Forward Looking Statements

This presentation has been prepared by us solely for information purposes. This presentation includes, and our responses to various questions may include, “forward-looking statements” within the meaning of Section 27A of the Securities Act of 1933, as amended, or the Securities Act, and Section 21E of the Securities Exchange Act of 1934, as amended, or the Exchange Act, which are subject to the “safe harbor” created by those sections. These forward-looking statements relate to, among other things, the expected timetable for development of our product candidates, our growth strategy and our future financial performance, including our operations, economic performance, financial condition, prospects and other future events. We have attempted to identify forward-looking statements by using such words as “anticipates,” “believes,” “can,” “continue,” “could,” “estimates,” “expects,” “forecasts,” “intends,” “may,” “plans,” “potential,” “should,” “suggest,” “will” or other similar expressions. The forward-looking statements are not historical facts, and are based upon the Company’s current expectations, beliefs, estimates, and projections, and various assumptions, many of which, by their nature, are inherently uncertain and beyond the Company’s control. The Company’s expectations, beliefs and projections are expressed in good faith and the Company believes there is a reasonable basis for them. However, there can be no assurance that management’s expectations, beliefs, estimates, and projections will result or be achieved, and actual results may vary materially from what is expressed in or indicated by the forward-looking statements. Forward-looking statements are subject to risks and uncertainties that could cause actual performance or results to differ materially from those expressed in the forward-looking statements. The Company assumes no obligation to update forward-looking statements to reflect actual results, subsequent events or circumstances or other changes affecting forward-looking information except to the extent required by applicable securities laws.

Information contained in this presentation has been compiled from sources believed to be credible and reliable. However, we cannot guarantee such credibility and reliability. The forecasts and projections of events contained herein are based upon subjective valuations, analyses and personal opinions.
Company Snapshot (NASDAQ: ATHX)

- Established international leader in the development of innovative cell therapy and regenerative medicines

- Multiple clinical programs – emphasis on critical care indications with substantial unmet need, high cost of care and quality-of-life burden & economic impact

- Lead program: MASTERS-2 registrational Phase 3 trial for Ischemic Stroke enrolling (with Fast Track and RMAT designations) and being conducted under Special Protocol Assessment (SPA) from FDA – accelerated approval pathway in U.S. and Europe

- Recent positive clinical data from ARDS program (leading to 3rd Fast Track designation)

- Partnered with HEALIOS K.K. in Japan, enrolling in TREASURE registrational trial for stroke and ONE-BRIDGE trial for ARDS, has received notification of Orphan designation from the PMDA – both leveraging accelerated regulatory path in Japan

- Robust clinical and preclinical pipeline

- Solid financial position ($40.4 million as of September 30, 2019)
## Summary Financial Data (Q3, 2019)

<table>
<thead>
<tr>
<th>Description</th>
<th>Amount</th>
</tr>
</thead>
<tbody>
<tr>
<td>Market Cap (as of September 30, 2019)</td>
<td>$207M</td>
</tr>
<tr>
<td>Shares Outstanding</td>
<td>155.3M</td>
</tr>
<tr>
<td>$ Thousands Nine Months ended September 30, 2019</td>
<td></td>
</tr>
<tr>
<td>Revenues</td>
<td>$5,346</td>
</tr>
<tr>
<td>Net loss</td>
<td>$(34,659)</td>
</tr>
<tr>
<td>Net cash used in operating activities</td>
<td>$(25,173)</td>
</tr>
<tr>
<td>Net cash provided by financing activities</td>
<td>$14,994</td>
</tr>
<tr>
<td>Cash and cash equivalents</td>
<td>$40,446</td>
</tr>
</tbody>
</table>
Our Focus: Development of best in class regenerative medicine therapies for areas of substantial unmet medical need

Neurological, Inflammatory & Immune, Cardiovascular, and Other Indications with an Emphasis in the Critical Care Segment
## ATHX Regenerative Medicine Pipeline

<table>
<thead>
<tr>
<th>Neurological</th>
<th>Preclinical</th>
<th>IND/CTA</th>
<th>Phase 1</th>
<th>Phase 2</th>
<th>Phase 3</th>
<th>NDA/BLA</th>
<th>Commercial</th>
</tr>
</thead>
<tbody>
<tr>
<td>Ischemic Stroke</td>
<td></td>
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<tr>
<td>Hemorrhagic Stroke</td>
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<tr>
<td>Traumatic Brain Injury</td>
<td></td>
<td></td>
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<td></td>
<td></td>
</tr>
<tr>
<td>Multiple Sclerosis</td>
<td></td>
<td></td>
<td></td>
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<td></td>
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<tr>
<td>Spinal Cord Injury</td>
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</tr>
</tbody>
</table>

- Ability to move directly to Phase 2

<table>
<thead>
<tr>
<th>Cardiovascular</th>
<th>Preclinical</th>
<th>IND/CTA</th>
<th>Phase 1</th>
<th>Phase 2</th>
<th>Phase 3</th>
<th>NDA/BLA</th>
<th>Commercial</th>
</tr>
</thead>
<tbody>
<tr>
<td>Acute Myocardial Infarction</td>
<td></td>
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<tr>
<td>PVD/PAD/CLI</td>
<td></td>
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<tr>
<td>Congestive Heart Failure</td>
<td></td>
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</tr>
</tbody>
</table>

- Ability to move directly to Phase 2

<table>
<thead>
<tr>
<th>Inflammatory, Immune &amp; Related</th>
<th>Preclinical</th>
<th>IND/CTA</th>
<th>Phase 1</th>
<th>Phase 2</th>
<th>Phase 3</th>
<th>NDA/BLA</th>
<th>Commercial</th>
</tr>
</thead>
<tbody>
<tr>
<td>HSC Transplant / GvHD</td>
<td></td>
<td></td>
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<td></td>
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<tr>
<td>Acute Respiratory Distress Syndrome</td>
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</tr>
</tbody>
</table>

- SPA agreement and Fast Track designation by FDA, Orphan drug designation by FDA and EMA
- MUST-ARDS (Athersys) - Positive Results Announced and Fast Track Designation by FDA
- ONE-BRIDGE (HEALIOS) - Orphan Designation - enrollment ongoing

<table>
<thead>
<tr>
<th>Trauma</th>
<th>Preclinical</th>
<th>IND/CTA</th>
<th>Phase 1</th>
<th>Phase 2</th>
<th>Phase 3</th>
<th>NDA/BLA</th>
<th>Commercial</th>
</tr>
</thead>
<tbody>
<tr>
<td>Solid Organ Transplant Support</td>
<td></td>
<td></td>
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</tr>
</tbody>
</table>

- Pending 150 patient 1/2 study with funding from the DOD (MTEC), and UTHealth
## Experienced Executive Leadership

<table>
<thead>
<tr>
<th>Name</th>
<th>Title</th>
<th>Prior Experience</th>
</tr>
</thead>
<tbody>
<tr>
<td>Gil Van Bokkelen, PhD</td>
<td>Chairman &amp; CEO</td>
<td></td>
</tr>
<tr>
<td>William BJ Lehmann, JD</td>
<td>President &amp; COO</td>
<td>McKinsey &amp; Company</td>
</tr>
<tr>
<td>John Harrington, PhD</td>
<td>CSO Exec. VP, Board member</td>
<td>Amgen, Scripps, Stanford University School of Medicine, University of California at San Diego</td>
</tr>
<tr>
<td>Ivor Macleod, MBA, CPA</td>
<td>Chief Financial Officer</td>
<td>Eisai, Merck, Roche</td>
</tr>
<tr>
<td>Manal Morsy, MD, PhD</td>
<td>Senior VP Global Regulatory Affairs</td>
<td>Merck, EVMS, Johnson &amp; Johnson</td>
</tr>
<tr>
<td>Laura Campbell, CPA</td>
<td>Senior VP Finance</td>
<td>EY, The Ohio State University</td>
</tr>
<tr>
<td>Greg Liposky, MBA</td>
<td>Senior VP Manufacturing</td>
<td>MedImmune, Mallinckrodt, Questcor Pharmaceuticals, Inc.</td>
</tr>
</tbody>
</table>
MultiStem Cell Therapy

Technology & Product Summary:

- **No Ethical Concerns**
  - MultiStem is derived from the bone marrow of healthy adult donors

- **Highly scalable**
  - Millions of doses may be produced from a single donor bank

- **Based on Proprietary MAPC Technology**
  - Broad IP estate covering core technology, methods of production & areas of use

- **Promotes Healing and Tissue Repair**
  - Works through multiple mechanisms of action

- **Given Systemically or Locally**
  - Off the shelf administration with no tissue matching or immune suppression required

- **Well Characterized Product with Long Shelf Life**
  - > 8 years of stability data on cryogenically stored product
Practical: Simple to Prepare & Easy to Administer

Hospital Pharmacy to Patient in < 1 hour
SIFU™ Technology

- Proprietary cryogenic storage system
- Simplifies product preparation to a single step after order entry
- Secure access – inventory is always protected
- Real time informatics on product inventory, storage, system access & usage
- Enables full “track and trace”... from end to end
- No special facilities or extensive training required
Scalable Manufacturing:
Key Competitive Advantage

Distinctive and Robust Expansion Profile + Integration with Advanced Bioreactor Technology
Enables Unprecedented Commercial Scale
Multimodal Biologic Product

INFLAMMATION REDUCTION

IMMUNOMODULATION
(reparative mechanisms)

NEUROPROTECTION

CYTOPROTECTION

ANGIO / VASCULGENESIS

CELLULAR REGENERATION
REPLACEMENT

MultiStem® expresses a combination of therapeutic proteins & factors to enhance healing and tissue repair in multiple ways.
**Opportunity for Cell Therapy in Ischemic Stroke**

- **Leading cause of disability** and third leading cause of mortality globally

- **Annually ~17 Million** globally, **~800,000** stroke victims in U.S., **~2.2 Million** across U.S. + EU + Japan combined

  Note: >3.4 Million strokes annually in China (including 2.35 Million first time ischemic strokes)

- **Tremendous unmet need**: tPA must be administered within 3 - 4½ hours of ischemic stroke & MR within 6 – 16 hours

- With an expanding aging population globally (and increasing obesity and diabetes in U.S.), the clinical need and commercial opportunity are expected to increase dramatically in years ahead
MultiStem Therapy Could Meaningfully Extend the Treatment Window for Stroke Patients

Time to Stroke Treatment

- **Tissue Plasminogen Activator (tPA)**: 3-4½ hours
  - Relevant to ≤ 10% Stroke patients
- **Mechanical Thrombectomy**: 6 hours
  - Relevant to ≤ 10% Stroke patients
  - Recent extension limited to specific types of patients
- **MultiStem® Therapy**: 36 hours
  - Relevant to potentially 90-95% of stroke patients
Spleen’s Role in Inflammatory Damage in the Brain following Stroke

In preclinical models of ischemic stroke, removing the spleen before a surgically induced stroke significantly reduces the inflammatory damage that typically occurs in the brain (but also creates permanent immunological impairment).

Note: MCAO = Middle Cerebral Artery Occlusion (i.e. ischemic stroke)

Deep Understanding of Therapeutic MOA’s of IV Administration of MultiStem for Stroke

MultiStem works through regulation of multiple factors and pathways important to brain recovery following a stroke.

1. Inflammation after stroke leads to greater tissue loss and scarring in the brain. Immune cells coming from the spleen play a major role this response.

2. MultiStem cells migrate to the spleen and peripheral immune system and affect key pathways in the brain.

3. Simultaneous downregulation of pro-inflammatory processes and upregulation of reparative immune responses promotes recovery.

Representative Publication in Stem Cells (2017):
MAPC’s Enhance Recovery After Stroke by Modulating the Immune Response from the Spleen
Intravenous multipotent adult progenitor cell therapy for traumatic brain injury: modulation of the resident microglia population

Intravenous Cellular Therapies for Acute Ischemic Stroke

Therapeutic time window of multipotent adult progenitor cell therapy after traumatic brain injury

Systemic multipotent adult progenitor cells improve long-term neurodevelopmental outcomes after preterm hypoxic-ischemic encephalopathy

Multipotent Adult Progenitor Cells Prevent Macrophage-Mediated Axonal Dieback and Promote Regrowth after Spinal Cord Injury
MultiStem Stroke Clinical Trial

Results from MASTERS-1 (Phase 2)

MultiStem Administration for Stroke Treatment and Enhanced Recovery Study
There are currently 2 well accepted regulatory endpoints for therapies being evaluated for efficacy in treating acute ischemic stroke:

- **Excellent Outcome**
  - Proportion of patients that achieve an excellent score in each of three established clinical rating scales: NIHSS, Barthel Index, Modified Rankin Scale
  - This essentially represents the proportion of patients that achieve full recovery over clinical assessment period

- **mRS Shift Analysis**
  - Reflects improvement across the entire disability spectrum during the clinical evaluation period
  - Note: PMDA expressed a slight preference for Excellent Outcome, whereas FDA and EMA expressed a slight preference for mRS shift analysis as primary assessments (each w/ the alternative endpoint as main secondary assessment)
Clinical Sites Participating in MASTERS-1 Trial

Double-blind, randomized, placebo-controlled Phase 2 study conducted at 33 leading international stroke centers across the U.S. and the U.K.
Trial Design Overview

- IV administration of MultiStem or placebo 24-36 hours post onset of ischemic stroke
  - Cortical cerebral ischemic strokes with substantial, persistent deficits
  - Randomized, double-blind, placebo-controlled
  - Dose escalation phase, followed by efficacy phase
  - Administered IV dose of 1.2 billion cells (efficacy phase)
  - Clinical assessments conducted at day 7, 30, 90 and one-year (with 90-day primary, and full blinding maintained through one-year final assessment)

- Safety evaluated
  - Adverse events, infusion reactions, infections, mortality

- Multiple clinical scales used to evaluate efficacy:
  - modified Rankin Scale (mRS) = Global disability
  - NIH Stroke Scale (NIHSS) = Neurological and motor skill deficits
  - Barthel Index (BI) = Ability to engage in activities of daily living (e.g., walking, dressing, feeding, toiletry, bathing)

- Exploratory endpoints evaluating MOA and clinical impact
  - Biomarkers (circulating immune cells and serum cytokine levels)
  - Hospitalization, time in ICU

Key Eligibility Criteria – Original Design

<table>
<thead>
<tr>
<th>Cortical Stroke</th>
</tr>
</thead>
<tbody>
<tr>
<td>NIHSS 8-20 at baseline (24 hours), stable deficit</td>
</tr>
<tr>
<td>Administration within 24-36 hours</td>
</tr>
<tr>
<td>tPA or device patients eligible if other criteria met</td>
</tr>
</tbody>
</table>

Key Changes to Accelerate Enrollment

<table>
<thead>
<tr>
<th>Cortical Stroke</th>
</tr>
</thead>
<tbody>
<tr>
<td>Administration window extended to 48 hours</td>
</tr>
<tr>
<td>Earlier treatment better. However, local cell processing limitations (e.g., HSC cell transplant units open limited hours Monday through Friday, need to work around their schedule) constrained enrollment rates</td>
</tr>
<tr>
<td>Included patients receiving both tPA-MR</td>
</tr>
<tr>
<td>Background rates/expectations for this group not well known</td>
</tr>
<tr>
<td>However, several of our sites treating patients receiving both tPA and mechanical reperfusion (MR), and such patients seemed to meet criteria</td>
</tr>
</tbody>
</table>
## Summary MASTERS-1 Baseline Demographics Information

<table>
<thead>
<tr>
<th>Patient sample</th>
<th>MultiStem n=65</th>
<th>Placebo n=61</th>
</tr>
</thead>
<tbody>
<tr>
<td>Age, mean, range</td>
<td>61.8</td>
<td>62.6</td>
</tr>
<tr>
<td></td>
<td>41-83</td>
<td>37-80</td>
</tr>
<tr>
<td>Sex, male</td>
<td>53.8%</td>
<td>54.1%</td>
</tr>
<tr>
<td>NIHSS, mean, median</td>
<td>13.4</td>
<td>13.3</td>
</tr>
<tr>
<td></td>
<td>13.0</td>
<td>13.0</td>
</tr>
<tr>
<td>MRI DWI Lesion size, mL, mean, median</td>
<td>51.6</td>
<td>54.8</td>
</tr>
<tr>
<td></td>
<td>42.3</td>
<td>41.1</td>
</tr>
<tr>
<td>Administered</td>
<td></td>
<td></td>
</tr>
<tr>
<td>iv tPA</td>
<td>44.6%</td>
<td>47.5%</td>
</tr>
<tr>
<td>iv tPA+device</td>
<td>12.3%</td>
<td>14.8%</td>
</tr>
</tbody>
</table>
**Final Trial Results: Treatment w/ MultiStem Shows Significant Benefit at One Year**

Proportion of Subjects Achieving **Excellent Outcome** Increases Over Time (Patients Achieving NIHSS 0 or 1 and mRS 0 or 1, and Barthel Index >95)

<table>
<thead>
<tr>
<th></th>
<th>Day 90</th>
<th>Δ at Day 90</th>
<th>Day 365</th>
<th>Δ at Day 365</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>ITT (All Trial Subjects):</strong></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>MultiStem (n=65)</td>
<td>15.4%</td>
<td>8.8%</td>
<td>23.1%</td>
<td>14.9%</td>
</tr>
<tr>
<td>Placebo (n=61)</td>
<td>6.6%</td>
<td></td>
<td>8.2%</td>
<td></td>
</tr>
<tr>
<td><strong>Early MultiStem Treatment (≤36 Hrs) vs All Placebo</strong></td>
<td></td>
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<td></td>
</tr>
<tr>
<td>MultiStem (n=31)</td>
<td>16.1%</td>
<td>9.5%</td>
<td>29.0%</td>
<td>20.8%</td>
</tr>
<tr>
<td>Placebo (n=61)</td>
<td>6.6%</td>
<td></td>
<td>8.2%</td>
<td></td>
</tr>
<tr>
<td><strong>Original Trial Protocol: Early MultiStem Treatment (≤36 hrs) vs Placebo</strong></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>MultiStem (n=27)</td>
<td>18.5%</td>
<td>14.7%</td>
<td>29.6%</td>
<td>23.8%</td>
</tr>
<tr>
<td>Placebo (n=52)</td>
<td>3.8%</td>
<td></td>
<td>5.8%</td>
<td></td>
</tr>
</tbody>
</table>

* As specified in original trial design, analysis includes patients that received either no reperfusion therapy, non-responder tPA or mechanical reperfusion (MR) patients in addition to investigational product (i.e., excludes a limited number of subjects receiving both tPA and MR)
MultiStem Improvement Evident Across the Severity Spectrum – Early-Treated MultiStem Subjects v. Placebo

mRS Shift Analysis

<table>
<thead>
<tr>
<th>Day</th>
<th>MultiStem</th>
<th>Placebo</th>
<th>p-value</th>
</tr>
</thead>
<tbody>
<tr>
<td>Day 7</td>
<td>9.7% 29.0% 38.7% 16.1% 3.2%</td>
<td>4.9% 26.2% 26.2% 42.6%</td>
<td>0.006</td>
</tr>
<tr>
<td>Day 30</td>
<td>12.9% 25.8% 29.0% 25.8% 3.2%</td>
<td>8.2% 16.4% 29.5% 24.6% 21.3%</td>
<td>0.015</td>
</tr>
<tr>
<td>Day 90</td>
<td>12.9% 29.0% 32.3% 19.4% 3.2%</td>
<td>11.5% 24.6% 32.8% 14.8% 16.4%</td>
<td>0.127</td>
</tr>
<tr>
<td>Day 365</td>
<td>9.7% 22.6% 16.1% 38.7% 9.7%</td>
<td>11.5% 29.5% 31.1% 8.2% 18.0%</td>
<td>0.066</td>
</tr>
</tbody>
</table>

Note: Early-treated means <36-hour administration, representing 31 MultiStem subjects
mRS Distribution (Shift) at One Year – ITT

More excellent outcomes
More independence
Fewer deaths, LTEAs

Excellent Outcome
Barthel Index >95

AMONG SUBJECTS WITH MRS = 2 OR 3

p=0.09
Improvement in Excellent Outcome Following Treatment with MultiStem Therapy

In contrast to longstanding clinical experience, MultiStem treated patients exhibit meaningful improvement beyond the initial 90-day recovery period.

Excellent Outcome (%)
All Treated Patients

Day 90 One year
MultiStem Placebo

Excellent Outcome (%)
Early-treated MultiStem vs All Placebo

Day 90 One year
MultiStem Placebo

Excellent Outcome = mRS ≤1, NIHSS ≤1, and BI ≥95
Note: Early-treated means <36-hour administration, representing 31 MultiStem subjects
Safety: Intravenous MultiStem well tolerated by stroke patients
- No infusional or allergic reactions, and no abnormal patterns in safety labs or vital signs
- Adverse events consistent with expectations and experience for stroke patients of this type

Administration of MultiStem within 36 hours associated with meaningfully better outcomes for patients, including:
- Substantially higher proportion of patients achieving excellent score in Barthel Index (activities of daily living), 67.7% (MultiStem treated) vs 44.3% (Placebo), p = 0.03
- Meaningful reductions in ICU time and initial hospitalization

Reduction in serious complications following stroke
- Reduction in life threatening AEs and death
- Reduced incidence of secondary infections

Benefits observed across treated population, e.g., age, stroke severity, reperfusion vs. no reperfusion therapy
Additional Clinical Observations from the MASTERS-1 Stroke Study

Among the most severely disabled stroke patients, a substantial reduction observed in serious and life-threatening adverse events

Severe Stroke (NIHSS 15+) Subjects

% Subjects with Grade 3-5 Adverse Events Through Day 30

<table>
<thead>
<tr>
<th>Events</th>
<th>MultiStem n=26</th>
<th>Placebo n=23</th>
</tr>
</thead>
<tbody>
<tr>
<td>Incr. WBCs</td>
<td>4%</td>
<td>43%</td>
</tr>
<tr>
<td>Acute Resp. Fail.</td>
<td></td>
<td></td>
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<tr>
<td>Sepsis</td>
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<tr>
<td>Acute Renal Fail.</td>
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<tr>
<td>Edema</td>
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<tr>
<td>Herniation</td>
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</tbody>
</table>

In the aftermath of a severe stroke, patients are highly susceptible to a range of severe and potentially life-threatening complications.
Clinical Biomarker Data: MultiStem has an Impact on Key Inflammatory Markers

Circulating CD3+ T-cell Levels, Safety / ITT Population

<table>
<thead>
<tr>
<th></th>
<th>MultiStem</th>
<th>Placebo</th>
</tr>
</thead>
<tbody>
<tr>
<td>Day 0</td>
<td>18.5%</td>
<td>18.8%</td>
</tr>
<tr>
<td>Day 2</td>
<td>18.4%</td>
<td>23.3%</td>
</tr>
<tr>
<td>Change</td>
<td>(0.1%)</td>
<td>4.5%</td>
</tr>
<tr>
<td></td>
<td>P=0.001</td>
<td></td>
</tr>
</tbody>
</table>

Note: Evaluates available data from ITT population; for cytokine analyses, controlling for differences in baseline values and outliers

Impact on key biomarkers (circulating immune cells and key inflammatory cytokines), provides direct support for therapeutic rationale and MOAs.
Development Focus in Japan

HEALIOS K.K. Partnership Overview

- Initial partnership with Healios established January 2016
  - $15 million up front payment with > $225 million total additional potential payments, subject to certain credits, plus tiered double-digit royalties

- Initial therapeutic focus: Ischemic stroke = most prevalent cardiovascular disease in Japan (leading cause of serious disability)
  - High proportional incidence
  - Rapidly expanding population of elderly (i.e., most susceptible)

- Well-positioned for accelerated approval under new regulatory framework
  - Rapid approval possible based on a single trial
  - March 2017 = Priority Review designation granted to Healios under Sakigake
  - Trial initiated and first patient enrolled in November 2017
  - High degree of clinical investigator enthusiasm

- Once approved, clear and efficient reimbursement pathway
  - Highly centralized with a defined, efficient process (single point of entry)
  - Reimbursement applies universally to all payer groups in Japan
  - Attractive price point possible (e.g., based on recent precedents for Regenerative Medicine products)
Recently Implemented Accelerated Approval System for Commercialization of Cell Therapy Products in Japan
Recent Alliance Expansion

Expanded Collaboration – Announced June 2018

- Healios obtains expanded exclusive license to certain programs in Japan and globally
  - Under terms of the collaboration expansion, Healios obtained development and commercialization rights to include treatment of ARDS and certain transplantation indications in Japan, plus defined Ophthalmological indications and rights to Organ Bud based treatments globally (for $20 million license fee)

- Economic impact for ATHX = $43.1 million (plus up to ~$360 million in additional potential milestone payments, subject to certain credits, plus tiered double-digit royalties)
  - Initial equity investment of $21.1 million completed in March 2018 (at premium) in exchange for an initial 8.7% equity stake
  - Plus $20 million in license fees - for license expansion
  - Healios also obtains ability to acquire up to 4 million shares in ATHX (via warrant)
    - Warrants priced at premium to recent market price (minimum of $1.76 per share)
MASTERS-2: Pivotal Phase 3 Study in Ischemic Stroke

(Authorized by FDA under SPA, w/ Fast Track and RMAT designations)

NASDAQ: ATHX

Trial Overview – (focused on North America and Europe – initiated and enrolling subjects)

- Intravenous administration of investigation product (MultiStem cell therapy or placebo) within 18 - 36 hours post onset of ischemic stroke...Note: may be administered on top of standard of care for eligible patients
  - 300 subjects
  - Double-blind, randomized, placebo-controlled study
  - 1:1 ratio (MultiStem [n=150] or placebo [n=150])
  - Same dosing profile for MASTERS-1 (1.2 B cells, administered IV)
  - NIHSS 8 – 20 at baseline
  - Cortical cerebral infarct
  - IV tPA, mechanical thrombectomy or both treatments (for limited number of subjects) allowed if patient not showing substantial improvement
  - 90-day primary clinical assessment, 12-month double-blind follow-up (e.g. secondary endpoints)

- Evaluating safety
  - Mortality, adverse events, infections, infusion reactions

- **Primary efficacy endpoint** = mRS score at Day 90 evaluated by shift analysis

- **Key secondary efficacy** variables include differences between MultiStem and placebo treatments with respect to the following:
  - Proportion of subjects achieving an Excellent Outcome (mRS ≤1, NIHSS ≤1 and Barthel Index ≥95) at day 365
  - Proportion of subjects achieving an Excellent Outcome (mRS ≤1, NIHSS ≤1 and Barthel Index ≥95) at day 90
  - Proportion of subjects with mRS score of ≤2 at Day 90

Note: Bolded indicate changes from Phase 2 B01-02 trial design.
Other Portfolio Programs
Acute Respiratory Distress Syndrome (ARDS) afflicts approximately 500,000 patients in Europe, the United States and Japan combined annually, and >670,000 patients in China.

ARDS represents a major area of unmet medical need, with high mortality and morbidity, and typically requires extended intensive care hospitalization (e.g., ICU).

- Very high cost of care and quality of life (QOL) impact
- Heterogenous causes that make it difficult to study and treat
- Extensive literature illustrates potential for marrow stromal cells to treat ARDS
- Represents a multibillion $ market opportunity
Acute Pulmonary Medicine

- MultiStem conveys benefit through multiple mechanisms relevant to acute pulmonary inflammatory damage
  - Published data illustrates impact on reducing inflammatory damage in pulmonary system
  - Upregulation of reparative cell types and pathways

- Athersys and collaborators evaluating potential of MultiStem in ARDS ➔
  Positive results from exploratory clinical trial announced January 2019
  - Fast Track designation from FDA announced May 14th, 2019
  - Presentation at American Thoracic Society meeting May 20th, 2019

- Healios obtained PMDA authorization of CTN and has commenced the ONE-BRIDGE trial in Japan
  - Presentation at American Thoracic Society meeting May 20th, 2019
  - Announced first patient was enrolled in April 2019
  - Received notification of Orphan designation from the PMDA
MultiStem Reduces Inflammation in Human Lungs with Ischemic Reperfusion Injury

Assessment of human lungs isolated from organ donors that exhibit significant inflammation prior to use in transplantation, perfused with MultiStem or vehicle (saline).

Note: Organ donor lungs originally designated for use in transplantation, but were disqualified due to pulmonary inflammation that occurs after harvest, resulting in poor pulmonary function.

Figure 3 Semi-quantitative scoring demonstrates significant decrease in overall inflammation in the MAPC-treated LLL compared to the vehicle-treated RLL in three out of four lungs and in aggregate. Means ± SD of pooled observations from three blinded observers are depicted.

Figure 4 Representative photomicrographs from lung 1 demonstrate (A) alveolar septal thickening, edema, and perivascular and peri-bronchial inflammatory cell infiltrates in the control-treated RLL vs (B) minimal to no significant inflammation in MAPC-treated LLL. Original Mag 200 x.
ARDS Trial – Initial Results  

Exploratory Ph. 2 MUST-ARDS Trial: Randomized, double-blind, placebo-controlled trial evaluating patients through 28-day clinical assessment (standard) with one year follow up.

<table>
<thead>
<tr>
<th>All Subjects</th>
<th>MultiStem</th>
<th>Placebo</th>
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</thead>
<tbody>
<tr>
<td>Number</td>
<td>20</td>
<td>10</td>
</tr>
<tr>
<td>Ventilator-free days (mean)</td>
<td>12.9</td>
<td>9.2</td>
</tr>
<tr>
<td>(median)</td>
<td>18.5</td>
<td>6.5</td>
</tr>
<tr>
<td>ICU-free days (mean)</td>
<td>10.3</td>
<td>8.1</td>
</tr>
<tr>
<td>(median)</td>
<td>12.5</td>
<td>4.5</td>
</tr>
<tr>
<td>Mortality (d28)</td>
<td>25%</td>
<td>40%</td>
</tr>
</tbody>
</table>

Patients w/ Low pulmonary function: PaO2/FiO2 < 150 mm at baseline

<table>
<thead>
<tr>
<th></th>
<th>MultiStem</th>
<th>Placebo</th>
</tr>
</thead>
<tbody>
<tr>
<td>Number</td>
<td>8</td>
<td>8</td>
</tr>
<tr>
<td>Ventilator-free days (mean)</td>
<td>14.6</td>
<td>8.0</td>
</tr>
<tr>
<td>(median)</td>
<td>18.5</td>
<td>3.5</td>
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<tr>
<td>ICU-free days (mean)</td>
<td>11.4</td>
<td>5.9</td>
</tr>
<tr>
<td>(median)</td>
<td>12.5</td>
<td>1</td>
</tr>
<tr>
<td>Mortality (d28)</td>
<td>25%</td>
<td>50%</td>
</tr>
</tbody>
</table>
The Healios ONE-BRIDGE study is evaluating pneumonia-induced ARDS

Post-hoc analysis of Pneumonia-Induced ARDS
(Severe cases – PaO₂/FiO₂ Ratios at Day 0, Pre-infusion < 150)

<table>
<thead>
<tr>
<th></th>
<th>MultiStem</th>
<th>Placebo</th>
</tr>
</thead>
<tbody>
<tr>
<td>Day-28 Mortality</td>
<td>20%</td>
<td>50%</td>
</tr>
<tr>
<td>Ventilator-free days</td>
<td>14.8</td>
<td>7.5</td>
</tr>
<tr>
<td>ICU-free days</td>
<td>12.0</td>
<td>5.0</td>
</tr>
</tbody>
</table>

Data for severe cases of pneumonia-induced ARDS shows an even greater difference in mortality rate, Vent-free and ICU-free days between the subjects treated with MultiStem and the patients in the placebo-controlled group.
Other Neurological Injury & Disease Areas of Interest

**IV Administration of MultiStem to Promote & Accelerate Healing & Repair**

Work conducted in preclinical models – with multiple publications in leading scientific journals

- **Ischemic Stroke**
  - Supported by StrokeMAP
- **Traumatic Brain Injury (TBI)**
  - Supported by NIH
- **Neonatal Hypoxic Ischemia**
  - Supported by NIH
- **Spinal Cord Injury**
  - Supported by Third Frontier

**Acute Neurological Injury**

- **Multiple Sclerosis**
  - Supported by Fast Forward, MS Society
- **Parkinson’s Disease**
  - Supported by Michael J. Fox Foundation

**Chronic CNS Disease**

Also: Orphan status granted by FDA for MPS-1 (Hurler’s Syndrome)
Opportunity in Trauma

- Trauma is the leading cause of death and serious disability among individuals age <45 in the U.S.
  - Leading cause of life years lost among individuals up to age 75... and third leading cause of death overall
  - Significant impact on youth, elderly and military personnel (battlefield trauma & VA patients)
  - Huge economic and quality of life impact

- ATHX team and independent collaborators have worked extensively in several areas of trauma (numerous publications)
  - Traumatic Brain Injury (TBI)
  - Spinal Cord injury
  - ARDS (e.g. precipitated by trauma)

- Mechanistically the hyperinflammatory response following trauma is the same as for stroke, w/ similar effects
  - Emanates from the spleen & peripheral immune system, causing secondary damage
  - Response frequently results in immunodepression – with patients susceptible to a range of complications that inhibit or complicate recovery (e.g., secondary infections)

- ATHX collaborating with leading Tier 1 Trauma Center in U.S. with funding provided by MTEC (Department of Defense) and UTH for planned Phase 2 trial (~150 patients – double-blind randomized, placebo-controlled)
Trauma Phase 2 Trial Objectives
(MATRICS-1) Multistem Administration for Trauma Related Inflammation and Complications

- Randomized, double-blind, placebo-controlled study (~150 patients) evaluating safety and efficacy

- Compare the incidence, severity and duration of AKI in multiply injured, post-hemorrhage patients administered MultiStem vs. patients administered placebo

- Compare the incidence, severity and duration of inflammatory complications (e.g., SIRS) in multiply injured, post-hemorrhage patients administered MultiStem vs. patients administered placebo

- Compare all-cause mortality at 30 days in multiply injured, post-hemorrhage patients administered MultiStem vs. patients administered placebo

- Determine the inflammatory profiles associated with incidence of AKI, other inflammatory complications and mortality
Multiple important objectives achieved recently

- Healios advancement of TREASURE trial (w/ Sakigake designation)
- Fast Track and RMAT designations granted by FDA for our Phase 3 Stroke program... Positive Scientific Advice from by EMA
- Launch of (and continued enrollment in) MASTERS-2 trial
- Expansion of contract manufacturing capabilities to include CMO sites in Europe and Japan
- 2018 Expansion of collaboration with Healios
- Maintained healthy balance sheet
- Completion of enrollment in MUST-ARDS trial – and successful results
- Fast Track designation for ARDS program
- Healios’ initiation of ONE-BRIDGE study for ARDS and Orphan designation from PMDA

Primary priorities for 2020

- Complete enrollment for TREASURE and ONE-BRIDGE studies
- Advancement of MASTERS-2 and other clinical programs
- Complete evaluation / implementation of key partnering initiatives
- Continued advancement of key process development and manufacturing initiatives