Plain Language Summary

Long term administration of intravenous Trappsol Cyclo (HP-β-CD)

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Plain Language Summary: Results of a Phase I/II trial of long-term administration of intravenous Trappsol Cyclo (HP-β-CD)

What is this study about?

This research study tested whether a medicine called Trappsol Cyclo (also known as HP-β-CD) can help people with Niemann-Pick Disease Type C1 (NPC1). NPC1 is a rare genetic disease that causes harmful substances to build up in cells throughout the body, leading to serious problems with the brain, liver, spleen, and other organs.

What did the researchers do?

The scientists conducted an international Phase I/II clinical trial that followed 12 patients for 48 weeks as they received the drug through an IV (into a vein). This type of study tests both the safety of a new treatment and whether it works. There were three different dose levels, and neither the patients nor the doctors knew which dose each patient was getting. There was no placebo group; all patients received the active drug.

How does Trappsol Cyclo work?

Trappsol Cyclo is a form of cyclodextrin, a special type of molecule that can help capture cholesterol and other fatty substances that get trapped inside the cells of people with NPC1. It can then help remove the cholesterol and other fatty substances, reducing damage to organs and possibly slowing down disease progression.

What the Study Found:

- **Participation:** 9 out of the 12 patients completed the full 48-week study. The others stopped for reasons not related to safety, like travel issues during the COVID-19 pandemic.
- **Drug in the body:** The drug was found in the spinal fluid, which means it reached the brain. It also seemed to help move cholesterol in the body, as shown by changes in cholesterol markers.
- **Improvements in patients:**
  - Most patients (8 out of 9 who finished) showed improvement in at least two areas of their symptoms, based on a special NPC severity scale.
  - Many also improved in areas that patients and their families felt were most important for their quality of life, such as speech, swallowing, movement, and thinking.
  - Doctors and patients generally felt that patients either improved or stayed stable, which is a positive outcome for a disease that usually gets worse.
  - Some patients even showed improvements in specific movement and balance tests.
  - Liver and spleen size either stayed the same or improved, with no worsening.
- **Safety:** The drug was generally well-tolerated. Most side effects were mild to moderate and were often related to the disease itself or common illnesses, not the study drug. No patients stopped the study because of side effects, and there were no deaths. Some patients had temporary or slight changes in hearing during tests, but these were not usually noticed by the patients or their families and often returned to normal.

Why does this matter?

NPC1 is a progressive disease, meaning it typically gets worse over time, often leading to severe disability and shortened lifespan. Until recently, treatment options were very limited. Finding new medicines that can stabilize or slow down the disease progression by removing cholesterol and fatty substances from cells could significantly improve quality of life and outcomes for patients and their families.

What this means for patients:

This study provided evidence that Trappsol Cyclo could offer clinical benefits to people with NPC1 by removing cholesterol and fatty substances from cells. The research showed that patients who received the treatment experienced stabilization or slowing of their disease progression over the 48-week study period. This suggests that the medicine could be a new treatment option.

The study also helped establish the safety profile of long-term IV administration of Trappsol Cyclo, which is important information for doctors considering this treatment for their patients. This research represents an important step forward in expanding treatment options for NPC.