Corporate Presentation

Leader in AAV Gene Therapy

REGENXBIO®

June 1, 2019

Forward-looking statements

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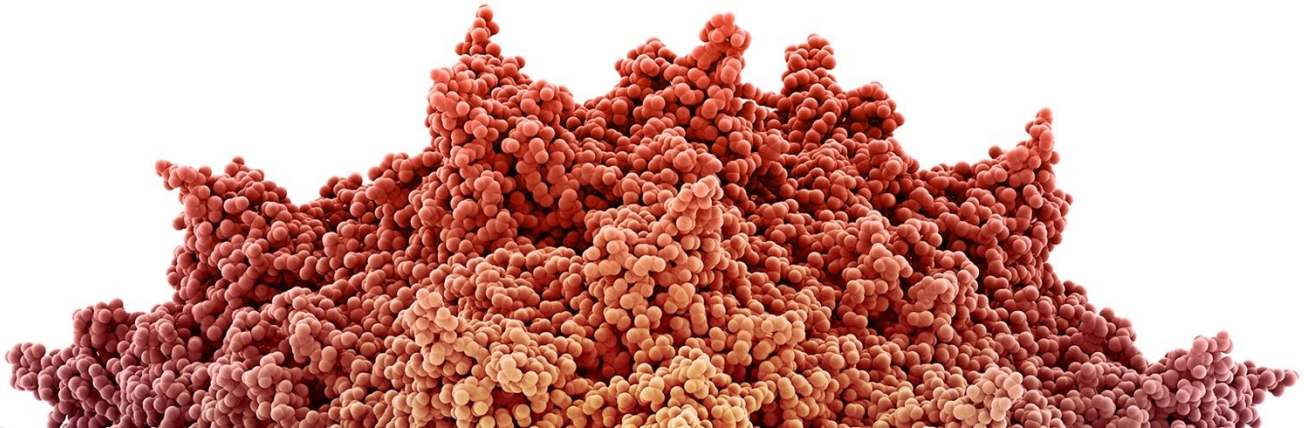
REGENXBIO: seeking to improve lives through the curative potential of gene therapy

4 clinical stage programs
with next data readout for RGX–314 expected in late 2019

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

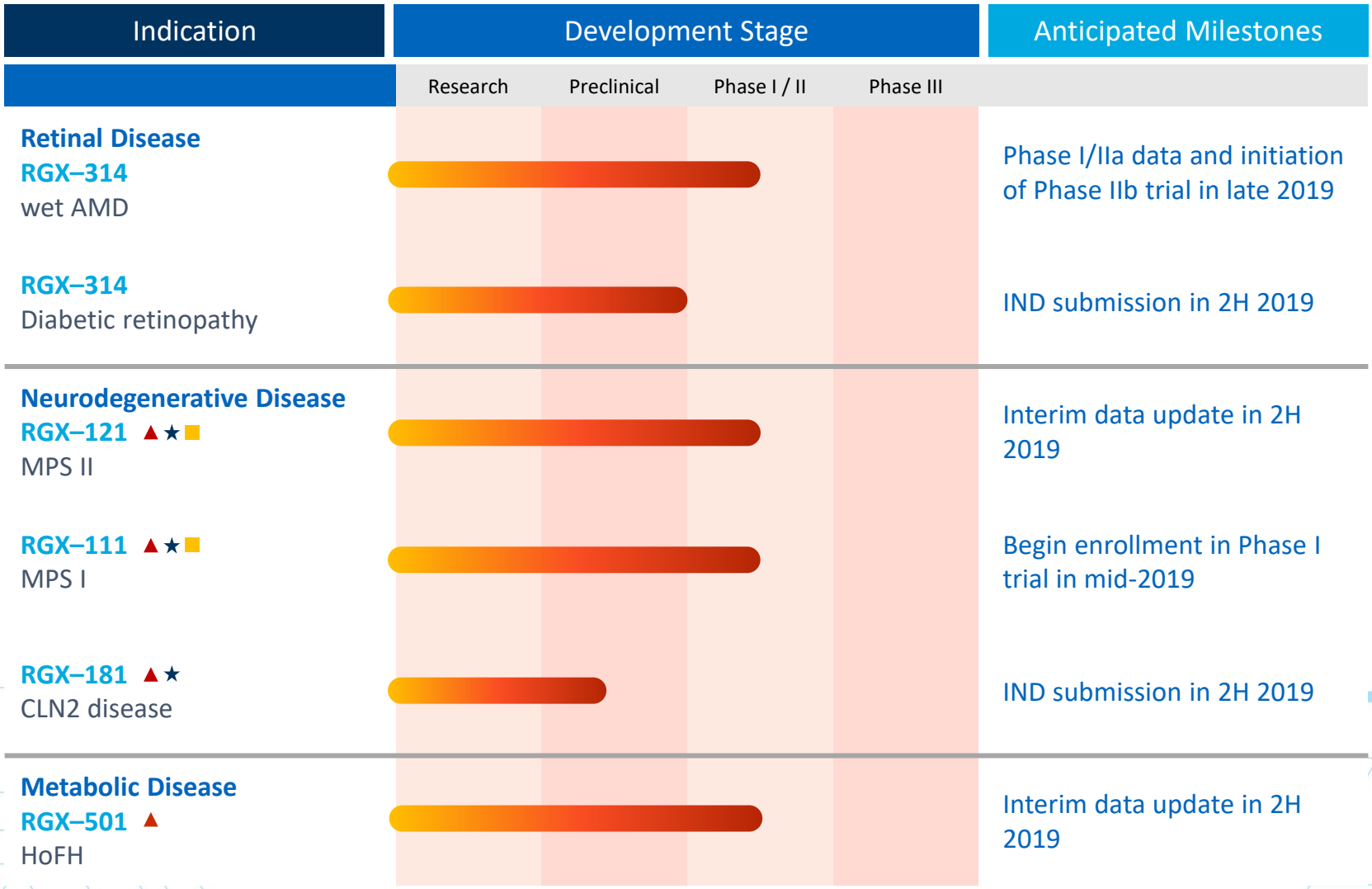
Proprietary **NAV® Technology Platform**
includes exclusive ***worldwide rights to over 100 AAV vectors***, including **AAV7, AAV8, AAV9** and **AAVrh10**

Management team are experienced drug developers and leaders in gene therapy



REGENXBIO's lead programs

Internally developed product candidates



REGENXBIO'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	PKU	ultragenyx	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	MPS IIIA	Abeona	MPS IIIA	LYSOGENE / SAREPTA
			CLN3	Abeona	MPS IIIA	ESTEVE		
			Parkinson's w/ GBA & Neuronopathic Gaucher	Prevail	MPS IIIB	Abeona		
			FTD-GRN	Prevail	CLN1	Abeona		
			Synucleinopathies (GBA + α-Syn RNAi)	Prevail				
Cardiac / skeletal muscle			Pompe Disease	AUDENTES	XLMTM	AUDENTES		
					CPVT	AUDENTES		
					Danon Disease	rocket pharma		

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

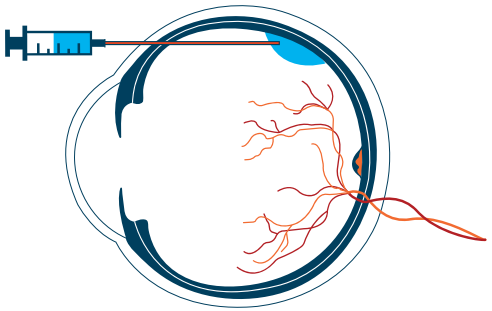
Vector: AAV8 | **Gene:** anti-VEGF Fab

Mechanism of action

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

Route of administration

Subretinal



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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: Up to 42 total

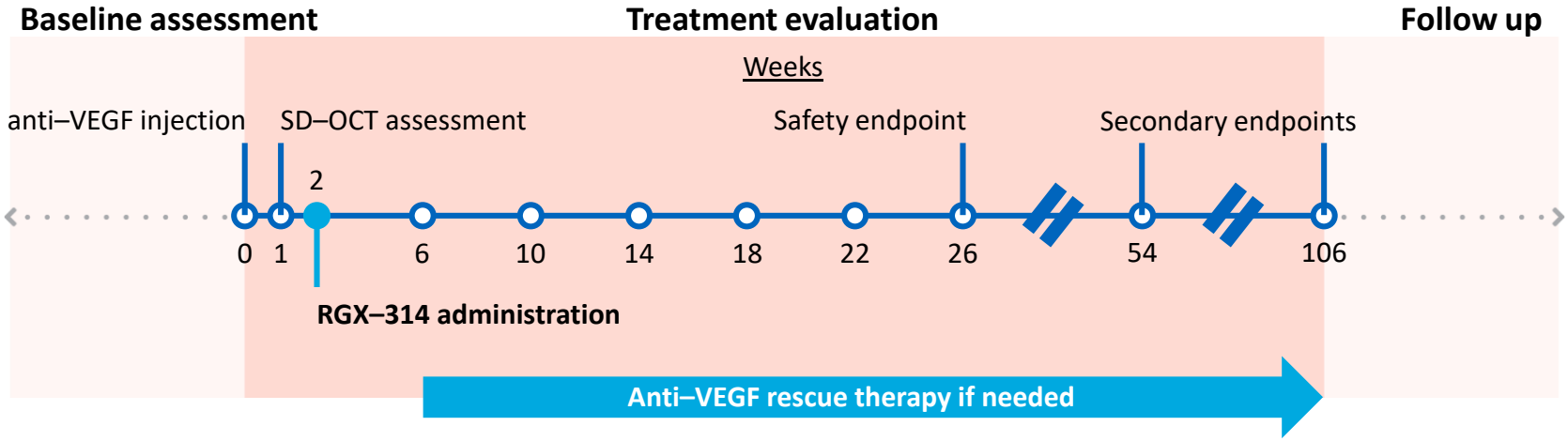
Sites: Eight leading retinal surgery centers across the United States

KEY INCLUSION CRITERIA

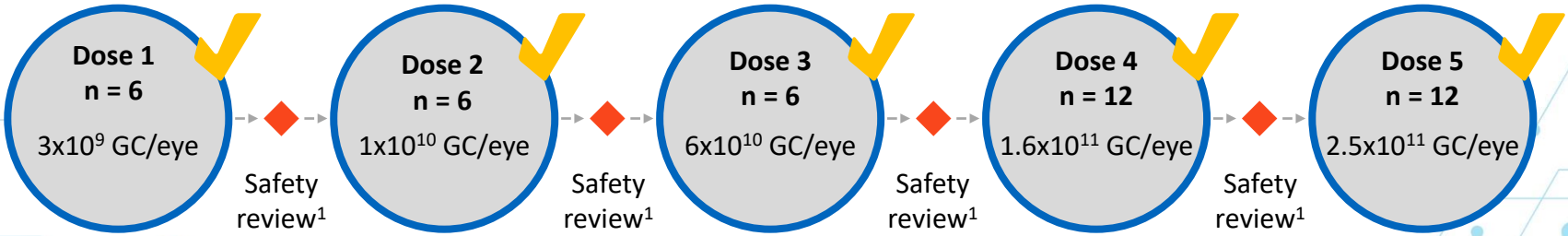
- Male or female ≥ 50 to 89 years of age
- Previously treated wet AMD subjects requiring ≥4 anti–VEGF injections in the 8 months prior to trial entry
- Documented response to anti–VEGF at trial entry (assessed by SD–OCT at week 1)
- Vision of 20/63 to 20/400 for the initial subject, then 20/40 to 20/400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



Anticipated dose escalation pathway



42 total subjects dosed across five cohorts

[1] 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 summary[1]

- *RGX–314 was **well–tolerated** (n=33)[2]*
- ***No drug-related AEs or drug-related SAEs***
- *Most AEs were assessed as mild (Grade 1 – 82%)*
- ***No observed clinically determined immune responses**, drug-related ocular inflammation, or any post-surgical inflammation beyond what is expected following routine vitrectomy*
- ***Eight SAEs that were not drug-related were reported in five subjects***

[1] As of April 18, 2019
[2] Includes 30 subjects in Cohorts 1 – 4 and 3 subjects from Cohort 5

RGX–314 clinical trial summary through one year

	Aqueous RGX–314 protein one month post–treatment	Aqueous RGX–314 protein one year post–treatment	Mean # of anti–VEGF injections through one year	Mean change in CRT one year post-treatment	Mean change in BCVA one year post-treatment
Cohort 1 $3x10^9$ GC/eye (n=6)	2.5 ng/ml[1]	2.2 ng/ml[2]	10.5 inj[3]	+33 µm[2]	-7 letters[2]
Cohort 2 $1x10^{10}$ GC/eye (n=6)	12.8 ng/ml	45.8 ng/ml	8.8 inj	+56 µm	+2 letters
Cohort 3 $6x10^{10}$ GC/eye (n=6)	**160.2 ng/ml**	**180.8 ng/ml**	**2.3 inj**	**-39 µm**	**+5 letters**

[1] N=5; one subject in Cohort 1 did not have aqueous sample taken at Week 6
[2] N=5; one subject in Cohort 1 discontinued from the study at four months
[3] One subject in Cohort 1 discontinued from the study at four months with four injections and was imputed as requiring thirteen injections through one year (or 52 weeks)

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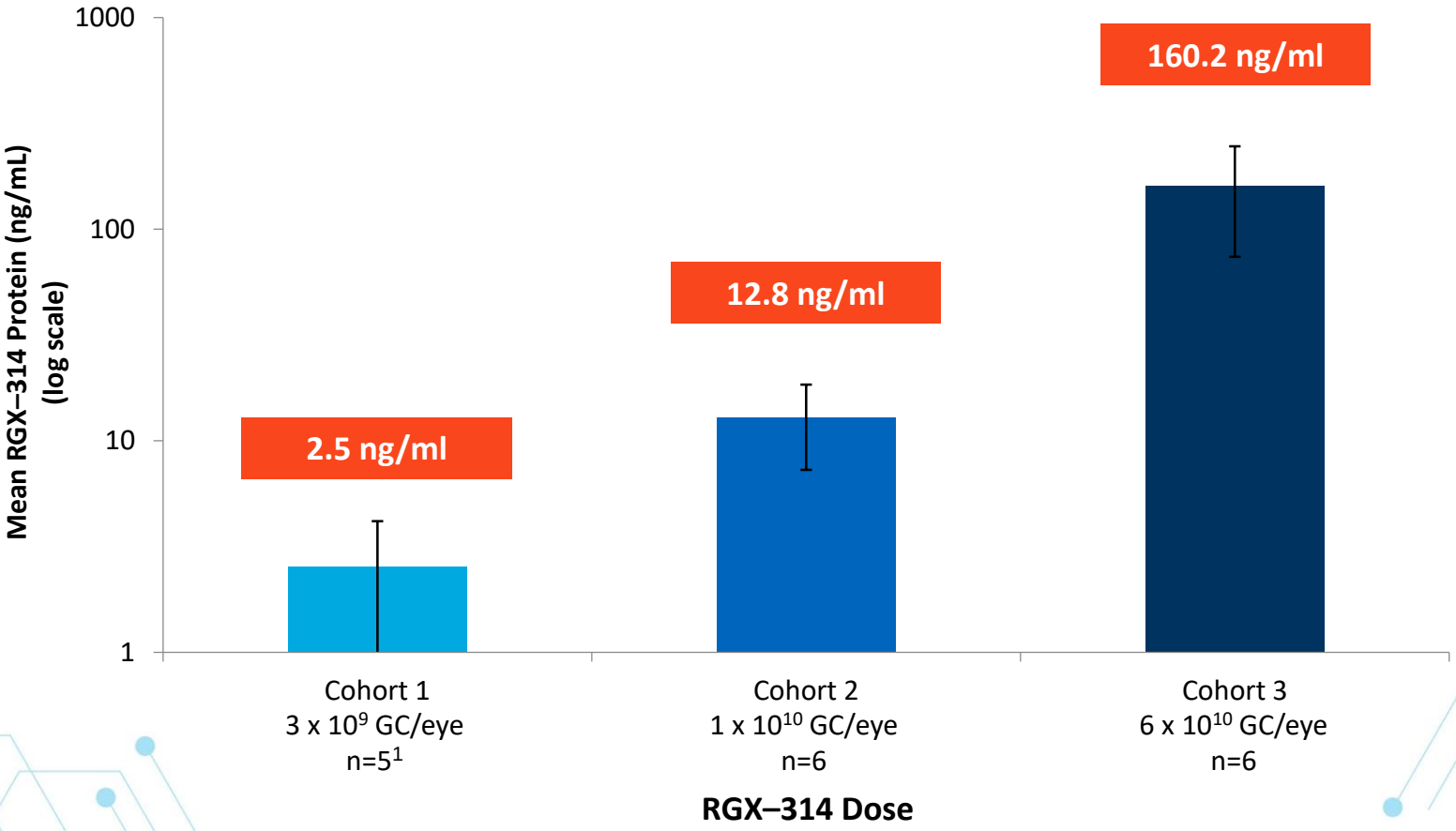
RGX–314 protein levels at one month

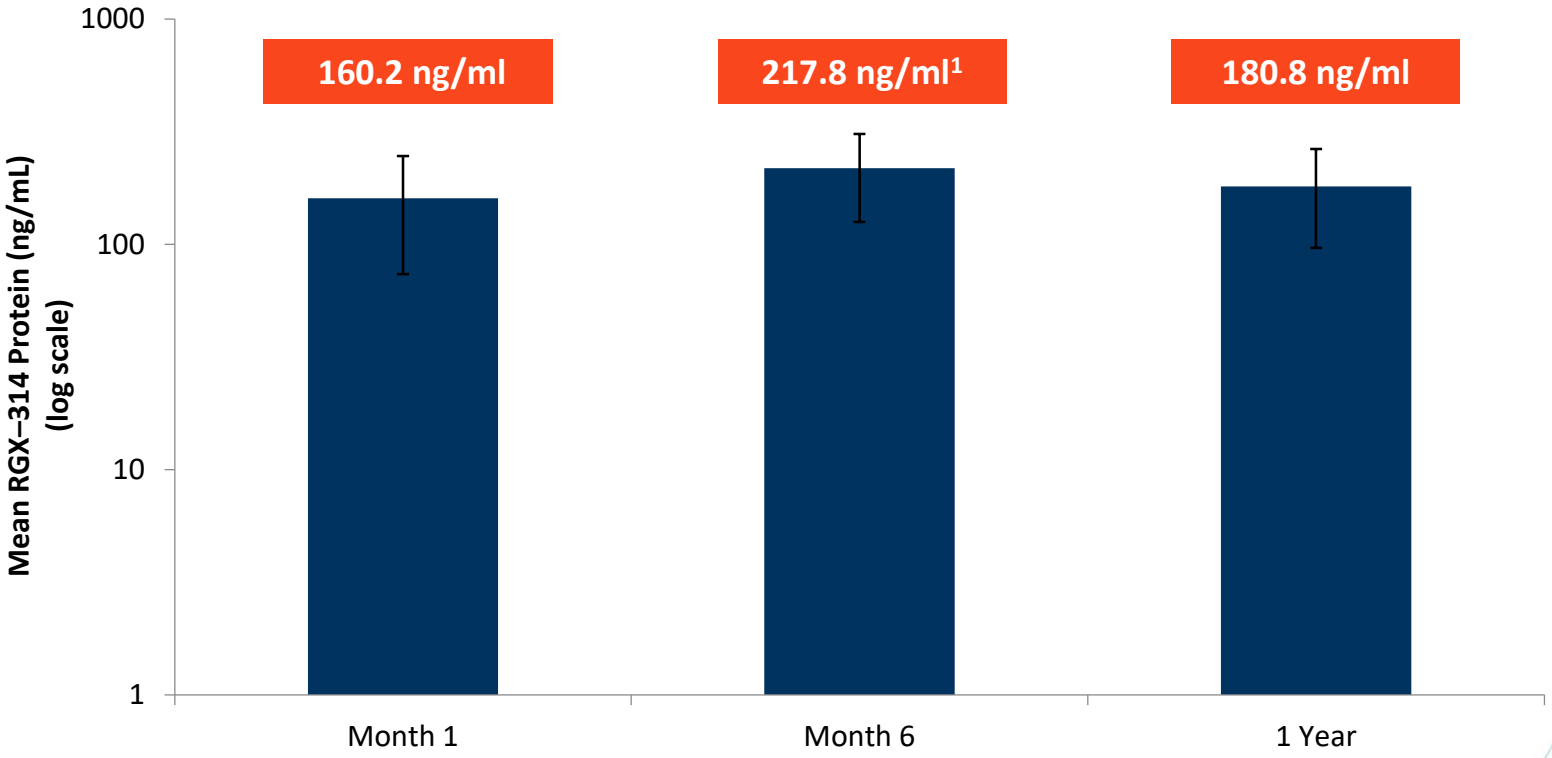
As measured from aqueous samples by ECL



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

RGX–314 Phase I/IIa trial: Sustained protein levels at one year

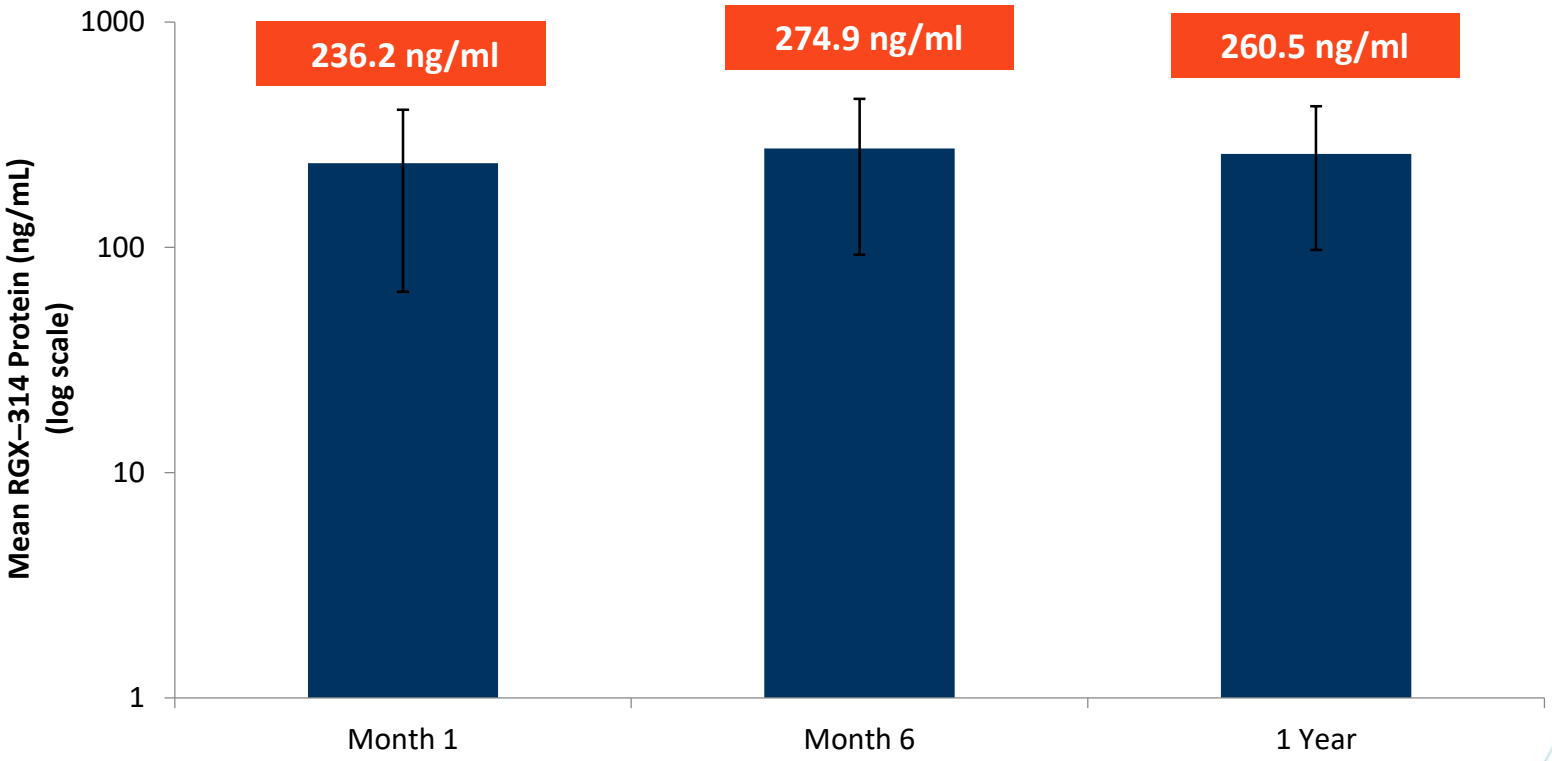
All subjects (n=6) in Cohort 3 (6 x 10^{10} GC/eye)



[1] One subject received an anti-VEGF rescue injection 1 month prior to sample

RGX–314 Phase I/IIa trial: Sustained protein levels at one year

Subjects with No Rescue Injections (n=3) in Cohort 3 (6 x 10^{10} GC/eye)

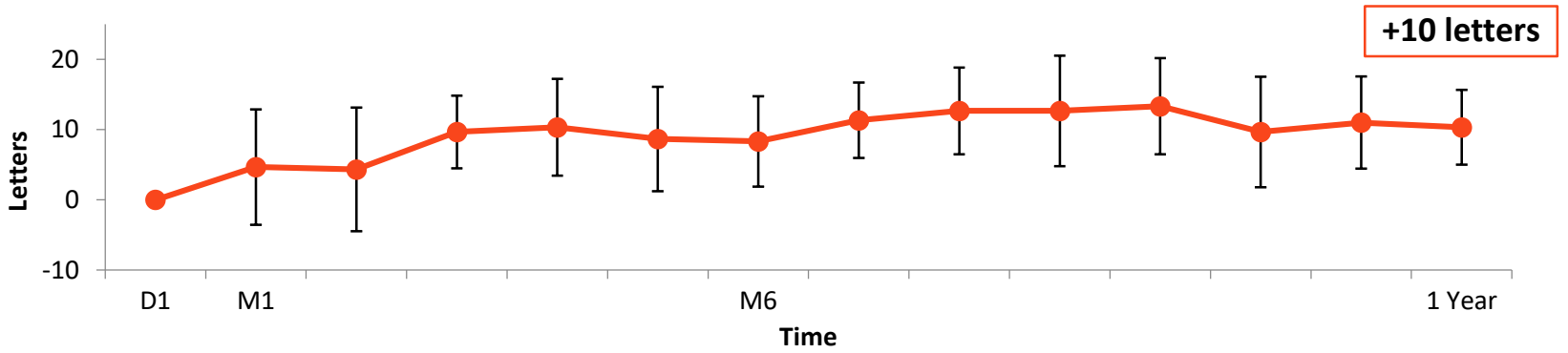


Cohort 3: Three subjects with no additional anti–VEGF injections through one year

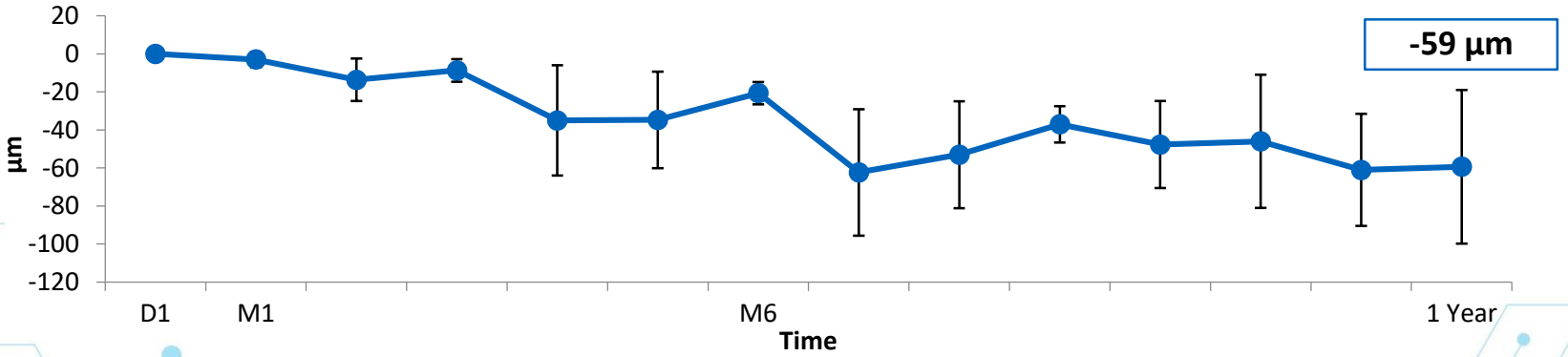
Previous therapy	▪ Study subjects received on average **>35 injections since wet AMD diagnosis**
Post-RGX–314 anti–VEGF injections	▪ **0 injections** through one year post-RGX–314
BCVA	▪ Mean **gain in BCVA of +10 ETDRS** letters from baseline through one year
SD–OCT	▪ Maintained with a **mean change in CRT of -59 µm** from baseline through one year

RGX–314 Phase I clinical trial – mean change in BCVA, CRT over one year in Cohort 3 subjects with no rescue injections (n=3)

Best Corrected Visual Acuity (BCVA)



Central Retinal Thickness (CRT) on SD-OCT



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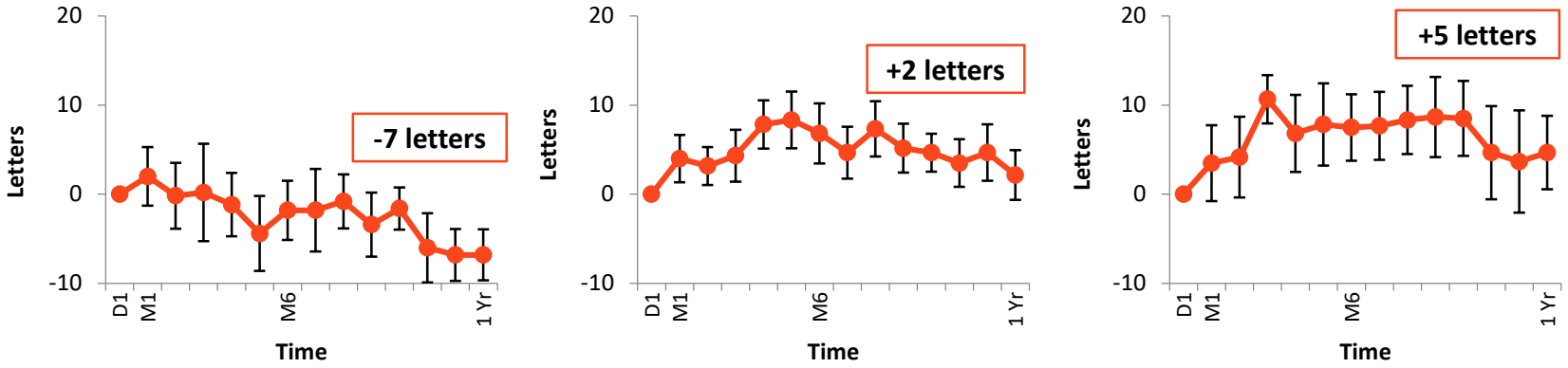
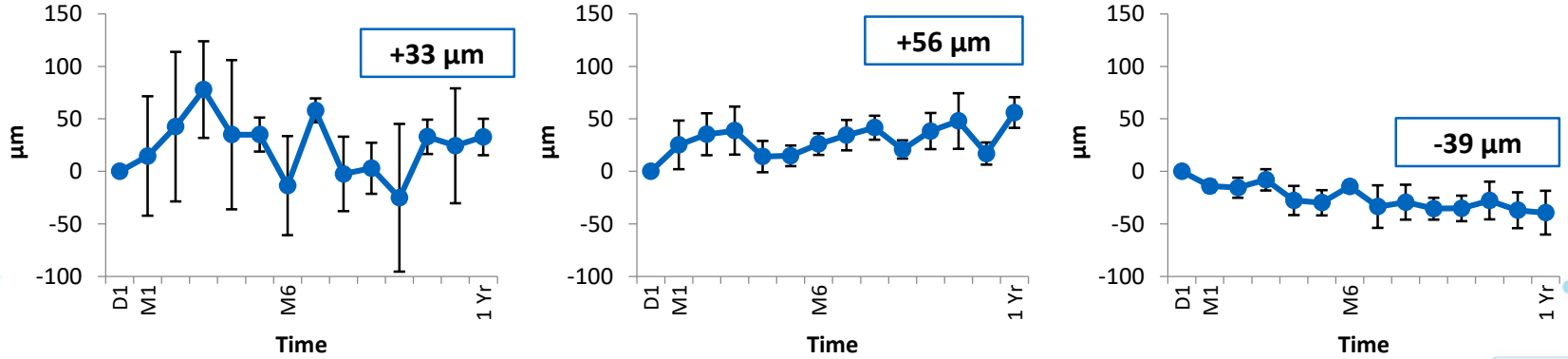
RGX–314 Phase I/IIa trial: Mean change in BCVA, CRT and average injections over one year

Best Corrected Visual Acuity (BCVA)



Central Retinal Thickness (CRT) on SD-OCT



Average Injections: 10.5	Average Injections: 8.8	Average Injections: 2.3
Cohort 1[1]	Cohort 2	Cohort 3

[1] One subject in Cohort 1 did not have aqueous sample taken at week 6; one subject in Cohort 1 discontinued from the study at four months with four injections and was imputed as requiring thirteen injections through one year.

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 and proliferative DR with or without macular edema
- Treatment options include anti-VEGF injections or panretinal laser treatment
- Approx. 8 million patients estimated in United States

RGX–314 PRODUCT CANDIDATE

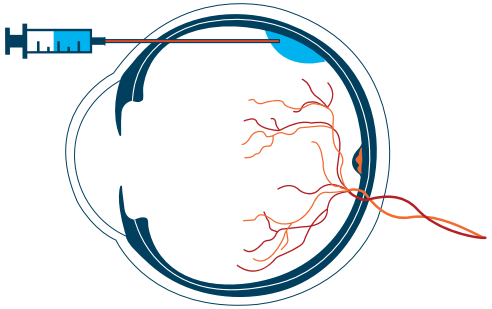
Vector: AAV8 | **Gene:** anti-VEGF Fab

Mechanism of action

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

Route of administration

Subretinal



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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

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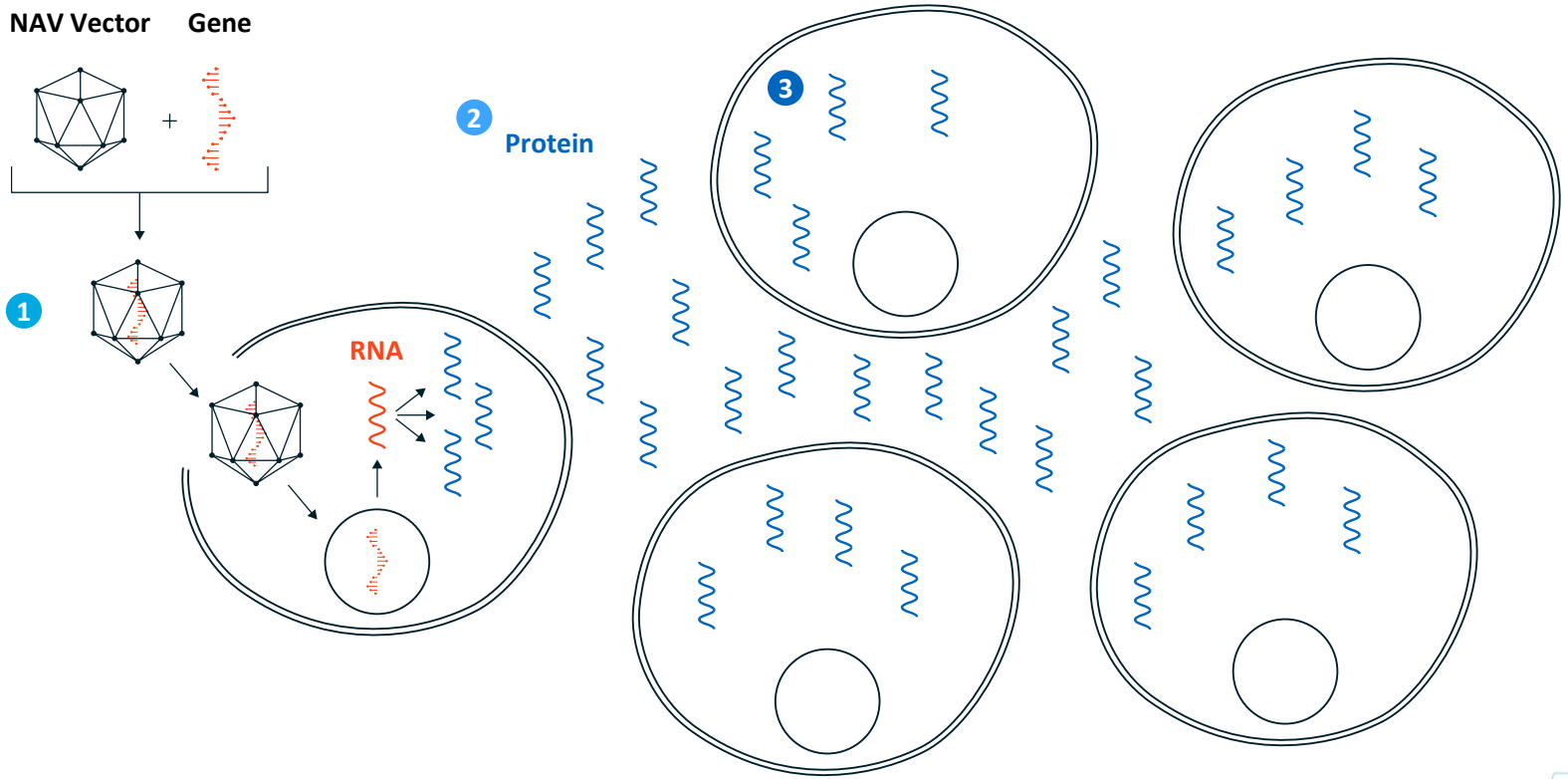
Cross–correction is a **key treatment advantage** in MPS and CLN2 disease

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

2 ***Protein secreted by transduced cells***

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



A single transduced cell has potential to correct many other cells

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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

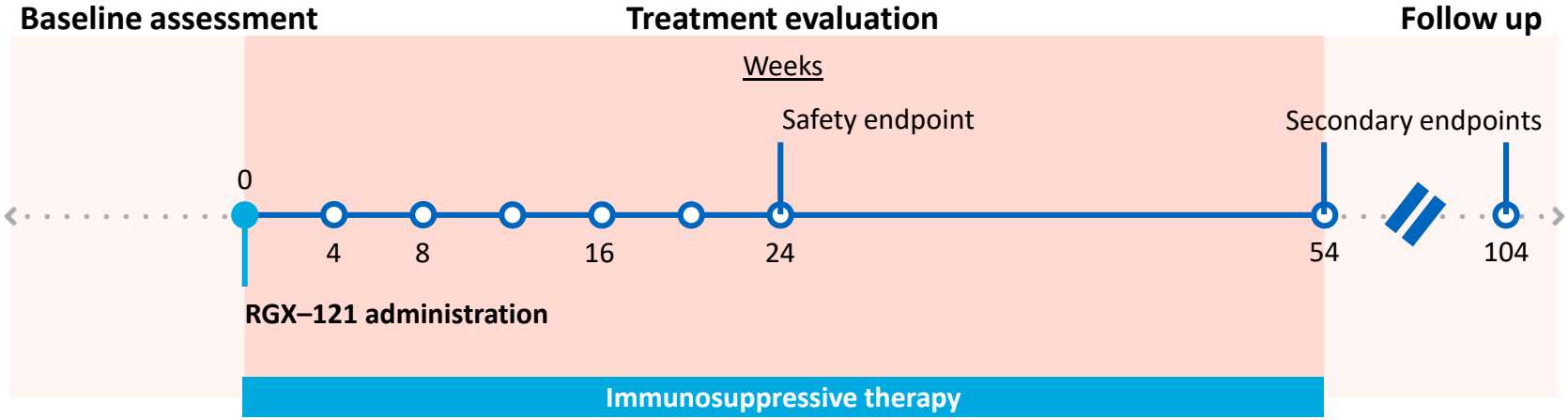
Sites: Leading U.S. and international lysosomal storage disease centers

Key inclusion criteria

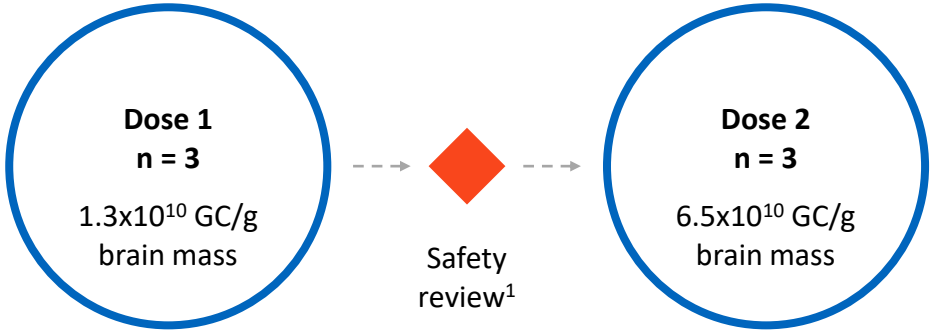
- Male subjects ≥4 months to <5 years of age
- Meeting one of the following criteria:
  - Diagnosis of MPS II and a score >55 and ≤77 on intelligent quotient testing OR
  - Diagnosis of MPS II and a score >55 and a decline of ≥1 standard deviation on intelligent quotient testing OR
  - Having a relative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



One subject dosed in the first cohort

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

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

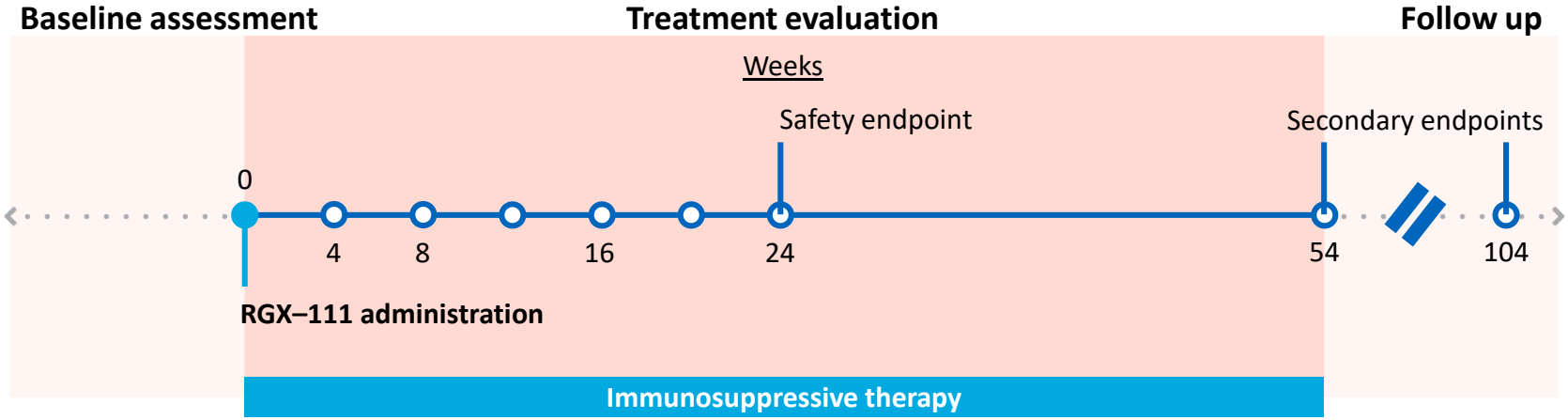
Sites: Leading U.S. and international lysosomal storage disease centers

Key inclusion criteria

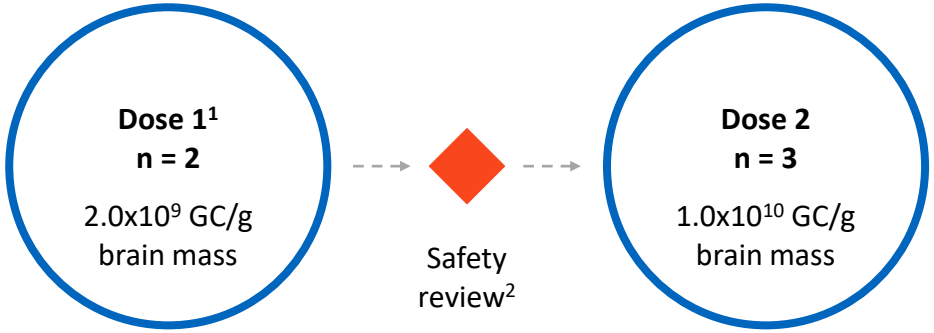
- Male or female
  - First subject ≥18 years of age
  - Subsequent subjects ≥6 years of age
- Documented evidence of early-stage neurocognitive deficit due to MPS I
  - A score of ≥1 standard deviation below mean on intelligent quotient testing or in one domain of neuropsychological function
  - A decline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



[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
- Approx. 11,000 patients worldwide

RGX–501 PRODUCT CANDIDATE

Vector: AAV8 | **Gene:** LDLR

Mechanism of action

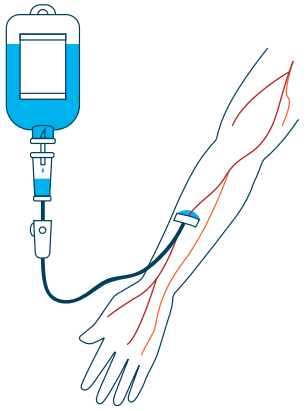
Correction of defective LDLR, reducing circulating LDL cholesterol

Route of administration

Intravenous

Special Regulatory Status

Orphan Drug Designation



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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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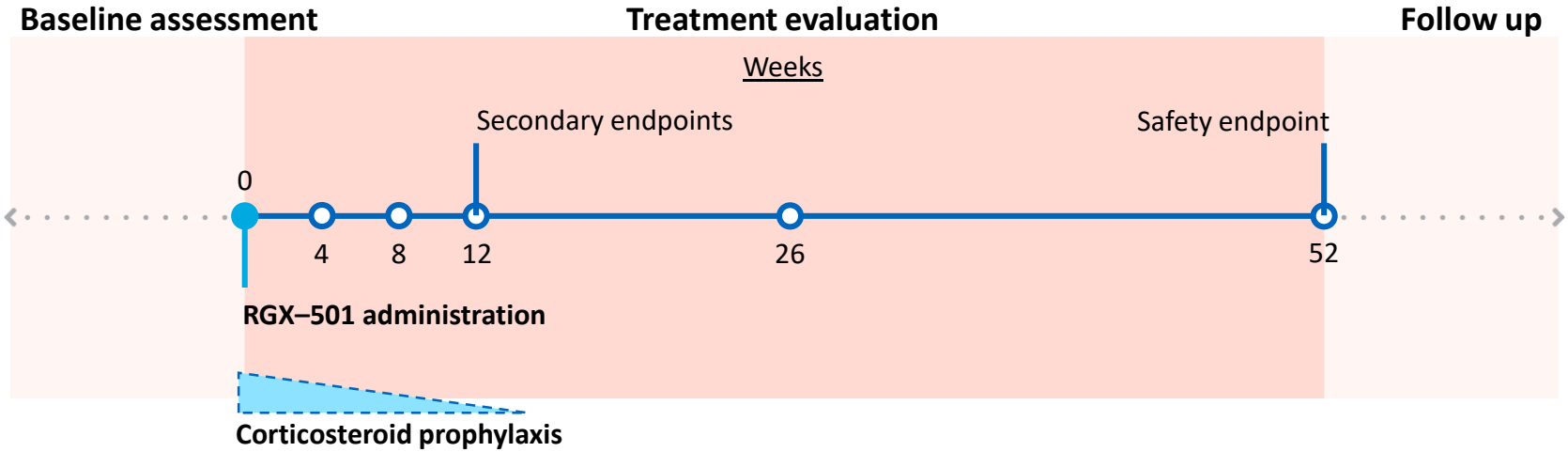
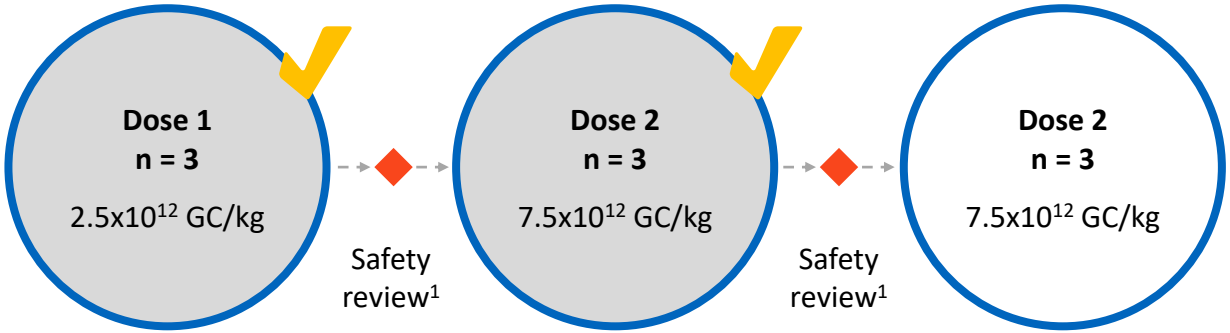
RGX–501 Phase I/II clinical trial – study design

Administration and follow-up timeline



Expected dose escalation pathway



Three subjects dosed in each of the first and second cohorts

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

RGX–501 Phase I/II clinical trial interim results and program update

Summary

- *Transaminase elevations observed in Cohort 2*
- *Administration of steroid appears to mitigate transaminase elevations and related effects*
- *Clinical trial protocol has been amended to allow for the enrollment of additional subjects using steroid prophylaxis*
- *U.S. IND application transferred to REGENXBIO from University of Pennsylvania in November 2018; transfer of the Clinical Trial Applications for all other participating countries is ongoing*

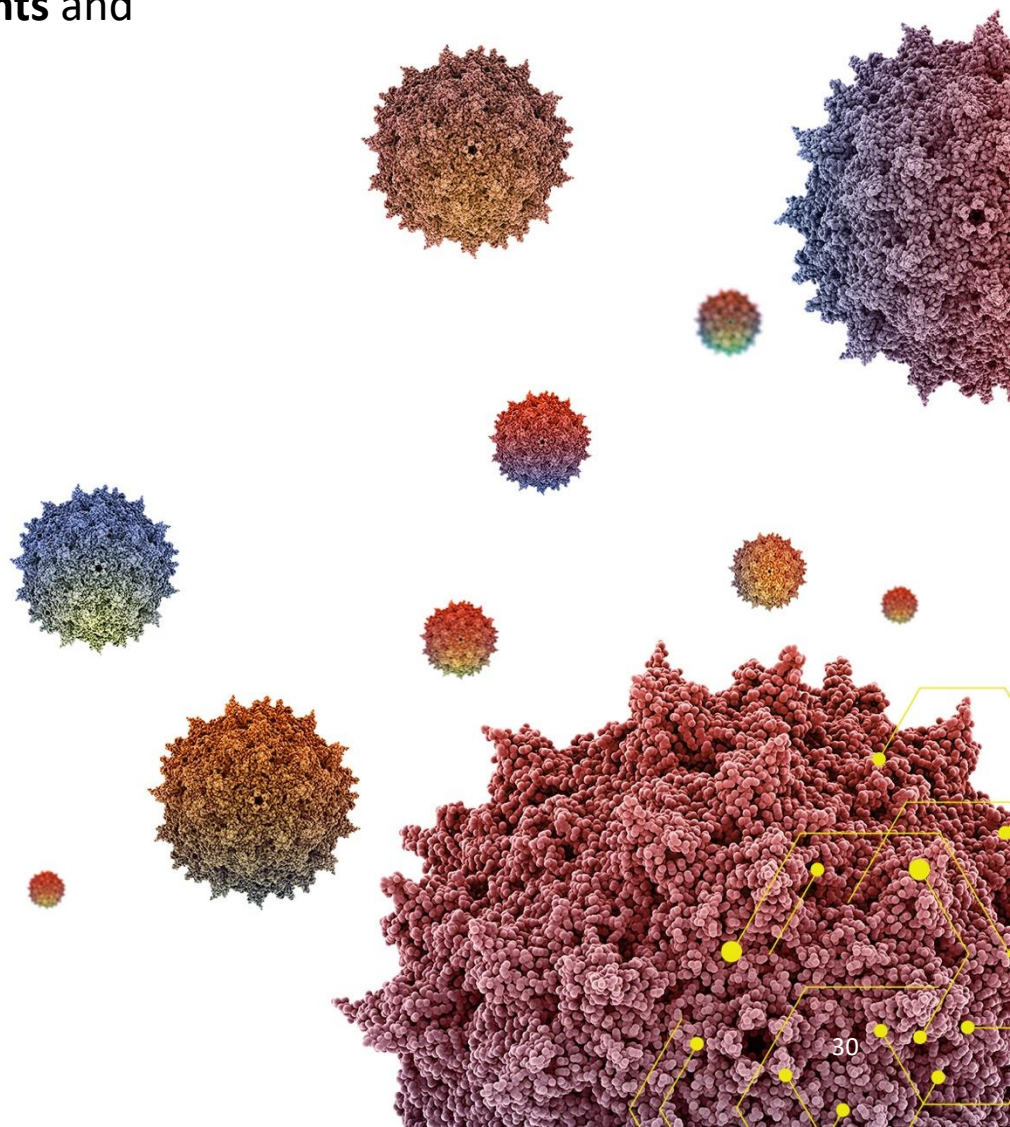
NAV® Technology Platform

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**

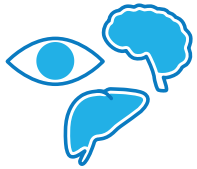
- AAV7, AAV8, AAV9, AAVrh10
- Over 100 other novel AAV sequences
- Sequences that are at least 95% identical to these capsids



Key features of **REGENXBIO's NAV Technology Platform**

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

nature biotechnology

Intravascular AAV9 Preferentially Targets Neonatal Neurons and Adult Astrocytes

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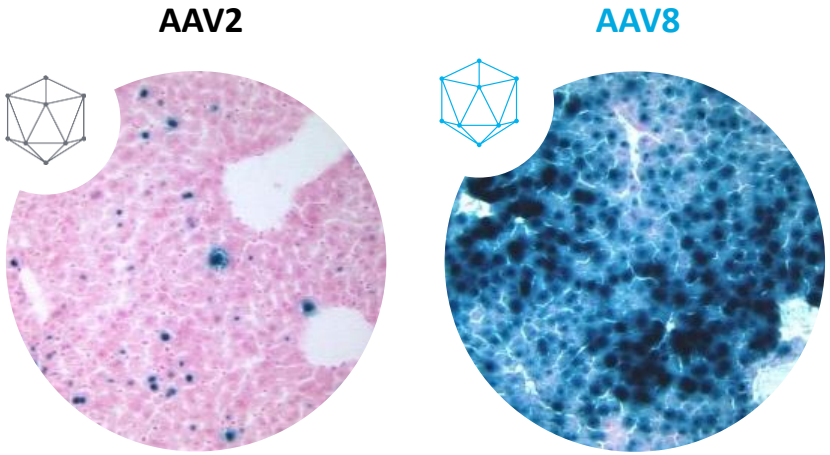
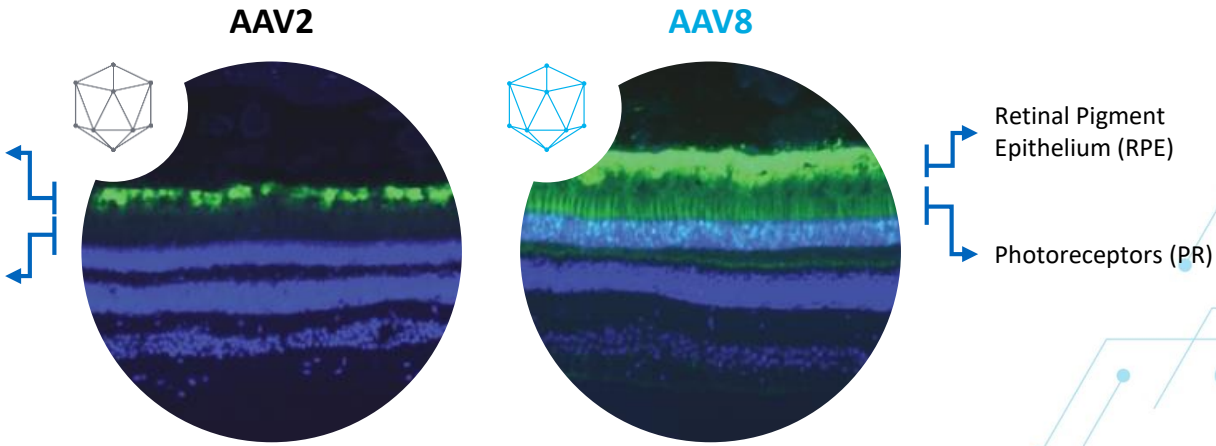
NAV Vectors: higher gene expression than early generation AAV vectors

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



NAV Vector AAV8: **More efficient gene delivery** to sites of most retinal dystrophies[1]



[1] Vandenberghe et al. 2011 *Science Translational Medicine*

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	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

Q1 2019 financials (mm)

R&D expense:	$25
G&A expense:	$12
Net loss:	$32
Basic share count (3/31/19):	36.6

Financial highlights

Ended Q1 2019 with **$444 million in cash**[1]
Pending approval by regulatory authorities and product launch, will begin recognizing **royalty revenue from commercial sales of Novartis' Zolgensma**

Program guidance and anticipated milestones

RGX–314	**wet AMD:** Phase I/IIa data and initiation of Phase IIb trial by end of 2019 **Diabetic retinopathy:** IND submission in 2H 2019
RGX–121	Interim data update in 2H 2019
RGX–111	IND active and subject recruiting ongoing; interim data update in 2H 2019
RGX–501	Interim data update in 2H 2019
RGX–181	IND submission in 2H 2019

2019 financial guidance:

*Expect 2019 ending cash balance to be between **$330 million and $350 million**, excluding any potential royalty revenue from commercial sales of Novartis' ZOLGENSMA for the treatment of SMA Type I*

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

Thank You

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