Are there any benefits to taking part?

Participants may not gain directly from taking part but the knowledge gained through the trial will be beneficial in the longer term as it will help us understand the best way to treat OI to prevent fractures

Are there any side effects or risks?

Like all medicines, the treatments used in the TOPaZ active treatment arm and the standard care arm can cause side effects. People who are interested in finding out more will be given detailed information by researchers and will have plenty of time to both ask questions and think about whether the trial is right for them.

The trial involves more scans (DEXA and x-rays) than a person with OI would normally have in routine clinical practice but the radiation exposure is still classed as a negligible risk.

Where are TOPaZ sites located?

The study will run at over 25 hospitals across the UK and Ireland. Even if you are not attending one of these hospitals it may still possible for you to take part in the trial by contacting the co-ordinating centre by telephone, email or post

TOPaZ sites across the UK & Ireland

Finding out more

TOPaZ Co-ordinating Centre
Edinburgh Clinical Trials Unit
University of Edinburgh
Western General Hospital
Edinburgh
EH4 2XU

Website: http://edin.ac/topaz-trial

Telephone: 00 44 (0) 131 537 2573

@TOPaZ_Trial

http://edin.ac/topaz-trial

The TOPaZ Trial

Treatment of Osteogenesis Imperfecta with Parathyroid hormone and Zoledronic Acid

BRITTLE BONE SOCIETY www.brittlebone.org

NHS National Institute for Health Research

accord Academic and Clinical Central Office for Research and Development

NHS Lothian

Version 1 (04/01/2017) IRAS 207747

If you have a diagnosis of Osteogenesis Imperfecta (brittle bone disease) and are aged 18 years or over, you may be eligible for the TOPaZ Trial

What is the TOPaZ Trial?

TOPaZ is a randomised open-label clinical trial for people with Osteogenesis Imperfecta (OI)

What does the trial aim to do?

We want to find out whether a two-year spell of treatment with a drug called Teriparatide (TPTD) followed by treatment with another drug called Zoledronic acid (ZA) reduces the risk of broken bones occurring in people with Osteogenesis Imperfecta. The idea is that TPTD treatment will build up new bone and the ZA will maintain the increase in bone strength that's occurred in response to the TPTD treatment.

People who enrol in the trial will have a 50% chance of having the TPTD/ZA therapy or continuing with their normal treatment. The reason that we are running the trial is that we don't know which is the best treatment – so both the group given the new treatment and the group receiving normal treatment are equally important.

What is a randomised open-label clinical trial?

TOPaZ is randomised controlled trial. These types of trial aim to make a fair comparison between a new treatment and an existing treatment to see which one works best. The decision about who gets one treatment or another is random – made by chance rather than by a doctor or by a participant. This means that researchers can be sure that any differences between the groups are due to the treatment and not to any other factor.

'Open-label' means that participants are randomised at the beginning of the trial and will know straight-away which treatment group they are part of.

Both treatment arms are equally important: we want to know which treatment is best but we will only know this by carrying out the trial!

Who is organizing the trial?

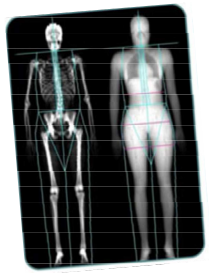
The TOPaZ Trial is organised by the University of Edinburgh and is funded by the UK National Institute for Health Research (NIHR) Efficacy and Mechanism Evaluation Programme.

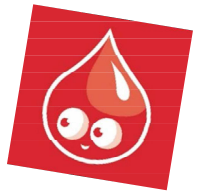
What's involved for participants?

Researchers at the hospital will provide information and an opportunity to discuss the trial. If someone then wants to take part they are first asked for their written consent.

Each participant is then asked to:

- Attend a 'Baseline' Visit at the hospital for
  - Safety blood samples
  - Research blood samples
  - Physical exam and a check of medical history
  - DEXA scan and spinal x-ray
  - HRQCT scan (at some hospitals only)
  - Questionnaire set





- Participants are then asked to keep a diary of any fractures and are contacted every 6 months by researchers by phone to see how they are
- There is a 12 and 24 month Visit at the hospital for repeat bloods, scans and questionnaires
- All participants are then asked to attend a final visit at the end of the trial for bloods, scans and questionnaires