



Plain Language Summary

Efficacy and safety of arimoclomol in NPC

Citation: Patterson MC, Vecchio D, Jacklin E, et al. Efficacy and safety of arimoclomol in Niemann-Pick disease type C: Results from a double-blind, randomised, placebo-controlled, multinational phase 2/3 trial of a novel treatment. *J Inherit Metab Dis.* 2021 Nov;44(6):1463-1480. doi: 10.1002/jimd.12428. Epub 2021 Sep 7.

<https://pmc.ncbi.nlm.nih.gov/articles/PMC9293014/>

Plain Language Summary: Results from a double-blind, randomized, placebo-controlled, Phase 2/3 trial of the efficacy and safety of arimoclomol in NPC.

What was this study about? This study looked at a medicine called arimoclomol for people with Niemann-Pick Disease Type C (NPC). NPC is a rare, serious genetic disease that causes problems with how the body handles fats, leading to damage in the brain, liver, and other organs. The main goals of this study were to see if arimoclomol was safe and if it could help stabilize or improve the symptoms of NPC.

Who participated in the study? The study included 50 patients with NPC that were 2-19 years old. These patients were from different countries around the world.

What was tested? The researchers tested arimoclomol, an oral medication. Patients were allowed to continue taking another NPC medicine called miglustat if they were already on it.

How was the study done? This was a "double-blind, randomized, placebo-controlled" study, which is a strong way to test medicines. This means:

- **Randomized:** Patients were randomly put into one of two groups.
- **Double-blind:** Neither the patients nor their doctors knew if they were getting the actual medicine (arimoclomol) or a placebo (a dummy pill with no medicine).
- **Placebo-controlled:** One group received arimoclomol, and the other received the placebo. Patients took their assigned medicine as capsules by mouth three times a day for 12 months.

What were the main findings? The study found that:

- **Overall Symptoms:** The main goal of the study was to see if arimoclomol improved NPC symptoms over 12 months, as measured by a special scale (the NPC Clinical Severity Scale). In the overall group of patients, arimoclomol showed an improvement compared to patients receiving placebo.
- **Safety:** Arimoclomol was generally safe and well-tolerated. The most common side effects were stomach and gut problems, like diarrhea.
- **Other Observations:**
 - When researchers looked closer, they found that patients who were taking miglustat at the start of the study seemed to show an enhanced benefit from arimoclomol.
 - The study also looked at a specific marker in the blood (called lysoSM-509) that is linked to NPC. Patients taking arimoclomol showed a decrease in this marker, which suggests the medicine was having an effect on the disease process.

What do these findings mean? This pivotal study demonstrated that arimoclomol can slow down the worsening of symptoms in NPC patients. The positive change in the blood marker also indicates that the medicine was active in the body.