REGENXBIO®

Leader in AAV Gene Therapy

Corporate Presentation

February 18, 2021

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,” ”assume,” “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, many of which are beyond the control of REGENXBIO. For a summary of certain of these risks and uncertainties, 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, 2019 and comparable “risk factors” sections of REGENXBIO’s Quarterly Reports on Form 10-Q and other filings, which have been filed with the U.S. Securities and Exchange Commission (SEC) and are available on the SEC’s website at www.sec.gov. 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

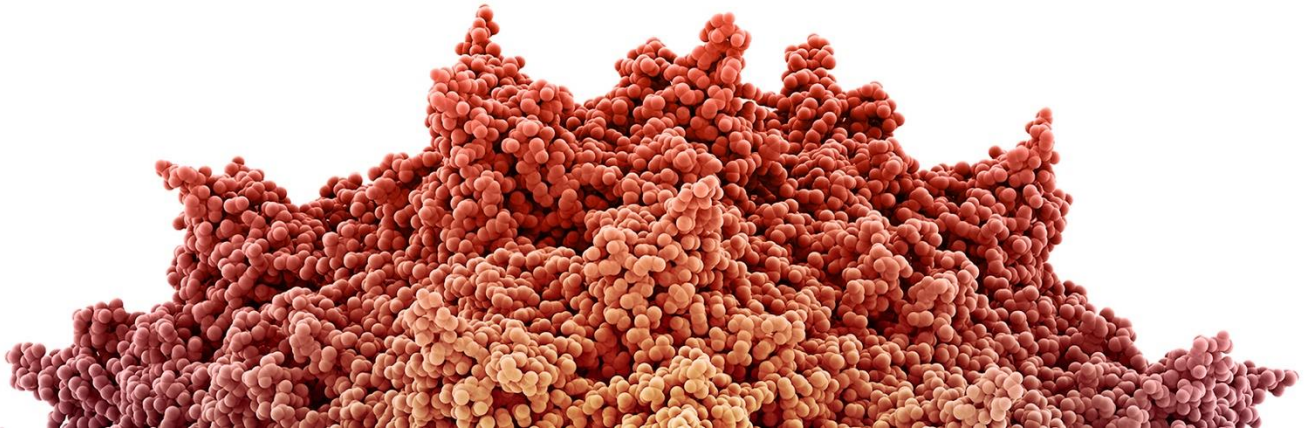
Pipeline focused on **AAV-mediated antibody delivery**
and **rare genetic diseases**
with **multiple clinical trials in 2021**

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

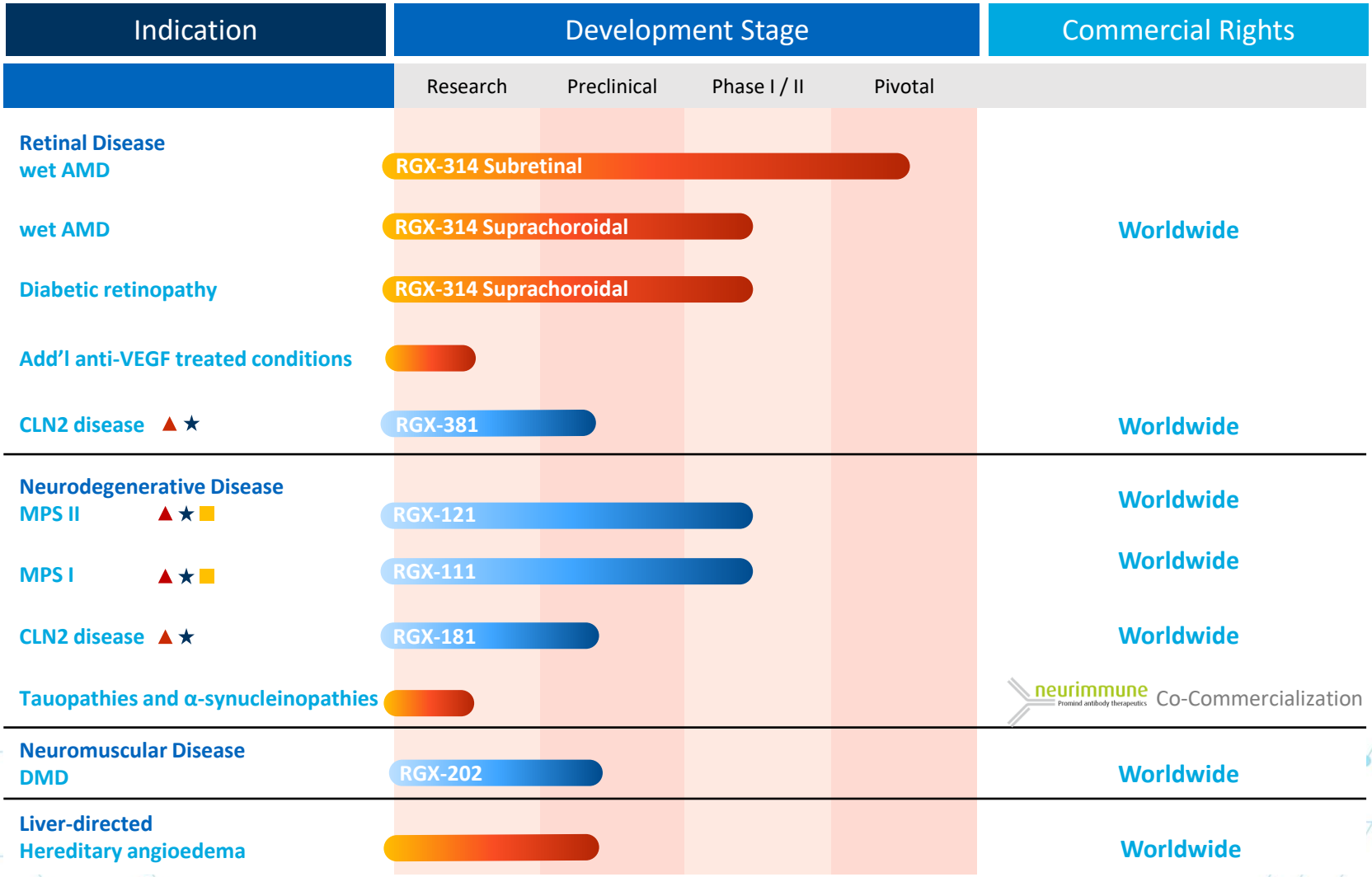
1 FDA-approved product using the NAV Technology Platform, and multiple clinical stage programs
being developed by third-party licensees across a broad range of therapeutic areas

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: Potential best-in-class, one-time gene therapy 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

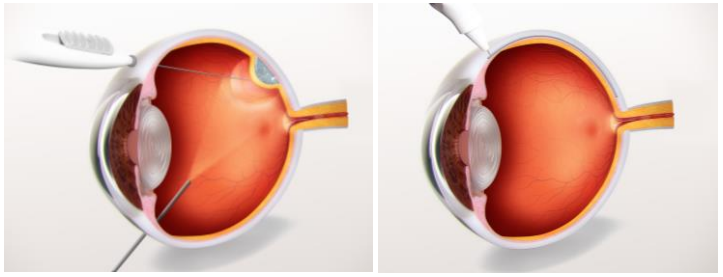
Vector: AAV8 | **Gene:** anti-VEGF Fab

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 for treatment of wet AMD: Current program status

Phase I/IIa subretinal dose-escalation study on-going[1]

- RGX-314 generally well-tolerated across all doses
- Durable treatment effect observed with stable to improved BCVA and CRT in Cohorts 3-5
- Long-term, meaningful reductions in anti-VEGF burden in Cohorts 3-5

Subretinal pivotal program is active and expected to support BLA filing in 2024

- Recently completed End of Phase 2 meeting with FDA
- Pivotal program to enroll a total of approximately 700 patients
- First trial, ATMOSPHERE™, is active and enrolling
- Clear path for cGMP manufacturing process to support BLA, bridging study expected to initiate in H1 2021

Phase II suprachoroidal AAVIATE trial on-going

- Cohort 1 enrollment complete; Cohort 2 enrollment expected to begin in Q1 2021
- Interim data from Cohort 1 expected in Q3 2021
- RGX-314 well-tolerated with no evidence of inflammation[2]

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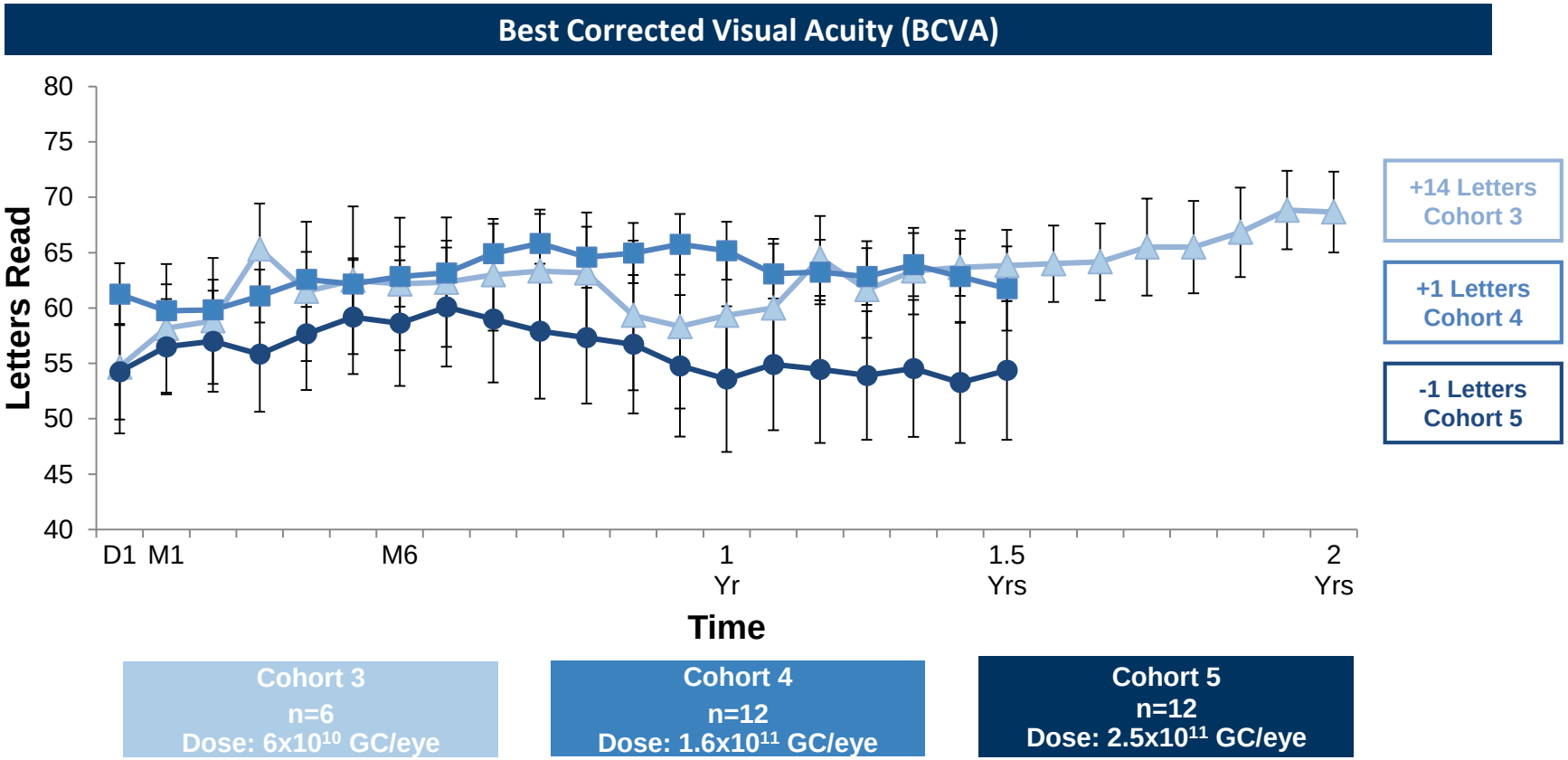
[1]Last data cut as of July 13, 2020
[2]As of December 31, 2020

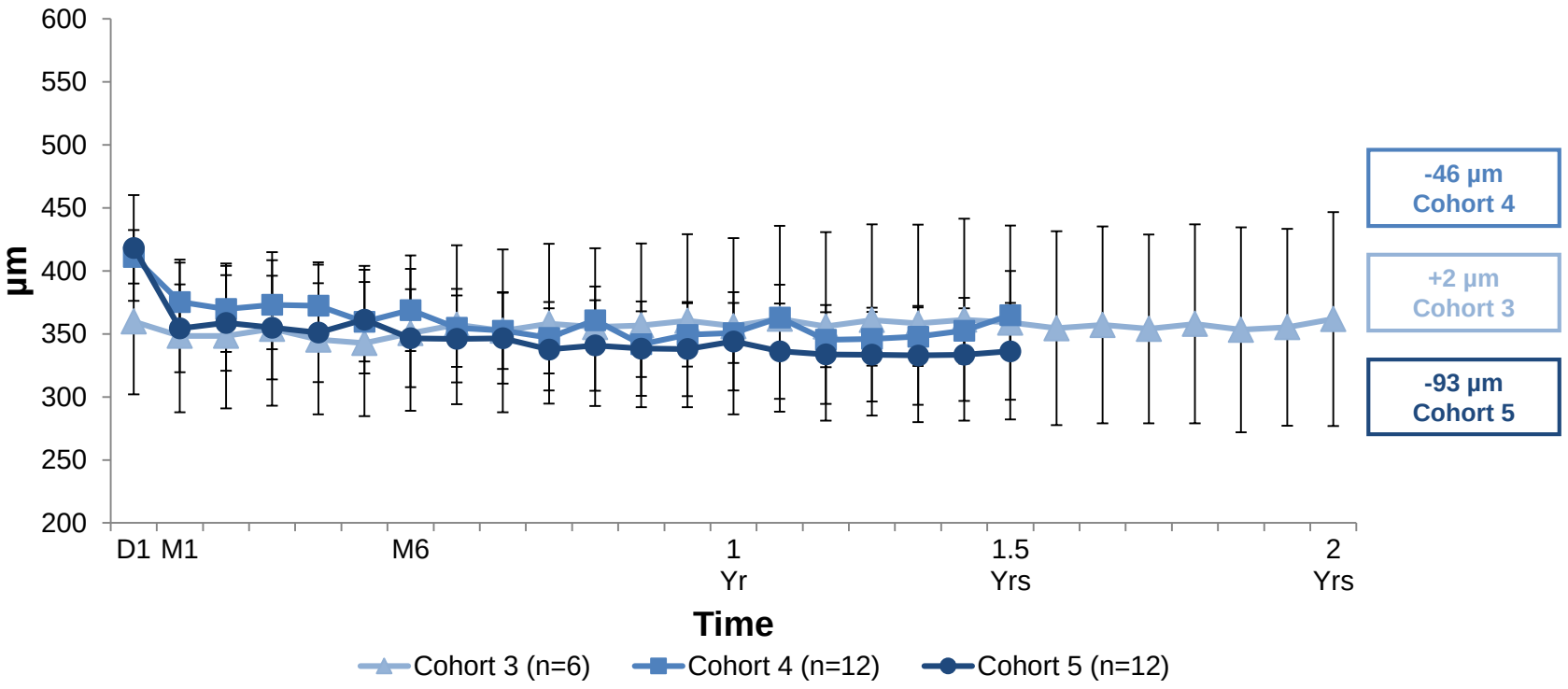
RGX–314 subretinal Phase I/IIa clinical trial: Mean BCVA over 2 years (Cohort 3) and 1.5 years (Cohorts 4&5)



Note: One patient in Cohort 5 discontinued the study prior to Week 22 visit. Another patient in Cohort 5 has missed the visits since Week 46 visit due to COVID-19. For this patient, missing visits were imputed using last observation carried forward (LOCF) through Week 74 visit. Six additional missing BCVA results were interpolated.

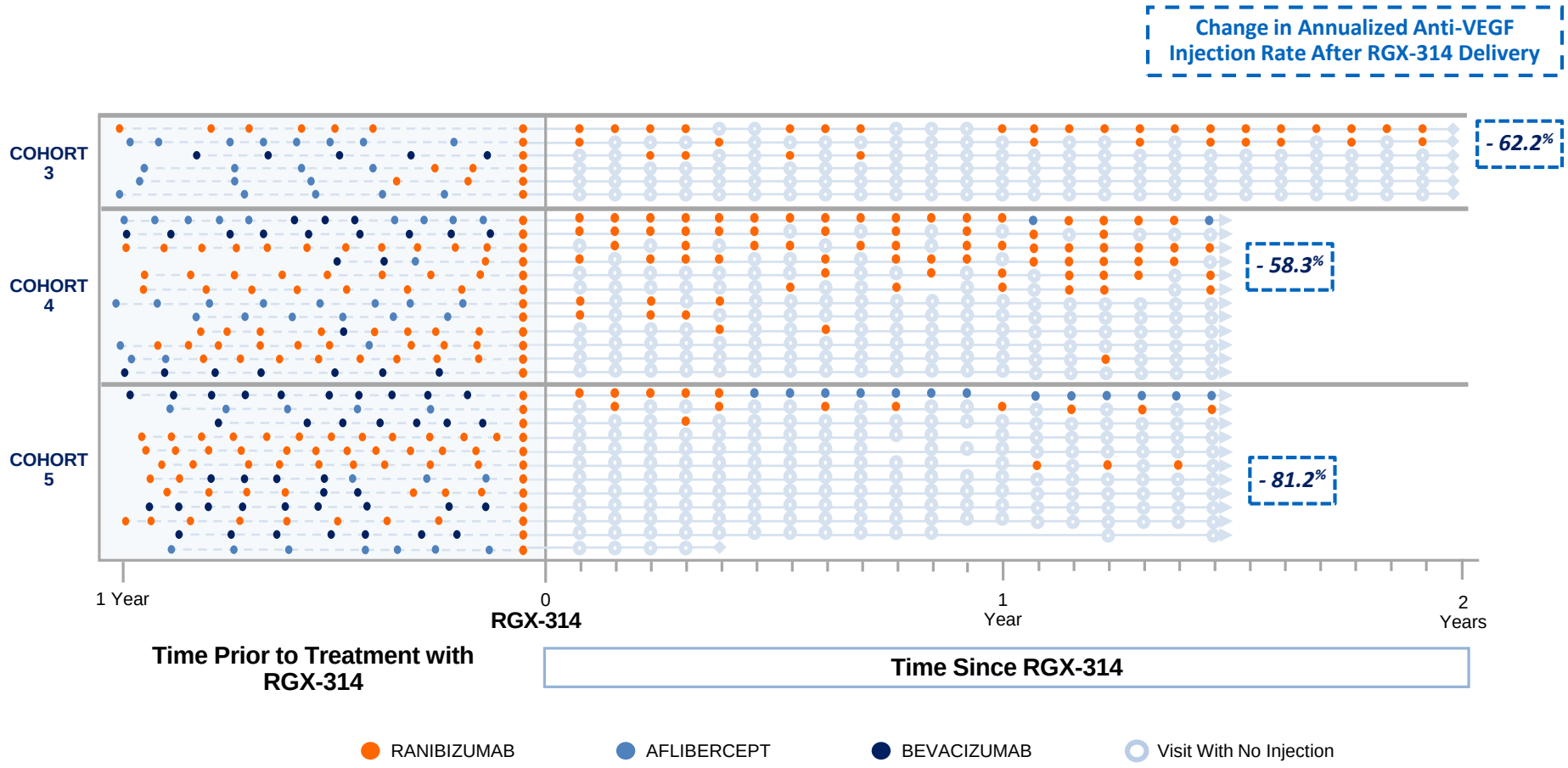
RGX–314 subretinal Phase I/IIa clinical trial: Mean CRT over 2 years (Cohort 3) and 1.5 years (Cohorts 4&5)

Central Retinal Thickness (CRT) by Central Reading Center



Note: One patient in Cohort 5 discontinued the study prior to Week 22 visit. Another patient in Cohort 5 has missed the visits since Week 46 visit due to COVID-19. For this patient, missing visits were imputed using last observation carried forward (LOCF) through Week 74 visit. Nine additional missing CRT results were interpolated.

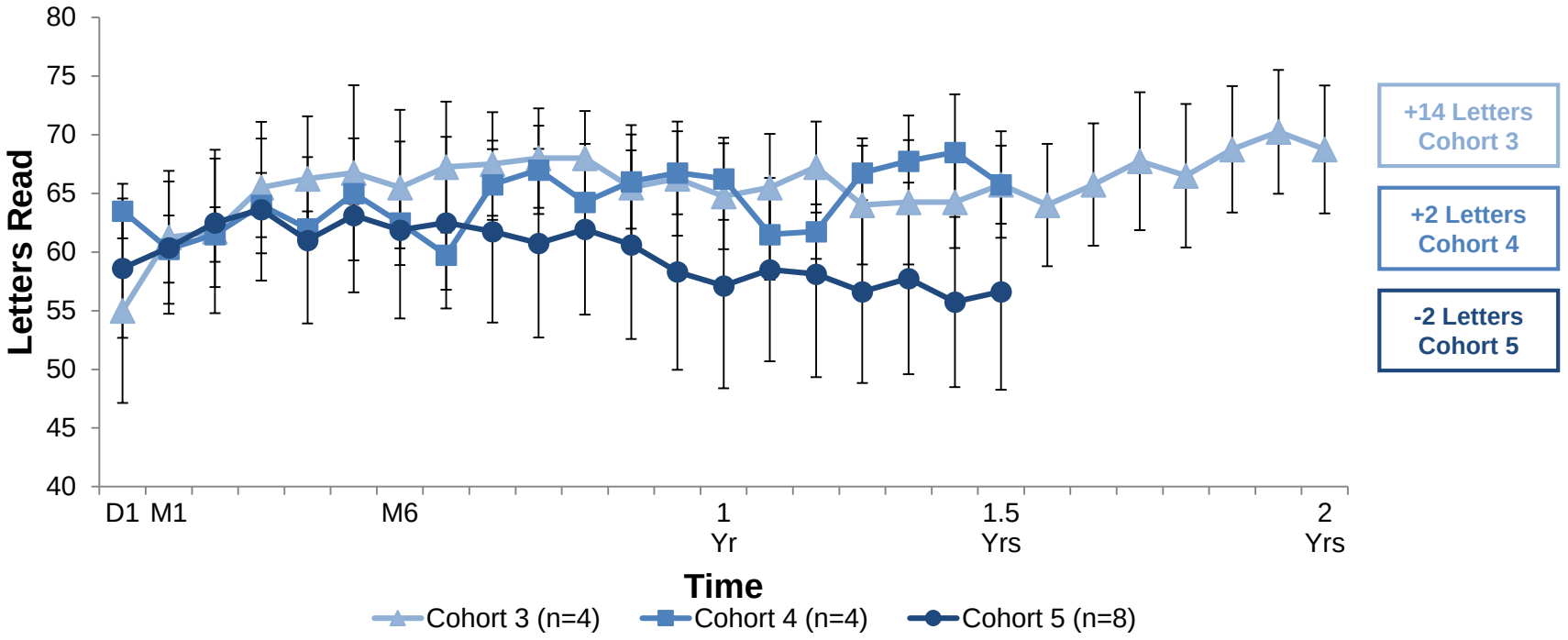
RGX–314 subretinal Phase I/IIa clinical trial: Cohort 3—5 injections pre- and post-RGX-314



RGX–314 subretinal Phase I/IIa clinical trial: Mean BCVA over 2 years (Cohort 3) and 1.5 years (Cohorts 4&5)

Patients with no anti-VEGF injections within 12 months of last visit

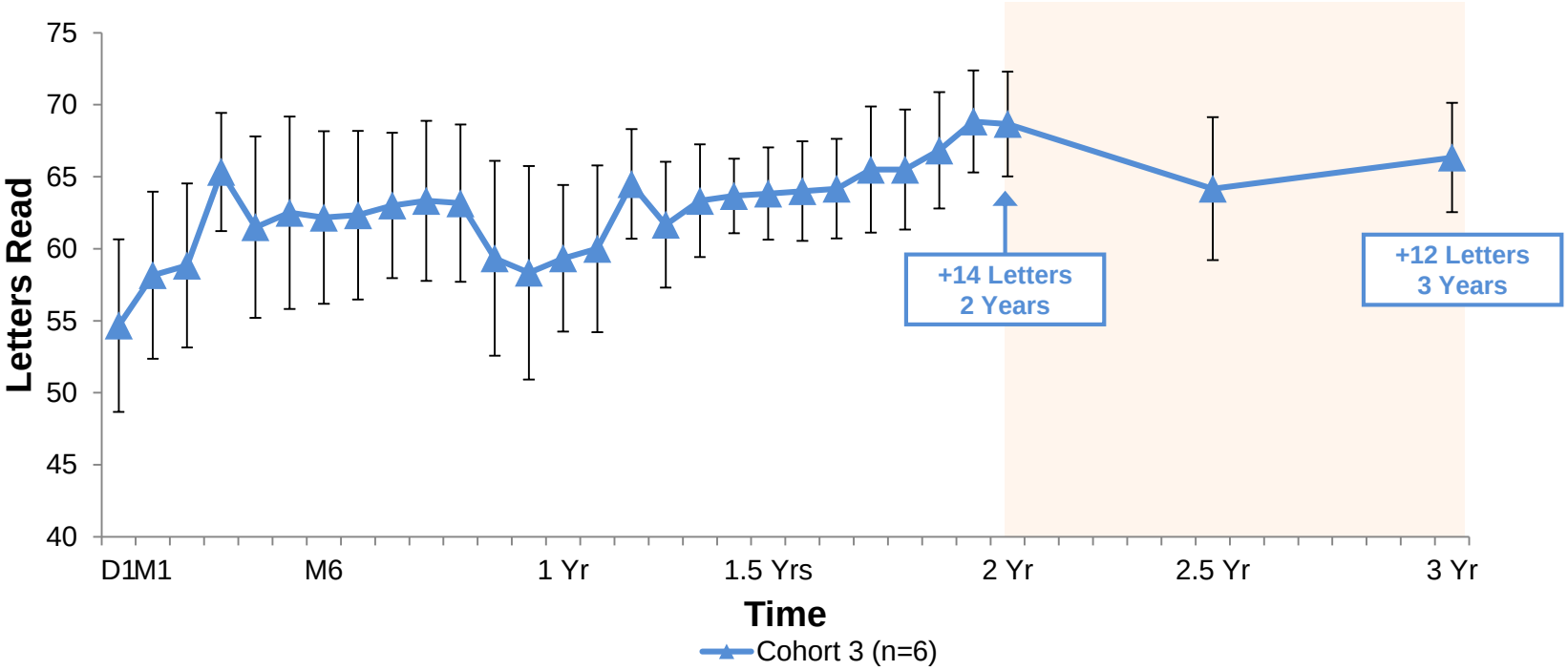
Best Corrected Visual Acuity (BCVA)



Note: One patient in Cohort 5 discontinued the study prior to Week 22 visit. Another patient in Cohort 5 has missed the visits since Week 46 visit due to COVID-19. For this patient, missing visits were imputed using last observation carried forward (LOCF) through Week 74 visit. Two additional missing BCVA results were interpolated.

RGX–314 subretinal Long-Term Follow-Up* trial: Mean BCVA over three years in Cohort 3

Best Corrected Visual Acuity (BCVA)



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* Subjects will roll over from the Phase I/IIa study to the Long-Term Follow-Up study to assess safety and efficacy up to five years after RGX-314 administration.

ATMOSPHERE™ pivotal clinical trial: RGX-314 for wet AMD

OBJECTIVES

Primary

- Non-inferiority in the mean change in BCVA for RGX–314 compared with monthly ranibizumab injection at 1 year

Secondary

- Safety and tolerability of RGX-314
- Effect of RGX–314 on vision and retinal anatomy
- Additional anti–VEGF injections post-RGX–314

Subjects: approximately 300 total

Route of administration: Subretinal

Sites: Up to 60 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 anti-VEGF therapy
- Documented response to anti–VEGF at trial entry (assessed by SD–OCT)
- Vision of 20/32 to 20/160
- Pseudophakic (status post cataract surgery)

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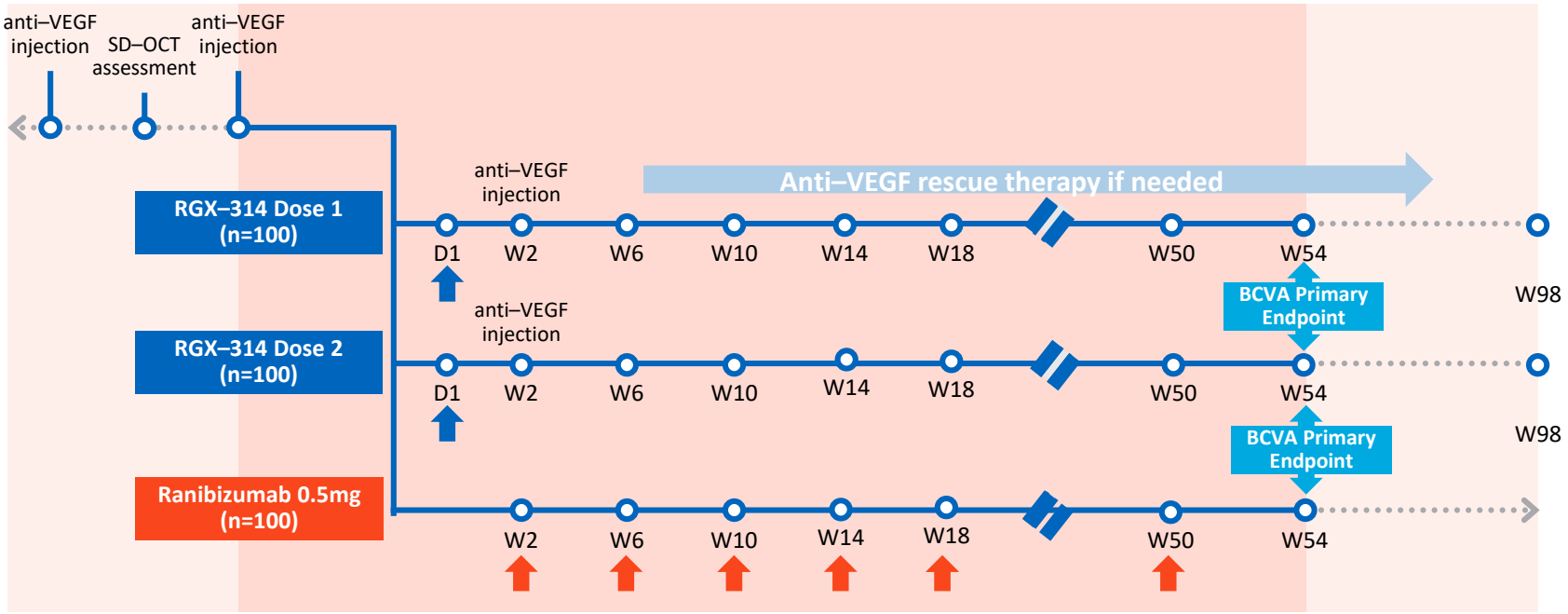
ATMOSPHERE™ pivotal trial design

Administration and follow-up timeline



Arms and Interventions

RGX-314 Dose 1	RGX-314 Dose 2	Ranibizumab
6.4x10^{10} GC/eye	1.3x10^{11} GC/eye	Comparator

A second pivotal trial is expected to be similar in design to ATMOSPHERE, planned to initiate in H2 2021

AAVIATE™ Phase II clinical trial: RGX-314 for wet AMD

OBJECTIVES

Primary

- To evaluate the mean change in BCVA for RGX-314 compared with ranibizumab monthly injection at Week 40

Secondary

- Safety and tolerability of RGX-314
- Change in central retinal thickness (CRT) as measured by Spectral Domain Optical Coherence Tomography (SD-OCT)
- Additional anti–VEGF injections post-RGX–314

Subjects: Up to 40 total (randomized 3:1)

Route of administration: Suprachoroidal using SCS Microinjector

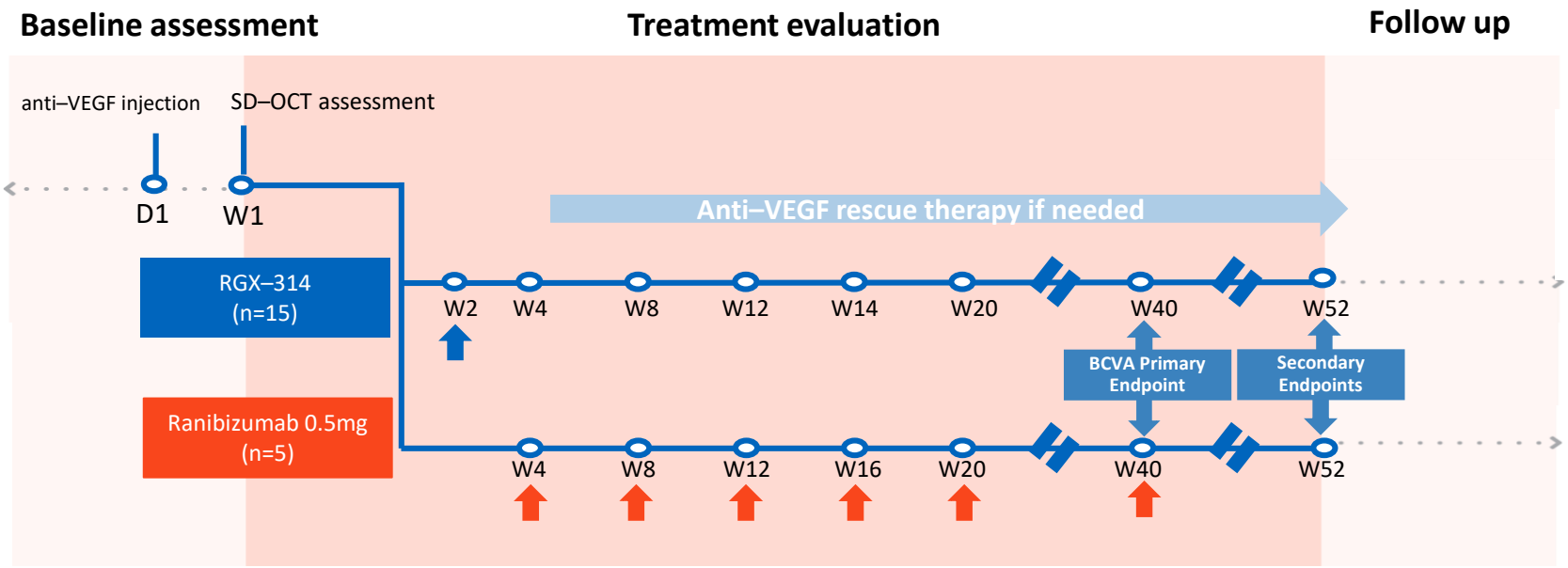
Sites: Fifteen leading retinal surgery centers across the United States

KEY INCLUSION CRITERIA

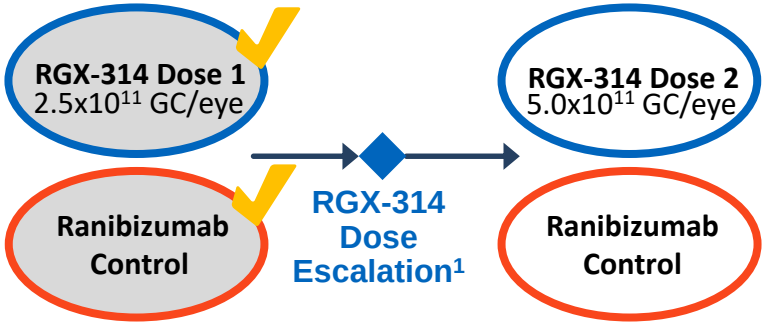
- Male or female ≥50 to 89 years of age
- Previously treated wet AMD subjects requiring no more than 10 anti–VEGF injections in the 12 months prior to trial entry
- Documented response to anti–VEGF at trial entry (assessed by SD–OCT)
- BCVA between ≤ 20/25 and ≥ 20/125 (≤ 83 and ≥ 44 Early Treatment Diabetic Retinopathy Study [ETDRS] letters) in the study eye

AAVIATE™ Phase II clinical trial design

Administration and follow-up timeline



Dose escalation



[1] Dose escalation safety review to occur two weeks after final subject in Cohort 1 has been dosed
SD-OCT = spectral domain optical coherence tomography

RGX–314 for treatment of Diabetic Retinopathy (DR)

THE DISEASE

- Leading cause of vision loss in adults between 24–75 years of age; average age of onset is 45-50 years of age
- As disease progresses from non-proliferative DR (NPDR) to proliferative DR (PDR), patients are at increased risk of developing vision threatening complications
- Vision threatening complications include diabetic macular edema (DME) and neovascularization that can lead to blindness
- Approximately 8 million patients estimated in United States alone

RGX–314 PRODUCT CANDIDATE

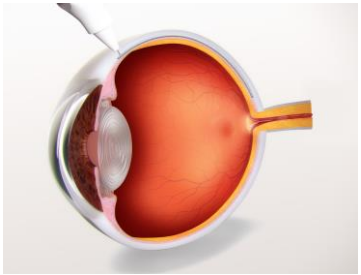
Vector: AAV8 | **Gene:** anti-VEGF Fab

Mechanism of action

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

Route of administration

Suprachoroidal



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ALTITUDE™ Phase II clinical trial in DR

OBJECTIVES

Primary

- Evaluate proportion of patients with ≥2 step improvement in severity on the Diabetic Retinopathy Severity Scale (DRSS) at 48 weeks

Secondary

- Safety and tolerability of RGX-314
- Development of DR-related ocular complications
- Need for additional standard of care interventions

Subjects: Up to 40 total (randomized 3:1)

Route of administration: Suprachoroidal using SCS Microinjector

Sites: Fifteen leading retinal surgery centers across the United States

KEY INCLUSION CRITERIA

- Male or female ≥ 25 to 89 years of age with DR secondary to diabetes mellitus Type 1 or Type 2
- Moderately severe NPDR, severe NPDR, or Mild PDR
- No active DME, CST < 320 µm
- Vision of 20/40 or better (≥ 69 Early Treatment Diabetic Retinopathy Study [ETDRS] letters) in the study eye

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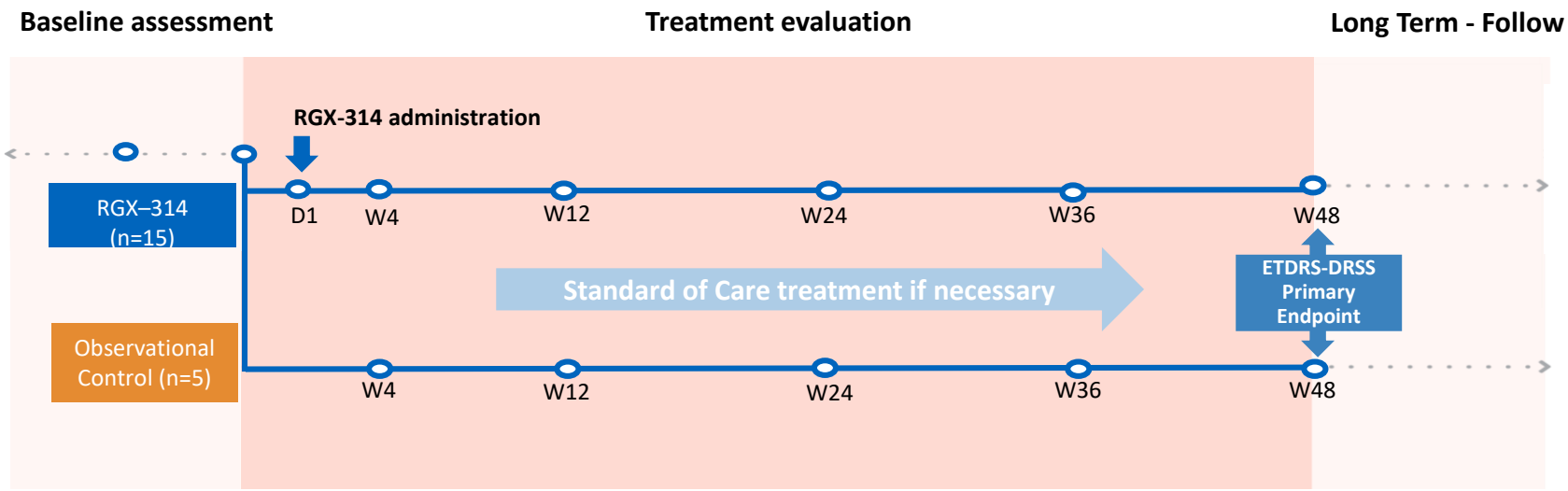
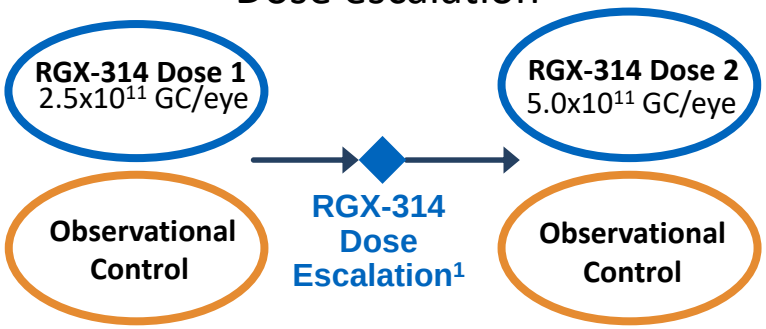
ALTITUDE™ Phase II clinical trial design

Administration and follow-up timeline



Dose escalation



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

RGX–202 for treatment of Duchenne Muscular Dystrophy (DMD)

THE DISEASE

- DMD is caused by mutations in the DMD gene which encodes for dystrophin, a protein involved in muscle cell structure and signaling pathways
- Without dystrophin, muscles throughout the body degenerate and become weak, eventually leading to loss of movement and independence, required support for breathing, cardiomyopathy and premature death
- Affects 1 in 3,500 to 5,000 male births worldwide

RGX–202 PRODUCT CANDIDATE

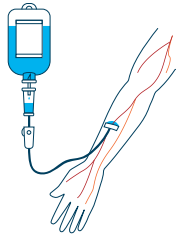
Vector: AAV8 | **Gene:** microdystrophin

Mechanism of action

Delivers transgene that encodes for novel microdystrophin which includes extended coding region of the C-Terminal Domain

Route of administration

Intravenous



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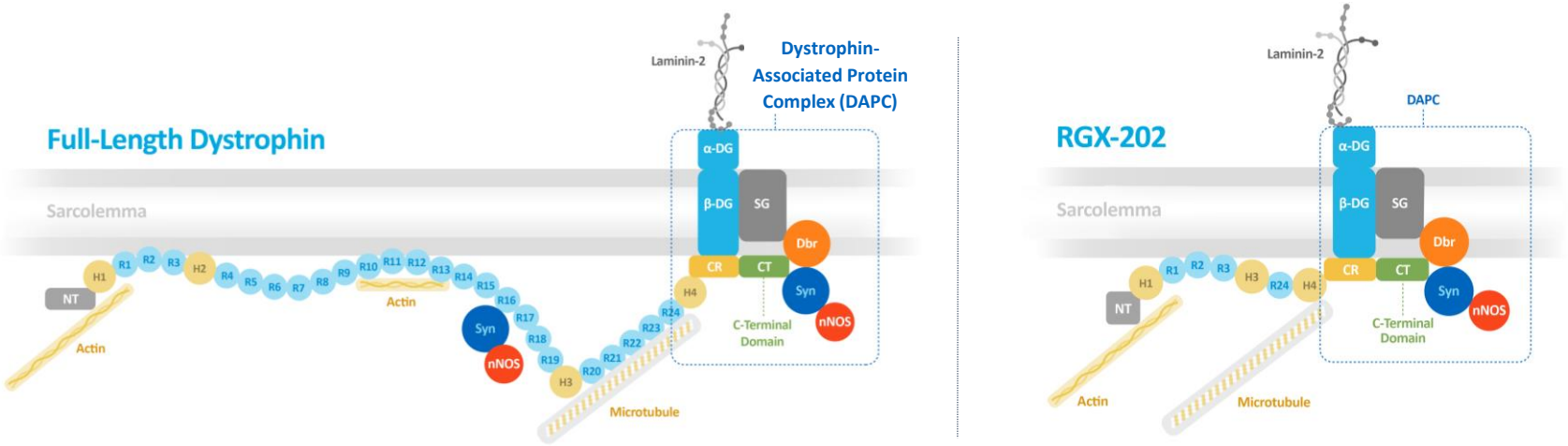
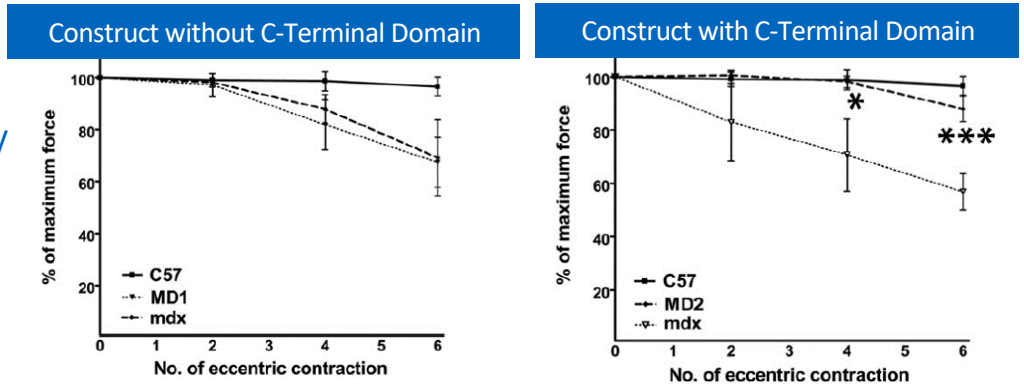
RGX-202 is designed to retain key elements of full-length dystrophin

CT Domain has been shown to recruit several key proteins to the muscle cell membrane (sarcolemma) including Syntrophin and Dystrobrevin, Neuronal nitric oxide synthase and other proteins[1]



Presence of CT Domain in microdystrophin significantly improved the muscle resistance to lengthening contraction–induced muscle damage in DMD^{mdx} mice[2]



[1] Allen et al, *Physiological Review*, 2016
[2] Koo et al, *Human Gene* Therapy, 2011

Syn: Syntrophin; Dbr: Dystrobrevin; CR: Cystein rich domain; nNOS: Neuronal nitric oxide synthase; DG: Dystroglycan; H: hinge; R: repeat

RGX-202 program has several features that provide potential benefits

	AAV Capsid	Promoter	Microdystrophin domain design	Transgene Size (bp)	CpG total (# Islands)
RGX-202	8	Spc5-12	ABD1 H1 R1 R2 R3 H3 R24 H4 CR CT	4,734	69 (1)
Other Investigational Intervention (Example)			ABD1 H1 R1 R2 H3 R22 R23 R24 H4 CR		

RGX-202 Features	Potential Benefits
Novel microdystrophin transgene includes extended coding region of dystrophin C-Terminal (CT) Domain	CT domain has been shown to recruit key proteins, leading to improved muscle resistance[1]
Codon optimization and CpG content reduction	May improve gene expression, increase translational efficiency and reduce immunogenicity[2]
NAV AAV8 vector and Spc5-12 muscle specific promoter	Designed to support the delivery and targeted expression of genes throughout skeletal and heart muscle[3, 4, 5]
Commercial-scale cGMP material already produced at 1000L capacity	Material expected to be used in clinical trials

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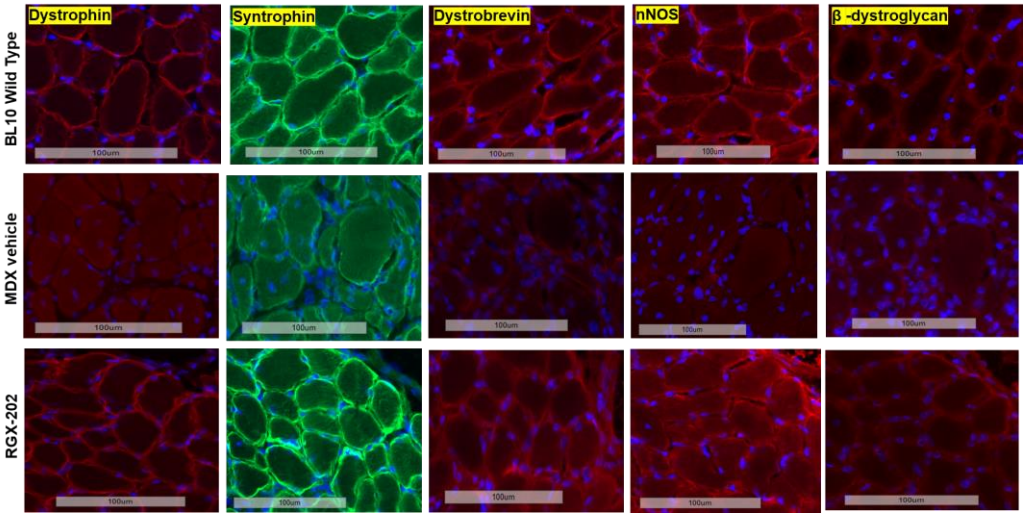
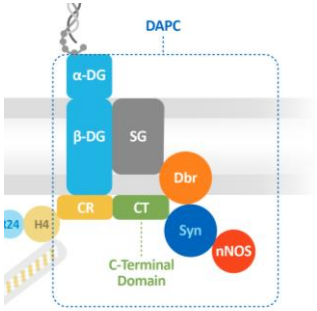
[1] Koo et al, *Human Gene* Therapy, 2011
[2] Faust, et al. *Journal of Clinical Investigation*, 2013
[3] Le Guiner, et al. *Nature Communications*, 2017
[4] Mack, et al. *Molecular Therapy*, 2017
[5] Shieh, et al. ASGCT 2019

RGX-202 Proof of concept in DMD^{mdx} mouse model

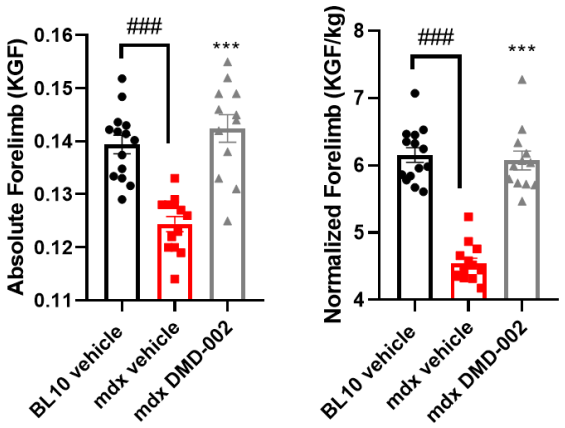


Histological evidence that RGX-202 recruits key proteins to DAPC

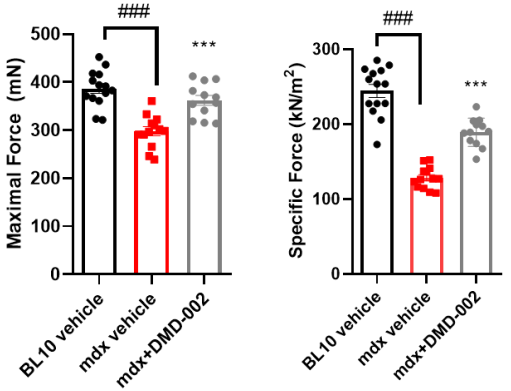


Significant strength and force improvements observed in DMD^{mdx} mice treated with RGX-202

Mouse Grip Strength (In-Life)



***Ex Vivo* Force Measurements**



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Data on file

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; stem cell 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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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: Approximately 12 patients

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 ≤77 on neurocognitive testing
  - Diagnosis of MPS II and a decline of ≥1 standard deviation on consecutive intelligent quotient testing
  - Having a relative diagnosed with severe MPS II who has the same IDS mutation as the subject
  - Having documented mutation(s) in *IDS* that is known to result in a neuronopathic phenotype
- No contraindications for intracisternal or intracerebroventricular injection and immunosuppressive therapy

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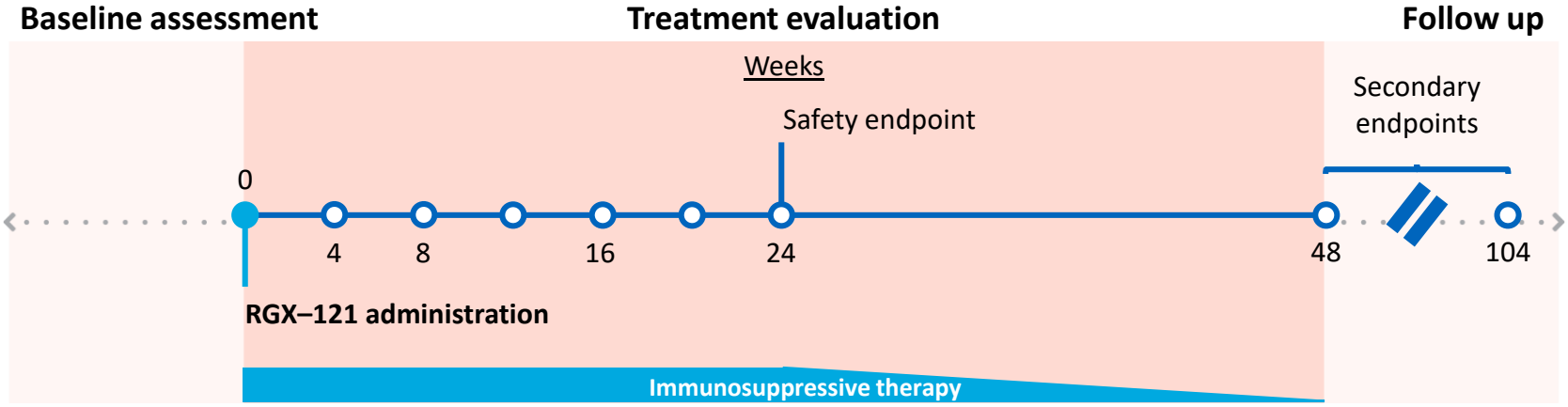
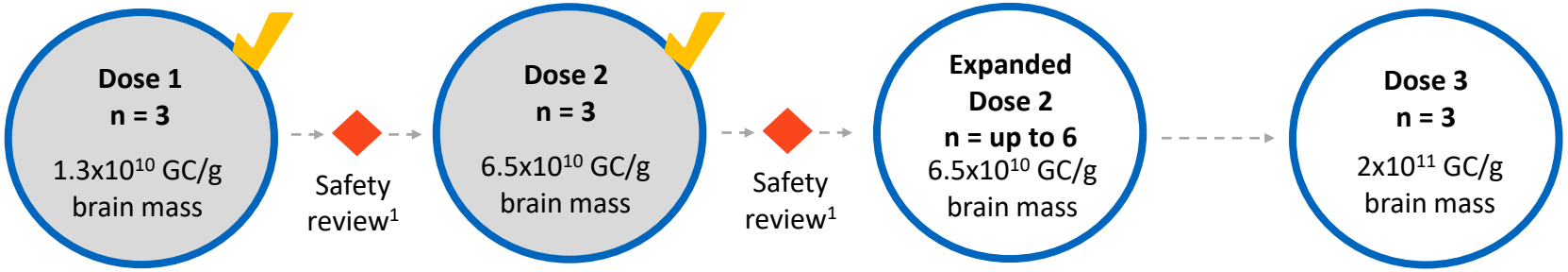
RGX–121 Phase I/II clinical trial: Administration and dose escalation

Administration and follow-up timeline



Dose escalation



Dosing in Cohort 3 expected to begin in Q1 2021

[1]Safety review to occur eight weeks after final subject in Cohort 1 has been dosed and after first three subjects in Cohort 2 have been dosed

RGX–121 Phase I/II clinical trial: Safety update and data summary from Cohorts 1 and 2[1]

- ***Well-tolerated following one-time RGX-121 administration*** *(n=8)*
  - No drug-related Serious Adverse Events
- ***Biomarkers and measures of neurodevelopmental function indicate CNS activity following RGX-121 administration***
  - Consistent reductions in CSF biomarkers up to 2 years after RGX-121 administration
  - Continued cognitive development and skill acquisition observed
- ***Evidence of systemic enzyme expression and biomarker activity***
  - Increased plasma enzyme levels
  - Rapid urine biomarker reductions observed in ERT[2]-naïve patients
  - Reduced liver and spleen volumes in one ERT-naïve patient

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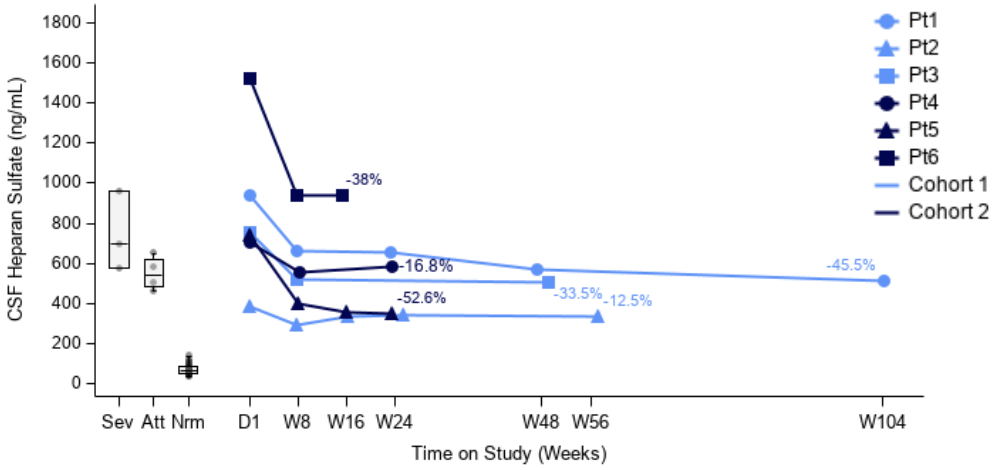
[1] As announced February 8, 2021

[2] Enzyme replacement therapy, standard of care for MPS II patients

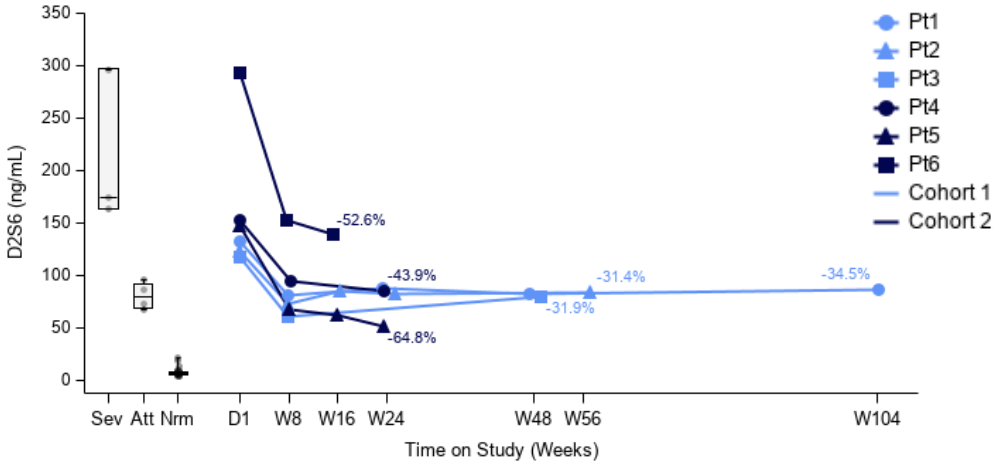
RGX–121 Phase I/II clinical trial: Consistent reductions in CSF biomarkers up to 2 years after RGX-121 administration

Heparan sulfate (HS) in cerebral spinal fluid, % change vs baseline



Median change from baseline: -30.3% at Week 8; -35.8% at last timepoint available

HS D2S6 disaccharide in cerebral spinal fluid, % change vs baseline



Median change from baseline: -44.2% at Week 8; -39.2% at last timepoint available

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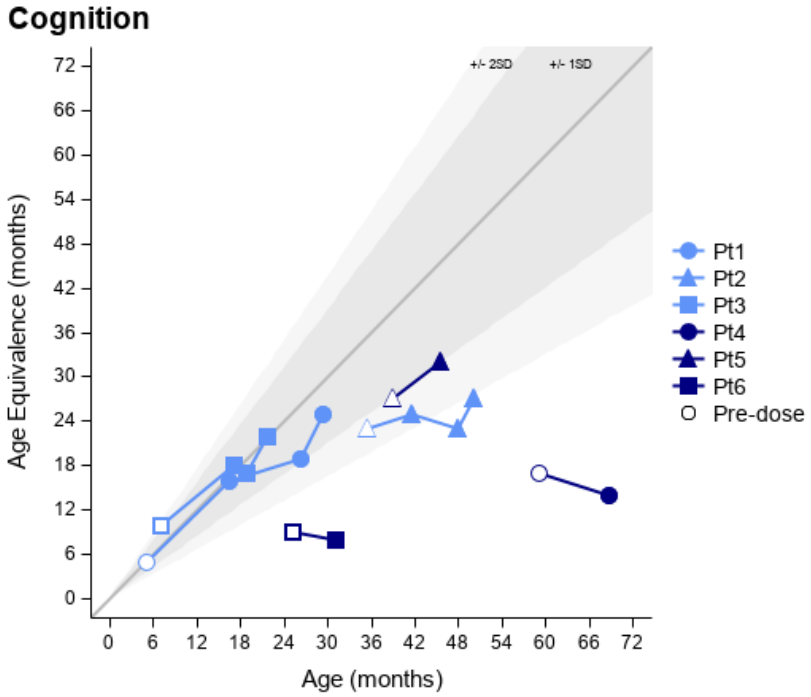
RGX–121 Phase I/II clinical trial: Continued cognitive development and skill acquisition observed in Cohorts 1 and 2 in patients with >6 months of follow-up

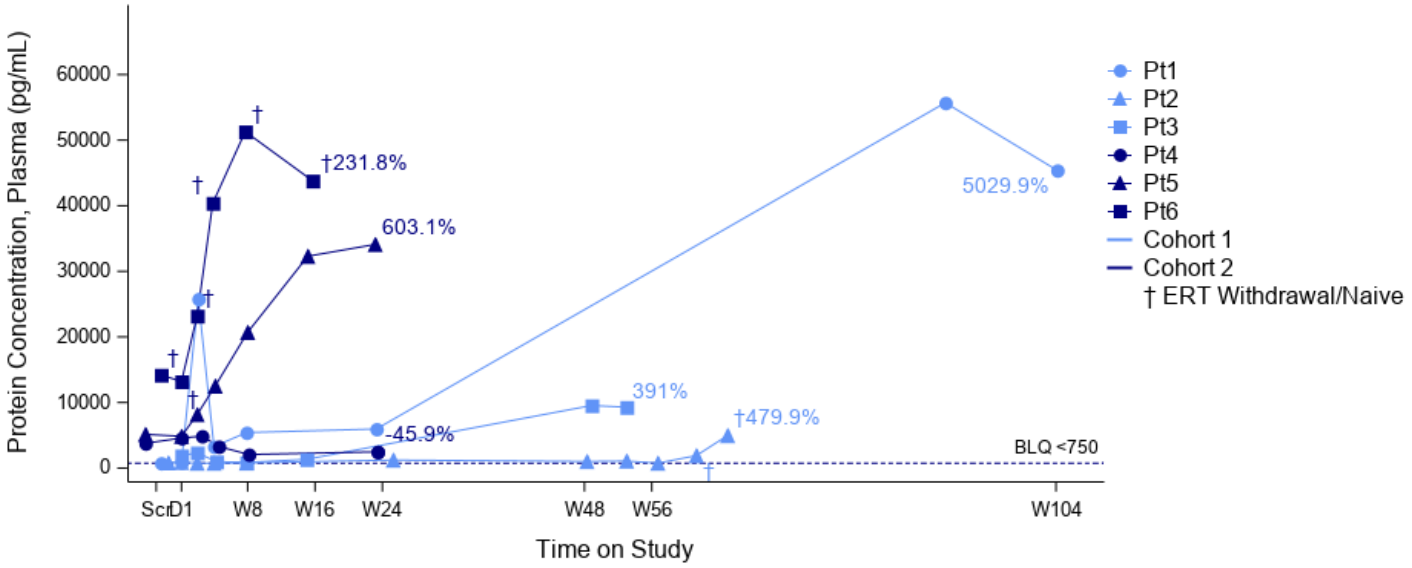
Bayley Scales of Infant and Toddler Development, 3rd Edition (BSID-III)



- Patients 1, 3 and 5 demonstrated continued cognitive development within a normal range
- Patients 2 and 4 entered the study with significant delay in neurocognitive development at baseline
  - Patient 2 has continued cognitive development
  - Patient 4 continued to acquire expressive and receptive language skills

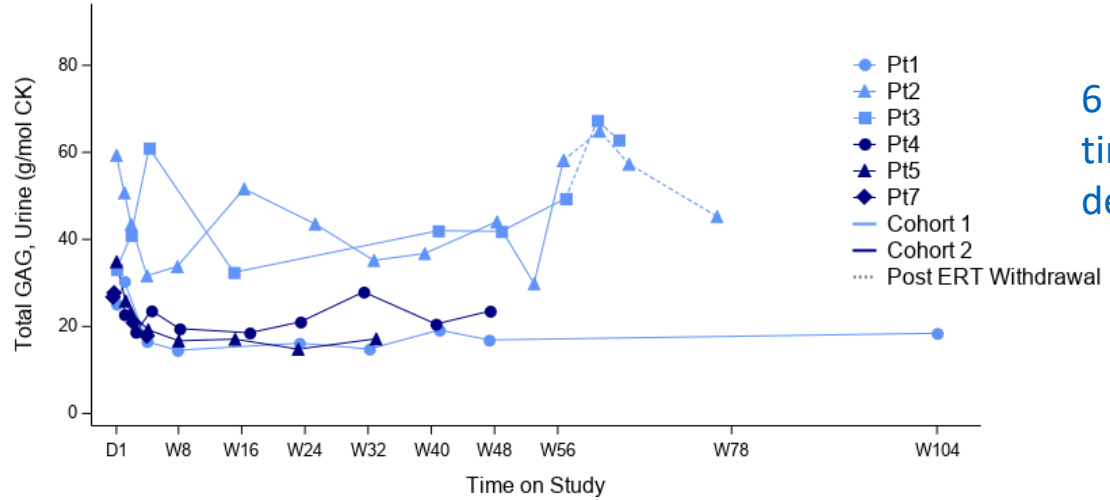
RGX–121 Phase I/II clinical trial: Evidence of systemic enzyme activity in Cohorts 1 and 2

I2S **enzyme** concentration levels, % change vs baseline



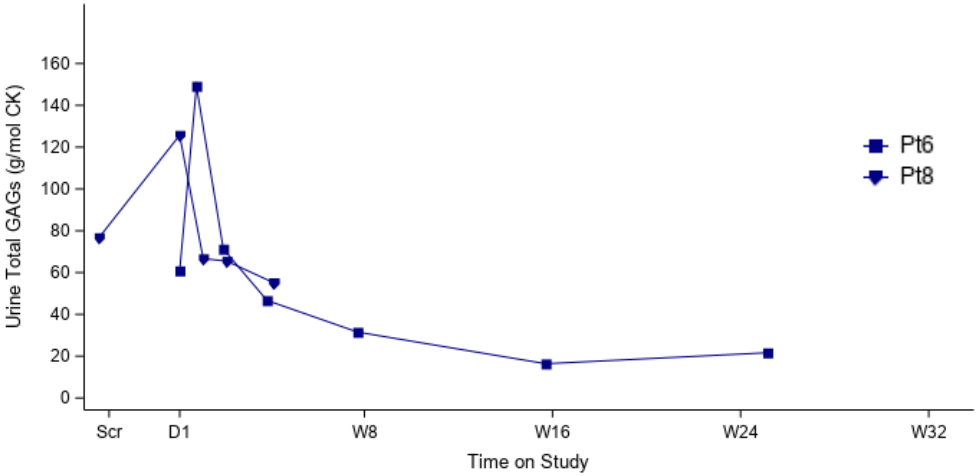
RGX–121 Phase I/II clinical trial: Urine biomarker reductions in Cohorts 1 and 2

Total GAG[1] in urine, patients with ERT



6 patients receiving ERT at the time of enrollment demonstrated decreases in total GAG levels

Total GAG in urine, patients without ERT



2 ERT-naïve patients demonstrated rapid decreases in total GAG levels

[1] Glycosaminoglycans

RGX–111 Phase I/II 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 lysosomal storage disease centers

Key inclusion criteria

- Male or female ≥ 4 months of age
- Documented evidence of CNS involvement due to MPS I or documented diagnosis of 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
  - Having documented biallelic mutation in *IDUA* predictive of severe MPS I or a relative diagnosed with severe MPS I
- No contraindications for intracisternal or intracerebroventricular injection or immunosuppressive therapy

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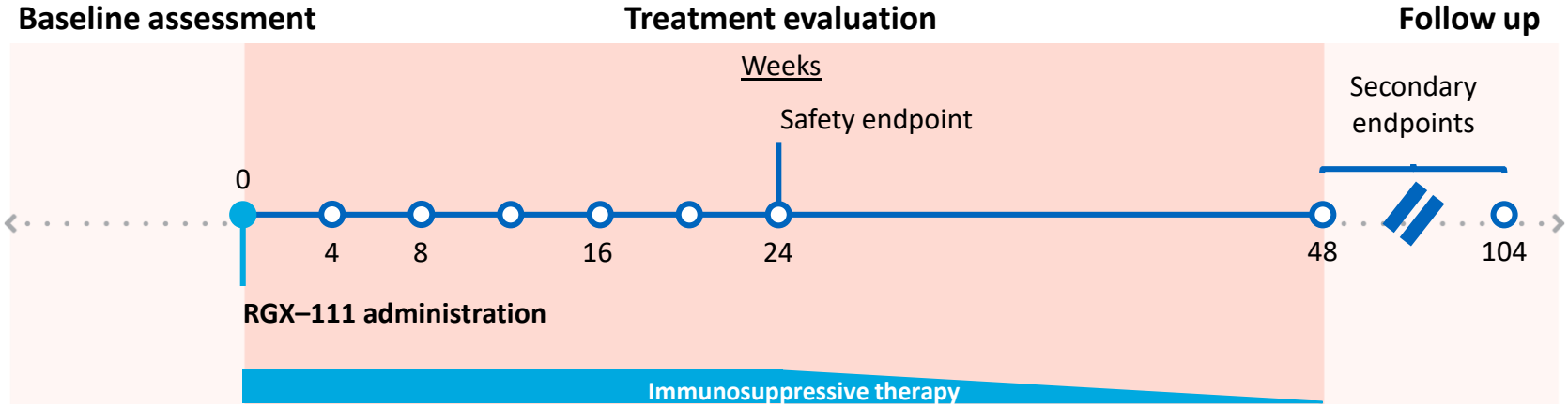
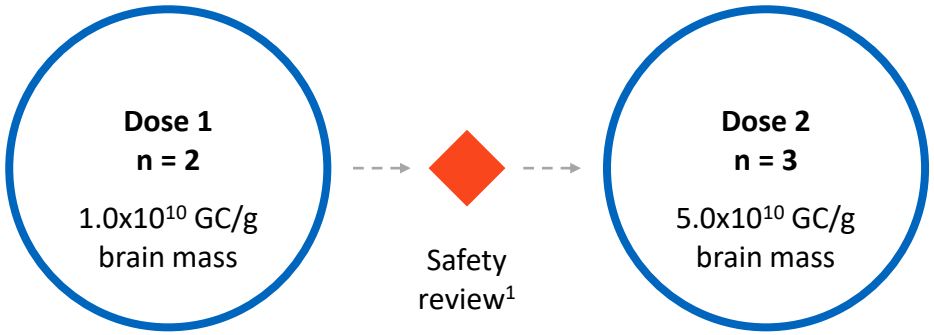
RGX–111 Phase I/II clinical trial: Administration and dose escalation

Administration and follow-up timeline



Dose escalation



First patient dosed

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

NAV® Technology Platform

REGENXBIO®

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 open in H1 2021
- cGMP manufacturing facility expected to be operational in H1 2022; will enable production at bioreactor scales up to 2,000L
- Integrated approach will allow for more efficient development and manufacturing of product candidates

REGENXBIO's **NAV Technology Platform** has been widely adopted

Multiple clinical stage programs being developed by NAV Technology Licensees across a broad range of therapeutic areas

	Research		Preclinical		Phase I / II		Phase III / Approved	
	Indication	Licensee	Indication	Licensee	Indication	Licensee	Indication	Licensee
Liver / hematologic	Undisclosed	ultragenyx			Hemophilia A	Takeda		
					Hemophilia A	ultragenyx, BAYER		
					OTC Deficiency	ultragenyx		
					GSDIa	ultragenyx		
					Wilson Disease	ultragenyx		
Central nervous system	CDKL5 Deficiency	ultragenyx	Rett Syndrome	NOVARTIS	SMA Type II / III	NOVARTIS	SMA Type I*	zolgensma, NOVARTIS
	Undisclosed	Prevail	ALS SOD1	NOVARTIS	Parkinson's w/ GBA & Neuronopathic Gaucher	Prevail	MPS IIIA	LYSOGENE, SAREPTA
	TLE	CORLIEVE	Friedreich's ataxia	Pfizer	MPS IIIA	ESTEVE		
			FTD-GRN	Prevail				
			Synucleinopathies (GBA + α-Syn RNAi)	Prevail				
Cardiac / skeletal muscle					Danon Disease	rocket pharma	XLMTM	astellas
					Pompe Disease	astellas		

*Zolgensma® is approved in the U.S., EU, and other geographies for the treatment of patients with SMA

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, Chief Operations and 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, Chief Legal Officer	Lumara Health™, WELLSTAT THERAPEUTICS
Laura Coruzzi, Ph.D., J.D.	SVP, Intellectual Property	JONES DAY
Shiva Fritsch	SVP, Chief People Officer	NOVAVAX, Human Genome Sciences

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

Financial results and guidance

2020 YTD financials as of 9/30/20 (mm)

Revenue:	$133.1
R&D expense:	$119.1
G&A expense:	$46.2
Net loss:	$65.0
Basic share count:	37.4

Recent financial highlights

- Expected to report between **$515 - $530 million in cash[1] as of December 31, 2020[2]**
- YTD Q3 2020 revenues include an **$80.0 million milestone payment** recognized upon the achievement of $1.0 billion in cumulative net sales of Zolgensma
- Completed partial monetization of Zolgensma royalty stream in Q4 2020 for **gross upfront payment of $200 million**
- **Gross proceeds of over $230 million** raised from underwritten public offering completed in January 2021[3]

Program guidance and anticipated milestones

RGX–314	**Subretinal wet AMD:** ATMOSPHERE™ to begin dosing patients in Q1 2021 **Suprachoroidal wet AMD:** Interim data from AAVIATE™ Cohort 1 expected in Q3 2021 **Suprachoroidal DR:** Initial data from ALTITUDE™ expected in 2021
RGX-202	**IND submission in mid-2021**
RGX–121	**Cohort 3 of Phase I/II trial to begin enrollment in Q1 2021**
RGX–111	**First patient recently dosed in Phase I/II trial**
RGX–181	**IND submission in Q1 2021**
RGX–381	**IND, or foreign equivalent, submission in H1 2021**

Financial guidance:

REGENXBIO expects to report that as of December 31, 2020, it had between $515 and $530 million in cash,[1, 2] which it believes is sufficient to fund its operations, including the completion of its internal manufacturing capabilities and clinical advancement of its product candidates, until late 2022.

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[1] Cash includes cash, cash equivalents and marketable securities for the purposes of this presentation
[2] Actual results may differ materially from these preliminary estimates. Please see "Preliminary Financial Information" in REGENXBIO's press release dated January 5, 2021 for further information.
[3] Includes full exercise of underwriters' option to purchase additional shares. Please see REGENXBIO's press release dated January 12, 2021 for further information.

Thank You

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