



We are investigating a medicine for the treatment of OI. Join us!

Why are we doing this study?

The purpose of this clinical trial is to investigate the efficacy and safety of a new medicine called setrusumab in pediatric and young adult patients with OI Types I, III, or IV. Current treatments for OI focus on preventing bone from breaking down (bone resorption). However, bone remains abnormal, and some patients still experience fractures. Setrusumab works differently than bisphosphonates. We are performing this study to see if treatment with setrusumab will reduce the total number of fractures a patient with OI experiences. Setrusumab is not approved by any country's health authority, other than for use in a clinical study such as this.

Consider participating if you:

- Are between the ages of 5 and 26
- Have OI Types I, III, or IV
- Had at least one fracture in the past year, two fractures in the past 2 years, or a tibia, humerus, or femur fracture in the past 2 years
 - Fractures can be self-treated or assessed by your doctor
- Agree to not take other therapies that may impact the bones during this study, including bisphosphonates

I need to stop my Bisphosphonates? Why?

The purpose of this study is to evaluate how setrusumab works in patients with OI. To assess the effects, it is important that we eliminate as many other factors as possible that may make it more difficult to interpret results. Therefore, we require that patients not take other medications that may impact their bones during this study.

What to expect:



Monthly infusion of study medicine



Blood and urine tests



Monthly physical examinations



Regular X-rays and bone density tests



Heart tests



A smartphone app to track your health and fractures (a smartphone will be provided if you don't have one)

What is involved?

Study duration:

Screening Period:
Up to 1 month



Study Treatment Period:

1-2 years, with a maximum study enrollment time of 24 months



Treatment Extension Period:

Receive setrusumab until commercial medicine is available



Study treatment:

- For Phase 3, you will be assigned to receive either the study medicine or placebo (a pretend medicine). Two people will receive study medicine for every one person who receives placebo. After the study treatment period, everyone (100%) will receive the study medicine.

Phase 3

- setrusumab



- placebo



Study visits:

- You will receive study medicine 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.



Support is provided for travel costs to clinic visits.



For additional OI study information, please contact OIstudyinfo@ultragenyx.com



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