

\$1 million for a drug used once

This is a story of precision medicines and their value.

The drug is alipogene tiparvovec (aka Glybera). Approved in 2012, it is “a medicine widely heralded as the “first gene therapy” in the Western world” – a treatment to correct DNA.

But when the Berlin physician Elisabeth Steinhagen-Thiessen wanted to give a patient Glybera last fall, it wasn't so easy. She says she had to prepare a submission as thick as “a thesis” for German regulators and then personally call the CEO of DAK, one of Germany's large sickness funds, or insurers, to ask him to pay the \$1 million price tag.

Last September, she gave 40 injections to the muscles of a 43-year-old woman with an ultra-rare disease called lipoprotein lipase deficiency. [...] Her patient had been hospitalized more than 40 times.

A dose of Glybera contains trillions of viruses harboring correct copies of the lipoprotein lipase gene. And Steinhagen-Thiessen says the treatment, at Charite Hospital in Berlin, was a success. The woman hasn't been back to the emergency room since the treatment and is now “living like you and me.”

But this single use of the drug just proves that Glybera is a flop. The problem is its staggering million-dollar price tag, too few patients, and questions about how effective it is. ...

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