# Corporate Presentation

*Leader in AAV Gene Therapy*

REGENXBIO®

July 8, 2020

# Forward-looking statements

This presentation includes “forward-looking statements,” within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended. These statements express a belief, expectation or intention and are generally accompanied by words that convey projected future events or outcomes such as “believe,” “may,” “will,” “estimate,” “continue,” “anticipate,” “design,” “intend,” “expect,” “could,” “plan,” “potential,” “predict,” “seek,” “should,” “would” or by variations of such words or by similar expressions. The forward-looking statements include statements relating to, among other things, REGENXBIO’s future operations, clinical trials, costs and cash flow. REGENXBIO has based these forward-looking statements on its current expectations and assumptions and analyses made by REGENXBIO in light of its experience and its perception of historical trends, current conditions and expected future developments, as well as other factors REGENXBIO believes are appropriate under the circumstances. However, whether actual results and developments will conform with REGENXBIO’s expectations and predictions is subject to a number of risks and uncertainties, including the timing of enrollment, commencement and completion and the success of clinical trials conducted by REGENXBIO, its licensees and its partners, the timing of commencement and completion and the success of preclinical studies conducted by REGENXBIO and its development partners, the timely development and launch of new products, the ability to obtain and maintain regulatory approval of product candidates, the ability to obtain and maintain intellectual property protection for product candidates and technology, trends and challenges in the business and markets in which REGENXBIO operates, the size and growth of potential markets for product candidates and the ability to serve those markets, the rate and degree of acceptance of product candidates, the impact of the COVID-19 pandemic or similar public health crises on REGENXBIO’s business, and other factors, many of which are beyond the control of REGENXBIO. Refer to the “Risk Factors” and “Management’s Discussion and Analysis of Financial Condition and Results of Operations” sections of REGENXBIO’s Annual Report on Form 10-K for the year ended December 31, 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

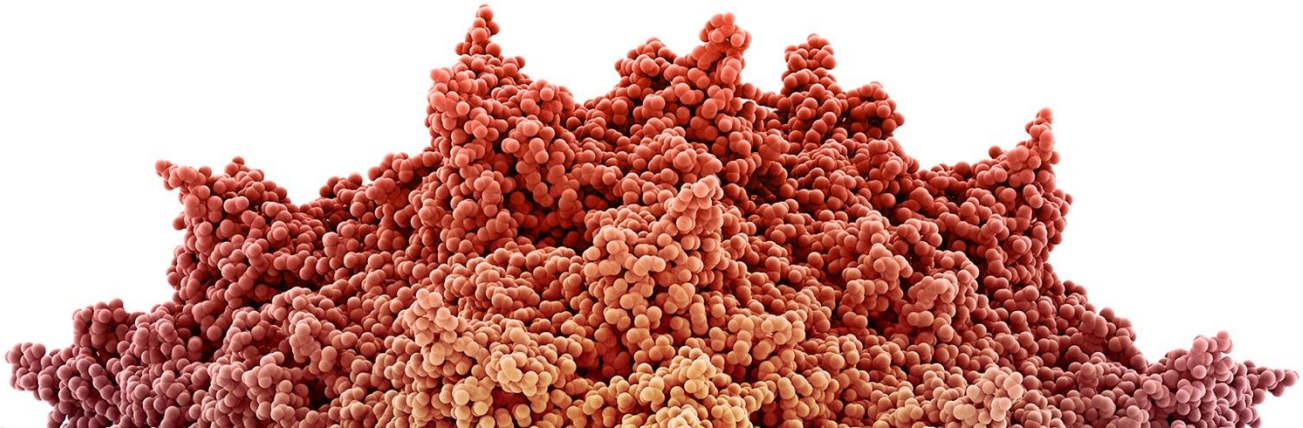
Pipeline focused on **AAV-mediated antibody delivery**
and **rare genetic diseases**
with **multiple clinical trials in 2020**

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

**1 FDA-approved product 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

| Indication | Development Stage (Research, Preclinical, Phase I / II, Phase III) | Commercial Rights |
|---|---|---|
| **Retinal Disease** | | |
| wet AMD | RGX-314 Subretinal | Worldwide |
| wet AMD | RGX-314 Suprachoroidal | Worldwide |
| Diabetic retinopathy | RGX-314 Suprachoroidal | Worldwide |
| Add'l anti-VEGF treated conditions | | 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** | | |
| HoFH ▲ | RGX-501 | Worldwide |
| Hereditary angioedema | | Worldwide |
| **Neuromuscular Diseases** | | |
| Undisclosed | | 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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# Internal Pipeline

# **RGX–314** for treatment of wet age-related macular degeneration **(wet AMD)**

## THE DISEASE

- Blurring of central vision and progressive vision loss due to formation of leaky blood vessels in the eye
- VEGF inhibitors are standard of care to treat fluid and associated vision loss
- Frequency / uncomfortable administration of current anti-VEGF therapies affects compliance and ultimately efficacy
- >2 million patients estimated in U.S., Europe and Japan

## RGX–314 PRODUCT CANDIDATE

**Vector:** AAV8

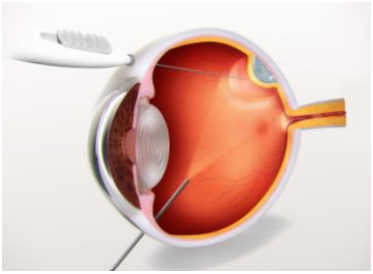
**Gene:** anti-VEGF Fab

### Mechanism of action

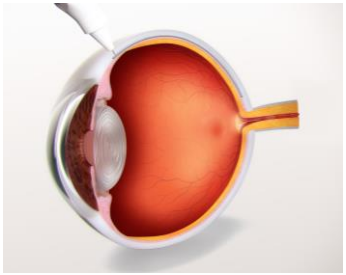
Reducing leaky blood vessel formation by giving retinal cells the ability to produce an anti-VEGF fab

### Routes of administration

Subretinal (SR)



Suprachoroidal (SC)



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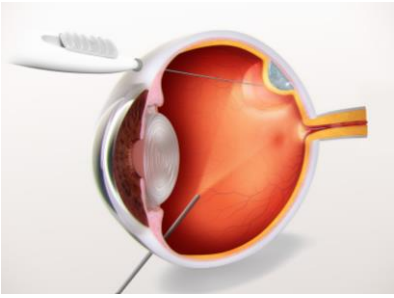
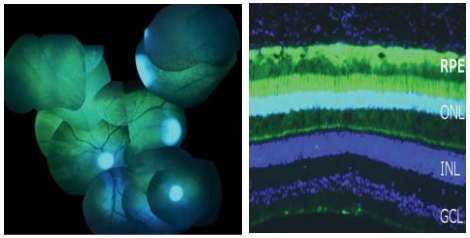
# **RGX-314 Routes of Administration:** Two approaches to reach the back of the eye with multiple advantages to broaden market opportunity

## Subretinal Delivery[1]





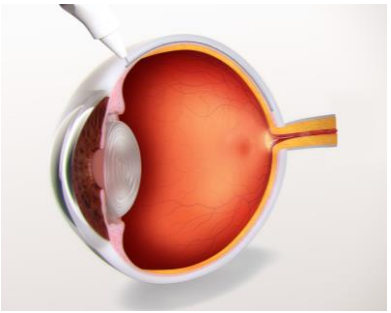

Retinal transduction achieved via subretinal delivery of AAV8 in non-human primates AAV8.GFP 1.0 x $10^{11}$ GC

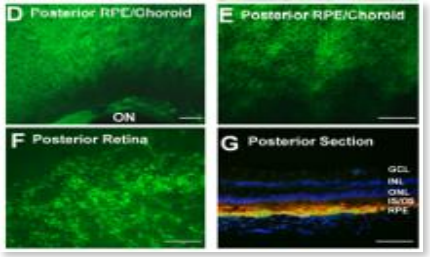
- Established route of delivery for gene therapy
- Direct and broad transduction of the retina observed
- Minimal exposure to the vitreous and anterior segment
  - Low risk of immune response
  - Low risk of inflammation
- No oral corticosteroid prophylaxis

### AAV Neutralizing Antibody (NAb) Status

- All patients eligible, regardless of NAb status

## Suprachoroidal Delivery[2]






Retinal transduction achieved via suprachoroidal delivery of AAV8 in non-human primates AAV8.GFP 4.75 x $10^{11}$ GC

- In-office, non-surgical approach using SCS Microinjector™
- Direct and broad transduction of the retina
- Minimal exposure to the vitreous and anterior segment
  - Low risk of immune response
  - Low risk of inflammation
- No oral corticosteroid prophylaxis

### AAV NAb Status

- ~70% patients without NAbs to AAV8[3]

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[1] Vandenberghe et al. 2011 *Science Translational Medicine*, [2] Ding, K., et al. *2019 Journal of Clinical Investigation*, [3] Calcedo R, et al. 2009 *Journal of Infectious Disease*

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

## OBJECTIVES

### Primary

- To determine the safety and tolerability of subretinal RGX–314 in subjects with wet AMD through six months

### Secondary

- Expression of RGX–314 protein in the eye
- Effect of RGX–314 on best corrected visual acuity (BCVA) and central retinal thickness (CRT) as measured by Spectral Domain Optical Coherence Tomography (SD–OCT)
- Additional anti–VEGF injections post-RGX–314

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**Subjects**: 42 total

**Route of administration:** subretinal

**Sites**: Eight leading retinal surgery centers across the United States

## KEY INCLUSION CRITERIA

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

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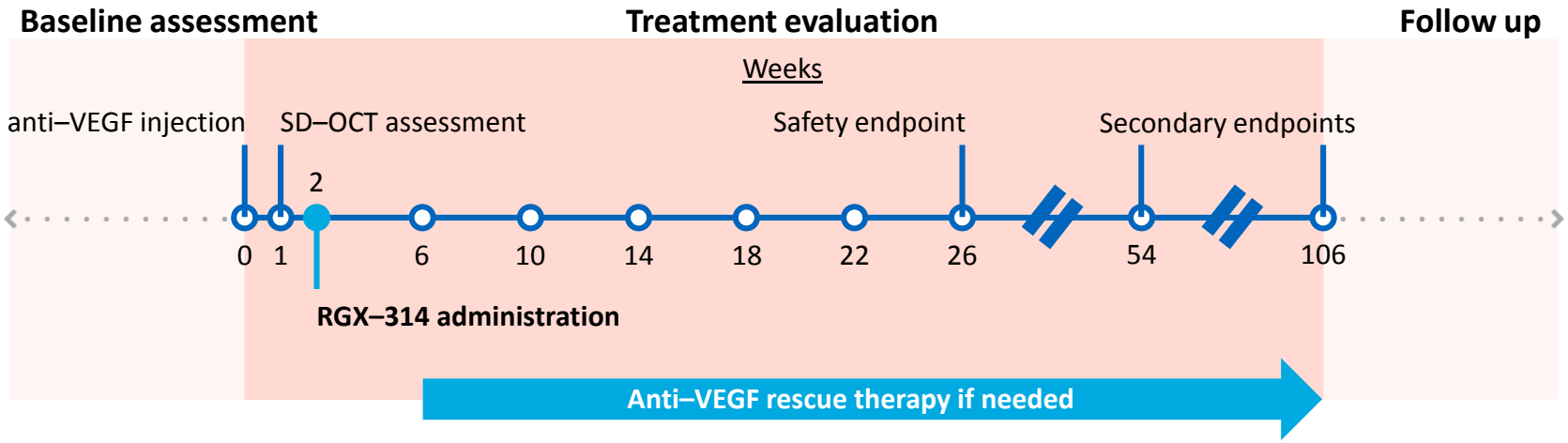
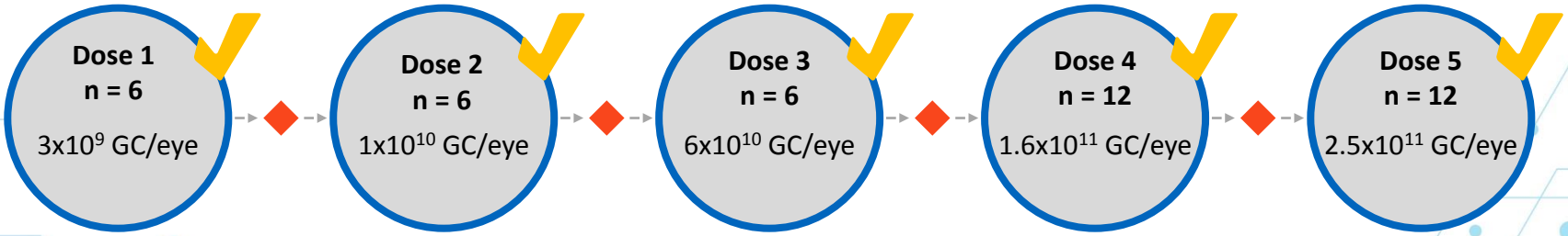
# **RGX–314 subretinal Phase I/IIa clinical trial:** dose escalation protocol

## Administration and follow-up timeline




## Dose escalation




**42 total subjects dosed across five cohorts**

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# **RGX–314 subretinal Phase I/IIa clinical trial:** Safety and data summary

- RGX–314 continued to be well–tolerated across all doses (n=42)
- No drug-related SAEs reported; 16 SAEs that were not drug-related reported in 10 patients[1]
- Common ocular AEs in the study eye included:
  - Post-operative conjunctival hemorrhage (69% of patients) – 100% mild, majority resolved within days to weeks
  - Mild to moderate retinal pigmentary changes[2] (67% of patients across all cohorts; 83% of patients in Cohorts 3-5) – 71% mild, none severe
    - No evidence of clinical symptoms or changes to visual acuity related to these observations
  - Post-operative inflammation[3] (36% of patients) – resolved within days to weeks, 100% mild
  - Post-operative visual acuity reduction (17% of patients) – majority resolved within days to weeks, 100% mild
  - Eye irritation (17% of patients) and eye pain (17% of patients) – 90% mild, none severe
  - Retinal hemorrhage (17% of patients) – an anticipated event in the severe wet AMD population, 100% mild
- No reports of clinically-determined immune responses, drug-related ocular inflammation, or post-surgical inflammation beyond what is expected following routine vitrectomy

Data cut April 6, 2020

[1]Includes two deaths unrelated to RGX-314; Two ocular procedure-related SAEs: peripheral retinal detachment which was repaired and an endophthalmitis post aqueous sample collection

[2]Retinal pigmentary changes observed were hypo and hyper pigmentation on imaging occurring in the bleb area or inferior retina

[3]Post-operative inflammation includes AC cells, flare, or inflammation

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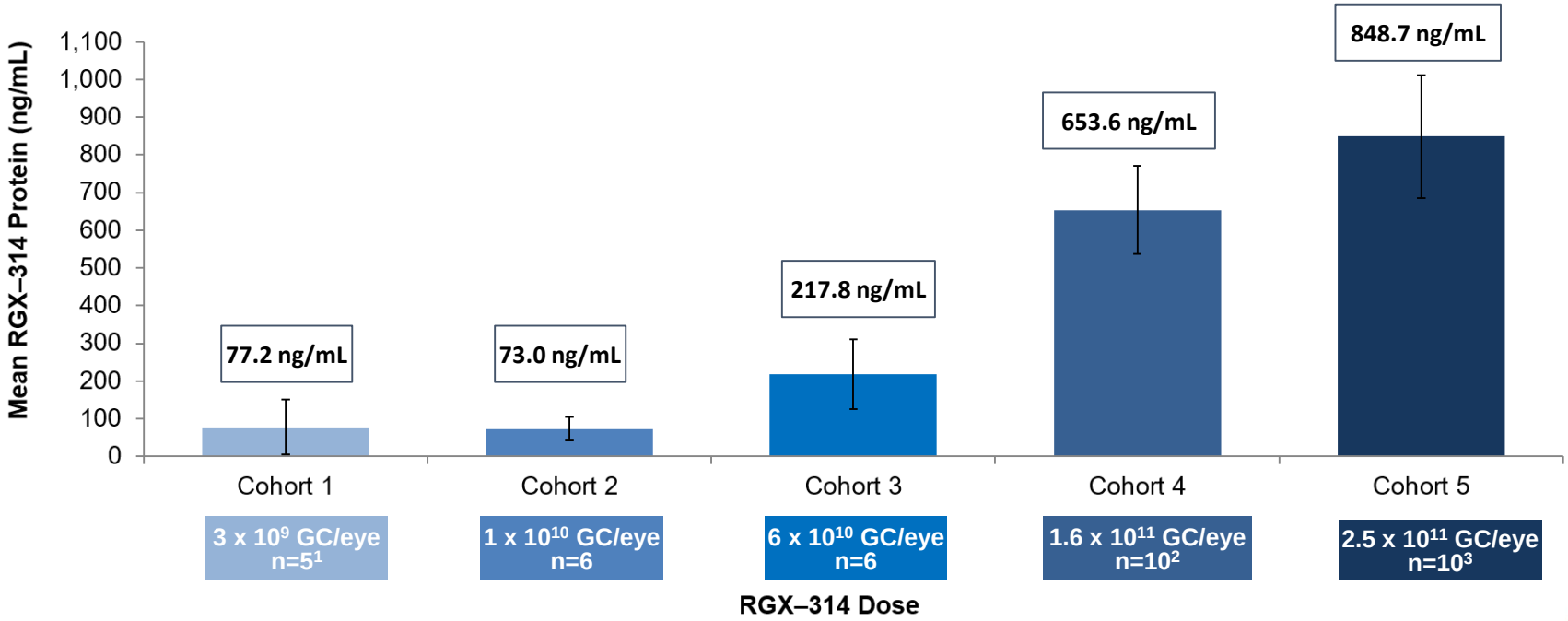
# **RGX–314 subretinal Phase I/IIa clinical trial:** RGX-314 protein levels at six months

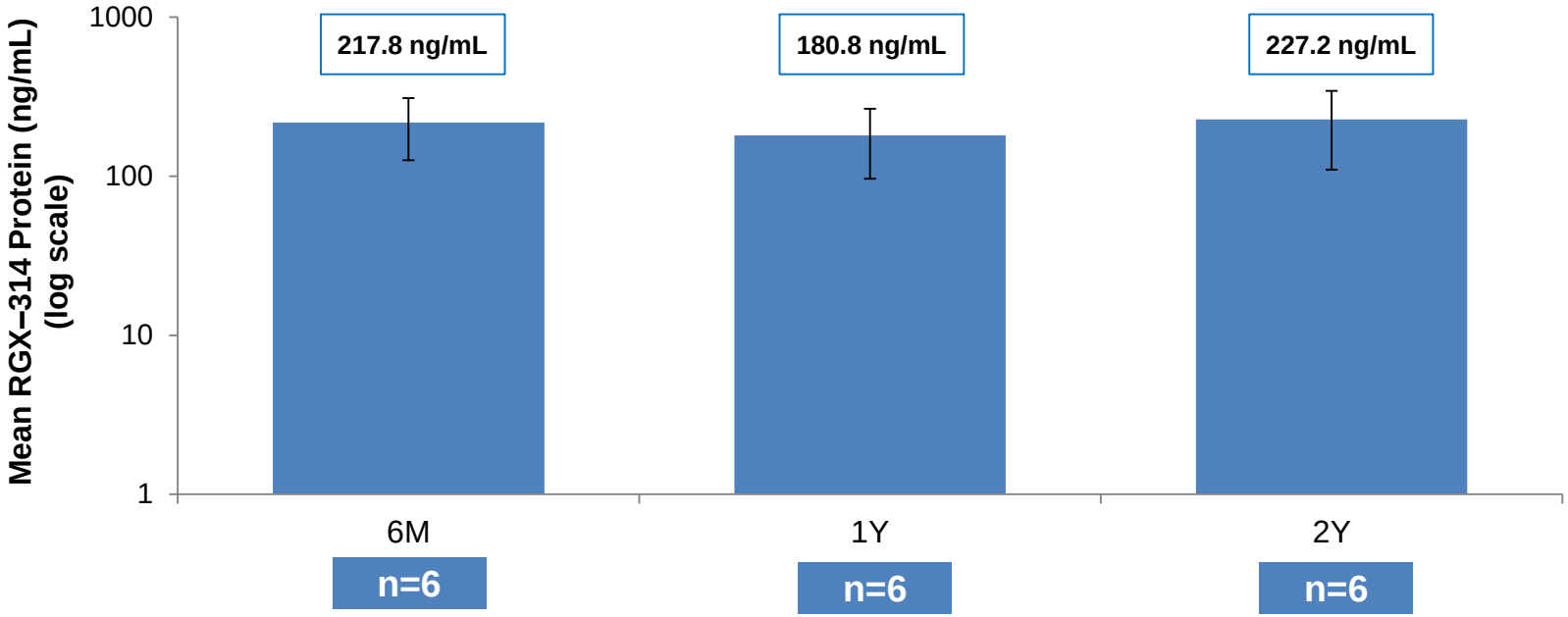
## As measured from aqueous samples by ECL




[1]One patient in Cohort 1 discontinued the study prior to Week 22 visit.
[2]Two patients in Cohort 4 did not have aqueous samples taken at Week 26
[3]One patient in Cohort 5 discontinued the study prior to Week 26 and another patient did not have aqueous sample taken at Week 26

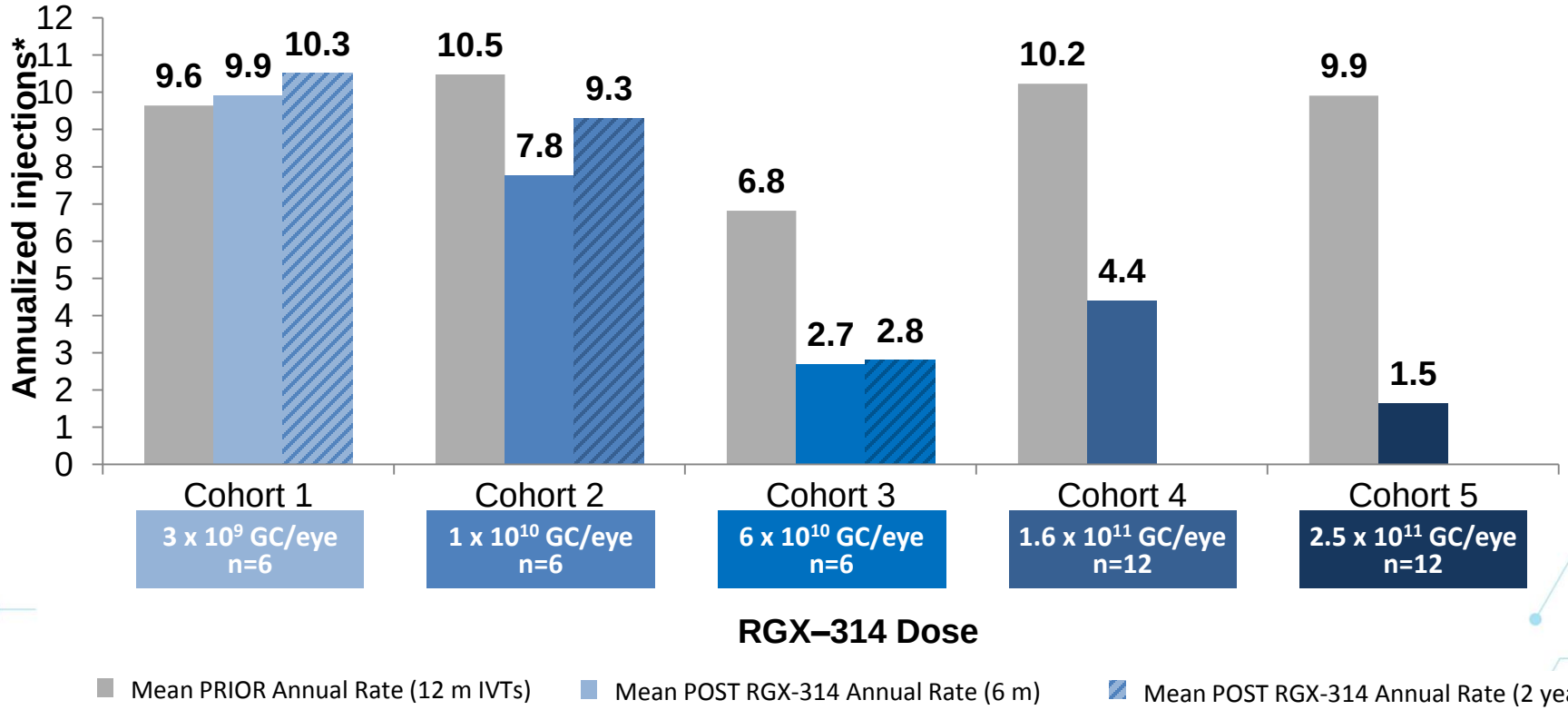
# **RGX–314 subretinal Phase I/IIa clinical trial:** RGX-314 protein levels over 2 years in Cohort 3

**As measured from aqueous samples by ECL**



# **RGX–314 subretinal Phase I/IIa clinical trial:** Mean change in annualized injection rate pre- and post-RGX-314

## Comparison of injection rate PRIOR and POST RGX-314




**Cohort 5 demonstrates over 80% reduction in anti-VEGF injections**

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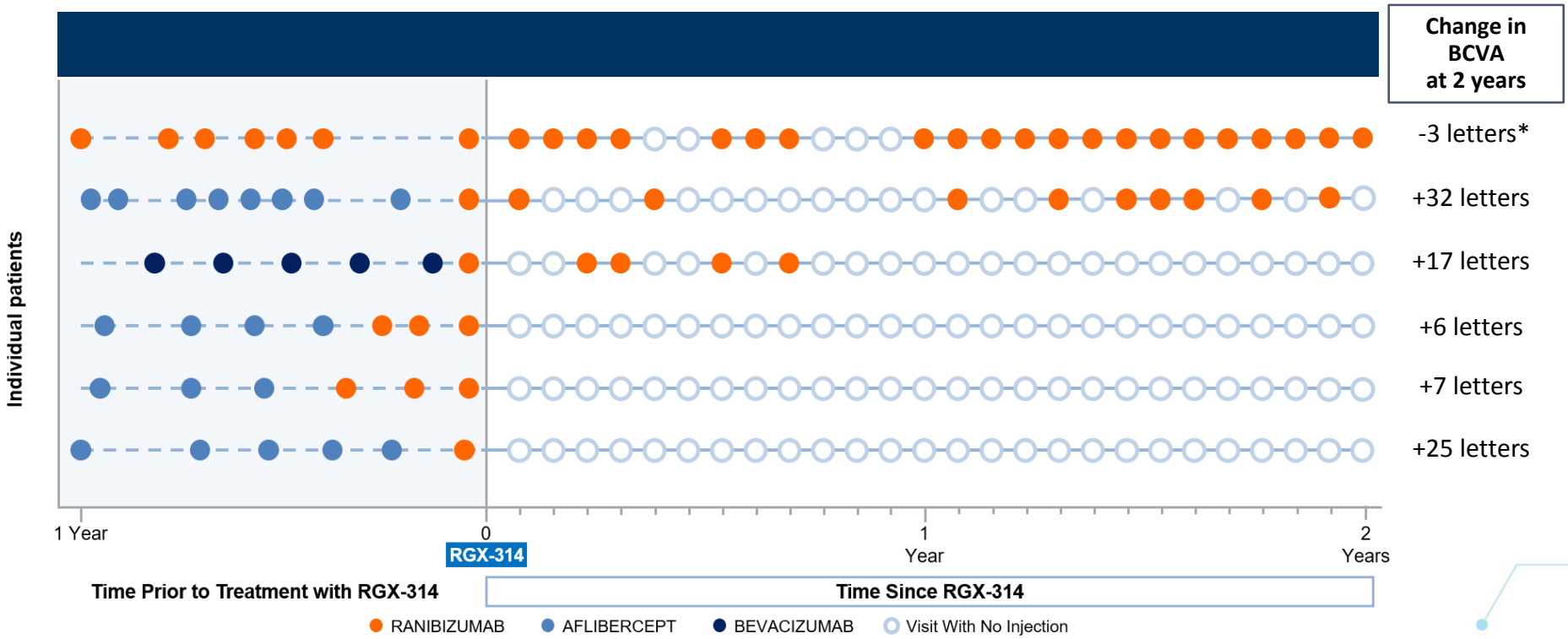
*Prior annual rate is (Total # of prior IVTs)/(minimum(366 days, Duration between first ever IVT and Day 1)/365.25). Post RGX-314 annual rate is (Total # of IVTs on Study)/(Duration on Study/365.25) where on Study is from RGX-314 administration through 24 months for C1-C3 and through 6 months for C4 –C5.

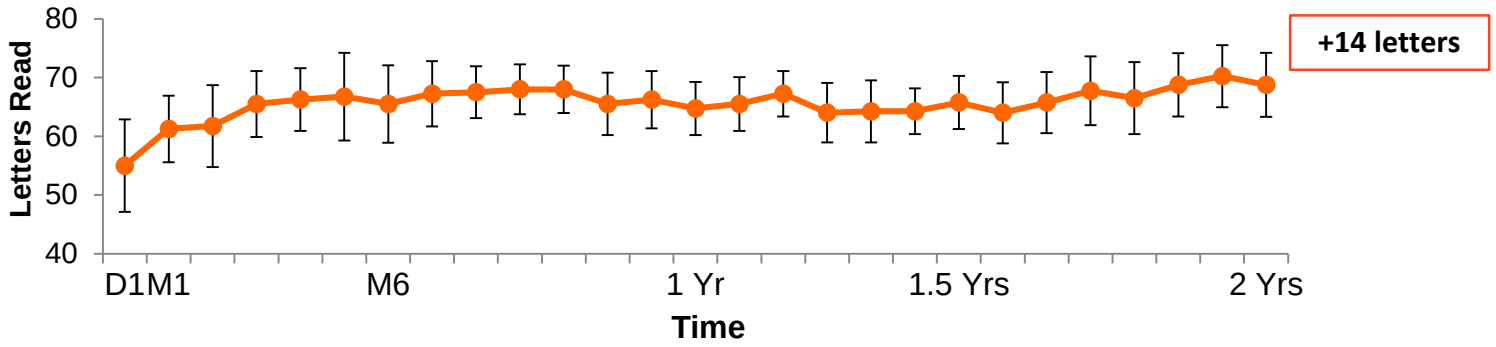
# **RGX–314 subretinal Phase I/IIa clinical trial:** Cohort 3 injections pre- and post-RGX-314



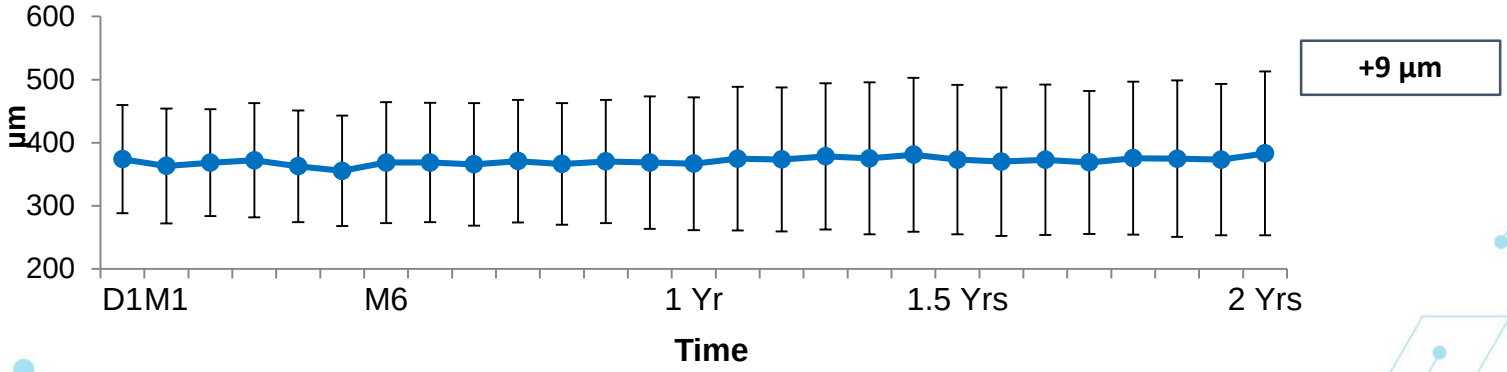

*Patient received incomplete dose at time of subretinal procedure

# **RGX–314 subretinal Phase I/IIa clinical trial:** Cohort 3 patients that are anti-VEGF injection-free after 9 months (n=4) continue to do well over 2 years

**Best Corrected Visual Acuity (BCVA)**

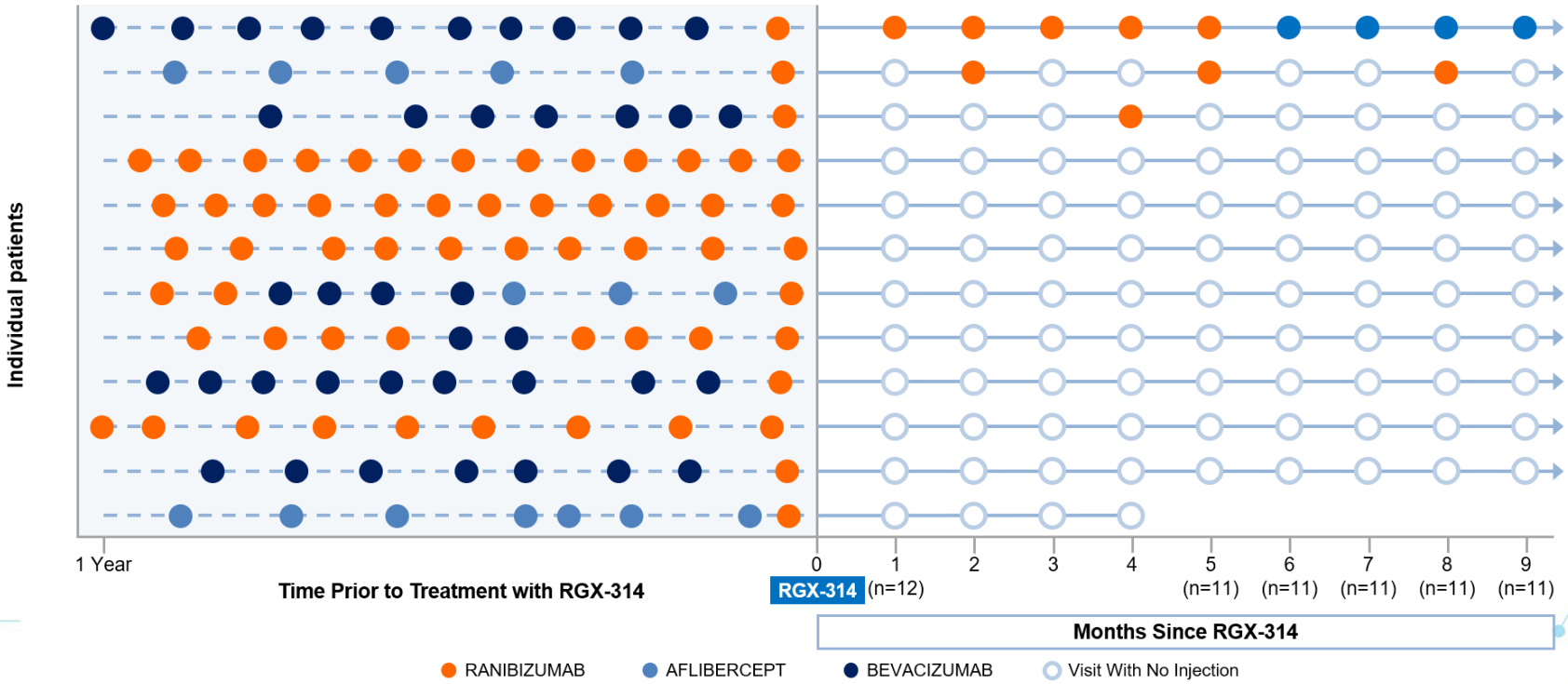



**Central Retinal Thickness (CRT) by Central Reading Center**




Note: one missing CRT value in Cohort 3 has been interpolated

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

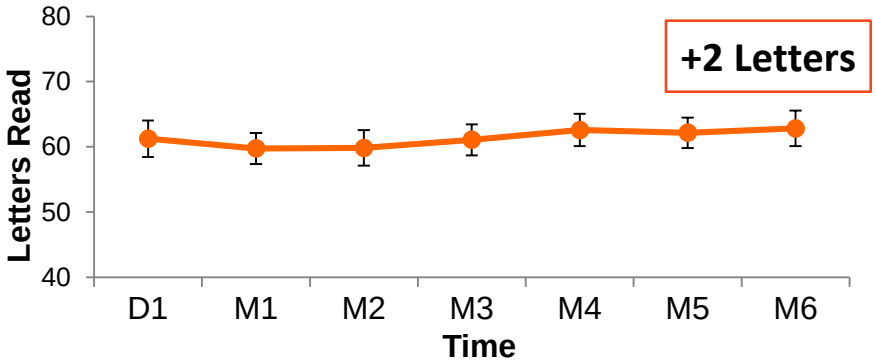


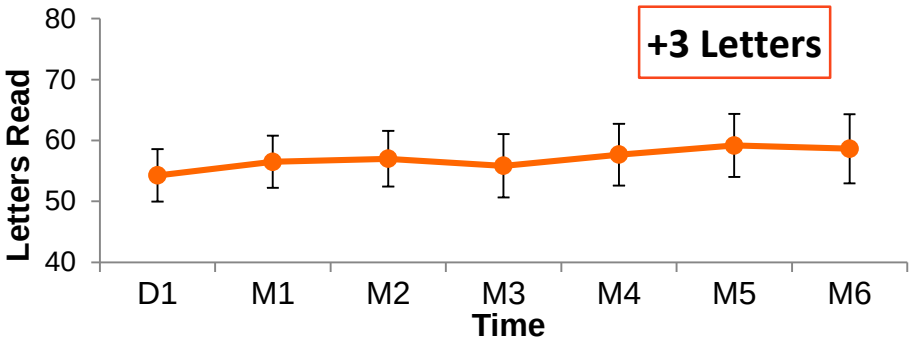

Subject #12 discontinued after 4 months

# **RGX–314 subretinal Phase I/IIa clinical trial:** BCVA, CRT and average injections over 6 months in cohorts 4 and 5

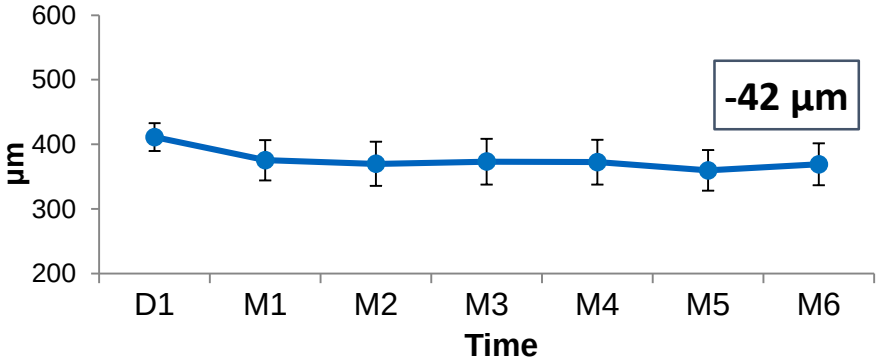
**Cohort 4 (n=12)** | **Cohort 5 (n=12)[1]**

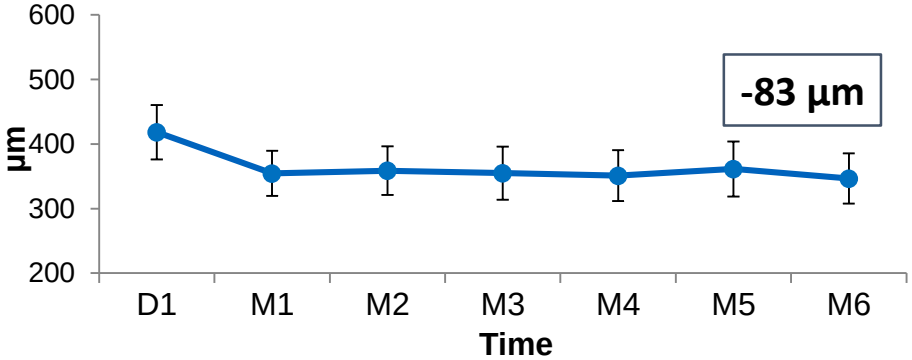
## Best Corrected Visual Acuity (BCVA)




## Central Retinal Thickness (CRT) on Heidelberg SD-OCT[2]




**Mean: 2.2 inj / 6 mo**
**42% (5 of 12) injection-free at 6 months**

**Mean: 0.8 inj / 6 mo**
**73%[3] (8 of 11) injection-free at 6 months**

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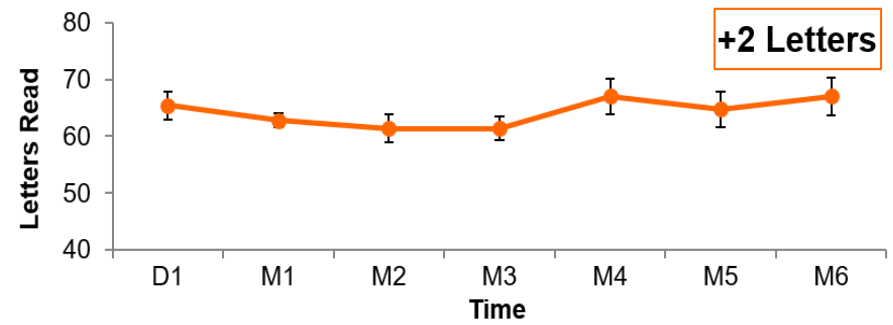
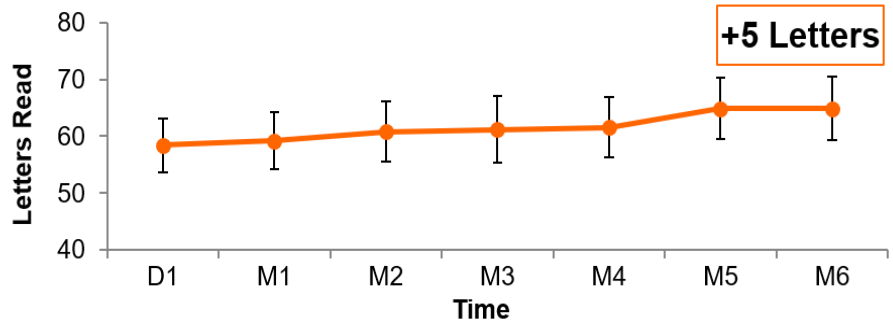
[1]One patient discontinued after 4 months
[2]SD-OCT data read by a central reading center (Duke Reading Center).
[3]Injection-free calculation (8 of 11) does not include the subject that discontinued after 4 months; subject was injection-free at time of discontinuation

# **RGX-314 subretinal Phase I/IIa clinical trial:** BCVA and CRT in anti-VEGF injection-free subjects from Cohorts 4 and 5 over 6 months

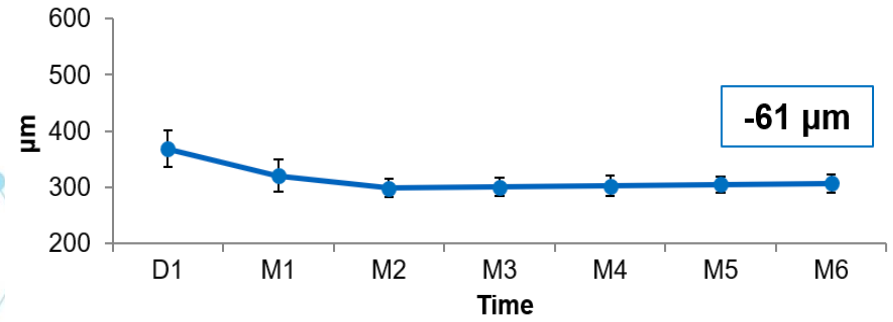
**Cohort 4 (n=5)** | **Cohort 5 (n=9)[2]**

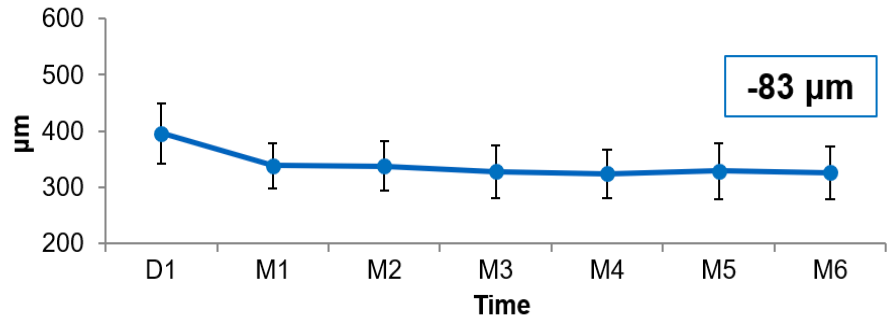
## Best Corrected Visual Acuity (BCVA)




## Central Retinal Thickness (CRT) on Heidelberg SD-OCT[1]




**0 Injections** | **0 Injections**

[1]SD-OCT data read by a central reading center (Duke Reading Center).
[2]One patient discontinued after 4 months (subject injection-free at time of discontinuation)

# **RGX–314** for treatment of Diabetic Retinopathy **(DR)**

## THE DISEASE

- Leading cause of vision loss in adults between 24 – 75 years of age
- Spectrum encompasses nonproliferative DR (NPDR) and proliferative DR (PDR) with or without diabetic macular edema (DME)
- Treatment options include anti-VEGF injections or panretinal laser treatment
- Approximately 8 million patients estimated in United States

## RGX–314 PRODUCT CANDIDATE

**Vector:** AAV8

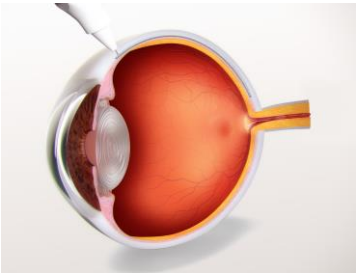
**Gene:** anti-VEGF Fab

### Mechanism of action

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

### Route of administration

Suprachoroidal



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# REGENXBIO's neurodegenerative disease franchise

| | RGX–121 for MPS II | RGX–111 for MPS I | RGX–181 for CLN2 disease |
|---|---|---|---|
| Disease | ▪ Reduced ability to process glycosaminoglycans (GAGs), leading to neurodegeneration and early death<br>▪ X-linked recessive disease<br>▪ Available treatment is inadequate to treat neurodegeneration<br>▪ Approximately 500 – 1,000 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>▪ Approximately 500 – 1,000 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 |
| Vector | AAV9 | AAV9 | AAV9 |
| Gene | IDS gene replacement | IDUA gene replacement | TPP1 gene replacement |
| Admin | Intracisternal | Intracisternal | Intracisternal |
| Desig-nations | ▲ 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 |

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# RGX–121 Phase I/II clinical trial in MPS II

## Objectives

### Primary

- To determine the safety and tolerability of RGX–121 in severe MPS II subjects who have or are at high risk of developing neurocognitive deficits

### Secondary

- Effect of RGX–121 on biomarkers of I2S activity in CSF, serum and urine
- Effect of RGX–121 on neurocognitive deficits

**Subjects**: Up to 6 total

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

### Key inclusion criteria

- Male subjects ≥4 months to <5 years of age
- Meeting one of the following criteria:
  - Diagnosis of MPS II and a score >55 and ≤77 on intelligent quotient testing OR
  - Diagnosis of MPS II and a score >55 and a decline of ≥1 standard deviation on intelligent quotient testing OR
  - Having a relative diagnosed with severe MPS II who has the same IDS mutation as the subject
- No contraindications for intracisternal injection and immunosuppressive therapy

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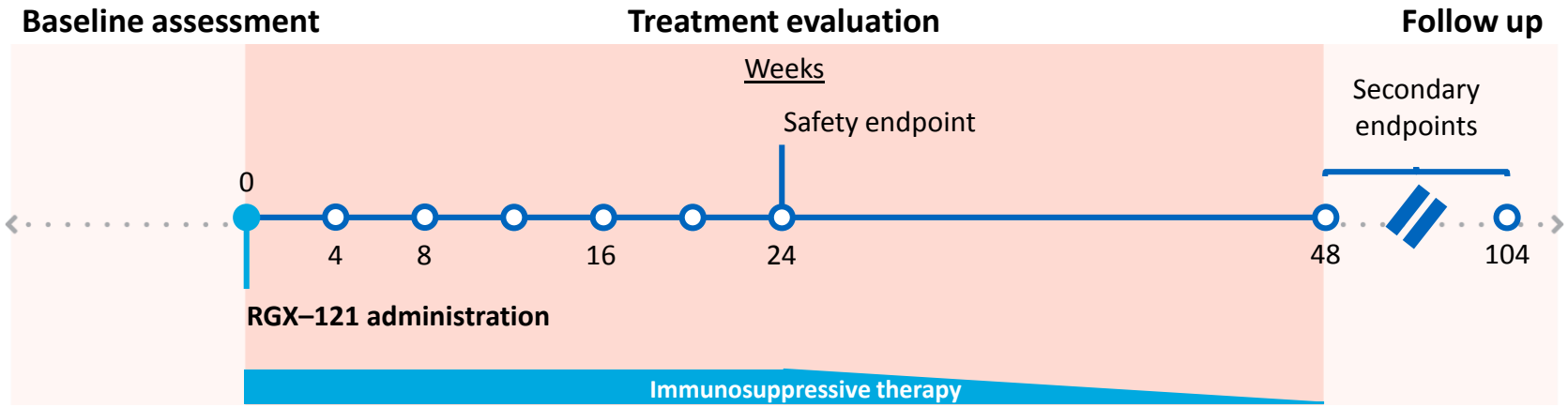
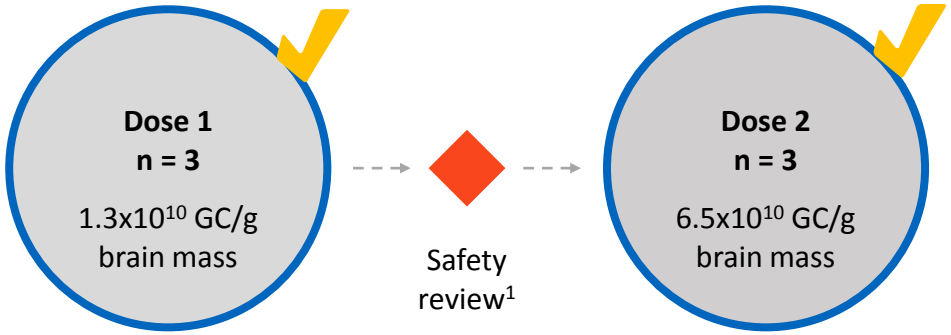
# **RGX–121 Phase I/II clinical trial:** Administration and dose escalation

## Administration and follow-up timeline




## Expected dose escalation pathway




**Dosing complete**

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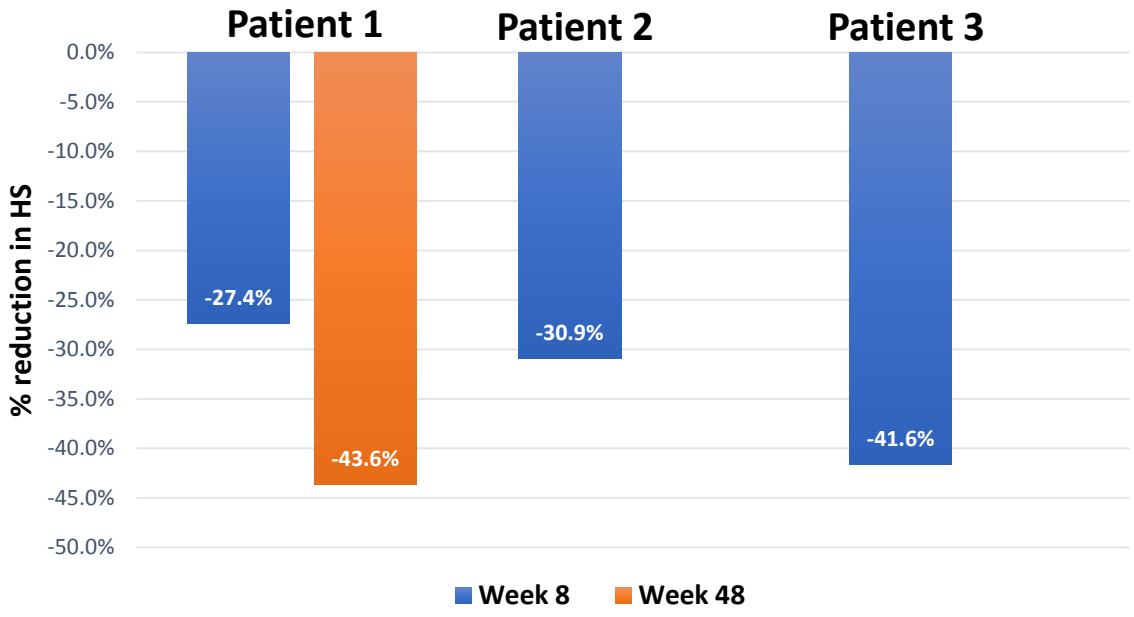
# **RGX–121 Phase I/II clinical trial:** Initial results from Cohort 1[1]

- *RGX-121 was **well-tolerated following one-time intracisternal administration** (n=3)*
  - No drug-related Serious Adverse Events (SAEs)
  - Patient 1 has completed immunosuppression regimen, per protocol
- *Demonstrated **consistent and sustained reduction in CSF levels of heparan sulfate,** a key biomarker of I2S activity*
- ***Early signs of neurocognitive stability observed***

**Heparan sulfate (HS) change from baseline as measured from cerebral spinal fluid**




[1] As announced December 18, 2019 and July 8, 2020

# RGX–111 Phase I clinical trial in MPS I

## Objectives

### Primary

- To determine the safety and tolerability of RGX–111 in MPS I subjects with neurocognitive deficits

### Secondary

- Effect of RGX–111 on biomarkers of IDUA activity in CSF, serum and urine
- Effect of RGX–111 on neurocognitive deficits

**Subjects**: Up to 5 total

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

### Key inclusion criteria

- Male or female ≥ 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
- No contraindications for intracisternal injection or immunosuppressive therapy

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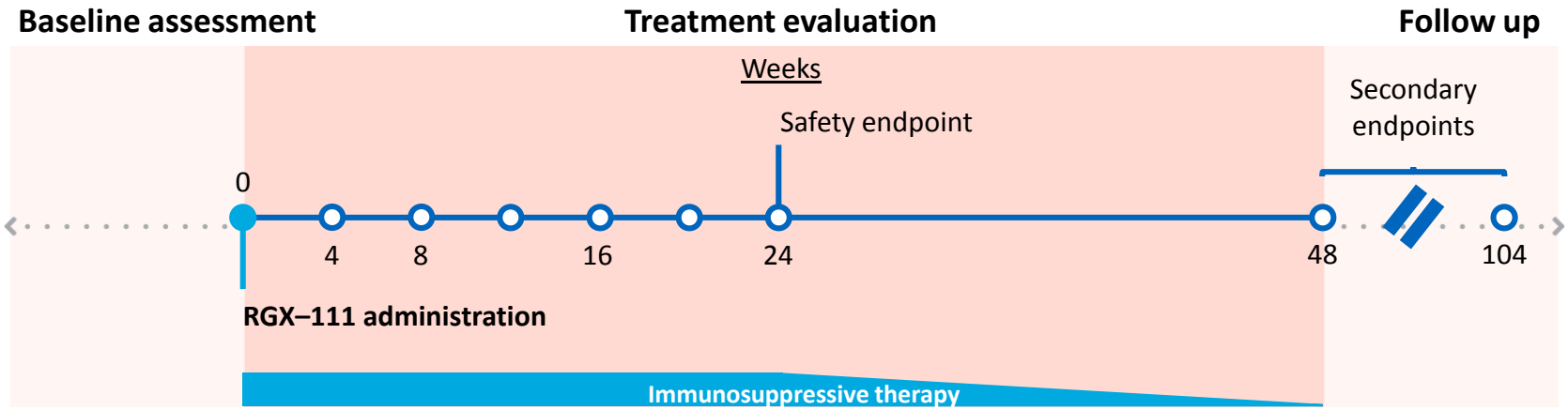
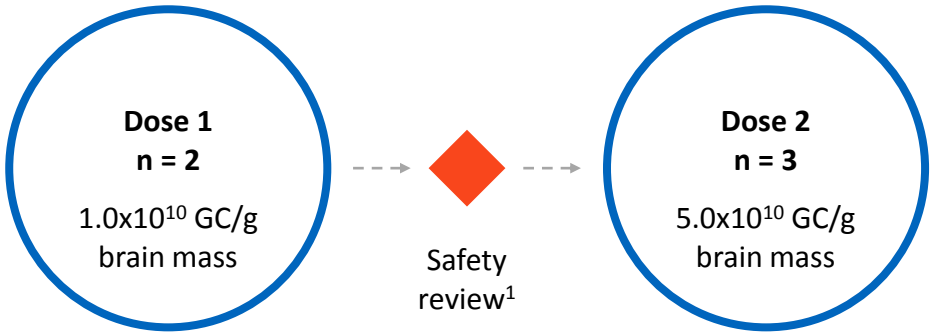
# **RGX–111 Phase I clinical trial:** Administration and dose escalation

## Administration and follow-up timeline




## Expected dose escalation pathway

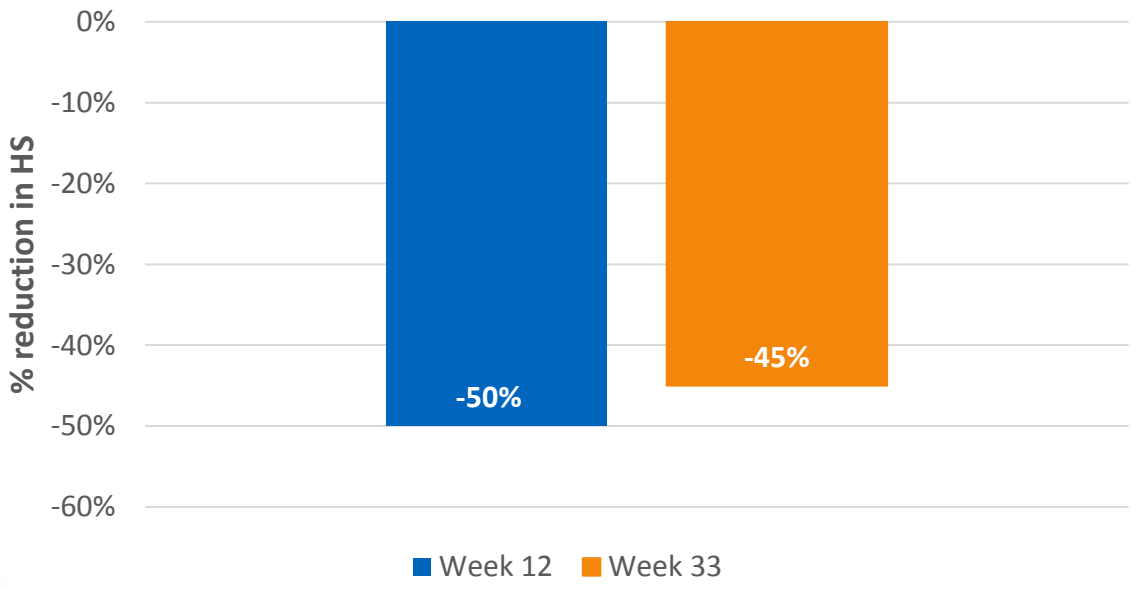



[1]Dose escalation safety review to occur eight weeks after final subject in cohort has been dosed

# **RGX–111 single-patient investigator-initiated IND:** Initial results[1]

- *RGX-111 was **well-tolerated following one-time intracisternal administration.***
  - No drug-related Serious Adverse Events (SAEs).
- *Demonstrated **increased IDUA enzyme activity in CSF** at Week 12, and **sustained reduction in CSF levels of heparan sulfate,** a key biomarker of IDUA activity.*
- *Patient has continued to **acquire cognitive developmental skills at a normal rate.***

**Heparan sulfate (HS) change from baseline as measured from cerebral spinal fluid**




[1] As announced July 8, 2020

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

## THE DISEASE

- Defective LDLR gene leads to limited / no LDL cholesterol receptors, allowing LDL to build up in bloodstream
- Total cholesterol levels >500 mg/dL
- Coronary artery disease at young age
- Even with existing standard of care therapies, the mean age of death is 32 years
- Approximately 11,000 patients worldwide

## RGX–501 PRODUCT CANDIDATE

**Vector:** AAV8

**Gene:** LDLR

### Mechanism of action

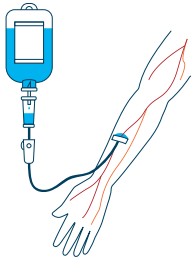
Correction of defective LDLR, reducing circulating LDL cholesterol

### Special Regulatory Status

Orphan Drug Designation

### Route of administration

Intravenous



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# RGX–501 Phase I/II clinical trial in HoFH

## Objectives

### Primary

- To determine the safety and tolerability of RGX-501 in subjects with HoFH

### Secondary

- Percent change in LDL-C levels at 12 weeks
- Other lipid outcome measures

**Subjects**: Up to 12 total

**Sites**: University of Pennsylvania as single center for RGX-501 administration. Penn or selected US and international sites for treatment evaluation and follow-up

**Key inclusion criteria**

- Male or female ≥ 18 years of age
- Untreated and/or treated LDL-C levels and clinical presentation consistent with the diagnosis of HoFH
- Molecularly defined LDLR mutations at both LDLR alleles
- Stable concurrent allowed lipid lowering medications
  - Statins, ezetimibe, bile acid sequestrants, PCSK9i

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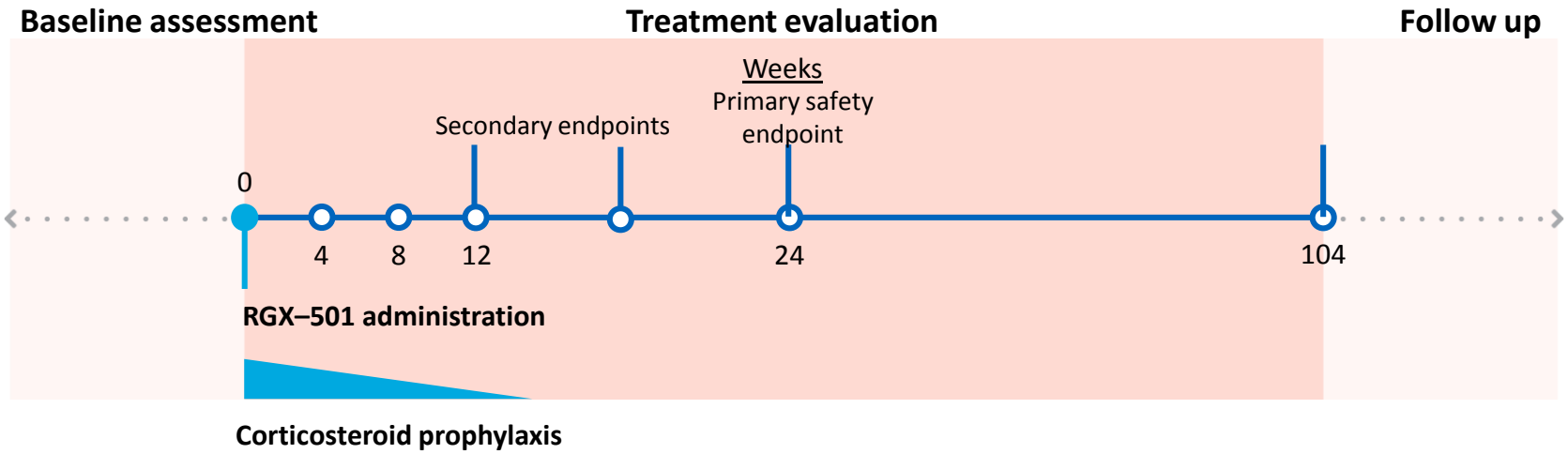
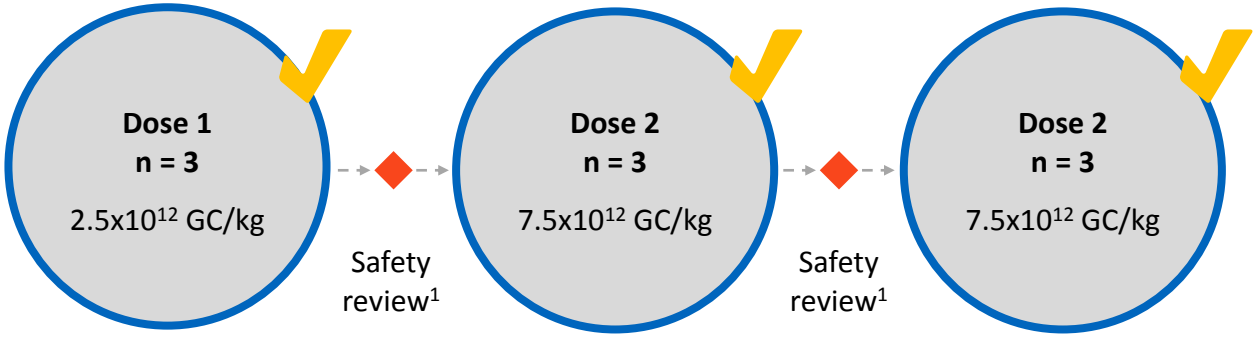
# RGX–501 Phase I/II clinical trial: Study design

## Administration and follow-up timeline




## Expected dose escalation pathway




**Dosing complete**

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

# NAV® Technology Platform

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# REGENXBIO | Industry leader in AAV production and manufacturing

***Deep in-house knowledge of vector characterization and strength in technical operations***

**3,000 ft$^2$** in-house GLP pilot plant with 3 X 200L bioreactor capacity
**18,000 ft$^2$** 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 be ready in late 2020
- cGMP manufacturing facility expected to be operational in 2021; will enable production at bioreactor scales up to 2,000L
- Integrated approach will allow for more efficient development and manufacturing of product candidates

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# 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

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## 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***

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

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*Zolgensma® is approved in the U.S. for the treatment of pediatric patients less than 2 years of age with SMA with bi-allelic mutations in the SMN1 gene

# 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

## 2020 YTD financials as of 3/31/20 (mm)

| | |
|---|---|
| **R&D expense:** | $37.0 |
| **G&A expense:** | $14.8 |
| **Net loss:** | $40.0 |
| **Basic share count:** | 37.2 |

## 2020 YTD financial highlights as of 3/31/20

| |
|---|
| Ended Q1 2020 with **$356.6 million in cash**[1] |
| Recognized YTD **$10 million in royalty** revenue from commercial sales of Novartis' Zolgensma |
| Recognized YTD **realized and unrealized loss of $5.1 million** on marketable equity securities of Prevail Therapeutics |

## Program guidance and anticipated milestones

| | |
|---|---|
| **RGX–314** | **Subretinal wet AMD:** Initiation of pivotal program in 2H 2020<br>**Suprachoroidal wet AMD:** Initiation of Phase II trial in 1H 2020<br>**Suprachoroidal DR:** IND submission in mid-2020 |
| **RGX–121** | **Interim data from Cohort 2 in 2H 2020** |
| **RGX–501** | **Interim data update in 1H 2020** |
| **RGX–111** | **Program update in 2H 2020** |
| **RGX–181** | **Program update in mid-2020**<br>**IND submission in 2H 2020** |

## 2020 financial guidance:

*As of March 31, 2020, REGENXBIO had **$356.6 million in cash**[1]. REGENXBIO expects these resources to fund completion of internal manufacturing capabilities and clinical advancement of its product candidates into 2022.*

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

# Thank You

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