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Gardener convicted of murdering expat lover

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A gene therapy treatment has been approved for production by a major pharmaceutical company for the first time, in what scientists hope will open up a long-promised field of medicine.

GlaxoSmithKline, the British drug company, will be allowed to sell a genetic therapy to treat an extremely rare immune disorder in children. The treatment works by taking cells from the child's own body, replacing a defective gene, and then reimplanting them.

No other such treatment has been approved before in children and none have been previously created by multinationals. The fact that the GSK has the green light for its Strimvelis treatment has been taken as a sign that after several false starts the research area may at last be coming to maturity.

In theory, gene therapy is an area with tremendous therapeutic promise — used to correct mutations that can have a devastating effect on people's lives and to let people's own cells save them. In practice, producing a working and safe treatment has proven extremely difficult.

Strimvelis is used to treat children suffering from a disease called ADA-SCID, in which the body does not make a particular enzyme. This leads to a build-up of toxins that slowly destroys the body's immune system. Untreated, children suffering from the disease will normally die of infections in the first two years of their lives.

The treatment works by taking bone marrow stem cells from the affected children, replacing the defective gene so that they can make the enzyme and reinserting them. These then effectively restore the immune system.

But for most people the significance of the treatment's approval was not so much about the fact that there is now a treatment for one of the rarest diseases in the world, but more a celebration that the method is workable. Martin Andrews, the head of GSK's rare diseases unit, said the company hoped to extend the approach to more common conditions. "Longer term, as the human genome gets unravelled, we believe strongly that we will find many diseases are not single diseases at all, but amalgams, and subsets and subsets. This is something we need to get used to – and find ways of economically developing treatments and bringing them to smaller populations."

Already, cancer researchers have shown that cancers such as breast cancer are dozens of separate diseases that have been grouped into one category. Mr Andrews said that gene therapy may be the best way of responding to this because it can harness people's own bodies to fight disease. "We think cells as medicines are the future. This we think could be a new frontier in therapeutic medicine."

He said they were investing in rare diseases, which are not very lucrative, because they are the easiest place to test the technology. "We have to start where the science is most tractable. Ultra rare diseases is an area where corrective gene therapy is viable. There is normally a very specific target, a single gene change. Ultimately, we believe if we can reprogram cells, there is a fairly wide range of diseases we could attack."