Could some inherited diseases be treated more effectively before birth?

Rresearch in mice suggests that treatment for haemophilia – and maybe other inherited diseases – could start in the womb, boosting the success of therapies after birth.

In haemophilia, genetic mutations cause a lack of blood clotting proteins. The most common type is caused by a lack of coagulation factor VIII. People born with the disorder can be given injections of factor VIII, but the immune systems of about one-fifth of people with haemophilia develop antibodies that render the protein ineffective.

To see if priming in the womb would make any difference to this immune response Sébastien Lacroix-Desmazes and his at at the French research institute INSERM attached parts of factor VIII to another protein that enabled it to cross the placenta between mother and fetus. The group then administered this to pregnant mice lacking factor VIII. Other similar pregnant mice received no treatment.

Once the pups were born, the team treated all of the offspring with a factor VIII therapy. The mice treated while in the womb were much more tolerant of the protein – on average, their immune systems produced 80 per cent less antibody against it than the control mice.

Sing Sing Way, an infectious disease physician and scientist at the Cincinnati Children's Hospital Medical Center in Ohio, says that exploring the idea of fetal immune therapy is worthwhile but it is early days. "The study shows that this approach can work in mice, but does little to say how it may actually work as a therapy or preventative strategy for humans."

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