ATHOS-3 Phase 3 Study of LJPC-501

Positive Topline Results

February 27, 2017
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La Jolla is dedicated to improving the lives of patients suffering from life-threatening diseases by discovering and developing innovative therapies.
Catecholamine Resistant Hypotension (CRH) Remains a Major Unmet Medical Need
Shock: Deadly, Costly and Prevalent

- A well-characterized syndrome\textsuperscript{1}
  - Occurs when the organs and tissue of the body do not receive an adequate flow of blood (oxygen) due to a lack of blood pressure (hypotension)

- Deadly
  - Mortality rate exceeds that of most acute conditions requiring hospitalization\textsuperscript{2}
  - Can kill old and young alike within hours\textsuperscript{2}

- Costly
  - Estimated costs are 2-3 times greater compared to other conditions

- Prevalent
  - Affects one-third of patients in the intensive care unit\textsuperscript{1}

MORTALITY RATES COMPARED

\begin{itemize}
\item Shock: \textgreater 50\% mortality in patients with shock in the ICU\textsuperscript{2}
\item AMI: 14\%
\item CHF: 12\%
\item Pneumonia: 16\%
\end{itemize}

Abbreviations: AMI=acute myocardial infarction; CHF=congestive heart failure.

CMS Covered Charges for CRH Population Are Much Greater Than for Other Acute Hospital Conditions

Source: CMS FY14 Inpatient Public Use File (IPUF)

Abbreviations: AMI=acute myocardial infarction; CHF=congestive heart failure.
U.S. Shock Patient Population and Treatment Paradigm

First-Line Standard-of-Care

Norepinephrine:
703,000 Patients per Year\(^1\)
$153 per Patient\(^2\)
$108MM Sales Run Rate

Second-Line Standard-of-Care

Vasopressin:
244,000 Patients per Year\(^3\)
$1,385 per Patient\(^2\)
$338MM Sales Run Rate

LJPC-501 Target Patient Population

Patients Who Do Not Adequately Respond to Norepinephrine and Vasopressin

196,000 Estimated Patients\(^4\)

2. Wolters Kluwer PriceRx Pro, 2017
3. 3.01MM annualized vials (251K vials sold in January 2017 X 12); Symphony Health Solutions, 2017. 81% of vials sold for hypotensive shock; estimate based on medical literature. 10 vials used per patient; estimate based on Dunser et al, *Circulation*, 107:2313-2319, 2003 and Gordon et al, *Crit Care Med*, 42(6):1325-1333, 2014
4. Decision Resources Group market research
Randomized Study of Vasopressin

VASST Overall Survival\(^1\)

Day 28 HR=0.90 (95% CI: 0.75-1.08)  P=0.27
Day 90 HR=0.88 (95% CI: 0.76-1.03)  P=0.10

No. at Risk
Vasopressin  397  301  272  249  240  234  232  230  226  220
Norepinephrine  382  289  247  230  212  205  200  194  193  191

VASST=Vasopressin and Septic Shock Trial

LJPC-501: The First Synthetic Human Angiotensin II Tested in a Randomized Phase 3 Study
LJPC-501 is a proprietary formulation of synthetic human angiotensin II, a naturally occurring regulator of blood pressure.

Angiotensin II has been shown to raise blood pressure in a pilot, randomized, placebo-controlled, pilot study in CRH\(^1\), as well as in animal models of hypotension.

Special Protocol Assessment (SPA) agreement reached with FDA for Phase 3 study design.
- Agreement reached that blood pressure can be the primary endpoint for approval.

ATHOS-3 enrollment completed in Q4 2016.

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Three Systems Work in Harmony to Regulate Blood Pressure

Existing Treatments for Shock Only Utilize Two Systems

THERAPIES AND ASSOCIATED ADVERSE EVENTS

CATECHOLAMINES¹: SYMPATHETIC NERVOUS
Prolonged elevated heart rate, tachyarrhythmia, acute cardiac arrest or death, pulmonary hypertension

VASOPRESSIN: ARGinine-VASOPRESSIN
Myocardial ischemia, decreases gut blood flow

RENIN ANGIOTENSIN-ALDOSTERONE
No current therapies

1. Catecholamines include: norepinephrine, epinephrine, dopamine, phenylephrine, ephedrine
ATHOS-3 (Angiotensin II for the Treatment of High-Output Shock) Study Design

Patient population:
- Adult patients with CRH
- N=344 enrolled
- N=321 treated

1:1 double-blind randomization

Placebo
N=158

LJPC-501
N=163

Primary endpoint
Percentage of patients achieving pre-specified target blood pressure response

Study Conducted In 74 Centers Across 9 Countries

1. Mean Arterial Pressure (MAP) ≥ 75 mmHg OR a 10 mmHg increase from baseline MAP at 3 hours following the initiation of study treatment without an increase in standard-of-care vasopressors
ATHOS-3 Topline Results: Primary Endpoint

- Analysis of primary efficacy endpoint was highly statistically significant

<table>
<thead>
<tr>
<th>Primary Efficacy Endpoint</th>
<th>Placebo</th>
<th>LJPC-501</th>
<th>P-Value</th>
</tr>
</thead>
<tbody>
<tr>
<td>Percentage of patients achieving pre-specified target blood pressure response¹</td>
<td>23%</td>
<td>70%</td>
<td>&lt;0.00001</td>
</tr>
</tbody>
</table>

1. Mean Arterial Pressure (MAP) ≥ 75 mmHg OR a 10 mmHg increase from baseline MAP at 3 hours following the initiation of study treatment without an increase in standard-of-care vasopressors
ATHOS-3 Topline Results: Mortality

- Trend toward longer survival observed

<table>
<thead>
<tr>
<th>Estimated Risk Reduction</th>
<th>Hazard Ratio(^1)</th>
<th>95% Confidence Interval</th>
<th>P-Value</th>
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<tbody>
<tr>
<td>22%</td>
<td>0.78</td>
<td>0.57-1.07</td>
<td>0.12</td>
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1. Proportional hazards estimate (unadjusted) of mortality to Day 28 comparing placebo-treated patients to LJPC-501-treated patients
ATHOS-3 Topline Results: Safety

- Throughout the study, safety outcomes were followed by an independent Data Safety Monitoring Board (DSMB)
  - The DSMB recommended that the study continue as originally planned

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<th>Placebo</th>
<th>LJPC-501</th>
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<td>Percentage of patients experiencing at least one adverse event</td>
<td>92%</td>
<td>87%</td>
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<tr>
<td>Percentage of patients discontinuing treatment due to an adverse event</td>
<td>22%</td>
<td>14%</td>
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We plan to present and publish detailed results from ATHOS-3 later this year

Thank You