First gene therapy to go on sale in Europe in 2013

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Dutch biotech company uniQure said Monday it would start selling the first human gene therapy to be approved in the West by mid-2013 and predicted an explosion of similar therapies to come.

The European Commission approved Glybera on October 25, making the drug for treating the extremely rare disorder lipoprotein lipase deficiency (LPLD) the first to be approved for sale in Europe or North America.

"We believe that after Glybera's approval gene therapy is at the beginning of a period of rapid growth similar to the development of the antibody business in the last decade," uniQure chief executive Joern Aldag said in a statement.

Gene therapy works by modifying a patient's DNA to combat a specific disease, and has been experimented with to treat everything from blindness to depression and brain wasting diseases.

But the relatively unknown treatments have struggled to obtain regulatory approval in the West, although authorities in China approved a gene therapy for treating head and neck cancer as long ago as 2003.

Gene medicine burst on the medical scene in the late 1990s and is one of the most alluring areas of biotechnology, offering the theoretical promise of blocking or reversing inherited disease.

But this new frontier has also been hit by occasional setbacks, notably an unexpected or uncontrollable response from the immune system.

So far, successes have been few, limited to single-gene disorders—as opposed to complex multi-gene disorders that account for the commonest diseases.

Setbacks included the tragic death of an 18-year-old US volunteer, Jesse Gelsinger, in 1999, and the development of cancer among two French children treated for "bubble baby" syndrome, a chronic lack of immune defences.

Glybera treats LPLD, an inherited disease that affects around one or two people in a million, by preventing them from metabolising certain fat particles in the blood.

"First commercial sales of Glybera in Europe are expected in summer 2013," uniQure said, with the company also seeking approval for the drug in North America.

The company said it was developing a raft of other gene therapies to treat diseases including blood clotting disorder haemophilia B, metabolic disorder acute intermittent porphyria, central nervous system disorder Parkinson's and enzyme disorder Sanfilippo B.