



Study Summary: Stakeholder views on stem cell transplantation for Osteogenesis Imperfecta (OI)

Background

The Boost Brittle Bones Before Birth (BOOSTB4) clinical trial is investigating transplanting mesenchymal stem cells during pregnancy and in the baby's first year to treat severe OI. The number of children with OI who have had stem cell transplantation is very small and the BOOSTB4 clinical trial will look at both the safety of the treatment and how well it works to treat OI. BOOSTB4 is an international study; led by researchers in Sweden and working with researchers in the UK, Germany and the Netherlands. As part of the BOOSTB4 study, we conducted interviews in the UK to explore people's views of using stem cell transplantation to treat OI.

Study findings

We interviewed three groups of people;

1. 26 adults who were either affected with OI (with or without children) or were parents of children affected with OI
2. 29 health professionals who work with patients affected with OI
3. 2 patient advocates from relevant patient support groups

We found that most people held positive views towards using stem cell transplantation to treat OI. Some of the potential benefits people discussed were;

- Early treatment could prevent fractures and reduce the severity OI.
- Reducing the severity of OI would mean fewer hospital visits and, as a result, more time at school and greater involvement in family and social activities.
- Stem cell transplantation would be an additional option for people with OI to consider when starting a family which might mean people have fewer concerns about having a child with the condition.

The most common concerns that people discussed were;

- Safety for child and mother when having the stem cell transplantation.
- Possible short/long-term side effects.
- Not knowing whether the stem cell transplantation would work.
- The potential for parents to have unrealistic expectations of how well the stem cells will work.

Some ethical concerns were raised about stem cell transplantation. The stem cells that will be used in the BOOSTB4 clinical trial are obtained when women who have chosen to have a termination of pregnancy for personal reasons before 12 weeks in pregnancy agree to donate cells from the fetus for use in stem cell transplantation research. Some people that we interviewed were "uncomfortable" or "worried" about the stem cells being fetal in origin. But most felt that using fetal cells for stem cell transplantation was an "opportunity for something good to come out of a bad situation".

An important part of our study was to ask people about the counselling and support needs of parents when they are deciding whether or not to have stem cell transplantation. Our participants highlighted that parents will need;

- Information about the stem cells that is explained in a way that all parents can understand
- Counselling that sets realistic expectations as we don't know yet if stem cell transplantation will work
- Psychological support
- Time for reflecting on the decision as decisions should not be rushed
- Counselling and support that is specific to individual families and considers their previous experience with OI

Update on the BOOSTB4 clinical trial

The clinical trial has ethical approval in Sweden and the UK and is due to start later in 2019. You can find out more information about the clinical trial on the study website: <http://boostb4.eu/>

During the BOOSTB4 clinical trial we will interview parents about their experiences of being offered stem cell transplantation and their involvement in the trial.