
# Corporate Presentation

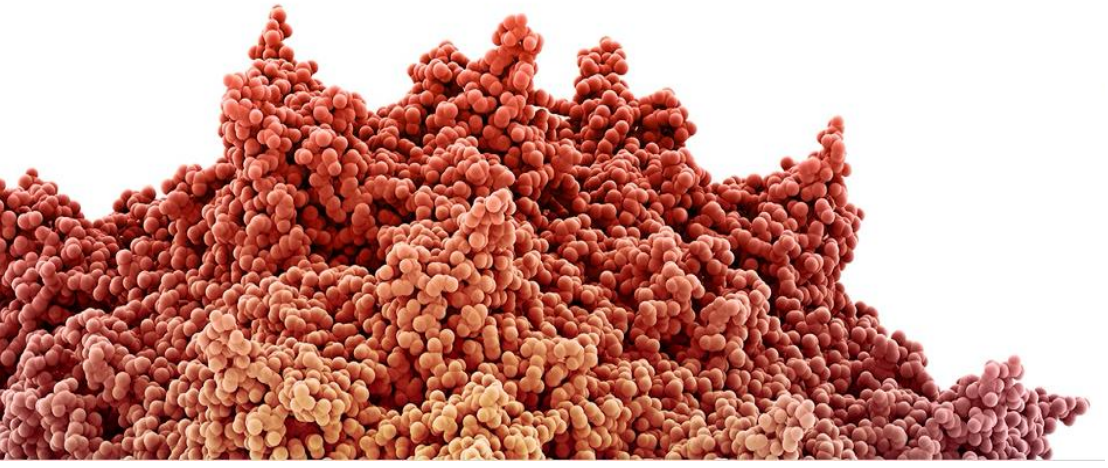
*Leader in AAV Gene Therapy*

9 | 13 | 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 proposed collaboration with AbbVie and 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 anticipated completion of REGENXBIO’s proposed transaction with AbbVie, the outcome of REGENXBIO’s proposed collaboration with AbbVie and other factors, 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, 2020 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. Except as required by law, 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.

# 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** 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**

# REGENXBIO's internal pipeline

| Indication | Development Stage | | | | Commercial Rights |
|---|---|---|---|---|---|
| | Research | Preclinical | Phase I / II | Pivotal | |
| **Retinal Disease**<br>wet AMD | RGX-314 Subretinal | | | | abbvie<br>U.S. Equal Profit Share<br>Ex-U.S. Tiered Royalties |
| wet AMD | RGX-314 Suprachoroidal | | | | |
| Diabetic retinopathy | RGX-314 Suprachoroidal | | | | |
| Add'l anti-VEGF treated conditions | | | | | |
| CLN2 disease ▲★ | RGX-381 | | | | Worldwide |
| **Neuromuscular Disease**<br>Duchenne muscular dystrophy | RGX-202 | | | | Worldwide |
| **Neurodegenerative Disease**<br>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**<br>Hereditary angioedema | | | | | Worldwide |

**AAV-mediated antibody delivery for chronic diseases**

**Monogenic gene replacement**

▲ Orphan Drug Designation

★ Rare Pediatric Disease Designation

■ Fast Track Designation

# 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

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## 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

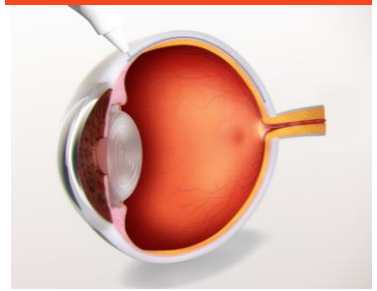
### Mechanism of action

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

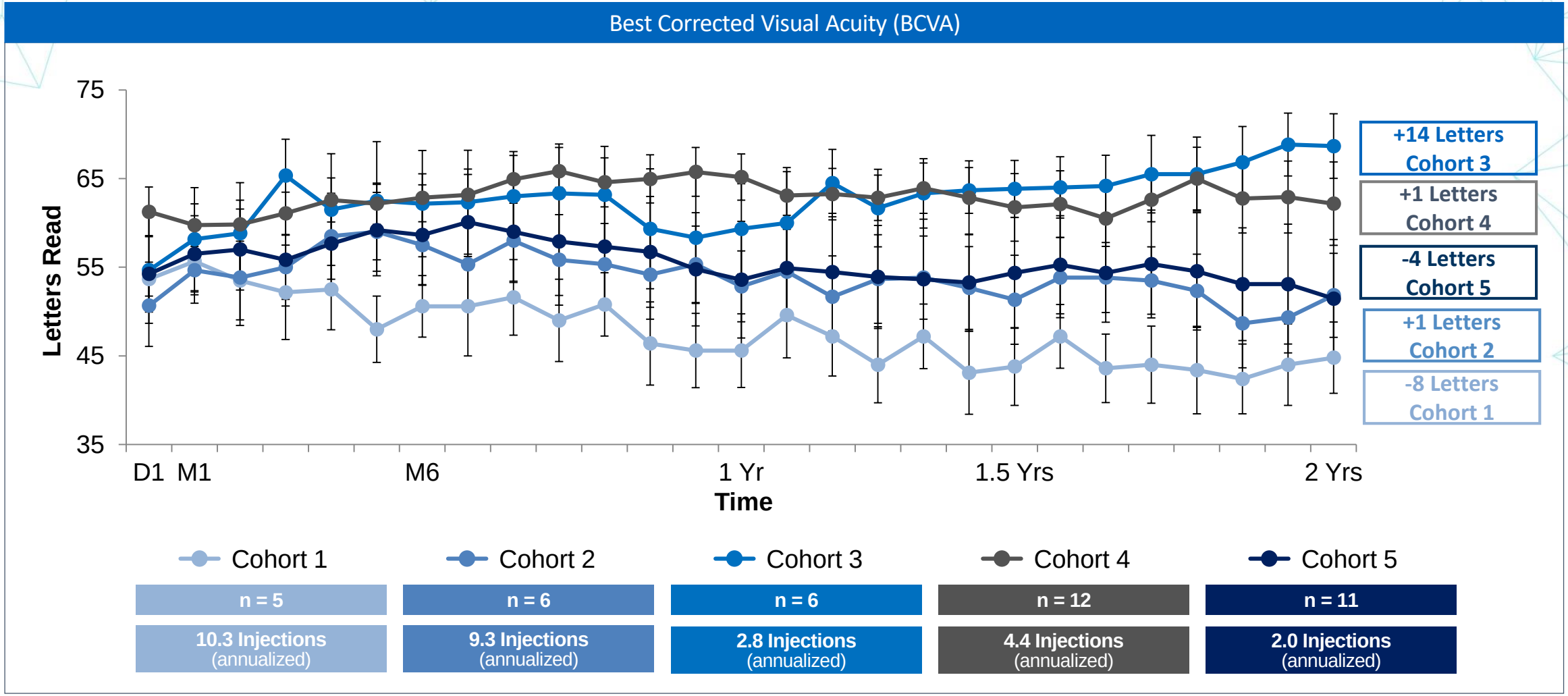
- Pivotal program to enroll a total of approximately 700 patients
- First trial, ATMOSPHERE™, is active and enrolling; a second pivotal trial is planned to initiate in Q4 2021
- cGMP manufacturing process bridging study is active, expected to support BLA filing

## Phase II suprachoroidal AAVIATE® trial on-going

- Cohort 1 enrollment complete, interim data to be presented at the Retina Society 54th Annual Scientific Meeting, Sept 29-Oct 2, 2021.
- Cohort 2 enrollment complete, interim data expected in Q4 2021
- Cohort 3 enrollment complete in NAb+ patients

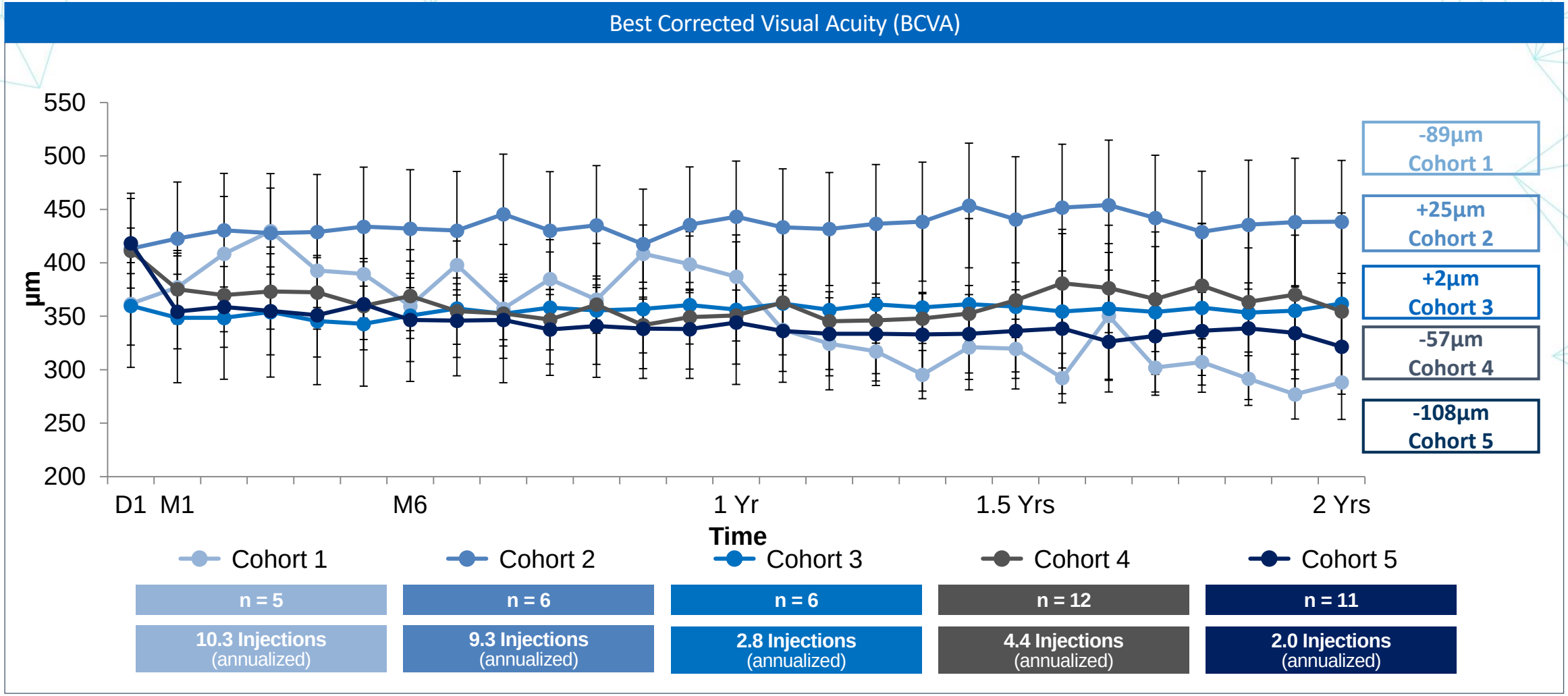
[1]Last data cut as of August 9, 2021
[2]Last data cut as of January 22, 2021

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



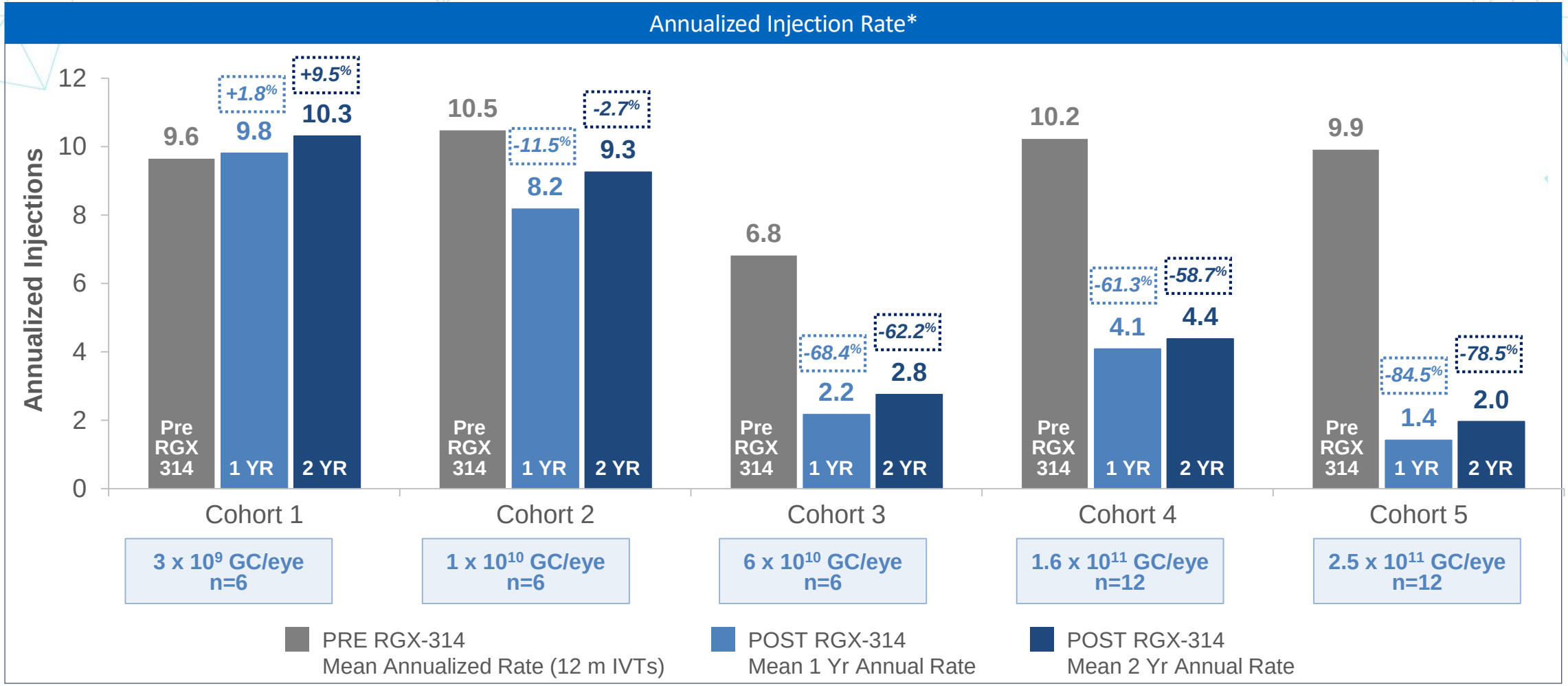

Note: One patient in Cohort 1 and one patient in Cohort 5 discontinued the study prior to Week 22 visit. Another patient in Cohort 5 has missed the visits due to COVID-19 from Week 50 through Week 74 and from Week 86 through Week 94. For this patient, missing visits were imputed using last observation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

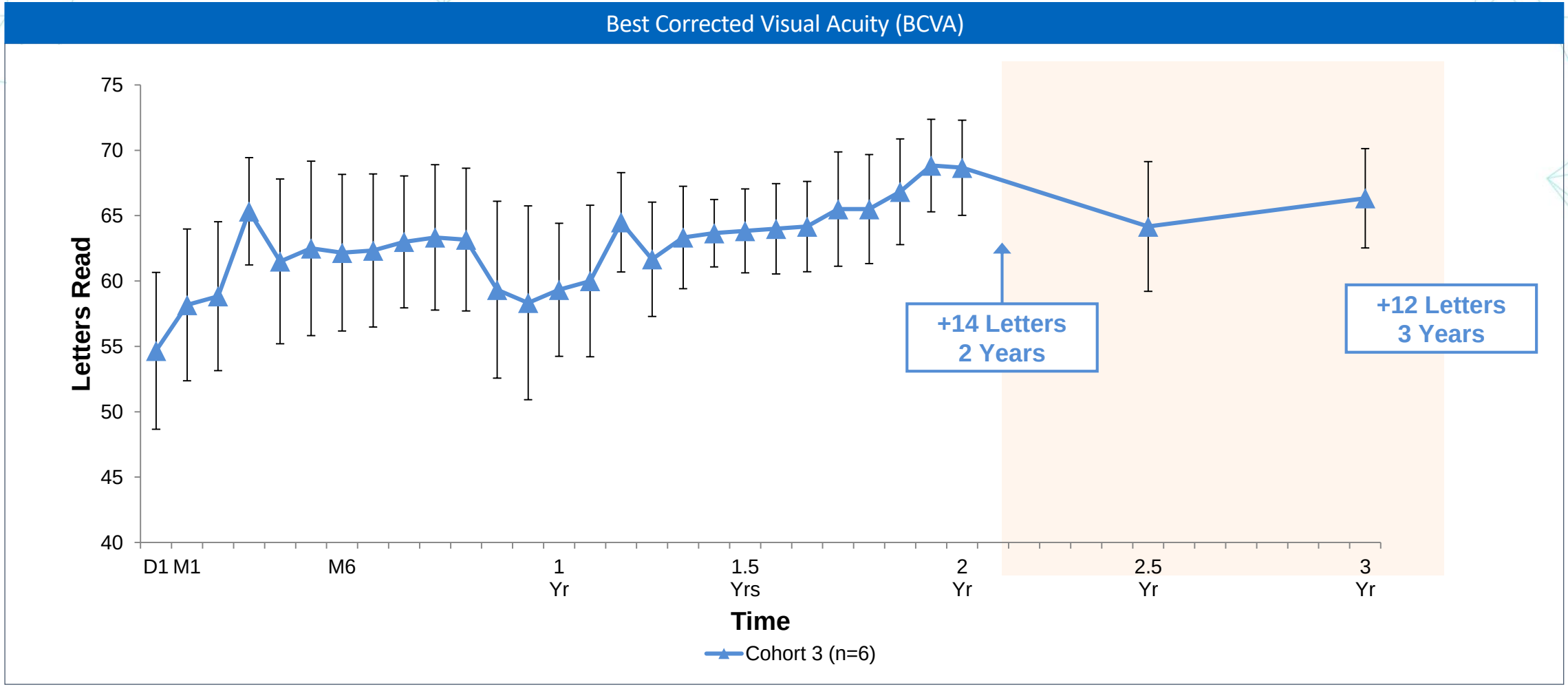
# RGX–314 subretinal Phase I/IIa clinical trial:
## Mean Change in Annualized Injection Rate PRE and POST RGX-314 in Cohorts 1–5




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

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## Subjects: approximately 300 total

**Route of administration:** Subretinal

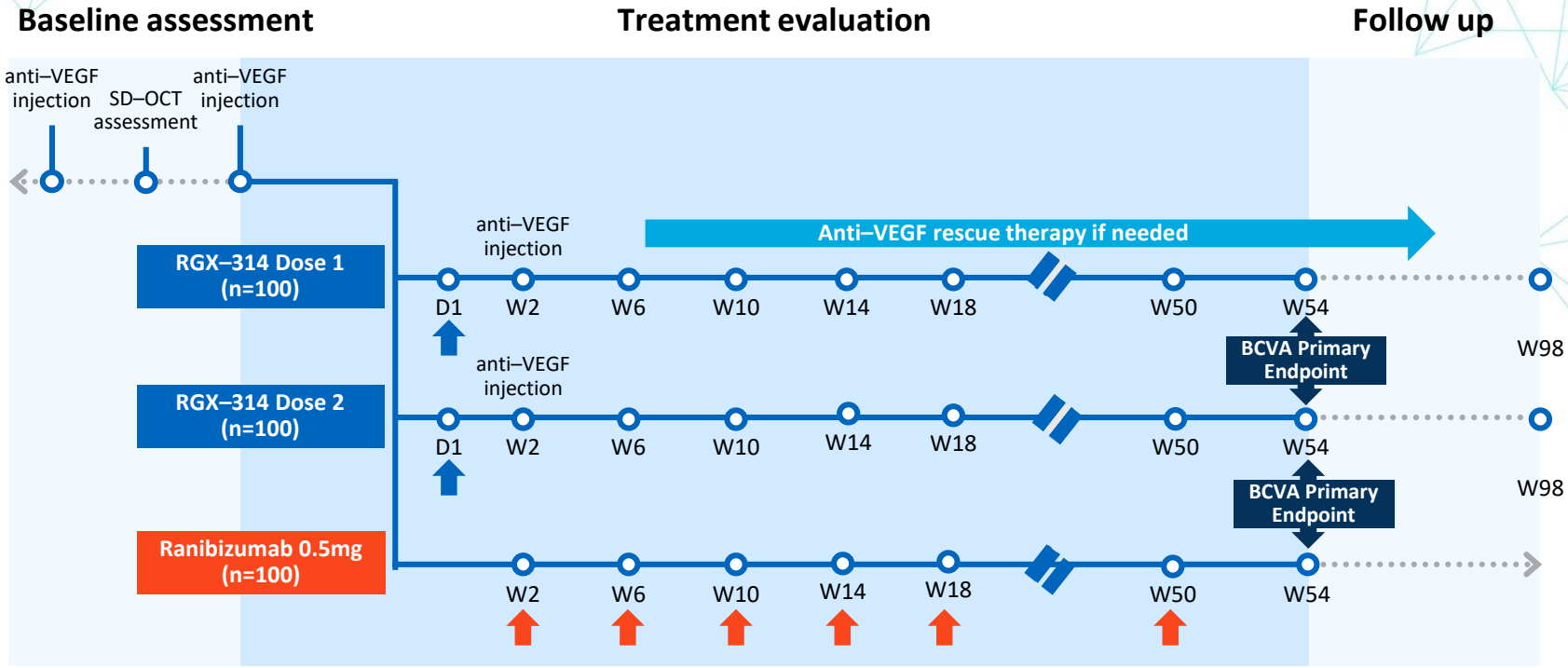
**Sites**: Up to 60 leading retinal surgery centers across the United States

## 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 |
|---|---|---|
| 6.4x10$^{10}$ GC/eye | 1.3x10$^{11}$ GC/eye | Comparator |

# 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

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

**Route of administration:** Suprachoroidal using SCS Microinjector

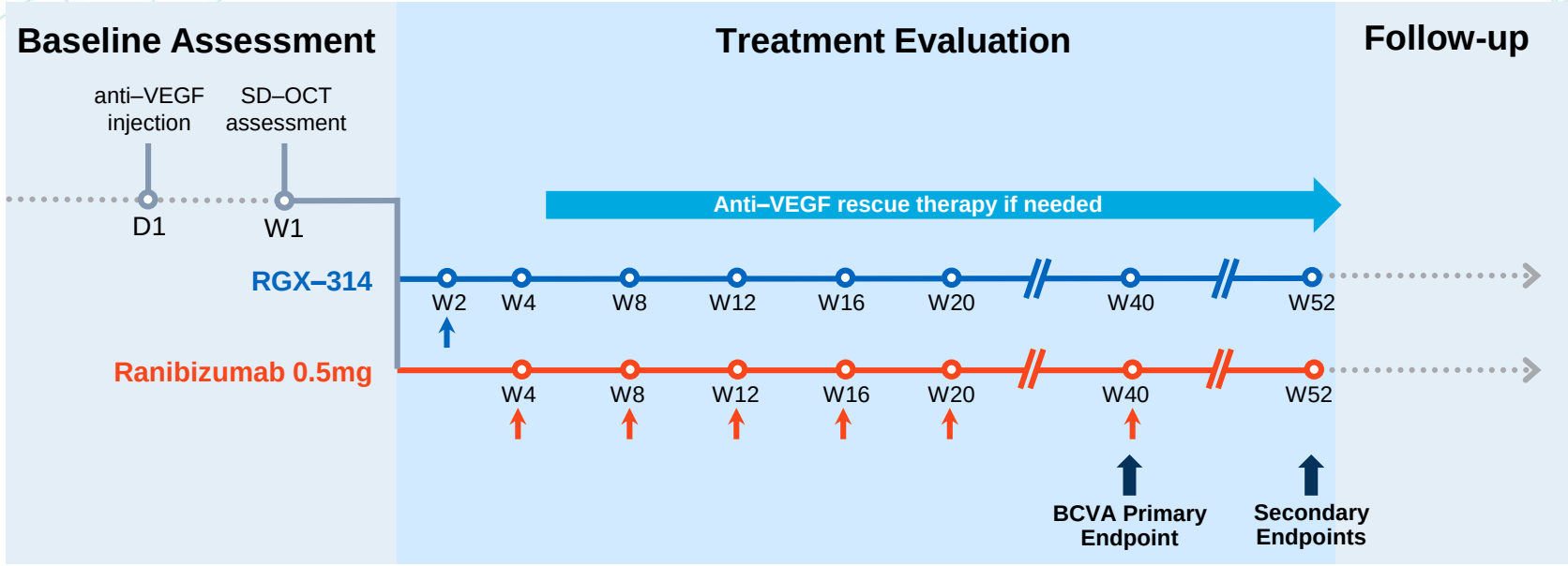
**Sites**: Fifteen leading retinal centers across the United States

## KEY INCLUSION CRITERIA

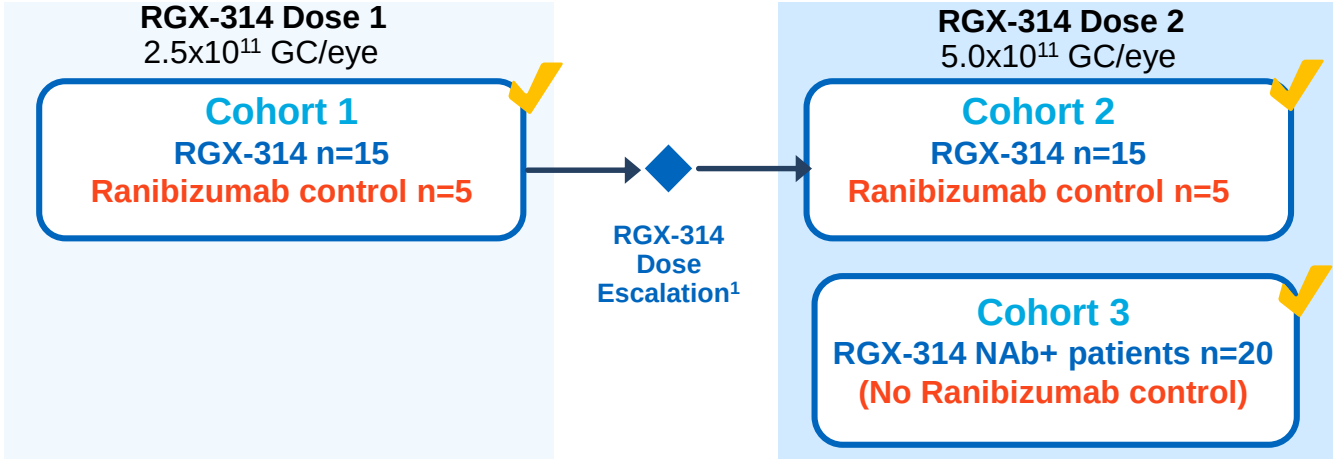
- Male or female ≥50 to 89 years of age
- Previously treated wet AMD subjects requiring no more than 10 anti–VEGF injections in the 12 months prior to trial entry
- Documented response to anti–VEGF at trial entry (assessed by SD–OCT)
- BCVA between ≤ 20/25 and ≥ 20/125 (≤ 83 and ≥ 44 Early Treatment 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
NAb+ = AAV8 neutralizing antibody positive

# **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 48 weeks

**Secondary**

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

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

**Route of administration:** Suprachoroidal using SCS Microinjector

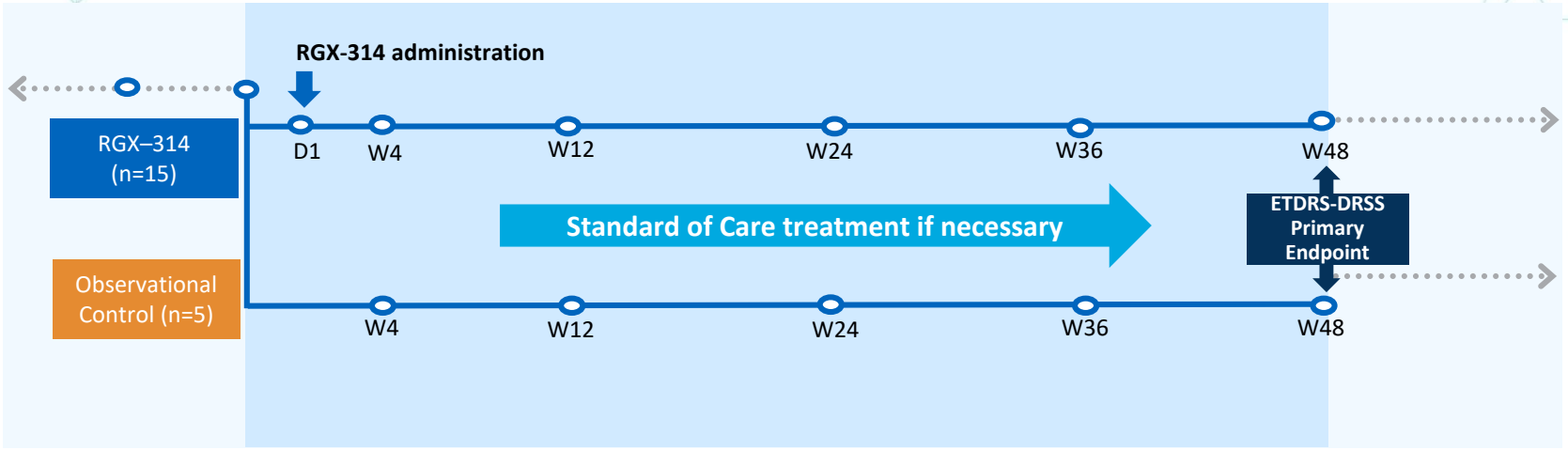
**Sites**: Fifteen leading retinal centers across the United States

## KEY INCLUSION CRITERIA

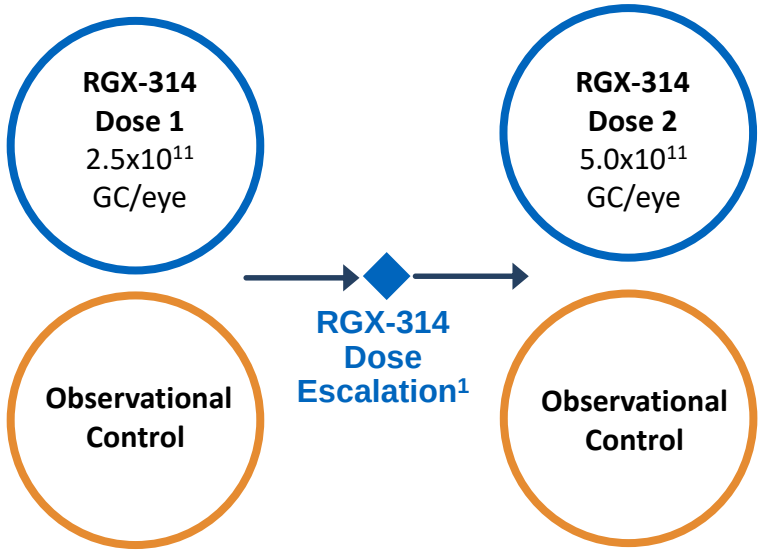
- Male or female ≥ 25 to 89 years of age with DR secondary to diabetes mellitus Type 1 or Type 2
- Moderately severe NPDR, severe NPDR, or Mild PDR
- No active DME, CST < 320 µm
- Vision of 20/40 or better (≥ 69 Early Treatment Diabetic Retinopathy Study [ETDRS] letters) in the study eye

# ALTITUDE™ Phase II clinical trial design

## Administration and follow-up timeline



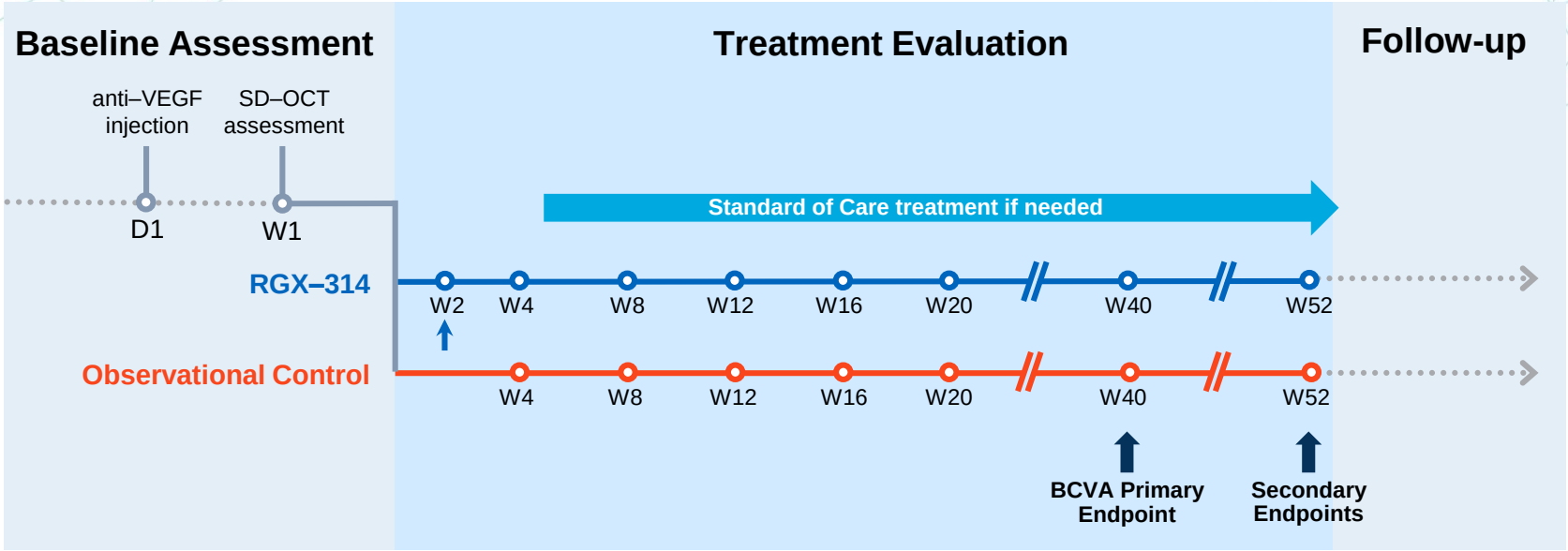

## Dose escalation



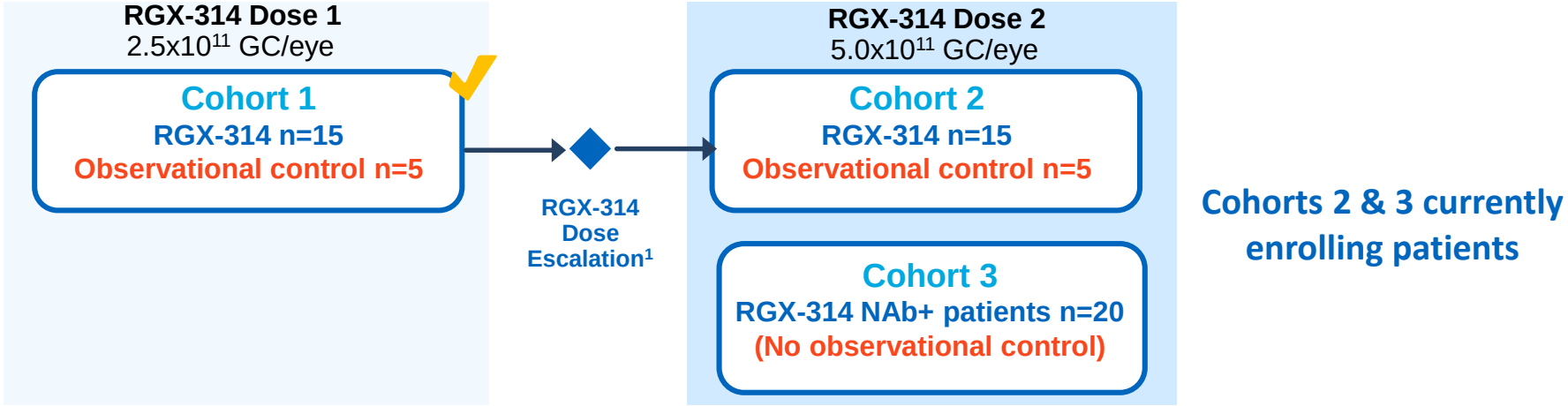

[1] Dose escalation safety review to occur two weeks after final subject in each cohort has been dosed

# 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 Cohort 1 has been dosed
SD-OCT = spectral domain optical coherence tomography
NAb+ = AAV8 neutralizing antibody positive

# **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

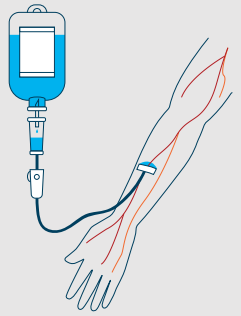
**Gene:** microdystrophin

### Mechanism of action

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

### Route of administration

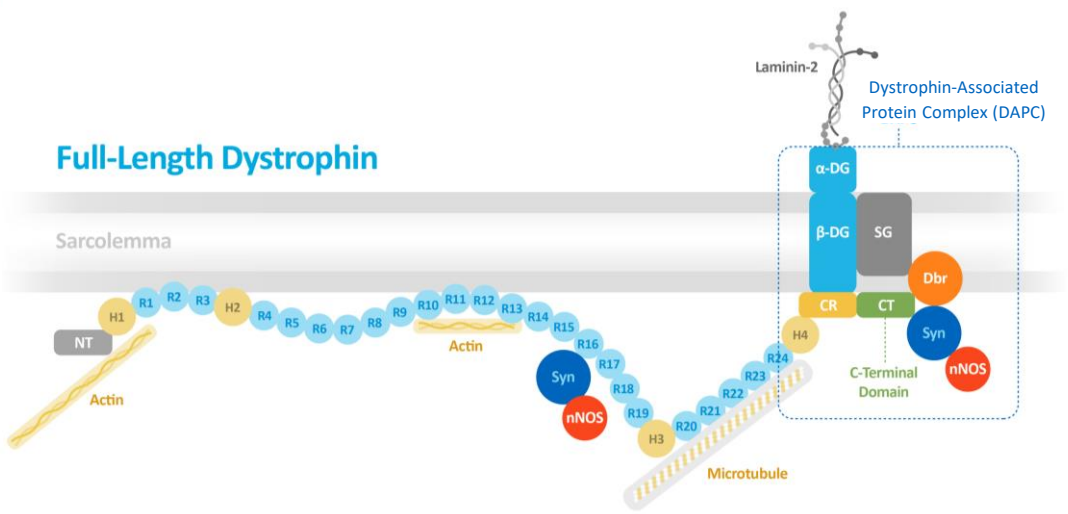
Intravenous

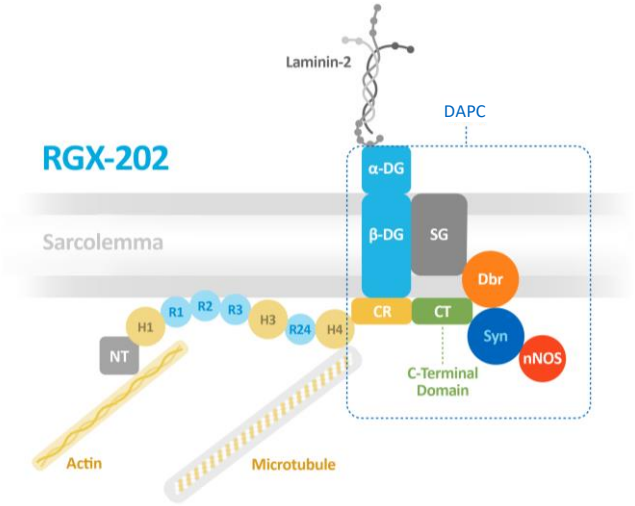


REGENXBIO®

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

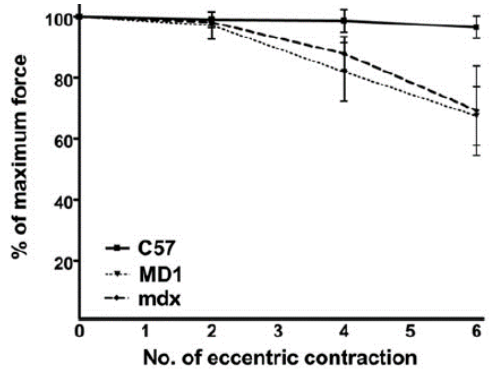
***CT Domain has been shown to recruit several key proteins to the muscle cell membrane (sarcolemma) including Syntrophin and Dystrobrevin, Neuronal nitric oxide synthase and other proteins[1]***



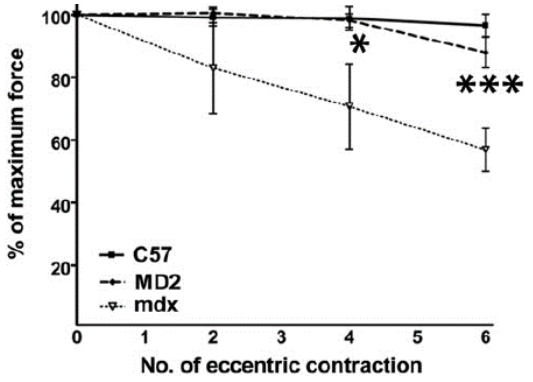




Presence of CT Domain in microdystrophin significantly improved the muscle resistance to lengthening contraction–induced muscle damage in DMD$^{mdx}$ mice[2]

**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

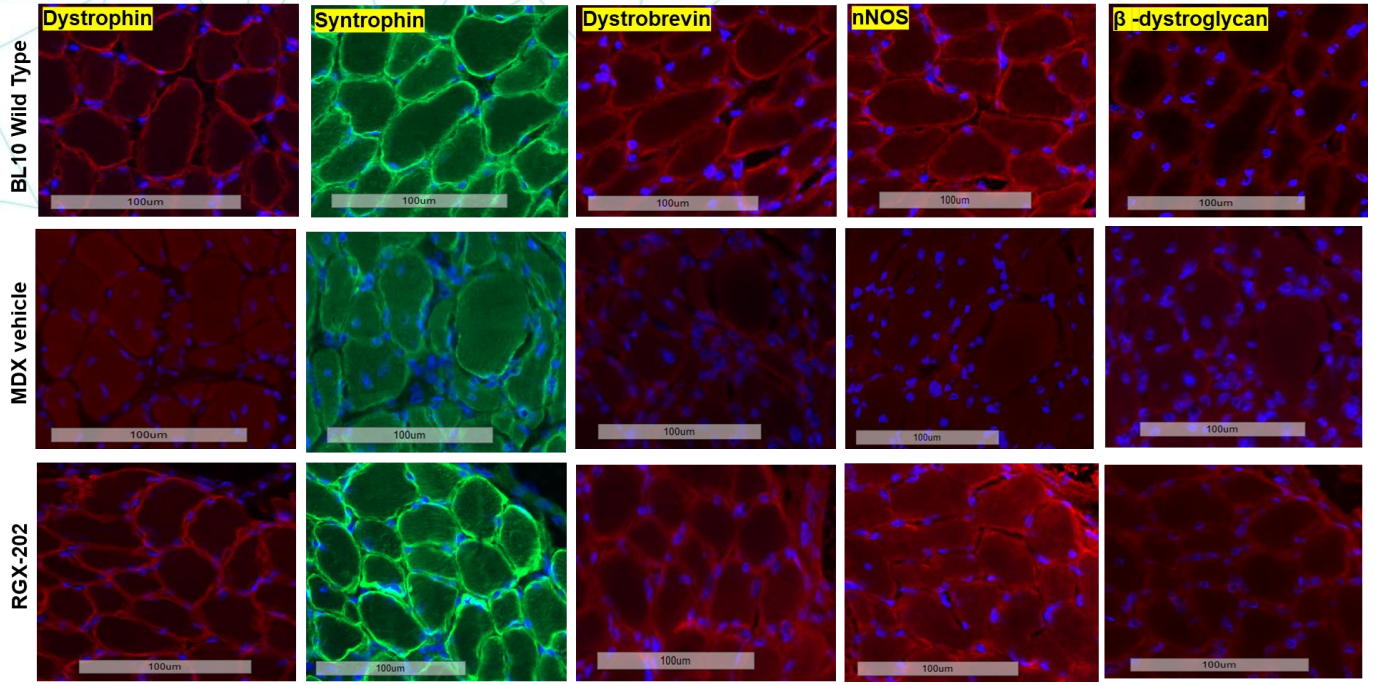
| | AAV Capsid | Promoter | Microdystrophin domain design | Transgene Size (bp) | CpG total (# Islands) |
|---|---|---|---|---|---|
| RGX-202 | 8 | Spc5-12 | ABD1 – H1 – R1 – R2 – R3 – H3 – R24 – H4 – CR – CT | 4,734 | 69 (1) |
| Other Investigational Intervention (Example) | | | ABD1 – H1 – R1 – R2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

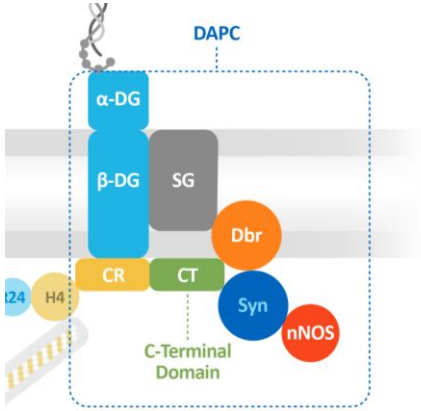
[1] Koo et al, *Human Gene* Therapy, 2011
[2] Faust, et al. *Journal of Clinical Investigation*, 2013
[3] Le Guiner, et al. *Nature Communications*, 2017
[4] Mack, et al. *Molecular Therapy*, 2017
[5] Shieh, et al. ASGCT 2019

REGENXBIO®

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

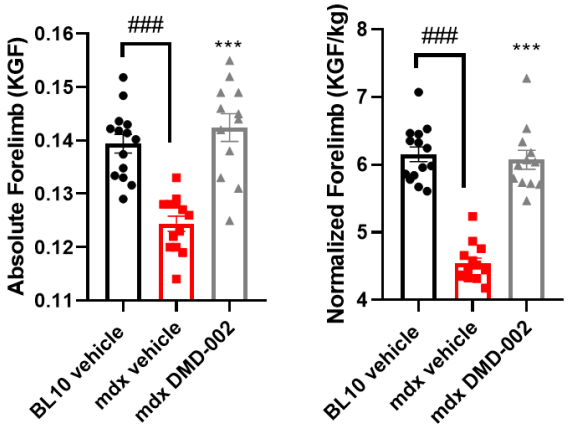



Histological evidence that RGX-202 recruits key proteins to DAPC

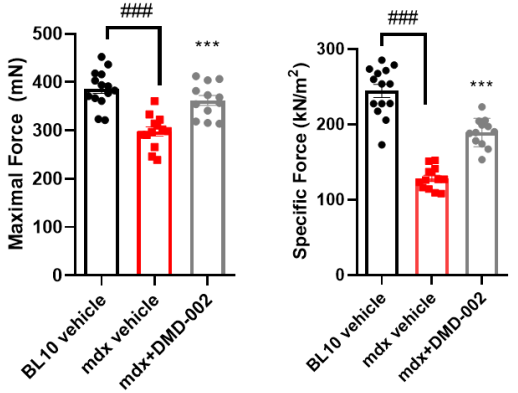



Significant strength and force improvements observed in DMD$^{mdx}$ mice treated with RGX-202

**Mouse Grip Strength (In-Life)**

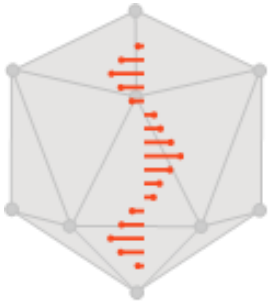


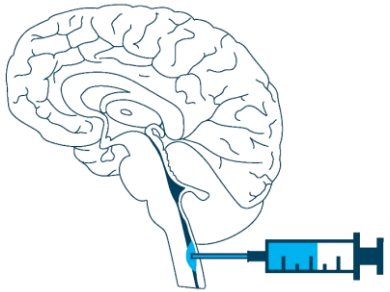

***Ex Vivo* Force Measurements**



# 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<br>▪ X-linked recessive disease<br>▪ Available treatment is inadequate to treat neurodegeneration<br>▪ More than 500 patients born annually worldwide | ▪ Reduced ability to process GAGs, leading to neurodegeneration and early death<br>▪ Autosomal recessive disease<br>▪ Available treatment is inadequate to treat neurodegeneration; stem cell transplant partially effective<br>▪ More than 500 patients born annually worldwide | ▪ Reduced ability to process cellular waste peptides, leading to seizures, vision loss, neurodegeneration and early death<br>▪ Autosomal recessive disease<br>▪ Available treatment requires frequent ICV infusions of ERT, shown to stabilize some but not all disease manifestations<br>▪ Approximately 500 patients born annually worldwide |
| **Gene** | **IDS** Gene Replacement | **IDUA** Gene Replacement | **TPP1** Gene Replacement |
| **Designations** | ▲ Orphan Drug Designation<br>★ Rare Pediatric Disease Designation<br>■ Fast Track Designation | ▲ Orphan Drug Designation<br>★ Rare Pediatric Disease Designation<br>■ Fast Track Designation | ▲ Orphan Drug Designation<br>★ 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

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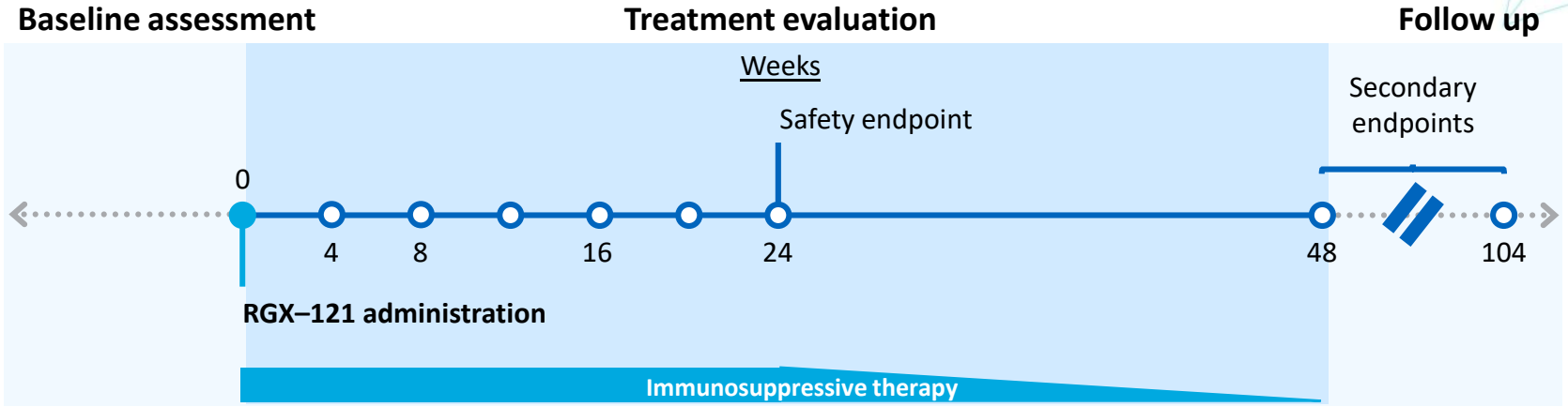
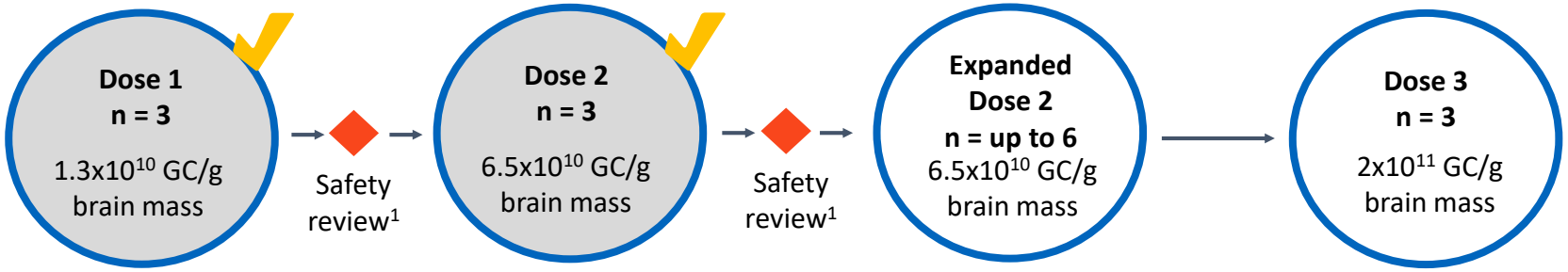
# **RGX–121 Phase I/II clinical trial:** Administration and dose escalation

## 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

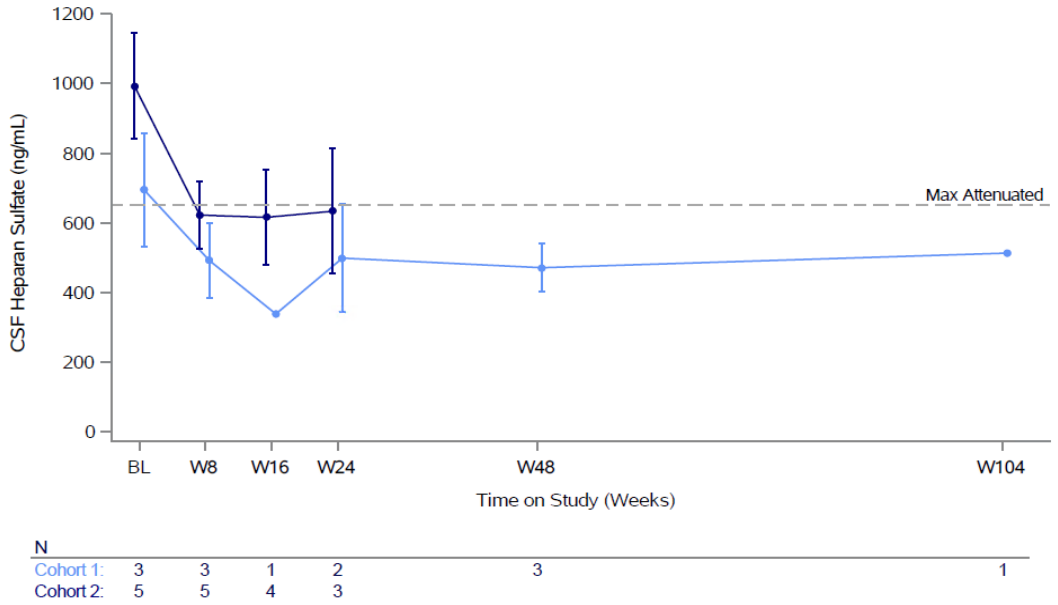
- **Emerging evidence of systemic I2S protein expression and biomarker activity in Cohorts 1 & 2**
  - Increased I2S protein concentration in plasma
  - Rapid reductions in urine biomarker levels observed in ERT[2]-naïve patients

[1] As announced May 14, 2021

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

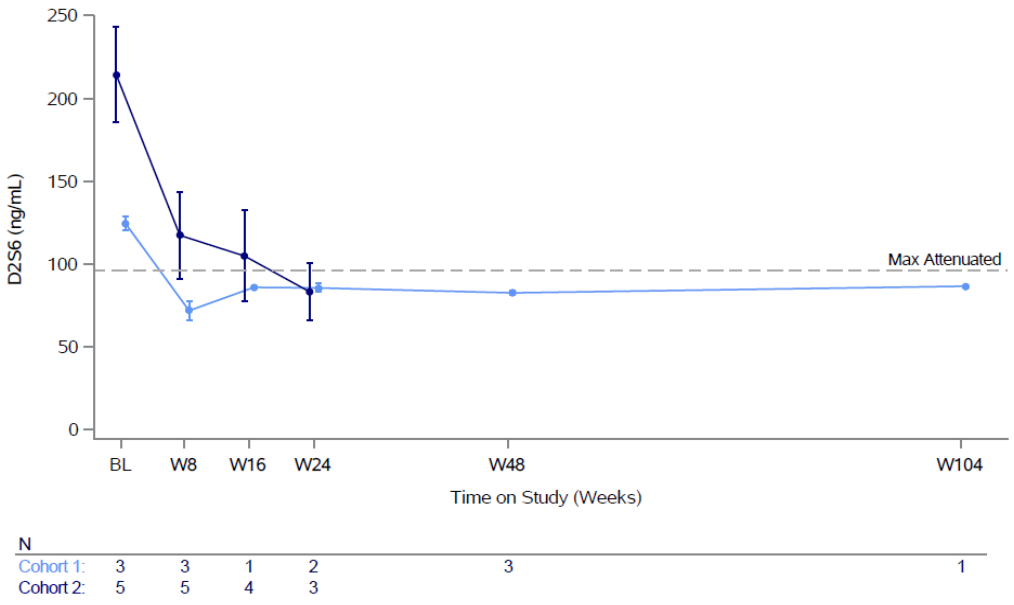
# **RGX–121 Phase I/II clinical trial:** Reductions in CSF biomarkers up to 2 years after RGX-121 administration[1]

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




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

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

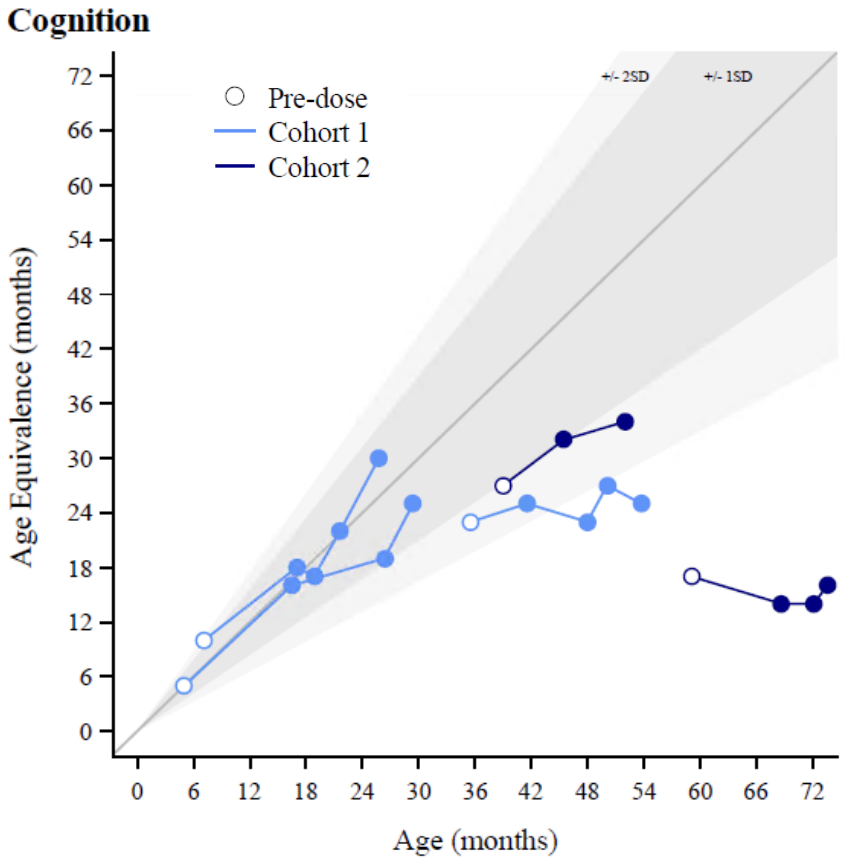



Median change from baseline:
-44.1% at Week 8; -40.4% at last timepoint available (n=8)

[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[1]

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




- 4 out of 5 subjects with greater than 6 months[2] of follow-up continued cognitive

[1] Presented at ASGCT on May 14, 2021

[2] 3 of 5 patients enrolled in cohort 2 had ≤ 6 months of follow up and are not reported here

# 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

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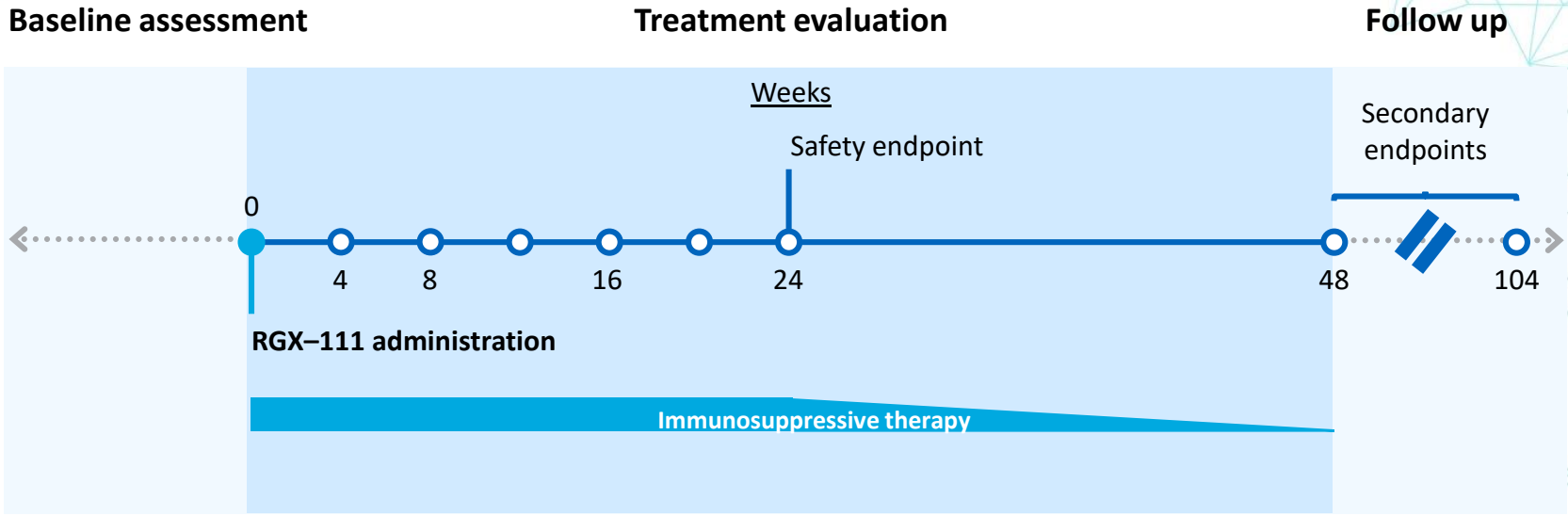
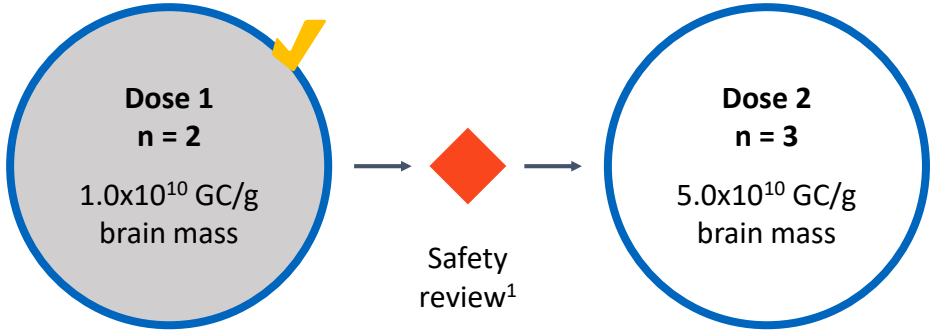
# **RGX–111 Phase I/II clinical trial:** Administration and dose escalation

## 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

REGENXBIO®

# 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 Q4 2021<br>**Suprachoroidal wet AMD:** Interim data from AAVIATE® Cohort 1 to be presented at Retina Society 54th Annual Meeting (Sept 29-Oct 2); interim data from Cohort 2 expected in Q4 2021<br>**Suprachoroidal DR:** Initial data from ALTITUDE™ expected in Q4 2021 |
| **RGX-202** | IND submission by end of 2021 |
| **RGX–121** | **Phase I/II trial in patients up to 5 years old**: enrollment ongoing<br>**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.*

[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