First ever gene therapy drug comes at high price

The Western world's first gene therapy drug is set to go on sale in <u>Germany</u> with a 1.1 million euro (\$1.4 million) price tag, a new record for a medicine to treat a rare disease.

The sky-high cost of Glybera, from Dutch biotech firm UniQure (QURE.O) and its unlisted Italian marketing partner Chiesi, shows how single curative therapies to fix faulty genes may upend the conventional pharmaceutical business model.

After a quarter century of experiments and several setbacks, gene therapy is finally throwing a life-line to patients by inserting corrective genes into malfunctioning cells – but paying for it poses a challenge.

The new drug fights an ultra-rare genetic disease called lipoprotein lipase deficiency (LPLD) that clogs the blood with fat. The medicine was approved in Europe two years ago but its launch was delayed to allow for the collection of six-year follow-up data on its benefits.

Now Chiesi has filed a pricing dossier with Germany's Federal Joint Committee, or G-BA, which will issue an assessment of the drug's benefits by the end of April 2015. The company is seeking a retail price of 53,000 euros per vial, or 43,870 euros ex-factory.

That equates to 1.11 million euros for an typical LPLD patient, averaging 62.5 kg in clinical trials, who will need 42 injections from 21 vials. This price will be subject to a standard 7 percent discount under Germany's drug pricing system.

Read full, original article: First gene therapy drug sets million-euro price record