



Corporate Presentation

Leader in AAV Gene Therapy

Forward-looking statements

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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*

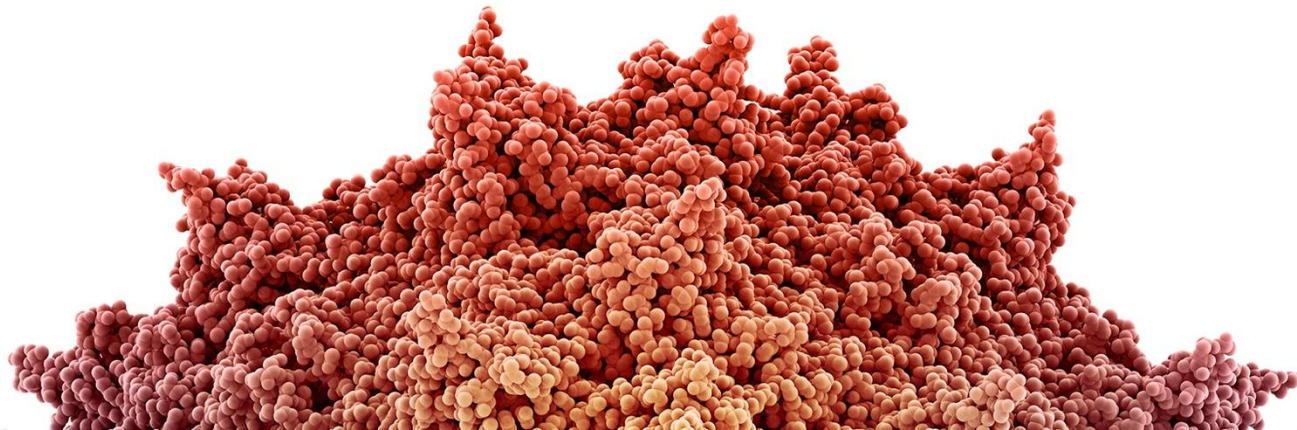
1 FDA approved product and

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

Proprietary **NAV[®] 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





























REGENXBIO®

- ▲ Orphan Drug Designation
- ★ Rare Pediatric Disease Designation
- Fast Track Designation

-  AAV-mediated antibody delivery for chronic diseases
-  Monogenic gene replacement

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	Licen see	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 + α -Syn RNAi)		CLN1			
Cardiac / skeletal muscle			Pompe Disease	AUDENTES 	CPVT	AUDENTES 	XLMTM	AUDENTES 
					Danon Disease			

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



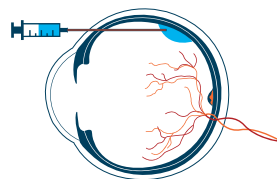
Gene: anti-VEGF Fab

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

Subjects: 42 total

Route of administration: subretinal

Sites: Eight leading retinal surgery centers across the United States

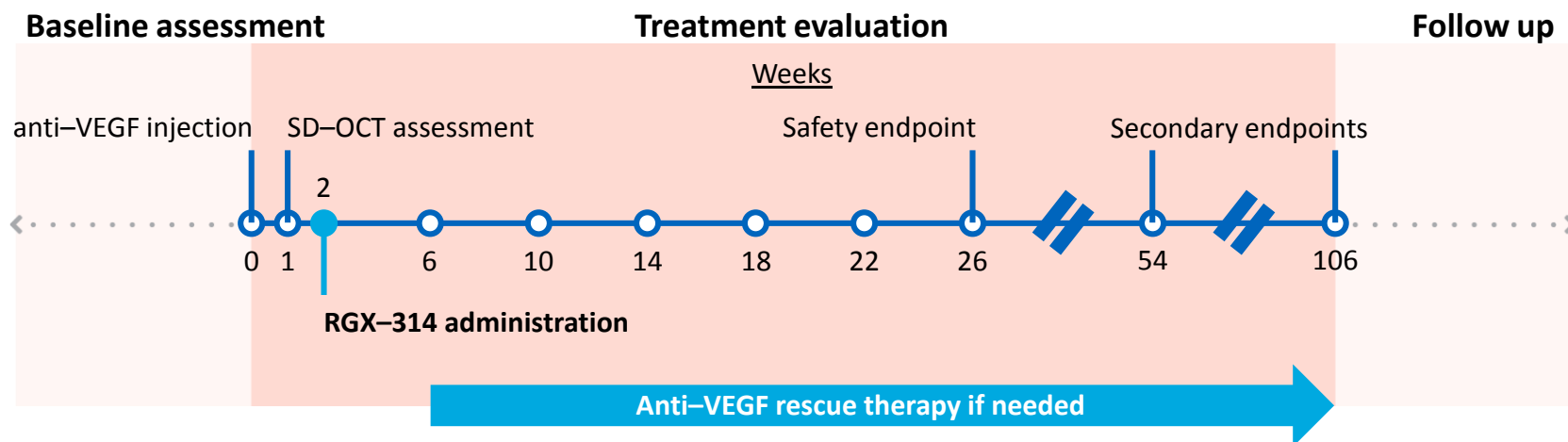


KEY INCLUSION CRITERIA

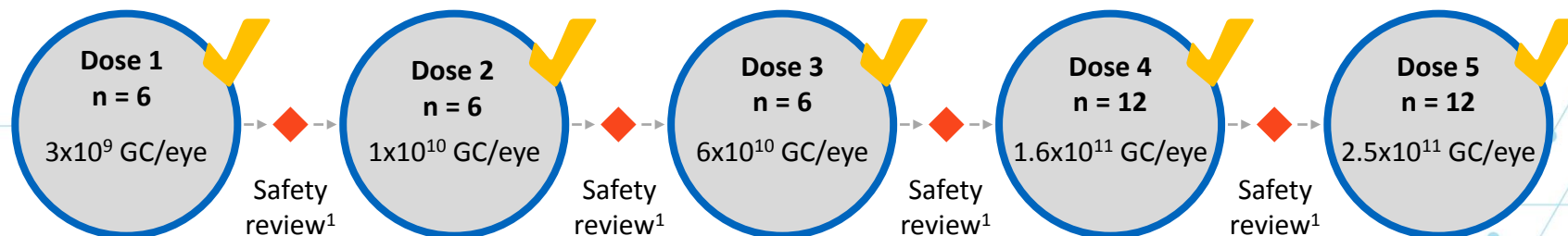
- Male or female ≥ 50 to 89 years of age
- Previously treated wet AMD subjects requiring ≥ 4 anti-VEGF injections in the 8 months prior to trial entry
- Documented response to anti-VEGF at trial entry (assessed by SD-OCT at week 1)
- Vision of 20/63 to 20/400 for the initial subject, then 20/40 to 20/400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



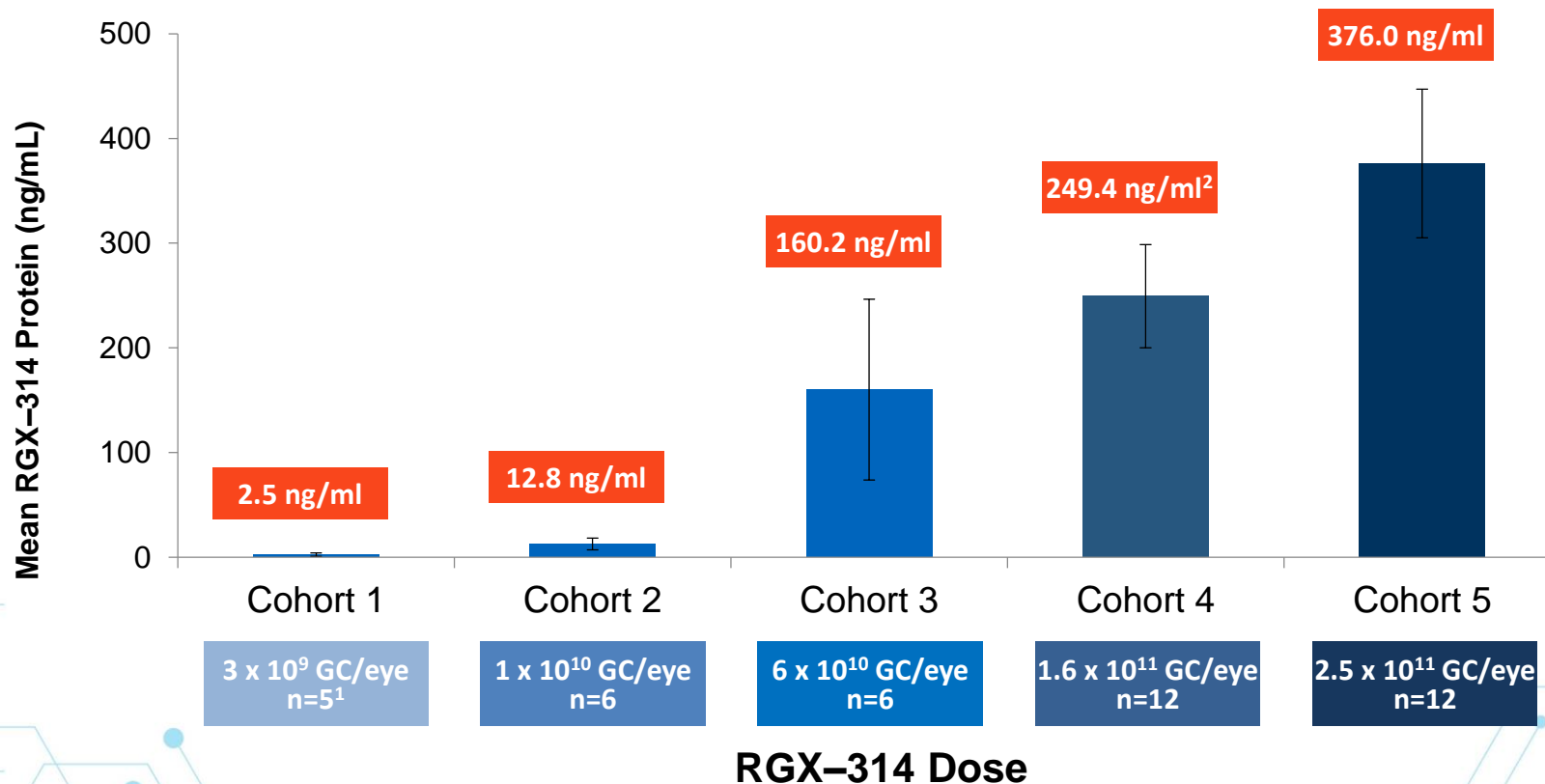
42 total subjects dosed across five cohorts

RGX-314 Phase I/IIa clinical trial: Safety and data summary¹

- 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

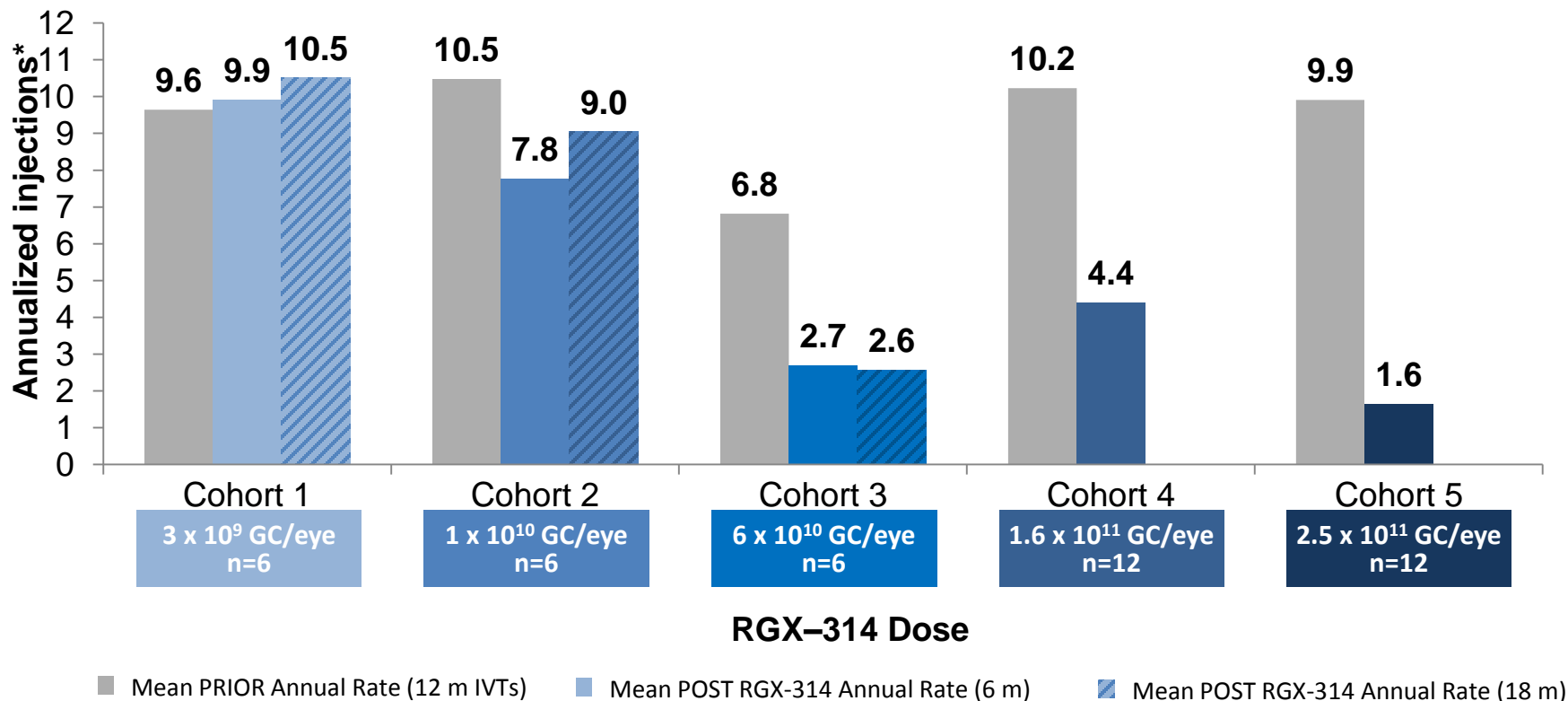


1. N=5; one subject in Cohort 1 did not have aqueous sample taken at Week 6

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

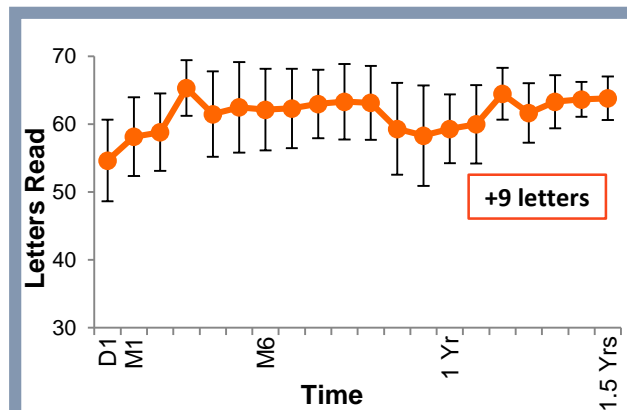
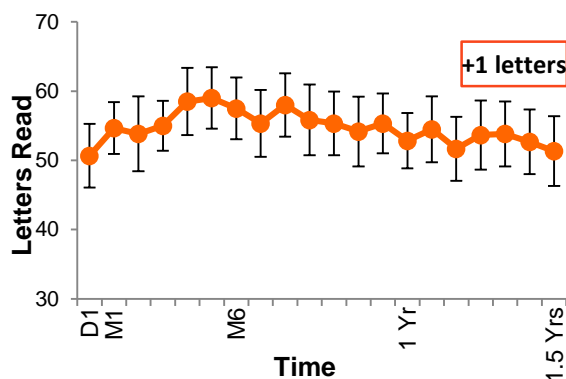
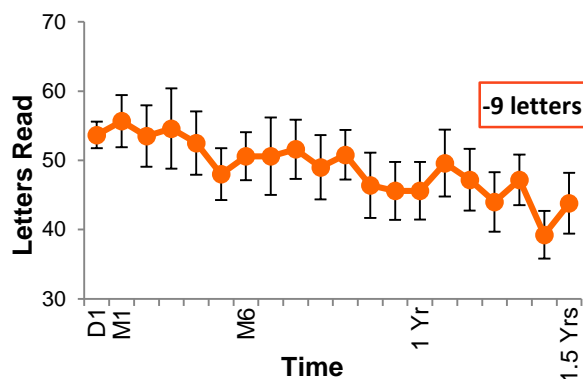
RGX-314 Phase I/IIa clinical trial: Mean change in BCVA, CRT and annualized injections over 1.5 years in cohorts 1-3

Cohort 1¹

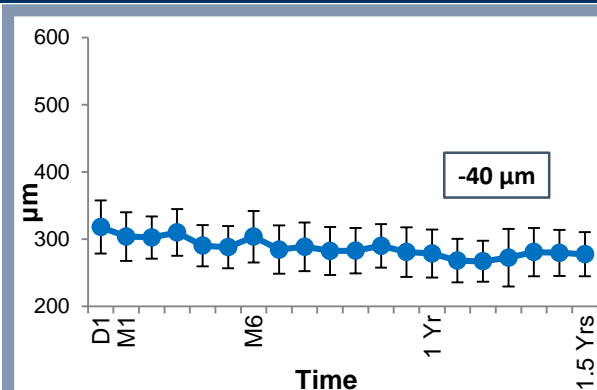
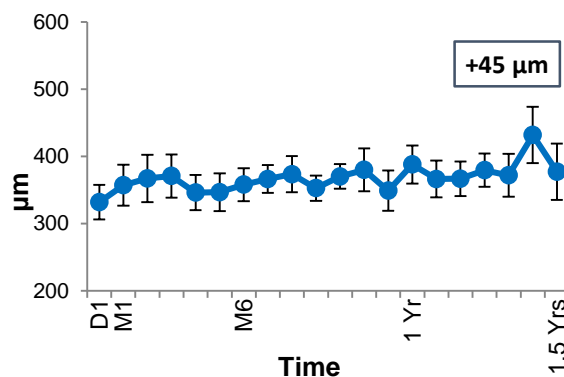
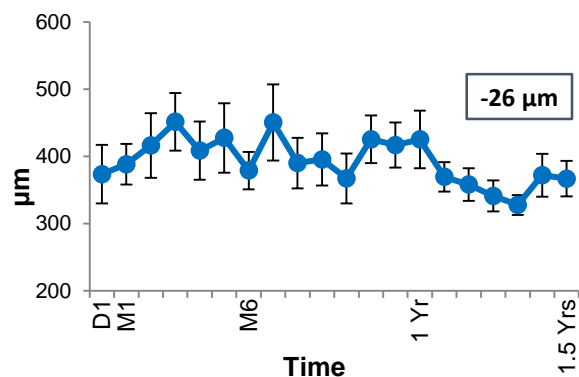
Cohort 2

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)

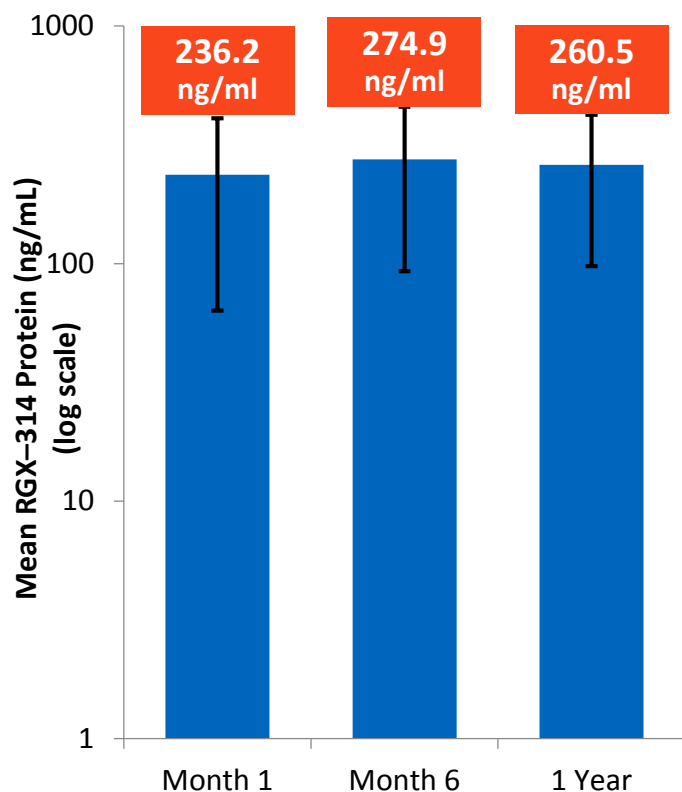


REGENXBIO®

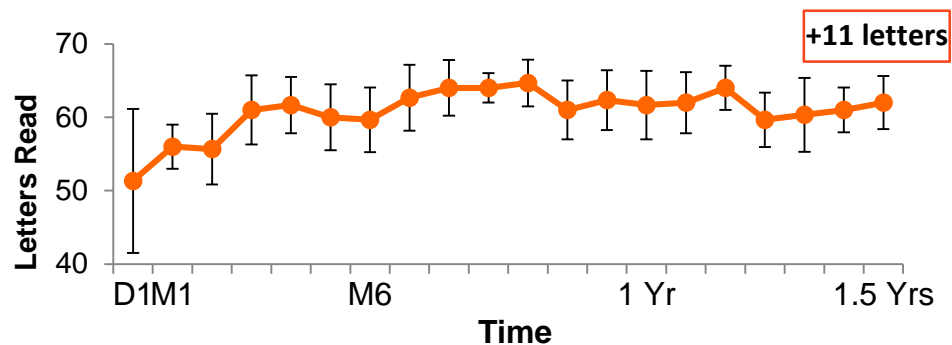
¹ One subject in Cohort 1 discontinued from the study at four months with four injections and was imputed as requiring one injection per every 4 weeks visit.

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



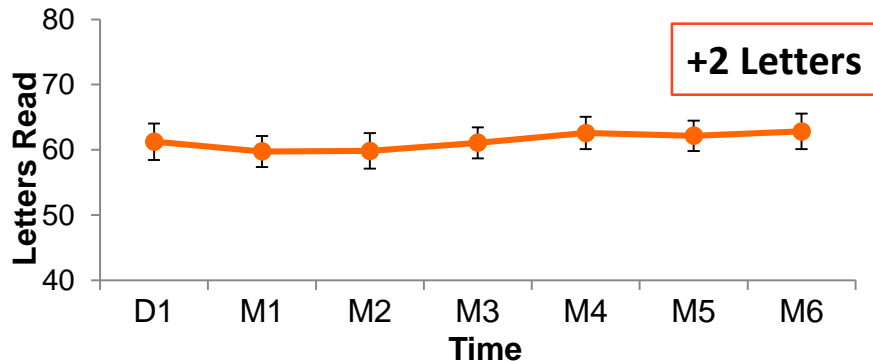
Best Corrected Visual Acuity (BCVA)



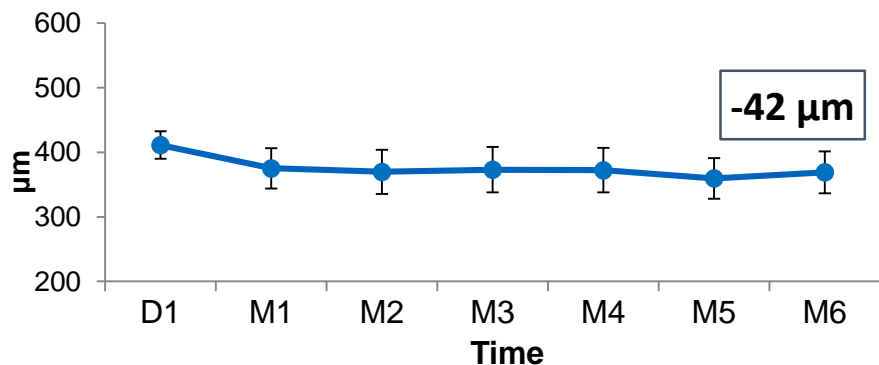
RGX-314 Phase I/IIa clinical trial: Mean change in BCVA, CRT and average injections up to 6 months in cohorts 4 and 5

Cohort 4 (n=12)

Best Corrected Visual Acuity (BCVA)



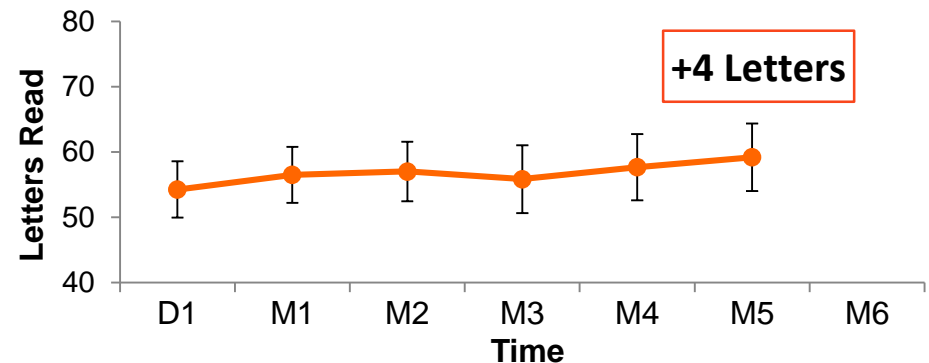
Central Retinal Thickness (CRT) on Heidelberg SD-OCT²



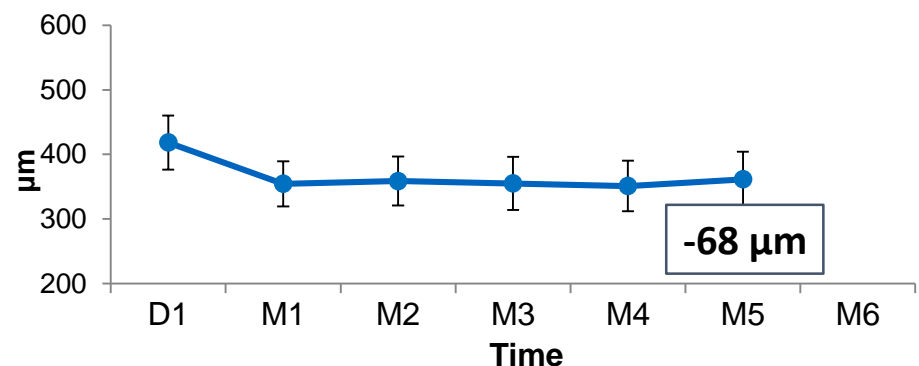
Mean: 2.2 inj / 6 mo
42% (5 of 12) injection-free at 6 months

Cohort 5 (n=12)¹

Best Corrected Visual Acuity (BCVA)



Central Retinal Thickness (CRT) on Heidelberg SD-OCT²



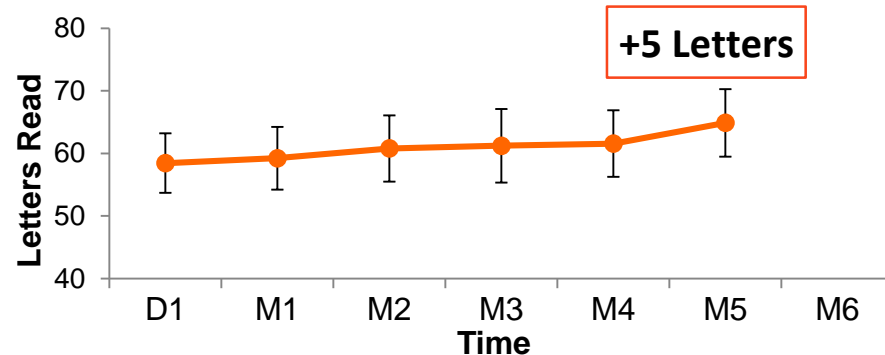
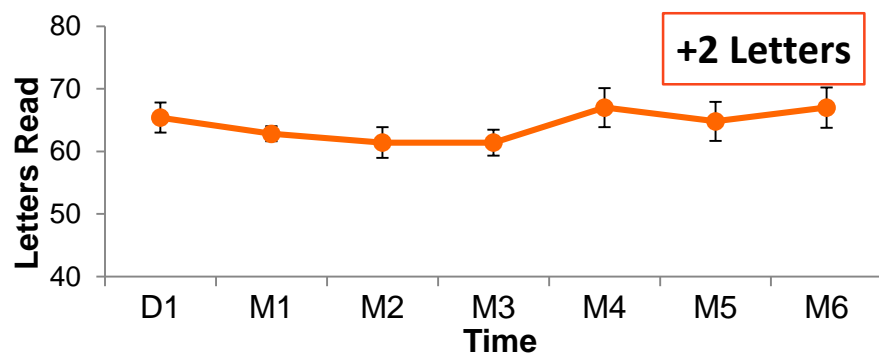
Mean: 0.8 inj / 5 - 6 mo
75% (9 of 12) injection-free at 5-6 months

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

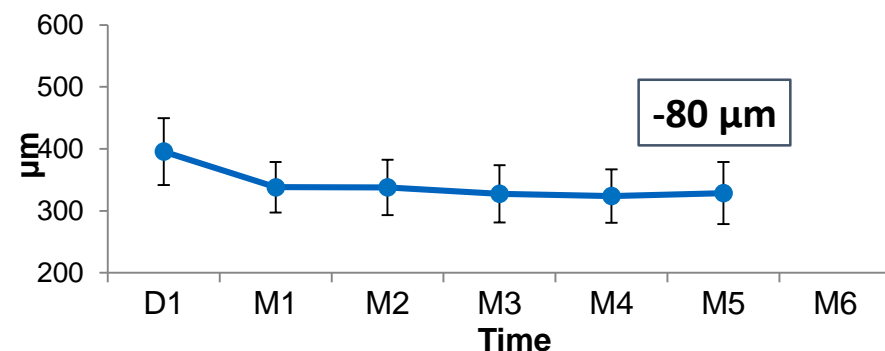
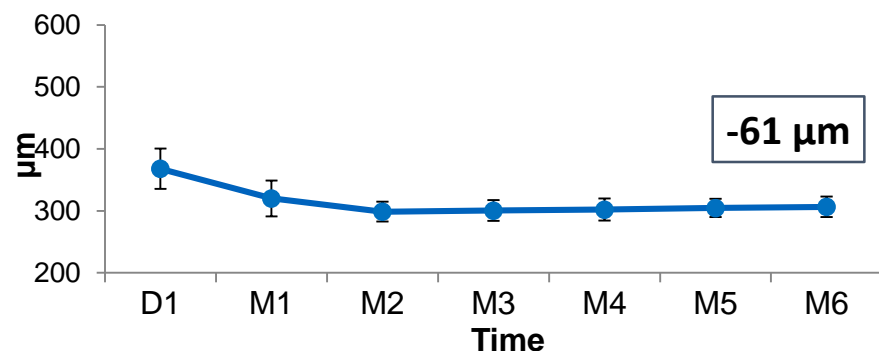
Cohort 4 (n=5)

Cohort 5 (n=9)¹

Best Corrected Visual Acuity (BCVA)



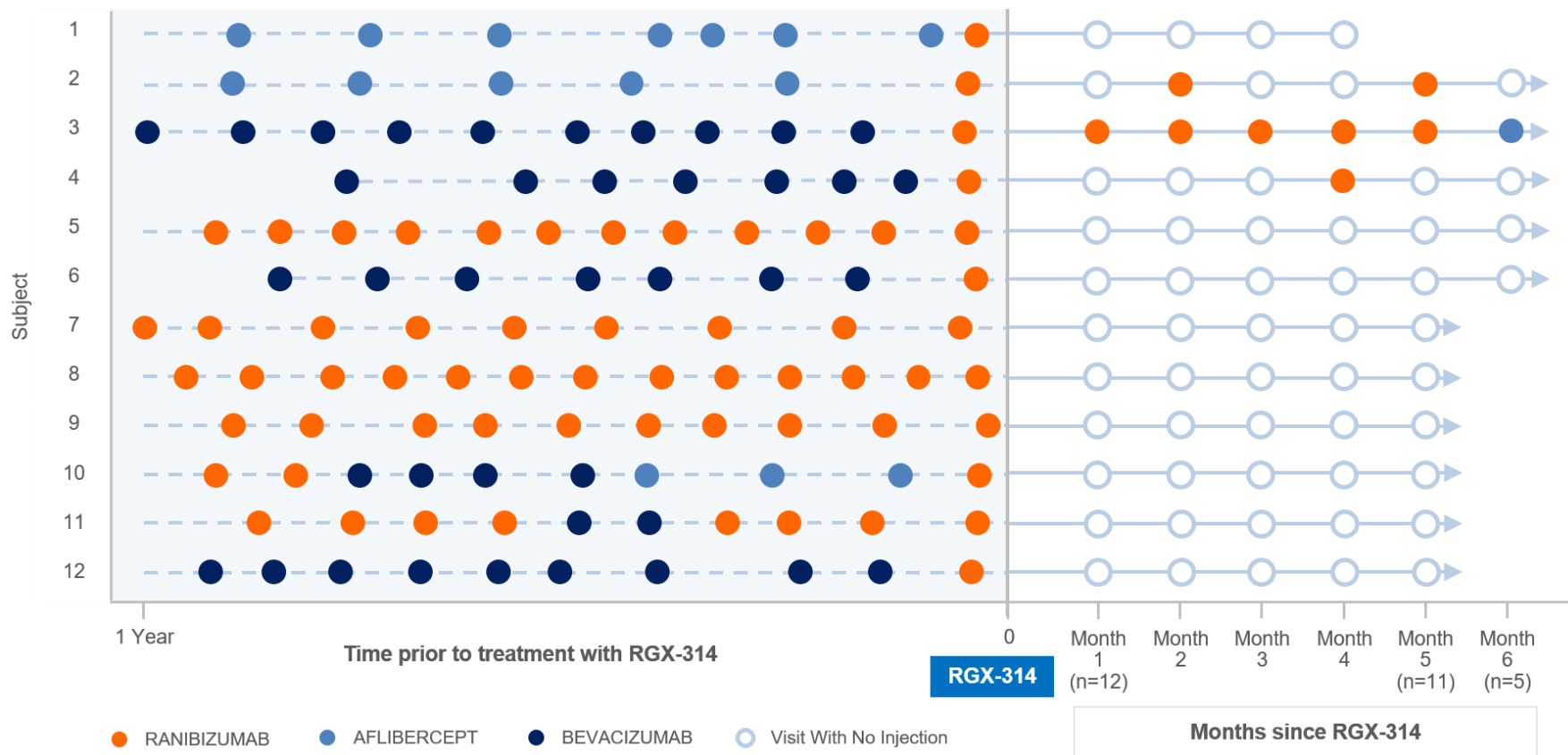
Central Retinal Thickness (CRT) on Heidelberg SD-OCT²



0 Injections

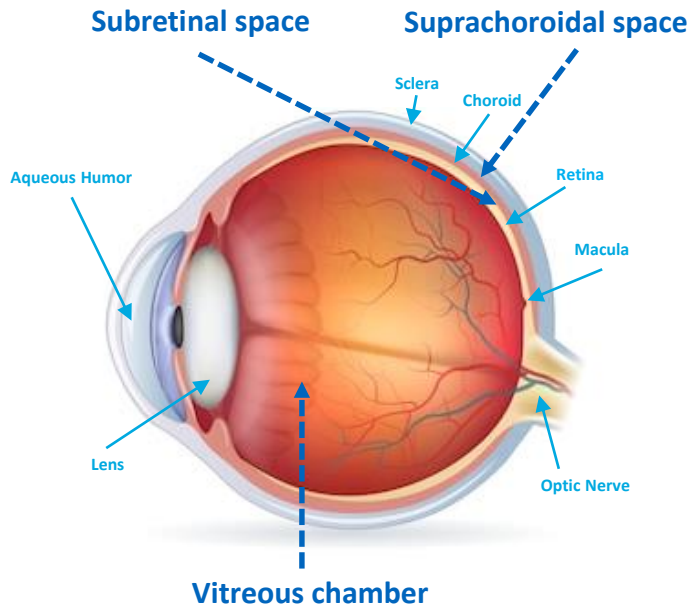
0 Injections

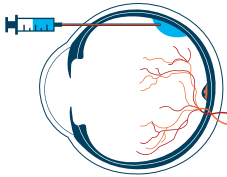
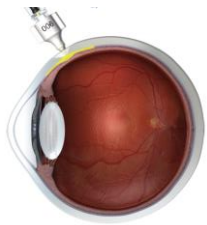

RGX-314 Phase I/IIa clinical trial: Cohort 5 Injections Pre- and Post-RGX-314



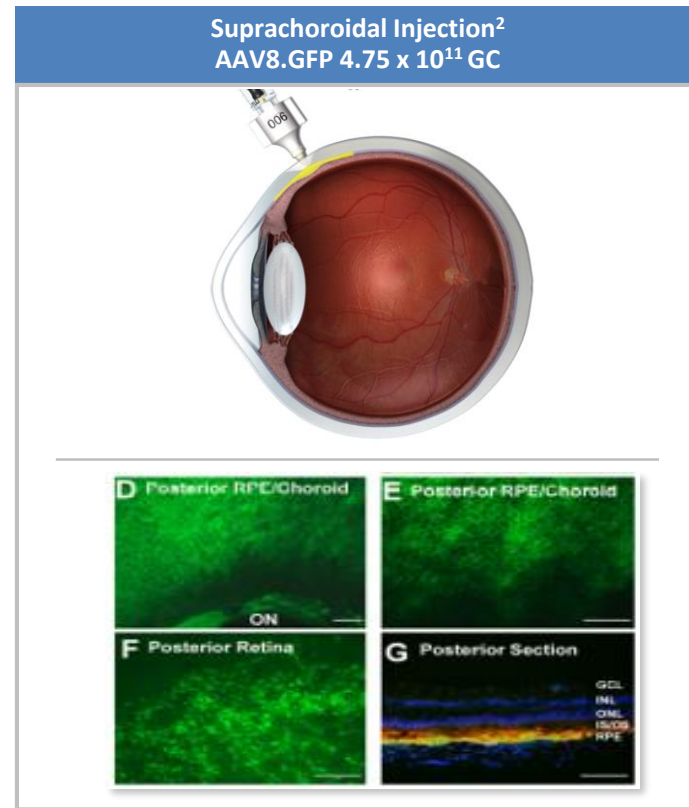
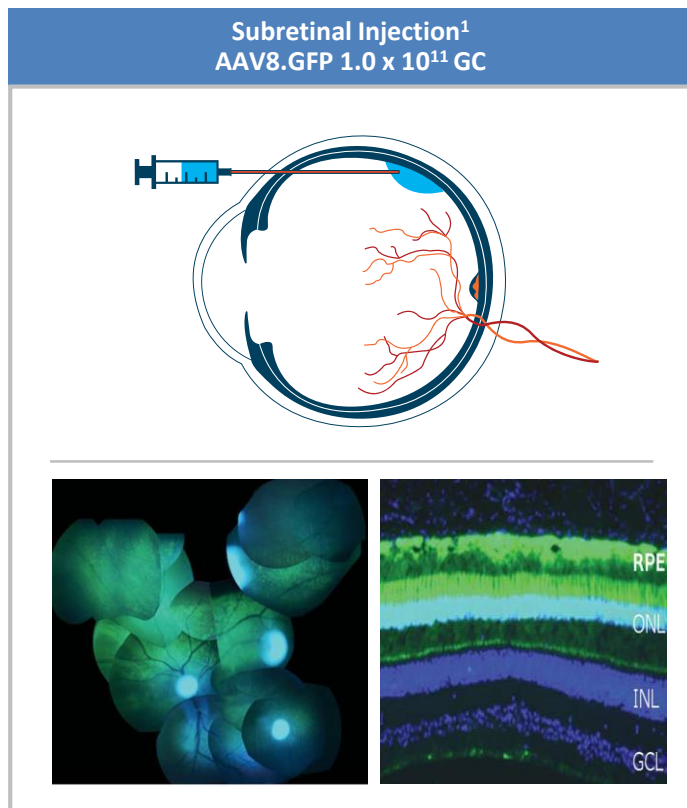
Ocular gene therapy delivery methods to reach the back of the eye

Comparative profiles



Delivery Space		Potential Patient Eligibility
 <p>Subretinal Space¹</p> <ul style="list-style-type: none"> Direct and broad transduction of the retina observed in preclinical studies Minimal exposure to the vitreous and anterior segment <ul style="list-style-type: none"> Low risk of immune response Low risk of inflammation 		<p>AAV Neutralizing Antibody (NAb) Status</p> <ul style="list-style-type: none"> All patients eligible, regardless of NAb status
 <p>Suprachoroidal Space (SCS)²</p> <ul style="list-style-type: none"> Direct and broad transduction of the retina observed in preclinical studies Minimal exposure to the vitreous and anterior segment <ul style="list-style-type: none"> Low risk of immune response Low risk of inflammation 		<p>AAV NAb Status</p> <ul style="list-style-type: none"> Patients without NAb to AAV⁸ <ul style="list-style-type: none"> ~70% for AAV8 ~30% for AAV2
 <p>Vitreous Chamber</p> <ul style="list-style-type: none"> Inner limiting membrane (ILM) presents physical barrier, potentially limiting direct transduction of the retina³ <ul style="list-style-type: none"> Limited transduction of the retina observed in preclinical studies⁴ Broad exposure to the vitreous and anterior segment <ul style="list-style-type: none"> High risk of immune response^{5,6} High risk of inflammation⁴ Potential oral systemic corticosteroid prophylaxis needed⁷ 		<p>AAV NAb Status</p> <ul style="list-style-type: none"> Patients without NAb to AAV⁸ <ul style="list-style-type: none"> ~70% for AAV8 ~30% for AAV2

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





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



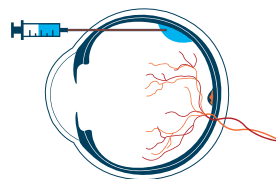
Gene: anti-VEGF Fab

Mechanism of action

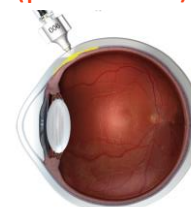
Regressing diabetic blood vessels by giving retinal cells the ability to produce an anti-VEGF fab

Routes of administration

Subretinal
(preclinical)

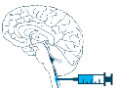
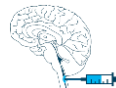



Suprachoroidal
(preclinical)





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"> Reduced ability to process glycosaminoglycans (GAGs), leading to neurodegeneration and early death X-linked recessive disease Available treatment is inadequate to treat neurodegeneration Approximately 500 – 1,000 patients born annually worldwide 	<ul style="list-style-type: none"> Reduced ability to process GAGs, leading to neurodegeneration and early death Autosomal recessive disease Available treatment is inadequate to treat neurodegeneration; bone marrow transplant partially effective Approximately 500 – 1,000 patients born annually worldwide 	<ul style="list-style-type: none"> Reduced ability to process cellular waste peptides, leading to seizures, vision loss, neurodegeneration and early death Autosomal recessive disease Available treatment requires frequent ICV infusions of ERT, shown to stabilize some but not all disease manifestations Approximately 500 patients born annually worldwide
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"> ▲ Orphan Drug Designation ★ Rare Pediatric Disease Designation ■ Fast Track Designation 	<ul style="list-style-type: none"> ▲ Orphan Drug Designation ★ Rare Pediatric Disease Designation ■ Fast Track Designation 	<ul style="list-style-type: none"> ▲ Orphan Drug Designation ★ Rare Pediatric Disease Designation



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

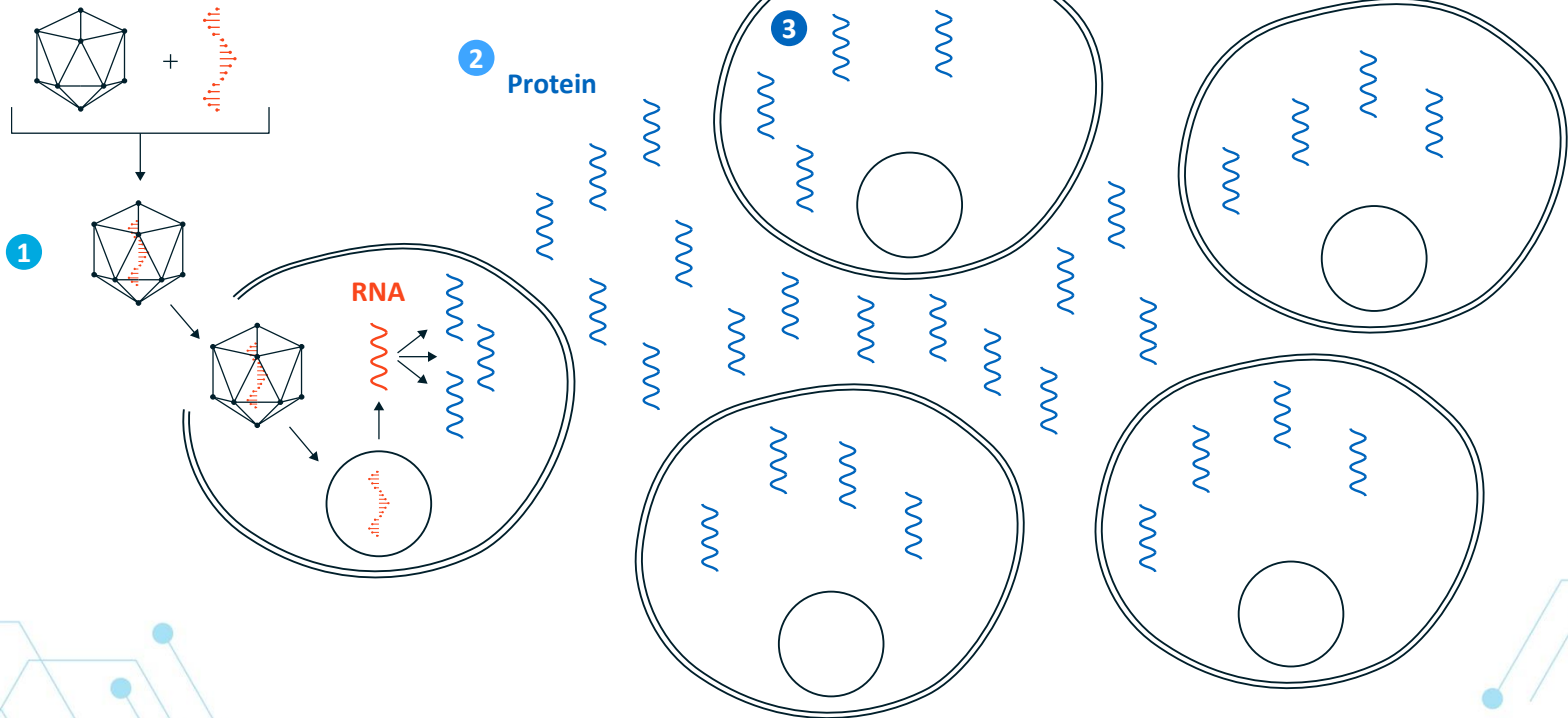
A single transduced cell has potential to correct many other cells

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



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

Subjects: Up to 6 total

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

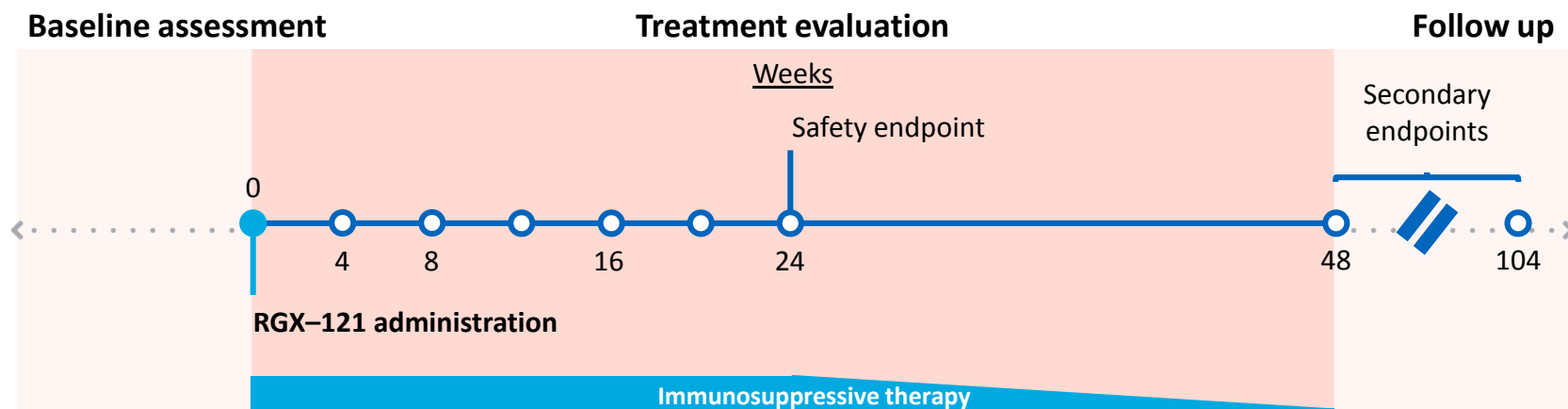


Key inclusion criteria

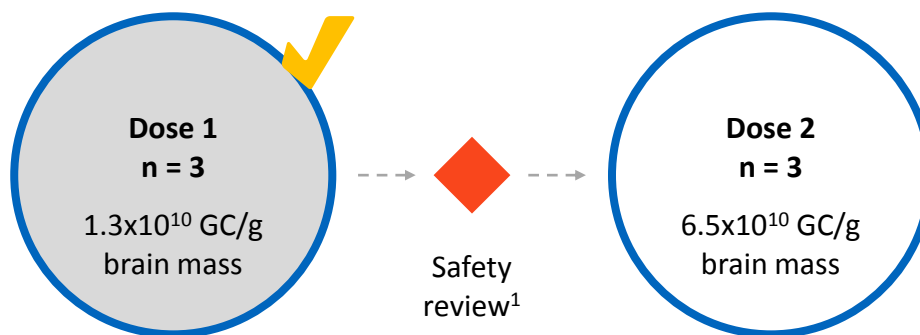
- Male subjects ≥ 4 months to < 5 years of age
- Meeting one of the following criteria:
 - Diagnosis of MPS II and a score > 55 and ≤ 77 on intelligent quotient testing OR
 - Diagnosis of MPS II and a score > 55 and a decline of ≥ 1 standard deviation on intelligent quotient testing OR
 - Having a relative 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

Subjects: Up to 5 total

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

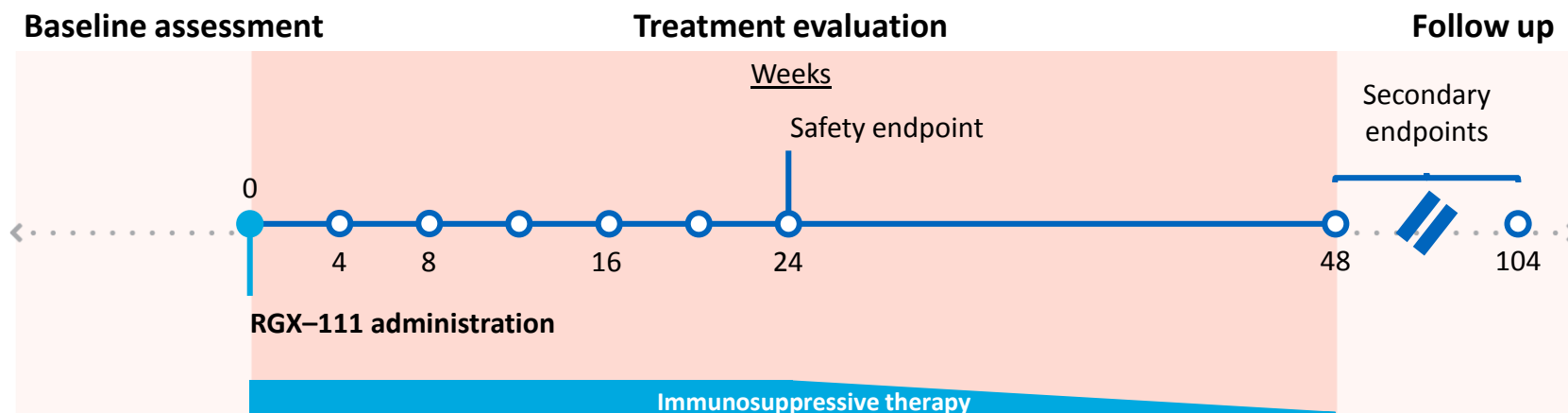


Key inclusion criteria

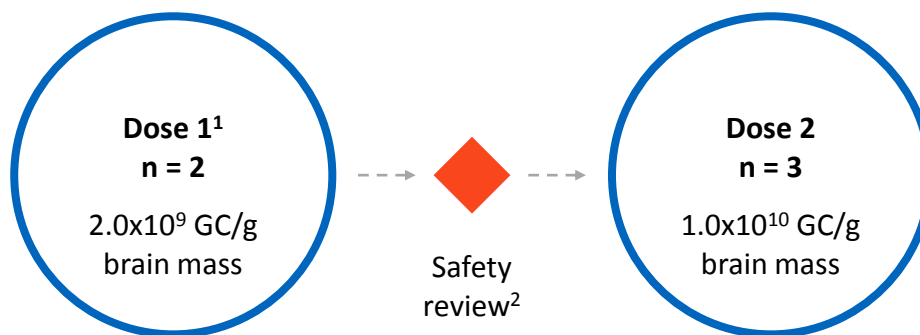
- Male or female
 - First subject ≥ 18 years of age
 - Subsequent subjects ≥ 6 years of age
- Documented evidence of early-stage neurocognitive deficit due to MPS I
 - A score of ≥ 1 standard deviation below mean on intelligent quotient testing or in one domain of neuropsychological function
 - A decline of ≥ 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





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

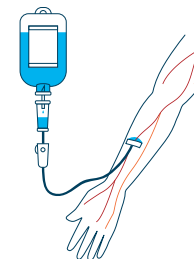
Correction of defective LDLR, reducing circulating LDL cholesterol

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

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

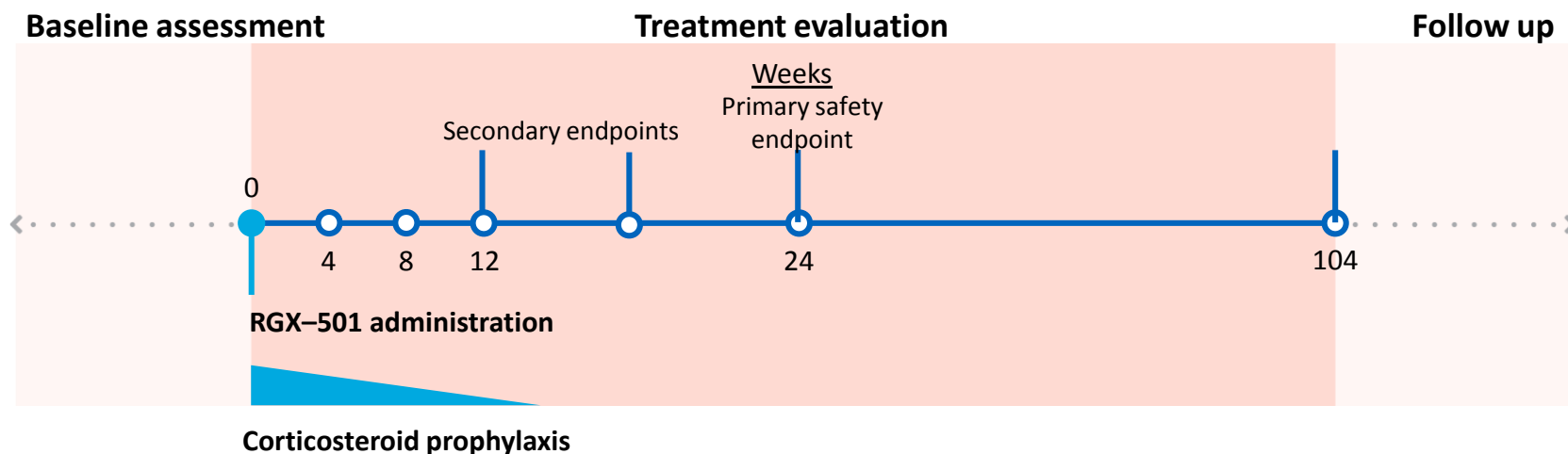


Key inclusion criteria

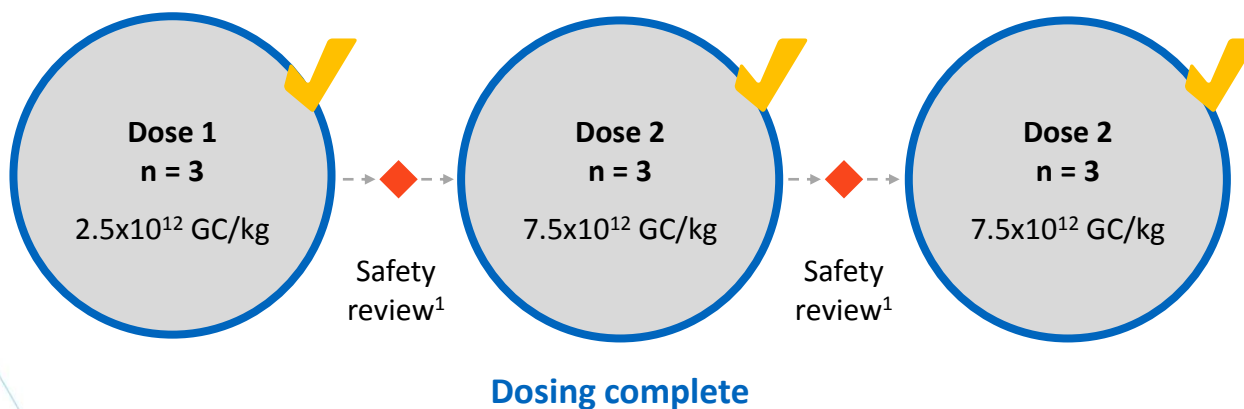
- Male or female ≥ 18 years of age
- Untreated and/or treated LDL-C levels and clinical presentation consistent with the diagnosis of HoFH
- Molecularly defined LDLR mutations at both LDLR alleles
- Stable concurrent allowed lipid lowering medications
 - Statins, ezetimibe, bile acid sequestrants, PCSK9i

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

Administration and follow-up timeline



Expected dose escalation pathway



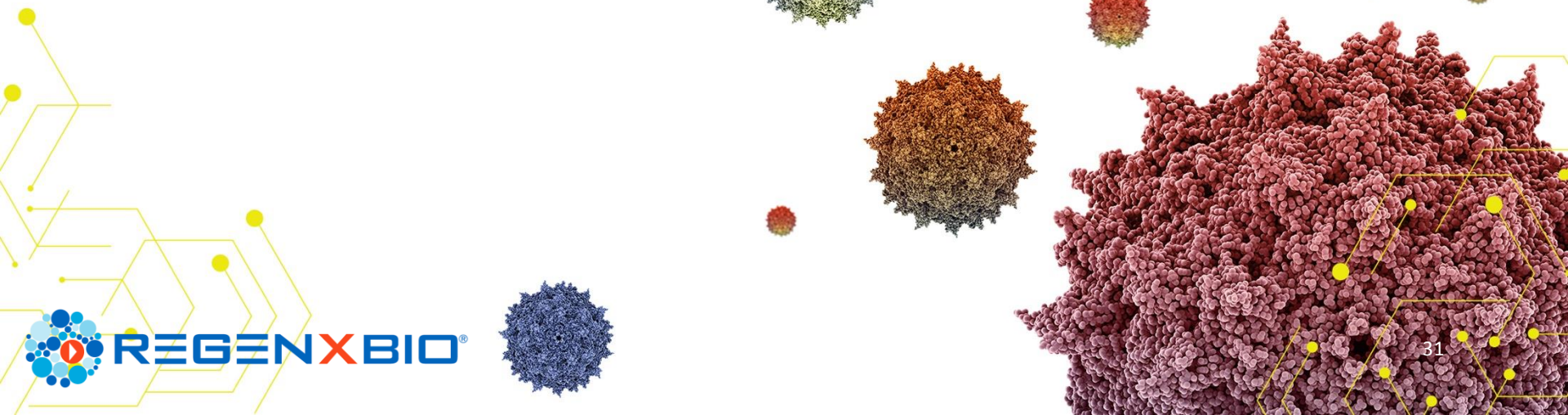


NAV[®] Technology Platform

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



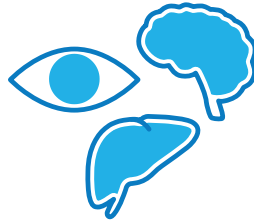
Key features of REGENXBIO's NAV Technology Platform



Higher gene expression



Longer-term gene expression



Broad and novel tissue selectivity



Lower immune response



Improved manufacturability



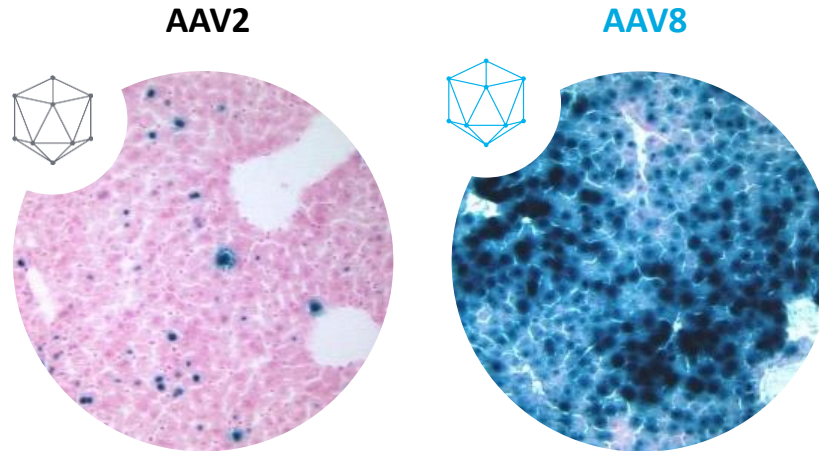
Long-Term Safety and Efficacy of Factor IX Gene Therapy in Hemophilia B



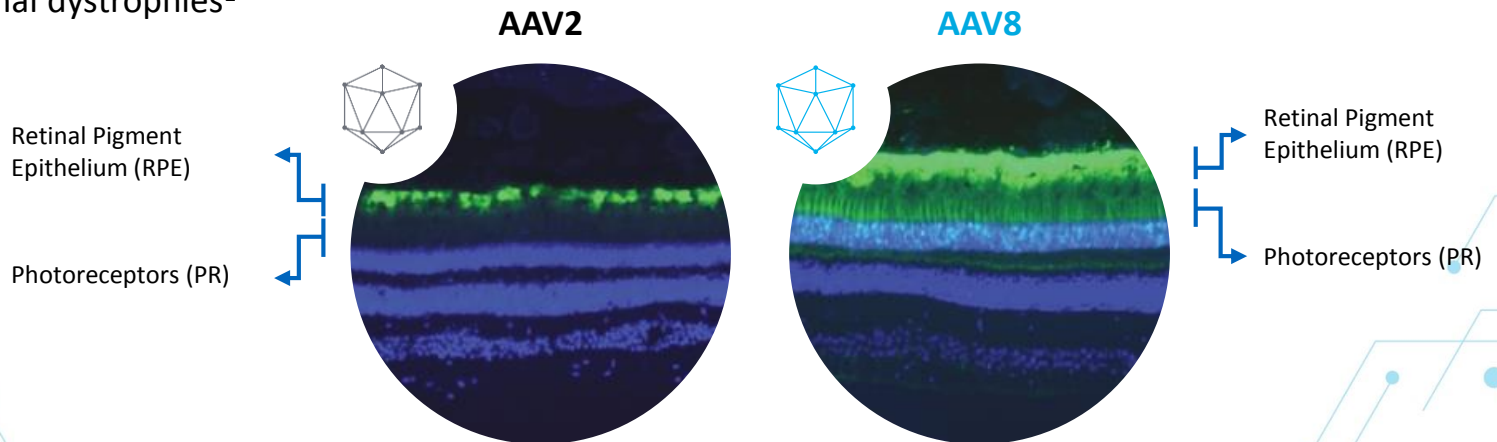
Single-Dose Gene-Replacement Therapy for Spinal Muscular Atrophy

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 dystrophies¹



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		 <i>A Member of the Roche Group</i>
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**¹

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	wet AMD: Initiation of Phase IIb trial in Q1 2020 Diabetic retinopathy: 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