Corporate Presentation

Leader in AAV Gene Therapy

10 | 21 | 2021

Forward-looking statements

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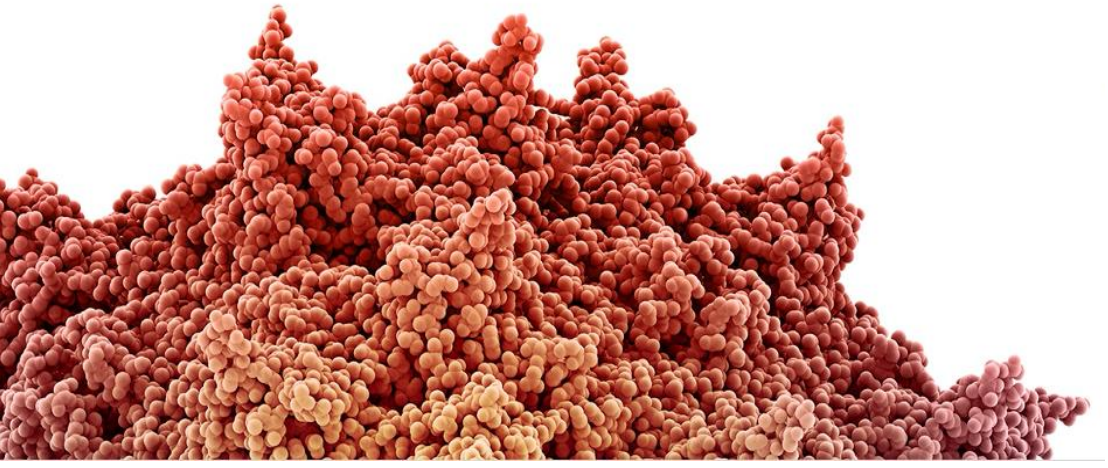
Seeking to improve lives through the curative potential of gene therapy



Advancing **broad pipeline of clinical-stage** gene therapy programs and research

Strategic partnership with AbbVie[1] to develop and commercialize gene therapy treatments for retinal disease

Industry-leading, robust AAV manufacturing and global supply platform

Experienced leaders in gene therapy and drug development

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

Strong balance sheet

[1] The transaction is expected to close by the end of 2021, subject to the satisfaction of customary closing conditions, including applicable regulatory approvals.

REGENXBIO's internal pipeline

Indication	Development Stage				Commercial Rights
	Research	Preclinical	Phase I / II	Pivotal	
Retinal Disease					
wet AMD	RGX-314 Subretinal				abbvie U.S. Equal Profit Share Ex-U.S. Tiered Royalties[1]
wet AMD	RGX-314 Suprachoroidal				
Diabetic retinopathy	RGX-314 Suprachoroidal				
Add'l anti-VEGF treated conditions					
CLN2 disease ▲ ★	RGX-381				Worldwide
Neuromuscular Disease					
Duchenne muscular dystrophy	RGX-202				Worldwide
Neurodegenerative Disease					
MPS II ▲ ★ ■	RGX-121				Worldwide
MPS I ▲ ★ ■	RGX-111				Worldwide
CLN2 disease ▲ ★	RGX-181				Worldwide
Tauopathies and α-synucleinopathies					neurimmune Promind antibody therapeutics Co-Commercialization
Liver-directed					
Hereditary angioedema					Worldwide

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

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

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[1] The transaction is expected to close by the end of 2021, subject to the satisfaction of customary closing conditions, including applicable regulatory approvals.

Internal Pipeline

Strategic partnership with AbbVie to develop and commercialize RGX-314, a potential one-time gene therapy for treatment of wet AMD and diabetic retinopathy

REGENXBIO

- Leadership and expertise in AAV and retinal gene therapy
- Strong in-house capabilities of AAV manufacturing

abbvie

- Leading eye care company
- Global development and commercial infrastructure

Details of Partnership[1]

- **$370 million upfront payment** with up to **$1.38 billion in additional development, regulatory and commercial milestones**
- Collaboration for the development and commercialization of RGX-314 with **equal share of profits in U.S. and REGENXBIO to receive royalties outside the U.S.**
- **REGENXBIO will lead the manufacturing of RGX-314** for clinical development and U.S. commercial supply

[1] The transaction is expected to close by the end of 2021, subject to the satisfaction of customary closing conditions, including applicable regulatory approvals.

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

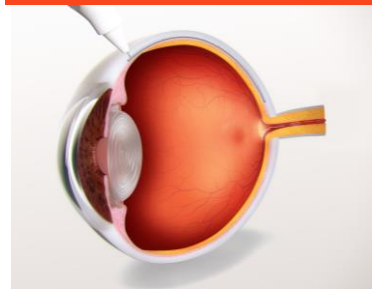
Mechanism of action

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

Routes of administration

Subretinal (SR)

Suprachoroidal (SC)



RGX–314 for treatment of wet AMD: Current program status

Phase I/IIa subretinal dose-escalation study

- RGX-314 generally well-tolerated across all doses
- Long-term, durable treatment effect demonstrated over 2 years (Cohorts 4&5)[1] and 3 years (Cohort 3)[2] post-RGX-314 administration
  - Stable to improved visual acuity and central retinal thickness
  - Meaningful reductions in anti-VEGF injection burden

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

- Pivotal program to enroll a total of approximately 700 patients
- First trial, ATMOSPHERE™, is active and enrolling; a second pivotal trial is planned to initiate in Q4 2021
- cGMP manufacturing process bridging study is active, expected to support BLA filing

Phase II suprachoroidal AAVIATE® trial on-going

- RGX-314 well tolerated in 50 patients in Cohorts 1-3 with no drug-related serious adverse events[3]
- Positive initial data presented3 from Cohort 1 (dose level: $2.5x10^{11}$ GC/eye) at six months after one-time treatment of RGX-314
  - Treatment effect observed with stable visual acuity and retinal thickness
  - Demonstrated meaningful reduction in anti-VEGF treatment burden
- Cohorts 2&3 enrollment complete (dose level: $5x10^{11}$ GC/eye)
- Trial expanded to include third dose level of RGX-314 ($1x10^{12}$ GC/eye)

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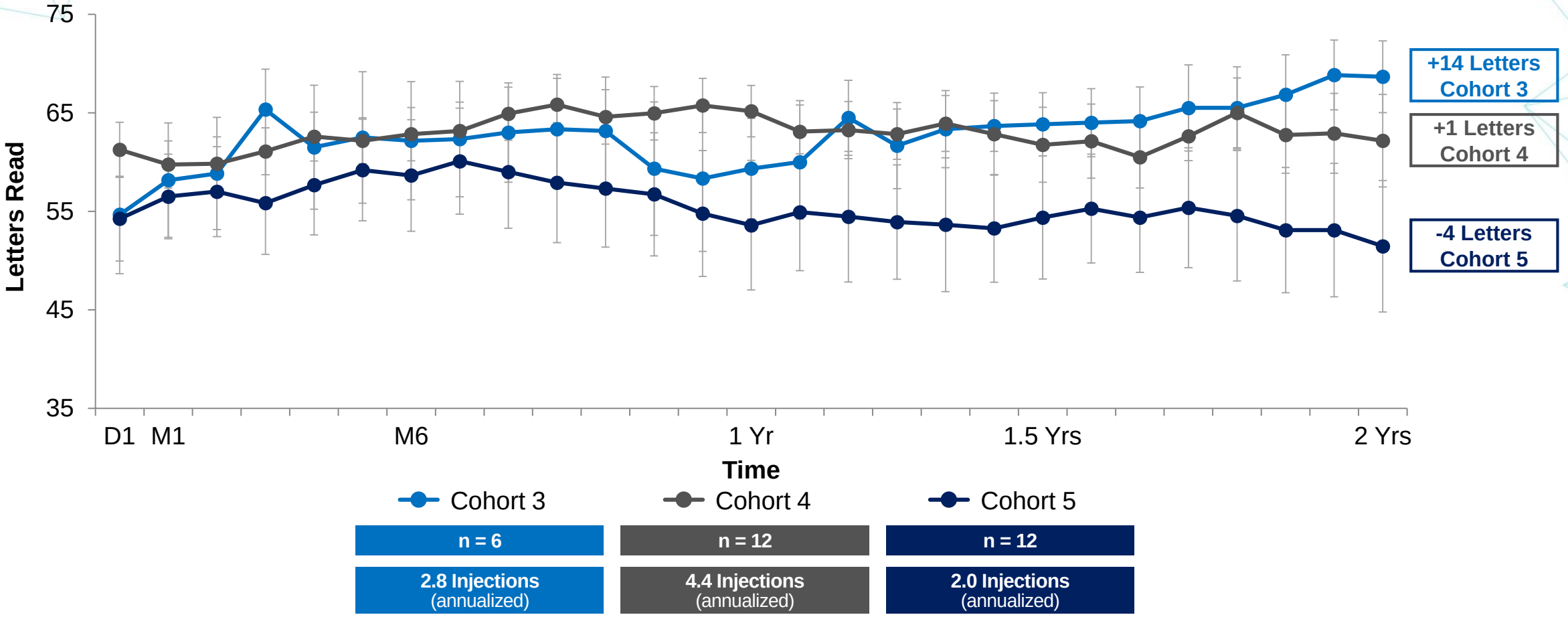
[1]Last data cut as of August 9, 2021
[2]Last data cut as of January 22, 2021
[3]As announced October 1, 2021

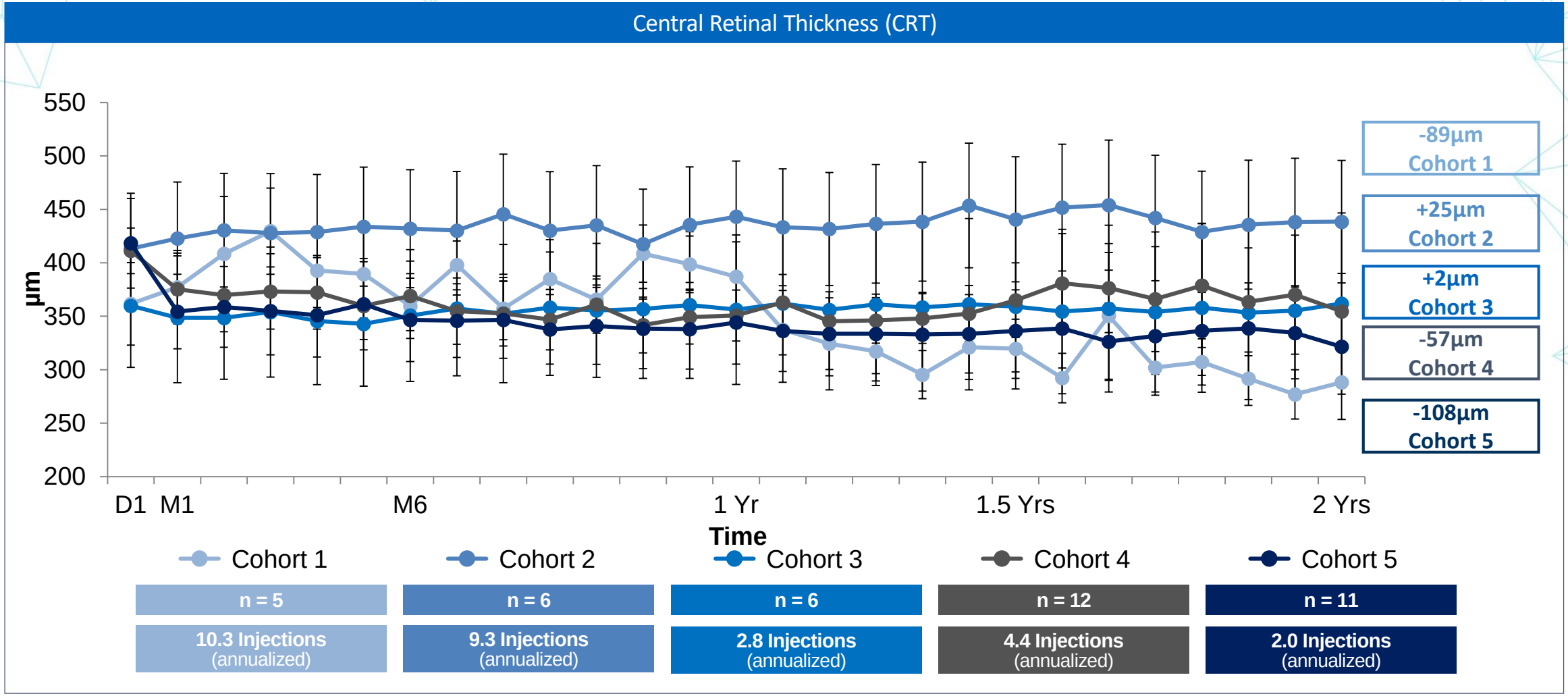
RGX–314 subretinal Phase I/IIa clinical trial:
Mean BCVA over 2 years in Cohorts 3-5

Best Corrected Visual Acuity (BCVA)



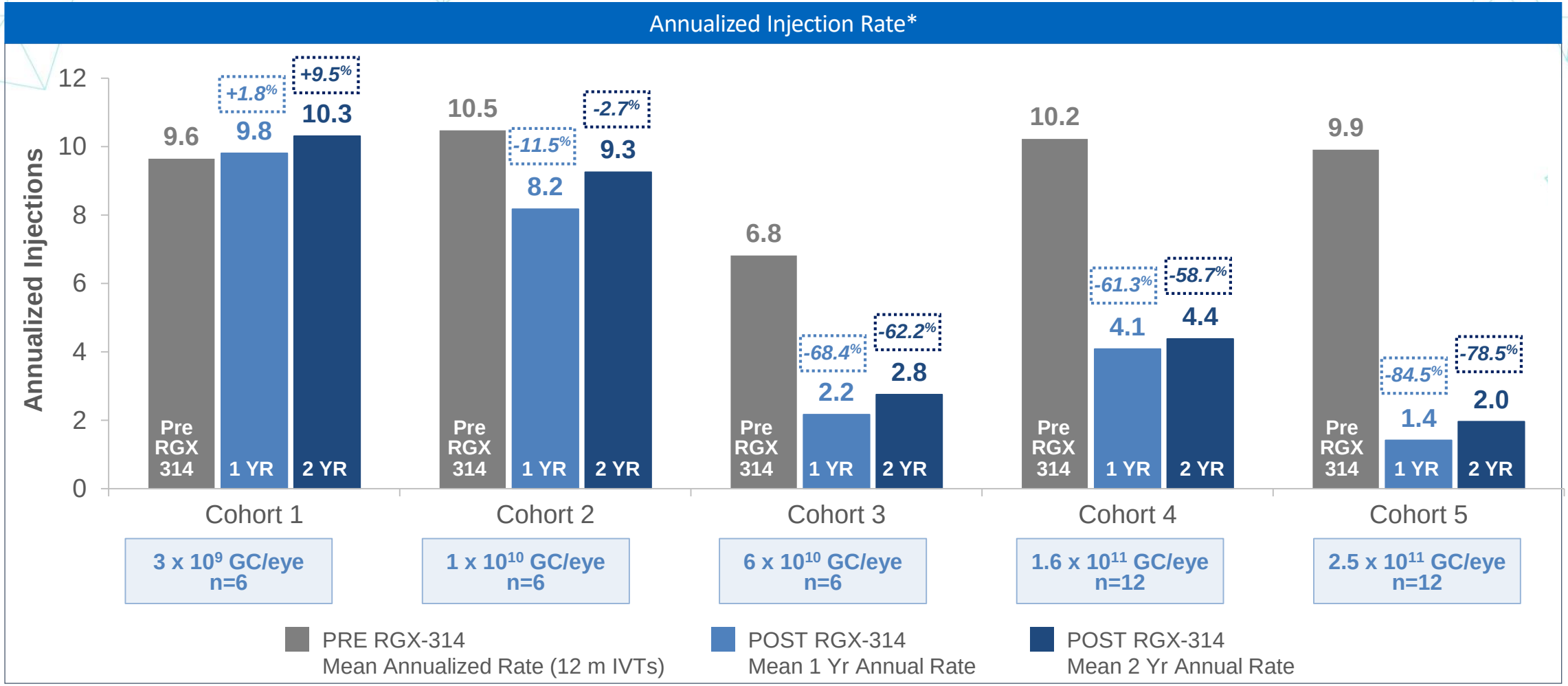
* One patient in Cohort 5 discontinued the study prior to the Week 22 visit and missing data post discontinuation was not imputed. Another patient in Cohort 5 has missed the visits due to COVID-19 from Week 50 through Week 74 and from Week 86 through Week 94. For this patient, missing visits were imputed using last observation carried forward (LOCF). Ten additional missing BCVA results were interpolated.

RGX–314 subretinal Phase I/IIa clinical trial:
Mean CRT over 2 years



Note: One patient in Cohort 1 and one patient in Cohort 5 discontinued the study prior to Week 22 visit. Another patient in Cohort 5 has missed the visits due to COVID-19 from Week 50 through Week 74 and from Week 86 through Week 94. For this patient, missing visits were imputed using last observation carried forward (LOCF). Seventeen additional missing CRT results were interpolated.

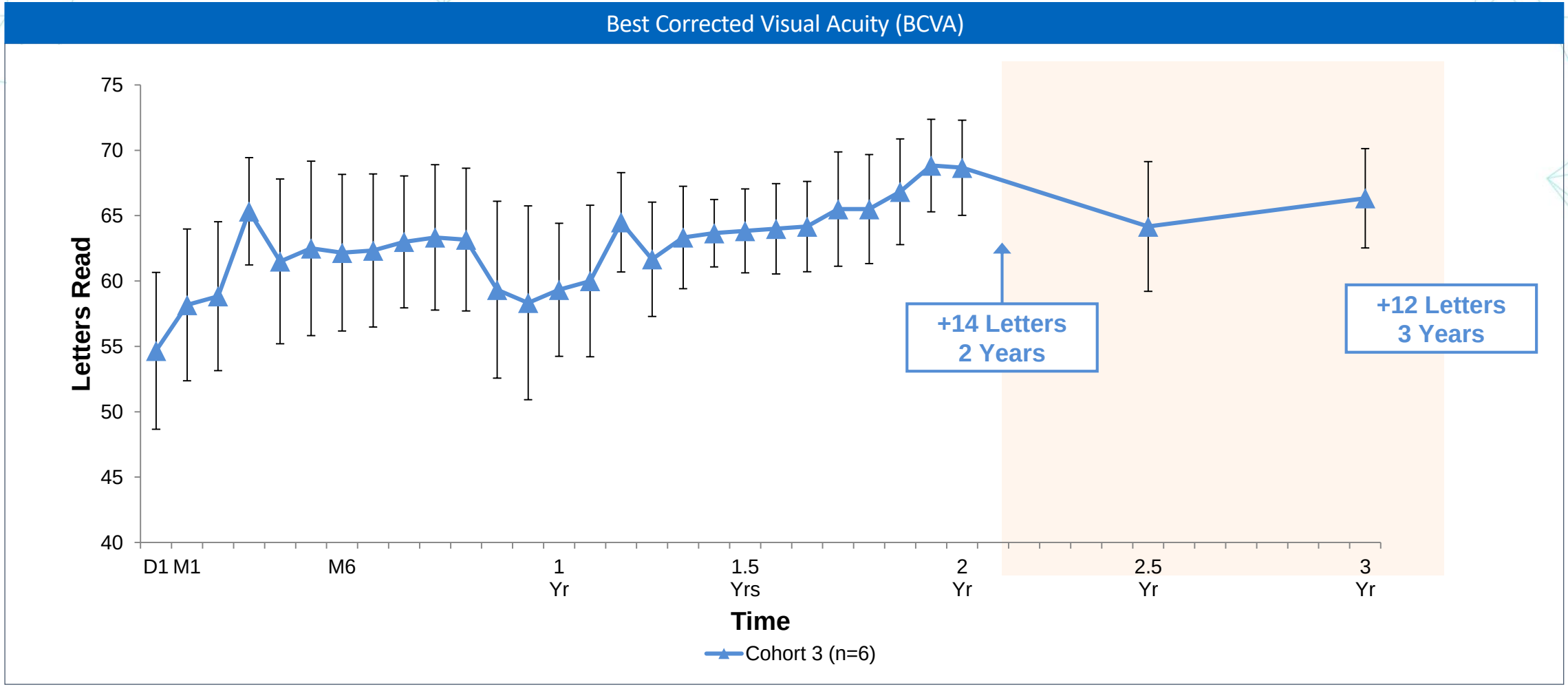
RGX–314 subretinal Phase I/IIa clinical trial:
Mean Change in Annualized Injection Rate PRE and POST RGX-314 in Cohorts 1–5



Retreatment Criteria: Any CNV-related increased, new, or persistent fluid; Vision loss of ≥5 letters associated with fluid; New ocular hemorrhage

* 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 defined from RGX-314 administration to a specified cut-off date.

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



* 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

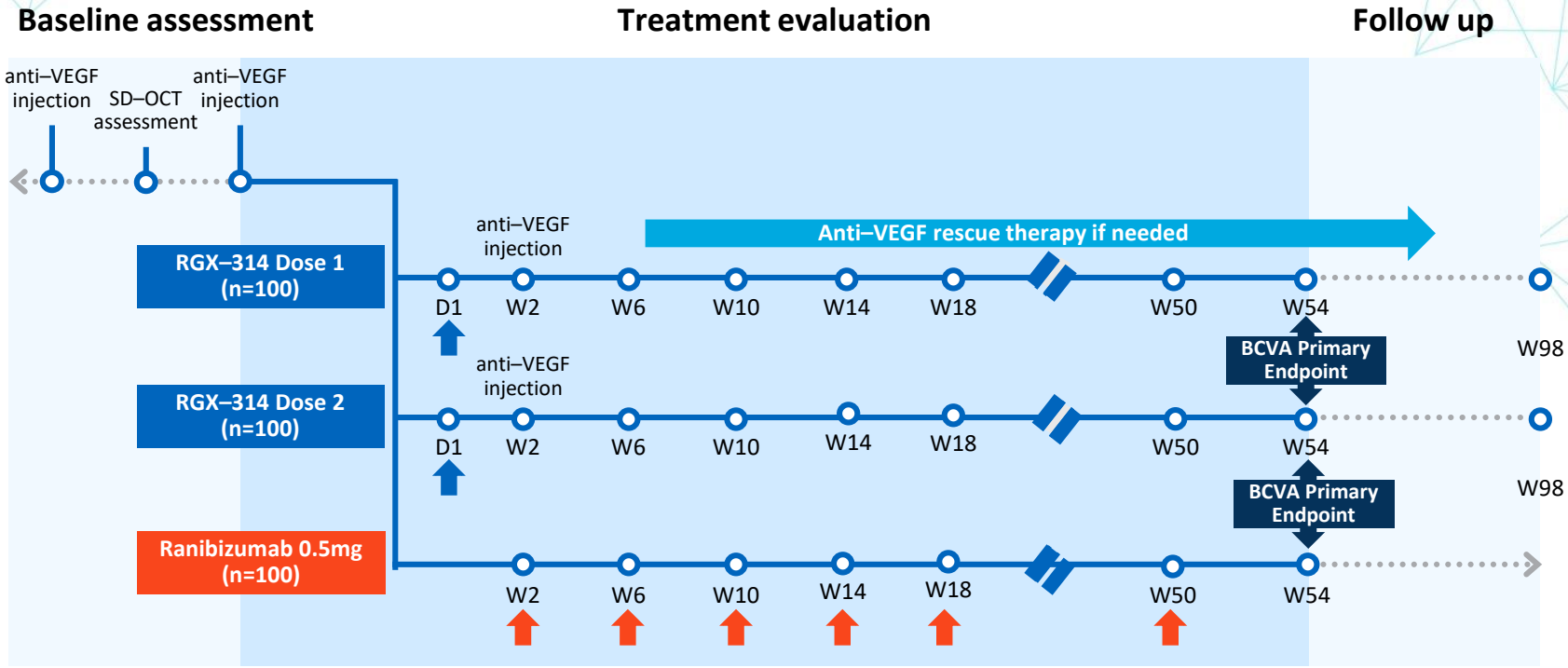
Sites: Up to 60 leading retinal surgery centers across the United States

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)

ATMOSPHERE™ pivotal trial design

Administration and follow-up timeline



Arms and Interventions

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

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 Month 9

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 95 total

Route of administration: Suprachoroidal using SCS Microinjector

Sites: Fifteen leading retinal centers across the United States

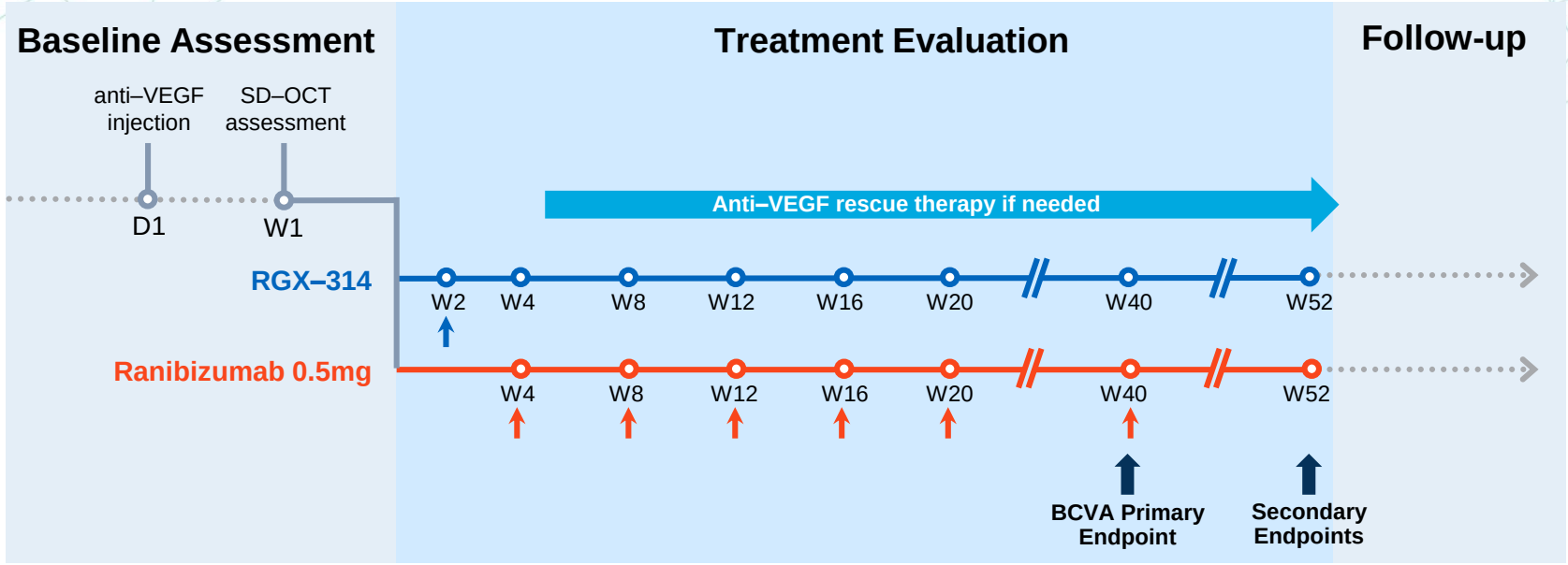
REGENXBIO®

KEY INCLUSION CRITERIA

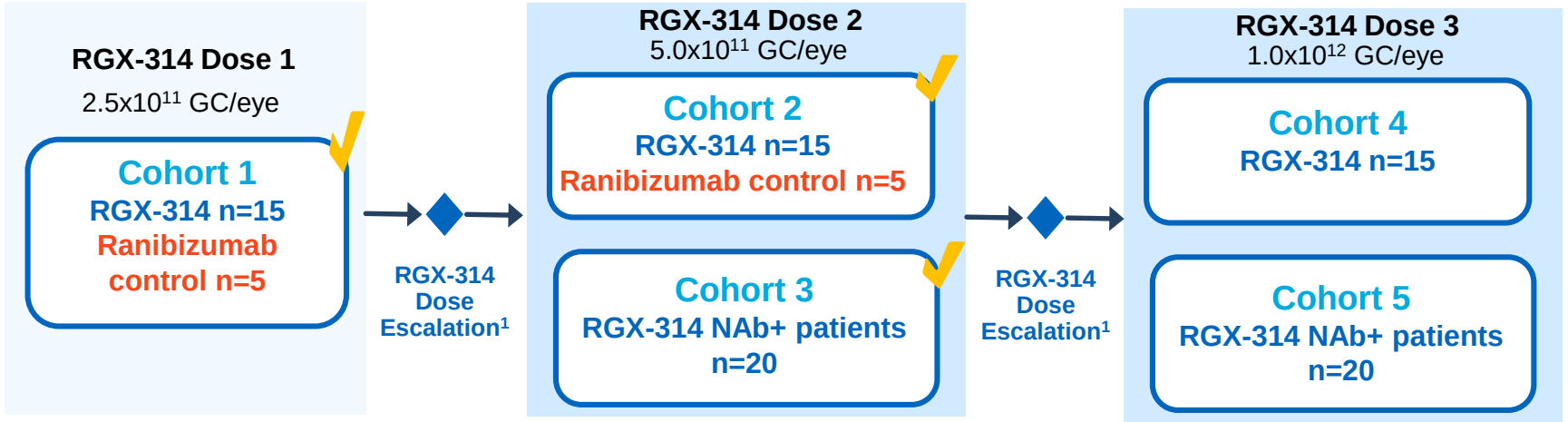
- Male or female ≥50 to 89 years of age
- Previously treated wet AMD subjects with fluid on OCT at trial entry
- Documented response to anti–VEGF at trial entry (assessed by reading center)
- BCVA between ≤ 20/25 and ≥ 20/125 (≤ 83 and ≥ 44 Early Treatment Diabetic Retinopathy Study [ETDRS] letters) in the study eye
- Phakic or pseudophakic

AAVIATE® Phase II clinical trial design

Administration and follow-up timeline



Dose escalation

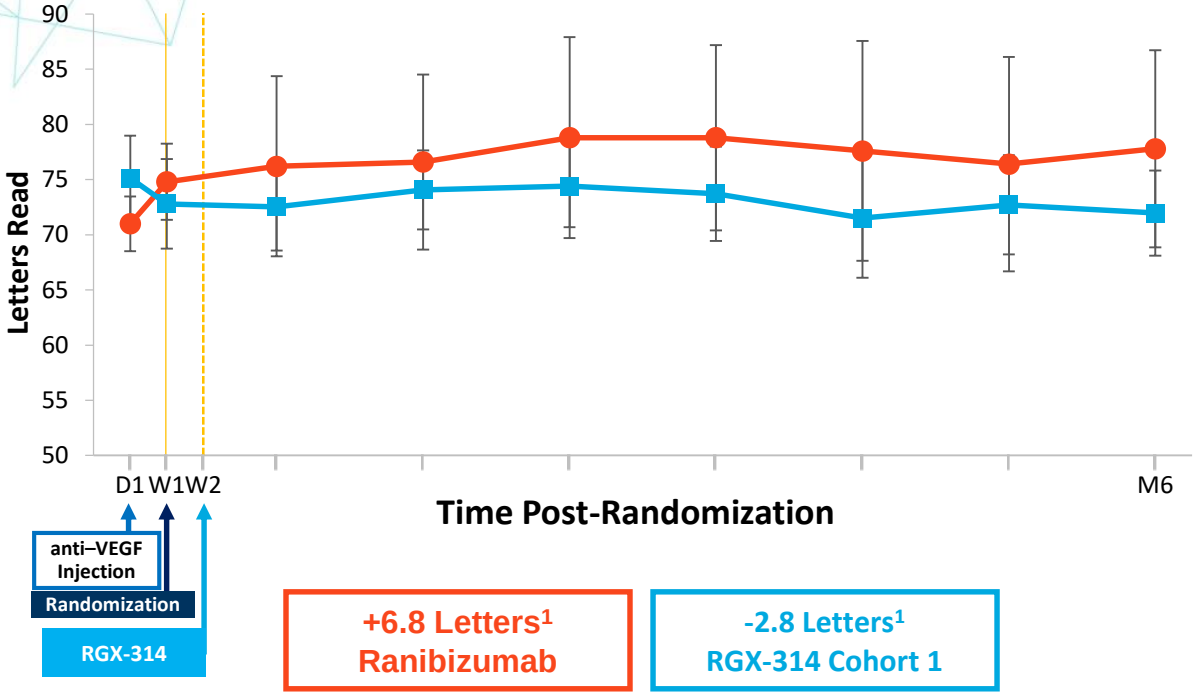


Cohorts 4 & 5 currently enrolling patients

[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
NAb+ = AAV8 neutralizing antibody positive

Cohort 1: Mean BCVA and CRT from Day 1 (Screening) Through Month 6

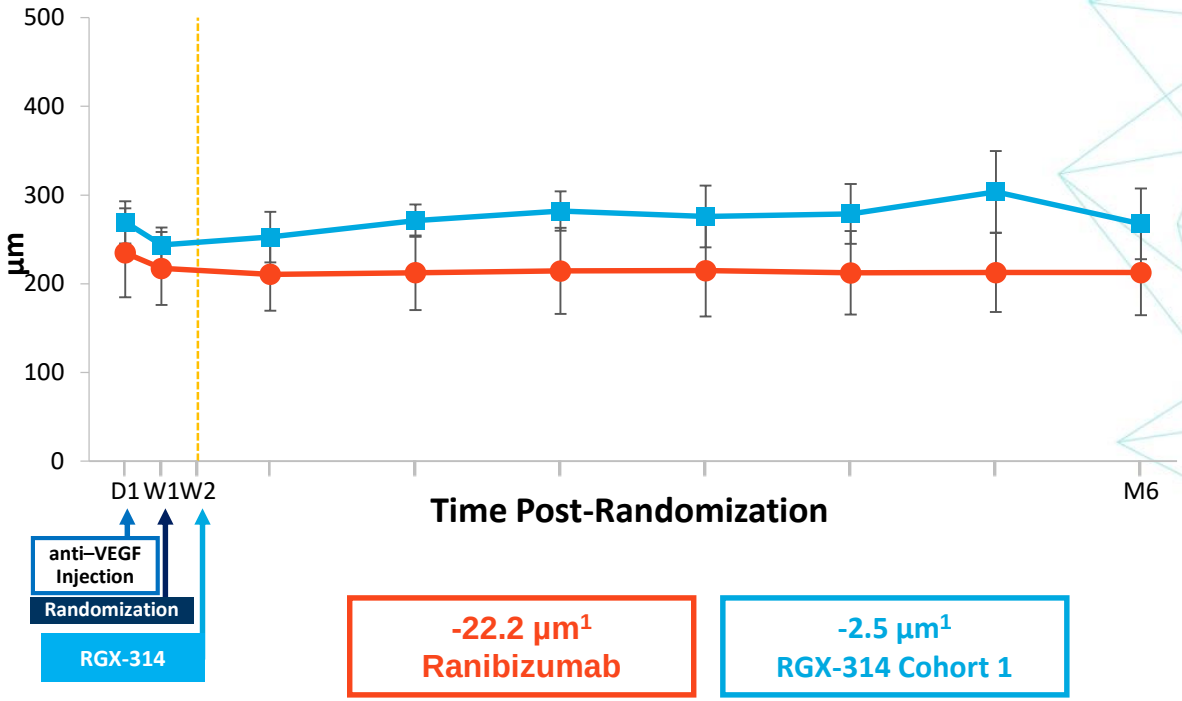
Best Corrected Visual Acuity (BCVA) 95% CI



+6.8 Letters[1] Ranibizumab

-2.8 Letters[1] RGX-314 Cohort 1

Central Retinal Thickness (CRT) 95% CI



-22.2 µm[1] Ranibizumab

-2.5 µm[1] RGX-314 Cohort 1

Ranibizumab Q4W (n=5)

RGX-314 (n=15[2])

7 Injections

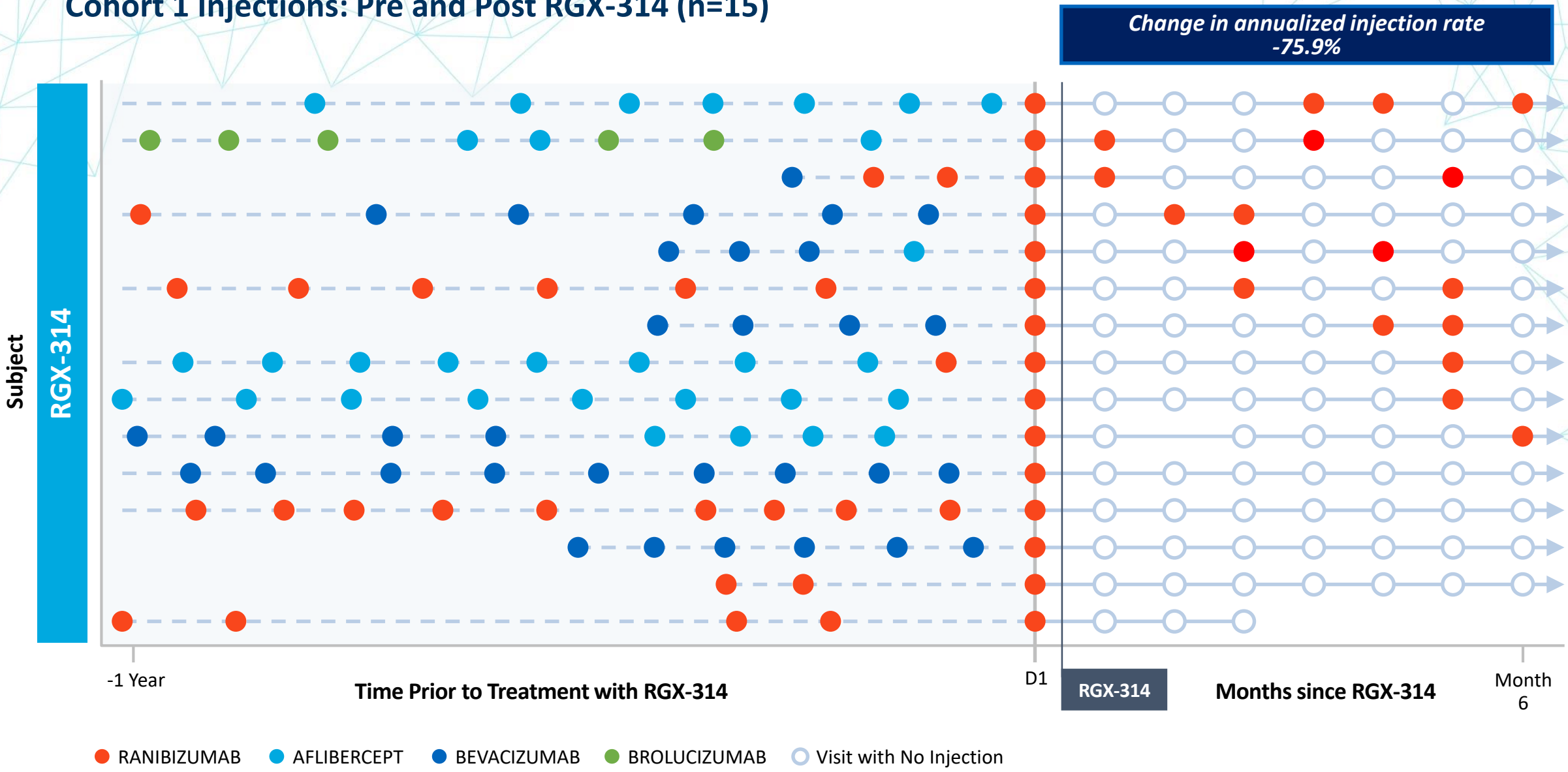
1.2 Injections

Mean Injections Post-Randomization

Data cut Sep 13, 2021

1. Values are mean change from Day 1.
2. One patient discontinued the study after Week 12, and only data up to week 12 is included for the subject. For one patient who has missing Weeks 8 and 28 visits, the missing data has been interpolated using the average of before and after the missing visit.

Cohort 1 Injections: Pre and Post RGX-314 (n=15)



Change in annualized injection rate is the difference between historical annualized injection rate and on-study annualized injection rate up to 6 months post-RGX-314.
Historical annualized injection rate is (Total # of prior injections)/(minimum(366 days, Duration between first injection and Day 1)/365.25).
On-study annualized injection rate is (Total # of injections on Study)/(Duration on Study/365.25) where on-study is defined from post-D1 to a specified cut-off date.

AAVIATE Safety Summary

RGX–314 was well–tolerated in Cohorts 1–3 (n=50) with follow-up ranging from 1 month – 12 months

- 4 SAEs: None were considered drug-related:
  - One death resulting from a complete atrioventricular block (Cohort 1)
  - One hospitalization due to intestinal obstruction (Cohort 1)
  - One CVA (Cohort 2)
  - One gastric ulcer (Cohort 3)

RGX-314: Cohort 1 (n=15)

- Common ocular TEAEs[1] in the study eye were generally mild with none severe:
  - Conjunctival Hemorrhage (5/15, 33%)
  - Mild Intraocular Inflammation[2] (4/15, 27%) observed on slit-lamp examination
    - All cases resolved within days to weeks on topical corticosteroids which have been discontinued
  - Worsening of nAMD[3] (3/15, 20%)
  - Conjunctival Hyperemia (2/15, 13%)
  - Dry Eye (2/15, 13%)
- No cases of chorioretinal vasculitis or occlusion, or hypotony were observed

Data cut Sep 13, 2021
1. Common ocular TEAEs defined as ≥ 10% of RGX-314 treated study eyes
2. 3 patients presented with anterior cell (+0.5, +2, +2) and 1 patient presented with vitreous cell (trace); onset range was 2-6 weeks post-dosing
3. All reported from one investigator at one site
CVA: Cerebrovascular accident; SAE: Serious Adverse Event; TEAE: Treatment Emergent Adverse Event

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 (SC)

REGENXBIO

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 1 year

Secondary

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

Subjects: Up to 60 total

Route of administration: Suprachoroidal using SCS Microinjector

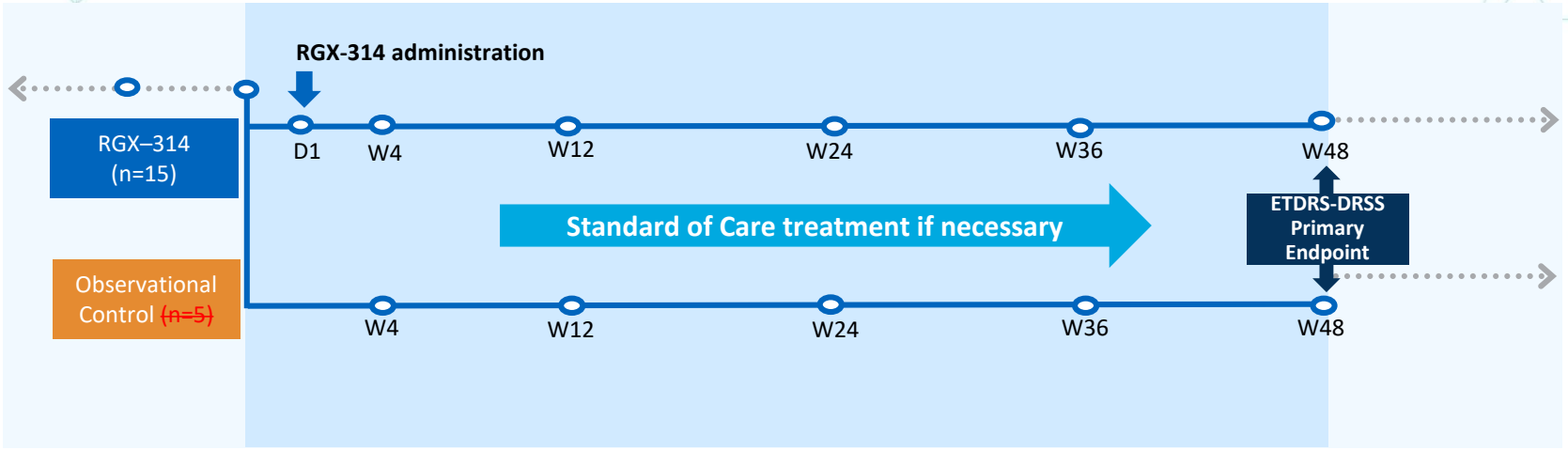
Sites: Eighteen leading retinal centers across the United States

KEY INCLUSION CRITERIA

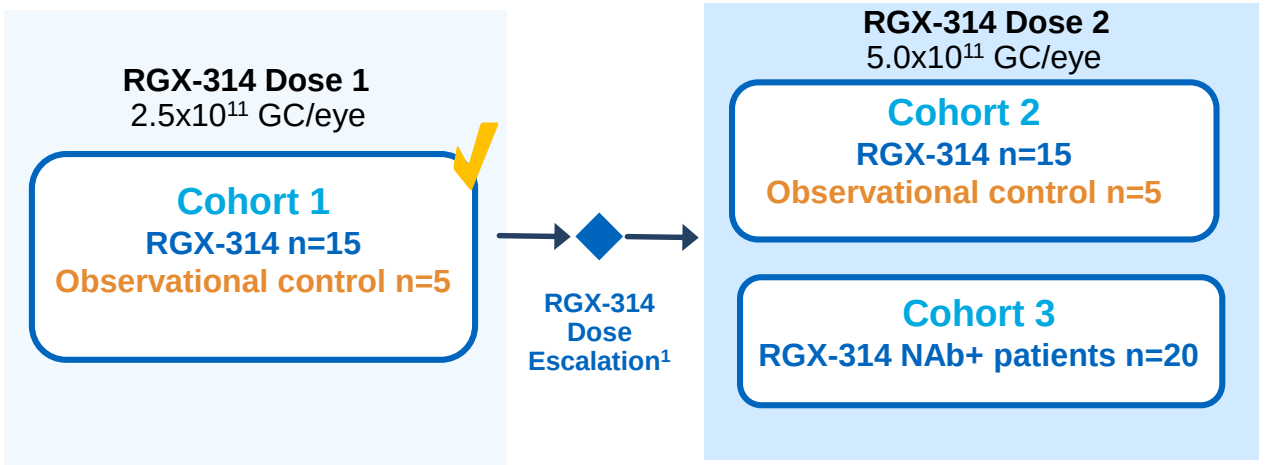
- Male or female ≥ 25 to 89 years of age with DR secondary to diabetes mellitus Type 1 or Type 2
- Moderately severe NPDR, severe NPDR, or Mild PDR (DRSS levels 47-61)
- No active CI-DME, CST < 320 µm
- Vision of 20/40 or better (≥ 69 Early Treatment Diabetic Retinopathy Study [ETDRS] letters) in the study eye
- No anti-VEGF injection(s) in prior 6 months

ALTITUDE™ Phase II clinical trial design

Administration and follow-up timeline



Dose escalation



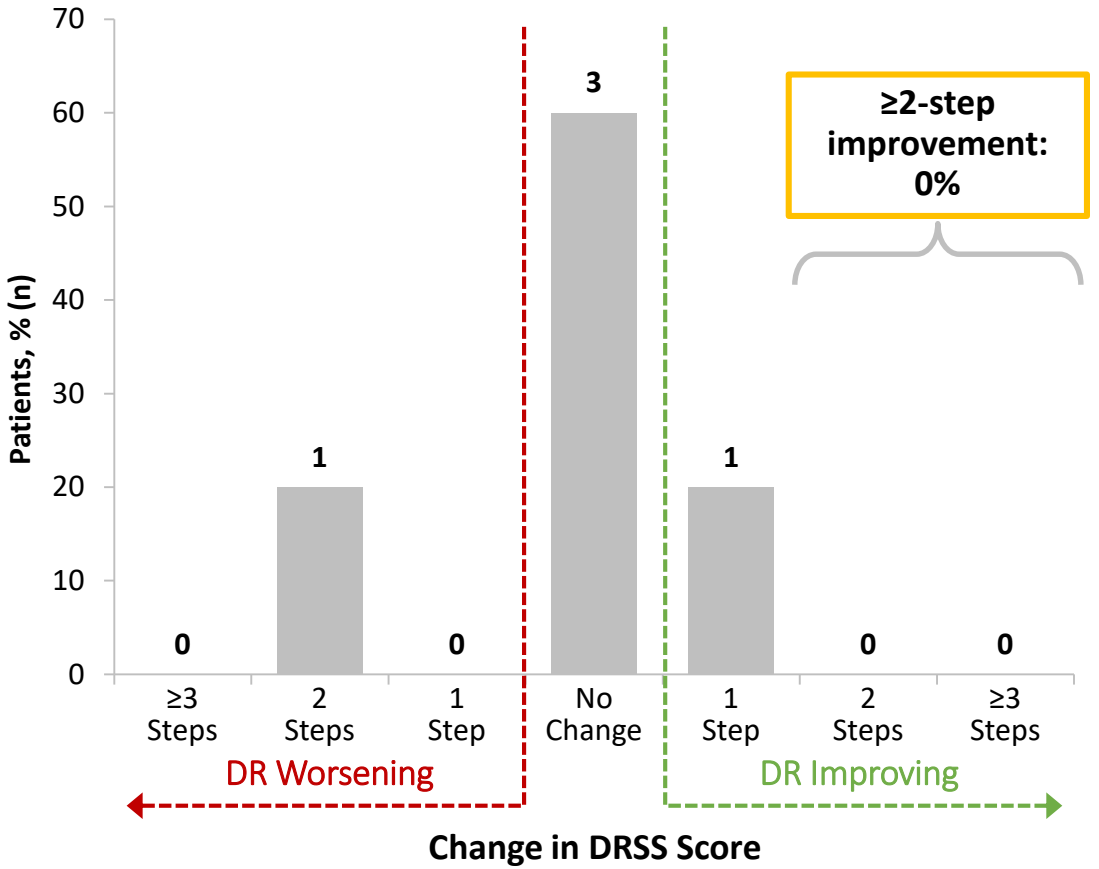
Cohorts 2 & 3 currently enrolling patients

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

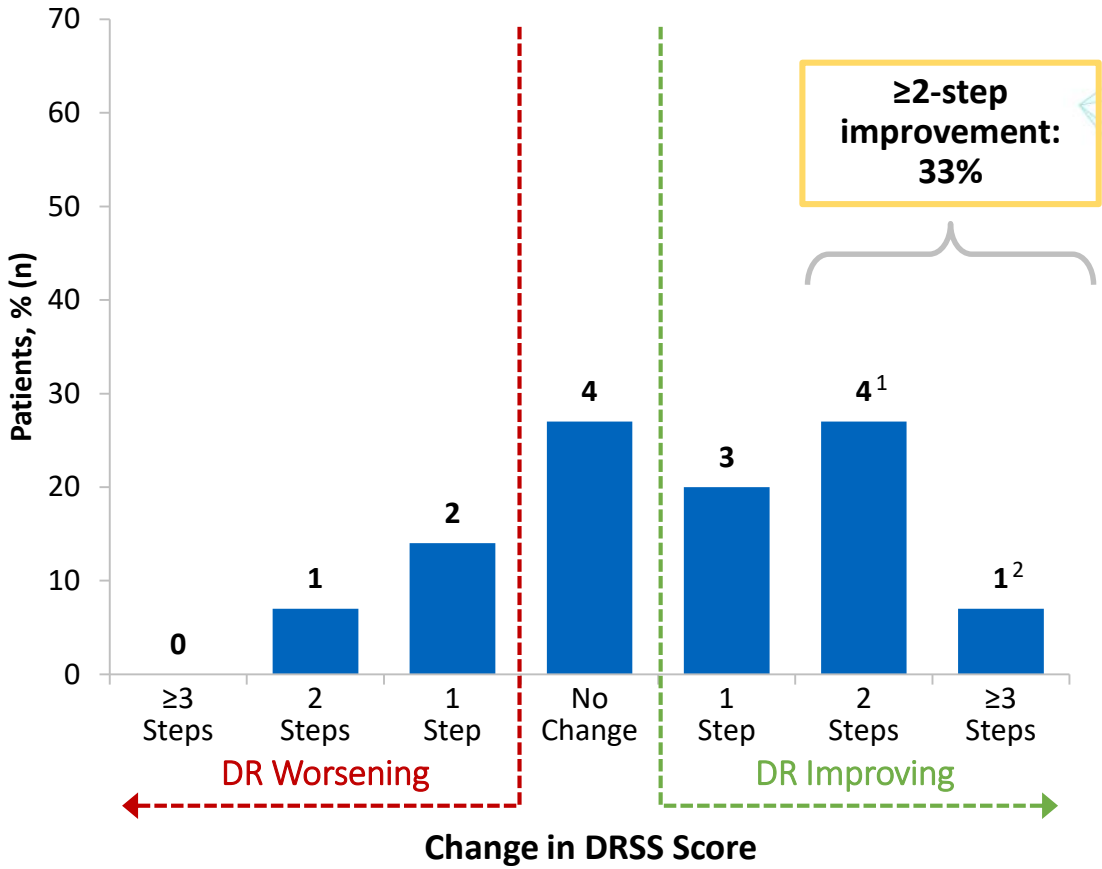
Cohort 1: Change in DRSS at Month 3

33% of RGX-314 Treated Patients Achieved a ≥2-Step Improvement

Observational Control (n=5)



RGX-314 (n=15)



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Data cut Sep 29, 2021

1. One study eye (DRSS 61 at baseline) received a single Lucentis injection 8 days after RGX-314 dosing for trace vitreous hemorrhage, which was 10 weeks prior to their 3 month visit when DRSS was assessed.
2. One patient had a 4-step improvement.

ALTITUDE Safety Summary: Cohort 1

RGX–314 was well-tolerated (n=15)

- 1 SAE that was not considered drug-related:
  - Vitreous hemorrhage in an untreated *fellow eye*

Common ocular TEAEs[1] in the study eye were not considered drug-related and were predominantly mild:

- Conjunctival hyperemia (2/15, 13%)
- Conjunctival hemorrhage (2/15, 13%)

One case of mild episcleritis[2] that resolved with topical corticosteroids

No intraocular inflammation observed on slit-lamp examination

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Data cut Sep 29, 2021
1. Common ocular TEAEs defined as ≥ 10% of RGX-314 treated study eyes.
2. Onset was 2-weeks post-dosing.
SAE: Serious Adverse Event; TEAE: Treatment Emergent Adverse Event.

RGX–202 for treatment of Duchenne Muscular Dystrophy

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

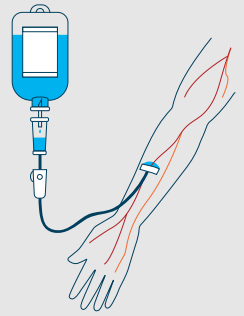
Gene: microdystrophin

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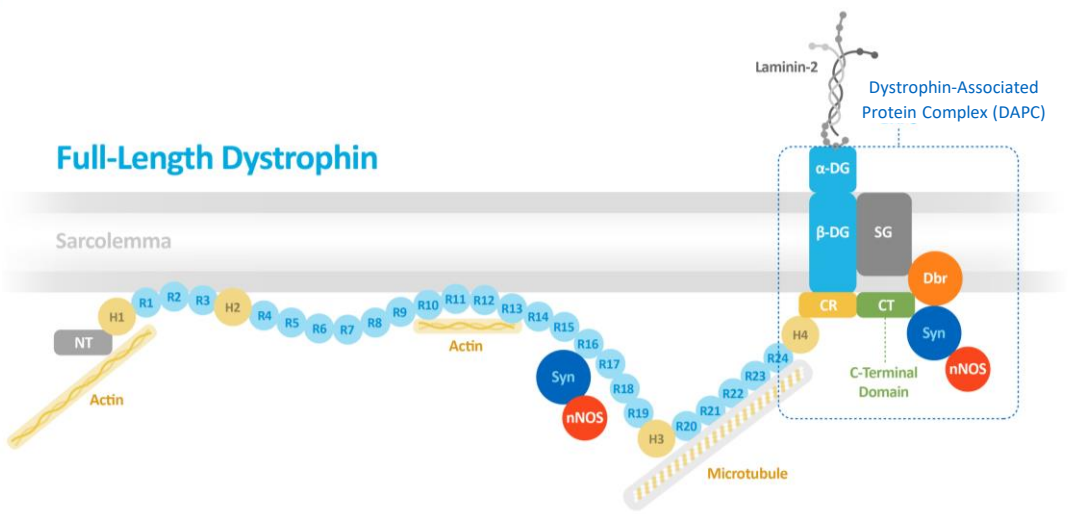
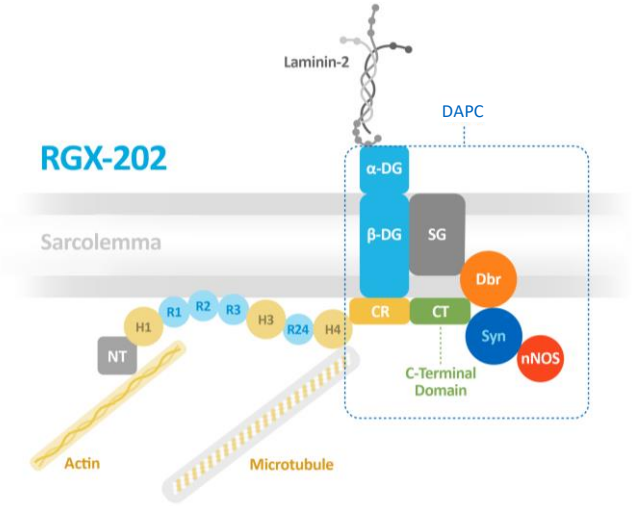
RGX-202 is designed to retain key elements of full-length dystrophin

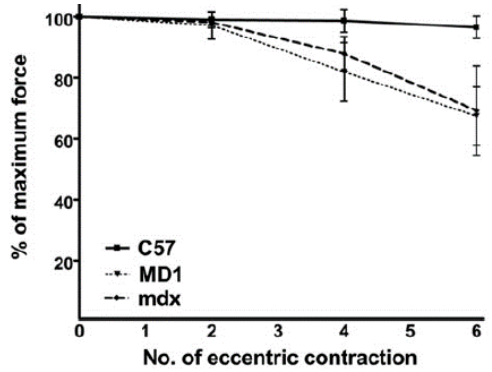
CT Domain has been shown to recruit several key proteins to the muscle cell membrane (sarcolemma) including Syntrophin and Dystrobrevin, Neuronal nitric oxide synthase and other proteins[1]



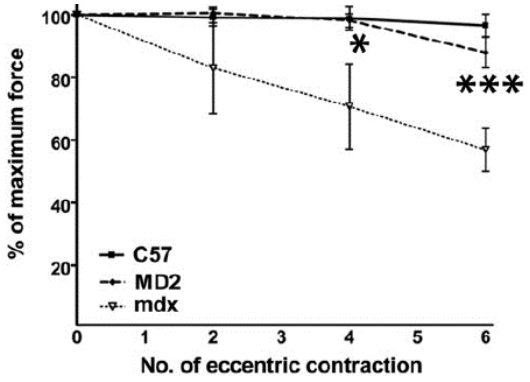


Presence of CT Domain in microdystrophin significantly improved the muscle resistance to lengthening contraction–induced muscle damage in DMD^{mdx} mice[2]

Construct without C-Terminal Domain



Construct with C-Terminal Domain



[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

[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

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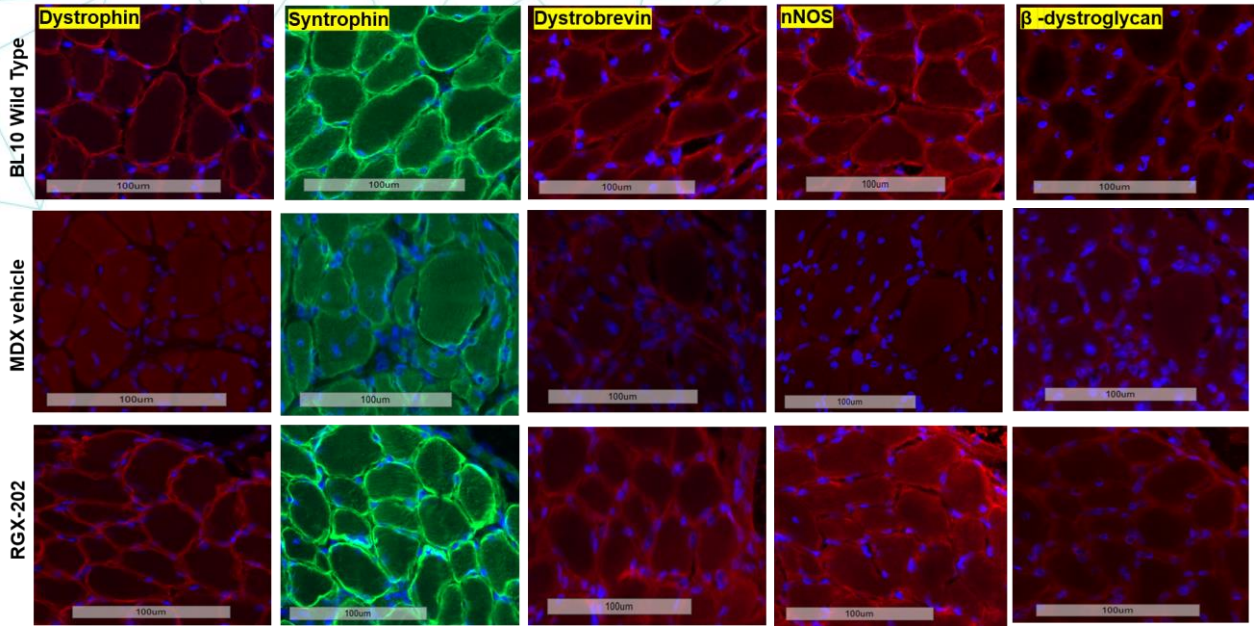
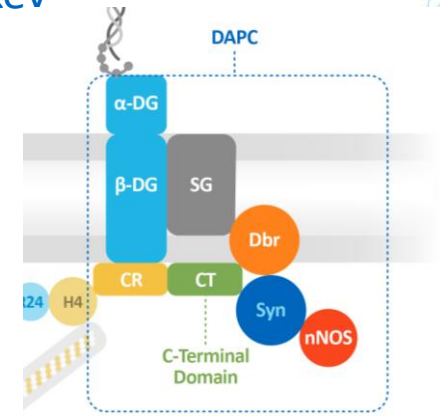
RGX-202 Proof of concept in DMD^{mdx} mouse model

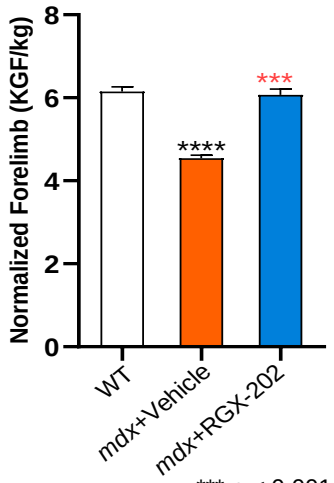


Histological evidence that RGX-202 recruits kev proteins to DAPC

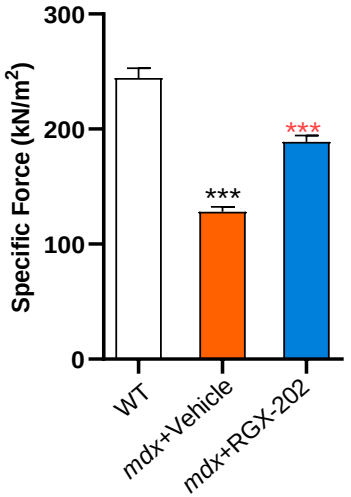


Significant strength and force improvements observed in DMD^{mdx} mice treated with RGX-202

Mouse Grip Strength (In-Life)



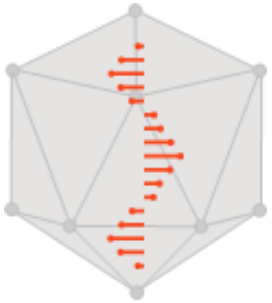
***Ex Vivo* Force Measurements**

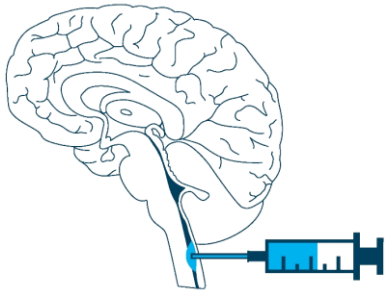


*** p < 0.001 vs wild-type historical control data;
*** p < 0.001 vs vehicle control *mdx*; student's t-test was used. Data are presented as mean ± SEM

REGENXBIO's neurodegenerative disease franchise

AAV9 vector





Intracisternal Delivery

	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 ▪ More than 500 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 ▪ More than 500 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
Gene	**IDS** Gene Replacement	**IDUA** Gene Replacement	**TPP1** Gene Replacement
Designations	▲ 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: 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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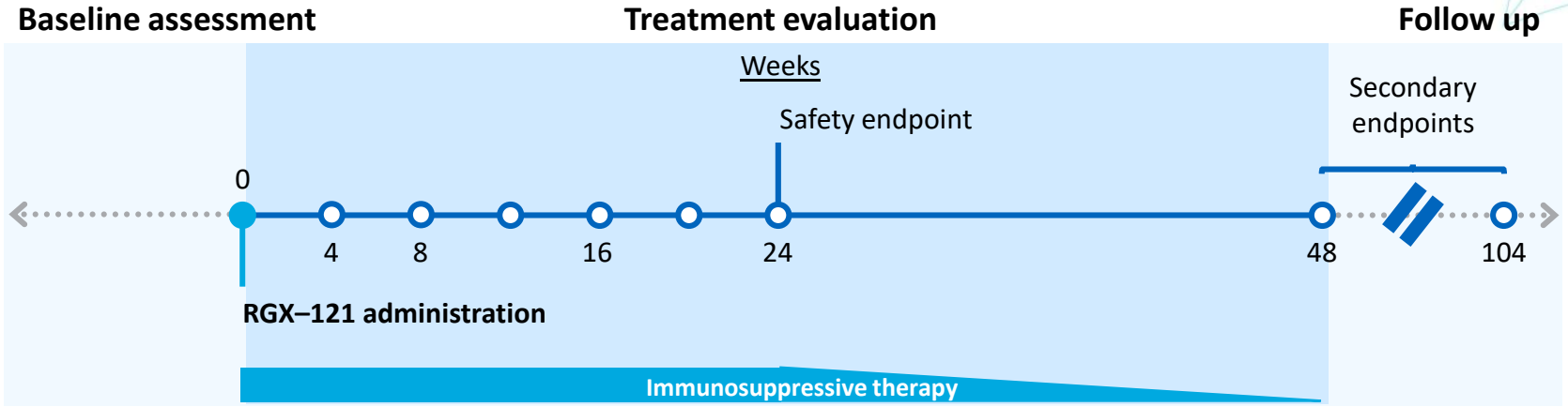
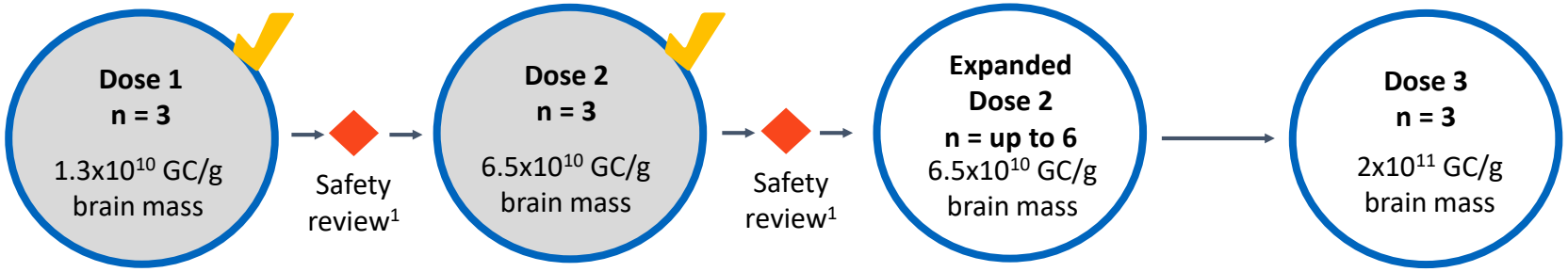
RGX–121 Phase I/II clinical trial: Administration and dose escalation

Administration and follow-up timeline



Dose escalation



Dosing in Cohort 3 is ongoing

[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 Cohorts 1 & 2 data summary[1]

- **Well-tolerated following one-time RGX-121 administration**
  - No drug-related Serious Adverse Events in 9 patients dosed in Cohorts 1-3

- **Biomarkers and measures of neurodevelopmental function indicate CNS activity in Cohorts 1 & 2 following RGX-121 administration**
  - Reductions in CSF biomarkers up to 2 years after RGX-121 administration
  - Continued cognitive development and language and/or motor skill acquisition observed

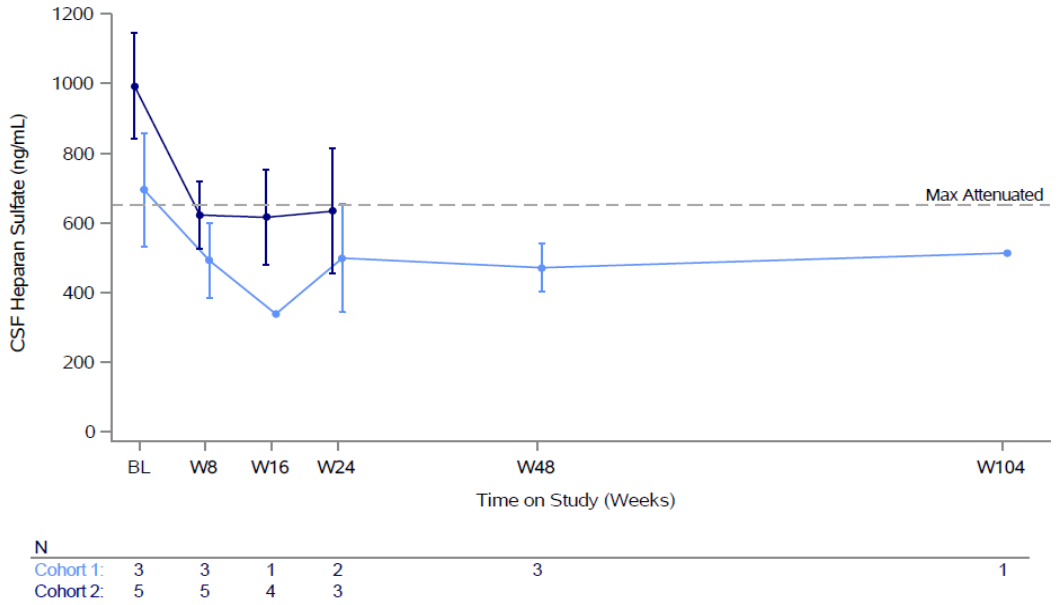
- **Emerging evidence of systemic I2S protein expression and biomarker activity in Cohorts 1 & 2**
  - Increased I2S protein concentration in plasma
  - Rapid reductions in urine biomarker levels observed in ERT[2]-naïve patients

[1] As announced May 14, 2021

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

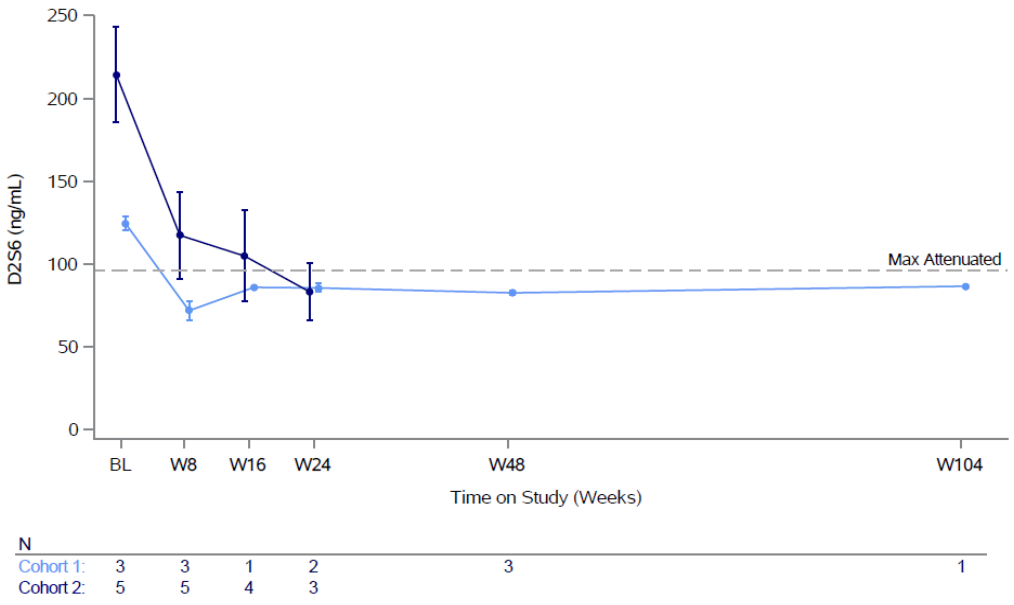
RGX–121 Phase I/II clinical trial: Reductions in CSF biomarkers up to 2 years after RGX-121 administration[1]

Heparan sulfate (HS) in cerebral spinal fluid, Mean +/- SE



Median change from baseline:
-30.3% at Week 8; -35.0% at last timepoint available (n=8)

HS D2S6 disaccharide in cerebral spinal fluid, Mean +/- SE

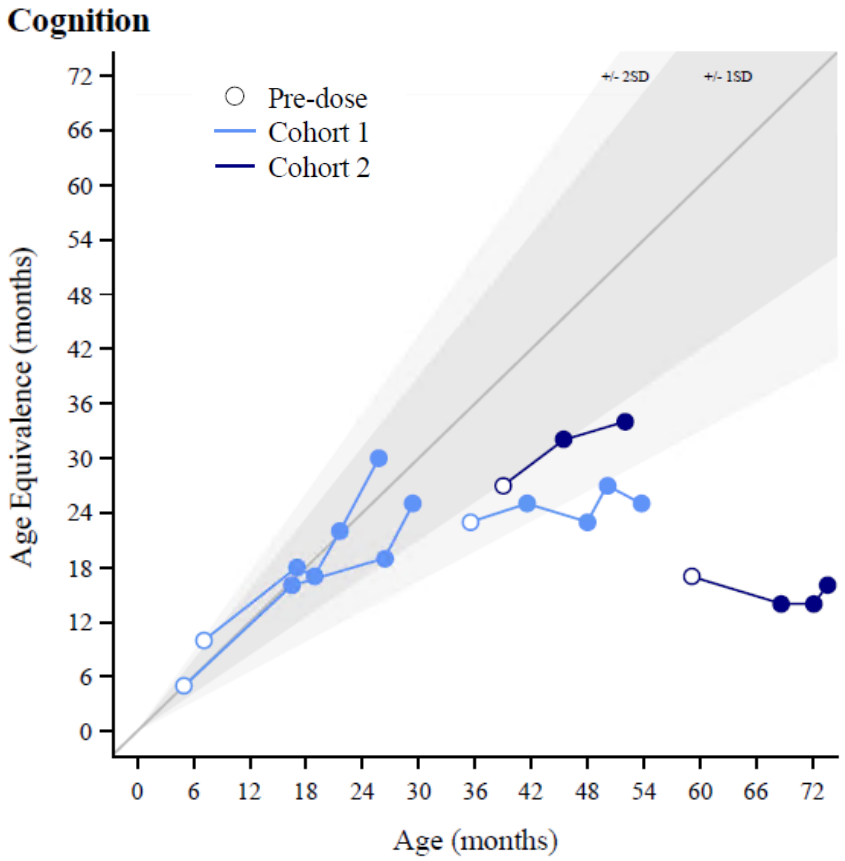


Median change from baseline:
-44.1% at Week 8; -40.4% at last timepoint available (n=8)

[1] Presented at ASGCT on May 14, 2021

RGX–121 Phase I/II clinical trial: Continued cognitive development 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)



- 4 out of 5 subjects with greater than 6 months[1] of follow-up continued cognitive development

Presented at ASGCT on May 14, 2021

[1]As of data cut-off date of April 25, 2021, 5 patients had > 6 months of follow- up: 3 in Cohort 1 and 2 in Cohort 3

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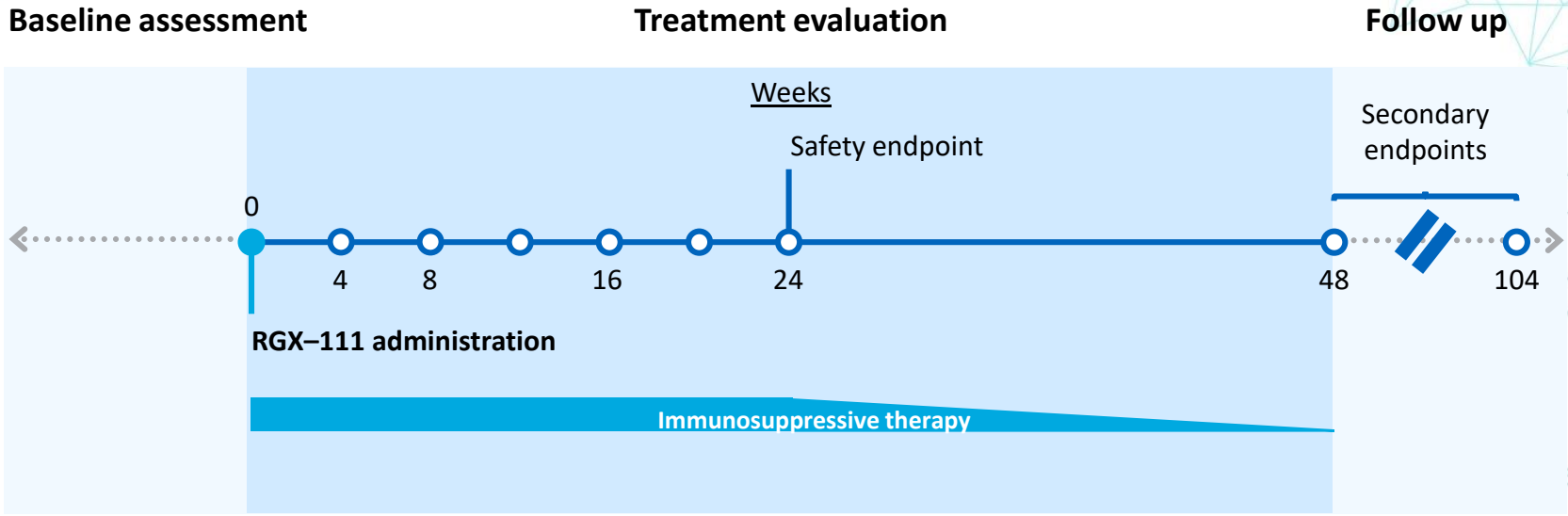
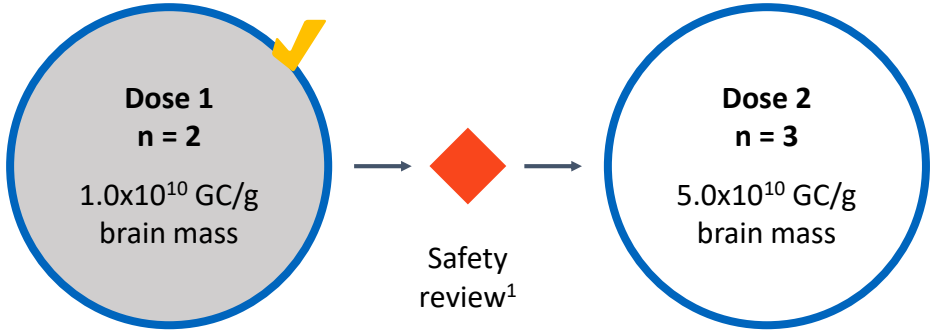
RGX–111 Phase I/II clinical trial: Administration and dose escalation

Administration and follow-up timeline



Dose escalation



Cohort 2 currently enrolling patients

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

NAV Technology Platform

The NAV Technology Platform is based on a *broad and deep IP portfolio*

Exclusive rights to more than **100 patents** and **patent applications worldwide**

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

Key features of REGENXBIO's NAV Technology Platform

- 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

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	OTC Deficiency	ultragenyx
					Hemophilia A	ultragenyx, BAYER	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	Lilly	Friedreich's ataxia	Pfizer	Parkinson's w/ GBA & Neuronopathic Gaucher	Lilly	MPS IIIA	LYSOGENE, SAREPTA THERAPEUTICS
			FTD-GRN	Lilly	MPS IIIA	ESTEVE		
			Synucleinopathies (GBA + α-Syn RNAi)	Lilly				
			TLE	uniQure				
Cardiac / skeletal muscle					Danon Disease	rocket pharma	XLMTM	astellas
					Pompe Disease	astellas		

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 open
- 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

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

Financial results and guidance

2021 YTD financials as of 6/30/21 (mm)

Revenue:	$40.9
R&D expense:	$85.6
G&A expense:	$36.3
Net loss:	$107.8
Basic share count:	42.5

2021 YTD financial highlights

- Ended Q2 2021 with **$593.0 million in cash, cash equivalents and marketable securities**
- Under terms of the partnership with AbbVie[1], REGENXBIO to **receive $370 million upfront payment, with potential to receive up to $1.38 billion in milestones**
- **Aggregate net proceeds of $216.1 million received** from follow-on offering of common stock completed in January 2021

Program guidance and anticipated milestones

RGX–314	**Subretinal wet AMD:** ATMOSPHERE™ currently enrolling patients; second pivotal trial to initiate in by end of 2021 **Suprachoroidal wet AMD:** Interim data from AAVIATE® Cohort 2 expected in Q4 2021 **Suprachoroidal DR:** Cohorts 2&3 enrollment ongoing
RGX-202	IND submission by end of 2021
RGX–121	**Phase I/II trial in patients up to 5 years old**: enrollment ongoing **Phase I/II trial in pediatric patients over 5 years old**: enrollment ongoing
RGX–111	Phase I/II trial Cohort 2 enrollment ongoing
RGX–181	Plan to provide program update in by end of 2021
RGX–381	Plan to provide program update in by end of 2021

Financial guidance:

Based on its current operating plan, REGENXBIO expects its balance in cash, cash equivalents and marketable securities of $593.0 million as of June 30, 2021, to fund its operations, including the completion of its internal manufacturing capabilities and clinical advancement of its product candidates, into the second half of 2023.

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[1] As announced on September 13, 2021. The transaction is expected to close in the second half of 2021, subject to the satisfaction of customary closing conditions, including applicable regulatory approvals.

Thank You