Visionary Science for Life Changing Cures 2018
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 reflect AGTC’s plans, estimates, assumptions and beliefs. Forward-looking statements include information concerning possible or assumed future results of operations, business strategies and operations, preclinical and clinical product development and regulatory progress, potential growth opportunities, potential market opportunities and the effects of competition. Forward-looking statements include all statements that are not historical facts and can be identified by terms such as "anticipates," "believes," "could," "seeks," "estimates," "expects," "intends," "may," "plans," "potential," "predicts," "projects," "should," "will," "would" or similar expressions and the negatives of those terms.

Actual results could differ materially from those discussed in the forward-looking statements, due to a number of important factors. Risks and uncertainties that may cause actual results to differ materially include, among others: gene therapy is still novel with only a few approved treatments so far; AGTC cannot predict when or if it will obtain regulatory approval to commercialize a product candidate or receive reasonable reimbursement; uncertainty inherent in clinical trials and the regulatory review process; risks and uncertainties associated with drug development and commercialization; factors that could cause actual results to differ materially from those described in the forward-looking statements are set forth under the heading "Risk Factors" in the Company’s most recently filed Annual Report on Form 10-K.

Given these uncertainties, you should not place undue reliance on these forward-looking statements. Also, forward-looking statements represent management’s plans, estimates, assumptions and beliefs only as of the date of this presentation. Except as required by law, AGTC assumes no obligation to update these forward-looking statements publicly or to update the reasons actual results could differ materially from those anticipated in these forward-looking statements, even if new information becomes available in the future.
AGTC’s Comprehensive Platform

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

Greater than fifteen years of gene therapy experience allows AGTC to design and construct all critical gene therapy elements and bring them together to develop promising treatments for patients
# Lead Product Pipeline

## Multiple Shots on Goal

<table>
<thead>
<tr>
<th></th>
<th>Phase</th>
<th>Patients – US &amp; EU</th>
<th>Partner</th>
<th>Key Platform</th>
<th>Next Milestone</th>
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<tbody>
<tr>
<td>XLRS RS1</td>
<td>Phase 1</td>
<td>35,000</td>
<td>Biogen</td>
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<td>Topline interim six-month data by end of 2018</td>
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<td>ACHM CNGB3</td>
<td>Phase 1</td>
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<td>Complete dose escalation in the first quarter of 2019</td>
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<td>CNGA3</td>
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<td>XLRP RPGR</td>
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<td>Optogenetics Unique ChR</td>
<td>IND Enabling</td>
<td>Multiple Indications</td>
<td>Bionic Sight, LLC</td>
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<td>File IND in the first half of 2019</td>
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<td>ALD ABCD1</td>
<td>Proof of Concept</td>
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<td>Biogen</td>
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<td>Option Decision</td>
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<td>Discovery Programs</td>
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<td>Biogen</td>
<td></td>
<td>Target Announcement</td>
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Lead Product Candidates
X-linked Retinoschisis (XLRS)

**Disease**
- Missing structural protein results in poor vision not correctable with eyeglasses
- ~35,000 patients in US and EU
- No current treatments

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

**Positioned for Success**
- Robust analysis of tissue targeting and functional improvement in animal models
- Understanding of human disease phenotype from natural history
- Accepted clinical endpoints
- Strong IP position
• 9 active trial sites in U.S.
  – All are leading centers and KOLs for inherited retinal diseases
• Initial targeted enrollment completed
  – Dose escalation phase (low, middle, and high dose)
    ✓ 12 patients enrolled
  – Additional middle dose pediatric group
    ✓ 5 children enrolled
  – High dose expansion group
    ✓ 10 adults enrolled
• Currently enrolling 5 additional pediatric patients
  – Extension of high dose expansion group
Achromatopsia (ACHM)

Disease
- 70% of ACHM is caused by mutations in the A3 and B3 genes. AGTC is currently working on these two genetics mutations that result in severely impaired vision and day blindness
- Missing cone photoreceptor protein results in poor vision not correctable with eyeglasses
- ~28,000 patients in US and EU
- No current treatments

Impact
- Extremely poor vision, legally blind
- Extreme light sensitivity (day blind)
- Complete loss of color discrimination

Positioned for Success
- Robust animal models showing potential to improve visual function
- Understanding of human disease phenotype from natural history
- Accepted clinical endpoints
- Strong IP position
ACHM - Phase 1/2 Current Status

• Enrollment as of Sept conference call
  – ACHM-B3
    • Group 1: Original Dose: 4 patients enrolled
    • Group 2: Low Dose: 2 patients enrolled
    • Group 3: Middle Dose: 2 patients enrolled
  – ACHM-A3
    • Group 1: Low Dose: 2 patients enrolled

• Site status
  – ACHM-B3: 5 sites actively enrolling patients
  – ACHM-A3: 5 sites actively enrolling patients
  – Actively identifying and initiating several additional sites
X-linked Retinitis Pigmentosa (XLRP)

**Disease**
- Missing protein results in degeneration of rods and cones
- ~20,000 patients in US and EU
- No current treatments

**Impact**
- Early night blindness, progressive constriction of visual fields
- Legally blind by age 45

**Positioned for Success**
- Robust animal models showing potential to improve vision
- Primate photoreceptor targeting
- Understanding of human disease phenotype from natural history
- Accepted clinical endpoints
- Strong IP position
XLRP - Phase 1/2 Current Status

• First Patient
  – Treated April 16\textsuperscript{th} 2018
  – Earned $2.5M BIIB milestone

• Fourth Patient
  – Treated July 17\textsuperscript{th} 2018
  – Earned $10.0M BIIB milestone

• Total of 5 patients enrolled as of Sept conference call
  – 3 sites actively enrolling patients
  – Actively identifying and initiating several additional sites
Optogenetics

• Collaboration with Bionic Sight
  – Bypass damaged tissue, directly stimulate cells to send visual information to the brain
• Pre-IND Meeting completed with FDA to get guidance on toxicology, CMC & clinical trial plans
• IND-enabling GLP mouse tox study completed
• GMP Clinical Trial Material in-process
• Clinical trial planning underway
  – Will include safety evaluation and preliminary light sensitivity testing
• IND submission targeted for first half 2019
Realizing the Promise of Gene Therapy
## Experienced Team

<table>
<thead>
<tr>
<th>Name</th>
<th>Title</th>
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<tbody>
<tr>
<td>Sue Washer</td>
<td>Chief Executive Officer</td>
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<tr>
<td>Stephen Potter</td>
<td>Chief Business Officer</td>
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<tr>
<td>Mark Shearman, Ph.D.</td>
<td>Chief Scientific Officer</td>
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<tr>
<td>Bill Sullivan</td>
<td>Chief Financial Officer</td>
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<tr>
<td>Matt Feinsod, M.D.</td>
<td>Chief Medical Officer</td>
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<tr>
<td>Lanita Scott, M.D.</td>
<td>VP, Clinical Research</td>
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<tr>
<td>Karen Carroll, RN</td>
<td>VP, Clinical Operations</td>
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Manufacturing Expertise and Capacity

• Manufacturing strength
  – Suspension system scalable to large volumes
  – Process adaptable to any serotype
  – **Productivity**
    • *Actual Phase 1/2 process; 25 L batch provides >2000 doses*
    • *New pivotal process; 10 fold higher productivity*

• Expertise
  – Successfully transferred to multiple parties
  – 7 successful cGMP batches completed
  – Completely integrated process & analytics
  – Analytics advancing to late stage readiness
Financial Summary
Strong Balance Sheet

$104.9 Million
Cash & Investment as of 6/30/2018
Represents > two years of cash

Sufficient Cash to:

Complete enrollment and analysis
of full data set from the ongoing Phase 1/2 human clinical trials for XLRS and both of the planned ACHM Phase 1/2 human clinical trials

Initiate and analyze
initial data from the Phase 1/2 human clinical trial for XLRP

Move pre-clinical optogenetic program into the Clinic

and fund planned R&D and PD
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