



Participate in GSD1a Research and Make an Impact

Why are we doing this study?

An investigational gene therapy called DTX401 is being tested for possible use in the treatment of glycogen storage disease type 1a (GSD1a).

Ultragenyx is sponsoring this study to find out if DTX401 is a safe and effective treatment for adults and children with GSD1a. The goal of this study is to evaluate if DTX401 can help people with GSD1a maintain or improve blood sugar levels with less or no cornstarch and reduce the risk of hypoglycemia.

Consider participating if you:

- Have been diagnosed with GSD1a
- Are 8 years of age or older
- Are currently receiving cornstarch regularly as part of your dietary regimen

What is involved?



Study

All participants will receive DTX401, either initially or after ~1 year (if still eligible)

- 9 clinic visits that require a 1-2-night stay
- 4 outpatient visits
- 16 outpatient/home health visits

All patients who receive DTX401 will be offered to enroll in an optional long-term follow-up study

What to expect:



Infusion of study medicine into your arm



Diet diary



Continuous glucose monitoring*



Medical questionnaires



Lab tests



Diet and cornstarch assessment



Controlled fasting challenge**

*Study participants will not see these results

**At the hospital



For additional study information, please contact TrialRecruitment@Ultragenyx.com

At Ultragenyx, we are committed to bringing patients new treatments for rare and ultra-rare diseases