Corporate Presentation

Developing Innovative Therapies for Patients Suffering from Life-threatening Diseases

NASDAQ: LJPC

January 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 Pharmaceutical’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 trials within projected time periods, the degree to which initial clinical study results are indicative of expected results for full studies and later studies, anticipated regulatory and patent exclusivity periods, the ability to manufacture clinical or commercial product successfully, the ability to resolve issues arising in the regulatory process, 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 is included in La Jolla Pharmaceutical’s periodic reports 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 (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 end of 2016

• LJPC-401 (hepcidin) for iron overload caused by diseases such as hereditary hemochromatosis (HH), thalassemia, sickle cell disease (SCD) and myelodysplasia (MDS)
  ▪ Phase 1 study ongoing; preliminary data encouraging

• LJPC-30Sa/b (gentamicin derivatives) for bacterial infections & rare genetic disorders
  ▪ Plan to initiate Phase 1 study following positive pre-IND meeting
### Product Pipeline

<table>
<thead>
<tr>
<th>Indication</th>
<th>IND</th>
<th>Phase 1</th>
<th>Phase 2</th>
<th>Phase 3</th>
</tr>
</thead>
<tbody>
<tr>
<td>LJPC-501: Angiotensin II</td>
<td>CRH</td>
<td>Q4 2015, Q1 2016</td>
<td>Q1 2015, Q4 2016</td>
<td></td>
</tr>
<tr>
<td>LJPC-401: Hepcidin</td>
<td>HH, Thalasemia, SCD and MDS</td>
<td>Q4 2015, Q1 2016</td>
<td></td>
<td></td>
</tr>
<tr>
<td>LJPC-30Sa &amp; LJPC-30Sb Gentamicin Derivatives</td>
<td>Bacterial Infections, Rare Genetic Disorders</td>
<td>Successful Pre-IND Meeting</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Other R&amp;D</td>
<td>Various</td>
<td></td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

- **Completed**
- **Underway**
- **Planned**
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• LJPC-501 (Angiotensin II) for CRH
• LJPC-401 (Hepcidin) for Iron Overload
• LJPC-30Sa/b (Gentamicin Derivatives) for Bacterial Infections and Rare Genetic Disorders
• 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: end of 2016

*Cardiovascular Sequential Organ Failure Assessment
**Catecholamine-resistant hypotension** is defined as requiring a norepinephrine-equivalent dose >20mg/day.


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**Total Annual Hospitalized U.S. Population with Severe Hypotension**: 555,479

**Applicable Severe Hypotension: 65%**: 361,684

**Catecholamine-Treated Patients: 92%**: 332,189

**Catecholamine-Resistant* Patients: 38%**: 126,232

* = LJPC-501 Target Market
**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>Panhematic</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

LJPC-501: Market Survey
Strong Interest Even among P&T Decision Makers

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

<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>

<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
• 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
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- Overview of LJPC
- LJPC-501 (Angiotensin II) for CRH
- LJPC-401 (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 hepcidin, a **naturally occurring** regulator of iron absorption and distribution

• Hereditary hemochromatosis (HH) is a disease characterized by a deficiency of hepcidin that results in excessive iron accumulation, which is toxic to vital organs such as the liver and heart
  - Most common genetic disease in Caucasians
  - Causes liver cirrhosis, liver cancer, heart disease and/or failure, dementia and diabetes

• Patients with thalassemia, sickle cell disease (SCD) and myelodysplasia (MDS) are treated with blood transfusions, resulting in acquired iron overload

• LJPC-401 has been shown to be effective at reducing serum iron in preclinical testing

• Phase 1 trial ongoing
  - Preliminary data encouraging

• Multiple points of potential proprietary protection
  - Potential regulatory exclusivity and Orphan Drug Designation (granted in EU)
  - IP licensed from INSERM
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
  - SQ formulation developed
  - Orphan Drug Designation granted (EU)
  - Phase 1 ongoing

GLP Toxicology Study in Rats

From: Blood Research Vol. 48, No. 1, p.10, March 2013
**OBJECTIVES**
Determine safety and tolerability of escalating, single doses of LJPC-401 in patients with conditions of iron overload; evaluate PK and effect on serum iron parameters

**PRIMARY ENDPOINT**
Safety and tolerability via review of: TEAEs, changes in clinical lab values, ECGs, vital sign and physical exam data

**SECONDARY ENDPOINTS**
PK & Iron Parameters including steady state plasma levels and half-life

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

- **Population**: Adult patients with refractory or hemolytic anemia of any type, or HH
- **Design**: Phase 1, open-label, dose-escalation, PK/PD/IM study
- **Iron studies**:
  - Serum iron, ferritin, transferrin
  - TIBC, UIBC
  - Non-transferrin bound iron
- **Duration of study**: single SC dose, 7-day observation
- **Study status**: preliminary patient data shows LJPC-401 to be safe and well-tolerated with a positive effect on serum iron parameters

**6 Cohorts Escalating Doses**

- Escalating dose cohorts
- 3 to 6 subjects each cohort

La Jolla Pharmaceutical
LJPC-401: Interim Phase 1 Data

- 6 patients enrolled and treated
- First 2 dose cohorts of potential 6
  - 1 mg and 5 mg
- No treatment emergent adverse events identified as dose limiting
- No adverse clinical lab changes noted
- Serum iron data available
LJPC-401: Interim Phase 1 Results – First Two Cohorts

Serum Iron % Change

Time Point

Cohort 1 (1 mg)
Cohort 2 (5 mg)
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, dementia 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

Inhibition/kill zone (mm)

<table>
<thead>
<tr>
<th></th>
<th>B. subtilis</th>
<th>K. pneumoniae (kill)</th>
<th>K. pneumoniae (inhib)</th>
</tr>
</thead>
<tbody>
<tr>
<td>placebo</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>next-generation gentamicin derivative</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>gentamicin</td>
<td></td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

Serum Creatinine mg/dL

- placebo
- next-generation gentamicin derivative
- 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
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
LJPC-30Sa/b: Clinical Proof-of-Concept in Cystic Fibrosis

- 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

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• Financial Position
# Financial Position

## Condensed Balance Sheet Data

<table>
<thead>
<tr>
<th></th>
<th>As of September 30, 2015 (in millions)</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Cash</strong></td>
<td>$135.1</td>
</tr>
<tr>
<td><strong>Total liabilities</strong></td>
<td>$4.3</td>
</tr>
<tr>
<td><strong>Total shareholders’ equity</strong></td>
<td>$133.6</td>
</tr>
</tbody>
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*Includes common stock, preferred stock (as-converted) & outstanding equity awards as of September 30, 2015

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

<table>
<thead>
<tr>
<th><strong>Fully Diluted, As-Converted Shares Outstanding</strong>*</th>
<th>27,304,103</th>
</tr>
</thead>
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