Corporate Presentation

Developing Innovative Therapies for Patients Suffering from Life-threatening Diseases

NASDAQ: LJPC

May 2019
Forward-Looking Statements

These slides contain forward-looking statements as that term is defined in the Private Securities Litigation Reform Act of 1995, including, but not limited to, statements related to La Jolla’s expectations regarding net sales and net cash used in operating activities for the full-year 2019, the expectation for future clinical and regulatory milestones, such as the new drug application submission and expected timing for commencement and completion of clinical studies. 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: our ability to successfully commercialize, market and achieve market acceptance of GIAPREZA; our ability to grow net sales of GIAPREZA; the effectiveness of GIAPREZA in the clinical setting, including the lack of negative and/or unintended side-effects; the timing and prospects for approval of GIAPREZA by the European Medicines Agency (EMA) or other regulatory authorities, the scope of product label(s) and potential market sizes; the timing, costs, conduct and outcome of clinical studies; risks relating to the development of drug candidates; the anticipated treatment of future clinical data by the U.S. Food and Drug Administration (FDA), EMA and other regulatory authorities, including whether such data will be sufficient for approval; 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.
LJPC Corporate Highlights

- GIAPREZA™ (angiotensin II)
  - Approved by FDA to increase blood pressure in adults with septic or other distributive shock
  - Marketing launch March 2018: 2018 net sales $10.1M
  - Marketing Authorisation Application (MAA) in European Union (EU) validated; decision expected June 2019
  - 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

- LJPC-0118: New Drug Application (NDA) planned for Q4 2019, for the treatment of severe malaria
  - Active ingredient shown to improve survival
  - April 2019 FDA has granted Breakthrough Therapy designation

- 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 Phase 1 study results reported
  - Agreement reached with EMA on pivotal study design in beta thalassemia. Study underway with data expected mid-2020
  - Phase 2 study initiated in patients with hereditary hemochromatosis with data expected second half 2019
## Product Pipeline

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

* In the European Union

### Indication

- **Iron overload due to beta thalassemia**  
  - Phase 2 study initiated in Dec. 2017

- **Iron overload due to hereditary hemochromatosis**  
  - Pivotal Study* initiated in Dec. 2017

- **Severe malaria**

### Other R&D Indications

- **Various**

* * In the European Union

### Completed/milestone achieved

- GIAPREZA
- LJPC-0118
- LJPC-401
- Other R&D

### Underway

- GIAPREZA
- LJPC-401

### Planned

- Other R&D

### FDA/EMA Review

- GIAPREZA
- LJPC-0118
- LJPC-401
- Other R&D

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**GIAPREZA**

- **Prototype**
- **Synthetic Human Angiotensin II**
- **Purpose**
  - Increases blood pressure in adults with septic or other distributive shock. Pediatric indication is being pursued.

**LJPC-0118**

- **Prototype**
- **Purpose**
  - Severe malaria

**LJPC-401**

- **Prototype**
- **Purpose**
  - Iron overload due to beta thalassemia  
  - Iron overload due to hereditary hemochromatosis

**Other R&D**

- **Purpose**
  - Various

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*In the European Union*
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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\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

<table>
<thead>
<tr>
<th>Condition</th>
<th>30-day mortality rate\textsuperscript{3}</th>
</tr>
</thead>
<tbody>
<tr>
<td>Shock</td>
<td>≥50% mortality in patients with shock in the ICU\textsuperscript{2}</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 Costly

Weighted Average CMS Covered Charges

- **Severe Distributive Shock**: $87,282
- **AMI**: $42,243
- **CHF**: $31,453
- **Pneumonia**: $30,702

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

- 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

Source: CMS FY14 Inpatient Public Use File (IPUF)
Distributive Shock is Prevalent

Role of GIAPREZA
A Significant Number of Patients Remain Hypotensive Despite Standard of Care Fluid And Vasopressor Therapy

Typical Treatment Cascade For Distributive Shock Patients

IV Fluids → 1st Line: Lower Dose Vasopressor (Norepinephrine equivalent <0.2 mcg/kg/min) → 2nd Line: Second Vasopressor initiated → 3rd Line: Increased Dose of Norepinephrine Equivalent of up to 0.4 mcg/kg/min → 4th Line: Norepinephrine Equivalent Greater or equal to 0.4 mcg/kg/min

Role for GIAPREZA*

Annual No. of DS Patients Presenting at US Hospitals Who Receive Each Line of Treatment:
- 833K for IV Fluids
- 768K for 1st Line
- 322K for 2nd Line
- 203K for 3rd Line
- 102K for 4th Line

Abbreviations: DS, distributive shock
*GIAPREZA and placebo were studied in conjunction with SoC vasopressors, including norepinephrine, epinephrine, dopamine, phenylephrine, and vasopressin.
GIAPREZA™ (angiotensin II) Injection for Intravenous Infusion

RENIN ANGIOTENSIN-ALDOSTERONE

CATECHOLAMINES¹: SYMPATHETIC NERVOUS

VASOPRESSIN: ARGinine-VASOPRESSIN

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

- 23rd International Conference on Advances in Critical Care Nephrology - AKI & CRRT 2018 on March 6, 2018
  - Outcomes in Patients with Acute Kidney Injury Receiving Angiotensin II for Vasodilatory Shock
  - Manuscript\(^1\) published online on March 6, 2018 in Critical Care Medicine

- Society of Critical Care Medicine’s (SCCM) 48th Critical Care Congress on February 18 -19, 2019
  - Effect of Disease Severity on Survival in Patients Receiving Angiotensin II for Vasodilatory Shock\(^2\)
  - GIAPREZA (angiotensin II), A Novel Treatment Option for Septic or Other Distributive Shock
  - Effect of Angiotensin II on Vasopressor Dose and Safety in Patients with Severe Vasodilatory Shock
  - Juvenile Developmental Toxicity of LJPC-501 (angiotensin II) in Newborn Sheep
  - Time Below Map Threshold in First 24 Hours of Initiation of Vasopressor Therapy and Mortality
  - Outcomes in Patients with Postoperative Vasoplegia Receiving Angiotensin II for Vasodilatory Shock

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

CI=Confidence Interval; HR=Hazard Ratio; RRT=Renal Replacement Therapy

“Outcomes in Patients with Acute Kidney Injury Receiving Angiotensin II for Vasodilatory Shock”
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”
• Positive Scientific Advice Fall 2017
  ▪ MAP identified as a clinically relevant endpoint

• Pre-MAA meeting held Jan. 2018
  ▪ Administrative tasks identified
  ▪ Follow up scientific advice on post-authorization study occurred in April 2018

• MAA validated by EMA in June 2018
  ▪ EMA decision expected June 2019

• Commercial assessment and preparation underway
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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 underway and fully recruited
  - April 2019 FDA has granted Breakthrough Therapy designation

- 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)
- 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 European Union (EU) for BT and SCD
- Agreement reached with European Medicines Agency (EMA) on pivotal study design in beta thalassemia
- Pivotal study initiated in patients with transfusion-dependent beta thalassemia
  - Topline data expected mid-2020
- Phase 2 study initiated in patients with hereditary hemochromatosis
  - Topline data expected second half 2019
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

LJPC-401: Phase 1 Results Overview

Patients with Iron Overload

- Fifteen patients dosed at escalating dose levels from 1 mg to 30 mg
  - Patient subtypes treated: HH; SCD; and thalassemia.

- 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
  - 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 Response not adjusted for multiple comparisons.

Individual dose p-values for change from baseline not adjusted for a potential regression to the mean effect.

Dose Group

- **1 mg (n=3)**: 
  - Percent Change in Serum Iron: -14.2%
  - p-value: 0.149

- **5 mg (n=3)**: 
  - Percent Change in Serum Iron: -26.7%
  - p-value: 0.304

- **10 mg (n=3)**: 
  - Percent Change in Serum Iron: -45.5%
  - p-value: 0.054

- **20 mg (n=6)**: 
  - Percent Change in Serum Iron: -58.1%
  - p-value: 0.001

Dose Response p=0.008
Enhanced bioavailability resulted in greater iron reduction at the same dose.
Pivotal Study in Beta Thalassemia

Study Currently Enrolling

- **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*

- **N = 100**
- **40 sites**
- **9 countries**

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

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BT= beta thalassemia, MRI = Magnetic resonance imaging, IO= Iron overload
Pivotal Study design agreed upon with EMA via scientific advice
Hereditary Hemochromatosis Phase 2  
*Study Currently Enrolling*

- 60 Patients, 25 sites, 4 countries (US, AUS, UK, FR)
- *Study Design*
  - Randomized 1:1 to LJPC-401 or Placebo
  - Population: Hereditary Hemochromatosis patients who have been receiving therapeutic phlebotomy
    - TSAT > 45%, Serum Ferritin 150 – 1,000 ng/mL, not on chelation
  - 4 month treatment
- *Primary endpoint: Change in TSAT*
- *Secondary endpoints:*
  - Phlebotomy Requirement
  - Serum Ferritin
  - Safety
LJPC-401 Conclusions

- LJPC-401 was well tolerated at doses between 1 mg and 30 mg, with the maximum iron-lowering effect observed at 20 mg.
- LJPC-401 showed significant decreases in serum iron levels compared with baseline, which were sustained in most patients for up to 8 days.
- In comparison to healthy adults, in whom LJPC-401 caused a decrease in serum iron levels that returned to baseline levels within 48 hours, the iron-lowering effect in iron overload patients was more sustained.
- 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 second half 2019.
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## Recent Corporate Highlights and Key Objectives

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<thead>
<tr>
<th>Description</th>
<th>Amount</th>
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<tbody>
<tr>
<td>Cash as of March 31, 2019</td>
<td>$140 million</td>
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<tr>
<td>2019 expected cash used in Operating Activity</td>
<td>$89 million to $94 million</td>
</tr>
<tr>
<td>First Quarter Net Sales of GIAPREZA</td>
<td>$4.4 million</td>
</tr>
<tr>
<td>Full-Year 2019 Net Sales Guidance for GIAPREZA</td>
<td>$24 million to $28 million</td>
</tr>
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**Cash resources expected to fund Company into late 2020**

### Fully Diluted, As-Converted Shares Outstanding

| Shares Outstanding | 33,828,404 |

1 Includes common stock, preferred stock (as-converted) as of March 31, 2019
Thank You