Single shot genetic cures are coming. Will insurance companies pay for these multimillion dollar therapies?

Some of Steven Pipe's hemophilia patients consider themselves cured. In a trial Pipe led from 2018 to 2021, they received a one-time gene therapy meant to override a DNA mutation that causes spontaneous bleeding episodes, some of them severe and life-threatening. Unlike most drugs, which relieve symptoms, gene therapy addresses the underlying cause of a disease. Thanks to the treatment, they haven't had to worry about serious bleeding for years.

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The therapy, called Hemgenix, <u>gained US approval</u> from the Food and Drug Administration on November 22 to treat patients with severe hemophilia B. Shortly after its approval, CSL Behring, the pharmaceutical company commercializing the drug, announced its price: \$3.5 million for a one-time dose. It's now the most expensive drug in the world. As part of a research study, Pipe's trial participants didn't have to pay for the therapy. But future patients and their insurers will.

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While most patients never pay the full cost of drugs out of pocket, people who are uninsured and those with high-deductive plans may have trouble accessing these therapies. CSL Behring says it plans to offer discounts, and other makers of gene therapies have also set up patient assistance programs.

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