Apellis

JPM Presentation



Forward looking statements

Apellis

Statements in this press release 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. These statements include, but are not limited to, statements relating to the implications of preliminary clinical data and planned or future clinical trials and the timing thereof. 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 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 such as the results referenced in this presentation will be indicative of results that will be generated in future clinical trials; whether APL-2 will successfully advance through the clinical trial process on a timely basis, or at all, and receive approval from the United States Food and Drug Administration or equivalent foreign regulatory agencies; whether, if Apellis' products receive approval, they will be successfully distributed and marketed; and other factors discussed in the "Risk Factors" section of Apellis' Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission on December 20, 2017, and the risks described in other filings that Apellis may make with the Securities and Exchange Commission. Any forward-looking statements contained in this press release 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.

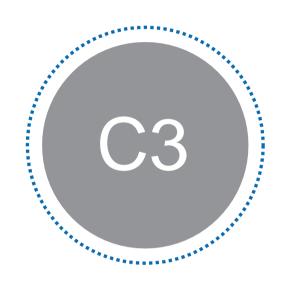


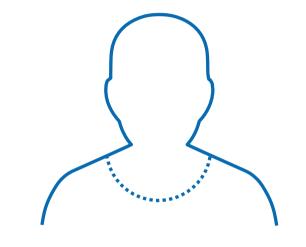
Geographic Atrophy Impacts One Million People in the U.S. Alone



What we do





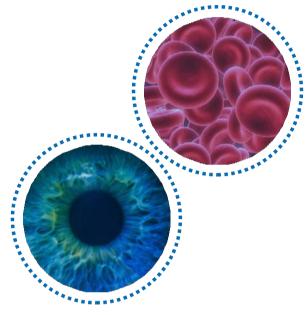


Pioneers in innate immunity & complement immunology

By regulating its core component C3

Value & patient outcomes at the center of our programs





Initially focused on AMD & PNH



Broad potential in other immune conditions

Pipeline

Product	Area	Disease	Pre-clinical	Phase 1	Phase 1b/2	Phase 3	Approval
APL-2 (intravitreal)	Ophthalmology	Geographic Atrophy (GA)					
APL-2 (subcutaneous)	Hematology	Paroxysmal Nocturnal Hemoglobinuria (PNH)					
		Auto-immune Hemolytic Anemia (AIHA)					
	Nephrology	Complement-dependent Nephropathies (CDN)					
APL-9 (intravenous)	Other	Undisclosed					



Apellis lead molecule: APL-2



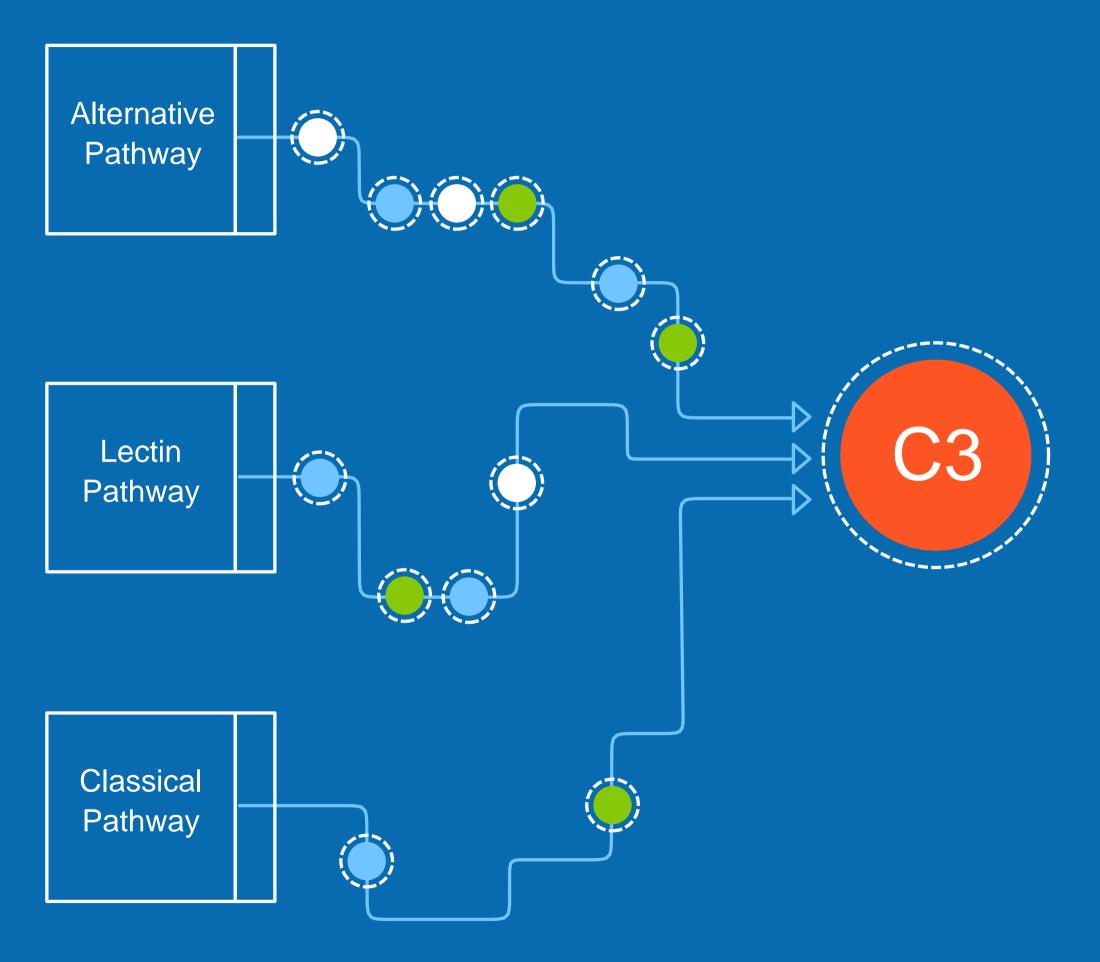
Peptides of the APL-2 family bind to a pocket of C3 and inhibit activation*

* Janssen, J. Biol. Chem., 282(40), 29241-29247, 2007



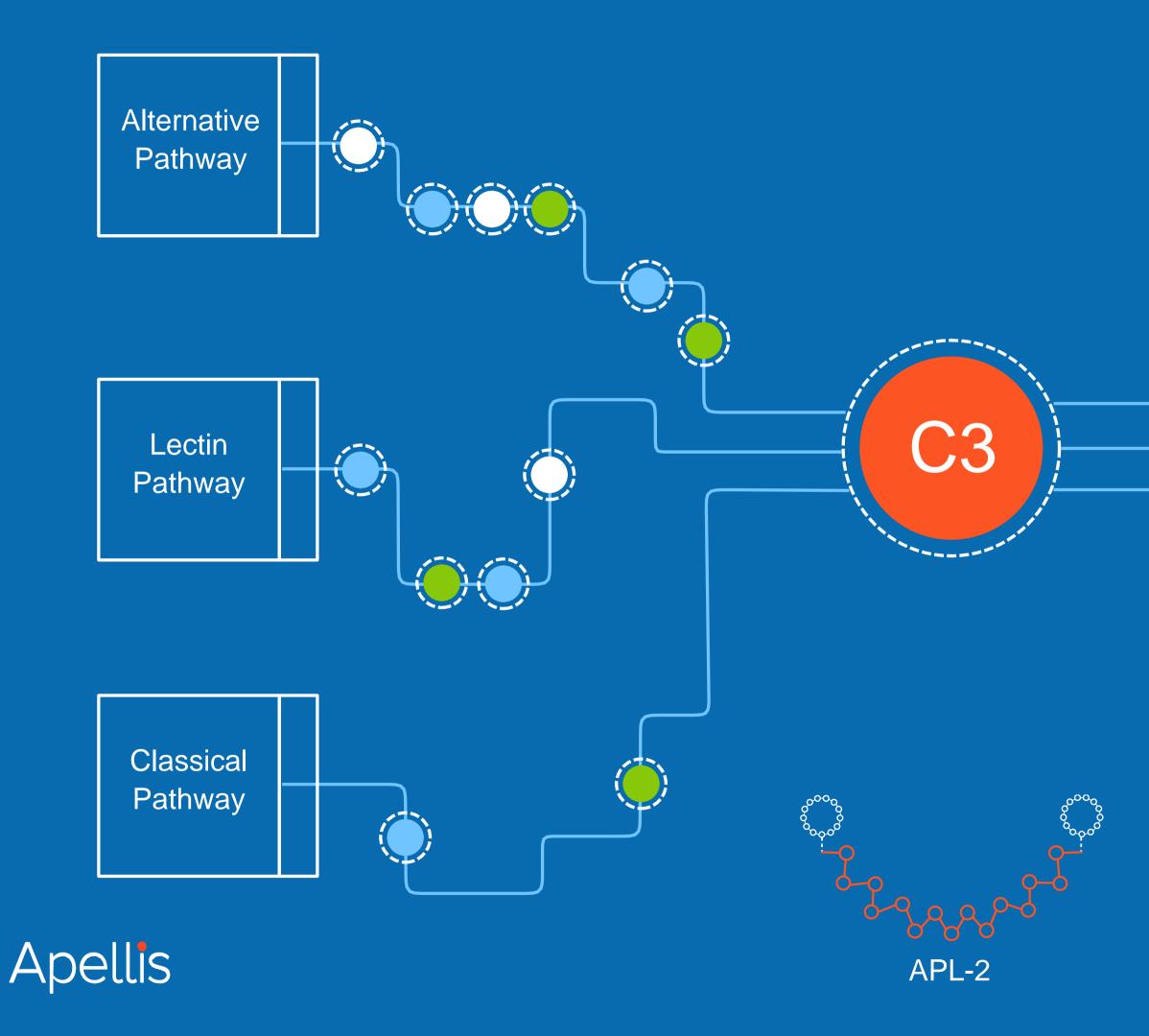


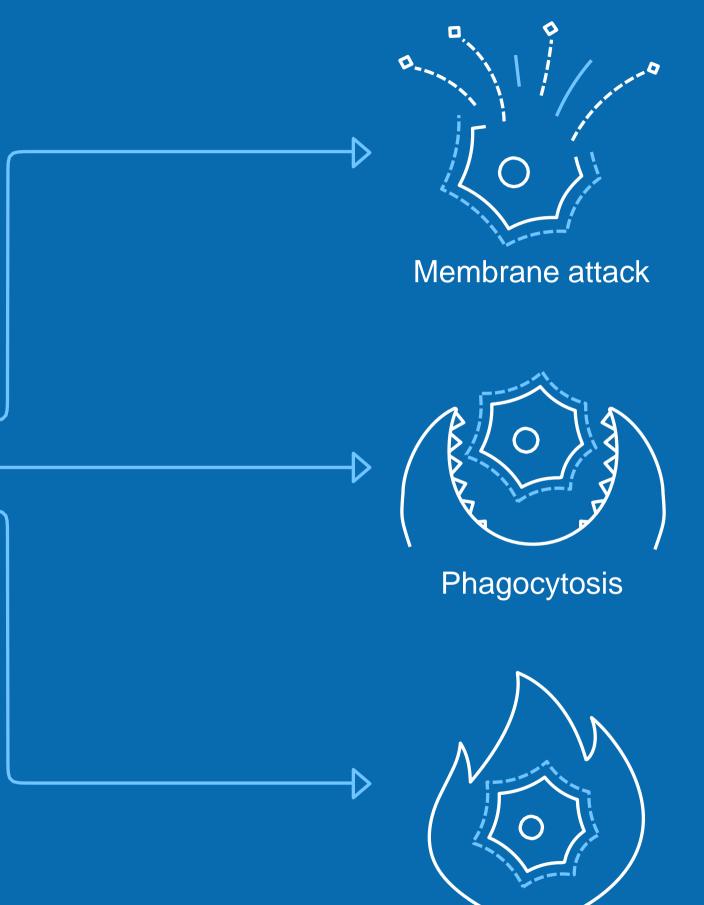
All pathways of complement converge on C3



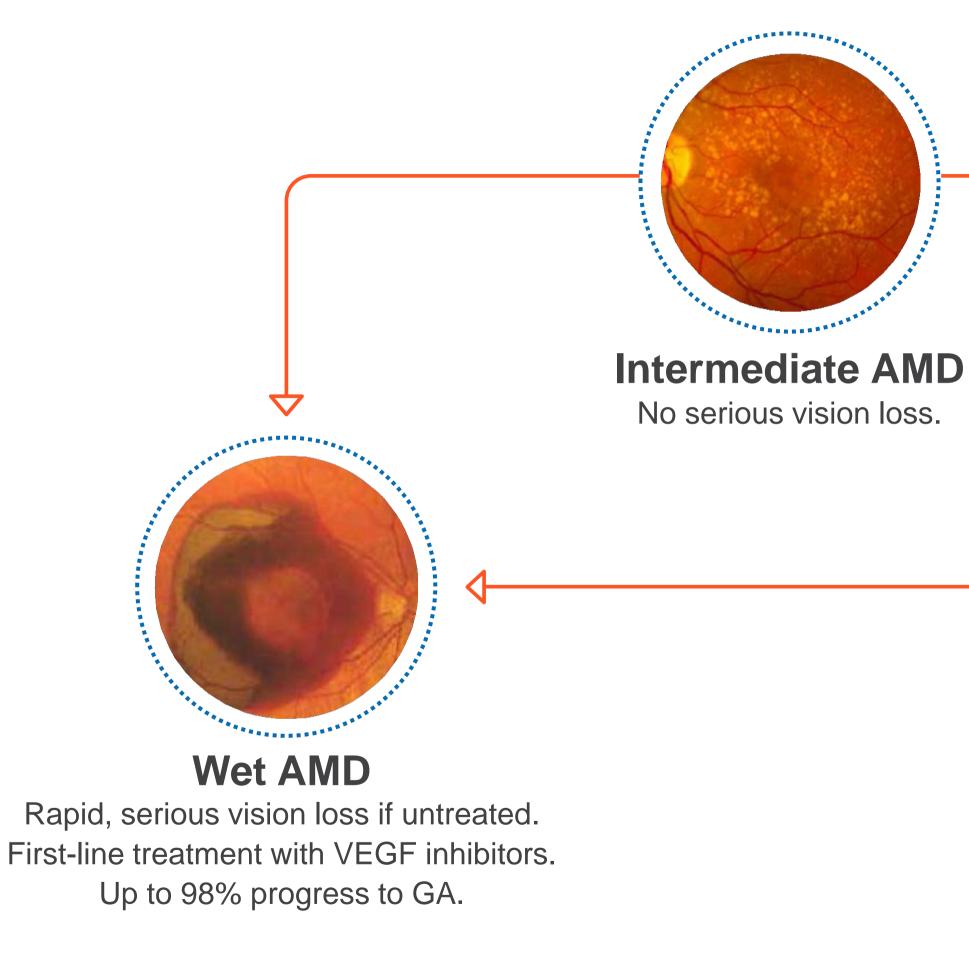


APL-2 targets C3 centrally in the complement cascade

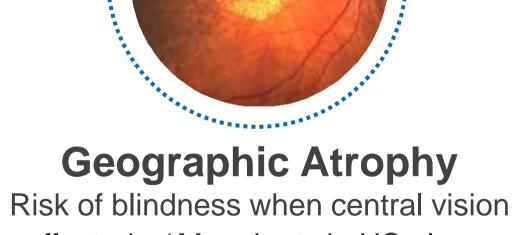




Geographic Atrophy - the leading cause of blindness



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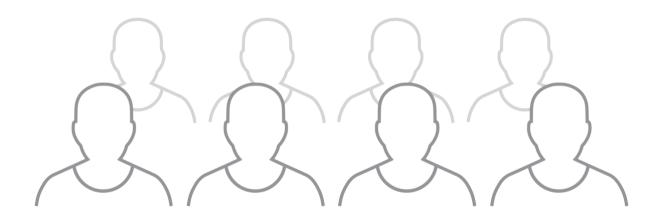
affected ~1M patients in US alone.

No approved therapies.

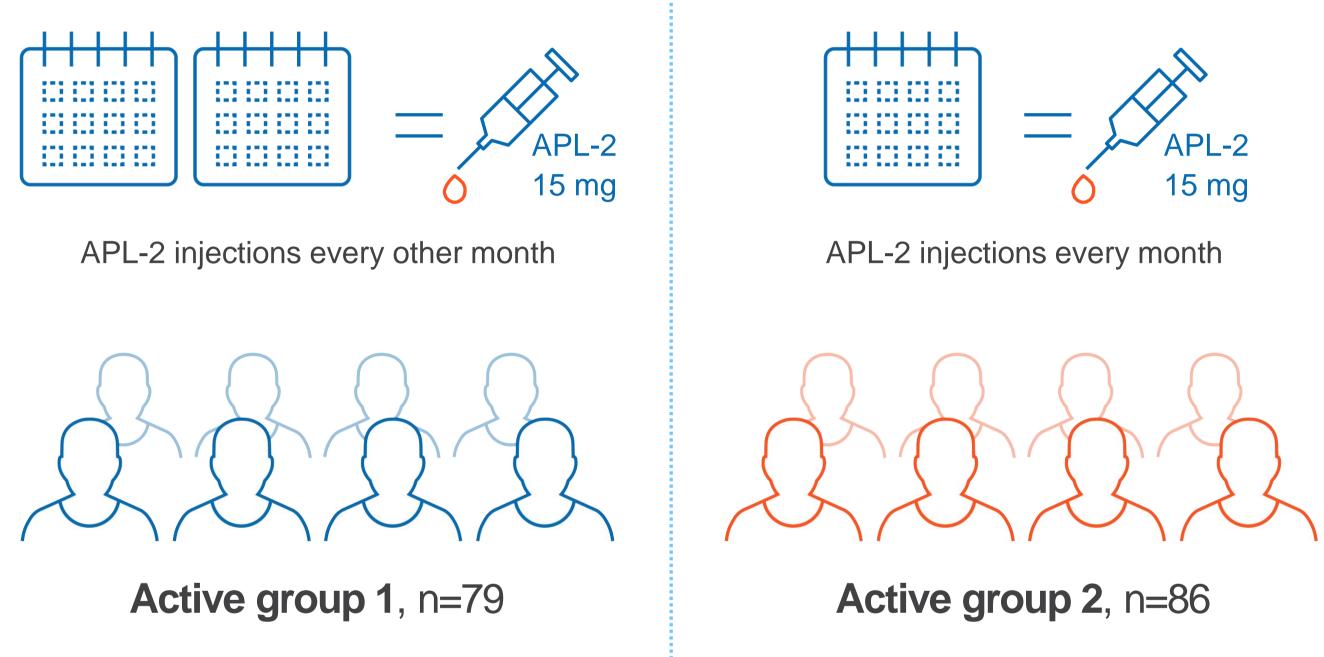
FILLY - Phase 2 study of APL-2 in Geographic Atrophy

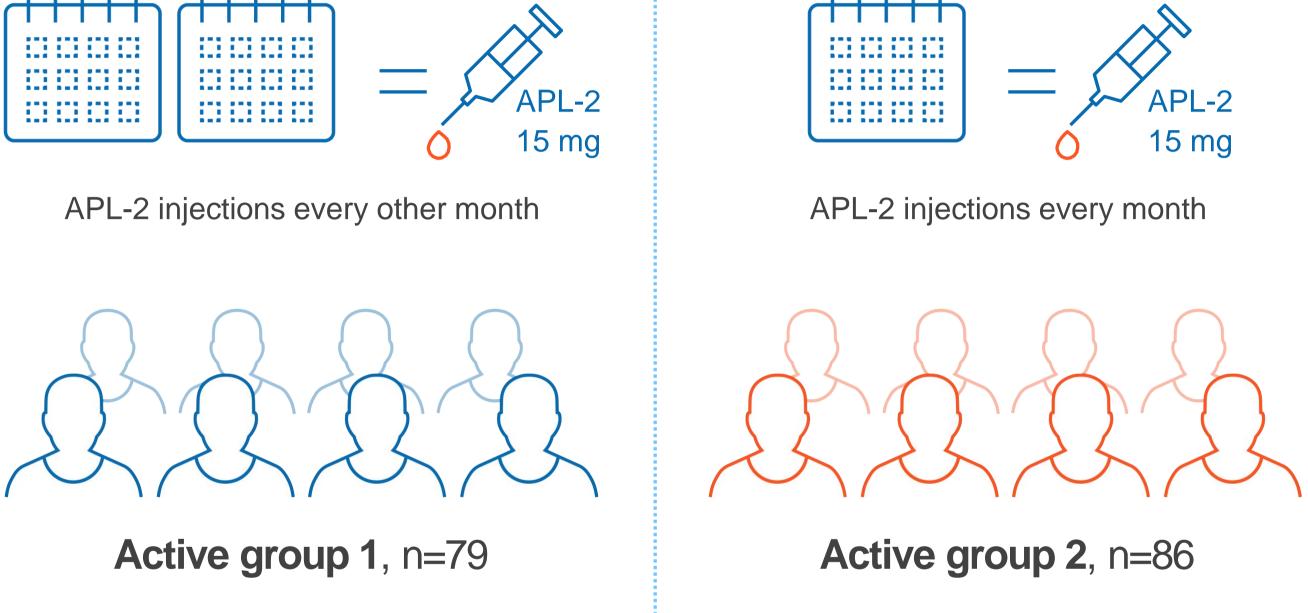


Sham injections



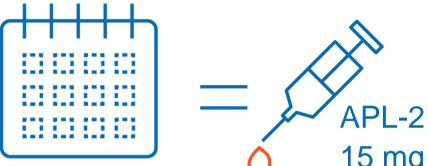
Sham group, n=81



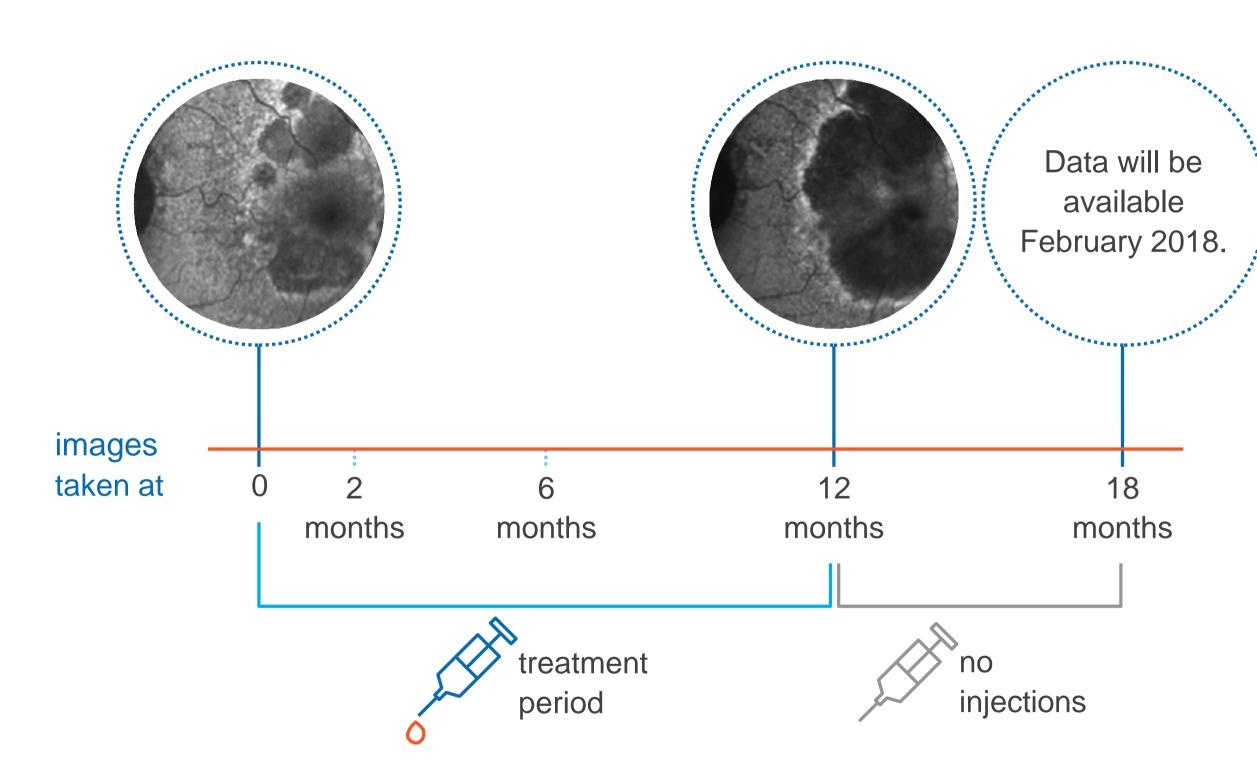


Phase 3 design finalized post FDA discussion

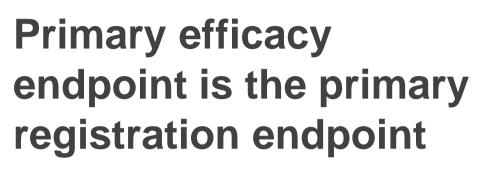




FILLY - primary endpoint



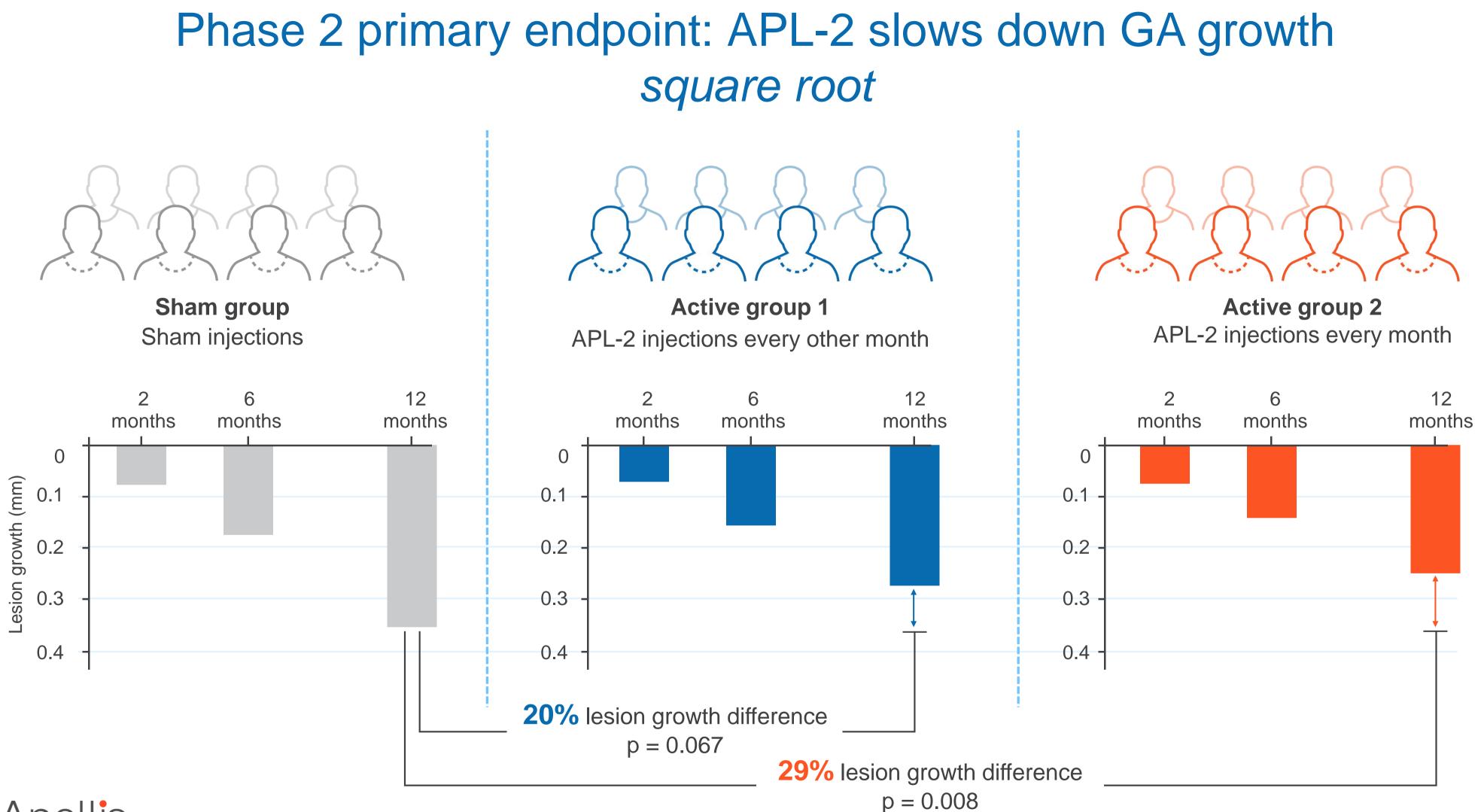




The primary endpoint is the change in geographic atrophy (GA) lesion size from baseline at month 12.

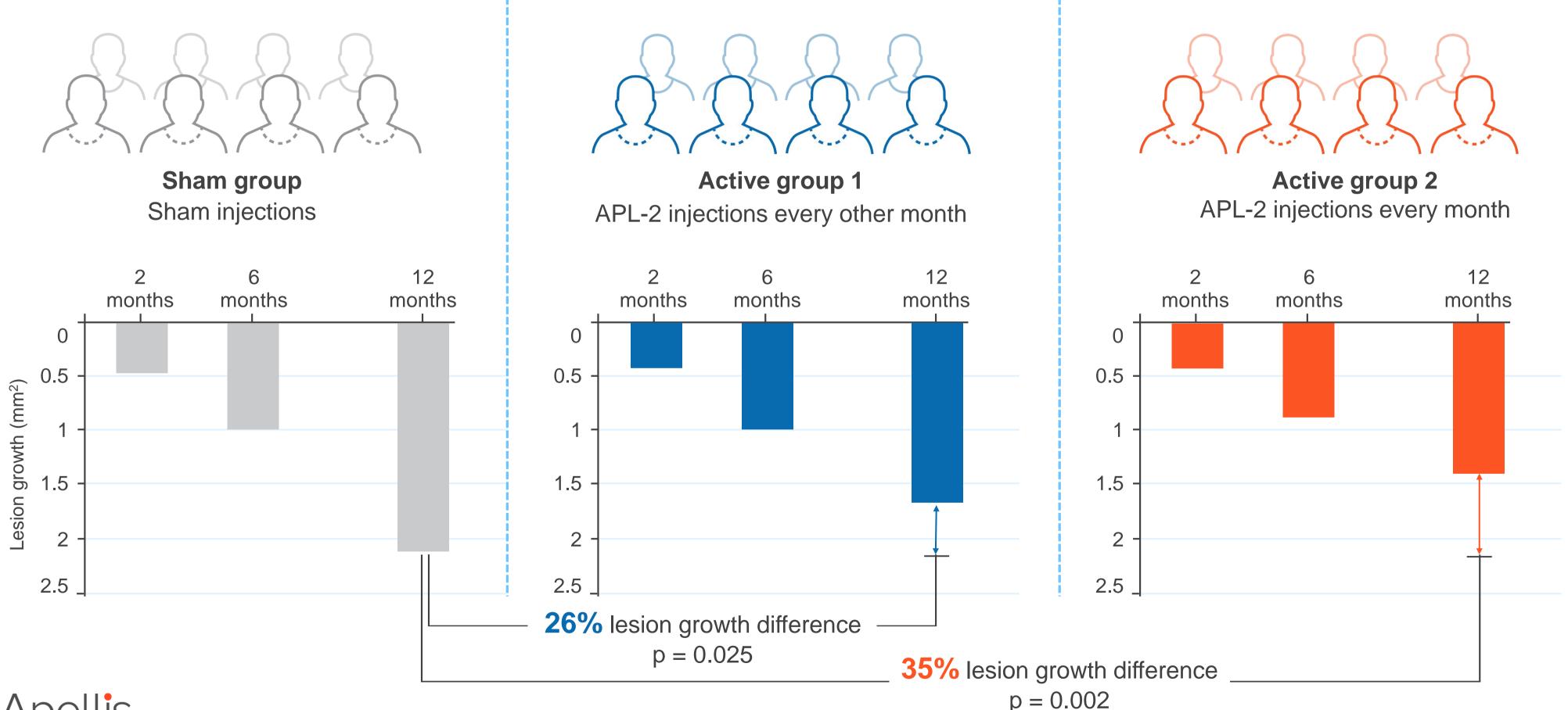
Primary safety endpoint

Number and severity of local and systemic treatment emergent adverse events (TEAE).



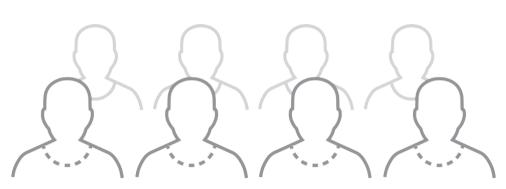
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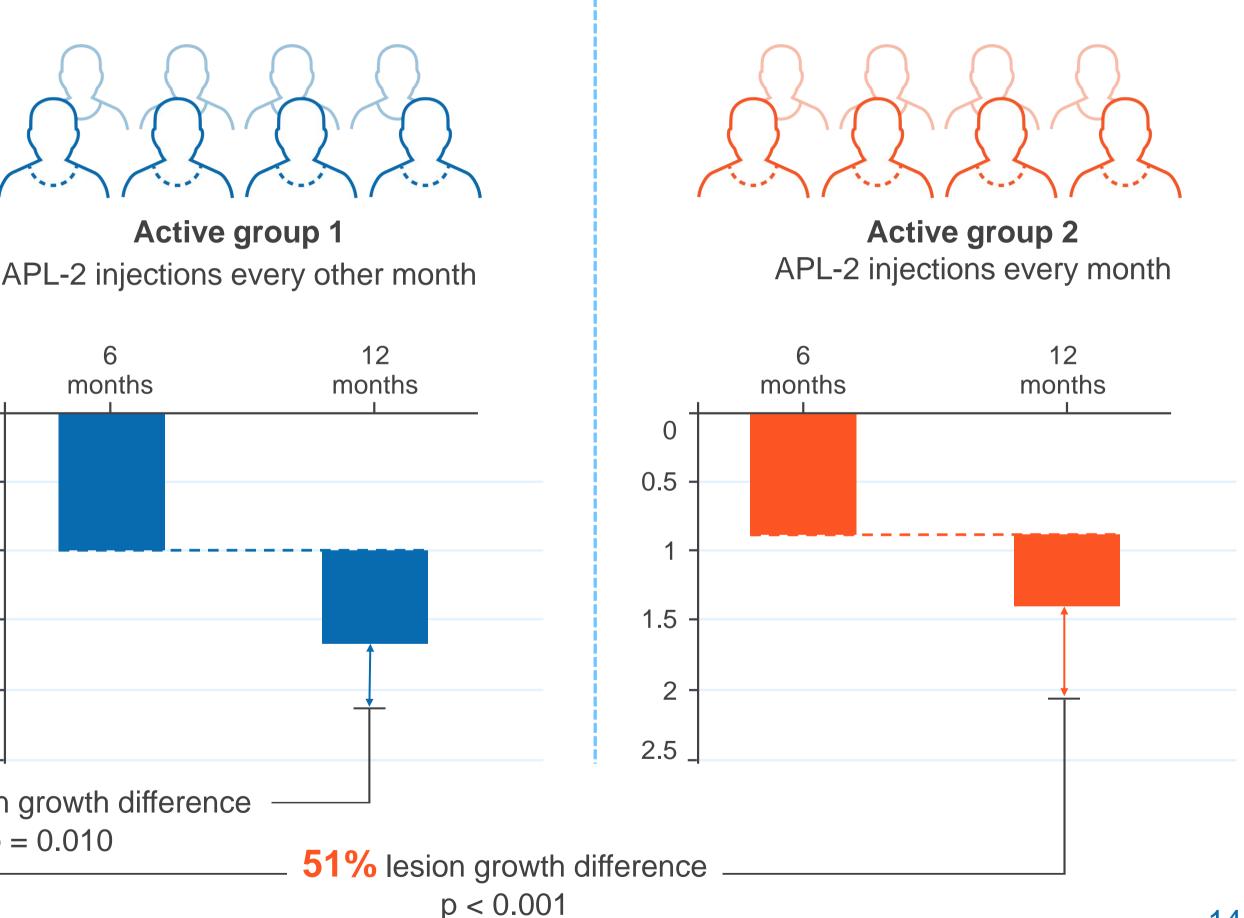
Phase 2 primary endpoint: APL-2 slows down GA growth absolute lesion size (Phase 3 primary endpoint)

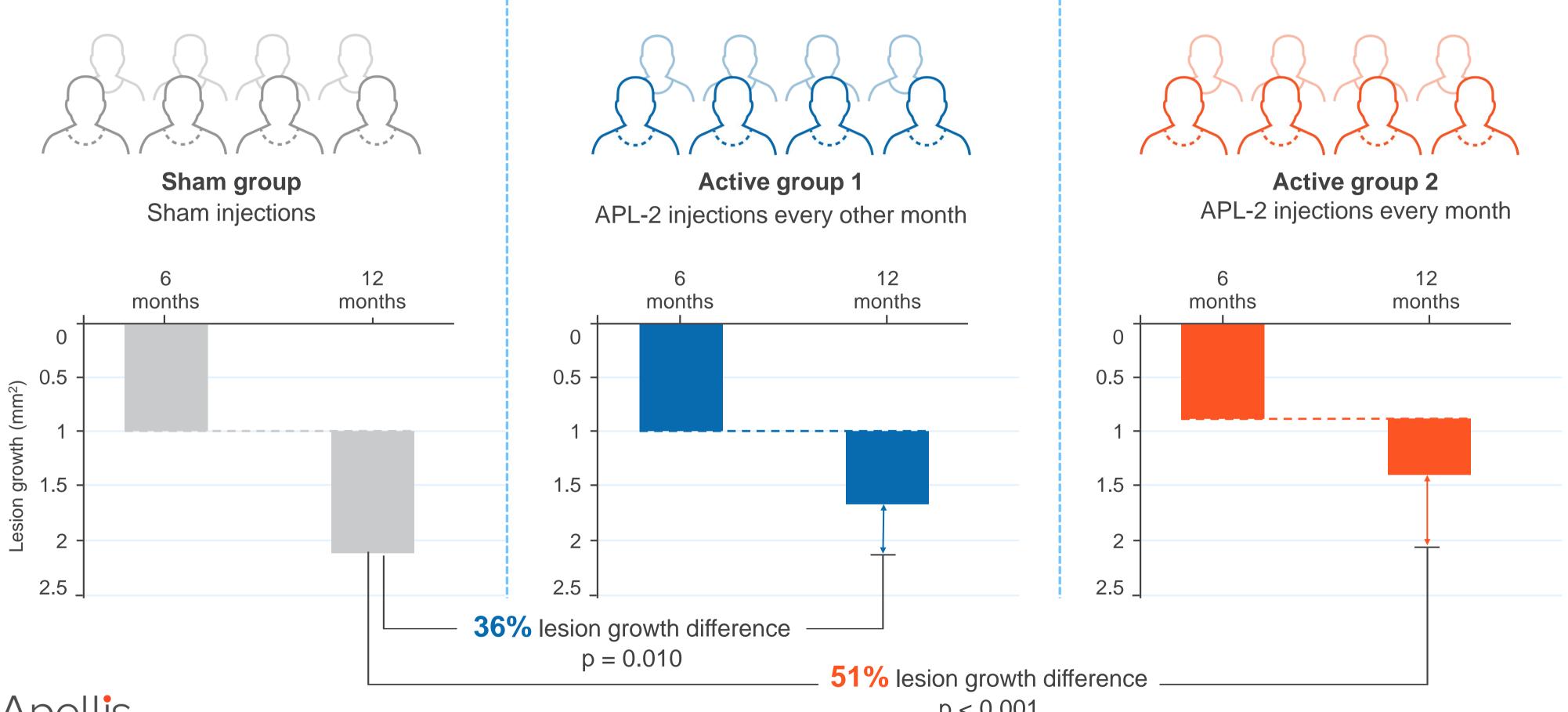


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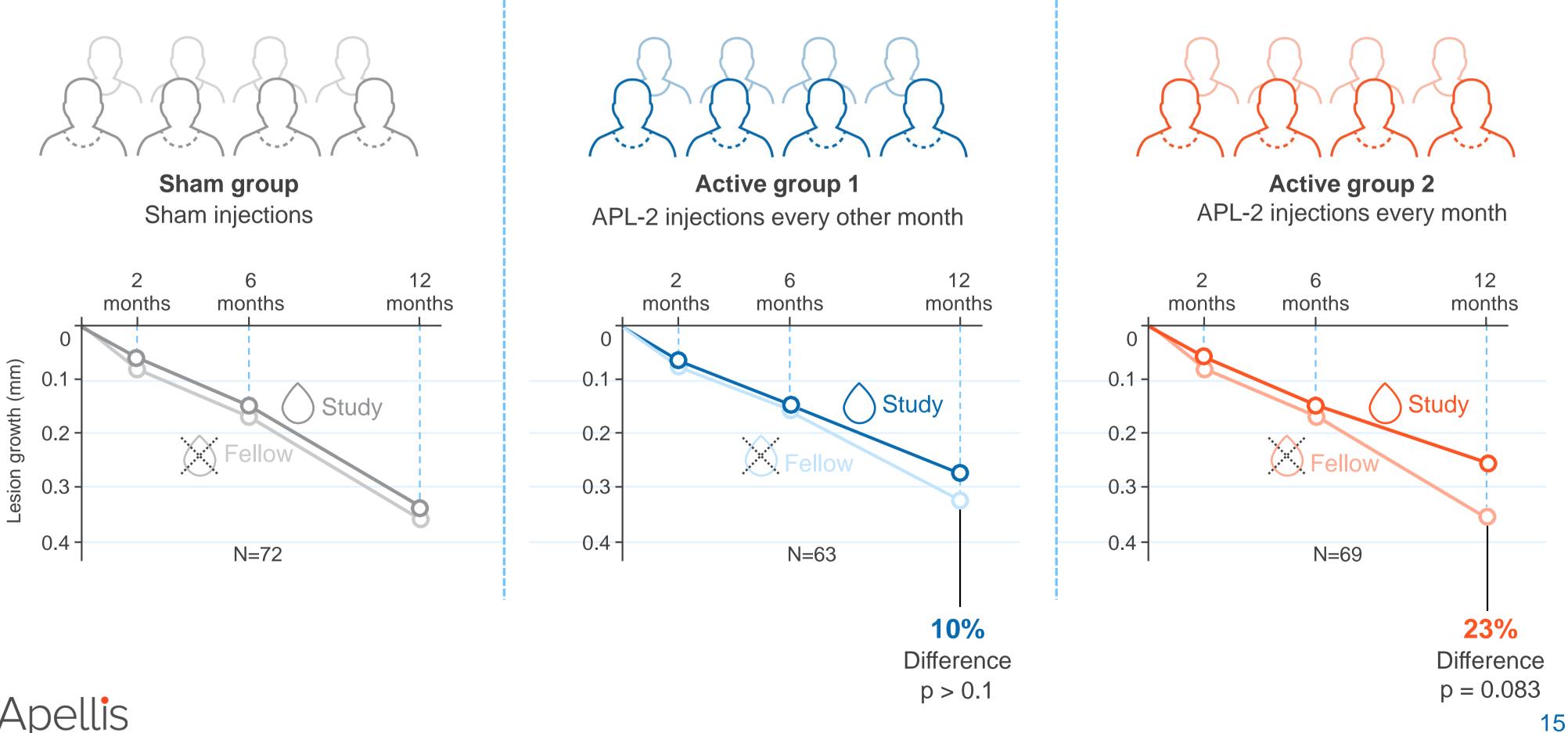
Phase 2 primary endpoint: APL-2 slows down GA growth absolute lesion size (Phase 3 primary endpoint)







GA growth comparison: fellow eye vs study eye

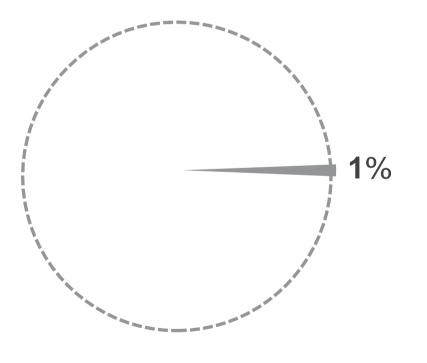


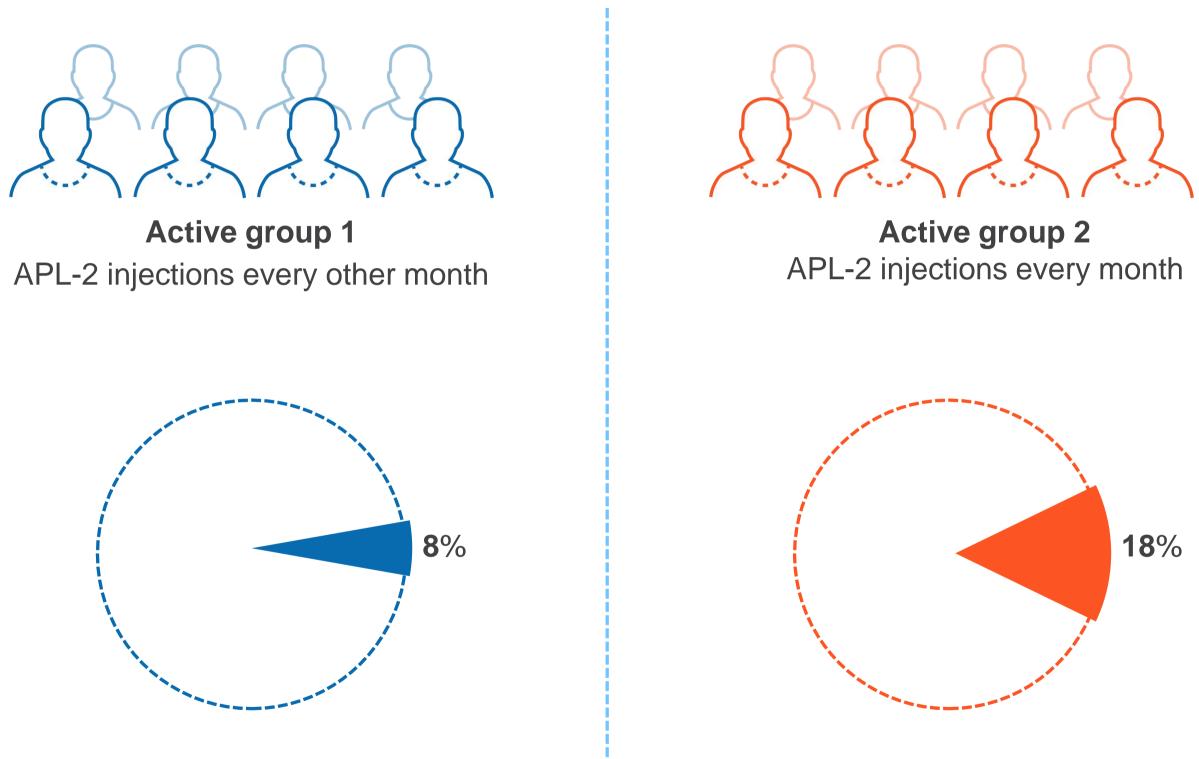


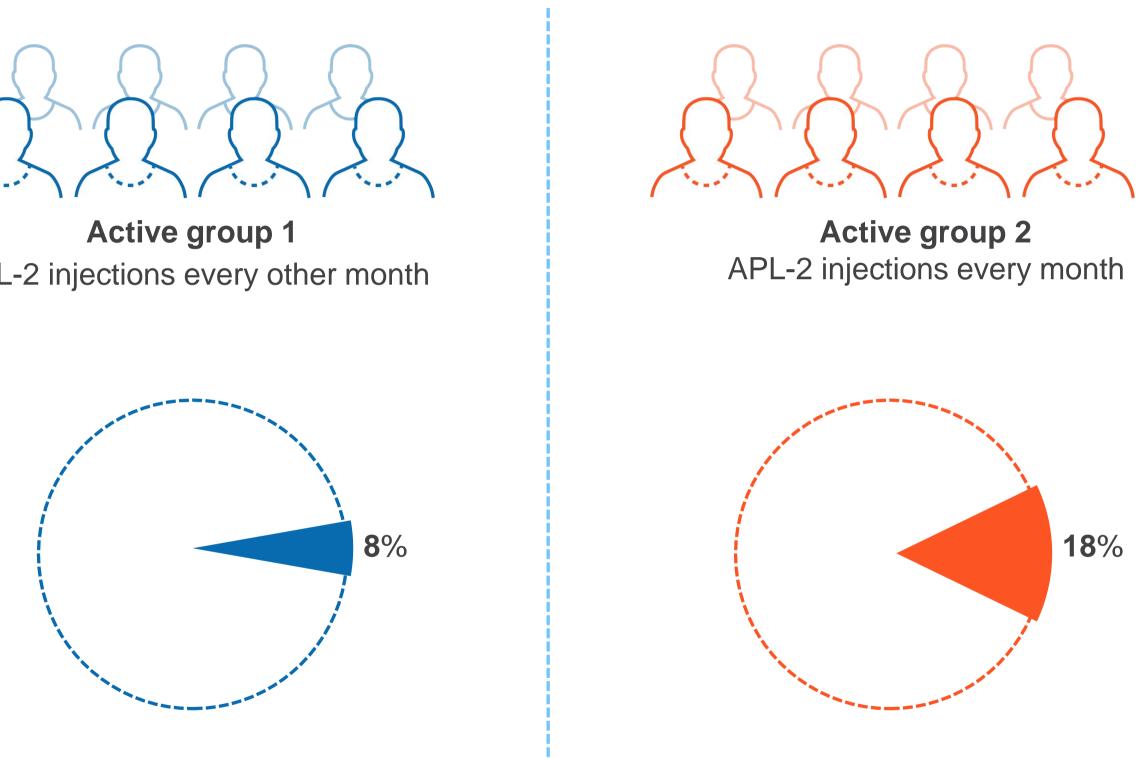
Conversions to Wet AMD



Sham group Sham injections

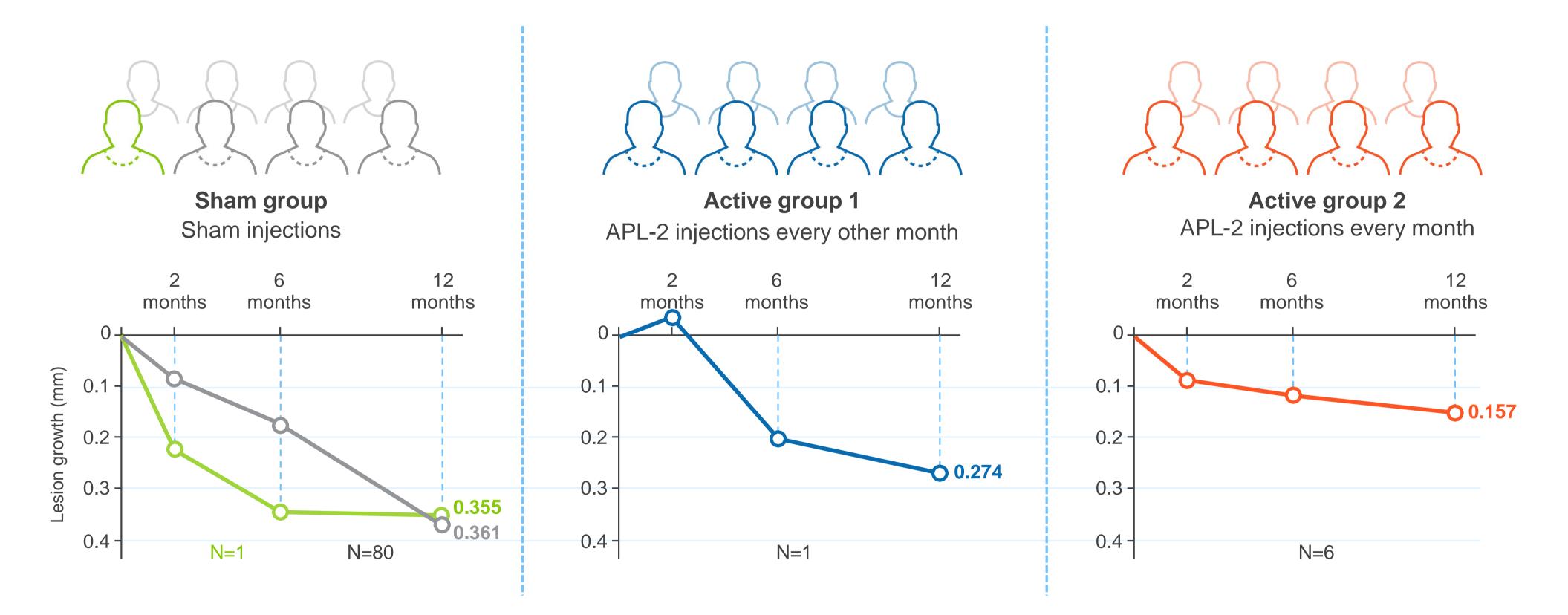








GA growth notably reduced in patients that converted to wet AMD

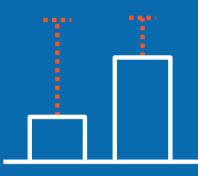


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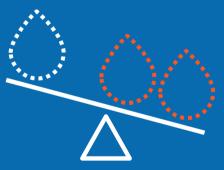
FILLY phase II trial



Preventing complement activation by blocking C3



Statistically significant data in largest Phase II in GA (n=246)



Results correlated to treatment frequency



Increased effect size over time

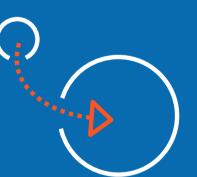




No specific genotype driving results



Further confidence in results from intra-patient control



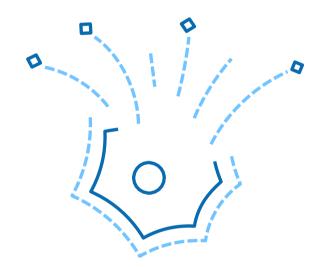
Notable response in patients that converted to wet AMD



Phase III design finalized

Paroxysmal Nocturnal Hemoglobinuria (PNH) is a rare, life-threatening blood disease

PNH characterized by uncontrolled hemolysis



Intravascular hemolysis

Red blood cell rupture in the circulation



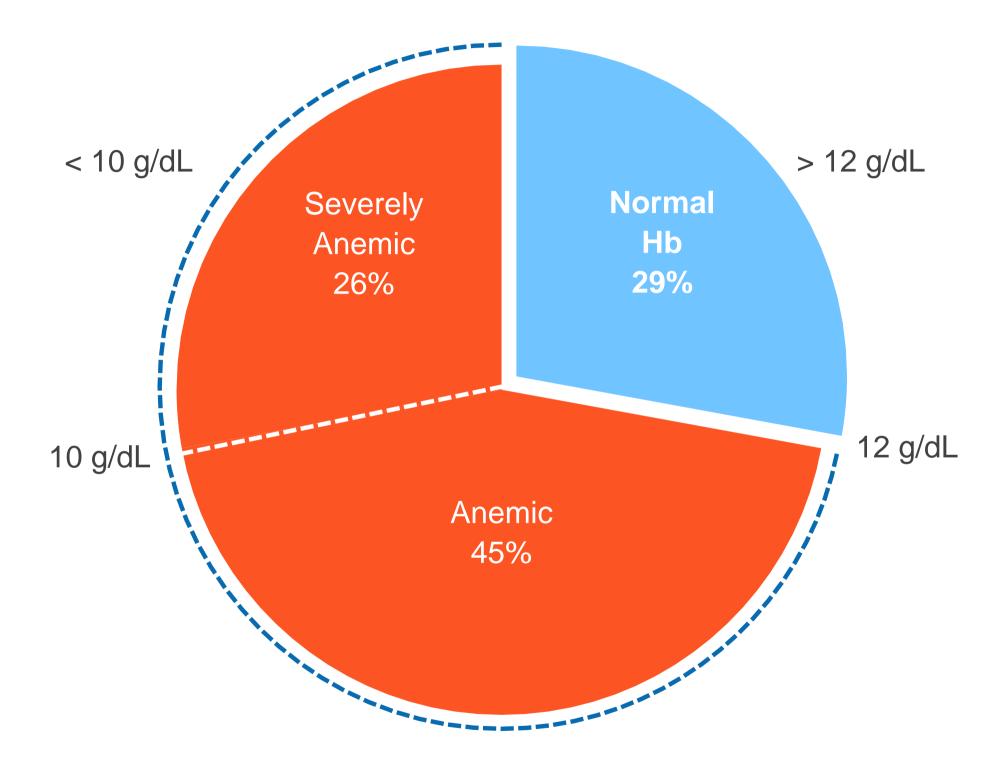
Extravascular hemolysis

Red blood cell destruction by macrophages in spleen and liver



- 5,000 patients in US.
- 35% 5-year mortality if untreated (thrombosis, severe anemia).
- Alexion's Soliris® (eculizumab) is only approved therapy.
- Treats only intravascular hemolysis.
- Approximate cost \$500,000 annually.

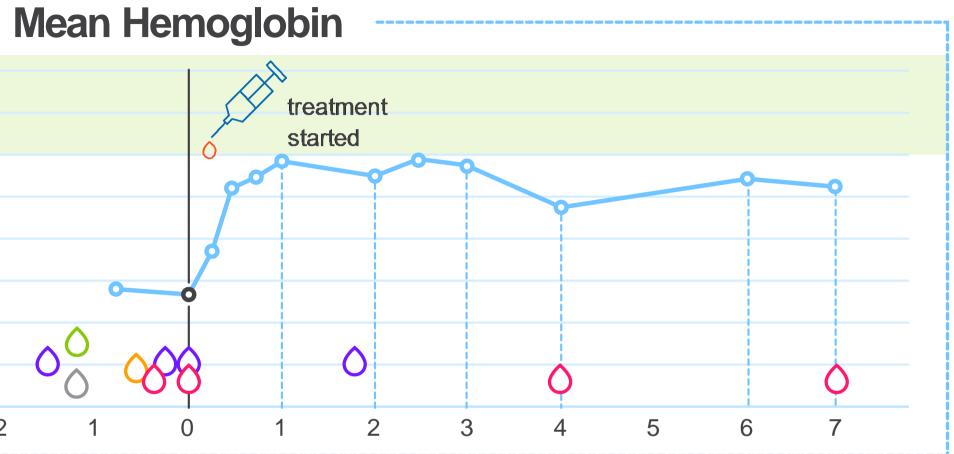
~70% of eculizumab-treated patients remain anemic due to extravascular hemolysis

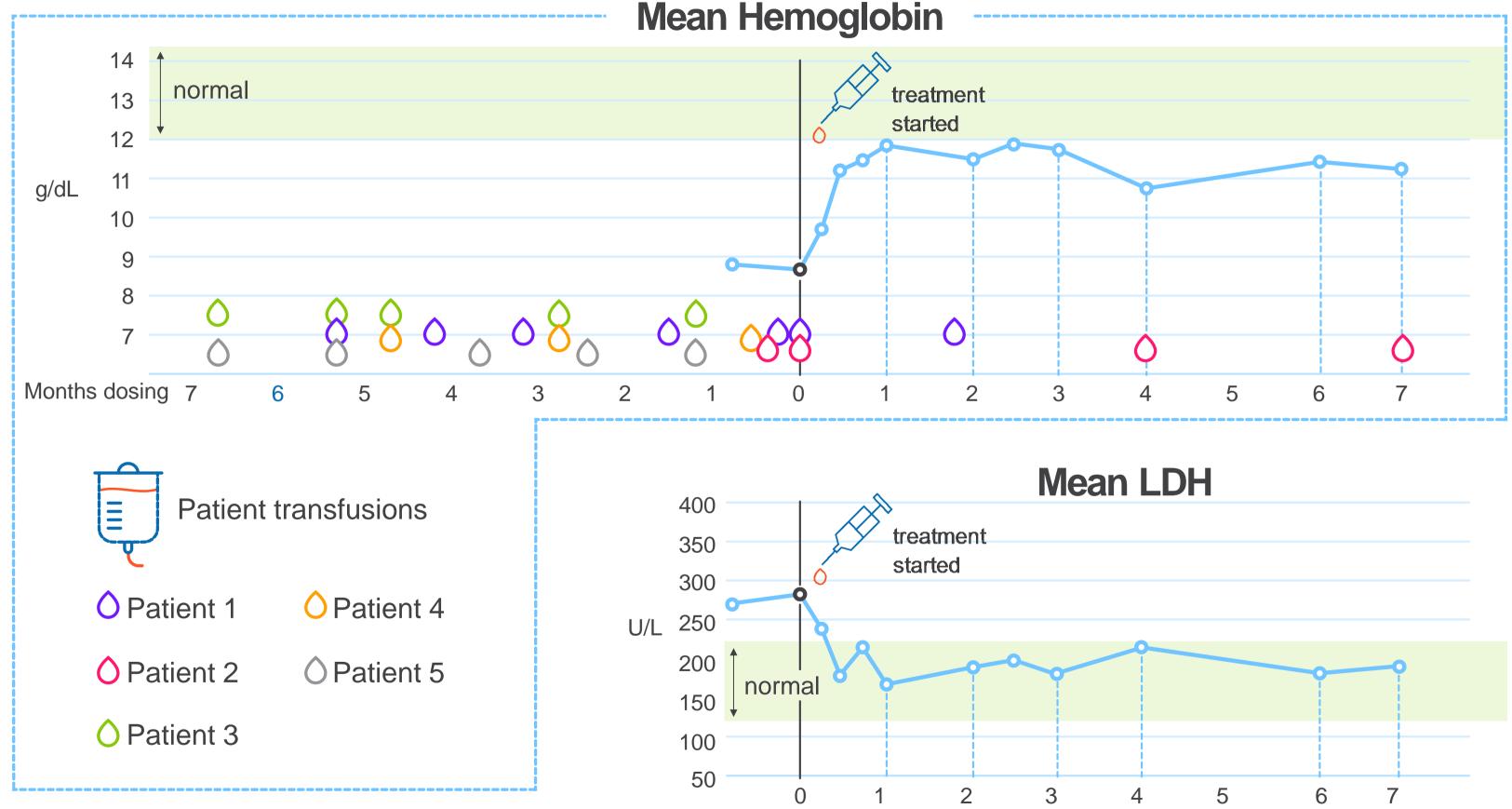


Hemoglobin (g/dL) in 141 random PNH Patients on Soliris®



APL-2 shows potential to improve eculizumab outcomes as add-on therapy in PNH – 270 mg/d, N=6

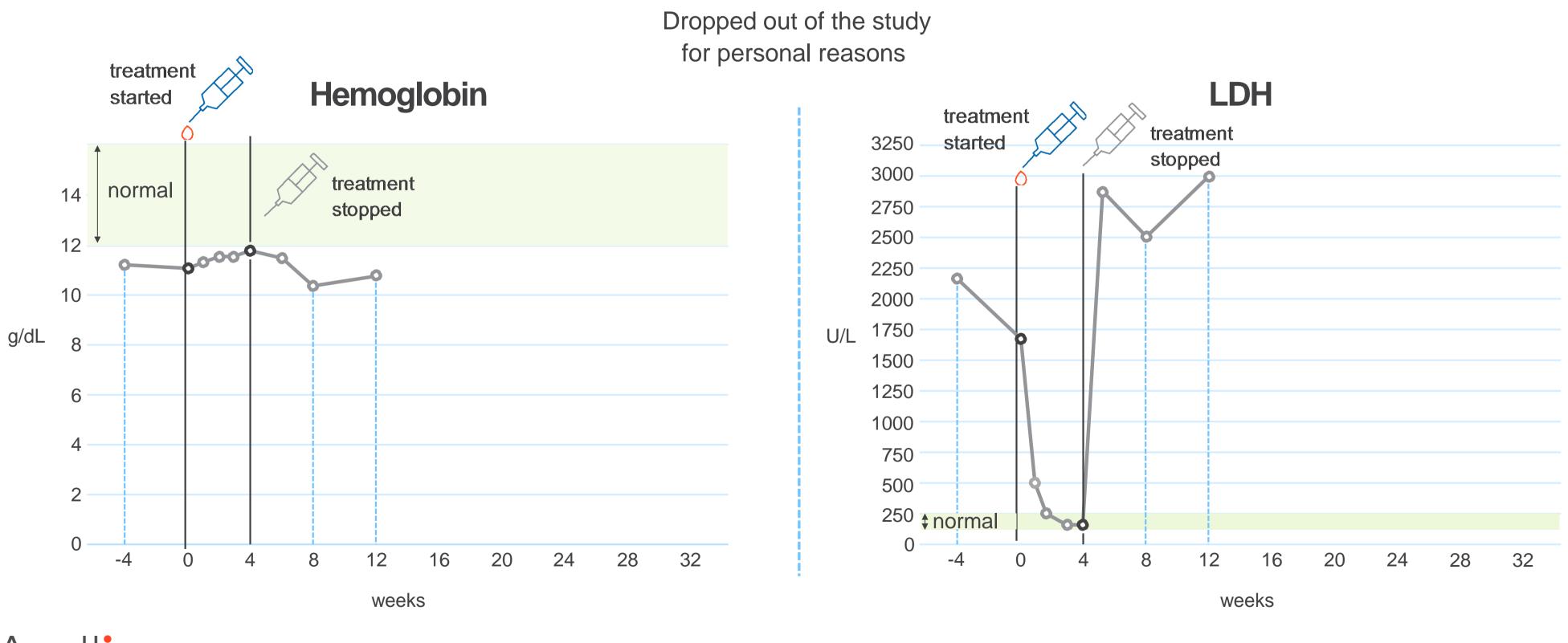






APL-2 monotherapy - 270 mg/d – Patient 1 of 3



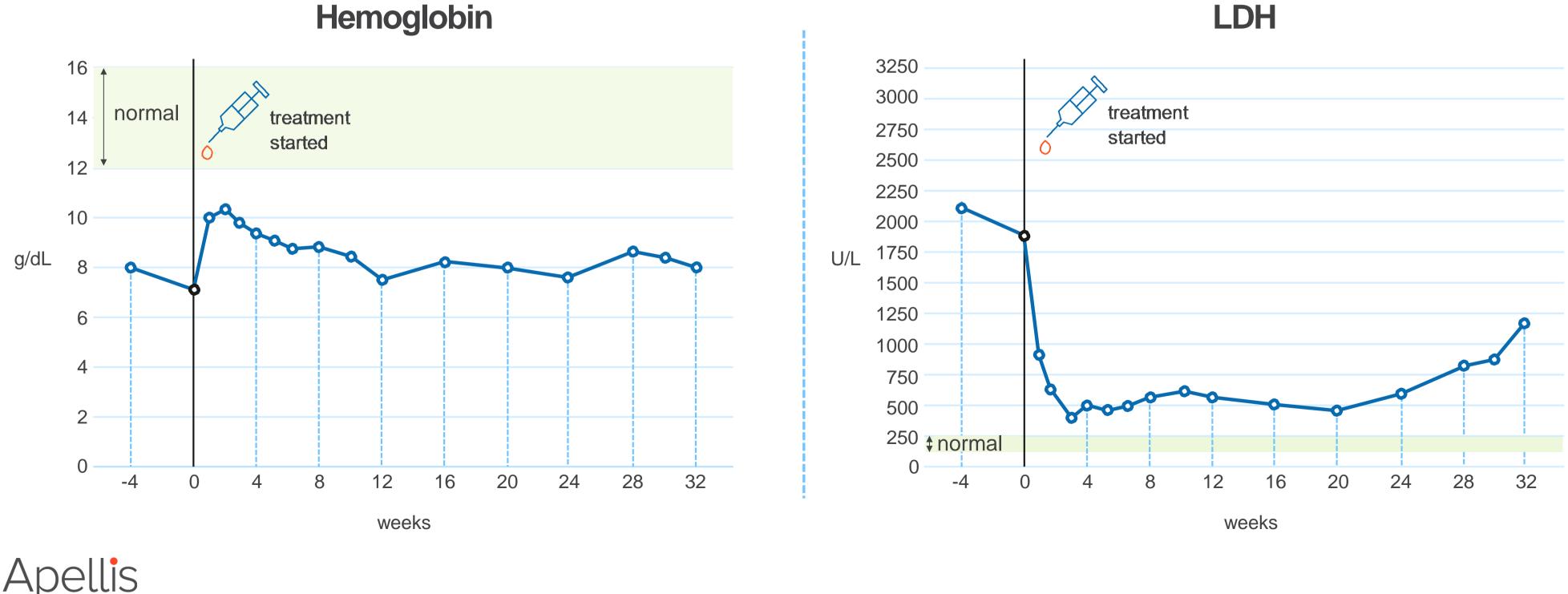


pellis

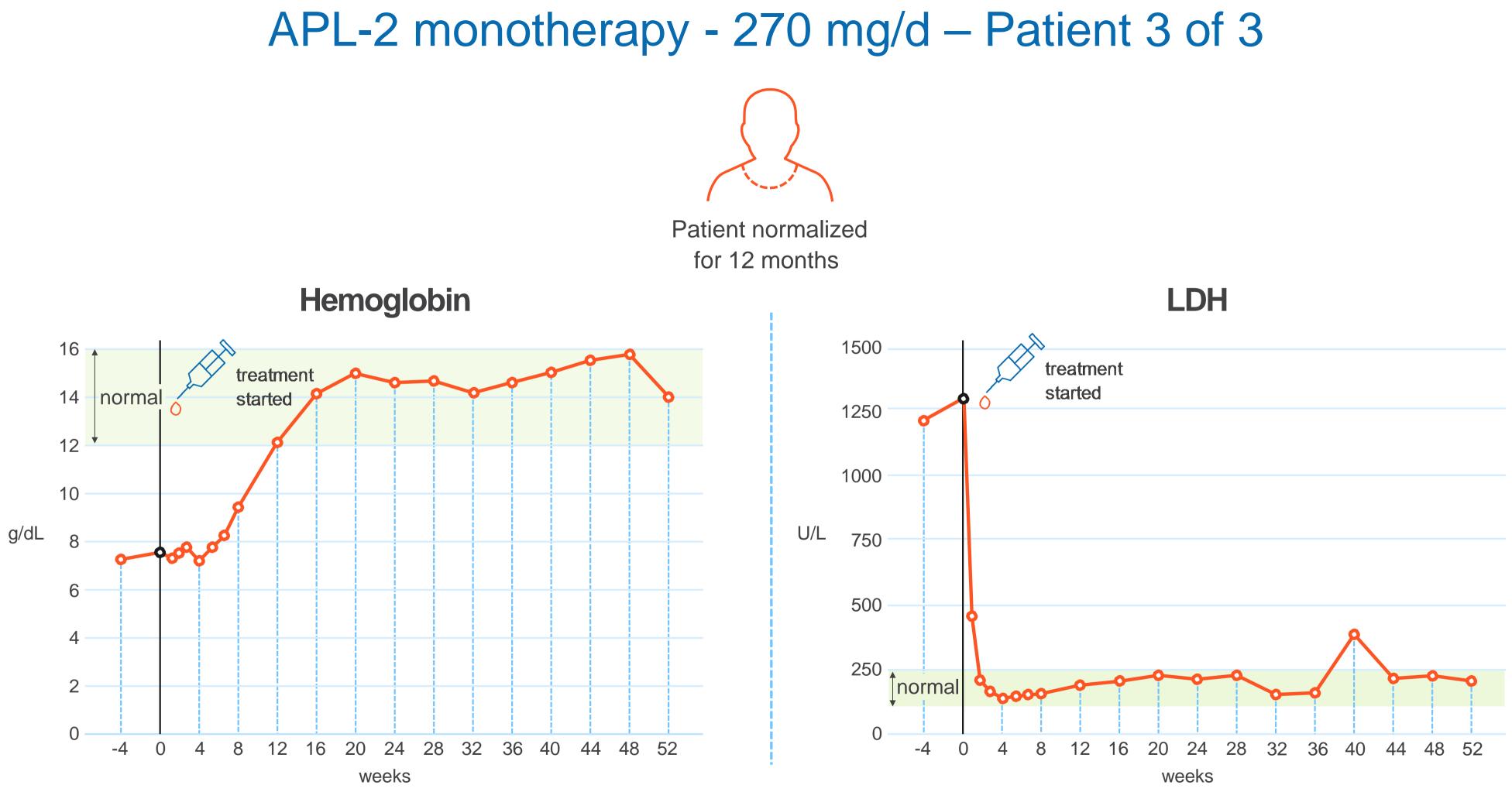
APL-2 monotherapy - 270 mg/d – Patient 2 of 3



This patient had ovarian cancer





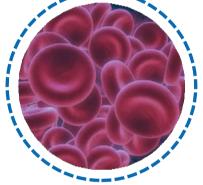


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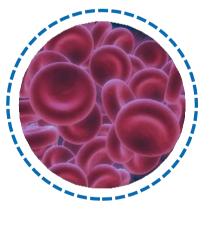
Key catalysts for 2018



18 month safety & efficacy data.



PNH: Phase 1b Soliris weaning & monotherapy expansion.



AIHA: Phase 2 POC monotherapy data.

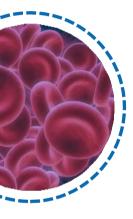
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H1





CDN: Phase 2 POC monotherapy data.



PNH:

Start of Phase 3 program.



GA: Start of Phase 3 program.

H2



Thank you

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