Corporate Presentation

Developing Innovative Therapies for Patients Suffering from Life-threatening Diseases

NASDAQ: LJPC

September 2016
Forward-Looking Statements

These slides contain "forward-looking" statements within the meaning of the Private Securities Litigation Reform Act of 1995. These statements may be identified by the use of forward-looking terminology such as "anticipate", "believe", "continue", "could", "estimate", "expect", "intend", "may", "might", "plan", "potential", "predict", "should" or "will" and include statements regarding La Jolla’s product candidates and clinical trial progress and results. These forward-looking statements are based on our current expectations and beliefs, speak only as of the date of this presentation and involve risks and uncertainties, many of which are outside of our control, that can cause actual results to differ materially from those anticipated in the forward-looking statements. Potential risks and uncertainties include, but are not limited to: our ability to commence and complete clinical studies within projected time periods, the degree to which initial clinical study results are indicative of expected results for future studies, anticipated regulatory and patent exclusivity periods, the ability to manufacture clinical or commercial products successfully, the ability to resolve regulatory issues, the ability to out-license programs, estimated market sizes and anticipated pricing levels for drug candidates, anticipated rates of physician adoption, if our drug candidates are approved, the ability to successfully develop our product candidates, including the results of ongoing and future clinical trials (including product safety issues and efficacy results), the ability to successfully prosecute patents and whether such patents will confer protection for our product candidates, and the expected duration of the Company’s operating runway based on current cash resources. Further information regarding these and other risks that could affect our future results of operations are included in La Jolla’s most recently filed Annual Report on Form 10-K and subsequent Quarterly Reports on Form 10-Q under the caption “Risk Factors,” as filed with the U.S. Securities and Exchange Commission at www.sec.gov. We disclaim any intent to update any forward-looking statements to reflect actual events that occur after the date of this presentation.
Agenda

• Overview of LJPC
  • LJPC-501 (Angiotensin II) for CRH
  • LJPC-401 (Synthetic Hepcidin) for Iron Overload
  • LJPC-30Sa/b (Gentamicin Derivatives) for Bacterial Infections and Rare Genetic Disorders
• 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 (angiotensin II) for catecholamine-resistant hypotension (CRH)
  - Phase 3 registration study actively enrolling
    - SPA agreement with FDA in place
    - Data expected in Q1 2017
- LJPC-401 (synthetic 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
    - Plan to initiate pivotal study mid-2017
- LJPC-30Sa/b (gentamicin derivatives) for bacterial infections & rare genetic disorders
  - Plan to initiate Phase 1 study following positive pre-IND meeting
**Product Pipeline**

- **LJPC-501**
  - Indication: CRH
  - Status: Completed

- **LJPC-401**
  - Indication: HH, Thalassemia, SCD and MDS
  - Status: Underway

- **LJPC-30Sa & LJPC-30Sb**
  - Indication: Bacterial Infections, Rare Genetic Disorders
  - Status: Underway

- **Other R&D**
  - Indication: Various
  - Status: Underway

**IND**

- **CRH**
  - Status: Underway

**Phase 1**

- **CRH**
  - Start: Q4 2015

**Phase 2**

- **CRH**
  - Start: Mid 2017

**Phase 3**

- **CRH**
  - Start: Q1 2015
  - End: Q1 2017

**La Jolla Pharmaceutical**
Agenda

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• LJPC-401 (Synthetic Hepcidin) for Iron Overload
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• Financial Position
LJPC-501: Overview

- LJPC-501 is a proprietary formulation of angiotensin II, a naturally occurring regulator of blood pressure.
- Catecholamine-resistant hypotension (CRH) is an acute, life-threatening condition in which blood pressure drops to dangerously low levels and is unresponsive to current treatments.
- LJPC-501 has been shown to raise blood pressure in a pilot RPC clinical trial in CRH, as well as animal models of hypotension.
- Special Protocol Assessment (SPA) agreement reached with FDA:
  - Agreement reached that blood pressure can be the primary endpoint for approval.
- Phase 3 trial actively enrolling.
- Issued patent claims covering potential product to 2034.
Current therapeutic options for the treatment of acute hypotension only leverage the adrenal system and vasopressin system.
High Doses of Catecholamines Increase Mortality

- Catecholamines (i.e., norepinephrine, epinephrine and dopamine) cause cardiac toxicity, digital necrosis and metabolic complications leading to higher mortality\(^1\)

- Blocking the cardiac toxicity of norepinephrine improves outcome\(^2\)

\(^1\)Sviri et al, J. of Crit Care, 29;157-160, 2014

Norepinephrine Dose Decreases with Angiotensin II Surrogate Effect on Blood Pressure

- Randomized, placebo-controlled, double-blind pilot trial
- Primary efficacy endpoint:
  - Catecholamine dose sparing; surrogate for BP effect
- Published October 2014 in Critical Care
- All angiotensin II patients experienced an increase in BP
- Strong proof-of-concept that angiotensin II increases blood pressure in CRH

Source: Chawla et al, Critical Care, 18:534, 2014
ATHOS (Angiotensin II for the Treatment of High-Output Shock) 3 trial initiated in March 2015
- Randomized, placebo-controlled, double-blind Phase 3 trial
- Patient population: catecholamine-resistant, based on amount of catecholamine required
- Primary endpoint: blood pressure at 3 hours
- Secondary endpoints: change in CV SOFA* score; safety and tolerability of LJPC-501 in CRH patients
- Size: ~315 patients
- Projected results: Q1 2017

*Cardiovascular Sequential Organ Failure Assessment
Catecholamine-resistant hypotension is defined as requiring a norepinephrine-equivalent dose >20mg/day.
### LJPC-501: Attractive Pricing Potential

**Prices of Hospital-Based Drugs Reimbursed under DRGs**

<table>
<thead>
<tr>
<th>Drug Name</th>
<th>Indication</th>
<th>Cost per Treatment Course</th>
</tr>
</thead>
<tbody>
<tr>
<td>Panhematin</td>
<td>Acute intermittent porphyria</td>
<td>$20,356</td>
</tr>
<tr>
<td>Cubicin</td>
<td>Complicated blood infection</td>
<td>$11,917</td>
</tr>
<tr>
<td>Kalbitor</td>
<td>Hereditary angioedema attack</td>
<td>$11,910</td>
</tr>
<tr>
<td>Xigris</td>
<td>Severe sepsis</td>
<td>$11,188</td>
</tr>
<tr>
<td>Firazyr</td>
<td>Hereditary angioedema attack</td>
<td>$9,896</td>
</tr>
<tr>
<td>Berinert</td>
<td>Hereditary angioedema attack</td>
<td>$8,055</td>
</tr>
<tr>
<td>Activase</td>
<td>Heart attack or pulmonary embolism</td>
<td>$7,610</td>
</tr>
<tr>
<td>Activase</td>
<td>Stroke</td>
<td>$4,794</td>
</tr>
<tr>
<td>Cubicin</td>
<td>Uncomplicated blood infection</td>
<td>$4,086</td>
</tr>
<tr>
<td>Cubicin</td>
<td>Skin infection</td>
<td>$2,384</td>
</tr>
<tr>
<td>Precedex</td>
<td>Sedation</td>
<td>$1,481</td>
</tr>
</tbody>
</table>

**Difference in Hospital Costs for ICU Hypotensive Patients**

<table>
<thead>
<tr>
<th></th>
<th>Catecholamine-Resistant*</th>
<th>Catecholamine-Responsive</th>
</tr>
</thead>
<tbody>
<tr>
<td>Average total cost per patient</td>
<td>$60,668</td>
<td>$53,555</td>
</tr>
<tr>
<td>Difference</td>
<td>$7,113</td>
<td></td>
</tr>
</tbody>
</table>

*Catecholamine-resistant hypotension is defined as requiring a norepinephrine-equivalent dose >20mg/day

Robust (N=100) survey of physicians, 50% of which are P&T decision makers, shows strong interest based on evidence in primary endpoint alone.

### Endpoints Achieved

<table>
<thead>
<tr>
<th>Endpoints Achieved</th>
<th>Scenario 1</th>
<th>Scenario 2</th>
<th>Scenario 3</th>
</tr>
</thead>
<tbody>
<tr>
<td>Improvement in MAP</td>
<td>X</td>
<td>X</td>
<td>X</td>
</tr>
<tr>
<td>Improvement in urinary output</td>
<td></td>
<td>X</td>
<td>X</td>
</tr>
<tr>
<td>Improvement in mortality</td>
<td></td>
<td></td>
<td>X</td>
</tr>
</tbody>
</table>

### Physician Type

<table>
<thead>
<tr>
<th>Physician Type</th>
<th>Estimated Prescription Share of LJPC-501</th>
</tr>
</thead>
<tbody>
<tr>
<td>All (n=100)</td>
<td>48%</td>
</tr>
<tr>
<td>P&amp;T committee members (n=50)</td>
<td>47%</td>
</tr>
<tr>
<td>Non-P&amp;T committee members (n=50)</td>
<td>48%</td>
</tr>
</tbody>
</table>

Source: IMS Health survey, 2015
Summary of LJPC-501

- LJPC-501 is a proprietary formulation of angiotensin II, a naturally occurring regulator of blood pressure

- Catecholamine-resistant hypotension (CRH) is an acute, life-threatening condition in which blood pressure drops to dangerously low levels and is unresponsive to current treatments

- LJPC-501 has been shown to raise blood pressure in a pilot RPC clinical trial in CRH, as well as animal models of hypotension

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

- Phase 3 trial actively enrolling

- Issued patent claims covering potential product to 2034
Agenda

• Overview of LJPC
• LJPC-501 (Angiotensin II) for CRH
• LJPC-401 (Synthetic Hepcidin) for Iron Overload
  • LJPC-30Sa/b (Gentamicin Derivatives) for Bacterial Infections and Rare Genetic Disorders
• Financial Position
LJPC-401: Overview

- LJPC-401 is a novel formulation of synthetic 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.
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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GLP Toxicology Study in Rats

- **Serum Iron (ug/dL)**
  - Time Points: 0 hr, 4 hr, 24 hr, 48 hr, Day 15
  - **25 mg/kg**

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La Jolla Pharmaceutical
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

- 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

1 mg (n=3) | 5 mg (n=3) | 10 mg (n=3) | 20 mg (n=6)

Serum Iron Percent Change

-14.2 | -26.7 | -45.5 | -58.1

p=0.149 | p=0.304 | p=0.054 | p=0.001

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.
LTEC-401: Percent Change in Serum Iron
Baseline through Day 7 for 20 mg Dose

Change from baseline through day 7: -21%
Fifteen patients received a single dose ranging from 1 mg to 20 mg

- Patient subtypes treated: HH = 10; SCD = 3; and thalassemia = 2

Well tolerated with no dose-limiting toxicities

- Mild to moderate, transient and self-limiting injection-site reactions

Profound and durable reduction in serum iron observed

- Statistically significant dose response (p=0.008)
- A single 20 mg dose resulted in a 58% reduction at hour 8, with levels still not returning to baseline through day 7 (21% reduction)
- Iron effect consistent with that observed in preclinical models
• 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 mid-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
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LJPC-30Sa/b: Overview

- LJPC-30Sa and LJPC-30Sb are **purified derivatives of gentamicin**, which retain biologic activity but lack traditional kidney toxicity.
- Gentamicin: FDA-approved, standard-of-care for serious Gram-negative bacterial infections
  - Mixture of several distinct but closely related chemical entities
  - >3 million vials of gentamicin used in the U.S. in 2014
  - Use is limited due to kidney toxicity, which is believed to be associated only with certain constituent components.
- Two parallel development paths
  - Bacterial infections: aminoglycosides = $500+ million market in the U.S.*
  - Rare genetic disorders: gentamicin’s mechanism may be leveraged for rare genetic disorders; proof-of-concept data exists in cystic fibrosis.
- Recent positive FDA feedback on Phase 1 proposal.
- Multiple points of potential proprietary protection
  - Potential regulatory exclusivity and Orphan Drug Designation
  - Antibiotic exclusivity: 8+ years including Hatch-Waxman + GAIN (QIDP)
  - IP optioned from IU and UAB.

*Adjusted for branded pricing of comparable hospital antimicrobials
LJPC-30Sa/b: Potential for Improved Clinical Profile

Next-generation improved gentamicin derivative

1. Retain activity
2. Improve safety

Graphs showing inhibition/kill zone (mm) for B.subtilus and K.pneumonieae (kill) and K.pneumonieae (inhib) for placebo, next-generation gentamicin derivative, and gentamicin.

Graph showing serum creatinine mg/dL over time from Baseline to Day 6 for placebo, next-generation gentamicin derivative, and gentamicin.
Gentamicin induces errors in the translation of genes into proteins.

- **Bacteria**
  - Alters protein synthesis leading to cell death
- **Humans**
  - Alters protein synthesis to allow read through of stop-codon mutations
Three, independent studies suggest gentamicin helps read through stop-codon mutations in cystic fibrosis:

- Study of 10 mg/kg IV gentamicin over 15 days in Y122X mutations leads to:
  - Improvement in cystic fibrosis clinical scores ($p=0.007$)
  - Improvements seen as early as day 4
  - Improvement in lung function (FEV1) independent of an antimicrobial effect ($p=0.04$)
  - Improvement in sweat chloride secretion ($p=0.03$) and nasal potential difference ($p=0.04$)

Dose-dependent effect suggests LJPC-30Sa/b could allow chronic dosing with better efficacy and no kidney toxicity.

LJPC-30Sa/b: Current Antibiotic Usage

- Antibiotic opportunity = $500+ million per year in U.S.
  - Market could expand with a safer alternative
    - Increased duration of therapy, increased penetration, and/or new indications

U.S. Aminoglycoside Market (2014)

- Other large potential market opportunity in rare genetic disorders, such as cystic fibrosis

Source: Source Healthcare Analytics
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## Financial Position

### Condensed Balance Sheet Data

<table>
<thead>
<tr>
<th></th>
<th>As of June 30, 2016 (in millions)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Cash</td>
<td>$100.6</td>
</tr>
<tr>
<td>Total liabilities</td>
<td>$5.2</td>
</tr>
<tr>
<td>Total shareholders’ equity</td>
<td>$99.6</td>
</tr>
</tbody>
</table>

**Cash resources expected to fund Company into 2018**

### Fully Diluted, As-Converted Shares Outstanding*

<table>
<thead>
<tr>
<th></th>
<th>28,307,476</th>
</tr>
</thead>
</table>

*Includes common stock, preferred stock (as-converted) & outstanding equity awards as of June 30, 2016
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Thank You