

# The Economist

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The  
end?



## SCIENCE AND TECHNOLOGY

## Sandhoff's disease

## An embryonic cure

RIYADH

IN SAUDI ARABIA, hundreds of years of desert isolation have led to a tradition of marrying into your own family. This has inevitable consequences, one of which is the prevalence of inherited diseases that would otherwise be rare. So numerous are such genetic disorders that the Saudis have opened an Inborn Errors of Metabolism Centre at King Faisal Hospital in Riyadh, where Dr Pinar Ozand, a Turkish-American pediatrician, leads the research.

One of the nastiest types of condition facing Dr Ozand and his colleagues goes un-

der the name of lysosomal storage disease. It establishes itself early during a fetus's development. An affected infant cannot break down wastes properly because it does not produce enough of the right sort of enzymes—and as a result will probably die between the ages of two and four. Although such conditions can be diagnosed long before birth, Islamic law and tradition does not allow affected fetuses to be aborted.

The best known variant of storage disease is Tay-Sachs, which affects Ashkenazi Jews in disproportionate numbers; Morquio disease in Quebec and Sanfilippo's syndrome in Holland are further examples. In Saudi Arabia the frequency of such conditions is 5-10 times higher than the world average of one in 100,000. The most prevalent in the kingdom is Sandhoff's disease, affecting one in every 2,000 people. It is closely related to Tay-Sachs. In Sandhoff's, an affected child lacks one of two components of an enzyme called hexosaminidase. As a result the child cannot digest certain sugar-containing compounds, which travel instead to the brain; by the age of one it is

blind and demented. Children with Tay-Sachs disease lack the other component of the enzyme.

There have been attempts to treat storage disease after birth (and at a cost of around \$100,000 per patient) with bone-marrow transplants; there are no proven therapies. But recently two doctors in America performed the first transplant operation in the womb to overcome storage disease before birth. The baby has just been born. Dr Esmail Zanjani of the University of Nevada in Reno and Dr William Krivit of the University of Minnesota in Minneapolis, who has done post-natal work on storage disease for many years, are evaluating the results and think that further use of their procedure is just a few months away.

Dr Zanjani thinks from his own animal studies of the subject, especially on monkeys, that there is little doubt that the transplant technique will work in the womb for people too. Their technique is to take certain "stem" cells from the livers of aborted fetuses and inject them into the belly or blood of a fetus with storage disease at about

the 12th week of its development. During gestation these cells, whose job is to produce red blood cells, spread from the liver to bone marrow and thence to all organs, including the brain. A fetus with healthy transplanted stem cells will produce enough of the crucial enzymes.

As well as Sandhoff's disease several other storage diseases should be treatable in the same way; the only differences between the diseases are in the particular enzymes affected. The method is an improvement over post-natal bone-marrow transplants because it should be able to stop the disease before it has set in, and should cost considerably less. But since abortions are illegal in Saudi Arabia there may be a considerable problem in getting hold of fetal cells for transplants there. An ethical committee at King Faisal Hospital has still to approve the procedure. If it is approved, frozen cells could perhaps be flown in from Europe or America. Doctors at the hospital feel that the desperate nature of the disease would justify such measures.