CTD Holdings to Present at WORLD Symposium on Lysosomal Storage Diseases

ALACHUA, FL – (Globe Newswire) – December 12, 2018 – CTD Holdings, Inc. (OTCQB: CTDH), a clinical stage biotechnology company that develops cyclodextrin-based products for the treatment of disease with unmet medical need, today announced acceptance of two presentations at the annual WORLD (We're Organizing Research on Lysosoal Diseases) Symposium to be held February 4 – 8, 2019 in Orlando, Florida. The Symposium is designed for basic, translational and clinical researchers, patient advocacy groups, clinicians and others interested in learning more about the latest discoveries related to lysosomal diseases and the clinical investigation of these advances. WORLD attracts participants from across the globe.

"CTD is pleased to have the opportunity to discuss our latest clinical findings with scientific and clinical colleagues and advocates for Niemann-Pick Disease Type C," said CTD Chairman and CEO, N. Scott Fine. "This gathering is a significant opportunity for us to share information and to engage with all stakeholders working to end suffering from this tragic disease."

Niemann-Pick disease type C (NPC) is a rare and fatal genetic disease characterized by the accumulation of cholesterol in every cell in the body. There is no cure.

CTD will present two posters as part of the Late-Breaker session:

Wednesday, February 6, 2019
4:30 pm to 6:30 pm
Hyatt Regency Orlando

The poster titles are:

"Initial findings from a Phase I clinical trial using hydroxypropyl beta cyclodextrin intravenously in Niemann-Pick type C patients"
Caroline Hastings MD; Benny Liu MD, Bryan Hurst MPhil, Bryan Murray MBBS and Sharon Hrynkow PhD. See the abstract Here.

"Initial safety and efficacy findings for a Phase I/II trial of hydroxypropyl beta cyclodextrins administered intravenously in patient with Niemann-Pick Type C disease."
Reena Sharma, MD; Martin Paucar-Arce, MD; Orna Staretz-Chacham MD; Caroline Hastings MD; Bryan Hurst MPhil; Bryan Murray MBBS; and Sharon Hrynkow PhD. See the abstract Here.

"While the ongoing trials are still blinded, the initial findings from both clinical trials using Trappsol® Cyclo™, CTD's proprietary formulation of hydroxypropyl beta cyclodextrin, are encouraging," said Sharon Hrynkow PhD, CTD's Senior Vice President for Medical

Affairs. “We are excited to share initial safety data along with biomarker and clinical efficacy data with the NPC community.”

The presentations will be made by Dr. Caroline Hastings, Principal Investigator for the Phase I study at the at UCSF Benioff Children’s Hospital Oakland, CA site and Dr. Reena Sharma, Coordinating Investigator for the Phase I/II EU/Israel study and Principal Investigator at the UK site at Salford Royal Foundation Trust, Salford, joined by Dr. Orna Staretz-Chacham, Principal Investigator for the Soroka Medical Center site in BeerSheva, Israel,and Dr. Hrynkow. CTD Chairman and CEO will attend WORLD and will join the presentations.

About CTD Holdings:
CTD Holdings, Inc. is a clinical-stage biotechnology company that develops cyclodextrin-based products for the treatment of disease. The company’s Trappsol® Cyclo™, an orphan drug designated product in the United States and Europe, is used to treat Niemann-Pick Disease Type C, a rare and fatal genetic disease, on a compassionate use basis as well as in two ongoing formal clinical trials (Clinical Trials.gov NCT02939547 and NCT02912793). Additional indications for the active ingredient in Trappsol® Cyclo™ are in development. For additional information, visit the company’s website: www.ctd-holdings.com

Safe Harbor Statement:
This press release contains “forward-looking statements” about the company’s current expectations about future results, performance, prospects and opportunities. Statements that are not historical facts, such as “anticipates,” “believes” and “expects” or similar expressions, are forward-looking statements. These statements are subject to a number of risks, uncertainties and other factors that could cause actual results in future periods to differ materially from what is expressed in, or implied by, these statements. The factors which may influence the company’s future performance include the company’s ability to obtain additional capital to expand operations as planned, success in achieving regulatory approval for clinical protocols, enrollment of adequate numbers of patients in clinical trials, unforeseen difficulties in showing efficacy of the company’s biopharmaceutical products, success in attracting additional customers and profitable contracts, and regulatory risks associated with producing pharmaceutical grade and food products. These and other risk factors are described from time to time in the company’s filings with the Securities and Exchange Commission, including, but not limited to, the company’s reports on Forms 10-K and 10-Q. Unless required by law, the company assumes no obligation to update or revise any forward-looking statements as a result of new information or future events.

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Phase I Study using Trappsol® Cyclo™

Initial findings from a Phase I clinical trial using hydroxypropyl beta cyclodextrin intravenously in Niemann-Pick Type C patients

Caroline Hastings, MD; Benny Liu, MD; Bryan Hurst, MPhil; Bryan Murray, MBBS; Sharon Hrynkow PhD

Niemann-Pick Disease Type C is a rare genetic disorder characterized by cholesterol accumulation in every cell of the body. Hydroxypropyl beta cyclodextrins (HPBCDs) have been found in pre-clinical studies to release cholesterol from cells, normalize cholesterol homeostasis, delay symptom onset, and increase lifespan. We present data from the first four patients in a Phase I study using Trappsol® Cyclo™, the proprietary formulation of HPBCD of CTD Holdings. The trial is randomized, double-blinded, with no control group (NCT02939547). Patients received either 1500 mg/kg or 2500 mg/kg of the drug intravenously over 8 to 9 hours every two weeks for 7 doses total. Results presented remain blinded to dose. Individual and cumulative safety data to date show the drug to be well tolerated. In particular, no clinically significant or permanent hearing problems were observed from intravenous dosing of Trappsol® Cyclo™ as measured by standard audiometric testing. Lathosterol, a validated serum biomarker reflecting whole body cholesterol synthesis, was reduced after intravenous administration of the drug, accompanied by a concomitant rise in cholesterol metabolites (24S-, 25-, 27-, and 4B- hydroxycholesterol), suggesting that trapped cholesterol is released and cleared from cells, and cells are responding by suppressing cholesterol synthesis. Cerebrospinal fluid (CSF) sampling at timed intervals following the start of intravenous administration showed increasing levels of drug in the CSF up to 12 hours, indicating that the drug crosses the blood-brain-barrier. Measurements of CSF tau taken at baseline and after the 7th dosing were reduced on average of 30% while two other biomarkers of neuroinflammation, TNF-alpha and GFAP, were below limits of detection at baseline and after the 7th dose. Results on liver ultrasound and elastography taken at baseline and after the 7th dose will be presented. Overall, initial findings and clinician impressions are encouraging.

Phase I/II Study using Trappsol® Cyclo™

Initial safety and efficacy findings for a phase I/II trial of hydroxypropyl beta cyclodextrins administered intravenously in patients with with Niemann-Pick Type C disease.

Reena Sharma, MD; Martin Paucar-Arce, MD; Orna Staretz-Chacham MD; Caroline Hastings, MD; Bryan Hurst, MPhil; Bryan Murray, MBBS; Sharon Hrynkow, PhD

Niemann-Pick Disease Type C is a rare and fatal genetic disorder characterized by cholesterol accumulation in every cell of the body. Hydroxypropyl beta cyclodextrins (HPBCDs) have been found in pre-clinical studies to release cholesterol from cells, normalize cholesterol homeostasis, delay symptom onset, and increase lifespan. We present data from the first four patients participating in a Phase I/II study using Trappsol® Cyclo™, the proprietary formulation of HPBCD of CTD Holdings. The trial is randomized, double-blinded, with no control group (NCT02912793). Patients were randomized to receive one of three doses of Trappsol® Cyclo™ (1500 mg/kg, 2000mg/kg or 2500 mg/kg) administered intravenously over 8 to 9 hours twice monthly for 48 weeks. Results presented remain blinded with respect to dose. The review of individual and cumulative safety data to date has shown the study drug to be well tolerated with no serious safety signals observed. In particular, no clinically significant or permanent hearing problems were observed from IV dosing of Trappsol® Cyclo™ as measured by standard audiometric testing. A plasma biomarker for severity of NPC disease, lysosphingomyelin-509, shows a clear downward trend with successive dosings: in 3 of 4 patients, there was a 30% to 50% reduction. Blinded results from standardized tests for ataxia, cognitive capacity, and NPC Severity Scores and Global Impression of Disease as well as subjective assessments from investigators show variation among patients in terms of outcome measures. Three of four patients showed improvement in one or more outcome measures, including ataxia, saccadic eye movements, speech and overall well-being. Initial impressions are encouraging. Clinical efficacy will be fully evaluated on unblinding.