First self-administered antibody therapy for HIV in late-stage clinical trials

LD Micro Invitational
June 6, 2017
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Lead product **PRO 140** is fully humanized monoclonal antibody

- Binds to CCR5 which disrupts the life cycle of HIV
- Two Phase 2b/3 clinical trials (one pivotal) and one label expansion
- World’s first self-injectable antibody in late-stage development
- Promising animal studies in cancer and other autoimmune diseases
- Patient enrollment ongoing in Phase 2 Graft versus Host Disease (GvHD)
$20 billion U.S. market for HIV therapies

Two Phase 2b/3 trials with PRO 140 for HIV therapy
- **Addition to HAART** - Phase 2b/3 Combination Trial - Pivotal
  - Study enrollment to be completed in 2Q17
  - Efficacy results announced shortly following enrollment completion
- **Alternative to HAART** - Phase 2b/3 Monotherapy Trial
  - Study completion expected in 2018

Multiple partnership opportunities with PRO 140 in immunologic indications
- GvHD – Phase 2 clinical trial underway
- Cancer – Positive animal study
- Multiple Sclerosis - Positive animal study
- Other immunologic indications being explored
HIV is No Longer a Death Sentence, BUT...

- Historically, the first goal in treating HIV was to stop this disease from advancing to AIDS and killing patients.

- Problem today is different, but very serious...
  - Transmission of the disease
  - Problems with HAART
    - Toxicity
    - Side effects
    - Compliance
    - Resistance

- Viral Load (VL) is number of HIV particles per milliliter of blood.

- If VL is <40 copies/mL of blood, Transmission Rate is ~ zero.
~3 years ago ~25% of HIV patients in U.S. have suppressed VL

Now ~30-35% HIV patients have suppressed VL

~45,000-50,000 new patients are infected with HIV annually...

This number has not changed for more than 15 years . . .

WHY?
HIV is no Longer a Deadly Disease, BUT...

- HAART (Highly Active Antiretroviral Therapy) is current standard of care
  - Comprised of 3 drugs from 2 classes
  - Works by interfering with the virus life cycle

- Multiple problems with HAART
  - Side effects
  - Toxicity
  - Compliance
  - Resistance
PRO 140 Advantages Over HAART

**PRO 140**

- No serious side effects and no serious adverse events (SAEs) observed in over 200 patients in 7 clinical trials
- Negligible toxicity
- No drug resistance in patients on monotherapy for >32 months
- Weekly, easy, subcutaneous injections

**HAART**

- Side Effects
  - Ranges from mild to severe

- Toxicity
  - Problems with short- and long-term toxicity

- Resistance
  - 76% of patients develop resistance

- Adherence
  - Daily lifeline dosing with only 30% of patients achieving complete VL supression
**PRO 140 – A Humanized Monoclonal Antibody**

**HIV Entry Receptor is CCR5**

- Humanized monoclonal antibody
- Binds to CCR5 co-receptor on white blood cells
- Blocks HIV entry into white blood cells

Diagrams:
- HIV life cycle: Entry, Reverse Transcription, Integration, Protease Activity, Budding
- Cell with HIV particles
- CCR5 and CD4 on the cell surface, PRO 140 blocking interaction

(OTCQB: CYDY) www.cytodyn.com
# PRO 140 for HIV: Clinical Trial Overview

<table>
<thead>
<tr>
<th>Study</th>
<th># patients</th>
<th>Design / Findings</th>
<th>Status</th>
<th>Stage</th>
</tr>
</thead>
<tbody>
<tr>
<td>2 Phase 1 studies</td>
<td>54</td>
<td>Healthy patients, no safety concerns</td>
<td>Complete</td>
<td></td>
</tr>
<tr>
<td>1302 IV Phase 1 study</td>
<td>39</td>
<td>Intravenous, single-dose VL reduction for 3 weeks</td>
<td>Complete</td>
<td></td>
</tr>
<tr>
<td>2301 IV Phase 2 study</td>
<td>31</td>
<td>Intravenous, single-dose VL reduction for 3 weeks</td>
<td>Complete</td>
<td></td>
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<tr>
<td>2101 SC Phase 2 study</td>
<td>44</td>
<td>Subcutaneous, long-acting, self-administered, proof-of-concept shown</td>
<td>Complete</td>
<td></td>
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<tr>
<td>CD01 Phase 2b</td>
<td>40</td>
<td>12-week drug-substitution monotherapy</td>
<td>Complete Jan. 2015</td>
<td></td>
</tr>
<tr>
<td></td>
<td></td>
<td>Long-term monotherapy extension: 14 patients with VL suppression at 12 weeks</td>
<td>Ongoing</td>
<td></td>
</tr>
<tr>
<td>CD02 Phase 2b/3 Pivotal-Fastest path to approval</td>
<td>30</td>
<td>Combination therapy in HAART failures, 1 week efficacy + 24 weeks safety and durability</td>
<td>Injection of 1st patient Oct. 2015</td>
<td></td>
</tr>
<tr>
<td>CD03 Phase 2b/3 Investigative Trial – Largest market size</td>
<td>300</td>
<td>Long-term monotherapy</td>
<td>Injection of 1st patients Dec. 2016</td>
<td></td>
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## PRO 140 Clinical and Regulatory Strategy in HIV

### Phase 2b/3 Pivotal Combination Therapy
- **1<sup>st</sup> patient in new protocol injected in October 2016**
- Several patients finished trial and are in a rollover study to continue with PRO 140
- Clinical trial for first approval
- Patient enrollment expected to be completed in 2Q17

### Phase 2b/3 Investigative Monotherapy
- **1<sup>st</sup> patients injected in December 2016**
- 48-week investigative trial
- Supported by long-term viral efficacy in Phase 2b extension study
- Provides safety data for pivotal combination study
HIV Patients on PRO 140 Monotherapy

Patient testimony from Phase 2b monotherapy extension trial

*Lower Toxicity and fewer Side Effects than HAART with completely suppressed VL*
- Target is CCR5 – receptor for chemokine responsible for immune cell trafficking, T-cell migration to sites of inflammation
- Trigger CCR5 immunologic activity
- CCR5 triggering plays crucial role in inflammatory responses
  - Regulation of cancer cell killing
  - Transplantation rejection reactions
  - Autoimmunity
  - Chronic inflammation
- Transplantation reaction, GvHD, is 1st immunologic indication (non-HIV)
  - 1st patient dosed in Phase 2 trial
## PRO 140 Important Milestone 2017/2018

<table>
<thead>
<tr>
<th>Milestones</th>
<th>Target Dates</th>
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<tbody>
<tr>
<td>HIV Fast Track Designation</td>
<td>Granted</td>
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<tr>
<td>Breakthrough Therapy Designation (application submitted)</td>
<td>2017</td>
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<tr>
<td>Primary Endpoint</td>
<td>2017</td>
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<tr>
<td>BLA (Biologic License Application) Submission</td>
<td>2018</td>
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<tr>
<td>Possible approval timeline</td>
<td>2018 w/BTD</td>
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<tr>
<td>Published studies – 2 in HIV; 2 in Inflammation</td>
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<tr>
<td>New Patents</td>
<td>2017</td>
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<tr>
<td>Conference Presentations at CROI and ASM Microbe</td>
<td>2017</td>
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<tr>
<td>Monotherapy Phase 2/3 study complete</td>
<td>2018</td>
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