UNITED STATES
SECURITIES AND EXCHANGE COMMISSION

WASHINGTON, DC 20549

FORM 10-Q

(Mark One)

☒ **QUARTERLY REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the quarterly period ended March 31, 2021

OR

☐ **TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the transition period from _____ to _____

Commission File Number: 001-38276

APELLIS PHARMACEUTICALS, INC.

(Exact Name of Registrant as Specified in its Charter)

Delaware	**27-1537290**
(State or other jurisdiction of incorporation or organization)	**(I.R.S. Employer Identification No.)**
100 Fifth Avenue, Waltham, MA	**02451**
(Address of principal executive offices)	**(Zip Code)**

Registrant's telephone number, including area code: (617) 977-5700

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock, $0.0001 par value per share	APLS	Nasdaq Global Select Market

Indicate by check mark whether the registrant (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. Yes ☒ No ☐

Indicate by check mark whether the registrant has submitted electronically every Interactive Data File required to be submitted pursuant to Rule 405 of Regulation S-T (§ 232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit such files). Yes ☒ No ☐

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, a smaller reporting company, or an emerging growth company. See the definitions of "large accelerated filer," "accelerated filer," "smaller reporting company," and "emerging growth company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer	☒	Accelerated filer	☐
Non-accelerated filer	☐	Small reporting company	☐
		Emerging growth company	☐

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act). Yes ☐ No ☒

As of April 23, 2021, the registrant had 80,496,771 shares of common stock, $0.0001 par value per share, outstanding.

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PART I—FINANCIAL INFORMATION

Item 1. Financial Statements.

APELLIS PHARMACEUTICALS, INC.
CONDENSED CONSOLIDATED BALANCE SHEETS
(Amounts in thousands, except per share amounts)

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	March 31, 2021	December 31, 2020
Assets	**(Unaudited)**	
Current assets:		
Cash and cash equivalents	$ 265,435	$ 565,779
Marketable securities	458,237	311,869
Prepaid assets	17,203	11,400
Restricted cash	1,552	1,266
Other current assets	31,198	26,878
Total current assets	773,625	917,192
Non-current Assets:		
Right-of-use assets	22,518	17,719
Property and equipment, net	7,077	6,803
Other assets	6,909	18,855
Total assets	$ 810,129	$ 960,569
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 4,158	$ 8,477
Accrued expenses	69,398	111,935
Current portion of development derivative liability	6,212	4,230
Current portion of right of use liabilities	3,902	3,685
Total current liabilities	83,670	128,327
Long-term liabilities:		
Convertible senior notes	386,152	358,830
Development derivative liability	268,740	253,638
Operating lease liabilities	19,909	15,217
Total liabilities	758,471	756,012
Commitments and contingencies (Note 13)	—	—
Stockholders' equity:		
Preferred stock, $0.0001 par value; 10,000 shares authorized, and zero shares issued and outstanding at March 31, 2021 and December 31, 2020	—	—
Common stock, $0.0001 par value; 200,000 shares authorized at March 31, 2021 and December 31, 2020; 80,438 shares issued and outstanding at March 31, 2021, and 76,130 shares issued and outstanding at December 31, 2020	8	8
Additional paid-in capital	1,147,263	1,131,013
Accumulated other comprehensive loss	(1,620)	(117)
Accumulated deficit	(1,093,993)	(926,347)
Total stockholders' equity	51,658	204,557
Total liabilities and stockholders' equity	$ 810,129	$ 960,569

See accompanying notes to unaudited condensed consolidated financial statements

APELLIS PHARMACEUTICALS, INC.
CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE INCOME/(LOSS)
(Unaudited)
(Amounts in thousands, except per share amounts)

	For the Three Months Ended March 31,	
	2021	2020
Operating expenses:		
Research and development	$ 84,012	$ 69,282
General and administrative	40,579	29,504
Net operating loss	(124,591)	(98,786)
Loss on conversion of debt	(39,487)	—
Loss from remeasurement of development derivative liability	(17,084)	(68,406)
Interest income	134	2,275
Interest expense	(4,175)	(3,919)
Other income, net	1,544	14
Net loss	(183,659)	(168,822)
Other comprehensive loss:		
Unrealized gain on marketable securities	79	1,394
Foreign currency loss	(1,582)	(230)
Total other comprehensive gain/ loss	(1,503)	1,164
Comprehensive loss, net of tax	$ (185,162)	$ (167,658)
Net loss per common share, basic and diluted	$ (2.32)	$ (2.29)
Weighted-average number of common shares used in net loss per common share, basic and diluted	79,219	73,720

See accompanying notes to unaudited condensed consolidated financial statements

APELLIS PHARMACEUTICALS, INC.
CONDENSED CONSOLIDATED STATEMENTS OF CHANGES IN STOCKHOLDERS' EQUITY
(Unaudited)
(Amounts in thousands)

	Common Stock		Additional Paid-In Capital	Accumulated Other Comprehensive Income/(Loss)	Accumulated Deficit	Total Stockholders' Equity
	Outstanding Shares	Amount				
Balance at January 1, 2021	76,130	$ 8	$ 1,131,013	$ (117)	$ (926,347)	$ 204,557
Impact of adoption of ASU 2020-06	—	—	(165,747)	—	16,013	(149,734)
Issuance of shares in exchange of 2019 Convertible Notes, including issuance costs	3,976	—	162,258	—	—	162,258
Forfeiture of accrued interest in exchange of 2019 Convertible Notes	—	—	1,668	—	—	1,668
Issuance of common stock upon exercise of stock options	285	—	2,588	—	—	2,588
Vesting of restricted stock units, net of shares withheld for taxes	47	—	(956)	—	—	(956)
Share-based compensation expense	—	—	16,439	—	—	16,439
Unrealized gain on available-for-sale investments	—	—	—	79	—	79
Net loss	—	—	—	—	(183,659)	(183,659)
Foreign currency loss	—	—	—	(1,582)	—	(1,582)
Balance at March 31, 2021	80,438	$ 8	$ 1,147,263	$ (1,620)	$ (1,093,993)	$ 51,658

	Common Stock		Additional Paid-In Capital	Accumulated Other Comprehensive Loss	Accumulated Deficit	Total Stockholders' Equity
	Outstanding Shares	Amount				
Balance at January 1, 2020	63,938	$ 6	$ 615,850	$ (154)	$ (581,473)	$ 34,229
Issuance of common stock in follow-on offering, net of offering costs	10,925	1	381,457	—	—	381,458
Issuance of common stock upon exercise of stock options	559	—	1,674	—	—	1,674
Share-based compensation expense	—	—	9,294	—	—	9,294
Unrealized gain on available-for-sale investments	—	—	—	1,394	—	1,394
Net loss	—	—	—	—	(168,822)	(168,822)
Foreign currency loss	—	—	—	(230)	—	(230)
Balance at March 31, 2020	75,422	$ 7	$ 1,008,275	$ 1,010	$ (750,295)	$ 258,997

See accompanying notes to unaudited condensed consolidated financial statements

APELLIS PHARMACEUTICALS, INC.
CONDENSED CONSOLIDATED STATEMENTS OF CASH FLOWS
(Unaudited)
(Amounts in thousands)

	For the Three Months Ended March 31,	
	2021	2020
Operating Activities		
Net loss	$ (183,659)	$ (168,822)
Adjustments to reconcile net loss to net cash used in operating activities:		
Share-based compensation expense	16,439	9,294
Loss on conversion of debt	39,487	—
Loss from remeasurement of development derivative liability	17,084	68,406
Forfeiture of accrued interest in exchange of convertible notes	1,668	—
Amortization of right-of-use assets	112	57
Depreciation expense	282	85
Amortization of debt discounts	360	—
Amortization of discounts for convertible notes, net of financing costs	—	1,994
Changes in operating assets and liabilities:		
Prepaid assets	(5,819)	(3,004)
Other current assets	(4,242)	(671)
Other assets	11,946	(460)
Accounts payable	(4,170)	(5,237)
Accrued expenses	(42,802)	(9,991)
Net cash used in operating activities	(153,314)	(108,349)
Investing Activities		
Purchase of property and equipment	(484)	(435)
Purchase of available-for-sale securities	(171,281)	(227,419)
Proceeds from maturity of available-for-sale securities	25,000	—
Net cash used in investing activities	(146,765)	(227,854)
Financing Activities		
Proceeds from issuance of common stock, net of issuance costs	—	381,593
Proceeds from development derivative liability	—	20,000
Proceeds from exercise of stock options	2,588	1,674
Payments of employee tax withholding related to equity-based compensation	(956)	—
Net cash provided by financing activities	1,632	403,267
Effect of exchange rate changes on cash, cash equivalents and restricted cash	(1,611)	(152)
Net increase in cash, cash equivalents and restricted cash	(300,058)	66,912
Cash, cash equivalents and restricted cash at beginning of period	567,045	351,985
Cash, cash equivalents and restricted cash at end of period	$ 266,987	$ 418,897
Reconciliation of cash, cash equivalents and restricted cash to the consolidated balance sheets:		
Cash and cash equivalents	$ 265,435	$ 417,881
Restricted cash	1,552	1,016
Total cash, cash equivalents, and restricted cash	$ 266,987	$ 418,897
Supplemental Disclosure of Financing Activities		
Cash paid for Interest	$ 6,893	$ 3,829
2019 Convertible Notes exchanged for common stock	$ 126,129	$ —

See accompanying notes to unaudited condensed consolidated financial statements

1. Nature of Organization and Operations

Apellis Pharmaceuticals, Inc. (the "Company") is a clinical-stage biopharmaceutical company focused on the development of novel therapeutic compounds to treat disease through the inhibition of the complement system, which is an integral component of the immune system, at the level of C3, the central protein in the complement cascade.

The Company was incorporated in September 2009 under the laws of the State of Delaware and has its principal office in Waltham, Massachusetts.

The Company's operations since inception have been limited to organizing and staffing the Company, acquiring rights to product candidates, business planning, raising capital and developing its product candidates.

The Company is subject to risks common in the biotechnology industry including, but not limited to, raising additional capital, development by its competitors of new technological innovations, its ability to successfully complete preclinical and clinical development of product candidates and receive timely regulatory approval of products, market acceptance of the Company's products, protection of proprietary technology, healthcare cost containment initiatives, and compliance with governmental regulations, including those of the U.S. Food and Drug Administration ("FDA"). Additionally, the Company is subject to risks arising from the Coronavirus Disease 2019 (COVID-19) pandemic, which could have adverse effects upon its business and operations, including on its ability to initiate, conduct and complete clinical trials, delay the initiation of planned and future clinical trials and could disrupt regulatory activities.

Adoption of ASU 2020-06 Debt – Debt with Conversion and Other Options (Subtopic 470-20)

Effective January 1, 2021, the Company early adopted ASU 2020-06, *Debt – Debt with Conversion and Other Options (Subtopic 470-20)* which reduces complexity in applying GAAP to certain financial instruments with characteristics of liability and equity. The ASU removes the guidance that requires entities to account for beneficial conversion features and cash conversion features in equity, separately from the host convertible debt or preferred stock. The ASU further revises the guidance to require entities to calculate diluted earnings per share for convertible instruments by using the if-converted method. In addition, entities must presume share settlement for purposes of calculating diluted earnings per share when an instrument may be settled in cash or shares. The impact of the adoption of the statement increased net debt outstanding and decreased net equity by $149.7 million as of January 1, 2021. Of the $149.7 million decrease to net equity, $16.0 million was recorded to retained earnings. See Note 5, Long Term Debt for additional information.

Convertible Notes Exchange

In January 2021, the Company entered into separate, privately negotiated exchange agreements to modify the conversion terms with certain holders of its convertible notes issued in September 2019 (the "2019 Convertible Notes"). Under the terms of these exchange agreements, the holders exchanged approximately $126.1 million in aggregate principal amount of 2019 Convertible Notes held by them for an aggregate of 3,906,869 shares of common stock issued by the Company. The Company also issued 69,491 shares for settlement of issuance costs paid to the Company's advisor. The Company recognized a total loss on conversion of debt in the unaudited condensed consolidated statement of operations of $39.5 million. As of March 31, 2021, the Company held as treasury Convertible Notes the $126.1 million principal amount of exchanged notes and such notes have not been cancelled. See Note 5, Long Term Debt for additional information.

Liquidity and Going Concern

The accompanying unaudited condensed consolidated financial statements have been prepared on the basis of the realization of assets and the satisfaction of liabilities and commitments in the normal course of business. As of April 28, 2021, the date of issuance of these unaudited condensed consolidated financial statements, the Company believes that its cash and cash equivalents of $265.4 million and marketable securities of $458.2 million as of March 31, 2021 will be sufficient to fund its operations and capital expenditure requirements for at least the next twelve months from the date of issuance of these unaudited condensed consolidated

financial statements. The future viability of the Company beyond that point is dependent on its ability to raise additional capital to finance its operations.

The Company is subject to risks common to other life science companies in the development stage including, but not limited to, uncertainty of product development and commercialization, lack of marketing and sales history, development by its competitors of new technological innovations, dependence on key personnel, market acceptance of products, product liability, protection of proprietary technology, ability to raise additional financing, and compliance with FDA and other government regulations. If the Company does not successfully commercialize any of its product candidates, it will be unable to generate recurring product revenue or achieve profitability. The Company's plans to meet its short-term and longer-term operating cash flow requirements include obtaining additional funding.

There are uncertainties associated with the Company's ability to (1) obtain additional debt or equity financing on terms that are favorable to the Company (2) enter into collaborative agreements with strategic partners to obtain funding, and (3) succeed in its future operations. If the Company is not able to obtain the required funding for its operations, or is not able to obtain funding on a timely basis on terms that are favorable to the Company, it could be forced to delay, reduce or eliminate its research and development programs or future commercialization efforts and its business could be materially harmed.

2. Basis of Presentation

The accompanying unaudited condensed consolidated financial statements include the accounts of the Company and its wholly owned subsidiaries, Apellis Australia Pty Ltd, Apellis Bermuda Limited, Apellis Germany GmbH, Apellis Ireland Ltd, Apellis Netherlands B.V., Apellis Switzerland GmbH, Apellis UK Limited, APL DEL Holdings LLC, APL Sales Corp I, LLC, APL PRG I, Corp. and Apellis MA Securities Corp. All intercompany balances and transactions have been eliminated in consolidation. The accompanying unaudited condensed consolidated financial statements have been prepared in accordance with U.S. generally accepted accounting principles ("U.S. GAAP") and following the requirements of the Securities and Exchange Commission (the "SEC"), for interim reporting. As permitted under those rules, certain footnotes or other financial information that are normally required by U.S. GAAP have been condensed or omitted and, accordingly, the consolidated balance sheet as of December 31, 2020 has been derived from audited consolidated financial statements at that date but does not include all of the information required by U.S. GAAP for complete financial statements. These financial statements have been prepared on the same basis as the Company's annual financial statements and, in the opinion of management, reflect all adjustments (consisting only of normal recurring adjustments) that are necessary for a fair presentation of the Company's financial information. The results of operations for the three months ended March 31, 2021 are not necessarily indicative of the results to be expected for the year ending December 31, 2021 or for any other interim period or for any other future year.

The accompanying unaudited condensed consolidated financial statements and related financial information should be read in conjunction with the audited consolidated financial statements and the related notes thereto for the year ended December 31, 2020 included in the Company's Annual Report on Form 10-K filed with the SEC on February 25, 2021.

Licensing and Collaboration Revenue

The Company analyzes license and collaboration arrangements pursuant to FASB ASC Topic 808, *Collaborative Arrangement Guidance and Considerations*, ("ASC 808") to assess whether such arrangements, or transactions between arrangement participants, involve joint operating activities performed by parties that are both active participants in the activities and exposed to significant risks and rewards dependent on the commercial success of such activities or are more akin to a vendor-customer relationship. In making this evaluation, the Company considers whether the activities of the collaboration are considered to be distinct and deemed to be within the scope of the collaborative arrangement guidance or if they are more reflective of a vendor-customer relationship and, therefore, within the scope of FASB ASC Topic 606, *Revenue from Contracts with Customer, (*"ASC 606"). This assessment is performed throughout the life of the arrangement based on changes in the responsibilities of all parties in the arrangement.

For elements of collaboration arrangements that are not accounted for pursuant to guidance in ASC 606, an appropriate recognition method is determined and applied consistently, generally by analogy to the revenue from contracts with customers guidance. Amounts related to transactions with a counterparty in a collaborative arrangement that is not a customer are presented as collaboration revenue and in a separate line item from revenue recognized from contracts with customers, if any, in the Company's consolidated statements of operations.

Pursuant to ASC 606, for arrangements or transactions between arrangement participants determined to be within the scope of the contracts with customers guidance, the Company performs the following steps to determine the appropriate amount of revenue to

be recognized as the Company fulfills its obligations: (i) identification of the promised goods or services in the contract; (ii) determination of whether the promised goods or services are performance obligations including whether they are distinct in the context of the contract; (iii) measurement of the transaction price, including the constraint on variable consideration; (iv) allocation of the transaction price to the performance obligations based on estimated selling prices; and (v) recognition of revenue when (or as) the Company satisfies each performance obligation.

The Company evaluates the performance obligations promised in the contract that are based on goods and services that will be transferred to the customer and determine whether those obligations are both (i) capable of being distinct and (ii) distinct in the context of the contract. Goods or services that meet these criteria are considered distinct performance obligations. The Company estimates the transaction price based on the amount expected to be received for transferring the promised goods or services in the contract. The consideration may include fixed consideration or variable consideration. At the inception of each arrangement that includes variable consideration, the Company evaluates the amount of potential transaction price and the likelihood that the transaction price will be received. The Company utilizes either the most likely amount method or expected value method to estimate the amount expected to be received based on which method best predicts the amount expected to be received. The amount of variable consideration that is included in the transaction price may be constrained and is included in the transaction price only to the extent that it is probable that a significant reversal in the amount of the cumulative revenue recognized will not occur in a future period. Arrangements that include rights to additional goods or services that are exercisable at a customer's discretion are generally considered options. The Company assesses if these options provide a material right to the customer and, if so, these options are considered performance obligations. The Company has not currently identified any such material rights.

Revenue is recognized when, or as, the Company satisfies a performance obligation by transferring a promised good or service to a customer. An asset is transferred when, or as, the customer obtains control of that asset. For performance obligations that are satisfied over time, the Company recognizes revenue using an input or output measure of progress that best depicts the satisfaction of the relevant performance obligation.

After contract inception, the transaction price is reassessed at every period end and updated for changes such as resolution of uncertain events. Any change in the overall transaction price is allocated to the performance obligations on the same methodology as at contract inception.

See Note 11, License and Collaboration Agreements, for further discussion related to the Collaboration and License Agreement with Swedish Orphan Biovitrum AB (Publ) ("Sobi").

Fair Value of Financial Instruments

The Company is required to disclose information on the fair value of financial instruments and inputs that enable an assessment of the fair value. The three levels of the fair value hierarchy prioritize valuation inputs based upon the observable nature of those inputs as follows:

Level 1 – Quoted prices in active markets for identical assets or liabilities;

Level 2 – Inputs other than quoted prices included within level 1 that are observable for the asset or liability, either directly or indirectly;

Level 3 – Unobservable inputs that reflect the Company's own assumptions about the assumptions market participants would use in pricing the asset or liability.

The Company's financial instruments, in addition to those presented in Note 5, Long-term Debt, Note 7, Marketable Securities, and Note 9, Fair Value Measurements, include cash and cash equivalents, other receivables, accounts payable and accrued liabilities. Management believes that the carrying amounts of certain cash and cash equivalents, other receivables, accounts payable and accrued expenses approximate the fair value due to the short-term nature of those instruments.

Cash and Cash Equivalents

Cash and cash equivalents are defined as cash in banks and investment instruments having maturities of three months or less from their acquisition date. The carrying amounts reported in the condensed consolidated balance sheets for certain cash and cash equivalents are valued at cost, which approximates their fair value. See Note 9, Fair Value Measurements, for additional information.

Restricted Cash

The Company is contingently liable under unused letters of credit with a bank, related to the Company's customs import bond and facility lease agreements of $1.6 million and $1.3 million as of March 31, 2021 and December 31, 2020, respectively. The Company records as restricted cash the collateral used to secure these letters of credit.

Foreign Currency

The financial position and results of operations of the Company's Australian, Irish and German subsidiaries are measured using the foreign subsidiary's local currency. Revenues and expenses of the subsidiaries have been translated into U.S. dollars at average exchange rates prevailing during the respective periods. Assets and liabilities have been translated at the rates of exchange on the balance sheet date. The resulting translation gain and loss adjustments are recorded directly as a separate component of stockholders' equity. The financial position and results of operations of the Company's Swiss subsidiary are measured and reported in U.S. dollars and transactions are translated to U.S. dollars at the end of the period.

3. Prepaid Assets and Accrued Expenses

Prepaid assets include $14.1 million and $8.0 million of prepaid research and development costs as of March 31, 2021 and December 31, 2020, respectively.

Accrued expenses are as follows (in thousands):

	March 31, 2021	December 31, 2020
Accrued research and development	$ 32,586	$ 47,879
Accrued license fee	—	25,050
Accrued payroll liabilities	11,857	22,896
Other	24,955	16,110
Total	$ 69,398	$ 111,935

4. Development Derivative Liability

On February 28, 2019, the Company entered into a development funding agreement, (the "SFJ Agreement"), with SFJ Pharmaceuticals Group ("SFJ"), under which SFJ agreed to provide funding to the Company to support the development of pegcetacoplan for the treatment of patients with PNH. SFJ paid the Company $60.0 million following the signing of the agreement, and agreed to pay the Company up to an additional $60.0 million in the aggregate in three equal installments upon the achievement of specified development milestones with respect to the Company's Phase 3 program for pegcetacoplan in PNH and subject to the Company having cash resources at the time sufficient to fund at least 10 months of the Company's operations.

On June 7, 2019, the Company and SFJ amended the development funding agreement, (the "SFJ Amendment"). Under the SFJ Amendment, SFJ agreed to make an additional $20.0 million funding payment to the Company to support the development of pegcetacoplan for the treatment of patients with PNH.

As of March 31, 2021, the Company has received a total of $140.0 million from SFJ as the Company met milestones as identified in the agreement. The Company did not receive any funds under the SFJ agreement in 2021. In the three months ended March 31, 2020, the Company received $20.0 million, from SFJ.

Under the SFJ Agreement following regulatory approval by the FDA or EMA for the use of pegcetacoplan as a treatment for PNH the Company will be obligated to pay SFJ an initial payment of up to $5.0 million (or a total of $10.0 million if regulatory approval is granted by the FDA and the EMA) and then up to an additional $226.0 million in the aggregate (or up to $452.0 million if regulatory approval is granted by the FDA and the EMA) in six additional annual payments with the majority of the payments being made from the third anniversary to the sixth anniversary of regulatory approval. Additionally, the Company granted a security interest

in all of its assets, excluding intellectual property and license agreements to which it is a party. In connection with the grant of the security interest, the Company agreed to certain affirmative and negative covenants, including restrictions on its ability to pay dividends, incur additional debt or enter into licensing transactions with respect to its intellectual property, other than specified types of licenses.

The SFJ Agreement is presented as a derivative liability on the condensed consolidated balance sheet and is considered a level three derivative, and, as such, is recorded at fair value and remeasured each quarter. The change in fair value due to the remeasurement of the development derivative liability resulted in a $17.1 million loss and a $68.4 million loss for the three months ended March 31, 2021 and 2020, respectively, recorded on the unaudited condensed consolidated statement of operations. The development derivative liability has a remeasured fair value of $275.0 million on the consolidated balance sheet at March 31, 2021. At March 31, 2021, $6.2 million of the $275.0 million of the development derivative liability fair market value is included in current liabilities.

The following table presents a rollforward of the development derivative liability (in thousands):

	For the Three Months Ended March 31,	
	2021	2020
Balance at fair market value, January 1,	$ 257,868	$ 134,839
Amounts received under the SFJ Agreement and SFJ Amendment	—	20,000
Loss recorded in loss from remeasurement of development derivative liability	17,084	68,406
Balance at fair market value, March 31,	$ 274,952	$ 223,245

The derivative is valued using a scenario-based discounted cash flow method, whereby each scenario makes assumptions about the probability and timing of cash flows, and such cash flows are present valued using a risk-adjusted discount rate. The analysis is calibrated such that the value of the derivative as of the date of the SFJ Agreement was consistent with an arm's-length transaction. Key inputs to the level 3 fair value model include (i) the probability and timing of achieving stated development milestones to receive the next tranches of funding, (ii) the probability and timing of achieving FDA and EMA approval, (iii) SFJ's cost of borrowing (8.0%), and (iv) the Company's cost of borrowing (11.76%).

SFJ's implied cost of borrowing was 8.0% and the Company's implied cost of borrowing was 11.76% as of the reporting date. These implied costs of borrowing were determined assuming the SFJ Agreement was initially executed with arm's-length terms. If the SFJ Agreement was instead not determined to be an arm's-length transaction, then implied discount rates could differ.

5. Long-term Debt

Convertible Senior Notes

On September 16, 2019, the Company completed a private offering of the 2019 Convertible Notes with an aggregate principal amount of $220.0 million issued pursuant to an indenture (the "Indenture") with U.S. Bank National Association, as trustee.

The net proceeds from the sale of the 2019 Convertible Notes were approximately $212.9 million after deducting the initial purchasers' discounts and commissions of $6.6 million and offering expenses of $0.5 million by the Company. The Company used $28.4 million of the net proceeds from the sale of the 2019 Convertible Notes to pay the cost of the capped call transactions in September 2019 described below.

On May 12, 2020, the Company issued the 2020 Convertible Notes with an aggregate principal amount of $300.0 million. The net proceeds from the sale of the 2020 Convertible Notes were approximately $322.9 million after deducting the purchasers' discounts and commission of $5.7 million and offering expenses of $0.3 million. The Company used $43.1 million of the net proceeds from the sale to pay the cost of the additional capped call transactions in May 2020 described below.

The Convertible Notes are senior unsecured obligations of the Company and bear interest at a rate of 3.5% per year payable semiannually in arrears on March 15 and September 15 of each year, beginning on March 15, 2020. The Convertible Notes will mature on September 15, 2026, unless converted earlier, redeemed or repurchased in accordance with their terms.

The Convertible Notes are convertible into shares of the Company's common stock at an initial conversion rate of 25.3405 shares per $1,000 principal amount of Convertible Notes (equivalent to an initial conversion price of approximately $39.4625 per share of common stock). The conversion rate is subject to customary anti-dilution adjustments. In addition, following certain events that occur prior to the maturity date or if the Company deliver a notice of redemption, the Company will increase the conversion rate for a holder who elects to convert its Convertible Notes in connection with such corporate event or a notice of redemption, as the case may be, in certain circumstances as provided in the indenture.

Prior to March 15, 2026, the Convertible Notes are convertible only upon the occurrence of certain events. On or after March 15, 2026 until the close of business on the second scheduled trading day immediately preceding the maturity date of the Convertible Notes, holders may convert the Convertible Notes at any time. Upon conversion of the Convertible Notes, the Company will pay or deliver, as the case may be, cash, shares of the Company's common stock or a combination of cash and shares of common stock, at the Company's election.

Prior to September 20, 2023, the Company may not redeem the Convertible Notes. The Company may redeem for cash all or a portion of the Convertible Notes, at its option, on or after September 20, 2023 if the last reported sale price of the Company's common stock has been at least 130% of the conversion price then in effect for at least 20 trading days (whether or not consecutive), including the trading day immediately preceding the date on which the Company provides a notice of redemption, during any 30 consecutive trading day period ending on, and including, the trading day immediately preceding the date on which the Company provides notice of redemption. The redemption price will be equal to 100% of the principal amount of the Convertible Notes to be redeemed, plus accrued and unpaid interest to, but excluding, the redemption date.

If the Company undergoes a "fundamental change," as defined in the Indenture, prior to maturity, subject to certain conditions, holders may require the Company to repurchase for cash all or any portion of their Convertible Notes at a fundamental change repurchase price equal to 100% of the principal amount of the notes to be repurchased, plus any accrued and unpaid interest to, but excluding, the fundamental change repurchase date.

Prior to the adoption of ASU 2020-06 effective January 1, 2021, the Company used an effective interest rate of 10.5% to determine the liability component of the 2019 and 2020 Convertible Notes. This resulted in the recognition of $145.1 million and $204.5 million as the liability component of the 2019 and 2020 Convertible Notes, respectively and the recognition of the residual amount of $74.9 million and $95.5 million as the debt discount with a corresponding increase to additional paid in capital for the equity component of the 2019 and 2020 Convertible Notes, respectively. The 2020 Convertible Notes aggregate debt issuance costs of $6.0 million were allocated to the liability and equity components in the amounts of $3.7 and $2.3 million, respectively. The 2019 Convertible Notes aggregate debt issuance costs of $7.1 million were allocated to the liability and equity components in the amounts of $4.7 million and $2.4 million, respectively.

Effective January 1, 2021, the Company adopted ASU 2020-06 using the modified retrospective method. Upon adoption, the Company increased net debt and reduced net equity by $149.7 million. The $149.7 million consisted of several items. The first item is the reclassification from equity to debt of the residual amounts originally identified as the equity components of the 2019 and 2020 Convertible Notes of $74.9 million and $95.5 million, respectively. The equity component reclassification was offset by the adjustment to retained earnings for the reversal of previous non-cash interest expense recorded for the amortization of the equity components of $17.1 million. The second item is the reclassification from equity to debt of the debt issuance costs originally allocated to equity for the 2019 and 2020 Convertible Notes of $2.4 million and $2.3 million, respectively. The debt issuance costs reclassification was offset by the adjustment to retained earnings for previous amortization of the debt issuance costs recorded of $1.1 million.

In January 2021, the Company entered into separate, privately negotiated exchange agreements to modify the conversion terms with certain holders of its 2019 Convertible Notes. Under the terms of these exchange agreements, the holders exchanged approximately $126.1 million in aggregate principal amount of 2019 Convertible Notes held by them for an aggregate of 3,906,869 shares of common stock issued by the Company. In accordance with ASC topic 470-20, "*Debt*,– Debt with Conversion and Other Options," the Company accounted for the exchange as an induced conversion based on the short period of time the conversion offer was open and the substantive conversion feature offer. The Company accounted for the conversion of the debt as an inducement by expensing the fair value of the shares that were issued in excess of the original terms of the Convertible Notes. The Company reduced net debt outstanding and increased net equity on the consolidated balance sheet by $122.8 million, consisting of the par value of the 2019 Convertible Notes exchanged of $126.1 million less the $3.3 million of remaining debt issuance costs associated with the exchanged notes. The Company also increased shares outstanding by 3,906,869 shares consisting of 3,196,172 shares issued at the initial conversion rate in the Indenture of 25.3405 plus an additional 710,697 shares. Additionally, the Company issued 69,491 shares as settlement of debt issuance costs paid to the Company's advisor in connection with the conversion transaction. The Company recorded a loss on conversion of debt of $39.5 million comprised of $36.4 million related to the value of the shares issued in excess of the original conversion terms at the fair market value and $3.1 million for the value of the 69,491 shares issued in payment of issuance

costs. Upon exchange of the 2019 Convertible Notes, the holders forfeited accrued interest through the date of the exchange of $1.7 million, which the Company charged to interest expense and to equity. As of March 31, 2021, the Company held as treasury Convertible Notes the $126.1 million principal amounts of exchanged notes and such notes had not been cancelled.

Interest expense for the Convertible Notes was $4.2 million and $3.9 million for the three months ended March 31, 2021 and 2020, respectively. For the three months ended March 31, 2021, interest expense included accrued semi-annual coupon payable of $3.8 million and amortization of debt issuance costs of $0.4 million. For the three months ended March 31, 2020 interest expense included the amortization of the discount on the Convertible Notes of $1.9 million, an accrued semi-annual coupon payable of $1.9 million and amortization of debt issuance costs of $0.1 million. As of March 31, 2021, $7.7 million of debt issuance costs was recorded on the unaudited condensed consolidated balance sheet as a reduction to the carrying amount of the Convertible Notes.

The aggregate principal balance of the Convertible Notes, net of unamortized debt issuance costs, as of March 31, 2021 and December 31, 2020 was $386.2 million and $358.8 million respectively.

Capped Call Transactions

On September 11, 2019, and May 6, 2020 concurrently with the pricing of the 2019 Convertible Notes and the 2020 Convertible Notes, respectively, the Company entered into capped call transactions with two counterparties. The capped call transactions are expected generally to reduce the potential dilution to the Company's common stock upon any conversion of Convertible Notes and/or offset any cash payments the Company is required to make in excess of the principal amount of converted Convertible Notes, as the case may be, in the event that the market price per share of the Company's common stock, as measured under the terms of the capped call transactions, is greater than the strike price of the capped call transactions, which is initially $39.4625 (the conversion price of the Convertible Notes) and is subject to anti-dilution adjustments substantially similar to those applicable to the conversion rate of such Convertible Notes. If, however, the market price per share of the Company's common stock, as measured under the terms of the capped call transactions, exceeds the cap price of the capped call transactions, which is initially $63.14 per share, there would nevertheless be dilution and/or there would not be an offset of such potential cash payments, in each case, to the extent that such market price exceeds the cap price of the capped call transactions.

Pursuant to ASC 815-40 *Derivatives and Hedging*, the Company determined that the capped call transactions should be classified as equity instruments and the capped call premium paid in the amount of $28.4 million and $43.1 million were recorded as reductions to additional paid-in capital at March 31, 2021 for the 2019 and 2020 Convertible Notes, respectively.

6. Leases

On January 1, 2019, the Company adopted ASU 2016-02 *Leases (Topic 842)* using a modified retrospective method. The underlying assets of the Company's leases primarily relate to office space leases, but also include some equipment leases. The Company determines if an arrangement qualifies as a lease at its inception.

As a practical expedient permitted under Topic 842, the Company elected to account for the lease and non-lease components as a single lease component for all leases of which it is the lessee. Lease payments, which may include lease and non-lease components, are included in the measurement of the Company's lease liabilities to the extent that such payments are either fixed amounts or variable amounts that depend on a rate or index as stipulated in the lease contract. When the Company cannot readily determine the rate implicit in the lease, the Company determines its incremental borrowing rate by using the rate of interest that it would have to pay to borrow on a collateralized basis over a similar term, an amount equal to the lease payments in a similar economic environment.

The Company enters into lease agreements with terms generally ranging from 2-7 years. Some of the Company's lease agreements include Company options to extend the lease on a month-to-month basis or for set periods for up to five years. Many of these leases also include options to terminate the leases within one year or per other contractual terms. Renewal and termination options were generally not included in the lease term for the Company's existing operating leases.

As of March 31, 2021 and December 31, 2020, all leases were classified as operating lease assets and liabilities. Additional information related to the operating lease assets and liabilities is as follows (in thousands):

	March 31, 2021	December 31, 2020
Operating Lease Assets	$ 22,518	$ 17,719
Operating Lease Liabilities	$ 23,811	$ 18,902
Weighted Average Remaining Term in years	5.35	4.66
Weighted Average discount rate used to measure outstanding lease liabilities	7.76%	7.74%

For the three months ended March 31, 2021 and 2020, the total lease cost for operating lease expense was $1.3 million and $1.0 million, respectively.

Supplemental cash flow information related to operating leases for the three months ended March 31 is as follows (in thousands):

	2021	2020
Operating cash flows for operating leases	$ 1,588	$ 968
Operating lease assets obtained in exchange for lease obligations	$ 5,675	$ 334

The maturities of the Company's operating lease liabilities as of March 31, 2021 are as follows (in thousands):

2021	$ 4,204
2022	5,415
2023	5,480
2024	4,811
2025 and thereafter	9,267
Total future minimum lease payments less	29,177
Imputed interest	(5,366)
Total operating lease liabilities	$ 23,811

7. Marketable Securities

The amortized cost, gross unrealized holding losses and fair value of available-for-sale debt securities by type of security as of March 31, 2021 and December 31, 2020 were as follows (in thousands):

	As of March 31, 2021			
	Amortized Cost	Gross Unrealized Holding Gains	Gross Unrealized Holding Losses	Fair Value
U.S. Government-related obligations	$ 458,158	$ 79	$ —	$ 458,237

	As of December 31, 2020			
	Amortized Cost	Gross Unrealized Holding Gains	Gross Unrealized Holding Losses	Fair Value
U.S. Government-related obligations	$ 311,877	$ 11	$ (19)	$ 311,869

All available-for-sale securities mature in one year or less.

8. Other Comprehensive Income and Accumulated Other Comprehensive Income

The following tables summarize the changes in accumulated other comprehensive income/(loss), by component for the three months ended March 31, 2021 and 2020 (in thousands):

	Unrealized Gains (Losses) from Marketable Securities	Foreign Currency Translation Adjustment	Total Accumulated Other Comprehensive Income (Loss)
Balances, December 31, 2020	$ (8)	$ (109)	$ (117)
Net other comprehensive income (loss)	79	(1,582)	(1,503)
Balances, March 31, 2021	71	(1,691)	(1,620)

	Unrealized Gains (Losses) from Marketable Securities	Foreign Currency Translation Adjustment	Total Accumulated Other Comprehensive Income (Loss)
Balances, December 31, 2019	$ —	$ (154)	$ (154)
Net other comprehensive income (loss)	1,394	(230)	1,164
Balances, March 31, 2020	1,394	(384)	1,010

9. Fair Value Measurements

The Company is required to disclose information on the fair value of financial instruments and inputs that enable an assessment of the fair value. The three levels of the fair value hierarchy prioritize valuation inputs based upon the observable nature of those inputs as follows:

Level 1 – Quoted prices in active markets for identical assets or liabilities;

Level 2 – Inputs other than quoted prices included within level 1 that are observable for the asset or liability, either directly or indirectly;

Level 3 – Unobservable inputs that reflect the Company's own assumptions about the assumptions market participants would use in pricing the asset or liability.

The following table presents the fair value of financial instruments recorded originally at amortized cost or fair value and not remeasured on a recurring basis (in thousands):

		March 31, 2021			
Balance Sheet Classification	Type of Instrument	Level 1	Level 2	Level 3	Total
Financial Assets:					
Cash and cash equivalents:	Money market funds	$ 213,175	$ —	$ —	$ 213,175
	Bank certificates of deposit	43,582	—	—	43,582
Total Financial Assets		$ 256,757	$ —	$ —	$ 256,757

		December 31, 2020			
Balance Sheet Classification	Type of Instrument	Level 1	Level 2	Level 3	Total
Financial Assets:					
Cash and cash equivalents	Money market funds	$ 427,515	$ —	$ —	$ 427,515
	Bank certificates of deposit	43,577	—	—	43,577
Total Financial Assets		$ 471,092	$ —	$ —	$ 471,092

* The convertible notes were measured and recognized at fair value on the consolidated balance sheet at inception.

The Company's Convertible Notes are Level 1 category within the fair value level hierarchy at March 31, 2021 due to the adoption of ASU 2020-06. The fair value of the Convertible Notes was $547.3 million at March 31, 2021. At December 31, 2020, the Company's Convertible Notes were Level 2 category within the fair value level hierarchy. At December 31, 2020, the fair value of debt was determined using broker quotes in a non-active market for valuation. As of December 31, 2020, the debt component of the Company's Convertible Notes was $676.2 million. The Convertible Notes accrue a semi-annual coupon at an annual rate of 3.5%, which was included in accrued expenses in the consolidated balance sheets at March 31, 2021 and December 31, 2020.

The following table presents the fair value of financial instruments recorded at fair value at inception and remeasured on a recurring basis (in thousands):

		March 31, 2021			
Balance Sheet Classification	Type of Instrument	Level 1	Level 2	Level 3	Total
Financial Assets:					
Marketable securities:	US government obligations	$ 458,237	—	—	$ 458,237
Total Financial Assets		$ 458,237	$ —	$ —	$ 458,237
Financial Liabilities:					
Development derivative liability	Development derivative liability	$ —	$ —	$ 274,952	$ 274,952
Total Financial Liabilities		$ —	$ —	$ 274,952	$ 274,952

		December 31, 2020			
Balance Sheet Classification	Type of Instrument	Level 1	Level 2	Level 3	Total
Financial Assets:					
Cash and cash equivalents	US government obligations	$ 89,990	$ —	$ —	$ 89,990
Marketable securities:	US government obligations	311,869	—	—	311,869
Total Financial Assets		$ 401,859	$ —	$ —	$ 401,859
Financial Liabilities:					
Development derivative liability	Development derivative liability	$ —	$ —	$ 257,868	$ 257,868
Total Financial Liabilities		$ —	$ —	$ 257,868	$ 257,868

The fair value of the SFJ Agreement is presented as a development derivative liability based on level 3 inputs. The derivative is valued using a scenario-based discounted cash flow method, whereby each scenario makes assumptions about the probability and timing of cash flows, and such cash flows are present valued using a risk-adjusted discount rate. The analysis is calibrated such that the value of the derivative as of the date of the SFJ Agreement was consistent with an arm's-length transaction. Key inputs to the level 3 fair value model include (i) the probability and timing of achieving stated development milestones to receive the next tranches of funding, (ii) the probability and timing of achieving FDA and EMA approval, (iii) SFJ's cost of borrowing (8.0%), and (iv) the Company's cost of borrowing (11.76%).

SFJ's implied cost of borrowing was 8.0% and the Company's implied cost of borrowing was 11.76% as of the reporting date. These implied costs of borrowing were determined assuming the SFJ Agreement was initially executed with arm's-length terms.

10. Income Taxes

Deferred tax assets and deferred tax liabilities are determined based on temporary differences between the financial reporting and tax bases of assets and liabilities and are measured using the enacted tax rates and laws that will be in effect when the differences are expected to reverse. A valuation allowance is recorded against deferred tax assets if it is more likely than not that some portion or all of the deferred tax assets will not be realized. The Company has recorded a full valuation allowance against its deferred tax assets for the period ended on March 31, 2021.

The Company does not recognize a tax benefit for uncertain tax positions unless it is more likely than not that the position will be sustained upon examination by tax authorities, including resolution of any related appeals or litigation processes, based on the technical merits of the position. The tax benefit that is recorded for these positions is measured at the largest amount of cumulative benefit that has greater than a 50 percent likelihood of being realized upon ultimate settlement. Deferred tax assets that do not meet these recognition criteria are not recorded and the Company recognizes a liability for uncertain tax positions that may result in tax payments. If such unrecognized tax benefits were realized and not subject to valuation allowances, the entire amount would impact the tax provision. The Company has not recorded any amounts for unrecognized tax positions for the period ended on March 31, 2021.

For the three months ended on March 31, 2021 and 2020, the Company did not record any current or deferred income tax expense or benefit.

11. License and Collaboration Agreements

Sobi Transaction

On October 27, 2020, the Company and its subsidiaries Apellis Switzerland GmbH and APL DEL Holdings, LLC entered into a Collaboration and License Agreement with Sobi, concerning the development and commercialization of pegcetacoplan and specified other structurally and functionally similar compstatin analogues or derivatives for use systemically or for local non-ophthalmological administration (collectively referred to as the "Licensed Products").

Under the collaboration agreement, the Company granted Sobi an exclusive (subject to certain retained rights of the Company), sublicensable license of certain patent rights and know-how to develop and commercialize Licensed Products in all countries outside of the United States. The Company retains the right to commercialize Licensed Products in the United States, and, subject to specified limitations, to develop Licensed Products worldwide for commercialization in the United States.

Under the collaboration agreement, the Company and Sobi have agreed to collaborate to develop Licensed Products for the treatment of paroxysmal nocturnal hemoglobinuria, cold agglutinin disease, hematopoietic stem cell transplantation-associated thrombotic microangiopathy, C3 glomerulopathy and immune complex membranoproliferative glomerulonephritis, and amyotrophic lateral sclerosis, and any other indications subsequently agreed upon by the parties, for commercialization by or on behalf of the Company in the United States and by or on behalf of Sobi outside of the United States. If the parties do not agree to jointly pursue any development activities for the Licensed Products (whether for an Initial Indication or otherwise), the party proposing to pursue such activities may conduct such activities at its sole expense (with the non-proposing party having the right to obtain rights to the data generated by such development activities by paying a specified percentage of that expense), subject to agreed-upon exceptions that limit each party's unilateral development rights.

The initial development plan sets forth the initial development activities to be conducted by each of the Company and Sobi, with the Company bearing all costs incurred in conducting the activities set forth in such initial development plan, as well as certain specified additional costs that are not included in the initial development plan that may be incurred by the parties in developing Licensed Products for paroxysmal nocturnal hemoglobinuria in the European Union and the United Kingdom. The Company and Sobi will form several governance committees to oversee the development and manufacture, and to review and discuss the commercialization, of Licensed Products.

The Company shall supply Licensed Products to Sobi for development and for commercialization outside of the United States in accordance with a supply agreement to be negotiated by the parties. The collaboration agreement grants Sobi the right to perform or have performed drug product manufacturing of Licensed Products for development and for commercialization outside the United States and to manufacture or have manufactured drug substance under certain circumstances.

Sobi paid the Company an upfront payment of $250.0 million in November 2020 and has agreed to pay up to an aggregate of $915.0 million upon the achievement of specified one-time regulatory and commercial milestone events, and to reimburse the Company for up to $80.0 million in development costs. The Company will also be entitled to receive tiered, double-digit royalties (ranging from high teens to high twenties) on sales of Licensed Products outside of the United States, subject to customary deductions and third-party payment obligations, until the latest to occur of: (i) expiration of the last-to-expire of specified licensed patent rights; (ii) expiration of regulatory exclusivity; and (iii) ten (10) years after the first commercial sale of the applicable Licensed Product, in each case on a Licensed Product-by-Licensed Product and country-by-country basis. Under the collaboration agreement, the Company remains responsible for its license fee obligations (including royalty obligations) to the University of Pennsylvania as a licensor of the Company and for its payment obligations to SFJ Pharmaceuticals.

Accounting Analysis

The Company has determined that the agreement is within the scope of ASC 808 as a contractual arrangement that involves a joint operating activity whereby both parties are (i) active participants in the activity and (ii) exposed to certain significant risks and rewards dependent on the commercial success of the activity. ASC Topic 808 does not address measurement or recognition matters but allows for analogizing to ASC 606. Pursuant to ASC 606, the Company performed the following five steps: (i) identified the contract(s) with a customer; (ii) identified the performance obligations in the contract; (iii) determined the transaction price; (iv) allocated the transaction price to the performance obligations in the contract; and (v) recognized revenue when (or as) the entity satisfies a performance obligation.

The Company identified the following material distinct promises under the Sobi Agreement: (1) licenses to develop and commercialize pegcetacoplan or, Licenses to IP, and (2) performance of research and development services. The Company determined the promises to be distinct because Sobi can benefit from each of the license and the development services on their own or with readily available services. The Company could have provided the license without any development services and Sobi would have been

able to benefit from it by obtaining development services from another provider as the Licensed Products are at a more mature stage in their life cycle.

Under the agreement, Sobi agreed to pay the Company

i) a fixed amount of $250.0 million in an upfront payment in November 2020;
ii) a fixed amount of an additional $80.0 million in development reimbursements, payable yearly in four tranches in amounts determined based upon actual expenses incurred by the Company;
iii) up to an aggregate of $915.0 million upon the achievement of specified one-time regulatory and commercial milestone events; and
iv) tiered, double-digit royalties, ranging from high teens to high twenties, on sales of Licensed Products outside of the United States, subject to customary deductions and third-party payment obligations.

At contract inception, the $250.0 million non-refundable payment and the $80.0 million reimbursements were fixed proceeds. The Company evaluated whether Sobi is a customer for either of the distinct promises in the agreement. Under the Licenses to IP, the Company determined that Sobi is a customer as the know-how provided and the right granted by the Company to Sobi are outputs of the Company's business activities for which the Company will receive consideration. With respect to research and development activity, management determined that there is no vendor relationship as performing research and development activities for others is not a part of the Company's ongoing central operations. Based upon the evaluation of the relative fair values, the Company allocated the purchase price of $250.0 million and the related milestones and royalties to the license of IP and $80.0 million to performance of research and development activities.

The milestone and royalty payments are subject to activities outside the control of the Company. Per ASC 606, the Company considers this to be a customer/ vendor relationship, therefore, the Company will include the regulatory milestone payments in the total transaction price when it is probable that a significant reversal of revenue would not occur in a future period. The Company will recognize commercial milestone and royalty revenue at the later of (i) when the related sales occur or (ii) when the performance obligation to which the commercial milestone or royalty has been allocated has been satisfied. In case of commercial milestone or royalty payments, the Company will recognize revenue in the same period that the sales are completed for which the Company is contractually entitled to the milestone or percentage-based royalty payment. To date, the Company has not recognized any commercial milestone or royalty revenue resulting from any of our licensing arrangements. Management will periodically assess the elements of the contract and re-evaluate revenue recognition as necessary.

Pursuant to ASC 606, for the year ended December 31, 2020 the Company recognized the $250.0 million in revenue as this is the amount allocated to the license. The $80.0 million reimbursement for research and development activities does not constitute a customer/vendor relationship and thus is not in the scope of ASC 606. As ASC 808 does not include recognition guidance, the Company has established an accounting policy to recognize the payments under the reimbursement as a receivable on the balance sheet in an amount that is to be reimbursed based upon expense incurred by the Company, with a contra- research and development expense recognized in the statement of operations, over time as the expenses are incurred.

Under the Sobi collaboration agreement, for the three months ended March 31, 2021, the Company did not recognize licensing revenue. As of March 31, 2021, the Company recognized $8.1 million for contra-research and development expense in the unaudited condensed consolidated statement of operations related to the $80.0 million reimbursement from Sobi. As of December 31, 2020, the Company had recorded a $43.0 million receivable for contra-research and development reimbursement on the consolidated balance sheet, with $25.0 million and $18.0 million in current and long-term assets, respectively. The Company received a $25.0 million payment from Sobi in January 2021. As of March 31, 2021, the Company has a receivable of $26.1 million for contra-research and development reimbursement from Sobi recognized on the unaudited condensed consolidated balance sheet with $20.0 million recognized in current assets and $6.1 million recognized in other assets.

12. License Agreements

In connection with its purchase of assets from Potentia in September 2014, the Company became party to a license agreement with the Trustees of the University of Pennsylvania ("Penn") as a result of an agreement to purchase substantially all the assets of Potentia Pharmaceuticals, Inc, for an exclusive, worldwide license to specified patent rights. The Company is required to pay annual maintenance fees of $0.1 million until the first sale of a licensed product. The Company is also required to make milestone payments aggregating up to $3.2 million based upon the achievement of specified development and regulatory milestones and up to $5.0 million based upon the achievement of specified annual sales milestones with respect to each licensed product, and to pay low single-digit royalties based on net sales of each licensed product and with minimum quarterly royalty thresholds. In addition, the Company is obligated to pay a specified portion of income it receives from sublicensees.

In addition, the Company is also party to a license agreement with Penn for an exclusive, worldwide license to specified patent rights for the development and commercialization of products in fields of use, as defined therein. The Company is required to pay annual maintenance fees of $0.1 million until the first sale of a licensed product. The Company is required to make milestone payments aggregating up to $1.7 million, based upon the achievement of development and regulatory approval milestones, and up to $2.5 million, based upon the achievement of annual sales milestones with respect to each of the first two licensed products. The license agreement also requires the Company to pay low single digit royalties based on net sales of each licensed product, subject to minimum quarterly royalty thresholds. In addition, the Company is obligated to pay a specified portion of income it receives from sublicensees.

In January 2021, the Company paid $25.0 million for royalty expense owed to Penn related to the Sobi Agreement and another licensing transaction. As of December 31, 2020 the $25.0 million was recognized in accrued expenses on the consolidated balance sheet and recognized in license fees on the consolidated income statement.

In addition to the license agreement with Penn, the Company contracts to conduct research and development activities with third parties. Certain of these contracts commit the Company to pay future milestone payments up to $15.0 million or to pay royalty fees ranging from 3-6% if any of the research results in regulatory approval or commercial revenue for a product.

13. Commitments and Contingencies

The Company contracts to conduct research and development activities with third parties. The scope of the services under the research and development contracts can be modified and the contracts cancelled by the Company upon written notice. In some instances, the contracts may be cancelled by the third party upon written notice. If the Company were to cancel these contracts as of March 31, 2021, the Company would be required to pay certain termination costs and other fees of approximately $3.2 million that would be incurred in future periods.

The Company has certain non-cancellable purchase obligations related to the manufacturing of drug substance and drug product, primarily with Bachem Americas, Inc., and Bachem AG, collectively (“Bachem”) for the drug substance for the finished dosage form of pegcetacoplan and with NOF Corporation and NOF America Corporation, collectively, (“NOF”) for a component of pegcetacoplan. As of March 31, 2021, the Company has non-cancellable purchase commitments for 2021 with Bachem and NOF in the amount of approximately $16.4 million and $32.4 million, respectively. Subsequent to March 31, 2021, the Company became obligated for additional non-cancellable purchase commitments to Bachem and NOF for 2021 of approximately $2.9 million and $6.4 million, respectively.

Following regulatory approval by the FDA or EMA of pegcetacoplan for the treatment of PNH, the Company has certain payment and other obligations under the SFJ Agreement, which are discussed above in Note 4.

Indemnifications—In the ordinary course of business, the Company enters into agreements that may include indemnification provisions. Pursuant to such agreements, the Company may indemnify, hold harmless and defend indemnified parties for losses suffered or incurred by the indemnified party. Some of the provisions will limit losses to those arising from third-party actions. In some cases, the indemnification will continue after the termination of the agreement. The maximum potential amount of future payments the Company could be required to make under these provisions is not determinable. The Company has not incurred any cost to defend lawsuits or settle claims related to these indemnification provisions.

Legal—During the normal course of business, the Company may be a party to legal claims that may not be covered by insurance. Management does not believe that any such claims would have a material impact on the Company’s consolidated financial statements.

14. Net Loss per Share

Since the Company was in a loss position for all periods presented, basic net loss per common share is the same as diluted net loss per common share for all periods presented as the inclusion of all potential common shares outstanding would have been anti-dilutive. Convertible notes and shares outstanding presented below were excluded from the calculation of diluted net loss per share, prior to the use of the treasury stock method, as their effect is anti-dilutive (in thousands):

	For the Three Months Ended March 31,	
	2021	2020
Convertible notes	9,981	5,575
Common stock options	13,404	9,434
Restricted stock units	949	298
Total	24,334	15,307

Item 2. Management's Discussion and Analysis of Financial Condition and Results of Operations.

The following discussion and analysis of our financial condition and results of operations should be read in conjunction with our unaudited condensed financial statements and related notes appearing elsewhere in this Quarterly Report on Form 10-Q and our audited financial statements and related notes for the year ended December 31, 2020 included in our Annual Report on Form 10-K filed with the Securities and Exchange Commission on February 25, 2021, which we refer to as the 2020 Annual Report on Form 10-K.

This Quarterly Report on Form 10-Q contains forward-looking statements that involve substantial risks and uncertainties. All statements, other than statements of historical facts, contained in this Quarterly Report on Form 10-Q, including statements regarding our strategy, future operations, future financial position, future revenue, projected costs, prospects, plans and objectives of management and expected market growth are forward-looking statements. The words "anticipate," "believe," "continue," "could," "estimate," "expect," "intend," "may," "plan," "potential," "predict," "project," "should," "target," "would" and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words.

We may not actually achieve the plans, intentions or expectations disclosed in our forward-looking statements, and you should not place undue reliance on our forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in the forward-looking statements we make. The following information and any forward-looking statements should be considered in light of factors discussed elsewhere in this Quarterly Report on Form 10-Q particularly including those risks identified in Part II—Item 1A "Risk Factors" and our other filings with the SEC.

Overview

We are a clinical-stage biopharmaceutical company focused on the development of novel therapeutic compounds to treat disease through the inhibition of the complement system, which is an integral component of the immune system, at the level of C3, the central protein in the complement cascade. We believe that this approach can result in broad inhibition of the principal pathways of the complement system and has the potential to effectively control a broad array of complement-dependent autoimmune and inflammatory diseases.

We have the most advanced clinical programs targeting C3 with Phase 3 clinical trials of our lead product candidate, pegcetacoplan, in multiple indications. We believe that pegcetacoplan has the potential to be a best-in-class treatment that may address the limitations of existing treatment options or provide a treatment option where there currently is none. Pegcetacoplan has already shown activity that we believe is clinically meaningful in clinical trials for several distinct medical conditions, including geographic atrophy in age-related macular degeneration, or GA; paroxysmal nocturnal hemoglobinuria, or PNH; cold agglutinin disease, or CAD; and C3 glomerulopathy, or C3G. We are developing pegcetacoplan and other product candidates, including APL-9, targeting C3 through various routes of administration and plan to conduct clinical trials of these compounds in additional complement-dependent indications.

In October 2020, we entered into a collaboration and license agreement, or the collaboration agreement, with Swedish Orphan Biovitrum AB (Publ), or Sobi. Under the collaboration agreement, we agreed to co-develop pegcetacoplan for systemic indications, including PNH, CAD and hematopoietic stem cell transplantation-associated thrombotic microangiopathy, or HSCT-TMA, in hematology; C3G and immune complex membranoproliferative glomerulonephritis, or IC-MPGN in nephrology; and amyotrophic lateral sclerosis, or ALS in neurology. Sobi has exclusive ex-U.S. commercialization rights for systemic pegcetacoplan. We retain commercialization rights for systemic pegcetacoplan in the United States and worldwide commercial rights for ophthalmological pegcetacoplan, which includes our GA program in addition to worldwide commercialization rights for APL-9 and other novel compounds targeting C3.

GA. We initiated a Phase 3 clinical program consisting of two Phase 3 clinical trials evaluating pegcetacoplan in patients with GA in September 2018. We refer to these trials as the DERBY and OAKS trials. Both trials are fully enrolled and we expect to announce top-line data from these trials in the third quarter of 2021. In our Phase 2 clinical trial of pegcetacoplan in patients with GA, treatment with pegcetacoplan resulted in a significant reduction in the rate of GA lesion growth over 12 months. Additionally, data released in April 2021 from a post hoc analysis of seven patients in our Phase 1b trial of pegcetacoplan in patients with advanced GA and low vision demonstrated a trend in reduced lesion growth in eyes treated with pegcetacoplan versus untreated fellow eyes after 24 months of treatment.

Systemic Pegcetacoplan. We are developing pegcetacoplan for systemic administration in several indications, including PNH, C3G, IC-MPGN, ALS, CAD and HSCT-TMA.

PNH. In June 2018, we initiated a Phase 3 clinical trial evaluating pegcetacoplan in 80 patients with PNH who exhibited signs of moderate to severe anemia, specifically with an inclusion criterion of hemoglobin level of less than 10.5 g/dL, while being treated with eculizumab, an approved therapy for PNH that is marketed as Soliris. We refer to this trial as the PEGASUS trial.

In January 2020, we announced top-line data from the PEGASUS trial that showed that pegcetacoplan met the trial's primary efficacy endpoint, demonstrating superiority to eculizumab, with a statistically significant improvement in adjusted means of 3.8 g/dL of hemoglobin at week 16 ($p < 0.0001$), and promising results in key secondary endpoints. Additional data from the PEGASUS trial presented in June 2020 and December 2020 demonstrated increased hemoglobin levels, reduced transfusion requirements and improved key markers of hemolysis across the patient population, both in patients with high transfusion requirements and in patients with low or no transfusion requirements, which improvements were sustained through 48 weeks of treatment. In the PEGASUS trial, the safety profile of pegcetacoplan was comparable to that of eculizumab.

In September 2019, we initiated a second Phase 3 clinical trial in patients with PNH who have not been treated with eculizumab within three months before entering the trial. This trial is fully enrolled and we intend to present top-line data in the second quarter of 2021. We refer to this trial as the PRINCE trial.

We submitted an NDA to the FDA, and an MAA to the EMA, for pegcetacoplan for the treatment of PNH in September 2020. The FDA accepted the NDA and set the Prescription Drug User Fee Act, or PDUFA, target action date for May 14, 2021. The EMA validated the MAA in October 2020, with the potential for a European Commission decision on the MAA in the second half of 2021.

C3G/IC-MPGN. We have initiated and will continue to lead our registrational program in C3G / IC-MPGN. We initiated the Phase 2 NOBLE trial in up to 12 patients with post-kidney transplant recurrence of C3G or IC-MPGN in October 2020. We expect to dose the first patient in the NOBLE trial in the first half of 2021. We also plan to begin a Phase 3 clinical trial in patients with native kidney or post-transplant recurrence of C3G or IC-MPGN, having reduction of proteinuria as its primary endpoint, in the second half of 2021.

ALS. We have initiated a randomized, placebo-controlled Phase 2 clinical trial of pegcetacoplan in approximately 200 adults with sporadic ALS. We refer to this trial as the MERIDIAN trial. We treated the first patient in MERIDIAN in November 2020 and expect to complete enrollment in MERIDIAN in the second half of 2021.

CAD and HSCT-TMA. Sobi will lead development activities for a Phase 3 clinical trial in CAD and a Phase 2 clinical trial in HSCT-TMA, both planned to begin in 2021. In our Phase 2 clinical trial of pegcetacoplan in patients with CAD, patients achieved increased hemoglobin levels, reduced reticulocytes and bilirubin levels, and reduced LDH levels compared to baseline.

Pipeline. We are developing pegcetacoplan and other product candidates, including APL-9, targeting C3 through various routes of administration. We plan to conduct clinical trials of these compounds in additional complement-dependent indications. APL-9 is a C3 modulator designed to be intravenously administered for acute use. In May 2020 we initiated a Phase 1/2 randomized, placebo-controlled clinical trial for APL-9 in 66 patients with respiratory failure including acute respiratory distress syndrome (ARDS) secondary to COVID-19. In March 2021 we announced that we will not pursue additional development of APL-9 for the treatment of severe COVID-19 after an interim review of data from the Phase 1/2 trial by an independent data monitoring committee found no meaningful reduction in the overall mortality rate in patients treated with APL-9 in combination with standard of care therapy compared to the standard of care alone. No safety signals were observed by the data monitoring committee. We expect to report results from this trial in the second quarter of 2021. We are also developing APL-9 for the prevention of complement immune system activation coincident with adeno-associated virus, or AAV, vector administration for gene therapies and other indications. We plan to advance three new product candidates into clinical development by the end of 2022.

Since our commencement of operations in May 2010, we have devoted substantially all of our resources to developing our proprietary technology, developing product candidates, undertaking preclinical studies and conducting clinical trials for pegcetacoplan, building our intellectual property portfolio, organizing and staffing our company, business planning, raising capital, preparing for the commercial launch of our products and providing general and administrative support for these operations.

To date, we have financed our operations primarily through $772.1 million in net proceeds from public offerings of our common stock, including our initial public offering, or IPO, $535.8 million in net proceeds from the private offerings of Convertible Notes, a $250.0 million upfront payment and a $25.0 million as a development reimbursement payment from Sobi each pursuant to the Sobi collaboration agreement, $112.6 million in proceeds from the private placement of shares of our convertible preferred stock prior to our IPO, $140.0 million under the SFJ agreement, $20.0 million in proceeds from borrowings under a term loan facility with Silicon Valley Bank, and $7.0 million in proceeds from our issuance and sale of a promissory note. We have repaid the term loan facility and the promissory note in full, and we exchanged $126.1 million of aggregate principal amount of 2019 Convertible Notes for shares of our common stock in January 2021.

We have not generated any revenue from product sales. We have incurred significant annual net operating losses in each year since our inception and expect to continue to incur net operating losses for the foreseeable future. Our net losses were $183.7 million and $168.8 million for the three months ended March 31, 2021 and March 31, 2020, respectively. As of March 31, 2021, we had an accumulated deficit of $1.1 billion. We expect to continue to incur significant expenses and increasing operating losses for the next several years. Our net losses may fluctuate significantly from quarter to quarter and year to year. We anticipate that our expenses will increase significantly if and as we continue to develop and conduct our ongoing and planned clinical trials of pegcetacoplan and APL-9; initiate and continue research and preclinical and clinical development efforts for any future product candidates; seek to identify and develop additional product candidates for complement-dependent diseases; seek regulatory and marketing approvals for our product candidates that successfully complete clinical trials, if any; establish sales, marketing, distribution and other commercial infrastructure to commercialize any products for which we may obtain marketing approval; require the manufacture of larger quantities of product candidates for clinical development and, potentially, commercialization; maintain, expand and protect our intellectual property portfolio; hire and retain additional personnel, such as clinical, quality control and scientific personnel; add operational, financial and management information systems and personnel, including personnel to support our product development and help us comply with our obligations as a public company; and add equipment and physical infrastructure to support our research and development programs.

As of March 31, 2021 we had cash, cash equivalents and marketable securities of $723.7 million. We believe that our existing cash, cash equivalents and marketable securities as of March 31, 2021, along with the committed development reimbursement payments from Sobi, will enable us to fund our operating and capital expenditure requirements at least into the second half of 2022. We have based this estimate on assumptions that may prove to be wrong, and we could exhaust our available capital resources sooner than we expect. See “Liquidity and Capital Resources.”

We temporarily closed our facilities in March 2020 in respect to the COVID-19 pandemic. We have since reopened our facilities on a limited basis, subject to compliance with strict safety guidelines, but most of our employees continue to work remotely. We do not believe that the COVID-19 pandemic has had a significant impact upon our operations, including our ongoing clinical trials and the manufacture and supply of our product candidates.

SFJ Agreement

On February 28, 2019, we entered into a development funding agreement, which we refer to as the SFJ agreement, with SFJ Pharmaceuticals Group, or SFJ, under which SFJ agreed to provide funding to us to support the development of pegcetacoplan for the treatment of patients with PNH. Pursuant to the agreement, SFJ paid us $60.0 million following the signing of the agreement and agreed to pay us up to an additional $60.0 million in the aggregate in three equal installments upon the achievement of specified development milestones with respect to our Phase 3 program for pegcetacoplan in PNH and subject to our having cash resources at the time sufficient to fund at least 10 months of our operations.

On June 7, 2019, we amended the SFJ agreement, which we refer to as the SFJ amendment. Under the SFJ amendment, SFJ agreed to make an additional $20.0 million funding payment to us to support the development of pegcetacoplan for the treatment of patients with PNH.

On June 27, 2019, we received $40.0 million from SFJ, consisting of $20.0 million as the first installment of the additional $60.0 million upon the achievement of a milestone and the $20.0 million payable under the SFJ amendment.

In September 2019, we received $20.0 million from SFJ, as the second installment of the additional $60.0 million due to the achievement of a milestone and in January 2020 received the remaining $20.0 million installment of the additional $60.0 million upon the announcement of the results of the PEGASUS phase 3 trial.

Under the SFJ Agreement, following regulatory approval by the FDA or EMA under the SFJ Agreement for the use of pegcetacoplan as a treatment for PNH, we will be obligated to pay SFJ an initial payment of up to $5.0 million (or a total of $10.0 million if regulatory approval is granted by the FDA and the EMA) and then up to an additional $226.0 million in the aggregate (or up to $452.0 million if regulatory approval is granted by the FDA and the EMA) in six additional annual payments with the majority of the payments being made from the third anniversary to the sixth anniversary of regulatory approval.

Convertible Notes

In September 2019, we issued and sold $220.0 million aggregate principal amount of 3.5% convertible senior notes due 2026, or the 2019 Convertible Notes, in a private offering. The net proceeds from the sale of the 2019 Convertible Notes were approximately $212.9 million after deducting the initial purchasers’ discounts and commissions and estimated offering expenses payable. We used $28.4 million of the net proceeds from the offering to pay the cost of the capped call transactions in September 2019 described below.

In May 2020, we issued and sold an additional $300.0 million aggregate principal amount of 3.5% convertible senior notes due 2026, or the 2020 Convertible Notes, in a private offering. The aggregate purchase price of the 2020 Convertible Notes was $328.9 million, which amount included accrued interest from March 15, 2020 to, but not including, May 12, 2020. The net proceeds from the sale of the 2020 Convertible Notes were approximately $322.9 million after deducting the initial purchasers' discounts and commissions and offering expenses payable by us. We used $43.1 million of the net proceeds from the offering to pay the cost of the capped call transactions in May 2020 described below. The 2020 Convertible Notes were issued as additional notes under the Indenture and form a single series with, and have the same terms as, the 2019 Convertible Notes, but have a different issue date, issue price, CUSIP number and different restrictions on transfer. We refer to the 2019 Convertible Notes and the 2020 Convertible Notes together as the Convertible Notes.

The Convertible Notes are convertible into shares of our common stock at an initial conversion rate of 25.3405 shares per $1,000 principal amount of notes (equivalent to an initial conversion price of approximately $39.4625 per share of common stock). The conversion rate is subject to customary anti-dilution adjustments. In addition, following certain events that occur prior to the maturity date or if we deliver a notice of redemption, we will increase the conversion rate for a holder who elects to convert its Convertible Notes in connection with such corporate event or a notice of redemption, as the case may be, in certain circumstances as provided in the indenture governing the Convertible Notes, or the Indenture.

Prior to March 15, 2026, the Convertible Notes are convertible only upon the occurrence of certain events. On or after March 15, 2026 until the close of business on the second scheduled trading day immediately preceding the maturity date of the Convertible Notes, holders may convert the Convertible Notes at any time. Upon conversion of the Convertible Notes, we will pay or deliver, as the case may be, cash, shares of our common stock or a combination of cash and shares of common stock, at our election.

Prior to September 20, 2023, we may not redeem the Convertible Notes. We may redeem for cash all or a portion of the Convertible Notes, at our option, on or after September 20, 2023 if the last reported sale price of our common stock has been at least 130% of the conversion price then in effect for at least 20 trading days (whether or not consecutive), including the trading day immediately preceding the date on which we provide a notice of redemption, during any 30 consecutive trading day period ending on, and including, the trading day immediately preceding the date on which we provide notice of redemption. The redemption price will be equal to 100% of the principal amount of the Convertible Notes to be redeemed, plus accrued and unpaid interest to, but excluding, the redemption date.

If we undergo a "fundamental change," as defined in the Indenture, prior to maturity, subject to certain conditions, holders may require us to repurchase for cash all or any portion of their Convertible Notes at a fundamental change repurchase price equal to 100% of the principal amount of the Convertible Notes to be repurchased, plus any accrued and unpaid interest to, but excluding, the fundamental change repurchase date.

The Indenture contains customary terms and covenants, including that upon certain events of default occurring and continuing, either the Trustee or the holders of at least 25% in principal amount of the outstanding Convertible Notes may declare 100% of the principal of, and accrued and unpaid interest, if any, on, all the Convertible Notes to be due and payable.

On January 6, 2021, we entered into separate, privately negotiated exchange agreements with certain holders of our 2019 Convertible Notes. Under the terms of these exchange agreements, the holders exchanged approximately $126.1 million in aggregate principal amount of 2019 Convertible Notes held by them for an aggregate of 3,906,869 shares of our common stock. The exchange transactions closed in January 2021.

Capped Call Transactions

In September 2019 and May 2020, concurrently with the pricing of the 2019 Convertible Notes and 2020 Convertible Notes, respectively, we entered into capped call transactions with two counterparties. The capped call transactions are expected generally to reduce the potential dilution to our common stock upon any conversion of Convertible Notes and/or offset any cash payments we are required to make in excess of the principal amount of converted Convertible Notes, as the case may be, in the event that the market price per share of our common stock, as measured under the terms of the capped call transactions, is greater than the strike price of the capped call transactions, which is initially $39.4625, the conversion price of the Convertible Notes, and is subject to anti-dilution adjustments substantially similar to those applicable to the conversion rate of such Convertible Notes. If, however, the market price per share of our common stock, as measured under the terms of the capped call transactions, exceeds the cap price of the capped call transactions, there would nevertheless be dilution and/or there would not be an offset of such potential cash payments, in each case, to the extent that such market price exceeds the cap price of the capped call transactions.

Collaboration Agreement with Sobi

On October 27, 2020, we entered into the collaboration agreement with Sobi, concerning the development and commercialization of pegcetacoplan and specified other structurally and functionally similar compstatin analogues or derivatives for use systemically or for local non-ophthalmological administration, collectively referred to as the licensed products. See "Business—Collaboration and License Agreement with Sobi" for a description of the key terms of our collaboration agreement with Sobi. We granted Sobi an exclusive (subject to certain rights retained by us), sublicensable license of certain patent rights and know-how to develop and commercialize licensed products in all countries outside of the United States. We retained the right to commercialize licensed products in the United States, and, subject to specified limitations, to develop licensed products worldwide for commercialization in the United States. Under the agreement, Sobi made an upfront payment of $250.0 million in November 2020, and agreed to pay up to an aggregate of $915.0 million upon the achievement of specified one-time regulatory and commercial milestone events, and to reimburse us for up to $80.0 million in development costs. In January 2021 we received a $25.0 million development reimbursement payment from Sobi and expect to receive the balance in installments over the next three years, subject to certain conditions. We are also entitled to receive tiered, double-digit royalties (ranging from high teens to high twenties) on sales of licensed products outside of the United States, subject to customary deductions and third-party payment obligations, until the latest to occur of: (i) expiration of the last-to-expire of specified licensed patent rights; (ii) expiration of regulatory exclusivity; and (iii) ten (10) years after the first commercial sale of the applicable licensed product, in each case on a licensed product-by-licensed product and country-by-country basis. We remain responsible for our license fee obligations (including royalty obligations) to the University of Pennsylvania and for our payment obligations to SFJ Pharmaceuticals.

Financial Operations Overview

Revenue

We have not generated any revenue from product sales. If we are able to obtain regulatory approval for pegcetacoplan for the treatment of PNH in the United States, we will expect to generate revenue from the sale of products within the next twelve months.

Licensing and Collaboration Revenue

We enter into licensing agreements from time to time in which we receive upfront payments, milestone payments and royalties. In 2020 we entered into a collaboration agreement with Sobi for the development and commercialization of systemic pegcetacoplan, described below, and two license agreements with third parties to use APL-9 in certain research projects.

We analyze our license and collaboration arrangements pursuant to FASB ASC Topic 808, Collaborative Arrangement Guidance and Considerations to assess whether such arrangements, or transactions between arrangement participants, involve joint operating activities performed by parties that are both active participants in the activities and exposed to significant risks and rewards dependent on the commercial success of such activities or are more akin to a vendor-customer relationship. In making this evaluation, we consider whether the activities of the collaboration are considered to be distinct and deemed to be within the scope of the collaborative arrangement guidance and those that are more reflective of a vendor-customer relationship and, therefore, within the scope of the revenue with contracts with customers guidance. This assessment is performed throughout the life of the arrangement based on changes in the responsibilities of all parties in the arrangement.

For elements of collaboration arrangements that are not accounted for pursuant to the revenue from contracts with customers guidance, an appropriate recognition method is determined and applied consistently, generally by analogy to the revenue from contracts with customers guidance. Amounts related to transactions with a counterparty in a collaborative arrangement that is not a customer are presented as collaboration revenue and on a separate line item from revenue recognized from contracts with customers, if any, in our consolidated statements of operations.

Revenue is recognized when a customer obtains control of promised goods or services, in an amount that reflects the consideration which we expect to receive in exchange for those goods or services. To determine revenue recognition for arrangements that we determine are within the scope of ASC 606, Revenue from Contracts with Customers ("ASC 606"), we perform the following five steps: (i) identify the contract(s) with a customer; (ii) identify the performance obligations in the contract; (iii) determine the transaction price; (iv) allocate the transaction price to the performance obligations in the contract; and (v) recognize revenue when (or as) the entity satisfies a performance obligation. We only apply the five-step model to contracts when it is probable that we will collect the consideration we are entitled to in exchange for the goods or services we transfer to the customer. At contract inception, once the contract is determined to be within the scope of ASC 606, we assess the goods or services promised within each contract, determine those that are performance obligations and assess whether each promised good or service is distinct. We then recognize as revenue the

amount of the transaction price that is allocated to the respective performance obligation when (or as) the performance obligation is satisfied.

Pursuant to ASC 606, we recorded the $250.0 million non-refundable upfront payment in revenue as the payment was associated with the transfer of the good or services in the form of the license to Sobi. The $80.0 million reimbursement for research and development activities does not constitute a customer/vendor relationship and thus is not in the scope of ASC 606. As ASC 808 does not include recognition guidance, we established an accounting policy to recognize the payments under the reimbursement as a receivable on the balance sheet in an amount that is probable to be reimbursed based upon expense incurred by us, with a contra-research and development expense recognized in the statement of operations.

For the three months ended March 31, 2021 we did not recognize licensing revenue. As of March 31, 2021, we recognized $8.1 million in contra-research and development expense in the unaudited condensed consolidated statement of operations related to the $80.0 million reimbursement from Sobi. As of December 31, 2020, we had recorded a $43.0 million receivable for contra-research and development reimbursement, with $25.0 million and $18.0 million recorded in current and long-term assets, respectively. In January 2021, we received a $25.0 million development reimbursement payment from Sobi. As of March 31, 2021, we have a receivable of $26.1 million for contra-research and development reimbursement from Sobi recognized on the unaudited condensed consolidated balance sheet, with $20.0 million of the receivable in current assets and $6.1 million in other assets.

Research and Development Expenses

Research and development expenses consist primarily of costs incurred for our research activities, including our drug discovery efforts, and the development of our product candidates, which include:

- employee-related expenses including salaries, bonuses, benefits and share-based compensation expense related to individuals performing research and development activities;
- expenses incurred under agreements with third parties, including contract research organizations, or CROs, that conduct clinical trials and research and development activities on our behalf, and contract manufacturing organizations that manufacture quantities of drug supplies for both our preclinical studies and clinical trials;
- the cost of consultants, including share-based compensation expense; and
- various other expenses incident to the management of our preclinical studies and clinical trials.

Research and development costs are expensed as incurred. Nonrefundable advance payments for goods or services to be received in the future for use in research and development activities are deferred and capitalized. The capitalized amounts are expensed as the related goods are delivered or the services are performed. We have not provided program costs since inception because historically we have not tracked or recorded our research and development expenses on a program-by-program basis.

The successful development of our product candidates is highly uncertain. Accordingly, at this time, we cannot reasonably estimate the nature, timing and costs of the efforts that will be necessary to complete the remainder of the development of these product candidates. We are also unable to predict when, if ever, material net cash inflows will commence from pegcetacoplan or any other potential product candidates. This is due to the numerous risks and uncertainties associated with developing therapeutics, including the uncertainties of:

- establishing an appropriate safety profile in preclinical studies;
- successful enrollment in, and completion of clinical trials;
- receipt of marketing approvals from applicable regulatory authorities;
- establishing commercial manufacturing capabilities or making arrangements with third-party manufacturers;
- obtaining and maintaining patent and trade secret protection and regulatory exclusivity for our product candidates;
- launching commercial sales of the products, if and when approved, whether alone or in collaboration with others; and
- an acceptable safety profile of the products following approval.

A change in the outcome of any of these variables with respect to the development of any of our product candidates would significantly change the costs and timing associated with the development of that product candidate.

Research and development activities are central to our business model. Product candidates in later stages of clinical development generally have higher development costs than those in earlier stages of clinical development, primarily due to the

increased size and duration of later-stage clinical trials. We expect research and development costs to increase for the foreseeable future as our product candidate development programs progress. However, we do not believe that it is possible at this time to accurately project total program-specific expenses through commercialization. There are numerous factors associated with the successful commercialization of any of our product candidates, including future trial design and various regulatory requirements, many of which cannot be determined with accuracy at this time based on our stage of development. Additionally, future commercial and regulatory factors beyond our control will impact our clinical development programs and plans.

General and Administrative Expenses

General and administrative expenses consist primarily of employee-related expenses including salaries, bonuses, benefits and share-based compensation. Other significant costs include facility costs not otherwise included in research and development expenses, legal fees relating to patent and corporate matters, and fees for accounting and consulting services.

We anticipate that our general and administrative expenses will increase in the future to support continued research and development activities, potential commercialization of our product candidates and increased costs of operating as a public company. These increases will likely include increased costs related to the hiring of additional personnel and fees to outside consultants, attorneys and accountants, among other expenses. Additionally, we anticipate increased costs associated with being a public company including expenses related to services associated with maintaining compliance with exchange listing and SEC requirements, insurance costs and investor relations costs.

Critical Accounting Policies and Estimates

This discussion and analysis of our financial condition and results of operations is based on our financial statements, which we have prepared in accordance with U.S. generally accepted accounting principles. The preparation of these financial statements requires us to make estimates and assumptions that affect the reported amounts of assets and liabilities and the disclosure of contingent assets and liabilities at the date of the financial statements, as well as the reported amounts of expenses during the reporting periods. On an ongoing basis, we evaluate our estimates and judgments, including those described in greater detail below. We base our estimates on historical experience and on various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying value of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimates under different assumptions or conditions.

The following critical accounting policies are described under the heading “Management’s Discussion and Analysis of Financial Condition and Results of Operations—Critical Accounting Policies and Estimates” in our 2020 Annual Report on Form 10-K and the notes to the unaudited condensed financial statements included in Item 1, “Unaudited Financial Statements,” of this Quarterly Report on Form 10-Q. We believe that of our critical accounting policies, the following accounting policies are the most critical to fully understanding and evaluating our financial condition and results of operations:

- Licensing and Collaboration Revenue
- Accrued Research and Development
- Convertible Notes
- Capped Call Transactions, and
- Development Derivative Liability

Results of Operations

Comparison of Three Months Ended March 31, 2021 and 2020

The following table summarizes our results of operations for the three months ended March 31, 2021 and 2020, together with the dollar increase or decrease and percentage change in those items (in thousands):

	For the Three Months Ended March 31,		Change	Change
	2021	2020	$	%
Operating expenses:				
Research and development	84,012	69,282	14,730	21%
General and administrative	40,579	29,504	11,075	38
Net operating loss	(124,591)	(98,786)	(25,805)	26
Loss on conversion of debt	(39,487)	—	(39,487)	100
Loss from remeasurement of development derivative liability	(17,084)	(68,406)	51,322	(75)
Interest income	134	2,275	(2,141)	(94)
Interest expense	(4,175)	(3,919)	(256)	7
Other income, net	1,544	14	1,530	10,929
Net loss	$ (183,659)	$ (168,822)	$ (14,837)	9

Research and Development Expenses

The following table summarizes our research and development expenses incurred during the three months ended March 31, 2021 and 2020, together with the dollar increase or decrease and percentage change in those items (in thousands):

	For the Three Months Ended March 31,		Change	Change
	2021	2020	$	%
Clinical trial costs	29,591	19,492	10,099	52%
Compensation and related personnel costs	24,557	16,708	7,849	47
Contract manufacturing	21,809	26,178	(4,369)	(17)
Sobi development milestone	(8,053)	—	(8,053)	100
Research / Innovation costs	3,678	3,282	396	12
Other development costs	10,403	2,547	7,856	308
Pre-clinical study expenses	1,919	720	1,199	167
Device development expenses	108	355	(247)	(70)
Total research and development expenses	$ 84,012	$ 69,282	$ 14,730	21

Research and development expenses increased by $14.7 million to $84.0 million for the three months ended March 31, 2021 from $69.3 million for the three months ended March 31, 2020, an increase of 21%. The increase in research and development expenses was primarily attributable to an increase of $10.1 million in clinical trial costs associated the on-going Phase 3 trials and the preparation for and commencement of our clinical trials in other indications, an increase of $7.8 million in personnel related costs primarily due to the hiring of additional personnel, an increase of $9.5 million in research and development supporting activities caused primarily by increased quality and medical affairs expenses. The increases were offset by a decrease of $8.1 million in contra research and development expense related to the Sobi transaction, $4.4 million in contract manufacturing expenses due to timing of drug supply and analytical activity and $0.2 million in device development expenses. We expect our research and development expenses to continue to increase as the number of patients in our trials increases and the number of ongoing trials increases.

General and Administrative Expenses

General and administrative expenses increased by $11.1 million to $40.6 million for the three months ended March 31, 2021, from $29.5 million for the three months ended March 31, 2020, an increase of 38%. The increase in general and administrative expenses was primarily attributable to an increase in employee related costs of $7.8 million, an increase in professional and consulting

fees and general commercial preparation activities of $3.2 million, an increase of $0.6 million in director stock compensation expense, and an increase in $0.1 million in insurance, offset by a decrease in general office costs and conference and travel related expenses of $0.6 million. The increase in employee related costs of $7.8 million consisted of a $4.7 million increase in salaries and benefits primarily due to the increase in the number of employees, $4.1 million related to stock expense associated with the grant of stock options and restricted stock units to employees offset by a decrease of $1.0 million in recruitment expense. The increase in other professional and consulting fees and general commercial preparation activities of $3.2 million primarily related to an increase in commercial related activity of $4.8 million, offset by a decrease in general professional fees of $1.3 million and a decrease in communication and public relations fees of $0.3 million.

Loss on Conversion of Debt

In January 2021, we entered into separate, privately negotiated exchange agreements to modify the conversion terms with certain holders of our 2019 Convertible Notes. Under the terms of these exchange agreements, the holders exchanged approximately $126.1 million in aggregate principal amount of 2019 Convertible Notes held by them for an aggregate of 3,906,869 shares of common stock issued by us. In accordance with ASC topic 470-20, "*Debt,*– Debt with Conversion and Other Options," we accounted for the exchange as an induced conversion based on the short period of time the conversion offer was open and the substantive conversion feature offer. We accounted for the conversion of the debt as an inducement by expensing the fair value of the shares that were issued in excess of the original terms of the Convertible Notes. We reduced net debt outstanding and increased equity on the consolidated balance sheet by $122.8 million, consisting of the par value of the 2019 Convertible Notes exchanged of $126.1 million less the $3.3 million of remaining debt issuance costs associated with the exchange notes. We also increased shares outstanding by 3,906,869 shares consisting of 3,196,172 shares issued at the initial conversion rate in the Indenture of 25.3405 plus an additional 710,697 shares. Additionally, we issued 69,491 shares as settlement of debt issuance costs paid to our advisor in connection with the conversion transaction. Upon exchange of the 2019 Convertible Notes, the holders forfeited accrued interest through the date of the exchange of $1.7 million, which we charged to interest expense and to equity. We recorded a loss on conversion of debt of $39.5 million comprised of $36.4 million related to the value of the shares issued in excess of the original conversion terms at the fair market value and $3.1 million for the value of the 69,491 shares issued in payment of issuance costs. As of March 31, 2021, we held as treasury Convertible Notes the $126.1 million principal amount of exchanged notes and such notes have not been cancelled.

Gain/Loss from Remeasurement of Development Derivative Liability

On February 28, 2019, we entered into the SFJ agreement under which SFJ agreed to provide funding to us to support the development of pegcetacoplan for the treatment of patients with PNH. The development derivative liability was initially recorded at the value of the $60.0 million aggregate cash received pursuant to the contractual terms.

The SFJ agreement was amended on June 7, 2019 to provide for additional funding and we received $20.0 million upon execution of the SFJ amendment in June 2019 and in each of September 2019 and January 2020, we achieved a $20.0 million development milestone under the terms of the agreement, resulting in the receipt of an aggregate of $60.0 million of additional funding from SFJ.

We remeasure the fair value of the liability as a level 3 derivative at the end of each quarter. The remeasurement as of March 31, 2021 and March 31, 2020 resulted in a change in fair value recorded as a loss in the unaudited condensed consolidated statements of operations of $17.1 million and $68.4 million for the three months ended March 31, 2021 and March 31, 2020, respectively.

Interest Expense

Interest expense was $4.2 million for the three months ended March 31, 2021 and $3.9 million for the three months ended March 31, 2020 primarily related to the 2019 and 2020 Convertible Notes. The increase in interest was primarily due to the increase in the Convertible Notes outstanding offset by the adoption of ASU 2020-06 which reduced the amount of non-cash interest recognized on the debt discount.

Interest Income

Interest income was $0.1 million for the three months ended March 31, 2021, a decrease of $2.1 million, compared to $2.2 million for the three months ended March 31, 2020. The decrease in interest income was primarily attributable to a decline in investment yields and interest rates.

Other Income, Net

Other income, net during the three months ended March 31, 2021, decreased $1.5 million compared to the three months ended March 31, 2020.

Liquidity and Capital Resources

Sources of Liquidity

To date, we have financed our operations primarily through $772.1 million in net proceeds from public offerings of our common stock, including our IPO, $535.8 million in net proceeds from offerings of Convertible Notes, a $250.0 million upfront payment and a $25.0 million development reimbursement payment from Sobi each pursuant to the Sobi collaboration agreement, $112.6 million in proceeds from the private placement of shares of our convertible preferred stock prior to our IPO, $140.0 million under the SFJ agreement, $20.0 million in proceeds from borrowings under a term loan facility with Silicon Valley Bank, and $7.0 million in proceeds from our issuance and sale of a promissory note. We have repaid the term loan facility and the promissory note in full, and we exchanged $126.1 million of aggregate principal amount of 2019 Convertible Notes for shares of our common stock in January 2021.

On April 23, 2018, we issued and sold 5,500,000 shares of our common stock in a follow-on public offering at a public offering price of $25.50 per share for net proceeds of $131.2 million, after deducting underwriting discounts and commissions of $8.4 million and offering expenses of $0.5 million.

On March 11, 2019, we issued and sold 6,900,000 shares of our common stock in a follow-on offering at a public offering price of $17.00. We received net proceeds of $109.6 million after deducting underwriting discounts and commissions of $7.0 million and offering costs of $0.7 million.

On September 16, 2019, we completed a private offering of $220.0 million aggregate principal amount of Convertible Notes. We received net proceeds of approximately $212.9 million after deducting the initial purchasers' discounts and commissions and offering costs of $7.1 million.

On January 13, 2020, we issued and sold 10,925,000 shares of our common stock in a follow-on offering at a public offering price of $37.00, including 1,425,000 shares sold pursuant to the underwriters' exercise in full of their option to purchase additional shares of common stock. We received total net proceeds of $381.4 million after deducting underwriting discounts and commissions of $22.2 million and offering costs of $0.5 million.

On May 12, 2020, we completed a private offering of $300.0 million aggregate principal amount of 2020 Convertible Notes. We received net proceeds of approximately $322.9 million, which included accrued interest March 15, 2020 to, but not including May 12, 2020, and the initial purchasers' discounts and commissions and offering costs of $6.0 million.

On January 6, 2021, we entered into separate, privately negotiated exchange agreements with certain holders of our 2019 Convertible Notes. Under the terms of these exchange agreements, the holders exchanged approximately $126.1 million in aggregate principal amount of 2019 Convertible Notes held by them for an aggregate of 3,906,869 shares of our common stock. The exchange transactions closed in January 2021.

In addition to our existing cash, cash equivalents and marketable securities, we expect to receive research and development reimbursements and are eligible to earn development and commercial milestone payments and royalties under our collaboration agreement with Sobi. Our ability to earn these milestone payments and the timing of earning these payments is dependent upon the outcome of our research and development and commercialization activities and is uncertain at this time.

The capped call transactions that we entered into concurrently with the issuance of the Convertible Notes are expected generally to reduce the potential dilution to our common stock upon any conversion of Convertible Notes and/or offset any cash payments we are required to make in excess of the principal amount of converted Convertible Notes, as the case may be, in the event that the market price per share of our common stock, as measured under the terms of the capped call transactions, is greater than the strike price of the capped call transactions, which is initially $39.4625, the conversion price of the Convertible Notes.

Cash Flows

The following table provides information regarding our cash flows for the three months ended March 31, 2021 and 2020 (in thousands):

	For the Three Months Ended March 31,	
	2021	2020
Net cash used in operating activities	$ (153,314)	$ (108,349)
Net cash used in investing activities	(146,765)	(227,854)
Net cash provided by financing activities	1,632	403,267
Effect of exchange rate changes on cash, cash equivalents and restricted cash	(1,611)	(152)
Net increase (decrease) in cash, cash equivalents and restricted cash	$ (300,058)	$ 66,912

Net Cash Used in Operating Activities

Net cash used in operating activities was $153.3 million for the three months ended March 31, 2021 and consisted primarily of a net loss of $183.7 million adjusted for $75.4 million of non-cash items, including a loss on early conversion of debt of $39.5 million and a loss from remeasurement of development derivative liability of $17.1 million, share-based compensation expense of $16.4 million, and the forfeiture of accrued interest in the exchange of the 2019 Convertible Notes of $1.7 million, a net increase in operating assets of $1.9 million, an decrease in accounts payable of $4.1 million and an decrease in accrued expenses of $42.8 million.

Net cash used in operating activities was $108.3 million for the three months ended March 31, 2020 and consisted primarily of a net loss of $168.8 million adjusted for $79.8 million of non-cash items, including a loss from remeasurement of development derivative liability of $68.4 million and share-based compensation expense of $9.3 million, a net increase in accounts payable, accrued expenses and other liabilities of $15.2 million and a net decrease in operating assets of $4.1 million.

Net Cash Used in Investing Activities

Net cash used in investing activities during the three months ended March 31, 2021 was $146.8 million due primarily to the purchase of marketable securities.

Net cash used in investing activities during the three months ended March 31, 2020 was $227.9 million due primarily to the purchase of marketable securities with proceeds from the January 2020 follow-on offering.

Net Cash Provided by Financing Activities

Net cash provided by financing activities was $1.6 million during the three months ended March 31, 2021 and consisted primarily of $2.6 million upon the exercise of stock options offset by $1.0 million for the payment of employee tax withholding related to equity-based compensation.

Net cash provided by financing activities was $403.3 million during the three months ended March 31, 2020 and consisted primarily of proceeds from the follow-on offering in January 2020 of $381.6 million, the receipt of $20.0 million from the SFJ agreement and $1.7 million upon the exercise of stock options.

Funding Requirements

We expect our expenses to increase in connection with our ongoing activities, particularly as we continue the research and development of, and seek marketing approval for, our product candidates. In addition, if we obtain marketing approval for any of our product candidates, we expect to incur significant commercialization expenses related to product sales, marketing, manufacturing and distribution. Furthermore, we expect to continue to incur additional costs associated with operating as a public company. Accordingly, we will need to obtain substantial additional funding in connection with our continuing operations. If we are unable to raise capital when needed or on attractive terms, we would be forced to delay, reduce or eliminate our research and development programs or future commercialization efforts.

We believe that our cash, cash equivalents and marketable securities as of March 31, 2021, along with the committed development reimbursement payments from Sobi, will enable us to fund our operating expenses and capital expenditure requirements at least into the second half of 2022. We have based this estimate on assumptions that may prove to be wrong, and we may use our available capital resources sooner than we currently expect. Although we expect that our cash, cash equivalents and marketable securities will be sufficient to allow us to complete the DERBY and OAKS clinical trials, we do not believe they will be sufficient to allow us to support both the systemic and ophthalmological pegcetacoplan programs through commercial launch. Because of the numerous risks and uncertainties associated with the development of pegcetacoplan and other potential product candidates, and because the extent to which we may enter into collaborations with third parties for the development of these product candidates is unknown, we are unable to estimate the amounts of increased capital outlays and operating expenses associated with completing the research and development of our product candidates. Our future funding requirements will depend on many factors, including:

- the scope, progress, timing, costs and results of clinical trials of, and research and preclinical development efforts for pegcetacoplan, APL-9 and future product candidates;
- our ability to maintain a productive collaborative relationship with Sobi with respect to pegcetacoplan, including our ability to achieve milestone payments under our agreement with Sobi;
- our ability to identify additional collaborators for any of our product candidates and the terms and timing of any collaboration agreement that we may establish for the development and any commercialization of such product candidates;
- the number and characteristics of future product candidates that we pursue and their development requirements;
- the outcome, timing and costs of clinical trials and of seeking regulatory approvals of pegcetacoplan and other product candidates we may pursue
- the costs of commercialization activities for any of our product candidates that receive marketing approval to the extent such costs are not the responsibility of any collaborators, including the costs and timing of establishing product sales, marketing, distribution and manufacturing capabilities;
- subject to receipt of marketing approval, revenue, if any, received from commercial sales of pegcetacoplan and our other product candidates;
- our headcount growth and associated costs as we expand our research and development and establish a commercial infrastructure;
- the costs of preparing, filing and prosecuting patent applications, maintaining and protecting our intellectual property rights and defending against intellectual property related claims;
- the effect of competing technological and market developments;
- the effect of the COVID-19 pandemic on the healthcare system and the economy generally and on our clinical trials and other operations specifically;
- our ability to obtain adequate reimbursement for any product we commercialize; and
- the costs of operating as a public company.

Identifying potential product candidates and conducting preclinical testing and clinical trials is a time-consuming, expensive and uncertain process that takes years to complete, and we may never generate the necessary data or results required to obtain marketing approval and achieve product sales. In addition, our product candidates, if approved, may not achieve commercial success. Our commercial revenues, if any, will be derived from sales of medicines that we do not expect to be commercially available for many years, if at all. Accordingly, we will need to continue to rely on additional financing to achieve our business objectives. Adequate additional financing may not be available to us on acceptable terms, or at all.

Until such time, if ever, as we can generate substantial product revenues, we expect to finance our cash needs through a combination of equity offerings, debt financings, collaborations, strategic alliances and licensing arrangements. We currently do not have any committed external source of funds. To the extent that we raise additional capital through the sale of equity or convertible debt securities, our stockholders' ownership interests will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect our stockholders' rights. Debt financing, if available, would result in fixed payment obligations and may involve agreements that include restrictive covenants that limit our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends, that could adversely impact our ability to conduct our business.

If we raise funds through collaborations, strategic alliances or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates or to grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds through equity or debt financings when needed, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

Contractual Obligations

The disclosure of our contractual obligations and commitments is set forth under the heading "Management's Discussion and Analysis of Financial Condition and Results of Operations—Contractual Obligations" in our 2020 Annual Report on Form 10-K. See Note 12 to our unaudited condensed financial statements included in Item 1, "Financial Statements," of this Quarterly Report on Form 10-Q for a discussion of obligations and commitments.

During the three months ended March 31, 2021, there were no material changes to our contractual obligations and commitments as of December 31, 2020 as described under "Management's Discussion and Analysis of Financial Condition and Results of Operations – Contractual Obligations" in our 2020 Annual Report on Form 10-K.

Off-Balance Sheet Arrangements

We did not have during the periods presented, and we do not currently have, any off-balance sheet arrangements, as defined under applicable SEC rules.

Item 3. Quantitative and Qualitative Disclosures About Market Risk.

We are exposed to market risk related to changes in interest rates. As of March 31, 2021, we had cash, cash equivalents and marketable securities of $723.7 million, consisting primarily of money market funds and U.S. Government obligations and marketable securities. Our primary exposure to market risk is interest rate sensitivity, which is affected by changes in the general level of U.S. interest rates. Due to the short-term duration of our investment portfolio and the low risk profile of our investments, an immediate 10% change in interest rates would not have a material effect on the fair market value of our investment portfolio. We have the ability to hold our marketable securities until maturity, and therefore we would not expect our operating results or cash flows to be affected to any significant degree by the effect of a change in market interest rates on our investments.

Item 4. Controls and Procedures.

Limitations on Effectiveness of Controls and Procedures

The term "disclosure controls and procedures," as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act of 1934 as amended, or the Exchange Act, refers to controls and procedures that are designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is recorded, processed, summarized and reported, within the time periods specified in the SEC's rules and forms. Our disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is accumulated and communicated to our management, including its principal executive and principal financial officers, or persons performing similar functions, as appropriate to allow timely decisions regarding required disclosure.

In designing and evaluating our disclosure controls and procedures, management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving the desired control objectives. In addition, the design of disclosure controls and procedures must reflect the fact that there are resource constraints and that management is required to apply judgment in evaluating the benefits of possible controls and procedures relative to their costs.

Evaluation of Disclosure Controls and Procedures

Our management, with the participation of our Chief Executive Officer and Chief Financial Officer, evaluated, as of the end of the period covered by this Quarterly Report on Form 10-Q, the effectiveness of our disclosure controls and procedures (as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act). Based on that evaluation, our Chief Executive Officer and Chief Financial Officer concluded that our disclosure controls and procedures were effective at the reasonable assurance level as of March 31, 2021.

Changes in Internal Control Over Financial Reporting

No change in our internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act) occurred during the three months ended March 31, 2021 that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

As a result of the COVID-19 pandemic, beginning in March 2020 certain of our employees began working remotely. We have not identified any material changes in the Company's internal control over financial reporting as a result of these changes to the working environment. We are continually monitoring and assessing the COVID-19 situation to determine any potential impacts on the design and operating effectiveness of our internal controls over financial reporting.

PART II—OTHER INFORMATION

Item 1. Legal Proceedings.

We are not currently subject to any material legal proceedings.

Item 1A. Risk Factors.

Careful consideration should be given to the following risk factors, in addition to the other information set forth in this Quarterly Report on Form 10-Q and in other documents that we file with the SEC, in evaluating our company and our business. Investing in our common stock involves a high degree of risk. If any of the following risks actually occur, our business, financial condition, results of operations and future growth prospects could be materially and adversely affected.

RISK FACTOR SUMMARY

Our business is subject to a number of risks that if realized could materially affect our business, financial condition, results of operations, cash flows and access to liquidity. These risks are discussed more fully in the "Risk Factors" section of this Annual Report on Form 10-K. Our principal risks include the following:

- We have incurred significant losses since inception, expect to incur significant and increasing losses for at least the next several years, and may never achieve or maintain profitability. Our net losses were $183.7 million and $168.8 million for the three months ended March 31, 2021 and 2020, respectively.

- We have not yet successfully obtained marketing approvals nor commercialized any pharmaceutical products, which may make it difficult to evaluate our future prospects. We will need to transition from a company with a development focus to a company capable of supporting commercial activities.

- We will need substantial additional funding, to allow us to support both the systemic and ophthalmological pegcetacoplan programs through commercial launch, and if we are unable to raise capital when needed, we could be forced to delay, reduce or eliminate certain product development programs or commercialization efforts. We believe that our existing cash, cash equivalents and marketable securities, along with the committed development reimbursement payments from Sobi, will enable us to fund our operating expenses and capital expenditure requirements at least into the second half of 2022.

- If we receive regulatory approval for the use of pegcetacoplan as a treatment for PNH or if our agreement with SFJ Pharmaceuticals Group, or SFJ, is terminated prior to receiving such approval in specified circumstances, we will be required to make substantial payments to SFJ pursuant to our development funding agreement. If we do not have sufficient funding or cash flow from our business to meet our payment obligations under the development funding agreement, SFJ could exercise its remedies as a holder of a first priority security interest in our assets and our business could be materially harmed.

- We are dependent on the successful development and commercialization of pegcetacoplan. If we are unable to develop, obtain marketing approval for or successfully commercialize pegcetacoplan or if we experience significant delays in doing so, our business could be harmed.

- The regulatory approval process is expensive, time consuming and uncertain and may prevent us or our collaborators such as Swedish Orphan Biovitrum AB (Publ), or Sobi, from obtaining approvals for the commercialization of pegcetacoplan or any of our product candidates that we develop. As a result, we cannot predict when or if, and in which territories, we, or our collaborators, will obtain marketing approval to commercialize pegcetacoplan or any other product candidate that we develop.

- The COVID-19 pandemic may affect our ability to conduct our ongoing clinical trials, delay the initiation of planned and future clinical trials, disrupt regulatory activities, or have other adverse effects on our business and operations. In addition, the COVID-19 pandemic has adversely impacted economies worldwide, which could result in adverse effects on our business and operations.

- There are no approved therapies that act by inhibiting C3 and only two approved therapies in our target indications that act by inhibiting the complement system. We may not be able to successfully develop and commercialize pegcetacoplan or other product candidates.

- If clinical trials of our product candidates fail to satisfactorily demonstrate safety and efficacy to the U.S. Food and Drug Administration, or FDA, the European Medicines Agency, or EMA, and other regulators, we may incur additional costs or experience delays in completing, or ultimately be able to complete, the development and commercialization of these product candidates.

- We rely on third parties to conduct our clinical trials and to manufacture and distribute our product candidates for our clinical trials. We contract with third parties for the manufacture, storage and distribution of our product candidates for clinical trials as well as in connection with our commercialization efforts. This reliance on third parties increases the risk that we will not have sufficient quantities of our product candidates or such quantities at an acceptable cost, which could delay, prevent or impair our development or commercialization efforts. If these third parties do not perform satisfactorily, our development or commercialization efforts could be delayed or impaired.

- Our prospects for the development and commercialization of our product candidates will depend in significant part on the success of our collaboration with Sobi for the global co-development and commercialization of systemic pegcetacoplan outside of the United States. Our ability to generate revenues from this collaboration will depend on Sobi.

- If we fail to comply with our obligations under our existing and any future intellectual property licenses with third parties, we could lose license rights that are important to our business, including our patent license agreements with the University of Pennsylvania under which we license patents with claim that recite a class of compounds generically covering our lead product candidates, pegcetacoplan and APL-9, and that specifically recite the active component.

Risks Related to Our Financial Position and Need for Additional Capital

We have incurred significant losses since inception, expect to incur significant and increasing losses for at least the next several years, and may never achieve or maintain profitability.

We have incurred significant annual net operating losses in every year since our inception. We expect to continue to incur significant and increasing net operating losses for at least the next several years. Our net losses were $183.7 million for the three months ended March 31, 2021 and $344.9 million for the year ended December 31, 2020. As of March 31, 2021, we had an accumulated deficit of $1.1 billion. We have not generated any revenues from product sales, have not completed the development of any product candidate and may never have a product candidate approved for commercialization. We have financed our operations to date primarily through the sale of our common stock in our initial public offering and subsequent follow-on offerings, the sale of convertible notes, private placements of our preferred stock prior to our initial public offering, the development funding agreement with SFJ Pharmaceuticals Group, or SFJ, the collaboration agreement with Swedish Orphan Biovitrum AB (Publ), or Sobi, borrowings under a term loan facility and the issuance and sale of a promissory note. We have devoted substantially all of our financial resources and efforts to research and development, including preclinical studies and our clinical trials. Our net losses may fluctuate significantly from quarter to quarter and year to year. Net losses and negative cash flows have had, and will continue to have, an adverse effect on our stockholders' equity and working capital.

We expect to continue to incur significant expenses and increasing operating losses for the foreseeable future. We anticipate that our expenses will increase substantially if and as we:

- continue to develop and conduct clinical trials with our lead product candidate, pegcetacoplan, and APL-9;
- initiate and continue research and preclinical and clinical development efforts for any future product candidates;
- seek to identify and develop additional product candidates for complement-dependent diseases;
- seek regulatory and marketing approvals for our product candidates that successfully complete clinical trials, if any;
- establish sales, marketing, distribution and other commercial infrastructure to commercialize any products for which we may obtain marketing approval;
- require the manufacture of larger quantities of product candidates for clinical development and, potentially, commercialization;
- maintain, expand and protect our intellectual property portfolio;
- hire and retain additional personnel, such as clinical, quality control and scientific personnel;

- add operational, financial and management information systems and personnel, including personnel to support our product development and help us comply with our obligations as a public company; and
- add equipment and physical infrastructure to support our research and development programs.

Our ability to become and remain profitable depends on our ability to generate revenue. We do not expect to generate significant revenue unless and until we are, or our collaborators, including Sobi, are able to obtain marketing approval for, and successfully commercialize, one or more of our product candidates. Successful commercialization will require achievement of key milestones, including completing clinical trials of our product candidates, obtaining marketing approval for these product candidates, manufacturing, marketing and selling those products for which we, or any of our collaborators, may obtain marketing approval, satisfying any post-marketing requirements and obtaining reimbursement for our products from private insurance or government payors. Because of the uncertainties and risks associated with these activities, we are unable to accurately predict the timing and amount of revenues, and if or when we might achieve profitability. We and any collaborators may never succeed in these activities and, even if we do, or any collaborators do, we may never generate revenues that are large enough for us to achieve profitability. Even if we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to become and remain profitable would decrease the value of our company and could impair our ability to raise capital, expand our business, maintain our research and development efforts, diversify our pipeline of product candidates or continue our operations. A decline in the value of our company could cause our stockholders to lose all or part of their investment.

We have not yet successfully obtained marketing approvals nor commercialized pharmaceutical products, which may make it difficult to evaluate our future prospects.

Our operations to date have been limited to financing and staffing our company, developing our technology and conducting preclinical research and Phase 1, Phase 2 and Phase 3 clinical trials for our product candidates. However, although we have begun planning commercial activities, we have not yet demonstrated an ability to successfully obtain marketing approvals, manufacture a commercial-scale product, or arrange for a third party to do so on our behalf, or conduct sales and marketing activities necessary for successful product commercialization. Accordingly, our stockholders should consider our prospects in light of the costs, uncertainties, delays and difficulties frequently encountered by clinical-stage biopharmaceutical companies such as ours. Any predictions made about our future success or viability may not be as accurate as they could be if we had a longer operating history or a history of successfully developing and commercializing pharmaceutical products.

We may encounter unforeseen expenses, difficulties, complications, delays and other known or unknown factors in achieving our business objectives. We will need to transition from a company with a development focus to a company capable of supporting commercial activities. We may not be successful in such a transition.

We expect our financial condition and operating results to continue to fluctuate significantly from quarter to quarter and year to year due to a variety of factors, many of which are beyond our control. Accordingly, our stockholders should not rely upon the results of any quarterly or annual periods as indications of future operating performance.

We will need substantial additional funding to allow us to support both the systemic and ophthalmological programs through commercial launch, and if we are unable to raise capital when needed, we could be forced to delay, reduce or eliminate our product development programs or commercialization efforts.

Developing pharmaceutical products, including conducting preclinical studies and clinical trials, is a very time-consuming, expensive and uncertain process that takes years to complete. We have consumed substantial amounts of cash since our inception. For example, in the three months ended March 31, 2021, and in the years ended December 31, 2020, 2019 and 2018, we used net cash of $153.3 million, $160.5 million, $211.1 million and $131.2 million respectively, in our operating activities substantially all of which related to research and development activities. As of March 31, 2021, our cash, cash equivalents and marketable securities were $723.7 million. We expect our expenses to increase in connection with our ongoing activities, particularly as we initiate new clinical trials of, initiate new research and preclinical development efforts for, seek marketing approval for and prepare for commercialization of, our product candidates. In addition, if we obtain marketing approval for pegcetacoplan or any of our other product candidates, we may incur significant commercialization expenses related to product sales, marketing, manufacturing and distribution to the extent that such sales, marketing, manufacturing and distribution are not the responsibility of the collaborator. Furthermore, we continue to incur significant costs associated with operating as a public company. Accordingly, we will need to obtain substantial additional funding in connection with our continuing operations, particularly if we commercialize the ophthalmological program outside the United States without a collaborator or if we fail to establish substantial commercial sales of systemic pegcetacoplan. If we are unable to raise capital when needed or on attractive terms, we may be forced to delay, reduce or eliminate our research and development programs or any future commercialization efforts.

We will be required to expend significant funds in order to advance the development of pegcetacoplan in multiple disease areas, as well as other product candidates we may seek to develop. In addition to our collaboration agreement with Sobi, we may seek one or more additional collaborators for future development of our product candidates for one or more indications. However, we may not be able to enter into an additional collaboration for any of our product candidates for such indications on suitable terms, on a timely basis or at all. Accordingly, we may be required to obtain further funding through public or private equity offerings, debt financings, collaborations and licensing arrangements or other sources to achieve our business objectives. We do not have any committed external source of funds other than Sobi's reimbursement obligations under the collaboration agreement. Adequate additional financing may not be available to us on acceptable terms, or at all. Our failure to raise capital as and when needed would have a negative impact on our financial condition and our ability to pursue our business strategy.

We believe that our existing cash, cash equivalents and marketable securities, along with the committed development reimbursement payments from Sobi, will enable us to fund our operating expenses and capital expenditure requirements at least into the second half of 2022. Our estimate as to how long we expect our cash, cash equivalents and marketable securities to be able to continue to fund our operations is based on assumptions that may prove to be wrong, and we could use our available capital resources sooner than we currently expect. Although we expect that our cash, cash equivalents and marketable securities will be sufficient to allow us to complete the DERBY and OAKS clinical trials, we do not believe they will be sufficient to allow us to support both the systemic and ophthalmological pegcetacoplan programs through commercial launch. Further, changing circumstances, some of which may be beyond our control, could cause us to consume capital significantly faster than we currently anticipate, and we may need to seek additional funds sooner than planned. Our future funding requirements, will depend on many factors, including:

- the scope, progress, timing, costs and results of clinical trials of, and research and preclinical development efforts for, pegcetacoplan, APL-9 and future product candidates;
- our ability to maintain a productive collaborative relationship with Sobi with respect to pegcetacoplan, including our ability to achieve milestone payments under our agreement with Sobi;
- our ability to identify additional collaborators for any of our product candidates and the terms and timing of any collaboration agreement that we may establish for the development and any commercialization of such product candidates;
- the number and characteristics of future product candidates that we pursue and their development requirements;
- the outcome, timing and costs of clinical trials and of seeking regulatory approvals of pegcetacoplan and other product candidates we may pursue;
- the costs of commercialization activities for any of our product candidates that receive marketing approval to the extent such costs are not the responsibility of our collaborators, including the costs and timing of establishing product sales, marketing, distribution and manufacturing capabilities;
- subject to receipt of marketing approval, revenue, if any, received from commercial sales of pegcetacoplan and other product candidates;
- our headcount growth and associated costs as we expand our research and development and establish a commercial infrastructure;
- the costs of preparing, filing and prosecuting patent applications, maintaining and protecting our intellectual property rights and defending against intellectual property related claims;
- the effect of competing technological and market developments;
- the effect of the COVID-19 pandemic on the healthcare system and the economy generally and on our clinical trials and other operations specifically;
- our ability to obtain adequate reimbursement for any product we commercialize; and
- the costs of operating as a public company.

Raising additional capital may cause dilution to our stockholders, restrict our operations or require us to relinquish rights to our technologies or product candidates.

We expect our expenses to increase in connection with our planned operations. To the extent that we raise additional capital through the sale of common stock, convertible securities or other equity securities, the ownership interest of our then-existing stockholders may be diluted, and the terms of these securities could include liquidation or other preferences and anti-dilution protections that could adversely affect the rights of our common stockholders. In addition, additional debt financing, if available, would result in fixed payment obligations and may involve agreements that include grants of security interests on our assets and

restrictive covenants that limit our ability to take specific actions, such as incurring additional debt, making capital expenditures, creating liens, redeeming stock or declaring dividends, that could adversely impact our ability to conduct our business.

For example, under our development funding agreement with SFJ, as amended, we have agreed that following regulatory approval by the U.S. Food and Drug Administration, or FDA, or the European Medicines Agency, or EMA, for the use of pegcetacoplan as a treatment for paroxysmal nocturnal hemoglobinuria, or PNH, we will pay to SFJ an initial payment of up to $5.0 million (or up to a total of $10.0 million if regulatory approval is granted by the FDA and the EMA) and then up to an additional $226.0 million in the aggregate (or up to $452.0 million if regulatory approval is granted by the FDA and the EMA) in six additional annual payments with the majority of the payments being made from the third anniversary to the sixth anniversary of regulatory approval. In September 2020, we submitted an NDA, to the FDA and a marketing approval authorization, or MAA, to the EMA for pegcetacoplan for the treatment of PNH. Additionally, we granted a security interest in all of our assets, excluding our intellectual property and license agreements to which we are a party. In connection with the grant of the security interest, we agreed to certain affirmative and negative covenants, including restrictions on our ability to pay dividends, incur additional debt or enter into licensing transactions with respect to our intellectual property other than specified types of licenses.

Future debt securities or other financing arrangements could contain similar or more restrictive negative covenants. In addition, securing financing could require a substantial amount of time and attention from our management and may divert a disproportionate amount of their attention away from day-to-day activities, which may adversely affect our management's ability to oversee the development of our product candidates.

In January 2021, we closed privately negotiated exchange agreements to modify the conversion terms with certain holders of our outstanding convertible notes issued in September 2019, or the 2019 Convertible Notes, under which we issued approximately 3.9 million shares of common stock in exchange for approximately $126.1 million in aggregate principal amount of 2019 Convertible Notes. The effective price per share of the common stock issued in the exchange transactions was lower than the trading price of the Company's common stock on the Nasdaq market at the time of settlement of the exchanges. We may in the future exchange additional principal amount of our Convertible Notes and the effective price per share of the common stock may be lower than the trading price at such time.

If we raise additional funds through collaborations or marketing, distribution or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams or product candidates or grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds when needed, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

If we receive regulatory approval for the use of pegcetacoplan as a treatment for PNH or if our agreement with SFJ is terminated prior to receiving such approval in specified circumstances, we will be required to make substantial payments to SFJ pursuant to our development funding agreement. If we do not have sufficient funding or cash flow from our business to meet our payment obligations under the development funding agreement, SFJ could exercise its remedies as a holder of a first priority security interest in our assets and our business could be materially harmed.

We submitted an NDA to the FDA and a MAA to the EMA for pegcetacoplan for the treatment of PNH in September 2020. If we receive regulatory approval for the use of pegcetacoplan as a treatment for PNH, we will be required to make substantial payments to SFJ pursuant to our development funding agreement. In addition, if the agreement is terminated prior to obtaining regulatory approval for the treatment of PNH, under specified circumstances, we also will be required to make substantial payments to SFJ. Our ability to make these required payments depends on our future performance and the future performance of Sobi, which is subject to economic, financial, competitive and other factors beyond our control. Our business may generate cash flow from operations in the future sufficient to meet our obligations under the development funding agreement. If we are unable to generate such cash flow or to obtain additional funding through public or private equity offerings, debt financings, collaborations and licensing arrangements or other sources on acceptable terms or at all, we could default on our payment obligations to SFJ.

Our payment obligations to SFJ could have significant consequences for our security holders and our business, results of operations and financial condition by, among other things:

- limiting our ability to obtain additional financing;
- requiring the dedication of a substantial portion of our cash flow from operations to service our meet our obligations under the development funding agreement, which will reduce the amount of cash available for other purposes; and
- limiting our flexibility to plan for, or react to, changes in our business;

Our business may not generate sufficient funds, and we may otherwise be unable to maintain sufficient cash reserves, to pay amounts due to SFJ, and our cash needs may increase in the future.

We have granted SFJ a first priority security interest in all of our assets other than our intellectual property and the license agreements to which we are a party. If we are unable to meet our payment obligations to SFJ, SFJ may exercise its remedies as a holder of a first priority security interest, which would result in a loss of our assets and our business would be materially harmed.

Our indebtedness could limit the cash flow available for our operations, expose us to risks that could adversely affect our business, financial condition and results of operations and impair our ability to satisfy our obligations under the Convertible Notes.

We incurred an aggregate of $520.0 million of indebtedness as a result of the issuance of convertible notes in September 2019 and May 2020, or the Convertible Notes, of which an aggregate of approximately $393.9 million is outstanding and held by third parties as of the date of this Quarterly Report on Form 10-Q. We issued approximately 3.9 million shares of our common stock in exchange for approximately $126.1 million of 2019 Convertible Notes in January 2021. We may also incur additional indebtedness to meet future financing needs. Our indebtedness could have significant negative consequences for our security holders and our business, results of operations and financial condition by, among other things:

- increasing our vulnerability to adverse economic and industry conditions;
- limiting our ability to obtain additional financing;
- requiring the dedication of a substantial portion of our cash flow from operations to service our indebtedness, which will reduce the amount of cash available for other purposes;
- limiting our flexibility to plan for, or react to, changes in our business;
- diluting the interests of our existing stockholders as a result of issuing shares of our common stock upon conversion of the Convertible Notes; and
- placing us at a possible competitive disadvantage with competitors that are less leveraged than us or have better access to capital.

Our business may not generate sufficient funds, and we may otherwise be unable to maintain sufficient cash reserves, to pay amounts due under the Convertible Notes, and our cash needs may increase in the future.

Servicing the Convertible Notes will require a significant amount of cash, and we may not have sufficient cash flow from our business to make payments on the Convertible Notes.

Our ability to make scheduled payments of the principal of, to pay interest on or to refinance the Convertible Notes depends on our future performance, which is subject to economic, financial, competitive and other factors beyond our control. Our business may not generate cash flow from operations in the future sufficient to service the Convertible Notes. If we are unable to generate cash flow, we may be required to adopt one or more alternatives, such as selling assets, restructuring debt or obtaining additional equity capital on terms that may be unfavorable to us or highly dilutive. Our ability to refinance our indebtedness will depend on the capital markets and our financial condition at the time we seek to refinance such indebtedness. We may not be able to engage in any of these activities or engage in these activities on desirable terms, which could result in a default on our debt obligations.

We may not have the ability to raise the funds necessary to settle conversions of the Convertible Notes in cash or to repurchase the Convertible Notes upon a fundamental change, and our future debt may contain limitations on our ability to pay cash upon conversion or repurchase of the Convertible Notes.

Holders of the Convertible Notes have the right to require us to repurchase all or a portion of their Convertible Notes upon the occurrence of a fundamental change at a price equal to the principal amount of the Convertible Notes to be repurchased, plus accrued and unpaid interest. In addition, upon conversion of the Convertible Notes, unless we elect to deliver solely shares of our common stock to settle such conversion (other than paying cash in lieu of delivering any fractional share), we will be required to make cash payments in respect of the Convertible Notes being converted. However, we may not have enough available cash or be able to obtain financing at the time we are required to make repurchases of Convertible Notes surrendered therefor or Convertible Notes being converted. In addition, our ability to repurchase the Convertible Notes or to pay cash upon conversions of the Convertible Notes may be limited by law, by regulatory authority or by agreements governing our future indebtedness. Our failure to repurchase Convertible

Notes at a time when the repurchase is required by the indenture or to pay any cash payable on future conversions of the Convertible Notes as required by the indenture would constitute a default under the indenture. A default under the indenture or the fundamental change itself could also lead to a default under agreements governing our existing or future indebtedness. If the repayment of the related indebtedness were to be accelerated after any applicable notice or grace periods, we may not have sufficient funds to repay the indebtedness and repurchase the Convertible Notes or make cash payments upon conversions thereof.

The conditional conversion feature of the Convertible Notes, if triggered, may adversely affect our financial condition and operating results.

In the event the conditional conversion feature of the Convertible Notes is triggered, holders of Convertible Notes will be entitled to convert the Convertible Notes at any time during specified periods at their option. If one or more holders elect to convert their Convertible Notes, unless we elect to satisfy our conversion obligation by delivering solely shares of our common stock (other than paying cash in lieu of delivering any fractional share), we would be required to settle a portion or all of our conversion obligation in cash, which could adversely affect our liquidity. In addition, even if holders do not elect to convert their Convertible Notes, we could be required under applicable accounting rules to reclassify all or a portion of the outstanding principal amount of the Convertible Notes as a current rather than long-term liability, which would result in a material reduction of our net working capital.

The accounting method for convertible debt securities could have a material effect on our reported financial results.

In May 2008, the Financial Accounting Standards Board, or FASB, issued FASB Staff Position No. APB 14-1, Accounting for Convertible Debt Instruments That May Be Settled in Cash Upon Conversion (Including Partial Cash Settlement), which has subsequently been codified as Accounting Standards Codification 470-20, Debt with Conversion and Other Options, or ASC 470-20. Under ASC 470-20, an entity must separately account for the liability and equity components of the convertible debt instruments (such as the Convertible Notes) that may be settled entirely or partially in cash upon conversion in a manner that reflects the issuer's economic interest cost. The effect of ASC 470-20 on the accounting for the Convertible Notes is that the equity component is required to be included in the additional paid-in capital section of stockholders' equity on our consolidated balance sheet, and the value of the equity component was treated as original issue discount for purposes of accounting for the debt component of the Convertible Notes. As a result, we have been required to record a greater amount of non-cash interest expense in current periods presented as a result of the amortization of the discounted carrying value of the Convertible Notes to their face amount over the term of the Convertible Notes. We have reported lower net income in our financial results because ASC 470-20 required interest to include both the current period's amortization of the debt discount and the instrument's coupon interest, which may have adversely affect our reported financial results, the trading price of our common stock and the trading price of the Convertible Notes.

In addition, under certain circumstances, convertible debt instruments (such as the Convertible Notes) that may be settled entirely or partly in cash are currently accounted for utilizing the treasury stock method, the effect of which is that the shares issuable upon conversion of the Convertible Notes are not included in the calculation of diluted earnings per share except to the extent that the conversion value of the Convertible Notes exceeds their principal amount. Under the treasury stock method, for diluted earnings per share purposes, the transaction is accounted for as if the number of shares of common stock that would be necessary to settle such excess, if we elected to settle such excess in shares, are issued. We cannot be sure that the accounting standards in the future will continue to permit the use of the treasury stock method. If we are unable to use the treasury stock method in accounting for the shares issuable upon conversion of the Convertible Notes, then our diluted earnings per share would be adversely affected.

In August 2020, the FASB issued ASU 2020-06, *Debt – Debt with Conversion and Other Options (Subtopic 470-20)* to reduce complexity in applying GAAP to certain financial instruments with characteristics of liability and equity. The Company early adopted this statement effective January 1, 2021 using the modified retrospective method. The impact of the adoption of the statement is to increase debt and decrease equity by the amount of the equity component of convertible notes recognized in equity and to decrease the interest expense by the non-cash portion of the discount amortization. Adoption of this statement or other changes to the current accounting standards related to the Convertible Notes could decrease our weighted average basic earnings per share or other financial metrics, but we do not believe that the adoption of this statement will have a materially adverse impact upon our financial statements.

Risks Related to the Discovery, Development and Commercialization of Our Product Candidates

There are no approved therapies that act by inhibiting C3, and we may not be able to successfully develop and commercialize pegcetacoplan or other product candidates.

Pegcetacoplan is a novel therapeutic compound and its potential benefit in controlling complement-dependent autoimmune and inflammatory diseases has not been established. Pegcetacoplan is designed to control disease through inhibition of C3. There are no approved therapies for our target indications that act by inhibiting C3 and only two approved therapies that act by inhibiting the

complement system. As a result, pegcetacoplan may not demonstrate in patients any or all of the pharmacological benefits we believe it may possess. We have not yet obtained marketing approval of any product candidate. We have evaluated pegcetacoplan in preclinical studies and in clinical trials and have advanced pegcetacoplan into Phase 3 clinical development in geographic atrophy, or GA, and PNH. We have submitted an NDA to the FDA and a MAA to the EMA for pegcetacoplan for the treatment of PNH in September 2020, but we have not obtained regulatory approval to sell pegcetacoplan, APL-9 or any product based on our therapeutic approaches.

If we are unsuccessful in our development efforts, we may not be able to advance the development of pegcetacoplan or any other product candidate, commercialize products, raise capital, expand our business or continue our operations.

The COVID-19 pandemic may affect our ability to initiate and complete preclinical studies and conduct our ongoing clinical trials, delay the initiation of planned and future clinical trials, disrupt regulatory activities, or have other adverse effects on our business and operations. In addition, this pandemic has adversely impacted economies worldwide, which could result in adverse effects on our business and operations.

The COVID-19 pandemic has caused many governments to implement measures to slow the spread of the outbreak through quarantines, strict travel restrictions, heightened border scrutiny, and other measures. The outbreak and government measures taken in response have had a significant impact, both direct and indirect, on businesses and commerce, as worker shortages have occurred; supply chains have been disrupted; facilities and production have been suspended; and demand for certain goods and services, such as medical services and supplies, has spiked, while demand for other goods and services, such as travel, has fallen. The future progression of the pandemic and its effects on our business and operations are uncertain.

We have re-opened our facilities on a limited basis with strict guidelines, but most of our employees continue to work remotely.

We have enrolled, and seek to enroll, patients in our ongoing clinical trials at sites located both in the United States and internationally. We may face difficulties recruiting and retaining patients in our ongoing clinical trials because of logistical effects arising from the pandemic, including increased difficulty for patients and health care providers to travel to or access clinical sites. If patients enrolled in our clinical trials are unable or unwilling to visit clinical trial sites, the data generated by the trials and the timing of completion of our clinical trials may be adversely affected particularly in clinical trials like DERBY and OAKS where patients are expected to travel to clinical sites on a monthly basis over an extended period of time. We may also face disruptions related to the ability to obtain necessary regulatory, institutional review board, or IRB, or other necessary site approvals, as well as other delays at clinical trial sites. In particular, site initiation, participant recruitment and enrollment, participant dosing, availability of drug product or clinical and laboratory supplies, distribution of clinical trial materials, study monitoring, and data analysis may be paused or delayed due to changes in hospital or university policies, federal, state or local regulations, prioritization of hospital resources toward pandemic efforts, or other reasons related to the pandemic. The potential suspension of clinical trial activity at clinical trial sites may have an adverse impact on our clinical trial plans and timelines.

Treatment with complement inhibitors, like pegcetacoplan and APL-9, has immunosuppressive effects. Elderly patients or patients with significantly compromised health, such as those in our clinical trials, could be more susceptible to infections and other complications as a result of treatment with complement inhibitors. The COVID-19 pandemic could lead to delayed enrollment in our trials, more frequent missed visits from ongoing trials and more frequent or severe adverse events during our trials.

We also may face disruptions as a result of the COVID-19 pandemic that affect our ability to procure items that are essential for our research and development activities, including, for example, raw materials used in the manufacturing of our product candidates and laboratory and clinical supplies for our clinical trials. If we experience supply issues, our clinical trial plans and business operations could be adversely affected.

The response to the COVID-19 pandemic may redirect resources with respect to regulatory and intellectual property matters in a way that would adversely impact our ability to progress regulatory approvals and protect our intellectual property. In addition, we may face impediments to regulatory meetings and in obtaining regulatory approvals due to measures intended to limit in-person interactions which could adversely impact the ability of regulatory authorities to take all steps needed to grant regulatory approval and could cause regulatory authorities to defer action on our regulatory submissions, including limitations or delays of inspections of facilities by regulatory authorities, which may impact approval timelines.

Any negative impact that the COVID-19 pandemic has on recruiting or retaining patients in our clinical trials or on the ability of our suppliers to provide materials for our product candidates could cause additional delays to clinical trial activities, which could materially and adversely affect our ability to obtain regulatory approval for and to commercialize our product candidates, increase our operating expenses, affect our ability to raise additional capital, and have a material adverse effect on our financial results.

The COVID-19 pandemic has significantly impacted economies worldwide, which could result in adverse effects on our business and operations. We cannot be certain what the overall impact of the continuation or worsening of the COVID-19 pandemic may be on our business. It has the potential to adversely affect our business, financial condition, results of operations, and prospects.

We are dependent on the successful development and commercialization of our lead product candidate, pegcetacoplan***. If we are unable to develop, obtain marketing approval for or successfully commercialize pegcetacoplan, either alone or through a collaboration, or if we experience significant delays in doing so, our business could be harmed.***

We currently have no products approved for sale and are investing a significant portion of our efforts and financial resources to fund the development of pegcetacoplan. We submitted an NDA to the FDA and a MAA to the EMA for pegcetacoplan for the treatment of PNH in September 2020. The FDA accepted the NDA and set the PDUFA target action date for May 14, 2021. The EMA validated the MAA in October 2020, with the potential for a European Commission decision on the MAA in the second half of 2021. Pursuant to our agreement with Sobi, we have granted to Sobi the exclusive right to commercialize systemic pegcetacoplan outside the United States. Our prospects are substantially dependent on our ability, or that of Sobi or any future collaborator, to develop, obtain marketing approval for and successfully commercialize pegcetacoplan in one or more disease indications. All of our other product candidates are in early stages of clinical development.

The success of pegcetacoplan will depend on several factors, including the following:

- successful recruitment of patients, enrollment in and completion of our ongoing and planned clinical trials;
- initiation and successful recruitment of patients, enrollment in and completion of additional clinical trials;
- safety, tolerability and efficacy profiles that are satisfactory to the FDA, EMA or any comparable foreign regulatory authority for marketing approval;
- our ability to identify success criteria and endpoints for our clinical trials and otherwise design our clinical trials such that the FDA, EMA, and other regulatory authorities will be able to determine the clinical efficacy and safety profile of any product candidates we may develop;
- timely receipt of marketing approvals from applicable regulatory authorities;
- the extent of any required post-marketing approval commitments to applicable regulatory authorities;
- establishment of supply arrangements with third-party suppliers and manufacturers of raw materials and drug intermediates;
- establishment of arrangements with third-party manufacturers to obtain finished products that are appropriately packaged for sale;
- obtaining pegcetacoplan drug product from third-party manufacturers of sufficient quality to be used in our clinical trials and for commercial sale;
- developing, validating and maintaining a commercially viable manufacturing process that is compliant with current good manufacturing practices, or cGMPs;
- the performance of Sobi and any future collaborators;
- obtaining and maintaining patent, trade secret protection and regulatory exclusivity, both in the United States and internationally;
- protection of our rights in our intellectual property portfolio;
- successful launch of commercial sales following any marketing approval;
- an acceptable safety profile following any marketing approval;
- commercial acceptance of our products, if approved, by patients, the medical community and third-party payors;
- our ability to compete with other therapies; and
- obtaining and maintaining healthcare coverage and adequate reimbursement.

Many of these factors are beyond our control, including clinical development, the regulatory submission process, potential threats to our intellectual property rights and the manufacturing, marketing and sales efforts of our collaborators, including Sobi. If we

are unable to develop, receive marketing approval for and successfully commercialize pegcetacoplan on our own or with a collaborator, or experience delays as a result of any of these factors or otherwise, our business could be substantially harmed.

If clinical trials of our product candidates fail to satisfactorily demonstrate safety and efficacy to the FDA and other regulators, we, may incur additional costs or experience delays in completing, or ultimately be unable to complete, the development and commercialization of these product candidates.

We are not permitted to commercialize, market, promote or sell any product candidate in the United States without obtaining marketing approval from the FDA. Foreign regulatory authorities, such as the EMA, impose similar requirements. Prior to September 2020, we had not previously submitted or similar drug approval filings to comparable foreign regulatory authorities for any of our product candidates. In September 2020, we submitted an NDA to the FDA and a MAA to the EMA for pegcetacoplan for the treatment of PNH. We may never receive such approvals. We must complete extensive preclinical development and clinical trials to demonstrate the safety and efficacy of our product candidates in humans before we will be able to obtain these approvals.

Clinical testing is expensive, is difficult to design and implement, can take many years to complete and is inherently uncertain as to outcome. We cannot guarantee that any clinical trials will be conducted as planned or completed on schedule, if at all. The clinical development of our product candidates is susceptible to the risk of failure inherent at any stage of product development, including failure to demonstrate efficacy in a clinical trial or across a broad population of patients, the occurrence of adverse events that are severe or medically or commercially unacceptable, failure to comply with protocols or applicable regulatory requirements and determination by the FDA or any comparable foreign regulatory authority that a product candidate may not continue development or is not approvable. It is possible that even if one or more of our product candidates has a beneficial effect, that effect will not be detected during clinical evaluation as a result of one or more of a variety of factors, including the size, duration, design, measurements, conduct or analysis of our clinical trials. Conversely, as a result of the same factors, our clinical trials may indicate an apparent positive effect of a product candidate that is greater than the actual positive effect, if any. Similarly, in our clinical trials we may fail to detect toxicity or intolerability caused by our product candidates, or mistakenly believe that our product candidates are toxic or not well tolerated when that is not in fact the case. Many companies in the pharmaceutical and biotechnology industries have suffered significant setbacks in late-stage clinical trials after achieving positive results in earlier development, and we cannot be certain that we will not face additional setbacks. It is possible that any of our development programs may be placed on full or partial clinical hold by regulatory authorities at any point, which would delay and possibly prevent further development of our product candidates.

In October 2018, we announced that we voluntarily implemented a pause in dosing in our Phase 3 clinical program in patients with GA due to observed cases of non-infectious inflammation in patients treated from a single manufacturing lot of pegcetacoplan ophthalmological drug product. We also voluntarily implemented a pause in our Phase 1b/2 trial of pegcetacoplan in patients with wet AMD, which we subsequently discontinued. A total of eight patients, four in the Phase 3 GA program and four in our Phase 1b/2 clinical trial of pegcetacoplan in patients with wet AMD, were treated with pegcetacoplan from this manufacturing lot and each patient developed non-infectious inflammation. Inflammation in all eight patients completely resolved. We modified our manufacturing process in order to eliminate an impurity in the active pharmaceutical ingredient and have manufactured sufficient supply of pegcetacoplan utilizing the modified manufacturing process to conduct the Phase 3 GA program. In March 2019, we restarted enrollment of our Phase 3 clinical program in GA, and we announced that we completed enrollment in July 2020.

In March 2021 we decided not to pursue additional development of APL-9 for the treatment of severe COVID-19. The decision followed an interim review of mortality data from the Phase 1/2 study by an independent data monitoring committee, or the DMC, which found no meaningful reduction in the overall mortality rate in patients treated with APL-9 in combination with standard of care therapy compared to standard of care alone. No safety signals were observed by the DMC.

Any inability to successfully complete preclinical and clinical development could result in additional costs to us and impair our ability to generate revenues from product sales, regulatory and commercialization milestones and royalties. Moreover, if we are required to conduct additional clinical trials or other testing of our product candidates beyond the trials and testing that we contemplate, if we are unable to successfully complete clinical trials of our product candidates or other testing or the results of these trials or tests are unfavorable, uncertain or are only modestly favorable, or there are unacceptable safety concerns associated with our product candidates, we may:

- incur additional unplanned costs;
- be delayed in obtaining marketing approval for our product candidates;
- not obtain marketing approval at all;
- obtain approval for indications or patient populations that are not as broad as intended or desired;

- obtain approval with labeling that includes significant use or distribution restrictions or significant safety warnings, including boxed warnings;
- be subject to additional post-marketing testing or other requirements; or
- be required to remove the product from the market after obtaining marketing approval.

Under our collaboration with Sobi, we are relying on Sobi to conduct certain clinical trials of pegcetacoplan and seek regulatory approval for pegcetacoplan outside the United States. If Sobi or any future collaborator are unable to successfully complete clinical trials of our product candidates and obtain regulatory approvals on a timely basis, or at all, our ability to generate revenues from product sales, regulatory and commercialization milestones and royalties may be materially impaired.

In addition, investigators for our clinical trials and other service providers may serve as scientific advisors or consultants to us from time to time and receive compensation in connection with such services, including equity awards and option grants, and may have other financial interests in our company. We are required to collect and provide financial disclosure notifications or certifications for our clinical investigators to the FDA. If the FDA concludes that a financial relationship between us and a clinical investigator has created a conflict of interest or otherwise affected interpretation of the trial, the FDA may question the integrity of the data generated at the applicable clinical trial site and the utility of the clinical trial itself may be jeopardized. This could result in a delay in approval, or rejection, of our marketing applications by the FDA and may ultimately lead to the denial of marketing approval of our current and future product candidates.

Our failure to successfully complete clinical trials of our product candidates and to demonstrate the efficacy and safety necessary to obtain regulatory approval to market any of our product candidates would significantly harm our business.

Adverse events or undesirable side effects caused by, or other unexpected properties of, any of our product candidates may be identified during development that could delay or prevent their marketing approval or limit their use.

Adverse events or undesirable side effects caused by, or other unexpected properties of, our product candidates could cause us, or any collaborator conducting clinical trials of our product candidates such as Sobi, an institutional review board or regulatory authorities to interrupt, delay or halt clinical trials of one or more of our product candidates and could result in a more restrictive label, or the delay or denial of marketing approval by the FDA or comparable foreign regulatory authorities. For example, by design pegcetacoplan has immunosuppressive effects and, in some cases, may be administered to patients with underlying significantly compromised health. Administration of our product candidates could make patients more susceptible to infection.

In addition, in preclinical studies of pegcetacoplan, we observed evidence of minimal to mild kidney toxicity when animals were administered relatively higher doses of pegcetacoplan than the doses we intend to use in the treatment of patients. We believe this kidney toxicity is likely associated with the presence of polyethylene glycol, or PEG, which is a component of pegcetacoplan. If such kidney toxicity, or other adverse effects, were to arise in patients being treated with pegcetacoplan or any other of our product candidates, it could require us to halt, delay or interrupt clinical trials of such product candidate or adversely affect our ability to obtain requisite approvals to advance the development and commercialization of such product candidate.

In our Phase 2 trial of pegcetacoplan in patients with GA, the most frequently reported adverse events were associated with the injection procedure in the study eye. These adverse events included two cases of confirmed endophthalmitis, which is inflammation in the eye typically caused by infection, and one case of presumed endophthalmitis where the culture tested negative for bacterial growth. In addition, during the 12-month treatment period and the subsequent six-month period during which no treatment was administered, we observed a higher incidence of new onset exudation, or fluid leakage in the retinas of eyes in which exudation had not previously been reported, in the study eyes treated with pegcetacoplan, predominantly in patients with a history of wet AMD in the non-study eye, or fellow eye. Specifically, we observed that, after the 12-month treatment period and the six-month monitoring period, 21% of patients who received administration of pegcetacoplan every month and 9% of patients who received administration of pegcetacoplan every other month showed new onset exudation in the study eye as compared to 1% of the sham group. As we continue development of pegcetacoplan for GA, if a significant number of patients develop new onset exudation, then we may need to limit development of ophthalmological pegcetacoplan to certain uses or subpopulations in which the undesirable side effects or other characteristics are less prevalent, less severe or more acceptable from a risk-benefit perspective.

In our Phase 3 clinical trial of pegcetacoplan in patients with GA and our Phase 1b/2 clinical trial of pegcetacoplan in patients with wet AMD, several patients treated from a single manufacturing lot of pegcetacoplan ophthalmological drug product experienced non-infectious inflammation. A total of eight patients, four in our Phase 3 GA program and four in our Phase 1b/2 clinical trial of pegcetacoplan in patients with wet AMD, were treated with this pegcetacoplan from this manufacturing lot and each patient developed non-infectious inflammation. Inflammation in these patients has completely resolved.

In our Phase 3 PEGASUS trial, the safety profile observed in the pegcetacoplan was comparable to the safety profile observed in the eculizumab arm and consistent with previously reported data. After the 48-week study period, twenty-four of 80 pegcetacoplan monotherapy-treated patients (30%) experienced a serious adverse event (SAE). Five of the SAEs (6%) were assessed to be possibly related to study treatment. No cases of meningitis were reported. One death was reported due to COVID-19 and was unrelated to study treatment. The most common adverse events (AEs) reported throughout the study were injection site reactions (36%), hemolysis (24%), and diarrhea (21%). Twelve out of 80 patients (15%) discontinued due to adverse events, with five discontinuations due to hemolysis. Sixty-four of the 67 patients (96%) who completed the open-label period opted to enter the extension study.

If any of our product candidates is associated with adverse events or undesirable side effects or has properties that are unexpected, we, or our collaborators, may abandon development or limit development of that product candidate to certain uses or subpopulations in which the undesirable side effects or other characteristics are less prevalent, less severe or more acceptable from a risk-benefit perspective. Many compounds that initially showed promise in clinical or earlier stage testing have later been found to cause undesirable or unexpected side effects that prevented further development of the compound.

In addition, clinical trials by their nature utilize a sample of the potential patient population. However, with a limited number of subjects and limited duration of exposure, rare and severe side effects of our product candidates may only be uncovered when a significantly larger number of patients are exposed to the product. If safety problems occur or are identified after one of our products reaches the market, the FDA or comparable non-U.S. regulatory authorities may require that we amend the labeling of our product, recall our product, or even withdraw approval for our product.

If we, or any collaborator conducting clinical trials of any of our product candidates such as Sobi, experience any of a number of possible unforeseen events in connection with clinical trials of our product candidates, potential clinical development, marketing approval or commercialization of our product candidates could be delayed or prevented.

We, or our collaborators, may experience numerous unforeseen events during, or as a result of, clinical trials that could delay or prevent clinical development, marketing approval or commercialization of our product candidates, including:

- clinical trials of our product candidates may produce unfavorable or inconclusive results;
- we, or our collaborators, may decide, or regulators may require us or them, to conduct additional clinical trials or abandon product development programs;
- the number of patients required for clinical trials of our product candidates may be larger than we, or our collaborators, anticipate, patient enrollment in these clinical trials may be slower than we, or our collaborators, anticipate or participants may drop out of these clinical trials at a higher rate than we, or our collaborators, anticipate;
- the cost of planned clinical trials of our product candidates may be greater than we anticipate;
- our third-party contractors or those of our collaborators, including those manufacturing our product candidates or components or ingredients thereof or conducting clinical trials on our behalf or on behalf of our collaborators, may deviate from the trial protocol, fail to comply with regulatory requirements or fail to meet their contractual obligations to us or our collaborators in a timely manner or at all;
- regulators or institutional review boards may not authorize us, our collaborators or our or their investigators to commence a clinical trial or conduct a clinical trial at a prospective trial site;
- we, or our collaborators, may have delays in reaching or fail to reach agreement on acceptable clinical trial contracts or clinical trial protocols with prospective trial sites;
- patients that enroll in a clinical trial may misrepresent their eligibility to do so or may otherwise not comply with the clinical trial protocol, resulting in the need to drop the patients from the clinical trial, increase the needed enrollment size for the clinical trial or extend the clinical trial’s duration;
- we, or our collaborators, may have to delay, suspend or terminate clinical trials of our product candidates for various reasons, including a finding that the participants are being exposed to unacceptable health risks, undesirable side effects or other unexpected characteristics of the product candidate;
- regulators or institutional review boards may require that we, or our collaborators, or our or their investigators suspend or terminate clinical research for various reasons, including noncompliance with regulatory requirements or their standards of conduct, a finding that the participants are being exposed to unacceptable health risks, undesirable side effects or other unexpected characteristics of the product candidate or findings of undesirable effects caused by a chemically or mechanistically similar product or product candidate;

- the FDA or comparable foreign regulatory authorities may disagree with our, or our collaborators', clinical trial designs or our or their interpretation of data from preclinical studies and clinical trials, including the data from our PEGASUS trial;
- the FDA or comparable foreign regulatory authorities may fail to approve or subsequently find fault with the manufacturing processes or facilities of third-party manufacturers with which we, or our collaborators, enter into agreements for clinical and commercial supplies;
- the supply or quality of raw materials, drug intermediates or manufactured product candidates, other products evaluated in our clinical trials or other materials necessary to conduct clinical trials of our product candidates may be insufficient, inadequate or not available at an acceptable cost, or we may experience interruptions in supply; and
- the approval policies or regulations of the FDA or comparable foreign regulatory authorities may significantly change in a manner rendering our clinical data insufficient to obtain marketing approval.

Should the COVID-19 pandemic persist, our clinical development plans could be affected, and we may be unable to conduct our clinical trials in the manner or on the timelines that we currently anticipate. Clinical trial participants and clinical investigators may not be able to comply with clinical trial protocols, if for example quarantines or other travel limitations impede participant movement, affect sponsor access to study sites, or interrupt healthcare services. The COVID-19 pandemic could lead to delayed enrollment in our trials, more frequent missed appointments and withdrawals from ongoing trials and more frequent or severe adverse events during our trials.

Product development costs for us will increase if we experience delays in testing or pursuing marketing approvals and we may be required to obtain additional funds to complete clinical trials and prepare for possible commercialization of our product candidates. We do not know whether any preclinical tests or clinical trials will begin as planned, will need to be restructured, or will be completed on schedule or at all. Significant preclinical study or clinical trial delays also could shorten any periods during which we, or our collaborators, may have the exclusive right to commercialize our product candidates or allow our competitors, or the competitors of our collaborators, to bring products to market before we, or our collaborators, do and impair our ability, or the ability of our collaborators, to successfully commercialize our product candidates and may harm our business and results of operations. In addition, many of the factors that lead to clinical trial delays may ultimately lead to the denial of marketing approval of any of our product candidates.

If we, or any collaborator conducting clinical trials of any of our product candidates such as Sobi, experience delays or difficulties in the enrollment of patients in clinical trials, our or their receipt of necessary regulatory approvals could be delayed or prevented.

We, or our collaborators, may not be able to initiate or continue clinical trials for any of our product candidates if we, or they, are unable to locate and enroll a sufficient number of eligible patients to participate in clinical trials as required by the FDA or comparable foreign regulatory authorities. Patient enrollment is a significant factor in the timing of clinical trials, and is affected by many factors, including:

- the size and nature of the patient population;
- the severity of the disease under investigation;
- the proximity of patients to clinical sites;
- the patient referral practices of physicians;
- the eligibility criteria for the trial;
- the design of the clinical trial;
- efforts to facilitate timely enrollment;
- competing clinical trials; and
- clinicians' and patients' perceptions as to the potential advantages and risks of the drug being studied in relation to other available therapies, including any new drugs that may be approved for the indications we are investigating.

Many of the indications for which we are developing product candidates are rare diseases with small patient populations, and many of those patients are treated with other therapies or products. Further, there are only a limited number of specialist physicians that regularly treat patients with these rare diseases and major clinical centers that support such treatment are concentrated in a few geographic regions. In addition, other companies are conducting clinical trials and have announced plans for future clinical trials that are seeking, or are likely to seek, to enroll patients with these rare diseases and patients are generally only able to enroll in a single trial at a time. Both patients and their physicians may be reluctant to forgo, discontinue or otherwise alter existing, approved life-saving therapeutic approaches. Given the severe and life-threatening nature of these indications and the expectation that many patients will be on treatment with other therapies or products, we may encounter difficulty in recruiting a sufficient number of patients for our trials including in particular our planned clinical trials. The small population of patients, competition for these patients, the nature of the disease and limited trial sites may make it difficult for us to enroll enough patients to complete our clinical trials of pegcetacoplan in a timely and cost-effective manner. These difficulties may be exacerbated as a result of the ongoing COVID-19 pandemic.

Our inability, or the inability of our collaborators, to enroll a sufficient number of patients for our, or their, clinical trials could result in significant delays or may require us or them to abandon one or more clinical trials altogether. Enrollment delays in our, or their, clinical trials may result in increased development costs for our product candidates, delay or halt the development of and approval processes for our product candidates and jeopardize our, or our collaborators', ability to commence sales of and generate revenues from our product candidates, which could cause the value of our company to decline and limit our ability to obtain additional financing, if needed.

Results of preclinical studies and Phase 1 and Phase 2 clinical trials may not be predictive of results of later clinical trials.

The outcome of preclinical studies and Phase 1 and Phase 2 clinical trials may not be predictive of the success of later clinical trials, and preliminary or interim results of clinical trials do not necessarily predict final results. Many companies in the pharmaceutical and biotechnology industries have suffered significant setbacks in late-stage clinical trials after achieving positive results in earlier stages of clinical development, and we could face similar setbacks. Similarly, the design of a clinical trial can determine whether its results will support approval of a product and flaws in the design of a clinical trial may not become apparent until the clinical trial is well advanced.

In addition, preclinical and clinical data are often susceptible to varying interpretations and analyses. Many companies that believed their product candidates performed satisfactorily in preclinical studies and clinical trials have nonetheless failed to obtain marketing approval for the product candidates. Even if we, or our collaborators, believe that the results of clinical trials for our product candidates warrant marketing approval, the FDA or comparable foreign regulatory authorities may disagree and may not grant marketing approval of our product candidates.

Some of the data we present on the use of pegcetacoplan for the treatment of GA is drawn from *post hoc* analyses of data subsets from our Phase 2 clinical trial. While we believe these data were useful in informing the design of future Phase 3 clinical trials for pegcetacoplan, *post hoc* analyses performed after unmasking trial results can result in the introduction of bias and may not be predictive of success in Phase 3 clinical trials.

In some instances, there can be significant variability in safety or efficacy results between different clinical trials of the same product candidate due to numerous factors, including changes in trial procedures set forth in protocols, differences in the size and type of the patient populations, changes in and adherence to the dosing regimen and other clinical trial protocols and the rate of dropout among clinical trial participants. For instance, the Phase 3 clinical trials in GA are similar in design to the Phase 2 clinical trial, except that patients will be treated with pegcetacoplan for 24 months rather than 12 months and there will not be a six-month monitoring period following treatment. Additionally, unlike the Phase 2 clinical trial, GA lesion size will be measured by total area rather than mean change in the square root of GA lesion size. In our Phase 3 clinical trials in GA, statistical significance is set at a p-value of 0.05 or less, meaning that there is a 1-in-20 or less probability that the observed results occurred by chance rather than as a treatment effect. In our Phase 2 clinical trial, we set statistical significance as a p-value of 0.1 or less, meaning that there is a 1-in-10 or less probability that the observed results occurred by chance. If we fail to receive positive results in clinical trials of our product candidates, the development timeline and regulatory approval and commercialization prospects for our most advanced product candidates, and, correspondingly, our business and financial prospects would be negatively impacted.

If we fail to develop and commercialize other product candidates, we may be unable to grow our business.

Although the development and commercialization of pegcetacoplan is our primary focus, as part of our growth strategy, we are developing a pipeline of product candidates for the treatment of complement-dependent diseases. These other product candidates will require additional, time-consuming and costly development efforts prior to commercial sale, including preclinical studies, clinical trials and approval by the FDA and/or applicable foreign regulatory authorities. All product candidates are prone to the risks of failure that are inherent in pharmaceutical product development, including the possibility that the product candidate will not be shown to be

sufficiently safe and effective for approval by regulatory authorities. In addition, there can be no assurance that any such products that are approved will be manufactured or produced economically, successfully commercialized or widely accepted in the marketplace or be more effective than other commercially available alternatives.

We have never obtained marketing approval for a product candidate and we may be unable to obtain, or may be delayed in obtaining, marketing approval for any of our product candidates.

We have never obtained marketing approval for a product candidate. We submitted an NDA to the FDA and a MAA to the EMA for pegcetacoplan for the treatment of PNH in September 2020. The FDA may not approve our NDA and the EMA may not approve or EMA for pegcetacoplan for the treatment of PNH. Although the FDA set the PDUFA target action date and the EMA validated the MAA in Europe, it is possible that the FDA or EMA may refuse to accept for substantive review any NDAs or MAAs that we submit for our product candidates. In addition, the FDA and EMA or may conclude after review of our data that our application is insufficient to obtain marketing approval of pegcetacoplan. If the FDA or EMA does not accept or approve our NDA or MAA for pegcetacoplan, it may require that we conduct additional clinical trials, preclinical studies or manufacturing validation studies and submit that data before it will reconsider our applications. Depending on the extent of these or any other required trials or studies, approval of any NDA, MAA or application that we submit may be delayed by several years or may require us to expend more resources than we have available. It is also possible that additional trials or studies, if performed and completed, may not be considered sufficient by the FDA or EMA to approve our NDAs or MAAs.

The FDA has advised us that, for the approval of a new treatment for PNH, hemoglobin stabilization in conjunction with change in transfusion dependence constitute accepted clinical benefit, but that a rise in hemoglobin levels may not translate to clinical benefit in patients who entered the trial with high hemoglobin levels, such as permitted by the inclusion criteria of the PEGASUS trial, and who do not require transfusions. We believe that the data from the PEGASUS trial support a finding of clinical benefit. We submitted an NDA to the FDA and an MAA to the EMA for pegcetacoplan for the treatment of PNH in September 2020.

Any delay in obtaining, or an inability to obtain, marketing approvals would prevent us from commercializing pegcetacoplan in the United States or EU, generating revenues and achieving and sustaining profitability. If any of these outcomes occur, either to pegcetacoplan or to any future product candidate for which we may seek marketing approval, we may be forced to abandon our development efforts for pegcetacoplan or such future product candidates, which could significantly harm our business.

Even if pegcetacoplan or one of our other product candidates that we develop receives marketing approval, we or others may later discover that the product is less effective than previously believed or causes undesirable side effects that were not previously identified, which could compromise our ability, or that of our collaborators, to market the product.

Clinical trials of our product candidates are conducted in carefully defined sets of patients who have agreed to enter into clinical trials. Consequently, it is possible that our clinical trials, or those of our collaborators, may indicate an apparent positive effect of a product candidate that is greater than the actual positive effect, if any, or alternatively fail to identify undesirable side effects. If, following approval of a product candidate, we, or others, discover that the product is less effective than previously believed or causes undesirable side effects that were not previously identified, any of the following adverse events could occur:

- regulatory authorities may withdraw their approval of the product or seize the product;
- we, or our collaborators, may be required to recall the product, change the way the product is administered or conduct additional clinical trials;
- additional restrictions may be imposed on the marketing of, or the manufacturing processes for, the particular product;
- we may be subject to fines, injunctions or the imposition of civil or criminal penalties;
- regulatory authorities may require the addition of labeling statements, such as a “black box” warning or a contraindication;
- we, or our collaborators, may be required to create a Medication Guide outlining the risks of the previously unidentified side effects for distribution to patients;
- we, or our collaborators, could be sued and held liable for harm caused to patients;
- the product may become less competitive; and
- our reputation may suffer.

Any of these events could harm our business and operations and could negatively impact our stock price.

Even if pegcetacoplan or one of our other product candidates that we develop receives marketing approval, it may fail to achieve the degree of market acceptance by physicians, patients, third-party payors and others in the medical community necessary for commercial success, in which case we may not generate significant revenues or become profitable.

We have never commercialized a product, and even if pegcetacoplan or one of our other product candidates that we develop is approved by the appropriate regulatory authorities for marketing and sale, it may nonetheless fail to gain sufficient market acceptance by physicians, patients, third-party payors and others in the medical community. Physicians are often reluctant to switch their patients from existing therapies even when new and potentially more effective or convenient treatments enter the market. Further, patients often acclimate to the therapy that they are currently taking and do not want to switch unless their physicians recommend switching products or they are required to switch therapies due to lack of reimbursement for existing therapies. Eculizumab (marketed as Soliris) and ravulizumab (marketed as Ultomiris) are the only therapies that have been approved for the treatment of PNH, and even if we are able to obtain marketing approval of pegcetacoplan for the treatment of PNH, we may not be able to successfully convince physicians or patients to switch from eculizumab or ravulizumab to pegcetacoplan. This may be particularly true with respect to eculizumab as many in the medical community believe that patients with PNH on eculizumab may experience sudden and excessive blood cell lysis, or rupture, leading to anemia, blood clots and other medical problems, when they stop receiving eculizumab. In addition, even if we are able to demonstrate our product candidates' safety and efficacy to the FDA and other regulators, safety concerns in the medical community may hinder market acceptance.

Efforts to educate the medical community and third-party payors on the benefits of our product candidates may require significant resources and may not be successful. If pegcetacoplan or any of our other product candidates that we develop is approved but does not achieve an adequate level of market acceptance, we may not generate significant revenues and we may not become profitable. The degree of market acceptance of pegcetacoplan or our other product candidates that we develop, if approved for commercial sale, will depend on a number of factors, including:

- the efficacy and safety of the product;
- the potential advantages of the product compared to competitive therapies;
- the prevalence and severity of any side effects;
- the clinical indications for which the product is approved;
- whether the product is designated under physician treatment guidelines as a first-, second- or third-line therapy;
- the price at which the product is offered for sale;
- the product's convenience and ease of administration compared to alternative treatments;
- the willingness of the target patient population to try, and of physicians to prescribe, the product;
- limitations or warnings, including distribution or use restrictions contained in the product's approved labeling;
- the strength of sales, marketing and distribution support;
- the approval of other new products for the same indications;
- the timing of market introduction of our approved products as well as competitive products;
- adverse publicity about the product or favorable publicity about competitive products;
- potential product liability claims;
- changes in the standard of care for the targeted indications for the product; and
- availability and amount of coverage and reimbursement from government payors, managed care plans and other third-party payors.

In addition, the potential market opportunity for pegcetacoplan in any indication is difficult to precisely estimate. Our estimates of the potential market opportunity for pegcetacoplan include several key assumptions based on our industry knowledge, industry publications, third-party research reports and other surveys. However, no independent source has verified such assumptions. If any of these assumptions proves to be inaccurate, then the actual market for pegcetacoplan could be smaller than our estimates of potential market opportunity. If the actual market for pegcetacoplan is smaller than we expect, our product revenue may be limited, and it may be more difficult for us to achieve or maintain profitability.

We may expend our limited resources to pursue a particular product candidate or indication and fail to capitalize on product candidates or indications that may be more profitable or for which there is a greater likelihood of success.

Because we have limited financial and managerial resources, we intend to focus on developing product candidates for specific indications that we identify as most likely to succeed, in terms of both their potential for marketing approval and commercialization. As a result, we may forego or delay pursuit of opportunities with other product candidates or for other indications that may prove to have greater commercial potential.

Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or profitable market opportunities. Our spending on current and future research and development programs and product candidates for specific indications may not yield any commercially viable product candidates. If we do not accurately evaluate the commercial potential or target market for a particular product candidate, we may relinquish valuable rights to that product candidate through collaboration, licensing or other royalty arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to the product candidate.

If we are unable to establish sales, marketing and distribution capabilities or enter into sales, marketing and distribution arrangements with third parties, we may not be successful in commercializing any product candidates if approved.

We are currently developing sales, marketing and distribution infrastructure in the United States to support commercialization of systemic pegcetacoplan for the treatment of PNH and worldwide infrastructure for our other product candidates, including pegcetacoplan as a treatment for GA.

We are building focused capabilities to commercialize development programs for certain indications where we believe that the medical specialists for the indications such as PNH are sufficiently concentrated to allow us to effectively promote the product with a targeted sales team. The development of sales, marketing and distribution capabilities requires substantial resources, is time-consuming and could delay any product launch. If the commercial launch of a product candidate for which we recruit a sales force and establish marketing and distribution capabilities is delayed or does not occur for any reason, we could have prematurely or unnecessarily incurred these commercialization costs. This may be costly, and our investment could be lost if we cannot retain or reposition our sales and marketing personnel. In addition, we may not be able to hire or retain a sales force in the United States that is sufficient in size or has adequate expertise in the medical markets that we plan to target. If we are unable to establish or retain a sales force and marketing and distribution capabilities, our operating results may be adversely affected. If a potential partner has development or commercialization expertise that we believe is particularly relevant to one of our products, then we may seek to collaborate with that potential partner even if we believe we could otherwise develop and commercialize the product independently.

In certain indications, we may seek to enter into collaborations that we believe may contribute to our ability to advance development and ultimately commercialize our product candidates. We may also seek to enter into collaborations where we believe that realizing the full commercial value of our development programs will require access to broader geographic markets or the pursuit of broader patient populations or indications. As a result of entering into arrangements with third parties to perform sales, marketing and distribution services, our product revenues or the profitability of these product revenues may be lower, perhaps substantially lower, than if we were to directly market and sell products in those markets. Furthermore, we may be unsuccessful in entering into the necessary arrangements with third parties or may be unable to do so on terms that are favorable to us. In addition, we may have little or no control over such third parties, and any of them may fail to devote the necessary resources and attention to sell and market our products effectively.

If we do not establish sales, marketing and distribution capabilities, either on our own or in collaboration with third parties, we will not be successful in commercializing any of our product candidates that receive marketing approval.

We have granted exclusive commercialization rights for systemic pegcetacoplan outside of the United States to Sobi under our agreement with Sobi. If Sobi is unable to meet its contractual obligations, we may be forced to focus our efforts internally to commercialize systemic pegcetacoplan outside of the United States without the assistance of a commercialization partner or seek another commercialization partner, either of which would result in us incurring greater expenses and could cause a delay in market penetration while we expand our commercial operations or seek an alternative commercialization partner. Such costs may exceed the increased revenues we would receive from direct systemic pegcetacoplan sales outside of the United States, at least in the near term. We would also be forced to declare a breach of the agreement with Sobi and seek a termination of the agreement which could result in an extended and uncertain dispute with Sobi, including arbitration or litigation, any of which would be costly.

We face substantial competition, which may result in others discovering, developing or commercializing products before or more successfully than we do.

The development and commercialization of new products is highly competitive, as described in "Competition," above. We expect that we, and our collaborators, will face significant competition from major pharmaceutical companies, specialty pharmaceutical companies and biotechnology companies worldwide with respect to any of our product candidates that we, or our collaborators, may seek to develop or commercialize in the future, including from therapies that act through the complement system and therapies that use different approaches.

Our competitors may succeed in developing, acquiring or licensing technologies and products that are more effective, have fewer side effects or more tolerable side effects or are less costly than any product candidates that we are currently developing or that we may develop, which could render our product candidates obsolete and noncompetitive.

Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize products that are safer, more effective, have fewer or less severe side effects, are more convenient or are less expensive than any products that we, or our collaborators, may develop. Our competitors also may obtain FDA or other marketing approval for their products before we, or our collaborators, are able to obtain approval for ours, which could result in our competitors establishing a strong market position before we, or our collaborators, are able to enter the market.

Many of our existing and potential future competitors have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining marketing approvals and marketing approved products than we do. Mergers and acquisitions in the pharmaceutical and biotechnology industries may result in even more resources being concentrated among a smaller number of our competitors. Smaller or early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These competitors also compete with us in recruiting and retaining qualified scientific and management personnel and establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, the development of our product candidates.

If the FDA or comparable foreign regulatory authorities approve generic versions of any of our products that receive marketing approval, or such authorities do not grant our products appropriate periods of data exclusivity before approving generic versions of our products, the sales of our products could be adversely affected.

Once an NDA is approved, the product covered thereby becomes a "reference-listed drug" in the FDA's publication, "Approved Drug Products with Therapeutic Equivalence Evaluations," or the Orange Book. Manufacturers may seek approval of generic versions of reference-listed drugs through submission of an ANDA in the United States. In support of an ANDA, a generic manufacturer need not conduct clinical trials. Rather, the applicant generally must show that its product has the same active ingredient(s), dosage form, strength, route of administration and conditions of use or labeling as the reference-listed drug and that the generic version is bioequivalent to the reference-listed drug, meaning it is absorbed in the body at the same rate and to the same extent. Generic products may be significantly less costly to bring to market than the reference-listed drug and companies that produce generic products are generally able to offer them at lower prices. Thus, following the introduction of a generic drug, a significant percentage of the sales of any branded product or reference-listed drug may be typically lost to the generic product.

The FDA may not approve an ANDA for a generic product until any applicable period of non-patent exclusivity for the reference-listed drug has expired. The Federal Food, Drug, and Cosmetic Act, or FDCA, provides a period of five years of non-patent exclusivity for a new drug containing a new chemical entity, or NCE. Specifically, in cases where such exclusivity has been granted, an ANDA may not be filed with the FDA until the expiration of five years unless the submission is accompanied by a Paragraph IV certification that a patent covering the reference-listed drug is either invalid or will not be infringed by the generic product, in which case the applicant may submit its application four years following approval of the reference-listed drug. It is unclear whether the FDA will treat the active ingredients in our product candidates as NCEs and, therefore, afford them five years of NCE data exclusivity if they are approved. If any product we develop does not receive five years of NCE exclusivity, the FDA may approve generic versions of such product three years after its date of approval, subject to the requirement that the ANDA applicant certifies to any patents listed for our products in the Orange Book. Manufacturers may seek to launch these generic products following the expiration of the applicable marketing exclusivity period, even if we still have patent protection for our product.

Competition that our products may face from generic versions of our products could negatively impact our future revenue, profitability and cash flows and substantially limit our ability to obtain a return on our investments in those product candidates.

Even if we, or any collaborator that is commercializing any of our product candidates such as Sobi, are able to commercialize any product candidate, the product may become subject to unfavorable pricing regulations, third-party payor reimbursement practices or healthcare reform initiatives, any of which could harm our business.

The commercial success of our product candidates will depend substantially, both domestically and abroad, on the extent to which the costs of our product candidates will be paid by third-party payors, including government health administration authorities and private health coverage insurers. If coverage and reimbursement is not available, or reimbursement is available only to limited levels, we, or our collaborators, may not be able to successfully commercialize our product candidates. Even if coverage is provided, the approved reimbursement amount may not be high enough to allow us, or our collaborators, to establish or maintain pricing sufficient to realize a sufficient return on our or their investments. In the United States, no uniform policy of coverage and reimbursement for products exists among third-party payors and coverage and reimbursement for products can differ significantly from payor to payor. As a result, the coverage determination process is often a time-consuming and costly process that will require us to provide scientific and clinical support for the use of our products to each payor separately, with no assurance that coverage and adequate reimbursement will be applied consistently or obtained in the first instance.

There is significant uncertainty related to third-party payor coverage and reimbursement of newly approved drugs. Marketing approvals, pricing and reimbursement for new drug products vary widely from country to country. Some countries require approval of the sale price of a drug before it can be marketed. In many countries, the pricing review period begins after marketing or product licensing approval is granted. In some foreign markets, prescription pharmaceutical pricing remains subject to continuing governmental control even after initial approval is granted. As a result, we, or our collaborators, might obtain marketing approval for a product in a particular country, but then be subject to price regulations that delay commercial launch of the product, possibly for lengthy time periods, which may negatively impact the revenues we are able to generate from the sale of the product in that country. Adverse pricing limitations may hinder our ability or the ability of our collaborators to recoup our or their investment in one or more product candidates, even if our product candidates obtain marketing approval.

Patients who are provided medical treatment for their conditions generally rely on third-party payors to reimburse all or part of the costs associated with their treatment. Therefore, our ability, and the ability of our collaborators, to commercialize any of our product candidates will depend in part on the extent to which coverage and reimbursement for these products and related treatments will be available from third-party payors. Third-party payors decide which medications they will cover and establish reimbursement levels. The healthcare industry is acutely focused on cost containment, both in the United States and abroad. Government authorities and other third-party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular medications, which could affect our ability or that of our collaborators to sell our product candidates profitably. These payors may not view our products, if any, as cost-effective, and coverage and reimbursement may not be available to our customers, or those of our collaborators, or may not be sufficient to allow our products, if any, to be marketed on a competitive basis. Cost-control initiatives could cause us, or our collaborators, to decrease the price we, or they, might establish for products, which could result in lower than anticipated product revenues. If the prices for our products, if any, decrease or if governmental and other third-party payors do not provide coverage or adequate reimbursement, our prospects for revenue and profitability will suffer.

The commercial potential of our products depends in part on reimbursement by government health administration authorities, private health insurers and other organizations. If we, or any collaborator that is commercializing our product candidates such as Sobi are unable to obtain coverage or reimbursement for our products, as monotherapy or in combination with other therapies, including possible combinations with eculizumab or ravulizumab, at the levels anticipated, our financial condition could be harmed. Additionally, if new compounds currently in development by potential competitors, including biosimilars of eculizumab or ravulizumab, obtain marketing approval, there may be downward pressure on reimbursement levels for therapies in our target disease areas, which could have a negative impact on our ability to achieve and maintain profitability.

There may also be delays in obtaining coverage and reimbursement for newly approved drugs, and coverage may be more limited than the indications for which the drug is approved by the FDA or comparable foreign regulatory authorities. Moreover, eligibility for reimbursement does not imply that any drug will be paid for in all cases or at a rate that covers our costs, including research, development, manufacture, sale and distribution. Reimbursement rates may vary, by way of example, according to the use of the product and the clinical setting in which it is used. Reimbursement rates may also be based on reimbursement levels already set for lower cost drugs or may be incorporated into existing payments for other services.

In addition, increasingly, third-party payors are requiring higher levels of evidence of the benefits and clinical outcomes of new technologies and are challenging the prices charged. We cannot be sure that coverage will be available for any product candidate that we, or any collaborator, including Sobi, commercialize and, if available, that the reimbursement rates will be adequate. Further, the net reimbursement for drug products may be subject to additional reductions if there are changes to laws that presently restrict imports of drugs from countries where they may be sold at lower prices than in the United States. An inability to promptly obtain coverage and adequate payment rates from both government-funded and private payors for any of our product candidates for which we, or our

collaborator, obtain marketing approval could significantly harm our operating results, our ability to raise capital needed to commercialize products and our overall financial condition.

Product liability lawsuits against us could divert our resources, cause us to incur substantial liabilities and limit commercialization of any products that we may develop.

We face an inherent risk of product liability claims as a result of the clinical testing of our product candidates despite obtaining appropriate informed consents from our clinical trial participants. We will face an even greater risk if we or our collaborators including Sobi, commercially sell any product that we may develop. For example, we may be sued if any product we develop allegedly causes injury or is found to be otherwise unsuitable during clinical testing, manufacturing, marketing or sale. Any such product liability claims may include allegations of defects in manufacturing, defects in design, a failure to warn of dangers inherent in the product, negligence, strict liability or a breach of warranties. Claims could also be asserted under state consumer protection acts. If we cannot successfully defend ourselves against product liability claims, we may incur substantial liabilities or be required to limit commercialization of our product candidates. Regardless of the merits or eventual outcome, liability claims may result in:

- decreased demand for our product candidates or products that we may develop;
- injury to our reputation and significant negative media attention;
- withdrawal of clinical trial participants;
- significant costs to defend resulting litigation;
- substantial monetary awards to trial participants or patients;
- loss of revenue;
- reduced resources of our management to pursue our business strategy; and
- the inability to commercialize any products that we may develop.

Although we maintain product liability insurance coverage in the amount of up to $20.0 million in the aggregate and clinical trial liability insurance of up to $20.0 million in the aggregate, this insurance may not fully cover potential liabilities that we may incur. The cost of any litigation or other proceeding, even if resolved in our favor, could be substantial. We will need to increase our insurance coverage if we commercialize any product that receives marketing approval. In addition, insurance coverage is becoming increasingly expensive. If we are unable to maintain sufficient insurance coverage at an acceptable cost or to otherwise protect against potential product liability claims, it could prevent or inhibit the development and commercial production and sale of our product candidates, which could harm our business, financial condition, results of operations and prospects.

Our internal information systems, or those of any contractors, consultants, vendors, business partners or other third parties, may fail or suffer security breaches, which could result in a material disruption of our product development programs.

We collect, store and transmit large amounts of confidential information, including personal information and information relating to intellectual property, on internal information systems and through the information systems of our contractors, consultants, vendors, business partners or other third parties.

Despite the implementation of security measures, our internal information systems and those of third parties are vulnerable to damage from computer viruses, malware, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failures. Such systems are also vulnerable to service interruptions or to security breaches from inadvertent or intentional actions by our employees, our collaborators, contractors, consultants, vendors, business partners and other third parties, or from cyber-attacks by malicious third parties over the Internet or through other mechanisms. Cyber-attacks are increasing in their frequency, sophistication and intensity, and have become increasingly difficult to detect. Cyber-attacks could include the deployment of harmful malware, ransomware, denial of service attacks, social engineering and other means to affect service reliability and threaten the confidentiality, integrity and availability of information. Cyber-attacks also could include phishing attempts or e-mail fraud to cause payments or information to be transmitted to an unintended recipient.

While we have not experienced any such material system failure, accident, cyber-attack or security breach to date, if such an event were to occur and cause interruptions in our operations, it could result in a material disruption of our development programs, clinical trials and business operations, whether due to a loss of our trade secrets or other proprietary information or other similar disruptions, in addition to possibly requiring substantial expenditures of resources to remedy. For example, the loss of clinical trial data from clinical trials could result in delays or termination of our regulatory approval efforts and significantly increase our costs to

recover or reproduce the data. In addition, as risks with respect to our information systems continue to evolve, we will incur additional costs to maintain the security of our information systems and comply with evolving laws and regulations pertaining to cybersecurity and related areas. To the extent that any disruption or security breach were to result in a loss of, or damage to, our data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur liability, including regulatory fines and other losses with respect to privacy claims, enrollment in our clinical trials could be negatively affected, our competitive position and reputation could be harmed and the further development and commercialization of our product candidates could be delayed.

Risks Related to Our Dependence on Third Parties

We rely on third parties to conduct our clinical trials. If they do not perform satisfactorily, our business could be harmed.

We do not independently conduct clinical trials of our product candidates. We rely, and expect to continue to rely, on third parties, such as contract research organizations, clinical data management organizations, medical institutions and clinical investigators, to conduct our clinical trials of pegcetacoplan and any other product candidate that we develop. Any of these third parties may terminate their engagements with us under certain circumstances. We may not be able to enter into alternative arrangements or do so on commercially reasonable terms. In addition, there is a natural transition period when a new contract research organization begins work. As a result, delays would likely occur, which could negatively impact our ability to meet our expected clinical development timelines and harm our business, financial condition and prospects.

Further, although our reliance on these third parties for clinical development activities limits our control over these activities, we remain responsible for ensuring that each of our trials is conducted in accordance with the applicable protocol, legal, regulatory and scientific standards. For example, notwithstanding the obligations of a contract research organization for a trial of one of our product candidates, we remain responsible for ensuring that each of our clinical trials is conducted in accordance with the general investigational plan and protocols for the trial. Moreover, the FDA requires us to comply with standards, commonly referred to as current Good Clinical Practices, or cGCPs, for conducting, recording and reporting the results of clinical trials to assure that data and reported results are credible and accurate and that the rights, integrity and confidentiality of trial participants are protected. The FDA enforces these cGCPs through periodic inspections of trial sponsors, principal investigators, clinical trial sites and institutional review boards. If we or our third-party contractors fail to comply with applicable cGCPs, the clinical data generated in our clinical trials may be deemed unreliable and the FDA may require us to perform additional clinical trials before approving our product candidates, which would delay the marketing approval process. We cannot be certain that, upon inspection, the FDA will determine that any of our clinical trials comply with cGCPs. Similar regulatory requirements apply outside the United States, including the International Council for Harmonisation of Technical Requirements for the Registration of Pharmaceuticals for Human Use, or ICH. We are also required to register clinical trials and post the results of completed clinical trials on a government-sponsored database, ClinicalTrials.gov, within certain timeframes. Failure to do so can result in fines, adverse publicity and civil and criminal sanctions.

Furthermore, the third parties conducting clinical trials on our behalf are not our employees, and except for remedies available to us under our agreements with such contractors, we cannot control whether or not they devote sufficient time, skill and resources to our ongoing development programs. These contractors may also have relationships with other commercial entities, including our competitors, for whom they may also be conducting clinical trials or other drug development activities, which could impede their ability to devote appropriate time to our clinical programs. In addition, these contractors may be adversely affected by the COVID-19 pandemic. If these third parties do not successfully carry out their contractual duties, meet expected deadlines or conduct our clinical trials in accordance with regulatory requirements or our stated protocols, we may not be able to obtain, or may be delayed in obtaining, marketing approvals for our product candidates. If that occurs, we will not be able to, or may be delayed in our efforts to, successfully commercialize our product candidates. In such an event, our financial results and the commercial prospects for any product candidates that we seek to develop could be harmed, our costs could increase and our ability to generate revenues could be delayed, impaired or foreclosed.

We contract with third parties for the manufacture, storage and distribution of our product candidates for clinical trials and expect to continue to do so in connection with our future development and commercialization efforts. This reliance on third parties increases the risk that we will not have sufficient quantities of pegcetacoplan or our other product candidates or such quantities at an acceptable cost, which could delay, prevent or impair our development or commercialization efforts.

We currently have no manufacturing facilities, and a relatively small number of personnel with manufacturing experience who can oversee the manufacturing process. We rely on contract manufacturers to manufacture, store and distribute both drug substance and drug product required for our clinical trials. We plan to continue to rely upon contract manufacturers, and, potentially collaboration partners, to manufacture commercial quantities of our products, if approved. We may be unable to establish any

agreements with contract manufacturers or to do so on acceptable terms, or to maintain such agreements as we may enter. Even if we are able to establish agreements with contract manufacturers, reliance on contract manufacturers entails additional risks, including:

- manufacturing delays if our third-party contractors give greater priority to the supply of other products over our product candidates or otherwise do not satisfactorily perform according to the terms of the agreements between us and them, or if unforeseen events in the manufacturing process arise;
- the possible termination or nonrenewal of agreements by our third-party contractors at a time that is costly or inconvenient for us;
- the possible breach by the third-party contractors of our agreements with them;
- the failure of third-party contractors to comply with applicable regulatory requirements;
- the possible mislabeling of clinical supplies, potentially resulting in the wrong dose amounts being supplied or active drug or placebo not being properly identified;
- the possibility of clinical supplies not being delivered to clinical sites on time, leading to clinical trial interruptions, or of drug supplies not being distributed to commercial vendors in a timely manner, resulting in lost sales; and
- the possible misappropriation of our proprietary information, including our trade secrets and know-how.

We currently rely, and expect to continue to rely, on a small number of third-party contract manufacturers to supply most of our supply of active pharmaceutical ingredients and required finished product for our preclinical studies and clinical trials. In particular, we have entered into commercial supply agreements with Bachem Americas, Inc., or Bachem, and NOF Corporation, or NOF, to purchase a significant portion of our requirements for the pegcetacoplan drug substance and drug intermediates, respectively, over the next five years. We have also entered into long-term commercial supply agreements with other suppliers of raw materials, drug intermediates, drug substance and drug product. If any of our existing manufacturers should become unavailable to us for any reason, we may incur delays in identifying or qualifying replacements. We also rely on other third parties to store and distribute drug supplies for our clinical trials. Any performance failure on the part of our contract manufacturers or distributors could delay clinical development or marketing approval of our product candidates or commercialization of any resulting products, producing additional losses and depriving us of potential product revenue. For example, in the past we experienced issues associated with the manufacturing process for pegcetacoplan that resulted in delays in the supply of pegcetacoplan. These delays resulted in us incurring additional costs and delays in our PNH development program. Additionally, in October 2018, we announced that we voluntarily implemented a pause in dosing in our clinical trials in patients with GA and wet AMD due to observed cases of non-infectious inflammation in patients treated from a single manufacturing lot of pegcetacoplan ophthalmological drug product that we believe occurred due to an impurity in the active pharmaceutical ingredient. If we experience other issues or delays in the future, our development of pegcetacoplan may be materially delayed and our business adversely affected.

Any manufacturing problem, the loss of a contract manufacturer or any loss of storage could be disruptive to our operations, delay our clinical trials and, if our products are approved for sale, result in lost sales. Accordingly, for example, if Bachem or NOF were to experience manufacturing and supply issues, we would have difficulty in procuring the drug intermediates and drug substance needed for the supply and manufacture of pegcetacoplan. Additionally, we rely on third parties to supply the raw materials needed to manufacture our product candidates. Any reliance on suppliers may involve several risks, including a potential inability to obtain critical materials and reduced control over production costs, delivery schedules, reliability and quality. If we or any third-party parties on which we rely are adversely impacted by restrictions or limitations resulting from the COVID-19 pandemic, our ability to manufacture and supply pegcetacoplan may be disrupted, which would limit our ability to conduct our clinical trials or prepare for our commercial launch. Any unanticipated disruption to our contract manufacturing caused by problems at suppliers could delay shipment of our product candidates, increase our cost of goods sold and result in lost sales with respect to any approved products.

If any of our product candidates are approved by any regulatory agency, we will need to enter into agreements with third-party contract manufacturers for the commercial production and distribution of those products. It may be difficult for us to reach agreement with a contract manufacturer on satisfactory terms or in a timely manner. In addition, we may face competition for access to manufacturing facilities as there are a limited number of contract manufacturers operating under cGMPs that can manufacture our product candidates. Consequently, we may not be able to reach agreement with third-party manufacturers on satisfactory terms, which could delay our commercialization efforts.

Third-party manufacturers are required to comply with cGMPs and similar regulatory requirements outside the United States, such as the ICH. Facilities used by our third-party manufacturers must be approved by the FDA after we submit an NDA and before potential approval of the product candidate. Similar regulations apply to manufacturers of our product candidates for use or sale in foreign countries. We do not control the manufacturing process and are completely dependent on our third-party manufacturers for compliance with the applicable regulatory requirements for the manufacture of our product candidates. If our manufacturers cannot successfully manufacture material that conforms to our specifications or the strict regulatory requirements of the FDA and any applicable foreign regulatory authority, they may not be able to meet our supply requirements for clinical and commercial operations and to secure the applicable approval for their manufacturing facilities. If these facilities are not approved for commercial manufacture, we may need to find alternative manufacturing facilities, which could result in delays in obtaining approval for the applicable product candidate.

In addition, our manufacturers are subject to ongoing periodic inspections by the FDA and corresponding state and foreign agencies for compliance with cGMPs and similar regulatory requirements both prior to and following the receipt of marketing approval for any of our product candidates. Some of these inspections may be unannounced. Failure by any of our manufacturers to comply with applicable cGMPs or other regulatory requirements could result in sanctions being imposed on us, including fines, injunctions, civil penalties, delays, suspensions or withdrawals of approvals, operating restrictions, interruptions in supply and criminal prosecutions, any of which could significantly impact the available supplies of our product candidates and harm our business, financial condition and results of operations.

We are developing a custom, on-body drug delivery system that would enable patients to self-administer pegcetacoplan through subcutaneous infusion. While this device is in development, we plan to use one or more commercially available ambulatory infusion pumps in our ongoing and planned clinical trials and for our commercial launch of pegcetacoplan as a treatment for PNH. The development of a custom drug delivery system may be delayed, or we may not be successful in developing a custom drug delivery system and may need to continue to rely on commercially available ambulatory infusion pumps. Any reliance on third-party infusion pumps may involve several risks, including reduced control over costs, delivery schedules, reliability and quality.

Our current and anticipated future dependence upon others for the manufacture of our product candidates may harm our future profit margins and our ability to commercialize any products that receive marketing approval on a timely and competitive basis.

Our prospects for the development and commercialization of our product candidates will depend in significant part on the success of our collaboration with Sobi and future collaborations.

We have entered into a collaboration with Sobi for the global co-development and commercialization outside of the United States of systemic pegcetacoplan and we may seek to enter into additional collaborations for the development and commercialization of certain of our product candidates. We may have limited control over the amount and timing of resources that our collaborators, including Sobi, will dedicate to the development or commercialization of our product candidates. Our ability to generate revenues from these arrangements will depend on our collaborators' abilities to successfully perform the functions assigned to them in these arrangements. In addition, our collaborators may have the right to abandon research or development projects and terminate applicable agreements, including funding obligations, prior to or upon the expiration of the agreed upon terms.

Collaborations involving our product candidates pose a number of risks, including the following:

- collaborators have significant discretion in determining the efforts and resources that they will apply to these collaborations;
- collaborators may not perform their obligations as expected;
- collaborators may not pursue development and commercialization of our product candidates or may elect not to continue or renew development or commercialization programs, based on clinical trial results, changes in the collaborators' strategic focus or available funding or external factors, such as an acquisition, that divert resources or create competing priorities;
- collaborators may delay clinical trials, provide insufficient funding for a clinical trial program, stop a clinical trial or abandon a product candidate, repeat or conduct new clinical trials or require a new formulation of a product candidate for clinical testing;
- collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with our product candidates;
- a collaborator with marketing and distribution rights to one or more products may not commit sufficient resources to the marketing and distribution of such product or products;

- disagreements with collaborators, including disagreements over proprietary rights, contract interpretation or the preferred course of development, might cause delays or termination of the research, development or commercialization of product candidates, might lead to additional responsibilities for us with respect to product candidates, or might result in litigation or arbitration, any of which would be time-consuming and expensive;
- collaborators may not properly maintain or defend our intellectual property rights or may use our proprietary information in such a way as to invite litigation that could jeopardize or invalidate our intellectual property or proprietary information or expose us to potential litigation;
- collaborators may infringe the intellectual property rights of third parties, which may expose us to litigation and potential liability;
- disputes may arise between the collaborators and us regarding ownership of or other rights in the intellectual property generated in the course of the collaborations; and
- collaborations may be terminated and, if terminated, may result in a need for additional capital to pursue further development or commercialization of the applicable product candidates.

For example, our agreement with Sobi is subject to early termination in the event of any uncured material breach of the agreement or under specific circumstances relating to insolvency. If we do not maintain a productive collaborative relationship with Sobi or if Sobi is unable to meet its contractual obligations or if there is an early termination of the agreement as described above, we would be forced to either establish a commercial infrastructure outside of the United States so that we could undertake the commercialization efforts which had been theretofore undertaken by Sobi or we would need to seek an alternative collaborator. The establishment of a commercial infrastructure and assumption by us of commercialization activities outside of the United States would require substantial resources, financial and otherwise, and could result in us incurring greater expenses than the increase in revenues from our direct sales of systemic pegcetacoplan. It could also cause a delay in market penetration while we expand our commercial operations. Seeking and obtaining an alternative collaborator outside the United States could also adversely impact sales of systemic pegcetacoplan and market penetration outside of the United States.

Collaboration agreements may not lead to development or commercialization of product candidates in the most efficient manner or at all. If our collaborators, including Sobi are involved in a business combination, it could decide to delay, diminish or terminate the development or commercialization of any product candidate licensed to it by us.

We have in the past established, and in the future, may seek to establish, additional collaborations and, if we are not able to establish them on commercially reasonable terms, we may have to alter our development and commercialization plans.

We entered into the collaboration agreement with Sobi in October 2020 concerning the development and commercialization of pegcetacoplan and specified other structurally and functionally similar compstatin analogues or derivatives for use systemically or for local non-ophthalmic administration. We may seek to establish one or more additional collaborators for the development and commercialization of one or more of our product candidates. Likely collaborators may include large and mid-size pharmaceutical companies, regional and national pharmaceutical companies and biotechnology companies. In addition, if we are able to obtain marketing approval for product candidates from foreign regulatory authorities, we intend to enter into strategic relationships with international biotechnology or pharmaceutical companies for the commercialization of such product candidates outside of the United States.

We face significant competition in seeking appropriate collaborators. Whether we reach a definitive agreement for a collaboration will depend, among other things, upon our assessment of the collaborator’s resources and expertise, the terms and conditions of the proposed collaboration and the proposed collaborator’s evaluation of a number of factors. Those factors may include the potential differentiation of our product candidates from competing product candidates, design or results of clinical trials, the likelihood of approval by the FDA or comparable foreign regulatory authorities and the regulatory pathway for any such approval, the potential market for the product candidate, the costs and complexities of manufacturing and delivering the product to patients and the potential of competing products. The collaborator may also consider alternative product candidates or technologies for similar indications that may be available for collaboration and whether such a collaboration could be more attractive than the one with us for our product candidate. If we elect to increase our expenditures to fund development or commercialization activities on our own, we may need to obtain additional capital, which may not be available to us on acceptable terms or at all. If we do not have sufficient funds, we may not be able to further develop our product candidates or bring them to market and generate product revenue.

Collaborations are complex and time-consuming to negotiate and document. Further, there have been a significant number of recent business combinations among large pharmaceutical companies that have resulted in a reduced number of potential future collaborators.

Any collaboration agreements that we enter into in the future may contain restrictions on our ability to enter into potential collaborations or to otherwise develop specified product candidates. Our collaboration agreement with Sobi, we agreed not to, directly or indirectly, alone or with or for any other person or entity, conduct any clinical development or commercialization of APL-9 for PNH, cold agglutinin disease, hematopoietic stem cell transplantation thrombotic microangiopathy, C3 glomerulopathy and immune complex membranoproliferative glomerulonephritis, and amyotrophic lateral sclerosis or any other indications subsequently agreed upon by the parties.

We may not be able to negotiate collaborations on a timely basis, on acceptable terms, or at all. If we are unable to do so, we may have to curtail the development of the product candidate for which we are seeking to collaborate, reduce or delay its development program or one or more of our other development programs, delay its potential commercialization or reduce the scope of any sales or marketing activities, or increase our expenditures and undertake development or commercialization activities at our own expense.

Risks Related to Our Intellectual Property

If we fail to comply with our obligations under our existing and any future intellectual property licenses with third parties, we could lose license rights that are important to our business.

We are a party to patent license agreements with The University of Pennsylvania, or Penn, under which we license patent rights relating to a family of compounds for use in all fields. The licensed patent rights include issued U.S. and foreign patents with claims that recite a class of compounds generically covering our lead product candidates, pegcetacoplan and APL-9, and that specifically recite the active component. We may enter into additional license agreements in the future. Our license agreements with Penn impose, and we expect that future license agreements will impose, various diligence, milestone payment, royalty, insurance and other obligations on us. If we fail to comply with our obligations under these licenses, our licensors may have the right to terminate these license agreements, in which event we might not be able to market any product that is covered by these agreements, or our licensors may convert the license to a non-exclusive license, which could negatively impact the value of the product candidate being developed under the license agreement. Termination of these license agreements or reduction or elimination of our licensed rights may also result in our having to negotiate new or reinstated licenses with less favorable terms.

If we are unable to obtain and maintain sufficient patent protection for our product candidates, or if the scope of the patent protection is not sufficiently broad, our competitors could develop and commercialize products similar or identical to ours, and our ability to successfully commercialize our product candidates may be adversely affected.

Our success depends in large part on our ability to obtain and maintain patent protection in the United States and other countries with respect to our proprietary product candidates. If we do not adequately protect our intellectual property rights, competitors may be able to erode or negate any competitive advantage we may have, which could harm our business and ability to achieve profitability. To protect our proprietary position, we file patent applications in the United States and abroad related to our novel product candidates that are important to our business; we also license or purchase patent applications filed by others. The patent application and approval process is expensive and time-consuming. We may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner.

Agreements through which we license patent rights may not give us control over patent prosecution or maintenance, so that we may not be able to control which claims or arguments are presented and may not be able to secure, maintain, or successfully enforce necessary or desirable patent protection from those patent rights. We have not had and do not have primary control over patent prosecution and maintenance for certain of the patents and patent applications we license, and therefore cannot guarantee that these patents and applications will be prosecuted in a manner consistent with the best interests of our business. We cannot be certain that patent prosecution and maintenance activities by our licensors have been or will be conducted in compliance with applicable laws and regulations or will result in valid and enforceable patents.

We, or any partners, collaborators, or licensees, may fail to identify patentable aspects of inventions made in the course of development and commercialization activities before it is too late to obtain patent protection on them. Therefore, we may miss potential opportunities to strengthen our patent position. Moreover, in some circumstances, we might not have the right to control the preparation, filing and prosecution of patent applications, or to maintain the patents, covering any technology that we may license from third parties in the future. These patents and applications may not be prosecuted and enforced in a manner consistent with the

best interests of our business. Our license agreements with Penn provide that Penn has the right under certain circumstances to control the preparation, prosecution and maintenance of the underlying patent rights.

It is possible that defects of form in the preparation or filing of our patents or patent applications may exist, or may arise in the future, for example with respect to proper priority claims, inventorship, claim scope, or patent term adjustments. If we or our partners, collaborators, licensees, or licensors, whether current or future, fail to establish, maintain or protect such patents and other intellectual property rights, such rights may be reduced or eliminated. If our partners, collaborators, licensees, or licensors are not fully cooperative or disagree with us as to the prosecution, maintenance or enforcement of any patent rights, such patent rights could be compromised. If there are material defects in the form, preparation, prosecution, or enforcement of our patents or patent applications, such patents may be invalid and/or unenforceable, and such applications may never result in valid, enforceable patents. Any of these outcomes could impair our ability to prevent competition from third parties, which may have an adverse impact on our business.

The patent position of biotechnology and pharmaceutical companies generally is highly uncertain. No consistent policy regarding the breadth of claims allowed in biotechnology and pharmaceutical patents has emerged to date in the United States or in many foreign jurisdictions. In addition, the determination of patent rights with respect to pharmaceutical compounds commonly involves complex legal and factual questions, which has in recent years been the subject of much litigation. As a result, the issuance, scope, validity, enforceability and commercial value of our patent rights are highly uncertain.

Pending patent applications cannot be enforced against third parties practicing the technology claimed in such applications unless and until a patent issues from such applications. Assuming the other requirements for patentability are met, currently, the first to file a patent application is generally entitled to the patent. However, prior to March 16, 2013, in the United States, the first to invent was entitled to the patent. Publications of discoveries in the scientific literature often lag behind the actual discoveries, and patent applications in the United States and other jurisdictions are typically not published until 18 months after filing, or in some cases not at all. Therefore, we cannot be certain that we were the first to make the inventions claimed in our patents or pending patent applications, or that we were the first to file for patent protection of such inventions. Similarly, we cannot be certain that parties from whom we do or may license or purchase patent rights were the first to make relevant claimed inventions, or were the first to file for patent protection for them. If third parties have filed patent applications on inventions claimed in our patents or applications on or before March 15, 2013, an interference proceeding in the United States can be initiated by such third parties to determine who was the first to invent any of the subject matter covered by the patent claims of our applications. If third parties have filed such applications after March 15, 2013, a derivation proceeding in the United States can be initiated by such third parties to determine whether our invention was derived from theirs.

Moreover, because the issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability, our patents or pending patent applications may be challenged in the courts or patent offices in the United States and abroad. There is no assurance that all of the potentially relevant prior art relating to our patents and patent applications has been found. If such prior art exists, it may be used to invalidate a patent, or may prevent a patent from issuing from a pending patent application. For example, such patent filings may be subject to a third-party preissuance submission of prior art to the U.S. Patent and Trademark Office, or USPTO, or to other patent offices around the world. Alternately or additionally, we may become involved in post-grant review procedures, oppositions, derivations, proceedings, reexaminations, inter partes review or interference proceedings, in the United States or elsewhere, challenging patents or patent applications in which we have rights, including patents on which we rely to protect our business. An adverse determination in any such challenges may result in loss of exclusivity or in patent claims being narrowed, invalidated or held unenforceable, in whole or in part, which could limit our ability to stop others from using or commercializing similar or identical technology and products, or limit the duration of the patent protection of our technology and products. In addition, given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. Furthermore, while it is our policy to require our employees and contractors who may be involved in the conception or development of intellectual property to execute agreements assigning such intellectual property to us, we may be unsuccessful in executing such an agreement with each party who, in fact, conceives or develops intellectual property that we regard as our own. As a result, the inventorship or ownership of our intellectual property may be challenged in the future.

Pending and future patent applications may not result in patents being issued which protect our business, in whole or in part, or which effectively prevent others from commercializing competitive products. Our issued patents or any patents that may issue in the future may be invalidated or interpreted narrowly, such that they fail to provide us with any significant competitive advantage. Changes in either the patent laws or interpretation of the patent laws in the United States and other countries may diminish the value of our patents or narrow the scope of our patent protection. In addition, the laws of foreign countries may not protect our rights to the same extent or in the same manner as the laws of the United States. For example, patent laws in various jurisdictions, including significant commercial markets such as Europe, restrict the patentability of methods of treatment of the human body more than United States law does.

Issued patents that we have or may obtain or license may not provide us with any meaningful protection, prevent competitors from competing with us or otherwise provide us with any competitive advantage. Our competitors may be able to circumvent our patents by developing similar or alternative technologies or products in a non-infringing manner. Our competitors may also seek approval to market their own products similar to or otherwise competitive with our products. Alternatively, our competitors may seek to market generic versions of any approved products by submitting ANDAs to the FDA in which they claim that patents owned or licensed by us are invalid, unenforceable or not infringed. In these circumstances, we may need to defend or assert our patents, or both, including by filing lawsuits alleging patent infringement. In any of these types of proceedings, a court or other agency with jurisdiction may find our patents invalid or unenforceable, or find that our competitors are competing in a non-infringing manner. Thus, even if we have valid and enforceable patents, these patents still may not provide protection against competing products or processes sufficient to achieve our business objectives.

Pursuant to the terms of some of our license agreements with third parties, some of our third-party licensors have the right, but not the obligation in certain circumstances to control enforcement of our licensed patents or defense of any claims asserting the invalidity of these patents. Even if we are permitted to pursue such enforcement or defense, we will require the cooperation of our licensors, and cannot guarantee that we would receive it and on what terms. We cannot be certain that our licensors will allocate sufficient resources or prioritize their or our enforcement of such patents or defense of such claims to protect our interests in the licensed patents. If we cannot obtain patent protection, or enforce existing or future patents against third parties, our competitive position and our financial condition could suffer.

If we are unable to protect the confidentiality of our trade secrets, the value of our technology could be negatively impacted and our business would be harmed.

In addition to the protection afforded by patents, we also rely on trade secret protection for certain aspects of our intellectual property. We seek to protect these trade secrets, in part, by entering into non-disclosure and confidentiality agreements with parties who have access to them, such as our employees, consultants, independent contractors, advisors, contract manufacturers, suppliers and other third parties. We also enter into confidentiality and invention or patent assignment agreements with employees and certain consultants. Any party with whom we have executed such an agreement may breach that agreement and disclose our proprietary information, including our trade secrets, and we may not be able to obtain adequate remedies for such breaches. Enforcing a claim that a party illegally disclosed or misappropriated a trade secret is difficult, expensive and time-consuming, and the outcome is unpredictable. Additionally, if the steps taken to maintain our trade secrets are deemed inadequate, we may have insufficient recourse against third parties for misappropriating the trade secret. Further, if any of our trade secrets were to be lawfully obtained or independently developed by a competitor, we would have no right to prevent such third party, or those to whom they communicate such technology or information, from using that technology or information to compete with us. If any of our trade secrets were to be disclosed to or independently developed by a competitor, our business and competitive position could be harmed.

We may become involved in lawsuits to protect or enforce our patents or other intellectual property, which could be expensive, time consuming and unsuccessful.

Competitors may infringe our patents, trademarks, copyrights or other intellectual property. To counter infringement or unauthorized use, we may be required to file infringement claims, which can be expensive and time consuming and divert the time and attention of our management and scientific personnel. Any claims we assert against perceived infringers could provoke these parties to assert counterclaims against us alleging that we infringe their patents, in addition to counterclaims asserting that our patents are invalid or unenforceable, or both. In any patent infringement proceeding, there is a risk that a court will decide that a patent of ours is invalid or unenforceable, in whole or in part, and that we do not have the right to stop the other party from using the invention at issue. There is also a risk that, even if the validity of such patents is upheld, the court will construe the patent's claims narrowly or decide that we do not have the right to stop the other party from using the invention at issue on the grounds that our patent claims do not cover the invention. An adverse outcome in a litigation or proceeding involving one or more of our patents could limit our ability to assert those patents against those parties or other competitors, and may curtail or preclude our ability to exclude third parties from making and selling similar or competitive products. Similarly, if we assert trademark infringement claims, a court may determine that the marks we have asserted are invalid or unenforceable, or that the party against whom we have asserted trademark infringement has superior rights to the marks in question. In this case, we could ultimately be forced to cease use of such trademarks.

Even if we establish infringement, the court may decide not to grant an injunction against further infringing activity and instead award only monetary damages, which may or may not be an adequate remedy. Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during litigation. There could also be public announcements of the results of hearings, motions or other interim proceedings or developments. If securities analysts or investors perceive these results to be negative, it could adversely affect the price of shares of our common stock. Moreover, there can be no assurance that we will have sufficient financial or other resources to file and pursue such infringement claims, which typically last for years before they are concluded. Even if we ultimately prevail in

such claims, the monetary cost of such litigation and the diversion of the attention of our management and scientific personnel could outweigh any benefit we receive as a result of the proceedings.

If we are sued for infringing intellectual property rights of third parties, such litigation could be costly and time consuming and could prevent or delay us from developing or commercializing our product candidates.

Our commercial success depends, in part, on our ability to develop, manufacture, market and sell our product candidates without infringing the intellectual property and other proprietary rights of third parties. Third parties may have U.S. and non-U.S. issued patents and pending patent applications relating to compounds and methods of use for the treatment of the disease indications for which we are developing our product candidates or relating to the use of complement inhibition that may cover our product candidates or approach to complement inhibition. For example, we are aware of a U.S. patent with claims that could be construed to cover pegcetacoplan. Although we believe that these claims, if construed to cover pegcetacoplan, would be invalid due to various prior art disclosures available more than a year before the priority date of the U.S. patent, there are no assurances that a court would agree. If any third-party patents or patent applications are found to cover our product candidates or their methods of use or our approach to complement inhibition, we may not be free to manufacture or market our product candidates as planned without obtaining a license, which may not be available on commercially reasonable terms, or at all.

There is a substantial amount of intellectual property litigation in the biotechnology and pharmaceutical industries, and we may become party to, or threatened with, litigation or other adversarial proceedings regarding intellectual property rights with respect to our products candidates, including interference proceedings before the USPTO. There may be third-party patents or patent applications with claims to materials, formulations, methods of manufacture or methods for treatment related to the use or manufacture of our product candidates. Because patent applications can take many years to issue, there may be currently pending patent applications which may later result in issued patents that our product candidates may be accused of infringing. In addition, third parties may obtain patents in the future and claim that use of our technologies infringes upon these patents. Accordingly, third parties may assert infringement claims against us based on existing or future intellectual property rights. The outcome of intellectual property litigation is subject to uncertainties that cannot be adequately quantified in advance. The pharmaceutical and biotechnology industries have produced a significant number of patents, and it may not always be clear to industry participants, including us, which patents cover various types of products or methods of use. The coverage of patents is subject to interpretation by the courts, and the interpretation is not always uniform. If we were sued for patent infringement, we would need to demonstrate that our product candidates, products or methods either do not infringe the patent claims of the relevant patent or that the patent claims are invalid or unenforceable, and we may not be able to do this. Proving invalidity is difficult. For example, in the United States, proving invalidity requires a showing of clear and convincing evidence to overcome the presumption of validity enjoyed by issued patents. Even if we are successful in these proceedings, we may incur substantial costs and the time and attention of our management and scientific personnel could be diverted in pursuing these proceedings, which could significantly harm our business and operating results. In addition, we may not have sufficient resources to bring these actions to a successful conclusion.

If we are found to infringe a third party's intellectual property rights, we could be forced, including by court order, to cease developing, manufacturing or commercializing the infringing product candidate or product. Alternatively, we may be required to obtain a license from such third party in order to use the infringing technology and continue developing, manufacturing or marketing the infringing product candidate or product. However, we may not be able to obtain any required license on commercially reasonable terms or at all. Even if we were able to obtain a license, it could be non-exclusive, thereby giving our competitors access to the same technologies licensed to us; alternatively or additionally it could include terms that impede or destroy our ability to compete successfully in the commercial marketplace. In addition, we could be found liable for monetary damages, including treble damages and attorneys' fees if we are found to have willfully infringed a patent. A finding of infringement could prevent us from commercializing our product candidates or force us to cease some of our business operations, which could harm our business. Claims that we have misappropriated the confidential information or trade secrets of third parties could have a similar negative impact on our business.

Some of our intellectual property that was discovered through government-funded programs may be subject to federal regulation such as "march-in" rights, certain reporting requirements, and a preference for U.S. industry. Compliance with such regulations may limit our exclusive rights, subject us to expenditure of resources with respect to reporting requirements and limit our ability to contract with foreign manufacturers.

Some of our in-licensed intellectual property with respect to our product candidates has been funded in part by the U.S. government and, therefore, would be subject to certain federal regulations pursuant to the Bayh-Dole Act of 1980, or the Bayh-Dole Act. As a result, the U.S. government may have certain rights to intellectual property embodied in our current or future product candidates pursuant to the Bayh-Dole Act. The "march-in" provisions of the Bayh-Dole Act allow the U.S. government under strictly limited circumstances to require the patent owners to grant exclusive, partially exclusive or non-exclusive rights to third parties for

intellectual property discovered through the government-funded program. The U.S. government can exercise its march-in rights if it determines that action is necessary because the patent owner fails to achieve practical application of the new invention or because action is necessary to alleviate health concerns or address the safety needs of the public. Intellectual property discovered under the government-funded program is also subject to certain reporting requirements, compliance with which may require us or our licensors to expend substantial resources. Such intellectual property is also subject to a preference for U.S. industry, which may limit our ability to contract with foreign product manufacturers for products covered by such intellectual property. Penn has requested a waiver of the U.S. manufacturing requirement, but there can be no assurance that such waiver will be granted.

Changes to the patent law in the United States and other jurisdictions could diminish the value of patents in general, thereby impairing our ability to protect our products.

As is the case with other biopharmaceutical companies, our success is heavily dependent on intellectual property, particularly patents. Obtaining and enforcing patents in the biopharmaceutical industry involves both technological and legal complexity and is therefore costly, time consuming and inherently uncertain. Recent patent reform legislation in the United States, including the Leahy-Smith America Invents Act, or the America Invents Act, could increase those uncertainties and costs. The America Invents Act was signed into law on September 16, 2011, and many of the substantive changes became effective on March 16, 2013. The America Invents Act reformed United States patent law in part by changing the U.S. patent system from a "first to invent" system to a "first inventor to file" system, expanding the definition of prior art, and developing a post-grant review system. This legislation changes United States patent law in a way that may weaken our ability to obtain patent protection in the United States for those applications filed after March 16, 2013.

Further, the America Invents Act created new procedures to challenge the validity of issued patents in the United States, including post-grant review and inter partes review proceedings, which some third parties have been using to cause the cancellation of selected or all claims of issued patents of competitors. For a patent with an effective filing date of March 16, 2013 or later, a petition for post-grant review can be filed by a third party in a nine-month window from issuance of the patent. A petition for inter partes review can be filed immediately following the issuance of a patent if the patent has an effective filing date prior to March 16, 2013. A petition for inter partes review can be filed after the nine-month period for filing a post-grant review petition has expired for a patent with an effective filing date of March 16, 2013 or later. Post-grant review proceedings can be brought on any ground of invalidity, whereas inter partes review proceedings can only raise an invalidity challenge based on published prior art and patents. These adversarial actions at the USPTO review patent claims without the presumption of validity afforded to U.S. patents in lawsuits in U.S. federal courts, and use a lower burden of proof than used in litigation in U.S. federal courts. Therefore, it is generally considered easier for a competitor or third party to have a U.S. patent invalidated in a USPTO post-grant review or inter partes review proceeding than invalidated in a litigation in a U.S. federal court. If any of our patents are challenged by a third party in such a USPTO proceeding, there is no guarantee that we or our licensors or collaborators will be successful in defending the patent, which would result in a loss of the challenged patent right to us.

The U.S. Supreme Court has ruled on several patent cases in recent years, either narrowing the scope of patent protection available in certain circumstances or weakening the rights of patent owners in certain situations. Additionally, there have been recent proposals for additional changes to the patent laws of the United States and other countries that, if adopted, could impact our ability to enforce our patents. In addition to increasing uncertainty with regard to our ability to obtain patents in the future, this combination of events has created uncertainty with respect to the value of patents once obtained. Depending on future actions by the U.S. Congress, the U.S. courts, the USPTO and the relevant law-making bodies in other countries, the laws and regulations governing patents could change in unpredictable ways that would weaken our ability to obtain new patents or to enforce our existing patents and patents that we might obtain in the future.

We may not be able to enforce our intellectual property rights throughout the world.

Filing, prosecuting and defending patents on our product candidates in all countries throughout the world would be prohibitively expensive, and our intellectual property rights in some countries outside the United States are less extensive than those in the United States. The requirements for patentability may differ in certain countries, particularly in developing countries; thus, even in countries where we do pursue patent protection, there can be no assurance that any patents will issue with claims that cover our products. Competitors may use our technologies in jurisdictions where we have not pursued and obtained patent protection to develop their own products and, further, may export otherwise infringing products to territories where we may obtain patent protection, but where patent enforcement is not as strong as that in the United States. These products may compete with our products in jurisdictions where we do not have any issued or licensed patents and any future patent claims or other intellectual property rights may not be effective or sufficient to prevent them from so competing.

Moreover, our ability to protect and enforce our intellectual property rights may be adversely affected by unforeseen changes in foreign intellectual property laws. Additionally, laws of some countries outside of the United States and Europe do not afford intellectual property protection to the same extent as the laws of the United States and Europe. Many companies have encountered significant problems

in protecting and defending intellectual property rights in certain foreign jurisdictions. The legal systems of some countries, including India, China and other developing countries, do not favor the enforcement of patents and other intellectual property rights. This could make it difficult for us to stop the infringement of our patents or the misappropriation of our other intellectual property rights. For example, many foreign countries have compulsory licensing laws under which a patent owner must grant licenses to third parties. Consequently, we may not be able to prevent third parties from practicing our inventions in certain countries outside the United States and Europe. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop and market their own products and, further, may export otherwise infringing products to territories where we have patent protection, if our ability to enforce our patents to stop infringing activities is inadequate. These products may compete with our products, and our patents or other intellectual property rights may not be effective or sufficient to prevent them from competing.

Agreements through which we license patent rights may not give us sufficient rights to permit us to pursue enforcement of our licensed patents or defense of any claims asserting the invalidity of these patents (or control of enforcement or defense) of such patent rights in all relevant jurisdictions as requirements may vary. For instance, under the Sobi collaboration, we retain the primary right to prosecute and defend its patent and other intellectual property rights, but Sobi has the primary right to enforce such rights against competitive infringement outside the United States.

Proceedings to enforce our patent rights in foreign jurisdictions, whether or not successful, could result in substantial costs and divert our efforts and resources from other aspects of our business. Moreover, such proceedings could put our patents at risk of being invalidated or interpreted narrowly and our patent applications at risk of not issuing and could provoke third parties to assert claims against us. We may not prevail in any lawsuits that we initiate and the damages or other remedies awarded, if any, may not be commercially meaningful. Furthermore, while we intend to protect our intellectual property rights in major markets for our products, we cannot ensure that we will be able to initiate or maintain similar efforts in all jurisdictions in which we may wish to market our products. Accordingly, our efforts to protect our intellectual property rights in such countries may be inadequate.

If we do not obtain patent term extension and data exclusivity for any product candidates we may develop, our business may be materially harmed.

Depending upon the timing, duration and specifics of any FDA marketing approval of any product candidates we may develop, one or more of our U.S. patents may be eligible for limited patent term extension under the Drug Price Competition and Patent Term Restoration Action of 1984, or Hatch-Waxman Amendments. The Hatch-Waxman Amendments permit a patent term extension of up to five years as compensation for patent term lost during the FDA regulatory review process. A patent term extension cannot extend the remaining term of a patent beyond 14 years from the date of product approval, only one patent may be extended and the extension only applies to those claims covering the approved drug, a method for using it, or a method for manufacturing it. However, we may not be granted an extension because of, for example, failing to exercise due diligence during the testing phase or regulatory review process, failing to apply within applicable deadlines, failing to apply prior to expiration of relevant patents, or otherwise failing to satisfy applicable requirements. Moreover, the applicable time period or the scope of patent protection afforded could be less than we request. If we are unable to obtain patent term extension or the term of any such extension is less than we request, our competitors may obtain approval of competing products following our patent expiration, and our business, financial condition, results of operations and prospects could be materially harmed.

We may be subject to claims by third parties asserting that our employees or we have misappropriated their intellectual property, or claiming ownership of what we regard as our own intellectual property.

Many of our employees, including our senior management, were previously employed at universities or at other biotechnology or pharmaceutical companies, including some which may be competitors or potential competitors. Some of these employees, including each member of our senior management, executed proprietary rights, non-disclosure, non-competition and non-solicitation agreements, or similar agreements, in connection with such previous employment. Although we try to ensure that our employees do not use the proprietary information or know-how of others in their work for us, we may be subject to claims that we or these employees have used or disclosed intellectual property, including trade secrets or other proprietary information, of any such third party. Litigation may be necessary to defend against such claims. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel or sustain damages. Such intellectual property rights could be awarded to a third party, and we could be required to obtain a license from such third party to commercialize our technology or products. Such a license may not be available on commercially reasonable terms or at all. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management.

In addition, while we typically require our employees, consultants and contractors who may be involved in the development of intellectual property to execute agreements assigning such intellectual property to us, we may be unsuccessful in executing such an

agreement with each party who in fact develops intellectual property that we regard as our own, which may result in claims by or against us related to the ownership of such intellectual property. If we fail in prosecuting or defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights. Even if we are successful in prosecuting or defending against such claims, litigation could result in substantial costs and be a distraction to our senior management and scientific personnel.

Obtaining and maintaining patent protection depends on compliance with various procedural, document submission, fee payment and other requirements imposed by governmental patent agencies, and our patent protection could be reduced or eliminated for non-compliance with these requirements.

Periodic maintenance fees, renewal fees, annuity fees and various other governmental fees on patents and applications are required to be paid to the USPTO and various governmental patent agencies outside of the United States in several stages over the lifetime of the patents and applications. The USPTO and various non-U.S. governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other similar provisions during the patent application process and after a patent has issued. While an inadvertent lapse can in many cases be cured by payment of a late fee or by other means in accordance with the applicable rules, there are situations in which non-compliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. Non-compliance events that could result in abandonment or lapse of a patent or patent application include, but are not limited to, the failure to respond to official actions within prescribed time limits, non-payment of fees and failure to properly legalize and submit formal documents. If we fail to maintain the patents and patent applications covering our product candidates, our competitive position would be adversely affected.

If we are unable to obtain licenses from third parties on commercially reasonable terms or fail to comply with our obligations under such agreements, our business could be harmed.

It may be necessary for us to use the patented or proprietary technology of third parties to commercialize our products, in which case we would be required to obtain a license from these third parties. If we are unable to license such technology, or if we are forced to license such technology on unfavorable terms, our business could be materially harmed. If we are unable to obtain a necessary license, we may be unable to develop or commercialize the affected product candidates, which could materially harm our business and the third parties owning such intellectual property rights could seek either an injunction prohibiting our sales, or, with respect to our sales, an obligation on our part to pay royalties and/or other forms of compensation. Even if we are able to obtain a license, it may be non-exclusive, which could enable our competitors to obtain access to the same technologies licensed to us.

If we fail to comply with our obligations under license agreements, our counterparties may have the right to terminate these agreements, in which event we might not be able to develop, manufacture or market, or may be forced to cease developing, manufacturing or marketing, any product that is covered by these agreements or may face other penalties under such agreements. Such an occurrence could materially adversely affect the value of the product candidate being developed under any such agreement. Termination of these agreements or reduction or elimination of our rights under these agreements may result in our having to negotiate new or reinstated agreements with less favorable terms, cause us to lose our rights under these agreements, including our rights to important intellectual property or technology, or impede, delay or prohibit the further development or commercialization of one or more product candidates that rely on such agreements.

Risks Related to Regulatory Approval and Marketing of Our Product Candidates and Other Legal Compliance Matters

The regulatory approval process is expensive, time consuming and uncertain and may prevent us or our collaborators such as Sobi from obtaining approvals for the commercialization of pegcetacoplan or any of our product candidates that we develop. As a result, we cannot predict when or if, and in which territories, we, or our collaborators, will obtain marketing approval to commercialize pegcetacoplan or any other product candidate that we develop.

The research, testing, manufacturing, labeling, approval, selling, marketing, promotion and distribution of products are subject to extensive regulation by the FDA and comparable foreign regulatory authorities. We are not permitted to market our product candidates in the United States or in other countries until we, or they, receive approval of an NDA from the FDA or marketing approval from applicable regulatory authorities outside the United States. Our product candidates are in various stages of development and are subject to the risks of failure inherent in drug development. In September 2020, we submitted an NDA to the FDA and a MAA to the EMA for pegcetacoplan for the treatment of PNH. We have limited experience in conducting and managing the clinical trials necessary to obtain marketing approvals, including FDA approval of an NDA.

The process of obtaining marketing approvals, both in the United States and abroad, is lengthy, expensive and uncertain. It may take many years, if approval is obtained at all, and can vary substantially based upon a variety of factors, including the type,

complexity and novelty of the product candidates involved. Securing marketing approval requires the submission of extensive preclinical and clinical data and supporting information, including manufacturing information, to regulatory authorities for each therapeutic indication to establish the product candidate's safety and efficacy. The FDA or other regulatory authorities may determine that our product candidates are not safe and effective, only moderately effective or have undesirable or unintended side effects, toxicities or other characteristics that preclude our obtaining marketing approval or prevent or limit commercial use. Any marketing approval we ultimately obtain may be limited or subject to restrictions or post-approval commitments that render the approved product not commercially viable.

In addition, changes in marketing approval policies during the development period, changes in or the enactment or promulgation of additional statutes, regulations or guidance or changes in regulatory review for each submitted product application, may cause delays in the approval or rejection of an application. Regulatory authorities have substantial discretion in the approval process and may refuse to accept any application or may decide that our data are insufficient for approval and require additional preclinical, clinical or other studies. In addition, varying interpretations of the data obtained from preclinical and clinical testing could delay, limit or prevent marketing approval of a product candidate. Any marketing approval we, or our collaborators, ultimately obtain may be limited or subject to restrictions or post-approval commitments that render the approved product not commercially viable. In addition, to the extent that we seek to develop a combination drug-device product for delivery of a product candidate or we rely on a previously cleared device to deliver a product candidate, we will also be dependent on FDA clearance or approval of such products.

Finally, disruptions at the FDA and other agencies may prolong the time necessary for new drugs to be reviewed and/or approved by necessary government agencies, which would adversely affect our business. For example, over the last several years, the U.S. government has shut down several times and certain regulatory agencies, such as the FDA, have had to furlough critical employees and stop critical activities. If a prolonged government shutdown occurs, it could significantly impact the ability of the FDA to timely review and process our regulatory submissions, which could have a material adverse effect on our business. The Trump Administration also took several executive actions that could impose significant burdens on, or otherwise materially delay, the FDA's ability to engage in routine regulatory and oversight activities.

Under our agreement with Sobi, Sobi is responsible for seeking regulatory approval outside the United States for systemic pegcetacoplan. A delay in obtaining or failure to obtain required approvals and clearances could negatively impact our ability or that of our collaborators, including Sobi, to generate revenue from the particular product candidate, which likely would result in significant harm to our financial position and adversely impact our stock price.

Failure to obtain marketing approval in foreign jurisdictions would prevent our product candidates from being marketed abroad. Any approval we are granted for our product candidates in the United States would not assure approval of our product candidates in foreign jurisdictions.

In order to market and sell pegcetacoplan or any of our other products in the European Union and other foreign jurisdictions, we, and our collaborators, such as Sobi, must obtain separate marketing approvals and comply with numerous and varying regulatory requirements. The approval procedure varies among countries and can involve additional testing. The time required to obtain approval may differ substantially from that required to obtain FDA approval. The marketing approval process outside the United States generally includes all of the risks associated with obtaining FDA approval. In addition, in many countries outside the United States, a product must be approved for reimbursement before the product can be approved for sale in that country. We, and our collaborators, such as Sobi, may not obtain approvals from regulatory authorities outside the United States on a timely basis, if at all. Approval by the FDA does not ensure approval by regulatory authorities in other countries or jurisdictions, and approval by one regulatory authority outside the United States does not ensure approval by regulatory authorities in other countries or jurisdictions or by the FDA. We may file for marketing approvals but not receive necessary approvals to commercialize our products in any market.

Additionally, we could face heightened risks with respect to seeking marketing approval in the United Kingdom as a result of the recent withdrawal of the United Kingdom from the European Union, commonly referred to as Brexit. Pursuant to the formal withdrawal arrangements agreed between the United Kingdom and the European Union, the United Kingdom withdrew from the European Union, effective December 31, 2020. On December 24, 2020, the United Kingdom and the European Union entered into a Trade and Cooperation Agreement. The agreement sets out certain procedures for approval and recognition of medical products in each jurisdiction. Since the regulatory framework for pharmaceutical products in the United Kingdom covering the quality, safety, and efficacy of pharmaceutical products, clinical trials, marketing authorization, commercial sales, and distribution of pharmaceutical products is derived from European Union directives and regulations, Brexit could materially impact the future regulatory regime that applies to products and the approval of product candidates in the United Kingdom. Any delay in obtaining, or an inability to obtain, any marketing approvals, as a result of Brexit or otherwise, would prevent us from commercializing any product candidates in the United Kingdom and/or the European Union and restrict our ability to generate revenue and achieve and sustain profitability. If any of these outcomes occur, we may be forced to restrict or delay efforts to seek regulatory approval in the United Kingdom and/or European Union for any product candidates, which could significantly and materially harm our business.

We, or our collaborators, may not be able to obtain orphan drug designation or orphan drug exclusivity for our product candidates and, even if we do, that exclusivity may not prevent the FDA or the EMA from approving other competing products.

Regulatory authorities in some jurisdictions, including the United States and Europe, may designate drugs for relatively small patient populations as orphan drugs. Under the Orphan Drug Act, the FDA may designate a product as an orphan drug if it is a drug intended to treat a rare disease or condition, which is generally defined as a patient population of fewer than 200,000 individuals annually in the United States. The FDA has granted orphan drug designation to pegcetacoplan for the treatment of PNH and for the treatment of C3 glomerulopathy. We, or our collaborators, may seek orphan drug designations for pegcetacoplan for other indications and for other product candidates and may be unable to obtain such designations.

Even if we, or our collaborators, obtain orphan drug designation for a product candidate, such as is the case for pegcetacoplan for the treatment of PNH, we, or they, may not be able to obtain orphan drug exclusivity for that product candidate. Generally, a product with orphan drug designation only becomes entitled to orphan drug exclusivity if it receives the first marketing approval for the indication for which it has such designation, in which case the FDA or the EMA will be precluded from approving another marketing application for the same drug for that indication for the applicable exclusivity period. The applicable exclusivity period is seven years in the United States and ten years in Europe. The European exclusivity period can be reduced to six years if a drug no longer meets the criteria for orphan drug designation or if the drug is sufficiently profitable so that market exclusivity is no longer justified. Orphan drug exclusivity may be lost if the FDA or the EMA determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the drug to meet the needs of patients with the rare disease or condition.

Even if we, or our collaborators, obtain orphan drug exclusivity for a product, that exclusivity may not effectively protect the product from competition because different drugs can be approved for the same condition. Even after an orphan drug is approved, the FDA can subsequently approve the same drug for the same condition if the FDA concludes that the later drug is clinically superior in that it is shown to be safer, more effective or makes a major contribution to patient care.

The FDA may further reevaluate the Orphan Drug Act and its regulations and policies. We do not know if, when, or how the FDA may change the orphan drug regulations and policies in the future, and it is uncertain how any changes might affect our business. Depending on what changes the FDA may make to its orphan drug regulations and policies, our business could be adversely impacted.

Fast track designation for one or more of our product candidates may not actually lead to a faster development or regulatory review or approval process.

We have received fast track designations for pegcetacoplan for the treatment of PNH and GA. If a product is intended for the treatment of a serious condition and nonclinical or clinical data demonstrate the potential to address unmet medical need for this condition, a product sponsor may apply for FDA fast track designation. Even though we have received fast track designation for pegcetacoplan for the treatment of PNH and GA, fast track designation does not ensure that we will receive marketing approval or that approval will be granted within any particular timeframe. We may not experience a faster development or regulatory review or approval process with fast rack designation compared to conventional FDA procedures. In addition, the FDA may withdraw fast track designation if it believes that the designation is no longer supported by data from our clinical development program. Fast track designation alone does not guarantee qualification for the FDA's priority review procedures.

Even if we, or our collaborators, obtain marketing approvals for our product candidates, the terms of approvals and ongoing regulation of our products may limit how we manufacture and market our products, which could impair our ability to generate revenue.

Once marketing approval has been granted, an approved product and its manufacturer and marketer are subject to ongoing review and extensive regulation. We, and our collaborators, must therefore comply with requirements concerning advertising and promotion for any of our product candidates which we or they market. Promotional communications with respect to prescription drugs are subject to a variety of legal and regulatory restrictions and must be consistent with the information in the product's approved labeling. Thus, we and our collaborators will not be able to promote any products we develop for indications or uses for which they are not approved.

In addition, manufacturers of approved products and those manufacturers' facilities are required to comply with extensive FDA requirements, including ensuring that quality control and manufacturing procedures conform to cGMPs, which include requirements relating to quality control and quality assurance as well as the corresponding maintenance of records and documentation and reporting requirements. We, our contract manufacturers, our collaborators and their contract manufacturers could be subject to periodic unannounced inspections by the FDA to monitor and ensure compliance with cGMPs.

Accordingly, assuming we, or our collaborators, receive marketing approval for one or more of our product candidates, we, and our collaborators, and our and their contract manufacturers will continue to expend time, money and effort in all areas of regulatory compliance, including manufacturing, production, product surveillance and quality control.

If we, and our collaborators, are not able to comply with post-approval regulatory requirements, we, and our collaborators, could have the marketing approvals for our products withdrawn by regulatory authorities and our, or our collaborators', ability to market any future products could be limited, which could adversely affect our ability to achieve or sustain profitability. Further, the cost of compliance with post-approval regulations may have a negative effect on our operating results and financial condition.

Any of our product candidates for which we, or our collaborators, obtain marketing approval in the future could be subject to post-marketing restrictions or withdrawal from the market and we, or our collaborators, may be subject to substantial penalties if we, or they, fail to comply with regulatory requirements or if we, or they, experience unanticipated problems with our products following approval.

Any of our product candidates for which we, or our collaborators, obtain marketing approval, as well as the manufacturing processes, post-approval studies and measures, labeling, advertising and promotional activities for such product, among other things, will be subject to ongoing requirements of and review by the FDA and other regulatory authorities. These requirements include submissions of safety and other post-marketing information and reports, registration and listing requirements, requirements relating to manufacturing, quality control, quality assurance and corresponding maintenance of records and documents, requirements regarding the distribution of samples to physicians and recordkeeping. Even if marketing approval of a product candidate is granted, the approval may be subject to limitations on the indicated uses for which the product may be marketed or to the conditions of approval, including the requirement to implement a Risk Evaluation and Mitigation Strategy.

The FDA may also impose requirements for costly post-marketing studies or clinical trials and surveillance to monitor the safety or efficacy of a product. The FDA and other agencies, including the Department of Justice, closely regulate and monitor the post-approval marketing and promotion of products to ensure that they are manufactured, marketed and distributed only for the approved indications and in accordance with the provisions of the approved labeling. The FDA imposes stringent restrictions on manufacturers' communications regarding off-label use and if we, or our collaborators, do not market any of our product candidates for which we, or they, receive marketing approval for only their approved indications, we, or they, may be subject to warnings or enforcement action for off-label marketing. Violation of the FDCA and other statutes, including the False Claims Act, relating to the promotion and advertising of prescription drugs may lead to investigations or allegations of violations of federal and state health care fraud and abuse laws and state consumer protection laws.

In addition, later discovery of previously unknown adverse events or other problems with our products or their manufacturers or manufacturing processes, or failure to comply with regulatory requirements, may yield various results, including:

- restrictions on such products, manufacturers or manufacturing processes;
- restrictions on the labeling or marketing of a product;
- restrictions on product distribution or use;
- requirements to conduct post-marketing studies or clinical trials;
- warning letters or untitled letters;
- withdrawal of the products from the market;
- refusal to approve pending applications or supplements to approved applications that we submit;
- recall of products;
- restrictions on coverage by third-party payors;
- fines, restitution or disgorgement of profits or revenues;
- suspension or withdrawal of marketing approvals;
- refusal to permit the import or export of products;
- product seizure; or
- injunctions or the imposition of civil or criminal penalties.

Current and future legislation may increase the difficulty and cost for us and our collaborators to obtain marketing approval of and commercialize our product candidates and affect the prices we, or they, may obtain.

In the United States and some foreign jurisdictions, there have been and continue to be a number of legislative and regulatory changes and proposed changes regarding the healthcare system that could, among other things, prevent or delay marketing approval of our product candidates, restrict or regulate post-approval activities and affect our ability, or the ability of our collaborators, to profitably sell any products for which we, or they, obtain marketing approval. We expect that current laws, as well as other healthcare reform measures that may be adopted in the future, may result in more rigorous coverage criteria and in additional downward pressure on the price that we, or our collaborators, may receive for any approved products.

In March 2010, President Obama signed into law the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act of 2010, or collectively the ACA. In addition, other legislative changes have been proposed and adopted since the ACA was enacted. In August 2011, the Budget Control Act of 2011, among other things, created measures for spending reductions by Congress. A Joint Select Committee on Deficit Reduction, tasked with recommending a targeted deficit reduction of at least $1.2 trillion for the years 2013 through 2021, was unable to reach required goals, thereby triggering the legislation's automatic reduction to several government programs. These changes included aggregate reductions to Medicare payments to providers of up to 2% per fiscal year, which went into effect in April 2013 and will remain in effect through 2029 unless additional Congressional action is taken. The Coronavirus Aid, Relief, and Economic Security Act, or the CARES Act, suspended the 2% Medicare sequester from May 1, 2020 through December 31, 2020, and extended the sequester by one year, through 2030. The American Taxpayer Relief Act of 2012, among other things, reduced Medicare payments to several providers and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. These new laws may result in additional reductions in Medicare and other healthcare funding and otherwise affect the prices we may obtain for any of our product candidates for which we may obtain regulatory approval or the frequency with which any such product candidate is prescribed or used.

Since enactment of the ACA, there have been, and continue to be, numerous legal challenges and Congressional actions to repeal and replace provisions of the law. For example, with enactment of the Tax Cuts and Jobs Act of 2017 (the "TCJA"), Congress repealed the "individual mandate." The repeal of this provision, which requires most Americans to carry a minimal level of health insurance, became effective in 2019. Further, on December 14, 2018, a U.S. District Court judge in the Northern District of Texas ruled that the individual mandate portion of the ACA is an essential and inseverable feature of the ACA, and therefore because the mandate was repealed as part of the Tax Cuts and Jobs Act, the remaining provisions of the ACA are invalid as well. On December 18, 2019, the Court of Appeals for the Fifth Circuit court affirmed the lower court's ruling that the individual mandate portion of the ACA is unconstitutional and it remanded the case to the district court for reconsideration of the severability question and additional analysis of the provisions of the ACA. Thereafter, the U.S. Supreme Court agreed to hear this case. Oral argument in the case took place on November 10, 2020. On February 10, 2021, the Biden Administration withdrew the federal government's support for overturning the ACA. A ruling by the Court is expected sometime this year. Litigation and legislation over the ACA are likely to continue, with unpredictable and uncertain results.

The Trump Administration also took executive actions to undermine or delay implementation of the ACA, including directing federal agencies with authorities and responsibilities under the ACA to waive, defer, grant exemptions from, or delay the implementation of any provision of the ACA that would impose a fiscal or regulatory burden on states, individuals, healthcare providers, health insurers, or manufacturers of pharmaceuticals or medical devices. On January 28, 2021, however, President Biden issued a new Executive Order which directs federal agencies to reconsider rules and other policies that limit Americans' access to health care, and consider actions that will protect and strengthen that access. Under this Order, federal agencies are directed to re-examine: policies that undermine protections for people with pre-existing conditions, including complications related to COVID-19; demonstrations and waivers under Medicaid and the ACA that may reduce coverage or undermine the programs, including work requirements; policies that undermine the Health Insurance Marketplace or other markets for health insurance; policies that make it more difficult to enroll in Medicaid and the ACA; and policies that reduce affordability of coverage or financial assistance, including for dependents. This Executive Order also directs the U.S. Department of Health and Human Services to create a special enrollment period for the Health Insurance Marketplace in response to the COVID-19 pandemic.

The prices of prescription pharmaceuticals in the United States and foreign jurisdictions is subject to considerable legislative and executive actions and could impact the prices we obtain for our products, if and when licensed.

The prices of prescription pharmaceuticals have also been the subject of considerable discussion in the United States. To date, there have been several recent U.S. congressional inquiries, as well as proposed and enacted state and federal legislation designed to, among other things, bring more transparency to drug pricing, review the relationship between pricing and manufacturer patient programs, reduce the costs of drugs under Medicare and reform government program reimbursement methodologies for products. To those ends, the Trump Administration issued several executive orders intended to lower the costs of prescription drug products.

Certain of these orders are reflected in recently promulgated regulations, including an interim final rule implementing the Trump Administration's most favored nation model, but such final rule is currently subject to a nationwide preliminary injunction. It remains to be seen whether these orders and resulting regulations will remain in force during the Biden Administration. Further, on September 24, 2020, the Trump Administration finalized a rulemaking allowing states or certain other non-federal government entities to submit importation program proposals to the FDA for review and approval. Applicants are required to demonstrate that their importation plans pose no additional risk to public health and safety and will result in significant cost savings for consumers. The FDA has issued draft guidance that would allow manufacturers to import their own FDA-approved drugs that are authorized for sale in other countries (multi-market approved products).

At the state level, individual states are increasingly aggressive in passing legislation and implementing regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing. In addition, regional health care organizations and individual hospitals are increasingly using bidding procedures to determine what pharmaceutical products and which suppliers will be included in their prescription drug and other health care programs. These measures could reduce the ultimate demand for our products, once approved, or put pressure on our product pricing. We expect that additional state and federal healthcare reform measures will be adopted in the future, any of which could limit the amounts that federal and state governments will pay for healthcare products and services, which could result in reduced demand for our product candidates or additional pricing pressures.

In countries outside of the United States, particularly the countries of the European Union, the pricing of prescription pharmaceuticals is subject to governmental control. In these countries, pricing negotiations with governmental authorities can take considerable time after the receipt of marketing approval for a product. To obtain reimbursement or pricing approval in some countries, we may be required to conduct a clinical trial that compares the cost-effectiveness of our product candidate to other available therapies. If reimbursement of our products is unavailable or limited in scope or amount, or if pricing is set at unsatisfactory levels, our business could be harmed, possibly materially.

Our relationships with customers and third-party payors, among others, will be subject to applicable anti-kickback, fraud and abuse and other healthcare laws and regulations, which could expose us to penalties, including criminal sanctions, civil penalties, contractual damages, reputational harm, fines, disgorgement, exclusion from participation in government healthcare programs, curtailment or restricting of our operations, and diminished profits and future earnings.

Healthcare providers, physicians and third-party payors will play a primary role in the recommendation and prescription of any products for which we obtain marketing approval. Our current and future arrangements with healthcare providers, and third-party payors and customers, if any, will subject us to broadly applicable fraud and abuse and other healthcare laws and regulations. The laws and regulations may constrain the business or financial arrangements and relationships through which we conduct clinical research, market, sell and distribute any products for which we obtain marketing approval. These include the following:

Anti-Kickback Statute. The federal Anti-Kickback Statute prohibits, among other things, persons and entities from knowingly and willfully soliciting, offering, receiving or providing remuneration (including any kickback, bribe or rebate), directly or indirectly, in cash or in kind, to induce or reward, or in return for, either the referral of an individual for, or the purchase, lease or order of a good, facility, item or service for which payment may be made under a federal healthcare program such as Medicare and Medicaid;

False Claims Laws. The federal false claims and civil monetary penalties laws, including the federal civil False Claims Act, impose criminal and civil penalties, including through civil whistleblower or *qui tam* actions against individuals or entities for, among other things, knowingly presenting or causing to be presented false or fraudulent claims for payment by a federal healthcare program or making a false statement or record material to payment of a false claim or avoiding, decreasing or concealing an obligation to pay money to the federal government, with potential liability including mandatory treble damages and significant per-claim penalties;

HIPAA. The federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, imposes criminal and civil liability for, among other things, executing a scheme, or making materially false statements in connection with the delivery of or payment for health care benefits, items, or services. Additionally, HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act and its implementing regulations, also imposes obligations on covered entities and their business associates that perform certain functions or activities that involve the use or disclosure of protected health information on their behalf, including mandatory contractual terms and technical safeguards, with respect to maintaining the privacy, security and transmission of individually identifiable health information;

Transparency Requirements. The federal Physician Payments Sunshine Act requires certain manufacturers of drugs, devices, biologics, and medical supplies for which payment is available under Medicare, Medicaid, or the Children's Health Insurance Program, with specific exceptions, to report annually to the Centers for Medicare & Medicaid Services, or CMS, information related

to payments or transfers of value made to physicians and teaching hospitals, as well as information regarding ownership and investment interests held by physicians and their immediate family members; and

Analogous State and Foreign Laws. Analogous state and foreign fraud and abuse laws and regulations, such as state anti-kickback and false claims laws, can apply to sales or marketing arrangements, and claims involving healthcare items or services reimbursed by non-governmental third-party payors, and are generally broad and are enforced by many different federal and state agencies as well as through private actions. Some state laws require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government and require drug manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers or marketing expenditures. Additionally, some state and local laws require the registration of pharmaceutical sales representatives in the jurisdiction. State and foreign laws also govern the privacy and security of health information in some circumstances, many of which differ from each other in significant ways and often are not pre-empted by HIPAA, thus complicating compliance efforts.

Because of the breadth of these laws and the narrowness of the statutory exceptions and safe harbors available, it is possible that some of our business activities could be subject to challenge under one or more of such laws. If our operations are found to be in violation of any of the laws described above or any other government regulations that apply to us, we may be subject to penalties, including civil and criminal penalties, damages, fines, exclusion from participation in government healthcare programs, such as Medicare and Medicaid, imprisonment and the curtailment or restructuring of our operations, any of which could adversely affect our business, financial condition, results of operations and prospects.

The provision of benefits or advantages to physicians to induce or encourage the prescription, recommendation, endorsement, purchase, supply, order or use of medicinal products is prohibited in the European Union. The provision of benefits or advantages to physicians is also governed by the national anti-bribery laws of European Union Member States, such as the UK Bribery Act 2010. Violation of these laws could result in substantial fines and imprisonment.

Payments made to physicians in certain European Union Member States must be publicly disclosed. Moreover, agreements with physicians often must be the subject of prior notification and approval by the physician's employer, his or her competent professional organization and/or the regulatory authorities of the individual European Union Member States.

These requirements are provided in the national laws, industry codes or professional codes of conduct applicable in the European Union Member States. Failure to comply with these requirements could result in reputational risk, public reprimands, administrative penalties, fines or imprisonment.

Efforts to ensure that our business arrangements with third parties, and our business generally, will comply with applicable healthcare laws and regulations will involve substantial costs. It is possible that governmental authorities will conclude that our business practices may not comply with current or future statutes, regulations or case law involving applicable fraud and abuse or other healthcare laws and regulations. If our operations are found to be in violation of any of these laws or any other governmental regulations that may apply to us, we may be subject to significant civil, criminal and administrative penalties, damages, fines, individual imprisonment, additional reporting requirements and oversight if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of non-compliance with these laws, exclusion of products from government funded healthcare programs, such as Medicare and Medicaid, disgorgement, contractual damages, reputational harm, and the curtailment or restructuring of our operations. Defending against any such actions can be costly, time-consuming and may require significant financial and personnel resources. Therefore, even if we are successful in defending against any such actions that may be brought against us, our business may be impaired. Further, if any of the physicians or other healthcare providers or entities with whom we expect to do business is found to be not in compliance with applicable laws, they may be subject to criminal, civil or administrative sanctions, including exclusions from government funded healthcare programs. Liabilities they incur pursuant to these laws could result in significant costs or an interruption in operations, which could have a material adverse effect on our business, financial condition, results of operations and prospects.

Compliance with global privacy and data security requirements could result in additional costs and liabilities to us or inhibit our ability to collect and process data globally, and the failure to comply with such requirements could subject us to significant fines and penalties, which may have a material adverse effect on our business, financial condition or results of operations.

The regulatory framework for the collection, use, safeguarding, sharing, transfer and other processing of information worldwide is rapidly evolving and is likely to remain uncertain for the foreseeable future. Globally, virtually every jurisdiction in which we operate has established its own data security and privacy frameworks with which we must comply. For example, the collection, use, disclosure, transfer, or other processing of personal data regarding individuals in the European Union, including personal health data, is subject to the EU General Data Protection Regulation, or the GDPR, which took effect across all member states of the European Economic Area, or EEA, in May 2018. The GDPR is wide-ranging in scope and imposes numerous requirements on companies that process personal data, including requirements relating to processing health and other sensitive data, obtaining consent of the individuals to whom the personal data relates, providing information to individuals regarding data processing activities, implementing

safeguards to protect the security and confidentiality of personal data, providing notification of data breaches, and taking certain measures when engaging third-party processors. The GDPR increases our obligations with respect to clinical trials conducted in the EEA by expanding the definition of personal data to include coded data and requiring changes to informed consent practices and more detailed notices for clinical trial subjects and investigators. In addition, the GDPR also imposes strict rules on the transfer of personal data to countries outside the European Union, including the United States and, as a result, increases the scrutiny that clinical trial sites located in the EEA should apply to transfers of personal data from such sites to countries that are considered to lack an adequate level of data protection, such as the United States. The GDPR also permits data protection authorities to require destruction of improperly gathered or used personal information and/or impose substantial fines for violations of the GDPR, which can be up to four percent of global revenues or 20 million Euros, whichever is greater, and it also confers a private right of action on data subjects and consumer associations to lodge complaints with supervisory authorities, seek judicial remedies, and obtain compensation for damages resulting from violations of the GDPR. In addition, the GDPR provides that European Union member states may make their own further laws and regulations limiting the processing of personal data, including genetic, biometric or health data.

Similar actions are either in place or under way in the United States. There are a broad variety of data protection laws that are applicable to our activities, and a wide range of enforcement agencies at both the state and federal levels that can review companies for privacy and data security concerns based on general consumer protection laws. The Federal Trade Commission and state Attorneys General all are aggressive in reviewing privacy and data security protections for consumers. New laws also are being considered at both the state and federal levels. For example, the California Consumer Privacy Act—which went into effect on January 1, 2020—is creating similar risks and obligations as those created by GDPR, though the Act does exempt certain information collected as part of a clinical trial subject to the Federal Policy for the Protection of Human Subjects (the Common Rule). Many other states are considering similar legislation. A broad range of legislative measures also have been introduced at the federal level. Accordingly, failure to comply with federal and state laws (both those currently in effect and future legislation) regarding privacy and security of personal information could expose us to fines and penalties under such laws. There also is the threat of consumer class actions related to these laws and the overall protection of personal data. Even if we are not determined to have violated these laws, government investigations into these issues typically require the expenditure of significant resources and generate negative publicity, which could harm our reputation and our business.

Given the breadth and depth of changes in data protection obligations, preparing for and complying with these requirements is rigorous and time intensive and requires significant resources and a review of our technologies, systems and practices, as well as those of any third-party collaborators, service providers, contractors or consultants that process or transfer personal data collected in the European Union. The GDPR and other changes in laws or regulations associated with the enhanced protection of certain types of sensitive data, such as healthcare data or other personal information from our clinical trials, could require us to change our business practices and put in place additional compliance mechanisms, may interrupt or delay our development, regulatory and commercialization activities and increase our cost of doing business, and could lead to government enforcement actions, private litigation and significant fines and penalties against us and could have a material adverse effect on our business, financial condition or results of operations.

Laws and regulations governing any international operations we may have in the future may preclude us from developing, manufacturing and selling certain products outside of the United States and require us to develop and implement costly compliance programs.

As we expand our operations outside of the United States, we must dedicate additional resources to comply with numerous laws and regulations in each jurisdiction in which we plan to operate. The Foreign Corrupt Practices Act, or FCPA, prohibits any U.S. individual or business from paying, offering, authorizing payment or offering of anything of value, directly or indirectly, to any foreign official, political party or candidate for the purpose of influencing any act or decision of the foreign entity in order to assist the individual or business in obtaining or retaining business. The FCPA also obligates companies whose securities are listed in the United States to comply with certain accounting provisions requiring us to maintain books and records that accurately and fairly reflect all transactions of the corporation, including international subsidiaries, and to devise and maintain an adequate system of internal accounting controls for international operations.

Compliance with the FCPA is expensive and difficult, particularly in countries in which corruption is a recognized problem. In addition, the FCPA presents particular challenges in the pharmaceutical industry, because, in many countries, hospitals are operated by the government, and doctors and other hospital employees are considered foreign officials. Certain payments to hospitals in connection with clinical trials and other work have been deemed to be improper payments to government officials and have led to FCPA enforcement actions.

Various laws, regulations and executive orders also restrict the use and dissemination outside of the United States, or the sharing with certain non-U.S. nationals, of information classified for national security purposes, as well as certain products and technical data relating to those products. As we expand our presence outside of the United States, it will require us to dedicate additional resources to comply with these laws, and these laws may preclude us from developing, manufacturing, or selling certain products and product candidates outside of the United States, which could limit our growth potential and increase our development costs.

The failure to comply with laws governing international business practices may result in substantial civil and criminal penalties and suspension or debarment from government contracting. The Securities and Exchange Commission, or SEC, also may suspend or bar issuers from trading securities on U.S. exchanges for violations of the FCPA's accounting provisions.

If we fail to comply with environmental, health and safety laws and regulations, we could become subject to fines or penalties or incur costs that could harm our business.

We are subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures and the handling, use, storage, treatment and disposal of hazardous materials and wastes. From time to time and in the future, our operations may involve the use of hazardous and flammable materials, including chemicals and biological materials, and may also produce hazardous waste products. Even if we contract with third parties for the disposal of these materials and waste products, we cannot completely eliminate the risk of contamination or injury resulting from these materials. In the event of contamination or injury resulting from the use or disposal of our hazardous materials, we could be held liable for any resulting damages, and any liability could exceed our resources. We also could incur significant costs associated with civil or criminal fines and penalties for failure to comply with such laws and regulations.

We maintain workers' compensation insurance to cover us for costs and expenses we may incur due to injuries to our employees resulting from the use of hazardous materials, but this insurance may not provide adequate coverage against potential liabilities. However, we do not maintain insurance for environmental liability or toxic tort claims that may be asserted against us.

In addition, we may incur substantial costs in order to comply with current or future environmental, health and safety laws and regulations. Current or future environmental laws and regulations may impair our research, development or production efforts. In addition, failure to comply with these laws and regulations may result in substantial fines, penalties or other sanctions.

Our employees may engage in misconduct or other improper activities, including non-compliance with regulatory standards and requirements, which could cause significant liability for us and harm our reputation.

We are exposed to the risk of employee fraud or other misconduct, including intentional failures to comply with FDA regulations or similar regulations of comparable foreign regulatory authorities, provide accurate information to the FDA or comparable foreign regulatory authorities, comply with manufacturing standards, comply with federal and state healthcare fraud and abuse laws and regulations and similar laws and regulations established and enforced by comparable foreign regulatory authorities, report financial information or data accurately or disclose unauthorized activities to us. Employee misconduct could also involve the improper use of information obtained in the course of clinical trials, which could result in regulatory sanctions and serious harm to our reputation. This could include violations of HIPAA, other U.S. federal and state law, and requirements of non-U.S. jurisdictions, including the European Union Data Protection Directive. It is not always possible to identify and deter employee misconduct, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to be in compliance with such laws, standards, regulations, guidance or codes of conduct. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business and results of operations, including the imposition of significant fines or other sanctions.

Risks Related to Employee Matters and Managing Growth

Our future success depends on our ability to retain our executive team and to attract, retain and motivate qualified personnel.

We are highly dependent on the pharmaceutical research and development and business development expertise of our executive team, including Cedric Francois, M.D., Ph.D., our President and Chief Executive Officer, and Pascal Deschatelets, Ph.D., our Chief Scientific Officer. The members of our executive team are employed "at will," meaning any of them may terminate his or her employment with us at any time with or without notice and for any reason or no reason. In the future, we may be dependent on other members of our management, scientific and development team.

Our ability to compete in the biotechnology and pharmaceuticals industries depends upon our ability to attract and retain highly qualified managerial, scientific and medical personnel. Our industry has experienced a high rate of turnover of management personnel in recent years. If we lose one or more of our executive officers or other key employees, our ability to implement our business strategy successfully could be seriously harmed. Furthermore, replacing executive officers or other key employees may be difficult and may take an extended period of time because of the limited number of individuals in our industry with the breadth of skills and experience required to develop, gain marketing approval of and commercialize products successfully. Competition to hire from this limited pool is intense, and we may be unable to hire, train, retain or motivate these additional key employees on acceptable terms given the

competition among numerous pharmaceutical and biotechnology companies for similar personnel. We also experience competition for the hiring of scientific and clinical personnel from universities and research institutions.

We rely on consultants and advisors, including scientific and clinical advisors, to assist us in formulating our research and development and commercialization strategy. Our consultants and advisors may be employed by other entities and may have commitments under consulting or advisory contracts with those entities that may limit their availability to us. If we are unable to continue to attract and retain highly qualified personnel, our ability to develop and commercialize our product candidates will be limited.

We expect to continue to expand our organization, and as a result, we may encounter difficulties in managing our growth, which could disrupt our operations.

We continue to expect to experience significant growth in the number of our employees and the scope of our operations, particularly in the areas of drug manufacturing, clinical, regulatory affairs and sales, marketing and distribution. During 2020, the number of our employees increased from 235 on December 31, 2019 to 374 on December 31, 2020. As of March 31, 2021, we had 391 employees and we expect the number of employees to continue to increase significantly in 2021. Our principal office is located in Massachusetts and we maintain additional offices in California, Australia and Switzerland. To manage these growth activities and separation of offices, we must continue to implement and improve our managerial, operational and financial systems and continue to recruit and train additional qualified personnel. Our management may need to devote a significant amount of its attention to managing these growth activities. Due to our limited financial resources and the limited experience of our management team in managing a company with such anticipated growth, we may not be able to effectively manage the expansion of our operations, retain key employees, or identify, recruit and train additional qualified personnel. Our inability to manage the expansion of our operations effectively may result in weaknesses in our infrastructure, give rise to operational mistakes, loss of business opportunities, loss of employees and reduced productivity among remaining employees. Our expected growth could also require significant capital expenditures and may divert financial resources from other projects, such as the development of additional product candidates. If we are unable to effectively manage our expected growth, our expenses may increase more than expected, our ability to generate revenues could be reduced and we may not be able to implement our business strategy, including the successful commercialization of our product candidates.

We temporarily closed our facilities in March 2020 in response to the COVID-19 pandemic. We have since re-opened our physical facilities on a limited basis, subject to compliance with strict safety guidelines, but most of our employees continue to work remotely. In the event of a renewal of shelter-in-place orders or and mandated local travel restrictions, our employees conducting research and development activities may not be able to access our facilities and our activities may be significantly limited or curtailed, possibly for an extended period of time. Furthermore, it is possible that over the long term our operational efficiency may be decreased if our employees and third-party collaborators are unable to meet and work in the same physical location.

Our employees, independent contractors, consultants, collaborators and contract research organizations may engage in misconduct or other improper activities, including non-compliance with regulatory standards and requirements, which could cause significant liability for us and harm our reputation.

We are exposed to the risk that our employees, independent contractors, consultants, collaborators and contract research organizations may engage in fraud or other misconduct, including intentional failures to comply with FDA regulations or similar regulations of comparable non-U.S. regulatory authorities, to provide accurate information to the FDA or comparable non-U.S. regulatory authorities, to comply with manufacturing standards we have established, to comply with federal and state healthcare fraud and abuse laws and regulations and similar laws and regulations established and enforced by comparable non-U.S. regulatory authorities, to report financial information or data accurately or to disclose unauthorized activities to us. Such misconduct could also involve the improper use of information obtained in the course of clinical trials, which could result in regulatory sanctions and serious harm to our reputation. It is not always possible to identify and deter misconduct, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to be in compliance with such laws, standards or regulations. These risks may be particularly acute given the rapid growth in the size of our company. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business and results of operations, including the imposition of significant criminal, civil and administrative sanctions including monetary penalties, damages, fines, disgorgement, individual imprisonment, and exclusion from participation in government funded healthcare programs, such as Medicare and Medicaid, additional reporting requirements and oversight if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of non-compliance with these laws, reputational harm, and we may be required to curtail or restructure our operations.

We may engage in acquisitions that could disrupt our business, cause dilution to our stockholders or reduce our financial resources.

In the future, we may enter into transactions to acquire other businesses, products or technologies. Because we have not made any acquisitions to date, our ability to do so successfully is unproven. If we do identify suitable candidates, we may not be able to make such acquisitions on favorable terms, or at all. Any acquisitions we make may not strengthen our competitive position, and these transactions may be viewed negatively by customers or investors. We may decide to incur debt in connection with an acquisition or issue our common stock or other equity securities to the stockholders of the acquired company, which would reduce the percentage ownership of our existing stockholders. We could incur losses resulting from undiscovered liabilities of the acquired business that are not covered by the indemnification we may obtain from the seller. In addition, we may not be able to successfully integrate the acquired personnel, technologies and operations into our existing business in an effective, timely and non-disruptive manner. Acquisitions may also divert management attention from day-to-day responsibilities, increase our expenses and reduce our cash available for operations and other uses. We cannot predict the number, timing or size of future acquisitions or the effect that any such transactions might have on our operating results.

Risks Related to Ownership of Our Common Stock

An active trading market for our common stock may not be sustainable. If an active trading market is not sustained, our ability to raise capital in the future may be impaired.

Our shares began trading on the Nasdaq Global Select Market on November 9, 2017. Given the limited trading history of our common stock, there is a risk that an active trading market for our shares may not be sustained, which could put downward pressure on the market price of our common stock and thereby affect the ability of stockholders to sell their shares. An inactive trading market for our common stock may also impair our ability to raise capital to continue to fund our operations by selling shares and impair our ability to acquire other companies or technologies by using our shares as consideration.

The trading price of our common stock is highly volatile, which could result in substantial losses for our stockholders.

The trading price of our common stock has been, and is likely to continue to be, highly volatile and could be subject to wide fluctuations in response to various factors, some of which are beyond our control. The stock market in general and the market for smaller pharmaceutical and biotechnology companies in particular have experienced extreme volatility that has often been unrelated to the operating performance of particular companies. As a result of this volatility, our stockholders may not be able to sell their common stock at or above the price they paid for their common stock. The market price for our common stock may be influenced by many factors, including:

- the timing and results of clinical trials of pegcetacoplan and any other product candidates;

- regulatory developments with respect to pegcetacoplan, including with respect to our efforts to obtain approval with respect to our NDA and MAA related to pegcetacoplan for the treatment of PNH;
- the success of existing or new competitive products or technologies;
- results of discussions with regulatory authorities and regulatory actions with respect to our product candidates or our competitors' products and product candidates;
- the effect of the COVID-19 outbreak on the healthcare system and the economy generally and on our clinical trials and other operations specifically;
- announcements by us or our competitors of significant acquisitions, strategic partnerships, joint ventures, collaborations or capital commitments;
- commencement or termination of collaborations for our development programs;
- failure or discontinuation of any of our product candidates or development programs;
- results of clinical trials of product candidates of our competitors;
- regulatory or legal developments in the United States and other countries;
- developments or disputes concerning patent applications, issued patents or other proprietary rights;
- the recruitment or departure of key personnel;
- the level of expenses related to any of our product candidates or clinical development programs;
- the results of our efforts to develop additional product candidates or products;
- actual or anticipated changes in estimates as to financial results or development timelines;
- announcement or expectation of additional financing efforts;
- sales of our common stock by us, our insiders or other stockholders;
- variations in our financial results or those of companies that are perceived to be similar to us;
- short positions, hedging or other transactions in our securities in connection with our Convertible Notes;
- changes in estimates or recommendations by securities analysts, if any, that cover our stock;
- changes in the structure of healthcare payment systems;
- market conditions in the pharmaceutical and biotechnology sectors;
- general economic, industry and market conditions; and
- the other factors described in this "Risk Factors" section.

We could be subject to securities class action litigation.

In the past, securities class action litigation has often been brought against a company following a decline in the market price of its securities. This risk is especially relevant for us because pharmaceutical companies have experienced significant stock price volatility in recent years. If we face such litigation, it could result in substantial costs and a diversion of management's attention and our resources, which could harm our business.

We have broad discretion in the use of our funds and may not use them effectively.

Our management will have broad discretion in the application of our cash, cash equivalents and marketable securities and could spend our funds in ways that do not improve our results of operations or enhance the value of our common stock. The failure by our management to apply these funds effectively could result in financial losses that could harm our business, cause the price of our common stock to decline and delay the development of our product candidates. Pending their use, we may invest our funds in a manner that does not produce income or that loses value.

We incur increased costs as a result of operating as a public company, and our management is required to devote substantial time to compliance initiatives and corporate governance practices.

As a public company, we incur significant legal, accounting and other expenses. The Sarbanes-Oxley Act of 2002, the Dodd-Frank Wall Street Reform and Consumer Protection Act, the listing requirements of the Nasdaq Global Select Market and other applicable securities rules and regulations impose various requirements on public companies, including establishment and maintenance of effective disclosure and financial controls and corporate governance practices. Our management and other personnel devote and will need to continue to devote, a substantial amount of time to these compliance initiatives. Moreover, these rules and regulations increase our legal and financial compliance costs and make some activities more time-consuming and costly.

If we identify a material weakness in our internal control over financial reporting, it could have an adverse effect on our business and financial results and our ability to meet our reporting obligations could be negatively affected, each of which could negatively affect the trading price of our common stock.

A material weakness is a deficiency, or a combination of deficiencies, in internal control over financial reporting, such that there is a reasonable possibility that a material misstatement of our annual or interim financial statements will not be prevented or detected on a timely basis. Accordingly, a material weakness increases the risk that the financial information we report contains material errors.

We regularly review and update our internal controls, disclosure controls and procedures, and corporate governance policies. In addition, we are required under the Sarbanes-Oxley Act of 2002 to report annually on our internal control over financial reporting. Our system of internal controls, however well-designed and operated, is based in part on certain assumptions and includes elements that rely on information from third parties. Our system can provide only reasonable, not absolute, assurances that the objectives of the system are met. If we, or our independent registered public accounting firm, determine that our internal controls over financial reporting are not effective, or we discover areas that need improvement in the future, these shortcomings could have an adverse effect on our business and financial results, and the price of our common stock could be negatively affected.

If we cannot conclude that we have effective internal control over our financial reporting, or if our independent registered public accounting firm is unable to provide an unqualified opinion regarding the effectiveness of our internal control over financial reporting, investors could lose confidence in the reliability of our financial statements, which could lead to a decline in our stock price. Failure to comply with reporting requirements could also subject us to sanctions and/or investigations by the SEC, The Nasdaq Stock Market or other regulatory authorities.

A sale of a substantial number of shares of our common stock could cause the market price of our common stock to decline significantly, even if our business is doing well.

Sales of a substantial number of shares of our common stock in the public market could occur at any time. These sales, or the perception in the market that the holders of a large number of shares of common stock intend to sell shares, could reduce the market price of our common stock.

We have registered all shares of common stock that we may issue under our equity compensation plans. As of March 31, 2021, we had options to purchase an aggregate of 13,404,381 shares of our common stock outstanding, of which options to purchase 6,642,110 shares were vested and 949,377 outstanding unvested restricted stock units that upon vesting would result in the issuance of 949,377 shares of our common stock. These shares can be freely sold in the public market upon issuance, subject to volume limitations applicable to affiliates. Moreover, holders of an aggregate of 11,098,982 shares of our common stock have rights, subject to conditions, to require us to file registration statements covering their shares or to include their shares in registration statements that we may file for ourselves or other stockholders. If these additional shares are sold, or if it is perceived that they will be sold, in the public market, the trading price of our common stock could decline.

Changes in tax laws or in their interpretation could adversely affect our business and financial condition.

Recent changes in tax law could adversely affect our business or financial condition. On December 22, 2017, the U.S. government enacted legislation, commonly referred to as the Tax Cuts and Jobs Act, or the TCJA, that significantly revised the Internal Revenue Code of 1986, as amended, or the Code. The TCJA, among other things, contained significant changes to corporate taxation, including reduction of the corporate tax rate from a top marginal rate of 35% to a flat rate of 21%, limitation of the tax deduction for interest expense to 30% of adjusted earnings (except for certain small businesses), limitation of the deduction for net operating losses to 80% of current year taxable income and elimination of net operating loss carrybacks for losses arising in taxable years ending after December 31, 2017 (though any such net operating losses may be carried forward indefinitely), one time taxation of offshore earnings at reduced rates regardless of whether they are repatriated, elimination of U.S. tax on foreign earnings (subject to

certain important exceptions), immediate deductions for certain new investments instead of deductions for depreciation expense over time, and modifying or repealing many business deductions and credits.

As part of Congress' response to the COVID-19 pandemic, the Families First Coronavirus Response Act, or FFCR Act, was enacted on March 18, 2020, and the Coronavirus Aid, Relief, and Economic Security Act, or CARES Act, was enacted on March 27, 2020. COVID relief provisions were also included in the Consolidated Appropriations Act, 2021, or CAA, which was enacted on December 27, 2020. The FFCR Act, the CARES Act, and the CAA contain numerous tax provisions. In particular, the CARES Act retroactively and temporarily (for taxable years beginning before January 1, 2021) suspends application of the 80%-of-income limitation on the use of net operating losses, which was enacted as part of the TCJA. It also provides that net operating losses arising in any taxable year beginning after December 31, 2017, and before January 1, 2021, are generally eligible to be carried back up to five years.The CARES Act also temporarily (for taxable years beginning in 2019 or 2020) relaxes the limitation on the tax deductibility of net interest expense by increasing the limitation from 30% to 50% of adjusted taxable income.

Regulatory guidance under the TCJA, the FFCR Act, the CARES Act, and the CAA is and continues to be forthcoming, and such guidance could ultimately increase or lessen the impact of these laws on our business and financial condition. Congress may enact additional legislation in connection with the COVID-19 pandemic, some of which could have an impact on our company. In addition, it is uncertain if and to what extent various states will conform to the TCJA, the FFCR Act, the CARES Act, or the CAA.

We might not be able to utilize a significant portion of our net operating loss carryforwards and research and development tax credit carryforwards.

As of December 31, 2020, we had both federal and state net operating loss carryforwards of $358.2 million and $405.0 million, respectively, and federal and state research and development tax credit carryforwards of $34.2 million and $6.5 million, respectively. Federal net operating loss carryforward generated post-2017 in the amount of $276.9 million may be carried forward indefinitely. The remaining net operating loss and research and development tax credit carryforwards will begin to expire in 2025. These net operating loss and tax credit carryforwards could expire unused and be unavailable to offset future income tax liabilities. Under the TCJA, as modified by the CARES act, federal net operating losses incurred in 2018 and in future years may be carried forward indefinitely, but the deductibility of such federal net operating losses in 2021 and future years is limited. Certain states have also enacted temporary suspension or limitation of the utilization of net operating loss carryforwards. In addition, under Section 382 of the Code, and corresponding provisions of state law, if a corporation undergoes an "ownership change," which is generally defined as a greater than 50% change, by value, in its equity ownership over a three-year period, the corporation's ability to use its pre-change net operating loss carryforwards and other pre-change tax attributes to offset its post-change income may be limited. We experienced a Section 382 ownership change in September 2015, which imposes annual limitations on our use of pre-change net operating loss carryforwards and other pre-change tax attributes. In addition, we may experience ownership changes in the future as a result of subsequent shifts in our stock ownership, some of which may be outside of our control. We have determined that our research and development credit carryforwards are also limited. These limitations upon our historical net operating loss and tax credit carryforwards may harm our future operating results by effectively increasing our future tax obligations. Refer to Note 14, "Income Taxes," of the financial statements in our 2020 Annual Report on Form 10-K for additional information related to our accounting for income taxes.

Taxing authorities could challenge our historical and future tax positions or our allocation of taxable income among our subsidiaries, and tax laws to which we are subject could change in a manner adverse to us.

We operate through various subsidiaries in a number of countries throughout the world. Consequently, we are subject to tax laws, treaties, and regulations in the countries in which we operate, and these laws and treaties are subject to interpretation. We have taken, and will continue to take, tax positions based on our interpretation of such tax laws. Our transfer pricing arrangements are not generally binding on applicable tax authorities. The price charged for products, services, or the royalty rates and other amounts paid for intellectual property rights, could be challenged by the various tax authorities, resulting in additional tax liability, interest, and/or penalties. There can be no assurance that a taxing authority will not have a different interpretation of applicable law and assess us with additional taxes. If we are assessed with additional taxes, this may result in a material adverse effect on our results of operations and/or financial condition.

Any changes to existing accounting pronouncements or taxation rules or practices may cause adverse fluctuations in our reported results of operations or affect how we conduct our business.

A change in accounting pronouncements or taxation rules or practices can have a significant effect on our reported results and may affect our reporting of transactions completed before the change is effective. New accounting pronouncements, taxation rules and varying interpretations of accounting pronouncements or taxation rules have occurred in the past and may occur in the future. The change to existing rules, future changes, if any, or the need for us to modify a current tax or accounting position may adversely affect our reported financial results or the way we conduct our business.

We do not anticipate paying any cash dividends on our capital stock in the foreseeable future. Accordingly, stockholders must rely on capital appreciation, if any, for any return on their investment.

We have never declared nor paid cash dividends on our capital stock. We currently plan to retain all of our future earnings, if any, to finance the operation, development and growth of our business. In addition, the terms of our development funding agreement with SFJ, precludes us from paying dividends, and any future debt or credit agreements may also preclude us from paying dividends. As a result, capital appreciation, if any, of our common stock will be our stockholders' sole source of gain for the foreseeable future.

Concentration of ownership of our common stock among our executive officers and directors, entities associated with our executive officers and directors and our largest stockholders may allow these stockholders to significantly influence matters submitted to our stockholders for approval, as well as our management and affairs.

As of March 31, 2021, our executive officers and directors, and entities associated or affiliated with our executive officers and directors, in the aggregate, beneficially owned shares representing approximately 24.7% of our outstanding common stock, including our largest stockholder, Morningside Venture Investments, Ltd., which beneficially owned approximately 15.6% of our outstanding common stock. As a result, if these stockholders were to choose to act together, they may have the ability to significantly influence all matters submitted to our stockholders for approval, as well as our management and affairs. For example, these persons, if they choose to act together, could substantially influence the election of directors and approval of any merger, consolidation or sale of all or substantially all of our assets. This concentration of ownership may:

- delay, defer or prevent a change in control;
- entrench our management or the board of directors; or
- impede a merger, consolidation, takeover or other business combination involving us that other stockholders may desire.

Some of these persons or entities may have interests different than those of our other investors. For example, because many of these stockholders purchased their shares at prices substantially below the price at which other investors purchased shares and have held their shares for a longer period, they may be more interested in selling our company to an acquirer than other investors or they may want us to pursue strategies that deviate from the interests of other stockholders.

Provisions in our corporate charter documents and under Delaware law may prevent or frustrate attempts by our stockholders to change our management or hinder efforts to acquire a controlling interest in us.

Provisions in our corporate charter and our bylaws may discourage, delay or prevent a merger, acquisition or other change in control of us that stockholders may consider favorable, including transactions in which our stockholders might otherwise receive a premium for their shares. These provisions could also limit the price that investors might be willing to pay in the future for shares of our common stock, thereby depressing the market price of our common stock. In addition, because our board of directors is responsible for appointing the members of our management team, these provisions may frustrate or prevent any attempts by our stockholders to replace or remove our current management by making it more difficult for stockholders to replace members of our board of directors. Among other things, these provisions:

- establish a classified board of directors such that all members of the board are not elected at one time;
- allow the authorized number of our directors to be changed only by resolution of our board of directors;
- limit the manner in which stockholders can remove directors from the board;
- establish advance notice requirements for nominations for election to the board of directors or for proposing matters that can be acted on at stockholder meetings;
- require that stockholder actions must be effected at a duly called stockholder meeting and prohibit actions by our stockholders by written consent;
- limit who may call a special meeting of stockholders;
- authorize our board of directors to issue preferred stock without stockholder approval, which could be used to institute a "poison pill" that would work to dilute the stock ownership of a potential hostile acquirer, effectively preventing acquisitions that have not been approved by our board of directors; and
- require the approval of the holders of at least 75% of the votes that all our stockholders would be entitled to cast to amend or repeal certain provisions of our charter or bylaws.

Moreover, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the General Corporation Law of the State of Delaware, which prohibits a person who owns in excess of 15% of our outstanding voting stock from merging or combining with us for a period of three years after the date of the transaction in which the person acquired in excess of 15% of our outstanding voting stock, unless the merger or combination is approved in a prescribed manner. This could discourage, delay or prevent someone from acquiring us or merging with us, whether or not it is desired by, or beneficial to, our stockholders. This could also have the effect of discouraging others from making tender offers for our common stock, including transactions that may be in the best interests of our stockholders. These provisions may also prevent changes in our management or limit the price that investors are willing to pay for our stock.

If securities or industry analysts do not publish research or publish inaccurate or unfavorable research about our business, our share price and trading volume could decline.

The trading market for our common stock will likely depend in part on the research and reports that securities or industry analysts publish about us or our business. We do not have any control over these analysts. There can be no assurance that analysts will continue to cover us or provide favorable coverage. Securities or industry analysts may elect not to provide research coverage of our common stock, and such lack of research coverage may negatively impact the market price of our common stock. In the event we do have analyst coverage, if one or more analysts downgrade our stock or change their opinion of our stock, our share price would likely decline. In addition, if one or more analysts cease coverage of our company or fail to regularly publish reports on us, we could lose visibility in the financial markets, which could cause our share price or trading volume to decline.

Our restated certificate of incorporation designates the state courts in the State of Delaware or, if no state court located within the State of Delaware has jurisdiction, the federal court for the District of Delaware, as the sole and exclusive forum for certain types of actions and proceedings that may be initiated by our stockholders, which could discourage lawsuits against our company and our directors, officers and employees.

Our restated certificate of incorporation provides that, unless we consent in writing to the selection of an alternative forum, the Court of Chancery of the State of Delaware (or, if the Court of Chancery does not have jurisdiction, the federal district court for the District of Delaware) will be the sole and exclusive forum for any derivative action or proceeding brought on our behalf, any action asserting a claim of breach of a fiduciary duty owed by any of our directors, officers or employees to our company or our stockholders, any action asserting a claim against us arising pursuant to any provision of the General Corporation Law of the State of Delaware or our certificate of incorporation or bylaws, or any action asserting a claim against us governed by the internal affairs doctrine. This exclusive forum provision will not apply to actions arising under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended. This exclusive forum provision may limit the ability of our stockholders to bring a claim in a judicial forum that such stockholders find favorable for disputes with us or our directors, officers or employees, which may discourage such lawsuits against us and our directors, officers and employees.

Item 2. Unregistered Sales of Equity Securities and Use of Proceeds.

Sale of Unregistered Securities

On February 9, 2021, we issued 69,491 shares of common stock to the placement agent as consideration for its services in connection with our exchange of the 2019 Convertible Notes for shares of common stock. The shares of common stock issued in reliance upon the exemption from the registration requirements of the Securities Act, as set forth in Section 4(a)(2) under the Securities Act. The recipient of the securities represented that it was an accredited investor and was acquiring the securities for its own account for investment purposes only and not with a view to, or for sale in connection with, any distribution thereof and that it could bear the risks of the investment and could hold the securities for an indefinite period of time and appropriate legends were affixed to the securities

Item 6. Exhibits.

Exhibit Number	Description
10.1*†	Amended and Restated Commercial Supply Agreement, dated March 10, 2021, by and between the Registrant, Apellis Switzerland GmbH and NOF Corporation
10.2	Form of Exchange Agreement (incorporated by reference to Exhibit 10.1 to the Company's Current Report on Form 8-K filed with the Securities and Exchange Commission on January 7, 2021)
31.1*	Certification of Principal Executive Officer Pursuant to Rules 13a-14(a) and 15d-14(a) under the Securities Exchange Act of 1934, as Adopted Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
31.2*	Certification of Principal Financial Officer Pursuant to Rules 13a-14(a) and 15d-14(a) under the Securities Exchange Act of 1934, as Adopted Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
32.1*	Certification of Principal Executive Officer Pursuant to 18 U.S.C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
32.2*	Certification of Principal Financial Officer Pursuant to 18 U.S.C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
101.INS	Inline XBRL Instance Document – the instance document does not appear in the Interactive Data File because its XBRL tags are embedded within the Inline XBRL document.
101.SCH	Inline XBRL Taxonomy Extension Schema Document
101.CAL	Inline XBRL Taxonomy Extension Calculation Linkbase Document
101.DEF	Inline XBRL Taxonomy Extension Definition Linkbase Document
101.LAB	Inline XBRL Taxonomy Extension Label Linkbase Document
101.PRE	Inline XBRL Taxonomy Extension Presentation Linkbase Document
104	Cover Page Interactive Data File (formatted as Inline XBRL and contained in Exhibit 101)

* Filed herewith.
† Portions of this exhibit have been omitted pursuant to Item 601(b)(10)(iv) of Regulation S-K

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned thereunto duly authorized.

Apellis Pharmaceuticals, Inc.

Date: April 28, 2021

By: /s/ Cedric Francois
Cedric Francois
President and Chief Executive Officer
(principal executive officer)

Date: April 28, 2021

By: /s/ Timothy Sullivan
Timothy Sullivan
Chief Financial Officer and Treasurer
(principal financial officer)

Exhibit 10.1

Certain identified information has been excluded from the exhibit because it is both (i) not material and (ii) is the type of information that the registrant treats as private or confidential. Double asterisks denote omissions.

AMENDED AND RESTATED SUPPLY AGREEMENT

by and between

APELLIS PHARMACEUTICALS, INC.,

together with

APELLIS SWITZERLAND GmbH

and

NOF CORPORATION

This **AMENDED AND RESTATED SUPPLY AGREEMENT** (this "Agreement") is entered into effective as of February 12, 2021 (the "Effective Date"), by and between:

(a) (i) **APELLIS PHARMACEUTICALS, INC.,** a corporation duly organized and existing under the laws of the State of Delaware and having its principal office at 100 Fifth Avenue, Waltham, Massachusetts 02451, U.S.A., together with its subsidiary, (ii) **APELLIS SWITZERLAND GmbH,** a corporation having its principal office at Zahlerweg 10, 6300 Zug, Switzerland CH-6302 (collectively, "Apellis")

and

(b) **NOF CORPORATION,** a corporation duly organized and existing under the laws of Japan and having its principal office at 20-3, Ebisu 4-chome, Shibuya-ku, Tokyo 150-6019 Japan (hereinafter referred to as "NOF").

WHEREAS, APELLIS PHARMACEUTICALS, INC. and NOF entered into the certain DEVELOPMENT AND CLINICAL SUPPLY AGREEMENT effective as of November 1, 2016 (the "Original Agreement"), pursuant to which NOF performed certain development services and manufactured certain materials for use in clinical trials; and

WHEREAS, Apellis and NOF wish to supersede the Original Agreement with this Agreement to, among other things, include commercial supply matters.

NOW THEREFORE, Apellis and NOF, intending to be legally bound, hereby agree as follows:

1. INTRODUCTION

1.1 Apellis desires that NOF supply activated polyethyleneglycol derivative ("SUNBRIGHT [**]" as defined in Section 2.51) in accordance with the terms and conditions set forth herein; and

1.2 NOF has the capability and expertise to Manufacture and supply the SUNBRIGHT [**] in conformity with applicable laws, rules and regulations and the other terms of this Agreement, and desires to Manufacture and supply Apellis the SUNBRIGHT [**] in accordance with the terms and conditions set herein.

2. DEFINITIONS

The following terms, whether used in the singular or plural, shall have the following meanings for purposes of this Agreement:

2.1 "Affiliate" means any entity directly or indirectly controlled by, controlling, or under common control with, a Party, where "control" means direct or indirect possession of (i) the power to direct or cause direction of the management and policies of such entity (whether through ownership of securities or partnership or other ownership interests, by contract or otherwise), or (ii) more than fifty percent (50%) of the voting securities of such entity.

2.2 "Apellis IP" means (i) all technology, know-how, inventions, discoveries, ideas, concepts, trade-secrets, improvements, processes, process improvements, information, or data, whether patentable or not, which are related to the Drug Substance or Drug Product, including those that arise from the Manufacturing services hereunder; and (ii) any intellectual property rights therein. For the avoidance of doubt, manufacturing process of SUNBRIGHT [**], including those that arise from the Manufacturing services hereunder is excluded from Apellis IP.

2.3 "Apellis Property" has the meaning set forth in Section 12.1.

2.4 "Batch" means the SUNBRIGHT [**] that results from a single Manufacturing process, inclusive of Materials and testing.

2.5 "Batch Record" means the complete written record of the history of the Batch and its production and processing thereof in accordance with the Master Batch Record, including Materials, raw data, production records, sampling documentation, in-process test results, release data, investigative and corrective action reports, deviation reports, all applicable processing data (including any pertinent output from instrumentation), the Batch Release, Certificate of Analysis and any other related controls required by cGMPs.

2.6 "Batch Release" means the final written approval, signed by NOF's (or its subcontractor's or CMO's, as applicable) relevant quality assurance ("QA")/quality control ("QC") officer, marking the culmination of the quality process through which a Batch is shown to conform to cGMPs, the applicable Specifications, and all applicable Laws.

2.7 "Business Day" has the meaning set forth in Section 15.4.

2.8 "Certificate of Analysis" means the written certificate of analysis for each Batch in a form reasonably acceptable to Apellis and signed by NOF's relevant QA/QC officer, which certifies the actual content of the Batch.

2.9 "CMO" means a subcontractor of NOF which performs a step in the Manufacture of SUNBRIGHT [**] on behalf of NOF as designated in Exhibit F. Exhibit F may be updated with the agreement of the Parties from time to time.

2.10 "cGMPs" means the part of quality assurance which ensures that the SUNBRIGHT [**] is consistently produced and controlled in accordance with the then applicable current good manufacturing practices relating to the Manufacture of SUNBRIGHT [**], including the current Good Manufacturing Practices as specified in the United States Code of Federal Regulations (the "CFR") Part 210, the European Union ("EU") Good Manufacturing Guidelines (Part II), the ICH Good Manufacturing Practice Guide for Active Pharmaceutical Ingredients (Q7), and the MHLW GMP/GQP ordinances and accompanying regulations in Japan, as applicable quality standards appropriate to their intended use as defined in ICH Q7 Guideline. If additional requests for the cGMPs are needed, Apellis and NOF shall cooperate with each other to fulfill such requests.

2.11 "Confidential Information" means all information, data, know-how, and all other proprietary and confidential business, technical, and financial data disclosed hereunder, by one Party (the "Disclosing Party") or any of its Affiliates to the other Party (the "Receiving Party") or any of its Affiliates, except to the extent such information: (i) at the time of disclosure, is generally available to the public, other than by a breach of the Receiving Party or any of its Affiliates of any confidentiality obligation owed to the Disclosing Party or any of its Affiliates; (ii) after disclosure hereunder, becomes generally available to the public, except through breach by the Receiving Party or any of its Affiliates of this Agreement or any other confidentiality obligation owed by the Receiving Party or any of its Affiliates to the Disclosing Party or any of its Affiliates; (iii) the Receiving Party can demonstrate by contemporaneous written records was in its or its Affiliate's possession prior to the time of such disclosure by the Disclosing Party or any of its Affiliates hereunder, and was not acquired directly or indirectly from the Disclosing Party or any of its Affiliates; (iv) becomes available to the Receiving Party from a Third Party (as defined in Section 2.44) that is not legally prohibited from disclosing such Confidential Information, provided such Confidential Information was not acquired directly or indirectly from the Disclosing Party or any of its Affiliates; or (v) the Receiving Party can demonstrate by contemporaneous written records was developed by or for the

Receiving Party or any of its Affiliates independently of the disclosure of Confidential Information by the Disclosing Party or any of its Affiliates. All Apellis Property, whether disclosed by Apellis or its Affiliates to NOF or its Affiliates or developed under this Agreement, is considered Confidential Information of Apellis and not of NOF, with Apellis considered the Disclosing Party and NOF considered the Receiving Party, and NOF may not rely on clauses (iii), (iv) or (v) with respect thereto.

2.12 "Delivery" or "Deliver" or "Delivered" means NOF's delivery of SUNBRIGHT [**] pursuant to a given Firm Order in accordance with the Delivery Terms and the provisions of this Agreement.

2.13 "Delivery Address" means, with respect to a given order of SUNBRIGHT [**], the address where the quantities of SUNBRIGHT [**] under such order are to be shipped, as set forth in the applicable order.

2.14 "Delivery Date" means the date by which Apellis shall take delivery of SUNBRIGHT [**] as set forth in a Firm Order.

2.15 "Delivery Terms" means DAP (Incoterms 2010) NOF's designated Facility for the finished, packaged and labelled SUNBRIGHT [**].

2.16 "Disclosing Party" has the meaning set forth in Section 2.11.

2.17 "Drug Product" means a finished dosage form of a product containing the Drug Substance.

2.18 "Drug Product IND" means an investigational new drug application for a Drug Product, and any supplements or amendments thereto, as may be filed with the FDA or applicable Regulatory Agency with jurisdiction over the Drug Product during or after the term hereof or any corresponding foreign application, registration, or certification of each jurisdiction in the Territory.

2.19 "Drug Product NDA" means new drug application for a Drug Product, and any supplements or amendments thereto, as may be filed with the FDA or applicable Regulatory Agency with jurisdiction over the Drug Product during or after the term hereof or any corresponding foreign application, registration, or certification of each jurisdiction in the Territory.

2.20 "Drug Substance" means the pharmaceutical ingredient obtained by conjugation with SUNBRIGHT [**].

2.21 "DSCSA" means the United States Drug Supply Chain Security Act (21 U.S.C. §581 et seq.) and applicable regulations promulgated thereunder, as amended from time to time.

2.22 "Effective Date" has the meaning given in the heading paragraph.

2.23 "Facility" means NOF's manufacturing facility at [**], or any other facility of NOF that Manufactures SUNBRIGHT [**] as may be agreed upon in writing by the Parties.

2.24 "FDA" means the United States Food and Drug Administration, or any successor entity.

2.25 "Firm Commitment" shall have the meaning set forth in Section 4.7(a).

2.26 "Firm Order" has the meaning set forth in Section 4.7(f).

2.27 "First Commercial Sale" means the first sale for use by the general public after approval of the Drug Product by the applicable Regulatory Agency.

2.28 "Indemnified Party" means the Party seeking indemnification for any Liabilities pursuant to Section 13.

2.29 "Indemnifying Party" means the Party responsible for indemnifying the Indemnified Party pursuant to Section 13.

2.30 "Laws" means (i) any present and future national, state, local or similar laws (whether under statute, rule, regulation or otherwise) and (ii) requirements under permits, orders, decrees, judgments or directives, and requirements of applicable Regulatory Agencies (including, without limitation, cGMP) (with respect to each of the foregoing, as amended or revised from time to time).

2.31 "Liabilities" has the meaning set forth in Section 13.1.

2.32 "Manufacture" or "Manufacturing" or "Manufactured" means with respect to SUNBRIGHT [**], all operations performed by or on behalf of NOF for the manufacture and supply of SUNBRIGHT [**] pursuant to this Agreement, including, as applicable, receipt (including testing) and storage of Materials, production, manufacture, visual inspection, packaging, labeling, handling, warehousing, quality control testing (including in-process, release and stability testing), release, as applicable, and shipping of SUNBRIGHT [**], and also including such activities as may be specified in the Master Batch Record.

2.33 "Marketing authorization application for the Territory" means the process of reviewing and assessing the dossier to support a medicinal product in view of its marketing which is performed within a legislative framework that defines the requirements necessary for application to the respective regulatory authority. The application dossier for marketing application is called Marketing Authorization Application in the Territory, or simply registration dossier.

2.34 "Master Batch Record" means the master production instructions for manufacture of a batch of SUNBRIGHT [**].

2.35 "Material" means any item, document or article used or related to the development or production of the SUNBRIGHT [**].

2.36 "Minimum Purchase Obligation" has the meaning set forth in Exhibit E.

2.37 "NOF Supplied Materials" has such meaning as set forth in Section 4.2.

2.38 "Party" means Apellis or NOF, as applicable.

2.39 "Permitted Variance Deficient Quantities" has the meaning set forth in Section 4.10.

2.40 "Permitted Variance Excess Quantities" has the meaning set forth in Section 4.10.

2.41 "QA" has the meaning set forth in Section 2.6.

2.42 "QC" has the meaning set forth in Section 2.6.

2.43 "Quality Agreement" means the Quality Agreement that defines the individual responsibilities of the Parties as to the quality aspects of Manufacturing and release of the SUNBRIGHT [**] to ensure compliance with the Specifications and applicable Laws, which agreement is attached as Exhibit G, as such agreement may be amended by the Parties from time to time in accordance therewith.

2.44 "Receiving Party" has the meaning set forth in Section 2.11.

2.45 "Regulatory Agency" means (i) the FDA and any successor authority; (ii) EMA (European Medicines Agency), European Commission or the Council of the European Union and any successor authorities, (iii) PMDA (Japanese Pharmaceuticals and Medical Devices Agency) and any successor authority; and (iv) any similar governmental agency in the Territory that is responsible for granting manufacturing, marketing, price or reimbursement price authorizations and includes any national, supra-national, state and local regulating group, department, bureau, commission, council and other governmental entity that has jurisdiction over the Drug Substance, or a Drug Product, as applicable, whether with respect to its development, manufacture, handling, storage, transportation, destruction, or otherwise.

2.46 "Regulatory Submission" means any application or filing identified in the applicable Statement of Work or required by FDA regulations, as amended from time to time, and the equivalent application or filing for each country or super-national jurisdiction in the Territory, including the Drug Product IND and the Drug Product NDA.

2.47 "Rolling Forecast" shall have the meaning set forth in Section 4.7(a).

2.48 "Shelf Life" means the period from Manufacturing date to retest date specified in Certificate of Analysis.

2.49 "SOPs" means the applicable standard operating procedures of NOF, including any SOPs relating to the SUNBRIGHT [**], any of the NOF Supplied Materials or any other Material.

2.50 "Specifications" means the applicable specifications, standards, criteria, limits, and other requirements for or related to the SUNBRIGHT [**] provided hereunder, as set forth on Exhibit C and as revised or supplemented by the Parties in writing from time to time pursuant to this Agreement to conform with the specifications for the SUNBRIGHT [**] set forth in the New Drug Application (NDA) approved by the FDA, as such specifications may be modified from time to time in response to actions by the FDA or another Regulatory Agency without the need to amend this Agreement.

2.51 "SUNBRIGHT [**]" means the polyethyleneglycol derivative identified in Exhibit A.

2.52 "Supply Failure" shall have the meaning set forth in Section 4.7(e).

2.53 "Supply Price" means the supply price to be paid to NOF for the Manufacture and supply of the SUNBRIGHT [**] as set forth on Exhibit B.

2.54 "Territory" means all countries in the world.

2.55 "Third Party" means any person or entity other than the Parties and their Affiliates.

3. PERFORMANCE OF THIS AGREEMENT BY NOF'S SUBSIDIARY. NOF appoints its Affiliate, NOF AMERICA CORPORATION, duly organized under the laws of the state of Delaware, and having its principal office at One North Broadway, Suite 912, White Plains, N.Y. 10601 U.S.A. ("NAC") as a distributor and representative of NOF for any of communication with Apellis, provided, however, NOF

shall cause NAC to abide by the terms and conditions of this Agreement, as if it were a Party hereto, and NOF shall be responsible for any performance of the obligations by NAC.

4. MANUFACTURE OF SUNBRIGHT [**]

4.1 Manufacture. NOF shall Manufacture the SUNBRIGHT [**] in accordance with SOPs, the Specifications, applicable Laws (including cGMPs), and the terms of this Agreement and the Quality Agreement. Except as may be specifically permitted pursuant to Section 4.3 to a specific approved subcontractor, NOF shall Manufacture the SUNBRIGHT [**] only in the Facility.

4.2 NOF Supplied Materials. Unless otherwise provided for in this Agreement, NOF shall obtain and maintain in the Facility equipment, raw materials, and other associated Materials and resources necessary to Manufacture the SUNBRIGHT [**] ("NOF Supplied Materials"). NOF shall establish a source of supply for all NOF Supplied Materials sufficient to ensure NOF's ability to fulfill its obligations hereunder in a timely manner.

4.3 Approval of Subcontracting. For supply of the SUNBRIGHT [**], NOF shall not subcontract, sublicense or otherwise delegate all or any portion of its obligations under this Agreement without Apellis's prior written approval; provided, however, that with respect to the specific activities to the specified subcontractors set forth on Exhibit F, a separate written approval shall not be required from Apellis. To the extent such approvals are granted or activities are subcontracted to NAC, NOF shall (i) fully qualify such subcontractor, and Apellis shall have the right to participate in such qualification process; (ii) ensure that all such qualified subcontractors comply with the provisions of this Agreement; and (iii) be responsible for each such subcontractor's performance hereunder (including any breach of this Agreement caused by such subcontractor), as if NOF were itself performing such activities. If additional Process Validation (PV) production at the CMO is needed according to Apellis' request, Apellis shall pay for cost associated with protocol, QC testing, and process validation reports provided that such costs will not exceed $[**] for each process validation campaign.

4.4 Compliance with Law. NOF shall perform all Manufacturing under this Agreement in conformance with all applicable Laws, including cGMPs.

4.5 Environmental; Health and Safety. In carrying out its obligations under this Agreement, NOF will comply with all applicable environmental and health and safety laws in Japan, U.S.A. and EU that NOF recognized at the time of this Agreement. In addition to the foregoing, NOF will take all reasonable actions necessary to avoid spills and other safety concerns to persons and damage to property or the environment resulting from the SUNBRIGHT [**] or any intermediates or raw materials thereof. NOF shall handle, label, package, store, transport and dispose of all wastes generated in the course of Manufacturing in accordance with all applicable Laws.

4.6 Schedules for Manufacture and Orders for SUNBRIGHT [**]. NOF will Manufacture and Deliver the SUNBRIGHT [**] in accordance with the Firm Orders set forth in Section 4.7.

4.7 Forecasts; Orders.

(a) On or before the [**] of each [**] during the Term of this Agreement, Apellis agrees to provide NOF with a written twenty-four (24) month rolling forecast of Apellis's projected delivery requirements of SUNBRIGHT [**] during the following twenty-four (24) month period ("Rolling Forecast"). The quantity of SUNBRIGHT [**] specified for a given month in a given Rolling Forecast shall be subject to the following limitations: (i) the first [**] of the Rolling Forecast shall represent a binding

order for the quantities of SUNBRIGHT [**] specified therein, for delivery during such [**] period ("Firm Commitment") and shall be one hundred percent (100%) binding; (ii) each of the next [**] of the Rolling Forecast shall specify quantities of SUNBRIGHT [**] which are no more than one hundred percent (100%) or less than [**] percent ([**]%) of the quantity of SUNBRIGHT [**] forecasted for such month in the immediately preceding Rolling Forecast provided to NOF; (iii) each of the next [**] of the Rolling Forecast after clause (ii) shall specify quantities of SUNBRIGHT [**] which are no more than one hundred percent (100%) or less than [**] percent ([**]%) of the quantity of SUNBRIGHT [**] forecasted for such month in the immediately preceding Rolling Forecast provided to NOF (each of clause (ii) and (iii) collectively, the "Collared Portion"); and (iv) each of the remaining months shall be good faith non-binding estimates.

(b) By way of example, if the Firm Commitment for SUNBRIGHT [**] is [**], then: (1) each of [**] through [**] of a given Rolling Forecast shall not vary from the immediately preceding Rolling Forecast provided to NOF [**]; (2) each of months [**] shall specify quantities of SUNBRIGHT [**] which are no more than one hundred percent (100%) or less than [**] percent ([**]%) of the quantity forecasted for such months when such months were the [**] months, respectively, of the last Rolling Forecast provided to NOF; (3) each of months [**] shall specify quantities of SUNBRIGHT [**] which are no more than one hundred percent (100%) or less than [**] percent ([**]%) of the quantity forecasted for such months when such months were the [**] months, respectively, of the last Rolling Forecast provided to NOF, and (4) the remaining months shall be non-binding good-faith estimate of the quantities of SUNBRIGHT [**] to be ordered for delivery during such period.

(c) An example of the foregoing Rolling Forecast and Collared Portions is attached hereto as Exhibit H.

(d) Within [**] of receipt of the Rolling Forecast, NOF shall reply in writing whether it accepts the quantities proposed and to meet the delivery dates proposed in the Rolling Forecast; provided, however, NOF shall have no ability to reject or not accept a Rolling Forecast or Firm Commitment to the extent such Rolling Forecast and Firm Commitments are consistent with the foregoing and the terms and conditions of this Agreement. If the Rolling Forecast is inconsistent with the foregoing or this Agreement, the Parties shall discuss in good faith and use their reasonable efforts to revise the Rolling Forecast; provided, however, that NOF shall use commercially reasonable effort to accept any part of the Rolling Forecast which is inconsistent with the Firm Commitment or the Collared Portion. NOF agrees to Manufacture and supply, and Apellis agrees to purchase, the quantities of SUNBRIGHT [**] set forth in the Firm Commitment, in accordance with this Agreement. In addition and in order to provide Apellis with the necessary transparency it needs to enable Apellis to provide NOF with reasonable forecasts, NOF will provide Apellis with a manufacturing and delivery plan and any related information into its or its subcontractors' capacity or ordering limitations. NOF shall provide access to and information regarding its CMO and ordering commitments therefrom.

(e) For purposes of clarity, the Parties acknowledge that (a) NOF is not the exclusive or partially exclusive supplier of SUNBRIGHT [**] or any polyethyleneglycol derivative to Apellis, (b) Apellis may purchase SUNBRIGHT [**] or any polyethyleneglycol derivative from any Third Party, and (c) Apellis has not limited its remedies hereby to only those specified for a Supply Failure or material breach by NOF. "Supply Failure" shall have the meaning that if NOF is unable to supply at least [**]% of any Firm Order for SUNBRIGHT [**] within the Firm Commitment within [**] after the Delivery Date set forth on the applicable Firm Order for any reason (other than Force Majeure not caused by Apellis).

(f) "Firm Order" means a purchase order for SUNBRIGHT [**] issued by Apellis under this Agreement that specifies the quantity of SUNBRIGHT [**] ordered, the required Delivery Date, and the Delivery Address (as well as any specific shipping instructions, if applicable), in each instance in

accordance with this Agreement. Apellis shall place Firm Orders for SUNBRIGHT [**] in accordance with the Firm Commitment portion of its Rolling Forecast for the relevant period at least [**] before the requested Delivery Date. Firm Orders will be made on such form of purchase order or document as Apellis may specify from time to time in writing; provided that the terms and conditions of this Agreement shall be controlling over any terms and conditions included in any Firm Order. Any term or condition of such Firm Order that is different from or contrary to the terms and conditions of this Agreement shall be void, unless otherwise agreed between the Parties in writing.

(g) NOF agrees to retain [**] of each lot of SUNBRIGHT [**] produced for either (i) [**] after product inventory at NOF runs out, or (ii) [**] after the retest date, whichever is longer.

4.8 Changes to Manufacture. Any material changes to the Manufacture process or the Specifications shall require the Parties' mutual written consent in accordance with the Quality Agreement, and an applicable change to the Master Batch Record in accordance with Section 15.7 of this Agreement, before implementation. Notwithstanding anything herein to the contrary or in the Quality Agreement, except as otherwise agreed to by Apellis in writing, NOF shall not amend, change, or supplement any of the following without Apellis's prior written consent: (1) the Specifications; (2) the Materials; (3) the specifications for Materials that have regulatory impact (e.g., specification is listed in the regulatory filing) or the potential for quality impact on the SUNBRIGHT [**]; (4) the source of Materials that have regulatory impact (e.g., supplier is listed in the regulatory filing) or the potential for quality impact on the SUNBRIGHT [**]; (5) the equipment and machinery, other than in-kind replacements, used in the Manufacture of SUNBRIGHT [**] that have a direct impact on the quality of the SUNBRIGHT [**]; (6) the test methods used in connection with the Manufacturing of SUNBRIGHT [**] that have regulatory impact (e.g., method is listed in the regulatory filing) or the potential for quality impact on the SUNBRIGHT [**]; or (7) the process for Manufacturing SUNBRIGHT [**]. If any of the foregoing changes are required by Regulatory Agency or a change in cGMP and such requirement is solely directed to SUNBRIGHT [**], then Apellis shall be responsible for reimbursing NOF for the costs of such required change and, in all other cases, NOF shall bear all costs of such change.

4.9 Minimum Purchase Obligation.

(a) Apellis agrees to purchase SUNBRIGHT [**] from NOF under the estimated annual forecasts in Exhibit D, to the extent set forth in the Firm Commitment portion of the Rolling Forecasts and subject to the Minimum Purchase Obligations set forth in Exhibit E. Except as expressly set forth in Exhibit E, Apellis shall order from NOF, and NOF shall supply, at least the minimum amount of the SUNBRIGHT [**] for delivery as provided in Exhibit E attached hereto and incorporated herein, or of its proportionate amount of such SUNBRIGHT [**] in case of less than [**]. If Apellis fails to purchase the quantity of SUNBRIGHT [**] which satisfies the Minimum Purchase Obligation for a particular time period, Apellis shall pay NOF the amount equal to [**]% of the remaining quantity of the Minimum Purchase Obligation for such time period without affecting the obligations of Apellis to purchase and pay for 100% of the Firm Orders previously issued. Beginning [**], the Parties shall discuss in good faith quantities of Minimum Purchase Obligation for calendar years [**].

(b) Notwithstanding the foregoing, Apellis may terminate the Minimum Purchase Obligation for the remaining term of this Agreement by providing notice to NOF on or before October 1 of the preceding year, subject to the buy-out payment obligations provided in this Section. In the case Apellis terminates the Minimum Purchase Obligation for the remaining Term of this Agreement ("Buy-Out Option "), then, as Apellis's exclusive liability and NOF's sole remedy, Apellis shall pay NOF an amount equal to the Buy-Out Calculation for [**] percent ([**]%) of the aggregate of the remaining Minimum Purchase Obligation for remaining time periods thereafter of the Term of this Agreement; provided,

however, if this Agreement is terminated by NOF for convenience or by Apellis for NOF's breach, Apellis shall have no obligation to pay any further Minimum Purchase Obligation and the Buy-Out Calculation shall be of no force or effect.

(c) The amount of the payment required to be made under Section 4.9(a) and Section 4.9(b) for Buy-Out Option shall be calculated based on the applicable per-gram Supply Price set forth in Exhibit B ("Buy-Out Calculation"). Invoices for the appropriate payment will be issued by NOF within [**] from the end of the applicable time period or the effective date of NOF's receipt of the applicable buy-out notice, as the case may be, and shall be payable within [**] of the date of receipt of the invoice from NOF.

(d) Nothing herein shall be construed as limiting the amount of SUNBRIGHT [**] that Apellis may purchase from NOF, nor the amount of SUNBRIGHT [**] that NOF may supply to Apellis, in any given calendar year during the Term of this Agreement, subject only to the applicable provisions relating to Rolling Forecasts, as set forth in Section 4.7.

(e) For purposes of clarification, Apellis may exercise Buy-Out Option pursuant to this Section 4.9 without terminating this Agreement, and such exercise of Buy-Out Option shall not be construed to be a termination of this Agreement but instead this Agreement shall remain as an agreement without the Minimum Purchase Obligation for the relevant time period.

4.10 Delivery Against Firm Orders. NOF will acknowledge all Firm Orders submitted under Section 4.7(f) within [**] following receipt of same. NOF shall Deliver SUNBRIGHT [**] only against specific Firm Orders. NOF shall make reasonable effort to Deliver SUNBRIGHT [**] under each Firm Order on the Delivery Date specified in the applicable Firm Order; provided, however, that Delivery of SUNBRIGHT [**] within [**] before or after the Delivery Date may be permissible. The Delivery Date may be changed with agreement of the Parties. NOF shall Deliver SUNBRIGHT [**] under each Firm Order in the quantities set forth in such Firm Order; provided that NOF shall be deemed to have satisfied its obligations with respect to the quantity of a given SUNBRIGHT [**] if the actual quantity of SUNBRIGHT [**] is within plus or minus [**] percent (+/-[**]%) of the quantity of such SUNBRIGHT [**] set forth in the applicable Firm Order (the amount of such excess quantity of SUNBRIGHT [**] actually Delivered that is above the amount requested in the Firm Order, if any, the "Permitted Variance Excess Quantities", and the amount of such deficient quantity of SUNBRIGHT [**] actually Delivered that is below the amount requested in the Firm Order, if any, the "Permitted Variance Deficient Quantities"). For the avoidance of doubt, Apellis shall have no obligation to pay for any quantity of SUNBRIGHT [**] delivered in excess of the Permitted Variance Excess Quantities. The Facility shall be indicated on documents accompanying each Delivery of SUNBRIGHT [**]. In the event NOF will fail to meet a Delivery Date set forth in a Firm Order, NOF shall bear the incremental costs required for expedited transport above and beyond the cost incurred by the method outlined in the Delivery Terms. In the event that Apellis fails to take delivery of the SUNBRIGHT [**] on the Delivery Date, Apellis will be responsible for any costs incurred by NOF in connection with a delay in delivery. For the avoidance of doubt, any Permitted Variance Excess Quantities shall count against the Minimum Purchase Obligations in the then current period.

4.11 Quality Control and Batch Release. NOF shall provide to Apellis copies of the current and approved Master Batch Record and validated analytical test methods and validation protocols and reports through a secure electronic portal, and all such documents must be provided in English and any updates or revisions thereto must also be provided in English. NOF and, where applicable, its subcontractor or CMO, shall perform such quality control tests as indicated in the Specifications, as required by applicable Law and regulatory requirements and in accordance with the validated analytical test methods and written

procedures. NOF shall provide through a secure electronic portal, and where applicable, its subcontractor's or CMO's Certificate of Analysis and investigations for Out of Specification (OOS) and for deviations of manufactured Batches must be provided in English to Apellis at least [**] prior to the Delivery Date of the corresponding Batches of SUNBRIGHT [**] in accordance with the Quality Agreement. Executed Batch Records and executed quality control data package for each Batch in the original language (Japanese) shall be provided through a secure electronic portal. Should any Batch fail to meet the standards set forth in the Specifications, Apellis may, at its option, investigate the cause of such failure or require NOF, its subcontractor or CMO to do so and to provide Apellis with a written report summarizing the results of such investigations. For clarity, all documents provided pursuant to this Section 4.11 through an electronic portal may be provided in read only format so long as NOF, its subcontractor or CMO maintains hard copies of all such documents and Apellis can access such documents through the electronic portal for a period of [**], or such longer period as may be required by cGMP. Notwithstanding the foregoing, from a security standpoint, all such documents may be removed from the portal after [**] of their upload; provided that (a) NOF notifies Apellis at least [**] prior to such removal and (b) during such [**] period or longer period as may be required by cGMP, all such documents shall be re-uploaded to the electronic portal for [**] within [**] upon Apellis's requests. In addition, Apellis agrees that Master Batch Record, Executed Batch Records and executed quality control data packages shall be handled based on the separately agreed Confidential Agreement effective as of [**].

4.12 NOF Obligations. NOF agrees that it shall not enter into any agreement with any Third Party that shall have a conflict of interest with performance of its obligations under this Agreement and neither NOF nor its Affiliates shall intentionally or knowingly conduct or otherwise carry out at any time during the Term any activities that may prejudice the quality, safety, efficacy, Manufacture or delivery of the SUNBRIGHT [**]. As between the Parties, NOF shall be solely responsible, at its sole cost and expense, for performance of all Manufacturing. In furtherance of the foregoing, NOF agrees to provide, at its sole cost and expense, all labor and expertise necessary for the performance of the Manufacturing of SUNBRIGHT [**] as well as all facilities, equipment, machinery and Materials (other than Apellis Supplied Materials) necessary to Manufacture the SUNBRIGHT [**], including maintaining sufficient stocks of Materials necessary to supply Apellis's requirements of SUNBRIGHT [**] under this Agreement.

4.13 Safety Stock. NOF shall maintain a safety stock of the [**] raw material in a quantity that is sufficient to Manufacture at least [**] of SUNBRIGHT [**] (the "Safety Stock") from [**] and thereafter throughout the Term. This Safety Stock shall remain separate and distinct from any other quantities of [**] inventory held at NOF's Facility and shall be stored by NOF. NOF will maintain the appropriate level of Safety Stock by promptly replenishing that quantity of [**] used in such supply in accordance with Section 4.7. NOF will manage Safety Stock to fulfill Apellis purchase orders on a routine basis to prevent units of [**] being held in the Safety Stock from exceeding their posted expiration date. NOF shall replenish its Safety Stock within [**] of use pursuant to this Section 4.13 (the "Replenishment Period"). NOF shall within [**] of the end of the Replenishment Period notify Apellis in writing of its inability to replenish the Safety Stock as aforesaid. Upon expiration or earlier termination of this Agreement, Apellis shall purchase the Safety Stock in accordance with Section 9.3.

4.14 Packaging. All SUNBRIGHT [**] supplied hereunder shall be packaged in accordance with the Specifications and the Quality Agreement. Without limiting the foregoing, all SUNBRIGHT [**] supplied hereunder shall also be labeled with a traceable batch number and the date of Manufacture.

4.15 Transfer of Title. Title to SUNBRIGHT [**] supplied hereunder shall pass to Apellis contemporaneously with the transfer of risk of loss, as established by the Delivery Terms.

5. PROJECT MANAGEMENT; CONTACTS

Each Party shall designate in writing one person to be that Party's project manager with overall responsibility for issues associated with the performance of this Agreement. Each Party may designate one or more additional contact persons whom the other Party may contact with respect to particular matters. Each Party may substitute or replace its project manager and additional contact persons at any time by providing written notice to the other Party. Each Party shall make its project manager and other contact persons available for status meetings, telephone consultation, and otherwise as reasonably required to facilitate the performance of the Manufacturing services in accordance with this Agreement. The project managers shall hold meetings at such times as the Parties so elect to do so but shall meet no less frequently [**]. The initial project managers and the additional contact persons who shall be contacted by NOF or Apellis in regard to various matters are:

Apellis:

Department	Function	Name	Tel. Number	E-mail
CMC	Manufacturing	[**]	[**]	[**]
QA	QA, Contract	[**]	[**]	[**]
Analytical development and QC	QC	[**]	[**]	[**]

NOF:

Department	Function	Name	Tel. Number	E-mail
NAC	Manager, Sales of DDS Product	[**]	[**]	[**]

6. FEES

6.1 Price. In full consideration for the Manufacture and supply of SUNBRIGHT [**], Apellis shall pay NOF the Supply Price for the SUNBRIGHT [**] provided under this Agreement as set forth in Exhibit B.

6.2 Invoice. NOF shall submit invoices of [**] percent ([**]%) of Supply Price to Apellis after the receipt of each Firm Order from Apellis hereunder and invoices of remaining [**] percent ([**]%) of Supply Price to Apellis simultaneously with the delivery to Apellis of the Batch Release documentation pursuant to Section 4.11. Apellis shall pay NOF [**]% of Supply Price within [**] of the invoice. Upon quality assurance disposition by Apellis and written acceptance of the Batch Release documentation, Apellis shall pay NOF [**]% of Supply Price within [**] of the invoice and prior to Delivery of the SUNBRIGHT [**], provided however, NOF may change the payment terms to any other terms, including, but not limited to advance payment, depending on the credit status of Apellis; provided, however, if the Delivery is received more than [**] after the required Delivery Date and Apellis accepts such delayed shipment, NOF shall issue, and Apellis shall receive, a [**] percent ([**]%) discount off the invoiced Supply Price for such shipment. If Apellis fails to pay any invoice by the due date, NOF is not required to ship the Firm Order until Apellis makes the payment. Notwithstanding the foregoing, if Apellis fails to pay by the payment due date under this Agreement, NOF may (i) reject Firm Orders submitted by Apellis or cancel accepted Firm Orders (applicable only for the initial invoice), or (ii) terminate this Agreement pursuant to Section 9.2.(a).

6.3 Taxes. Apellis shall have obligation to pay all taxes and duties allocated to it under DAP certain location agreed by the Parties (Incoterms 2010).

7. STORAGE; DELIVERY; ACCEPTANCE AND REJECTION

7.1 Storage, Delivery and Shipping Terms. NOF is responsible for maintaining and monitoring the appropriate temperature storage conditions for all Material and SUNBRIGHT [**] while Material and SUNBRIGHT [**] are under its control. NOF is also responsible for packaging SUNBRIGHT [**] in a manner that will ensure maintenance of the appropriate physical and temperature conditions during shipment. All Apellis orders shall be shipped DAP (Incoterms 2010) from Facility.

7.2 Acceptance and Rejection. In the event that any SUNBRIGHT [**] delivered to Apellis or any Apellis designated location fails to conform to the Specifications, was not Manufactured in accordance with cGMP or the Quality Agreement, is damaged or defective, is otherwise adulterated or misbranded under applicable Laws, or has not been stored or shipped in such a manner that ensures maintenance of the appropriate physical and temperature conditions or has obvious defects, Apellis may reject such Batch by providing NOF written notice within [**] of the Delivery of the SUNBRIGHT [**]. Obvious defects shall include any defects reasonably discoverable on visual examination of representative samples of a consignment. Apellis shall notify NOF in writing of any non-obvious defects promptly upon discovery thereof but in no event later than after the Shelf Life of the applicable SUNBRIGHT [**] that are not rejected within the foregoing applicable period will be deemed to have been accepted by Apellis, and any defect or nonconformity thereof will be deemed waived by Apellis. Apellis shall use commercially reasonable efforts to test a quantity of the SUNBRIGHT [**] delivered by NOF in order to verify that such quantity satisfies the Specifications. Any notice of rejection given hereunder shall specify the applicable nonconformity and furnish such other written evidence or other documentation as may be reasonably requested by NOF. Apellis shall ship, at NOF's expense, such allegedly nonconforming SUNBRIGHT [**] to NOF's facility for inspection and testing by NOF and, if NOF's inspection and testing reveals to NOF's reasonable satisfaction or the independent laboratory confirms pursuant to Section 7.3 or it is resolved pursuant to arbitration set forth in Section 15.16 that any quantity of the SUNBRIGHT [**] supplied by NOF does not conform to the Specifications or is otherwise defective, NOF shall, at Apellis's discretion, either (a) replace such nonconforming SUNBRIGHT [**] in a timely manner at no additional cost to Apellis or (b) refund Apellis the total Supply Price paid or payable by Apellis with respect to such non-conforming SUNBRIGHT [**]. Any dispute between the Parties regarding the quality of any quantity of the SUNBRIGHT [**] supplied shall be resolved in accordance with the procedure set forth in Section 7.3. The Parties shall determine in good faith how to dispose of any nonconforming SUNBRIGHT [**], provided that Apellis shall have no financial liability for the disposition or resale of nonconforming SUNBRIGHT [**]. EXCEPT FOR SUCH OTHER REMEDIES AND OBLIGATIONS SET FORTH IN THIS AGREEMENT, INCLUDING NOF'S INDEMNIFICATION OBLIGATIONS AND APELLIS'S RIGHT TO TERMINATE THIS AGREEMENT, THIS SECTION 7.2 SETS FORTH THE SOLE REMEDY OF APELLIS AND ENTIRE LIABILITY OF NOF FOR FAILURE OF SUNBRIGHT [**] TO CONFORM TO THE SPECIFICATIONS OR FOR THE SALE OF SUNBRIGHT [**] THAT IS DEFECTIVE, OR IS OTHERWISE ADULTERATED OR MISBRANDED UNDER APPLICABLE LAWS, OR HAS NOT BEEN MANUFACTURED IN ACCORDANCE WITH CGMP OR THE QUALITY AGREEMENT OR OTHERWISE STORED OR SHIPPED IN SUCH A MANNER THAT ENSURES MAINTENANCE OF THE APPROPRIATE PHYSICAL AND TEMPERATURE CONDITIONS.

7.3 Conflict Resolution. In the event that a dispute arises between the Parties under Section 7.2 due to analytical issues, the resolution shall conform to cGMP guidance on out-of-Specification results and shall proceed in stages as follows: The first stage requires immediate communication between scientists

representing both Parties to determine that the methods are being executed in the same manner at both sites, if applicable. Secondly, carefully controlled and split samples shall be exchanged to attempt to resolve the issue, if applicable. Should there be a failure to achieve resolution within a reasonable time, scientists from both Parties shall meet to work through the analysis of one or more mutually agreed sample(s). If the dispute is not resolved within [**], the Parties agree to submit a sample of the quantity in question to an independent test facility to be agreed upon by both Parties, such agreement not to be unreasonably withheld, and to accept the results of the testing performed by that facility as binding with regard to the quantity from which the sample was taken for purposes of this Agreement. Apellis will engage the independent test facility and pay the charges unless the results show that the quantity was nonconforming, in which case NOF shall pay the charges. If the Parties are unable to settle a dispute under Section 7.2 that is not due to analytical issues, then upon the demand of either Party, the matter shall be submitted to binding arbitration set forth in Section 15.16.

8. REGULATORY MATTERS

8.1 Regulatory Support. NOF shall cooperate with Apellis as reasonably requested with respect to Regulatory Submissions in the Territory regarding the Drug Substance or Drug Product or that are otherwise necessary to conduct clinical trials with the Drug Product, to obtain or maintain Regulatory Submissions and/or any Drug Product NDA, and/or to market and sell the Drug Product, including providing Apellis with all reports, authorizations, certificates, methodologies, specifications and other documentation and/or information in the possession or under the control of NOF (or any of its Affiliates) relating to the Manufacture of SUNBRIGHT [**] or any component thereof reasonably requested by Apellis for any of the foregoing; provided that (a) to the extent the reports, authorizations, certificates, methodologies, specifications and other documentation and/or information are within the scope or of a similar nature to those set forth on Exhibit I or similar or of the same scope as that which has previously been provided to Apellis (including in connection with the Original Agreement) ("NOF Specific Information"), NOF shall be required to provide Apellis with all such authorizations, certificates, methodologies, specifications and other documentation and/or information, provided that the Parties discuss and agree the extent of both provision by NOF of and use by Apellis of NOF Specific Information to be used for Regulatory submissions to [**] regulatory agency prior to the submission, and (b) to the extent authorizations, certificates, methodologies, specifications and other documentation and/or information are not within the scope of the foregoing clause (a), the Parties shall discuss the conditions under which such shall be provided to Apellis or made directly available to the applicable Regulatory Agency. As stated in the Quality Agreement, NOF shall make available to Apellis at the Facility all documents reasonably requested by Apellis regarding the SUNBRIGHT [**] and the Manufacturing thereof that may be necessary or helpful for preparing Regulatory Submissions or communicating with Regulatory Agencies relating to the Drug Substance or Drug Product and/or obtaining or maintaining any Drug Product NDA. NOF may charge Apellis fees if NOF conducts additional tests to get data to cooperate with Apellis with respect to the Regulatory Submissions and Apellis shall pay the fees. The Parties shall discuss and agree amounts of the fees from time to time.

8.2 Ownership of Regulatory Filings. Except as specifically agreed otherwise by the Parties, Apellis shall be the sole owner of all Regulatory Submissions and all governmental approvals obtained from any Regulatory Agency with respect to the Drug Substance or the Drug Product, including any Drug Product NDA.

8.3 Interactions with Regulatory Authorities. Apellis shall be responsible for the preparation and filing of any Regulatory Submissions and for all contacts and communications with any Regulatory Authorities with respect to matters specifically relating to Manufacture of SUNBRIGHT [**] or any component thereof. NOF shall notify Apellis immediately (and in no event later than [**]) after NOF

receives any contact or communication from any Regulatory Authority related in any way to the Manufacture of SUNBRIGHT [**] or which could be reasonably expected to have an adverse effect on the Manufacture of SUNBRIGHT [**]. NOF will provide Apellis with copies of any such correspondence or other communication and shall consult with Apellis regarding the response to any inquiry or observation from any Regulatory Authority, and Apellis, at its discretion, shall control and/or participate in any further contacts or communications relating to the Manufacture of SUNBRIGHT [**].

9. TERM AND TERMINATION

9.1 Term. This Agreement shall commence on the Effective Date and shall continue until December 31, 2025, unless earlier terminated pursuant to this Section 9 (the "Term").

9.2 Termination.

(a) *Termination for Breach*. Either Party may terminate this Agreement by giving written notice to the other Party if such other Party is in material breach of any material term or obligation under this Agreement, including any material breach of any material term or obligation under the Quality Agreement, and such termination shall become effective [**] after such notice given if such other Party has failed to cure the breach within [**] period. For clarity, the Parties agree that if NOF delivers [**] or more batches of SUNBRIGHT [**] that fail to conform to the Specifications, are defective, not Manufactured in accordance with cGMP or the Quality Agreement or are otherwise adulterated or misbranded under applicable Laws, or if there are [**] or more Supply Failures, NOF shall be deemed to be in material breach of this Agreement.

(b) *Termination for Adverse Health or Safety Reasons*. Apellis may terminate this Agreement hereunder upon written notice in the event that: (i) Apellis determines, in its discretion, that there are significant safety or efficacy problems relating to the Drug Substance or the Drug Product; (ii) authorization or permission to use the Drug Substance or the Drug Product is withdrawn by an applicable Regulatory Agency; (iii) either Party receives information from a Regulatory Agency that indicates that approval of a Regulatory Submission is highly unlikely or is irrevocably rejected; or (iv) Apellis irrevocably withdraws the Regulatory Submission.

(c) *Termination for Bankruptcy*. To the extent permitted under applicable Law, either Party may terminate this Agreement in its entirety effective immediately with written notice if the other Party files for bankruptcy, is adjudicated bankrupt, files a petition under insolvency Laws, is dissolved or has a receiver appointed for substantially all of its property.

(d) *Termination by Mutual Agreement*. The Parties may terminate this Agreement at any time by mutual written agreement.

(e) *Termination for Convenience*. Either Party may terminate this Agreement at any time for convenience by providing twenty-four (24) months' written notice to the other Party.

9.3 Consequences of Termination.

(a) Notwithstanding anything to the contrary provided in this Agreement, expiration or termination of this Agreement shall not relieve the Parties of any obligations accruing prior to the effective date of such expiration or termination, and shall not terminate any then-outstanding Firm Orders and NOF shall continue to Manufacture and supply any SUNBRIGHT [**] that is the subject of any then outstanding Firm Order. Upon the expiration or termination of this Agreement, each Party shall (i) return to the other Party or destroy, at the election of the other Party, all documents and written materials

containing, reflecting, incorporating or based on the Confidential Information of such other Party; (ii) permanently erase all of the other Party's Confidential Information from its computer systems (except for that which is stored in archival back-up in accordance with such Party's standard practice); and (iii) certify in writing to the other Party that it has complied with the requirements of this clause; provided that NOF shall continue to comply with its continuing obligations under Section 8.

(b) If this Agreement is terminated (i) by NOF pursuant to Section 9.2(a) (material breach) or Section 9.2(c) (bankruptcy) or (ii) by Apellis other than pursuant to Section 9.2(a) (material breach) or Section 9.2(c) (bankruptcy), then, in addition to its obligation to perform under the outstanding Firm Orders (including those to be issued pursuant to Section 9.3(c)), Apellis, in accordance with Section 9.3(d), shall pay NOF an amount equal to [**]-percent ([**]%) of the remaining quantity of the Minimum Purchase Obligation for the time period in which such termination occurred ("Initial MPO Period") and [**] percent ([**]%) of the then remaining aggregate unpaid Minimum Purchase Obligation for the remaining time period thereafter ("Remaining MPO Period"), except to the extent the Minimum Purchase Obligation has been earlier terminated pursuant to Section 4.9.

(c) If this Agreement is terminated (i) by NOF pursuant to Section 9.2(a) (material breach) or Section 9.2(c) (bankruptcy) or (ii) by Apellis pursuant to Section 9.2(e) (convenience), then, in addition to its obligation to perform under the outstanding Firm Orders, Apellis shall place Firm Orders for SUNBRIGHT [**] in accordance with the remaining Firm Commitment and NOF shall deliver such SUNBRIGHT [**] and Apellis, in accordance with Section 9.3(d), shall purchase and pay for such delivered SUNBRIGHT [**]; provided, however, that the foregoing amounts shall be creditable against any Minimum Purchase Obligation payments to be paid pursuant to Section 9.3(b).

(d) If a termination of this Agreement falls within Section 9.3(b) and/or 9.3(c), Apellis shall pay NOF for the amount of the Firm Commitment not yet paid plus the percentage amount of the Minimum Purchase Obligation for the Initial MPO Period and the Remaining MPO Period (less the Firm Commitment). Invoices for the appropriate payment will be issued by NOF, and shall be payable within [**] of the date of receipt of the invoice from NOF by Apellis. By way of example and without limiting the foregoing (and using round numbers for simplicity), if a termination of this Agreement falls within the Initial MPO Period and with the following assumptions:

(i) Apellis has a Minimum Purchase Obligation of $[**] ($[**] of which is during the Initial MPO Period and $[**] of which is for the Remaining MPO Period); and

(ii) a Firm Commitment of $[**];

Then, Apellis shall pay NOF an amount equal to $[**] calculated as follows:

$[**] (for the outstanding Firm Commitment (payable in accordance with this Agreement after delivery of the relevant SUNBRIGHT [**]))

+

$[**] (for the Minimum Purchase Obligation for the Initial MPO Period [**]

+

$[**] (the Minimum Purchase Obligation for the Remaining MPO Period)).

(e) If this Agreement is terminated (i) by Apellis pursuant to 9.2(a) (material breach) or Section 9.2(c) (bankruptcy), or (ii) by the Parties pursuant to Section 9.2(d) (mutual agreement), then the Minimum Purchase Obligation shall be no longer of any force or effect and Apellis shall have no obligation to pay any Minimum Purchase Obligation.

(f) If this Agreement is terminated (i) by NOF pursuant to 9.2(a) (material breach) or Section 9.2(c) (bankruptcy) or (ii) by Apellis other than pursuant to Section 9.2(a) (material breach) or Section 9.2(c) (bankruptcy), Apellis shall also be required to purchase from NOF the quantity of Safety Stock existing as of the time of such termination (if any), provided that all such Safety Stock meets the representations, warranties and covenants set forth in this Agreement), and in connection therewith, NOF shall Deliver all such quantities of Safety Stock in accordance with this Agreement, and Apellis shall pay NOF the cost of the Safety Stock. Notwithstanding the foregoing or anything to the contrary contained herein, from and after the delivery of any notice of termination pursuant to this Agreement, NOF shall not replenish or otherwise add to the quantity of Safety Stock then being held for Apellis pursuant to Section 4.13. Invoices for the appropriate payment(s) will be issued by NOF, and shall be payable within [**] of the date of receipt of the invoice from NOF by Apellis.

(g) If this Agreement is terminated (i) by NOF pursuant to 9.2(e) (convenience) or (ii) by Apellis pursuant to Section 9.2(a) (material breach) or Section 9.2(c) (bankruptcy), at the request of Apellis, NOF shall assist Apellis in effecting a smooth transition to an alternate supplier(s) for the manufacture of SUNBRIGHT [**] and [**]. Without limiting the generality of the foregoing, at the request of Apellis, NOF shall provide the manufacturing process and analytical methods description including specifications for SUNBRIGHT [**] and [**] to support the technical transfer of the Manufacture of SUNBRIGHT [**] to an alternate source(s); provided, however, that with respect to [**], which is the raw material of [**], NOF shall not be obligated to provide the manufacturing process and analytical methods description including specifications therefor. The Parties shall discuss supply and purchase of [**] in good faith. In addition to the Manufacture and supply of any SUNBRIGHT [**] that is the subject of an issued Firm Order, if this Agreement is terminated by Apellis pursuant to Section 9.2(a) or Section 9.2(c) or by NOF pursuant to Section 9.2(e), NOF, for up to an additional [**] period, shall Manufacture and supply SUNBRIGHT [**] to Apellis until Apellis is able to validate and qualify an alternative supplier of SUNBRIGHT [**].

10. REPRESENTATIONS AND WARRANTIES

10.1 NOF Representations and Warranties

(a) *Product Quality*. NOF represents and warrants that all SUNBRIGHT [**] delivered under this Agreement shall: (i) for its Shelf Life meet the Specifications and shall not be adulterated or misbranded under any Laws; (ii) have been Manufactured in conformation with all applicable SOPs, and cGMPs and all applicable Laws and the Quality Agreement; and (iii) be transferred to Apellis free and clear of any security interests, lien or encumbrances. NOF represents and warrants that it will Manufacture the SUNBRIGHT [**] in a professional and workmanlike manner and in compliance with the all applicable Laws, including cGMPs. Without limiting the foregoing, NOF represents and warrants that NOF will store, handle, and process all NOF Supplied Materials and Apellis-supplied Materials used in processing or the provision of Manufacturing hereunder in accordance with all SOPs, and all applicable Laws, including cGMPs and the Quality Agreement. The product warranty contained in Section 10.1(a)(i) does not apply to any SUNBRIGHT [**] that has been subjected to abuse, misuse, neglect, negligence, accident, improper testing, improper installation, improper storage, improper handling, abnormal physical stress, abnormal environmental conditions or use contrary to any written instructions issued by NOF; has been reconstructed, repaired or altered by persons other than NOF or its authorized representative; or has

been used with any Third Party products, hardware or product that has not been previously approved in writing by NOF. EXCEPT FOR SUCH OTHER REMEDIES AND OBLIGATIONS SET FORTH IN THIS AGREEMENT, INCLUDING NOF'S INDEMNIFICATION OBLIGATIONS AND APELLIS'S RIGHT TO TERMINATE THIS AGREEMENT, THE SOLE REMEDY OF APELLIS AND THE ENTIRE LIABILITY OF NOF FOR ANY BREACH OF THE PRODUCT WARRANTY SET FORTH IN SECTION 10.1(a)(i) ARE SET FORTH IN SECTION 7.2.

(b) *Personnel*. NOF represents and warrants that (a) NOF was not, is not and will not be, and it did not, does not, and will not use in any capacity the services of any person, disqualified or debarred under the Generic Drug Enforcement Act, 21 USC 335a, or otherwise disqualified or debarred by the FDA or any other Regulatory Agency and (b) NOF was not, is not and will not be, and it did not, does not, and will not use in any capacity the services of any person who has been charged or convicted of a crime as defined under the Generic Drug Enforcement Act. If, during the term of this Agreement, NOF becomes aware that either it or any person performing Manufacturing activities hereunder on behalf of NOF has become or may become disqualified or debarred, NOF shall immediately notify Apellis in writing.

(c) *Compliance with Regulatory Agency*. Without limiting any representations and warranties herein, NOF represents and warrants that neither it nor any member of its staff has been charged with or convicted under federal law for conduct relating to the development or approval of any filing or submission to any Regulatory Agency, any active pharmaceutical ingredient, any pharmaceutical product or any services of the type provided by NOF hereunder, or otherwise relating to the regulation of any drug product under any relevant Law. If at any time NOF or any member of its staff is charged with or convicted under federal Law for conduct relating to the development or approval of any filing or submission to any Regulatory Agency, or otherwise relating to the regulation of any drug product under any relevant Law, NOF shall immediately notify Apellis in writing.

(d) *No Third Party Infringement*. To its actual knowledge of NOF, NOF represents, warrants and covenants that in Manufacturing the SUNBRIGHT [**] it has not knowingly infringed, at the time of Effective Date, and shall not knowingly infringe upon the intellectual property rights of any Third Party.

10.2 Apellis Representations and Warranties.

(a) Apellis represents and warrants that it has the rights to transfer the Apellis-supplied Materials to NOF for the purposes contemplated herein and to grant the rights granted to NOF hereunder.

(b) Apellis represents and warrants that the SUNBRIGHT [**] will be used solely for the purpose of manufacturing Drug Substance and/or Drug Product.

10.3 Mutual Warranties. Each Party represents and warrants to the other that (i) to its knowledge, it has the right to provide the Confidential Information provided hereunder; (ii) it has all requisite power and authority (corporate and otherwise) to enter into this Agreement; (iii) the execution and delivery of this Agreement by the officer or individual whose name is signed on its behalf below has been duly authorized by all necessary actions; (iv) such Party's execution and delivery of this Agreement and the performance of its obligations hereunder do not and will not conflict with or result in a breach of or a default under its organizational instruments or any other agreement, instrument, order, or Law applicable to it or by which it may be bound; and (v) this Agreement has been duly and validly executed and delivered by it and constitutes its valid and legally binding obligation, enforceable in accordance with its terms, except as enforcement may be limited by bankruptcy, insolvency or other Laws of general application relating to

or affecting the enforcement of creditors' rights and except as enforcement is subject to general equitable principles.

10.4 Disclaimer. EXCEPT AS EXPRESSLY SET FORTH HEREIN, EACH PARTY DISCLAIMS ALL OTHER WARRANTIES, EXPRESS, IMPLIED, STATUTORY OR OTHERWISE, INCLUDING THE IMPLIED WARRANTIES OF MERCHANTABILITY, FITNESS FOR A PARTICULAR PURPOSE AND NON-INFRINGEMENT OF THIRD PARTY INTELLECTUAL PROPERTY RIGHTS.

11. CONFIDENTIALITY

11.1 NOF Confidentiality Obligations. During the Term of this Agreement and for a period of [**] thereafter, NOF shall maintain in confidence and shall not use Apellis's Confidential Information except as authorized under this Agreement and shall not disclose Apellis's Confidential Information to any Third Party other than: (i) employees, consultant, agents or permitted subcontractors of NOF or any of NOF's Affiliates who are bound by similar obligations of confidentiality and nonuse and who have a need to know such information in order to perform their duties in carrying out NOF's obligations under this Agreement; or (ii) as directed by Apellis in writing.

11.2 Apellis Confidentiality Obligations. During the Term of this Agreement and for a period of [**] thereafter, Apellis shall maintain in confidence and shall not use NOF's Confidential Information except as authorized under this Agreement and shall not disclose any NOF's Confidential Information to any Third Party other than: (i) employees, consultants, agents or contractors of Apellis or any of Apellis's Affiliates who are bound by similar obligations of confidentiality and nonuse and who have a need to know such information in order to perform their duties in carrying out Apellis's obligations under this Agreement, or in exercising Apellis's rights under this Agreement, or in order to provide direction or services to Apellis regarding production, testing, storage or quality of the Drug Substance or Drug Product, or regulatory, compliance or other issues related to the Drug Substance or Drug Product; or (ii) Regulatory Agencies in connection with communication(s) or Regulatory Submission(s) regarding the Drug Substance or Drug Product.

11.3 Required Disclosure. Notwithstanding Section 11.1 and Section 11.2, in case the Receiving Party is required (by oral questions, interrogatories, requests for information or documents, subpoena, civil investigative demand or similar process) to disclose any of the Disclosing Party's Confidential Information pursuant to legal, administrative or judicial action, the Receiving Party may disclose the Confidential Information to comply with the request; provided, that the Receiving Party, to the extent permitted by applicable Law, (a) timely informs the Disclosing Party, (b) uses its best efforts to limit the disclosure and maintain confidentiality to the extent possible and (c) permits the Disclosing Party to attempt by appropriate legal means to limit such disclosure.

11.4 Responsibility for Compliance with Confidentiality and Nonuse Obligations. Each Party shall be responsible for any disclosure, misuse or misappropriation, by such Party, its Affiliates, or the employees, consultants, agents or contractors of such Party or such Party's Affiliates, of the other Party's Confidential Information.

11.5 Terms of Agreement. Except for (i) any disclosure that is deemed necessary, in the reasonable judgment of the responsible Party, to comply with applicable Laws (including the rules and regulations of any national stock exchange on which such Party's securities are traded) or (ii) disclosure to a Party's or any of its Affiliates' employees consultants, advisors, agents, contractors or actual or potential licensees, financing sources, investors or acquirers, under reasonable conditions of confidentiality, neither

Party shall, without the prior written consent of the other Party, disclose in any manner to any Third Party the terms and conditions of this Agreement or use the other Party's name in any press release, publicity, or advertising without prior written consent. If Apellis deems it necessary to file this Agreement in accordance with U.S. securities Laws, Apellis shall use reasonable efforts to seek confidential treatment, to the extent consistent with such Laws, for the contents of the Exhibits of this Agreement.

12. INTELLECTUAL PROPERTY

12.1 Ownership. NOF agrees that, as between the Parties, Apellis owns all right, title, and interest in and to the Drug Substance and the Drug Product, as applicable, and all Apellis IP (collectively "Apellis Property"), and to the extent NOF or any of its Affiliates has or may acquire or be deemed to have acquired any rights in Apellis Property, NOF hereby agrees, on behalf of itself and its Affiliates, to transfer and assign, and hereby transfers and assigns, its and its Affiliates' right, title, and interest in such property to Apellis. Upon Apellis's request at any time, NOF shall, and shall require its Affiliates to, deliver to Apellis any and all documents and information necessary to protect Apellis's interest in the Apellis Property to the extent that such documents and information are not confidential information of a Third Party. NOF shall promptly notify Apellis in writing of any Apellis Property that arises from the Manufacturing services.

13. INDEMNIFICATION

13.1 Indemnification by NOF. NOF shall indemnify, defend, and hold Apellis and its Affiliates and their directors, officers, and employees harmless from and against any and all damages, judgments, claims, suits, actions, costs and expenses (including reasonable attorneys' fees) (collectively, "Liabilities") arising out of any Third Party claim to the extent resulting from (i) NOF's breach of this Agreement, including the Quality Agreement; (ii) any inability to process or release the Drug Substance or Drug Product due to NOF's failure to comply with applicable Laws; or (iii) the grossly negligent acts or omissions or willful misconduct of NOF or any of its directors, officers, employees, subcontractors or agents in NOF's performance of this Agreement.

13.2 Indemnification by Apellis. Apellis shall indemnify, defend and hold NOF and its Affiliates and their directors, officers, and employees harmless from and against all Liabilities arising out of any Third Party claim to the extent resulting from (i) Apellis's breach of this Agreement including any of its warranties or representations hereunder; (ii) the use of SUNBRIGHT [**] or the manufacture, sale, transfer or other disposition of the Drug Product by Apellis; or (iii) the grossly negligent acts or omissions or willful misconduct of Apellis or any of its directors, officers, employees, subcontractors or agents in Apellis's performance of this Agreement.

13.3 Indemnification Procedure. Upon notice of any Liability, the Indemnified Party shall promptly notify the Indemnifying Party in writing. Failure of the Indemnified Party to so notify the Indemnifying Party shall not relieve the Indemnifying Party of its indemnification obligations except to the extent the failure or delay is prejudicial. The Indemnifying Party shall have control over the defense and any settlement of any such claim for Liability; provided however that (i) the Indemnified Party shall be entitled to participate in the defense of such claim and to employ counsel at its own expense to assist in the handling of such claim; and (ii) the Indemnifying Party shall obtain the prior written approval (not to be unreasonably withheld) from the Indemnified Party before entering into any settlement of such claim. The Parties shall cooperate in furnishing such information and attending such conferences and hearings as reasonably requested in connection with the defense or prosecution of any Liabilities. In the event the Indemnifying Party fails to act within a reasonable time after receiving notice, the Indemnified Party shall have the right to employ its own counsel at the expense of the Indemnifying Party.

13.4 Insurance. NOF shall maintain products and completed operations liability insurance through the term of this Agreement, which insurance shall afford limits of not less than US$[**] for each occurrence and US$[**] in the aggregate per annum for product liability. If requested by Apellis, NOF will provide Apellis with a certificate of insurance evidencing the above and showing the name of the issuing company, the policy number, the effective date, the expiration date and the limits of liability. The insurance certificate shall further provide for a minimum of [**] written notice to NOF and to Apellis of a cancellation of, or material change in, the insurance.

14. LIMITATION OF LIABILITY

14.1 EXCEPT FOR LIABILITIES ARISING FROM (I) THE INTENTIONAL MISUSE OR MISAPPROPRIATION OF THE OTHER PARTY'S CONFIDENTIAL INFORMATION OR (II) EACH PARTY'S INDEMNIFICATION OBLIGATIONS SET FORTH IN SECTION 13, IN NO EVENT SHALL EITHER PARTY BE LIABLE TO THE OTHER OR ANY OF ITS AFFILIATES FOR ANY CONSEQUENTIAL, INCIDENTAL, INDIRECT, SPECIAL, PUNITIVE OR EXEMPLARY DAMAGES (INCLUDING LOST PROFITS, BUSINESS OR GOODWILL) SUFFERED OR INCURRED BY SUCH OTHER PARTY OR ITS AFFILIATES IN CONNECTION WITH THIS AGREEMENT, WHETHER BASED UPON BREACH OF CONTRACT OR TORT, INCLUDING NEGLIGENCE, EVEN IF ADVISED OF THE POSSIBILITY OF SUCH DAMAGES. IN ADDITION TO THE LIMITATION OF LIABILITY SET FORTH ABOVE, EXCEPT FOR (A) THE INTENTIONAL MISUSE OR MISAPPROPRIATION OF THE OTHER PARTY'S CONFIDENTIAL INFORMATION OR (B) DAMAGES ARISING OUT OF SUCH PARTY'S GROSS NEGLIGENCE OR WILLFUL MISCONDUCT, NOF'S MAXIMUM AGGREGATE LIABILITY TO COMPENSATE APELLIS FOR ALL DAMAGES UNDER THIS AGREEMENT WILL BE SET ON A PER CALENDAR YEAR BASIS AND FOR THE CALENDAR YEAR IN WHICH THE CAUSE OF SUCH LIABILITY LIES OR EXISTS (WHETHER IN CONTRACT, TORT, STRICT LIABILITY, STATUTE, OR OTHERWISE) AND SHALL BE LIMITED TO THE AMOUNTS PAID OR PAYABLE BY APELLIS TO NOF IN THE THEN MOST RECENTLY COMPLETED CALENDAR YEAR.

15. GENERAL PROVISIONS

15.1 Independent Contractors. For purposes of this Agreement, the Parties shall be deemed independent contractors, and neither of the Parties shall be deemed or construed, by virtue of this Agreement, to be the agent, representative, partner or joint venturer of the other Party. Neither Party shall have any express or implied right or authority to assume or create any obligation on behalf of, or in the name of the other Party, nor to bind the other Party to any contract, agreement or undertaking with any Third Party. NOF shall make clear to all Third Parties and Affiliates with whom it deals that NOF is a separate entity from Apellis and that NOF does not have the authority to bind Apellis.

15.2 Choice of Law. This Agreement shall be governed by and construed in accordance with the laws of the State of New York (for clarity, including, with respect to the purchase and sale of SUNBRIGHT [**], the Uniform Commercial Code therein), without regard to its conflicts of law principles. For the avoidance of doubt, the United Nations Convention on the International Sale of Goods shall not apply to this Agreement.

15.3 Binding Effect; No Third Party Beneficiaries. This Agreement shall be binding upon and inure to the benefit of, and shall be enforceable only by, the Parties and their respective successors and permitted assigns. It is the explicit intention of the Parties that no person or entity, other than the named Parties or their successors or permitted assigns, is or shall be entitled to bring any action to enforce any provision of this Agreement, as a third party beneficiary or otherwise.

15.4 Notices. Any notices or other communication given pursuant to this Agreement shall be written and delivered either by (i) personal delivery, (ii) certified mail, postage prepaid and return receipt requested, (iii) any recognized overnight air courier service, (iv) confirmed facsimile, or (v) email to the relevant Party as follows or to such other address of which the receiving Party gives notice pursuant to this Section 15.4:

If to **Apellis**:

Apellis Pharmaceuticals, Inc.
100 Fifth Avenue, 3rd Floor
Waltham, MA 02451
Attn: [**]
Email: [**]
(primary recipient)

With a copy to

Apellis Pharmaceuticals, Inc.
100 Fifth Avenue, 3rd Floor
Waltham, MA 02451
Attn: [**]
Email: [**]

If to **NOF**:

NOF CORPORATION
20-3, Ebisu 4-chome, Shibuya-ku,
Tokyo 150-6019, Japan
Attn: Planning & Administration Dept.
DDS Development Division
Telephone:[**]
Facsimile:[**]
Email: [**]
(primary recipient)

With a copy to:

NOF AMERICA CORPORATION
1 North Broadway, Suite 912
White Plains, NY 10601 U.S.A.
Attn: [**]
Telephone:[**]
Facsimile:[**]
Email: [**]

All notices shall take effect as follows: (i) upon receipt if delivered either in person or by confirmed facsimile on any business day in the delivery location (the relevant "Business Day") prior to 6 pm local time; or (ii) on the next succeeding Business Day if delivered either in person or by confirmed facsimile on a non-Business Day or after 6 pm local time; (iii) one (1) Business Day after having been delivered to a recognized air courier for overnight delivery; (iv) the delivery date if delivered by certified mail, return receipt requested; or (v) if delivered by email, when the primary recipient, by an email sent to the email address for the sender stated in this Section 15.4 or by a notice delivered by another method in accordance with this Section 15.4, acknowledges having received that email, with an automatic "read receipt" not constituting acknowledgment of an email for purposes of this Section 15.4.

15.5 Assignment. The Parties shall not assign this Agreement or any of the rights or obligations hereunder without the prior written consent of the other Party; provided, however, that (a) Apellis shall have the right, without the prior consent of NOF, to assign this Agreement, in whole or in part, to any Affiliate of Apellis, and (b) Apellis shall have the right, without the prior consent of NOF, to assign this Agreement, in whole or in part, to any Third Party (other than to a Third Party whose primary business is manufacture and supply of polyethyleneglycol derivatives) in connection with a sale of all or substantially all of the assets of Apellis to which this Agreement relates, whether by merger, transfer of a going concern, sale of stock, sale of assets or other similar transaction (including by operation of law), in each case upon notice to NOF. This Agreement shall bind and inure to the benefit of any successors and permitted assignees. Any prohibited assignments shall be void. No assignment relieves the assigning Party of any of its obligations under this Agreement.

15.6 Force Majeure. Neither Party shall be liable to the other Party for non-performance or delay in performance to the extent such failure or delay in performing any of its obligations hereunder is not due to its negligence and arises from or caused by any Force Majeure, if the impacted Party shall give

prompt notice of any such Force Majeure to the other Party. The impacted Party giving such notice shall thereupon be excused from such of its obligations hereunder as it is thereby disabled from performing for so long as it is so disabled, provided, however, that such impacted Party shall have used reasonable efforts to avoid such occurrence and to commence and continue to take reasonable and diligent actions to recommence performance as quickly as possible. “Force Majeure” shall mean any event beyond the reasonable control of an impacted Party including, strikes, labor troubles, riots, embargo, blockages, transport and customs clearance delays, Acts of God, storms, fires, explosions, earthquakes, floods, tsunamis, pandemics, epidemics, war, acts of authorities and prohibitions. Notwithstanding the foregoing, the COVID-19 Pandemic shall not be considered a Force Majeure event, unless (i) a Party's non-performance or delay in performance is result of laws, regulations or advisory of a governmental authority to combat the COVID-19 Pandemic which the addressees are legally obliged to comply with (e.g. closure of the Facility, quarantine regulations, or requisition of production capacities) and/or (ii) such Party has taken commercially reasonable measures to avoid harmful effects of the COVID-19 Pandemic. In the event of a Force Majeure that lasts for more than [**] or [**] in any twelve month period, the other Party shall have the right upon written notice to the delayed Party to terminate this Agreement.

15.7 Entire Agreement. This Agreement, which includes any exhibits, schedules, and attachments as well as the Quality Agreement, constitutes the entire understanding of the Parties in connection with the Manufacture of SUNBRIGHT [**] and a complete and exclusive statement of the terms of their agreement with respect thereto (and supersedes and replaces the Original Agreement). Any term or condition of any purchase order, sales acknowledgement, or document with respect to the Manufacture of the SUNBRIGHT [**] which is in addition to, different from, or contrary to the terms and conditions of this Agreement shall be void.

15.8 Amendment. This Agreement or any provision hereof shall not be amended, supplemented, or waived except in a writing signed by each of the Parties.

15.9 Breach of the Quality Agreement. Any breach or material breach of the Quality Agreement shall also be deemed a breach or material breach, respectively, of this Agreement.

15.10 Waiver. No failure or delay on the part of the Parties to exercise any right, power, or privilege hereunder or under any instrument executed pursuant hereto shall operate as a waiver; nor shall any single or partial exercise of any right, power, or privilege preclude any other or further exercise thereof or the exercise of any right, power, or privilege.

15.11 Severability. If either (i) a court of competent jurisdiction hold that a particular provision or requirement of this Agreement violates any applicable Law or (ii) a government agency with jurisdiction definitively advises the Parties that a feature or provision of this Agreement violates Laws over which such department or agency has jurisdiction, then each such provision, feature or requirement shall be fully severable and: (a) this Agreement shall be construed and enforced as if such illegal, invalid or unenforceable provision has never comprised a part hereof; (b) the remaining provisions hereof shall remain in full force and effect and shall not be affected by the severable provision; and (c) the Parties shall in good faith negotiate and substitute a provision as similar to such severable provisions as may be possible and still be legal, valid and enforceable.

15.12 Further Assurances. Each Party shall execute, acknowledge and deliver such further instruments, and take such further actions as may be reasonably necessary or appropriate to carry out the purposes and intent of this Agreement.

15.13 Headings. All headings in this Agreement are for convenience of reference only and shall affect the interpretation of this Agreement.

15.14 Survival. The rights and obligations of the Parties under Sections 8.2, 9.3, 10.4, 11, 12.1, 13, 14.1 and 15 shall survive the expiration or earlier termination of this Agreement and the obligations of the Parties under Section 11 shall survive for [**] from the expiration or earlier termination of this Agreement.

15.15 Counterparts. This Agreement and any amendment or supplement hereto may be executed in several counterparts, each of which shall be deemed to be an original, and all of which taken together shall constitute one and the same instrument.

15.16 Interpretation. Each Party acknowledges and agrees that: (i) it and its representatives have reviewed and negotiated the terms and provisions of this Agreement and have contributed to its revision; (ii) it has been advised by counsel during the course of negotiation of this Agreement; and (iii) the terms and provisions of this Agreement shall be construed fairly as to each Party and not in favor of or against either Party regardless of which Party was generally responsible for the preparation or drafting of this Agreement. The headings, captions and table of contents in this Agreement are for convenience of reference only and shall not be considered in construing this Agreement. In construing this Agreement, except where the context acquires otherwise, (a) use of the singular includes the plural and vice versa; (b) the words "include" "including", "includes" and "e.g." means "including without limitation"; (c) the word "or" is used in the inclusive sense that is typically associated with the phrase "and/or"; (d) the words "herein," "hereof" and "hereunder," and words of similar import, refer to this Agreement in its entirety and not to any particular provision hereof; (e) the verb "will" shall be construed to have the same meaning and effect as the word "shall"; (f) use of any gender includes any other gender; (g) whenever this Agreement refers to a number of days, such number shall refer to calendar days unless Business Days are specified; (h) references to a particular Law mean such Law as in effect as of the relevant time, including all rules and regulations thereunder and any successor Law in effect as of the relevant time, and including the then-current amendments thereto; (i) references to a particular person include such person's successors and assigns to the extent not prohibited by this Agreement; (j) a capitalized term not defined herein but reflecting a different part of speech than a capitalized term which is defined herein shall be interpreted in a correlative manner; (k) the words "Dollar" and "dollar" and the symbol "$" mean U.S. Dollars; (l) the word "notify" or "notice" means a notice in writing; and (m) all references herein to Articles, Sections or Exhibits shall be construed to refer to Articles, Sections and Exhibits of this Agreement.

15.17 Arbitration. The Parties shall finally settle all disputes, controversies or differences which arise between the Parties, out of or in relation to or in connection with this Agreement, which cannot be satisfactorily settled by the Parties, by arbitration in New York City, the State of New York, the United States of America, pursuant to the Commercial Arbitration Rules of the American Arbitration Association. The Parties shall conduct the arbitration proceedings in the English language. The award shall be final and binding upon the Parties. Each Party may have judgment upon the award entered in any court having jurisdiction thereof. At any time, a Party may seek or obtain injunctive relief from the arbitrators or from a court.

[Remainder of Page Intentionally Left Blank]

IN WITNESS WHEREOF, the Parties have executed this Agreement, as of the Effective Date.

APELLIS PHARMACEUTICALS, INC. (Apellis)

By:/s/ Nur Nicholson
Nur Nicholson

Title: Chief Technical Officer

Date signed: Feb. 15, 2021

NOF CORPORATION (NOF)

By:/s/ Tsuneharu Miyazaki
Tsuneharu Miyazaki

Title: Managing Executive Officer,
General Manager, DDS Development Div.

Date signed: Feb. 24, 2021

APELLIS SWITZERLAND GmbH

By:/s/ Thomas Lackner

Thomas Lackner

Title: SVP, GM International

Date signed: Feb. 13, 2021

EXHIBIT A: SUNBRIGHT [**]

EXHIBIT B: Supply Price

EXHIBIT C: Specifications

EXHIBIT D: Nonbinding Estimated Annual Forecast

EXHIBIT E: Minimum Purchase Obligation

EXHIBIT F: CMO

EXHIBIT G: Quality Agreement

EXHIBIT H: Rolling Forecast Example

EXHIBIT I: Types of Regulatory Data and Information

Exhibit 31.1

CERTIFICATION OF PRINCIPAL EXECUTIVE OFFICER PURSUANT TO EXCHANGE ACT RULES 13a-14(a) AND 15d-14(a), AS ADOPTED PURSUANT TO SECTION 302 OF SARBANES-OXLEY ACT OF 2002

I, Cedric Francois, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Apellis Pharmaceuticals, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

   (a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

   (b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

   (c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

   (d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

   (a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

   (b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: April 28, 2021

By: /s/ Cedric Francois
Cedric Francois
Chief Executive Officer

Exhibit 31.2

CERTIFICATION OF PRINCIPAL FINANCIAL OFFICER PURSUANT TO EXCHANGE ACT RULES 13A-14(A) AND 15D-14(A), AS ADOPTED PURSUANT TO SECTION 302 OF SARBANES-OXLEY ACT OF 2002

I, Timothy Sullivan, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Apellis Pharmaceuticals, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

   (a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

   (b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

   (c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

   (c) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

   (a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

   (b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: April 28, 2021

By: /s/ Timothy Sullivan
Timothy Sullivan
Chief Financial Officer and Treasurer

Exhibit 32.1

CERTIFICATION PURSUANT TO 18 U.S.C. SECTION 1350, AS ADOPTED PURSUANT TO SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with the Quarterly Report of Apellis Pharmaceuticals, Inc. (the "Company") on Form 10-Q for the period ending March 31, 2021 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned, Cedric Francois, President and Chief Executive Officer of the Company, hereby certifies, pursuant to 18 U.S.C. Section 1350, that:

(1) The Report fully complies with the requirements of section 13(a) or 15(d) of the Securities Exchange Act of 1934; and

(2) The information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: April 28, 2021

By: /s/ Cedric Francois
Cedric Francois
President and Chief Executive Officer

A signed original of this written statement required by Section 906 has been provided to the Company and will be retained by the Company and furnished to the Securities and Exchange Commission or its staff upon request.

Exhibit 32.2

CERTIFICATION PURSUANT TO 18 U.S.C. SECTION 1350, AS ADOPTED PURSUANT TO SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with the Quarterly Report of Apellis Pharmaceuticals, Inc. (the "Company") on Form 10-Q for the period ending March 31, 2021 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned, Timothy Sullivan, Chief Financial Officer and Treasurer of the Company, hereby certifies, pursuant to 18 U.S.C. Section 1350, that:

(1) The Report fully complies with the requirements of section 13(a) or 15(d) of the Securities Exchange Act of 1934; and

(2) The information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: April 28, 2021

By: /s/ Timothy Sullivan
Timothy Sullivan
Chief Financial Officer and Treasurer

A signed original of this written statement required by Section 906 has been provided to the Company and will be retained by the Company and furnished to the Securities and Exchange Commission or its staff upon request.