ASPIRO Study
Interim data as of December 21, 2017

Courageous Patients. Bold Effort.
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AT132 OVERVIEW

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### XLMTM Overview

#### A fatal disease with no treatment options

| Serious, life-threatening rare disease | ➤ Significant impairment of respiratory and neuromuscular function  
➤ Estimated 50% mortality by 18 months  
➤ Incidence: 1 in 50K newborn males |
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<tr>
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<tbody>
<tr>
<td>Monogenic, well-understood biology</td>
<td>➤ MTM1 gene encodes myotubularin, an enzyme required for normal development and function of skeletal muscle</td>
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</table>
| Target for AAV gene therapy | ➤ AAV8 effectively penetrates skeletal muscle  
➤ Muscle tissue is otherwise reasonably healthy and exhibits no dystrophic or inflammatory change |
| Clear clinical measures | ➤ Multiple endpoints to evaluate neuromuscular and respiratory function  
➤ Developmental milestones  
➤ Muscle biopsy |

<table>
<thead>
<tr>
<th><strong>AT132 Clinical Development Program</strong></th>
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### RECENSUS
Retrospective Medical Chart Review of Patients with XLMTM
- N=140
- Characterize aspects of the disease and medical management of XLMTM
- Identify potential outcome measures for ASPIRO
- Initial presentation Q1:17; First publication Q4:17

### INCEPTUS
Prospective Natural History Run-in Study in XLMTM Patients
- N≈16
- Longitudinal baseline and within-patient control for ASPIRO
- Facilitates enrollment in and operational aspects of ASPIRO
- Prelim. data shared Q4:17

### ASPIRO
A Phase 1/2 Clinical Study in XLMTM Patients
- N=12
- Assessment of safety and tolerability and preliminary efficacy
- Focus on neuromuscular & respiratory measurements
- First patient dosed Q3:17; Positive interim data reported for Cohort 1 in Q1:18

- Orphan Drug Designation received from FDA and EMA
- Rare Pediatric Disease and Fast Track designations granted by FDA
AUDENTES

ASPIRO: TO ASPIRE...

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# ASPIRO Phase 1/2 Clinical Study

Open-label, ascending-dose, safety and preliminary efficacy study

## Inclusion Criteria
- Subject is male
- <5 yrs old, or enrolled in INCEPTUS
- Genetically confirmed XLMTM
- Requires ventilator support

## Key Efficacy Assessments

### Neuromuscular
- CHOP INTEND
- MFM-20
- Bayley III
- Muscle biopsy
- Developmental milestones

### Respiratory
- Max Inspiratory Pressure (MIP)
- Ventilator use
- Respiratory sprinting

## Design
- N=12, roll-over from INCEPTUS
- 3 ascending-dose cohorts (3 active plus a delayed-treatment concurrent control)
- Doses: $1 \times 10^{14}$, $3 \times 10^{14}$, $5 \times 10^{14}$ vg/kg

## AT132 administration

- Subjects from INCEPTUS
- Baseline - Wk 1
- Wk 1 - 4: Prednisolone
- Wk 5 - 9 taper
- Long-term follow-up: Wk 12, 6 mos., 9 mos., 12 mos.

## Assessments

<table>
<thead>
<tr>
<th></th>
<th>Neuromuscular</th>
<th>Respiratory</th>
<th>Developmental milestones</th>
<th>Muscle biopsy</th>
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<tbody>
<tr>
<td>Baseline</td>
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<td>Wk 1</td>
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<td>Wk 2</td>
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<td>Wk 12</td>
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<td>6 mos.</td>
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<td>9 mos.</td>
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<tr>
<td>12 mos.</td>
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ASPIRO Clinical Study Sites

Same study sites as INCEPTUS natural history run-in study

- Ann & Robert H. Lurie Children's Hospital of Chicago, Illinois, USA
- SickKids, Ontario, Canada
- UCLA Health, California, USA
- NIH, Maryland, USA
- UF, Florida, USA
- London, UK
- Paris, France
- München, Germany
ASPIRO Cohort 1
All four Cohort 1 patients rolled over from INCEPTUS to ASPIRO

<table>
<thead>
<tr>
<th>Subject Number</th>
<th>Age at ASPIRO Baseline (yrs)</th>
<th>Age at baseline and follow up duration</th>
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<tbody>
<tr>
<td></td>
<td></td>
<td>0 -12 months</td>
</tr>
<tr>
<td>Pt 1</td>
<td>0.8</td>
<td></td>
</tr>
<tr>
<td>Pt 2</td>
<td>4.1</td>
<td></td>
</tr>
<tr>
<td>Pt 3</td>
<td>2.6</td>
<td></td>
</tr>
<tr>
<td>Pt 4 (Ctl)</td>
<td>4.0</td>
<td></td>
</tr>
</tbody>
</table>

Ventilator status at baseline:
- Patient 1: 12 hours of BiPAP per day
- Patient 2: 17 hours of invasive ventilation per day
- Patient 3: 24 hours of invasive ventilation per day
- Patient 4: 12 hours of BiPAP per day

Interim data as of December 21, 2017

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SAFETY AND TOLERABILITY

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AT132 Safety and Tolerability Profile at $1 \times 10^{14}$ vg/kg

- No tolerability issues during study drug administration
- Two serious adverse events (SAEs), both in Patient 3
  - Hospitalization for pneumonia (week 2), not treatment-related
  - Hospitalization for GI infection and elevated troponin levels (week 7), is responding to IV steroids and supportive care; probably treatment-related
- Two possibly/probably treatment-related adverse events (AEs)
  - Patient 1
    • Mild, clinically asymptomatic exacerbation of preexisting hyperbilirubinemia, resolved; possibly treatment-related
  - Patient 2
    • Clinically asymptomatic liver enzyme elevation, controlled by extended steroid coverage; probably treatment-related
- Two additional non-treatment related AEs
NEUROMUSCULAR FUNCTION

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Significant Improvements in Neuromuscular Function as Assessed by the CHOP-INTEND Scale

Maximum score = 64 points (normally reached by 3-6 months of age)

Interim data as of December 21, 2017

<table>
<thead>
<tr>
<th>Pt #</th>
<th>Median score INCEPTUS</th>
<th>Baseline Score ASPIRO</th>
<th>Most recent score (Wk)</th>
<th>Change from baseline (%)</th>
</tr>
</thead>
<tbody>
<tr>
<td>1</td>
<td>29</td>
<td>29</td>
<td>56 (Wk 12)</td>
<td>27 (93%)</td>
</tr>
<tr>
<td>2</td>
<td>45</td>
<td>45</td>
<td>56 (Wk 8)</td>
<td>11 (24%)</td>
</tr>
<tr>
<td>3</td>
<td>28</td>
<td>34</td>
<td>36 (Wk 4)</td>
<td>2 (6%)</td>
</tr>
<tr>
<td>4 (Control)</td>
<td>49</td>
<td>49</td>
<td>46 (Wk 4)</td>
<td>-3 (-6%)</td>
</tr>
</tbody>
</table>
# Multiple Motor Milestones Achieved at 12 Weeks in First Treated Patient

**First-year developmental milestones in healthy children**

<table>
<thead>
<tr>
<th>Activity</th>
<th>Baseline</th>
<th>Week 12</th>
</tr>
</thead>
<tbody>
<tr>
<td>Rolling over</td>
<td>-</td>
<td>+</td>
</tr>
<tr>
<td>Head Control</td>
<td>-</td>
<td>+</td>
</tr>
<tr>
<td>Sitting unassisted &gt;5 sec</td>
<td>-</td>
<td>+</td>
</tr>
</tbody>
</table>

Interim data as of December 21, 2017
Significant Improvements in Respiratory Function as Assessed by Maximal Inspiratory Pressure (MIP)

Interim data as of December 21, 2017

Estimated normal minimal pressures in healthy children

<table>
<thead>
<tr>
<th>Pt #</th>
<th>Median Pressure in INCEPTUS</th>
<th>Baseline Pressure in ASPIRO</th>
<th>Most recent Pressure (Wk)</th>
<th>Change from baseline (%)</th>
</tr>
</thead>
<tbody>
<tr>
<td>1</td>
<td>29</td>
<td>33</td>
<td>80 (Wk 12)</td>
<td>47 (142%)</td>
</tr>
<tr>
<td>2</td>
<td>34</td>
<td>44</td>
<td>77 (Wk 4)</td>
<td>33 (76%)</td>
</tr>
<tr>
<td>3</td>
<td>24</td>
<td>26</td>
<td>44 (Wk 4)</td>
<td>18 (70%)</td>
</tr>
<tr>
<td>4 (Control)</td>
<td>65</td>
<td>58</td>
<td>--</td>
<td>--</td>
</tr>
</tbody>
</table>
QUALITATIVE ASSESSMENTS
Progressive Qualitative Improvements Observed in Disease Severity in All Treated Patients

- Increased trunk and limb strength and activity
  - Early indicator of gross muscle function improvement
  - Velocity and accuracy of movement has also improved

- Reductions in ventilator settings (pressure, rate and volume of mechanical ventilation) Patients 1 and 2
  - First step toward weaning off mechanical ventilation

- Improvements in airway clearance control (swallowing, coughing)
  - Critical for reducing aspiration risk

- Increased vocalization – improved ability to communicate with caregivers

- Initial exposure to oral feeding (Patient 1)

Interim data as of December 21, 2017
PATIENT 1
CHOP INTEND VIDEO ASSESSMENTS
BASELINE VS WEEK 12

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ASPIRO Key Findings and Program Next Steps

- Six adverse events to date (3 treatment-related); all resolved or responding to treatment
- Significant improvements in neuromuscular function as assessed by the CHOP-INTEND scale
- Multiple developmental milestones achieved at 12 weeks in Patient 1
- Significant improvements in respiratory function as assessed by Maximal Inspiratory Pressure (MIP)
- Progressive qualitative improvements observed in disease severity in all treated patients
- Interim efficacy and safety data review underway with independent Data Monitoring Committee prior to initiating enrollment of next cohort
- Next study update is scheduled for Q2:18
ASPIRO Study
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