Corporate Presentation

Leader in AAV Gene Therapy

REGENXBIO®

January 10, 2020

Forward-looking statements

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

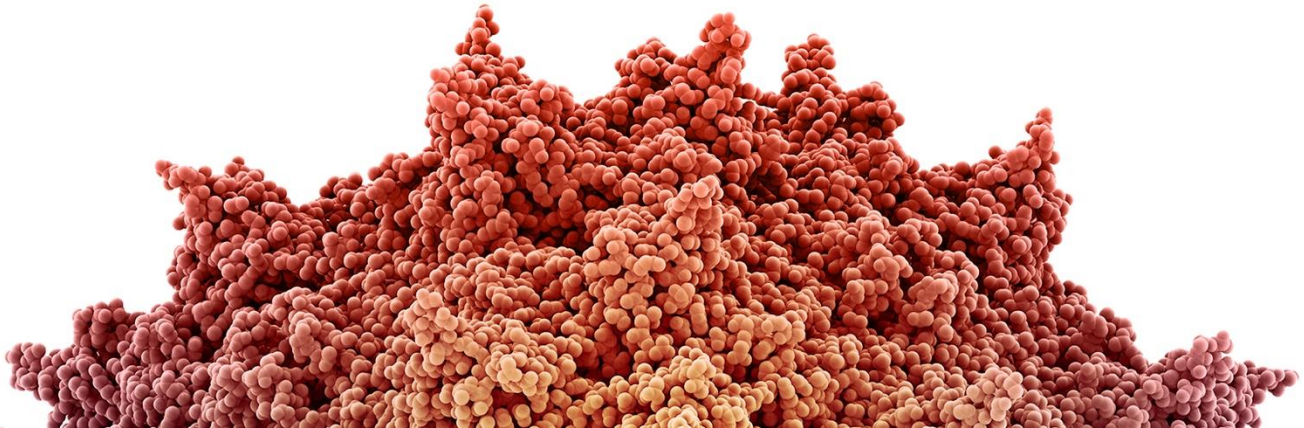
Pipeline focused on **AAV-mediated antibody delivery**
and **rare genetic diseases**
with **multiple clinical trials in 2020**

Proprietary **NAV® Technology Platform**
includes exclusive ***worldwide rights to over 100 AAV vectors***, including
AAV7, AAV8, AAV9 and **AAVrh10**

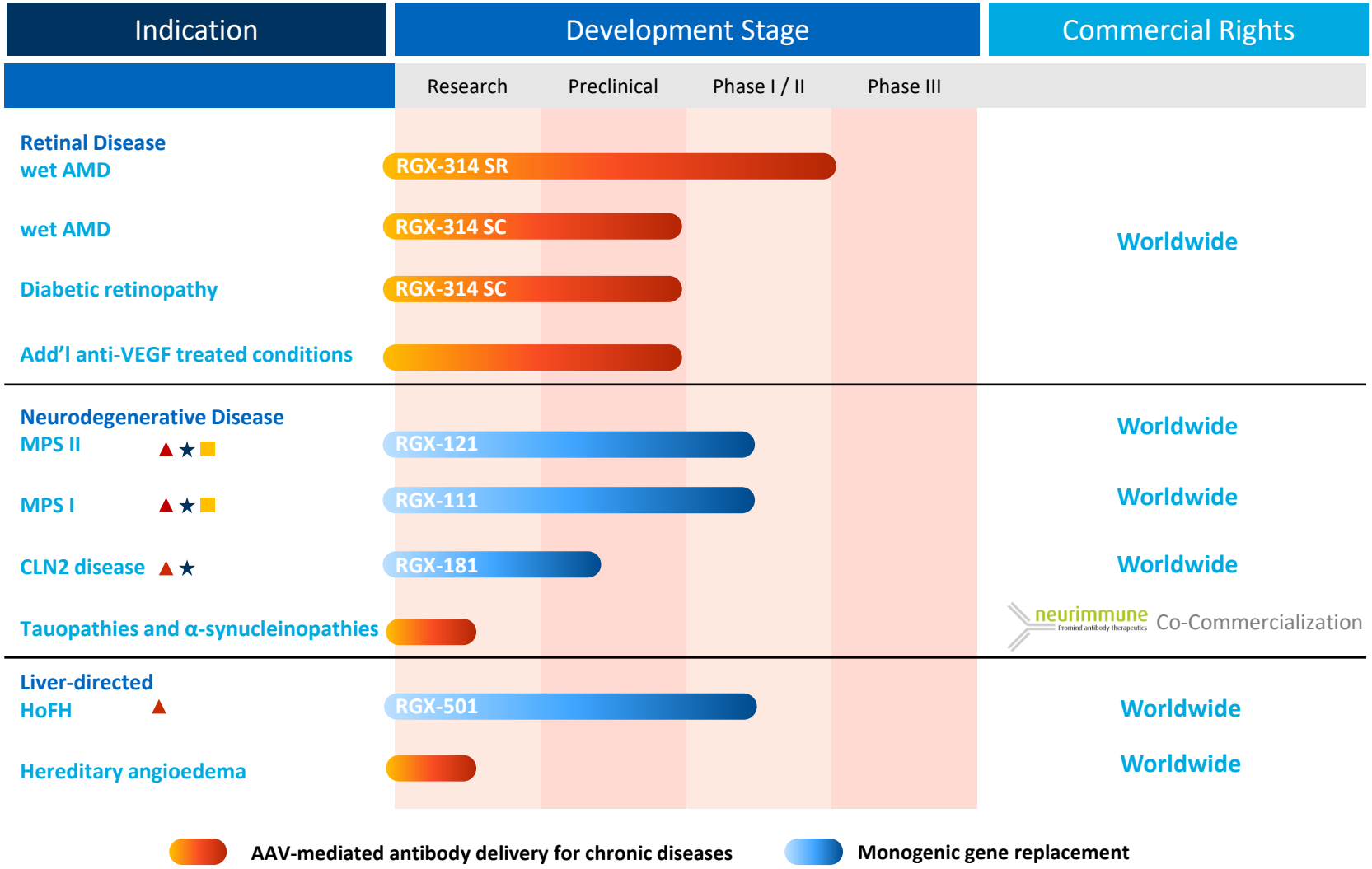
1 FDA-approved product and
15 clinical stage product candidates
being developed by third-party licensees;
over 20 partnered programs in total

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

Industry leader in AAV manufacturing



REGENXBIO's internal pipeline



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Internal Pipeline

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

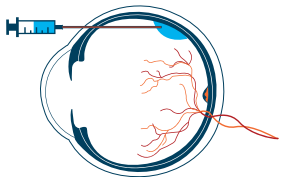
Vector: AAV8 | **Gene:** anti-VEGF Fab

Mechanism of action

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

Routes of administration

Subretinal (SR)



Suprachoroidal (SC)

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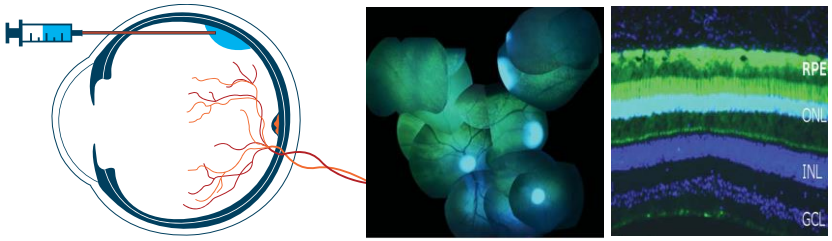
RGX-314 Routes of Administration: Two approaches to reach the back of the eye with multiple advantages to broaden market opportunity

Subretinal Delivery[1]



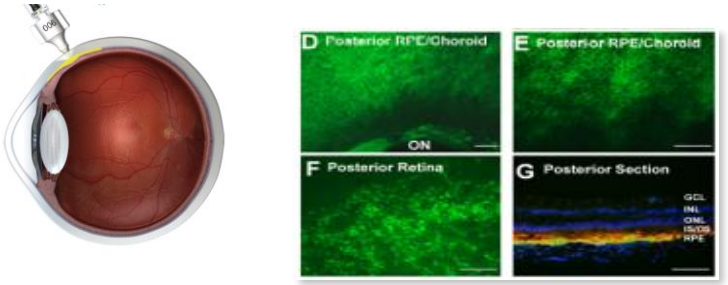
Retinal transduction achieved via subretinal delivery of AAV8 in non-human primates AAV8.GFP 1.0 x 10^{11} GC

- Established route of delivery for gene therapy
- Direct and broad transduction of the retina observed[1]
- Minimal exposure to the vitreous and anterior segment
  - Low risk of immune response
  - Low risk of inflammation
- No oral corticosteroid prophylaxis

AAV Neutralizing Antibody (NAb) Status

- All patients eligible, regardless of NAb status

Suprachoroidal Delivery[2]



Retinal transduction achieved via suprachoroidal delivery of AAV8 in non-human primates AAV8.GFP 4.75 x 10^{11} GC

- In-office, non-surgical approach using SCS Microinjector™
- Direct and broad transduction of the retina
- Minimal exposure to the vitreous and anterior segment
  - Low risk of immune response
  - Low risk of inflammation
- No oral corticosteroid prophylaxis

AAV NAb Status

- ~70% patients without NAbs to AAV8[3]

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[1] Vandenberghe et al. 2011 *Science Translational Medicine*, [2] Ding, K., et al. *2019 Journal of Clinical Investigation*, [3] Calcedo R, et al. 2009 *Journal of Infectious Disease*

RGX–314 subretinal Phase I/IIa clinical trial in wet AMD

OBJECTIVES

Primary

- To determine the safety and tolerability of subretinal 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)

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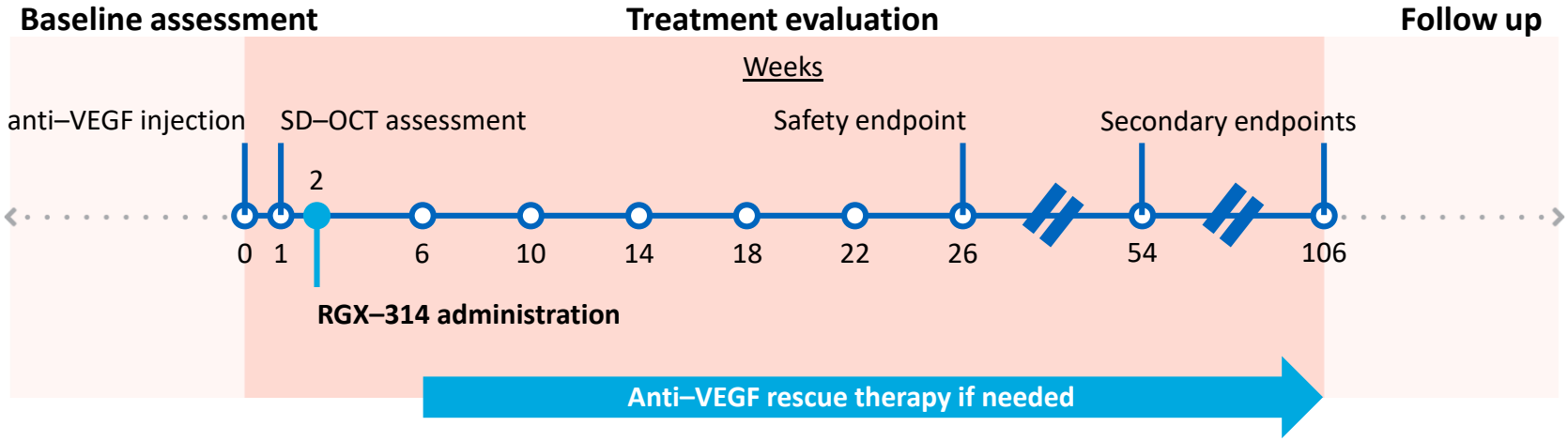
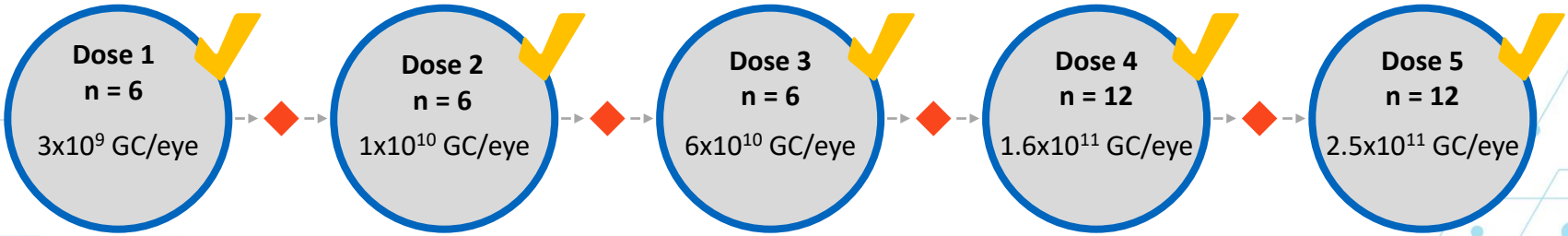
RGX–314 subretinal Phase I/IIa clinical trial: dose escalation protocol

Administration and follow-up timeline



Dose escalation



42 total subjects dosed across five cohorts

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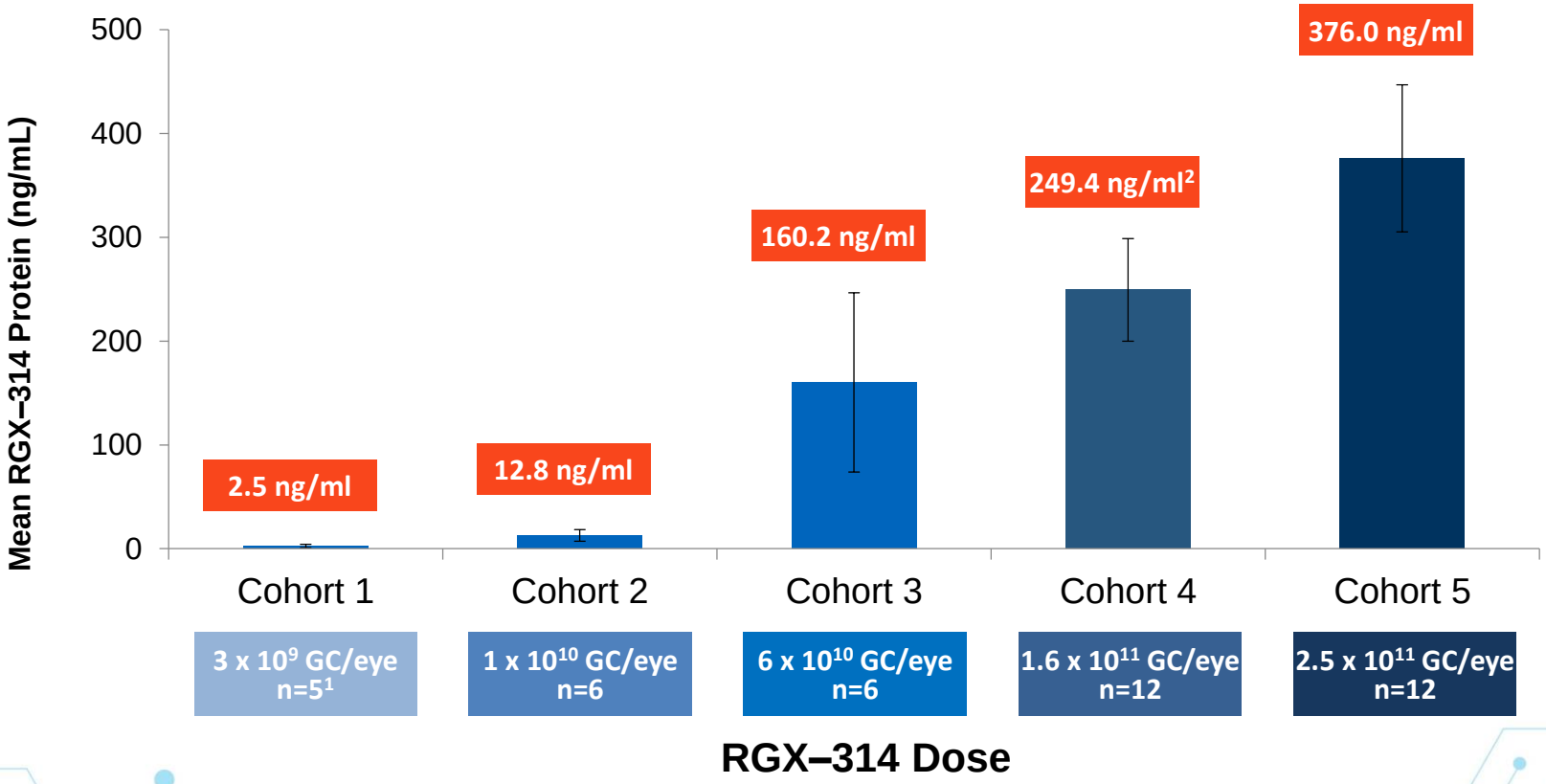
RGX–314 subretinal Phase I/IIa clinical trial: Safety and data summary[1]

- RGX–314 was **well–tolerated** (n=42)
- **No drug-related SAEs**
- Most AEs were assessed as mild (Grade 1 – 77%)
- **No observed clinically determined immune responses**, drug-related ocular inflammation, or any post-surgical inflammation beyond what is expected following routine vitrectomy

[1] Data cut December 6, 2019

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

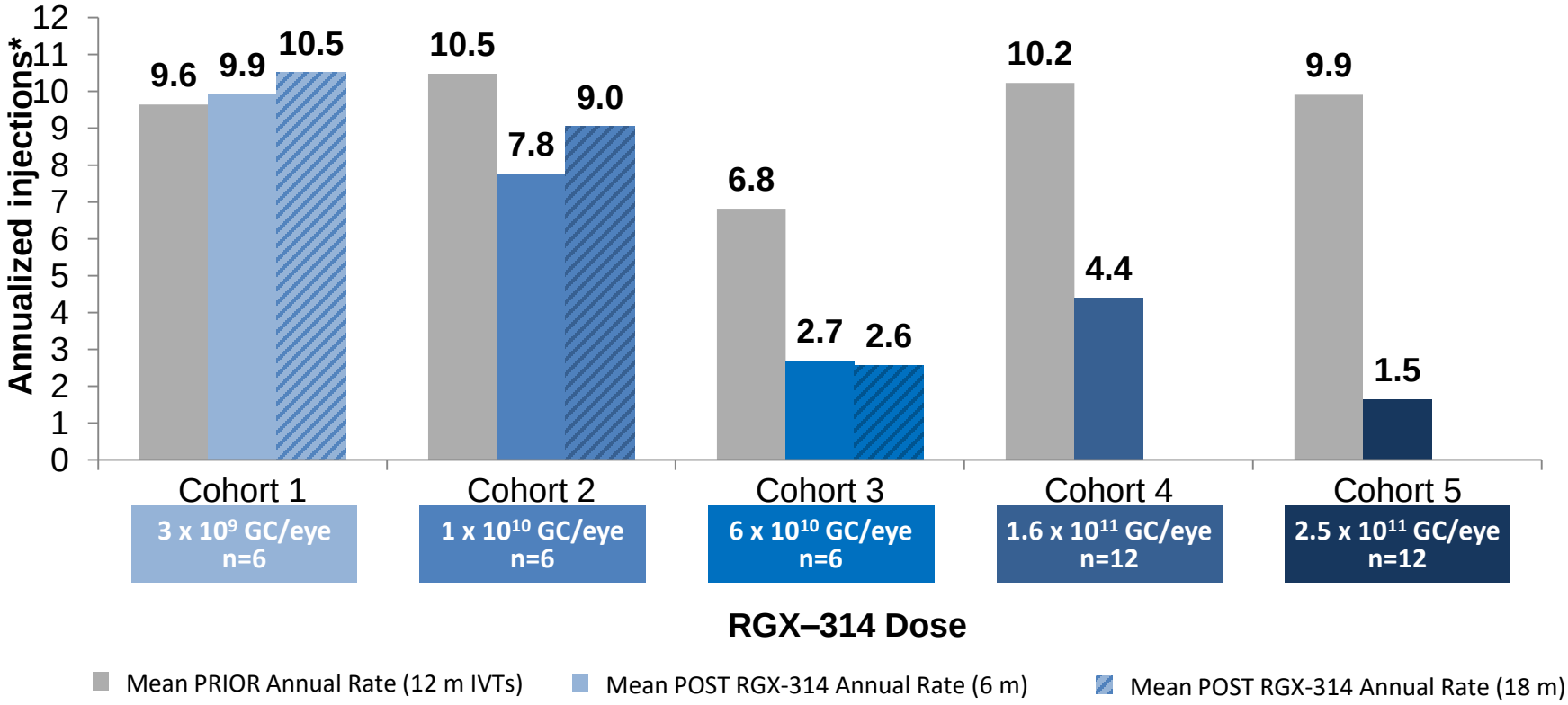
As measured from aqueous samples by ECL



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

RGX–314 subretinal 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

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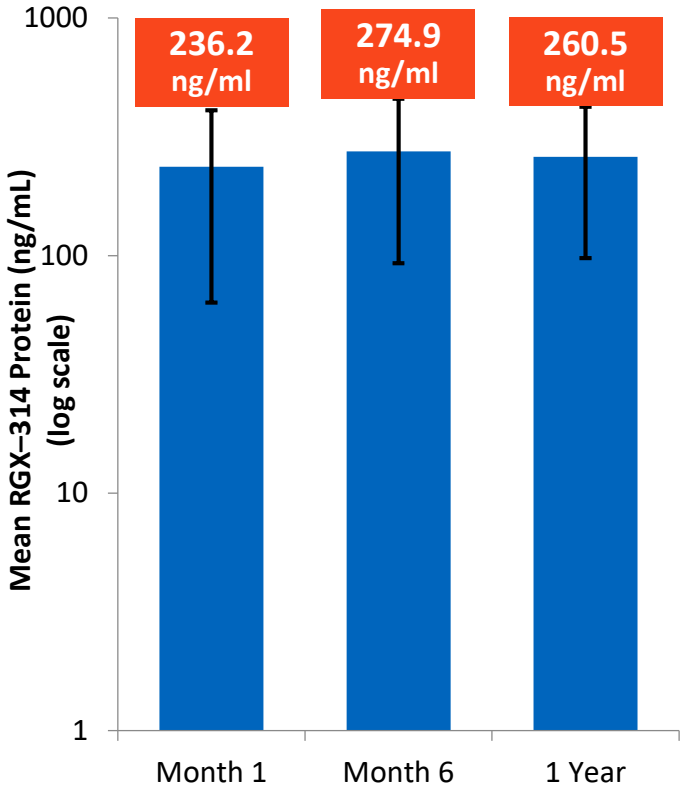
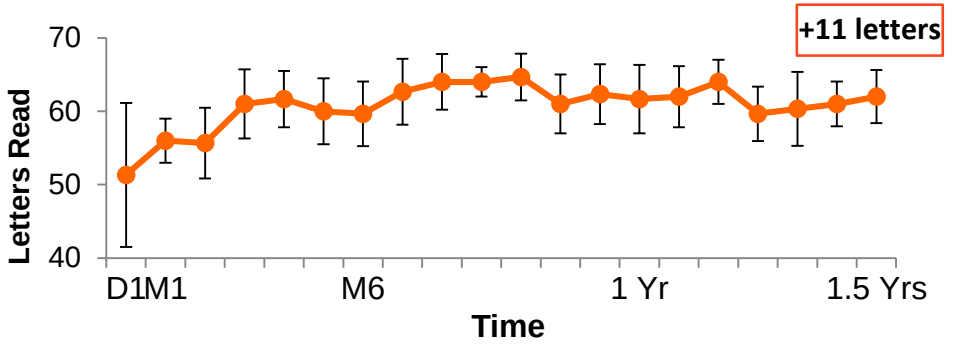
*Prior annual rate is (Total # of prior IVTs)/(minimum(366 days, Duration between first ever IVT and Day 1)/365.25). Post RGX-314 annual rate is (Total # of IVTs on Study)/(Duration on Study/365.25) where on Study is from RGX-314 administration through 18 months for C1-C3 and through 6 months for C4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

RGX–314 subretinal Phase I/IIa clinical trial: Cohort 3 anti-VEGF injection-free subjects (n=3 of 6) continue to do well over 1.5 years

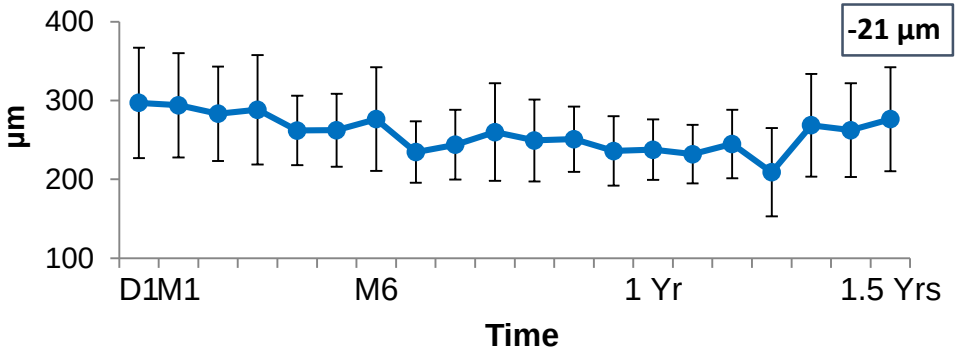
Sustained RGX-314 Protein Levels Over 1 Year



Best Corrected Visual Acuity (BCVA)



Central Retinal Thickness (CRT) on Heidelberg SD-OCT



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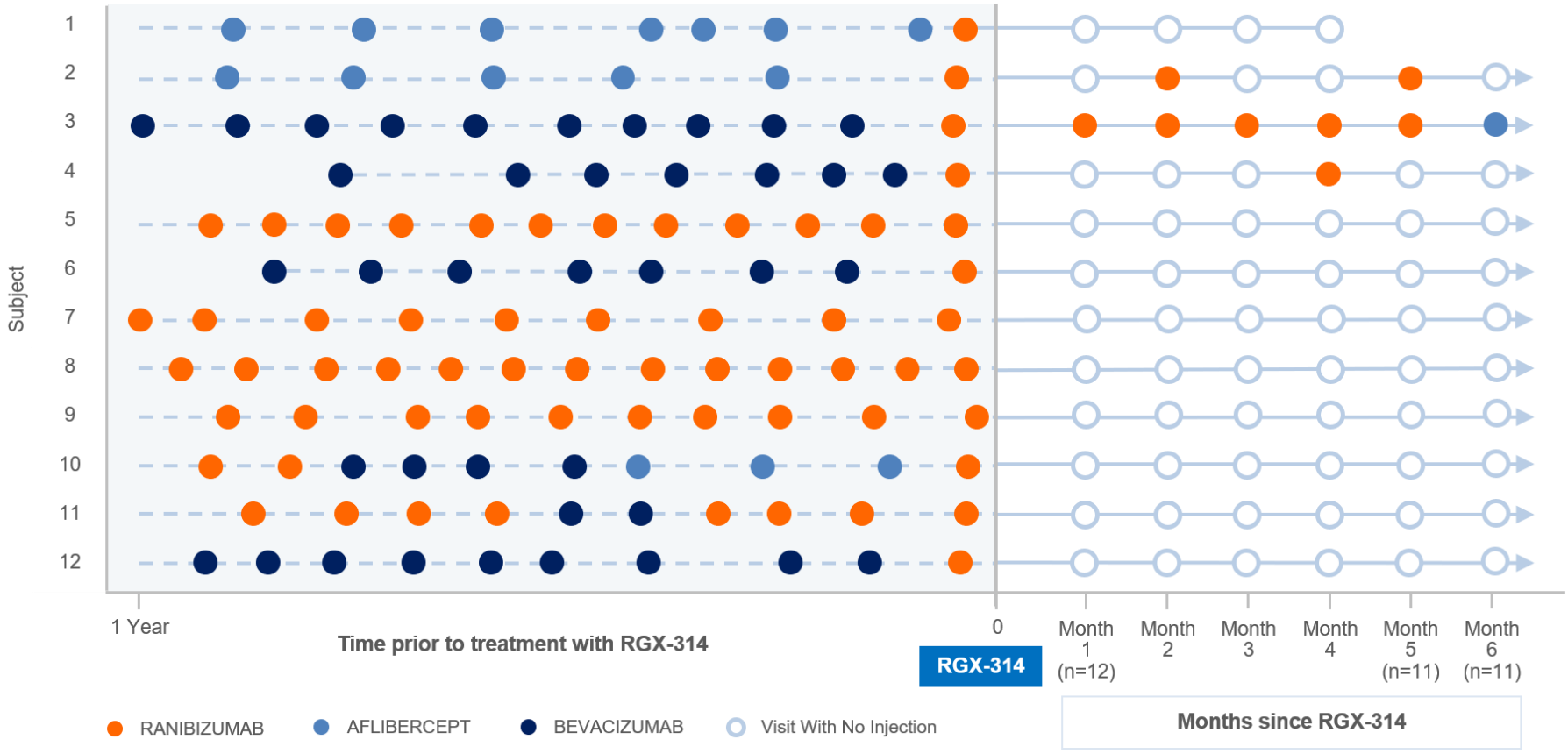
RGX–314 subretinal Phase I/IIa clinical trial: Cohort 5 injections pre- and post-RGX-314



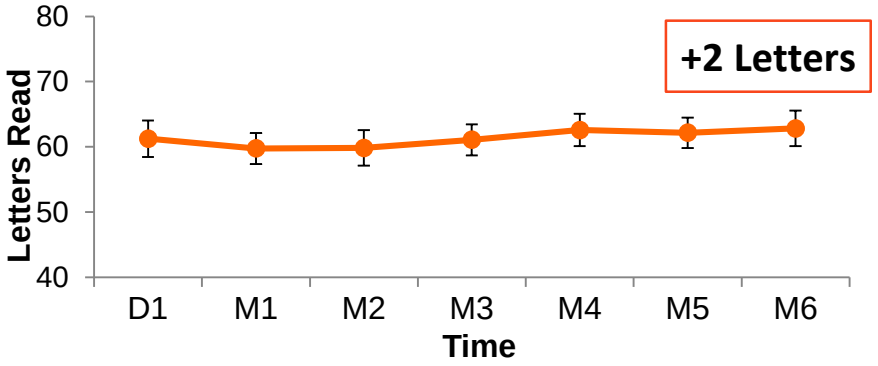
Subject #1 discontinued after 4 months

* Data cut December 6, 2019

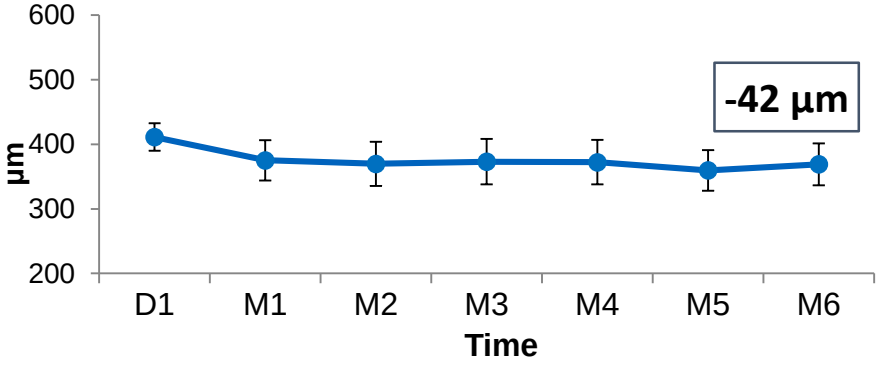
RGX–314 subretinal Phase I/IIa clinical trial: Mean change in BCVA, CRT and average injections up to 6 months in cohorts 4 and 5

Cohort 4 (n=12)

Best Corrected Visual Acuity (BCVA)



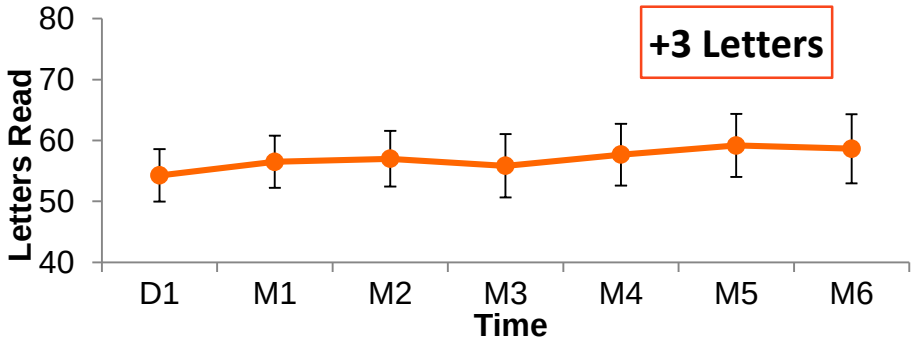
Central Retinal Thickness (CRT) on Heidelberg SD-OCT[2]



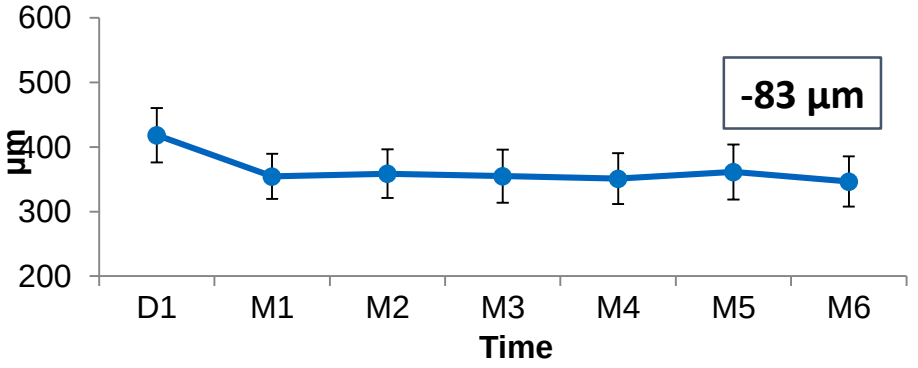
Mean: 2.2 inj / 6 mo
42% (5 of 12) injection-free at 6 months

Cohort 5 (n=12)[1]

Best Corrected Visual Acuity (BCVA)



Central Retinal Thickness (CRT) on Heidelberg SD-OCT[2]



Mean: 0.8 inj / 6 mo
73%[3] (8 of 11) injection-free at 6 months

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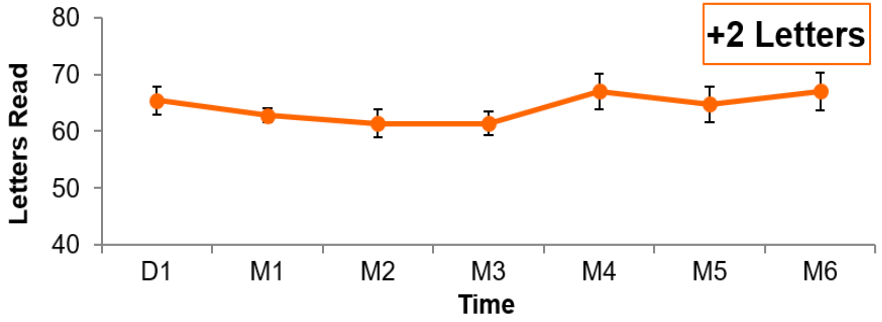
[1]One patient discontinued after 4 months
[2]SD-OCT data read by a central reading center (Duke Reading Center).
[3]Injection-free calculation (8 of 11) does not include the subject that discontinued after 4 months; subject was injection-free at time of discontinuation

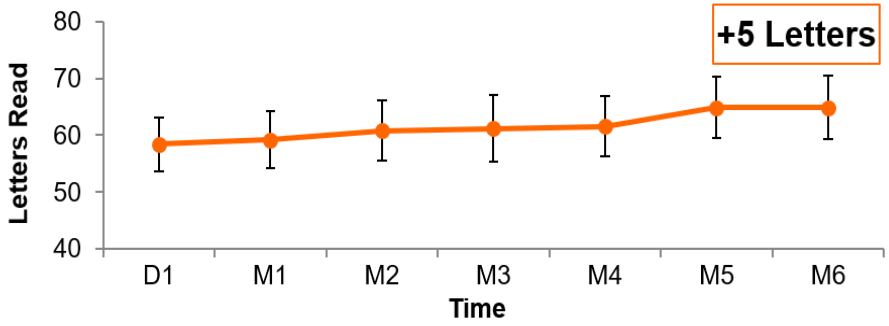
RGX-314 subretinal Phase I/IIa clinical trial: BCVA and CRT in anti-VEGF injection-free subjects from Cohorts 4 and 5

Cohort 4 (n=5)

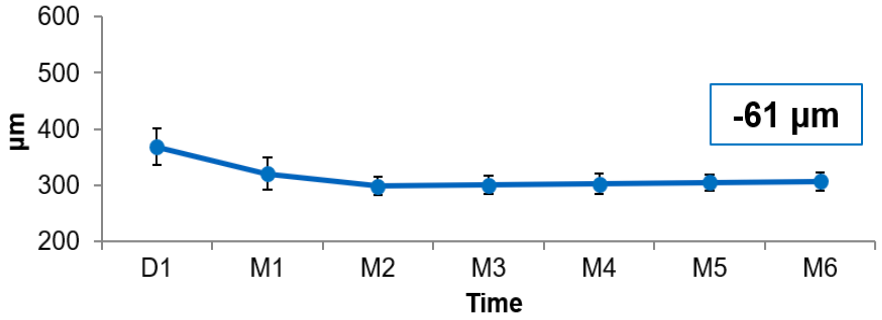
Cohort 5 (n=9)[2]

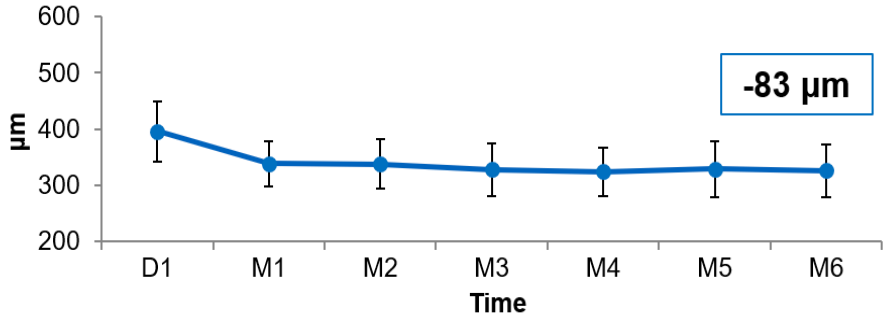
Best Corrected Visual Acuity (BCVA)





Central Retinal Thickness (CRT) on Heidelberg SD-OCT[1]





0 Injections

0 Injections

[1]SD-OCT data read by a central reading center (Duke Reading Center).
[2]One patient discontinued after 4 months (subject injection-free at time of discontinuation)

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

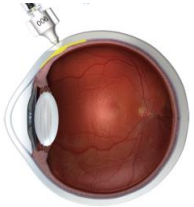
Gene: anti-VEGF Fab

Mechanism of action

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

Route of administration

Suprachoroidal



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REGENXBIO's neurodegenerative disease franchise

	RGX–121 for MPS II	RGX–111 for MPS I	RGX–181 for CLN2 disease
Disease	▪ 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	▪ 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	▪ 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
Desig-nations	▲ Orphan Drug Designation ★ Rare Pediatric Disease Designation ■ Fast Track Designation	▲ Orphan Drug Designation ★ Rare Pediatric Disease Designation ■ Fast Track Designation	▲ Orphan Drug Designation ★ Rare Pediatric Disease Designation

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

Subjects: Up to 6 total

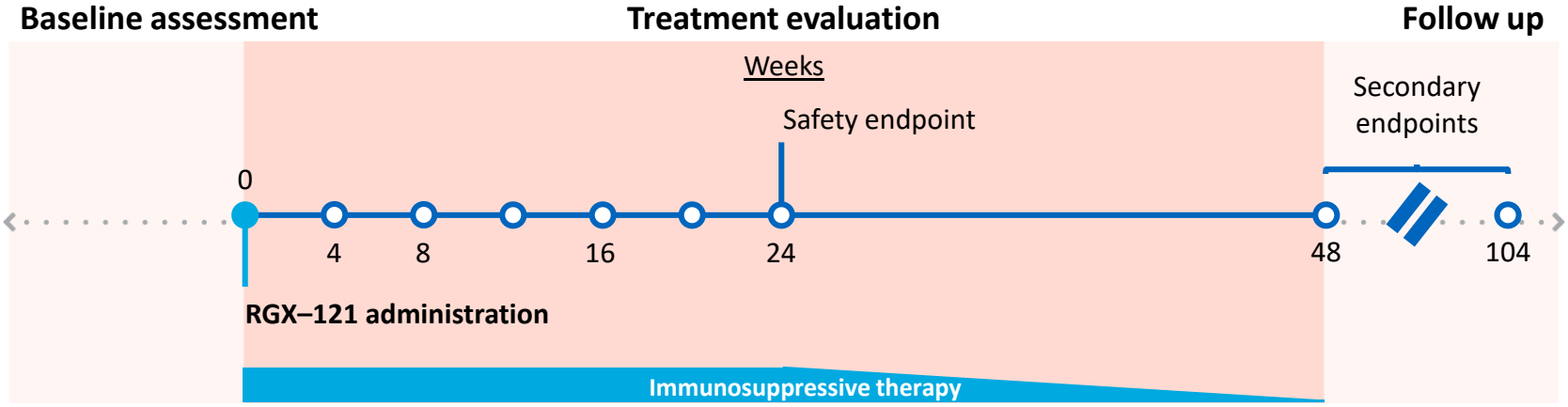
Sites: Leading U.S. and international lysosomal storage disease centers

Key inclusion criteria

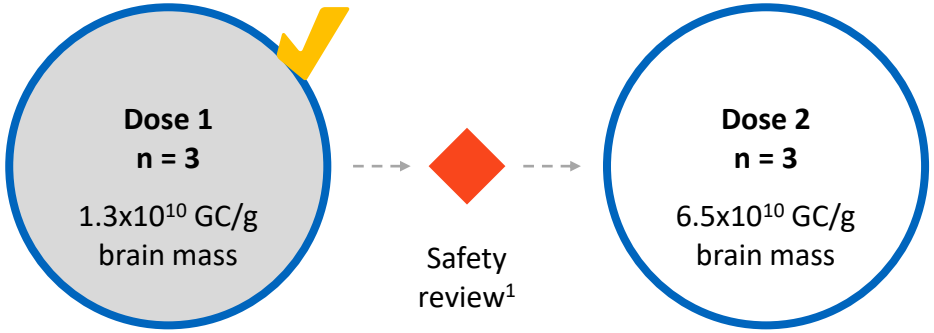
- Male subjects ≥4 months to <5 years of age
- Meeting one of the following criteria:
  - Diagnosis of MPS II and a score >55 and ≤77 on intelligent quotient testing OR
  - Diagnosis of MPS II and a score >55 and a decline of ≥1 standard deviation on intelligent quotient testing OR
  - Having a relative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



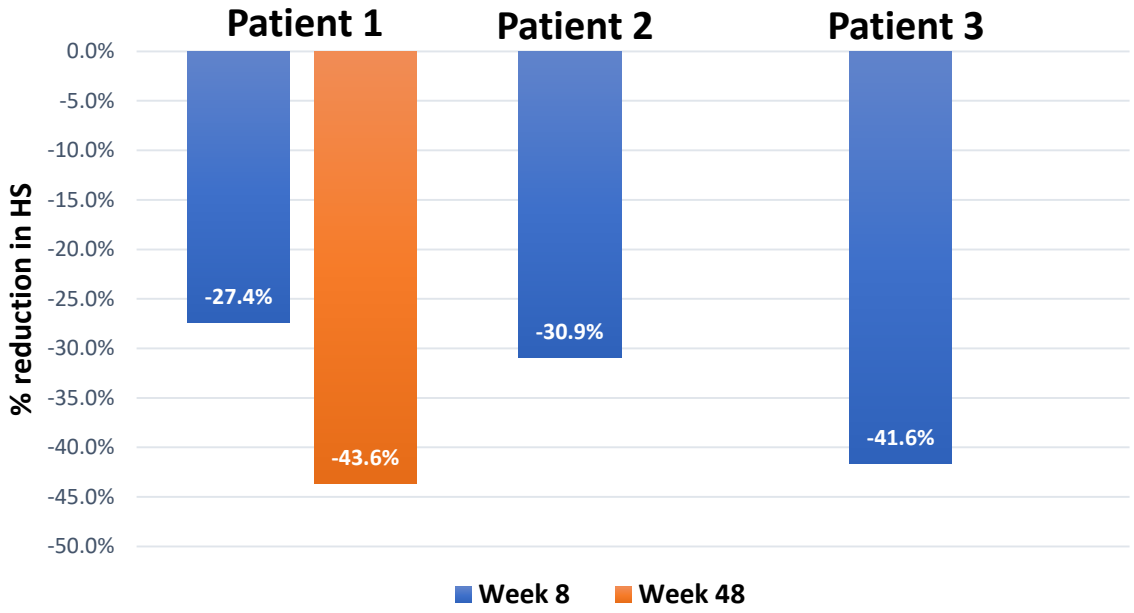
Dosing complete in the first cohort; dosing in second cohort has begun

[1] Dose escalation safety review to occur eight weeks after final subject in cohort has been dosed

RGX–121 Phase I/II clinical trial: Initial results from Cohort 1[1]

- *RGX-121 was **well-tolerated following one-time intracisternal administration** (n=3)*
  - No drug-related Serious Adverse Events (SAEs)
  - Patient 1 has completed immunosuppression regimen, per protocol
- *Demonstrated **consistent and sustained reduction in CSF levels of heparan sulfate,** a key biomarker of I2S activity*
- ***Early signs of neurocognitive stability observed***

Heparan sulfate (HS) change from baseline as measured from cerebral spinal fluid



[1] As announced December 18, 2019

RGX–111 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

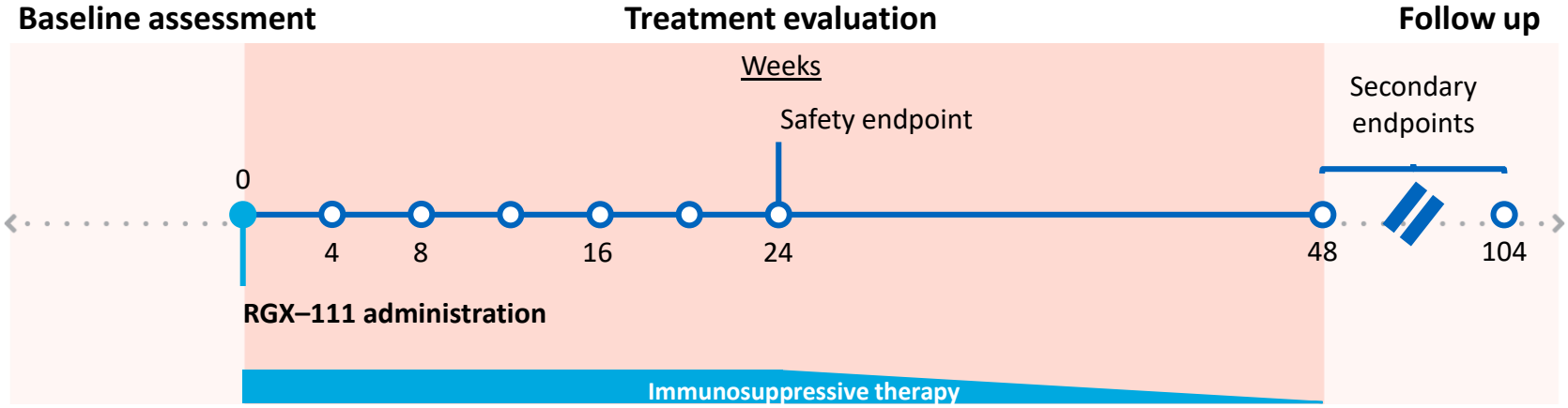
Sites: Leading U.S. and international lysosomal storage disease centers

Key inclusion criteria

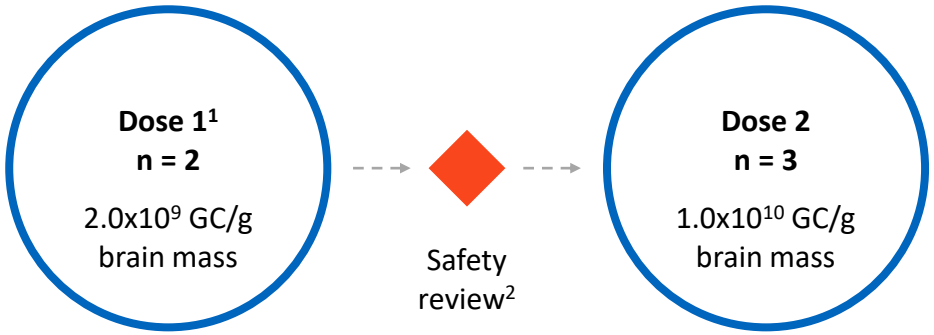
- Male or female
  - First subject ≥18 years of age
  - Subsequent subjects ≥6 years of age
- Documented evidence of early-stage neurocognitive deficit due to MPS I
  - A score of ≥1 standard deviation below mean on intelligent quotient testing or in one domain of neuropsychological function
  - A decline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

RGX–111 Phase I clinical trial: Administration and dose escalation

Administration and follow-up timeline



Expected dose escalation pathway



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[1] First subject to be ≥18 years of age
[2] 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

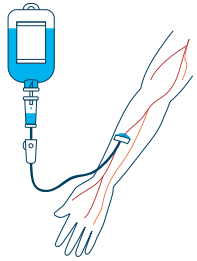
Correction of defective LDLR, reducing circulating LDL cholesterol

Special Regulatory Status

Orphan Drug Designation

Route of administration

Intravenous



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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

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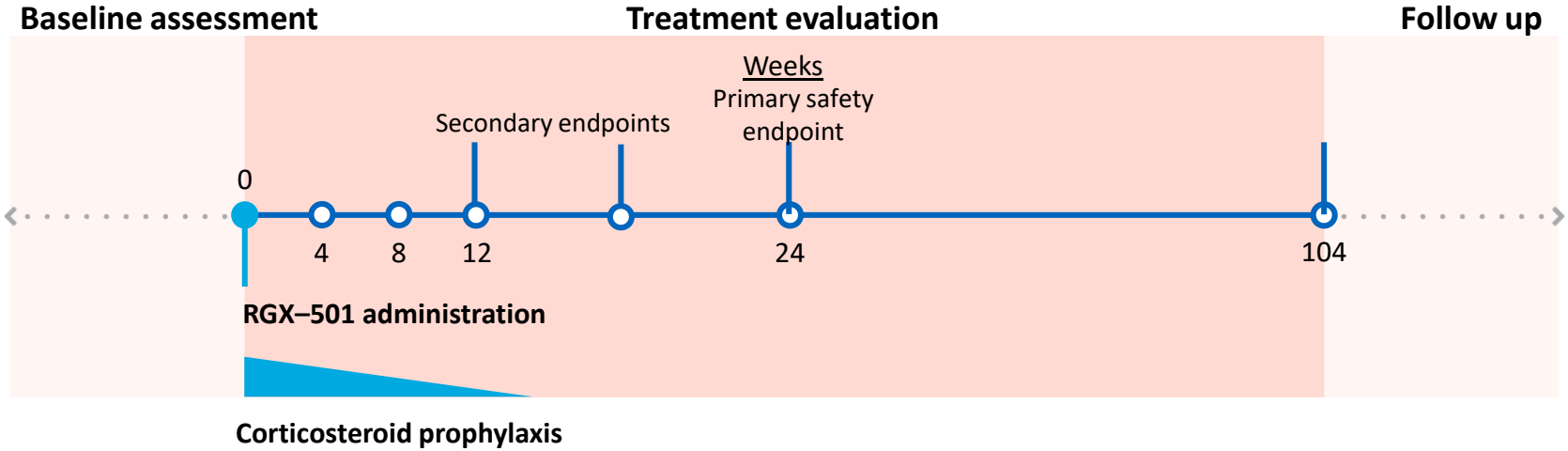
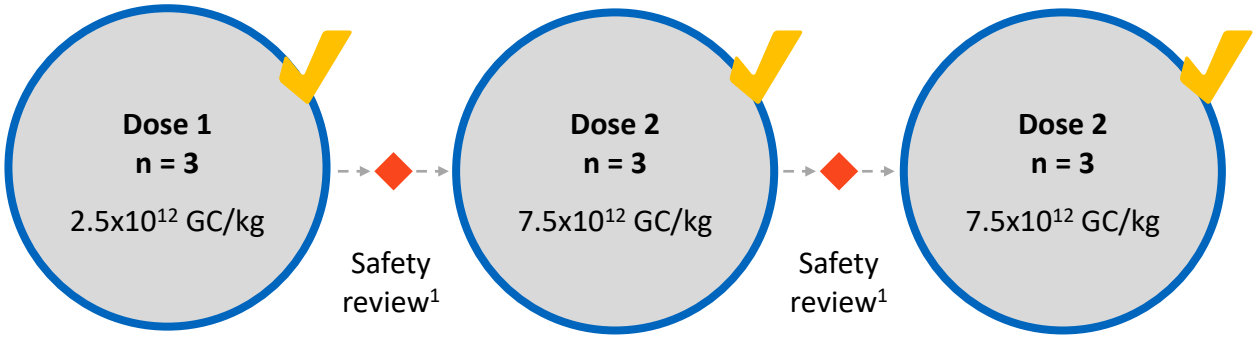
RGX–501 Phase I/II clinical trial: Study design

Administration and follow-up timeline



Expected dose escalation pathway



Dosing complete

[1] Dose escalation safety review to occur four weeks after final subject in each cohort has been dosed

NAV® Technology Platform

REGENXBIO®

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

- Broad and novel tissue selectivity
- Improved manufacturability
- Higher gene transfer
- Longer-term gene expression

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

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	ultragenyx	Hemophilia A	Takeda		
					Hemophilia A	ultragenyx / Bayer		
					OTC Deficiency	ultragenyx		
					GSDIa	ultragenyx		
					Crigler-Najjar	AUDENTES		
Central nervous system	CDKL5 Deficiency	ultragenyx	Rett Syndrome	NOVARTIS	SMA Type II / III	NOVARTIS	SMA Type I	NOVARTIS
	Undisclosed	Prevail	ALS SOD1	NOVARTIS	Parkinson's w/ GBA & Neuronopathic Gaucher	Prevail	MPS IIIA	LYSOGENE / SAREPTA
			CLN3	Abeona	MPS IIIA	Abeona		
			Friedreich's ataxia	Pfizer	MPS IIIA	ESTEVE		
			FTD-GRN	Prevail	MPS IIIB	Abeona		
			Synucleinopathies (GBA + α-Syn RNAi)	Prevail	CLN1	Abeona		
Cardiac / skeletal muscle			Pompe Disease	AUDENTES	CPVT	AUDENTES	XLMTM	AUDENTES
					Danon Disease	rocket pharma		

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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

REGENXBIO | Industry leader in AAV production and manufacturing

Deep in-house knowledge of vector characterization and strength in technical operations

3,000 ft² in-house GLP pilot plant with 3 X 200L bioreactor capacity

18,000 ft² of fully-operational advanced manufacturing and analytics lab space

30+ batches of cGMP bulk drug substance product covering multiple programs

Flexible, large-scale cGMP capacity

Candidate selection to clinical material in 12 months

Robust suspension cell culture-based production

Integrated process optimization to enable scale and quality

Analytical capabilities to ensure quality for patients

Key highlights of REGENXBIO's new headquarters

- Corporate, research and manufacturing headquarters to be ready in late 2020
- cGMP manufacturing facility expected to be operational in 2021; will enable production at bioreactor scales up to 2,000L
- Integrated approach will allow for more efficient development and manufacturing of product candidates

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Team and Conclusion

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The REGENXBIO team

Name	Position	Prior Affiliations
Ken Mills	President, CEO & Co-Founder; Director	FOXKISER, MSD
Olivier Danos, Ph.D.	SVP and Chief Scientific Officer	Biogen, cnrs
Vit Vasista	SVP and Chief Financial Officer	PRTM, MSD
Steve Pakola, M.D.	SVP and Chief Medical Officer	aerpio, amakem THERAPEUTICS
Curran Simpson	SVP, Product Development and Chief Technology Officer	gsk, Human Genome Sciences
Ram Palanki, Pharm.D.	SVP, Commercial Strategy and Operations	Santen, Genentech A Member of the Roche Group
Patrick Christmas, J.D.	SVP and General Counsel	Lumara Health™, WELLSTAT THERAPEUTICS
Laura Coruzzi, Ph.D., J.D.	SVP, Intellectual Property	JONES DAY
Shiva Fritsch	SVP, Human Resources	NOVAVAX, Human Genome Sciences

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	**Subretinal wet AMD:** Initiation of pivotal program in 2H 2020 **Suprachoroidal wet AMD:** Initiation of Phase II trial in 1H 2020 **Suprachoroidal DR:** IND submission in 1H 2020
RGX–121	**Additional data from Cohort 1 in early 2020** **Interim data from Cohort 2 in mid-2020**
RGX–501	**Interim data update in 1H 2020**
RGX–111	**Program update in 2H 2020**
RGX–181	**Program update in mid-2020** **IND submission in 2H 2020**

2020 financial guidance:

*As of December 31, 2019, REGENXBIO had **$400 million in cash**[1]. REGENXBIO expects these resources to fund completion of internal manufacturing capabilities and clinical advancement of its product candidates into 2022.*

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[1] Cash includes cash, cash equivalents and marketable securities for the purposes of this presentation

Thank You

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