# Therapeutic Update

**Trappsol® Cyclo™ Clinical Development Program**

The team at Cyclo Therapeutics, Inc., formerly known as CTD Holdings, is proud to have made great strides in development of Trappsol® Cyclo™ for the NPC1 indication since the last issue of the INPDA Newsletter.

Phase I study based in United States – Top line results out soon

In October 2019, we were excited to report completion of enrollment in our Phase I study using Trappsol® Cyclo™ intravenously in NPC patients (ClinicalTrials.gov NCT02939547), and in February 2020 announced Last Patient Last Visit in the same study. This paves the way for top-line results in the near term on safety and tolerability of Trappsol® Cyclo™ in NPC. While the company has over 10 years of safety data using the drug in a variety of compassionate use programs, the formal Phase I trial results will be an important milestone in the pathway to market registration.

The Cyclo team, joined by Co-Principal Investigators at the UCSF Benioff Children's Hospital Oakland, Dr. Caroline Hastings and Dr. Benny Liu, were pleased to present blinded data from the Phase I study at the WORLD Symposium, Orlando, Florida, also in February 2020. The presentation showed that Trappsol® Cyclo™ at a dose as low as 1500 mg/kg could reduce the amount of cholesterol in liver tissue of NPC1 patients by up to 84%. On unblinding of the study, we will be positioned to comment further on dose, safety, and, to a limited extent, efficacy. As a final note on our Phase I study, we are pleased to support an Extension Study, inclusive of home-based infusions. All indications from those participating in this study is that the home-based infusions are a welcomed alternative to clinic visits!

Phase I/II study based in the UK, Sweden and Israel – Enrollment completed

We were excited to have announced on February 20 "Last Patient In" in the Phase I/II study of Trappsol® Cyclo™ in NPC. This 48-week efficacy trial is based at a number of clinical sites in the UK, Sweden and Israel, with participants as young as 2 years old. The route of administration in this trial is intravenous, same as in the Phase I study. See ClinicalTrials.gov NCT02912793.

Pivotal trial – Upcoming meetings with Regulators

Based on the encouraging preliminary data from the Phase I and Phase I/II studies, the Cyclo Therapeutics team expects to meet with regulators in the US and in Europe in the early part of 2020 to discuss the design of the global pivotal trial for Trappsol® Cyclo™ in NPC. Many in the community have asked about eligibility criteria and potential sites to be involved in the global pivotal study. We look forward to sharing information just as soon as possible. Physicians interested in learning more about the pivotal program should be in touch Dr. Sharon Hrynkow, the company's CSO and Senior VP for Medical Affairs, at Sharon.Hrynkow@cyclodex.com.