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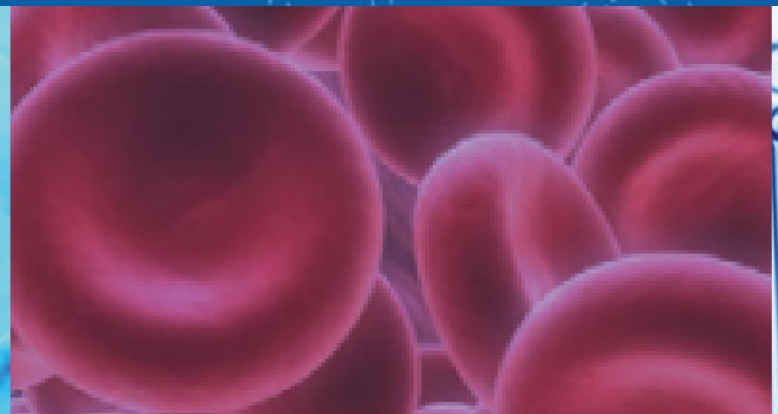
Welcome to Apellis at the Apella

R&D Day 2018



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Welcome & Today's Objectives

Cedric Francois, MD, PhD,
Founder and Chief Executive Officer,
Apellis Pharmaceuticals

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’ Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission on April 30, 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.

2018 R&D Day Agenda

Introduction	
2:00 pm - 2:15 pm	Welcome and Today's Objectives
Geographic Atrophy (GA)	
2:15 pm - 2:30 pm	GA: Disease Burden, Health Care Resource Use and Progression
2:30 pm - 2:35 pm	GA: Patient Perspective
2:35 pm - 2:50 pm	APL-2 in GA: Phase II FILLY Trial 18-Month Results
2:50 pm - 3:05 pm	APL-2 in GA: Phase III Program
3:05 pm - 3:25 pm	Geographic Atrophy Q&A
3:25 pm - 3:35 pm	10 Minute Break

Paroxysmal Nocturnal Hemoglobinuria (PNH)	
3:35 pm - 3:50 pm	APL-2 in PNH: Unmet Need & PADDOCK Trial Update
3:50 pm - 3:55 pm	PNH: Patient Perspective
3:55 pm - 4:10 pm	APL-2 in PNH: PHAROAH Trial Update & Phase III Program
4:10 pm - 4:30 pm	Paroxysmal Nocturnal Hemoglobinuria Q&A
Early Stage Pipeline	
4:30 pm - 4:45 pm	Early Stage Pipeline: APL-2 in AIHA and CDN
Closing Remarks	
4:45 pm – 5:00 pm	Closing Remarks

Cocktail Reception
5:00 pm – 6:00 pm

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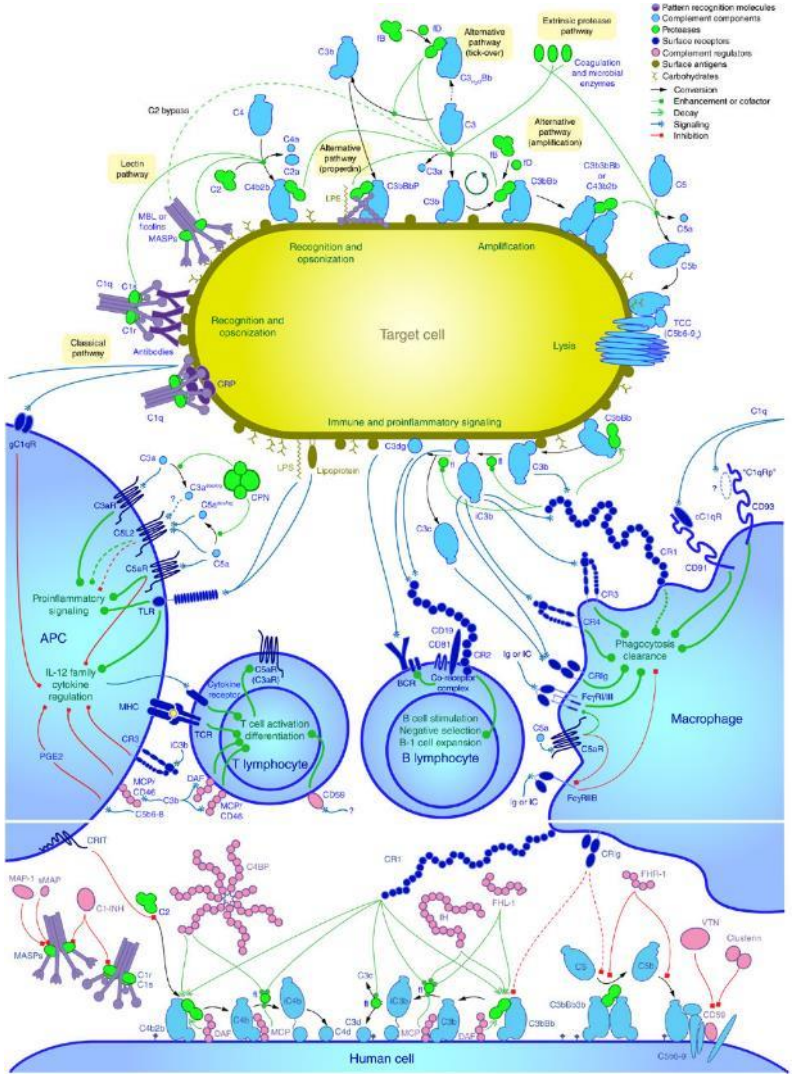
The Complement System



Complement central to innate immunity

Innate

"Shock and Awe"

Adaptive

"Self vs Non-Self"

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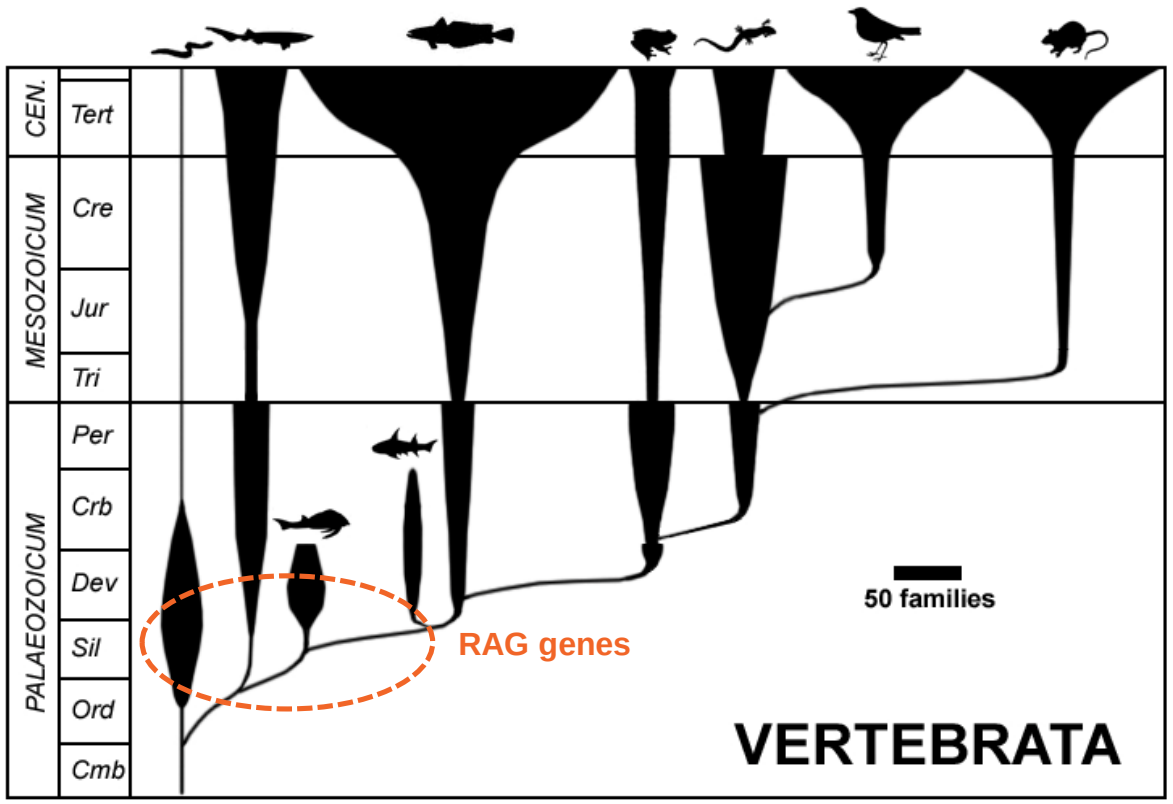
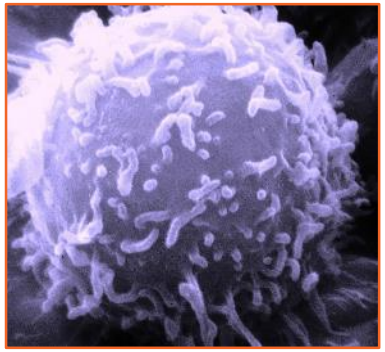
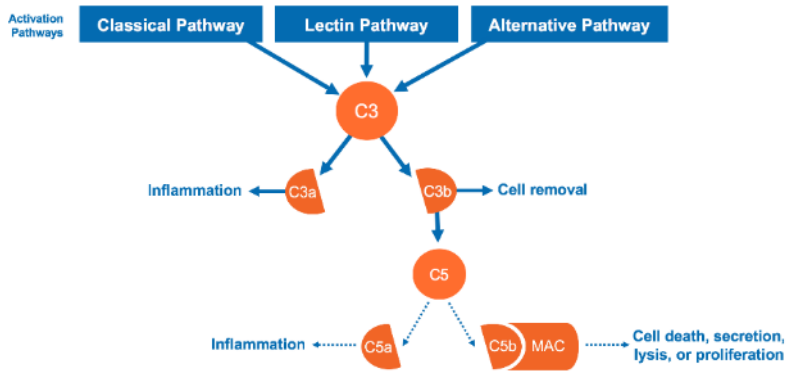
Rise of Adaptive Immunity







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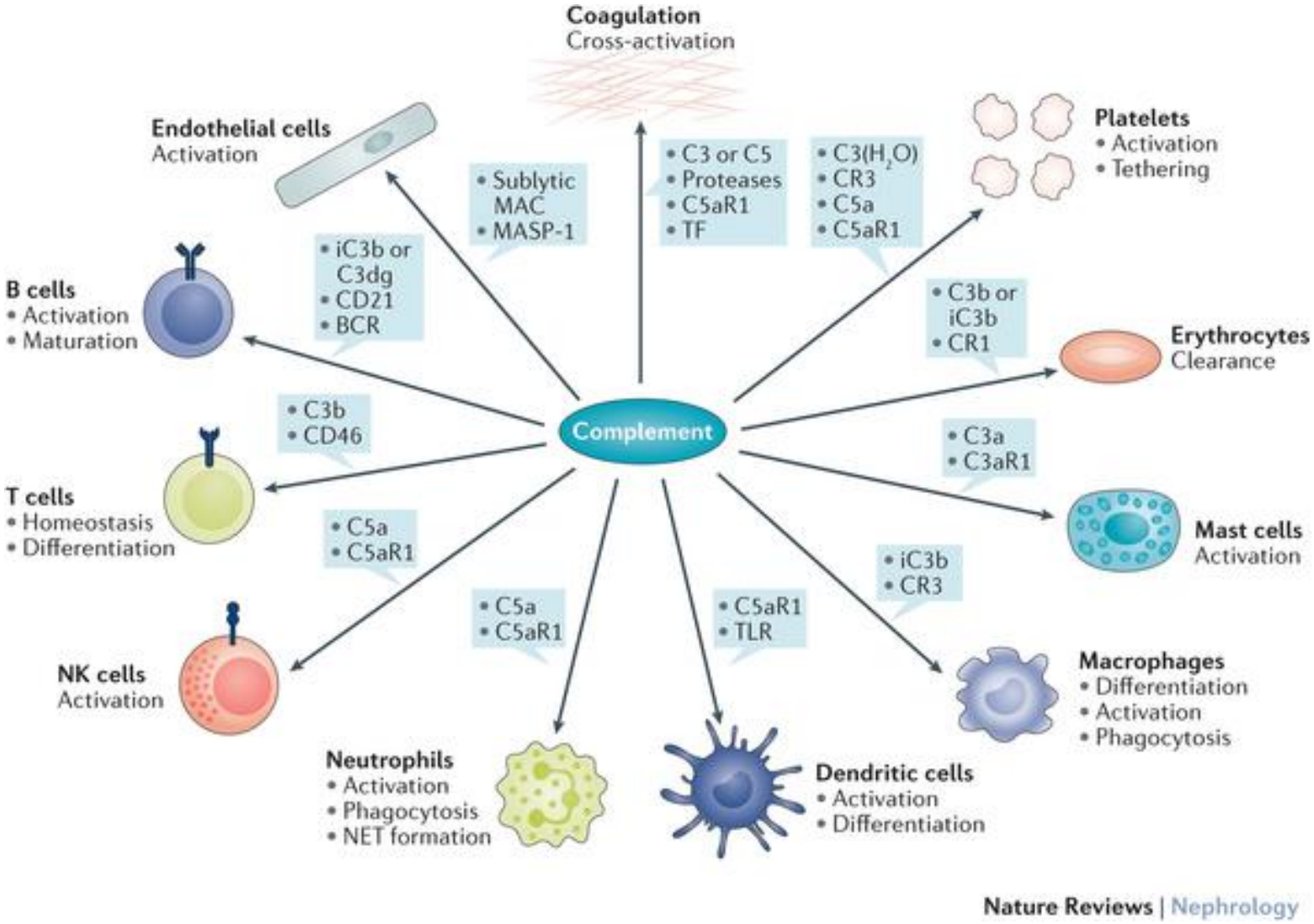
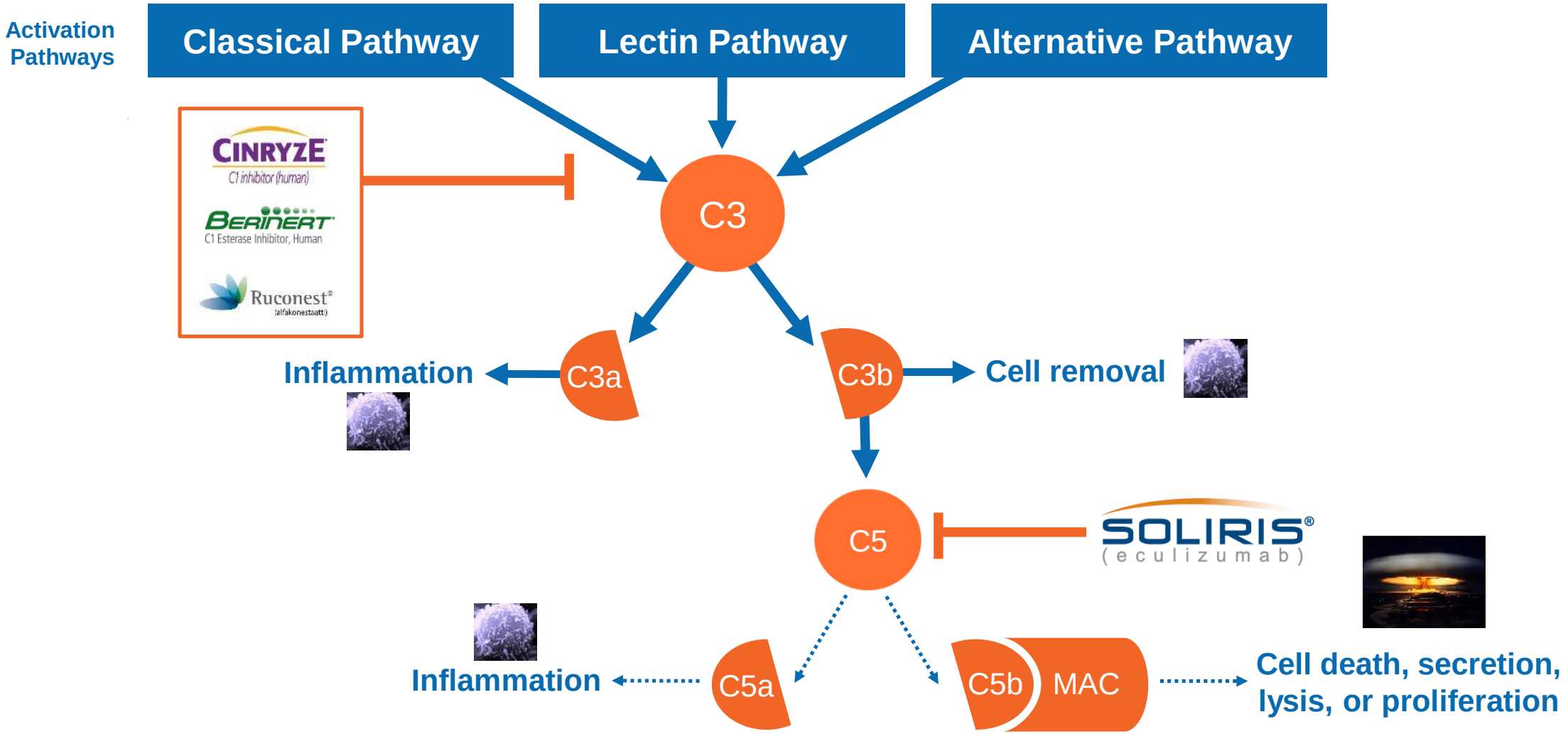
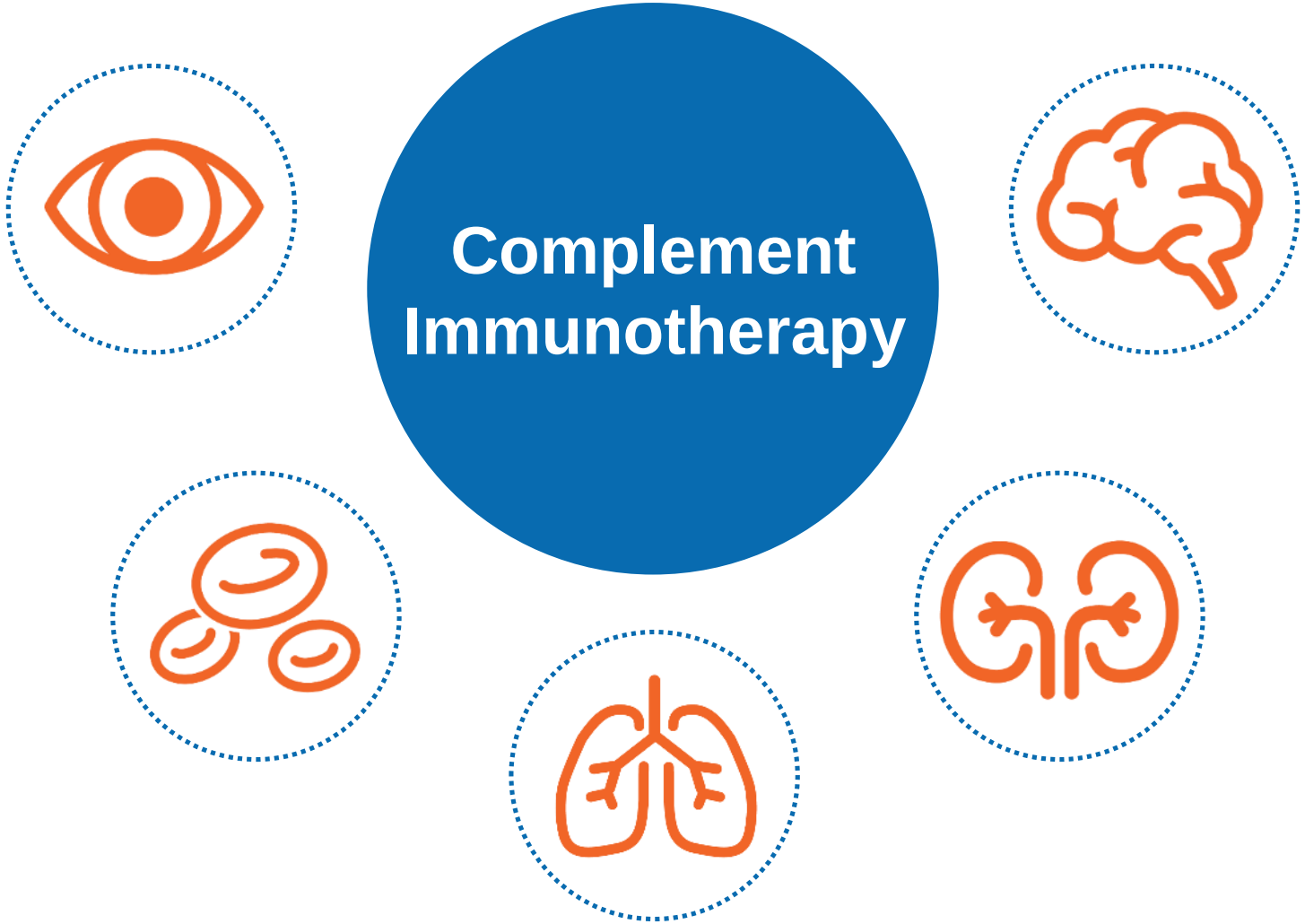
Roles of Complement



Approved Complement Inhibitors

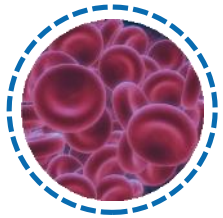


Therapeutic Potential



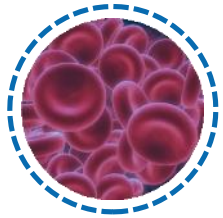
Key Milestones for 2018

GA:
Phase 2: 18 month safety & efficacy data

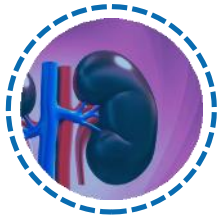


PNH:
Phase 1b: monotherapy expansion (interim)

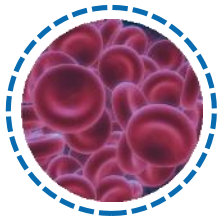
Phase 1b: Soliris weaning in add-on study



AIHA:
Phase 2: POC monotherapy data



CDN:
Phase 2: POC monotherapy data



PNH:
Start of Phase 3 program

GA:
Start of Phase 3 program

H1 H2

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GEOGRAPHIC ATROPHY (GA)

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Geographic Atrophy: Disease Burden, Health Care Resource Use and Progression

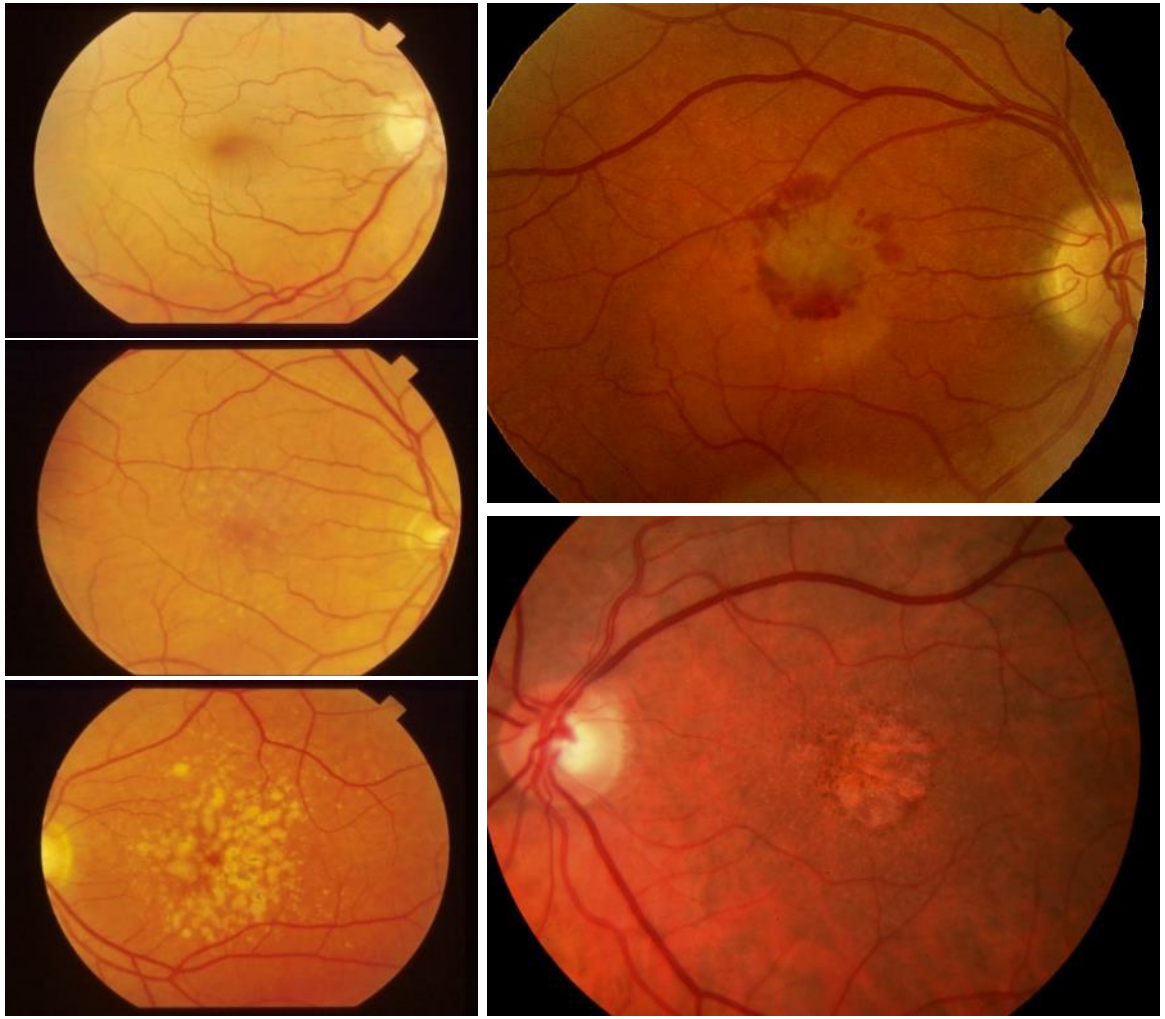
Usha Chakravarthy, MD, PhD,
Professor of Ophthalmology and
Consultant Ophthalmic Surgeon,
Queen's University of Belfast

Key Takeaways

GA is a cause of similar levels of visual morbidity as CNV

- Real-world data confirm that GA is a progressive disease and is associated with high levels of visual impairment, with impacts on mobility and independence
- Proportion of eyes progressing to blindness is greater than that recorded in previous clinical studies
- GA and CNV are manifestations of the same disorder with specific temporal relationships

Early and late age-related macular degeneration



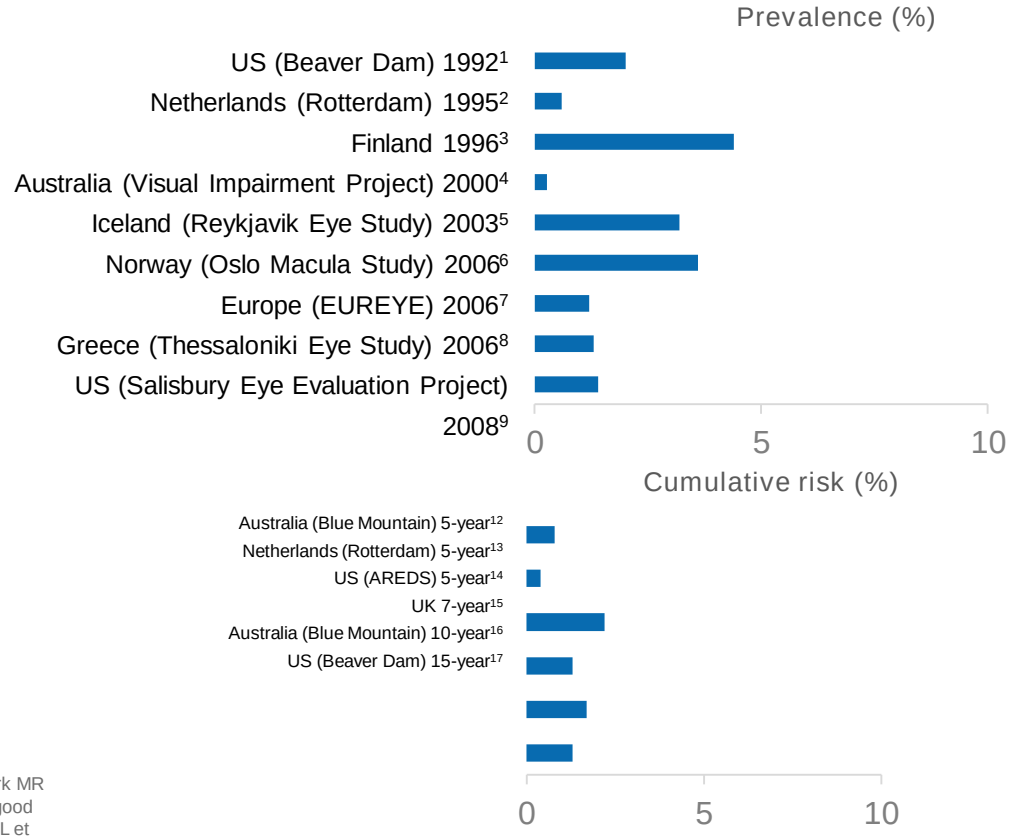
Introduction

- Geographic atrophy (GA) is an advanced manifestation of age-related macular degeneration (AMD) characterised by progressive and irreversible loss of the retinal pigment epithelium (RPE), photoreceptors and underlying choriocapillaris[1,2]
- Patients experience visual function abnormalities, such as difficulty with reading and seeing in low-light conditions
- Major cause of legal blindness: ~26% in UK, ~20% in North America
- No approved treatment available[2]

1. Boyer DS et al. Retina. Nov 29 2016 [Epub ahead of print]; 2. Holz FG et al. Ophthalmology. 2014;121:1079-91.

Prevalence and Incidence of GA

- GA affects >5 million people worldwide[10]
- Variable prevalence reported in the different studies[10,11]
  - Particularly when photographic grading has not been used
- GA prevalence
  - Quadruples per decade increase in age ≥50 years[11]
- A meta-analysis estimated annual incidence of GA at 1.6 cases per 1000, equivalent to 160,000 new cases per year[18]



1. Klein R et al. Ophthalmology. 1992;99:933-43; 2. Vingerling JR et al. Ophthalmology. 1995;102:205-10; 3. Hirvela H et al. Ophthalmology. 1996;103:871-7; 4. VanNewkirk MR et al. Ophthalmology. 2000;107:1593-600; 5. Jonasson F et al. Arch Ophthalmol. 2003;121:379-85; 6. Björnsson OM et al. Acta Ophthalmol Scand. 2006;84:636-41; 7. Augood CA et al. Arch Ophthalmol. 2006;124:529-35; 8. Topouzis F et al. Am J Ophthalmol. 2006;142:1076-9; 9. Bressler SB et al. Arch Ophthalmol. 2008;126:241-5; 10. Wong WL et al. Lancet Glob Health. 2014;2:e106-16; 11. Rudnicka AR et al. Ophthalmology. 2012;119:571-80; 12. Mitchell P et al. Ophthalmology. 2002;109:1092-7; 13. van Leeuwen R et al. Arch Ophthalmol. 2003;121:519-26; 14. Davis MD et al. Arch Ophthalmol. 2005;123:1484-98; 15. Sparrow JM et al. Eye. 1997;11:315-24; 16. Wang JJ et al. Ophthalmology. 2007;114:92-8; 17. Klein R et al. Ophthalmology. 1997;104:7-21; 18. Rudnicka AR et al. Am J Ophthalmol. 2015;160:85-93.e3.
AMD, age-related macular degeneration; GA, geographic atrophy; nAMD, neovascular age-related macular degeneration.

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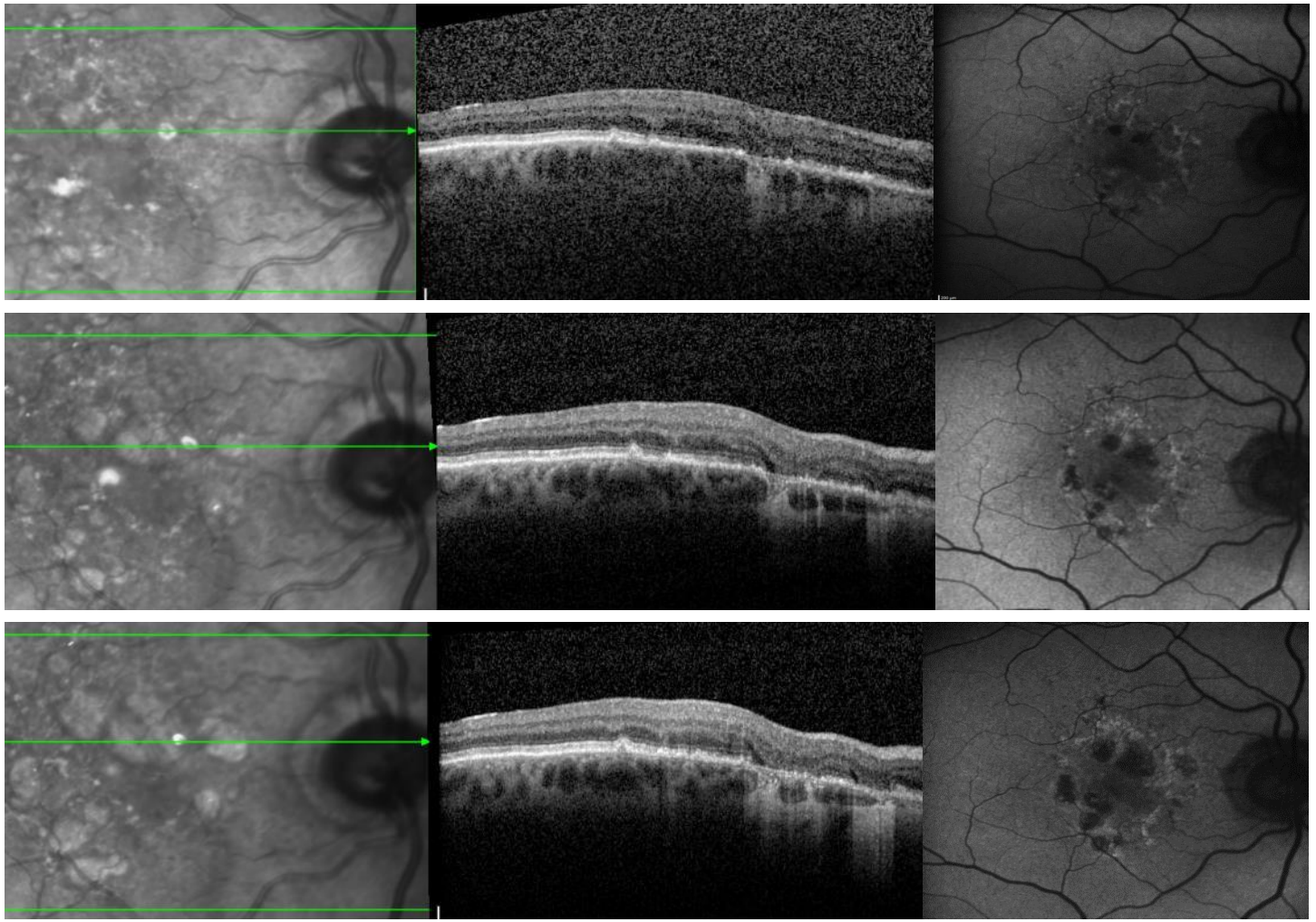
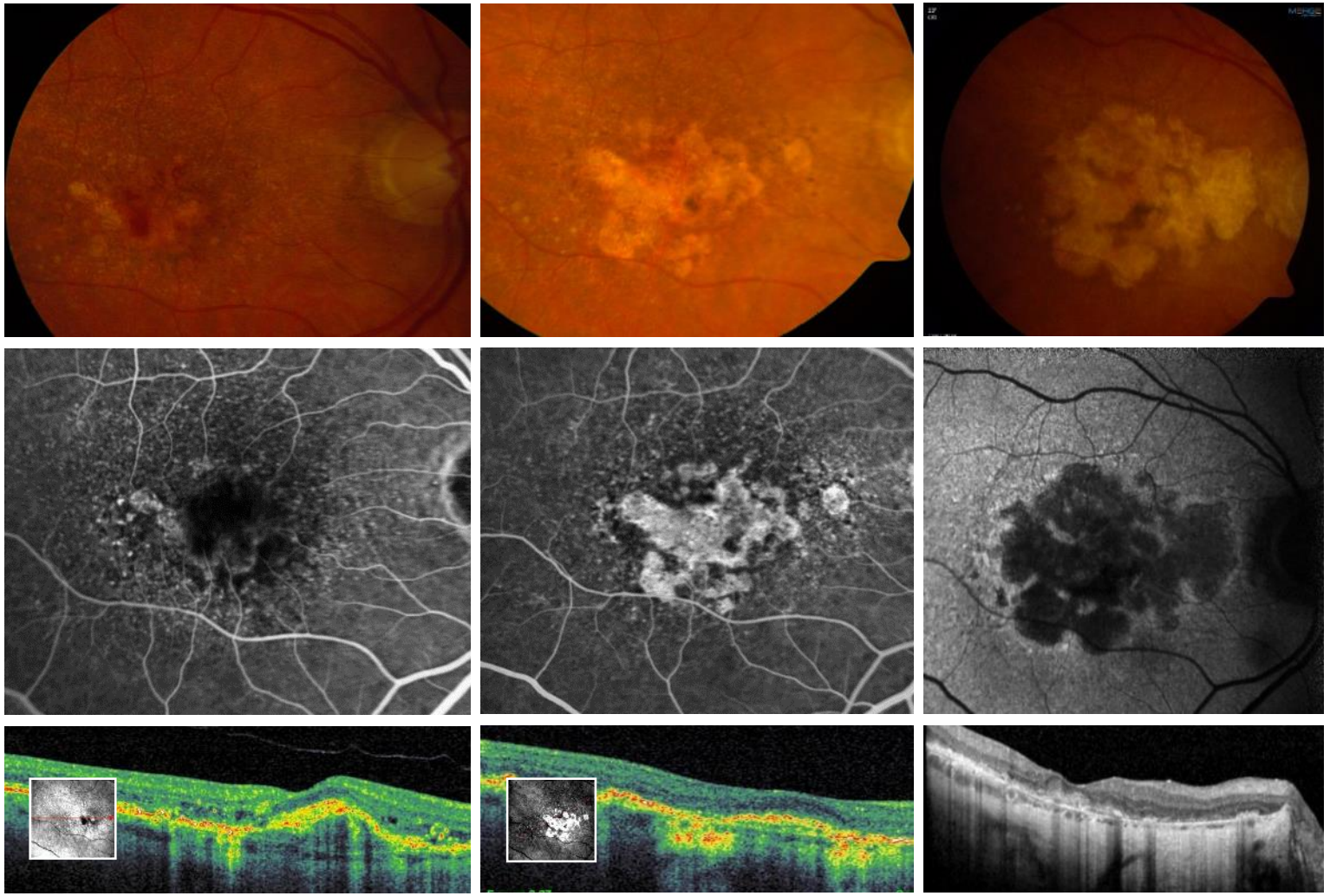
Rapid progression over 18M to multifocal GA



GA is responsible for sight loss in patients with wet AMD despite treatment with anti VEGF

98% of wet AMD patients developed GA, despite the treatment with anti-VEGF therapy when followed for 7 years as showed by the Seven-UP study



Ophthalmology. 2013 Nov;120(11):2292-9. doi: 10.1016/j.ophtha.2013.03.046. Epub 2013 May 3. Seven-year outcomes in ranibizumab-treated patients in ANCHOR, MARINA, and HORIZON: a multicenter cohort study (SEVEN-UP). Rofagha S1, Bhisitkul RB, Boyer DS, Sadda SR, Zhang K; SEVEN-UP Study Group.

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Rationale for UK EMR study

- Recent evidence suggesting that GA is the default pathway in AMD
- Increasing awareness that early forms of GA may be more common than previously recognised
- The extent of visual impact of GA may have been underestimated
  - The frequency of severe visual impairment /blindness arising from GA
  - The severity of visual impairment and consequent handicap due to GA

The UK EMR AMD Study

Study Design	• Retrospective analysis of a clinical dataset • Study plan and aims defined prior to data cut • EMR contains prospectively defined data fields (similar to electronic case report forms used in clinical trials rather than a retrospective chart review)
Data Source	• Medisoft EMR
Site Selection	• 10 NHS retina clinics contributed to the study
Period Covered	• Data cut made in Feb 2016 (period covered Oct 2000–Feb 2016) • Periods of EMR usage varied by centre as not all centres implemented EMR at the same time

AMD, age-related macular degeneration; EMR, electronic medical record; NHS, National Health Service. UK EMR GA study Chakravarthy et al Ophthalmology

Study Population – GA Cohorts

Anonymised data from 83,425 patients (diagnosis of any AMD)
10 UK retinal centres between 2000 and 2016

Disease definition

Cases with at least 1 eye with a clinical record of GA recorded at any visit **N = 11,240 (13.5%)**

Main Exclusions:
- Age at index <50 years
- CNV in both eyes at index date
- Study eye with <30 days' follow-up
- No information for the fellow eye in the EMR system, or fellow eye is not classifiable
- Missing VA data ±90 days of index date

Eligible GA cohort - 4769 (5.7%)

GA:GA 1901 (39.9%)	GA:CNV 1696 (35.6%)	GA:Early AMD 1172 (24.6%)
Both study and fellow eye with GA	Study eye with GA; fellow eye with CNV	Study eye with GA; fellow eye with Early/ Intermediate AMD

AMD, age-related macular degeneration; CNV, choroidal neovascularisation; EMR, electronic medical record; GA, geographic atrophy; VA, visual acuity.

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Outcome Measures: Bilateral GA Cohort

GA:GA Cohort

Populations Analysed			
	GA:GA ALL **1901**	Did not meet definition of UK blindness at baseline* **1693**	Eligible to drive at baseline† (UK standard) **523**
Outcomes	• Progression to CNV	• Progression to blindness • Loss of ≥10 letters of VA • Loss of ≥15 letters of VA	• Progression to loss of eligibility to drive

* Blindness definition (UK): VA measurement of <20 ETDRS letters or Snellen 3/60 in the better-seeing eye.† Driving eligibility standard (UK): VA measure in better-seeing eye of >70 letters or Snellen 6/12. CNV, choroidal neovascularisation; ETDRS, Early Treatment Diabetic Retinopathy Study; GA, geographic atrophy; VA, visual acuity.

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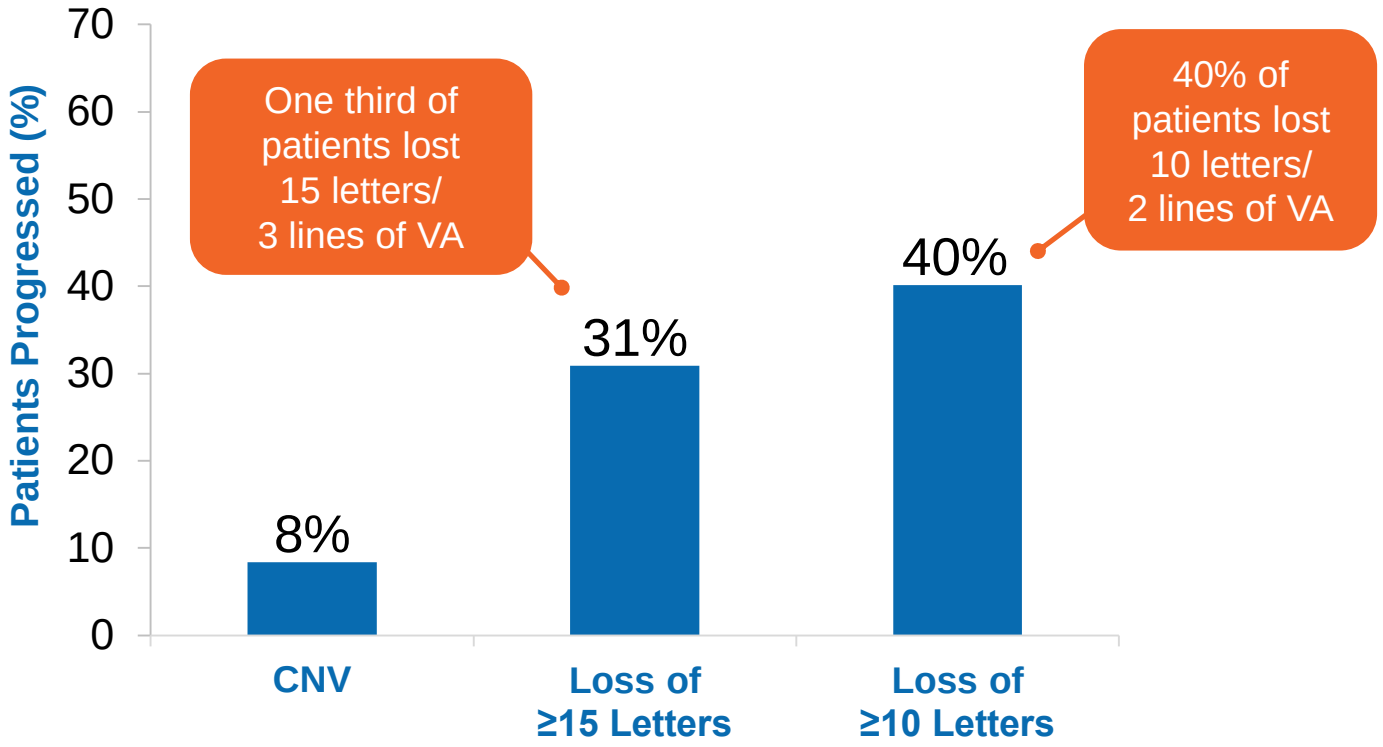
Progression to CNV and VA Loss: Bilateral GA Cohort



Study sample eligible for analyses (N)	1901	1693	1693
Median (IQR) time to outcome (years)	1.1 (0.3–2.0)	3.3 (1.5–6.2)	2.4 (1.1–5.0)

CNV, choroidal neovascularisation; GA, geographic atrophy; IQR, interquartile range; VA, visual acuity.

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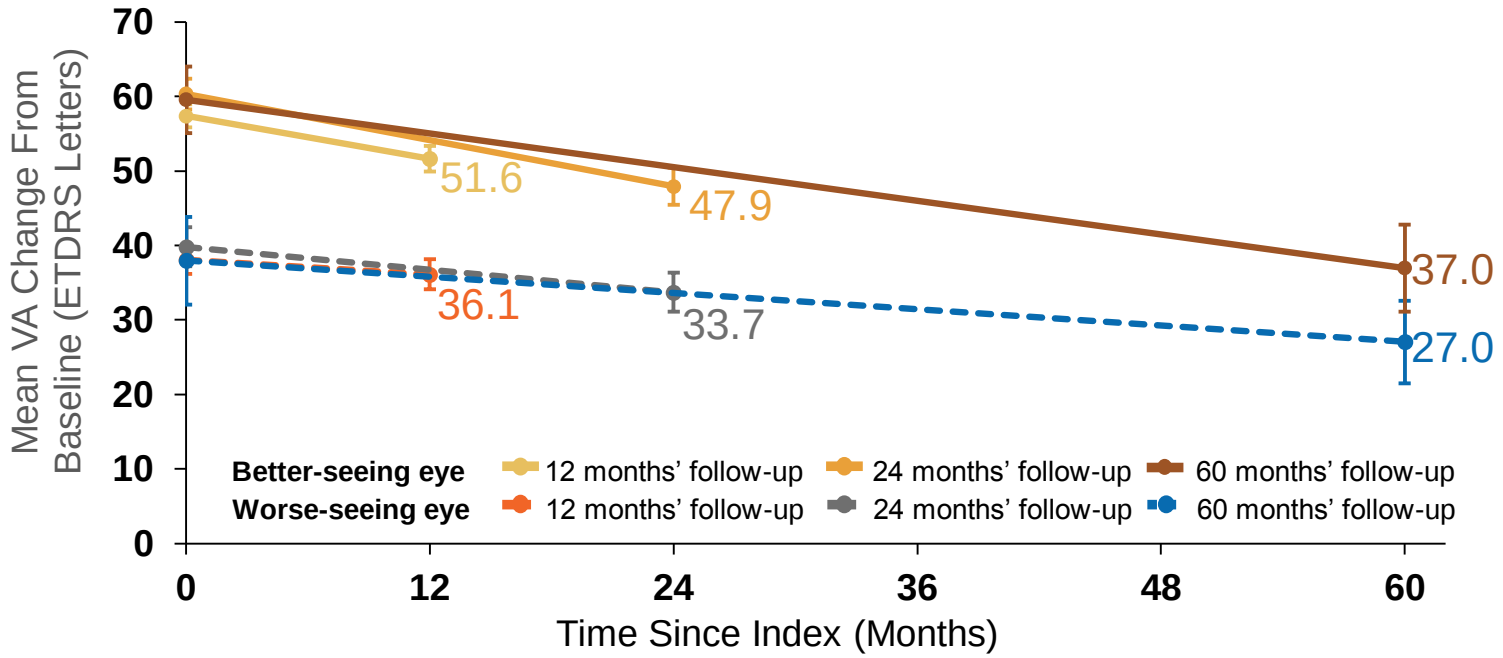
Change in Mean VA from Baseline in Better-Seeing and Worse-Seeing Eyes Over Time: Bilateral GA Cohort



Eye	Mean letter difference (95% CI)		
	12 Months	24 Months	60 Months
Better-seeing eye	**-5.7 (4.6-6.9)**	**-12.4 (10.6-14.2)**	**-22.6 (17.3-27.9)**
Worse-seeing eye	**-2.0 (0.7-3.3)**	**-6.1 (4.3-7.9)**	**-10.9 (5.5-16.4)**

For each time point, patients had to have VA measurement at index and at the specific time point of 12, 24 or 60 months post index. For the better-seeing eye, the sample comprised 726, 414 and 80 patients for the 12, 24 and 60 time points, respectively. For the worse-seeing-eye, the sample comprised 724, 413 and 80 patients for the 12, 24 and 60 time points, respectively. ETDRS, Early Treatment Diabetic Retinopathy Study; VA, visual acuity.

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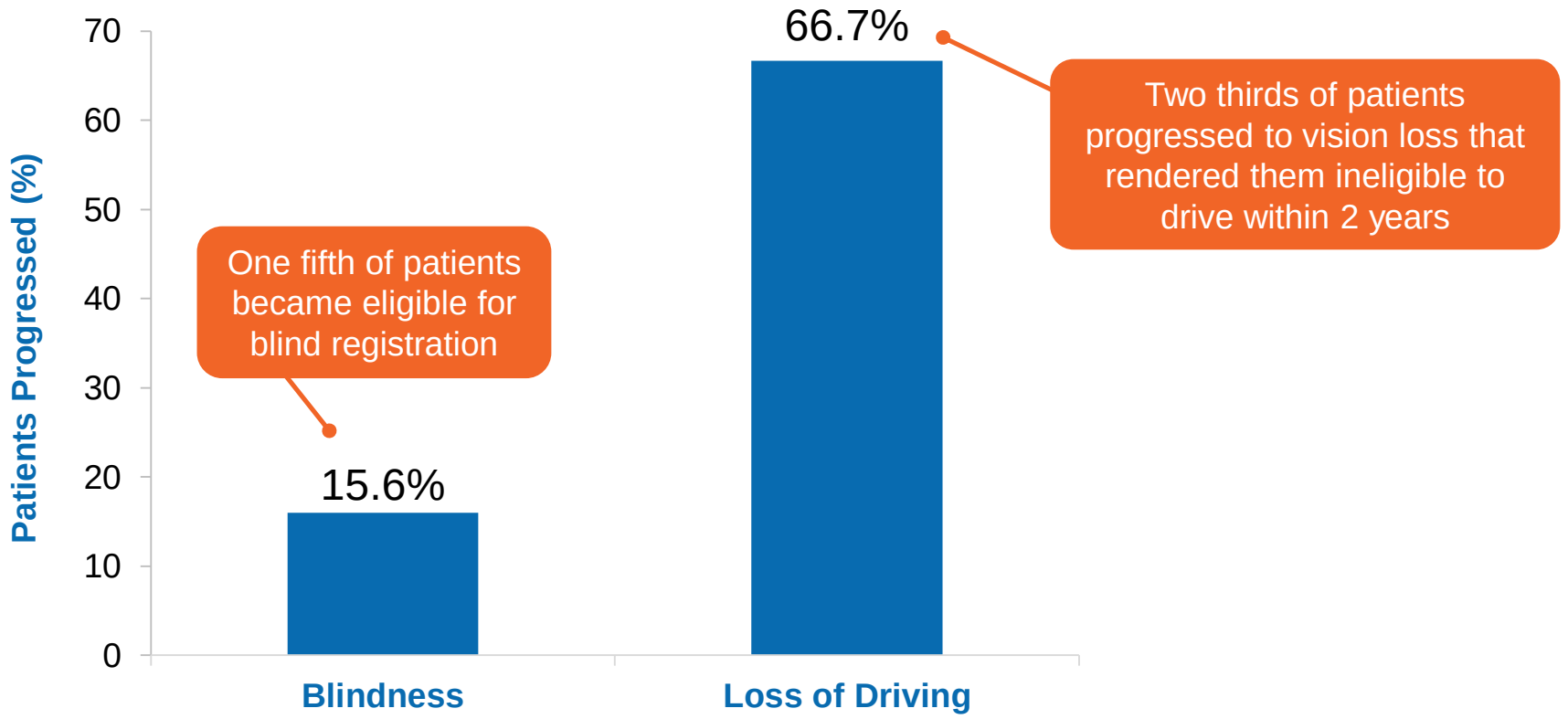
Progression of Vision To Blindness and VA Worse Than 20/40 in Better Eye: Bilateral GA Cohort*



Study sample eligible for analyses (N)	1693	523
Median (IQR) time to outcome (years)	6.2 (3.3–8.5)	1.6 (0.7-2.7)

* Results shown previously in All GA Cohorts section; included here for completeness. GA, geographic atrophy; IQR, interquartile range; VA, visual acuity.

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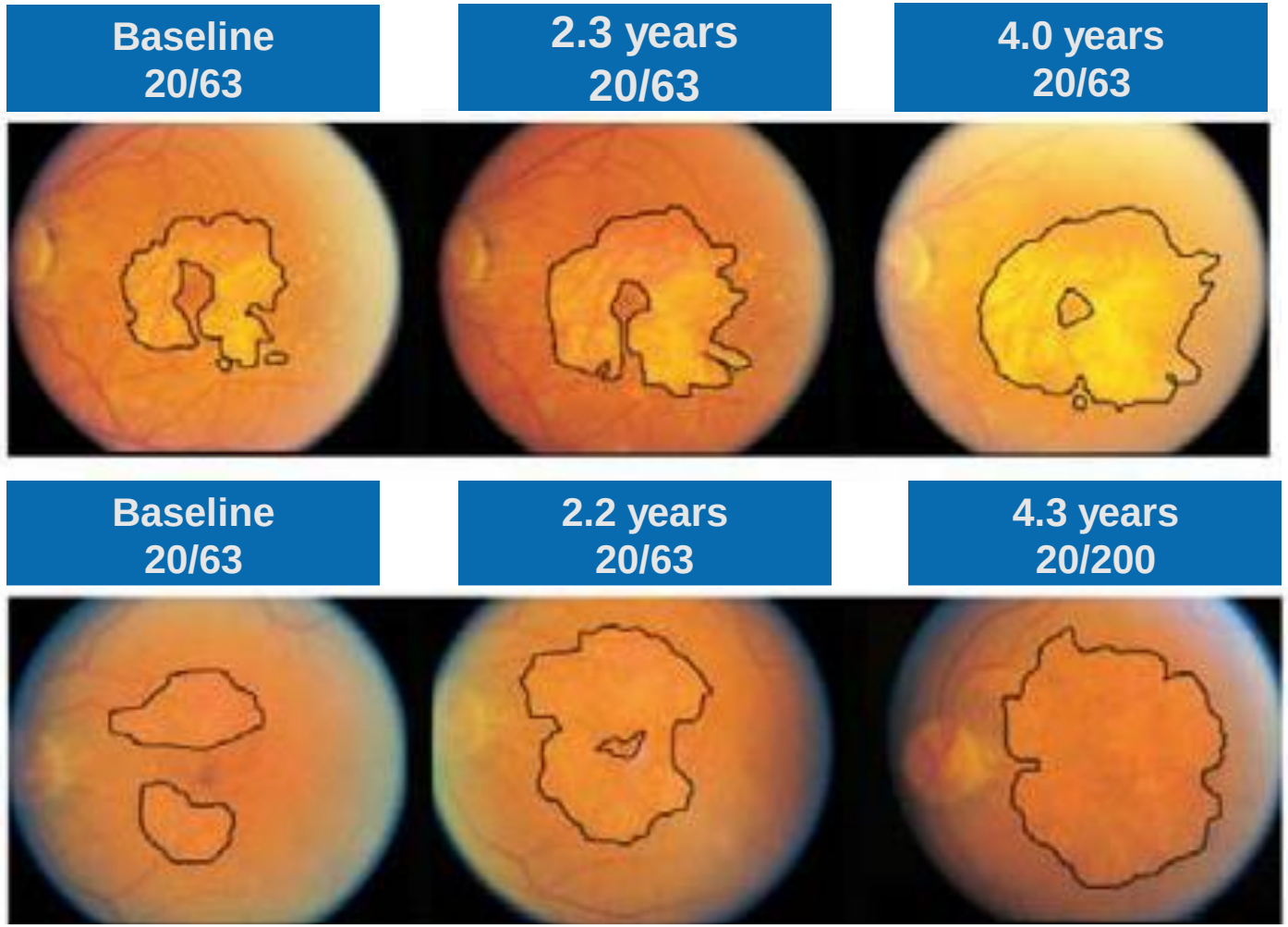
Progression of GA
Distance VA may not change until GA covers fovea



Courtesy of Frank Holz

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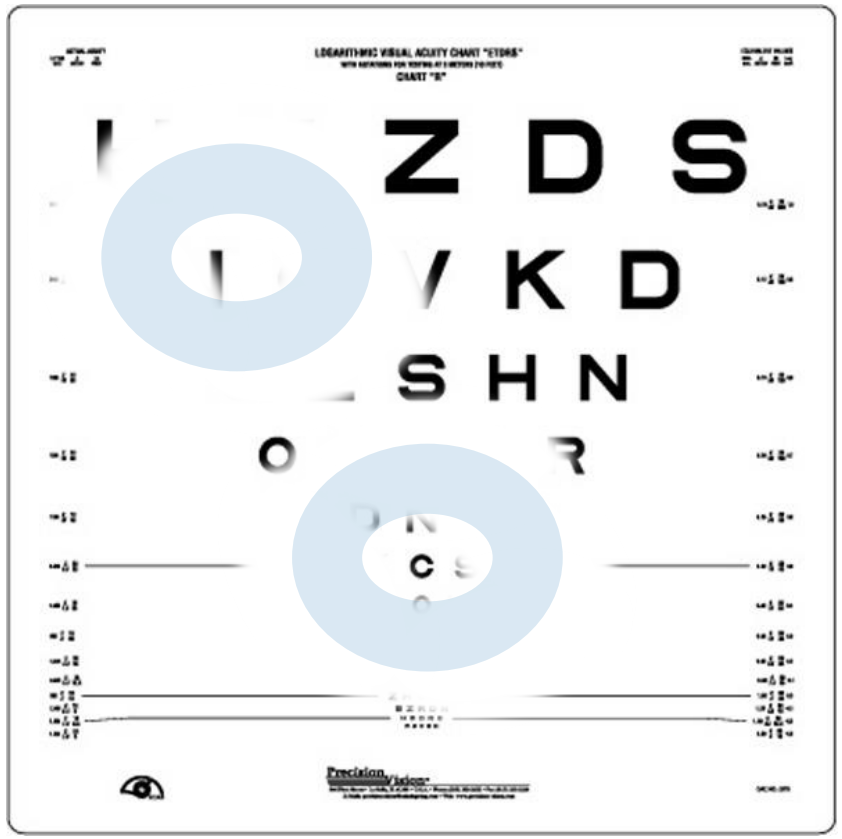
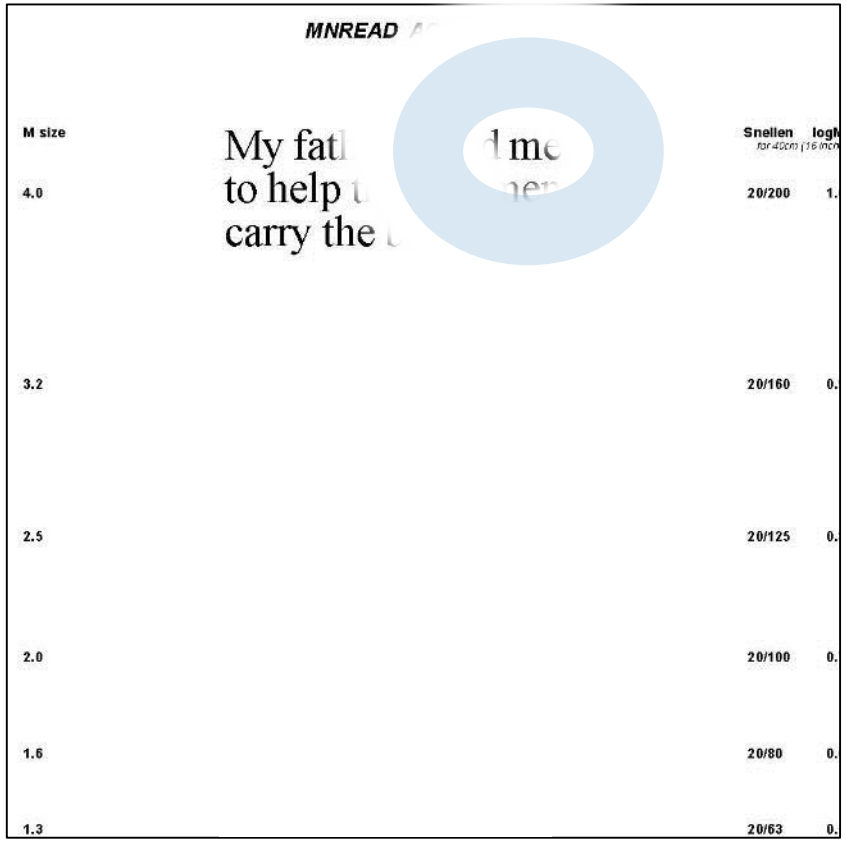
Good distance VA does not mean that reading ability is maintained





Self-reported visual function in patients with GA: change over time

	n=55		n=38	
	Baseline	Year 1	Baseline	Year 2
Reading*	71%	85%	74%	95%
Hazy	41%	43%	50%	45%
Faces*	65%	78%	58%	84%
Finding sign	57%	71%	74%	86%
Dim environment	85%	91%	81%	95%

*Significant difference at p=0.05 level for both Year 1 and Year 2 comparisons

Sunness et al. IOVS e-abstract 2011;52:91

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Key Takeaways

GA is a cause of similar levels of visual morbidity as CNV

- Real-world data confirm that GA is a progressive disease and is associated with high levels of visual impairment, with impacts on mobility and independence
- Proportion of eyes progressing to blindness is greater than that recorded in previous clinical studies
- GA and CNV are manifestations of the same disorder with specific temporal relationships

Geographic Atrophy: Patient Perspective

ROBERT SCHONFELD

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APL-2 in GA: Phase II FILLY Trial 18-Month Results

FILLY

Jeff Heier, MD
Co-President and Medical Director,
Ophthalmic Consultants of Boston

Key Takeaways

- APL-2 reduced the progression of GA secondary to AMD in the largest Phase 2 GA trial (n=246)
- Results correlated to treatment frequency with increasing effect size over time
- Further confidence in results from intra-patient control
- Upon discontinuation of APL-2, treatment effect declines

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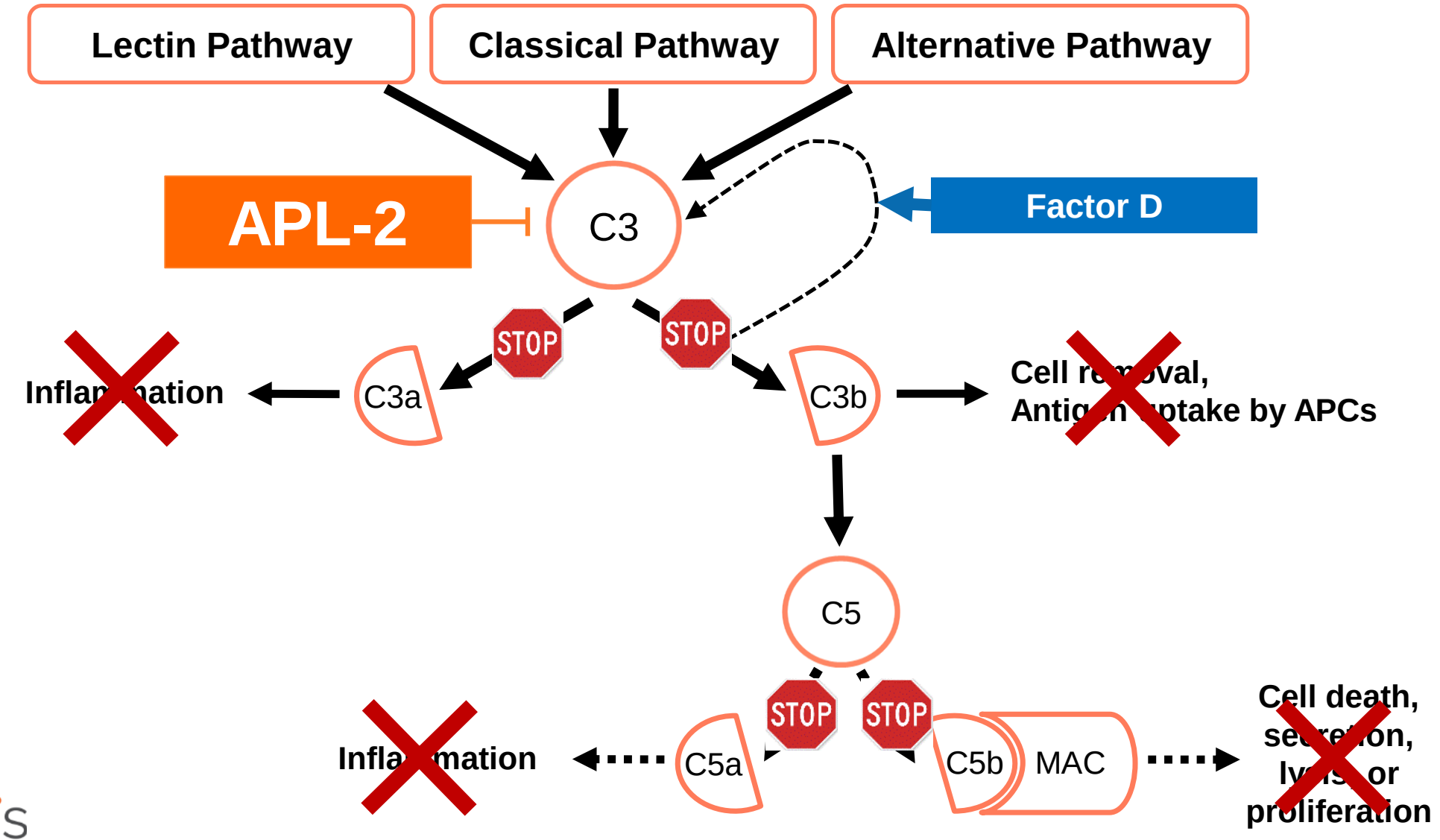
The Complement Pathway and Geographic Atrophy



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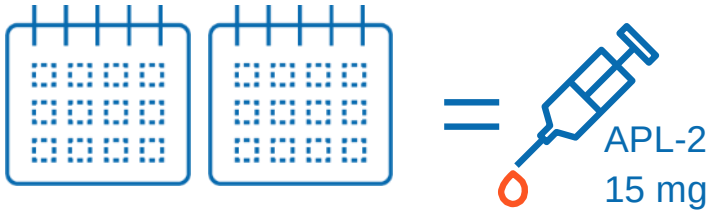
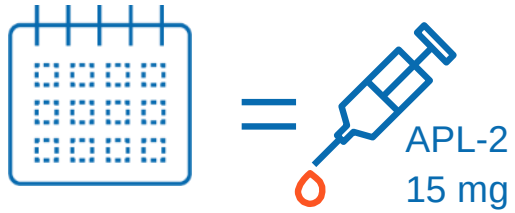
FILLY - Phase 2 study of APL-2 in Geographic Atrophy

Sham Injections n= 81

APL-2 injections every other month n= 79



APL-2 injections every month n= 86



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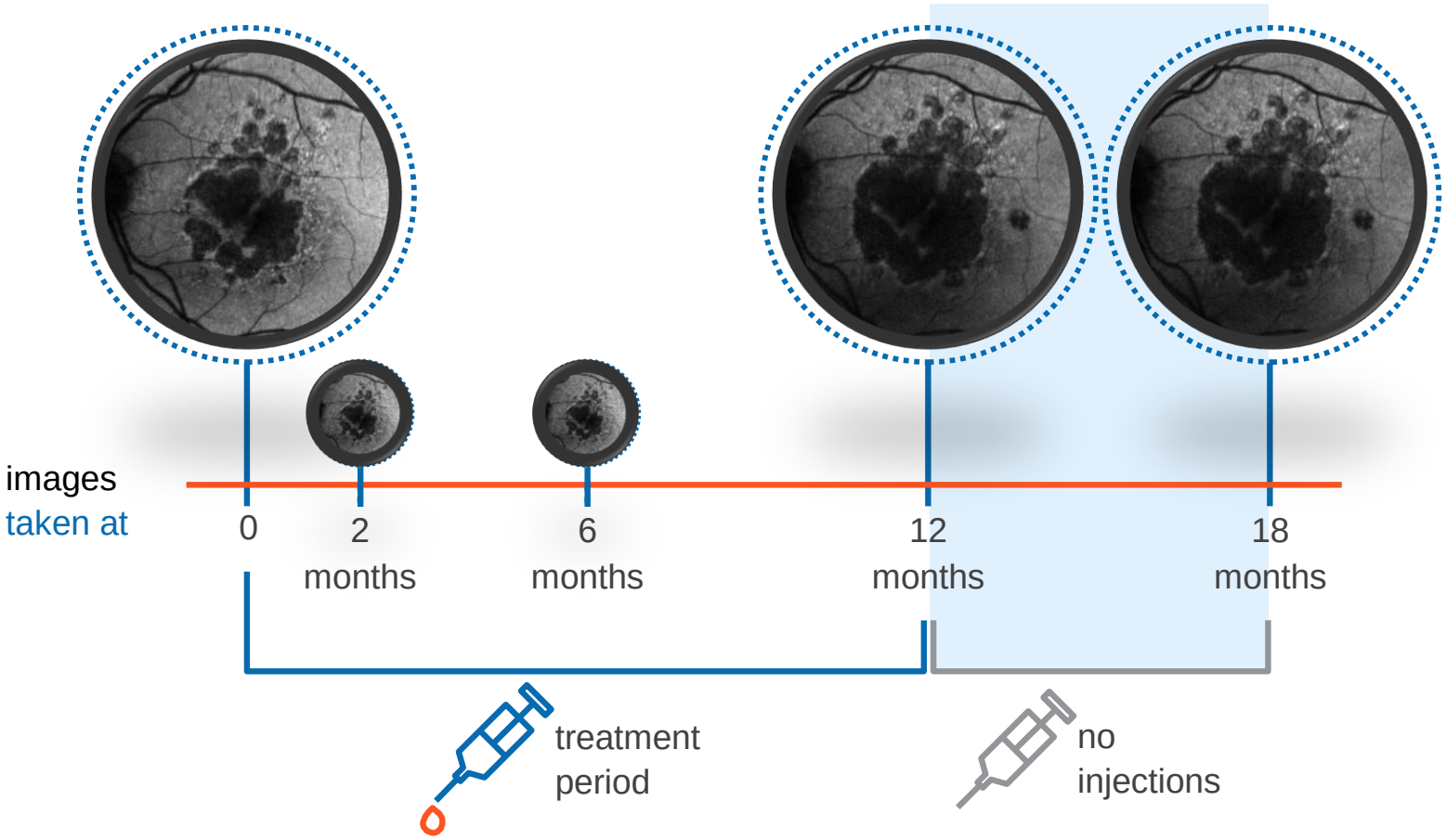
FILLY – timeline and endpoints



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).

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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

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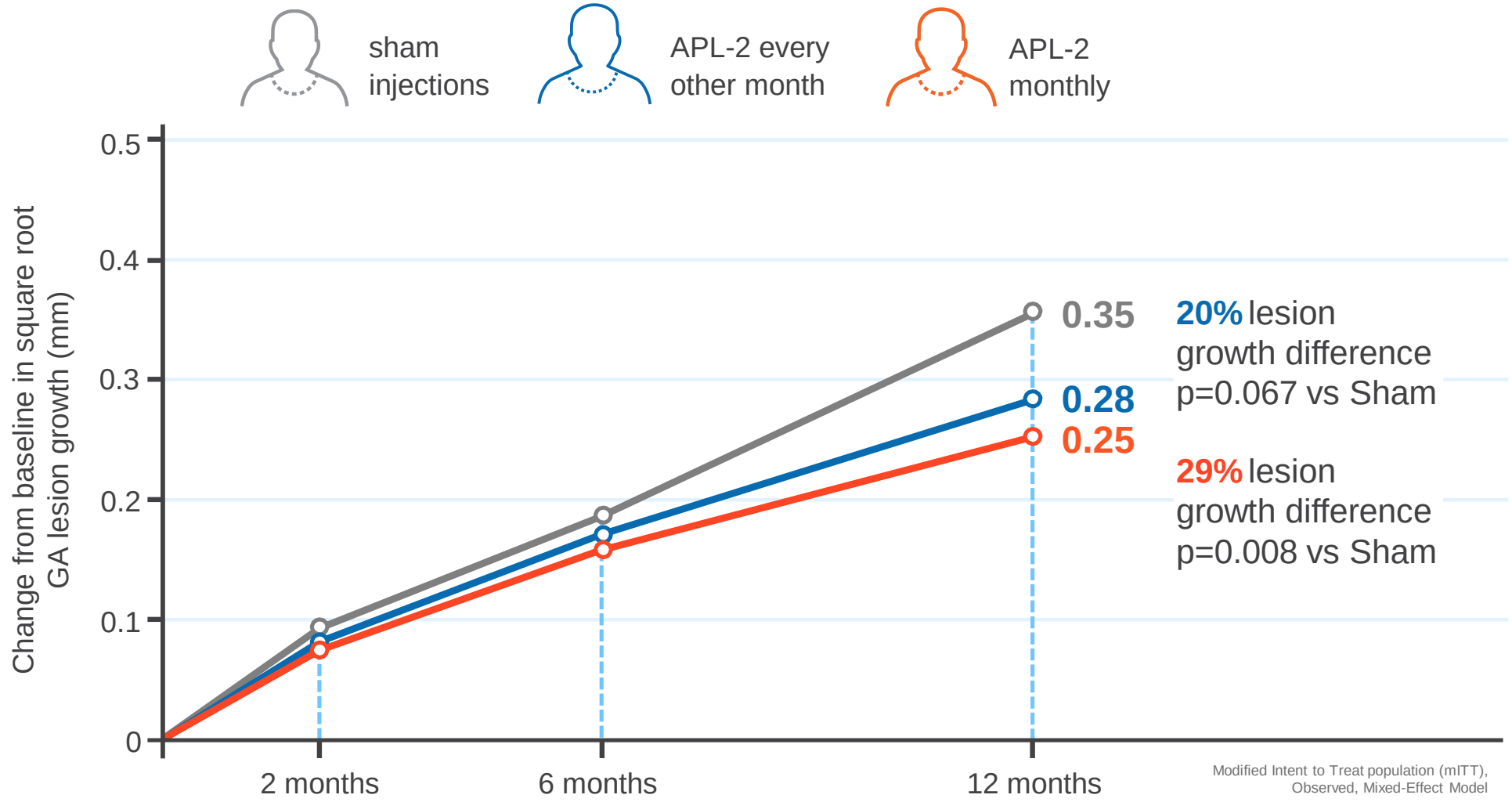
APL-2 slows GA growth at 12 months (*square root)*



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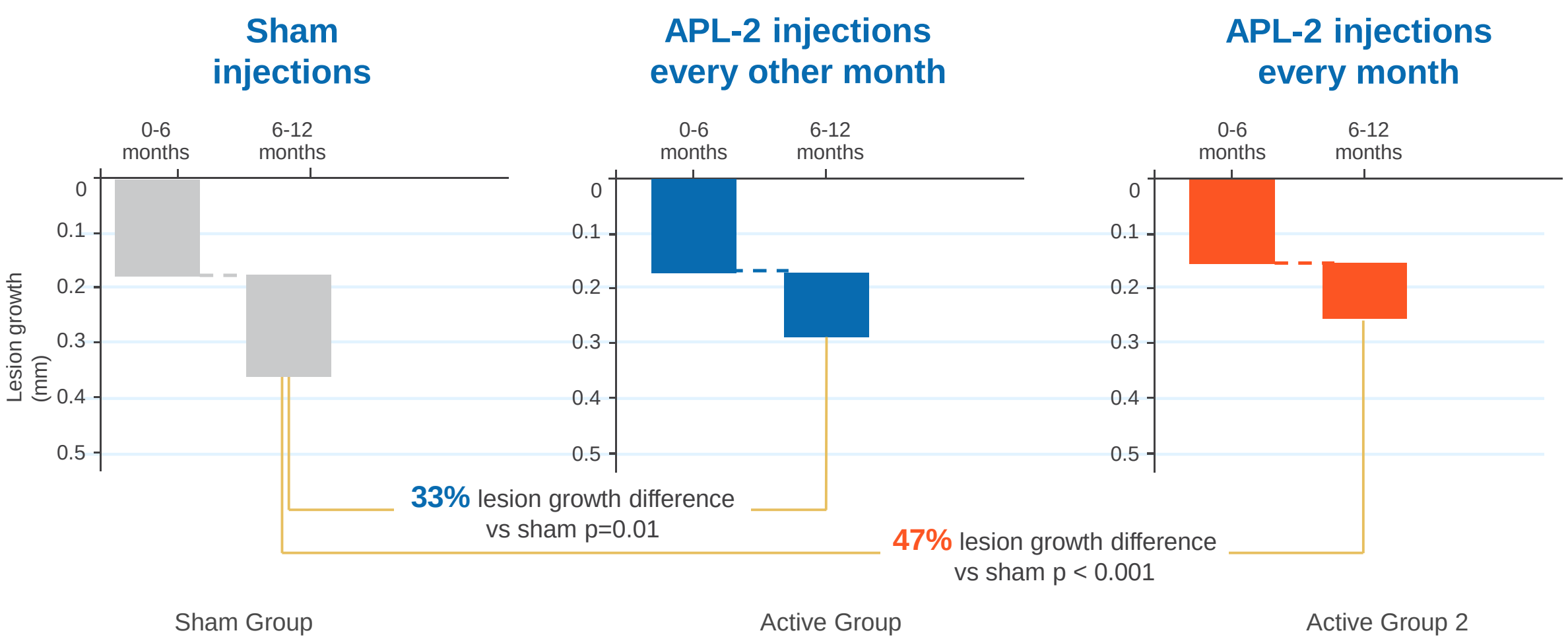
Lesion growth by six-month periods (*square root) – 12 months*



Data from subjects with a measurable GA lesion size at both Months 6 & 12

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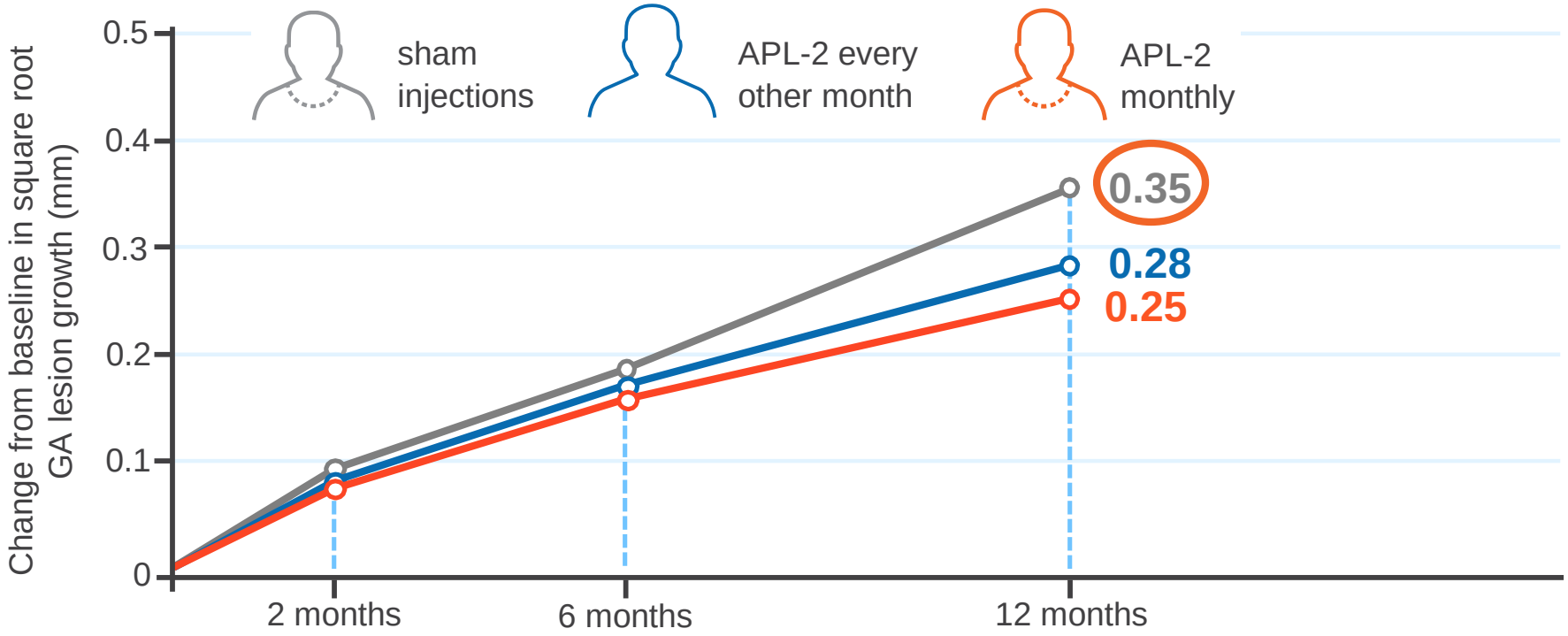
FILLY sham group behaved consistently with recent publication



Measure	Sham	Lampalizumab, 10 mg	
	Pooled (n=58)	q4w (n=596)	q6w (n603)
Change from baseline in square root of GA area at 48 wk, mm			
Adjusted mean (SE)	**0.342 (0.007)**	**0.349 (0.007)**	**0.352 (0.007)**
Difference in means (vs sham pooled)		**0.006**	**0.010**

Holz, F.G., et al., Efficacy and Safety of Lampalizumab for Geographic Atrophy Due to Age-Related Macular Degeneration: Chroma and Spectri Phase 3 Randomized Clinical Trials. JAMA Ophthalmol, 2018

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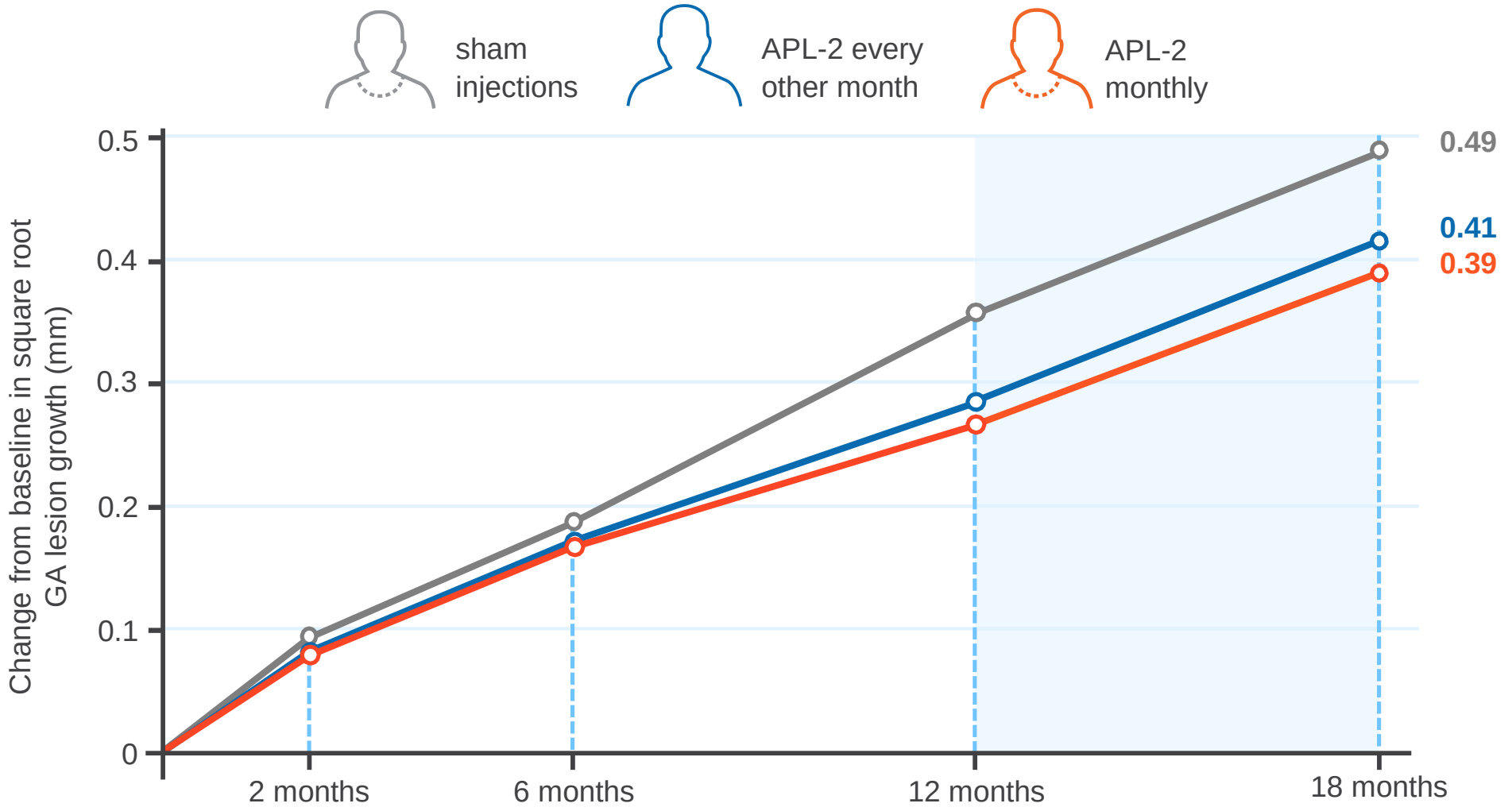
After cessation of treatment at 12 months, GA growth resumes but treatment effect is maintained through 18 months (*square root)*



16% lesion growth difference p=0.097 vs Sham

20% lesion growth difference p=0.044 vs Sham

Modified Intent to Treat population (mITT), Observed, Mixed-Effect Model

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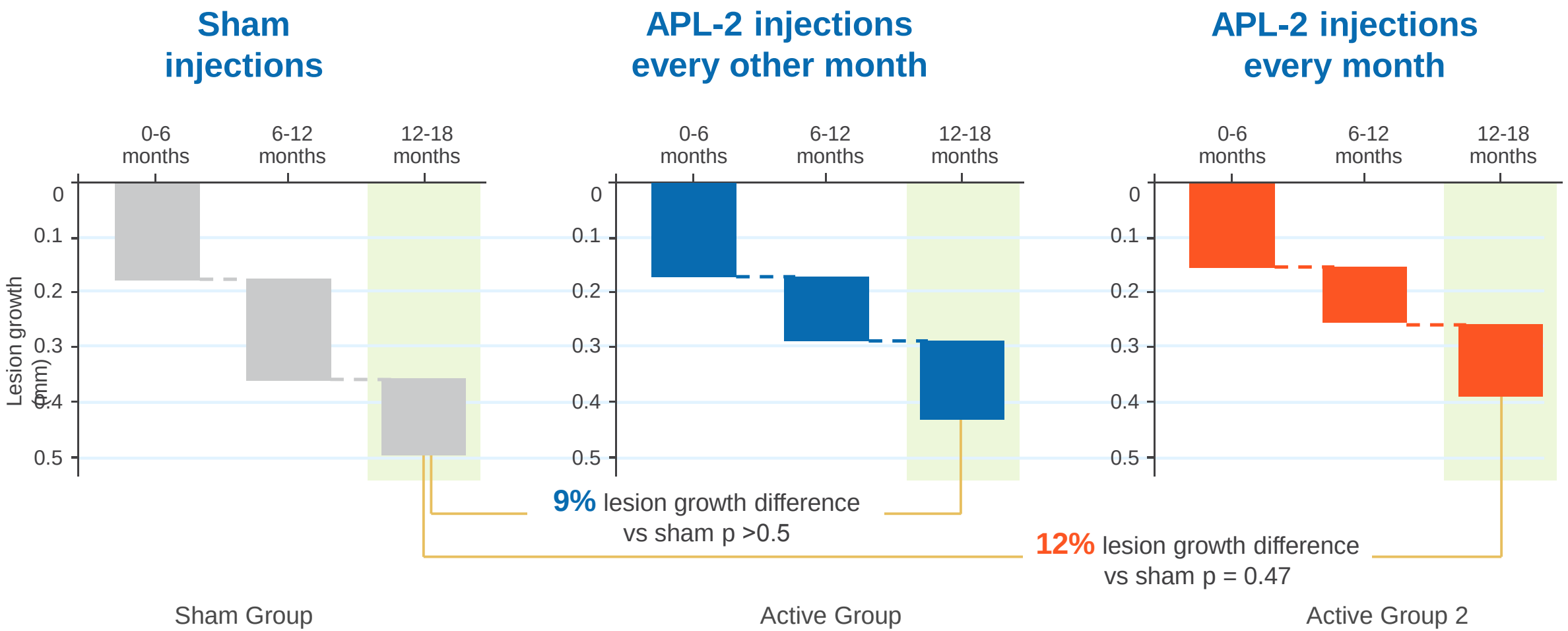
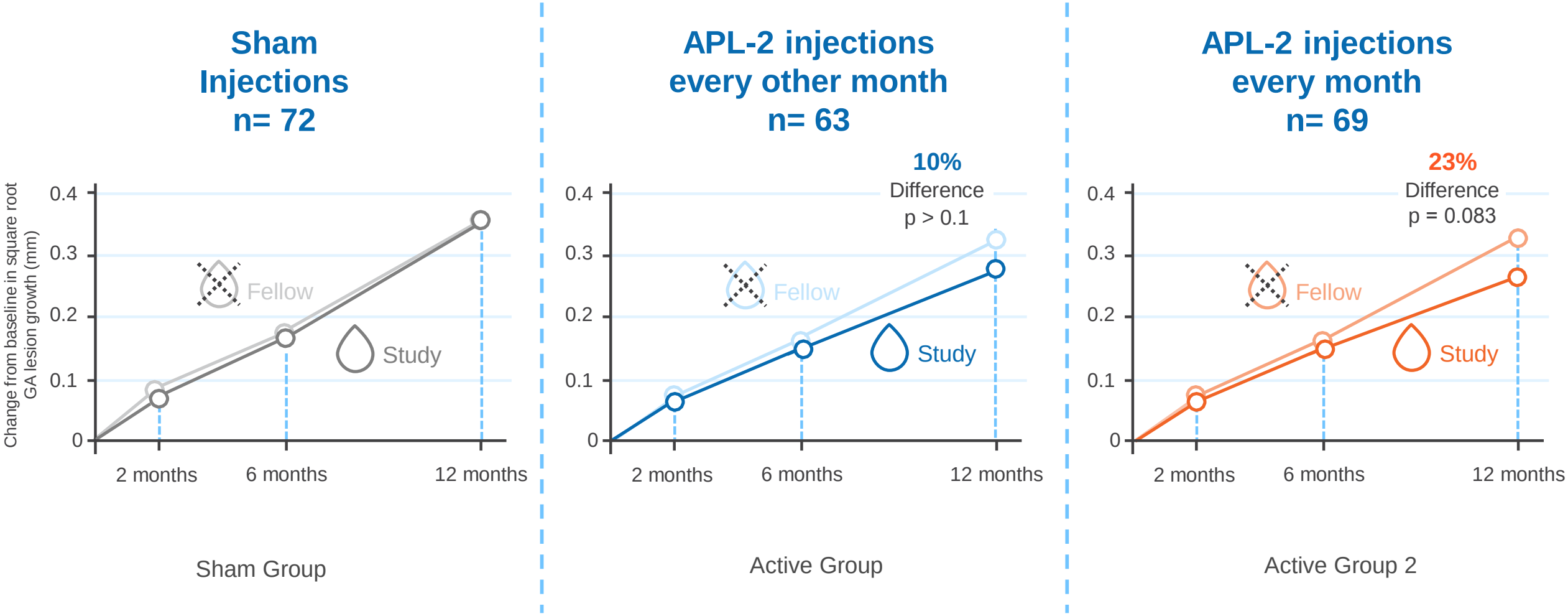
Lesion growth by six-month periods (*square root) – 18 months*



Data from subjects with a measurable GA lesion size at Months 6 & 12 & 18

GA growth comparison: fellow eye vs study eye *post hoc analysis*



Includes patients from the Bilateral GA Population

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New onset exudation – 18 months

	APL-2 Monthly	APL-2 EOM	Sham Pooled
All Subjects	n = 86	n =79	n = 81
Subjects with wAMD in Study eye (%)	18 (20.9%)	7 (8.9%)	1 (1.2%)
With History of CNV in Fellow Eye	n = 36	n =28	n = 29
Subjects with wAMD in Study eye (%)	13 (36.1%)	5 (17.9%)	0
No CNV History in Fellow Eye	n = 50	n =51	n = 52
Subjects with wAMD in Study eye (%)	5 (10.0%)	2 (3.9%)	1 (1.9%)

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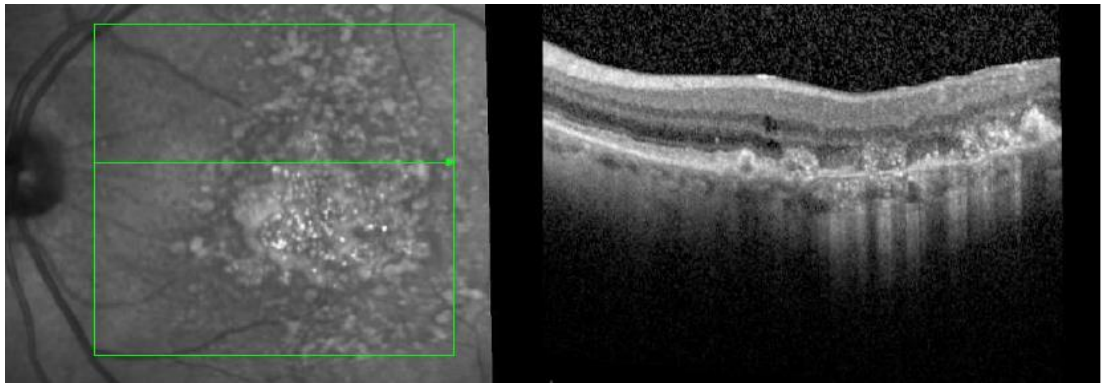
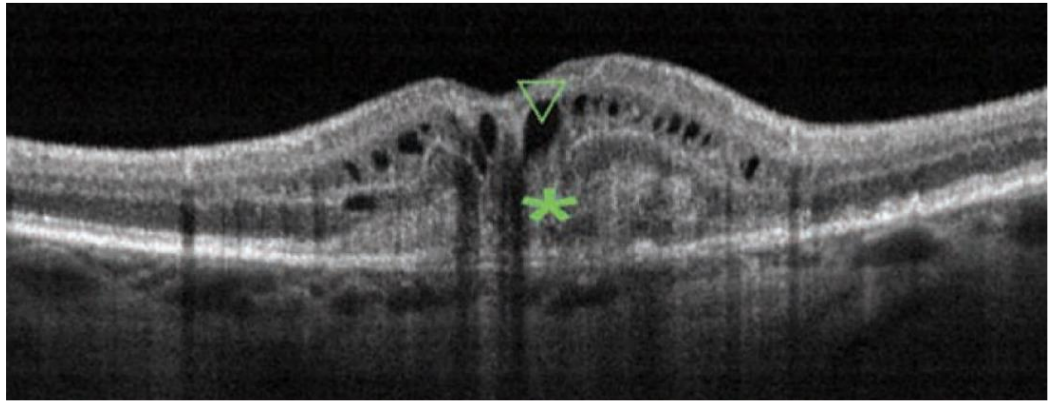
New onset exudation

- Subjects that developed exudation had minor loss of vision and were treated with anti-VEGF
- 6 patients developed wet AMD in the 12-18 month non-treatment period
  (5/6 had fellow eye wet AMD)

Subject 2 (monthly): Exudative AMD (Month 11)



Illustrative OCT scan of an exudative AMD with intraretinal fluid and RPE detachment



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Key Takeaways

- APL-2 reduced the progression of GA secondary to AMD in the largest Phase 2 GA trial (n=246)
- Results correlated to treatment frequency with increasing effect size over time
- Further confidence in results from intra-patient control
- Upon discontinuation of APL-2, treatment effect declines

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APL-2 in GA: Phase III Program

Jason S. Slakter, MD
Clinical Professor of Ophthalmology, NYU School of Medicine
Director Digital Angiography Reading Center

Key Takeaways

- Apellis on track to start 2 Global Phase 3 studies H2 2018
- Same study population as FILLY Phase 2 trial
- Primary endpoint is the change in the total area of GA lesions measured by FAF at Month 12
- Addition of secondary endpoints to evaluate changes in visual function

Phase 3 Study Overview

2 Global Studies	
Population	Patients with Geographic Atrophy secondary to AMD
1º 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

GA Phase 3 - Study population

No changes in Inclusion/Exclusion criteria from Filly Phase 2

- Key inclusion criteria - Study eye:
  - BCVA > 24 letters by ETDRS (20/320 Snellen equivalent)
  - The GA lesion must:
    a. Total GA: ≥ 2.5 and ≤ 17.5 mm^2
    b. If GA is multifocal, at least one focal lesion must be ≥ 1.25 mm^2 (0.5 DA)
    c. Presence of any pattern of hyperautofluorescence in the junctional zone of GA. Absence of hyperautofluorescence (i.e. pattern = none) is exclusionary
- Neovascular AMD in fellow eye is <u>not exclusionary</u>

Key Endpoints

- Primary:
  - Change from baseline to Month 12 in total area of GA lesion(s) in the study eye (in mm^2) based on Fundus Autoflourescence (FAF)
- Secondary:
  - Best-corrected visual acuity (BCVA), low luminance BCVA, low luminance deficit in the study eye
  - Reading speed (study eye), as assessed by Minnesota Reading or Radner Reading (MNRead) Charts (in select sites)
  - Microperimetry
  - National Eye Institute Visual Functioning Questionnaire 25-Item Version (NEI VFQ-25) distance activity subscale score
  - Functional Reading Independence Index (FRI) composite score in the study eye

Key Phase 3 Improvements

- Double masked
- Increase frequency of imaging timepoints
- Inclusion of two vision function assessments:
  - Reading speed
  - Microperimetry
- Inclusion of two patient reported outcomes:
  - National Eye Institute Visual Functional Questionnaire (NEI VFQ-25)
  - Functional Reading Independent (FRI) Index
- Subjects that develop exudative AMD will continue to receive APL-2 in combination with anti-VEGF

Visual Function Endpoints

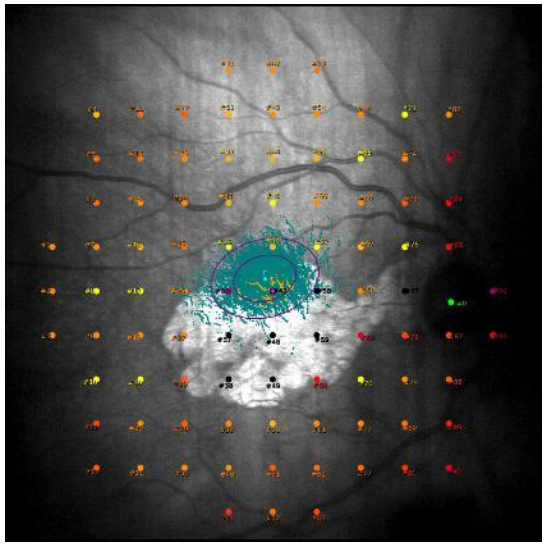
Patients with GA may have good best-corrected visual acuity owing to central fovea preservation, but very poor function because of scotoma surround the fovea

Reading speed:

- GA patients reported reading as the most important visual function activity perceives to decline over time
- Objective measurement of a patient's ability to read continuous text

Microperimetry:

- Objective measurement of retinal sensitivity to different light stimulus



Patient-Reported Outcome

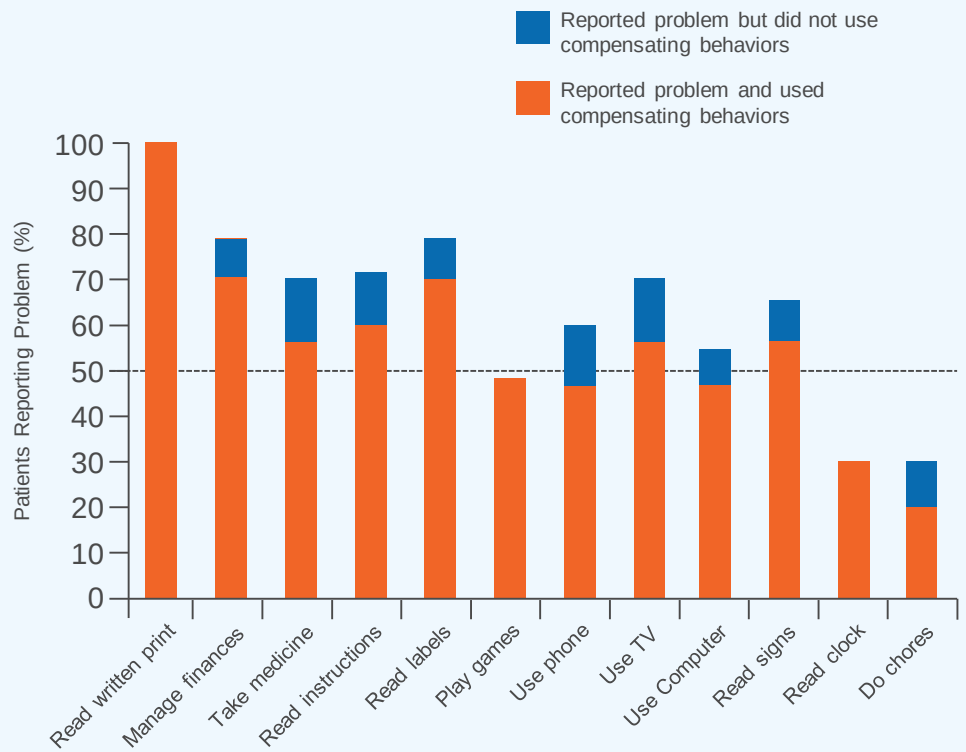
- NEI-VFQ 25
  - Questionnaire designed to assess the influence of visual impairment on vision-related daily activities
- Functional Reading Independence (FRI) Index
  - Evaluates the level of independence patients have in performing activities that require reading, such as writing a check or reading a prescription

VFQ-25 in Patients with AMD

NEI VFQ-25 Subscale	AMD Group [Mean (SD)]	P	Reference Group [Mean (SD)]	P
General Vision	53.0 (20.0)	<0.001	83.0 (15.0)	<0.001
Difficulty with distance tasks	56.0 (29.0)	<0.001	93.0 (11.0)	<0.001
Difficulty with near tasks	54.0 (27.0)	<0.001	92.0 (13.0)	<0.001
Peripheral vision	77.0 (27.0)	0.011	97.0 (10.0)	<0.001
Color Vision	85.0 (25.0)	<0.001	98.0 (8.0)	<0.001

Cahill, M.T., et al., Vision-related quality of life in patients with bilateral severe age-related macular degeneration. Ophthalmology, 2005. 112(1): p. 152-8.

FRI Index in Patients with AMD



Kimel, M., et al., Functional Reading Independence (FRI) Index: A New Patient-Reported Outcome Measure for Patients With Geographic Atrophy. Invest Ophthalmol Vis Sci, 2016. 57(14): p. 6298-6304

Management of New Onset Exudation

- In case of suspected new onset exudation, multiple imaging modalities will be used to confirm diagnosis
- Patients will continue to receive treatment with APL-2 in addition to anti-VEGF therapy

Key Takeaways

- Apellis on track to start 2 Global Phase 3 studies H2 2018
- Same study population as FILLY Phase 2 trial
- Primary endpoint is the change in the total area of GA lesions measured by FAF at Month 12
- Addition of secondary endpoints to evaluate changes in visual function

Geographic Atrophy

Q & A

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10 MINUTE BREAK

Apellis 2018 R&D Day

APL-2 IN PAROXYSMAL NOCTURNAL HEMOGLOBINURIA (PNH)

APL-2 in PNH: Unmet Need & PADDOCK Trial Update

Peter Hillmen, MD, PhD,
Professor of Experimental Haematology,
University of Leeds

APL-2 in PNH: Unmet Need - Key Takeaways

- Eculizumab prevents intravascular hemolysis in PNH
- All patients receiving eculizumab have C3-loading on their PNH rbc leading to extravascular hemolysis
- Hemoglobin is universally reduced in PNH patients receiving eculizumab compared to a normal population
- 36% patients on eculizumab continue to require transfusions but many more have mild to moderate symptoms

PNH – Triad of Clinical Features

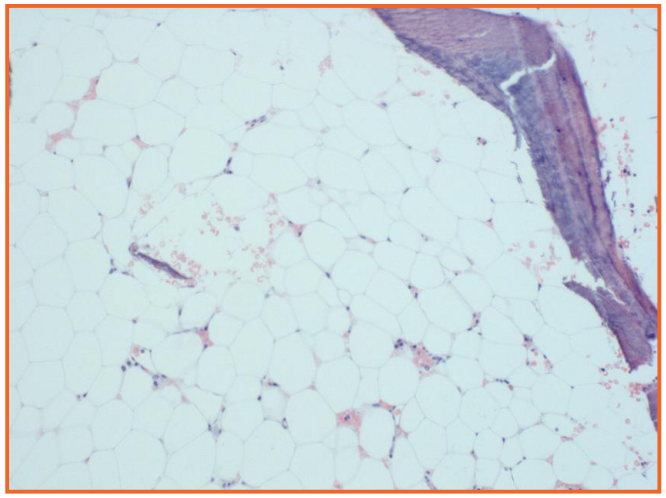
Hemoglobinuria

Intravascular hemolysis
- disabling symptoms
  - abdominal pain
  - dysphagia
  - erectile failure
  - severe lethargy

Budd-Chiari syndrome

Thrombosis
- liver, cerebral
- 50% of patients
- 33% of patients is fatal

Aplastic anaemia



Bone Marrow Failure
- often precedes PNH
- selects for PNH clone

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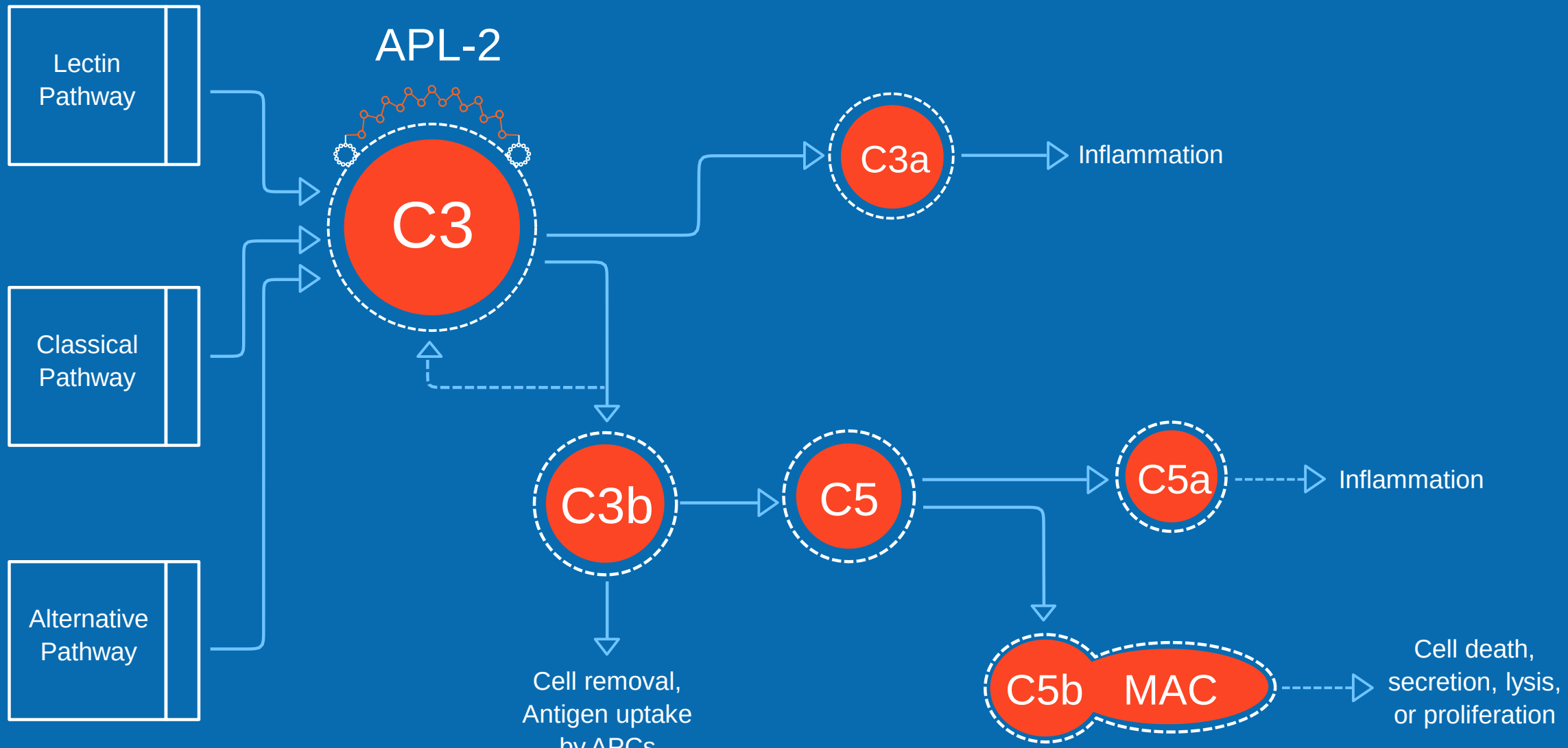
Central inhibition of complement



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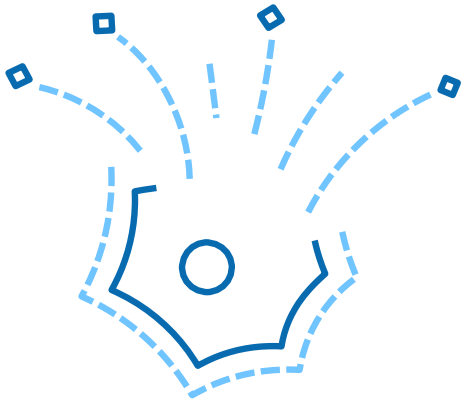
Paroxysmal Nocturnal Hemoglobinuria (PNH) is a rare, life-threatening blood disease

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

- ~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.
- Approximate cost $500,000 annually.

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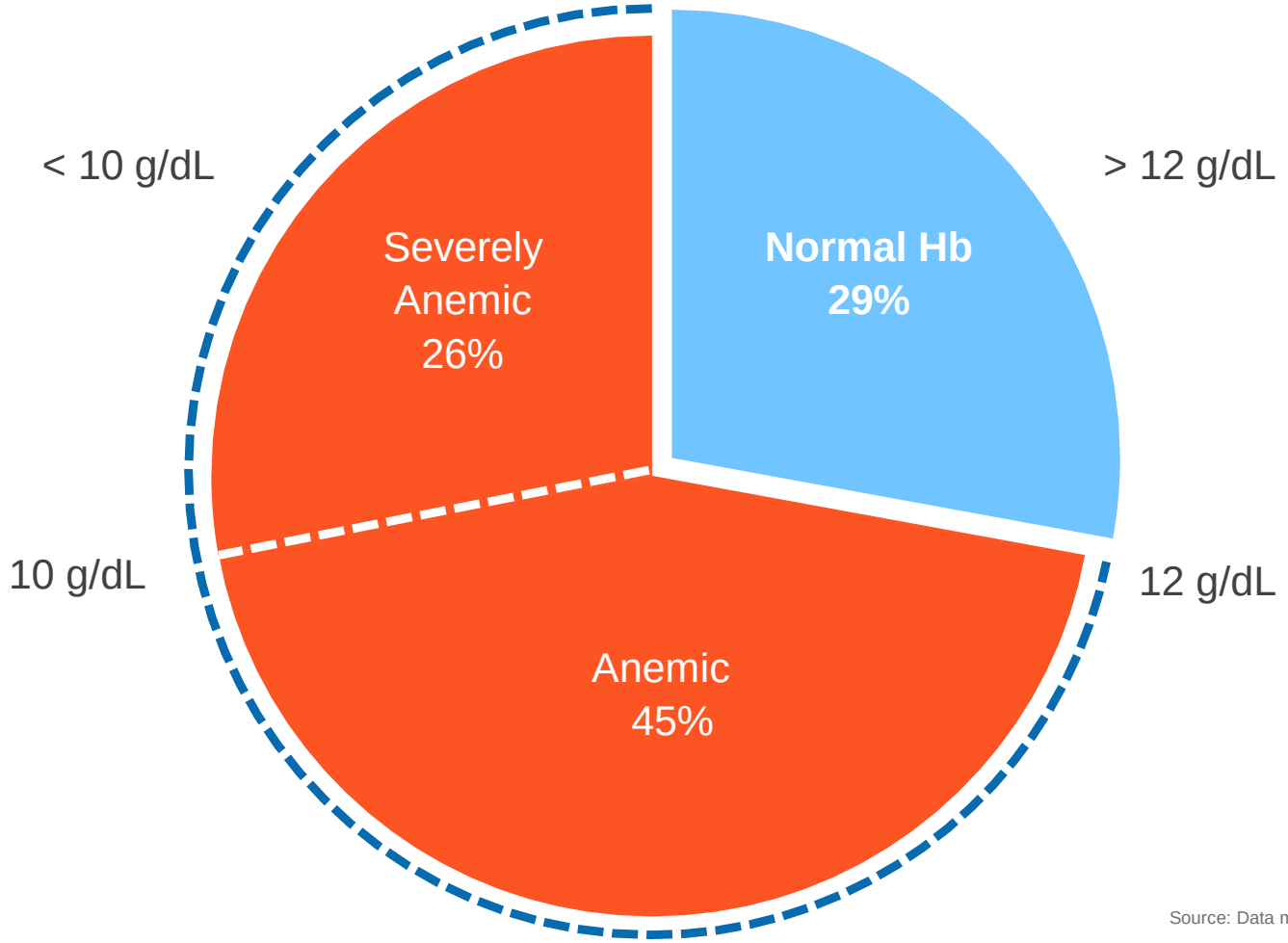
~70% of eculizumab-treated patients remain anemic due to extravascular hemolysis



Source: Data measured from 141 PNH patients courtesy of Dr. Pete Hillmen and Dr. Anita Hill in Apellis sponsored research collaboration.

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

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Extravascular Hemolysis Due to C3-Loading in Patients with PNH Treated with Eculizumab: Defining the Clinical Syndrome

Claire McKinley[1], Stephen Richards[4], Talha Munir[2], Morag Griffin[2], Lindsay Mitchell[3], Louise Arnold[2], Kathryn Riley[2], Nicola Copeland[2], Darren Newton[1], Anita Hill[2] and Peter Hillmen[1,2]

[1] Section of Experimental Haematology, University of Leeds, [2] Department of Haematology, St James's University Hospital, Leeds, [3] Department of Haematology, Monklands Hospital, Airdrie, [4]Haematological Malignancy Diagnostic Service, St James's University Hospital, Leeds

Methods

Data from patients with PNH treated in the Leeds centre of the UK PNH National Service was reviewed retrospectively. Criteria for inclusion in the analysis were:

- Treatment with eculizumab for ≥13 months
- Mean platelet count >50 x 10^9/L
- Mean absolute neutrophil count >0.5 x 10^9/L

(to exclude patients with significant bone marrow failure)

For each patient, mean values for haemoglobin, absolute reticulocyte count, LDH ratio (LDH adjusted for upper limit of normal), total bilirubin and the proportion of PNH red cells loaded with C3 were calculated and collated covering a 48-month period.

C3-loading of red cells was assessed by flow cytometry (Sutherland et al., Curr Protoc Cytom. 2015). Red cell transfusion requirements for the latest 12 months and latest eculizumab dosage were also collected.

Data between groups was compared by Mann-Whitney Tests.

141 patients were identified for inclusion by the above criteria; 70 males and 71 females.

median hemoglobin level for all patients = 109g/l.

72% had a mean hemoglobin below 120g/l despite treatment with eculizumab

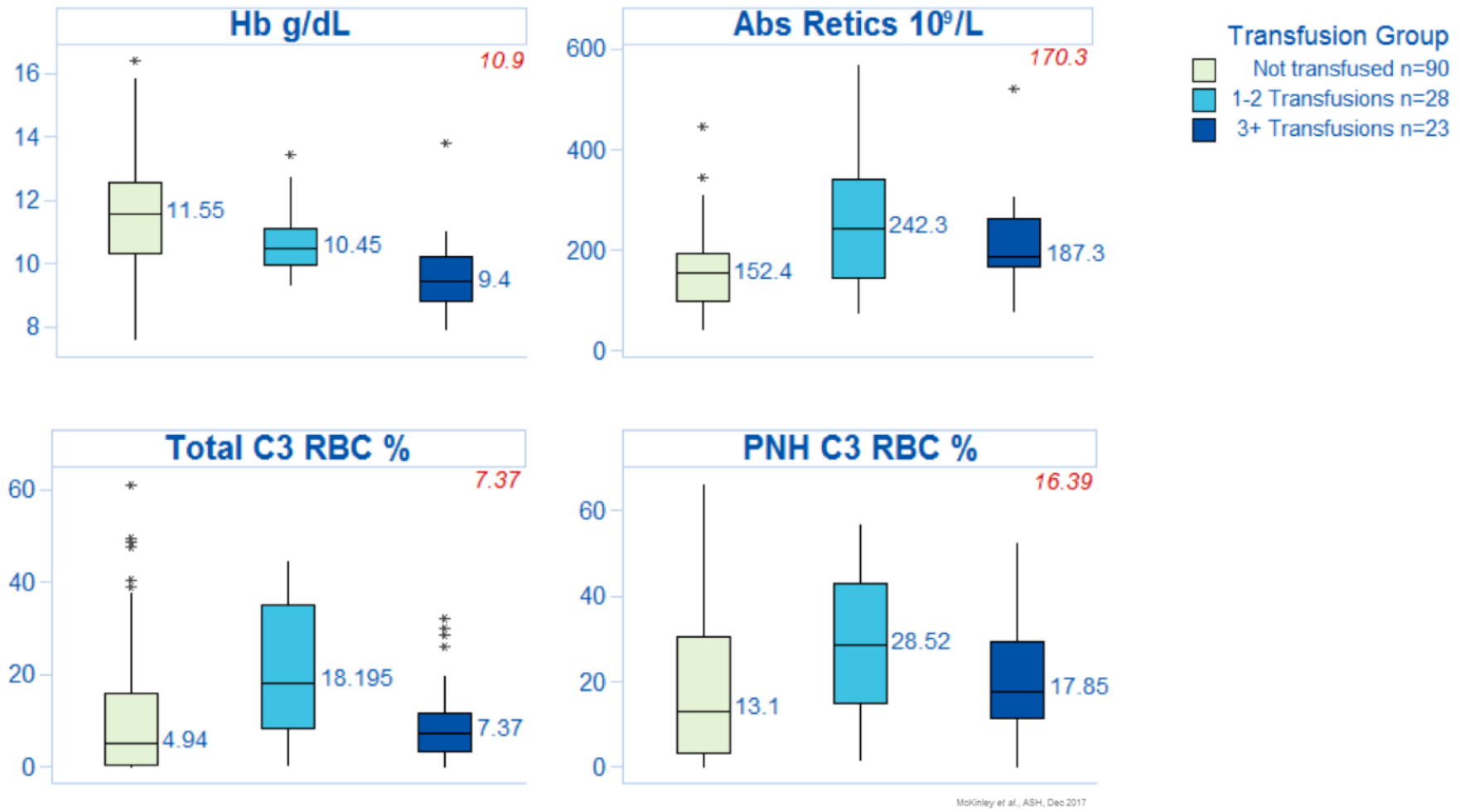
all patients had C3 on their red cells → some degree of extravascular hemolysis is always seen

During the latest 12 months

- 51 (36%) patients had at least one transfusion,
- 23 (16%) requiring 3 or more transfusions.

McKinley *et al.*, ASH, Dec 2017

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



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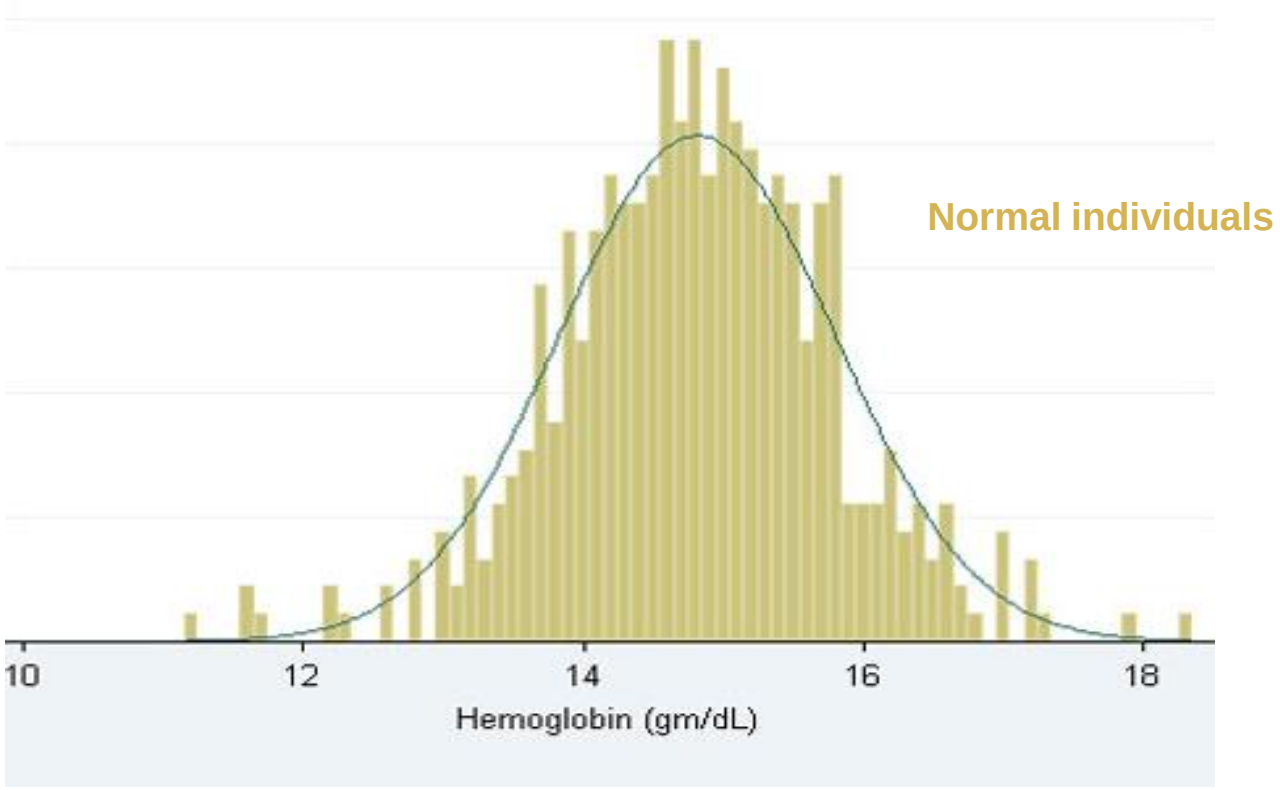
Hemoglobin levels in patients with PNH receiving eculizumab (n=141; all hemolytic)



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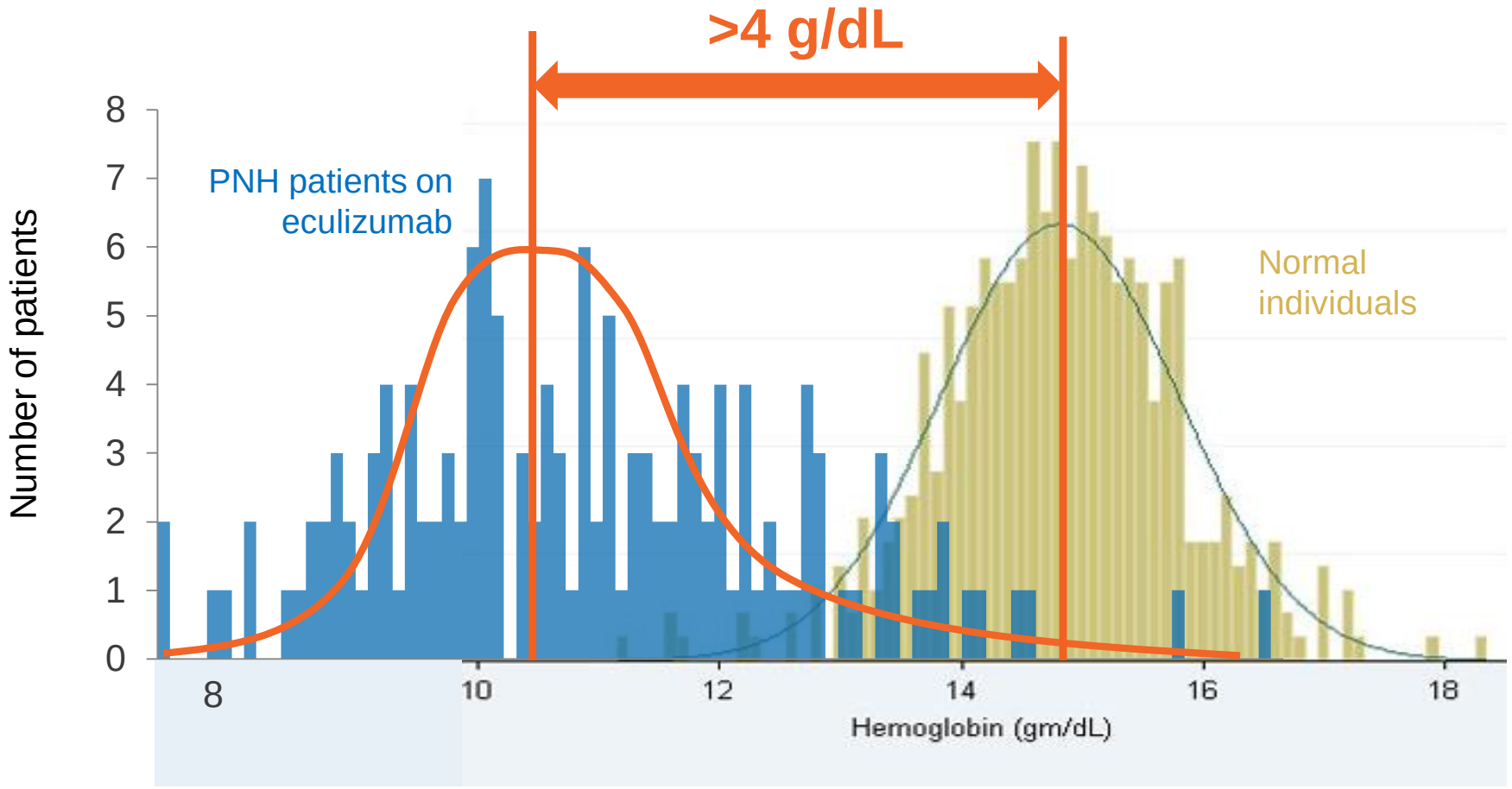
Hemoglobin levels in patients with PNH receiving eculizumab (n=141; all hemolytic)



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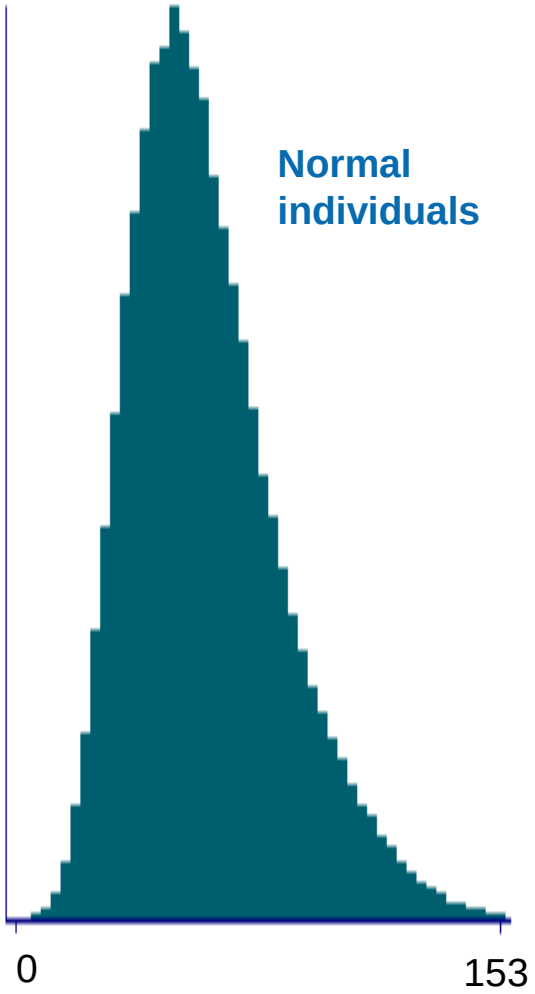
Increased absolute reticulocyte counts in almost all patients with PNH receiving eculizumab (n=141; all hemolytic)



Mean absolute reticulocytes count (x 10^9/l)

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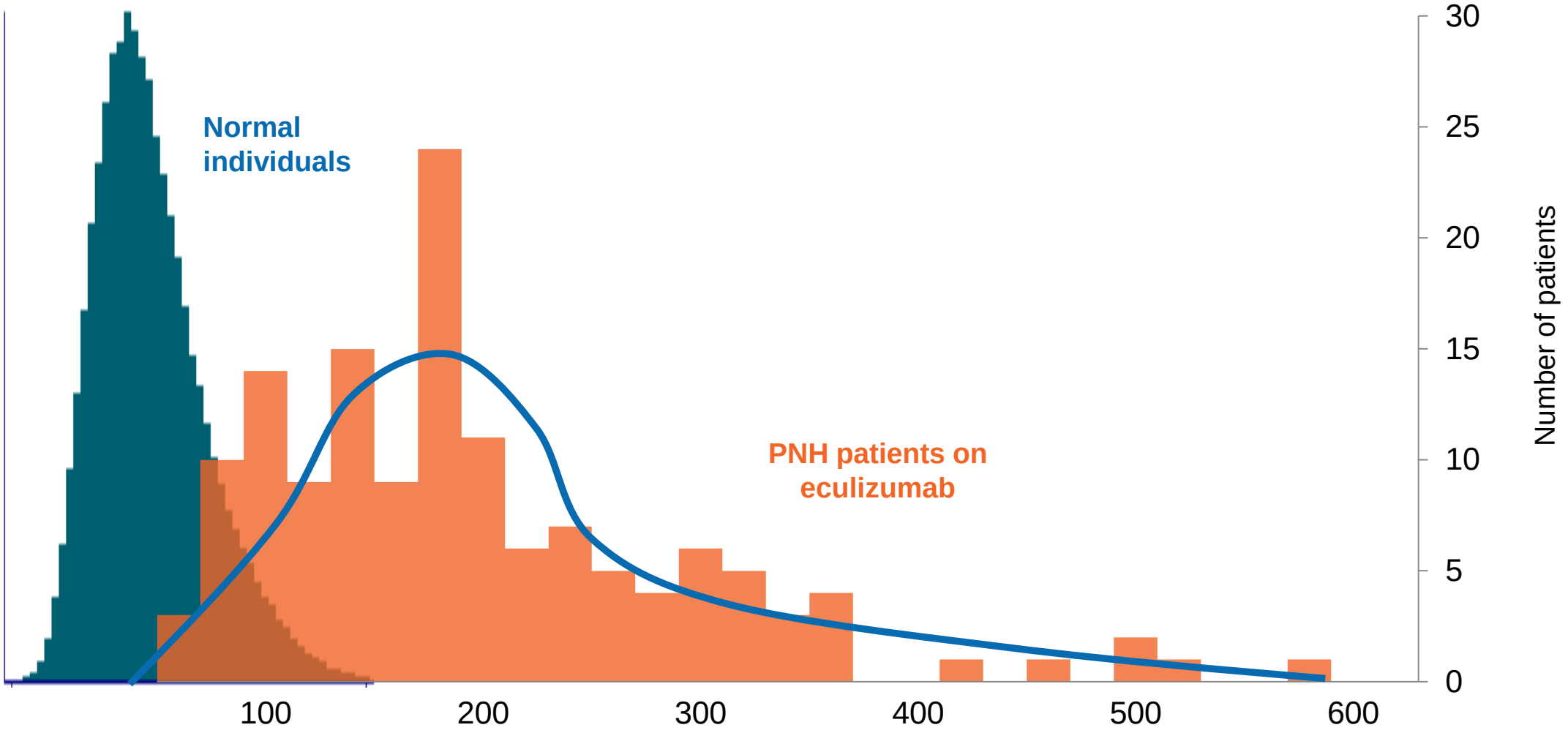
Increased absolute reticulocyte counts in almost all patients with PNH receiving eculizumab (n=141; all hemolytic)



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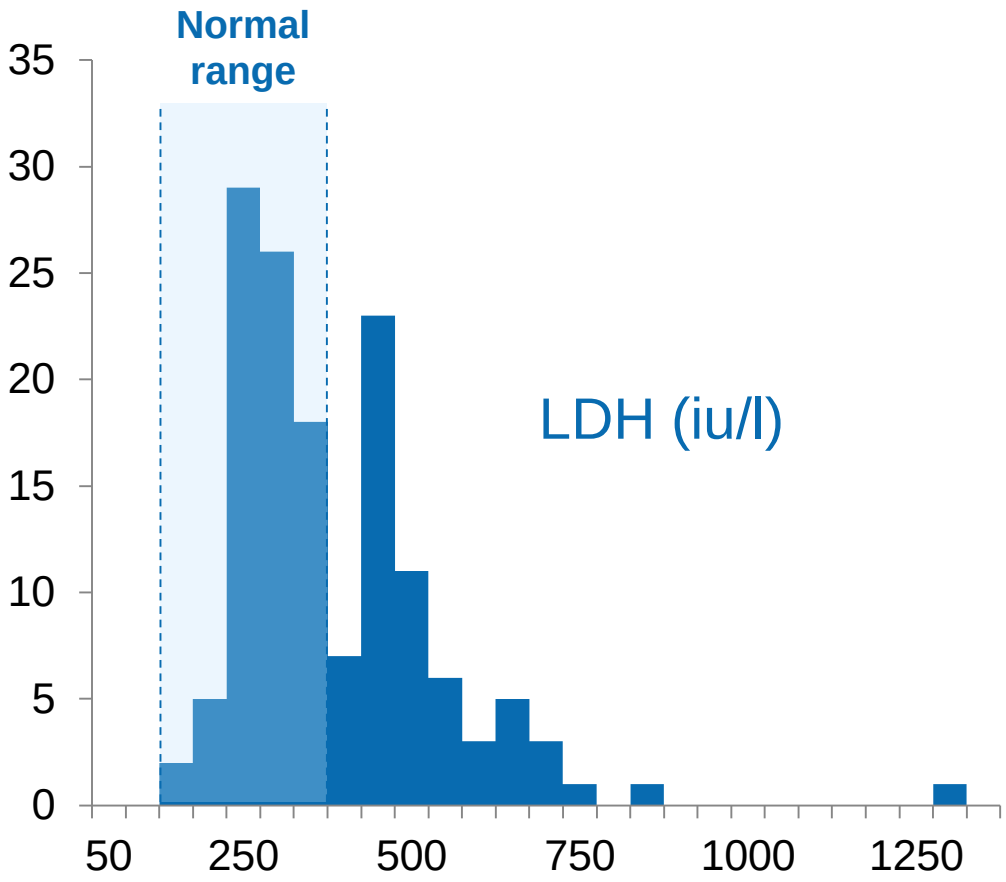
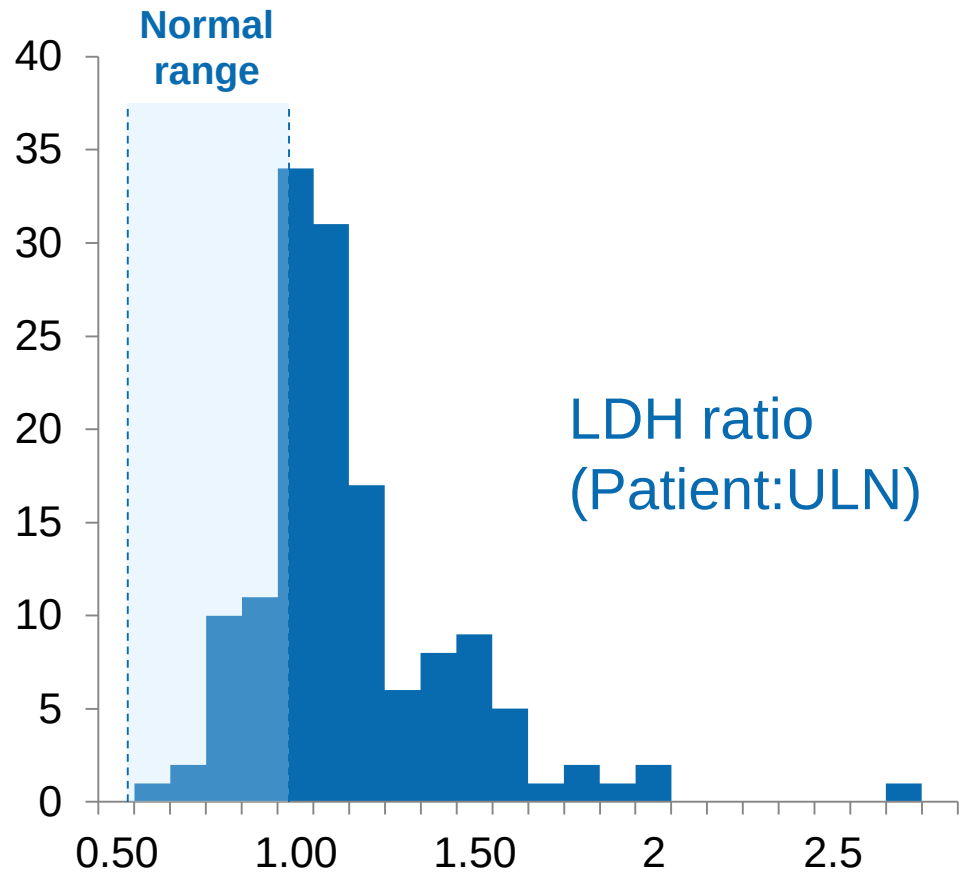
LDH levels are slightly raised in patients with PNH receiving eculizumab (n=141; all hemolytic)





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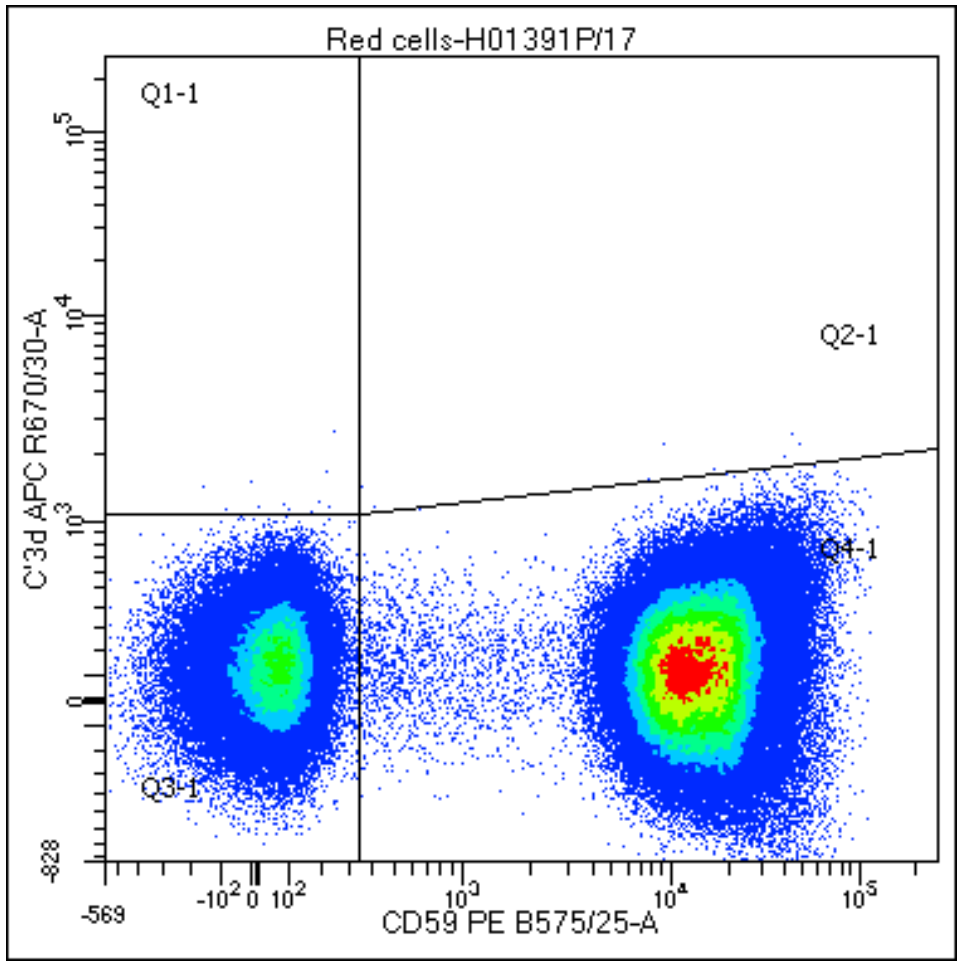
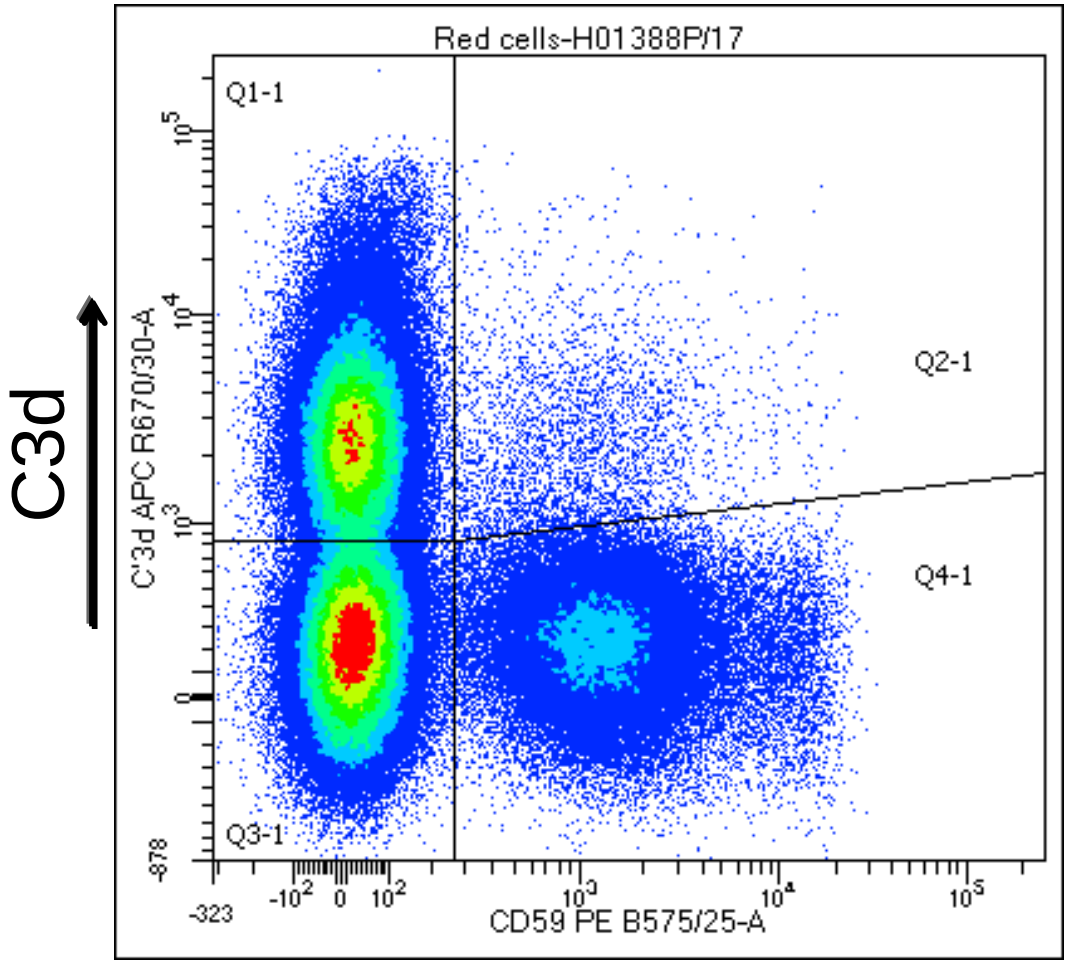
C3 red cell loading in PNH

PNH patient not on eculizumab



PNH patient on eculizumab



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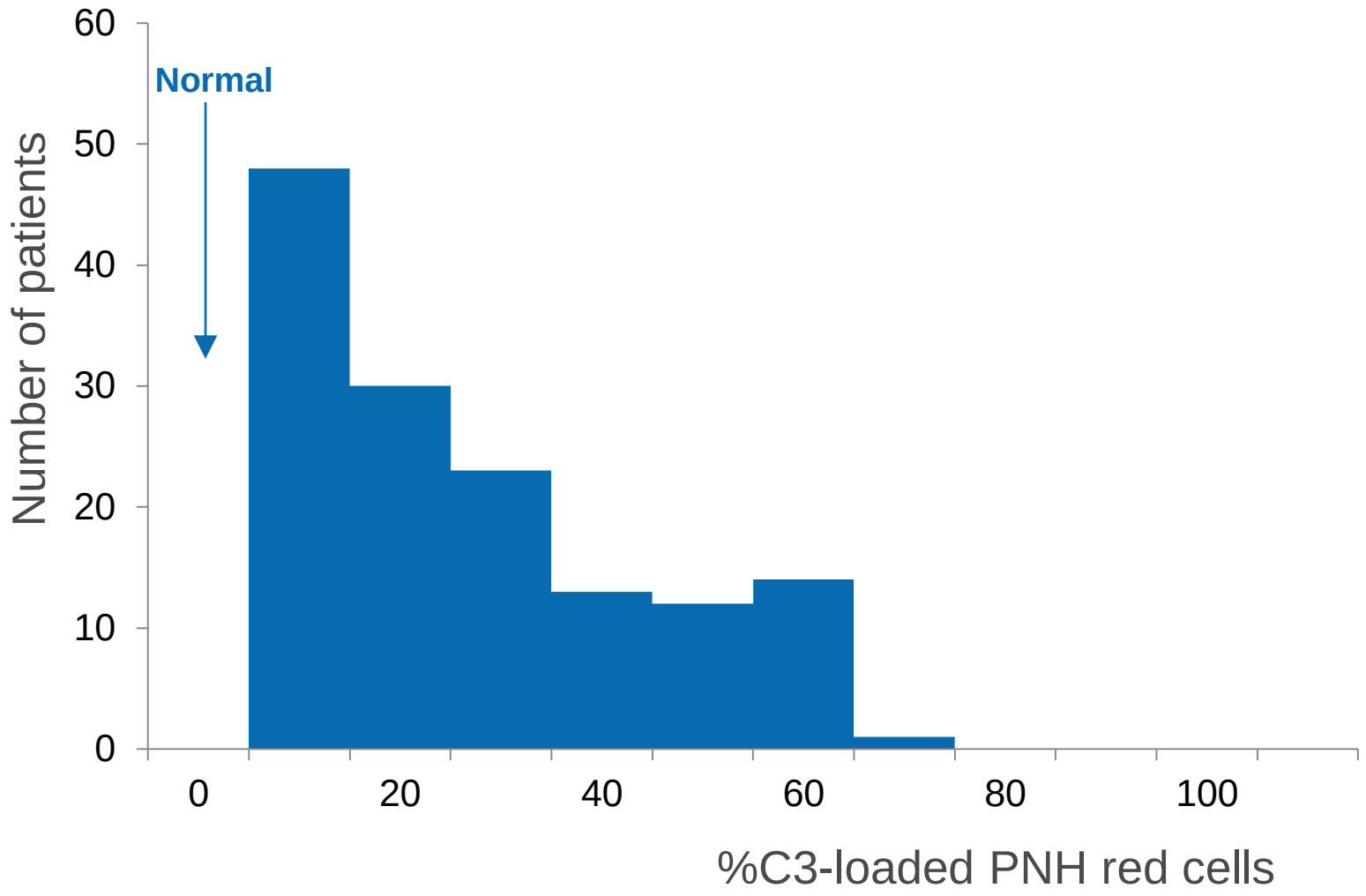
%C3-loaded PNH red cells in patients with PNH receiving eculizumab (n=141; all hemolytic)



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APL-2 in PNH: Unmet Need - Key Takeaways

- Eculizumab prevents intravascular hemolysis in PNH
- All patients receiving eculizumab have C3-loading on their PNH rbc leading to extravascular hemolysis
- Hemoglobin is universally reduced in PNH patients receiving eculizumab compared to a normal population
- 36% patients on eculizumab continue to require transfusions but many more have mild to moderate symptoms

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APL-2 In PNH: PADDOCK Update

Peter Hillmen, MD, PhD,
Professor of Experimental Haematology,
University of Leeds

APL-2 in PNH: PADDOCK - Key Takeaways

- Hemoglobin increases are clinically meaningful and different compared to C5 inhibitors
- “Normalization” of hemoglobin, reticulocyte count and bilirubin rarely seen with C5 inhibition
- APL-2 is well tolerated – two lead-in transfusions (not on pharmacology). Safety profile of APL-2 to date is very good
- Potential for meaningful superiority vs. C5 inhibitors

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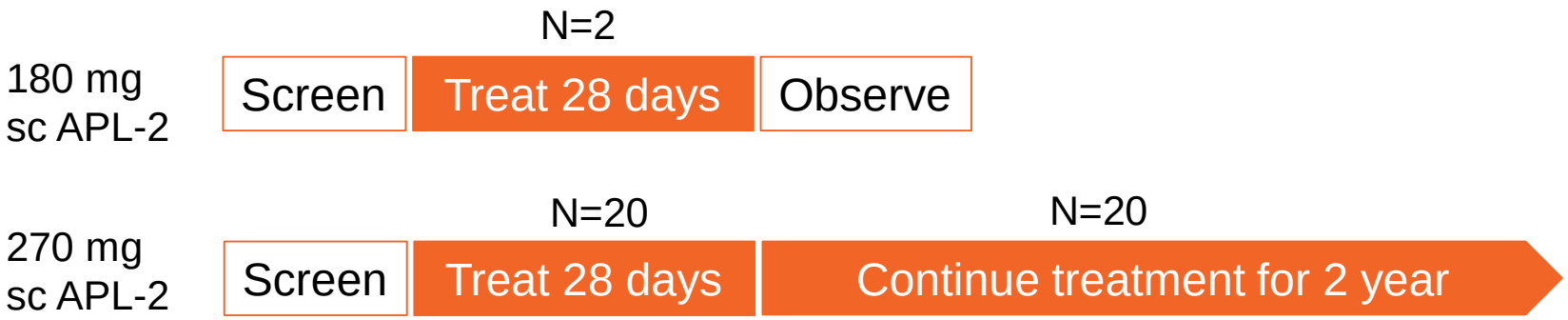
PADDOCK Study Design: APL-2 monotherapy in eculizumab-naïve PNH

Key eligibility criteria:

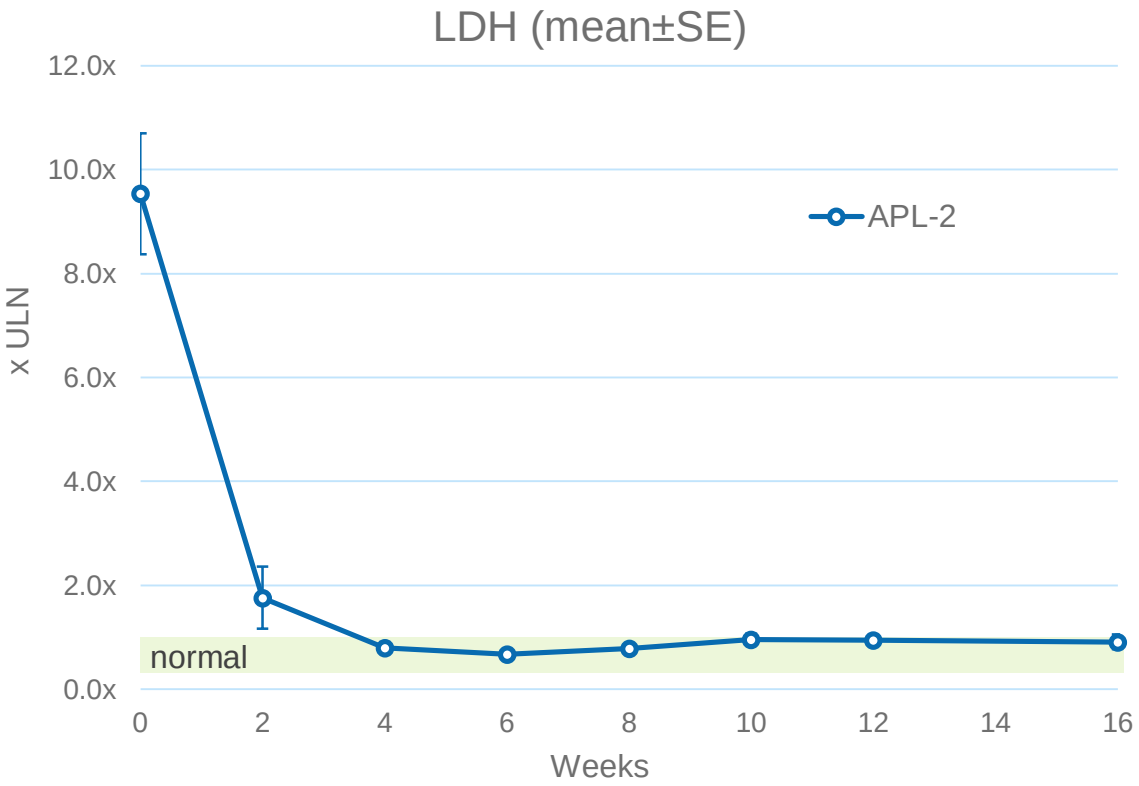
- Diagnosis of PNH (WBC clone > 10%)
- LDH > 2X ULN
- Last transfusion within 12 months prior to screening
- Platelet count of > 30,000/mm3 and Absolute neutrophil count > 500 x109/L

Study design – Open label MAD



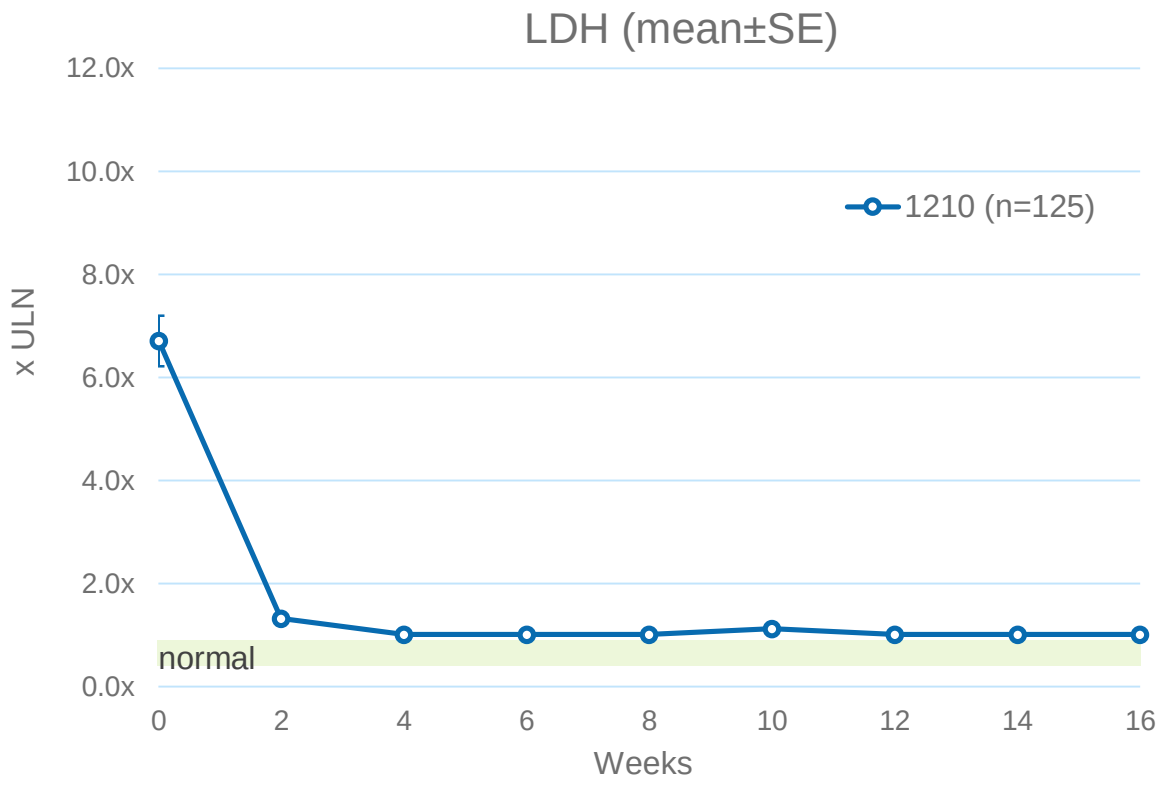
* Cohort 2 expanded to include up to 20 subjects

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



Week	0	2	4	6	8	10	12	14	16
n	n=13	n=13	n=13	n=10	n=8	n=5	n=5	Not Taken	n=4
Multiple of ULN	9.54x	1.76x	0.80x	0.66x	0.79x	0.95x	0.94x		0.91x

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.



Week	0	2	4	6	8	10	12	14	16
n=125									
Multiple of ULN	6.7x	1.3x	1.0x	1.0x	1.0x	1.1x	1.0x	1.0x	1.0x

ALXN-1210 data taken from Alexion's presentation of topline results of a Phase 3 clinical trial on March 15, 2018. Apellis has not conducted a head-to-head comparison of APL-2 and ALNX-1210. There may be differences between trial designs and subject characteristics of the Paddock APL-2 trial and the Phase 3 trial of ALXN-1210

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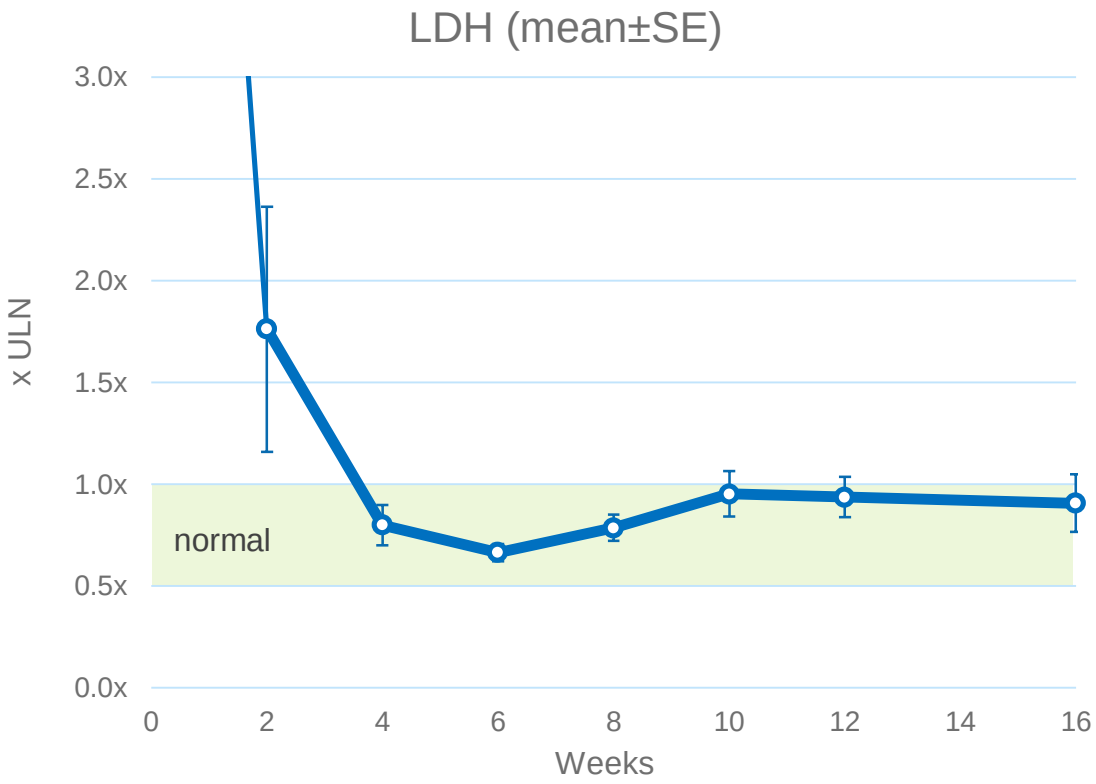
PADDOCK (interim): Preliminary results with APL-2 are encouraging in comparison to ALXN-1210 (data not from a head to head study)



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n	n=13	n=13	n=13	n=10	n=8	n=5	n=5	Not Taken	n=4
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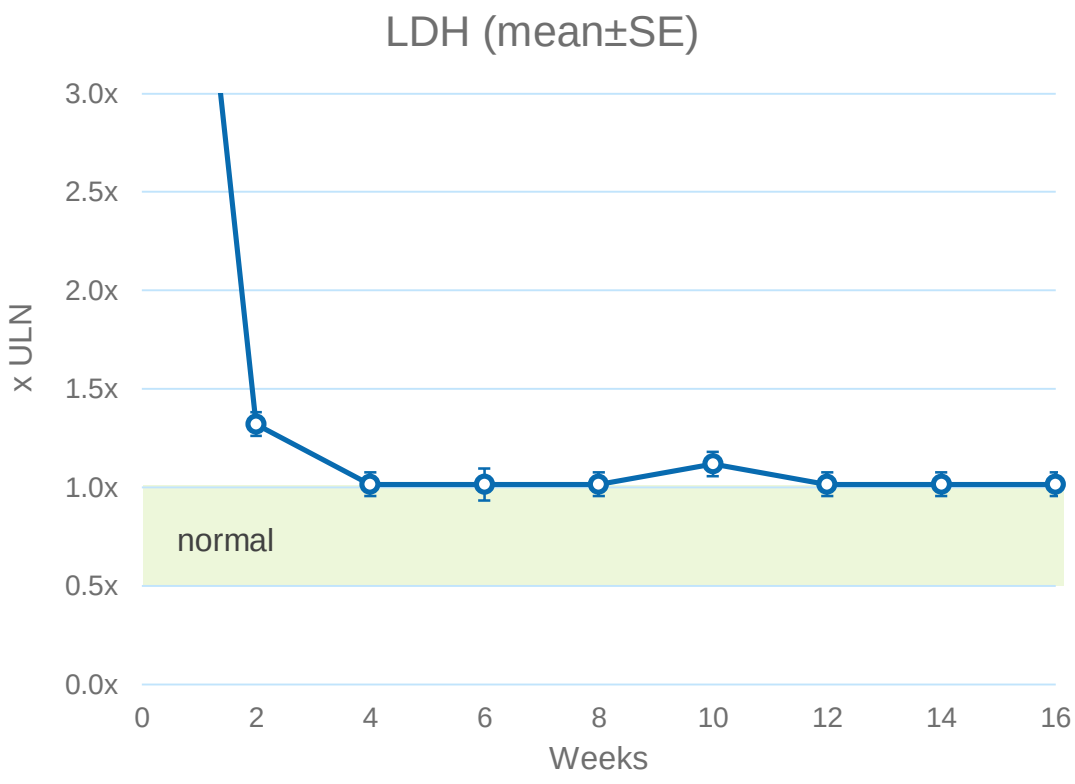

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ALXN-1210 data taken from Alexion's presentation of topline results of a Phase 3 clinical trial on March 15, 2018. Apellis has not conducted a head-to-head comparison of APL-2 and ALNX-1210. There may be differences between trial designs and subject characteristics of the Paddock APL-2 trial and the Phase 3 trial of ALXN-1210

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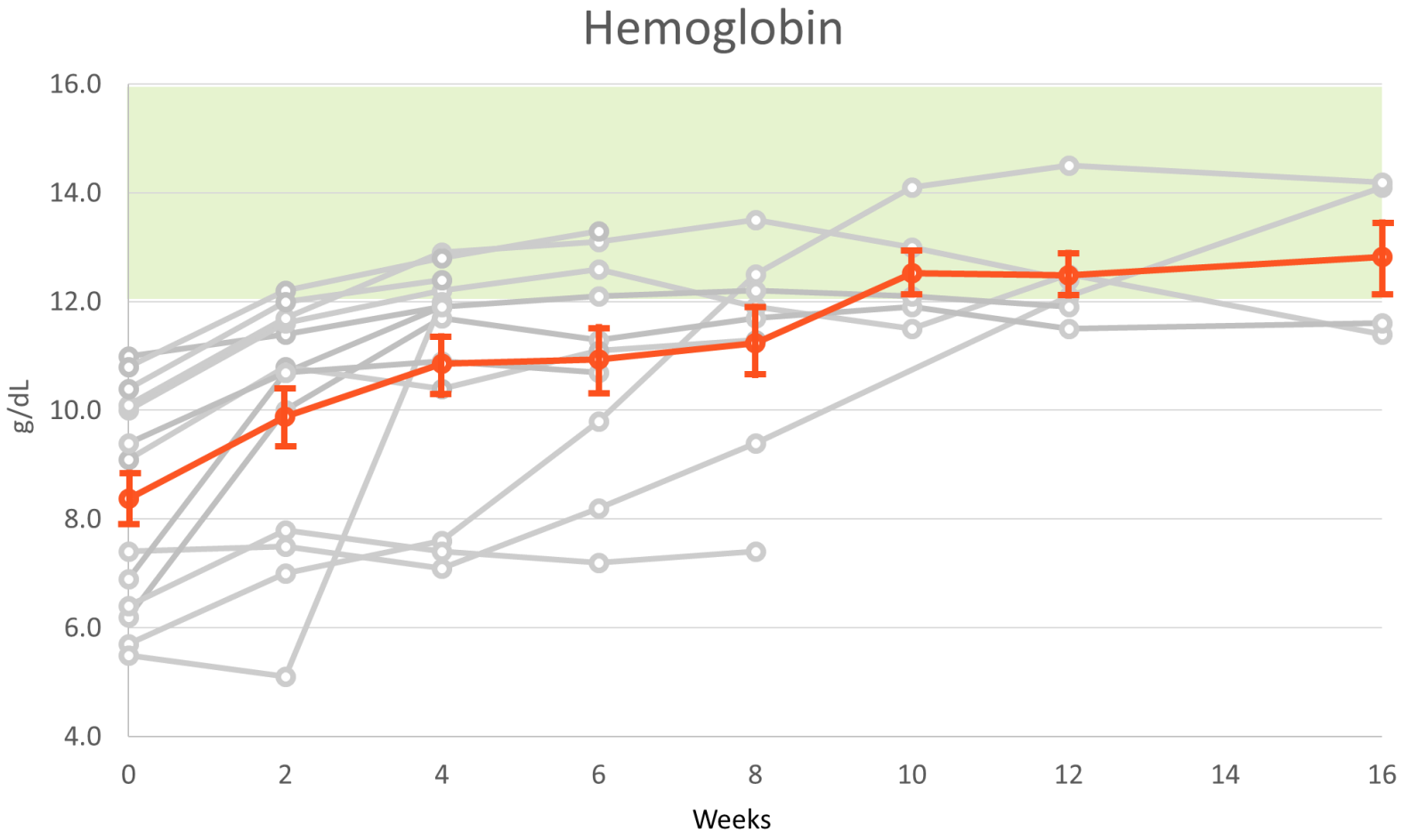
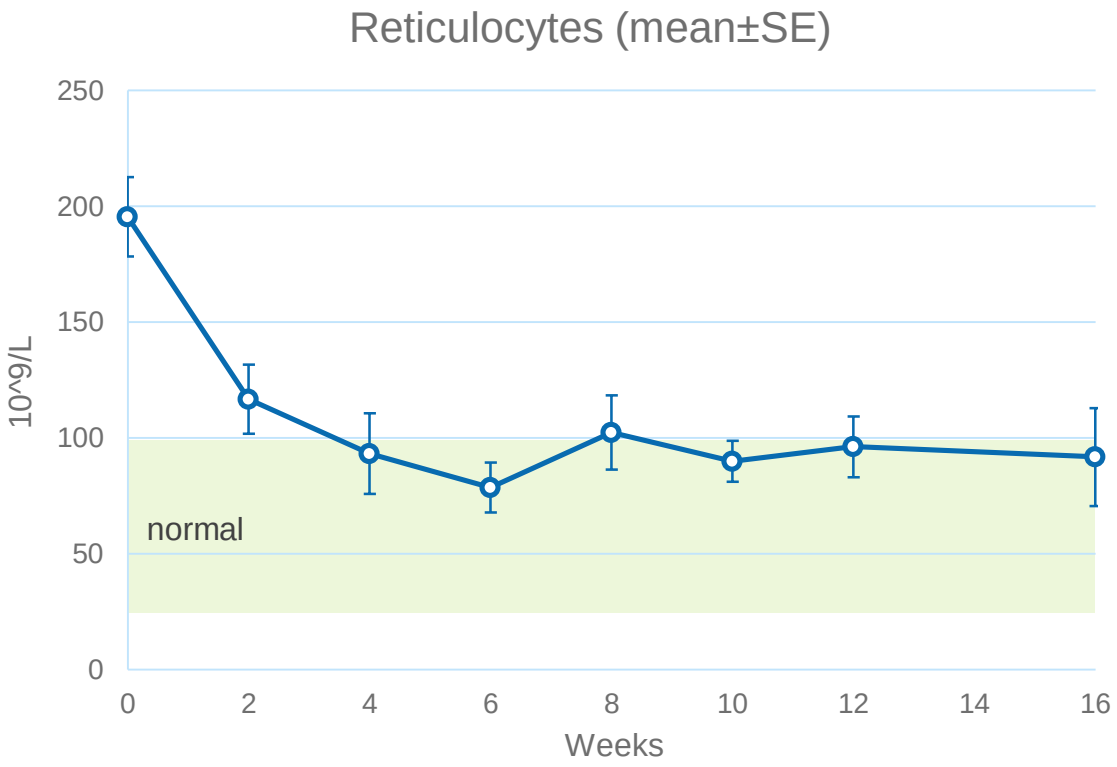
PADDOCK (interim): Mean hemoglobin improvement was 3.5 g/dL† (n=13) showing an impact on both intravascular and extravascular hemolysis



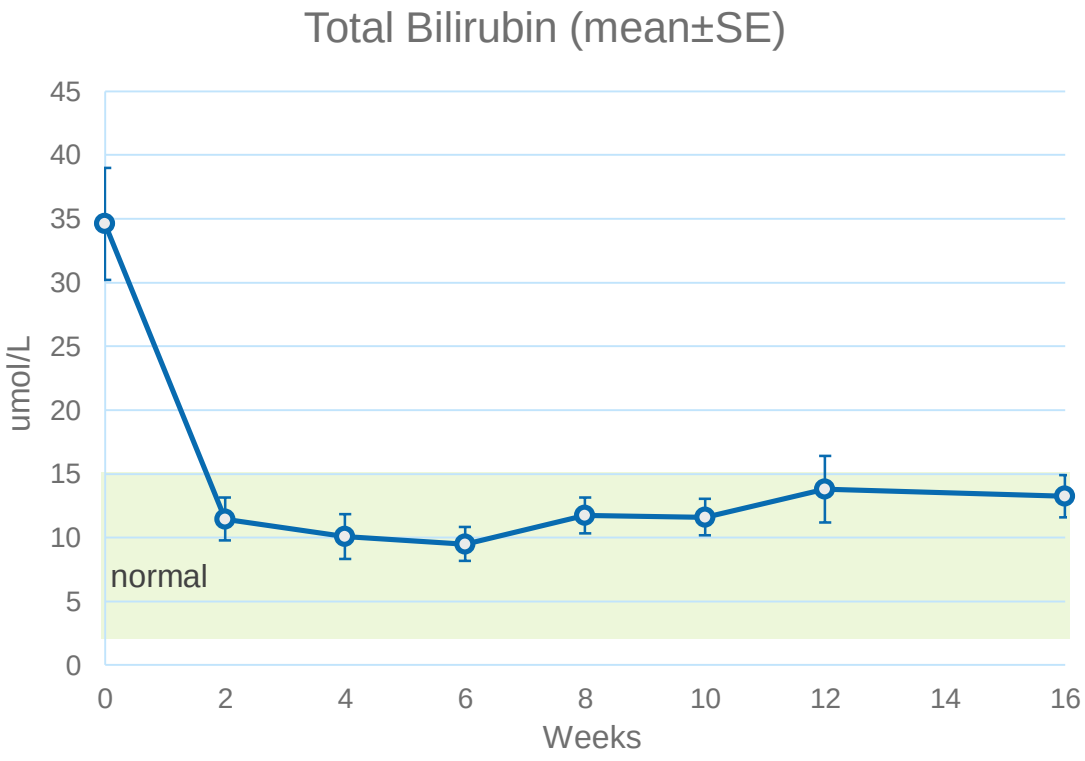
2/13 patients had transfusions, one at day 2 and a non-compliant patient at day 14; it is believed that neither patient had yet reached sufficient exposure to APL-2 for hematological benefit

† 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		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		13

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APL-2 in PNH: PADDOCK - Key Takeaways

- Hemoglobin increases are clinically meaningful and different compared to C5 inhibitors
- “Normalization” of hemoglobin, reticulocyte count and bilirubin rarely seen with C5 inhibition
- APL-2 is well tolerated – two lead-in transfusions (not on pharmacology). Safety profile of APL-2 to date is very good
- Potential for meaningful superiority vs. C5 inhibitors

Apellis

Paroxysmal Nocturnal Hemoglobinuria (PNH): Patient Perspective

BARRY KATSOF

President, Canadian Association of PNH Patients

Apellis

APL-2 in PNH: PHAROAH Trial Update

Anita Hill, MBChB (Hons), MRCP, FRCPath, PhD
Consultant Haematologist and Lead Clinician
for the National PNH Service, UK

APL-2 in PNH: PHAROAH - Key Takeaways

4 Patients Ongoing	• Hemoglobin stable & transfusion independent
Soliris Dose Reduction	• Successful dose reduction to approved 900mg/EOW in all high dose patients (100%) • All hematologically unchanged (Hb, LDH stable) • No transfusions required post-Soliris dose reduction
Soliris withdrawal -> APL-2 Monotherapy	• APL-2 increase from 270mg -> 360 mg/day • 3/4 successfully withdrawn from Soliris (1×270 mg, 2×360 mg) • 1 patient TBD: - Two unsuccessful attempts (1×270mg, 1×360mg) - Patient morbidly obese (BMI 46) - IRB approval for for dose increase to 440mg

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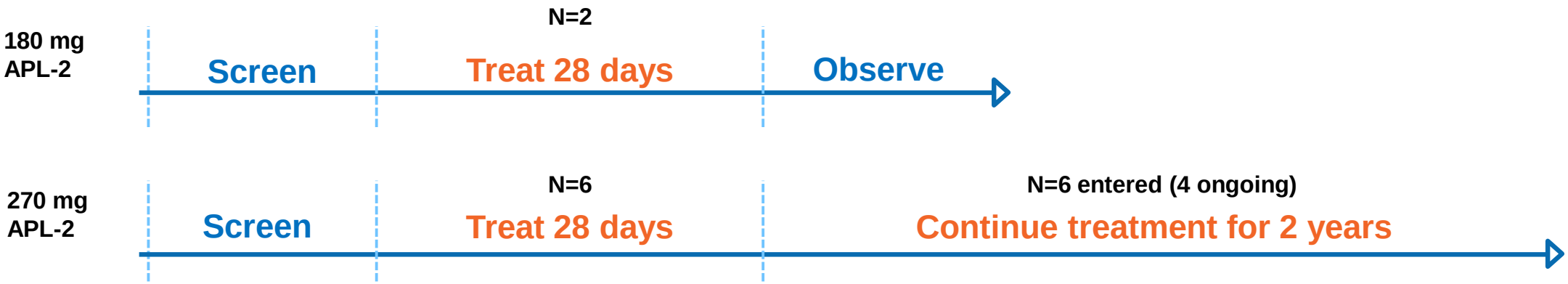
PHAROAH

Key eligibility criteria:

- Diagnosis of PNH (WBC clone > 10%)
- On treatment with eculizumab for at least 3 months
- Hb < 10 g/dL at screening OR have received at least one transfusion with 12 months prior to screening
- Platelet count of > 30,000/mm^3 and ANC > 500 x 10^9/L

Study design – Open label MAD



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PHAROAH - Baseline characteristics

6 Subjects enrolled by October 2016

- 35 combined transfusions in the 12 months before APL-2
- 5 out of 6 receiving higher than approved Soliris dose

Investigator and patient decision to stepwise change to standard of care based on effect observed:

- Reduce Soliris to approved dose of 900 mg bi-weekly
- Soliris withdrawal and continue with APL-2 monotherapy

	M/F	Age	BMI	Soliris dose		PRBC Transfusions in last 52 weeks		Hb NR (12-15)	LDH NR (110-209)	Bilirubin NR (0.2-1.2)	Reticulocytes (NR 39-123)	Months on Study	Ongoing
		(Y)		mg	freq	N	Units	g/dL	l/U	mg/dL	10^9/L		Y/N
Patient 1	F	50	35.2	1200	x2w	1	1	8.4	199	1.8	362	22	Y
Patient 2	F	52	22.8	900	x2w	9	18	9.6	225	1.9	296	21	Y
Patient 3	F	35	59.2	1200	x2w	1	1	7.4	535	1.8	160	7	N
Patient 4	F	57	27.8	900	x1w	4	6	10.5	211	2.0	272	20	Y
Patient 5*	F	38	25.6	1200	x2w	10	15	9.8	307	1.3	515	8	N
Patient 6	F	48	45.9	900	x1w	9	9	7.0	204	2.7	398	20	Y
MEAN								**8.8**	**280 x1.3 ULN**	**1.9 X1.6 ULN**	**334 (x3 ULN)**		

* Number given is true baseline in year before entering study for the 1st time

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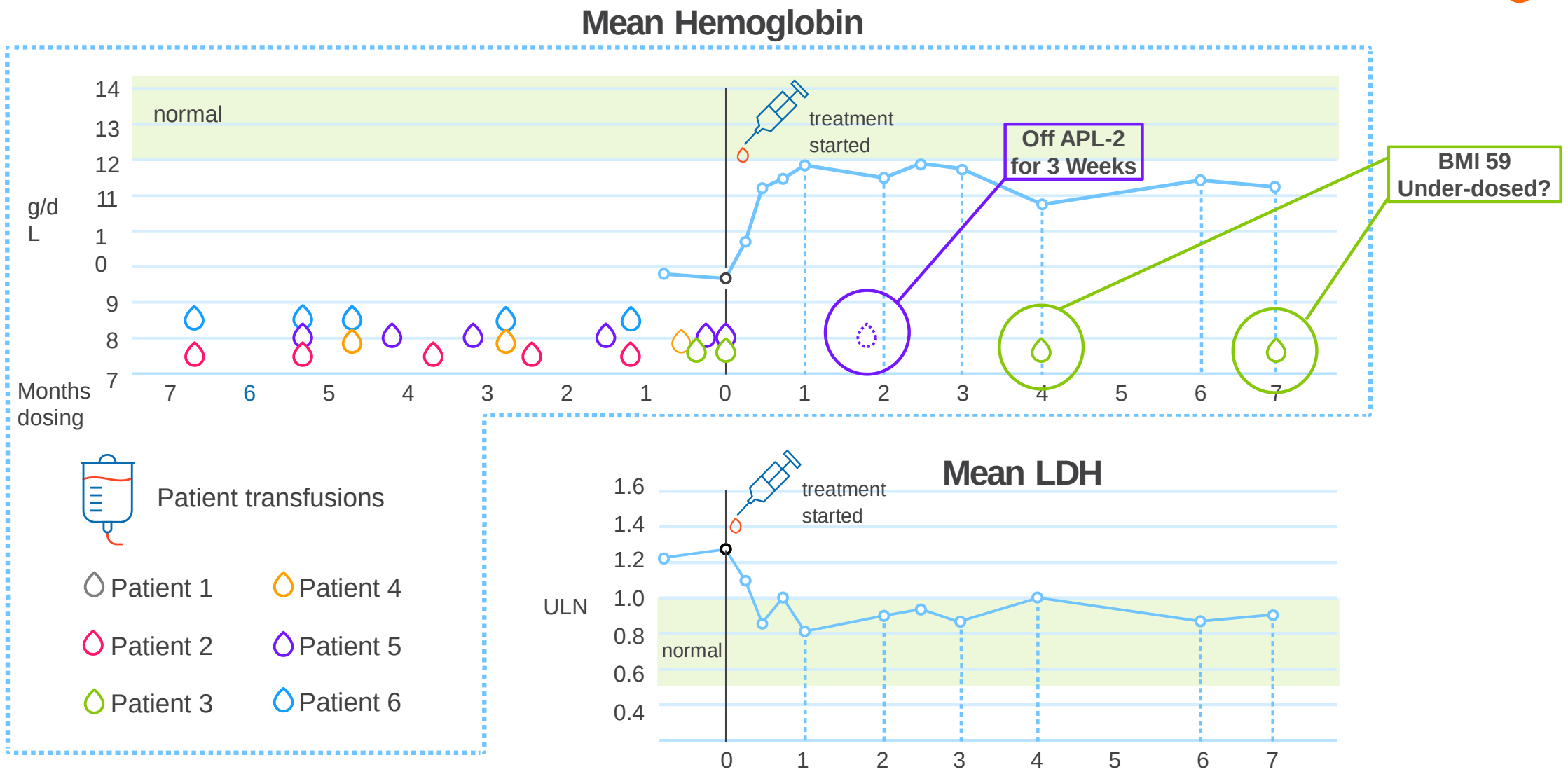
PHAROAH (interim): APL-2 shows potential to improve eculizumab outcomes as add-on therapy in PNH – 270 mg/day, (n=6)



Interim data as reported June 2017

PHAROAH Status – Month 12

2 Patients discontinued from the study:

Patient 3: Discontinued at month 7

- Morbidly obese (BMI 59)
- History of MS and renal/urinary co-morbidities
- Required x2 transfusions whilst on APL-2
- APL-2 dose increase to 360 mg appeared to improve response but subject had to withdraw after 28 days

Patient 5: Discontinued at month 8

- Subject participated in 3 dose-escalation cohorts at her request
- APL-2 was temporarily discontinued to allow investigation of a biliary obstruction
- Required transfusion while off APL-2 for 3 weeks
- Withdrawn from study due to pregnancy at month 8

Both Patients and their Physicians have requested that patients resume APL-2

4 Patients ongoing and continue to receive daily doses of APL-2

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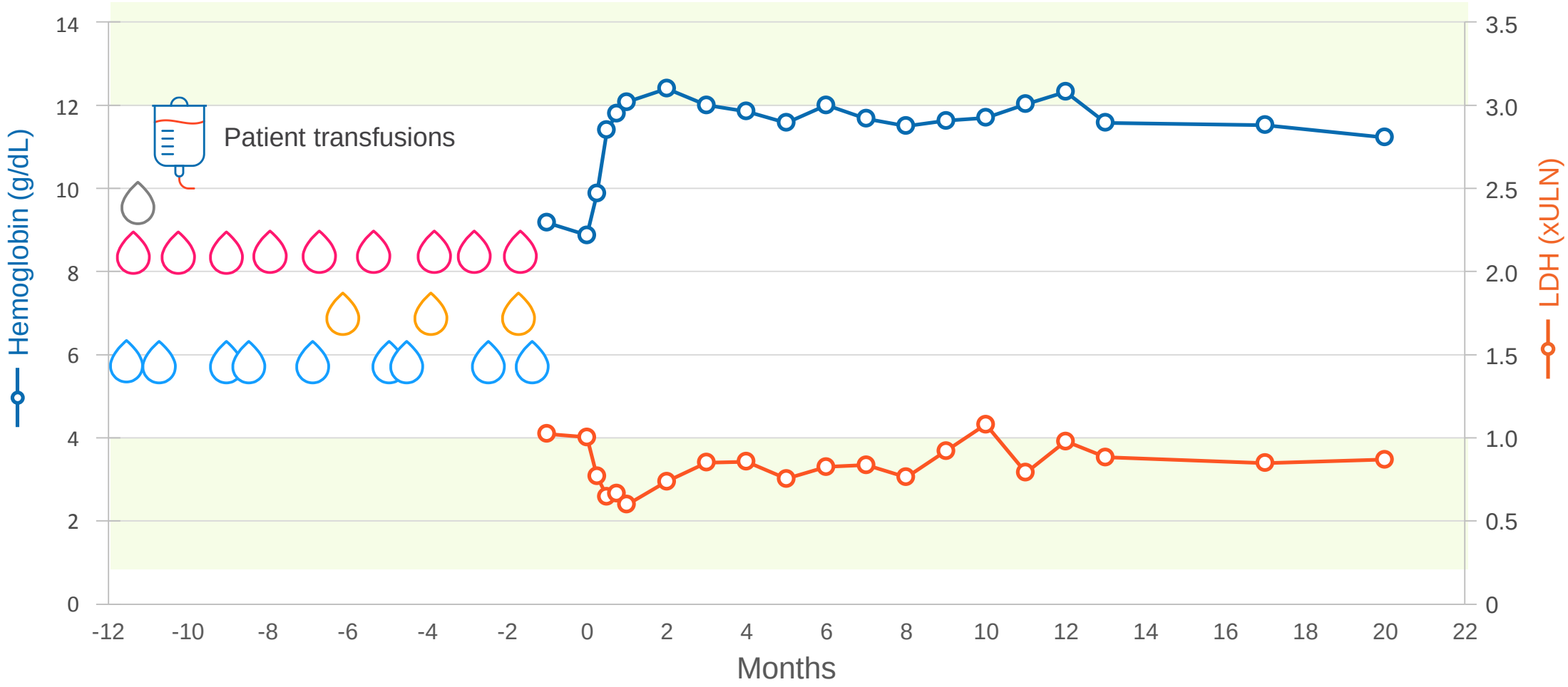
PHAROAH : APL-2 add-on to Soliris® four patients -1 yr.



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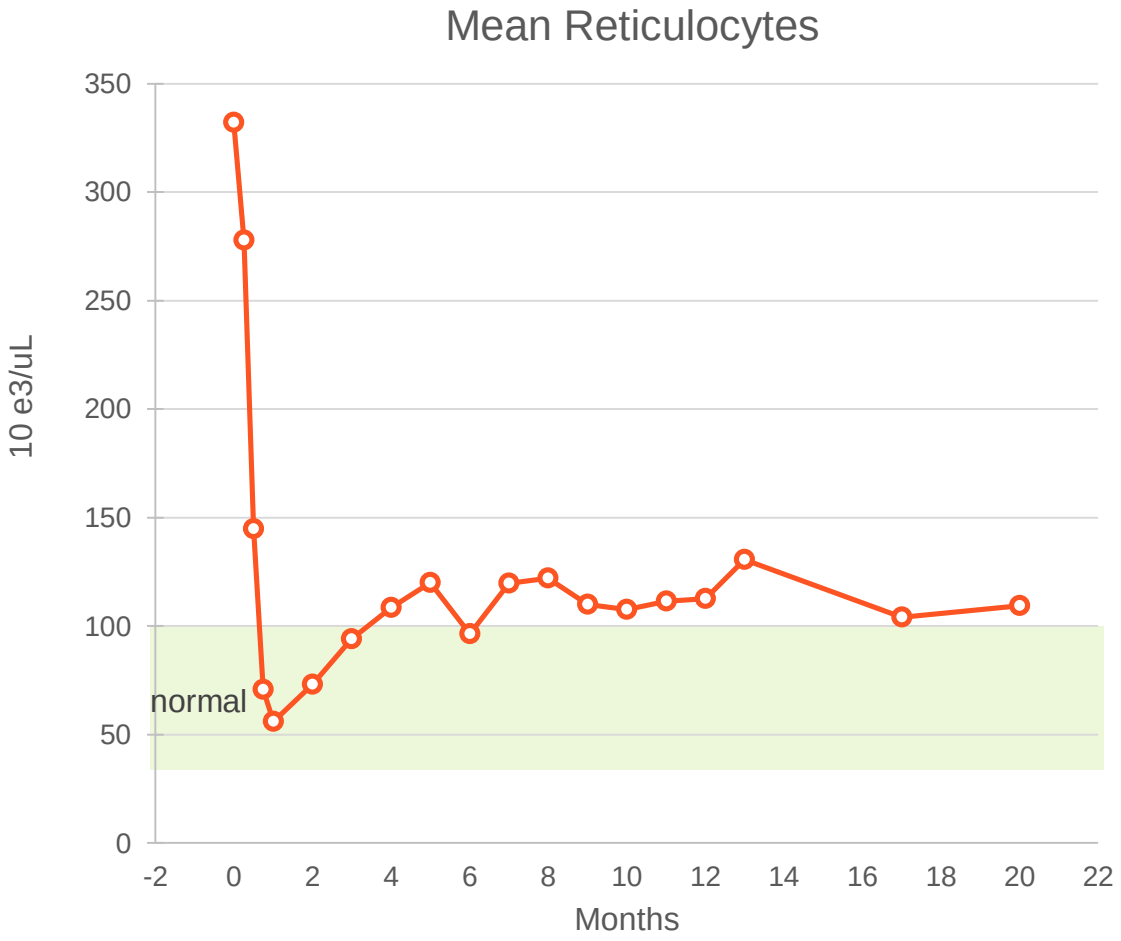
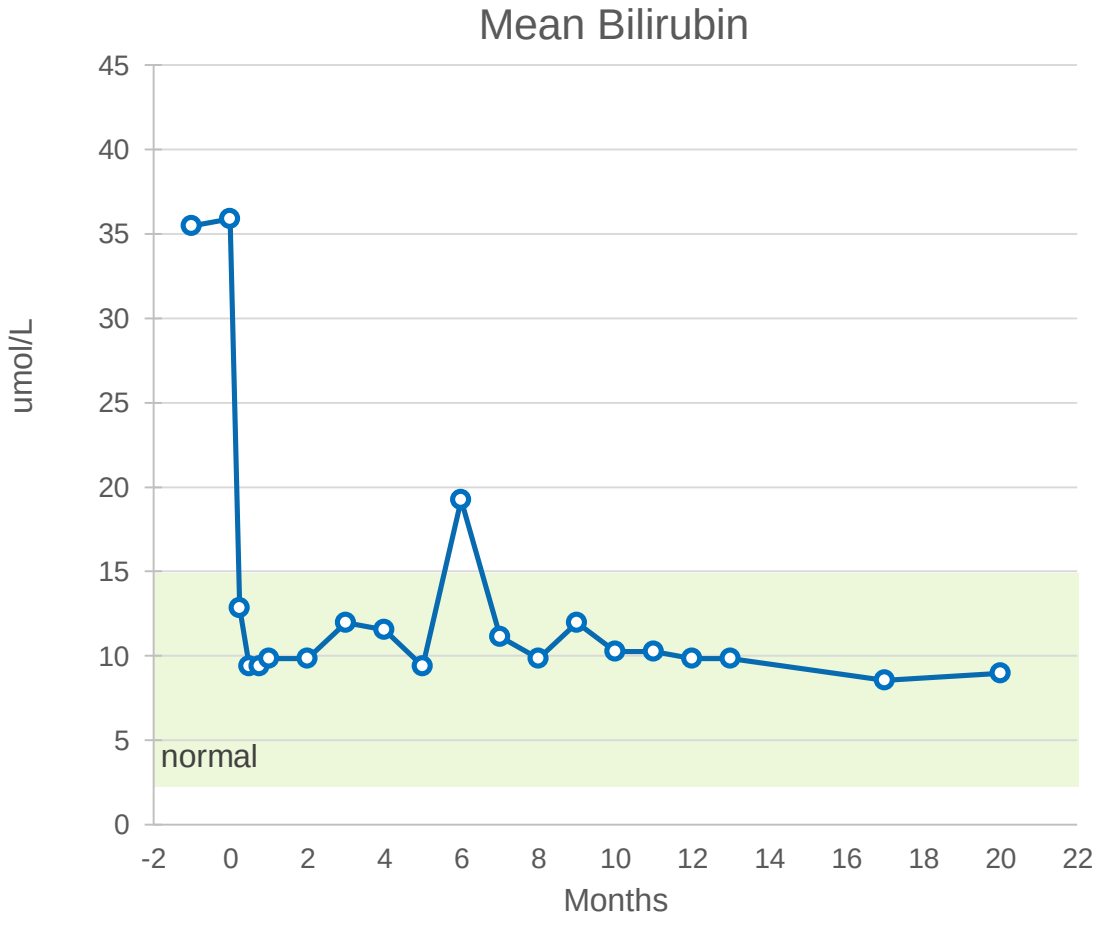
PHAROAH other measures of anemia meaningfully improved with APL-2 including reticulocytes and bilirubin (n=4)





Only for patients on study at 1 year (Patients 1,2,4,6)

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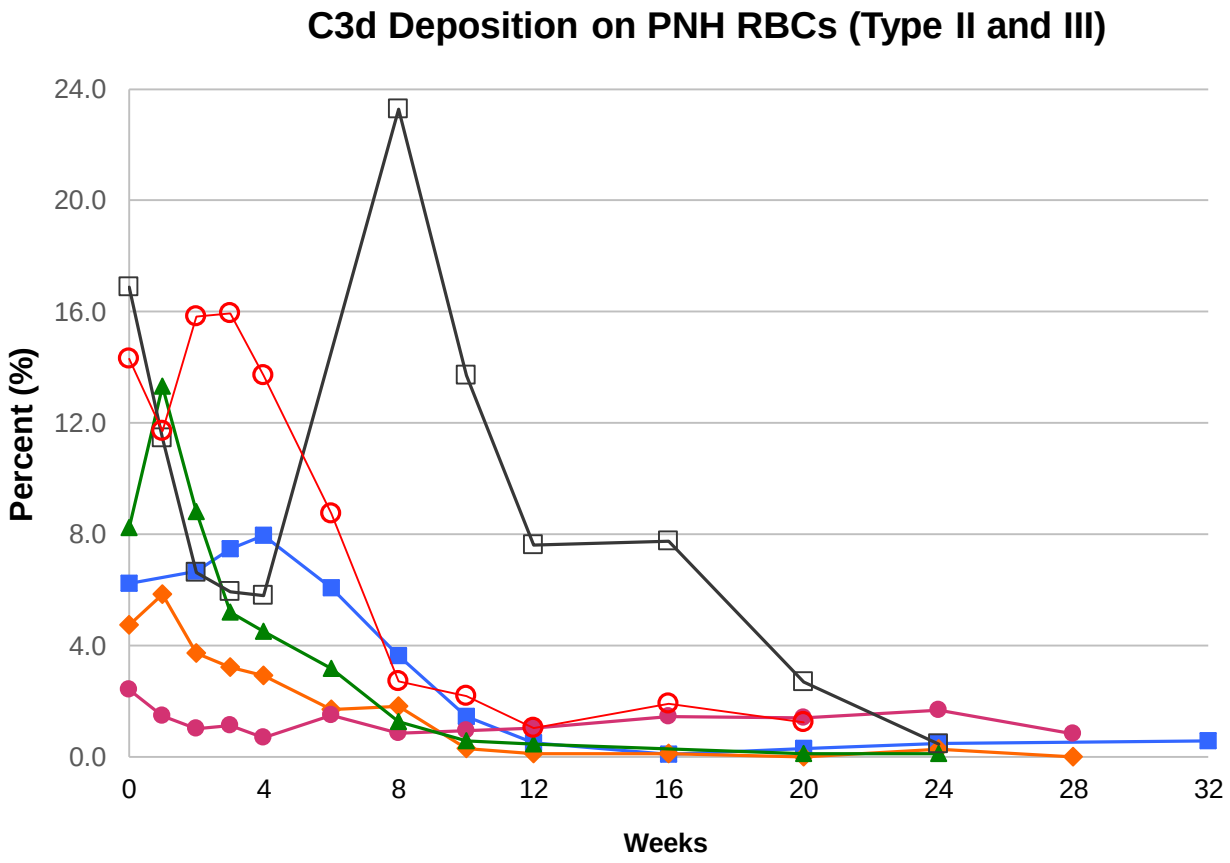
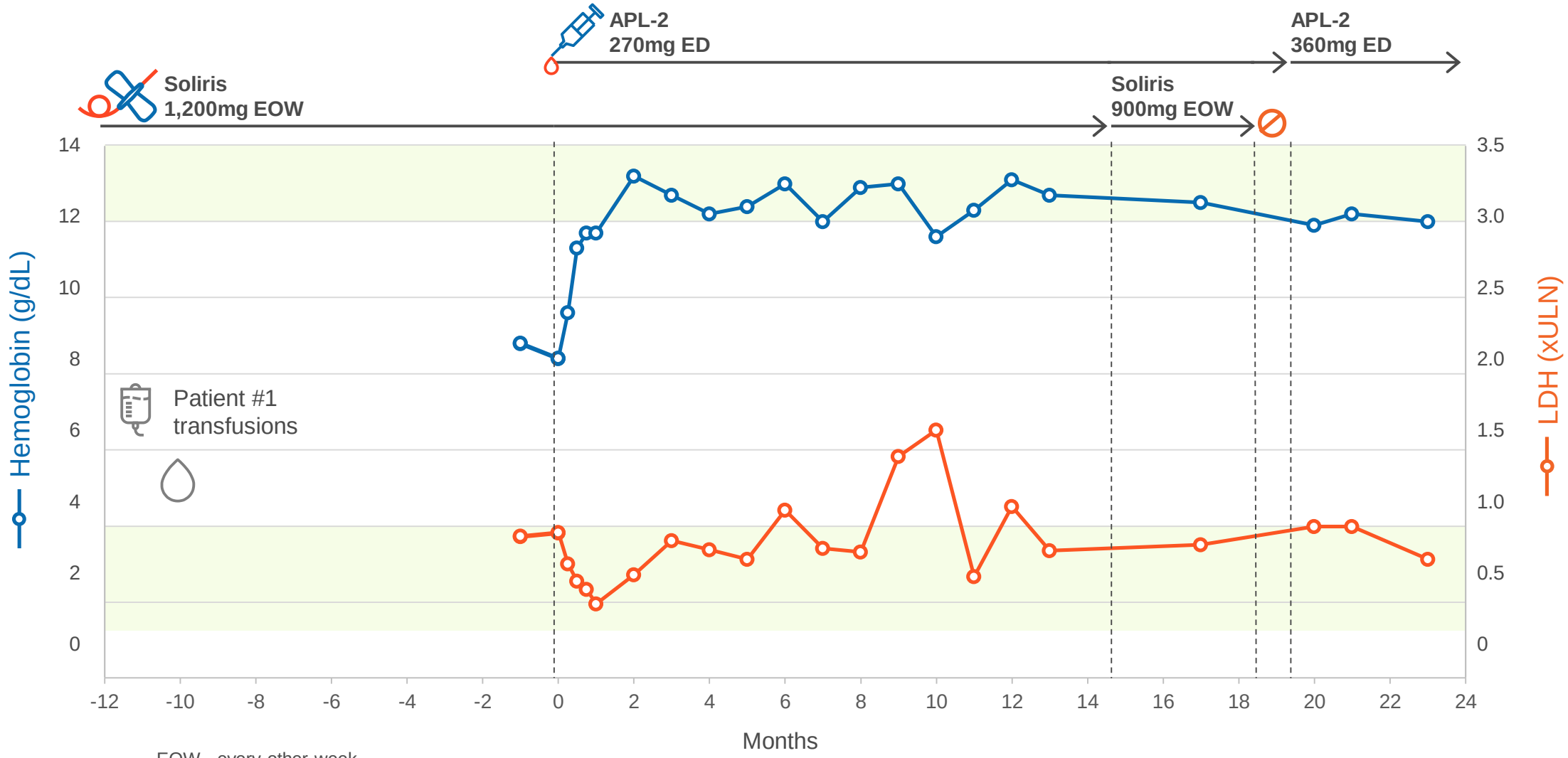
PHAROAH: C3d deposition



Patient #1



EOW - every other week
ED - every day

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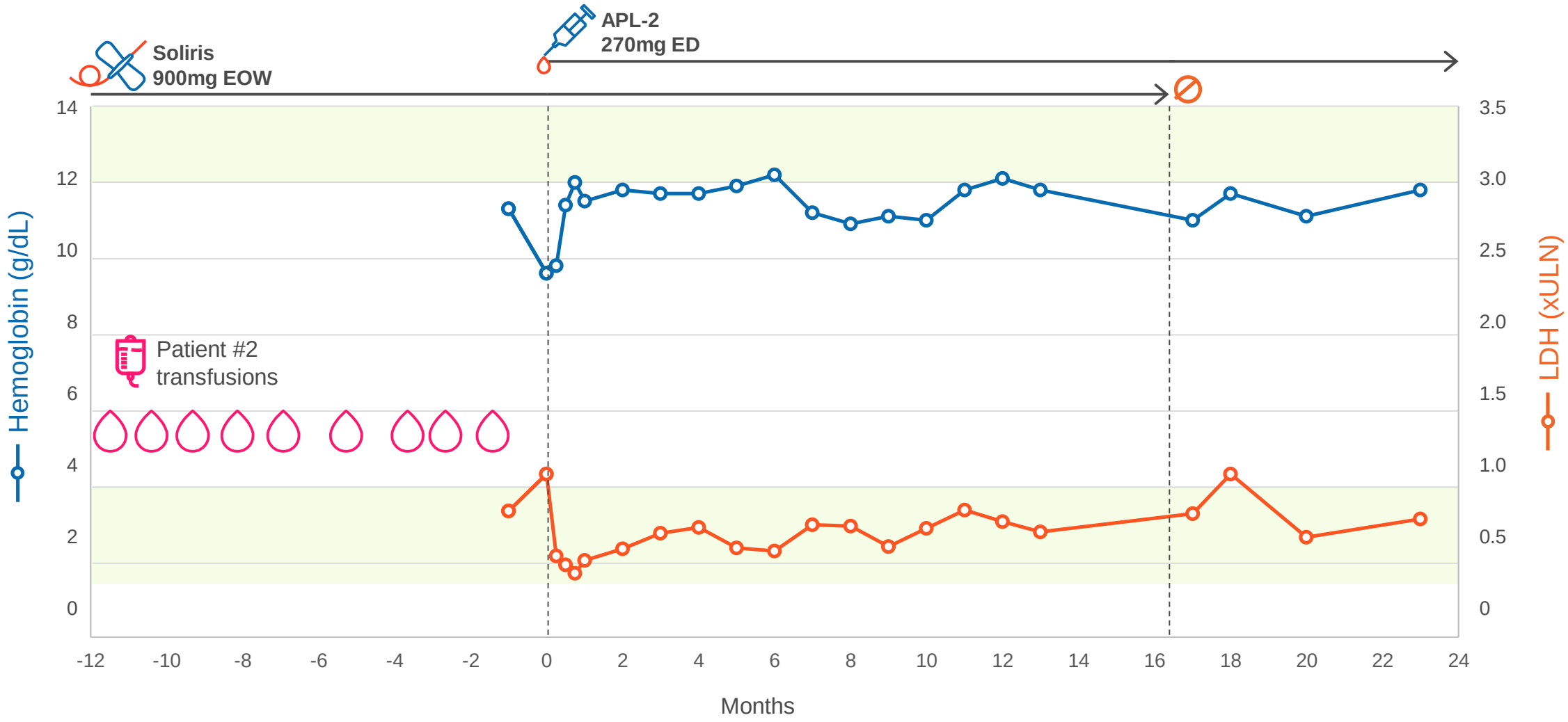
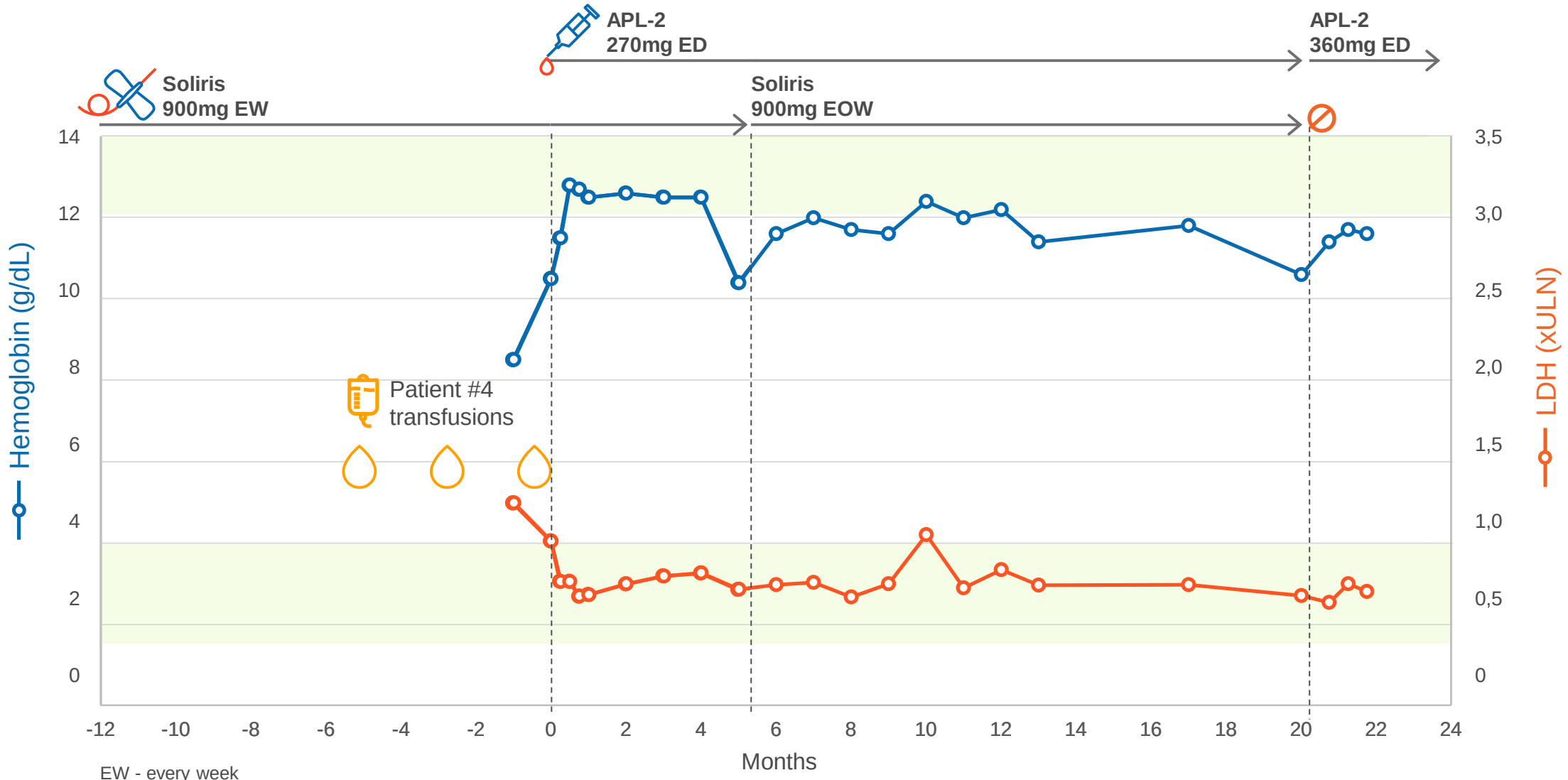
Patient #2



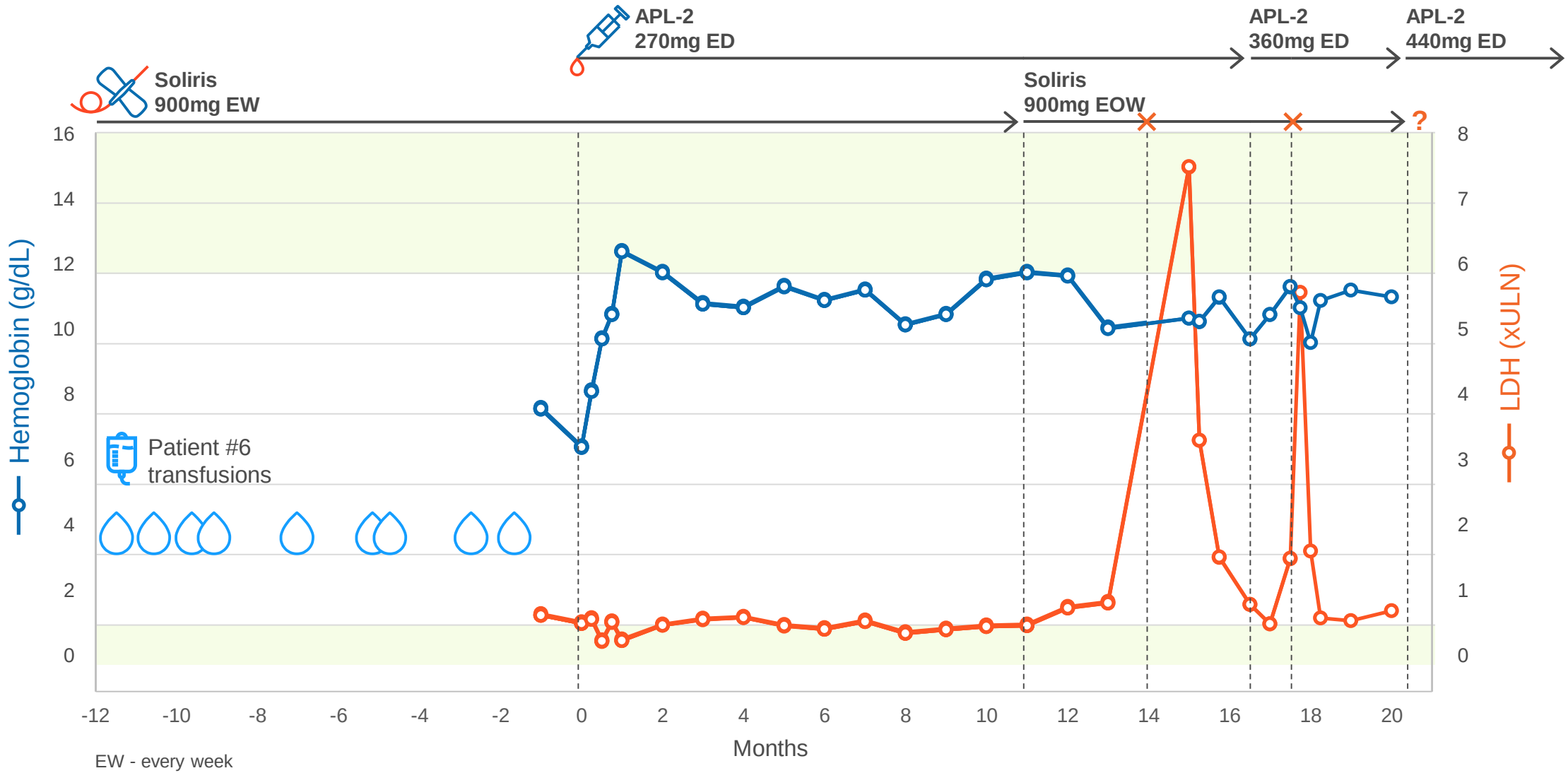
EOW - every other week
ED - every day

Patient #4



EW - every week
EOW - every other week
ED - every day

Patient #6



EW - every week
EOW - every other week
ED - every day

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APL-2 in PNH: PHAROAH - Key Takeaways

4 Patients Ongoing	• Hemoglobin stable & transfusion independent
Soliris Dose Reduction	• Successful dose reduction to approved 900mg/EOW in all high dose patients (100%) • All hematologically unchanged (Hb, LDH stable) • No transfusions required post-Soliris dose reduction
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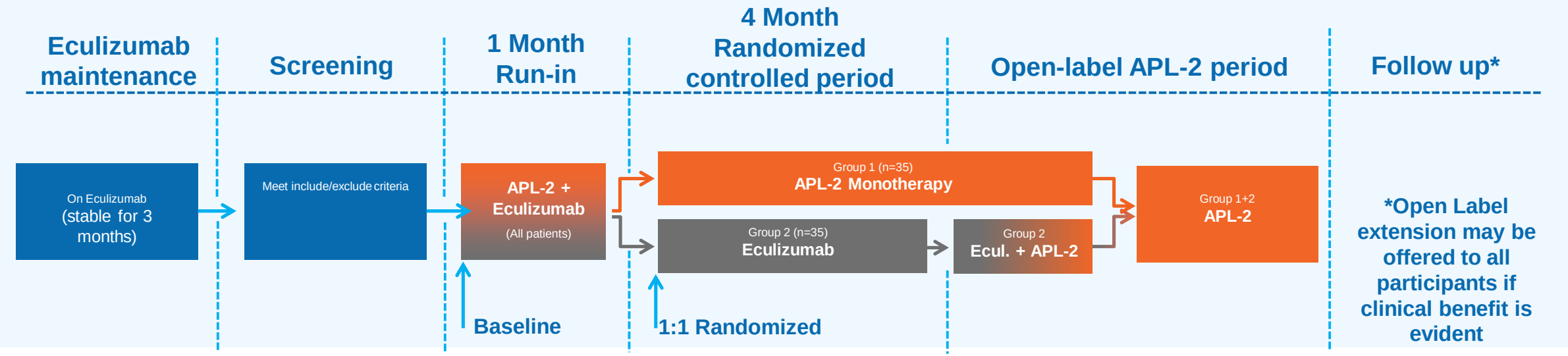
APL-2 In PNH – Phase III Program

Anita Hill, MBChB (Hons), MRCP, FRCPath, PhD
Consultant Haematologist and Lead Clinician
for the National PNH Service, UK

Phase III Program - Key Takeaways

- Phase III designed for superiority and patient/physician reality
- Data to date suggest markedly improved hematology vs. Soliris
- Blocking extravascular hemolysis likely to translate into clinically meaningful improvement in Hb (e.g. transfusion avoidance), reticulocytes, bilirubin, and comparable LDH to C5 inhibitors

PEGASUS (n=70) - Study outline



Parameter	
Population	PNH patients on eculizumab who continue to be anemic (Hb < 10.5 g/dL at screening)
Primary Endpoint	Week 16 change from baseline in hemoglobin level
Design	Randomized 1:1 APL-2:eculizumab. 1 Month Run In; 4 Month Randomization;6 Month Open Label; 1 Month Follow Up
Posology	Dose 1080 mg 2/week, SC infusion via injection pump
Sample size	70 patients (35/group) randomized 1:1 provides 90% power with 2-sided=0.05 to detect a difference of 1 mg/dL.

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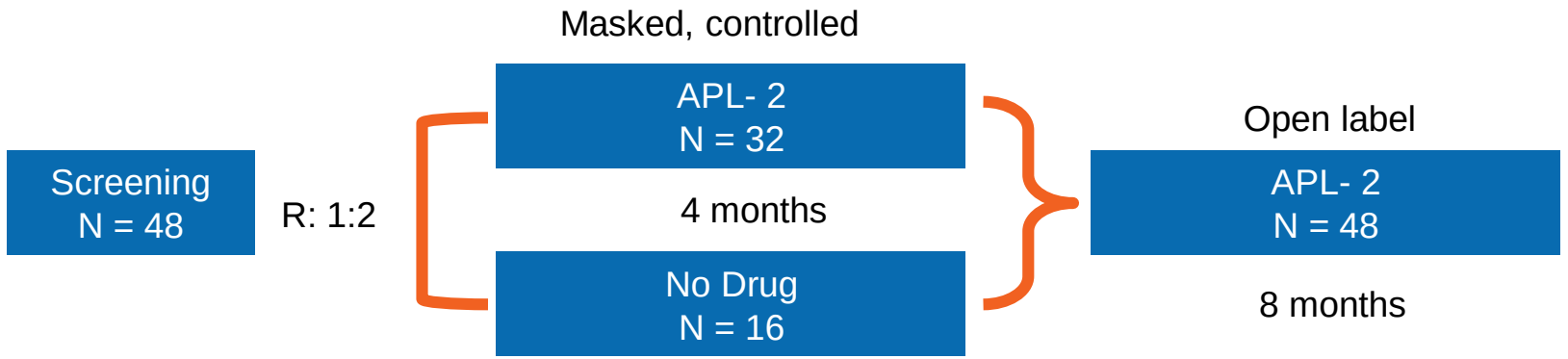
Tx Naïve Study (n=48) - Study outline



Parameter	
Population	Patients with PNH (not on eculizumab)
1º Endpoint	Hb stabilization, defined as avoidance of a decrease in Hb levels from baseline in the absence of transfusion through Week 16
Design	Randomized 2:1 APL-2:No-treatment, 4 months No-treatment controlled period followed by 8 months open label period
Posology	Dose 1080 mg, twice/week SC injection via injection pump
Sample size	48 patients randomized 2:1 (32:16) to APL-2:No-treatment provides 90% power with 2-sided =0.05 to detect a 45% difference in Hb stabilization (assuming a No-treatment rate of 5% and an APL-2 rate of 50%).

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Phase III Program - Key Takeaways

- Phase III designed for superiority and patient/physician reality
- Data to date suggest markedly improved hematology vs. Soliris
- Blocking extravascular hemolysis likely to translate into clinically meaningful improvement in Hb (e.g. transfusion avoidance), reticulocytes, bilirubin, and comparable LDH to C5 inhibitors

APL-2 in PNH - Key Takeaways

- Hematological correction significant & clinically meaningful
- Only one patient out of 20 required transfusions while on adequate APL-2 (5%), likely under-dosed (BMI = 59)
- Switch from eculizumab (C5) achieved with retained superior hematology to eculizumab monotherapy
- Well tolerated

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APL-2 In Paroxysmal Nocturnal Hemoglobinuria

Q&A

Apellis 2018 R&D Day

EARLY STAGE PIPELINE

Early Stage Pipeline:
APL-2 in Autoimmune Hemolytic Anemia (AIHA) & Complement Dependent Nephropathies (CDN)

Cedric Francois, MD, PhD,
Founder and Chief Executive Officer,
Apellis Pharmaceuticals

Apellis

APL-2 in Autoimmune Hemolytic Anemia (AIHA)

PLAUDIT

https://aihaclinicaltrial.com/

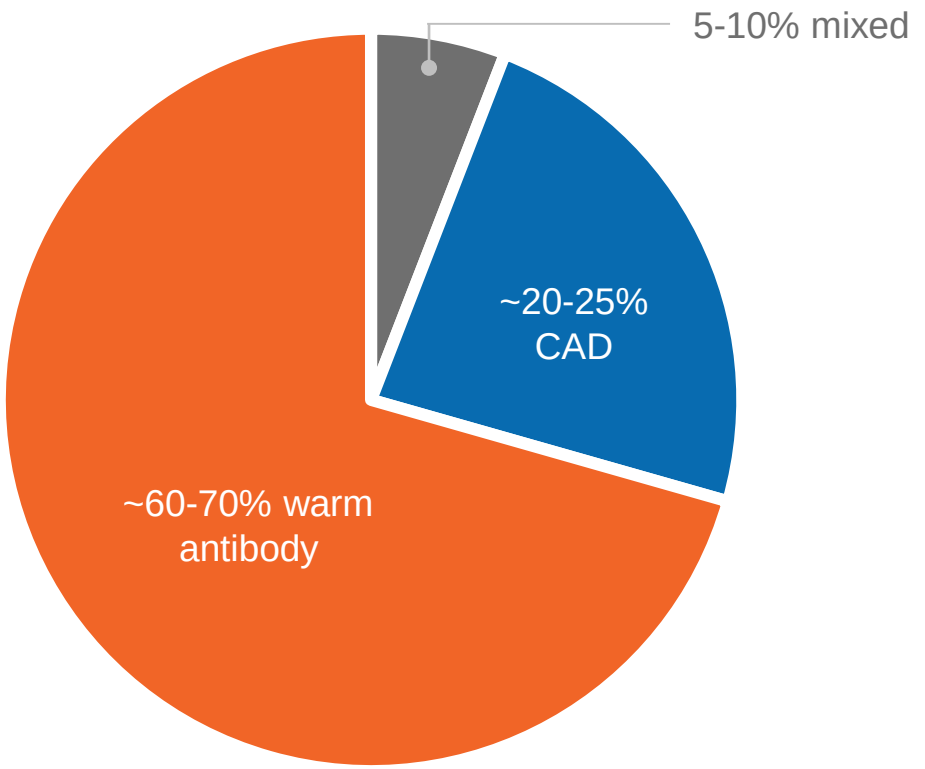
AIHA Overview

DISEASE

- Increased destruction of RBCs due to the presence of autoantibodies (decreased RBC life span)
- Estimated incidence 1-3 per 100,000/year, prevalence 17 per 100,000, mortality 8-11%
- Hemolysis mediated by complement:
  - Warm antibody (wAIHA): ~20% of patients
  - Cold agglutinin disease (CAD): majority of patients

STANDARD OF CARE

- wAIHA: Corticosteroids, Rituximab, splenectomy and transfusion.
- CAD: no effective treatment therapy, corticosteroids, alkylating agents, Rituximab (off label), IVIG, or transfusion.



AIHA Population

Reynaud Q., Durieu I., Dutertre M., et al. Efficacy and safety of rituximab in auto-immune hemolytic anemia: a meta-analysis of 21 studies. Autoimmunity Reviews. 2015;14(4):304–313.

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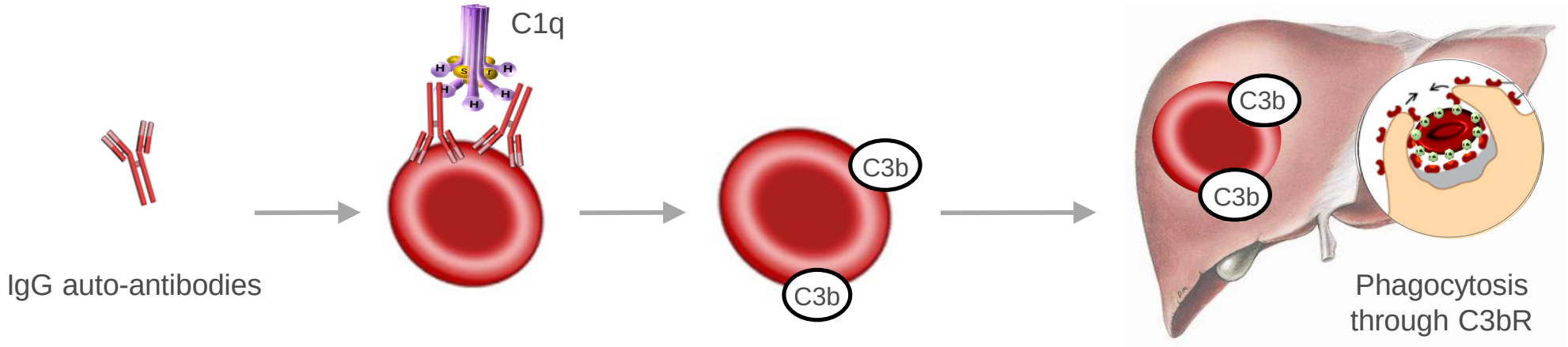
Role of Complement in Warm AIHA

Optimum 37ºC

Autoantibody antigen complex activates complement classical pathway



Extravascular hemolysis

Role of Complement in CAD

At 37ºC

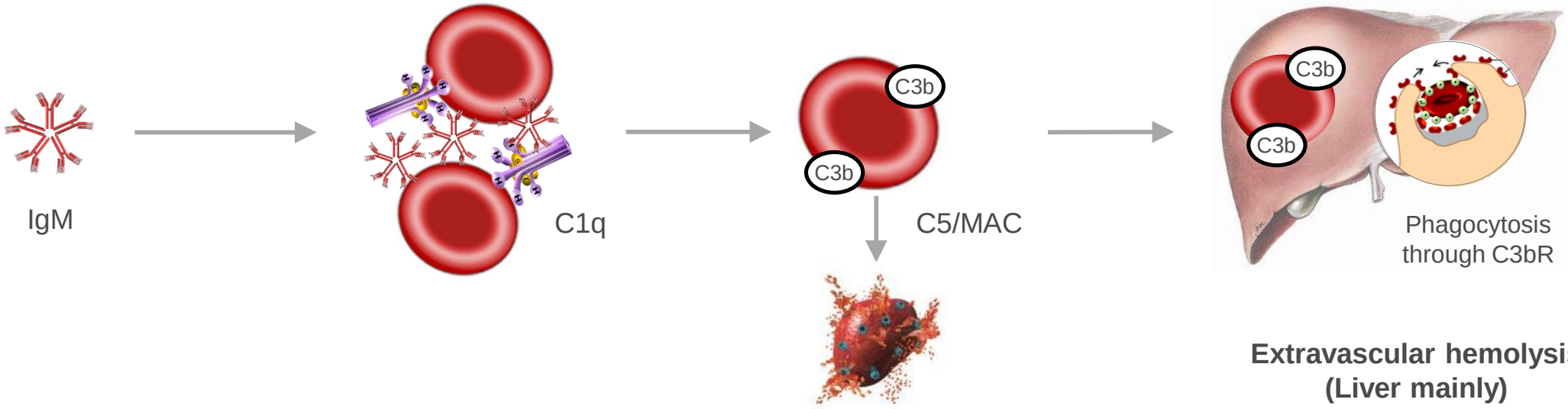
Production of cold agglutinin (CA) autoantibodies

Below 30ºC

In body extremities: IgM mediates RBC agglutination leads strong activation of CP

At 37ºC

Hemolysis upon recirculation to central parts of the body



Intravascular hemolysis
(patients w/ severe acute exacerbation)

Extravascular hemolysis (Liver mainly)

Unmet Need in AIHA

EFFICACY

- wAIHA: 15-30% non-responders to corticosteroids. Splenectomy: 33% patients w/ relapses.
- CAD: Steroids and splenectomy are not generally effective (10-14% response). Rituximab overall response: 45-66%, median time to response: 1.5 months, median duration of remission: 11 months.

SAFETY

- Long-term safety issue associated with high doses of corticosteroids
- Toxicity, clinically significant AE (neutropenia, infection) associated with Rituximab
- Rituximab also associated with rare disastrous outcomes, including fatal pulmonary fibrosis and PML
- Splenectomy associated with sepsis, postoperative thrombosis and pulmonary embolism. Transfusion can be life threatening.

1 Zanella A, Barcellini W. Treatment of autoimmune hemolytic anemias. Haematologica. 2014;99(10):1547-1554. doi:10.3324/haematol.2014.114561. 2 Swiecick,P, Livia T.,Hegerova and Morie A. Gertz Blood 2013 122:1114-1121

AIHA Study Overview

OVERVIEW

The study is to assess the safety, tolerability, preliminary efficacy, and pharmacokinetics of multiple subcutaneous (SC) doses of APL-2 in subjects with Warm Antibody Autoimmune Hemolytic Anemia (wAIHA) or Cold Agglutinin Disease (CAD)

PRIMARY SAFETY ENDPOINT

The incidence and severity of treatment emergent adverse events (TEAEs) following administration of multiple doses of subcutaneous (SC) APL-2

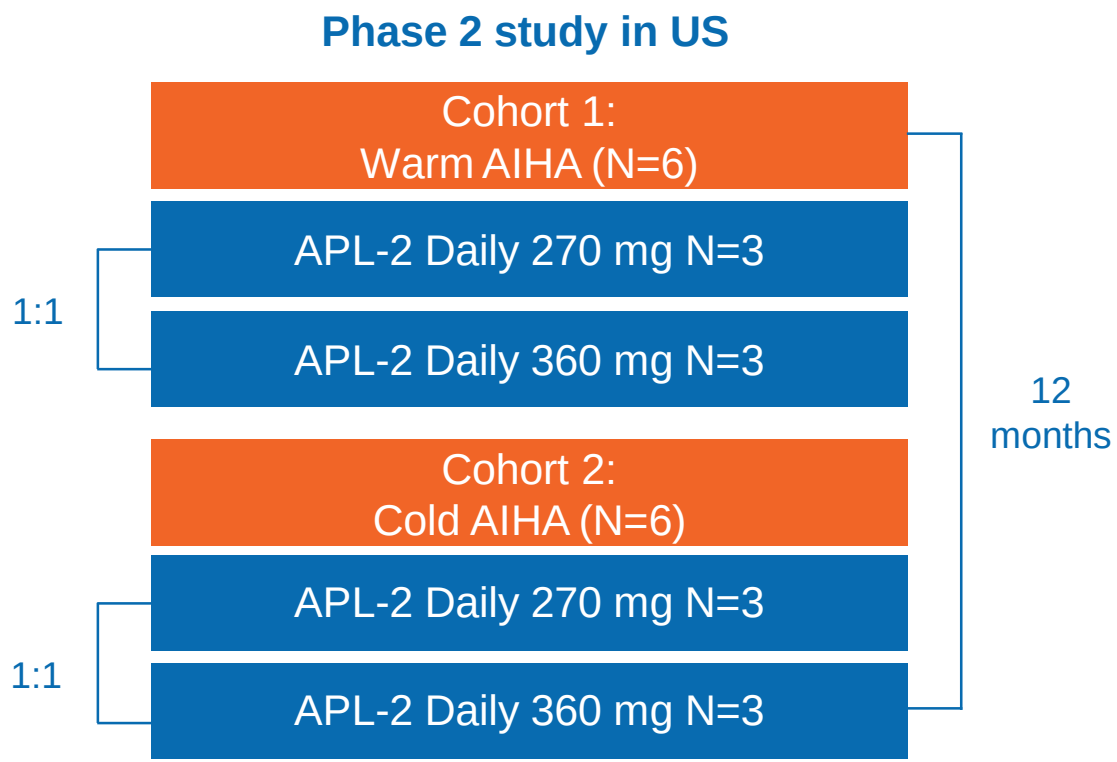
SECONDARY ENDPOINTS

Change from baseline in:

- Hb
- RBC transfusions during study
- absolute reticulocyte count
- LDH
- Haptoglobin
- Bilirubin
- Functional Assessment of Chronic Illness Therapy (FACIT) Scale
- Linear Analog Scale Assessment scale (LASA)

AIHA Proof of Concept Phase II Study Overview

Parameters	
Population	Patients with warm or cold AIHA (treatment naïve or previously treated)
Design	1:1 APL-2 270 mg/day: APL-2 360 mg/day, 12-month treatment
Posology	270 mg and 360 mg daily SC injection
Sample size	12 patients, 6 patients with warm AIHA and 6 patients with cold AIHA



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AIHA Key Inclusion Criteria

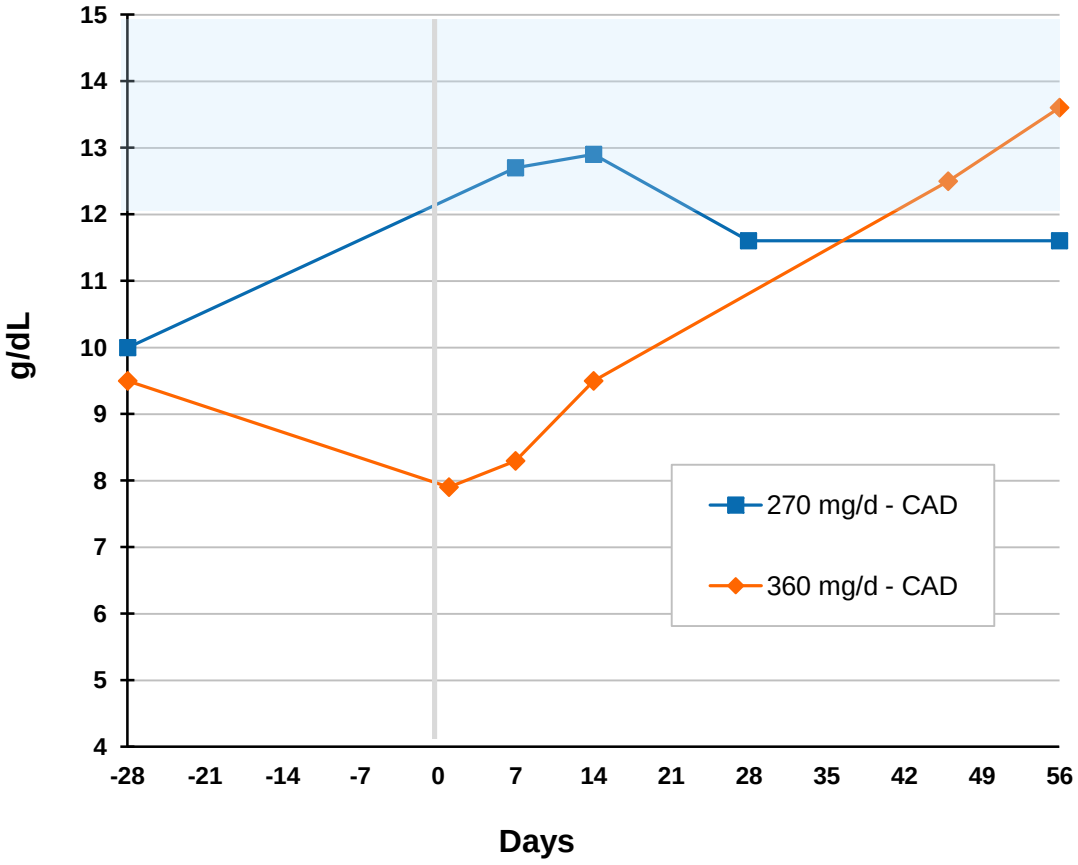
1. >18 years of age, <125 kg
2. Subjects must have a primary diagnosis of wAIHA or CAD defined by the presence of hemolytic anemia and positive DAT for wAIHA (IgG) or CAD (C3).
3. Hemoglobin <11 g/dL.
4. Specific for wAIHA: Relapsed from, did not respond to, or did not tolerate at least one prior wAIHA treatment regimen (such as prednisone, rituximab).

AIHA Key Exclusion Criteria

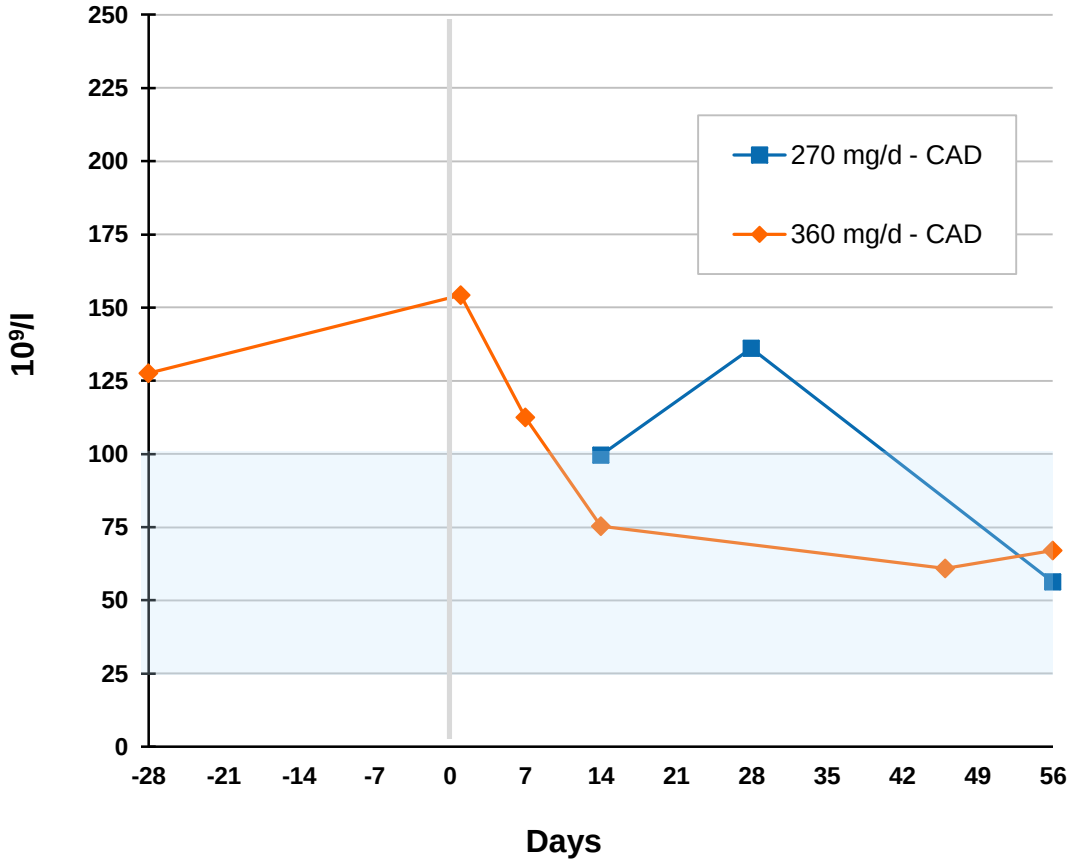
1. Prior treatment with rituximab within 90 days.
2. Deficiency of iron, folic acid and vitamin B12 prior to treatment phase.
3. Abnormal liver function (direct bilirubin above normal, and/or an AST/ALT level >2x upper limit of normal). Elevated indirect bilirubin due to hemolysis is not an exclusion criteria.
4. Active aggressive lymphoma requiring therapy or an active nonlymphatic malignant disease other than basal cell carcinoma or carcinoma in situ (CIS) of the cervix.
5. Presence or suspicion of active bacterial or viral infection at screening or severe recurrent bacterial infections
6. Pregnant, breast-feeding, or intending to conceive during the course of the study, including the Post-Treatment Phase.
7. Myocardial infarction, CABG, coronary or cerebral artery stenting and /or angioplasty, stroke, cardiac surgery, or hospitalization for congestive heart failure within 3 months or > Class 2 Angina Pectoris or NYHA Heart Failure Class >2

APL-2 in Cold Agglutinin Disease (n=2)

Hemoglobin



Reticulocytes



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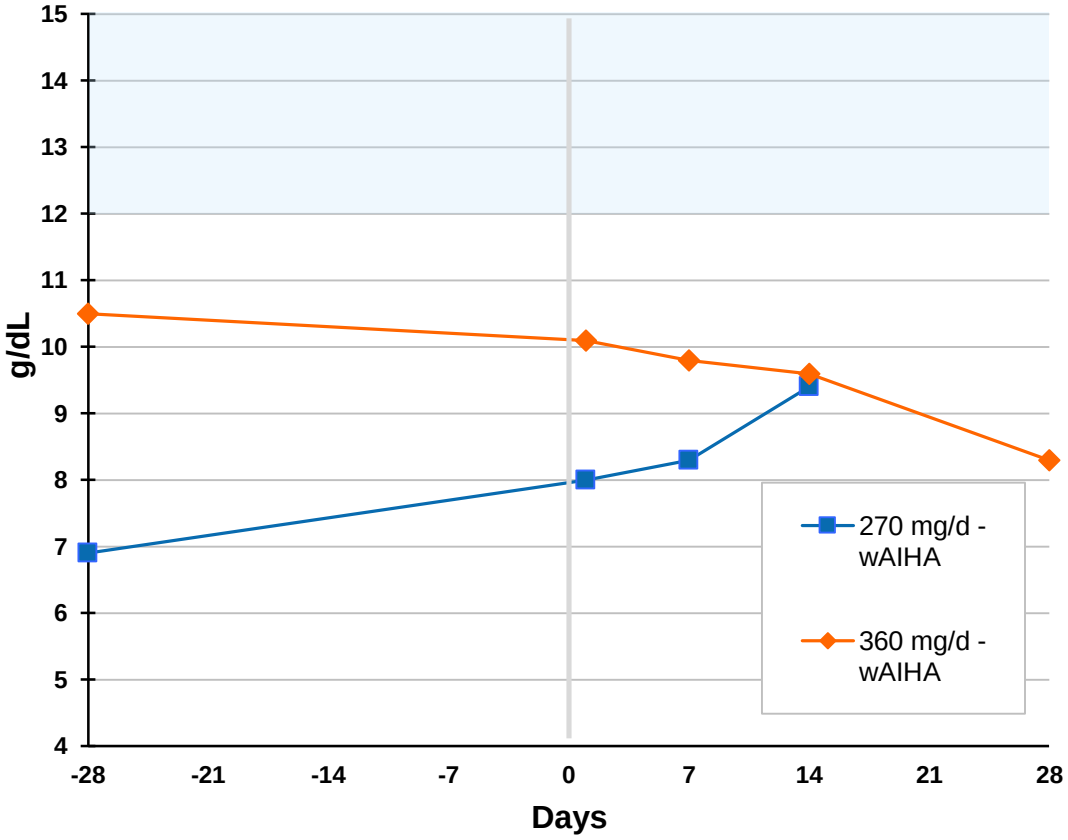
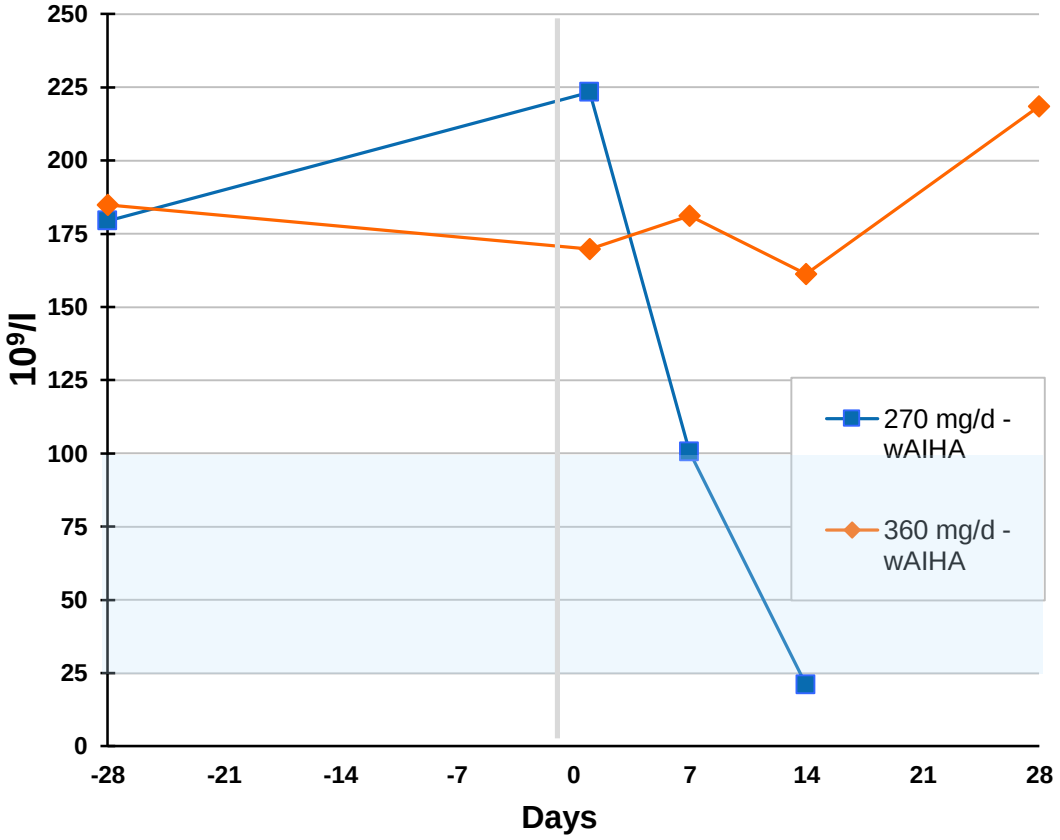
APL-2 in Warm Antibody AIHA (n=2)

Hemoglobin



Reticulocytes



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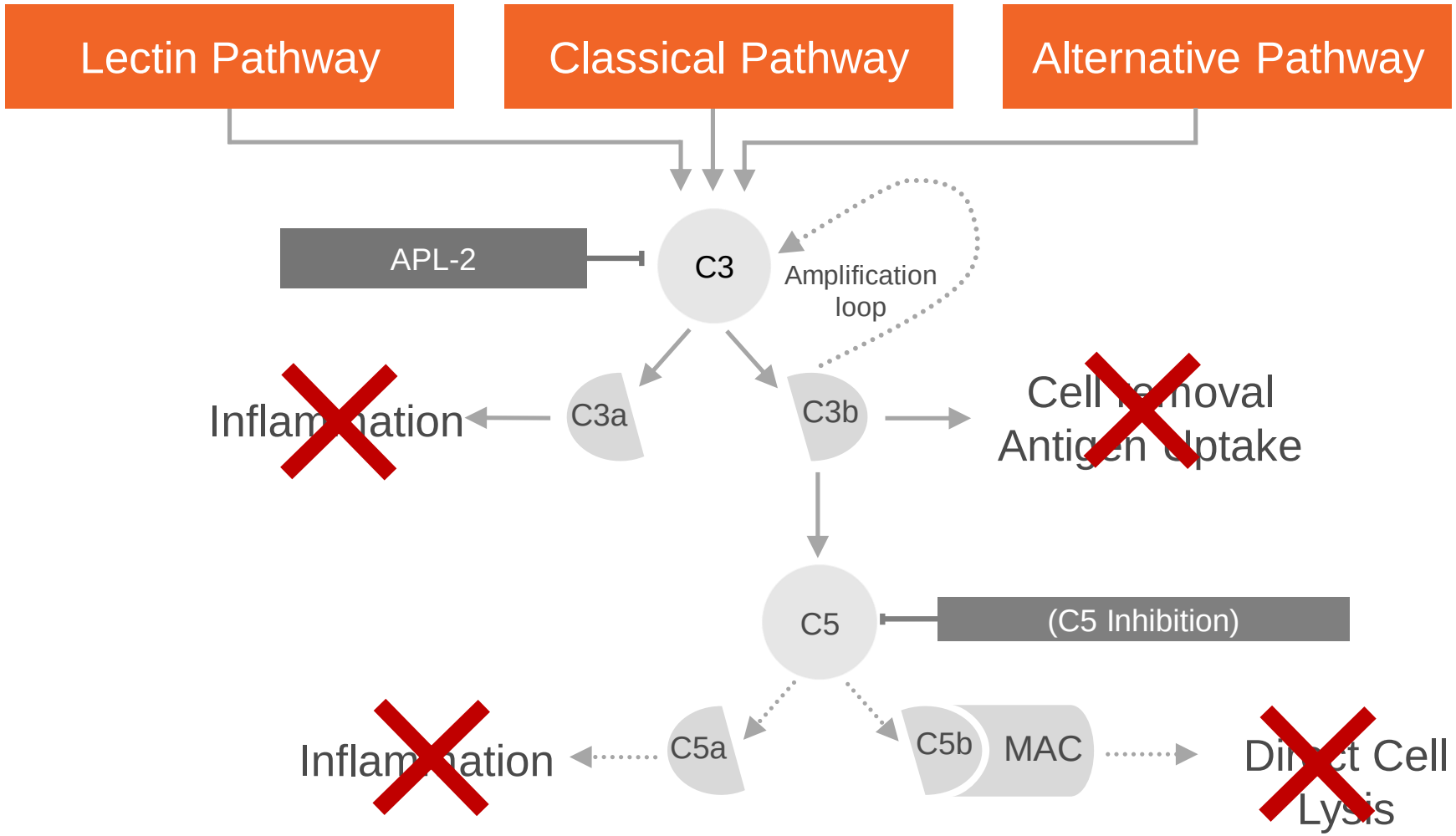
APL2-201

A Phase 2 Study to Evaluate the Safety and Biologic Activity of APL-2 in Patients With IgA Nephropathy, Lupus Nephritis, Primary Membranous Nephropathy, or C3 Glomerulopathy (C3 Glomerulonephritis and Dense Deposit Disease)

DISCOVERY

APL-2 Prevents Inflammation and MAC Formation



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Rationale for APL-2 in Glomerulopathies

Subtypes	Complement pathways activated	Incidence/ Prevalence[1-4]	End-stage Renal Disease (ESRD) Hemodialysis	Competition
IgA nephropathy	• **Alternative** • Lectin (more severe cases)	• Inc: 6,200* • Prev: 130,000	• ESRD: 40% within 20 years • Hemodialysis: 72%	• OMS721 (Omeros) phase 2
Membranous nephropathy	• **Lectin** • **Alternative**	• Inc: 2,980* • Prev: Not determined	• ESRD: 30-40% within 5-15 years • Hemodialysis: 82%	• Eculizumab - did not reach primary clinical endpoints • OMS721 (Omeros) phase 2
Lupus Nephritis	• **Alternative** • **Classical** • Lectin	• Inc: 17,000* • Prev: 80,000	• ESRD: 10-30% within 15 years • Hemodialysis: 85%	• Rituximab phase 3 • Eculizumab, one case report • OMS721 (Omeros) phase 2
C3 glomerulopathy	• **Alternative**	• Inc: Not determined • Prev: 600	• ESRD: ~50% within 10 years	• Eculizumab ~15 promising cases studies, C3 deposition on biopsy • OMS721 (Omeros) phase 2 • ACH-0144471 (Achillon) phase 2

* Based on US adult population, 2016 248 million adults

1. National Organization for Rare Diseases, IgA Nephropathy, 2005, www.rarediseases.org
2. Hou, J. et al. Toward a working definition of C3 glomerulopathy by immunofluorescence. *Kidney Intl.* 2014. 85(2) 450-56
3. Tickering, TD. *et al.* Dense Deposit Disease and C3 Glomerulopathy. *Seminars in Nephrology*. 33(6): 493-507
4.. McGrogan, A. *et al.,* The incidence of primary glomerulonephritis worldwide: a systemic review of the literature. *Nephrology Dialysis Transplantation* 2011. 26(2): 414-30
5. Hoover, PJ and Costenbader, KH. Insights into the Epidemiology and Management of Lupus Nephritis from the US Rheumatologist's Perspective. *Kidney Intl. 2016. 90(3): 487-492*

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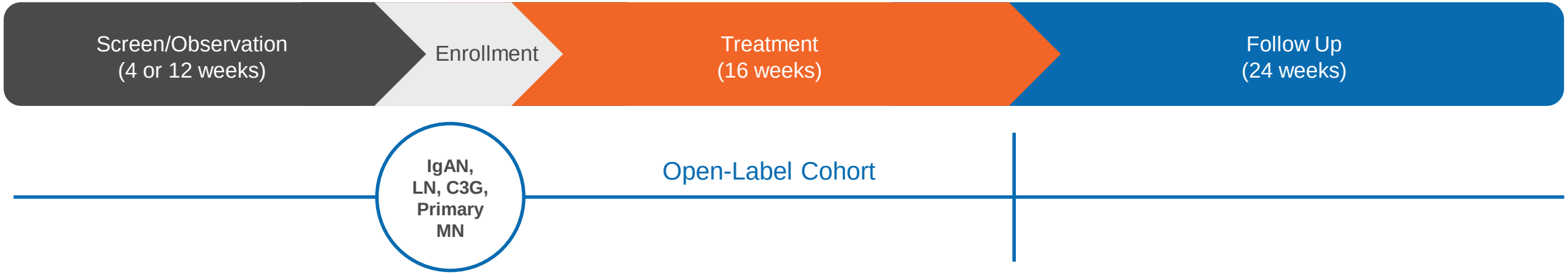
APL2-201: Study Design

Parameter	
Population	Patients with either: IgA Nephropathy (IgAN), Lupus Nephritis (LN), Primary Membranous Nephropathy (Primary MN) or C3 Glomerulopathy (C3G)
1º Endpoint	Proteinuria reduction
Design	Single, open-label, treatment arm with APL-2 daily dosing
Posology	360mg (40mg/mL) subcutaneous infusion
Sample size	Up to 48 patients (6-12 per indication)



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APL2-201: Key Inclusion Criteria (Following Protocol Amendment)

1. 18+ years of age at screening
2. Urinary protein to creatinine ratio >750mg/g (~1.0g/day)
3. Stable blood pressure (<140/90mmHg)
4. 3+ months of optimized ACE/ARB therapy (if applicable)
5. 2+ months of optimized immunosuppressive therapy (if applicable)
6. eGFR ≥ 30 mL/min/1.73 m^2 calculated by CKD-EPI
7. Prior diagnosis by renal biopsy
8. 4 Week Screening Eligibility:
   - 6+ months of uPCR levels >750mg/g, stable blood pressure (<140/90mmHg), and optimized ACE/ARB

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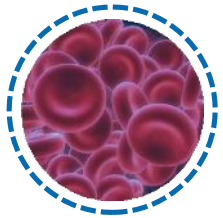
APL2-201: Key Exclusion Criteria

1. Use of belimumab, eculizumab, or rituximab within 6 months prior to Screening
2. Previous treatment with APL-2
3. History of organ transplant
4. Current unstable kidney function for other reasons, e.g. toxin induced acute kidney injury

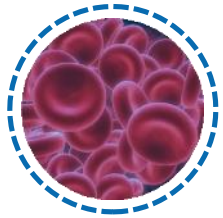
Key Milestones for 2018

GA:
Phase 2: 18 month safety & efficacy data ✓

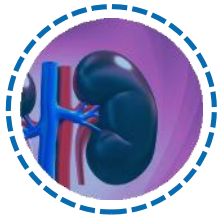


PNH:
Phase 1b: monotherapy expansion (interim) ✓

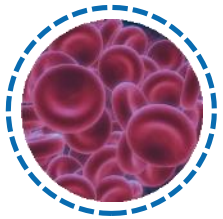
Phase 1b: Soliris weaning in add-on study ✓



AIHA:
Phase 2: POC monotherapy data ✓



CDN:
Phase 2: POC monotherapy data



PNH:
Start of Phase 3 program ✓

GA:
Start of Phase 3 program

H1 H2

2018

Apellis