Corporate Presentation

Developing Innovative Therapies for Patients Suffering from Life-threatening Diseases

NASDAQ: LJPC

January 2018
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 expectations regarding future events or La Jolla’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. La Jolla 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 La Jolla’s filings with the U.S. Securities and Exchange Commission (SEC), all of which are available free of charge on the SEC’s website www.sec.gov. These risks include, but are not limited to, risks relating to: the timing for commercial launch of GIAPREZA™ (angiotensin II); the degree of physician or pharmacy and therapeutics committee adoption of GIAPREZA; La Jolla’s success in commercializing GIAPREZA; the timing and availability of GIAPREZA in the market; risks relating to the scope of the GIAPREZA product label; potential market sizes, including for septic or other distributive shock; the anticipated treatment of future clinical data by the FDA, the EMA or other regulatory authorities, including whether such data will be sufficient for approval of GIAPREZA in the EMA, or for approval of LJPC-401 by either the FDA or EMA; the timing, costs, conduct and outcome of clinical studies; the impact of pharmaceutical industry regulation and healthcare legislation in the United States; the impact of unexpected hurdles which may be influenced by, among other things, the occurrence of adverse safety events, or failure to protect intellectual property and other proprietary rights; 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.
Agenda

• Overview of LJPC
• GIAPREZA™ (angiotensin II)
• 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

• GIAPREZA™ (angiotensin II)
  ▪ U.S. Food and Drug Administration (FDA) has approved GIAPREZA to increase blood pressure in adults with septic or other distributive shock
  ▪ Planned availability to hospitals for patients in the U.S. in March 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 study results reported
  ▪ Agreement reached with EMA on pivotal study design in beta thalassemia
  ▪ Pivotal study initiated in patients with transfusion-dependent beta thalassemia in December 2017
  ▪ Phase 2 study initiated in patients with hereditary hemochromatosis in December 2017
### LJPC-401
*Synthetic Human Hepcidin*

**Indication**
- Iron overload due to beta thalassemia
- Iron overload due to hereditary hemochromatosis

**Other R&D**
- Various

**Phase 1**
- Completed/milestone achieved

**Phase 2**
- Pivotal Study
  - Pivotal Study initiated in Dec. 2017
  - Phase 2 study initiated in Dec. 2017

**Phase 3**
- Planned

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*In the European Union*
Overview of LJPC

GIAPREZA™ (angiotensin II)

LJPC-401 (Synthetic Human Hepcidin) for Iron Overload

Financial Position
GIAPREZA™ (angiotensin II) Injection for Intravenous Infusion is indicated to increase blood pressure in adults with septic or other distributive shock

"We appreciate FDA's rapid review and approval of GIAPREZA and are especially grateful to the patients, families and dedicated critical care teams who made the development of GIAPREZA possible. We look forward to bringing this new treatment option to the many critically ill patients suffering from septic or other distributive shock."

GIAPREZA is classified as a new chemical entity exclusivity (NCE) with 5 years of market exclusivity
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

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

MORTALITY RATES COMPARED

- Shock: ≥50% mortality in patients with shock in the ICU
- 30-day mortality rate
  - 14% for AMI
  - 12% for CHF
  - 16% for Pneumonia

Distributive Shock is the Most Common Type of Shock in the Inpatient Setting

- **Prevalence**
  - Cardiogenic: 16%
  - Hypovolemic: 16%
  - Obstructive: 2%
  - **Distributive (94% is Septic Shock):** 66%

- **Types of Shock**
  - **Distributive Shock**
  - **Hypovolemic Shock**
  - **Cardiogenic Shock**
  - **Obstructive Shock**

Hypotension, or Abnormally Low Blood Pressure, is an Important Hemodynamic Marker for a Vasodilatory or Distributive Shock

Distributive Shock is Costly

Source: CMS FY14 Inpatient Public Use File (IPUF)

<table>
<thead>
<tr>
<th>Condition</th>
<th>Weighted Average CMS Covered Charges</th>
</tr>
</thead>
<tbody>
<tr>
<td>Severe Distributive Shock</td>
<td>$87,282</td>
</tr>
<tr>
<td>AMI</td>
<td>$42,243</td>
</tr>
<tr>
<td>CHF</td>
<td>$31,453</td>
</tr>
<tr>
<td>Pneumonia</td>
<td>$30,702</td>
</tr>
</tbody>
</table>

An average day in the ICU costs between $4,500 to $6,000

Mechanical ventilation adds ~$1,500

Hospitals are implementing multiple quality initiatives to improve patient care and maximize CMS reimbursements

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

Source: CMS FY14 Inpatient Public Use File (IPUF)
GIAPREZA Provides a New Approach For Increasing Blood Pressure In Distributive Shock

THERAPIES AND MECHANISMS

GIAPREZA™ (angiotensin II) Injection for Intravenous Infusion

RENIN ANGIOTENSIN-ALDOSTERONE

CATECHOLAMINES¹: SYMPATHETIC NERVOUS

VASOPRESSIN: ARGININE-VASOPRESSIN

1. Catecholamines include: norepinephrine, epinephrine, dopamine, phenylephrine, ephedrine
GIAPREZA: A Novel Vasopressor For Patients with Distributive Shock

Robust Response - 70% Patients achieved and maintained target MAP primary endpoint at hour 3 as compared to 23% in placebo arm

Rapid Response - Median response time to reach target MAP was 5 minutes

Sustained Response – Maintained throughout the treatment period

Mortality Trend – Mortality through Day 28 was 46% on GIAPREZA and 54% on placebo (HR 0.78; CI 0.57– 1.07)

Safety – Percent of patients with AEs were similar between the two treatment arms¹

• There is a potential for venous and arterial thromboembolic events (AEs 12.9% v 5.1%, DVT SAEs 1.8% v 0%)*

Abbreviations: AEs=Adverse Events; CI=Confidence Interval; DVT=Deep Vein Thrombosis; HR=Hazard Ratio; MAP=Mean Arterial Pressure; SAEs=Serious Adverse Events


*Use concurrent venous thromboembolism (VTE) prophylaxis
Distributive Shock is Prevalent

**Distributive Shock Patients**

- **First Line Standard of Care**
  - 745,000 Patients per Year\(^1\)

- **Second-Line Standard of Care**
  - 313,000 Patients\(^2\) per Year


2. SHA Integrated Pack Units from Aug 2017 – Jul 2017 3.01M, Filtered for hospitals, applying an estimated 10% stocking adjustment, inpatient percentage and average vials/patient based on Premier data inpatient Vasostrict patients projected to national numbers.
Preparing For Commercialization in 2018

**Commercial**
- Market Research
- Healthcare Professional Marketing
- Multi-Channel Marketing
- Professional Education
- Commercial Insights & Operations
- Market Access

**Medical Affairs**
- Medical Science Liaisons*
- Medical Communications
- HEOR Team
- Medical Education

**Field Sales**
- National and Regional Sales Leadership*
- Critical Care Nurse Educators*
- Hospital Critical Care Field Representative*
- Market Access Team*
- Sales Training
- Field Trade

*~136 Customer Facing FTEs
Agenda

- Overview of LJPC
- GIAPREZA™ (angiotensin II)
- 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 in beta thalassemia.

- Pivotal study initiated in patients with transfusion-dependent beta thalassemia.

- Phase 2 study initiated in patients with hereditary hemochromatosis.
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

GLP Toxicology Study in Dogs

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 signs, and physical exam data

**SECONDARY ENDPOINT**
Serum iron

- Escalating dose levels
  - 3 to 6 subjects at each dose level

Data Monitoring Committee (DMC) made dose-escalation decisions.
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
LJPC-401: Percent Change in Serum Iron
Baseline to Hour 8

Dose Group

<table>
<thead>
<tr>
<th>Dose Group</th>
<th>Sample Size</th>
<th>Percent Change</th>
<th>p-value</th>
</tr>
</thead>
<tbody>
<tr>
<td>1 mg (n=3)</td>
<td>14.2</td>
<td>0.149</td>
<td></td>
</tr>
<tr>
<td>5 mg (n=3)</td>
<td>-26.7</td>
<td>0.304</td>
<td></td>
</tr>
<tr>
<td>10 mg (n=3)</td>
<td>-45.5</td>
<td>0.054</td>
<td></td>
</tr>
<tr>
<td>20 mg (n=6)</td>
<td>-58.1</td>
<td>0.001</td>
<td></td>
</tr>
</tbody>
</table>

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%
**LJ401-BT01 Beta Thalassemia EU Registration Study**

- **Population:** Transfusion dep. BT, cardiac iron levels by MRI T2* of 6 to 35 mSec
- **Design:** Phase 2, randomized, parallel arm study for treatment of refractory cardiac IO.

- **Primary Endpoint:** Mean change from baseline in cardiac iron level as measured by MRI T2*
- **Dosing:** Individualized based on Transferrin Saturation

- **N = 100**
- **40 sites**
- **9 countries**
LJ401-HH01
Phase 2 Hereditary Hemochromatosis – Study Design

- 60 Patients, 25 sites, 5 countries (US, AUS, UK, Ire, Fr)

- Study Design
  - Randomized 1:1 to: LJ401 or Placebo
  - Population: Hereditary Hemochromatosis patients who have been receiving therapeutic phlebotomy
    - TSAT > 45%, S.Ferritin 150 – 1,000 ng/mL, not on chelation
  - 3 month treatment

- Primary endpoint: Change in TSAT

- Secondary endpoints:
  - Phlebotomy requirement
  - Serum Ferritin
  - Safety
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
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<table>
<thead>
<tr>
<th>Condensed Balance Sheet Data</th>
<th>As of September 30, 2017 (in millions)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Cash</td>
<td>$120.8</td>
</tr>
<tr>
<td>Total liabilities</td>
<td>$10.3</td>
</tr>
<tr>
<td>Total shareholders’ equity¹</td>
<td>$119.8</td>
</tr>
</tbody>
</table>

Cash resources expected to fund Company into second half of 2018

| Fully Diluted, As-Converted Shares Outstanding¹ | 34,017,102                             |

¹ Includes common stock, preferred stock (as-converted), and outstanding equity awards as of September 30, 2017
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Thank You