Corporate Presentation

Developing Innovative Therapies for Patients Suffering from Life-threatening Diseases

NASDAQ: LJPC

September 2019
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 as well as preliminary results subject to audit by La Jolla's independent registered public accounting firm. 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: commercialization plans in Europe, including the timing for commercial launch of GIAPREZA in Europe; risks relating to the scope of the GIAPREZA product label; our ability to grow net sales of GIAPREZA; the anticipated timing for regulatory filings and regulatory actions; the timing and status of LJPC-0118 at time of FDA approval; the consistency between the full data set, topline data and interim results from the LJ401-HH01 study; the anticipated treatment of future clinical data by the U.S. Food and Drug Administration (FDA), European Commission (EC) and other regulatory authorities, including whether such data will be sufficient for approval; the timing, costs, conduct and outcome of clinical studies; risks relating to the development of drug candidates; 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; the expected duration over which La Jolla’s cash balances will fund its operations; and other risks and uncertainties identified in our filings with the SEC. 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-0118 for Severe Malaria
- 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.
• GIAPREZA™ (angiotensin II)
  ▪ Approved by FDA to increase blood pressure in adults with septic or other distributive shock
  ▪ Q2 US net sales $5.7M
  ▪ Centers for Medicare & Medicaid Services (CMS) has granted a New Technology Add-on Payment (NTAP). The NTAP is effective for the CMS 2019 fiscal year, which began on October 1, 2018
  ▪ Approved by European Medicines Agency’s (EMA)

• LJPC-0118: New Drug Application (NDA) planned for Q4 2019, for the treatment of severe malaria
  ▪ Active ingredient shown to improve survival
  ▪ FDA Breakthrough Therapy and Orphan designations

• LJPC-401 (synthetic human hepcidin) for iron overload caused by diseases such as hereditary hemochromatosis (HH), thalassemia, sickle cell disease (SCD) and myelodysplasia syndrome (MDS)
  ▪ Positive results interim analysis of phase 2 study in patients with HH
  ▪ Agreement reached with EMA on pivotal study design in beta thalassemia. Study underway with data expected mid-2020
**Product Pipeline**

<table>
<thead>
<tr>
<th>Indication/ Proposed Indication</th>
<th>IND</th>
<th>Phase 1</th>
<th>Phase 2</th>
<th>Phase 3</th>
<th>FDA/EMA Review</th>
</tr>
</thead>
</table>
| **GIAPREZA**  
*Synthetic Human Angiotensin II*  
Increases blood pressure in adults with septic or other distributive shock. Pediatric indication is being pursued | | | | | **Approved U.S.** |
| **LJPC-0118**  
Severe malaria | | | | | **Now Approved EU** |
| **LJPC-401**  
*Synthetic Human Hepcidin*  
Iron overload due to beta thalassemia  
Iron overload due to hereditary hemochromatosis | | | | | **NDA planned for Q4 2019** |
| **Other R&D**  
Various | | | | | |

* In the European Union

* Completed/milestone achieved  
* Underway  
* Planned

**GIAPREZA**
- Indication: Iron overload due to beta thalassemia
- Phase 1: IND
- Phase 2: Underway
- Phase 3: Underway
- FDA/EMA Review: Approved U.S.

**LJPC-0118**
- Indication: Severe malaria
- Phase 1: IND
- Phase 2: Underway
- Phase 3: Underway
- FDA/EMA Review: Now Approved EU

**LJPC-401**
- Indication: Iron overload due to hereditary hemochromatosis
- Phase 1: IND
- Phase 2: Underway
- Phase 3: Underway
- FDA/EMA Review: NDA planned for Q4 2019

**Other R&D**
- Indication: Various
- Phase 1: Completed
- Phase 2: Planned
- Phase 3: Planned

*Pivotal Study* initiated in Dec. 2017

Positive results announced from pre-specified interim analysis

**GIAPREZA**
- Indication: Iron overload due to hereditary hemochromatosis
- Phase 1: Underway
- Phase 2: Underway
- FDA/EMA Review: Approved U.S.

**LJPC-0118**
- Indication: Severe malaria
- Phase 1: Completed
- Phase 2: Underway
- Phase 3: Underway
- FDA/EMA Review: Now Approved EU

**LJPC-401**
- Indication: Iron overload due to hereditary hemochromatosis
- Phase 1: Completed
- Phase 2: Planned
- Phase 3: Underway
- FDA/EMA Review: NDA planned for Q4 2019

**Other R&D**
- Indication: Various
- Phase 1: Planned
- Phase 2: Planned
- Phase 3: Planned

*Pivotal Study* initiated in Dec. 2017

Positive results announced from pre-specified interim analysis

* In the European Union
Overview of LJPC

GIAPREZA™ (angiotensin II)

LJPC-0118 for Severe Malaria

LJPC-401 (Synthetic Human Hepcidin) for Iron Overload

Financial Position
GIAPREZA: Now Approved to Increase Blood Pressure in Adults with Septic or Other 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
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.

Distributive Shock is Prevalent

Role of GIAPREZA
A Significant Number of Patients Remain Hypotensive Despite Standard of Care Fluid And Vasopressor Therapy\textsuperscript{1,2}

Typical Treatment Cascade For Distributive Shock Patients

<table>
<thead>
<tr>
<th>1st Line</th>
<th>2nd Line</th>
<th>3rd Line</th>
<th>4th Line</th>
</tr>
</thead>
<tbody>
<tr>
<td>Lower Dose Vasopressor (Norepinephrine equivalent &lt;0.2 mcg/kg/min)</td>
<td>Second Vasopressor initiated</td>
<td>Increased Dose of Norepinephrine Equivalent of up to 0.4 mcg/kg/min</td>
<td>Norepinephrine Equivalent Greater or equal to 0.4 mcg/kg/min</td>
</tr>
</tbody>
</table>

Role for GIAPREZA*

Annual No. of DS Patients Presenting at US Hospitals Who Receive Each Line of Treatment\textsuperscript{1}

833K → 768K → 322K → 203K → 102K

*GIAPREZA and placebo were studied in conjunction with SoC vasopressors, including norepinephrine, epinephrine, dopamine, phenylephrine, and vasopressin.

Abbreviations: DS, distributive shock

GIAPREZA Provides a New Approach For Increasing Blood Pressure In Distributive Shock

THERAPIES AND MECHANISMS

GIAPREZA™
(angiotensin II)
Injection for Intravenous Infusion

RENNIN ANGIOTENSIN-ALDOSTERONE

CATECHOLAMINES¹: SYMPATHETIC NERVOUS

VASOPRESSIN: ARGinine-VASOPRESSIN

1. Catecholamines include: norepinephrine, epinephrine, dopamine, phenylephrine, ephedrine
Additional Published Data on GIAPREZA (angiotensin II)

More Patients on GIAPREZA Discontinued Renal Replacement Therapy (RRT)

- 38% of patients in the Ang II group discontinued RRT compared with 15% in the placebo group
- 105 AKI patients receiving RRT at baseline were included (Ang II = 45, Placebo = 60)
  (adjusted HR = 2.90; 95% CI, 1.29-6.52; \( p = .007 \))
- Sensitivity analysis using logistic regression models confirms these findings

23rd International Conference on Advances in Critical Care Nephrology - AKI & CRRT 2018
“Outcomes in Patients with Acute Kidney Injury Receiving Angiotensin II for Vasodilatory Shock”

CI=Confidence Interval; HR=Hazard Ratio; RRT=Renal Replacement Therapy
Survival Was Significantly Longer on GIAPREZA

- 53% of patients in the Ang II group with AKI at baseline were alive at day 28 compared with 30% in the placebo group (unadjusted HR = 0.52; 95% CI, 0.30-0.87; P = .012)

- Sensitivity analysis using a Cox proportional hazards model confirms these findings

23rd International Conference on Advances in Critical Care Nephrology - AKI & CRRT 2018

“Outcomes in Patients with Acute Kidney Injury Receiving Angiotensin II for Vasodilatory Shock”
GIAPREZA Regulatory Status in EU

• Approved by The European Medicines Agency’s (EMA) for the treatment of refractory hypotension in adults with septic or other distributive shock who remain hypotensive despite adequate volume restitution and application of catecholamines and other available vasopressor therapies

• Commercial assessment and preparation underway
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- Financial Position
LJPC-0118 for the Treatment of Severe Malaria

- Severe Malaria: a fatal and debilitating disorder
  - Systemic infection leading to central nervous system compromise and coma; shock with hypotension and death in many cases
  - Approximately 1,500 annual cases of malaria in US
    - ~17% severe; often fatal and debilitating
  - Almost 500,000 deaths worldwide

- LJPC-0118
  - Active pharmaceutical ingredient shown to improve survival in 2 randomized clinical trials
  - Current clinical trial needed for NDA completed
  - FDA Breakthrough Therapy and Orphan designations

- NDA expected Q4 2019
  - May qualify for tropical disease priority review voucher (PRV)
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LJPC-401: Overview

Multiple Potential Indications

• 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

• Secondary iron overload
  ▪ Patients with thalassemia (including beta thalassemia), sickle cell disease (SCD) and myelodysplasia syndrome (MDS)

• Positive interim analysis of phase 2 study in patients with HH
  ▪ Top-line results expected in Q4 2019.

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

• Orphan Drug Designation granted European Union (EU) for beta thalassemia (BT) and SCD

• Agreement reached with European Medicines Agency (EMA) on pivotal study design in BT

• Pivotal study initiated in patients with transfusion-dependent BT
  ▪ Topline data expected mid-2020
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 and interim Phase 2 study results
  - Agreement with EMA on pivotal study design

From: Blood Research Vol. 48, No. 1, p.10, March 2013
Primary Endpoint: Change in TSAT.

Designed (with 80% statistical power) to detect a 15% improvement in TSAT between LJPC-401 and placebo arm assuming a common standard deviations of 18% and 2-sided alpha of 5%.

Secondary Endpoints: number of phlebotomies from Day 2 to End of Study (EOS), change in serum ferritin

Safety Endpoints: adverse events, change in clinical laboratory evaluations including serum iron parameters, change in vital signs, change in ECG, use of concomitant medications, physical exam findings, immunogenicity

Randomization Strata:
- Screening TSAT (>45% to 70% vs. >70%)
- Phlebotomy frequency over the prior 12 months (0 to 3 vs. >3)
## Primary Analysis: High-level Summary

<table>
<thead>
<tr>
<th>Primary Efficacy Endpoint: Week 16 post-dose Change in TSAT Mean (SD)</th>
<th>Placebo (N=14)</th>
<th>LJPC-401 (N=12)</th>
<th>Statistics</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>-6.0 (17.28)</td>
<td>-41.5 (16.43)</td>
<td>p&lt;0.0001</td>
</tr>
</tbody>
</table>

| Secondary Efficacy Endpoint: Number of Phlebotomies Mean (SD) | 1.71 (1.729) | 0.25 (0.622) | p=0.0030 |

| Injection Site Reactions (ISR) | 6.5% | 79% All mild/mod | p<0.01 |

| ISR leading to early termination | 0% | 0% |
Pivotal Study (HELIOS)
Beta Thalassemia

**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:**
Fixed weekly dosing during the primary efficacy period

- N = 100
- 40 sites
- 9 countries

BT = beta thalassemia, MRI = Magnetic resonance imaging, IO = Iron overload
Pivotal Study design agreed upon with EMA via scientific advice
LJPC-401 Conclusions

- Positive interim analysis of phase 2 study in patients with HH. Top line results expected in Q4 2019
- New formulation has improved PK exposure and PD effect with no corresponding increase of injection site reaction severity or duration
- Ongoing studies to further explore the iron-regulating effects in patients with iron-overload disorders: milestones expected in second half 2019
  1. Pivotal study in beta thalassemia (HELIOS) topline data expected mid-2020
  2. Phase 2 study in HH (HERCULES) topline data expected Q4 2019
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## Financial Position

### Recent Corporate Highlights and Key Objectives

<table>
<thead>
<tr>
<th>Description</th>
<th>Amount</th>
</tr>
</thead>
<tbody>
<tr>
<td>Cash as of June 30, 2019</td>
<td>$123 million</td>
</tr>
<tr>
<td>2019 expected cash used in Operating Activity</td>
<td>$89 million to $94 million</td>
</tr>
<tr>
<td>Six Month Net Sales of GIAPREZA</td>
<td>$10.1 million</td>
</tr>
<tr>
<td>Full-Year 2019 Net Sales Guidance for GIAPREZA</td>
<td>$24 million to $28 million</td>
</tr>
</tbody>
</table>

*Cash resources expected to fund Company into late 2020*

### Fully Diluted, As-Converted Shares Outstanding

<table>
<thead>
<tr>
<th>Description</th>
<th>Quantity</th>
</tr>
</thead>
<tbody>
<tr>
<td>Fully Diluted, As-Converted Shares Outstanding¹</td>
<td>33,861,000</td>
</tr>
</tbody>
</table>

¹Includes common stock, preferred stock (as-converted) as of June 30, 2019
LJPC Corporate Highlights

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Thank You