

# European regulator: Gene therapy treatment OK (Update)

July 20 2012, by MARIA CHENG

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(AP) — The European Medicines Agency is recommending the first-ever approval of a gene therapy treatment in the EU, in a significant move for a type of treatment that has so far failed to deliver on its promise to cure diseases.

In a statement on Friday, the EMA said Glybera, made by Dutch company uniQure, should be approved across Europe for the treatment of an extremely rare disorder that leaves people unable to digest fat. The treatment consists of a gene that makes a protein to break down fat.

Gene therapy is an experimental technique that tries to cure diseases by replacing genes that don't work. It has never been approved in the U.S. and most trials over the past two decades have failed. China was the first country to approve a gene therapy treatment in 2003 for cancer.

Scientists have struggled to find ways to deliver the genes safely, often by using a harmless virus. There are also concerns that inserting a gene at the wrong spot could cause cancer or that the body's immune system might attack the new gene and the virus used to deliver it.

The EMA previously rejected Glybera three times but it was reconsidered at the request of the European Commission. The agency recommended approval under tough restrictions and will require the company to set up a registry to closely track patients. Previous trials of the treatment only tested it in 27 patients.

"It is only meant for patients with the greatest need," said Monika Benstetter, an EMA spokeswoman, explaining the gene therapy is intended for people with no other treatment options. She said only a handful of gene therapy treatments had been considered before — one was recommended for approval but its manufacturer withdrew it before it was finalized.

Recommendations by the EMA are usually given final approval by the European Commission.

Patients with lipoprotein lipase deficiency, the inherited disease Glybera is intended to treat, often cannot eat a normal meal because it can lead to an extremely painful inflammation of the pancreas. Many patients with the disorder have a very restricted diet and only eat a fraction of their daily recommended calories. The condition affects only one to two people per million.

Jorn Aldag, CEO of uniQure, said the company was developing similar treatments for other diseases beyond rare conditions, including Parkinson's. "We believe that just like antibodies, gene therapy will one day be a mainstay in clinical practice," he said in a statement.

Benstetter said there are no other gene therapy treatments currently under consideration by the EMA and was unsure if the agency would get more applications based on Glybera's approval.

"The regulatory pathway is there for gene therapy," she said. "The criteria are the same for gene therapy as for any other medicines. ... Ultimately the companies have to demonstrate the benefits outweigh the risks."

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