

# **41<sup>st</sup> Annual J.P. Morgan Healthcare Conference 2023**

January 9, 2023

Apellis

# Forward-looking statements

Statements in this presentation about future expectations, plans and prospects, as well as any other statements regarding matters that are not historical facts, may constitute “forward-looking statements” within the meaning of The Private Securities Litigation Reform Act of 1995. The words “anticipate,” “believe,” “continue,” “could,” “estimate,” “expect,” “intend,” “may,” “plan,” “potential,” “predict,” “project,” “should,” “target,” “will,” “would” and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Actual results may differ materially from those indicated by such forward-looking statements as a result of various important factors, including: whether results obtained in preclinical studies and clinical trials will be indicative of results that will be generated in future clinical trials; whether the results of the FILLY, DERBY, and OAKS trials are sufficient to support regulatory submissions; whether a submission for approval of intravitreal pegcetacoplan for GA on the basis of the FILLY, DERBY and OAKS trials will be accepted by foreign regulatory agencies; whether intravitreal pegcetacoplan will receive approval from the FDA or equivalent foreign regulatory agencies for GA when expected or at all; whether the company’s clinical trials

will be fully enrolled and completed when anticipated; whether preliminary or interim results from a clinical trial will be predictive of the final results of the trial; whether results obtained in preclinical studies and clinical trials will be indicative of results that will be generated in future clinical trials; whether pegcetacoplan will successfully advance through the clinical trial process on a timely basis, or at all; whether the results of the company’s clinical trials will warrant regulatory submissions and whether pegcetacoplan will receive approval from the FDA or equivalent foreign regulatory agencies for CAD, C3G, IC-MPGN, HSCT-TMA, ALS or any other indication when expected or at all; whether, if Apellis’ products receive approval, they will be successfully distributed and marketed; and other factors discussed in the “Risk Factors” section of Apellis’ Annual Report on Form 10-K filed with the Securities and Exchange Commission on February 28, 2022 and the risks described in other filings that Apellis may make with the Securities and Exchange Commission. Any forward-looking statements contained in this presentation speak only as of the date hereof, and Apellis specifically disclaims any obligation to update any forward-looking statement, whether as a result of new information, future events or otherwise.

# Apellis is revolutionizing complement science to deliver life-changing medicines

*Targeting C3 for comprehensive control of complement*



**Advancing the first potential treatment for geographic atrophy (GA)**  
**U.S. PDUFA: Feb 26, 2023**



 **EMPAVELI<sup>®</sup>**  
(pegcetacoplan) injection  
1080 mg/20 mL solution



**750+**  
employees  
globally



**12**  
clinical and pre-  
clinical programs



Focused on  
**PATIENTS**

# 2022 was a transformational year



## Geographic Atrophy (GA): Ready to launch first-ever treatment

- ✓ Submitted NDA to U.S. FDA
- ✓ Submitted MAA to EMA
- ✓ U.S. commercial preparations complete; deployed ~100 field-based employees mid-2022
- ✓ Appointed leading retina specialist, Dr. Caroline Bauman as Chief Medical Officer (effective Jan 2023)



## Paroxysmal Nocturnal Hemoglobinuria (PNH): Continued growth in demand

- ✓ ~\$65 million<sup>1</sup> in U.S. net product sales
- ✓ Submitted sNDA for PRINCE & 48-week PEGASUS data
- ✓ Submitted sNDA for EMPA VELI injector
- ✓ Completed >800 patient years of systemic dosing<sup>2</sup>



## Pipeline: Leaders in complement

- ✓ Progressed late-stage clinical studies with partner Sobi
- ✓ Advanced early-stage pipeline across therapeutic areas
- ✓ Completed enrollment in Phase 2 ALS study

# Positioning Apellis for an extraordinary 2023

## *Retina in focus*

1

Deliver the  
**first-ever treatment  
to patients with GA**  
in the U.S.

2

Prepare for  
**ex-U.S. approvals  
in GA**

3

Maximize  
the value of  
 **EMPAVELI**®

4

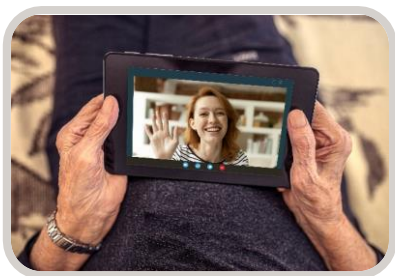
Advance early  
pipeline and Beam  
collaboration

*... with compassion and commitment to patients*

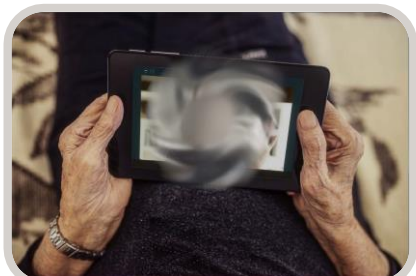
# GA is the leading cause of blindness worldwide

**5M**  
PATIENTS  
GLOBALLY<sup>1</sup>

.....  
1M with GA  
in US<sup>1</sup>



Normal vision



Vision with advanced GA

## GAINS, a global patient survey, showed:

- Nearly 7 in 10 (68%) believe **impact on independence and quality of life** due to visual decline **is worse than expected**
- ~80% agree their **vision was impacted faster than expected**
- ~70% of patients find it **hard to enjoy life** as much as they had prior to GA diagnosis

Note: The Geographic Atrophy Insights Survey (GAINS) was conducted by The Harris Poll and was sponsored by Apellis.

# Pegcetacoplan positioned to be first-ever treatment for GA

In Phase 3 DERBY and OAKS studies at 24 months, pegcetacoplan showed:

✓ **Increasing treatment effects over time**



✓ **Robust effects in two dosing regimens**



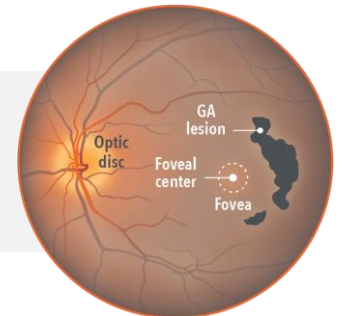
EVERY OTHER MONTH

OR



MONTHLY

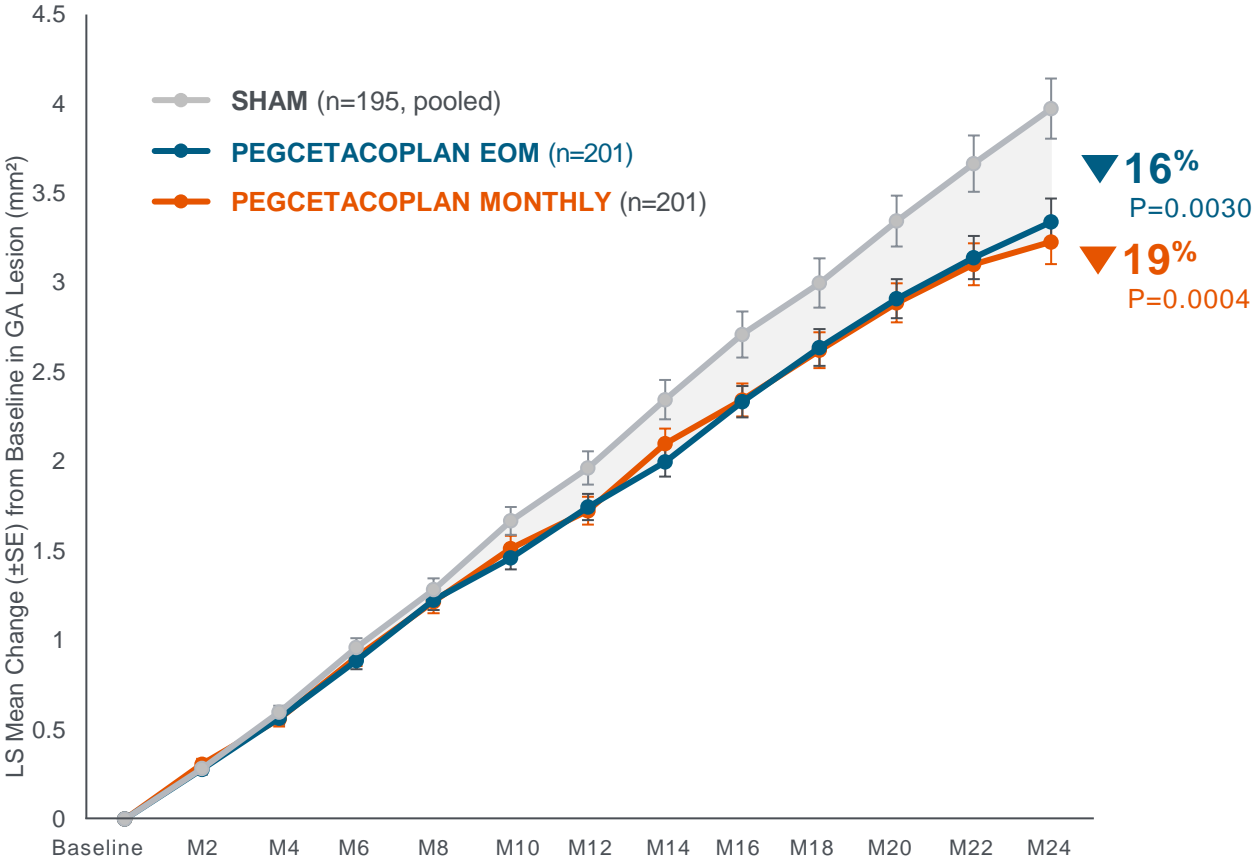
✓ **Slowed GA progression regardless of lesion location**



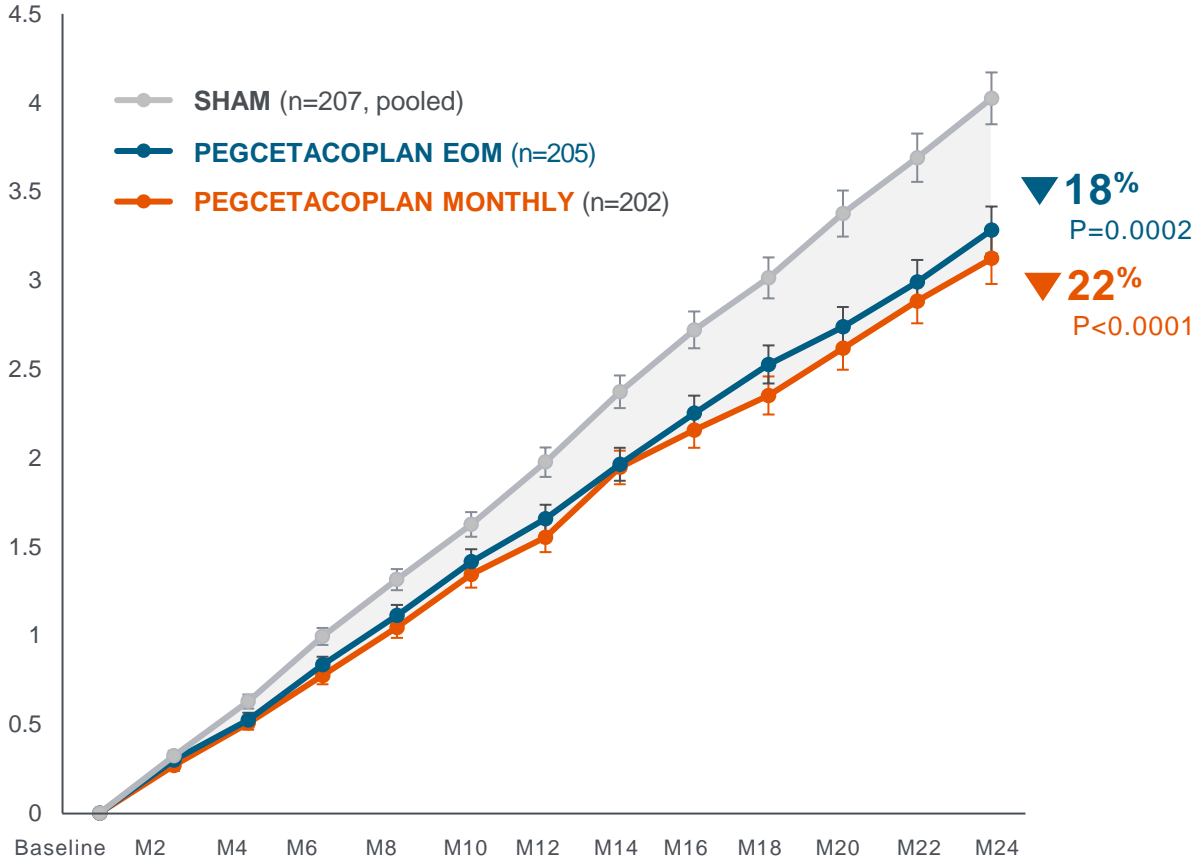
# Pegcetacoplan slowed GA progression with every-other-month and monthly dosing

P values are nominal (not controlled for multiplicity)

## DERBY



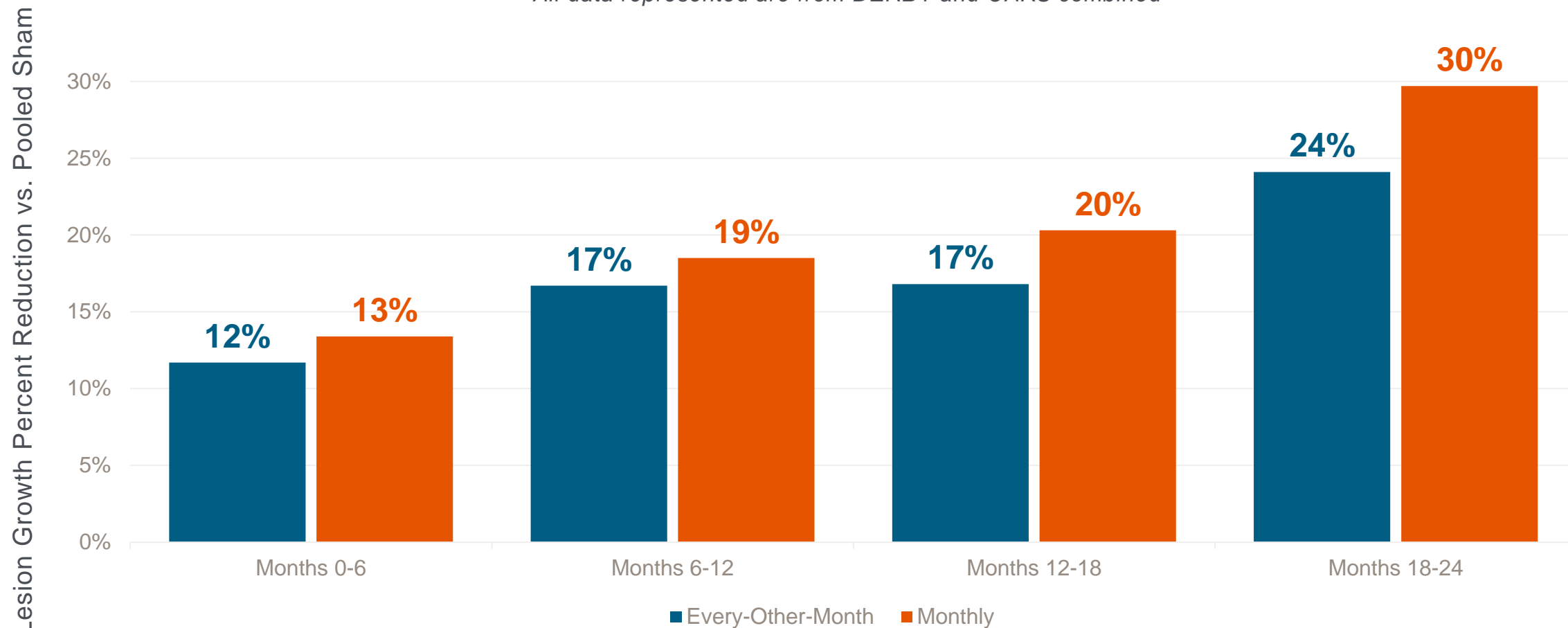
## OAKS



GA= geographic atrophy; SE= standard error. Least square (LS) means estimated from a mixed-effects model for repeated measures (MMRM). The mITT population was used for the analysis, defined as all randomized patients who received at least 1 injection of pegcetacoplan or sham and have baseline and at least one post-baseline value of GA lesion area in the study eye.

# Pegcetacoplan showed increasing treatment effects over time

All data represented are from DERBY and OAKS combined



P values for monthly and EOM, respectively, at **6 months**:  $P=0.0058$ ,  $P=0.0141$ ; **12 months**:  $P=0.0005$ ,  $P=0.0012$ ; **18 months**:  $P<0.0001$ ,  $P=0.0004$ ; **24 months**:  $P<0.0001$ ,  $P<0.0001$ . P values are nominal (not controlled for multiplicity)

Percent reduction vs. pooled sham for Month 0 to Month 24 was estimated from a piecewise linear slope model with 6-month segments using the combined patient-level data, not a simple average of results, from the two studies. Point estimates for the Month 0 to Month 18 segments vary marginally from previously reported numbers due to the inclusion of the Month 20 to Month 24 data into the statistical model.

# Pegcetacoplan slowed GA progression regardless of lesion location

*All data represented are from DERBY and OAKS combined between months 18 and 24*

	Subfoveal Lesions (foveal)	Non-subfoveal Lesions (extrafoveal)
Monthly	34%	28%
Every-Other-Month	28%	28%

# Pegcetacoplan demonstrated a favorable safety profile

All data represented are from DERBY and OAKS combined

	EXUDATIONS <sup>1</sup>		
	EOM	Monthly	Sham Pooled
0-12 months	4.1%	6.0%	2.4%
0-24 months (cumulative)	6.7%	12.2%	3.1%



Rates of endophthalmitis and intraocular inflammation generally in line with those reported in studies of other intravitreal therapies

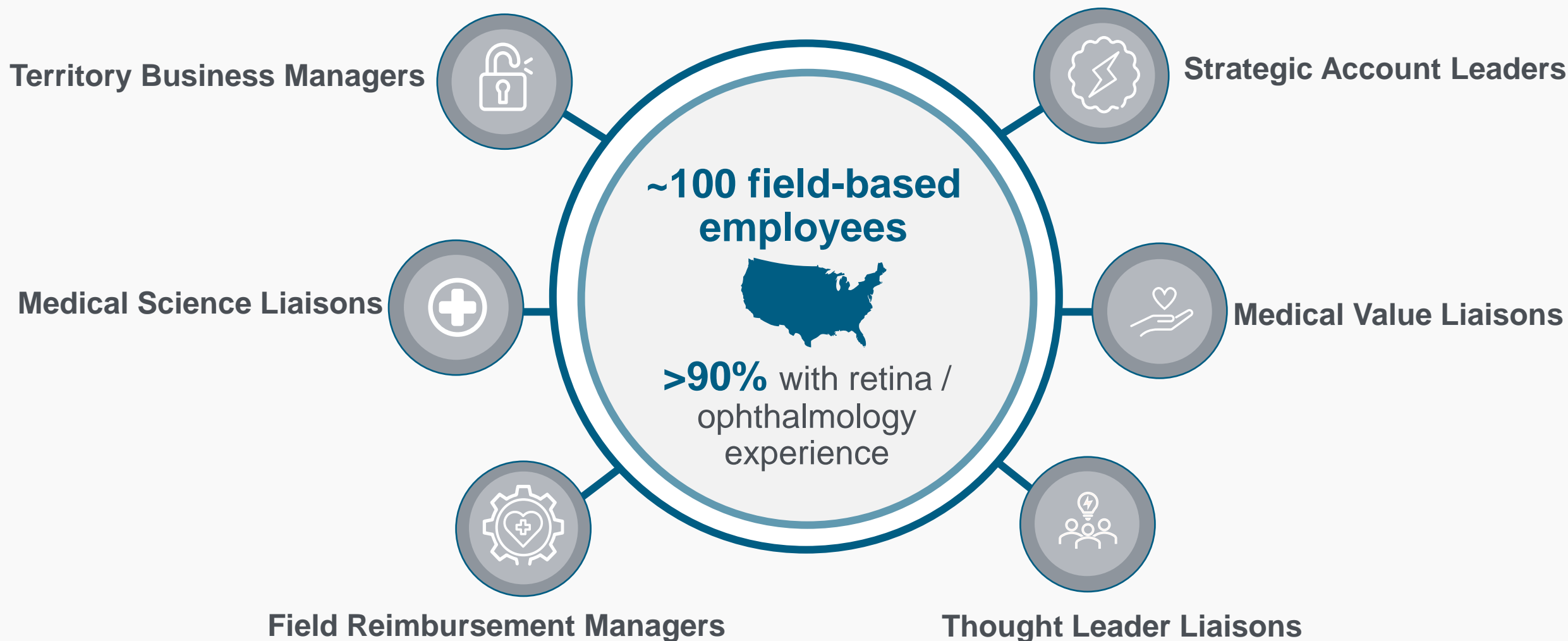
*...across 1,200 patients and nearly 12,000 injections*

1. Exudations include all adverse events reported by the investigator as choroidal neovascularization (CNV) or neovascular AMD, whether or not there was reading center confirmation

# Significant commercial opportunity with physicians eager to treat with pegcetacoplan



# Built a best-in-class team highly experienced in the retina; fully deployed in August 2022



# GA pre-launch activities underway ahead of February 26 PDUFA

## MEDICAL

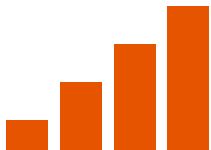


**MOSAIC**

MOSAIC GA Patient  
& Caregiver Burden  
of Illness Study



**>20** Scientific  
Education Programs



**>100** Additional Platform  
Presentations & Publications

*...throughout 2022*

## COMMERCIAL



**>65** Physician Disease  
State Education (DSE)  
Campaign and Peer-to-  
Peer Meetings

Targeted media  
consistently reach **99%** of  
retina specialists



Launched patient education efforts to support increased  
understanding of GA

*...since September 2022*

## PAYER

Engagement with National Payers  
representing **~80%** of Medicare  
Advantage Lives



Distribution model and patient  
support services and resources  
finalized



# Pegcetacoplan positioned to be first-ever treatment for GA

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Compelling product profile to address unmet need globally



>90% of surveyed retina specialists worldwide plan to use pegcetacoplan



Best-in-class field-based team deployed and ready for launch

# Positioning Apellis for an extraordinary 2023



*... with compassion and commitment to patients*

# Successful EMPAVELI launch, elevating the standard of care in PNH



As of December 31, 2022:

- **~\$65 million<sup>1</sup>** in FY 2022 sales
- **>260 HCPs** with **REMS certifications**
- **>230 start forms** submitted
- **~10%** of 2022 start forms were **naïve patients**
- **98% real world patient compliance rate**

“My hemoglobin is in the 12's as compared to the 8's. This is the first time in 6 years that I do not have to get bloodwork weekly or monthly ... I don't get repeat bloodwork for 6 months! EMPAVELI is life changing [for me].”

– EMPAVELI patient

*Individual results may vary*

# Strengthening the EMPAVELI label and the patient experience in 2023



**Supplemental NDA U.S. PDUFA Date**

**Feb. 8, 2023**

*Enhancing our label with treatment-naïve data from PRINCE and 48-week PEGASUS results*



**EMPAVELI Injector U.S. PDUFA Date**

**March 15, 2023**

*Improving the patient experience and administration*

# Strong safety profile demonstrated through real-world experience



Over 800 patient years of systemic pegcetacoplan exposure<sup>1</sup>



Zero reported cases of meningococcal infection<sup>1</sup>



Thrombosis rate of 1.13 events per 100 patient-years<sup>2</sup>

# Expanding potential indications

LATE STAGE PROGRAMS	IC-MPGN / C3G 	ALS 	CAD 	HSCT-TMA 
U.S MARKET OPPORTUNITY	~5,000 <sup>1</sup>	~19,000 <sup>2</sup>	~5,000 <sup>1</sup>	~4,000 <sup>3</sup>
CURRENT STATUS	Phase 3 ongoing (Apellis)	Mid-2023: Phase 2 top line results (Apellis)	Phase 3 ongoing (Sobi)	H2 2023: Phase 2 data (Sobi)

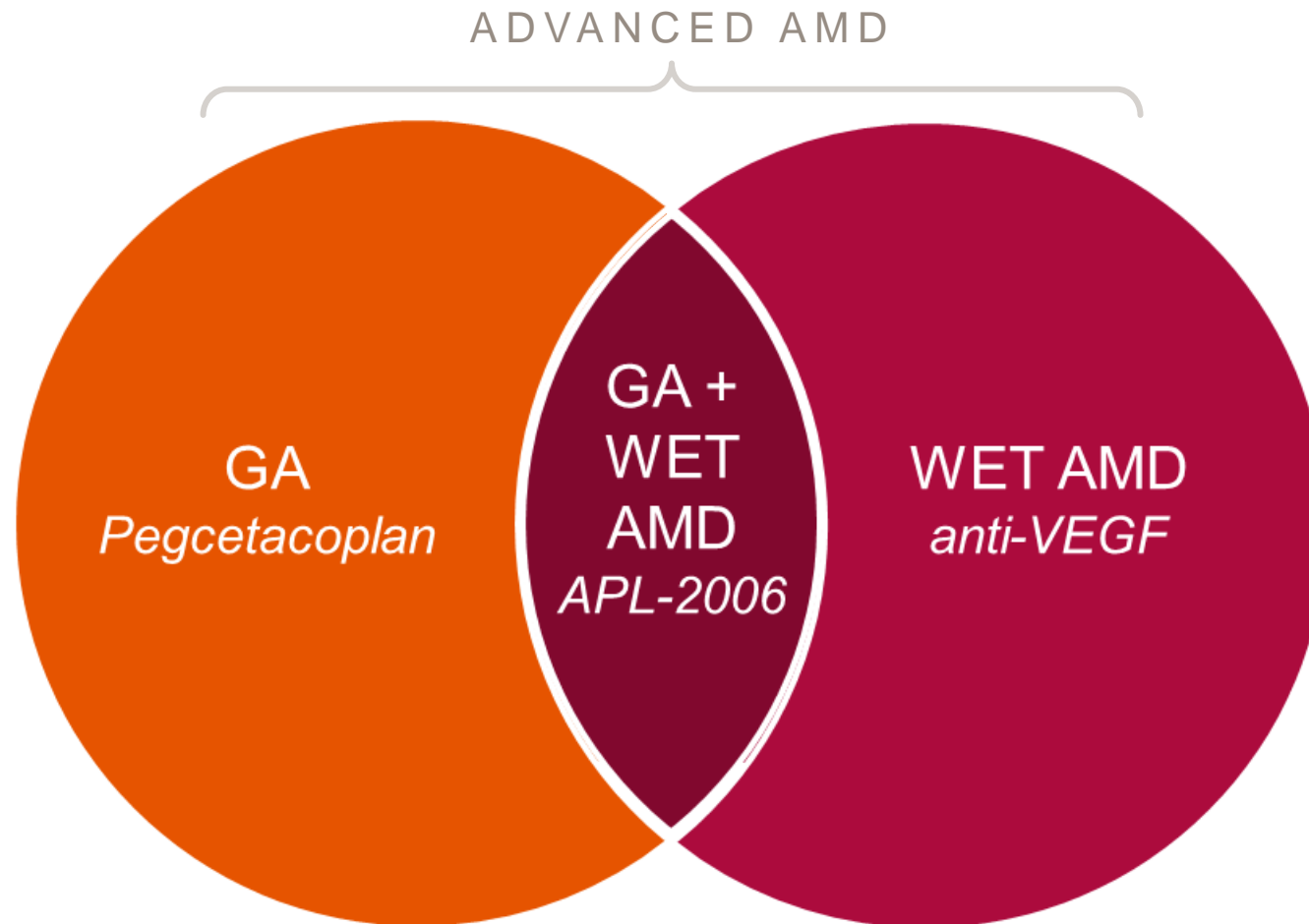
1. Based on moderate & severe patient population. CAD: Catenion using physician and literature consensus. Passweg et al, BMT. 2019, 38: 1575–1585 sus. C3G: ClearView Analysis using physician and literature consensus.  
 2. Based on sporadic only, patients seeking treatment, and non-monotherapy patients. ALS: ClearView Analysis based on physician interviews. 3. Based on TMA patients who display at least one high-risk feature. Phelan, R., Arora, M., Chen, M. Current use and outcome of hematopoietic stem cell transplantation: CIBMTR US summary slides, 2020.. Jodele et al, Blood. 2014, 124(4): 645–653. Sobi has global co-development and ex-U.S. commercialization rights for systemic pegcetacoplan.

# Positioning Apellis for an extraordinary 2023



*... with compassion and commitment to patients*

# APL-2006: Developing potential best-in-class treatment for patients with wet AMD + GA; IND planned H1 2023



# Next generation C3 siRNA + EMPA VELI



**Reduce treatment frequency** of EMPA VELI independent of indication



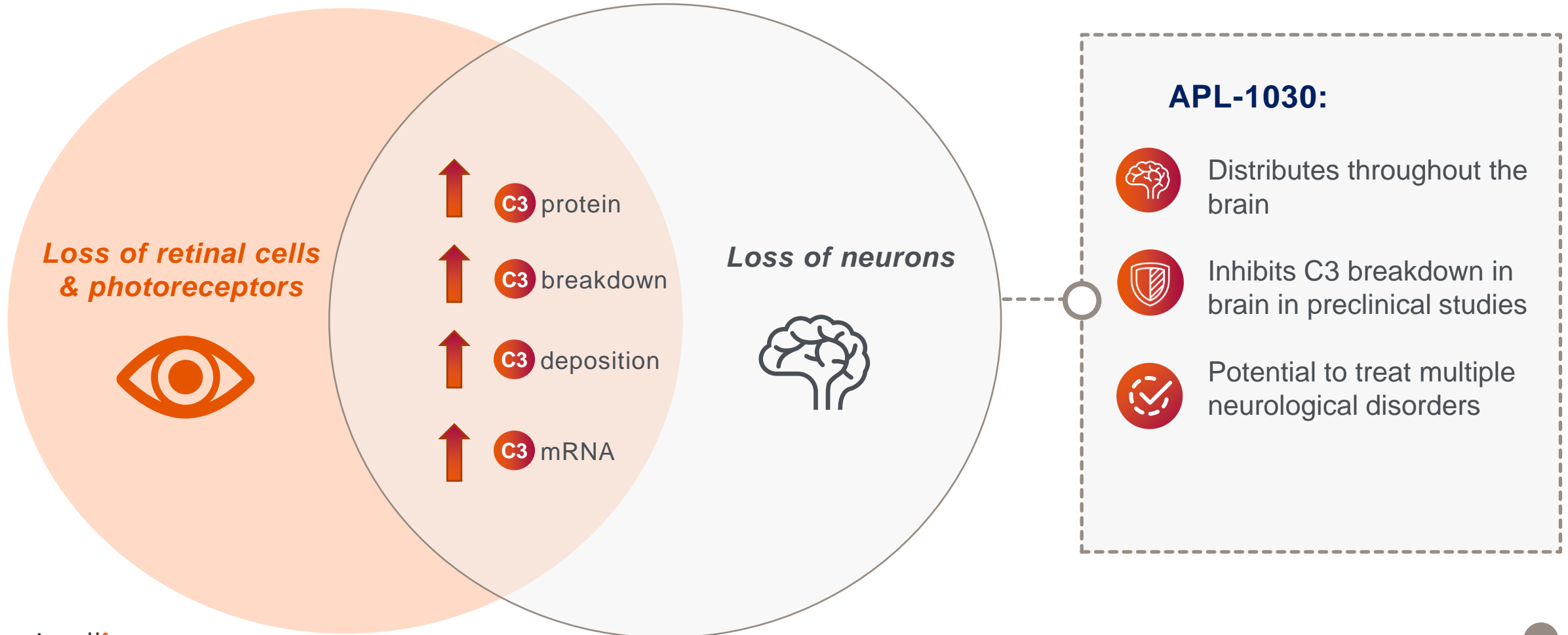
Silenced C3 expression **>90%** in pre-clinical studies



**IND planned H1 2023**

# Advancing APL-1030 to establish proof of concept for C3 inhibition in neurological diseases

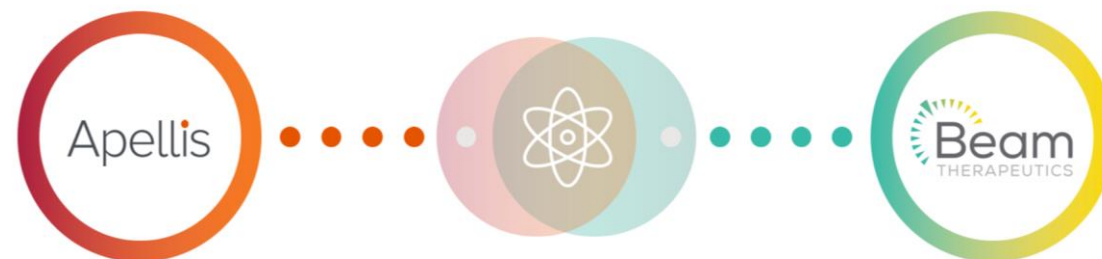
*The eye as a window to the brain:*



# Advancing our Beam collaboration

## Beam COLLABORATION:

- ✓ Research programs targeting eye, liver and brain advancing in preclinical studies



**6 research programs**  
over 5 years



**Base editing technology**  
to discover precision  
medicines for diseases of  
the eye, liver, and brain



**Focused on C3**  
and other  
complement targets



**Modulating**  
a complex biological  
system

# Pipeline

	PRODUCT	DISEASE	PRECLINICAL	PHASE 1	PHASE 2	PHASE 3	APPROVED
RARE DISEASE	EMPAVELI® (systemic pegcetacoplan)*	PNH	Marketed in the US				
		IC-MPGN & C3G					
		ALS	* Data expected mid-2023				
		CAD					
		HSCT-TMA	Phase 2 data expected H2 2023				
	siRNA + EMPAVELI**	Existing + new indications	IND in 1H'23				
OPHTHALMOLOGY	Intravitreal pegcetacoplan	GA	U.S. PDUFA Feb. 26 '23				
	APL-2006	GA & Wet AMD	IND in 1H'23				
	Gene therapies	Wet AMD, Intermediate AMD & GA					
NEUROLOGY	APL-1030	Undisclosed					
	Brain shuttle	Undisclosed					
	Gene therapies	Undisclosed					
MULTIPLE THERAPEUTIC AREAS	Systemic pegcetacoplan	Control of host attack for gene therapies					
	Oral alternative pathway inhibitor	Mild C3G and other indications					
	Gene-edited therapies (Beam)	Undisclosed					

\*Sobi has global co-development and ex-U.S. commercialization rights for systemic pegcetacoplan \*\*Initial IND for siRNA

The safety and efficacy of the agents for the indications under investigation have not been established.

\* Potential to be registrational

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