Apellis

Cowen Conference



Forward looking statements

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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. 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.

Apellis

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



What we do





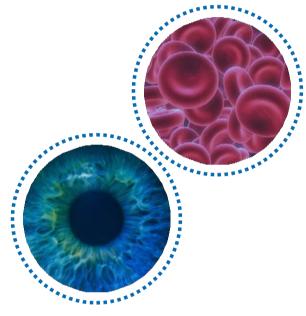


Pioneers in innate immunity & complement immunology

By regulating its core component C3

Value & patient outcomes at the center of our programs





Initially focused on AMD & PNH



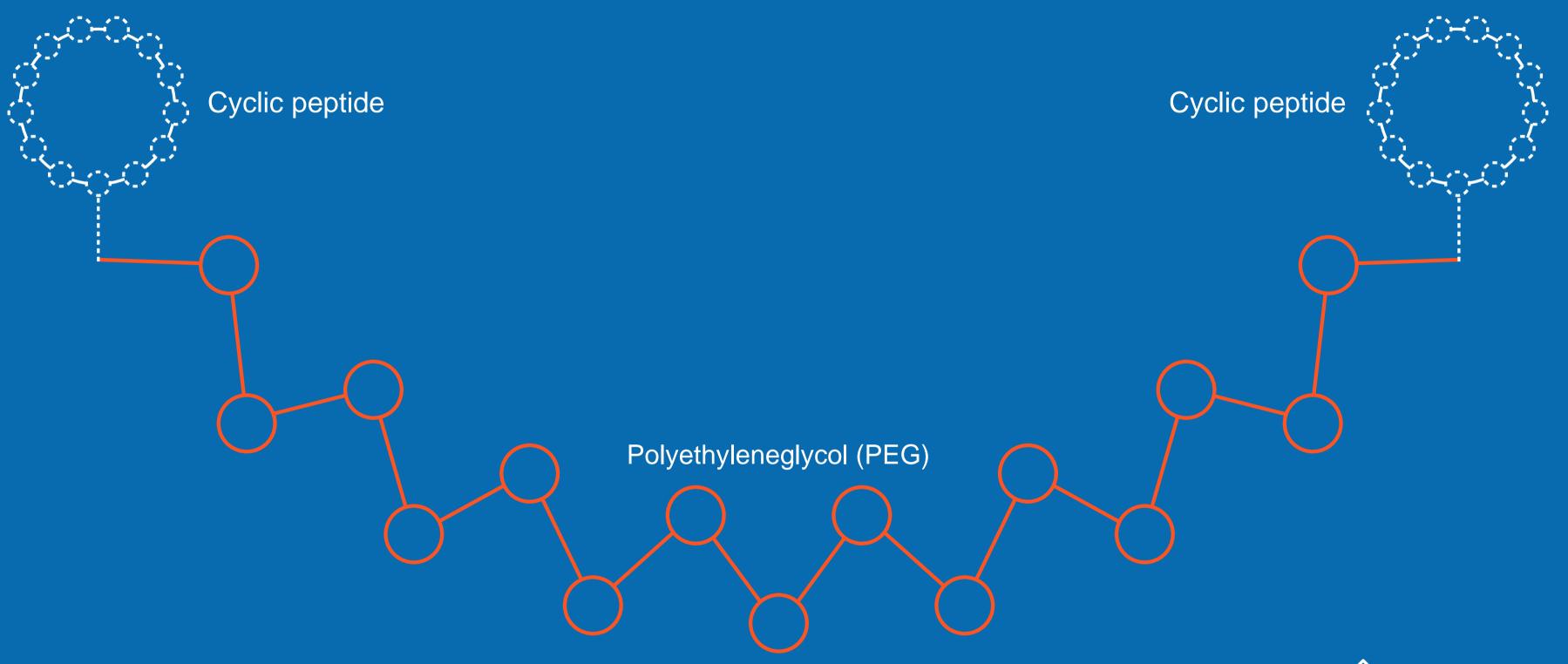
Broad potential in other immune conditions

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					



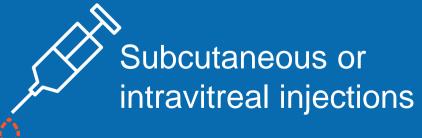
Apellis lead molecule: APL-2



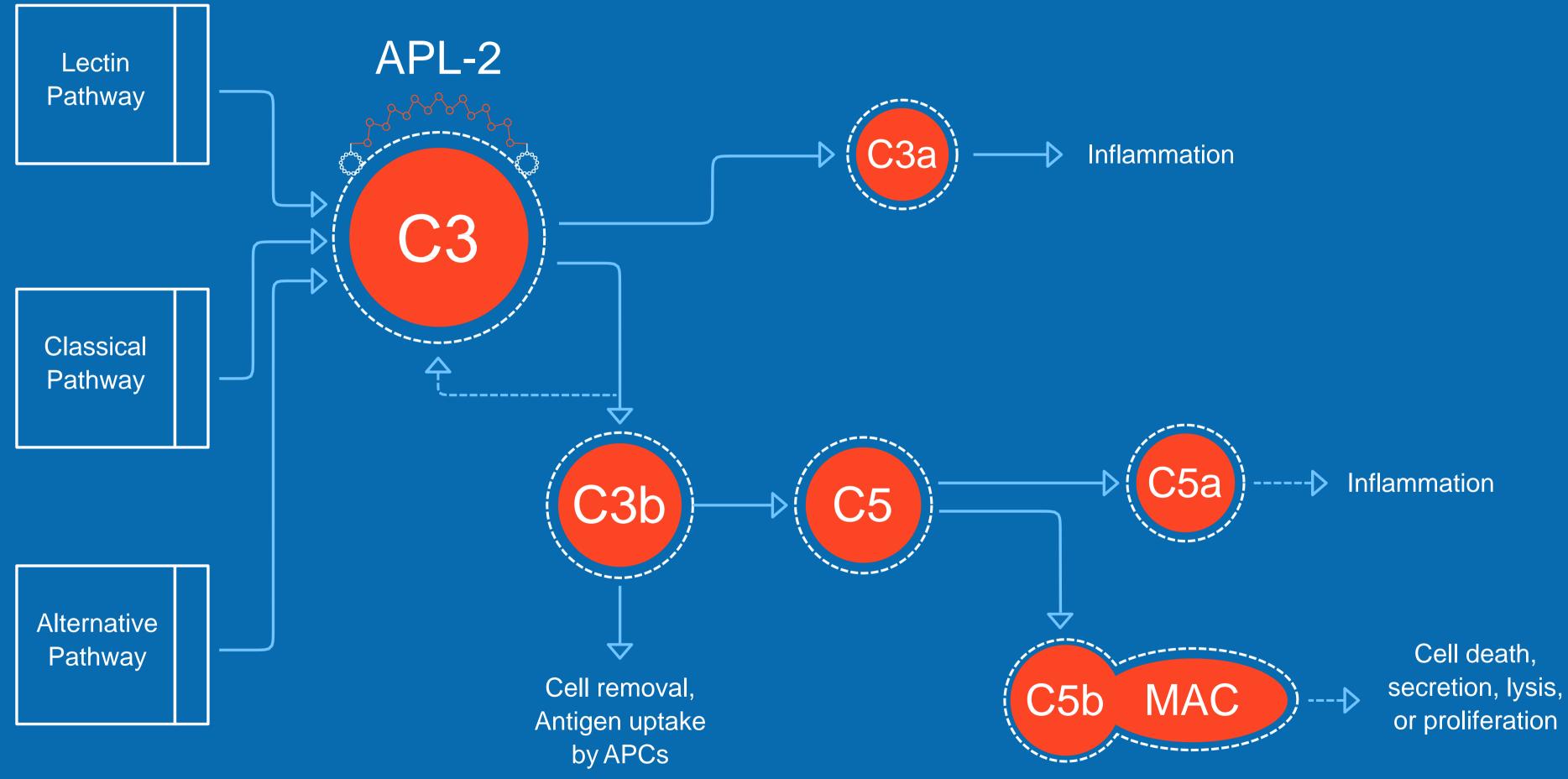
Peptides of the APL-2 family bind to a pocket of C3 and inhibit activation*

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



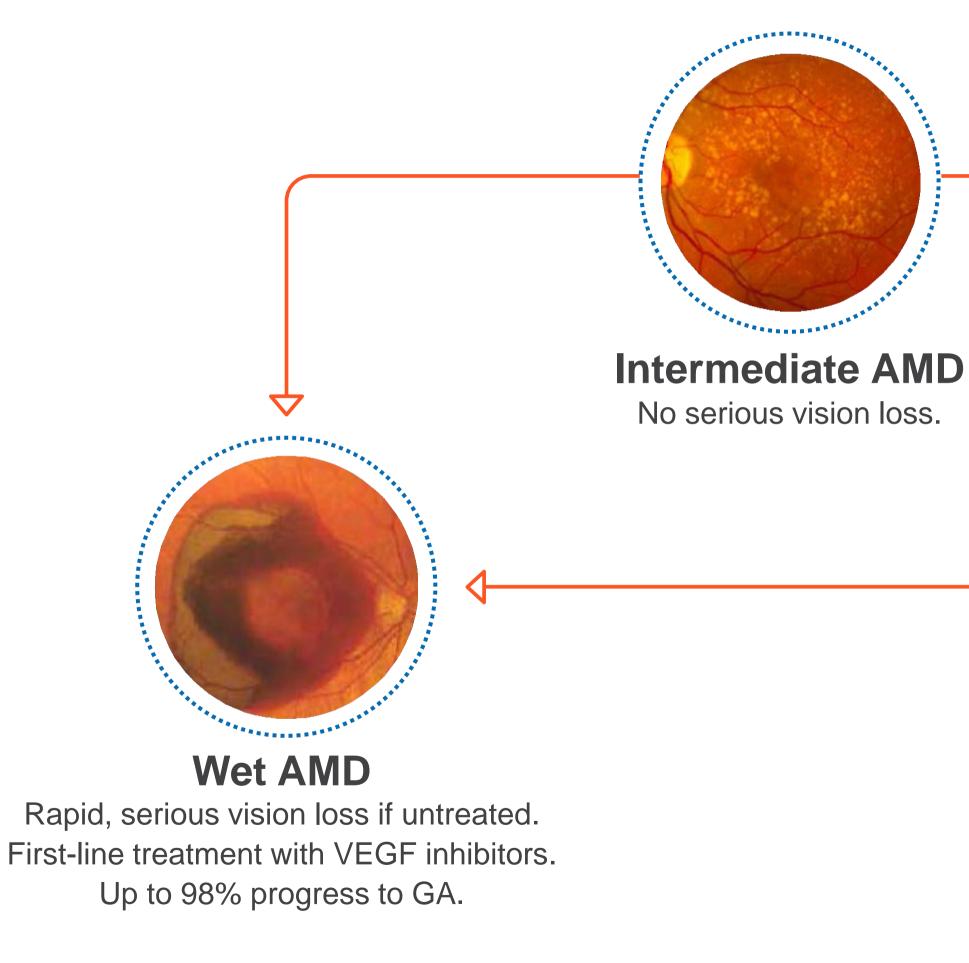


Central inhibition of complement

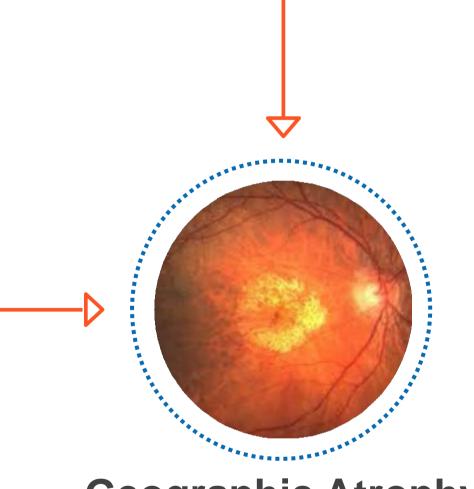




Geographic Atrophy - the leading cause of blindness



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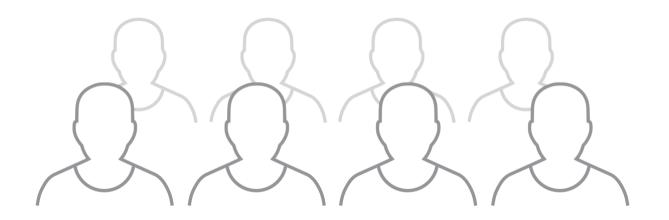
Geographic Atrophy

Risk of blindness when central vision affected ~1M patients in US alone. No approved therapies.

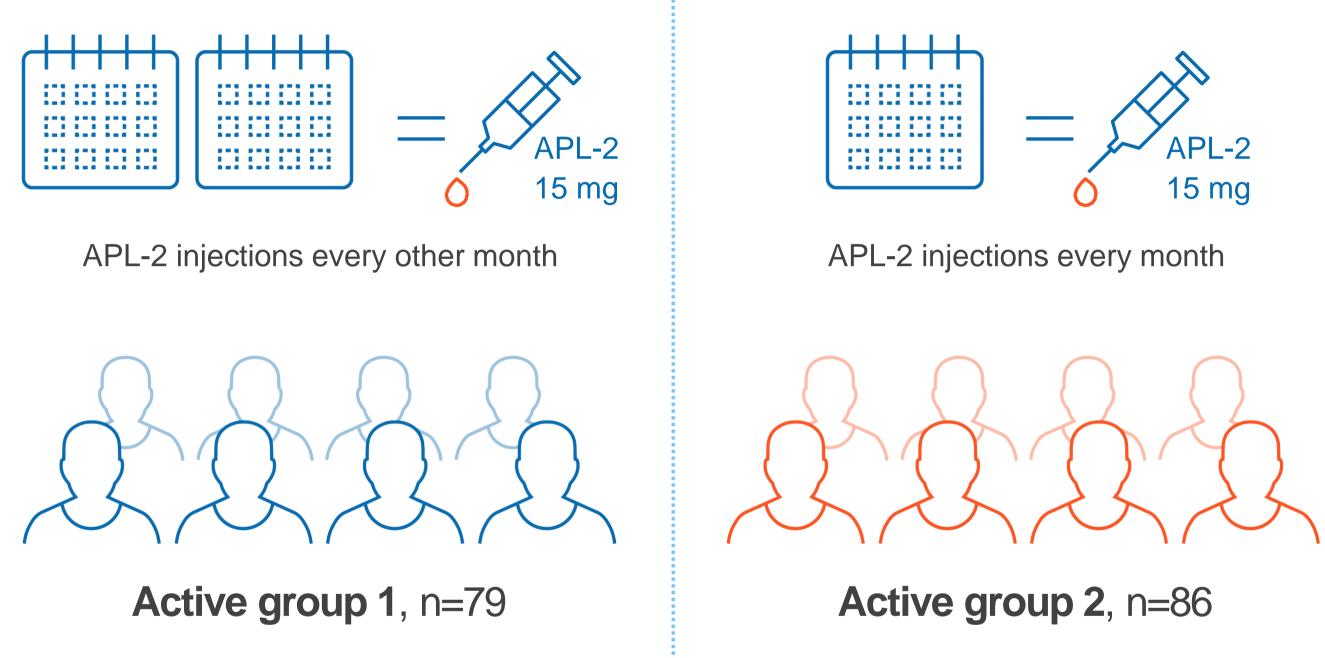
FILLY - Phase 2 study of APL-2 in Geographic Atrophy

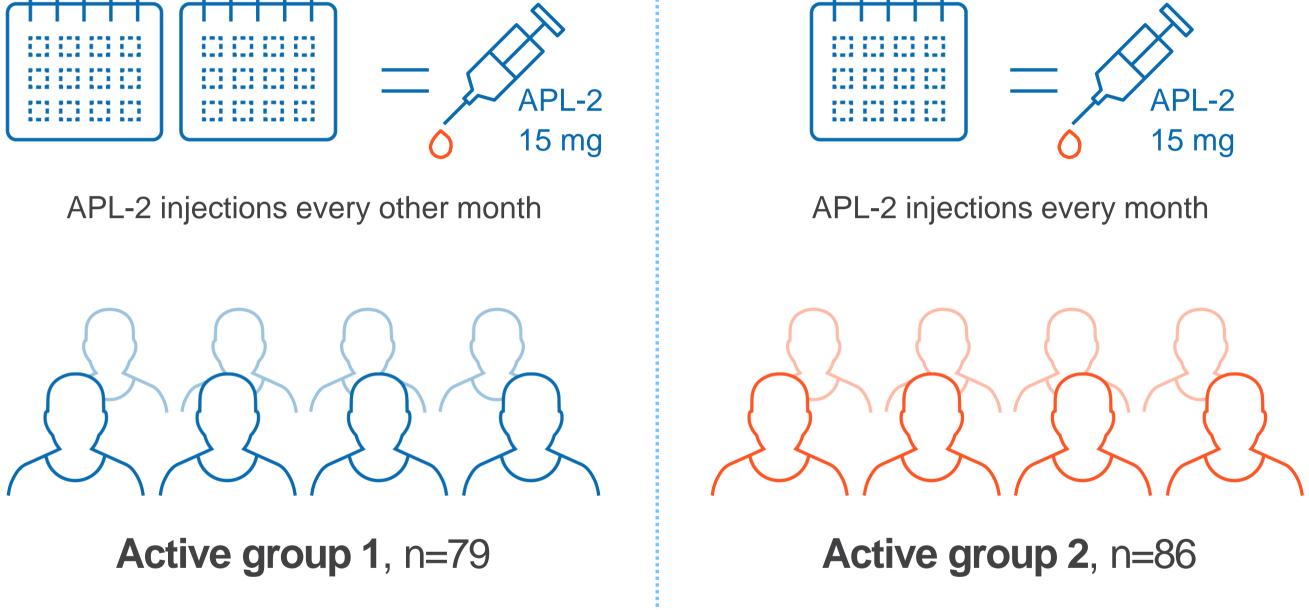


Sham injections



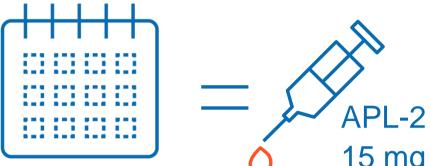
Sham group, n=81



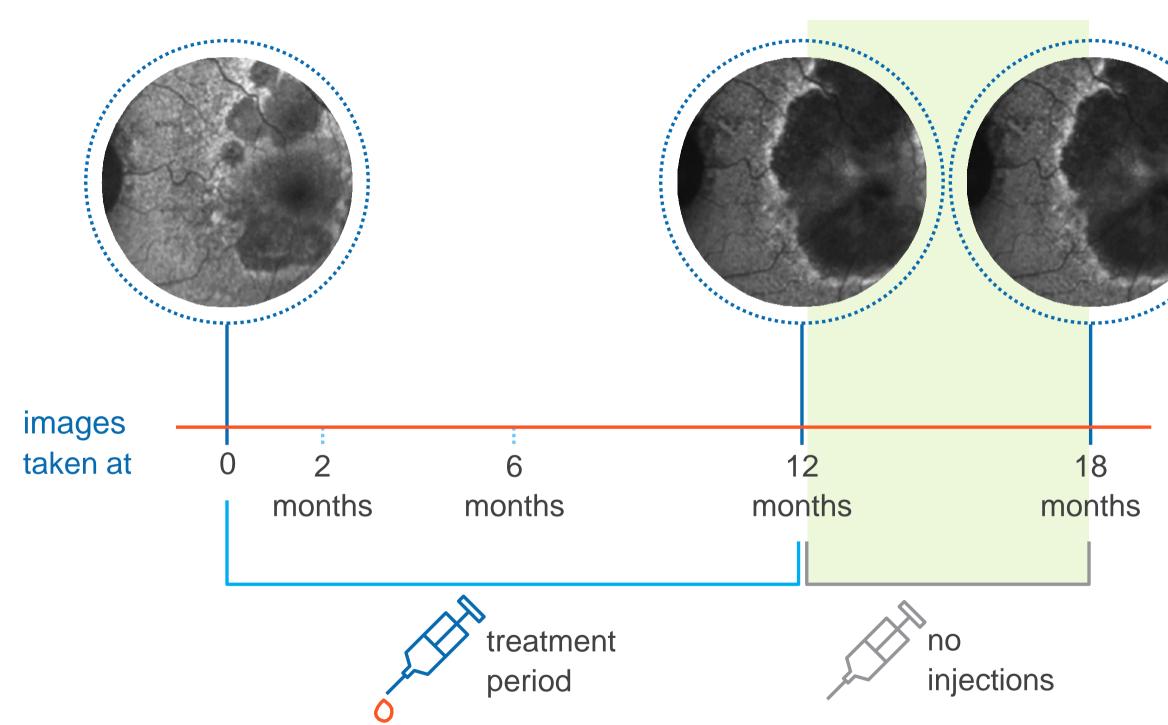


Phase 3 design finalized post FDA discussion

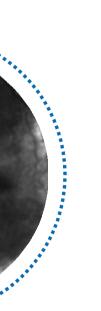




FILLY – timeline and endpoints







Primary efficacy endpoint is the primary registration endpoint

Change in geographic atrophy (GA) lesion size from baseline to month 12.

Primary safety endpoint

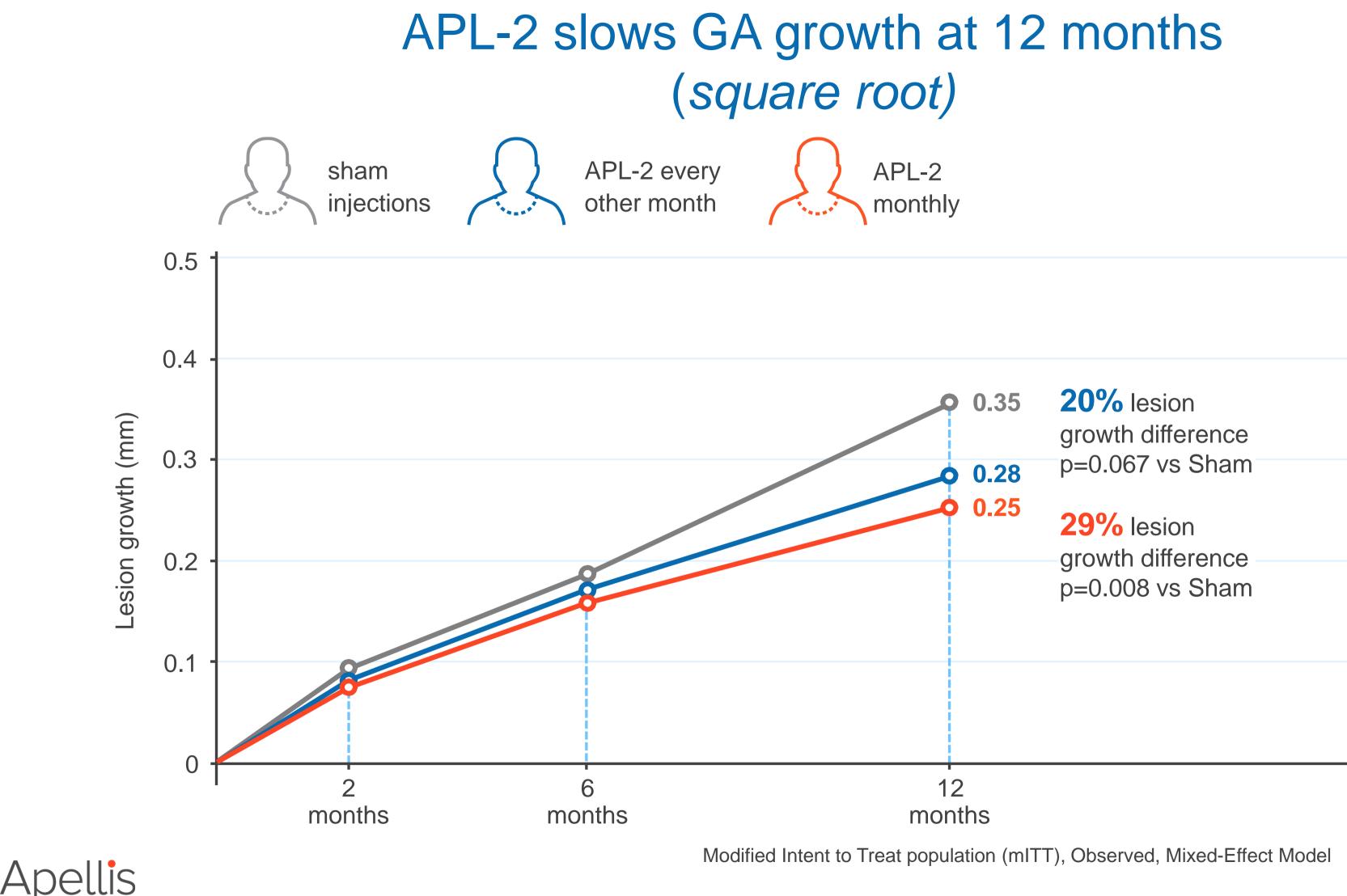
Number and severity of local and systemic treatment emergent adverse events (TEAEs).

Baseline characteristics

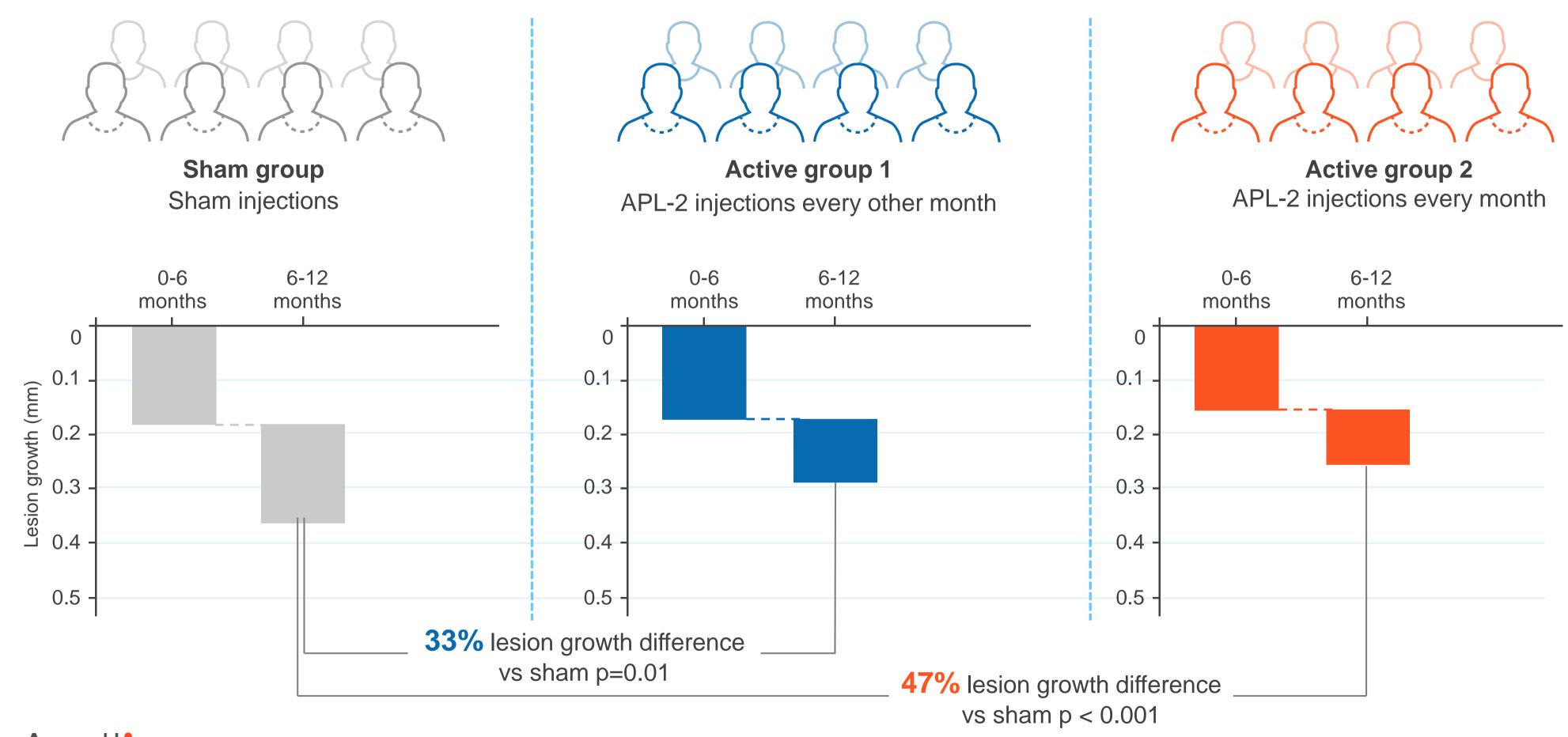
	Sham injections N=81	APL-2 every other month N=79	APL-2 monthly N=86
Bilateral GA, n (%)	72 (90.0%)	64 (82.1%)	71 (85.5%)
History of CNV in Fellow Eye, n (%)	29 (35.8%)	28 (35.4%)	36 (41.9%)
GA lesion size, mean, mm ² (SD)	8.2 (4.1)	8.9 (4.5)	8.0 (3.8)
BCVA score, mean letters (SD)	59.8 (17.2)	58.4 (16.0)	59.8 (15.7)
BCVA score (Snellen equivalent)	20/63	20/80	20/63
LL-BCVA score, mean letters (SD)	33.6 (17.8)	31.4 (17.1)	36.3 (16.6)

Groups were well balanced as to age, gender and race



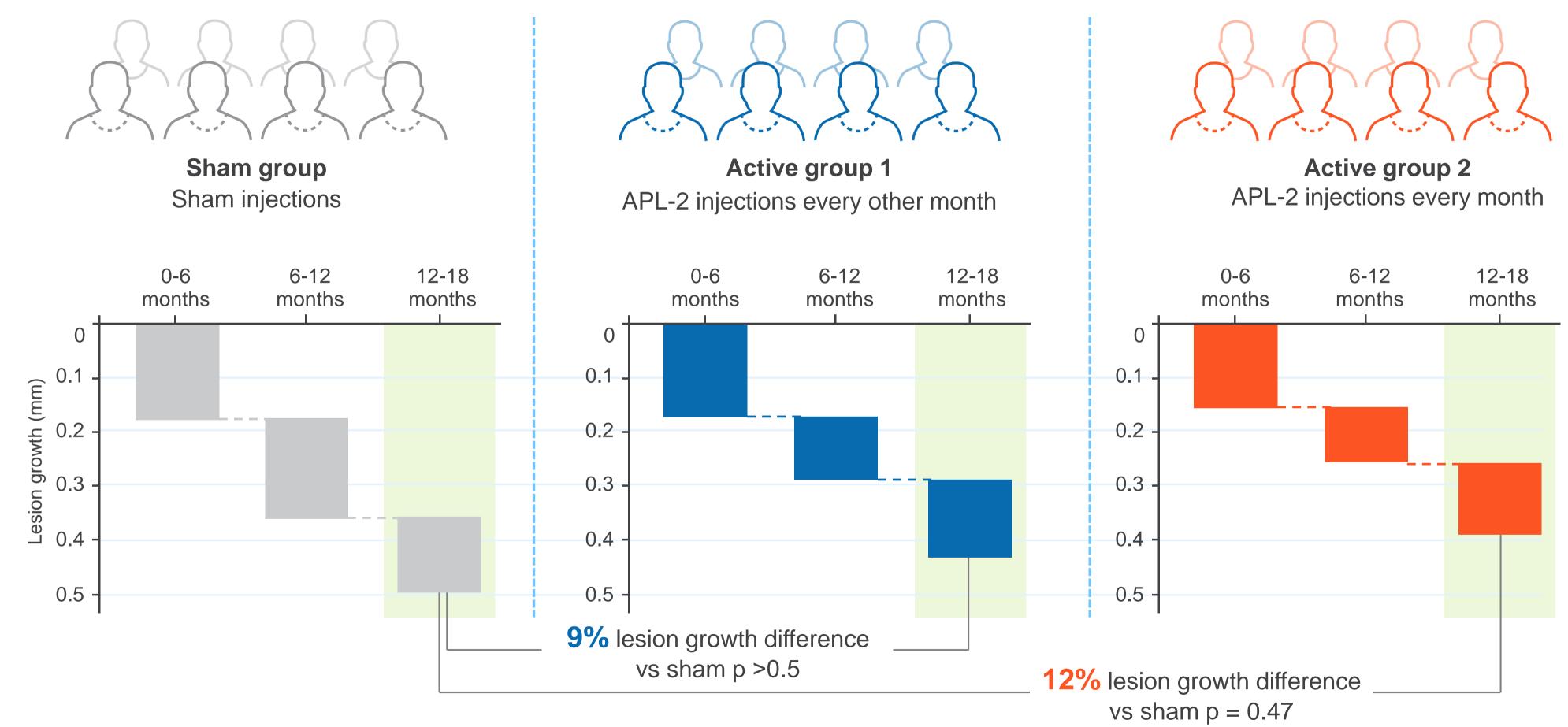


Lesion growth by six-month periods (square root)



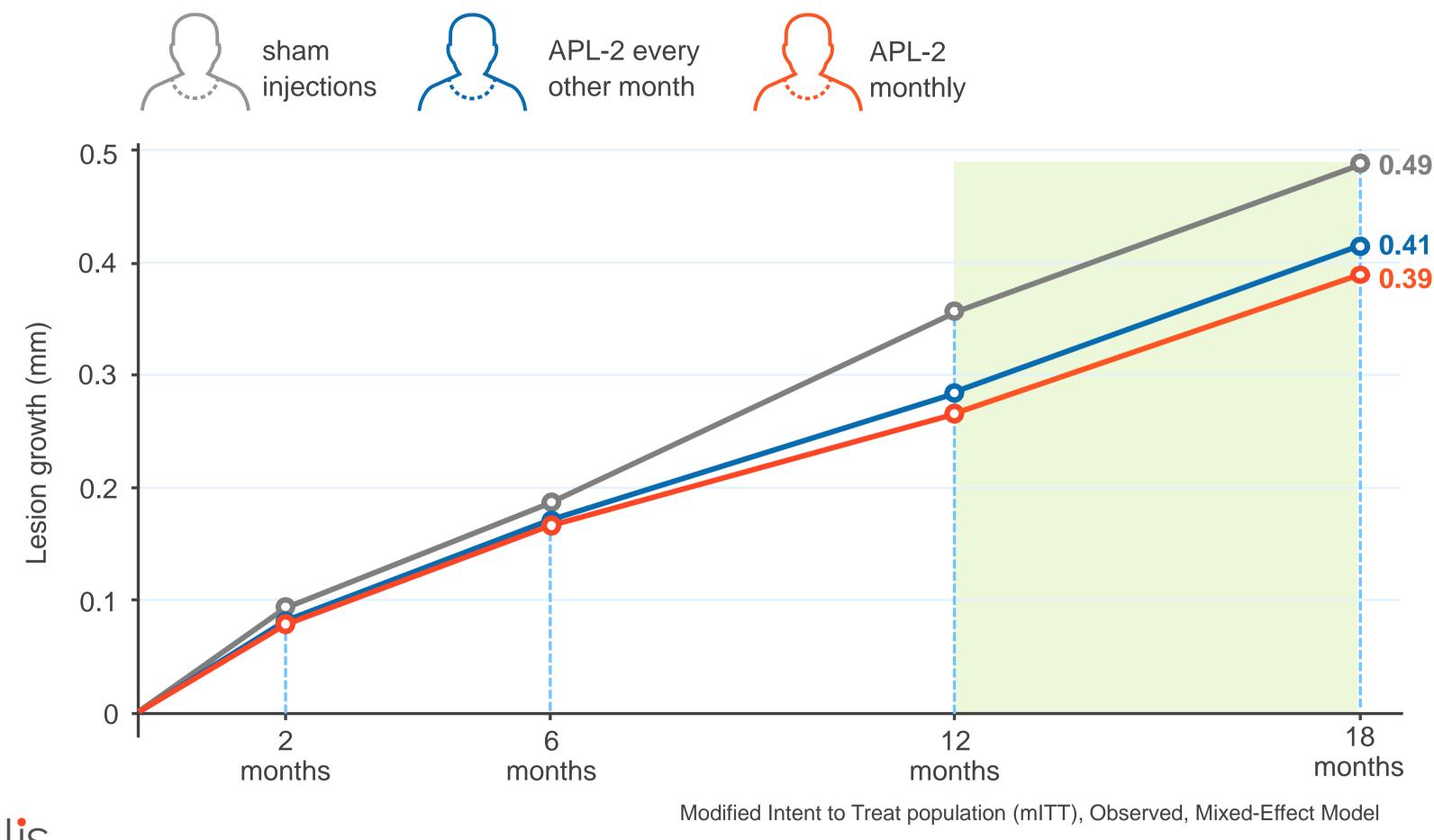
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Lesion growth by six-month periods (square root)



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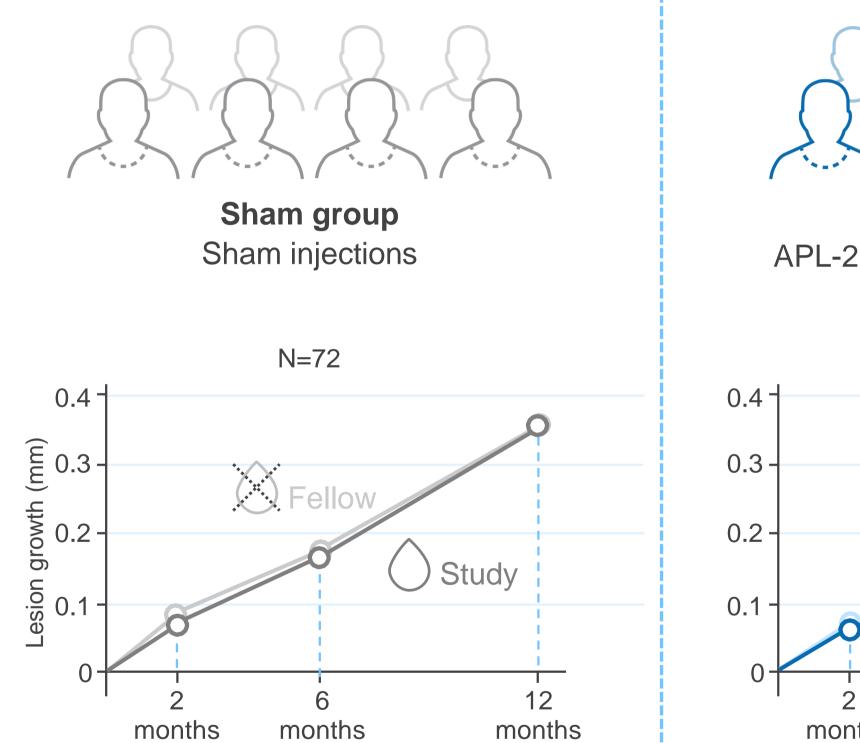
After cessation of treatment at 12 months, GA growth resumes but treatment effect is maintained through 18 months (square root)



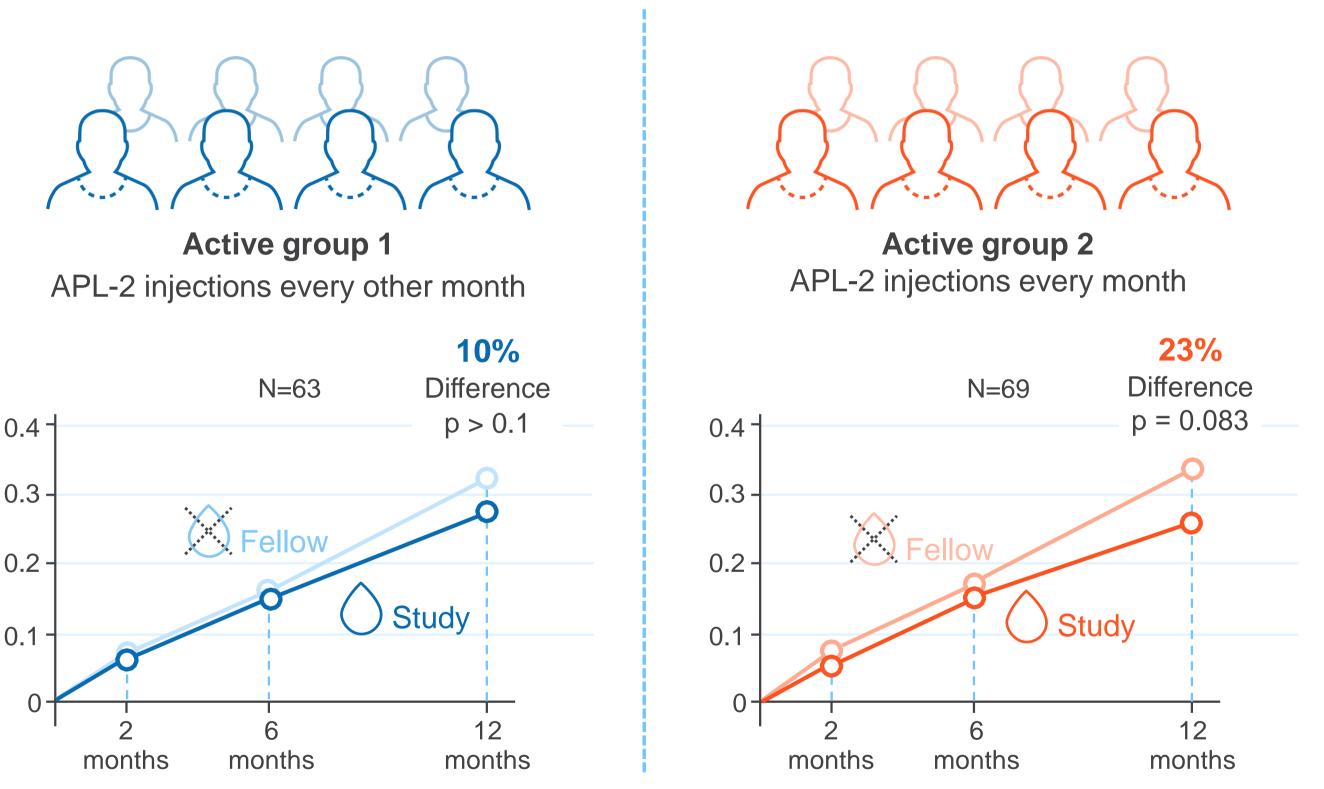
16% lesion growth difference p=0.097 vs Sham

20% lesion growth difference p=0.044 vs Sham

GA growth comparison: fellow eye vs study eye



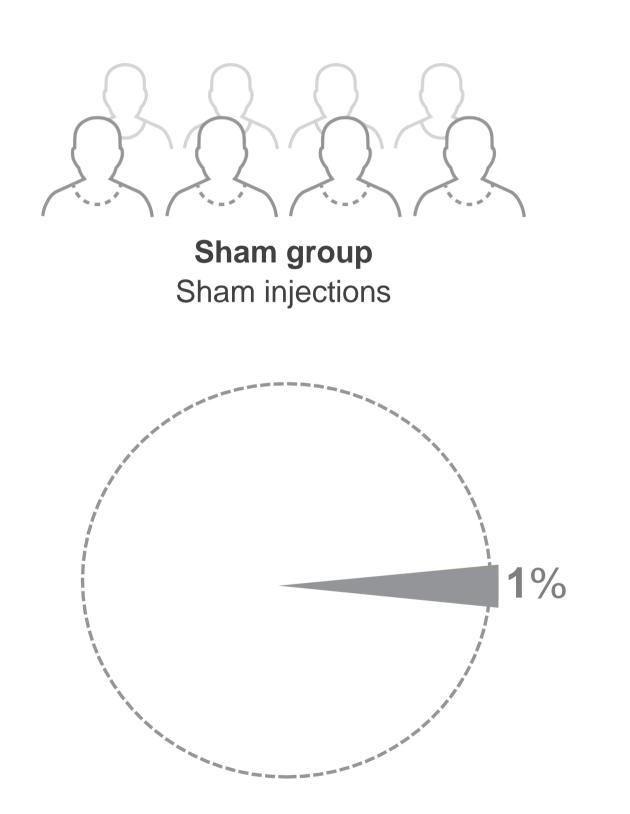
Active group 1 N=63

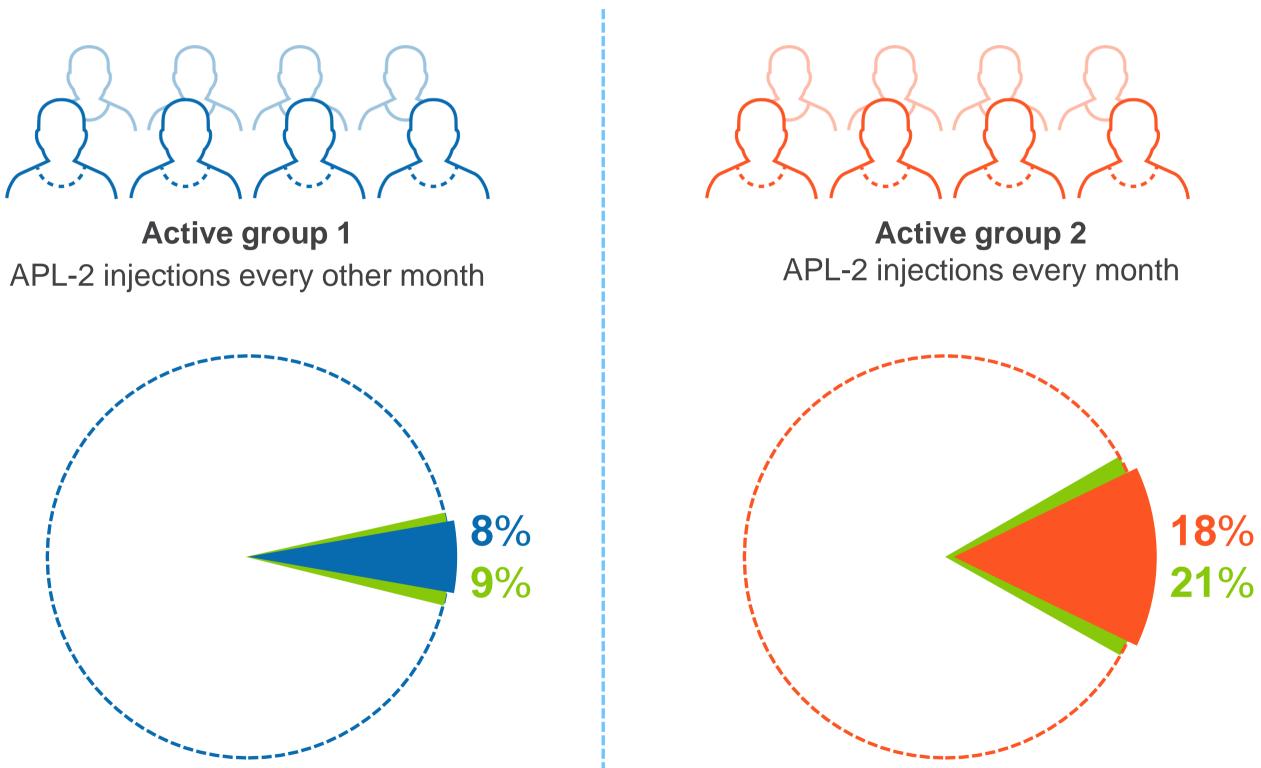


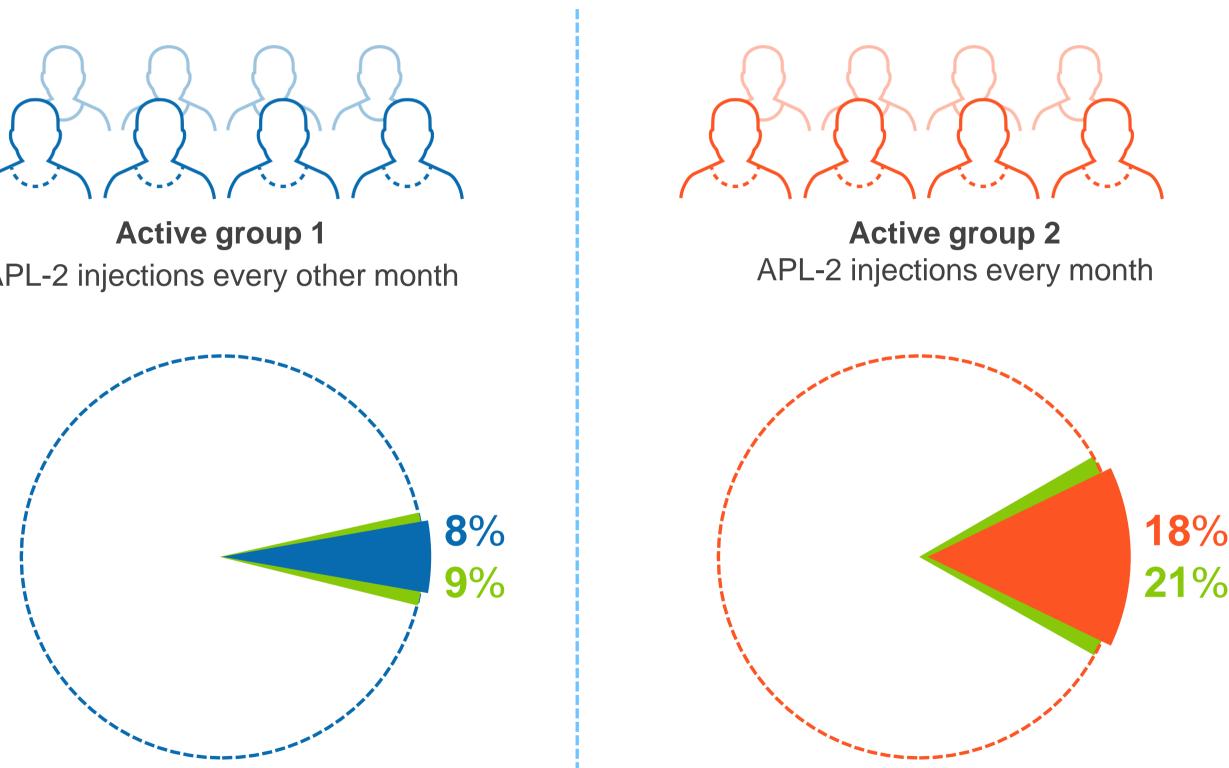
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Includes patients from the Bilateral GA Population

New onset wet AMD





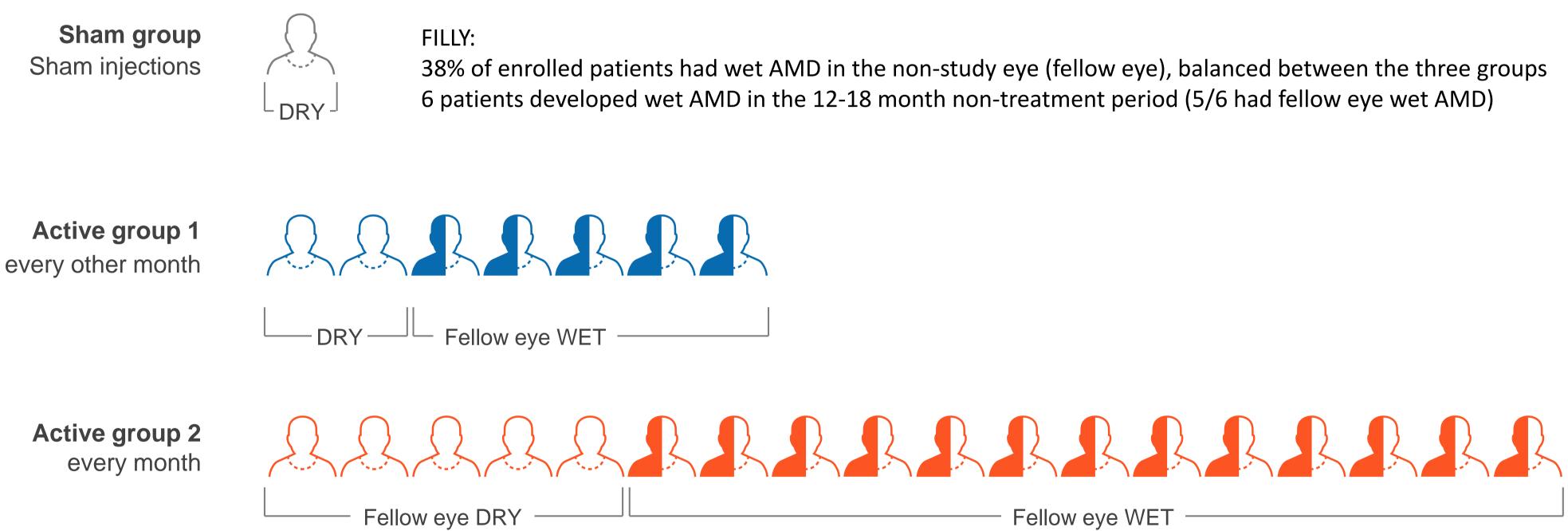






18-month outcomes

New onset wet AMD – 18 months



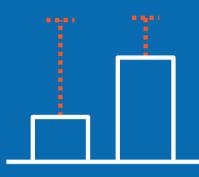
"Expected" based on natural history ~2%/yr for Dry fellow eye patients (Sunness et al 1999) and ~10%/yr for Wet fellow eye patients (Marques et al. 2013)



FILLY phase 2 trial



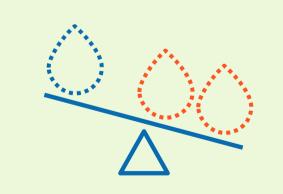
Preventing complement activation by blocking C3



Statistically significant data in largest Phase 2 in GA (n=246)



Results correlated to treatment frequency with increasing effect size over time

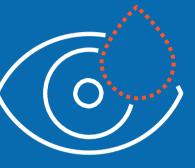


Risk benefit profile confirmed at 18 months supporting decision to move to Phase 3





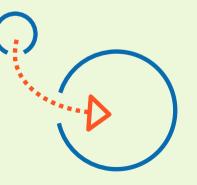
No specific genotype driving results



Further confidence in results from intra-patient control



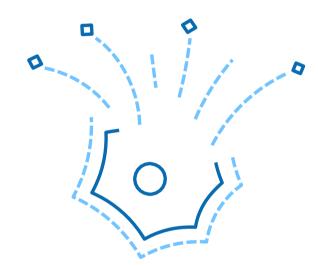
Phase 3 design finalized



Upon discontinuation of APL-2, treatment effect declines

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



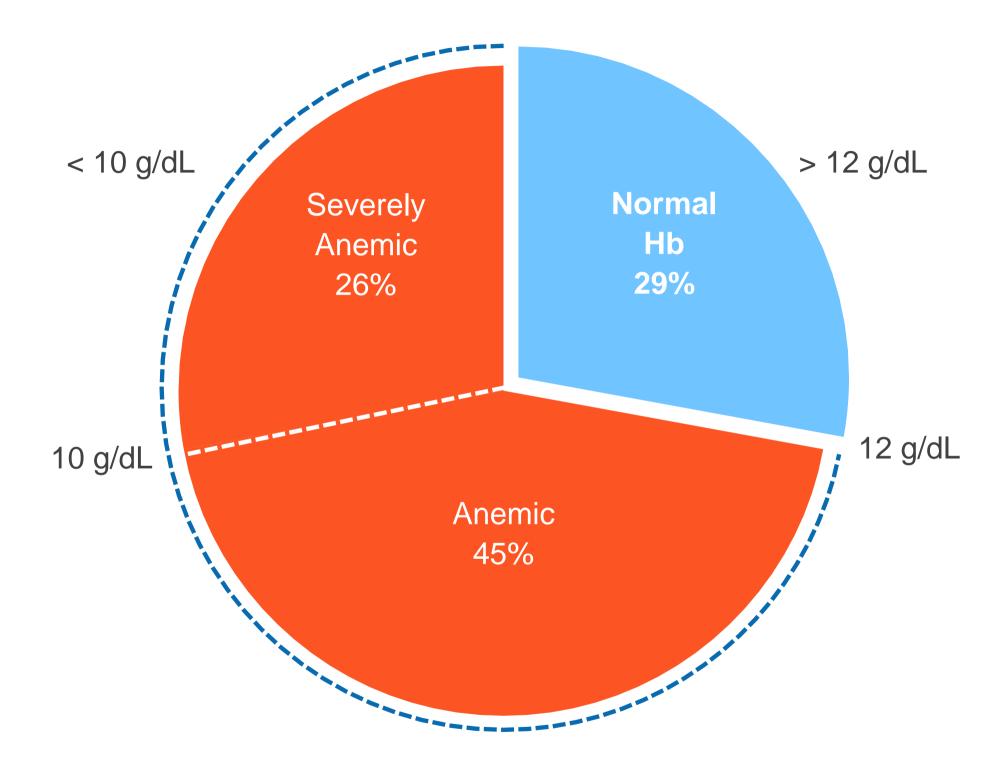
Extravascular hemolysis

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.

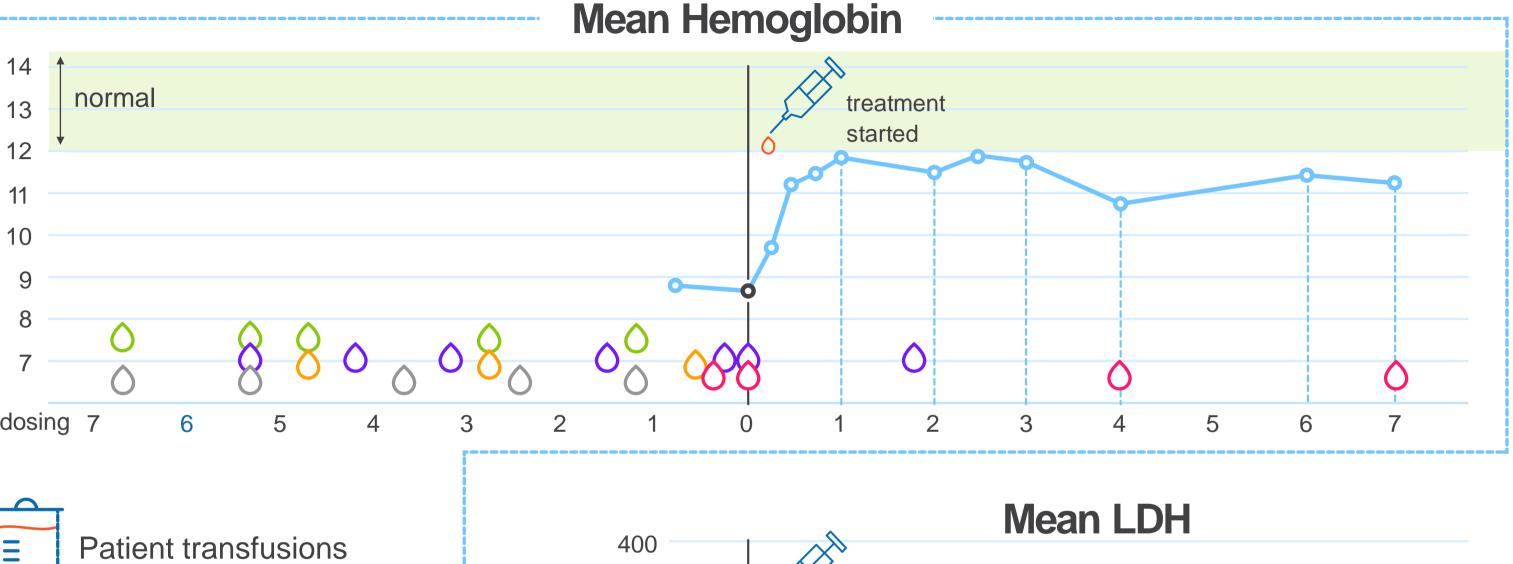
~70% of eculizumab-treated patients remain anemic due to extravascular hemolysis

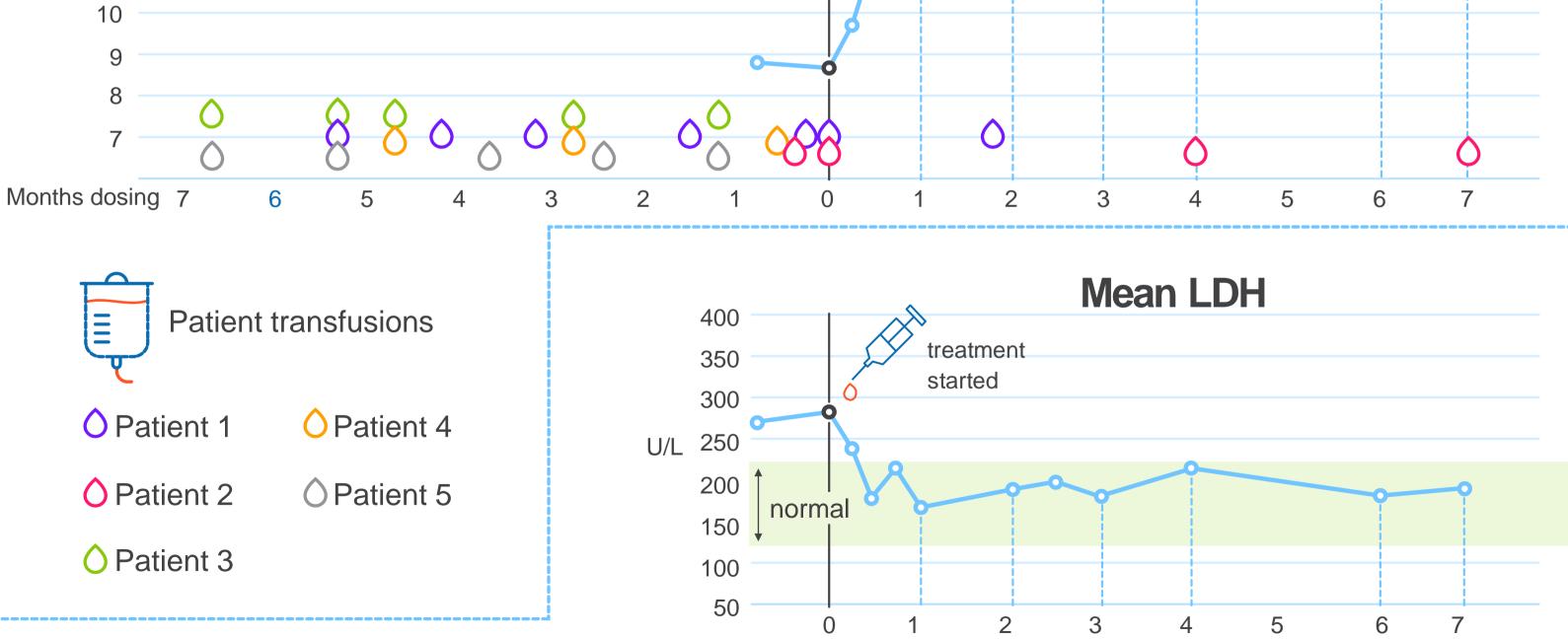


Hemoglobin (g/dL) in 141 random PNH Patients on Soliris®



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



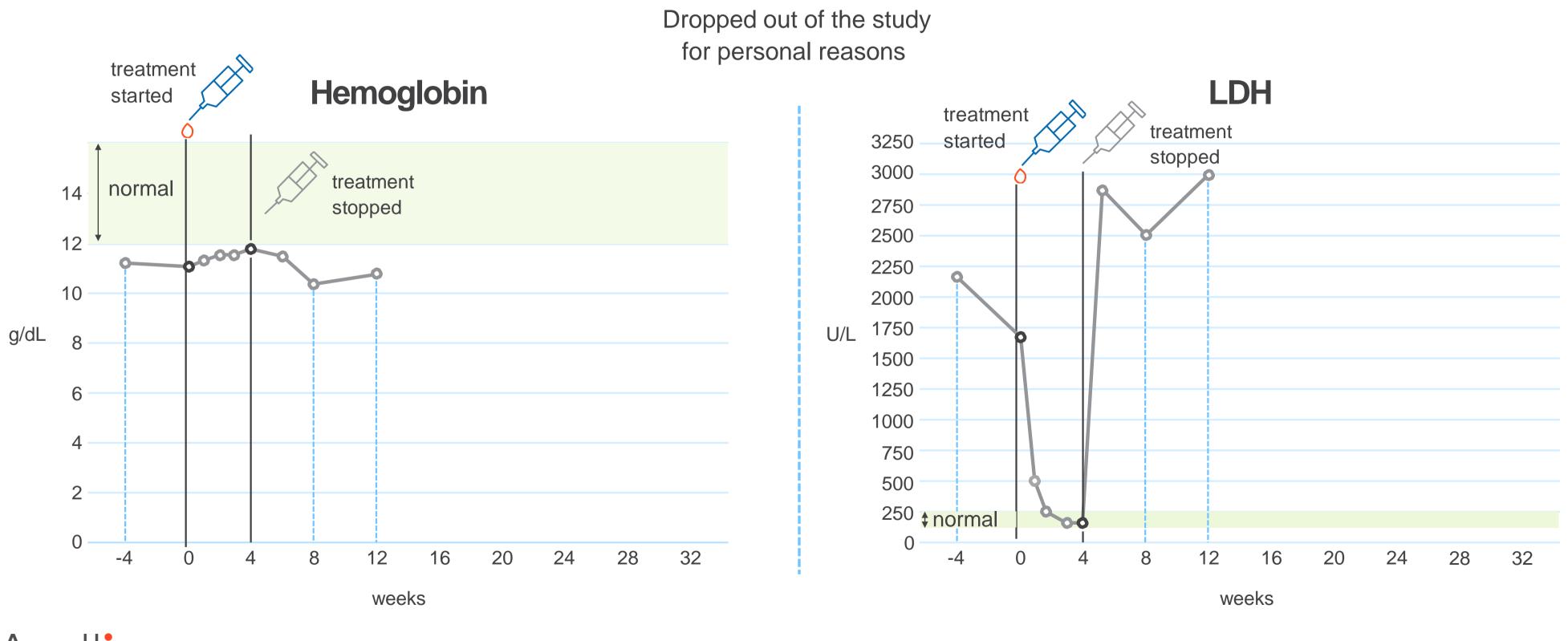




g/dL

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



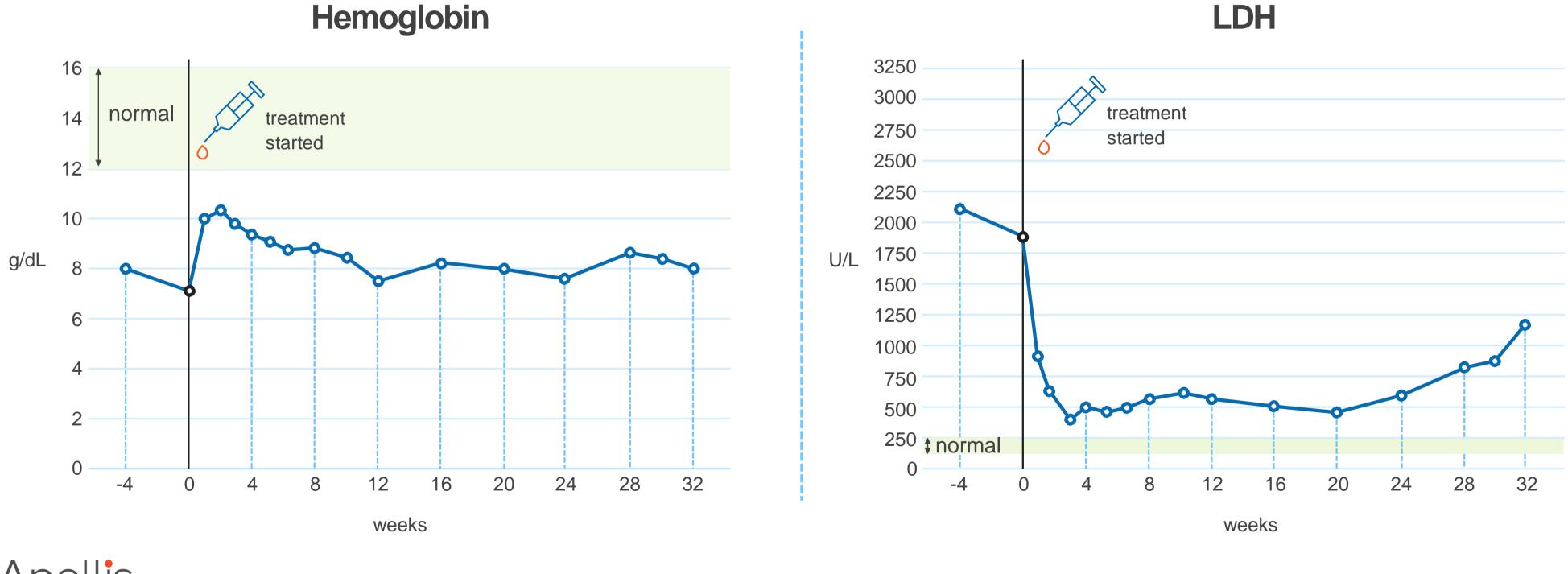


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APL-2 monotherapy - 270 mg/d – Patient 2 of 3

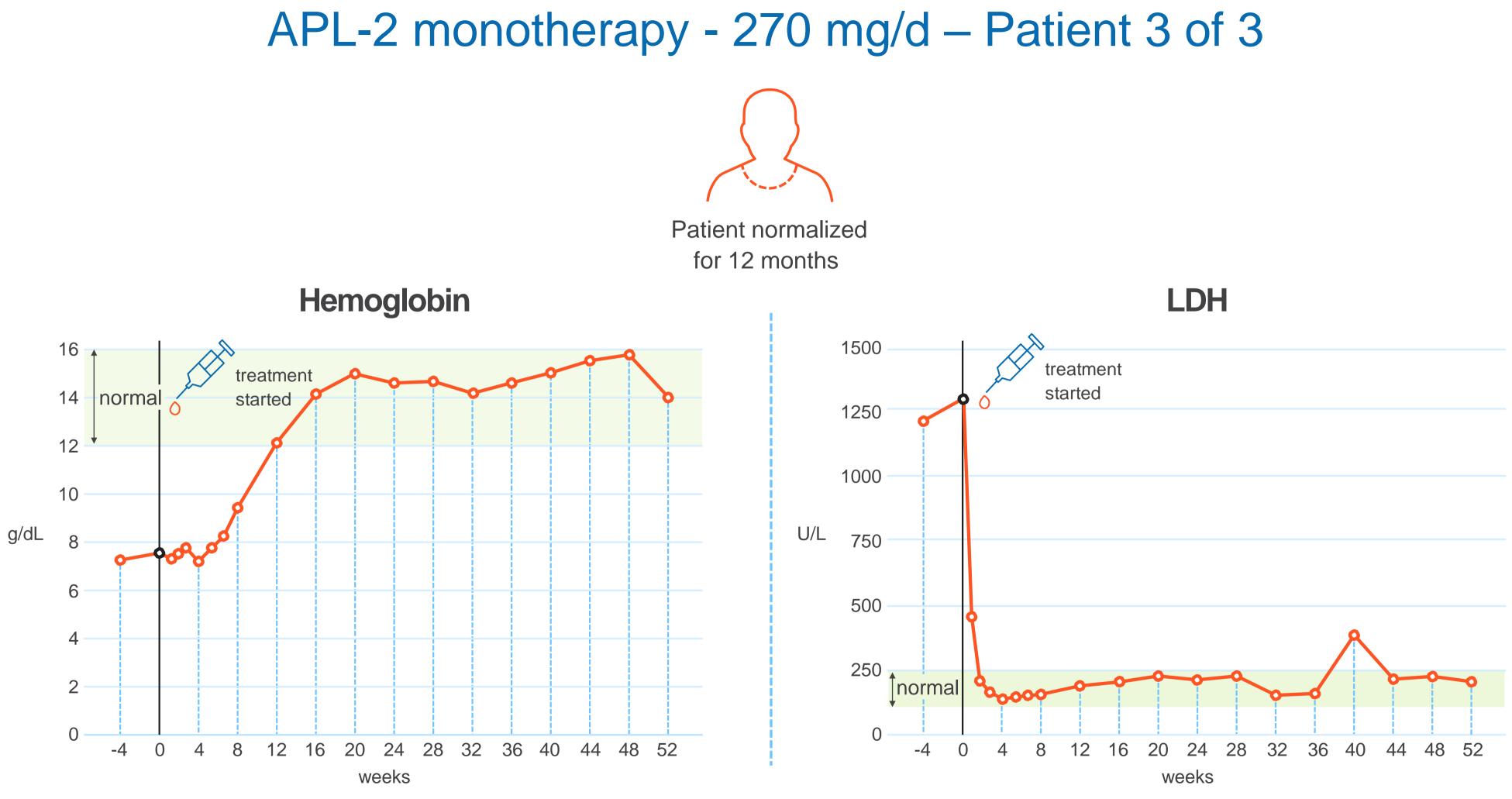


This patient had ovarian cancer



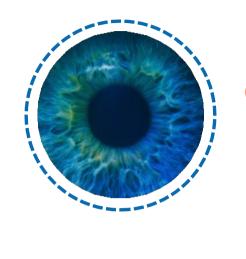
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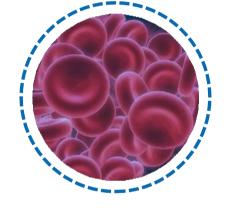


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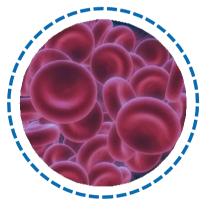
Key catalysts for 2018



GA: ✓ 18 month safety & efficacy data.



PNH: Phase 1b Soliris weaning & monotherapy expansion.



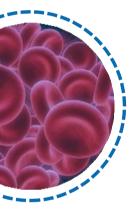
AIHA: Phase 2 POC monotherapy data.

H1





CDN: Phase 2 POC monotherapy data.



PNH:

Start of Phase 3 program.



2018

GA: Start of Phase 3 program.

H2



Thank you

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