
Jefferies Healthcare Conference
Roger Tung, Ph.D., President and CEO
June 3, 2015
This presentation contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. These forward-looking statements involve substantial risks and uncertainties. All statements, other than statements of historical facts, contained in this presentation, including statements regarding our strategy, future operations, future financial position, future revenue, projected costs, prospects, plans and objectives of management and expected market growth, are forward-looking statements. The words “anticipate,” “believe,” “continue,” “could,” “estimate,” “expect,” “intend,” “may,” “plan,” “potential,” “predict,” “project,” “should,” “target,” “would,” and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words.

We may not actually achieve the plans, intentions or expectations disclosed in our forward-looking statements, and you should not place undue reliance on our forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in the forward-looking statements we make as a result of various important factors, including the factors discussed in the "Risk Factors" section of our most recent Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission and in other filings that we make with the Securities and Exchange Commission. The forward-looking statements contained in this presentation reflect our current views with respect to future events, and we assume no obligation to update any forward-looking statements except as required by applicable law.
Investment Highlights

**Leader in Deuterium Chemistry**

- **Robust Pipeline**: Innovative approach to develop new medicines for patients
- **Validating Partnerships**: Avanir/Otsuka, Celgene and Jazz Pharmaceuticals
- **Leverageable Technology**: Platform enables efficient, cost-effective generation of Phase 1 proof-of-concept clinical data
- **Broad IP**: >60 Issued US patents including composition-of-matter coverage of clinical candidates
- **Well Capitalized**: $113M as of March 31, 2015
Deuterium: Powerful Tool for Pharmaceutical R&D

- Increased chemical bond stability provides potential for unique properties:
  - No material change to compound’s biochemical potency or selectivity
  - May result in improved effectiveness, safety or tolerability
- Deuterated analogs of approved drugs amenable to expedited path
## Robust Pipeline of Clinical Candidates

<table>
<thead>
<tr>
<th>Product Candidate</th>
<th>Lead Indication(s)</th>
<th>Preclinical</th>
<th>Phase I</th>
<th>Phase 2</th>
<th>Phase 3</th>
<th>Worldwide Rights</th>
</tr>
</thead>
<tbody>
<tr>
<td>AVP-786</td>
<td>Alzheimer’s Agitation</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td></td>
<td>Major Depressive Disorder</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>CTP-499</td>
<td>Diabetic Nephropathy</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>CTP-656</td>
<td>Cystic Fibrosis</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>JZP-386</td>
<td>Narcolepsy</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>CTP-730</td>
<td>Inflammatory Diseases</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>CTP-354</td>
<td>Spasticity associated with SCI and MS</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
</tbody>
</table>
AVP-786: Potential Late-Stage Investigational Compound

- Next-generation neurological and psychiatric agent advancing into late stage clinical evaluation

- Potential to leverage and extend known efficacy of dextromethorphan (Nuedexta®), reduce quinidine exposure

- Broad development opportunities
  - Alzheimer's Agitation
  - Major Depressive Disorders
  - Additional neurological and psychiatric indications

- FDA has agreed to an expedited development pathway, similar to 505(b)(2)

- Multiple issued US patents to 2028+, broad overseas IP

- A key asset in $3.5B acquisition of Avanir by Otsuka
AVP-786: Near Term Opportunity to Advance into Phase 3

- Lead indication: agitation in patients with Alzheimer’s disease
- Estimated 5.1M Americans have Alzheimer’s disease
  - Approximately 50% of patients experience agitation
  - Unmet medical need with no currently approved therapies

- Clinical development
  - Phase 3 in Alzheimer’s agitation expected to begin Q3 2015
  - Phase 2 in resistant major depressive disorder expected completion Q2 2016

- Concert: mid-single to low-double digit worldwide royalties
  - Potential $170MM upfront and milestones to Concert; $6 million received
  - Avanir responsible for all development and sales expenses
AVP-786: Planned Phase 3 Design

- Two multicenter, randomized, double-blind, placebo-controlled efficacy studies
  - Completers eligible for long term safety and efficacy extension study
- Assess efficacy, safety and tolerability for the treatment of agitation in patients with dementia of the Alzheimer's type
  - Primary outcome measure: NPI Agitation/Aggression Domain
  - Time Frame: 12 weeks
- Two doses of AVP-786 vs. placebo twice-daily
- Estimated enrollment: 325 and 380 patients
- Expected study start date: September 2015

Source: ClinicalTrials.gov
CTP-656: Novel Disease-Modifying Agent for Cystic Fibrosis

Expanding patient options

- Deuterated analog of ivacaftor
  - Novel, proprietary CFTR potentiator

- Potential use as monotherapy and in combination with other CFTR modulators
  - Potentiators are likely to be part of future combination CF treatments

- Preclinical data indicate greater metabolic stability than ivacaftor

- Potential for rapid development
Deuteration Improves Metabolic Stability to CYP3A4

Metabolic Stability in Human CYP3A4 Supersomes

<table>
<thead>
<tr>
<th>Compound</th>
<th>$t_{1/2}$ (min) Ave ± SD (% change)</th>
</tr>
</thead>
<tbody>
<tr>
<td>ivacaftor</td>
<td>5.5 ± 0.1 (NC)</td>
</tr>
<tr>
<td>D9-ivacaftor</td>
<td>8.1 ± 0.2 (+47%)</td>
</tr>
<tr>
<td>D18-ivacaftor</td>
<td>8.1 ± 0.0 (+47%)</td>
</tr>
</tbody>
</table>

1 uM compound, 50 pmol/mL supersomes
Deuterium Modification Improves Ivacaftor Oral PK *In Vivo*

**Rat Oral Pharmacokinetics**

<table>
<thead>
<tr>
<th>Compound</th>
<th>C\textsubscript{max} (ng/mL)</th>
<th>AUC\textsubscript{0-24 hr} (hr*ng/mL)</th>
<th>C\textsubscript{24 hr} (ng/mL)</th>
<th>t\textsubscript{1/2} (hr)</th>
<th>Dog PK Parameters</th>
</tr>
</thead>
<tbody>
<tr>
<td>Ivacaftor</td>
<td>1913</td>
<td>22177</td>
<td>346</td>
<td>8.0±2.0</td>
<td>2255</td>
</tr>
<tr>
<td>D9-ivacaftor</td>
<td>1970</td>
<td>24260</td>
<td>413</td>
<td>8.5±0.2</td>
<td>3463 +62%</td>
</tr>
<tr>
<td>D18-ivacaftor</td>
<td>2460 +29%</td>
<td>31556 +42%</td>
<td>623 +80%</td>
<td>9.7±0.4 +21%</td>
<td>3030</td>
</tr>
</tbody>
</table>

**Dog Oral Pharmacokinetics**

- Increased half life may enable once-daily dosing, ameliorate drug-drug interactions
CTP-656: Phase 1 Clinical Evaluation Underway

- Phase 1 program
  - Planned for 45 healthy volunteers
- Candidate selection completed
  - Single-dose crossover
- Single ascending dose study and multiple ascending dose study planned
  - Multiple ascending dose study initiation expected H2 2015
- Phase 1 top-line data expected late 2015/early 2016
CTP-499: Partnering Opportunity for Diabetic Nephropathy

Potential first-in-class treatment to slow progression to kidney failure

- Multi-subtype selective anti-fibrotic PDE inhibitor
  - CTP-499 expected to be additive to standard-of-care
- Promising 48-week study results
  - Appears to protect renal function
  - Generally well tolerated
  - Biomarkers suggest potential broad anti-fibrotic effect
- Successful End-of-Phase 2 FDA meeting
- Next step: negotiate Special Protocol Assessment (SPA)
CTP-499 Benefit Greater in Patients with Higher Baseline UACR

- Higher UACR is known to correlate with more rapid disease progression
- FDA supportive of enriched population in Phase 3 development

Results presented at NKF Spring Clinical Meeting April 2014
Value Creating Collaborations

Three active, prominent collaborations

- Combined milestone potential of $1.6 billion plus worldwide royalties for each product (mid-single to low-double digit)
  - $45 million realized to date
- All programs actively advancing in development
- Leverages and extends known efficacy of non-deuterated compound
- Phase 1 clinical testing considered proof-of-concept
- Potential for expedited development pathway
JZP-386: Potential Treatment for Narcolepsy

- Next generation neurological agent
  - Potential to leverage and extend known efficacy of sodium oxybate, the active ingredient in Xyrem®
    - 2015 Xyrem revenue guidance: $950-$970 million

- Phase 1 PK/PD demonstrated improved profile compared to Xyrem
  - Higher serum concentrations and increased PD effects
    - Similar safety profile
    - No increased variability

- Next steps: Jazz Pharmaceuticals intends to explore formulation options to further enhance positive effects of JZP-386

- $117M upfront/milestones
- $4M received
- Mid-single to low double digit royalties
CTP-730: High Value Collaboration with Celgene

- Next generation anti-inflammatory agent
- Phase 1 clinical program completion expected in 2015
  - Single and multiple ascending dose studies to assess safety and pharmacokinetics
- Potential to realize $8 million milestone in 2H 2015
- Collaboration has potential to include 3 additional compounds

- $1.4B upfront/milestones
- $35M received
- Mid-single to low-double digit royalties
Financial Results

**Strong Financial Position**

<table>
<thead>
<tr>
<th>March 31, 2015</th>
<th></th>
</tr>
</thead>
<tbody>
<tr>
<td>Cash and Investments*</td>
<td>$113 M</td>
</tr>
<tr>
<td>Revenue</td>
<td>$1.3 M</td>
</tr>
<tr>
<td>Operating Expenses</td>
<td>$10.2 M</td>
</tr>
<tr>
<td>Debt balance</td>
<td>$5.1 M</td>
</tr>
<tr>
<td>Shares outstanding**</td>
<td>21.7 M</td>
</tr>
</tbody>
</table>

*Does not include any potential payment that may be due to the company from Teva's acquisition of Auspex

**As of May 5, 2015**
### Multiple Potential Value Creating Events in 2015

#### 2015 Goals

<table>
<thead>
<tr>
<th><strong>AVP-786 (Avanir)</strong></th>
<th><strong>JZP-386 (Jazz Pharmaceuticals)</strong></th>
</tr>
</thead>
<tbody>
<tr>
<td>- Conduct end of Phase 2 FDA meeting</td>
<td>- Conduct Phase 1 trial</td>
</tr>
<tr>
<td>- Initiate Phase 3 Alzheimer’s Agitation</td>
<td>- Determine next steps Q2 2015</td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th><strong>CTP-499</strong></th>
<th><strong>CTP-730 (Celgene)</strong></th>
</tr>
</thead>
<tbody>
<tr>
<td>- Negotiate Special Protocol Assessment</td>
<td>- Complete multiple ascending dose Phase 1 trial H2 2015</td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th><strong>CTP-354</strong></th>
<th><strong>CTP-656 (D-Ivacaftor)</strong></th>
</tr>
</thead>
<tbody>
<tr>
<td>- Conduct non-clinical testing</td>
<td>- Initiate clinical evaluation</td>
</tr>
<tr>
<td>- Determine development next steps</td>
<td></td>
</tr>
</tbody>
</table>

- Identify additional lead candidate