Plain Language Summary

Intravenous 2-hydroxypropyl-β-cyclodextrin (Trappsol Cyclo)

Citation: Caroline Hastings, Benny Liu, Bryan Hurst, Gerald F. Cox, Sharon Hrynkow, Intravenous 2-hydroxypropyl-β-cyclodextrin (Trappsol Cyclo) demonstrates biological activity and impacts cholesterol metabolism in the central nervous system and peripheral tissues in adult subjects with Niemann-Pick Disease Type C1: Results of a phase 1 trial, Molecular Genetics and Metabolism, Volume 137, Issue 4, 2022, Pages 309-319, ISSN 1096-7192, https://doi.org/10.1016/j.ymgme.2022.10.004.

https://www.sciencedirect.com/science/article/pii/S109671922200419X?via%3Dihub

Plain Language Summary: Results of a Phase I trial of intravenous 2-hydroxypropyl-β-cyclodextrin (Trappsol Cyclo) in adults with NPC.

What is this study about?
This research study tested whether a medicine called Trappsol Cyclo (also known as 2-hydroxypropyl-β-cyclodextrin or HP-β-CD) can help adults with Niemann-Pick Disease Type C1 (NPC1). NPC1 is a rare genetic disease where cholesterol and other fatty substances get trapped inside cells throughout the body, causing serious damage to the brain, liver, spleen, and other organs.

What did the researchers do?
The scientists conducted a Phase 1 clinical trial, which is the first step in testing a new medicine in humans. This type of study focuses mainly on safety—making sure the treatment doesn't cause harmful side effects—while also looking for early signs that it might work. Adult patients with NPC1 received Trappsol Cyclo through an IV (intravenous infusion) directly into their bloodstream, and researchers carefully monitored what happened in their bodies.

How does Trappsol Cyclo work?
Trappsol Cyclo is a special type of cyclodextrin molecule that acts like a molecular "helper." It can grab onto cholesterol and other fatty substances that get stuck inside cells in people with NPC1. By helping to move these trapped substances out of cells, the medicine may help reduce the damage they cause, especially in the brain and other important organs.

Why does this matter?
NPC1 is a progressive disease that typically gets worse over time, often leading to severe neurological problems and shortened lifespan. The buildup of cholesterol in brain cells is a major part of what makes this disease so devastating. Finding a treatment that can impact cholesterol metabolism in the brain could be impactful for patients and families.

What this means for patients:
This Phase 1 study provided the first scientific evidence that Trappsol Cyclo can reach the brain and affect cholesterol metabolism in people with NPC1. The research showed that the medicine demonstrated "biological activity," meaning it was doing something measurable in patients' bodies. This proof-of-concept data shows the treatment could potentially help.

The study also established that IV administration of Trappsol Cyclo appeared to be safe in adult NPC1 patients, which was essential information needed before moving forward with larger, longer studies. This research laid the groundwork for the later Phase I/II trial that explore safety and clinical outcomes over 48 weeks.