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, which will be filed with the U.S. Securities and Exchange Commission (SEC) in the first quarter of 2019, and comparable "risk factors" sections of REGENXBIO's Quarterly Reports on Form 10-Q and other filings, which have been filed with the SEC and are available on the SEC's website at 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**
with next data readout for RGX–314
expected in late 2019

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**
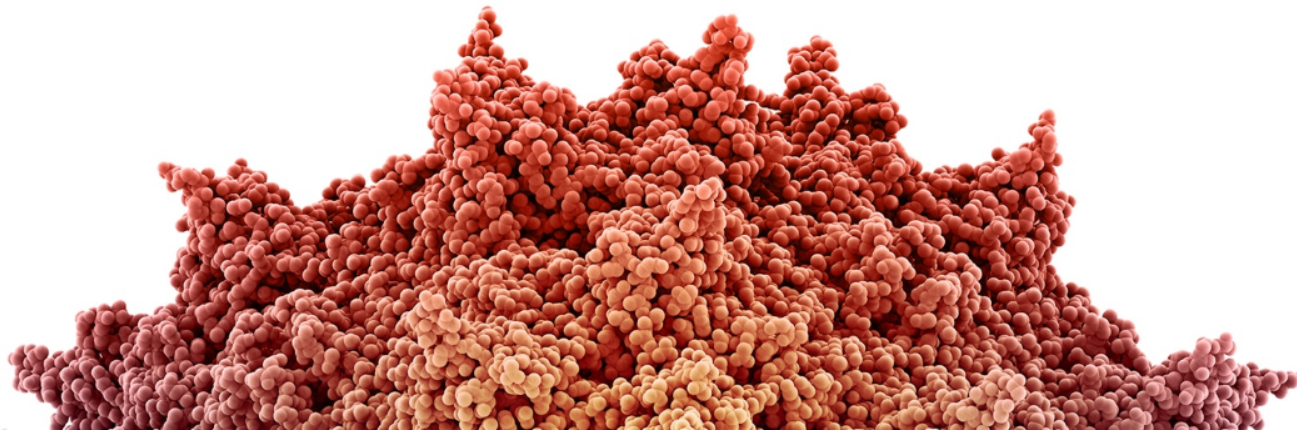
REGENXBIO's lead programs

Internally developed product candidates

Indication	Development Stage				Anticipated Milestones
	Research	Preclinical	Phase I / II	Phase III	
Retinal Disease **RGX–314** wet AMD					Phase I/IIa data and initiation of Phase IIb trial in late 2019
RGX–314 Undisclosed indication					IND submission in 2H 2019
Neurodegenerative Disease **RGX–121** ▲★■ MPS II					Interim data update in 2H 2019
RGX–111 ▲★■ MPS I					Begin enrollment in Phase I trial in mid-2019
RGX–181 ▲★ CLN2 disease					IND submission in 2H 2019
Metabolic Disease **RGX–501** ▲ HoFH					Interim data update in 2H 2019

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

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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	
	Indication	Licensee	Indication	Licensee	Indication	Licensee	Indication	Licensee
Liver / hematologic	Citrullinemia Type I	ultragenyx			Hemophilia A	Takeda		
	PKU	ultragenyx			Hemophilia A	ultragenyx / Bayer		
	Wilson Disease	ultragenyx			OTC Deficiency	ultragenyx		
					GSDIa	ultragenyx		
					Crigler-Najjar	AUDENTES		
Retina	Achromatopsia	Biogen						
	Choroideremia	Biogen						
Central nervous system	Parkinson's w/ GBA	Prevail THERAPEUTICS	Rett Syndrome	NOVARTIS	SMA Type II / III	NOVARTIS	SMA Type I	NOVARTIS
	Undisclosed	Voyager genzyme	ALS SOD1	NOVARTIS	MPS IIIA	Abeona THERAPEUTICS		
	CDKL5 Deficiency	ultragenyx	ALS SOD1	Voyager THERAPEUTICS	MPS IIIA	LYSOGENE / SAREPTA THERAPEUTICS		
			CLN1	Abeona THERAPEUTICS	MPS IIIA	ESTEVE		
			CLN3	Abeona THERAPEUTICS	MPS IIIB	Abeona THERAPEUTICS		
Cardiac / skeletal muscle	Friedreich's Ataxia	Voyager genzyme	Pompe Disease	AUDENTES	XLMTM	AUDENTES		
					CPVT	AUDENTES		
					Danon Disease	rocket pharma		

Simultaneous global regulatory submissions filed in U.S., Europe and Japan

REGENXBIO®

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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 ocular cells the ability to produce an anti-VEGF fab

Route of administration

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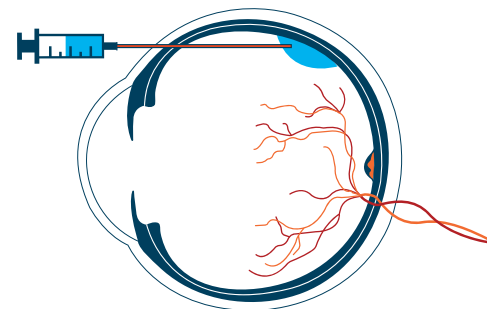
RGX–314 Phase I/IIa clinical trial in wet AMD

Objectives

Primary

- To determine the safety and tolerability of RGX–314 in subjects with wet AMD though 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: Seven leading retinal surgery centers across the United States

Key inclusion criteria

- Male or female ≥ 50 to 89 years of age

- 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

Baseline assessment | **Treatment evaluation** | **Follow up**

Weeks

anti–VEGF injection   SD–OCT assessment   Safety endpoint   Secondary endpoints

2

0 1   6   10   14   18   22   26   54   106

RGX–314 administration

Anti–VEGF rescue therapy if needed
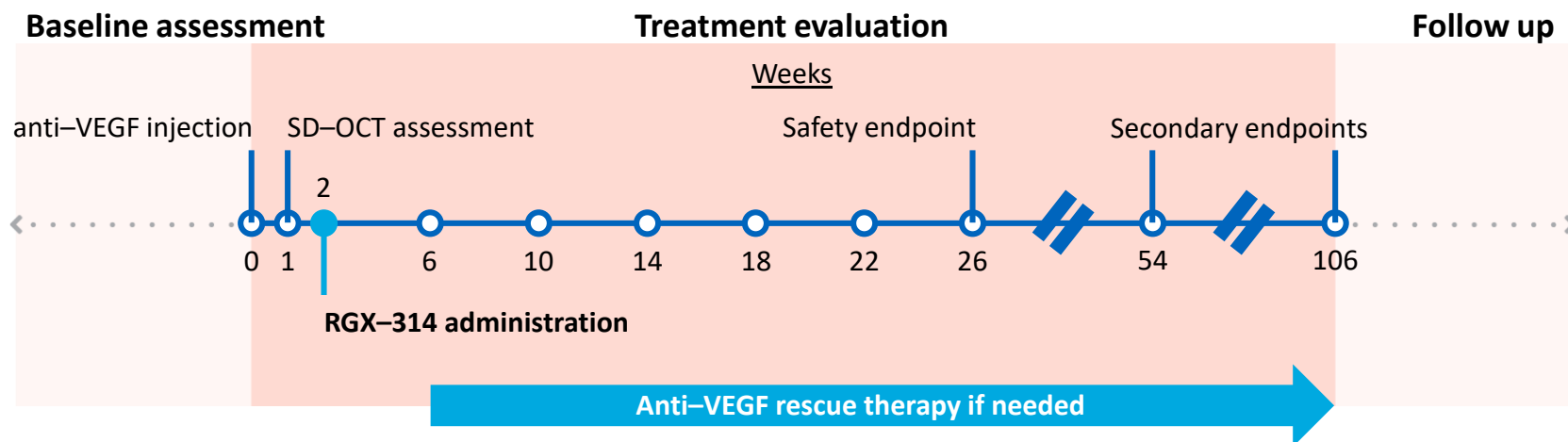
Anticipated dose escalation pathway

Dose 1
n = 6
3×10^9 GC/eye

Safety review[1]

Dose 2
n = 6
1×10^{10} GC/eye

Safety review[1]

Dose 3
n = 6
6×10^{10} GC/eye

Safety review[1]

Dose 4
n = 12
1.6×10^{11} GC/eye

Safety review[1]

Dose 5
n = 12
2.5×10^{11} GC/eye
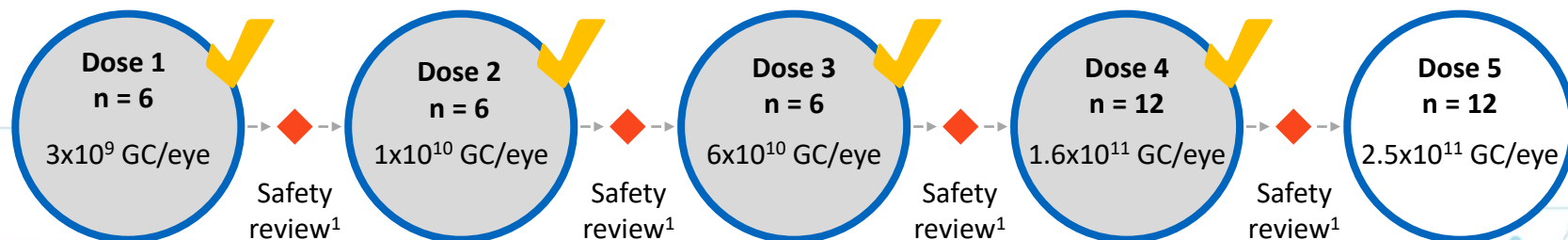
30 total subjects dosed across four cohorts

RGX–314 Phase I/IIa clinical trial – safety summary[1]

- *RGX–314 was **well–tolerated** (n=24)*

- ***No drug-related AEs or drug-related SAEs***

- *Most AEs were assessed as mild (Grade 1 – 83%)*

- ***No observed clinically determined immune responses**, drug-related ocular inflammation, or any post-surgical inflammation beyond what is expected following routine vitrectomy*

- ***Six SAEs that were not drug-related were reported in four subjects***

RGX–314 clinical trial summary through six months

	Aqueous RGX–314 protein one month post–treatment	Mean # of anti–VEGF injections through six months	Mean change in CRT through six months (range)	Mean change in BCVA through six months
Cohort 1 3x10^9 GC/eye (N=6)	2.4 ng/ml	4.7 inj*	-14 μm** (-181μm to +92 μm)	-2 letters** (-8 to +10 letters)
Cohort 2 1x10^{10} GC/eye (N=6)	12.8 ng/ml	3.8 inj	+26 μm (-7μm to +62 μm)	+7 letters (-4 to +15 letters)
Cohort 3 6x10^{10} GC/eye (N=6)	**160.2 ng/ml**	**1.3 inj**	**-14 μm** **(-27μm to +7 μm)**	**+8 letters** **(0 to +21 letters)**

* One subject in Cohort 1 discontinued from the study at four months with four injections and was imputed as requiring six injections through six months

** N=5; one subject in Cohort 1 discontinued from the study at four months

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RGX–314 Phase I/IIa clinical trial – sustained protein levels at six months

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

*One subject received an anti-VEGF rescue injection 1 month prior to sample
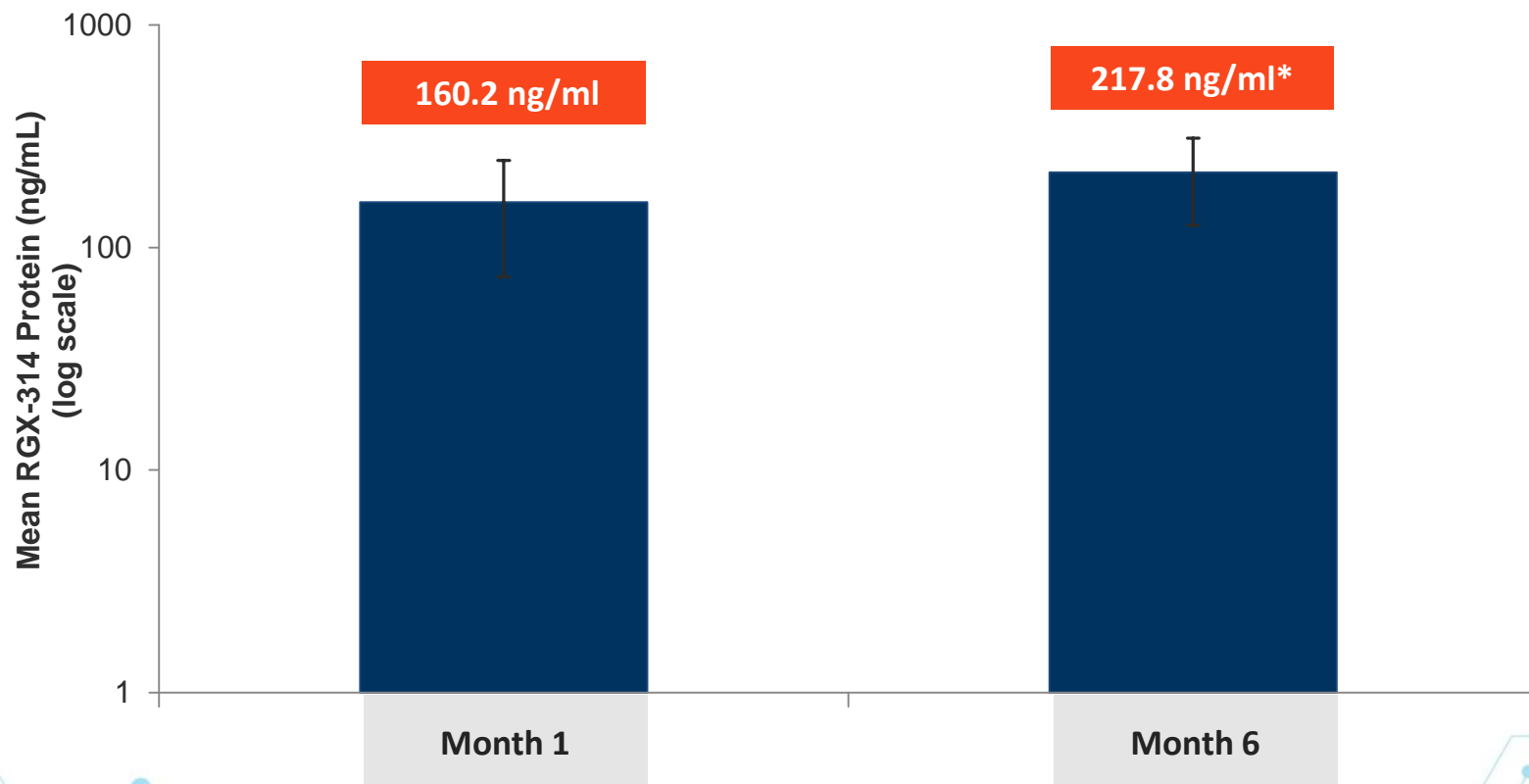
Cohort 3: Three subjects with no additional anti–VEGF injections through nine months

Previous therapy	▪ Study subjects received on average **>35 injections since wet AMD diagnosis**
Post-RGX–314 anti–VEGF injections	▪ **0 injections** through nine months post-RGX–314
BCVA	▪ Mean gain in BCVA of **+13 ETDRS** letters from baseline through nine months
SD–OCT	▪ Maintained with a **mean change in CRT of -37 µm** from baseline through nine months

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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

REGENXBIO®

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*

NAV Vector   Gene

1

RNA

2 Protein

3
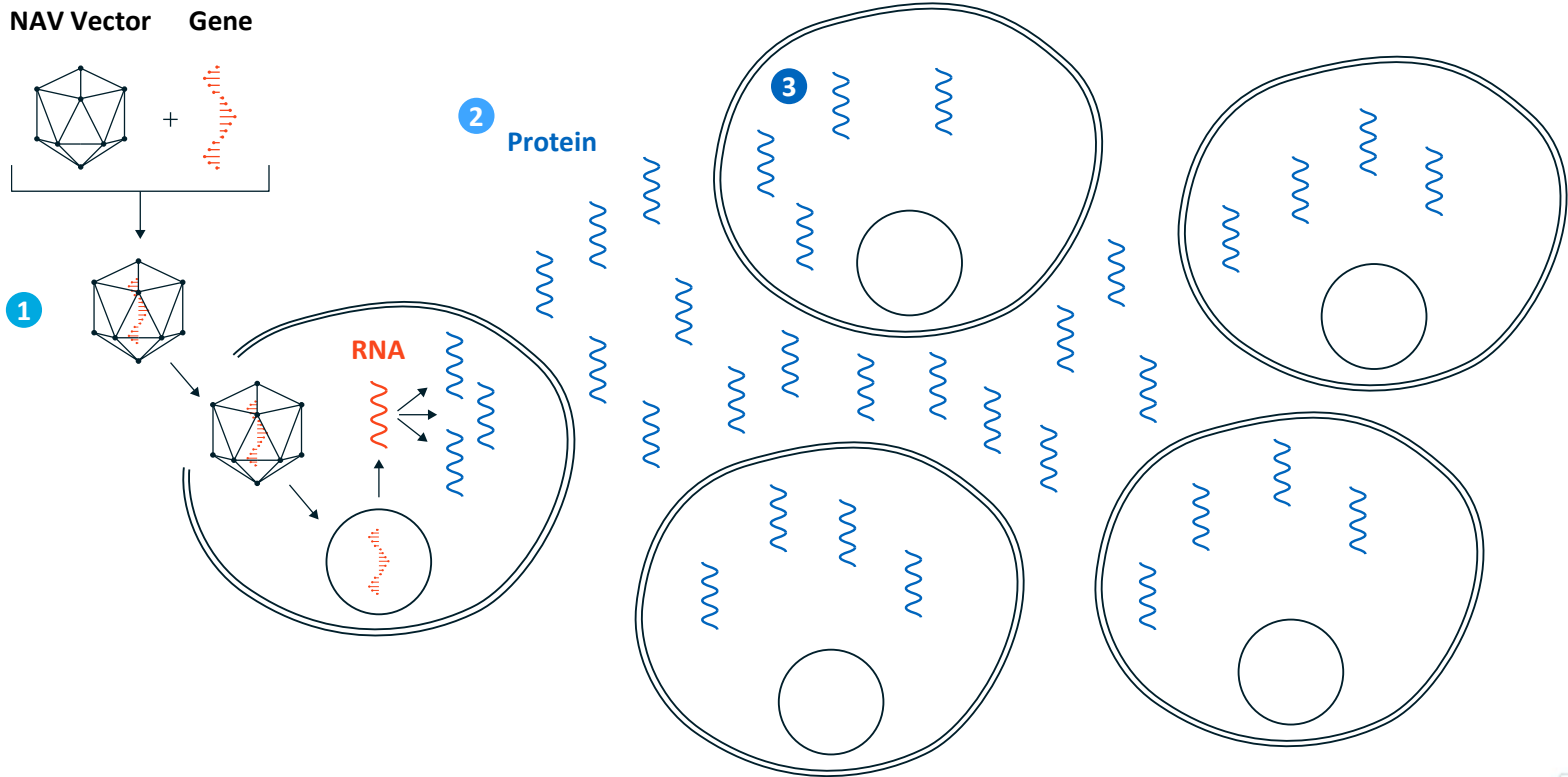
A single transduced cell has potential to correct many other cells

logo

REGENXBIO®

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

Baseline assessment   **Treatment evaluation**   **Follow up**

<u>Weeks</u>

Safety endpoint   Secondary endpoints

0   4   8   16   24   54   104

RGX–121 administration

Immunosuppressive therapy

Expected dose escalation pathway

**Dose 1
n = 3**

1.3×10^{10} gc/g
brain mass

Safety
review[1]

**Dose 2
n = 3**

6.5×10^{10} gc/g
brain mass

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

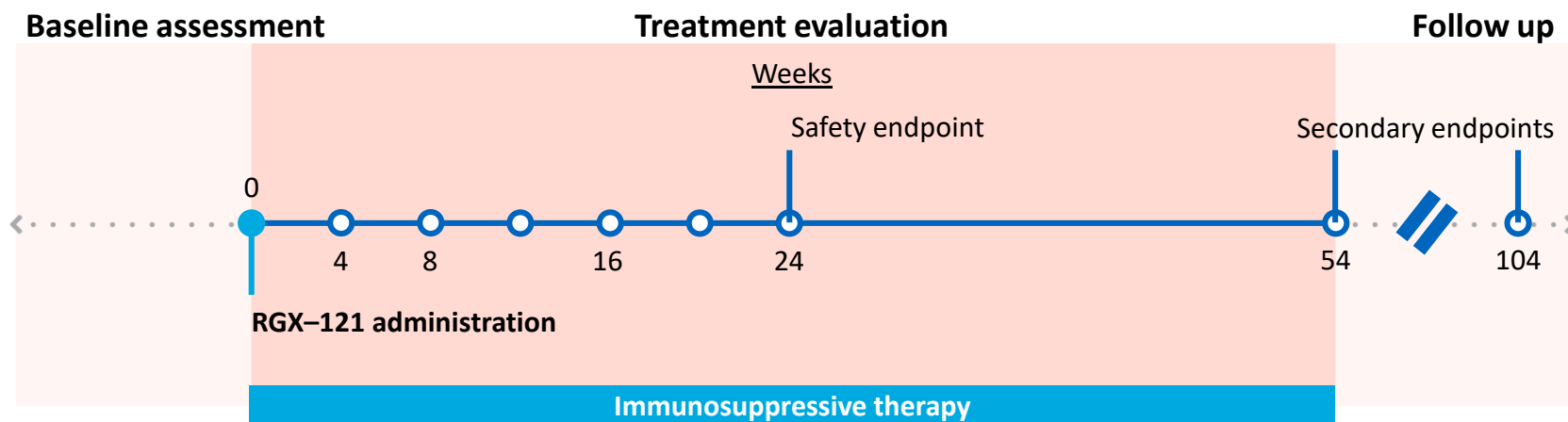**Sites**: Leading U.S. and international lysosomal storage disease centers
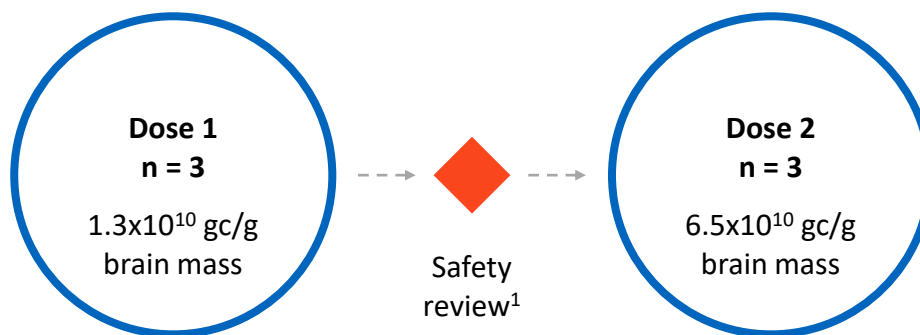

Key inclusion criteria

- 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

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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
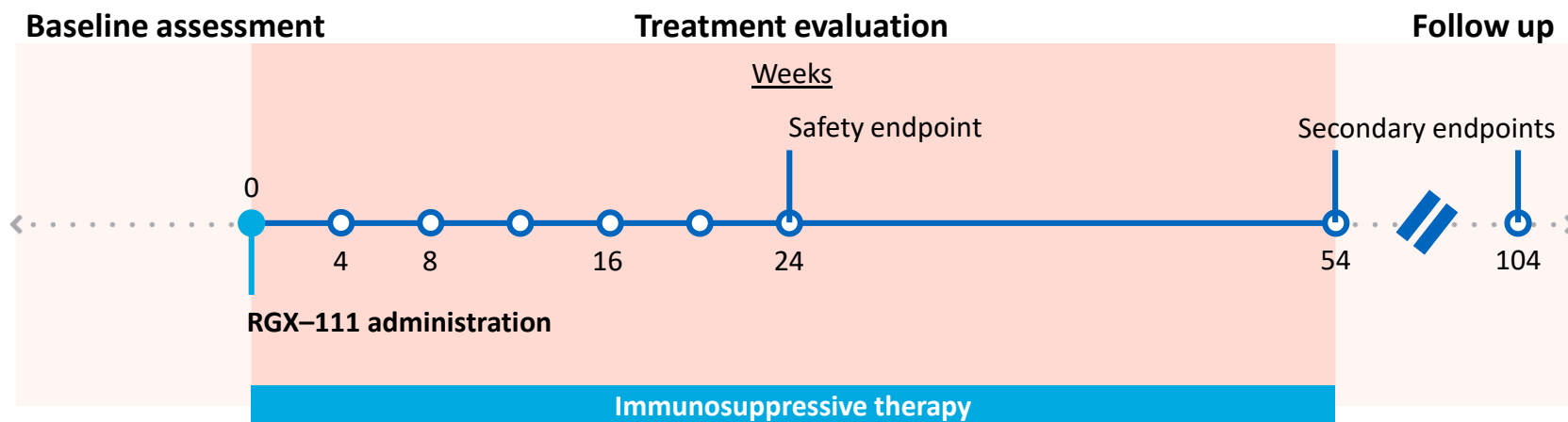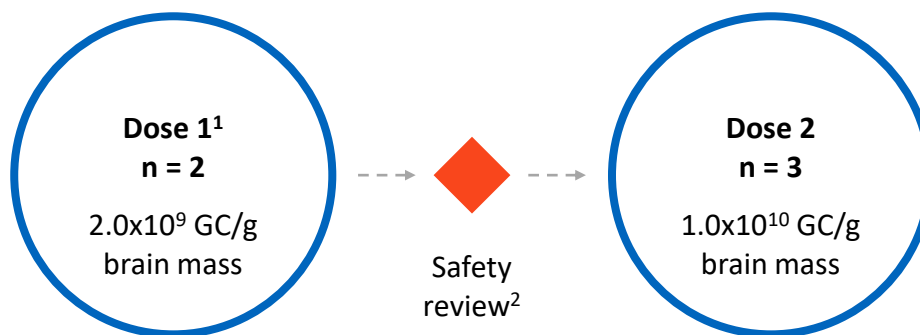
RGX–501 for treatment of homozygous familial hypercholesterolemia (HoFH)

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
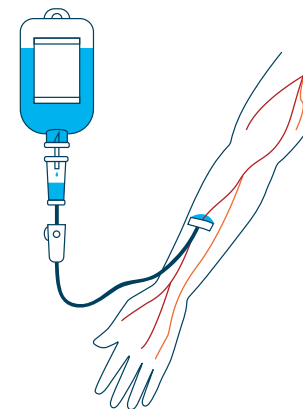
RGX–501 Phase I/II clinical trial in HoFH

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

Baseline assessment   **Treatment evaluation**   **Follow up**

Weeks

Secondary endpoints   Safety endpoint

0   4   8   12   26   52

RGX–501 administration

Corticosteroid prophylaxis[2]

Expected dose escalation pathway

Dose 1
n = 3
2.5×10^{12} gc/kg

Safety review[1]

Dose 2
n = 3
7.5×10^{12} gc/kg

Safety review[1]

Dose 2
n = 3
7.5×10^{12} gc/kg
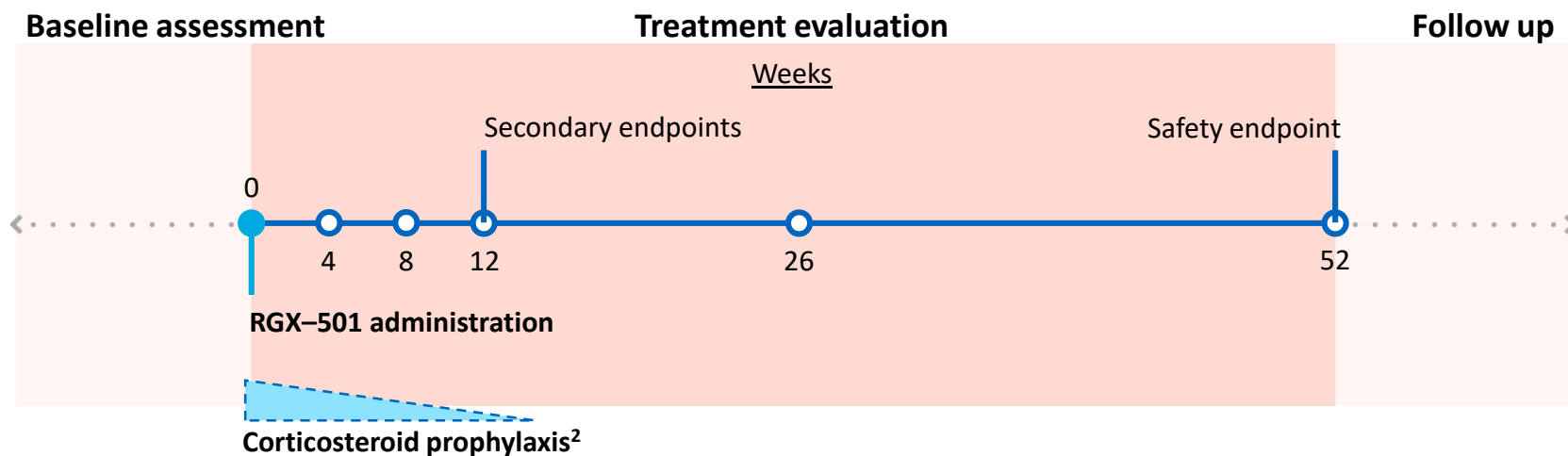
Three subjects dosed in each of the first and second cohorts

[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 amendment has been submitted to health authorities 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

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
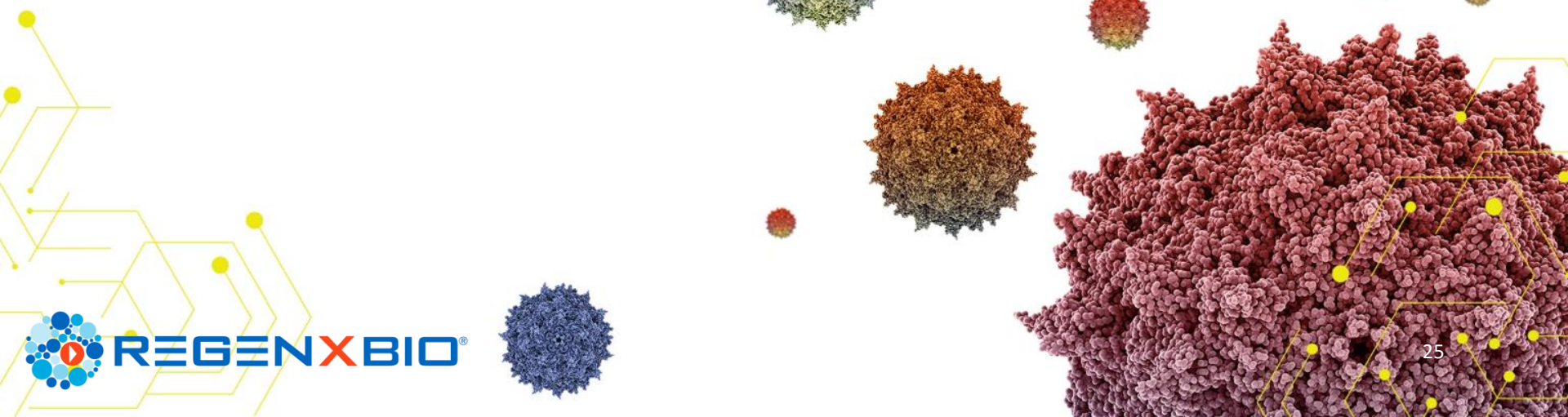
REGENXBIO®

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

Higher gene expression

Longer-term gene expression
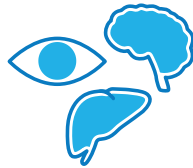
Broad and novel tissue selectivity

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

NAV Vectors: higher gene expression than early generation AAV vectors

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

AAV2
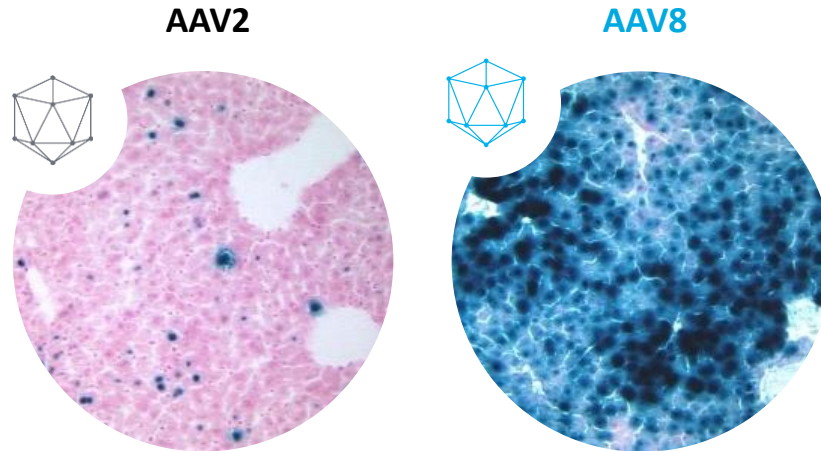
AAV8

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

AAV2

AAV8

Retinal Pigment Epithelium (RPE)

Photoreceptors (PR)

Retinal Pigment Epithelium (RPE)

Photoreceptors (PR)

[1] Vandenberghe et al. 2011 *Science Translational Medicine*
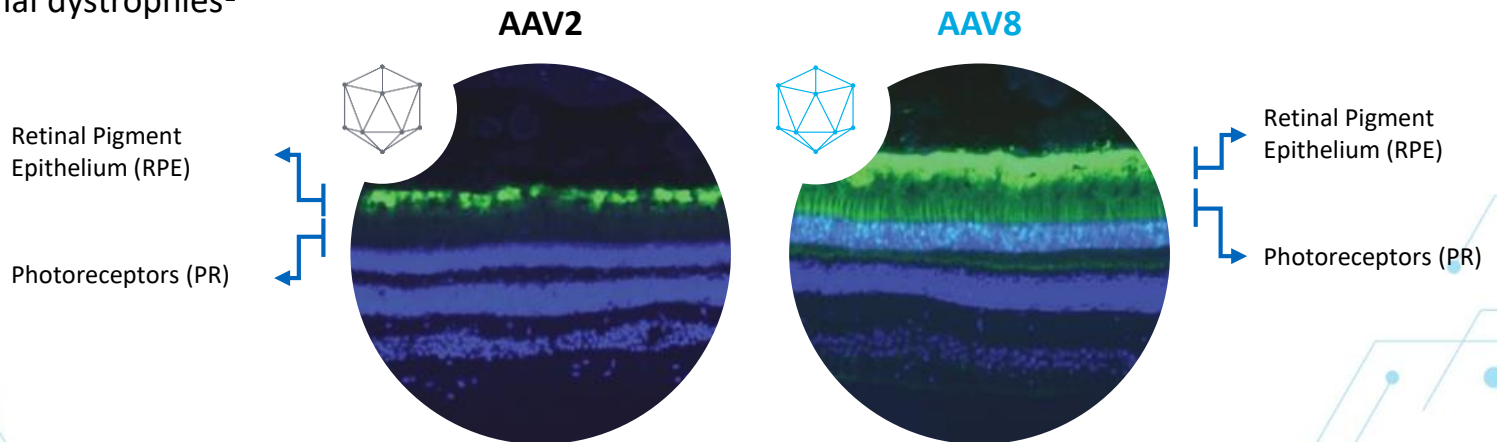
REGENXBIO | cGMP Manufacturing

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
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

2018 full year financials (mm)

R&D expense:	$84
G&A expense:	$37
Net income:	$100
Basic sharecount:	36.1

Financial highlights

In 2018, received **$180 million from AveXis** for amended SMA license agreement

Closed public offering in August 2018, raising **over $200 million in gross proceeds**

Ended 2018 with more than **$470 million in cash**[1]

Program guidance and anticipated milestones

RGX–314	**wet AMD:** Phase I/IIa data and initiation of Phase IIb trial in late 2019 **Undisclosed indication:** Disclose indication and IND submission in 2H 2019
RGX–121	Interim data update in 2H 2019
RGX–111	IND active and subject recruiting initiated; begin enrollment in Phase I trial in mid-2019
RGX–181	IND submission in 2H 2019
RGX–501	Interim data update in 2H 2019

2019 financial guidance:

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

Thank You