



# Corporate Presentation

*Leader in AAV Gene Therapy*

## Forward-looking statements

This presentation includes “forward-looking statements,” within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended. These statements express a belief, expectation or intention and are generally accompanied by words that convey projected future events or outcomes such as “believe,” “may,” “will,” “estimate,” “continue,” “anticipate,” “design,” “intend,” “expect,” “could,” “plan,” “potential,” “predict,” “seek,” “should,” “would” or by variations of such words or by similar expressions. The forward-looking statements include statements relating to, among other things, REGENXBIO’s future operations, clinical trials, costs and cash flow. REGENXBIO has based these forward-looking statements on its current expectations and assumptions and analyses made by REGENXBIO in light of its experience and its perception of historical trends, current conditions and expected future developments, as well as other factors REGENXBIO believes are appropriate under the circumstances. However, whether actual results and developments will conform with REGENXBIO’s expectations and predictions is subject to a number of risks and uncertainties, including the timing of enrollment, commencement and completion and the success of clinical trials conducted by REGENXBIO, its licensees and its partners, the timing of commencement and completion and the success of preclinical studies conducted by REGENXBIO and its development partners, the timely development and launch of new products, the ability to obtain and maintain regulatory approval of product candidates, the ability to obtain and maintain intellectual property protection for product candidates and technology, trends and challenges in the business and markets in which REGENXBIO operates, the size and growth of potential markets for product candidates and the ability to serve those markets, the rate and degree of acceptance of product candidates, and other factors, many of which are beyond the control of REGENXBIO. Refer to the “Risk Factors” and “Management’s Discussion and Analysis of Financial Condition and Results of Operations” sections of REGENXBIO’s Annual Report on Form 10-K for the year ended December 31, 2018 and comparable “risk factors” sections of REGENXBIO’s Quarterly Reports on Form 10-Q and other filings, which have been filed with the U.S. Securities and Exchange Commission (SEC) and are available on the SEC’s website at [www.sec.gov](http://www.sec.gov). All of the forward-looking statements made in this presentation are expressly qualified by the cautionary statements contained or referred to herein. The actual results or developments anticipated may not be realized or, even if substantially realized, they may not have the expected consequences to or effects on REGENXBIO or its businesses or operations. Such statements are not guarantees of future performance and actual results or developments may differ materially from those projected in the forward-looking statements. Readers are cautioned not to rely too heavily on the forward-looking statements contained in this presentation. These forward-looking statements speak only as of the date of this presentation. REGENXBIO does not undertake any obligation, and specifically declines any obligation, to update or revise any forward-looking statements, whether as a result of new information, future events or otherwise.

**REGENXBIO:** seeking to improve lives through the curative potential of gene therapy

## 4 clinical stage programs

Expect to initiate *Phase IIb study of RGX-314 for wet AMD in Q1 2020*, and file IND for *Phase II study of RGX-314 for DR in Q1 2020*

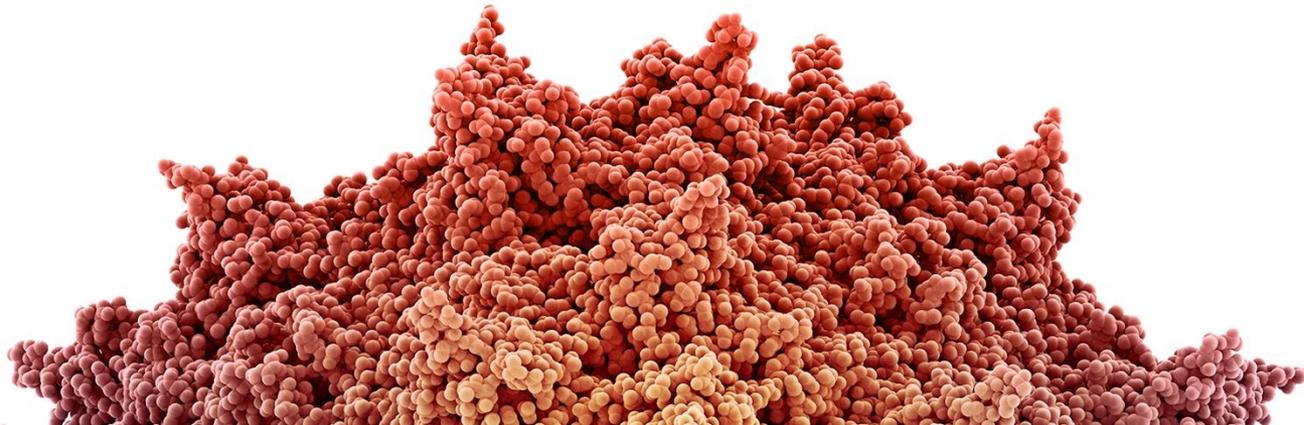
## 1 FDA approved product and

**15 clinical stage product candidates** being developed by third-party licensees; *over 20 partnered programs in total*

### Proprietary **NAV<sup>®</sup> Technology Platform**

includes exclusive *worldwide rights to over 100 AAV vectors*, including **AAV7, AAV8, AAV9** and **AAVrh10**

Management team are **experienced drug developers** and **leaders in gene therapy**



# REGENXBIO's lead programs

Internally developed product candidates

Indication	Development Stage				Commercial Rights
	Research	Preclinical	Phase I / II	Phase III	
Retinal Disease wet AMD	RGX-314				Worldwide
Diabetic retinopathy	RGX-314				
Add'l anti-VEGF treated conditions	RGX-314				
Neurodegenerative Disease MPS II ▲ ★ ■	RGX-121				Worldwide
MPS I ▲ ★ ■	RGX-111				Worldwide
CLN2 disease ▲ ★	RGX-181				Worldwide
Tauopathies					 neurimmune <small>Prominal antibody therapeutics</small> Co-Commercialization
Liver-directed HoFH ▲	RGX-501				Worldwide
Hereditary angioedema					Worldwide

# REGENXBIO's NAV Technology Platform has been widely adopted

Over 20 partnered product candidates being developed by NAV Technology Licensees

Research		Preclinical		Phase I / II		Phase III / Approved		
Indication	Licensee	Indication	Licensee	Indication	Licensee	Indication	Licensee	
Liver / hematologic		Wilson Disease		Hemophilia A				
				Hemophilia A				
				OTC Deficiency				
				GSDIa				
				Crigler-Najjar	AUDENTES 			
Central nervous system	CDKL5 Deficiency		Rett Syndrome		SMA Type II / III		SMA Type I	
	Undisclosed		ALS SOD1		Parkinson's w/ GBA & Neuronopathic Gaucher		MPS IIIA	
			CLN3		MPS IIIA			
			Friedreich's ataxia		MPS IIIA			
			FTD-GRN		MPS IIIB			
			Synucleinopathies (GBA + $\alpha$ -Syn RNAi)		CLN1			
Cardiac / skeletal muscle		Pompe Disease	AUDENTES 	CPVT	AUDENTES 	XLMTM	AUDENTES 	
				Danon Disease				



Zolgensma® is approved in the U.S. for the treatment of pediatric patients less than 2 years of age with SMA with bi-allelic mutations in the SMN1 gene

# Internal Development Programs





## RGX-314 for treatment of wet age-related macular degeneration (wet AMD)

### THE DISEASE

- Blurring of central vision and progressive vision loss due to formation of leaky blood vessels in the eye
- VEGF inhibitors are standard of care to treat fluid and associated vision loss
- Frequency / uncomfortable administration of current anti-VEGF therapies affects compliance and ultimately efficacy
- >2 million patients estimated in U.S., Europe and Japan

### RGX-314 PRODUCT CANDIDATE



Vector: AAV8



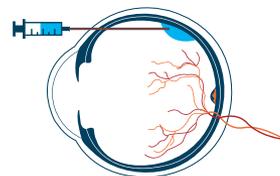
Gene: anti-VEGF Fab

### Mechanism of action

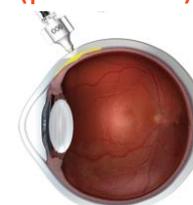
Reducing leaky blood vessel formation by giving retinal cells the ability to produce an anti-VEGF fab

### Routes of administration

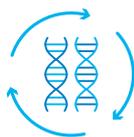
Subretinal  
(clinical)



Suprachoroidal  
(preclinical)



# RGX-314 Phase I/IIa clinical trial in wet AMD



## OBJECTIVES

### Primary

- To determine the safety and tolerability of RGX-314 in subjects with wet AMD through six months

### Secondary

- Expression of RGX-314 protein in the eye
- Effect of RGX-314 on best corrected visual acuity (BCVA) and central retinal thickness (CRT) as measured by Spectral Domain Optical Coherence Tomography (SD-OCT)
- Additional anti-VEGF injections post-RGX-314

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**Subjects:** 42 total

**Route of administration:** subretinal

**Sites:** Eight leading retinal surgery centers across the United States

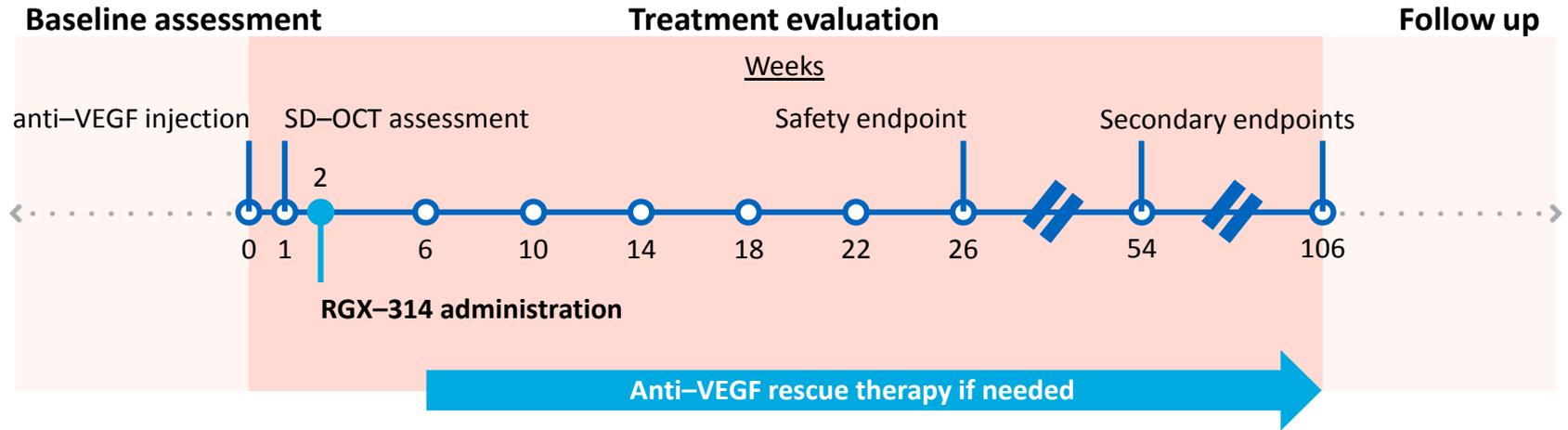


## KEY INCLUSION CRITERIA

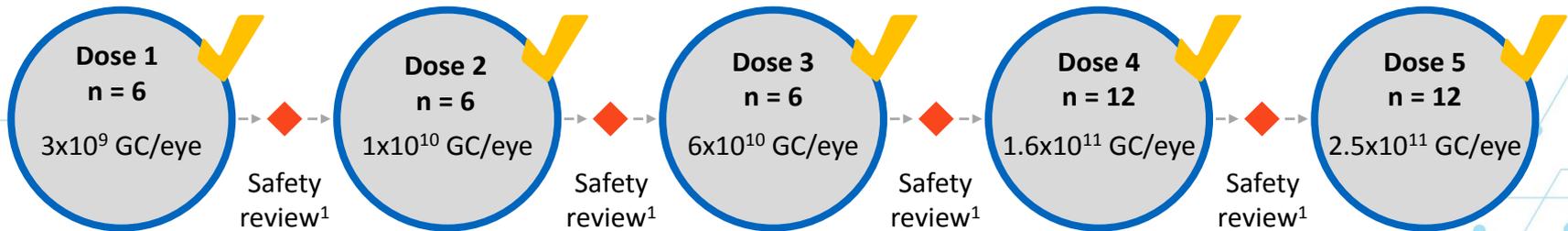
- Male or female  $\geq 50$  to 89 years of age
- Previously treated wet AMD subjects requiring  $\geq 4$  anti-VEGF injections in the 8 months prior to trial entry
- Documented response to anti-VEGF at trial entry (assessed by SD-OCT at week 1)
- Vision of 20/63 to 20/400 for the initial subject, then 20/40 to 20/400 for the rest of each cohort
- Pseudophakic (status post cataract surgery)

# RGX-314 Phase I/IIa clinical trial: Administration and dose escalation

## Administration and follow-up timeline



## Dose escalation pathway



42 total subjects dosed across five cohorts

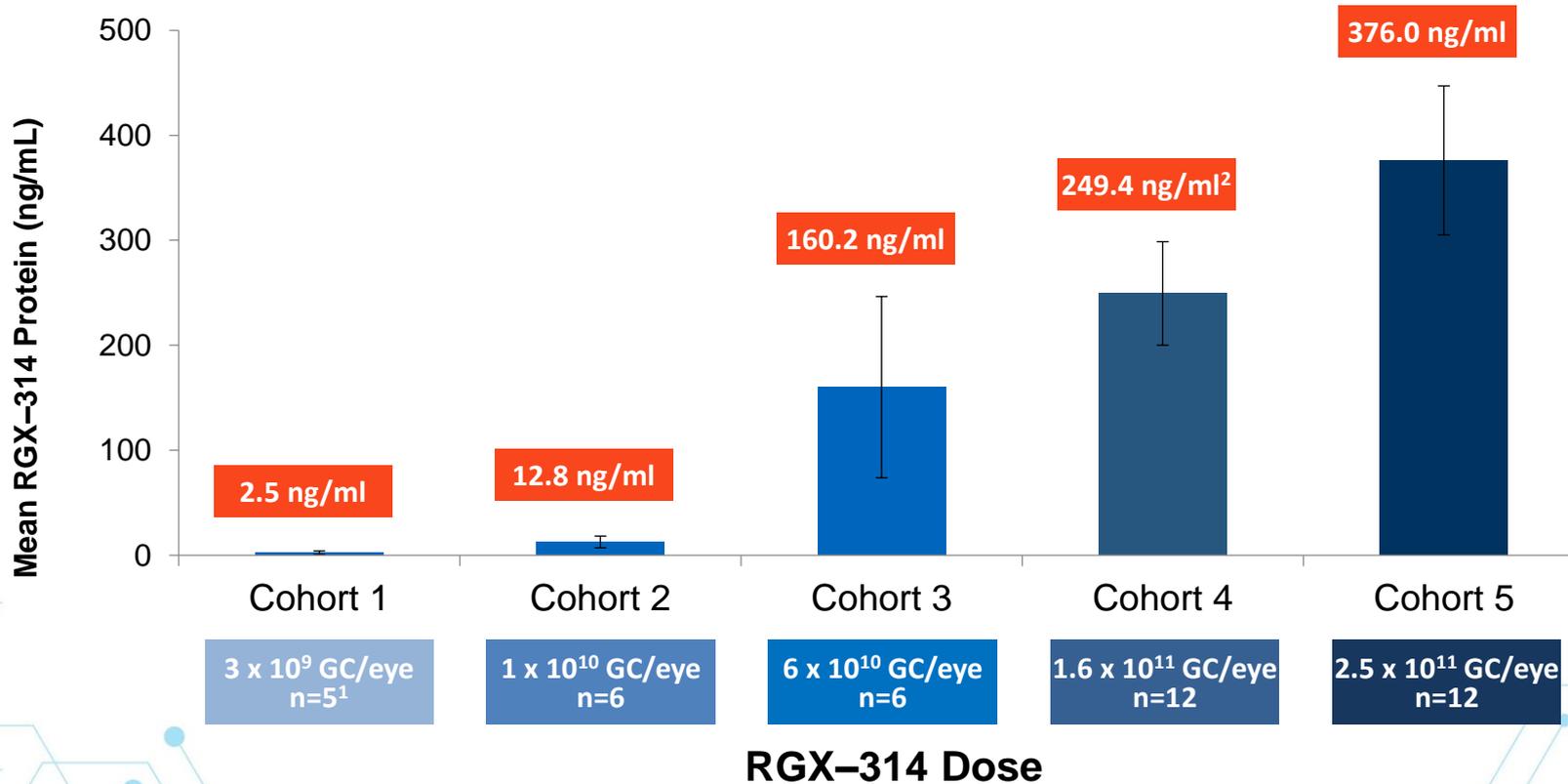
<sup>1</sup> Dose escalation safety review to occur four weeks after final subject in each cohort has been dosed

# RGX-314 Phase I/IIa clinical trial: Safety and data summary<sup>1</sup>

- RGX-314 was **well-tolerated** (n=42)
- **No drug-related SAEs**
- Most AEs were assessed as mild (Grade 1 – 79%)
- **No observed clinically determined immune responses**, drug-related ocular inflammation, or any post-surgical inflammation beyond what is expected following routine vitrectomy
- **Fifteen SAEs** that were not drug-related were **reported in nine subjects**
  - **Two deaths unrelated to RGX-314**
  - **Two ocular procedure-related SAEs:** peripheral retinal detachment which was repaired and an endophthalmitis post aqueous sample collection

# RGX-314 Phase I/IIa clinical trial: RGX-314 protein levels at one month

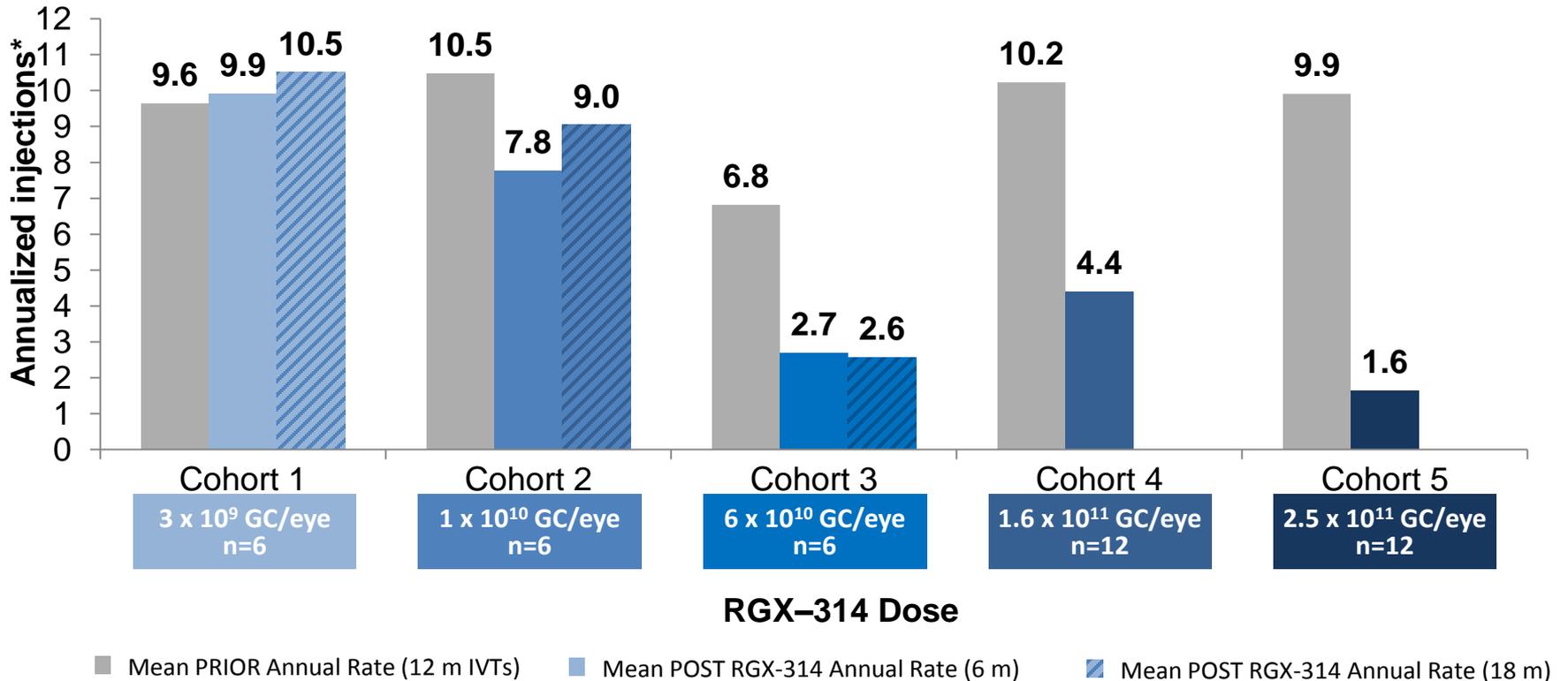
As measured from aqueous samples by ECL



1. N=5; one subject in Cohort 1 did not have aqueous sample taken at Week 6
2. One subjects protein concentration measured at Day 17 post RGX-314 administration (no 4 week sample available)

# RGX-314 Phase I/IIa clinical trial: Mean change in annualized injection rate pre- and post-RGX-314

## Comparison of injection rate PRIOR and POST RGX-314



**Cohort 5 demonstrates over 80% reduction in anti-VEGF injections**



\*Prior annual rate is (Total # of prior IVTs)/(minimum(366 days, Duration between first ever IVT and Day 1)/365.25). Post RGX-314 annual rate is (Total # of IVTs on Study)/(Duration on Study/365.25) where on Study is from RGX-314 administration through 18 months for C1-C3 and up to 6 months for C4-C5.

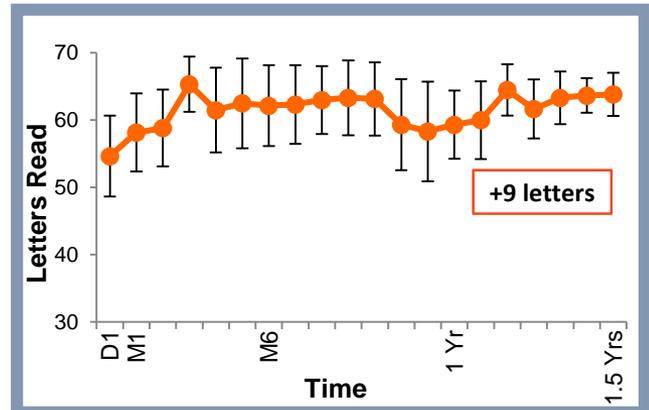
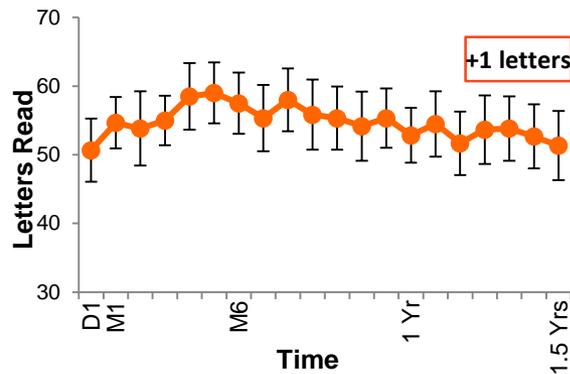
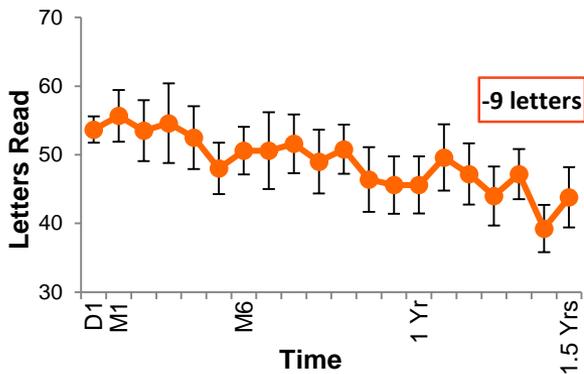
# RGX-314 Phase I/IIa clinical trial: Mean change in BCVA, CRT and annualized injections over 1.5 years in cohorts 1-3

## Cohort 1<sup>1</sup>

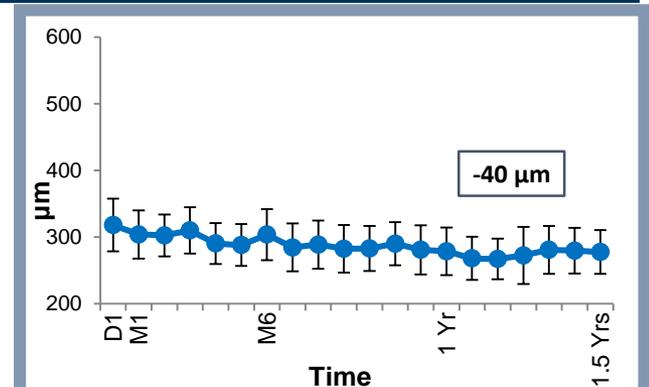
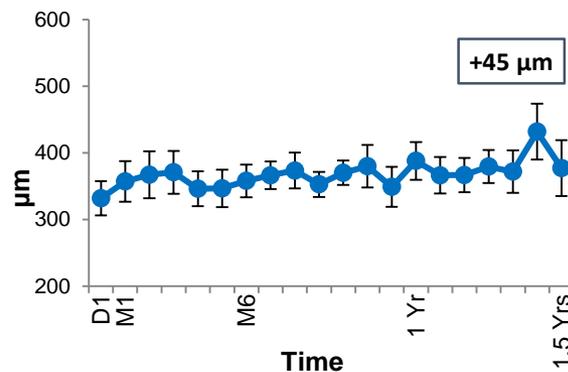
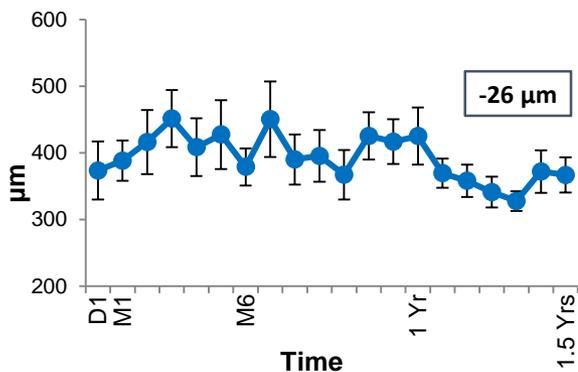
## Cohort 2

## Cohort 3

### Best Corrected Visual Acuity (BCVA)



### Central Retinal Thickness (CRT) on Heidelberg SD-OCT



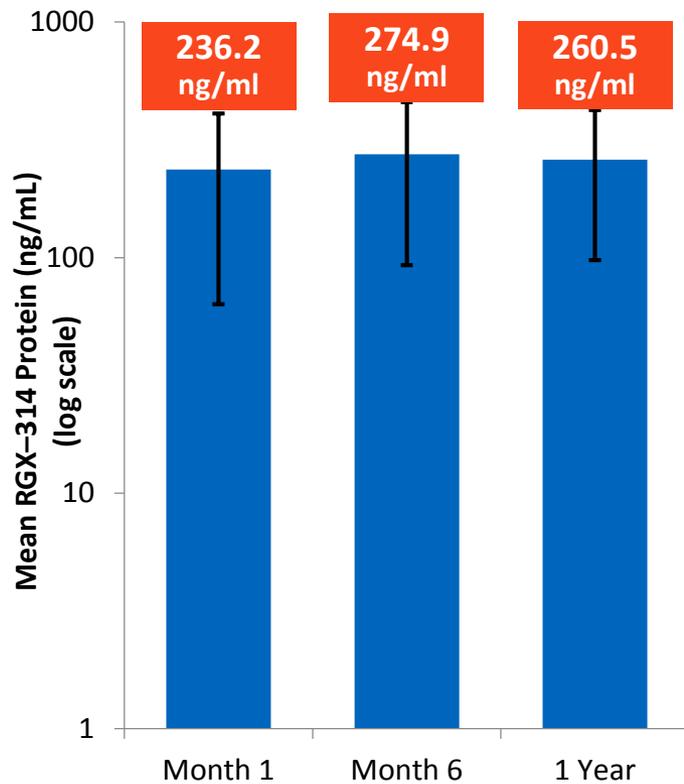
10.5 Injections (annualized)

9.0 Injections (annualized)

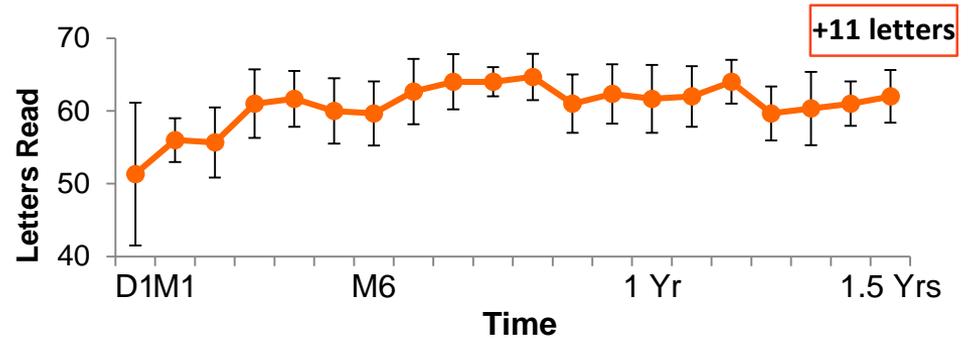
2.6 Injections (annualized)

# RGX-314 Phase I/IIa clinical trial: Cohort 3 anti-VEGF injection-free subjects (n=3 of 6) Continue to Do Well Over 1.5 Years

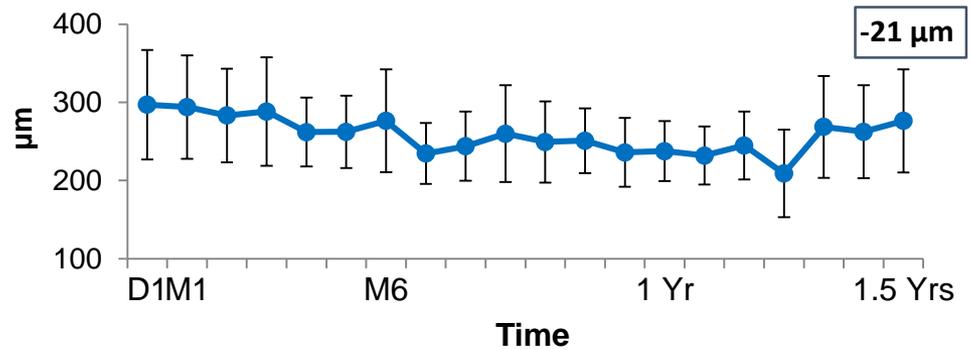
## Sustained RGX-314 Protein Levels Over 1 Year



## Best Corrected Visual Acuity (BCVA)



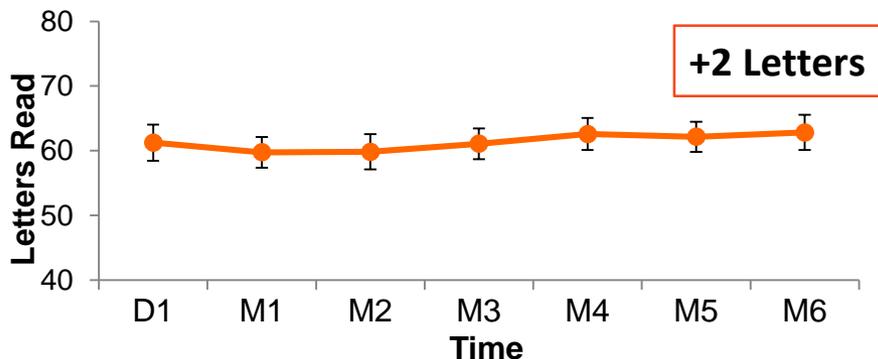
## Central Retinal Thickness (CRT) on Heidelberg SD-OCT



# RGX-314 Phase I/IIa clinical trial: Mean change in BCVA, CRT and average injections up to 6 months in cohorts 4 and 5

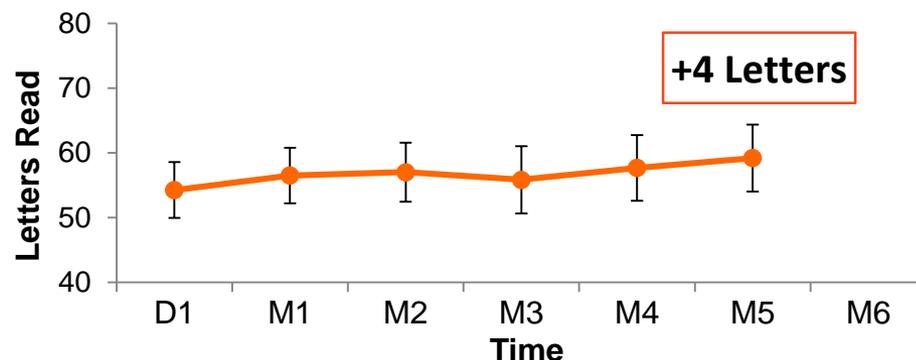
## Cohort 4 (n=12)

### Best Corrected Visual Acuity (BCVA)

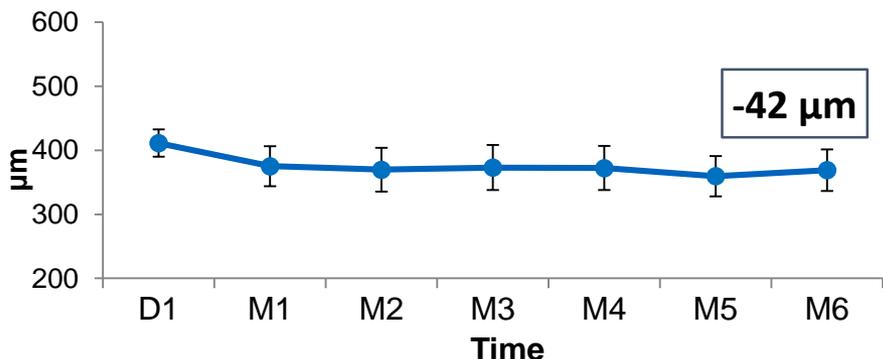


## Cohort 5 (n=12)<sup>1</sup>

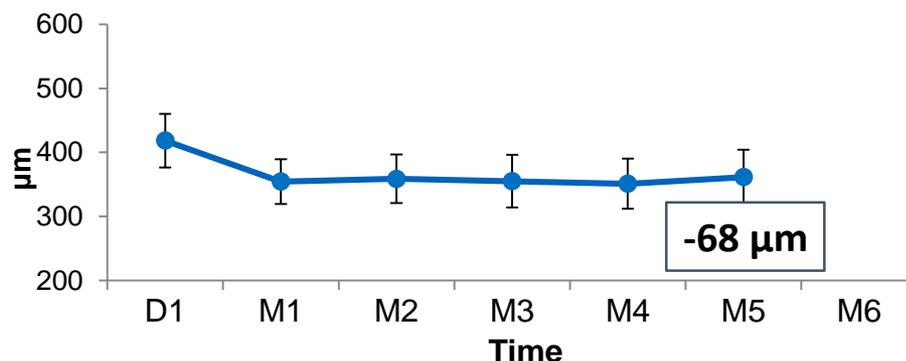
### Best Corrected Visual Acuity (BCVA)



### Central Retinal Thickness (CRT) on Heidelberg SD-OCT<sup>2</sup>



### Central Retinal Thickness (CRT) on Heidelberg SD-OCT<sup>2</sup>



Mean: 2.2 inj / 6 mo

42% (5 of 12) injection-free at 6 months

Mean: 0.8 inj / 5 - 6 mo

75% (9 of 12) injection-free at 5-6 months

<sup>1</sup>1 subject in Cohort 5 discontinued after 4 months

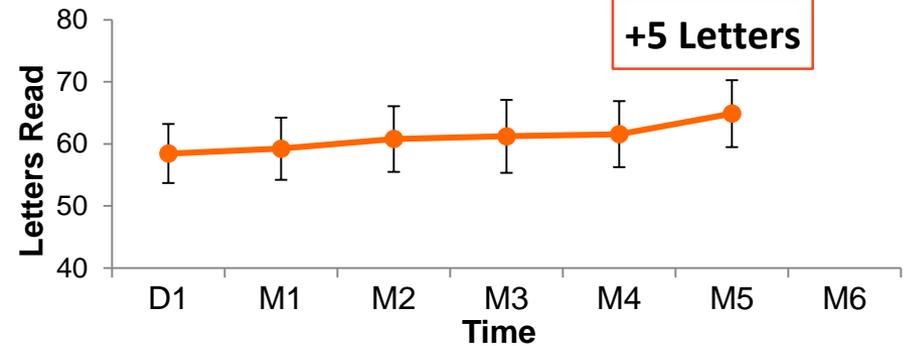
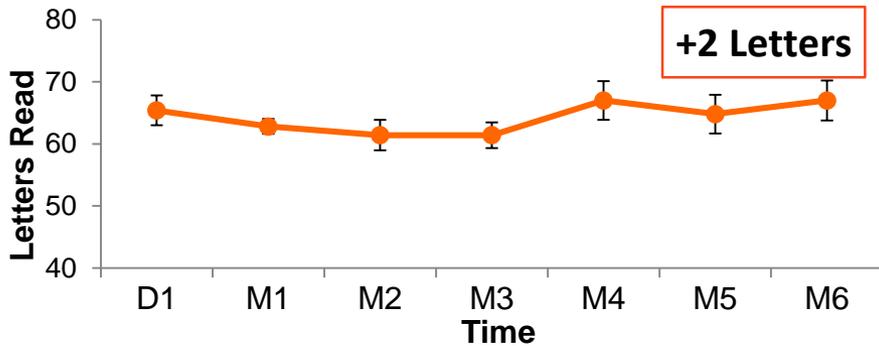
<sup>2</sup>SD-OCT data read by a central reading center (Duke Reading Center).

# RGX-314 Phase I/IIa clinical trial: BCVA and CRT in anti-VEGF injection-free subjects from Cohorts 4 and 5

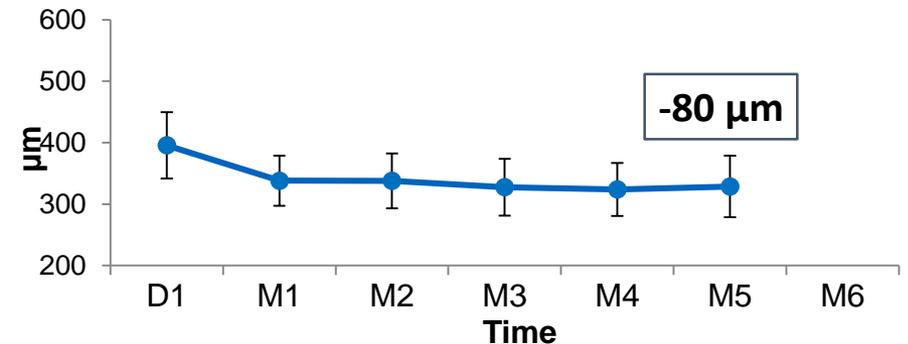
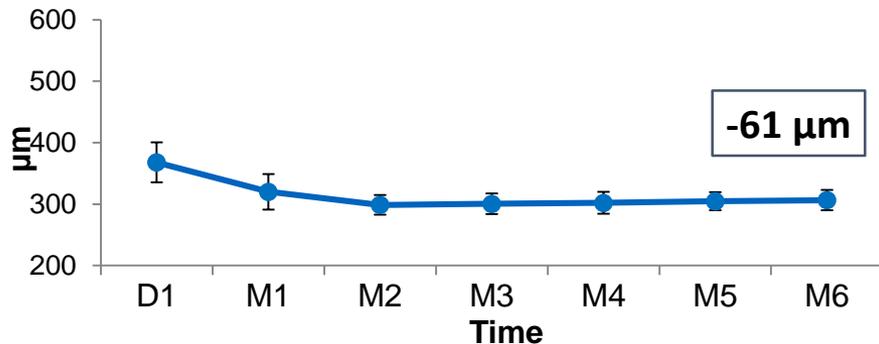
## Cohort 4 (n=5)

## Cohort 5 (n=9)<sup>1</sup>

### Best Corrected Visual Acuity (BCVA)



### Central Retinal Thickness (CRT) on Heidelberg SD-OCT<sup>2</sup>



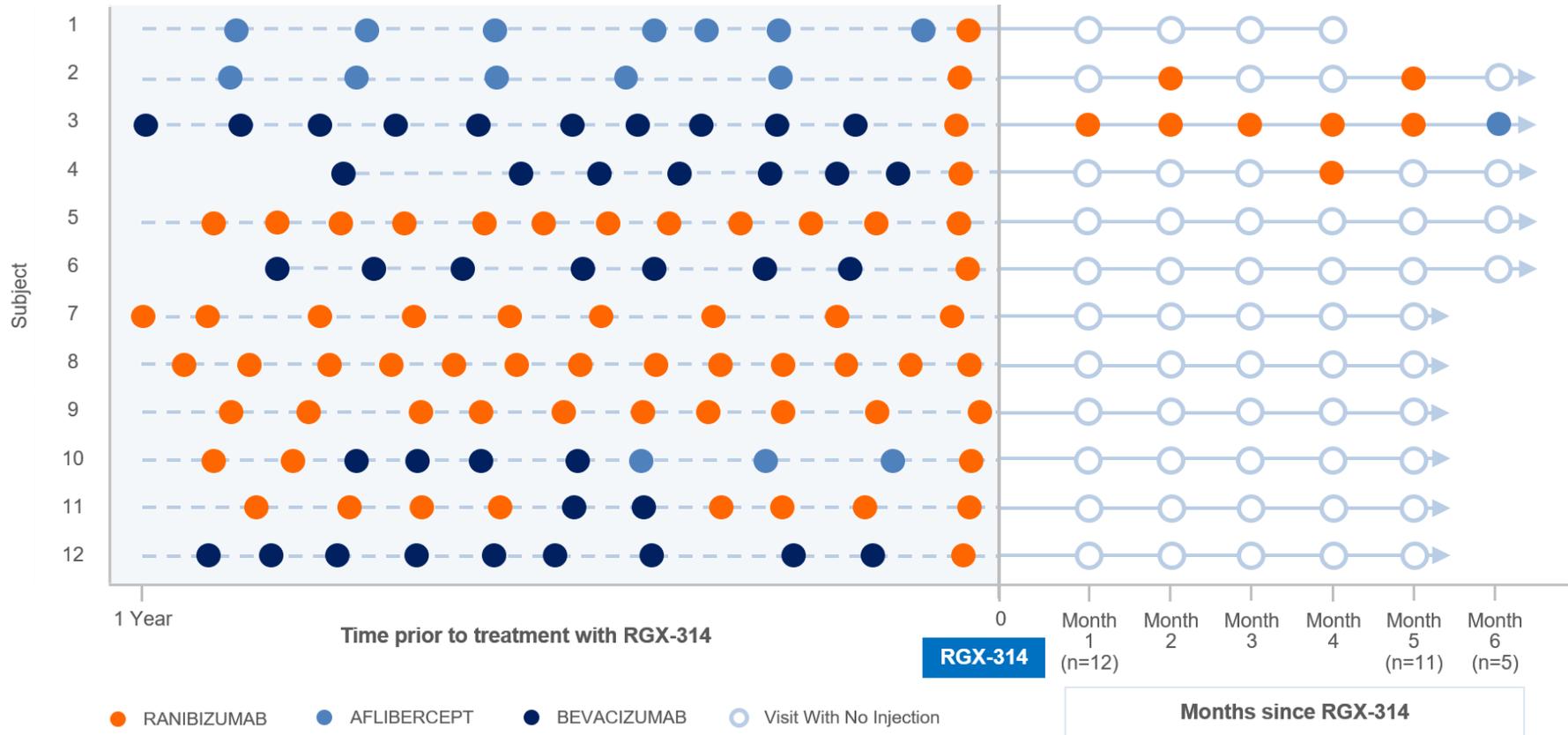
0 Injections

0 Injections

<sup>1</sup>1 subject in Cohort 5 discontinued after 4 months

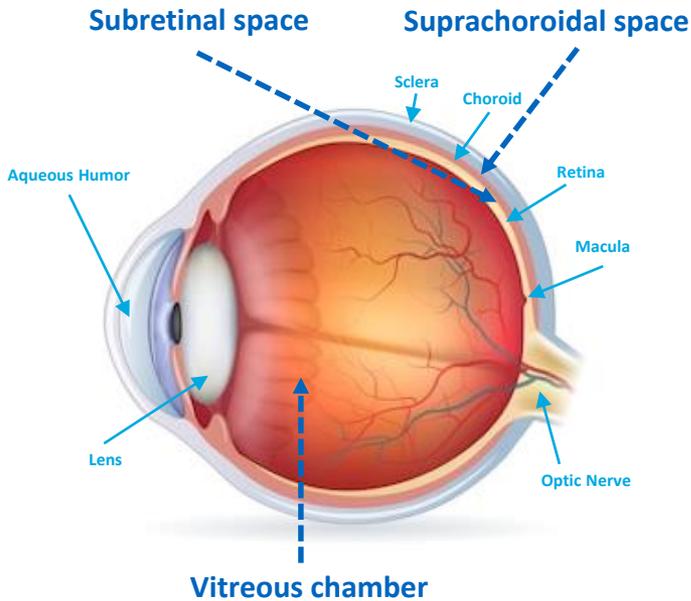
<sup>2</sup>SD-OCT data read by a central reading center (Duke Reading Center).

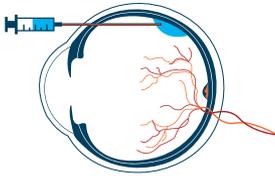
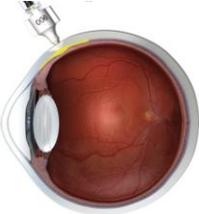
# RGX-314 Phase I/IIa clinical trial: Cohort 5 Injections Pre- and Post-RGX-314



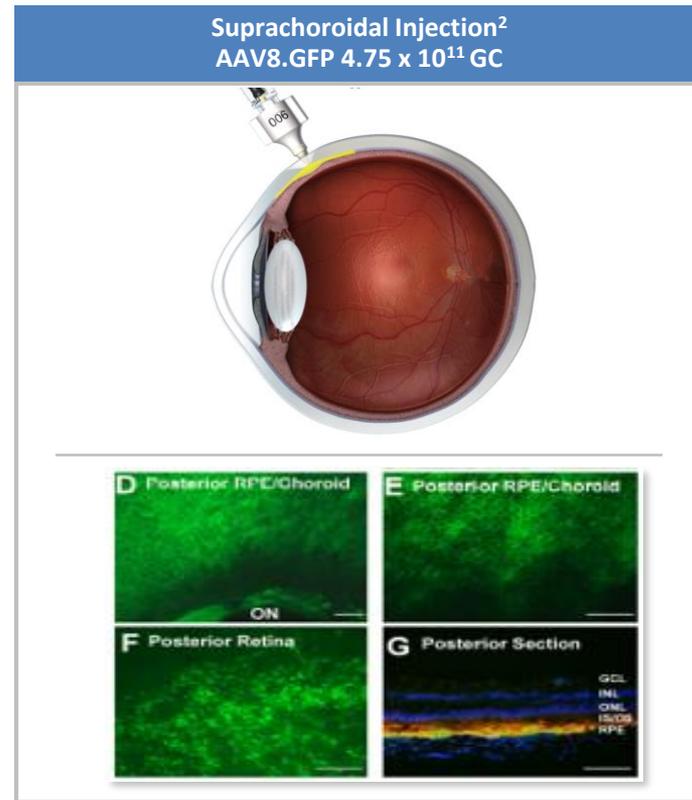
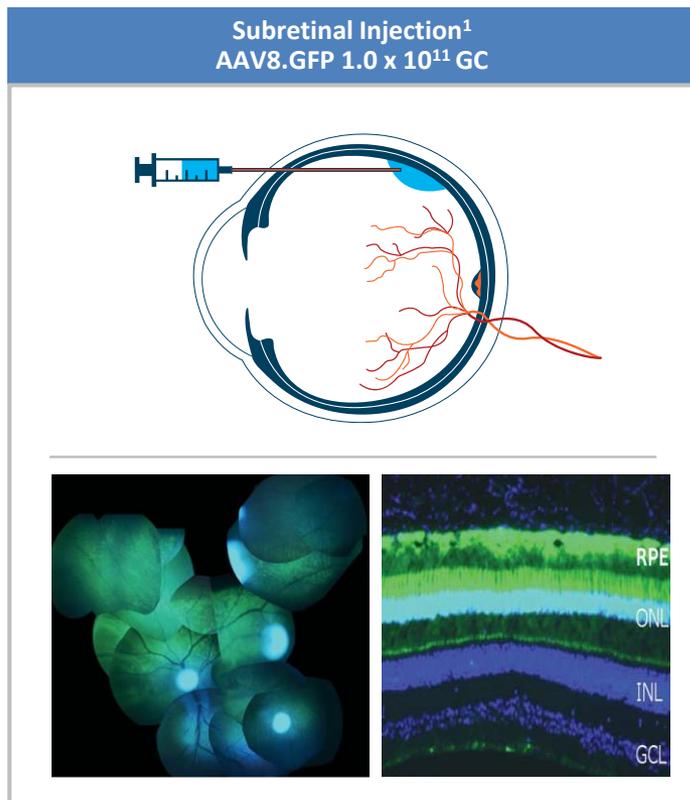
# Ocular gene therapy delivery methods to reach the back of the eye

## Comparative profiles



Delivery Space	Potential Patient Eligibility
 <p><b>Subretinal Space<sup>1</sup></b></p> <ul style="list-style-type: none"> <li>Direct and broad transduction of the retina observed in preclinical studies</li> <li>Minimal exposure to the vitreous and anterior segment                             <ul style="list-style-type: none"> <li>Low risk of immune response</li> <li>Low risk of inflammation</li> </ul> </li> </ul>	<p><b>AAV Neutralizing Antibody (NAb) Status</b></p> <ul style="list-style-type: none"> <li>All patients eligible, regardless of NAb status</li> </ul>
 <p><b>Suprachoroidal Space (SCS)<sup>2</sup></b></p> <ul style="list-style-type: none"> <li>Direct and broad transduction of the retina observed in preclinical studies</li> <li>Minimal exposure to the vitreous and anterior segment                             <ul style="list-style-type: none"> <li>Low risk of immune response</li> <li>Low risk of inflammation</li> </ul> </li> </ul>	<p><b>AAV NAb Status</b></p> <ul style="list-style-type: none"> <li>Patients without NAb to AAV<sup>8</sup> <ul style="list-style-type: none"> <li>~70% for AAV8</li> <li>~30% for AAV2</li> </ul> </li> </ul>
 <p><b>Vitreous Chamber</b></p> <ul style="list-style-type: none"> <li>Inner limiting membrane (ILM) presents physical barrier, potentially limiting direct transduction of the retina<sup>3</sup> <ul style="list-style-type: none"> <li>Limited transduction of the retina observed in preclinical studies<sup>4</sup></li> </ul> </li> <li>Broad exposure to the vitreous and anterior segment                             <ul style="list-style-type: none"> <li>High risk of immune response<sup>5,6</sup></li> <li>High risk of inflammation<sup>4</sup></li> <li>Potential oral systemic corticosteroid prophylaxis needed<sup>7</sup></li> </ul> </li> </ul>	<p><b>AAV NAb Status</b></p> <ul style="list-style-type: none"> <li>Patients without NAb to AAV<sup>8</sup> <ul style="list-style-type: none"> <li>~70% for AAV8</li> <li>~30% for AAV2</li> </ul> </li> </ul>

# Widespread retinal transduction achieved via subretinal and suprachoroidal delivery of AAV8 in non-human primates



<sup>1</sup> Vandenberghe LH, et al. 2011 *Science Translational Medicine*

<sup>2</sup> Ding, K., et al. 2019 *Journal of Clinical Investigation*



## RGX-314 for treatment of Diabetic Retinopathy (DR)

### THE DISEASE

- Leading cause of vision loss in adults between 24 – 75 years of age
- Spectrum encompasses nonproliferative DR (NPDR) and proliferative DR (PDR) with or without diabetic macular edema (DME)
- Treatment options include anti-VEGF injections or panretinal laser treatment
- Approximately 8 million patients estimated in United States

### RGX-314 PRODUCT CANDIDATE



**Vector:** AAV8



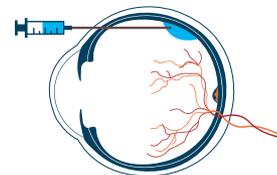
**Gene:** anti-VEGF Fab

### Mechanism of action

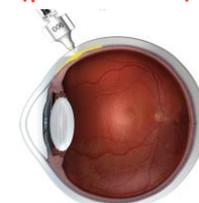
Regressing diabetic blood vessels by giving retinal cells the ability to produce an anti-VEGF fab

### Routes of administration

Subretinal  
(preclinical)



Suprachoroidal  
(preclinical)





## REGENXBIO's neurodegenerative disease franchise

	RGX-121 for MPS II	RGX-111 for MPS I	RGX-181 for CLN2 disease
Disease	<ul style="list-style-type: none"> <li>Reduced ability to process glycosaminoglycans (GAGs), leading to neurodegeneration and early death</li> <li>X-linked recessive disease</li> <li>Available treatment is inadequate to treat neurodegeneration</li> <li>Approximately 500 – 1,000 patients born annually worldwide</li> </ul>	<ul style="list-style-type: none"> <li>Reduced ability to process GAGs, leading to neurodegeneration and early death</li> <li>Autosomal recessive disease</li> <li>Available treatment is inadequate to treat neurodegeneration; bone marrow transplant partially effective</li> <li>Approximately 500 – 1,000 patients born annually worldwide</li> </ul>	<ul style="list-style-type: none"> <li>Reduced ability to process cellular waste peptides, leading to seizures, vision loss, neurodegeneration and early death</li> <li>Autosomal recessive disease</li> <li>Available treatment requires frequent ICV infusions of ERT, shown to stabilize some but not all disease manifestations</li> <li>Approximately 500 patients born annually worldwide</li> </ul>
Vector	AAV9	AAV9	AAV9
Gene	IDS gene replacement	IDUA gene replacement	TPP1 gene replacement
Admin	Intracisternal 	Intracisternal 	Intracisternal 
Designations	<ul style="list-style-type: none"> <li>▲ Orphan Drug Designation</li> <li>★ Rare Pediatric Disease Designation</li> <li>■ Fast Track Designation</li> </ul>	<ul style="list-style-type: none"> <li>▲ Orphan Drug Designation</li> <li>★ Rare Pediatric Disease Designation</li> <li>■ Fast Track Designation</li> </ul>	<ul style="list-style-type: none"> <li>▲ Orphan Drug Designation</li> <li>★ Rare Pediatric Disease Designation</li> </ul>

# Cross-correction is a **key treatment advantage** in MPS and CLN2 disease

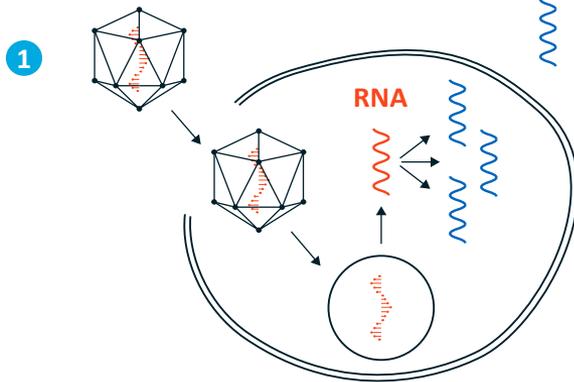
**A single transduced cell has potential to correct many other cells**

**1** NAV Vector delivers healthy gene to cells

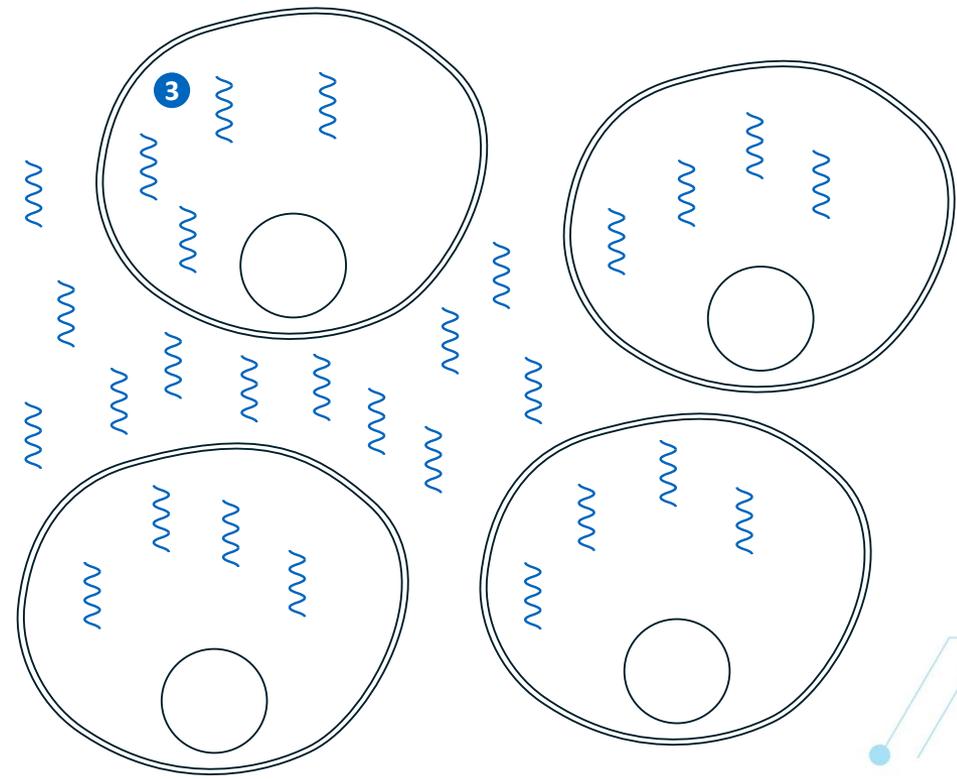
**2** Protein secreted by transduced cells

**3** Protein taken up by non-transduced cells

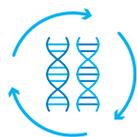
NAV Vector + Gene



**2** Protein



# RGX-121 Phase I/II clinical trial in MPS II



## Objectives

### Primary

- To determine the safety and tolerability of RGX-121 in severe MPS II subjects who have or are at high risk of developing neurocognitive deficits

### Secondary

- Effect of RGX-121 on biomarkers of I2S activity in CSF, serum and urine
- Effect of RGX-121 on neurocognitive deficits

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**Subjects:** Up to 6 total

**Sites:** Leading U.S. and international lysosomal storage disease centers

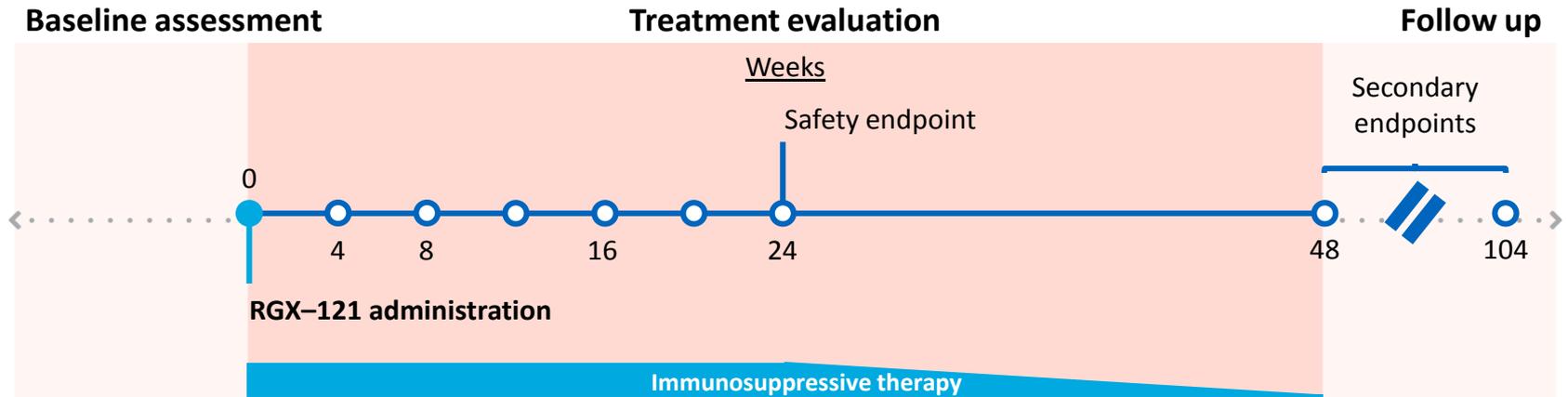


### Key inclusion criteria

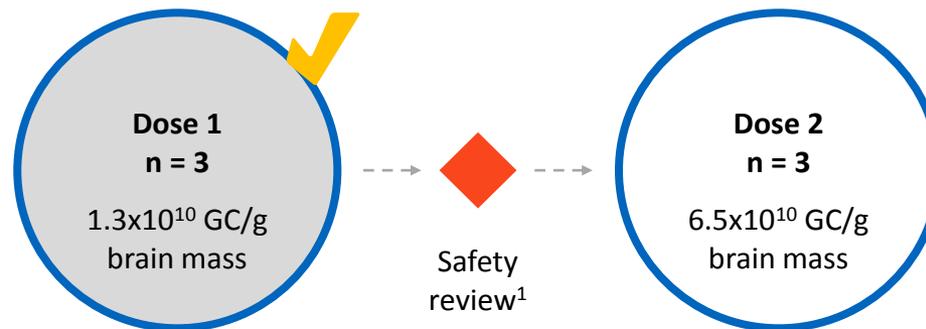
- Male subjects  $\geq 4$  months to  $< 5$  years of age
- Meeting one of the following criteria:
  - Diagnosis of MPS II and a score  $> 55$  and  $\leq 77$  on intelligent quotient testing OR
  - Diagnosis of MPS II and a score  $> 55$  and a decline of  $\geq 1$  standard deviation on intelligent quotient testing OR
  - Having a relative diagnosed with severe MPS II who has the same IDS mutation as the subject
- No contraindications for intracisternal injection and immunosuppressive therapy

# RGX-121 Phase I/II clinical trial: Administration and dose escalation

## Administration and follow-up timeline

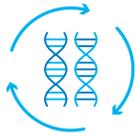


## Expected dose escalation pathway



**Dosing complete in the first cohort**

# RGX-111 U.S. Phase I clinical trial in MPS I



## Objectives

### Primary

- To determine the safety and tolerability of RGX-111 in MPS I subjects with neurocognitive deficits

### Secondary

- Effect of RGX-111 on biomarkers of IDUA activity in CSF, serum and urine
- Effect of RGX-111 on neurocognitive deficits

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**Subjects:** Up to 5 total

**Sites:** Leading U.S. and international lysosomal storage disease centers

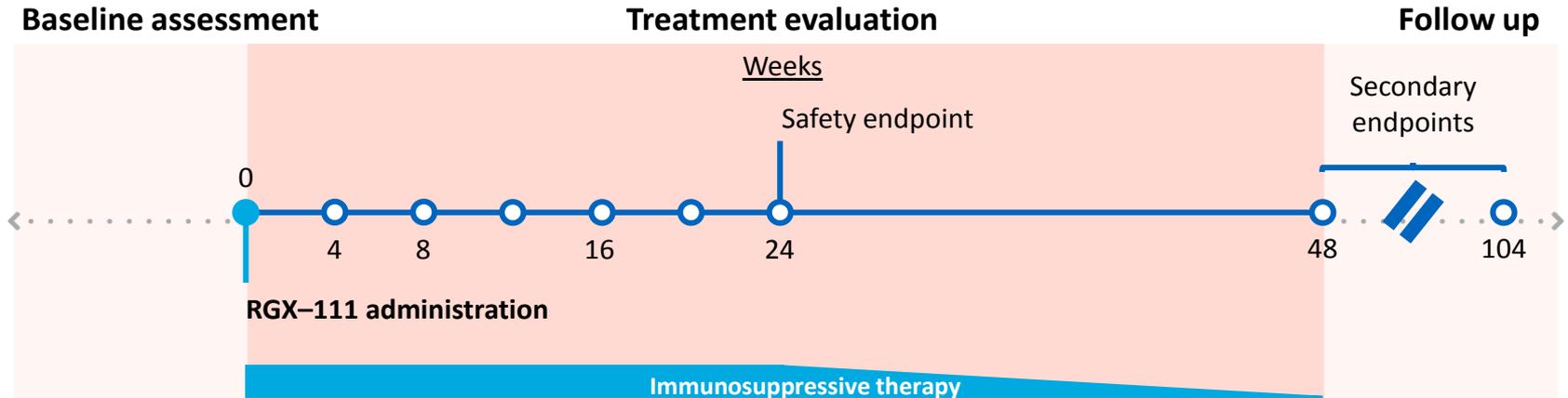


### Key inclusion criteria

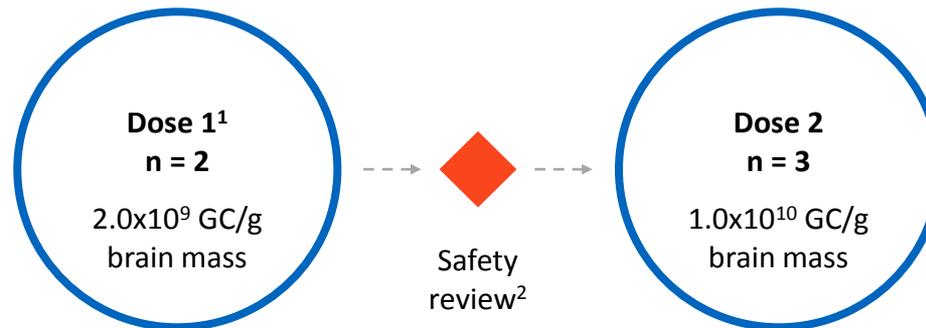
- Male or female
  - First subject  $\geq 18$  years of age
  - Subsequent subjects  $\geq 6$  years of age
- Documented evidence of early-stage neurocognitive deficit due to MPS I
  - A score of  $\geq 1$  standard deviation below mean on intelligent quotient testing or in one domain of neuropsychological function
  - A decline of  $\geq 1$  standard deviation on sequential testing
- No contraindications for intracisternal injection or immunosuppressive therapy

# RGX-111 U.S. Phase I clinical trial: Administration and dose escalation

## Administration and follow-up timeline



## Expected dose escalation pathway



<sup>1</sup> First subject to be  $\geq 18$  years of age

<sup>2</sup> Dose escalation safety review to occur eight weeks after final subject in cohort has been dosed



## RGX-501 for treatment of homozygous familial hypercholesterolemia (HoFH)

### THE DISEASE

- Defective LDLR gene leads to limited / no LDL cholesterol receptors, allowing LDL to build up in bloodstream
- Total cholesterol levels >500 mg/dL
- Coronary artery disease at young age
- Even with existing standard of care therapies, the mean age of death is 32 years
- Approximately 11,000 patients worldwide

### RGX-501 PRODUCT CANDIDATE



Vector: AAV8



Gene: LDLR

### Mechanism of action

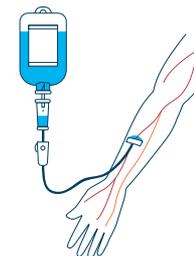
Correction of defective LDLR, reducing circulating LDL cholesterol

### Special Regulatory Status

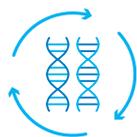
Orphan Drug Designation

Route of administration

Intravenous



# RGX-501 Phase I/II clinical trial in HoFH



## Objectives

### Primary

- To determine the safety and tolerability of RGX-501 in subjects with HoFH

### Secondary

- Percent change in LDL-C levels at 12 weeks
- Other lipid outcome measures

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**Subjects:** Up to 12 total

**Sites:** University of Pennsylvania as single center for RGX-501 administration. Penn or selected US and international sites for treatment evaluation and follow-up

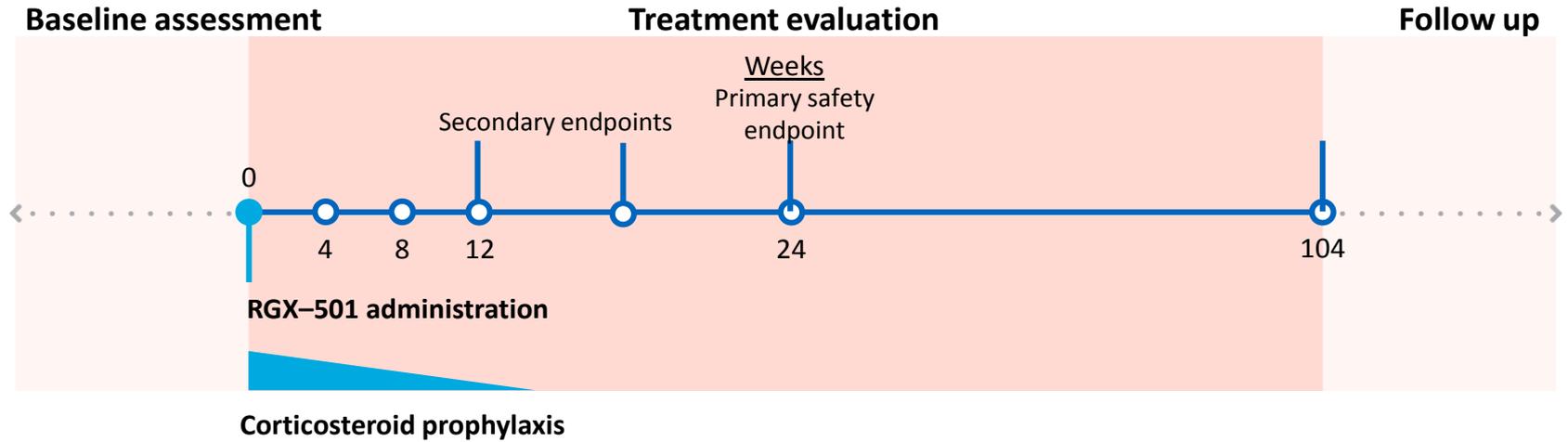


### Key inclusion criteria

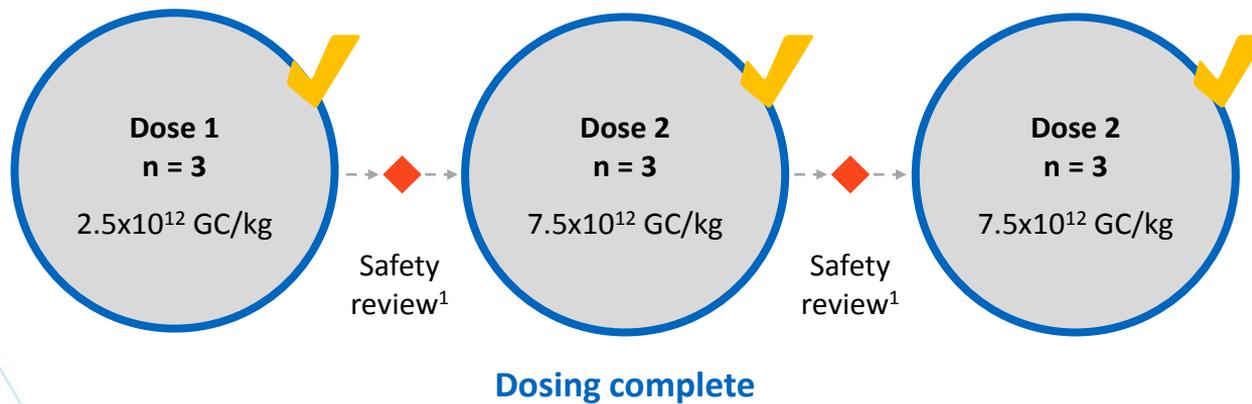
- Male or female  $\geq 18$  years of age
- Untreated and/or treated LDL-C levels and clinical presentation consistent with the diagnosis of HoFH
- Molecularly defined LDLR mutations at both LDLR alleles
- Stable concurrent allowed lipid lowering medications
  - Statins, ezetimibe, bile acid sequestrants, PCSK9i

# RGX-501 Phase I/II clinical trial: Study design

## Administration and follow-up timeline



## Expected dose escalation pathway



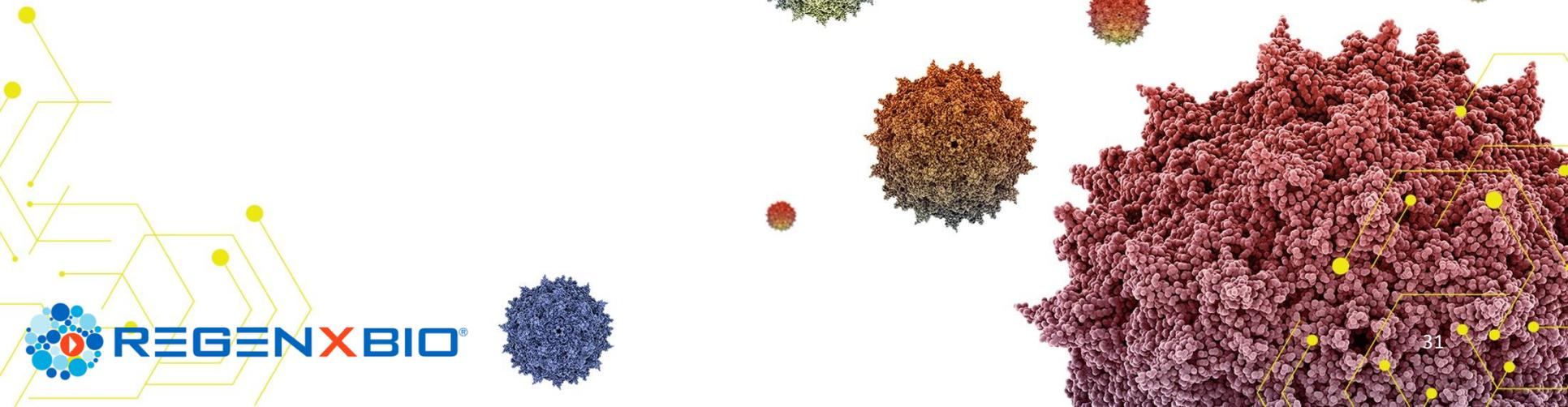


# NAV<sup>®</sup> Technology Platform

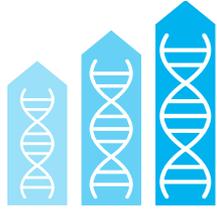
## The NAV Technology Platform is based on a *broad and deep IP portfolio*

Exclusive rights to more than **100 patents** and **patent applications worldwide**

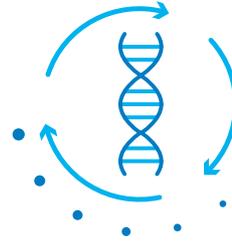
- AAV7, AAV8, AAV9, AAVrh10
- Over 100 other novel AAV sequences
- Sequences that are at least 95% identical to these capsids



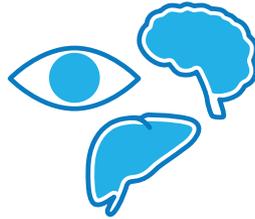
# Key features of REGENXBIO's NAV Technology Platform



**Higher gene expression**



**Longer-term gene expression**



**Broad and novel tissue selectivity**



**Lower immune response**



**Improved manufacturability**

 The NEW ENGLAND  
JOURNAL of MEDICINE

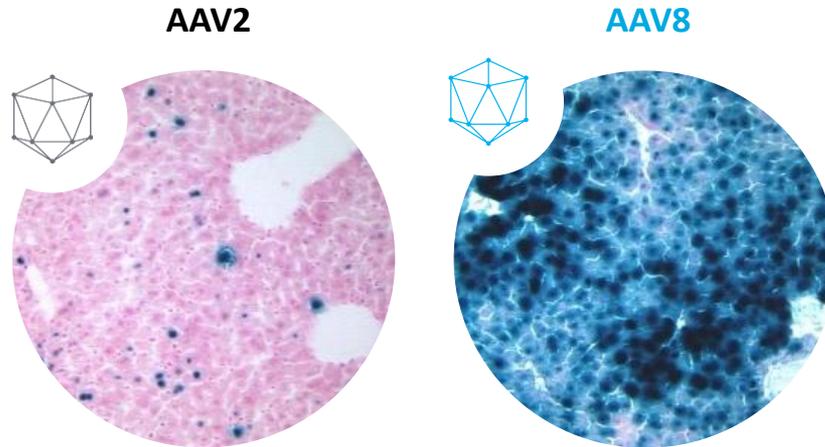
***Long-Term Safety and Efficacy of Factor IX Gene Therapy in Hemophilia B***

 The NEW ENGLAND  
JOURNAL of MEDICINE

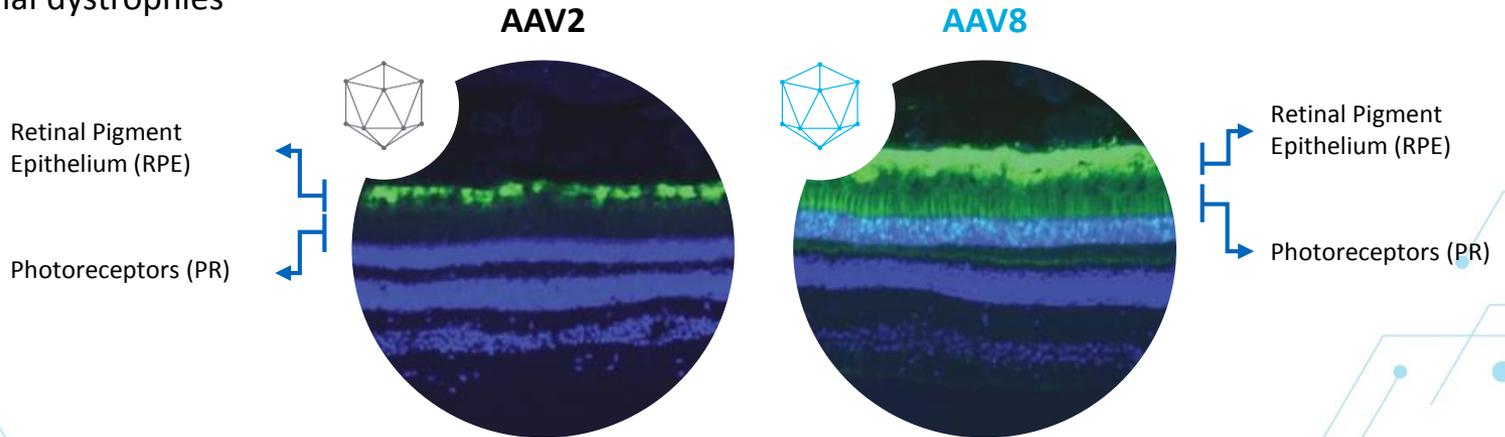
***Single-Dose Gene-Replacement Therapy for Spinal Muscular Atrophy***

# NAV Vectors: higher gene expression than early generation AAV vectors

NAV Vector AAV8: **10x–100x greater gene expression**



NAV Vector AAV8: **More efficient gene delivery** to sites of most retinal dystrophies<sup>1</sup>



# REGENXBIO | cGMP Manufacturing

*Strength in AAV production and deep experience in biologics scale up and commercialization*



## Mammalian cell-based production

- Natural host for AAV
- Robust process utilizing mammalian cell lines with known regulatory history
- Core in-house capability in adapting adherent cell lines to suspension cell culture-based systems
  - Suspension cell culture process developed and transferred to CMO



## Focus on process, quality and analytics

- Deep in-house knowledge of AAV characterization and production
- Focused efforts on integrated upstream and downstream process optimization and scale-up
- Significant expertise and investment in quality systems and downstream analytics



## Large-scale cGMP capacity at CMOs

- Agreements with multiple leading biologics CMOs for production of materials under cGMP, including secured large-scale (up to 2,000L) capacity and commercial production at FUJIFILM
- REGENXBIO platform processes transferred to all CMO partners with robust performance and yields
- FUJIFILM relationship supports clinical development and potential future commercial needs
- Leveraging flexibility and scale at CMOs to ensure supply while managing capital investment



## Clinical manufacturing status

- Completed production of investigational product for four lead product candidates in an amount which is expected to supply on-going clinical trials; GMP campaign in progress for RGX-181
- In-house GMP testing established to accelerate release of clinical supplies
- Capability to progress from candidate selection to clinical material in 12 months



# Team and Conclusion

## The REGENXBIO team

Name	Position	Prior Affiliations	
Ken Mills	President, CEO & Co-Founder; Director		
Olivier Danos, Ph.D.	SVP and Chief Scientific Officer		
Vit Vasista	SVP and Chief Financial Officer		
Steve Pakola, M.D.	SVP and Chief Medical Officer		
Curran Simpson	SVP, Product Development and Chief Technology Officer		
Ram Palanki, Pharm.D.	SVP, Commercial Strategy and Operations		
Patrick Christmas, J.D.	SVP and General Counsel		
Laura Coruzzi, Ph.D., J.D.	SVP, Intellectual Property		
Shiva Fritsch	SVP, Human Resources		

## Financial results and guidance

### 2019 YTD financials as of 9/30/19 (mm)

R&D expense:	\$90
G&A expense:	\$37
Net loss:	\$68
Basic share count:	36.8

### 2019 Financial highlights as of 9/30/19

Ended Q3 2019 with **\$417 million in cash**<sup>1</sup>

Recognized YTD **\$10.1 million in royalty** revenue from commercial sales of Novartis' Zolgensma, which commenced in Q2 2019

Recognized YTD **unrealized gain of \$29 million** on marketable equity securities of Prevail Therapeutics

### Program guidance and anticipated milestones

RGX-314	<b>wet AMD:</b> Initiation of Phase IIb trial in Q1 2020 <b>Diabetic retinopathy:</b> IND submission in Q1 2020
RGX-121	Interim data update in 2H 2019
RGX-111	IND active and subject recruitment ongoing
RGX-501	Interim data update in 2H 2019
RGX-181	IND, or foreign equivalent, submission in 2H 2020

### 2019 financial guidance:

*Expect 2019 ending cash balance to be **at least \$365 million***



**Thank You**