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Unique stem cell brittle-bone study starts

A study is to be conducted for the first time involving the transplantation of stem cells into unborn babies with the brittle-bone disease osteogenesis imperfecta, which causes repeated fractures, often before birth. The study is to be coordinated by Karolinska Institutet but is to be run as a collaboration between several leading European research centres and companies.

Babies born with the severe form of osteogenesis imperfecta, or congenital brittle-bone disease, are often seriously ill. Repeated fractures in all parts of the skeleton give rise to physical disabilities, postural abnormalities, and stunted growth. The child's breathing and pulmonary function can also be affected with the narrowing of the rib cage. There is currently no cure.

Collagen is a thread-like protein found in bone that has a similar reinforcing function to iron rods in concrete. Since the disease is caused by an inability of the developing body to form collagen, scientists at Karolinska Institutet have produced a special strain of stem cell, which when injected into the body of sufferers targets and strengthens the bone by producing collagen. Studies on mice have shown a positive response to the treatment, which has also been tried on a couple of children with the disease.

"The oldest child to have received the treatment is now 13 and is performing better than expected and is still growing," says Cecilia Götherström, a researcher at Karolinska Institutet's Department of Clinical Sciences, Intervention and Technology. "But we believe that we can improve the treatment for other patients by administering it to the unborn baby and again in repeated doses during the child's first years of life."

Dr Götherström will be leading the study, which will test the treatment on unborn babies. Thirty babies will be included in the study, half of whom will receive stem cells before birth, and half after. New treatments will then be done at six-monthly intervals over a period of two years to enhance the effect. The researchers will then assess the results by analysing the development of bones and counting the number of fractures that the children in both groups suffer and compare the results with children who have not been treated with stem cells.

The "Boost Brittle Bones Before Birth" (BOOSTB4) project will start in January 2016 and will be coordinated by Karolinska Institutet. Other participating research centres and companies include University College London, Great Ormond Street Hospital, London, the University of Leicester, Universitair Medisch Centrum Utrecht, Leiden University Medical Centre, Uniklinik Köln, Lund University, Cell Protect Nordic Pharmaceuticals AB, Nordic Health Economics, MedSciNet and Euram Ltd. The study will also include patients from other European countries. Funding has been obtained from several sources, including the Swedish Research Council (grant agreement number E0720901, € 1,760,000) and the European Union's Horizon 2020 research and innovation programme under grant agreement No 681045 (€ 6,608,752).

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