Visionary Science for Life Changing Cures
Forward Looking Statements

Today's presentation includes forward-looking statements intended to qualify for the Safe Harbor from liability established by the Private Securities Litigation Reform Act of 1995. These forward-looking statements, including statements regarding our planned pre-clinical and clinical studies, timing or ability to close partnerships, regulatory approval process and demand for our product candidates, are subject to risks, uncertainties and other factors that could cause actual results to differ materially from those suggested by our forward-looking statements.

These factors include, but are not limited to, the following:

- We have incurred significant losses since inception and anticipate that we will continue to incur significant losses for the foreseeable future.
- Our ability to generate revenue from product sales is highly uncertain.
- We may need to raise additional funding in the future, which may not be available on acceptable terms, or at all.
- No gene therapy products have been approved in the United States, and we may not be able to obtain regulatory approvals for our product candidates.
- We have encountered and may continue to encounter substantial delays in our clinical trials or fail to demonstrate safety and efficacy to the satisfaction of applicable regulatory authorities.
- We rely on third parties to conduct, supervise and monitor our clinical trials and to conduct certain aspects of our product manufacturing and protocol development.
- The insurance coverage and reimbursement status of our product candidates is uncertain.
- Negative public opinion and increased regulatory scrutiny of gene therapy and genetic research may adversely affect public perception of our product candidates and prospects for our business.
- If we are unable to obtain and maintain adequate patent protection for our technology and products our competitors could develop and commercialize technology and products similar or identical to ours.
Company Highlights

AGTC is developing genetic therapies to treat patients with inherited diseases. Treatments are precisely designed to meet the needs of each specific genetic disorder. AGTC’s most advanced gene therapy programs are designed to restore visual function in patients with rare blinding diseases.

- **Clear Vision**: Become the leader in ophthalmology and otology gene therapy
- **Deep Expertise**: In vector selection, design, manufacturing and delivery
- **Extensive IP Portfolio**: >100 patents and patent applications protecting candidate genes, vector capsids, manufacturing and delivery
- **Broad Pipeline**: Multiple opportunities to provide long-term value to patients
- **Key Partnership**: Broad collaboration with Biogen to develop gene therapies in ophthalmology & other indications
# Pipeline: Multiple Shots on Goal

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<th>Program</th>
<th>Gene</th>
<th>Proof -of-Concept</th>
<th>IND-Enabling</th>
<th>Phase 1</th>
<th>Pivotal</th>
<th>Partnering</th>
<th>Next Key Milestones</th>
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<tr>
<td>XLRS *</td>
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<td>Biogen</td>
<td>Complete Enrollment</td>
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<td>ACHM</td>
<td>CNGB3</td>
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<td>Target announcement</td>
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</tbody>
</table>

- **Target-1** and **Target-2** are Wholly Owned projects.
- **RS1**, **CNGB3**, and **CNGA3** are Wholly Owned projects.
- **Biogen** is the Partnering company for **RS1** and **RPGR**.
- **Complete Enrollment** is the next key milestone for **XLRS**.
- **Complete Enrollment** is the next key milestone for **ACHM**.
- **File IND** is the next key milestone for **CNGA3**.
- **File IND** is the next key milestone for **XLRP**.
- **Target announcement** is the next key milestone for **Target-1** and **Target-2**.
AGTC’s Technology Platform

Genetic therapies are complex with interdependent components that must work in harmony.

Fifteen years of gene therapy experience allows AGTC to design and construct all critical gene therapy elements and bring them together to develop successful treatments for patients.
Why Ophthalmology

**Significant Unmet Medical Need**

- No current treatments
- Many people fear blindness
- Blinding eye diseases significantly affect quality of life

**Growing Scientific Support**

- Extensive preclinical data supports safety and efficacy
- Well-defined clinical endpoints
- Preliminary evidence of safety and efficacy in humans

**Restoring Sight**

- Child with achromatopsia outdoors
- Same child indoors in dim light

- Day blindness
- 20/200
The Eye

The Retina - a highly organized tissue

- Retinal pigment epithelial cell
- Rod cell
- Cone cell
- Mueller cell
- Horizontal cell
- Bipolar cell
- Amacrine cell
- Ganglion cell

Light enters retina

- Rod diseases
- Cone diseases
- AMD

- Pupil
- Iris
- Cornea
- Lens
- Retina
- Vitreous humour
- Optic nerve
- Fovea
Clear Opportunity for Expansion

290 genetic causes of blindness and over 65 genetic causes of deafness mapped to a single locus

Scientific Criteria

Strategic Criteria

Selection

Design

Dev

Significant Opportunity for Value Creation
Lead Product Candidates
**X-linked Retinoschisis (XLRS)**

**Disease**
- Poor vision caused by missing structural protein
- No current treatments

**Impact**
- Poor vision (20/100) detected by school age
- Difficulty reading, driving, or recognizing faces
- 30% chance of retinal detachment or vitreous hemorrhage at any age

**Positioned for Success**
- Robust animal models
- Defined clinical endpoints
- Strong IP position
- Ongoing clinical trial
**XLRS – Study Design**

**Dose level**
- **Highest**: $6 \times 10^{11}$ vg
- **Middle**: $3 \times 10^{11}$ vg
- **Lowest**: $1 \times 10^{11}$ vg

**Cohort 1a**
- n=3

**Cohort 1b**
- n=3

**Cohort 2**
- n=3

**Cohort 3**
- N=3

**Cohort 4**
- N=15

**Age:**
- $\geq 18$ years
- $\geq 6$ years

**BCVA:**
- $\leq 20/80$
- $\leq 20/63$
- $\leq 20/50$

**STOP**
- **DSMB Review**

- Dose escalation in adults, then MTD in adults and children
- Early cohorts have worse visual acuity
- Primary endpoint is safety
Current Status

• Natural History Study (NHS)
  – Enrollment complete
  – Analyzing data in preparation for publication
  – Important step to inform active trial

• Phase 1/2 Clinical Study
  – Active at 7 centers in the U.S.
  – Eight patients enrolled
  – Multiple patients in screening program
  – Study agent is well tolerated
  – No significant improvements in analyzed efficacy endpoints at lowest dose
But What Came Before?

• Early Academic Research
  – Funded by NIH and FFB

• Design and Screening of Gene Therapy Vector
  – Funded by FFB and AGTC

• Formal Pre-Clinical Safety Studies
  – Funded by FFB and AGTC

• Natural History Study
  – Funded by FFB and AGTC

*FFB funding critical to early data; AGTC funding critical to accelerating path to clinic*
Achromatopsia

**Disease**
- Cone photoreceptors do not function due to missing cell membrane protein
- No current treatments

**Impact**
- Patients are legally blind
- Extremely light sensitive
- No color discrimination

**Positioned for Success**
- Robust animal models
- Defined clinical endpoints
- Strong IP position
- Ongoing clinical trial

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**Day vision**
*Cone-mediated vision predominates under brightly lit conditions*

**Night vision**
*Rod-mediated vision predominates under dimly lit conditions*
**ACHM Study Design**

**Dose level**
- **Highest**
  - $1.35 \times 10^{12} \text{ vg}$
- **Middle**
  - $4.5 \times 10^{11} \text{ vg}$
- **Lowest**
  - $1.8 \times 10^{11} \text{ vg}$

**Cohort 1**
- $n=3$

**Cohort 2**
- $n=3$

**Cohort 3**
- $n=3$

**Cohort 4**
- $N=15$

**Age:**
- $\geq 18$ years
- $\geq 6$ years

- Dose escalation in adults, then expanded cohort includes children
- Primary endpoint is safety

**DSMB Review**
Current Status

• Natural History Study (NHS)
  – Enrollment complete
  – Analyzing data in preparation for publication
  – Important step to inform active trial

• Phase 1/2 Clinical Study
  – Active at 4 centers in the U.S.
  – Two patients enrolled
  – Trial could be fully enrolled from NHS patients
But What Came Before?

• Early Academic Research
  – Funded by NIH and AGTC

• Design and Screening of Gene Therapy Vector
  – Funded by NIH and AGTC

• Formal Pre-Clinical Safety Studies
  – Funded by NIH and AGTC

• Natural History Study
  – Funded by NIH and AGTC

*NIH funding critical to initiating ACHM program; AGTC funding and experience accelerated path to clinic*
Partnership Benefits

• Sponsored Research
  – Often first money in to get key first data
  – From initial grant to expanded partnership
    • Later stage partnerships often include “Payback” of investment upon product commercialization

• Provides Important Credibility

• Scientific Discussion; Key Opinion Leader Access

• Patient Input; Patient Access

• Leverage for Success
  – Combine funding sources
  – Combine areas of expertise
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