Stem cell treatment for brittle bones in the womb

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The extraordinary results of an in utero stem cell treatment could lead to a new treatment for babies with brittle bones, as well as a range of other disabling conditions, according to a maternal-fetal medicine researcher, now based at The University of Queensland (UQ).

Action Medical Research has announced the outcomes of an Imperial College London study, conducted by a team led by Professor Nicholas Fisk, that could lead to a stem cell treatment for babies with brittle bones - before they are even born.

Professor Fisk, who now heads the new $66m UQ Centre for Clinical Research, said the work held potential for improving treatment of other disabling conditions such as muscular dystrophy and congenital brain diseases.

Brittle bone disease or Osteogenesis imperfecta (OI), as the inherited disease is known, affects babies whilst they are inside their mother's womb. This is because collagen, one of the main building blocks for bone, fails to develop properly. The disease is detected by DNA testing or ultrasound before birth and leads to weak bones and stunted growth.

The team, led by Professor Nicholas Fisk, transplanted specially manipulated stem cells into 14 day old mouse fetuses that had OI. These mice had a reduction in long bone fractures of two thirds, compared with an untreated group, by the time they were twelve weeks old. They also found that the bones of these mice were stronger, thicker and longer than

those with the disease that had not received the transplants. These outstanding results published in the journal Blood suggest that, with further research, this treatment could be translated to human babies in pregnancies that are affected by OI.

Dr Yolande Harley of charity Action Medical Research, which funded the project, said:

“Professor Fisk's work is a real breakthrough. It suggests that if stem cells could be successfully transplanted before a baby with OI is even born, it could mean a significant improvement in the child's health and quality of life.

“This is a tremendous piece of work that could have implications for many more diseases and conditions,” he said.

Professor Fisk said the research had shown “a profound therapeutic benefit” of intrauterine stem cell therapy.

“It has significance not only for treating this and other disabling conditions in affected fetuses inside the womb, but also for future related work. It will help us to understand what it is that leads to such a marked effect after a single transplant of stem cells, so that this can be harnessed to improve the results of stem cell therapy in repairing adult tissues and degenerative conditions.

“Our work suggests that, in the future, it could be possible to take stem cells from an unborn baby carrying the abnormal OI gene, manipulate them to correct the errant gene and then put them back into the fetus to allow it to develop properly”.

Source: Research Australia