



Adeno-associated viruses (AAVs) are naturally occurring viruses that are used in gene therapy. People who have been exposed to AAV naturally and have antibodies to the virus may not be able to take AAV-based gene therapies. This is a study to better understand how common it is for patients with OTC deficiency, GSD1a, or Wilson disease to have antibodies to certain types of AAVs.

## STUDY DESIGN

### Screening



#### You may qualify if:

- You are 18 years of age or older with a diagnosis of OTC deficiency, GSD1a, or Wilson disease



#### You may **not** qualify if:

- You have received an AAV-based gene therapy
- You have had a liver transplant
- You are currently in or have previously participated in an Ultragenyx clinical study
- You have any condition that might prevent you from safely participating in the study

#### During screening, you will be asked to:

- Sign an agreement to participate once the study has been explained to you
- Tell us about your medical history
- Provide information including gender, age, height and weight

### Tests

#### During a single visit either at home or at a clinic:



#### OTC deficiency

- A blood sample will be taken to test whether you have antibodies to the AAV8 serotype of the AAV virus
- A blood sample will be taken to study your *OTC* gene



#### GSD1a

- A blood sample will be taken to test whether you have antibodies to the AAV8 serotype of the AAV virus
- A blood sample will be taken to study your *G6PC* gene



#### Wilson disease

- A blood sample will be taken to test whether you have antibodies to the AAV9 serotype of the AAV virus
- A blood sample will be taken to study your *ATP7B* gene



### About Ultragenyx

Ultragenyx Pharmaceutical Inc. is a biopharmaceutical company committed to bringing patients novel products for the treatment of rare and ultra-rare diseases, with a focus on debilitating genetic diseases for which there are no approved therapies.

## WHAT TO EXPECT DURING THE STUDY

### KEY FORMS AND ASSESSMENTS



Agreement to participate



Medical questionnaires



Genetic testing



Lab tests



If you are interested in participating in this study and would like additional information, please contact **877-223-3576** and enter access code **89431**.