Ken Mills
Chief Executive Officer

Leader in AAV Gene Therapy

October 25th, 2018
Forward-looking statements

This presentation includes “forward-looking statements,” within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended. These statements express a belief, expectation or intention and are generally accompanied by words that convey projected future events or outcomes such as “believe,” “may,” “will,” “estimate,” “continue,” “anticipate,” “design,” “intend,” “expect,” “could,” “plan,” “potential,” “predict,” “seek,” “should,” “would” or by variations of such words or by similar expressions. The forward-looking statements include statements relating to, among other things, REGENXBIO’s future operations, clinical trials, costs and cash flow. REGENXBIO has based these forward-looking statements on its current expectations and assumptions and analyses made by REGENXBIO in light of its experience and its perception of historical trends, current conditions and expected future developments, as well as other factors REGENXBIO believes are appropriate under the circumstances. However, whether actual results and developments will conform with REGENXBIO’s expectations and predictions is subject to a number of risks and uncertainties, including the timing of enrollment, commencement and completion and the success of clinical trials conducted by REGENXBIO, its licensees and its partners, the timing of commencement and completion and the success of preclinical studies conducted by REGENXBIO and its development partners, the timely development and launch of new products, the ability to obtain and maintain regulatory approval of product candidates, the ability to obtain and maintain intellectual property protection for product candidates and technology, trends and challenges in the business and markets in which REGENXBIO operates, the size and growth of potential markets for product candidates and the ability to serve those markets, the rate and degree of acceptance of product candidates, and other factors, many of which are beyond the control of REGENXBIO. Refer to the “Risk Factors” and “Management’s Discussion and Analysis of Financial Condition and Results of Operations” sections of REGENXBIO’s Annual Report on Form 10-K for the year ended December 31, 2017 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

**4 clinical stage programs**
with additional data readouts for RGX-314
and RGX-501 expected in Q4 2018

**12 clinical stage product candidates**
being developed by third-party licensees;
*over 20 partnered programs in total*

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

Management team and scientific advisors are
leaders in gene therapy
Key features of **REGENXBIO’s NAV® Technology Platform**

- Higher gene expression
- Longer-term gene expression
- Broad and novel tissue selectivity
- Lower immune response
- Improved manufacturability

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*The New England Journal of Medicine*

**Long-Term Safety and Efficacy of Factor IX Gene Therapy in Hemophilia B**

*nature biotechnology*

**Intravascular AAV9 Preferentially Targets Neonatal Neurons and Adult Astrocytes**
REGENXBIO’s **NAV Technology Platform** has been widely adopted

*Over 20 partnered product candidates being developed by NAV Technology Licensees*

<table>
<thead>
<tr>
<th>Research</th>
<th>Phase I / II</th>
<th>Phase III</th>
</tr>
</thead>
<tbody>
<tr>
<td>Indication</td>
<td>Licensee</td>
<td>Indication</td>
</tr>
<tr>
<td>Liver / hematologic</td>
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</tr>
<tr>
<td>Citrullinemia Type I</td>
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<td>Hemophilia A</td>
</tr>
<tr>
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<td><a href="#">ubravue</a></td>
<td>Hemophilia A</td>
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<td><a href="#">ubravue</a></td>
<td>OTC Deficiency</td>
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<td></td>
<td>Crigler-Najjar</td>
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<tr>
<td>Achromatopsia</td>
<td><a href="#">Biogen</a></td>
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<tr>
<td>Choroideremia</td>
<td><a href="#">Biogen</a></td>
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<td><a href="#">Prevaill</a></td>
<td>ALS SOD1</td>
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<td><a href="#">Voyager genome</a></td>
<td>Rett Syndrome</td>
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<tr>
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<td><a href="#">Voyager genome</a></td>
<td>MPS IIIA</td>
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<tr>
<td>Cardiac / skeletal muscle</td>
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<tr>
<td>Friedreich’s Ataxia</td>
<td><a href="#">Voyager genome</a></td>
<td>XLMTM</td>
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<tr>
<td></td>
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<td>Pompe Disease</td>
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<td></td>
<td></td>
<td>CPVT</td>
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</table>
# REGENXBIO’s lead programs

*Internally developed product candidates*

<table>
<thead>
<tr>
<th>Indication</th>
<th>Development Stage</th>
<th>Anticipated Milestones</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Retinal Disease</strong></td>
<td></td>
<td></td>
</tr>
<tr>
<td>RGX–314 Wet age-related macular degeneration (wet AMD)</td>
<td>Research</td>
<td>Preclinical</td>
</tr>
<tr>
<td><strong>Metabolic Disease</strong></td>
<td></td>
<td></td>
</tr>
<tr>
<td>RGX–501 Homozygous familial hypercholesterolemia (HoFH)</td>
<td>Research</td>
<td>Preclinical</td>
</tr>
<tr>
<td><strong>Neurodegenerative Disease</strong></td>
<td></td>
<td></td>
</tr>
<tr>
<td>RGX–111 Mucopolysaccharidosis Type I (MPS I)</td>
<td>Research</td>
<td>Preclinical</td>
</tr>
<tr>
<td>RGX–121 Mucopolysaccharidosis Type II (MPS II)</td>
<td>Research</td>
<td>Preclinical</td>
</tr>
<tr>
<td>RGX–181 Late-infantile neuronal ceroid lipofuscinosis Type 2 (CLN2 disease)</td>
<td>Research</td>
<td>Preclinical</td>
</tr>
</tbody>
</table>

▲ Orphan Drug Designation
★ Rare Pediatric Disease Designation
■ Fast Track Designation
RGX-314
Single treatment anti-VEGF program for wet AMD
More than 11M people estimated to be suffering from wet AMD, DME, and RVO worldwide.

Source: Global data, NHANES data, WHO publications, Peer reviewed articles as published in Pubmed.
CATT: 5-year outcomes in wet AMD patients

Over 3 Years of Clinical Follow-up, Mean Number of Injections Was 15.4

Across the Total Cohort, Initial Vision Gains Were Lost Over 5 Years

At Year 5:
- 83% of patients had retinal fluid
- 61% had intraretinal fluid
- 38% had subretinal fluid
- 36% had sub-RPE fluid

Strong correlation between frequent anti-VEGF treatment & vision gain

Clinical Utilization of Anti-VEGF Agents and Disease Monitoring in Neurovascular Age-Related Macular Degeneration

Wet AMD Anti-VEGF Treatment: Treatment Patterns & Efficacy in Clinical Practice Differ from CATT

Studies enforce that patients gain significant vision with increased compliance to anti-VEGF treatment

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

**RGX–314 PRODUCT CANDIDATE**

- **Vector:** AAV8
- **Gene:** anti-VEGF fab
- **Route of administration:** Subretinal

**Mechanism of action:**
Reducing leaky blood vessel formation by giving ocular cells the ability to produce an anti-VEGF fab

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Improved AAV vector technology

- AAV8
- AAV2

More efficient gene delivery to the RPE

Leveraging current standard of care in transgene

- Current standard of care includes FDA-approved mAbs and mAb fragments that inhibit VEGF
- RGX–314 gene encodes an anti-VEGF mAb fragment (fab)

**RGX–314:**

- AAV8 encoding anti–VEGF fab

**Potential for long-term therapeutic anti-VEGF expression**

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1 Vandenberge et al. 2011 Science Translational Medicine
RGX–314 has **significant potential advantages** over earlier generation candidates for wet AMD gene therapy

<table>
<thead>
<tr>
<th>Sponsor</th>
<th>genzyme⁴</th>
<th>AVALANCHE²</th>
<th>REGENXBIO¹</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Vector</strong></td>
<td>AAV2</td>
<td>AAV2</td>
<td>AAV8</td>
</tr>
<tr>
<td><strong>ROA</strong></td>
<td>Intravitreal</td>
<td>Subretinal</td>
<td>Subretinal</td>
</tr>
<tr>
<td><strong>Transgene</strong></td>
<td>sFLT01</td>
<td>sFlt</td>
<td>anti-VEGF fab</td>
</tr>
<tr>
<td><strong>Dose</strong> (GC/eye)</td>
<td>2.4e10</td>
<td>8.0e11</td>
<td>1.0e11</td>
</tr>
<tr>
<td><strong>Max. expression</strong> (ng/ml)²</td>
<td>528</td>
<td>0.217</td>
<td>4,992</td>
</tr>
</tbody>
</table>

¹ Wielechowski et al. 2016 Poster session presented at the meeting of the American Society of Gene & Cell Therapy, Washington, DC
² Maximum expression in the anterior chamber of non-human primate eyes
³ Lai et al. 2012 Gene Therapy
⁴ MacLachlan et al. 2011 Molecular Therapy
RGX–314 Phase I clinical trial – study overview

**Objectives**

**Primary**
- To determine the safety and tolerability of RGX–314 in subjects with wet AMD though six months

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

**Subjects:** 24 subjects dosed

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

**Key inclusion criteria**
- Male or female ≥ 50 to 89 years of age
- Wet AMD subjects requiring frequent anti–VEGF therapy, with a documented history of response
- Documented response to anti–VEGF at trial entry (assessed by SD–OCT at week 1)
- Vision of 20/63 to 20/400 for the initial subject, then 20/40 to 20/400 for the rest of each cohort
- Pseudophakic (status post cataract surgery)
RGX–314 Phase I clinical trial – administration and dose escalation

Administration and follow-up timeline

Expected dose escalation pathway

Dose 1
n = 6
3x10⁸ GC/eye

Dose 2
n = 6
1x10¹⁰ GC/eye

Dose 3
n = 6
6x10¹⁰ GC/eye

Dose 4
n = 6
1.6x10¹¹ GC/eye

Safety review¹

Safety review¹

Safety review¹

Anti–VEGF rescue therapy if needed

Baseline assessment

Treatment evaluation

Follow up

Weeks

anti–VEGF injection

SD–OCT assessment

Safety endpoint

Secondary endpoints

0 1 2 6 10 14 18 22 26 54 106

RGX–314 administration

Baseline assessment

Treatment evaluation

Follow up

Weeks

anti–VEGF injection

SD–OCT assessment

Safety endpoint

Secondary endpoints

0 1 2 6 10 14 18 22 26 54 106

RGX–314 administration

Dosing completed in four cohorts (24 total patients)

¹ Dose escalation safety review to occur four weeks after final subject in each cohort has been dosed
RGX–314 standardized automated subretinal delivery procedure

Performed under local anesthesia in the OR

- Standard small gauge vitrectomy to perform a core vitrectomy
- Automated delivery with a MedOne subretinal cannula attached to the vitrectomy machine
- Inject 250μl to create a single bleb subretinally in a healthy area of retina
- Target superior to the superotemporal arcade vessel or outside the arcades
- Can select a second bleb area if needed
- Keep margin of the bleb at least 2DA away from the fovea

Air fluid exchange and sub-conjunctival steroids at the end of procedure; No positioning mandated and patient is discharged home with follow-up the next day
RGX–314 Phase I clinical trial – safety summary for Cohorts 1-3*

- **RGX–314 was well-tolerated**
- **No drug-related AEs or drug-related SAEs**
- Most AEs were assessed as mild (Grade 1 – 83%)
- **No observed immune responses**, drug-related ocular inflammation, or any post-surgical inflammation beyond what is expected following routine vitrectomy
- **Five SAEs that were not drug-related were reported in three subjects**

*As of July 27th, 2018
RGX–314 Phase I clinical trial - protein levels at one month for Cohorts 1-3

As Measured from Aqueous Samples by ECL

<table>
<thead>
<tr>
<th>Cohort</th>
<th>RGX–314 Protein (ng/mL)</th>
<th>N</th>
</tr>
</thead>
<tbody>
<tr>
<td>1</td>
<td>2.4 x 10^9 GC/eye</td>
<td>6</td>
</tr>
<tr>
<td>2</td>
<td>12.8 x 10^9 GC/eye</td>
<td>6</td>
</tr>
<tr>
<td>3</td>
<td>160.2 x 10^9 GC/eye</td>
<td>6</td>
</tr>
</tbody>
</table>
# RGX-314 Phase I clinical trial - summary through six months for Cohorts 1-3

<table>
<thead>
<tr>
<th>Cohort</th>
<th>Aqueous RGX-314 Protein 1 Month Post-treatment</th>
<th>Mean # of Anti-VEGF Injections Through 6 Months</th>
<th>Mean Change in CRT Through 6 Months (Range)</th>
<th>Mean Change in BCVA Through 6 Months</th>
</tr>
</thead>
<tbody>
<tr>
<td>Cohort 1</td>
<td>2.4 ng/ml</td>
<td>4.7 inj*</td>
<td>-14 µm** (-181 to +92 µm)</td>
<td>-2 letters** (-8 to +10 letters)</td>
</tr>
<tr>
<td>3x10^9 GC/eye (N=6)</td>
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<td></td>
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</tr>
<tr>
<td>Cohort 2</td>
<td>12.8 ng/ml</td>
<td>3.8 inj</td>
<td>+26 µm (-7 to +62 µm)</td>
<td>+7 letters (-4 to +15 letters)</td>
</tr>
<tr>
<td>1x10^10 GC/eye (N=6)</td>
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<td></td>
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</tr>
<tr>
<td>Cohort 3</td>
<td>160.2 ng/ml</td>
<td>1.3 inj</td>
<td>-14 µm (-27 to +7 µm)</td>
<td>+8 letters (0 to +21 letters)</td>
</tr>
<tr>
<td>6x10^10 GC/eye (N=6)</td>
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</table>

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

**N=5; one subject in Cohort 1 discontinued from the study at four months
Cohort 3: Three subjects with no additional anti-VEGF injections through month six

- **Previous therapy**
  - Study subjects received on average \textbf{>35 injections since wet AMD diagnosis}

- **Post-RGX–314 anti-VEGF injections**
  - \textbf{0 injections} through six months post-RGX–314

- **BCVA**
  - Mean gain in BCVA of \textbf{+8 ETDRS} letters from baseline through six months

- **SD–OCT**
  - Maintained with a \textbf{mean change in CRT of -21 µm} from baseline through six months
## The REGENXBIO team

<table>
<thead>
<tr>
<th>Name</th>
<th>Position</th>
<th>Prior Affiliations</th>
</tr>
</thead>
<tbody>
<tr>
<td>Ken Mills</td>
<td>President, CEO &amp; Co-Founder; Director</td>
<td><a href="#">Foxkiser</a></td>
</tr>
<tr>
<td>Olivier Danos, Ph.D.</td>
<td>SVP and Chief Scientific Officer</td>
<td><a href="#">Biogen</a></td>
</tr>
<tr>
<td>Vit Vasista</td>
<td>SVP and Chief Financial Officer</td>
<td><a href="#">PRTM</a></td>
</tr>
<tr>
<td>Stephen Yoo, M.D.</td>
<td>SVP and Chief Medical Officer</td>
<td><a href="#">MedImmune</a>; <a href="#">Abbott</a></td>
</tr>
<tr>
<td>Curran Simpson</td>
<td>SVP, Product Development and Chief Technology Officer</td>
<td><a href="#">GSK</a>; <a href="#">Human Genome Sciences</a></td>
</tr>
<tr>
<td>Ram Palanki, Pharm.D.</td>
<td>SVP, Commercial Strategy and Operations</td>
<td><a href="#">Santen</a>; <a href="#">Genentech</a></td>
</tr>
<tr>
<td>Patrick Christmas, J.D.</td>
<td>SVP and General Counsel</td>
<td><a href="#">Lumara Health</a>; <a href="#">Wellspring Therapeutics</a></td>
</tr>
<tr>
<td>Laura Coruzzi, Ph.D., J.D.</td>
<td>SVP, Intellectual Property</td>
<td><a href="#">Jones Day</a></td>
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<tr>
<td>Shiva Fritsch</td>
<td>SVP, Human Resources</td>
<td><a href="#">Novavax</a></td>
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Financial results and guidance

2018 1H financials (mm)

<p>| | |</p>
<table>
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<tbody>
<tr>
<td>R&amp;D expense:</td>
<td>$41</td>
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<tr>
<td>G&amp;A expense:</td>
<td>$17</td>
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<tr>
<td>Net income:</td>
<td>$115</td>
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<tr>
<td>Basic sharecount¹:</td>
<td>35.4</td>
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Program guidance and anticipated milestones

<table>
<thead>
<tr>
<th>Program</th>
<th>Milestone</th>
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<tbody>
<tr>
<td>RGX–314</td>
<td>Complete dosing of Cohort 4 Q4 2018 and initiate Phase II trial in 2019</td>
</tr>
<tr>
<td>RGX–501</td>
<td>Additional topline data Q4 2018</td>
</tr>
<tr>
<td>RGX–111</td>
<td>IND active and subject recruiting initiated; begin enrollment in Phase I</td>
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<tr>
<td></td>
<td>trial Q4 2018</td>
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<tr>
<td>RGX–121</td>
<td>IND active and subject recruiting initiated; begin enrollment in Phase I/II</td>
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<tr>
<td></td>
<td>trial Q4 2018</td>
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<tr>
<td>RGX–181</td>
<td>IND submission 2019</td>
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Financial highlights

- In June 2018, received $100 million from AveXis for amended SMA license agreement
- Ended Q2 2018 with over $306 million in cash
- Closed public offering in August 2018, raising over $200 million in gross proceeds

2018 financial guidance:

Expect 2018 ending cash balance to be between $440 million and $450 million²

¹ Basic sharecount of 32.3 million shares as of August 3, 2018 plus 3.1 million shares issued in public offering in August 2018
² Cash includes cash, cash equivalents and marketable securities for the purposes of this presentation. The expected 2018 ending cash balance represents the previously announced expected range of $250–$260 million after including the effect of REGENXBIO’s underwritten public offering of common stock in August 2018