Gene therapy to be authorised for first time in EU

Geoff Watts

London

The European Medicines Agency has recommended that alipogene tiparvovec (marketed as Glybera) be authorised for sale in the European Union. Intended to treat lipoprotein lipase deficiency in patients with severe pancreatitis, it is the first gene therapy in Europe to be recommended for this status and is yet to be approved in the United States.

The deficiency is a genetic disorder thought to affect no more than one or two people in a million. The new treatment relies on an adeno-associated virus to deliver functional copies of the lipoprotein lipase gene to the body’s muscle cells.

“It’s fantastic news for everyone involved in gene therapy because it gives a boost to the whole field and is particularly good for attracting investors,” said Deborah Gill, an Oxford University scientist who works on treatments for cystic fibrosis.

“There are a lot of people doing developmental work in gene therapy,” she added, “but to have something taken right through to market authorisation is huge step.”

People trying to manage lipoprotein lipase deficiency through strict dietary fat reduction face a daunting task, and many have life threatening episodes of pancreatitis, requiring hospital admission.

Glybera’s passage through the agency has been anything but smooth. First submitted in December 2009, the application was initially turned down by the agency’s Committee for Medicinal Products for Human Use and by its Committee for Advanced Therapies. After reconsideration in October 2011, the members of the second committee but not the first changed their minds.

Tomas Salmonson, acting chairman of the Committee for Medicinal Products for Human Use, said, “Our established ways of assessing the benefits and risks of Glybera were challenged by the extreme rarity of the condition and also by uncertainties associated with data provided.”

It was only when the European Commission asked the agency to re-evaluate the application with respect to use of the treatment only for patients facing the most severe or frequent attacks of pancreatitis that both committees gave their agreement.

Even now the company that markets the treatment, the Dutch biotechnology company UniQure, will have to provide the agency with data from a registry set up to monitor outcomes in patients undergoing treatment. Price will also be a problem, with Glybera probably turning out to be among the industry’s costliest ever products.

The next development in gene therapy for a more common condition is likely to depend on the outcome of research currently being conducted by the UK Cystic Fibrosis Gene Therapy Consortium. Gill, a member of the consortium, told the BMJ that she expects the results of the current study, which uses a non-viral vector to transfer the gene, to be available in 2014.

“Then it’s all a question of how rapidly we can take it into a phase III trial,” she said. If all goes well, a product could be ready for licensing well within the next decade. Meanwhile, she and her colleagues view the authorisation of Glybera as a great morale booster.

Cite this as: BMJ 2012;345:e5009

© BMJ Publishing Group Ltd 2012