Transformative Therapies from Bench to Bedside
This presentation contains forward-looking statements and information that are based on the beliefs of the management of Capricor Therapeutics, Inc. (Capricor) as well as assumptions made by and information currently available to Capricor. All statements other than statements of historical fact included in this presentation are forward-looking statements, including but not limited to statements identified by the words “anticipates,” “believes,” “estimates,” and “expects” and similar expressions. Such forward-looking statements also include any expectation of or dates for commencement of clinical trials, IND filings, similar plans or projections and other matters that do not relate strictly to historical facts. These statements reflect Capricor’s current views with respect to future events, based on what we believe are reasonable assumptions; however, the statements are subject to a number of risks, uncertainties and assumptions. There are a number of important factors that could cause actual results or events to differ materially from those indicated by such forward-looking statements. More information about these and other risks that may impact our business are set forth in our Annual Report on Form 10-K for the year ended December 31, 2014, as filed with the Securities and Exchange Commission on March 16, 2015, in our Registration Statement on Form S-1, as filed with the Securities and Exchange Commission on March 6, 2015 and in our Form 10-Q for the quarter ended March 31, 2015, as filed with the Securities and Exchange Commission on May 13, 2015. Should one or more of these risks or uncertainties materialize, or should underlying assumptions prove incorrect, actual results may vary materially from those in the forward-looking statements. Further, Capricor’s management does not intend to update these forward-looking statements and information after the date of this presentation.
Clinical-stage biotechnology company with a diversified pipeline focusing on cardiovascular diseases including orphan indications

- Cardiac-derived stem cells (CDCs)
- Peptide therapy for heart failure (Cenderitide)
- Micro-RNA Platform (Exosomes)
## Capricor: Key Metrics

<table>
<thead>
<tr>
<th>Select Data (approximate)</th>
<th>As of 3.31.15</th>
</tr>
</thead>
<tbody>
<tr>
<td>Cash</td>
<td>$24.5M</td>
</tr>
<tr>
<td>(Includes restricted cash and marketable securities)</td>
<td></td>
</tr>
<tr>
<td>Publicly traded: NASDAQ</td>
<td>CAPR</td>
</tr>
<tr>
<td>52 week range</td>
<td>$3.05-$10.68</td>
</tr>
<tr>
<td>Shares outstanding</td>
<td>16.2M</td>
</tr>
<tr>
<td>Fully diluted shares outstanding</td>
<td>23M</td>
</tr>
<tr>
<td>Cash through</td>
<td>~Q3 2016</td>
</tr>
<tr>
<td>Non-dilutive capital funding to date</td>
<td>$39.5M</td>
</tr>
<tr>
<td>Exclusive Licenses</td>
<td>Johns Hopkins University, Cedars-Sinai Medical Center, Mayo Foundation for Medical Education and Research and The University of Rome</td>
</tr>
<tr>
<td>Headquarters</td>
<td>Los Angeles, CA</td>
</tr>
<tr>
<td>Employees</td>
<td>32</td>
</tr>
</tbody>
</table>
CELL THERAPY TECHNOLOGY
Lead Product: CDCs (CAP-1002)

Cardiac Tissue → Explants → Explant-derived cells (EDCs) → Cardiospheres (CSps) → Cardiosphere-derived cells (CDCs)

### Features

<table>
<thead>
<tr>
<th>Cell Type</th>
<th>Human cardiac derived stem cell</th>
</tr>
</thead>
<tbody>
<tr>
<td>Characteristics</td>
<td>Panel of cellular markers and secreted factors</td>
</tr>
<tr>
<td>Clinical Trials</td>
<td><strong>CADUCEUS</strong> – completed autologous Phase I (n=25), showed regeneration in CDC-treated post-MI patients (sponsored by CSMC)</td>
</tr>
<tr>
<td></td>
<td><strong>ALLSTAR</strong> – completed Phase I (n=14), now in Phase II with allogeneic CDCs in post-MI patients</td>
</tr>
<tr>
<td></td>
<td><strong>DYNAMIC</strong> – ongoing study of allogeneic CDCs in heart failure patients</td>
</tr>
<tr>
<td>MOA</td>
<td>Largely paracrine:</td>
</tr>
<tr>
<td></td>
<td>▪ <em>Prevent cardiomyocyte apoptosis</em> (programmed cell death)</td>
</tr>
<tr>
<td></td>
<td>▪ <em>Promote cardiomyocyte proliferation and angiogenesis</em> (cell growth and blood vessel formation)</td>
</tr>
<tr>
<td></td>
<td>▪ <em>Attract endogenous stem cells</em></td>
</tr>
<tr>
<td></td>
<td>▪ <em>Anti-fibrotic</em> (anti-scarring)</td>
</tr>
</tbody>
</table>
CADUCEUS - Positive First-in-Man Data

Intracoronary cardiosphere-derived cells for heart regeneration after myocardial infarction (CADUCEUS): a prospective, randomised phase 1 trial


- Published in Lancet, 2012
- Autologous CDCs - 25M cells
- Patients with reduced ejection fraction following MI
- Sponsored by Cedars-Sinai with Johns Hopkins
- Intracoronary delivery
- 25 patients
  - 17 CDCs
  - 8 Controls
CDC Therapy Reduced Scar Size & Increased Healthy Heart Muscle in the CADUCEUS study

CDC patients had a significant reduction in infarct size. We hypothesize improvement in clinical outcomes.
Advantages of Allogeneic CDCs

- Donors pre-screened by organ procurement organizations
- Single donor permits manufacturing of many doses
- Freezing permits off-the-shelf product availability
- COGS reduced by 10X compared to autologous cells

Patient Dose of CAP-1002
## CDCs: Clinical Development

<table>
<thead>
<tr>
<th>Status</th>
<th>Indication</th>
<th>Clinical Development</th>
<th>Development</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>ALLSTAR Clinical Trial</strong></td>
<td>Post myocardial infarction (30 days – 1 year after MI)</td>
<td>Phase II</td>
<td>Collaboration with Janssen Biotech (J&amp;J), funded in part by CIRM</td>
</tr>
<tr>
<td><strong>DYNAMIC Clinical Trial</strong></td>
<td>NYHA Class III or ambulatory Class IV heart failure</td>
<td>14 patient trial</td>
<td>Funded in part by the NIH</td>
</tr>
<tr>
<td><strong>HOPE Clinical Trial</strong></td>
<td>Duchenne muscular dystrophy-related cardiomyopathy</td>
<td>IND submitted</td>
<td>Orphan designation granted</td>
</tr>
</tbody>
</table>

**Data**
- **Enrollment complete**: ~Q4 2015
- **Data anticipated**: ~Q4 2016-Q1 2017
- **Targeting trial initiation**: 2H 2015
ALLSTAR Phase I – 12 month MRI Analysis

- **ALLSTAR Phase I**
  - Met safety endpoint (1 month)
  - No control group
- Preliminary 12 month MRI analysis on Phase II equivalent population (defined by tissue type compatibility)
  - **Ejection fraction improved by 5.2%**
  - **Relative reduction in scar size of 20.7%**
  - Measurements of viable mass and regional function also showed quantifiable improvements
Duchenne Muscular Dystrophy
Duchenne Muscular Dystrophy

- Prevalence: 1 in 3,500 male births worldwide
  - ~20,000 male children affected in the US (~275,000 worldwide)
  - DMD is fatal; a majority of deaths occur due to cardiomyopathy
- Compelling preclinical data presented at AHA in November 2014
  - IND submitted 1H 2015
  - HOPE clinical trial planned for 2H 2015
- Intent to complement other dystrophin-correcting therapies for skeletal muscle
- Orphan disease – presents potential billion dollar market opportunity

Rationale for CDCs Use in DMD

CDCs
- Anti-oxidative
- Anti-inflammatory
- Anti-apoptotic
- Anti-remodeling
- Regenerative

DMD pathophysiology
- Oxidative/Nitrosative stress
- Inflammation
- Apoptosis
- Remodeling

CDC administration may be beneficial in DMD cardiomyopathy
Tested in mdx mouse model
**mdx Mouse Model of Duchenne Muscular Dystrophy**

- Mimics the target indication, DMD
- Aged to the point at which cardiac dysfunction becomes evident: ≥10 months old

Reference: Cedars-Sinai Heart Institute
Global Cardiac Function is Improved in *mdx* Mice

**Single Dose**

- Mdx+CDC
- Mdx+Vehicle
- CTL(WT)

**Repeat Dosing**

- Mdx+CDC
- Mdx+Vehicle

*EF (%) vs. Time*

- **Baseline**
- **Wk3**
- **M2**
- **M3**

**Reference:** Cedars-Sinai Heart Institute

**Presented at AHA - November 2014, Chicago, IL**

**Presented at ISEV - April 2015, Washington DC**
CDCs Increased Maximal Exercise Capacity

Distance (m)

**CTL**

**Mdx+CDC**

**Mdx+Vehicle**

n = 6-11

Presented at AHA - November 2014, Chicago, IL
Reference: Cedars-Sinai Heart Institute
Duchenne Cardiomyopathy and CDCs

- Injection of CDCs into \textit{mdx} hearts
  - Improves global function
  - Decreases fibrosis
  - Improves exercise capacity
  - Exerts potent anti-oxidant effects
  - Reverses abnormalities in mitochondrial abundance, structure and function
  - Increases cardiomyocyte proliferation and activation/recruitment of endogenous repair

Reference: Cedars-Sinai Heart Institute

Presented at AHA - November 2014, Chicago, IL
Natriuretic Peptide Technology
Cenderitide: A Unique Protein Drug

- Developed by scientists at the Mayo Clinic and derived from the venom of the green mamba snake

Cenderitide:
- Cardiac unloading
- Renal function preserved
- Aldosterone suppressing
- Anti-fibrotic, apoptotic, and hypertrophic

- 270 patients with acute decompensated heart failure have been treated
Continuous Subcutaneous Infusion Using the Insulet Omnipod® Technology

- Current pump delivery systems are robust, simple, and well tolerated
  - In use by more than 300,000 patients worldwide
  - Pump use is simple for all types of patients, not just diabetics
- Continuous delivery is ideal for worry-free dosing throughout the day and night

Sample shown: Omnipod®
Cenderitide’s Development

The Opportunity

1st Phase II clinical trial complete
Cenderitide for Outpatient and Ambulatory Heart Failure

Target Indication

Prevention of re-hospitalization in patients with a recent acute heart failure admission as well as other potential indications

Phase II Trial enrollment complete

- 14 patients treated
- Patients with stable chronic heart failure
- Trial assessing the safety and tolerability, pharmacokinetics profiles, and pharmacodynamic response to increasing dose levels of Cenderitide

- Early results suggest tolerability and physiologic effect
- Will announce further plans in late 2015, early 2016
Exosomes: Micro-RNA Platform Technology
Exosomes: Micro-RNA Platform Technology

- Nanometer-sized lipid-bilayer vesicles
- Rich in miRNAs
- Present in virtually all body fluids
- Released by nearly all cell types
- Potential for broad therapeutic applicability
CDCs Release Bioactive Exosomes that Recapitulate their Therapeutic Effects in Cardiac Injury

Ibrahim et al., Stem Cell Reports, 2014
CDCs (lines 155 or 220) or inert
NHDFs (dermal fibroblasts)
Culture in SF Media
(15 days)

Collect Conditioned Media

Exosome Isolation
- Precipitate by ExoQuick (in vivo studies)
- Isolate by ultracentrifugation (in vitro studies in iPS cells)

Exosomal Markers
CD63 (~53kDa)
CD9 (~28kDa)
CD81 (~26kDa)
HSP70 (~70kDa)

CDC Marker
CD105 (~80kDa)

Nanosight

Presented at ISEV - April 2015, Washington DC
Reference: Cedars-Sinai Heart Institute
Advantages for Exosomes for Tissue Repair

- Novel delivery vehicle reproducing the beneficial effects of living cells
- Natural capacity to protect their bioactive cargo
- Potential for simple manufacturing protocols
- Reduced immunogenicity
Capricor Investment Opportunity

- Ongoing Phase II clinical trials with 2 product candidates
  - IND for Duchenne Muscular Dystrophy Indication under review

- **New cell free regenerative medicine platform technology**

- Operational leverage - $39.5M of non-dilutive capital since inception

- License Option and Development Collaboration with Johnson and Johnson

- Deep scientific and clinical expertise in target markets

- ~16-18 months cash on hand
2015 Targeted Milestones

- **1H 2015**
  - Complete DYNAMIC trial enrollment – **completed**
  - Complete Cenderitide Phase II enrollment – **completed**
  - Receive Orphan Designation for DMD – **completed**
  - Submit IND for DMD-associated cardiomyopathy – **completed**

- **2H 2015**
  - Initiate HOPE-DUCHENNE trial
  - Report initial DYNAMIC results
  - Report initial Cenderitide results
  - Announce development program for natriuretic peptides
  - Announce 1st indication for exosomes
Senior Management & Board of Directors

Senior Management

- **Chief Executive Officer**
  Linda Marbán, Ph.D. (Founder)
- **Scientific Advisory Board Chairman**
  Dr. Eduardo Marbán (Founder, JHU, Cedars-Sinai)
- **VP of Research and Development**
  Rachel Smith, Ph.D. (Johns Hopkins)
- **EVP & General Counsel**
  Karen Krasney, J.D. (Biosensors)
- **VP of Medical Affairs**
  Andrew Hamer, M.D. (Chairman – New Zealand Cardiac Network)
- **VP of Clinical Operations**
  Shane Smith (Nektar, Genentech)

Board of Directors

- **Executive Chairman**
  Frank Litvack, M.D. (ConorMed)
- **Scientific Advisory Board Chairman**
  Dr. Eduardo Marbán, Ph.D.
- **VP of Research and Development**
  Dave Musket (ProMed Partners)
- **VP of Medical Affairs**
  Earl M. (Duke) Collier, Jr. (Genzyme)
- **VP of Clinical Operations**
  George W. Dunbar, Jr. (Aastrom)
- **VP of Clinical Operations**
  Joshua Kazam (Kite, Two-River)
- **VP of Medical Affairs**
  Gregory Schafer (Aduro, Onyx)
- **VP of Clinical Operations**
  Louis Manzo (Investor)
- **VP of Clinical Operations**
  Louis J. Grasmick (Investor)