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CTD Holdings, Inc. (OTCQB “CTDH”), a clinical-stage biotechnology company, is developing cyclodextrin-based products for the treatment of diseases with unmet medical need.

- Lead drug candidate, Trappsol® Cyclo™, being developed as treatment for orphan indication, Niemann-Pick Disease Type C (“NPC”), an orphan disease.
  - On market approval of Trappsol® Cyclo™ for NPC, CTD will apply for Priority Review Voucher
- Founded in 1990
- Corporate headquarters in Alachua, FL
- 6 employees
- Cash of $2.3M provides runway to Q'3 2017
- Market Cap: approx. $36.5M as of 4/21/17
### Investment Highlights

**Trappsol® Cyclo™**

*has potential to address significant unmet medical need in NPC*

- Phase II in Europe and Phase I in U.S. initiated in Q1 ’17
- Compassionate Use Program ongoing in multiple countries
  - Compelling efficacy data generated
- Granted orphan drug designation by the U.S. Food and Drug Administration and European Union Medical Agency

### Large Market Opportunity

- NPC represents significant $400M+ annual addressable market

### Additional Indications

- Multiple potential additional indications in which Cyclodextrins may be effective
  - Atherosclerosis
  - Acute Viral Infections

### Exclusivity & Fast Track

- Clear orphan drug strategy

### Right leadership to execute the plan

- Seasoned management team with broad expertise

### Uplisting

- Expected up listing in 2017
<table>
<thead>
<tr>
<th>Name</th>
<th>Position</th>
<th>Logos</th>
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<tbody>
<tr>
<td><strong>N. Scott Fine</strong></td>
<td>Chairman &amp; CEO</td>
<td><img src="green_mountain_coffee.png" alt="Green Mountain Coffee" /></td>
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<tr>
<td><strong>Dr. Sharon H. Hrynkow</strong></td>
<td>SVP, Medical Affairs</td>
<td><img src="university_of_minnesota.png" alt="University of Minnesota" /></td>
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<tr>
<td><strong>Dr. Jeffrey L. Tate</strong></td>
<td>COO, CSO, Director</td>
<td><img src="cdc.png" alt="Centers for Disease Control and Prevention" /></td>
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<td><strong>C.E. “Rick” Strattan</strong></td>
<td>Director &amp; Founder</td>
<td><img src="nih.png" alt="NIH" /></td>
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<tr>
<td><strong>Markus Sieger</strong></td>
<td>Lead Director</td>
<td><img src="usps.png" alt="United States Postal Service" /></td>
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<tr>
<td><strong>Judge Joseph J. Farnan</strong></td>
<td>Director</td>
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<tr>
<td><strong>William S. Shanahan</strong></td>
<td>Director</td>
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<tr>
<td><strong>F. Patrick Ostronic</strong></td>
<td>Director</td>
<td><img src="colgate_palmolive.png" alt="Colgate-Palmolive" /></td>
</tr>
<tr>
<td><strong>CTD Holdings, Inc.</strong></td>
<td></td>
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Niemann-Pick Disease Type C: Overview

An autosomal recessive lysosomal storage disease

1/80,000 - 1/120,000 Live Birth Incidences

The disease is associated with accumulation of cholesterol in late endosomes and lysosomes due to loss of normal function of the NPC protein (NPC1 in 95% of cases, NPC2 in 5% of cases).

NPC is highly variable, presenting usually in young children, who often do not survive into adulthood, and also has a later onset presentation which leads to longer term disability. The disease is difficult to diagnose and, therefore, under-diagnosed.

NPC damage can be found in the brain, liver, and other body tissues. Depending on severity of the disease, cognitive impairment, movement disorders, swallowing, lung, liver and other normal functions are affected.
Overview of Functional Defects in NPC Disease-Affected Cell Types
(Maetzel et al., Genetic and Chemical Correction of Cholesterol Accumulation and Impaired Autophagy in Hepatic and Neural Cells Derived from Niemann-Pick Type C Patient-Specific iPS Cells. Stem Cell Reports 2014; 2: 866–880)

Under normal conditions NPC-1, located on the late endosomal/lysosomal (LE/L) compartments, regulates cholesterol efflux. However, in NPC mutations in the NPC-1 gene on both alleles lead to accumulation of cholesterol in the LE/L compartments by inhibiting its efflux, and to a block in autophagic flux, which causes accumulation of autophagosomes and autophagy substrate due to impaired formation of amphisomes.

As described, HPβCD-mediated cholesterol release - independent of the function of both NPC1 and NPC2 proteins – has the potential to bring significant benefit to patients with NPC.
<table>
<thead>
<tr>
<th>Existing NPC Cases</th>
<th>Number of live births</th>
<th>Incidence</th>
<th>New Cases / Year</th>
<th>Annual Diagnosis Rate</th>
<th>Patient Penetration</th>
<th>Treatable Cases/Year</th>
<th>Annual IV Orphan Drug Price (Avg.)</th>
</tr>
</thead>
<tbody>
<tr>
<td>3,000*</td>
<td>137,000,000</td>
<td>1/80,000**</td>
<td>1,713</td>
<td>25%</td>
<td>50%</td>
<td>214</td>
<td>$404,737***</td>
</tr>
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</table>

Multiple Manifestations of NPC

No two patients suffer in the same manner

**Systemic**
- Liver Disease and Failure
- Hepatomegaly
- Splenomegaly
- Respiratory Dysfunction

**Central Nervous System (CNS)**
- Impaired Motor Functions
- Behavioral Disturbance
- Loss of Cognition
- Vertical Supranuclear Gaze Palsy (VSGP)

Impaired Quality of Life & Death
NPC Symptoms

- **Jaundice** at birth
- **Enlarged Spleen** or Liver
- Difficulty with eye movements - **Vertical Supranuclear Gaze Palsy**
- **Difficulty in posturing of limbs** (dystonia)
- **Unsteadiness of gait**, clumsiness, problems in walking (ataxia)
- **Hearing loss**
- **Learning difficulties and progressive intellectual decline** (Cognitive dysfunction – “dementia”)
- **Slurred, irregular speech** (dysarthria)
- **Tremors** accompanying movement and, in some cases, **seizures**
- Swallowing problems (dysphagia)
- **Enlarged Spleen or Liver**
- Sudden loss of muscle strength from head nodding to complete collapse/ falls (cataplexy)
There is no cure for NPC.

Patients are treated for their symptoms to help with posture, speech, movement, digestion, breathing among others.

There is only one approved treatment for NPC in the EU, and no approved treatments in the US.

Miglustat (Zavesca®) is approved for NPC in the EU

- It targets the neurological manifestations in adult and pediatric patients with NPC.
- Miglustat has no effect on the systemic manifestations of NPC (e.g. lung, liver, spleen and other organs).
HPβCD is a donut-shaped molecule comprised of seven glucopyranose units (see 2-CH(CH3)-O]n-H. Its hydrophilic exterior allows it to move easily through the body, and its hydrophobic core allows it to capture and hold certain types of molecules. The exact mechanism by which Trappsol® Cyclo™ releases cholesterol from cells and how it causes improvements in NPC symptoms is not known. This is an active area of research. HPβCD is used as an excipient in a number of products including Sporanox (broad-spectrum anti-fungal), eye drops, and mouthwash. Based on animal studies and compassionate use data with Trappsol® Cyclo™, the CTD clinical program was launched with the understanding that HPβCD could be used as an active drug ingredient (rather than as an excipient) at higher doses in NPC.
CTDH’s clinical program is based on:

- **The seminal work of Dr. Benny Liu**, which demonstrated that cyclodextrins could have a positive effect on NPC symptoms and progression in a mouse model. This led to the compassionate use of cyclodextrins in the US and internationally. CTD provided Trappsol® Cyclo™ to families for compassionate use beginning in 2009.

- **Compassionate use data derived from 21 NPC patients** who have been treated with Trappsol® Cyclo™, some for more than 6 years. Multiple patients showed marked improvements in their neurological symptoms of NPC with no significant, unexpected, safety concerns.

- Trappsol® Cyclo™ has been given to NPC patients **via IV administration**, IT and ICV. CTD’s EU and US study protocol of Trappsol® Cyclo™ specifies IV administration.
Clinical data have allowed treating physicians to continue to use HP-β-CD compassionately for more than 6 years in some cases.

<table>
<thead>
<tr>
<th>IV Trappsol® Cyclo™ has been administered to &gt; 20 patients worldwide</th>
<th>Individual patients exhibit objective Systemic/CNS responses</th>
</tr>
</thead>
<tbody>
<tr>
<td>• Favorable tolerability profile among patients treated to date.</td>
<td>• Reduction in hepatic volume and improvement in transaminases.</td>
</tr>
<tr>
<td>• Safety profile has enabled physicians to continue treatment &gt; 6 years.</td>
<td>• Restoration of language skills.</td>
</tr>
<tr>
<td></td>
<td>• Resolution of interstitial lung disease.</td>
</tr>
<tr>
<td></td>
<td>• Improvement in fine and gross motor skills.</td>
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<td></td>
<td>• Improvement in quality of life.</td>
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</tbody>
</table>
Age at time of Diagnosis correlates roughly with rate of disease progression
In a set of patients for which CTD has years of data, IV Trappsol® Cyclo™ either stabilizes NPC disease (lowermost line and upper right lines) or is associated with clinical improvements, as exhibited by lowering of NPC Severity Scores following IV Trappsol® Cyclo™ administration over time. Note: all patients added intrathecal administration to IV within 1 year to 2 years, with limited additional benefit observed.
(Trappsol® Cyclo™)
Phase I/II Study to Evaluate Safety and Efficacy

Randomization 4:4:4 Between Dose Groups

| Trappsol® Cyclo™: Bi-weekly 8 hour intravenous treatment for a period of 48 weeks |
|----------------------------------|------------------|------------------|------------------|
| **Dose Group 1**                 | **Dose Group 2**  | **Dose Group 3**  |
| 1500 mg/kg                       | 2000 mg/kg       | 2500 mg/kg       |

**Randomize (N=12)**

**Primary Endpoint**
- Plasma levels of Trappsol® Cyclo™

**Secondary Endpoint**
- Markers of Cholesterol metabolism/synthesis
- CSF Levels of Trappsol® Cyclo™
- Clinical Outcomes (motor Skills, cognition, eye movements, liver morphology et al)
- Global impression of disease

**Exploratory Endpoint**
- CSF biomarkers of NPC Disease

**Niemann-Pick Disease Type C**
- Confirmed diagnosis of NPC – 1
- NIH NPC Severity Score <30 and with no more than 4 individual domains with a score of > 3
- Age range: 2 years upwards

**Total Sites: 4-5 in 3 Countries**
- UK, Sweden, Italy
- Aptus/Synteract is supporting Trappsol® Cyclo™ by doing site setup and monitoring

**Trial Timeline (estimated)**
- First patient enrollment: Q’2 17
- Last patient enrollment: Q’4 17
- Final data readout: Q’4 18
(Trappsol® Cyclo™) Phase I Study to Evaluate Safety and Impact On Biomarkers of NPC Disease

**Randomization 6:6 Between Dose Groups**

<table>
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<tr>
<th>Trappsol® Cyclo™: Bi-weekly 8 hour intravenous treatment for a period of 14 weeks</th>
</tr>
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<tbody>
<tr>
<td><strong>Dose Group 1</strong></td>
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<tr>
<td>1500 mg/kg</td>
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**Primary Endpoint**
- Plasma levels of Trappsol® Cyclo™

**Secondary Endpoint**
- Markers of Cholesterol metabolism/synthesis
- CSF Levels of Trappsol® Cyclo™
- hepatic and splenic morphology
- global impression of disease

**Exploratory Endpoint**
- CSF biomarkers of NPC Disease

**Niemann-Pick Disease Type C**
- Confirmed diagnosis of NPC – 1
- NIH NPC Severity Score <30 and with no more then 4 individual domains with a score of > 3
- Age range: 18 years upwards

**Total Sites: 1 in United States**
- Emmes is supporting Trappsol® Cyclo™ by acting as Site Management Organization

**Trial Timeline (estimated)**
- First patient enrollment: Q’2 17
- Last patient enrollment: Q’4 17
- Final data readout: Q’1 18
Follow-on indications for use of Cyclodextrins in other therapeutic areas:

- **Acute Viral Infections**: Preliminary discussions with partners on in vitro study
- **Next Generation Trappsol® Cyclo™**: Creating novel Trappsol® Cyclo™ capable of crossing blood-brain-barrier – moving to closure on agreement with top academic laboratory
- **Atherosclerosis**: In discussion with two groups
<table>
<thead>
<tr>
<th>Exclusivity</th>
<th>CTD’s clinical trials expected to lead to <em>exclusivity of Trappsol® Cyclo™ administered intravenously</em> in NPC patients in the US and EU (7 and 10 years, respectively)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Fast Track</td>
<td>Granted <em>Fast Track Designation in the United States</em> allowing CTD increased opportunity for dialogue with FDA</td>
</tr>
<tr>
<td>Innovative</td>
<td>CTD supported research will lead to <em>novel forms of Trappsol®</em>, the IP of which would belong to CTD</td>
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Near-Term Milestones Expected to Drive Value

**Upcoming Clinical Milestone**
- Last Patient in Phase I/II Europe
- Last Patient in Phase I US

**Upcoming Clinical Milestone**
- Begin work on additional Indications

**Upcoming Corporate Milestone**
- Pursue Up listing on National Exchange

**Clinical Milestones**
- Fast Track Designation US (Granted)

**Regulatory Milestones**
- MPA CTA Acceptance (Accepted)
- File CTA with AIFA (Italy) (Completed)

**Regulatory Milestones**
- File IND with FDA (Accepted)
- File CTA with MHRA (UK) (Completed)
- File CTA with MPA (Sweden) (Completed)
- MHRA CTA Acceptance (Accepted)
# Financial Summary

<table>
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<tr>
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<th>2015</th>
<th>2016</th>
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<tr>
<td><strong>CURRENT CASH</strong></td>
<td>$2.3MM</td>
<td></td>
<td></td>
</tr>
<tr>
<td><strong>CURRENT DEBT</strong></td>
<td>NONE</td>
<td></td>
<td></td>
</tr>
<tr>
<td><strong>Revenue</strong></td>
<td>$1,567</td>
<td>$950</td>
<td>$1,503</td>
</tr>
<tr>
<td><strong>OP Ex</strong></td>
<td>$2,130</td>
<td>$3,357</td>
<td>$5,709</td>
</tr>
<tr>
<td><strong>Net Gain (Loss)</strong></td>
<td>($2,380)</td>
<td>($1,842)</td>
<td>($4,206)</td>
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Thank You