An Overview of CTD's Phase I Clinical Trial on Trappsol® Cyclo™ for Niemann-Pick Disease Type C

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CTD Holdings, Inc.

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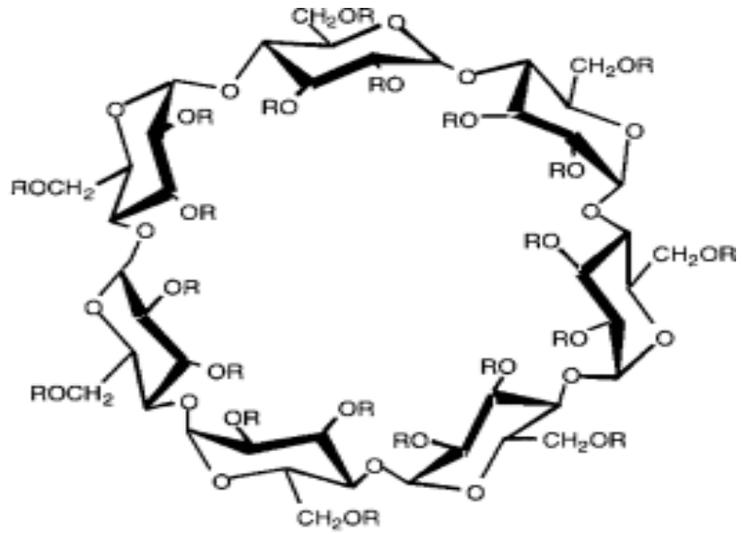
Today's Presentation

- A little history on cyclodextrin use for NPC
- Parameters of Phase I trial and Phase I/II trial in EU/Israel
- Data from quality control of Phase I and Phase I/II trials

Trappsol® Cyclo™



β-Cyclodextrin, R=H
HPβCD, R=$OCH_2CH(CH_3)OH$ or H

This schematic represents interaction of cylinder shaped cyclodextrins and cholesterol in a 1:1 or 1:2 ratio

Figure is courtesy of David Begley, Kings College

- 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
- HPβCD such as Trappsol® Cyclo™ are thought to mobilize stores of cholesterol from the endolysosome to cytosol, having positive effect on cholesterol homeostasis. Academic researchers continue to study mechanism of action
- 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

Trappsol® Cyclo™ Development Program – Historical Context

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 increased lifespan in the NPC mouse. 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, beginning with the Hempels.
- **CTD has gathered compassionate use data derived from >20 NPC patients globally** 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**, Intrathecal (IT) and Intracerebroventricular (ICV). CTD's EU and US study protocol of Trappsol® Cyclo™ specifies IV administration.

US – Phase I Study to Evaluate Safety and Impact On Biomarkers of NPC

Randomization 6:6 Between Dose Groups

Trappsol® Cyclo™: Bi-weekly 8 hour intravenous treatment for a period of 14 weeks

RANDOMIZE (N=12)

Dose Group 1	Dose Group 2
1500 mg/kg	**2500 mg/kg**

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
  - First patient enrollment: Q'3 17
  - First patient dosed Q'3 17

Extension Protocol

April 30, 2018

CTD announced FDA approval of an Extension Protocol, “An Open-Label Extension Study of the Long-Term Safety and Efficacy of Intravenous Trappsol ® Cyclo ™ (HPBCD) in Patients with Niemann-Pick Disease type C (NPC1)”

Protocol allows for continued access to Trappsol ® Cyclo™ while safety data are collected on a long-term basis. Will continue until market registration.

Principal Investigator: Dr. Caroline Hastings, UCSF Benioff Children’s Hospital Oakland

Europe/Israel – Phase I/II Study to Evaluate Safety and Efficacy of Trappsol ® Cyclo™ in NPC

Randomization 4:4:4 Between Dose Groups

Trappsol® Cyclo™: Bi-weekly 8 hour intravenous treatment for a period of 48 weeks

RANDOMIZE (N=12)

Dose Group 1	Dose Group 2	Dose Group 3
1500 mg/kg	**2000 mg/kg**	**2500 mg/kg**

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 then 4 individual domains with a score of > 3
  - Age range: 2 years upwards
- Total Sites: 5-6 in 4 Countries
  - UK, Sweden, Italy, Israel
  - Aptus/Synteract is supporting Trappsol® Cyclo™ by doing site setup and monitoring
- Trial Timeline
  - First patient enrollment: Q'2 17
  - First patient dosed Q'3 17

CTD's SEC 8-K Filing

February 6, 2018

CTD has preliminary data suggesting that Trappsol ® Cyclo ™ crosses the blood-brain-barrier in individuals suffering from NPC.

Data were derived from initial subjects participating in CTD's phase I clinical trial in the US and the phase I/II trial in Europe and Israel.

None of the PIs nor others involved in the conduct or analysis of the data know what doses were used in the subjects from which the data were obtained.

Following IV administration of Trappsol ® Cyclo ™ to study subjects, it was detected in subjects' cerebrospinal fluid.

The significance of these findings will be determined as part of the final analysis of both clinical trials.

Safety Review Committees

March 29, 2018

CTD announced initial review of safety data for the Phase I and Phase I/II trials.

Data examined included AEs and SAEs.

Members and Chairs, Co-Chairs of the Committees are: Professor Alan Boyd (UK), Dr. Caroline Hastings (US), Dr. Reena Sharma (UK), Dr. Bryan Murray (UK

Review of all available data led to conclusion for both studies: Positive Benefit:Safety Ratio

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Reference on Slide 8: ** Noda T, Todani T, Watanabe Y, Yamamoto S. Liver volume in children measured b computed tomography. Pediatr Radiol 1997:27(3):250-2.

Thank You!