Corporate Presentation

Developing Innovative Therapies for Patients Suffering from Life-threatening Diseases

NASDAQ: LJPC

September 2017
Forward-Looking Statements

These slides contain forward-looking statements as that term is defined in the Private Securities Litigation Reform Act of 1995. These statements relate to future events or the company’s future results of operations. These statements are only predictions or statements of current expectations and involve known and unknown risks, uncertainties and other factors, that may cause actual results to be materially different from those anticipated by the forward-looking statements. The company cautions readers not to place undue reliance on any such forward-looking statements, which speak only as of the date they were made. Certain of these risks, uncertainties, and other factors are described in greater detail in the company’s filings with the U.S. Securities and Exchange Commission (SEC), all of which are available free of charge on the SEC’s web site www.sec.gov. These risks include, but are not limited to, risks relating to: the timing and the prospects for approval of LJPC-501 by the FDA, the EMA or other regulatory authorities; risks relating to the scope of product label(s) (if approved) and other matters that could affect the availability or commercial potential of LJPC-501; the potential market sizes, as well as the broader commercial opportunity of the product candidate; the anticipated timing for regulatory actions; the impact of pharmaceutical industry regulation and health care legislation in the United States; the success of future development activities; potential indications for which the company’s product candidates may be developed; the timing, costs, conduct, and outcome of our clinical trials and future preclinical studies and clinical trials, including the timing of the initiation and availability of data from such trials; and the expected duration over which the company’s cash balances will fund its operations. Subsequent written and oral forward-looking statements attributable to the company or to persons acting on its behalf are expressly qualified in their entirety by the cautionary statements set forth in the company’s reports filed with the SEC. The company expressly disclaims any intent to update any forward-looking statements.
Overview of LJPC

LJPC-501 (Synthetic Human Angiotensin II) for the treatment of hypotension in adults with distributive or vasodilatory shock who remain hypotensive despite fluid and vasopressor therapy

LJPC-401 (Synthetic Human Hepcidin) for Iron Overload

Financial Position
La Jolla is dedicated to improving the lives of patients suffering from life-threatening diseases by discovering and developing innovative therapies.
LJPC Corporate Highlights

- Focused on de-risked product opportunities
  - Naturally occurring peptides with well-understood biological functions
  - Derivative components of FDA-approved products
- **LJPC-501** (synthetic human angiotensin II) for the treatment of hypotension in adults with distributive or vasodilatory shock who remain hypotensive despite fluid and vasopressor therapy
  - Phase 3 registration study conducted under SPA, positive topline results reported February 2017
  - Detailed study results published in May 2017 by The New England Journal of Medicine (NEJM)
  - New Drug Application accepted and Priority Review granted in August 2017
- **LJPC-401** (synthetic human hepcidin) for iron overload caused by diseases such as hereditary hemochromatosis (HH), thalassemia, sickle cell disease (SCD) and myelodysplasia (MDS)
  - Positive Phase 1 study results reported in September 2016
  - Agreement reached with EMA on pivotal study design
- **LJPC-30S** (gentamicin derivative) for bacterial infections and rare genetic diseases
<table>
<thead>
<tr>
<th>Product Pipeline</th>
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</thead>
<tbody>
<tr>
<td><strong>LJPC-501</strong></td>
</tr>
<tr>
<td>Synthetic Human Angiotensin II</td>
</tr>
<tr>
<td><strong>LJPC-401</strong></td>
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<tr>
<td>Synthetic Human Hepcidin</td>
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<tr>
<td><strong>LJPC-30S</strong></td>
</tr>
<tr>
<td>Gentamicin Derivative</td>
</tr>
<tr>
<td><strong>Other R&amp;D</strong></td>
</tr>
<tr>
<td>Various</td>
</tr>
</tbody>
</table>

**Indication**
- LJPC-501: Hypotension in adults w/ distributive or vasodilatory shock who remain hypotensive despite fluid and vasopressor therapy
- LJPC-401: Iron overload due to HH, Thalassemia, SCD and MDS
- LJPC-30S: Bacterial Infections/Rare Genetic Diseases
- Other R&D: Various

**IND**
- LJPC-501: Successful Pre-IND Meeting
- LJPC-401: Q4 2015
- Other R&D: Various

**Phase 1**
- LJPC-401: Q4 2015
- LJPC-30S: Successful Pre-IND Meeting
- Other R&D: Various

**Phase 2**
- LJPC-401: H2 2017
- LJPC-30S: Successful Pre-IND Meeting
- Other R&D: Various

**Phase 3**
- LJPC-501: Pivotal Study
- LJPC-401: Pivotal Study
- LJPC-30S: Successful Pre-IND Meeting
- Other R&D: Various

**NDA**
- LJPC-501: Priority Review Granted
- LJPC-401: Priority Review Granted
- LJPC-30S: Successful Pre-IND Meeting
- Other R&D: Various

**Legend**
- Completed/milestone achieved
- Underway
- Planned
Agenda

• Overview of LJPC

• LJPC-501 (Synthetic Human Angiotensin II) for the treatment of hypotension in adults with distributive or vasodilatory shock who remain hypotensive despite fluid and vasopressor therapy

• LJPC-401 (Synthetic Human Hepcidin) for Iron Overload

• Financial Position
LJPC-501: Overview

- LJPC-501 is a proprietary formulation of synthetic human angiotensin II, a naturally occurring regulator of blood pressure being developed for the treatment of hypotension in adults with distributive or vasodilatory shock who remain hypotensive despite fluid and vasopressor therapy.
- Shock is deadly, costly and prevalent.
- Phase 3 conducted under Special Protocol Assessment (SPA).
- New Drug Application (NDA) filed and Priority Review granted.
  - The Prescription Drug User Fee Act (PDUFA) is February 28, 2018.
  - FDA stated that it does not currently plan to hold an advisory committee meeting to discuss this application.
- Issued patent claims covering potential product to 2034.
Shock: Deadly, Costly and Prevalent

- A well-characterized syndrome
  - 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
  - Can kill old and young alike within hours
- Costly
  - Estimated costs are 2-3 times greater compared to other conditions
- Prevalent
  - Affects one-third of patients in the intensive care unit

### MORTALITY RATES COMPARED

<table>
<thead>
<tr>
<th>Condition</th>
<th>30-day mortality rate</th>
</tr>
</thead>
<tbody>
<tr>
<td>Shock</td>
<td>≥50%</td>
</tr>
<tr>
<td>AMI</td>
<td>14%</td>
</tr>
<tr>
<td>CHF</td>
<td>12%</td>
</tr>
<tr>
<td>Pneumonia</td>
<td>16%</td>
</tr>
</tbody>
</table>

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

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

Weighted Average CMS Covered Charges

CRH: $87,282
AMI: $42,243
CHF: $31,453
Pneumonia: $30,702

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

Source: CMS FY14 Inpatient Public Use File (IPUF)
U.S. Shock Patient Population and Treatment Paradigm

First-Line Standard-of-Care

Norepinephrine:
703,000 Patients per Year
$153 per Patient
$108MM Sales Run Rate

Second-Line Standard-of-Care

Vasopressin:
244,000 Patients per Year
$1,385 per Patient
$338MM Sales Run Rate

LJPC-501 Target Patient Population

Patients Who Do Not Adequately Respond to Norepinephrine and Vasopressin

196,000 Estimated Patients

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**

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**

<table>
<thead>
<tr>
<th>Vasopressin</th>
<th>397</th>
<th>301</th>
<th>272</th>
<th>249</th>
<th>240</th>
<th>234</th>
<th>232</th>
<th>230</th>
<th>226</th>
<th>220</th>
</tr>
</thead>
<tbody>
<tr>
<td>Norepinephrine</td>
<td>382</td>
<td>289</td>
<td>247</td>
<td>230</td>
<td>212</td>
<td>205</td>
<td>200</td>
<td>194</td>
<td>193</td>
<td>191</td>
</tr>
</tbody>
</table>

VASST=Vasopressin and Septic Shock Trial

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 randomized, placebo-controlled, pilot study\(^1\), 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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1. Chawla et al. Critical Care 2014, 18:534
Three Systems Work in Harmony to Regulate Blood Pressure

Existing Treatments for Shock Only Utilize Two Systems

THERAPIES AND ASSOCIATED ADVERSE EVENTS

**CATECHOLAMINES**: 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

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1. Catecholamines include: norepinephrine, epinephrine, dopamine, phenylephrine, ephedrine
Primary endpoint: Percentage of patients achieving pre-specified target blood pressure response.

Specifically, percentage of patients achieving a 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. The primary endpoint was agreed upon with FDA and is reflected in our Special Protocol Assessment (SPA) agreement dated February 2, 2015 (before study initiation) and has never been changed. ATHOS-3 was conducted without any amendment to any part of the clinical protocol, including the primary and all other endpoints.
ATHOS-3 was conducted as a phase 3 registration study under a special protocol assessment agreement with the US Food and Drug Administration.

This agreement conveys concurrence with the acceptability and adequacy of critical elements of protocol design (e.g., primary endpoint) and analysis (e.g., statistical plan).

AE, adverse event; CV SOFA, cardiovascular Sequential Organ Failure Assessment score; SOC, standard of care.

ATHOS-3 Study: Endpoints

- **Primary endpoint:** MAP response at hour 3
  - Defined as MAP ≥ 75 mmHg, or increased from baseline by ≥ 10 mmHg, without increase of background vasopressor dose
  - MAP response established as the mean of 3 determinations, taken 15 minutes apart in triplicate, at baseline and at hour 3

- **Secondary endpoints:** changes between baseline and hour 48 in
  - CV-SOFA score
  - Total SOFA score

- **Safety assessments:**
  - Serious adverse events
  - Adverse event-related drug discontinuations
  - All adverse events
  - All-cause mortality at 7 and 28 days

CV-SOFA, cardiovascular Sequential Organ Failure Assessment; MAP, mean arterial pressure.

ATHOS-3 Study: Primary Endpoint

MAP response defined as an increase from baseline of ≥ 10 mm Hg or an increase to ≥ 75 mm Hg, without an increase in the dose of background vasopressors.

MAP, mean arterial pressure; NE, norepinephrine.

## ATHOS-3 Study: Other Endpoints

<table>
<thead>
<tr>
<th></th>
<th>Angiotensin II N = 163</th>
<th>Placebo N = 158</th>
<th>Statistics</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Secondary Efficacy Endpoints</strong></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Mean (SD) change in cardiovascular SOFA score at hour 48</td>
<td>-1.75 (1.77)</td>
<td>-1.28 (1.65)</td>
<td>P = 0.01</td>
</tr>
<tr>
<td>Mean (SD) change in total SOFA score at hour 48</td>
<td>1.05 (5.50)</td>
<td>1.04 (5.34)</td>
<td>P = 0.49</td>
</tr>
<tr>
<td><strong>Additional Endpoints</strong></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Mean (SD) change in norepinephrine equivalent dose, baseline to hour 3</td>
<td>-0.03 (0.10)</td>
<td>0.03 (0.23)</td>
<td>P &lt; 0.001</td>
</tr>
<tr>
<td>All-cause mortality at day 7, n (%)</td>
<td>47 (29)</td>
<td>55 (35)</td>
<td>Hazard ratio, 0.78 (95% CI, 0.53–1.16) P = 0.22</td>
</tr>
<tr>
<td>All-cause mortality at day 28, n (%)</td>
<td>75 (46)</td>
<td>85 (54)</td>
<td>Hazard ratio, 0.78 (95% CI, 0.57–1.07) P = 0.12</td>
</tr>
</tbody>
</table>


mITT population
ATHOS-3 Study: Survival After Initiation of Therapy

ATHOS-3 Study: Survival After Initiation of Therapy Adjusted for Age (Continuous) and Gender

<table>
<thead>
<tr>
<th>Parameter</th>
<th>Hazard Ratio (95% CI)</th>
<th>P Value</th>
<th>Overall P Value</th>
</tr>
</thead>
<tbody>
<tr>
<td>Treatment</td>
<td>0.784 (0.574, 1.070)</td>
<td>0.13</td>
<td>0.046</td>
</tr>
<tr>
<td>Age</td>
<td>1.013 (1.002, 1.024)</td>
<td>0.022</td>
<td></td>
</tr>
<tr>
<td>Gender</td>
<td>0.931 (0.677, 1.280)</td>
<td>0.66</td>
<td></td>
</tr>
</tbody>
</table>

# ATHOS-3 Study: Safety Summary

## Patients With Event, n (%)

<table>
<thead>
<tr>
<th>Adverse events (any grade)</th>
<th>Angiotensin II N=163</th>
<th>Placebo N=158</th>
</tr>
</thead>
<tbody>
<tr>
<td>Adverse events leading to discontinuation of study drug</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Events leading to discontinuation in ≥ 2 patients in either arm</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Septic shock</td>
<td>8 (4.9)</td>
<td>4 (2.5)</td>
</tr>
<tr>
<td>Multi-organ failure</td>
<td>6 (3.7)</td>
<td>6 (3.8)</td>
</tr>
<tr>
<td>Cardiogenic shock</td>
<td>2 (1.2)</td>
<td>4 (2.5)</td>
</tr>
<tr>
<td>Cardiac arrest</td>
<td>0</td>
<td>5 (3.2)</td>
</tr>
<tr>
<td>Serious adverse events</td>
<td>99 (60.7)</td>
<td>106 (67.1)</td>
</tr>
<tr>
<td>Serious AE occurring in ≥ 3% of patients in either arm</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Multiorgan failure</td>
<td>25 (15.3)</td>
<td>23 (14.6)</td>
</tr>
<tr>
<td>Septic shock</td>
<td>18 (11.0)</td>
<td>10 (6.3)</td>
</tr>
<tr>
<td>Respiratory failure</td>
<td>8 (4.9)</td>
<td>11 (7.0)</td>
</tr>
<tr>
<td>Cardiac arrest</td>
<td>7 (4.3)</td>
<td>9 (5.7)</td>
</tr>
<tr>
<td>Atrial fibrillation</td>
<td>5 (3.1)</td>
<td>5 (3.2)</td>
</tr>
<tr>
<td>Ventricular tachycardia</td>
<td>5 (3.1)</td>
<td>3 (1.9)</td>
</tr>
<tr>
<td>Cardiorespiratory arrest</td>
<td>3 (1.8)</td>
<td>5 (3.2)</td>
</tr>
<tr>
<td>Acute respiratory failure</td>
<td>3 (1.8)</td>
<td>5 (3.2)</td>
</tr>
<tr>
<td>Hypotension</td>
<td>5 (3.1)</td>
<td>3 (1.9)</td>
</tr>
<tr>
<td>Peripheral ischemia</td>
<td>5 (3.1)</td>
<td>3 (1.9)</td>
</tr>
</tbody>
</table>

# ATHOS-3 Study: Adverse Events (Any Grade) of Special Interest

<table>
<thead>
<tr>
<th>Patients With Event, n (%)</th>
<th>Angiotensin II N=163</th>
<th>Placebo N=158</th>
</tr>
</thead>
<tbody>
<tr>
<td>Overall</td>
<td>51 (31.3)</td>
<td>55 (34.8)</td>
</tr>
<tr>
<td>Events occurring in ≥ 2 patients in either arm</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Cardiac disorders</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Acute myocardial infarction</td>
<td>2 (1.2)</td>
<td>3 (1.9)</td>
</tr>
<tr>
<td>Arrhythmia</td>
<td>3 (1.8)</td>
<td>0</td>
</tr>
<tr>
<td>Atrial fibrillation</td>
<td>22 (13.5)</td>
<td>21 (13.3)</td>
</tr>
<tr>
<td>Atrial flutter</td>
<td>2 (1.2)</td>
<td>5 (3.2)</td>
</tr>
<tr>
<td>Bundle branch block right</td>
<td>2 (1.2)</td>
<td>0</td>
</tr>
<tr>
<td>Sinus tachycardia</td>
<td>3 (1.8)</td>
<td>1 (0.6)</td>
</tr>
<tr>
<td>Supraventricular tachycardia</td>
<td>3 (1.8)</td>
<td>4 (2.5)</td>
</tr>
<tr>
<td>Tachycardia</td>
<td>8 (4.9)</td>
<td>4 (2.5)</td>
</tr>
<tr>
<td>Ventricular extrasystoles</td>
<td>1 (0.6)</td>
<td>3 (1.9)</td>
</tr>
<tr>
<td>Ventricular fibrillation</td>
<td>2 (1.2)</td>
<td>0</td>
</tr>
<tr>
<td>Ventricular tachycardia</td>
<td>5 (3.1)</td>
<td>8 (5.1)</td>
</tr>
<tr>
<td>Vascular disorders</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Peripheral ischemia</td>
<td>7 (4.3)</td>
<td>4 (2.5)</td>
</tr>
<tr>
<td>Poor peripheral circulation</td>
<td>2 (1.2)</td>
<td>0</td>
</tr>
<tr>
<td>Gastrointestinal disorders</td>
<td>Intestinal ischemia</td>
<td>1 (0.6)</td>
</tr>
<tr>
<td>Hepatobiliary disorders</td>
<td>Ischemic hepatitis</td>
<td>2 (1.2)</td>
</tr>
<tr>
<td>Investigations</td>
<td>Electrocardiogram QT prolonged</td>
<td>1 (0.6)</td>
</tr>
</tbody>
</table>

Adverse events were coded according to the Medical Dictionary for Regulatory Activities (MedDRA). [http://www.meddra.org](http://www.meddra.org).

No differences between treatment groups were statistically significant.

ATHOS-3 Study: Summary

- A MAP response at hour 3 (primary endpoint) was achieved by significantly more patients receiving angiotensin II vs placebo.

- The mean reduction of norepinephrine-equivalent vasopressor dose was significantly greater in the angiotensin II group at 3 hours:
  - Mean doses of background vasopressors in the angiotensin II group were consistently less than those in the placebo group.

- Angiotensin II, compared with placebo, was associated with significantly greater improvement in cardiovascular SOFA scores at 48 hours:
  - Changes in total SOFA score were comparable.

- Rates of adverse events were comparable in the angiotensin II and placebo groups.

LJPC-501: Path Forward

- Phase 3 conducted under Special Protocol Assessment (SPA)
  - Agreement reached that blood pressure can be the primary endpoint for approval
- Positive top-line results announced in February 2017; detailed study results published in May 2017 by The New England Journal of Medicine (NEJM)
- New Drug Application (NDA) filed and Priority Review granted
- The Prescription Drug User Fee Act (PDUFA) is February 28, 2018
- FDA stated that it does not currently plan to hold an advisory committee meeting to discuss this application
- Issued patent claims covering potential product to 2034
Agenda

- Overview of LJPC
- LJPC-501 (Synthetic Human Angiotensin II) for the treatment of hypotension in adults with distributive or vasodilatory shock who remain hypotensive despite fluid and vasopressor therapy
- LJPC-401 (Synthetic Human Hepcidin) for Iron Overload
- Financial Position
LJPC-401: Overview

- LJPC-401 is a proprietary formulation of synthetic human hepcidin, a naturally occurring regulator of iron absorption and distribution.

- Primary iron overload
  - Hereditary hemochromatosis (HH) is characterized by a genetic deficiency of hepcidin resulting in excessive iron accumulation
    - Most common genetic disease in Caucasians
    - Causes liver cirrhosis, liver cancer, heart disease and/or failure, and diabetes

- Secondary iron overload
  - Patients with thalassemia (including beta thalassemia), sickle cell disease (SCD) and myelodysplasia (MDS) have physiologically low hepcidin levels and are treated with blood transfusions, resulting in acquired iron overload.

- LJPC-401 has been shown to be effective at reducing serum iron levels in preclinical and Phase 1 human testing.

- Scalable manufacturing process capable of producing a pure, properly folded, stable hepcidin formulation developed.

- Orphan Drug Designation granted (EU).

- Agreement reached with European Medicines Agency (EMA) on pivotal study design.
Hepcidin: The Insulin of Iron Metabolism

- **Hepcidin: the insulin of iron metabolism**
  - Regulates iron absorption and disposition in all organs
  - Rapid and sustained lowering of iron levels

- **Progress**
  - SC formulation developed
  - Orphan Drug Designation granted (EU)
  - Positive Phase 1 study results
  - Agreement with EMA on pivotal study design

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**LJPC-401: Phase 1 Study Design**

- **Population:** Adult patients at risk of iron overload (e.g., HH, thalassemia, SCD)
- **Design:** Phase 1, open-label, dose-escalation, study
- **Study Duration:** Single SC dose, 7-day observation

**PRIMARY ENDPOINT**
Safety and tolerability via review of:
- Treatment Emergent Adverse Events (TEAEs), changes in clinical lab values, ECGs, vital sign and physical exam data

**SECONDARY ENDPOINT**
Serum iron

Data Monitoring Committee (DMC) made dose-escalation decisions

**Escalating dose levels**
3 to 6 subjects at each dose level
LJPC-401: Phase 1 Results Overview

- Fifteen patients dosed at escalating dose levels from 1 mg to 20 mg
  - Patient subtypes treated: HH = 10; SCD = 3; and thalassemia = 2
- Safety observations
  - No dose-limiting toxicities at any dose level
  - 1 SAE at 1 mg dose level unrelated to study drug
    - Hospitalization for acute sickle cell crisis; fully resolved
  - 9 injection-site reactions – all were mild or moderate in severity, self-limiting, and fully resolved
  - No significant changes in serum chemistries or hematology other than serum iron parameters
- Pharmacodynamic results
  - Dose-dependent, statistically significant reduction in serum iron (p=0.008)
  - Maximum serum iron reduction observed at 8 hours post-dose
  - Durable effect observed through last observation on Day 7
Individual dose p-values for change from baseline not adjusted for a potential regression to the mean effect.
Dose response not adjusted for multiple comparisons.
LJPC-401: Percent Change in Serum Iron
Baseline through Day 7 for 20 mg Dose

Change from baseline through day 7: -21%
LJPC-401: Update on Registration Plan

Agreement Reached on Pivotal Study

- Agreement reached with European Medicines Agency (EMA) on pivotal study design
- Randomized, controlled, multi-center study in beta thalassemia patients suffering from iron overload
  - A major unmet medical need in an orphan patient population
- Primary endpoint is a clinically relevant measurement directly related to iron overload
- Plan to initiate study H2, 2017
LJPC-401: Patient Need

- Hereditary Hemochromatosis (HH)
  - Most common genetic disease in Caucasians
  - Silent Killer - Iron accumulation can lead to liver cirrhosis, liver cancer, heart disease and/or failure, and diabetes
  - No FDA-approved treatment
  - Current treatments don’t address the underlying disease pathology and/or can have lethal side effects
    - Iron chelators may cause kidney failure, liver failure or gastrointestinal hemorrhage
    - Phlebotomy creates heavy patient burden with weekly procedures for >1 year
  - Significantly underdiagnosed despite simple, inexpensive and readily available genetic and serum iron tests
    - ~250,000 people in U.S. have clinically significant iron overload due to HH
- Acquired Iron Overload: thalassemia, sickle cell disease (SCD) and myelodysplasia (MDS)
  - Attractive treatment alternative for iron overload in lieu of chelation therapy
- EMA orphan designation for beta thalassemia and SCD
Agenda

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• LJPC-401 (Synthetic Human Hepcidin) for Iron Overload

• Financial Position
Financial Position

<table>
<thead>
<tr>
<th>Condensed Balance Sheet Data</th>
<th>As of June 30, 2017 (in millions)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Cash</td>
<td>$141.3</td>
</tr>
<tr>
<td>Total liabilities</td>
<td>$8.3</td>
</tr>
<tr>
<td>Total shareholders’ equity¹</td>
<td>$140.3</td>
</tr>
</tbody>
</table>

Cash resources expected to fund Company into second half of 2018

| Fully Diluted, As-Converted Shares Outstanding¹ | 33,631,994 |

¹ Includes common stock, preferred stock (as-converted), and outstanding equity awards as of June 30, 2017
LJPC Corporate Highlights

- Focused on de-risked product opportunities
  - Naturally occurring peptides with well-understood biological functions
  - Derivative components of FDA-approved products
- LJPC-501 (synthetic human angiotensin II) for the treatment of hypotension in adults with distributive or vasodilatory shock who remain hypotensive despite fluid and vasopressor therapy
  - Expanded Access Program launched in August 2017
  - New Drug Application accepted and Priority Review granted in August 2017
  - The Prescription Drug User Fee Act (PDUFA) is February 28, 2018
- LJPC-401 (synthetic human hepcidin) for iron overload caused by diseases such as hereditary hemochromatosis (HH), thalassemia, sickle cell disease (SCD) and myelodysplasia (MDS)
  - Positive Phase 1 results: statistically significant reduction in serum iron
  - Agreement reached with EMA on pivotal study design
- LJPC-30S (gentamicin derivatives) for bacterial infections and rare genetic diseases
Thank You