

The Apellis logo is centered on a blue background with a repeating pattern of white chemical structures. The word "Apellis" is written in a white, sans-serif font. The dot above the letter 'i' is a small, solid red circle.

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. 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’ Annual Report on Form 10-K filed with the Securities and Exchange Commission on July 31, 2018, 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.

Key milestones for 2018



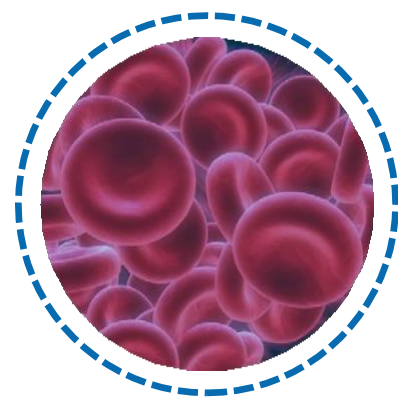
GA:

Phase 2: 18 month safety & efficacy data



GA:

Start of Phase 3 program

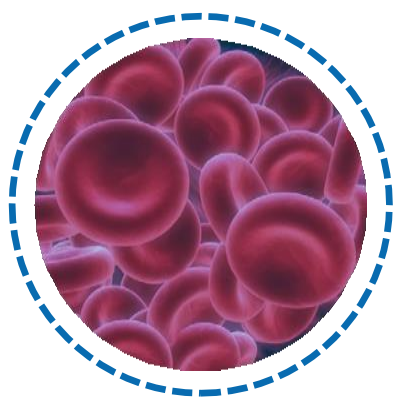


PNH:

Phase 1b: monotherapy expansion

Phase 1b: Soliris weaning in add-on study

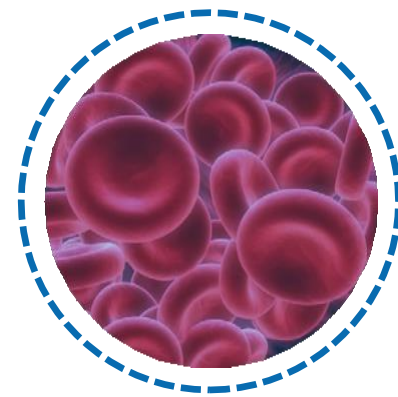
Start of Phase 3 program



AIHA:

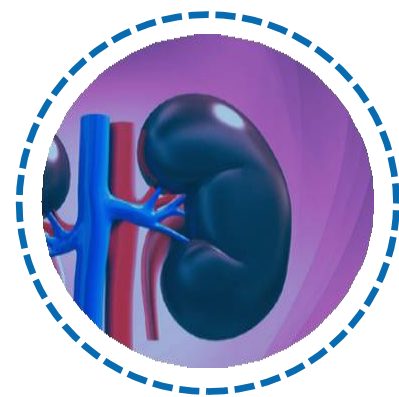
Phase 2: POC data in CAD

Phase 2: POC data in wa-AIHA



AIHA:

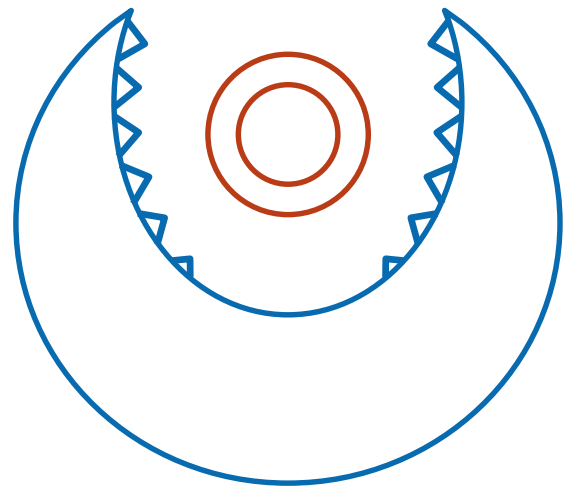
Preliminary data in CAD & wa-AIHA



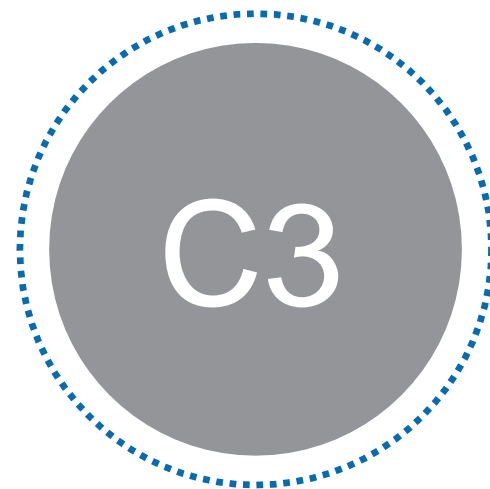
CDN:

Phase 2: POC monotherapy data

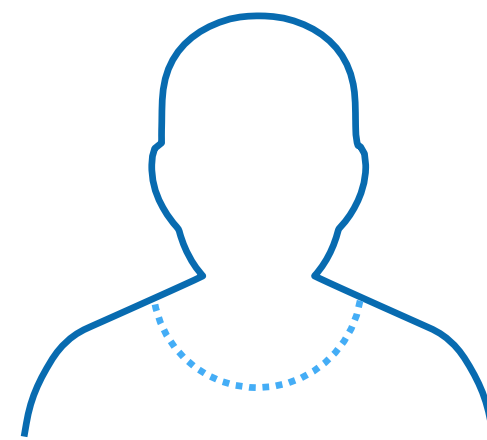
What we do



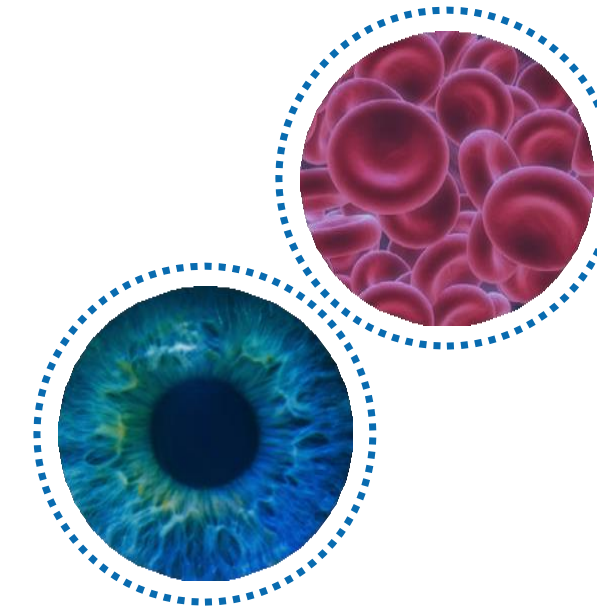
**Pioneers in innate
immunity
& complement
immunology**



**By regulating
its core
component C3**



**Value & patient
outcomes at the
center of our
programs**

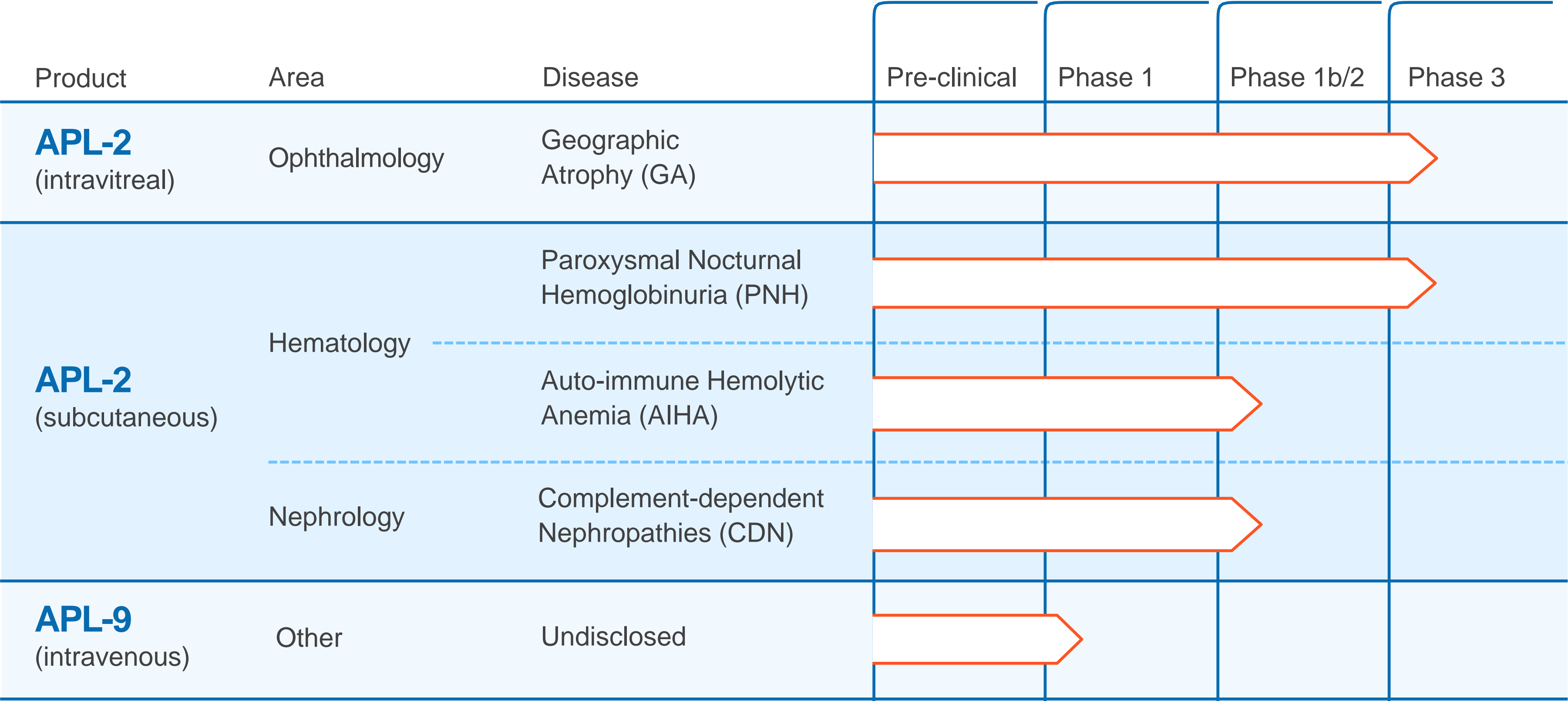


**Initially
focused on
AMD & PNH**

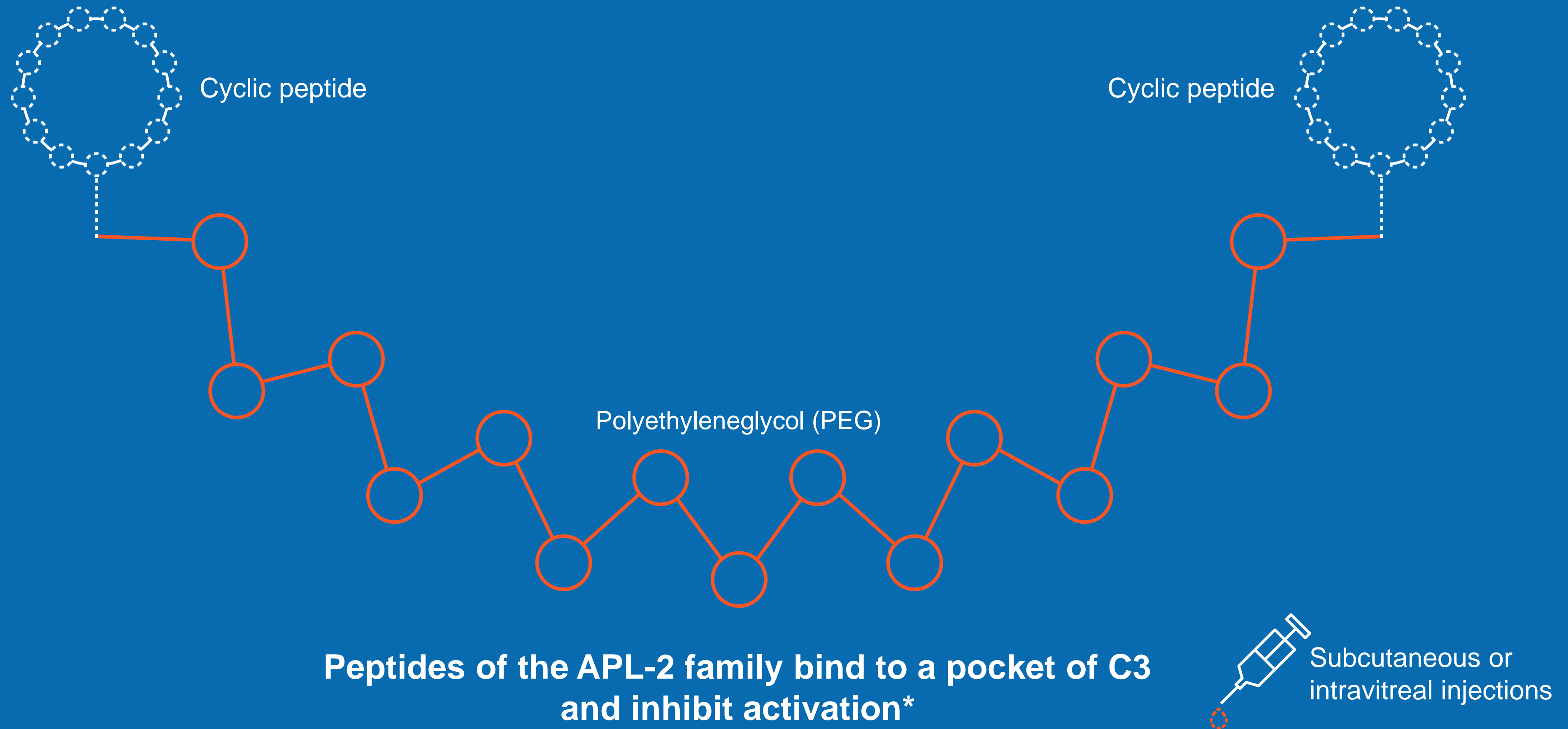


**Broad potential
in other immune
conditions**

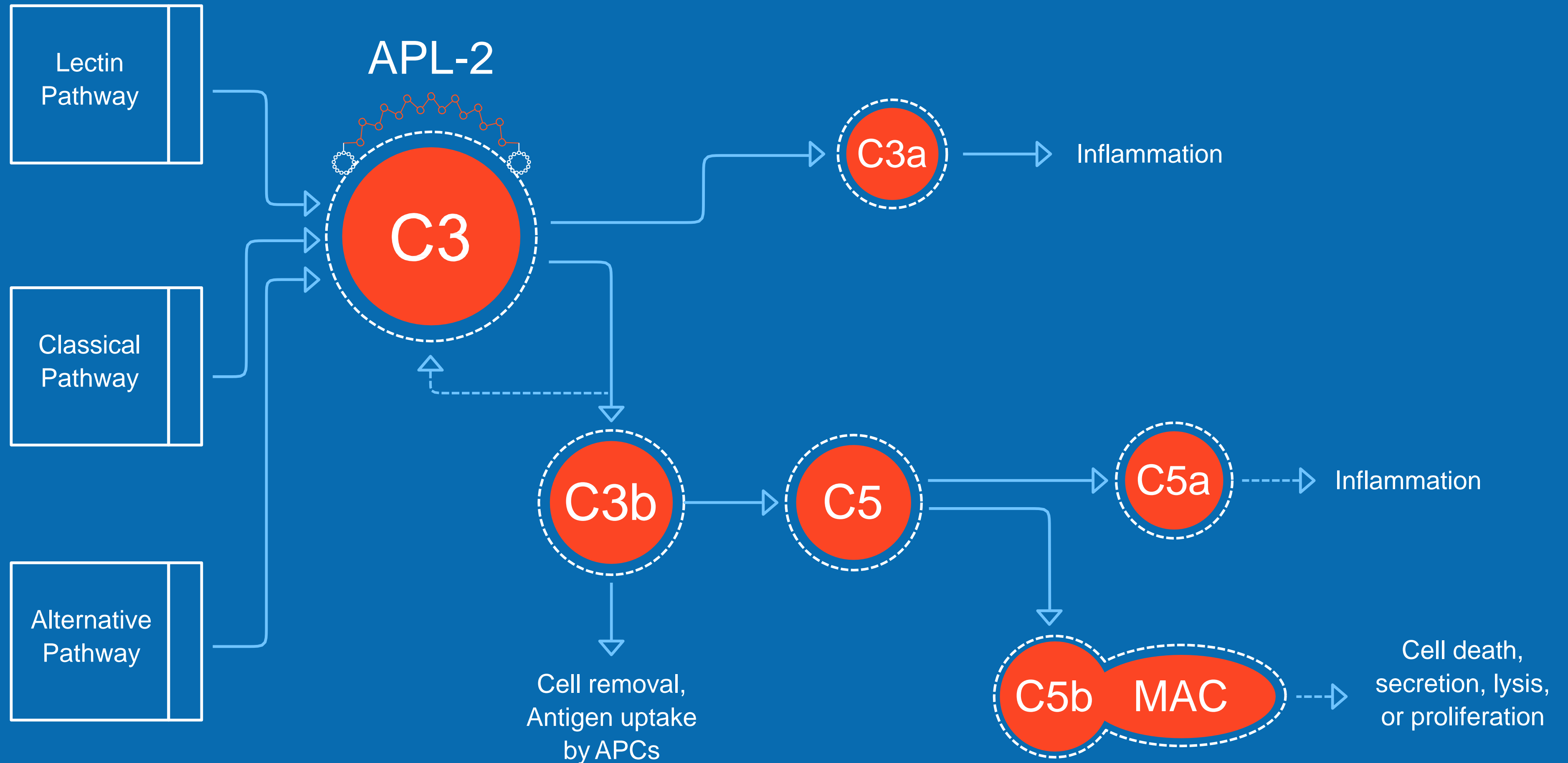
Pipeline



Apellis lead molecule: APL-2



Central inhibition of complement



Paroxysmal Nocturnal Hemoglobinuria (PNH)

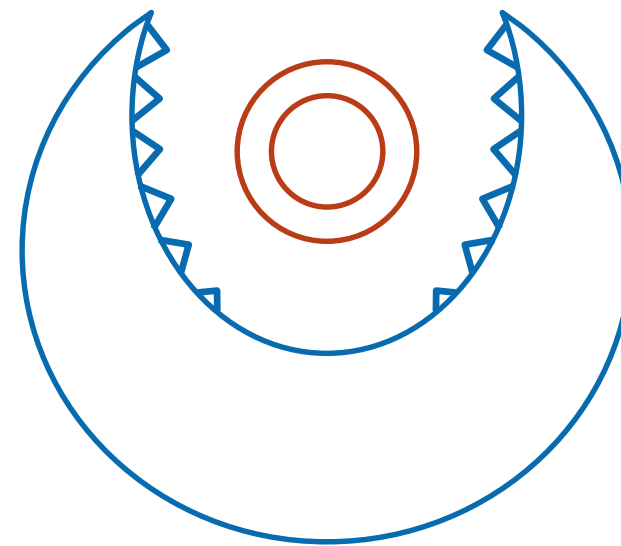
is a rare, life-threatening blood disease

PNH characterized by uncontrolled hemolysis



Intravascular hemolysis

Red blood cell rupture
in the circulation

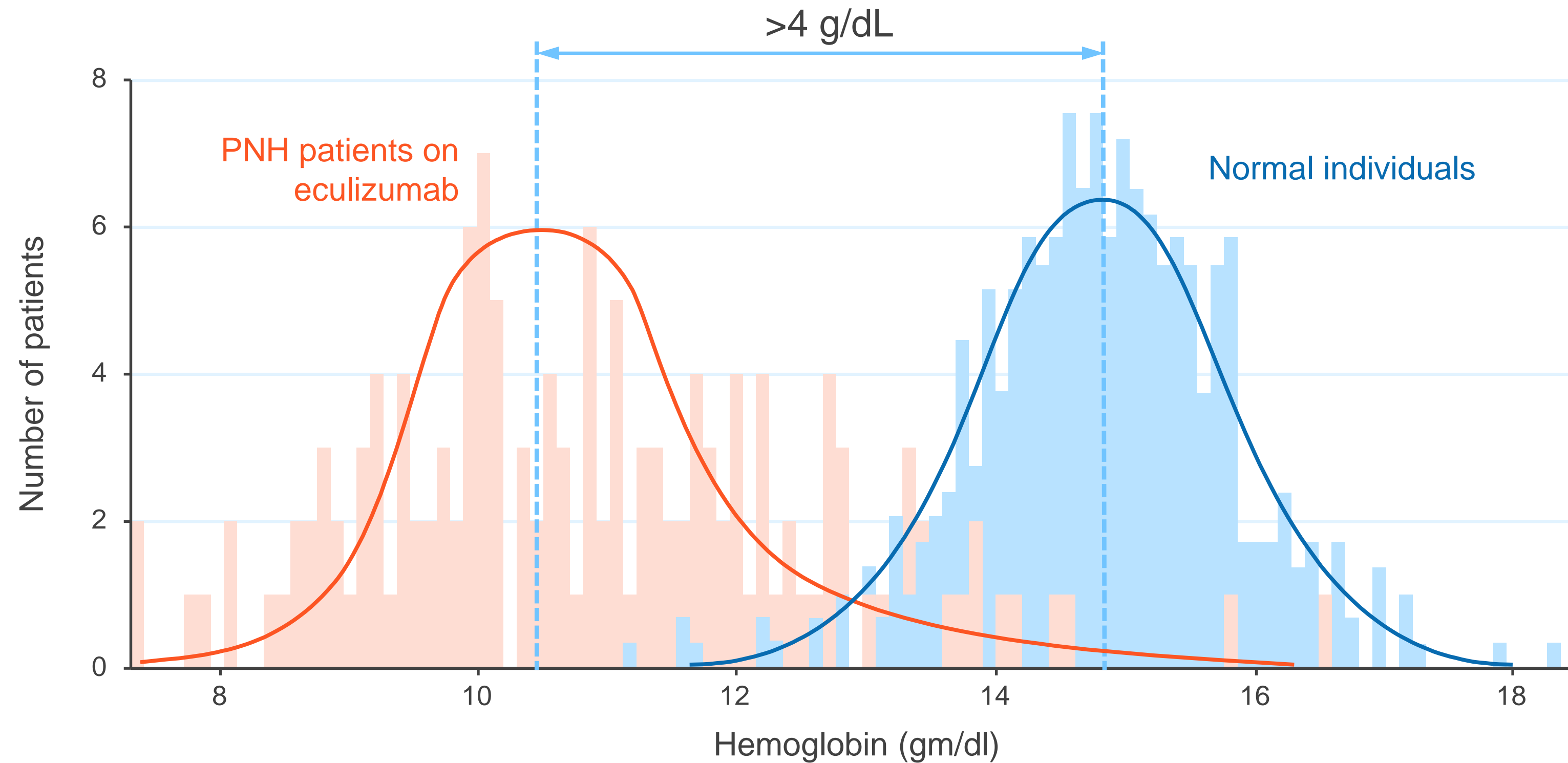


Extravascular hemolysis

Red blood cell destruction by
macrophages in spleen and liver

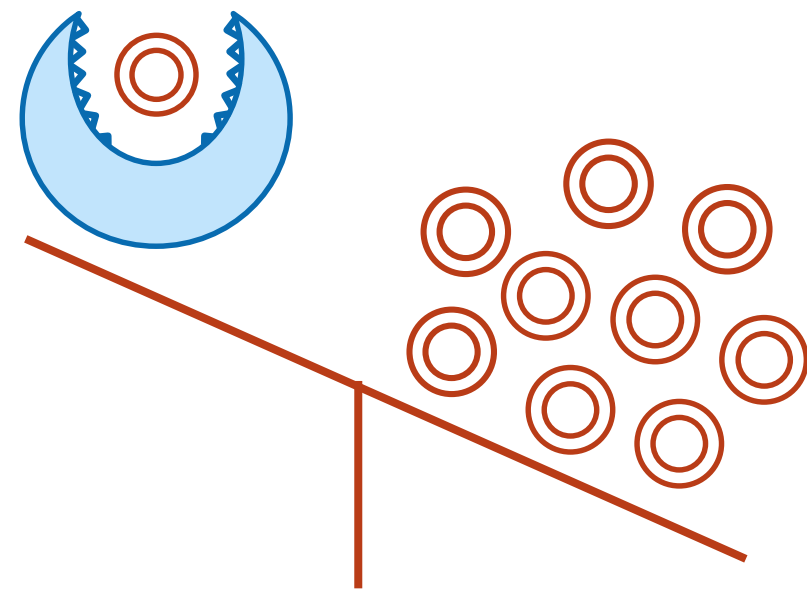
- ~12,000 prevalent patients in US, EU & Japan
 - ~4,700 patients in US
 - 35% 5-year mortality if untreated (thrombosis, severe anemia)
- Alexion's Soliris® (eculizumab) is only approved therapy
 - Treats only intravascular hemolysis
 - Many Soliris treated patients remain anemic and transfusion dependent due to extravascular hemolysis

Hemoglobin levels in patients with PNH receiving eculizumab (n=141; all hemolytic)




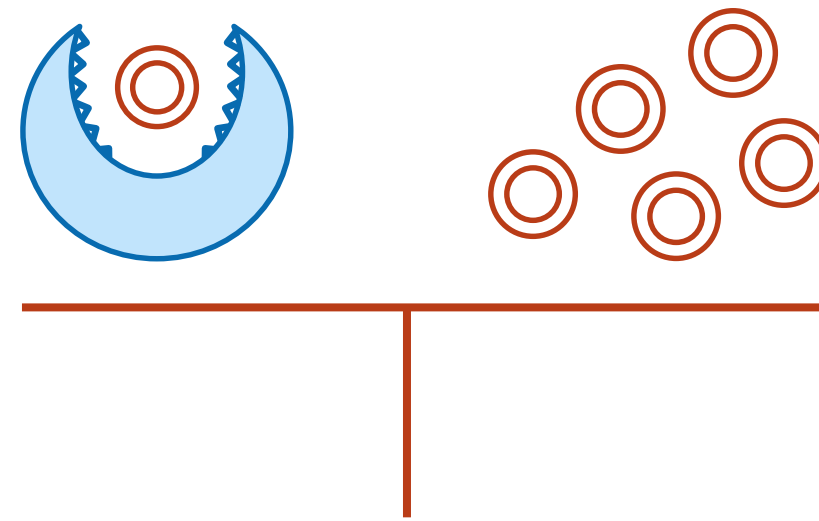
Source: Peter Hillmen, Professor of Experimental Haematology, University of Leeds

Only 30% of patients on Soliris® have bone marrow function strong enough to keep patients from experiencing anemia and/or transfusion




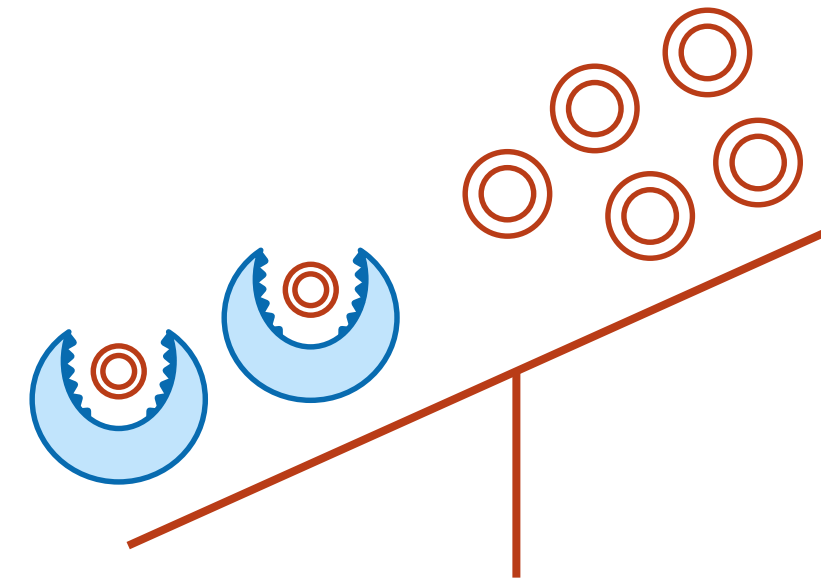
~30% of Patients

- Transfusion - 
- Hb >12




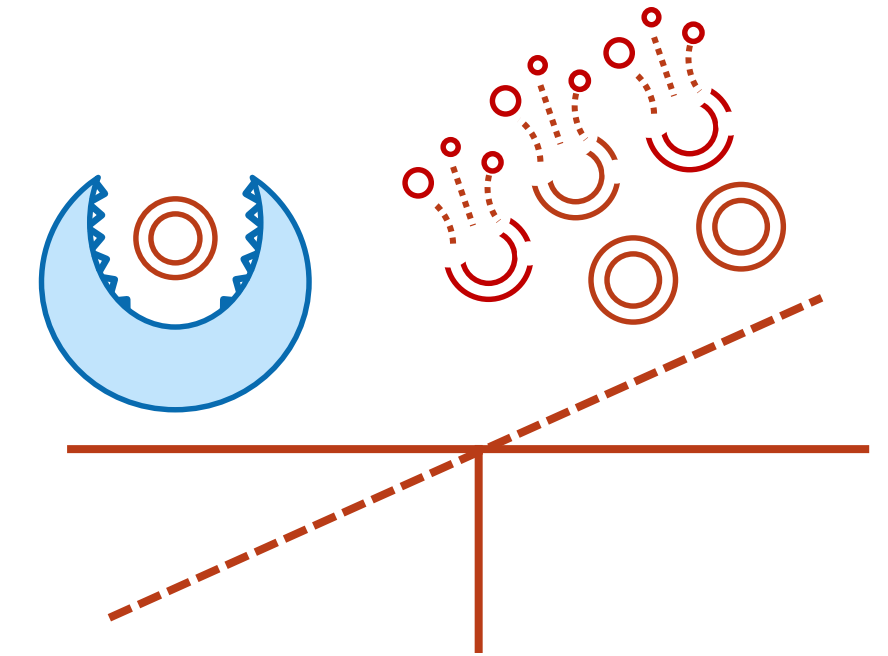
~40% of Patients

- Transfusion - 
- Hb <12




~20% of Patients

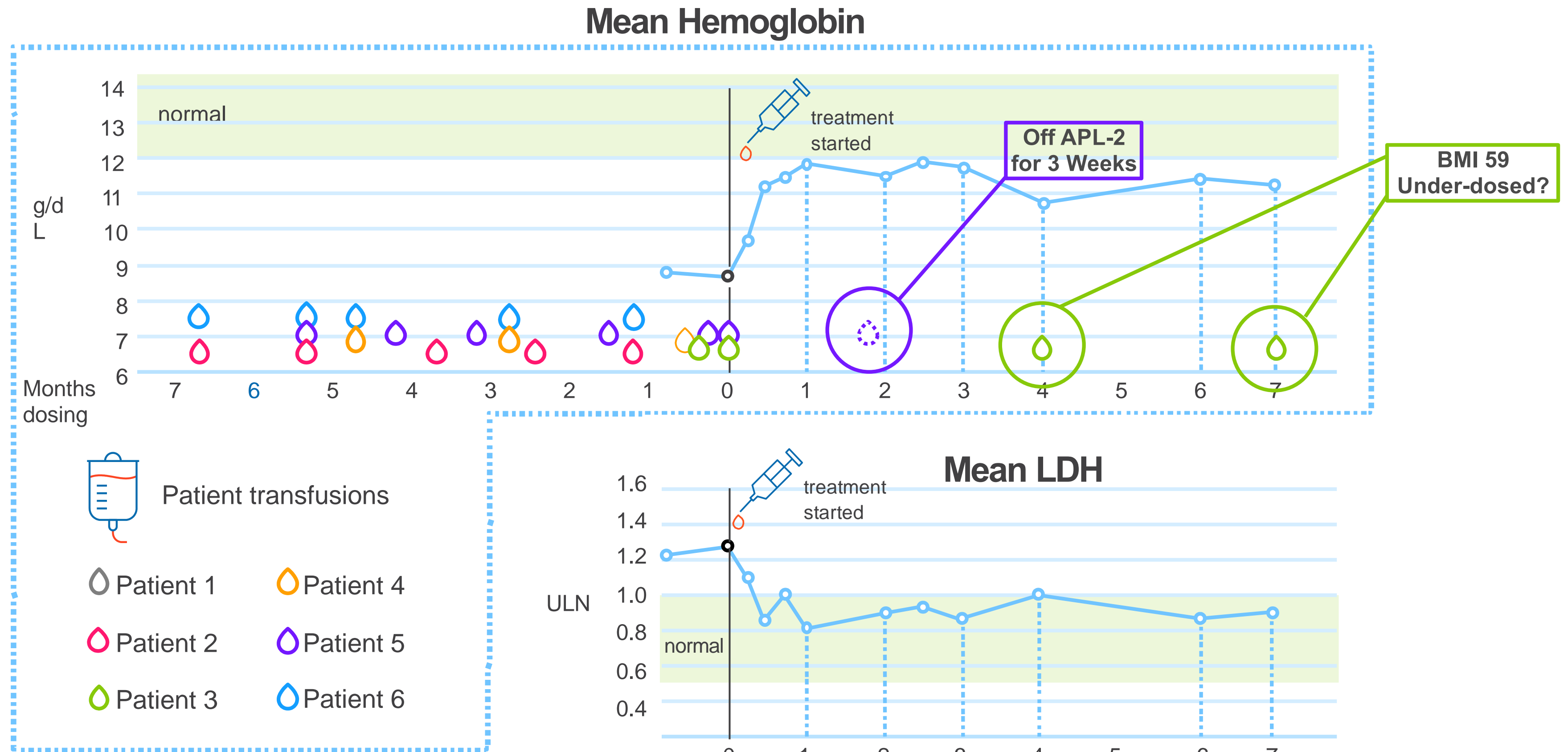
- Transfusion ++ 
- Hb <10



~10% of Patients

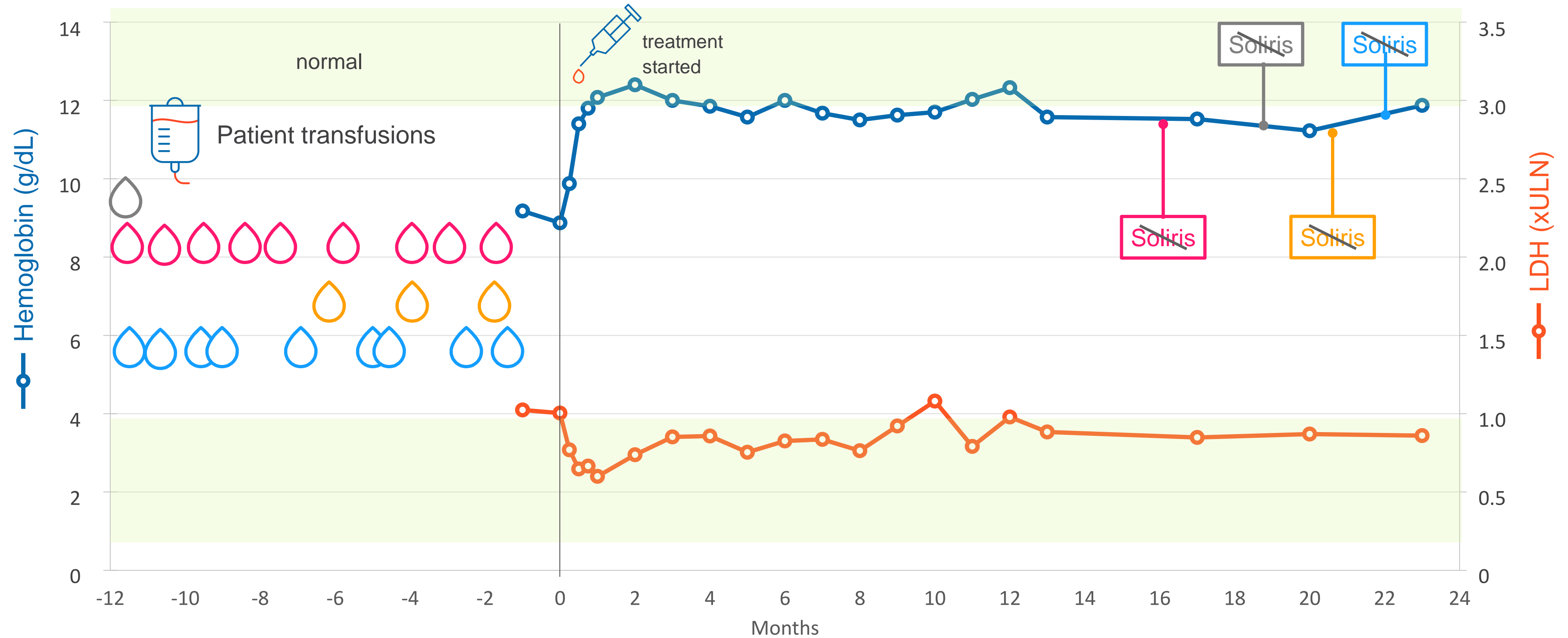
- Breakthrough ++ 
- Hb = any

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



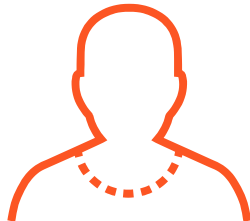


Interim data as reported June 2017

PHAROAH: APL-2 add-on to Soliris® - all four patients successfully transitioned to APL-2 monotherapy



PHAROAH: APL-2 add-on to Soliris® - all four patients successfully transitioned to APL-2 monotherapy

	 Eculizumab Monotherapy ⁱ	 APL-2 + Eculizumab ⁱⁱ	 APL-2 Monotherapy ⁱⁱⁱ
Hemoglobin (g/dL) *	8.9	11.9	11.4
Annual Transfusions (avg.)	6.0	0	0
LDH (ULN) *	1.0x	0.8x	0.9x
Reticulocytes (ULN)*	2.7x	1.2x	0.8x
Patient Years (Total)	NA	5.9 Years	1.9 Years
Multiple of Eculizumab Label Dose (900mg x 2wk.)	1.6x	1.0x	-

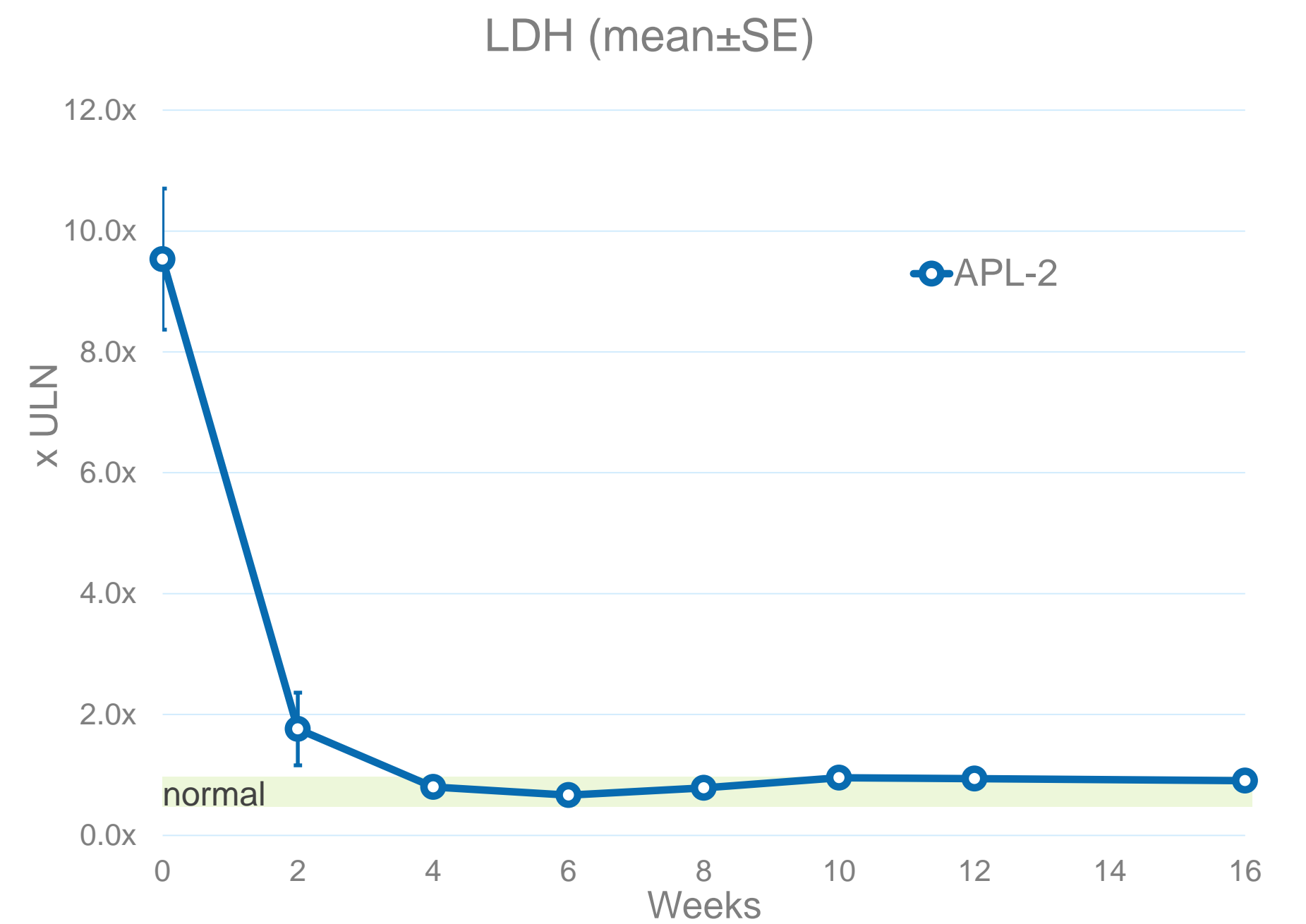
*Average last available reading for all four patients on each dosing regimen

i) last reading during eculizumab monotherapy prior to co-treatment with APL-2

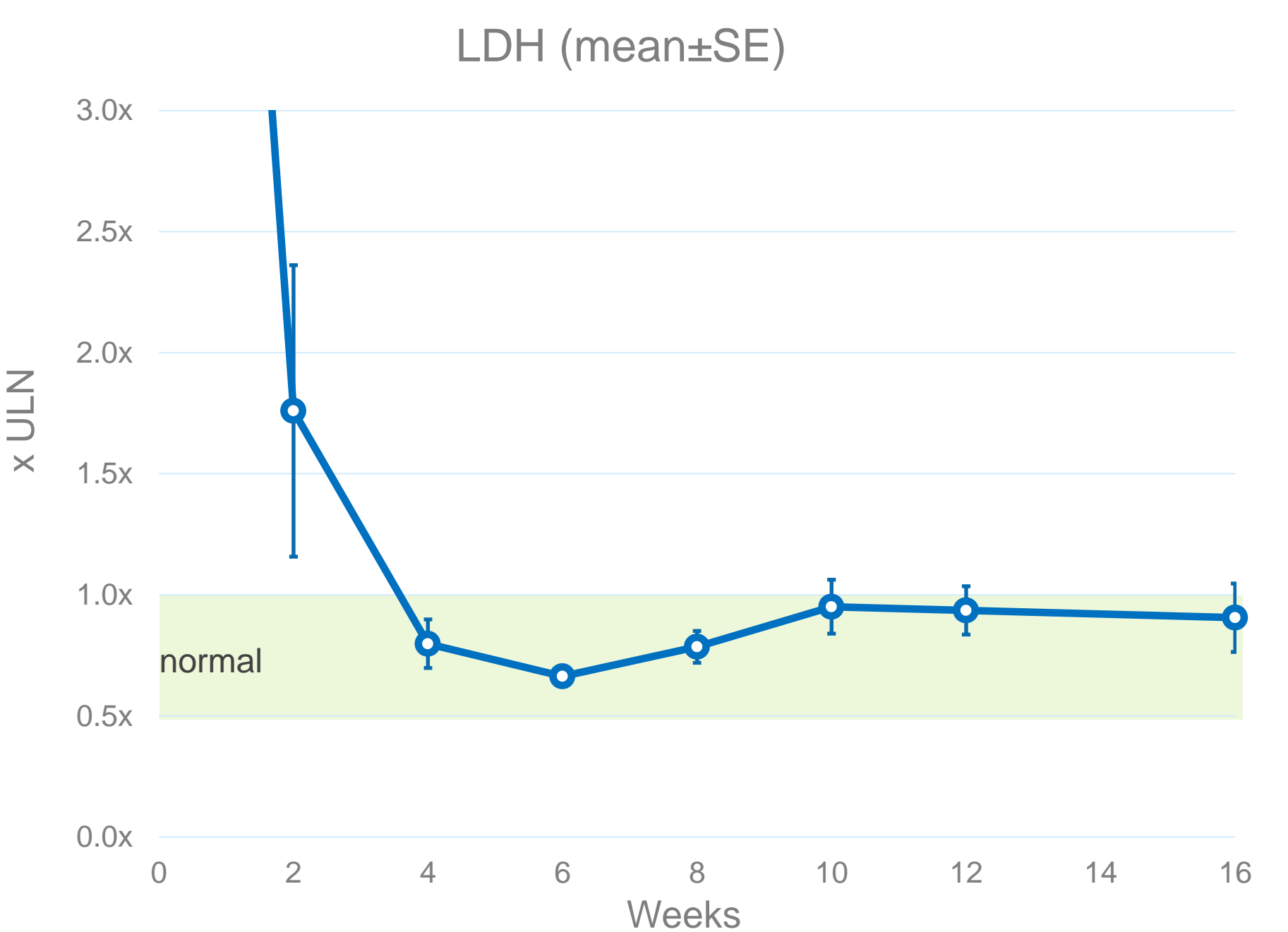
(ii) last reading during co-treatment and prior to APL-2 monotherapy

(iii) last reading while on APL-2 monotherapy

PADDOCK (interim): APL-2 shows potential to reach normal LDH levels as monotherapy in treatment in naïve PNH patients – 270 mg/day

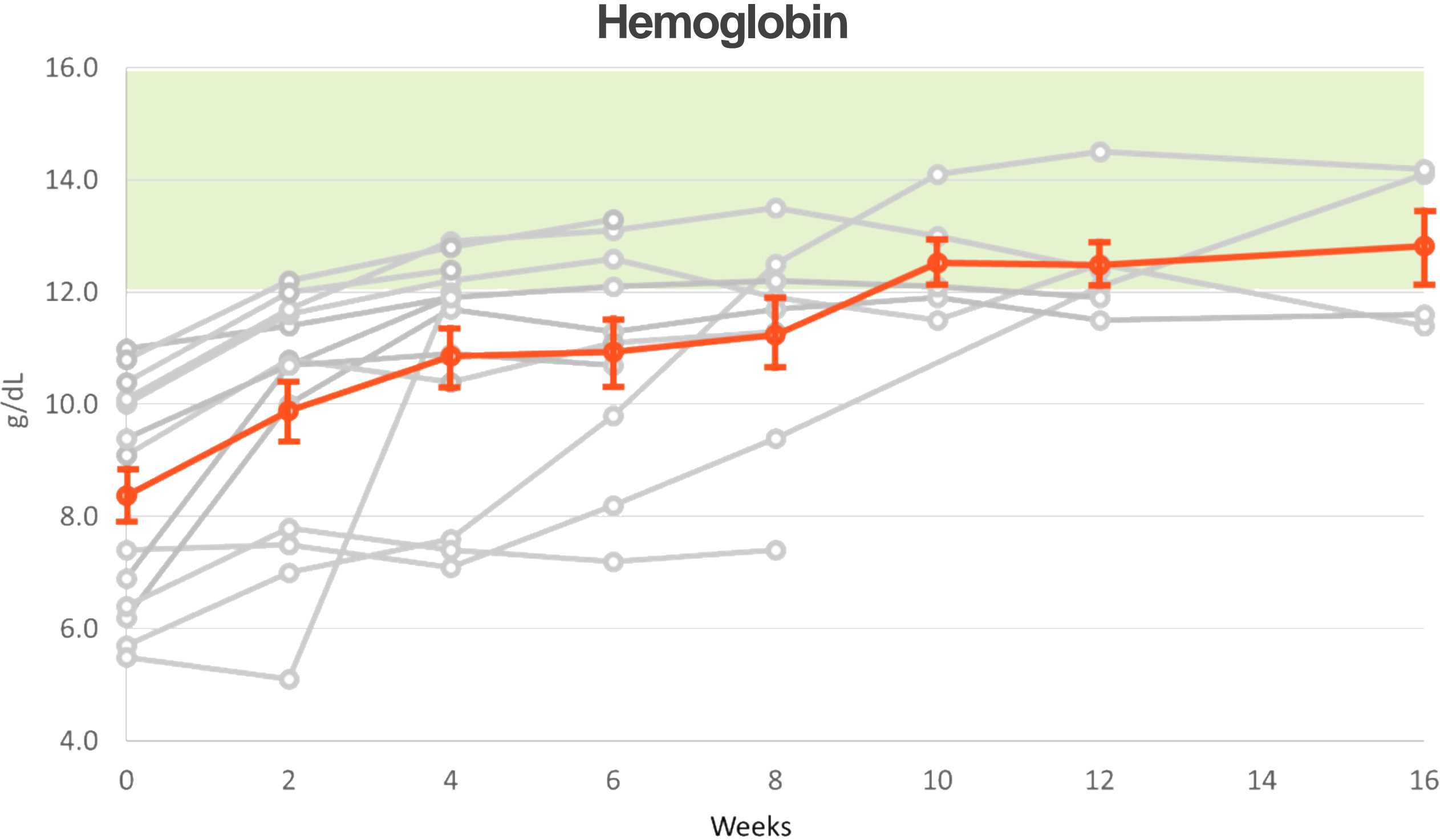


Excludes results from one of the original three patients, who had underlying metastatic ovarian cancer with a chronic low gastrointestinal bleed, unknown at the time of screening.



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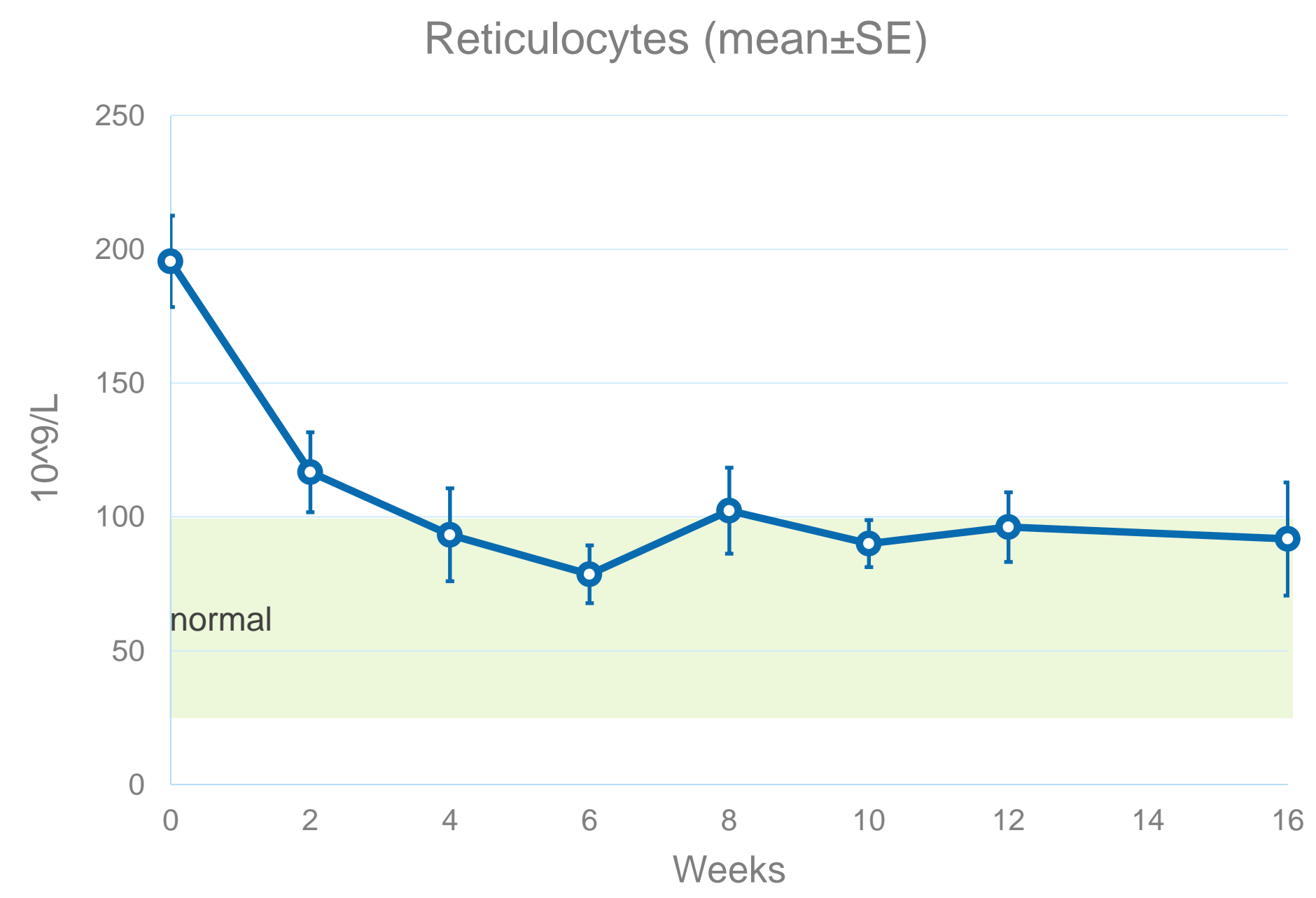
PADDOCK (interim): Mean hemoglobin improvement was 3.5 g/dL[†] (n=13) showing an impact on both intravascular and extravascular hemolysis



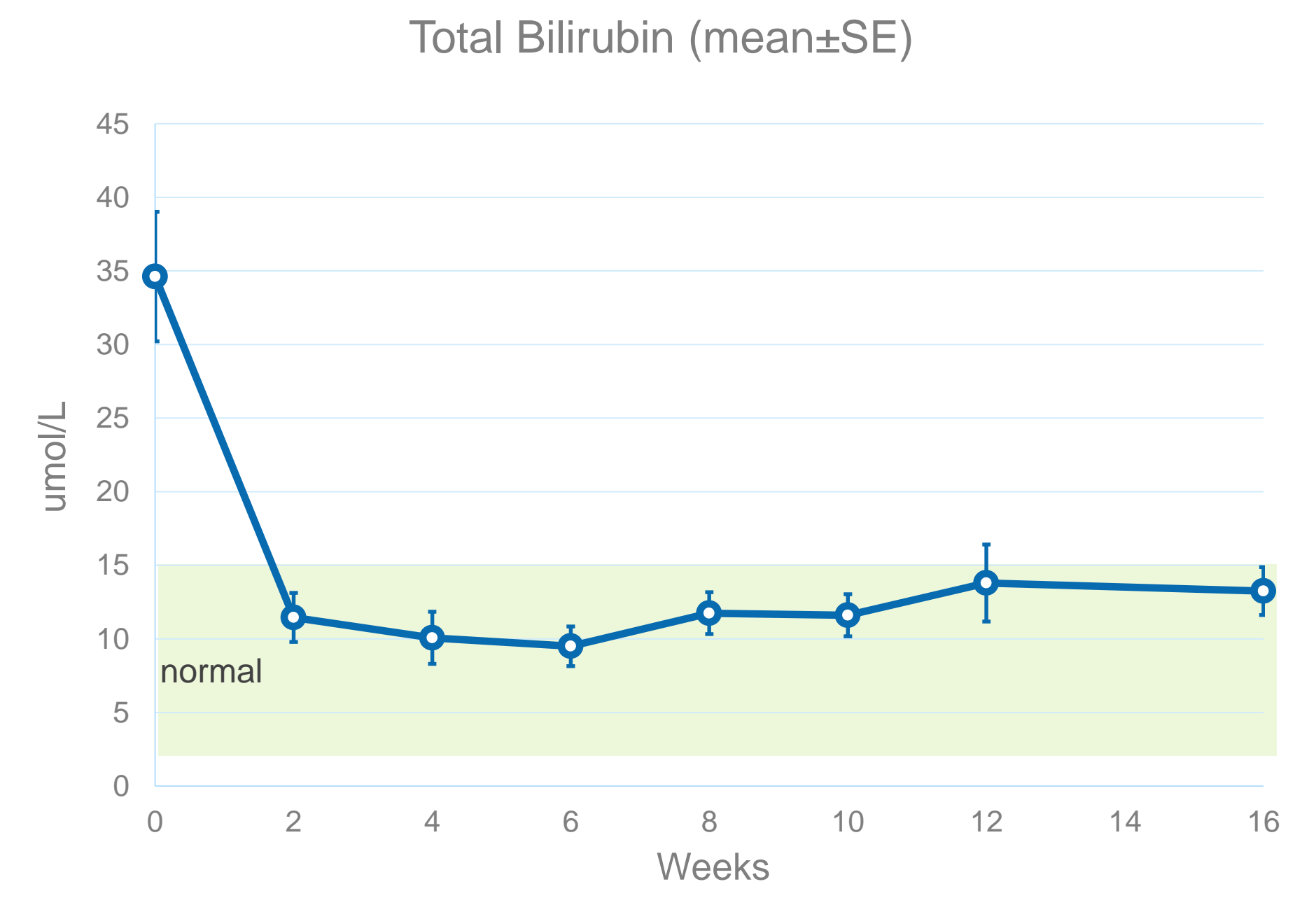
- 2/13 patients had transfusions, one at day 2 and a non-compliant patient at day 14
- It is believed that neither patient had yet reached sufficient exposure to APL-2 for hematological benefit

[†] At last measure; excludes one patient who had underlying metastatic ovarian cancer with a chronic low gastrointestinal bleed, unknown at the time of screening, which resulted in artificially low Hb and high LDH levels that were determined to be unrelated to PNH.

PADDOCK (interim): other measures of anemia meaningfully improved with APL-2 including reticulocytes and bilirubin

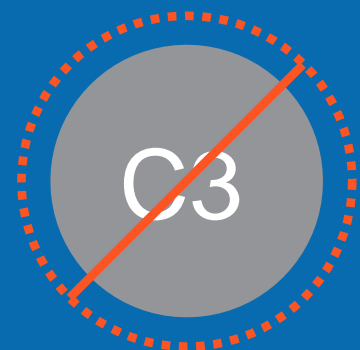


Week	0	2	4	6	8	10	12	14	16
n	n=13	n=13	n=13	n=11	n=8	n=5	n=5	Not Taken	n=4
Mean Retics (10 ⁹ /L)	196	117	93	79	102	90	96	Not Taken	92

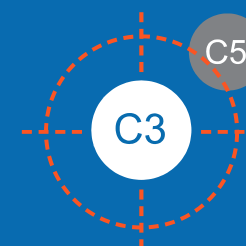


Week	0	2	4	6	8	10	12	14	16
n	n=13	n=13	n=13	n=11	n=8	n=5	n=5	Not Taken	n=4
Mean Bilirubin (umol/L)	35	11	10	10	12	12	14	Not Taken	13

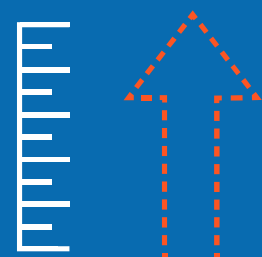
APL-2 PNH program – early data suggests potentially differentiated efficacy



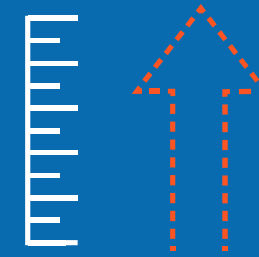
APL-2 blocks C3 and inhibits intravascular & extravascular hemolysis



LDH reduction appears to be equivalent to or better than C5 inhibitors



Significant increase in HgB levels to normal ranges in most patients



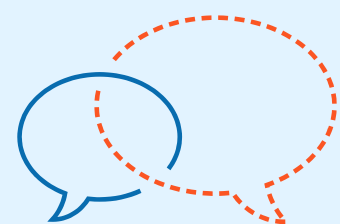
Other hematological measures meaningfully improved including reticulocytes and bilirubin



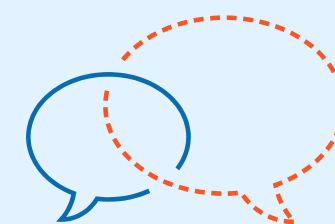
Ameliorates transfusion dependence in sub-optimal responding patients on high dose C5 inhibitors (PHAROAH)



Favorable product profile with convenient sub-q dosing & stability at room temperature for several months



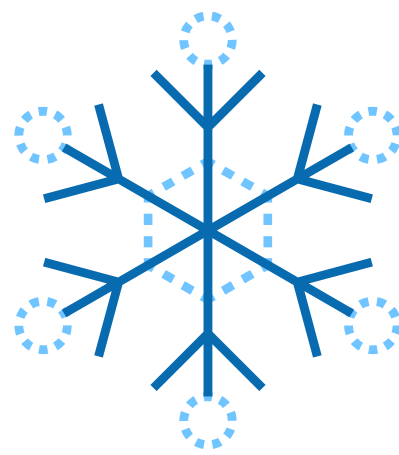
“While the LDH corrections seen with APL-2 monotherapy in these patients with PNH are truly remarkable, it is the significant hemoglobin correction that is most clinically meaningful.” Dr. Peter Hillmen



“Elevated reticulocytes and bilirubin are important markers of anemia resulting from extravascular hemolysis and are not known to improve in patients treated with eculizumab or other C5 inhibitors.” Dr. Anita Hill

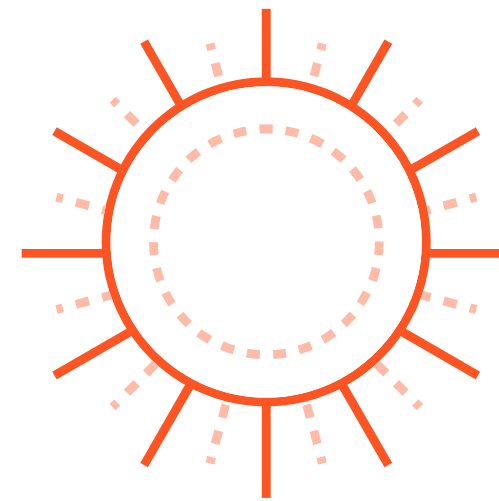
Autoimmune hemolytic anemia (AIHA) is a group of rare autoimmune disorders characterized by the premature hemolysis of red blood cells (RBCs) by autoantibodies

AIHA presents in two common forms



Cold Agglutinin Disease

Typically associated with
IgM autoantibodies
– 20-25% of cases

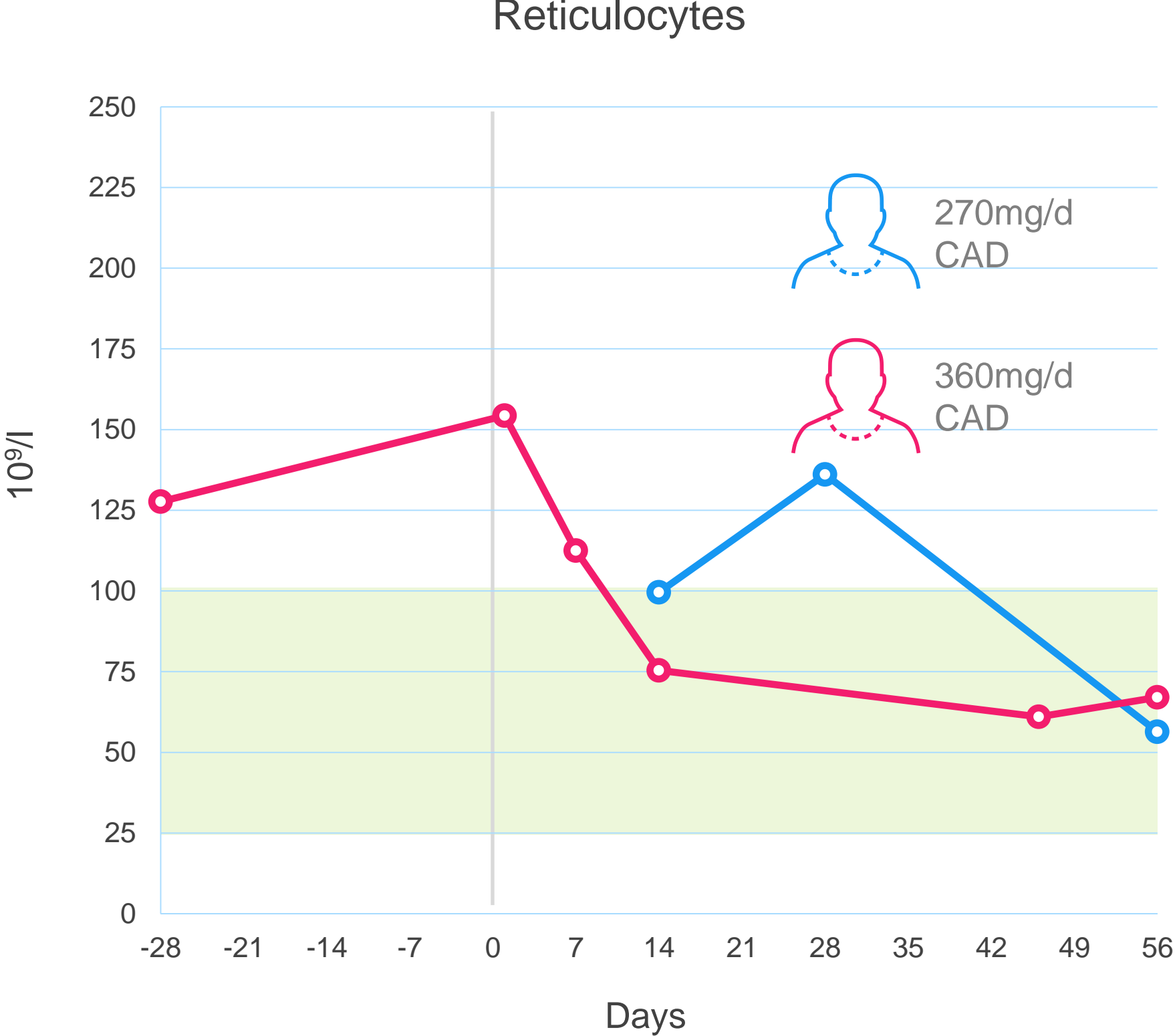
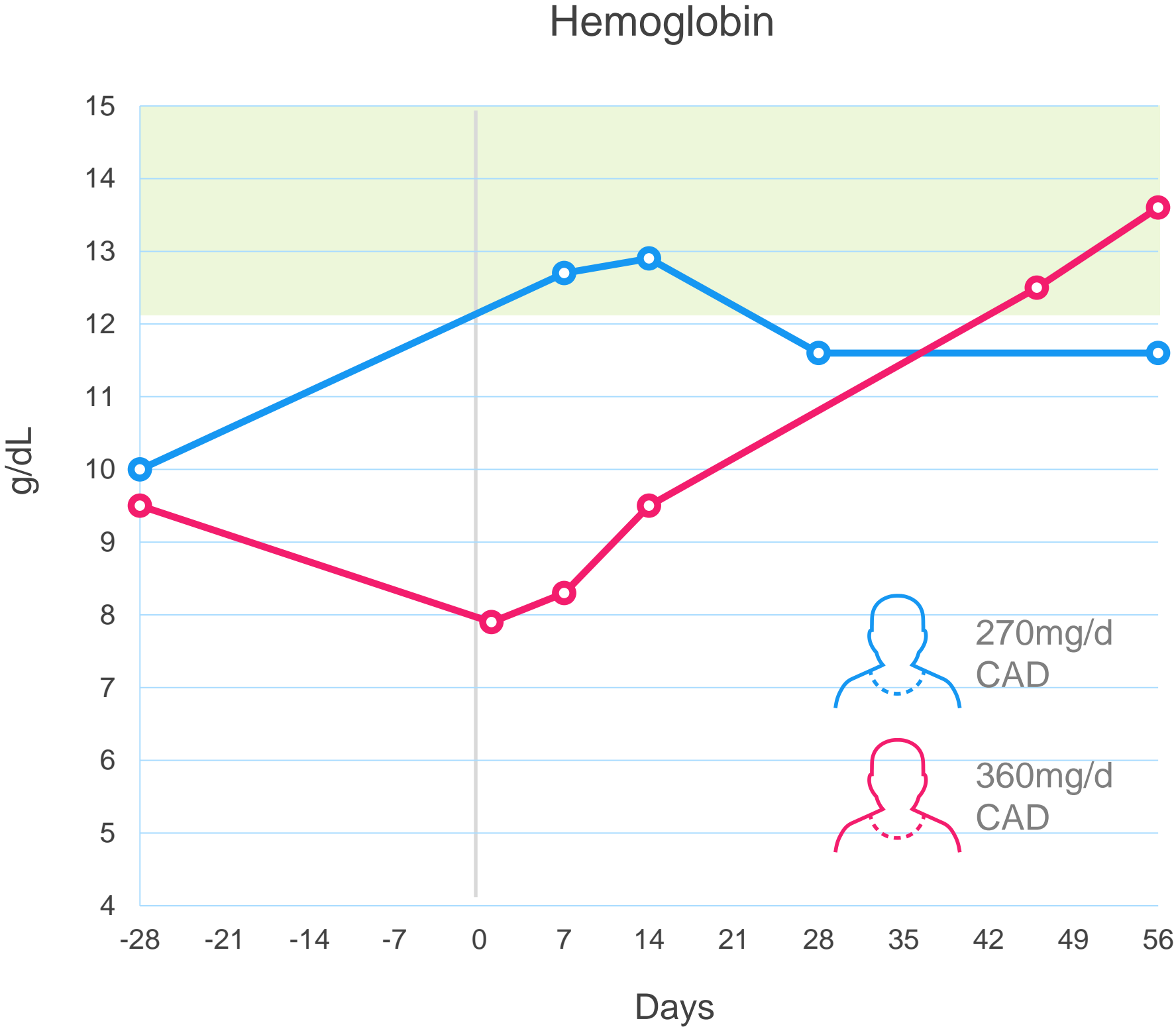


Warm Antibody AIHA

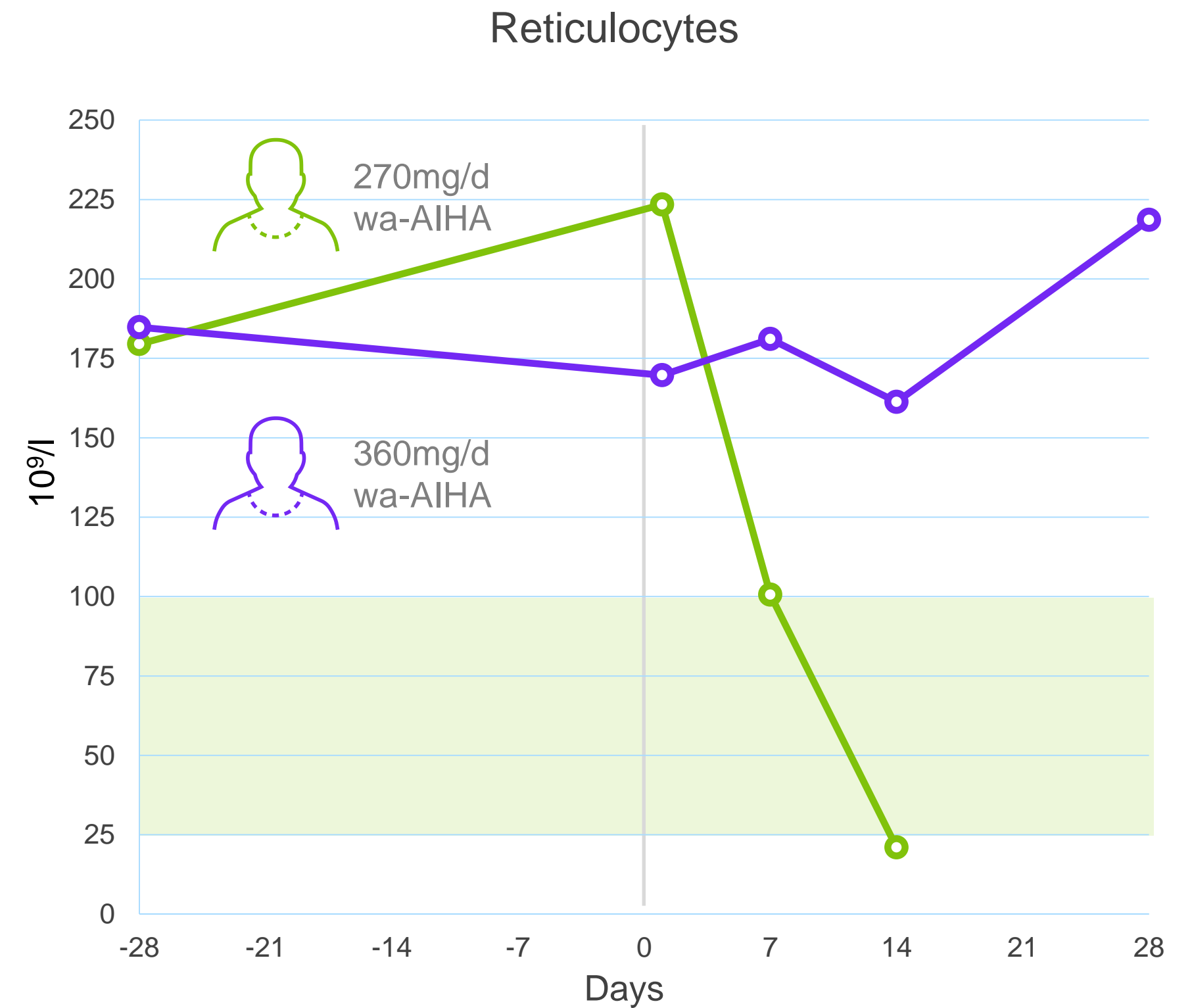
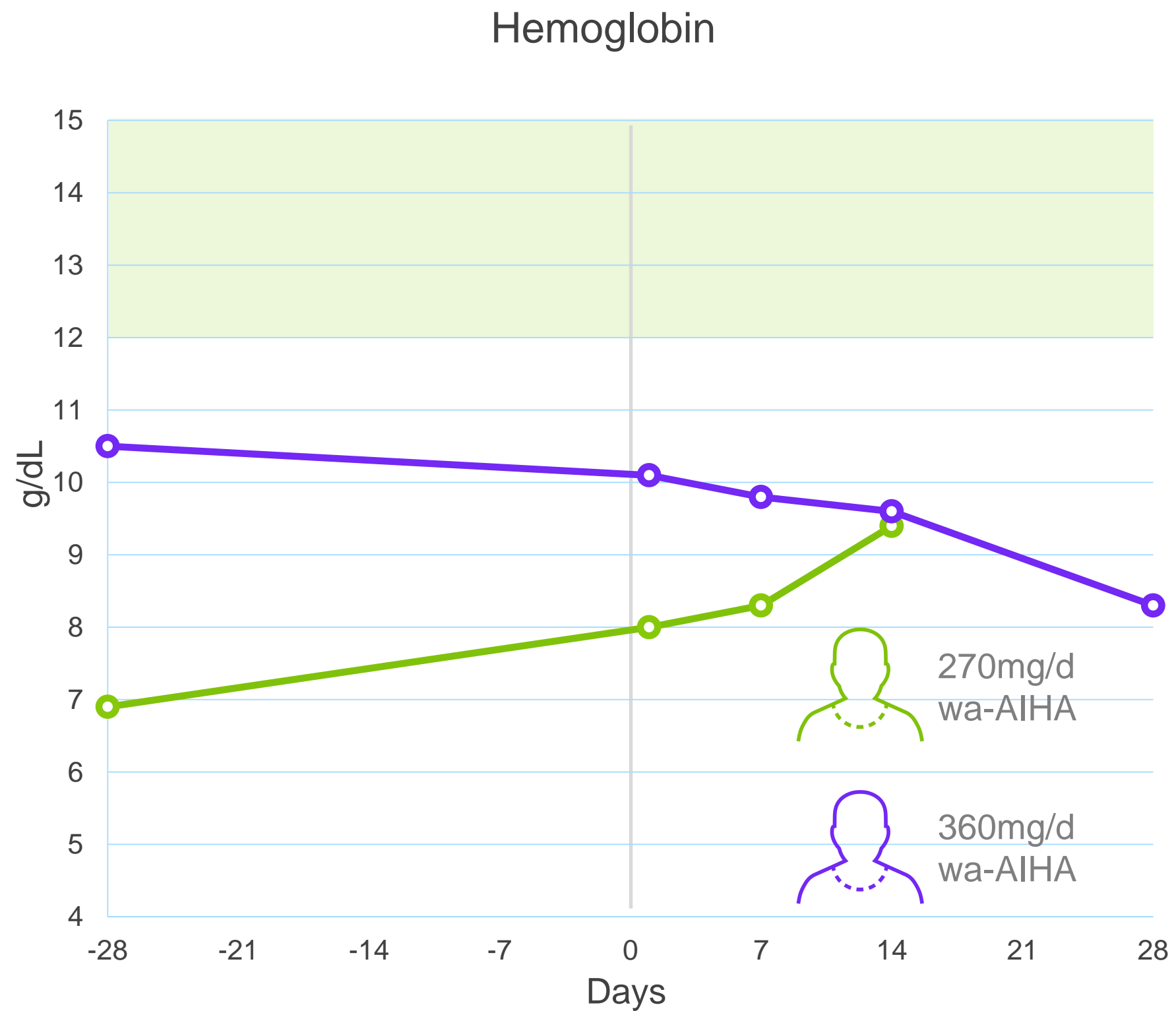
Typically associated with
IgG autoantibodies
- 60-70% of cases

- ~25,000 AIHA patients in US
- AIHA patients present with anemic symptoms similar to PNH
- Overall mortality of 11%
- IgG and IgM antibodies are the main cause of AIHA resulting in RBC phagocytosis and lysis
- Corticosteroids are first line therapy
- Many patients progress to splenectomy or Rituxan (off-label)

APL-2 in Cold Agglutinin Disease (n=2) – Preliminary Data



APL-2 in Warm Antibody AIHA (n=2) – Preliminary Data



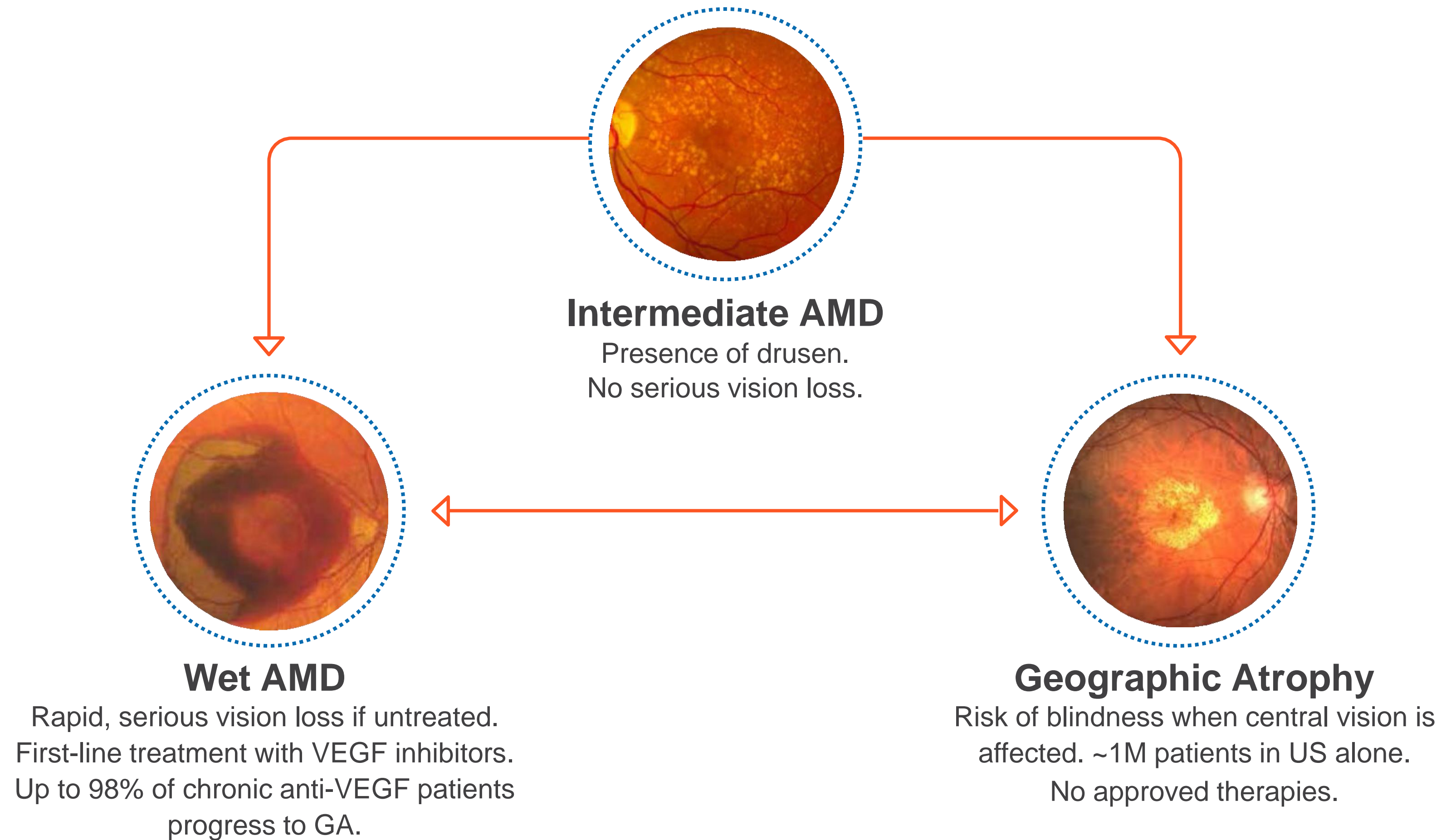
Geographic Atrophy Impacts

One Million People

in the U.S. Alone

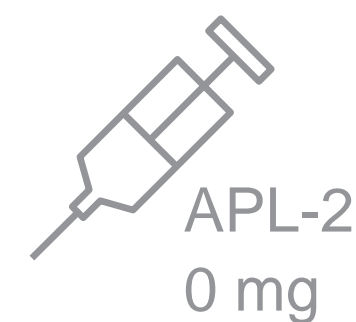


Geographic Atrophy - the leading cause of blindness



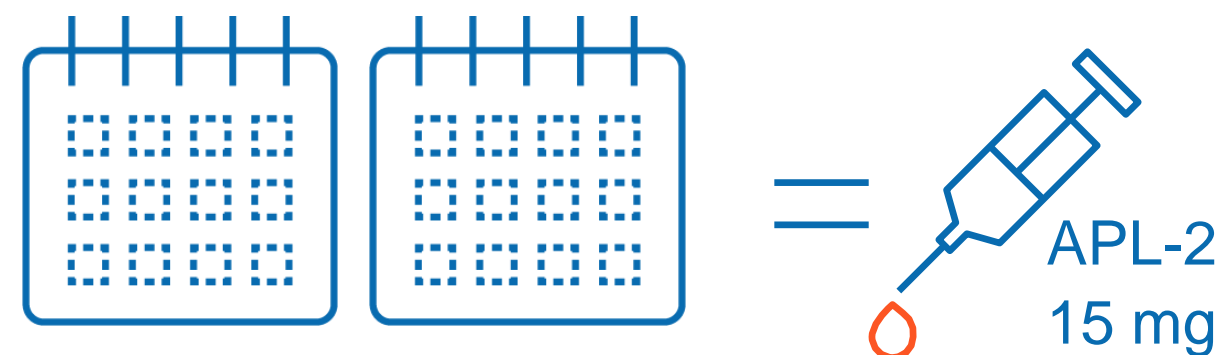
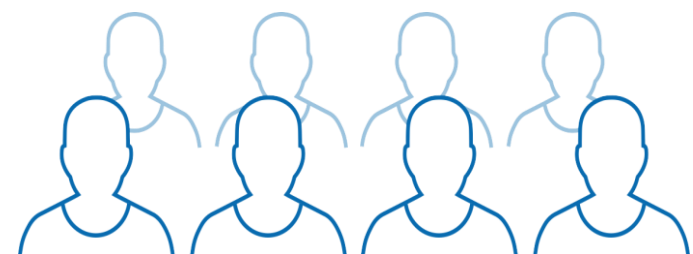
FILLY - Phase 2 study of APL-2 in Geographic Atrophy

Sham group, n=81 (pooled)



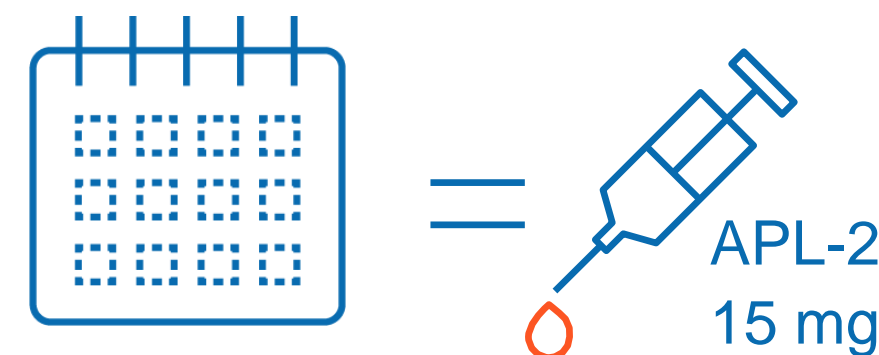
Sham injections

APL-2 EOM, n=79



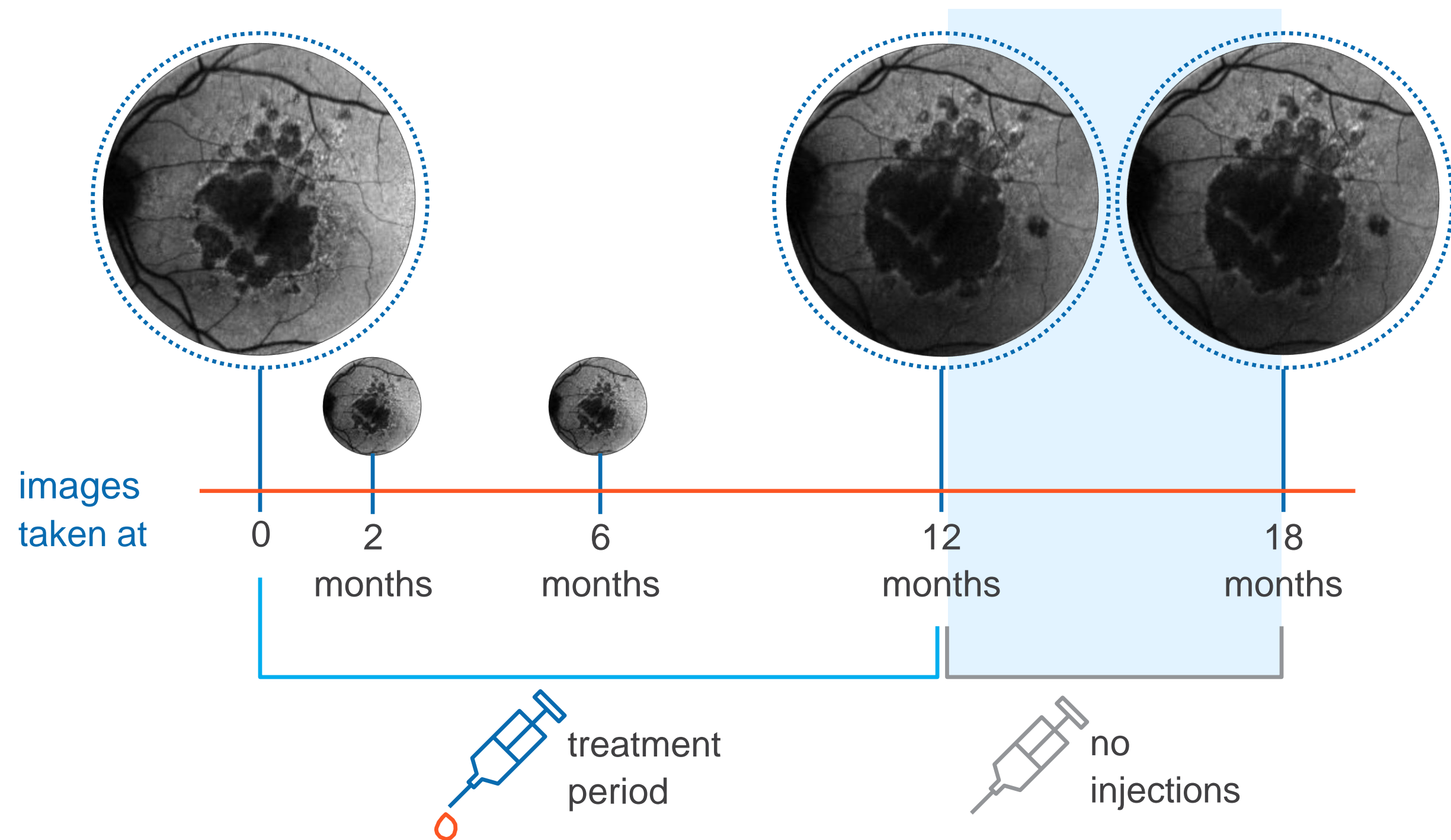
APL-2 injections every other month

APL-2 Monthly, n=86



APL-2 injections every month

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).

Filly 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)

APL-2 slowed GA growth at 12 months (square root) – primary endpoint



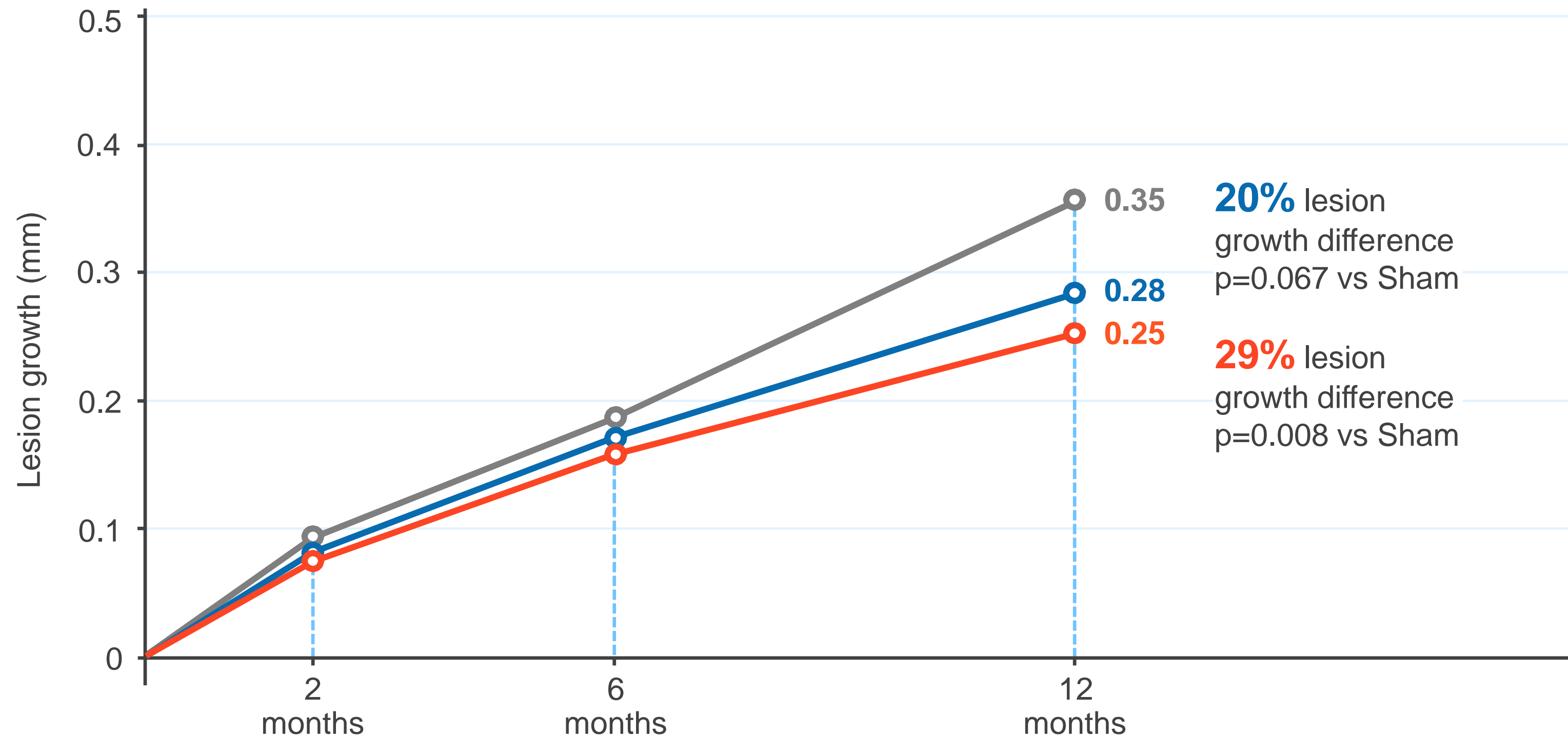
sham
injections



APL-2 every
other month

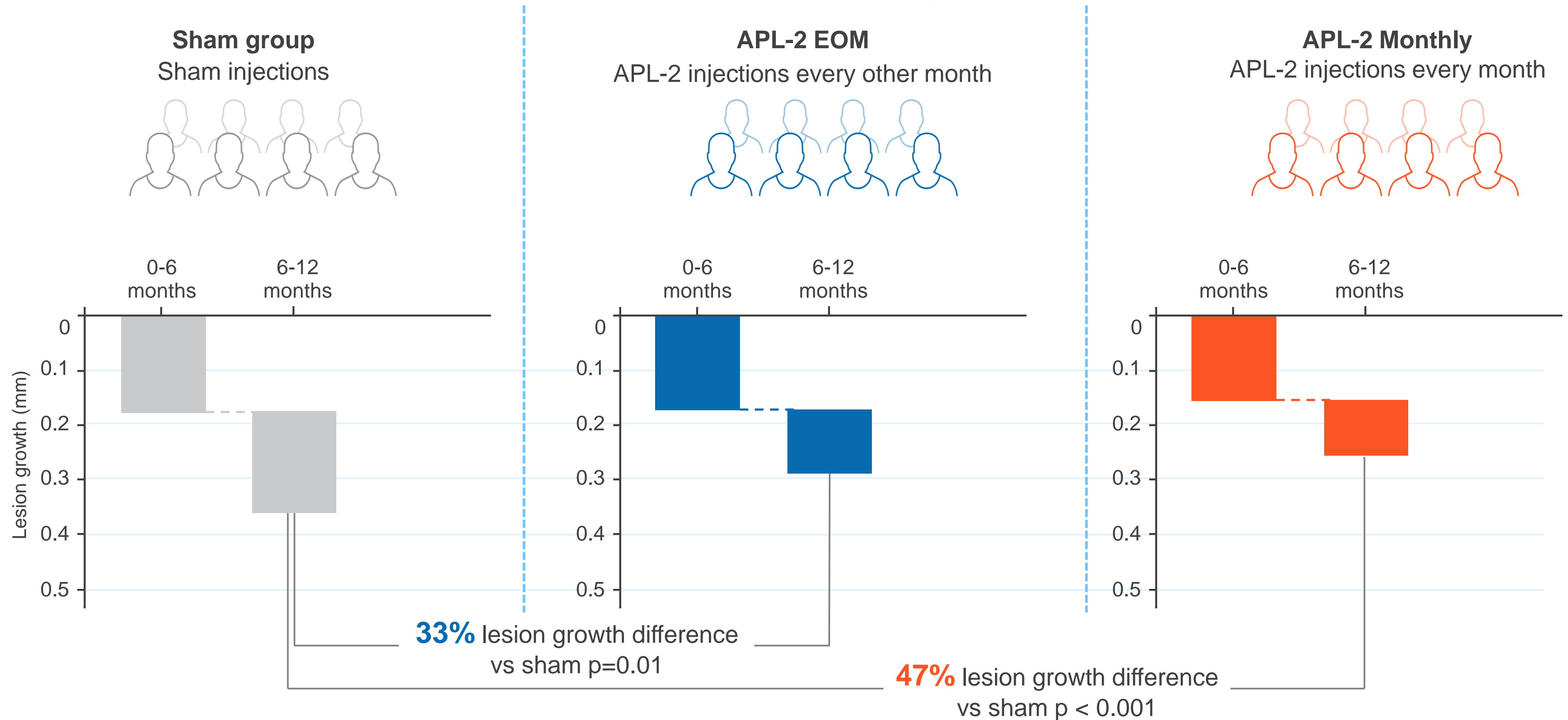


APL-2
monthly



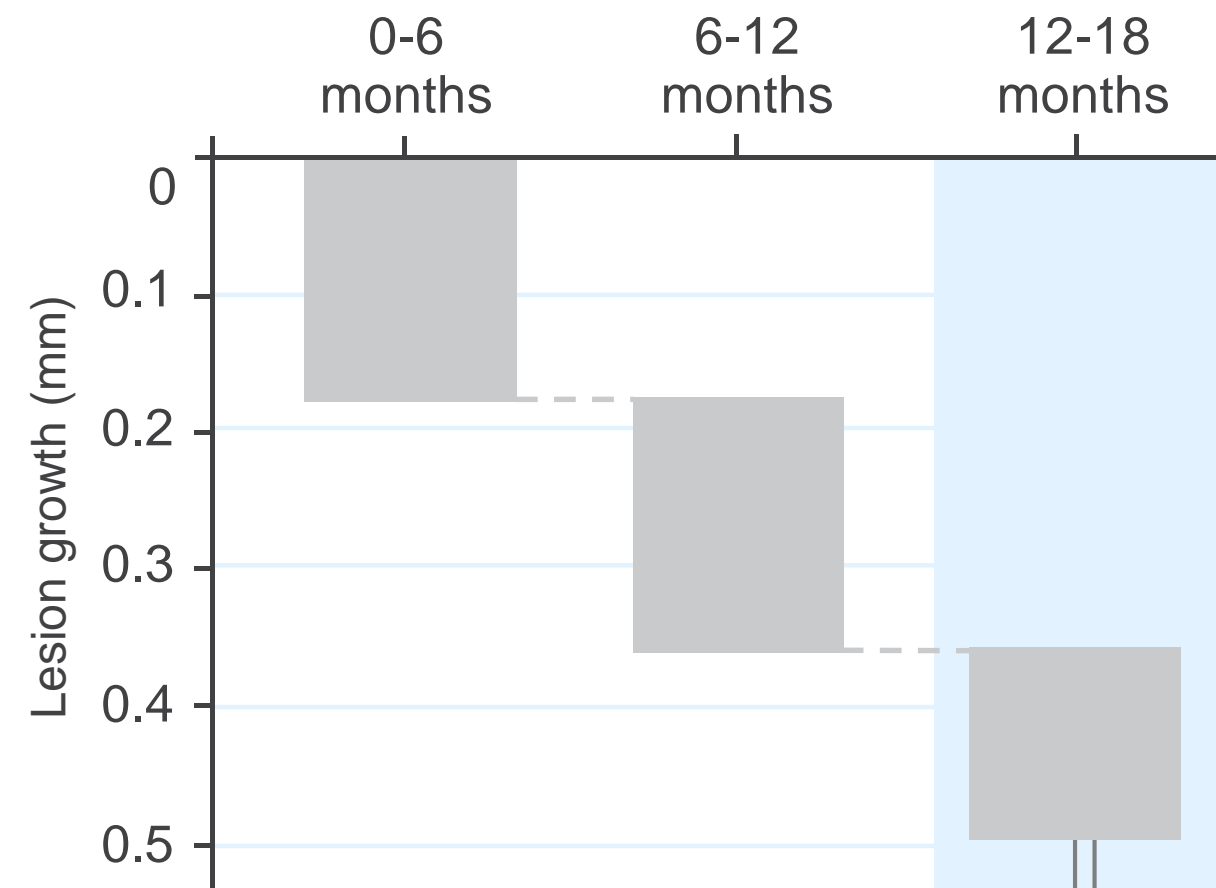
Lesion growth by six-month periods (*square root*)

- *post hoc analysis*

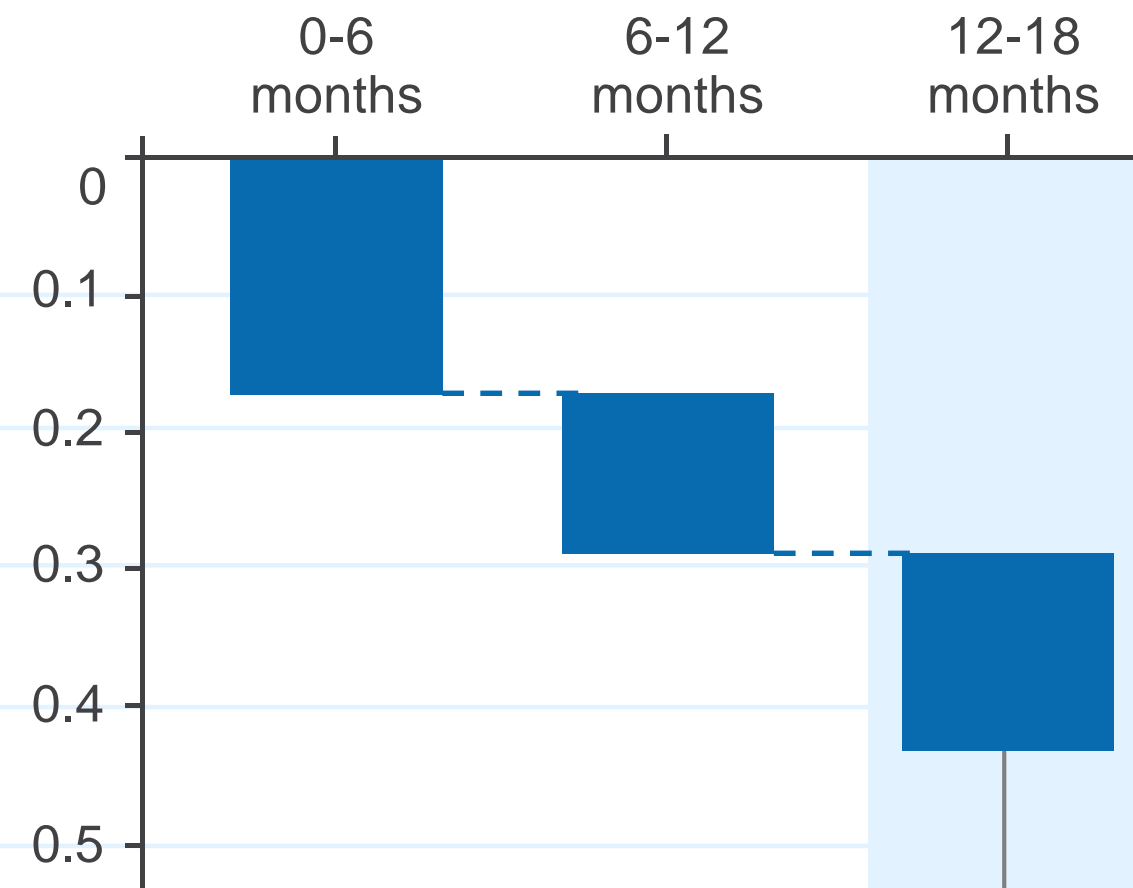
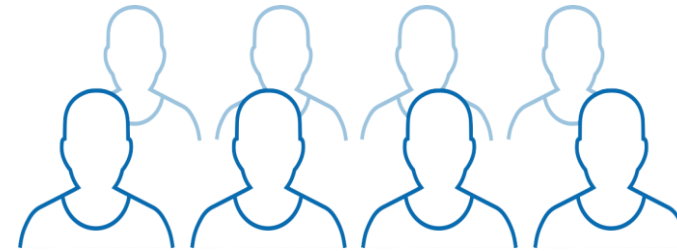


Lesion growth by six-month periods (*square root*) - *post hoc analysis*

Sham group
Sham injections

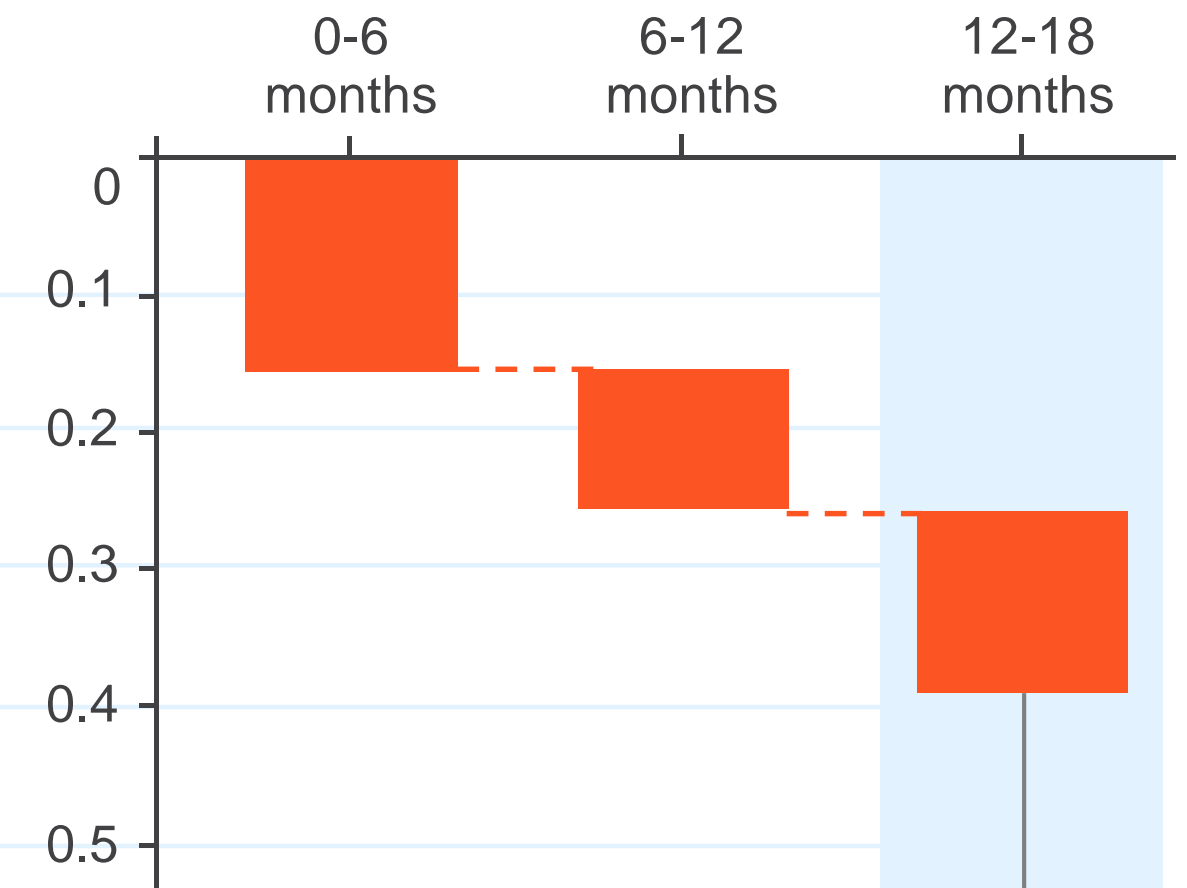


APL-2 EOM
APL-2 injections every other month



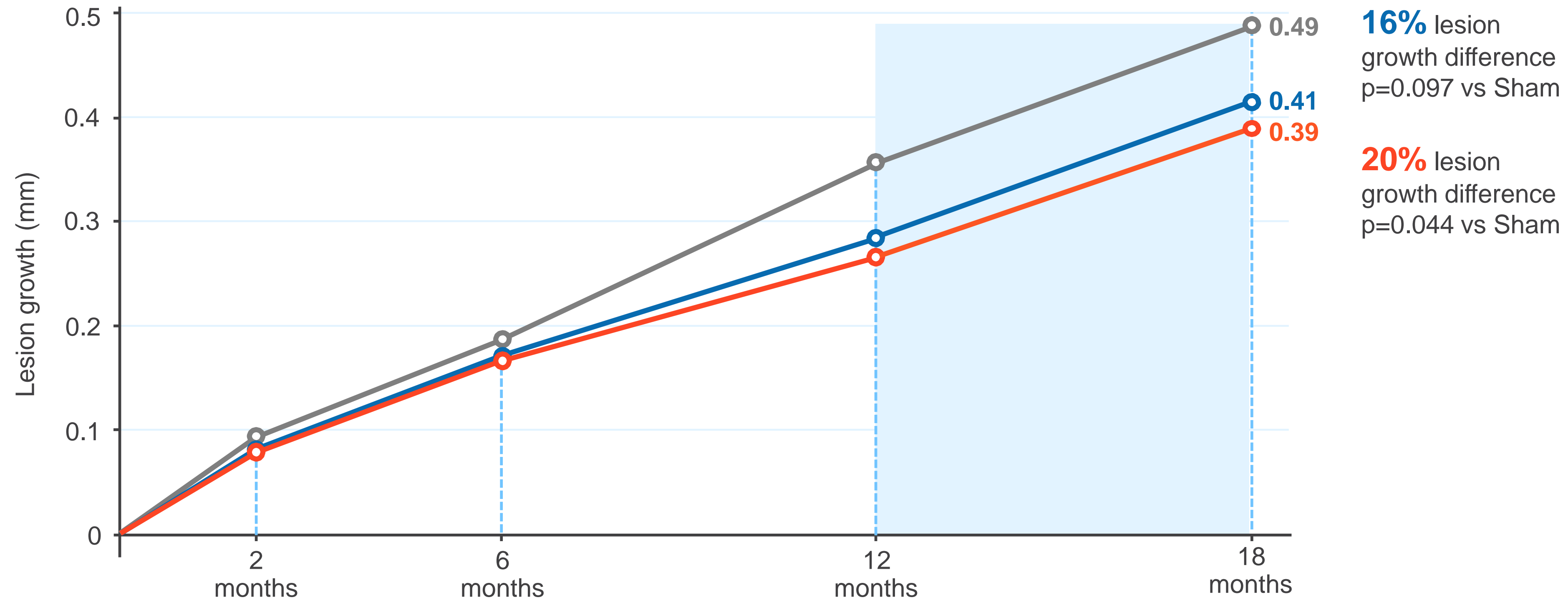
9% lesion growth difference
vs sham $p > 0.5$

APL-2 Monthly
APL-2 injections every month



12% lesion growth difference
vs sham $p = 0.47$

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

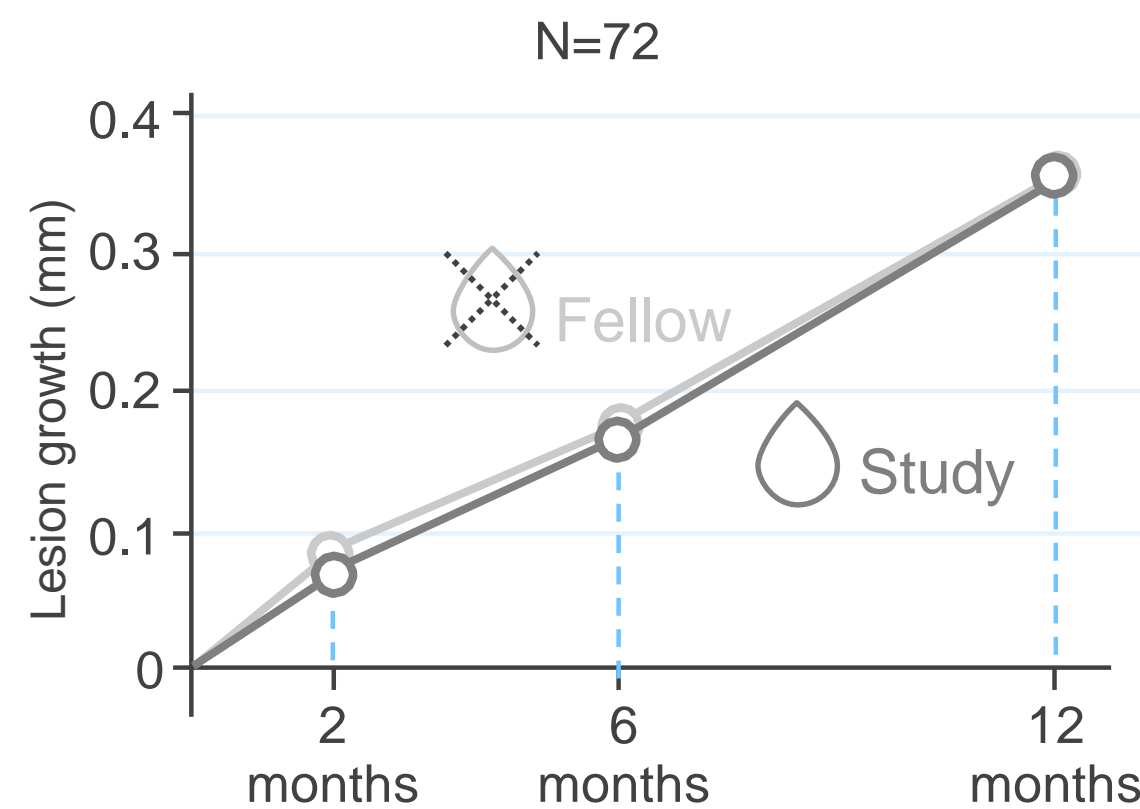


GA growth comparison: fellow eye vs study eye

- *post hoc analysis*

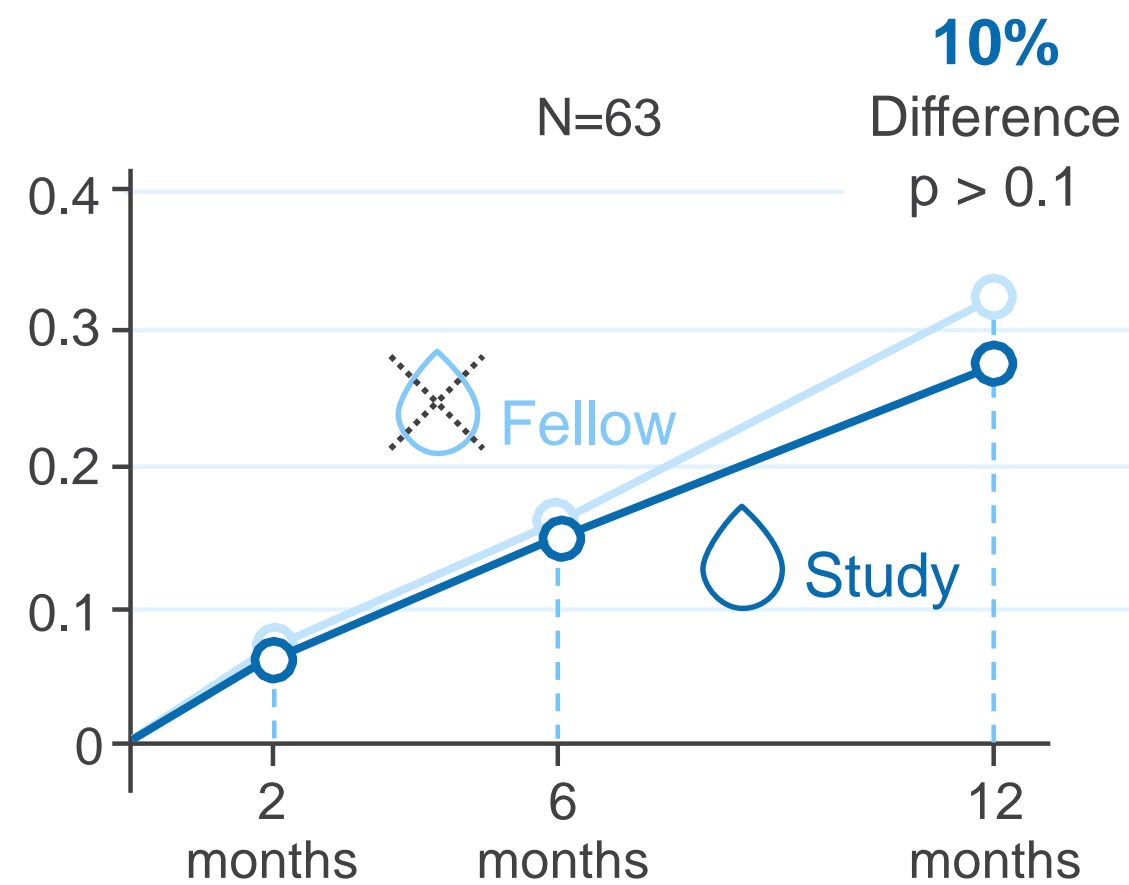
Sham group

Sham injections



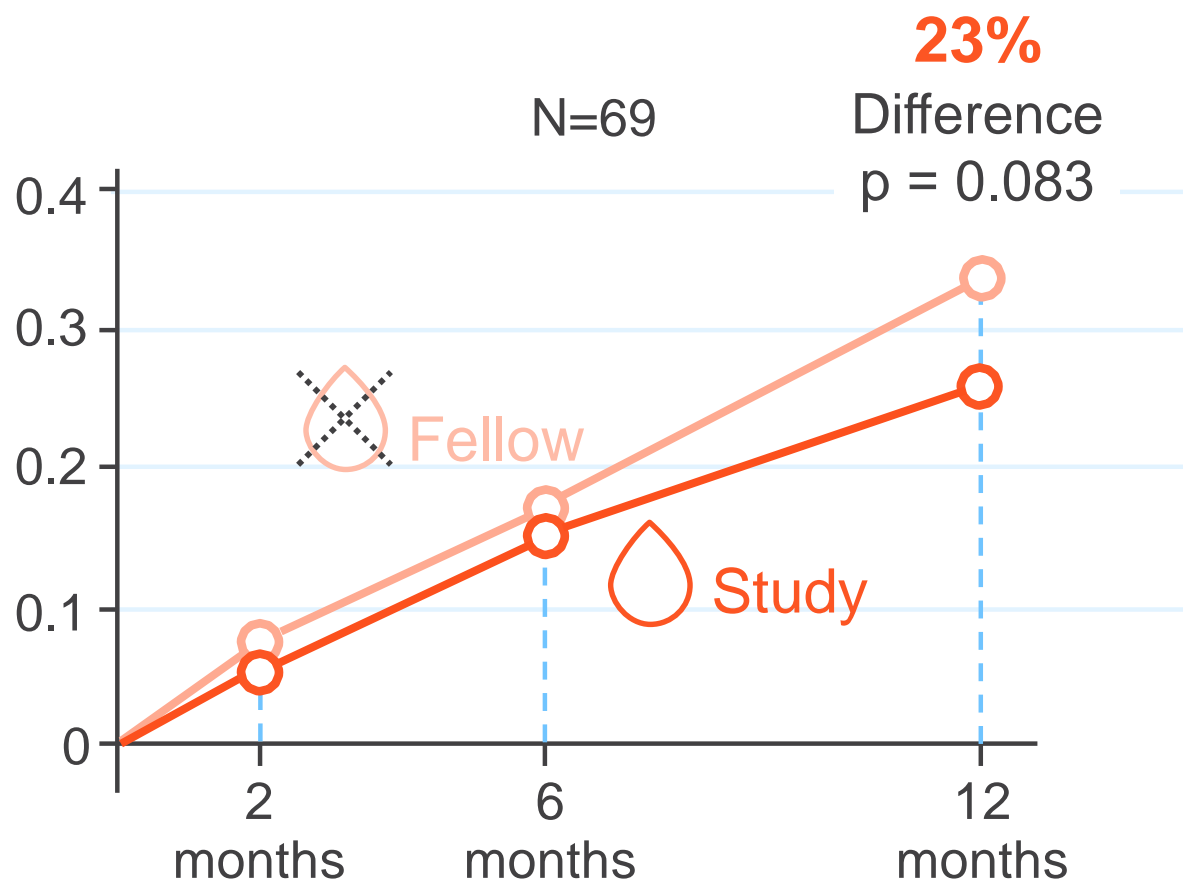
APL-2 EOM

APL-2 injections every other month



APL-2 Monthly

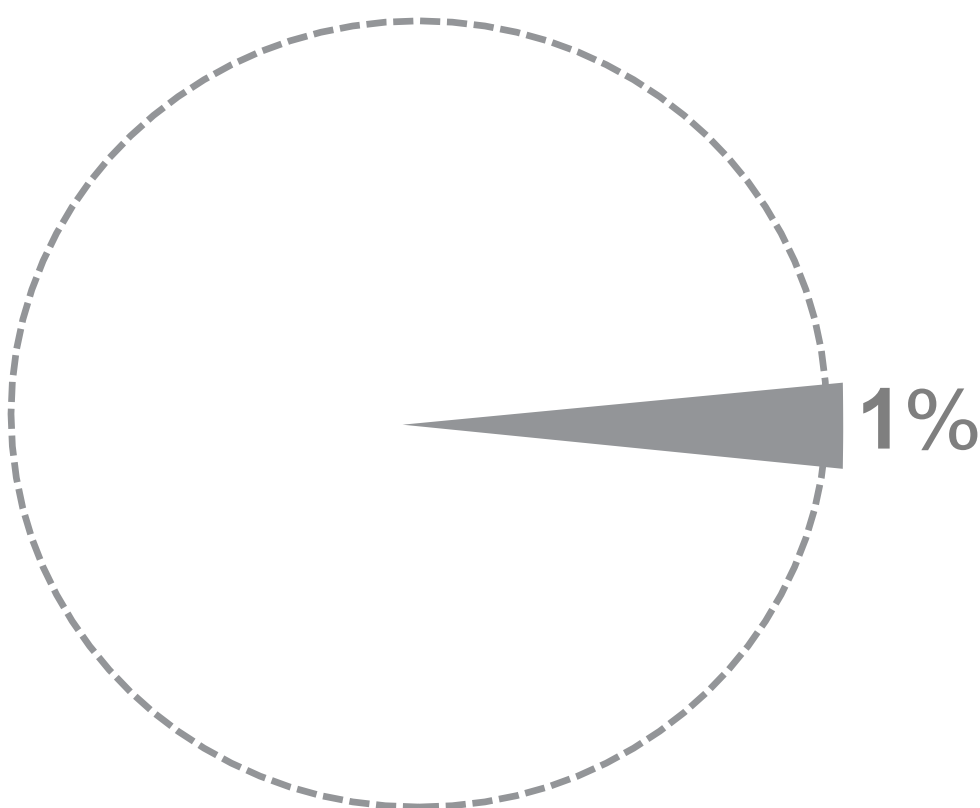
APL-2 injections every month



New onset wet AMD

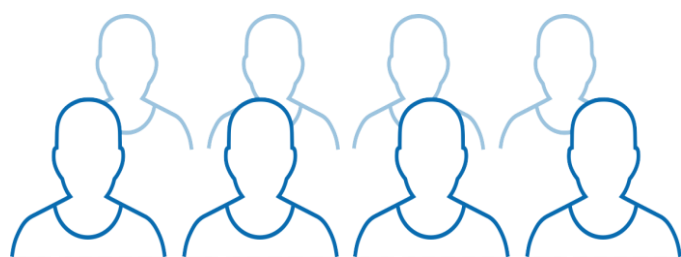
Sham group

Sham injections



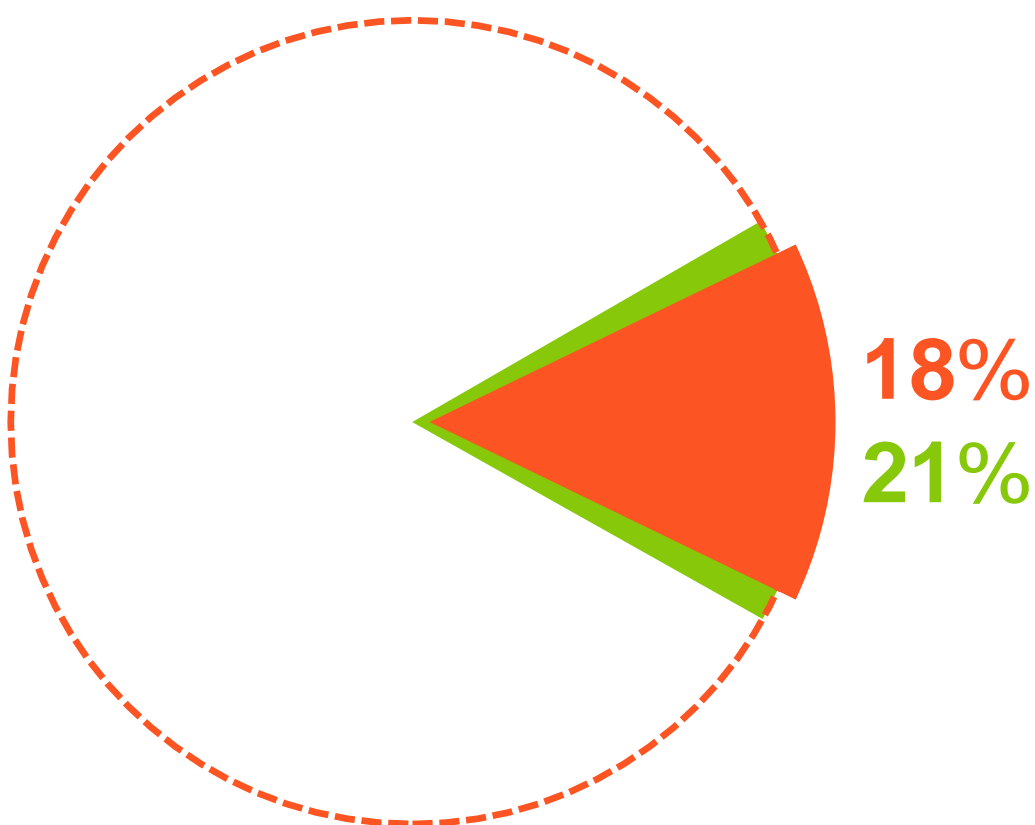
APL-2 EOM

APL-2 injections every other month



APL-2 Monthly

APL-2 injections every month



■ ■ ■ 12-month outcomes

■ 18-month outcomes

New onset wet AMD

FILLY:
38% of enrolled patients had wet AMD in the non-study eye (fellow eye), balanced between the three groups
6 patients developed wet AMD in the 12-18 month non-treatment period (5/6 had fellow eye wet AMD)

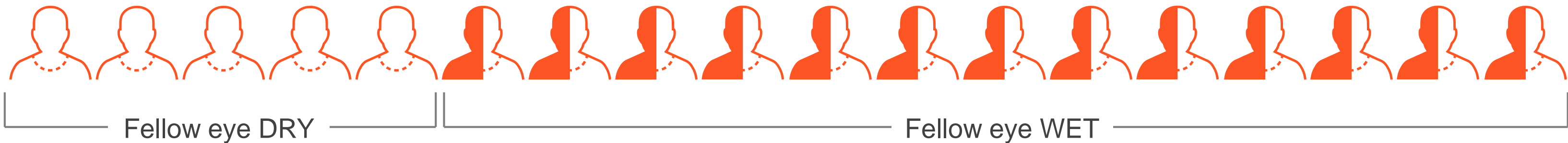
Sham group
Sham injections



APL-2 EOM
every other month



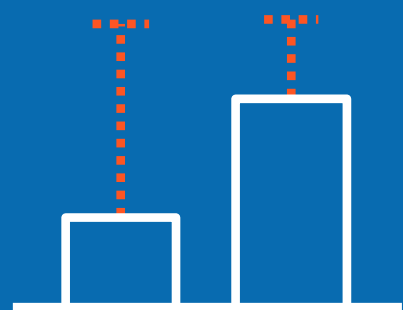
APL-2 Monthly
every month



FILLY phase 2 trial



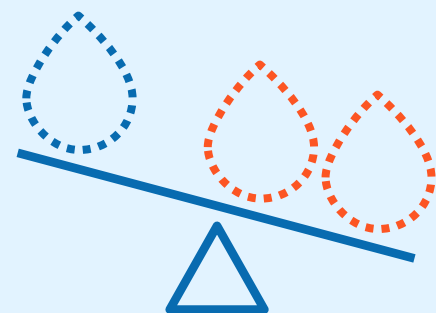
Preventing complement activation by blocking C3



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



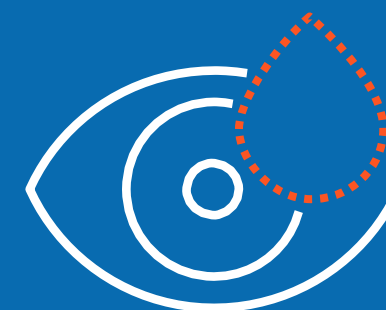
Results correlated to treatment frequency with increasing effect size over time



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



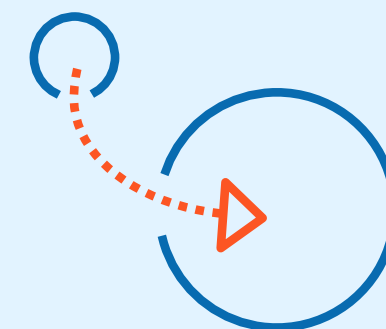
No specific genotype driving results



Further confidence in results from intra-patient control



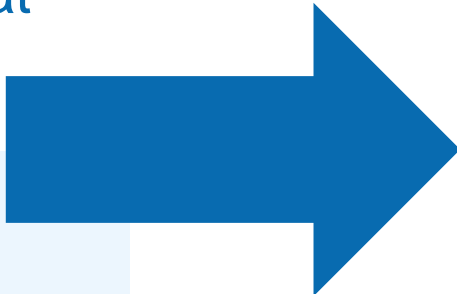
Phase 3 design finalized



Upon discontinuation of APL-2, treatment effect is maintained through 18 months

DERBY & OAKS - Phase 3 Program Overview

2 Global Studies	
Population	Patients with Geographic Atrophy secondary to AMD
1° Endpoint	Change in total area of GA lesion(s) based on FAF at Month 12
Design	Double Masked, Randomized 2:1:2:1
Treatment	15 mg/0.1 mL Intravitreal Injection vs. Sham Injection
Sample size	600 Subjects from approx. 100 multinational sites per study



Each study will have the following design:

Screening - R:2:1:2:1

———— 2 years ————

APL-2 Monthly N = 200

APL-2 EOM N = 200

Sham Monthly N = 100

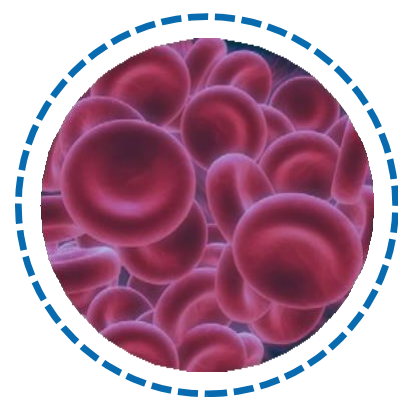
Sham EOM N = 100

Key milestones for 2018



GA:

Phase 2: 18 month safety & efficacy data

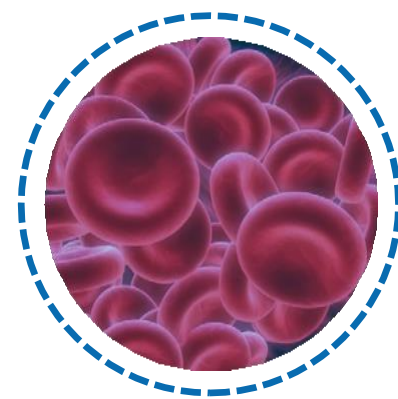


PNH:

Phase 1b: monotherapy expansion

Phase 1b: Soliris weaning in add-on study

Start of Phase 3 program



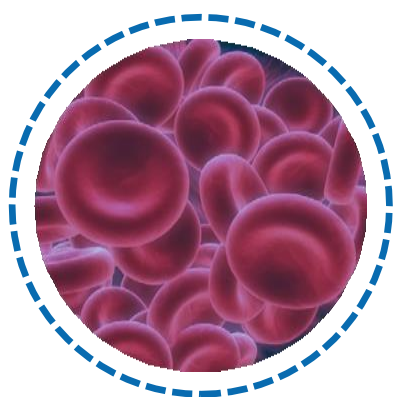
AIHA:

Preliminary data in CAD & wa-AIHA



GA:

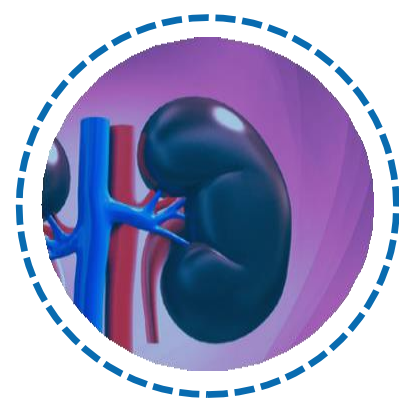
Start of Phase 3 program



AIHA:

Phase 2: POC data in CAD

Phase 2: POC data in wa-AIHA



CDN:

Phase 2: POC monotherapy data

A group of people are gathered outdoors for a birthday celebration. In the center, a woman with blonde hair, wearing an orange dress, is blowing out candles on a chocolate cake. She is surrounded by family members, including a man in a red shirt, a young man in a grey shirt, a woman in a yellow shirt, and an older man in a white shirt. A young girl in the foreground is holding a small blue gift box. To the right, a woman is holding a bouquet of yellow tulips. The background shows a white building with a grid of windows. The word "Hope" is overlaid in large white letters across the center of the image.

Hope

The background is a solid blue color with a repeating pattern of white chemical structures. These structures include various organic molecules such as benzene rings, alcohols, and esters, rendered in a simplified, line-art style.

Thank you

design by
THEORIA
CREATIVE