

\$500K gene therapy treatment? Some see a bargain

The idea behind gene therapy is that genetic material is used as a “living drug” to treat disease. Scientists have been trying to get gene therapies to work for decades, so Kymriah’s approval is historic. But the therapy’s \$475,000 price tag was immediately met with alarm and criticism from patient advocates. More gene therapies are in development, and those too could cost hundreds of thousands of dollars. And while some see sticker shock, others see a bargain.

That’s because many gene therapies promise to be outright cures with a one-time injection, a fundamentally different approach to medicine.

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[T]he average annual cost of health care for sickle cell patients ranged from \$10,704 for children up to nine years old to \$34,266 for those aged 30 to 39 years. Lifetime health-care costs for a 45-year-old with sickle cell disease reached \$953,640. Earlier this year, biotech company Bluebird Bio [reported](#) that its experimental gene therapy cured a teenage boy in France.

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One question that will remain even after some of these therapies get approved is how long their effects last. “The minute we launch a drug, we usually don’t have a full picture of what the long-term effect of the drug is,” [Ed Schoonveld, a drug pricing and market access expert] says.

The GLP aggregated and excerpted this blog/article to reflect the diversity of news, opinion, and analysis. Read full, original post: [Even at \\$500K, Gene Therapy Could Be a Bargain for Some Diseases](#)