



Participate in OI Research and Help Make an Impact.

Why are we doing this study?

Setrusumab is a monoclonal antibody being developed for the treatment of osteogenesis imperfecta (OI). The purpose of this study is to investigate the efficacy and safety of setrusumab in pediatric and young adult patients with OI Types I, III, or IV.

Consider participating if you:

- Are at least 5 but not yet 26 years of age
- Have a confirmed diagnosis of OI Types I, III, or IV
- Had at least one fracture in the past year or at least two fractures in the past 2 years
- Are willing to not receive bisphosphonate therapy during the study

Why is it important to be willing to not receive bisphosphonate therapy during the study?

This study is designed to see the effect of setrusumab on the effects of OI. That effect can be more clear if all participants are not receiving bisphosphonates during the study. All participants will be closely monitored throughout the study. If you do experience frequent fractures during the study (while potentially receiving placebo), then you would be eligible to receive active drug after as little as 12 months.

What is involved?

Study duration:

- **Screening Period:**
Up to 1 month
- **Study Treatment Period:**
1 to 2 years (depending on when you enroll)
- **Treatment Extension Period:**
Receive setrusumab until commercial drug is available



Study treatment:

- Setrusumab (2 in 3 chance) or placebo (1 in 3 chance), given by intravenous (IV) infusion once a month



Study visits:

- You will receive study treatment and have lab tests and assessments at monthly study visits. The number of visits you will have will depend on when you enroll in the study and when commercial drug is available.

What to expect:



Infusion of study medication into your arm



Blood and urine tests



Physical exams and vital signs



X-rays and bone density tests



Heart tests



Dental health and hearing assessments



Electronic diary and questionnaires



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

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