CTD Holdings Announces MHRA Approval of Clinical Trial Application for Treatment of NPC With Trappsol(R) Cyclo(TM)

Enrollment of Study Participants to Begin in the Coming Weeks

ALACHUA, FL -- (Marketwired) -- 09/15/16 -- CTD Holdings, Inc. (OTCQB: CTDH), a biotechnology company that develops cyclodextrin-based products for the treatment of disease, today announced that on September 12, 2016 the UK Medicines and Healthcare Products Agency (MHRA) approved its Clinical Trial Application (CTA) for its orphan drug product, Trappsol® Cyclo™, in the treatment of Niemann-Pick Type C (NPC) disease. CTD will launch a Phase I/II study to investigate safety, tolerability, pharmacokinetic parameters and pharmacological effects of the intravenous administration of Trappsol® Cyclo™, CTD's proprietary formulation of hydroxypropyl beta cyclodextrin. Trappsol® Cyclo™ has Orphan Drug Designation in both the EU and the US.

"We are delighted to receive approval of the CTA so Trappsol® Cyclo™ can move forward into its formal clinical trial," said N. Scott Fine, CTD Chairman and CEO. "With patients soon to be enrolled in the Phase I/II Clinical Trial in the UK, and with the complementary Phase I Clinical Trial recently approved in the US, this is an exciting time for the NPC families who are awaiting an approved cyclodextrin-based treatment, and for all of CTD's stakeholders and partners."

Niemann-Pick Type C is a rare and fatal genetic disease that impacts primarily children but is increasingly diagnosed in older patients who may live with disability for many years. NPC impacts the brain and major organs through abnormal accumulation of cholesterol in cells.

"The MHRA approval of this study is critical in setting the course toward market approval," said Professor Alan Boyd, senior advisor to CTD and an internationally recognized expert in Pharmaceutical Medicine. "We all look forward to further dialogue with the MHRA and other Regulatory Agencies as clinical data are gathered and as our clinical studies progress."

The clinical study is a randomized, double-blind, parallel group and multi-center study examining three doses of Trappsol® Cyclo™ in patients as young as two years old to adulthood. One of the following doses will be administered intravenously every two weeks for a 48-week treatment period: 1500 mg/kgBW; 2000 mg/kgBW; or 2500 mg/kgBW. The study will evaluate the safety and tolerability of Trappsol® Cyclo™, impacts on cholesterol synthesis and other biomarkers of NPC disease, and the clinical benefits of Trappsol® Cyclo™ relating to gross and fine motor skills, lung and liver function, behavioral and cognitive skills, and quality of life.

Dr. Reena Sharma, an expert in inherited metabolic diseases at the Mark Holland Metabolic Unit, Salford Royal NHS Foundation Trust, serves as the study site director for Salford and the coordinating Principal Investigator for the EU study, which will include sites in Sweden and Italy as well as in the UK.

Dr. Sharma said, "NPC is a systemic disease, impacting multiple organ systems. This intravenous trial allows us to examine multiple tissues to understand how Trappsol® Cyclo™ moves throughout the body and what impact it has on different cell types in NPC patients. This study builds on the ground-breaking work of Dr. Caroline Hastings and others who have used Trappsol® Cyclo™ in compassionate IV programs for years and who report the low-risk nature of this administration route." Dr. Hastings, of UCSF Benioff Children's Hospital Oakland, is senior clinical advisor on the EU study and is the Principal Investigator on the US Phase I study.

The second UK site participating in the study is led by Dr. Robin Lachmann at the Charles Dent Metabolic Unit, National Hospital for Neurology and Neurosurgery, University College London. CTD announced recently its filing of a CTA with the Swedish MPA for a site led by Dr. Martin Paucar at the Karolinska University Hospital, Stockholm.

"We remain deeply grateful to the many NPC families who have encouraged us to launch an intravenous trial for Trappsol® Cyclo™, joined by the physicians who have used our product on a compassionate use basis for years," said Dr. Sharon H. Hrynkow, CTD's Senior Vice President for Medical Affairs and Co-Chair of the company's Family and Physicians Listening Circle. "We look forward to continued engagement with families and physicians globally as we seek to bring effective treatments to NPC patients."

About the Company:
CTD Holdings, Inc. is a 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 Type C, a rare and fatal genetic disease. 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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