$4.25 million: World’s most expensive drug targets genetic disease that disables and kills toddlers. Will anyone pay for it?

There is a new most expensive drug ever—a gene therapy that costs as much as a Brooklyn brownstone or a Miami mansion, and more than the average person will earn in a lifetime.

Lenmeldy is a gene treatment for metachromatic leukodystrophy (MLD) and was approved in the U.S. on [March 18]. Its maker, Orchard Therapeutics, said [on March 20] the $4.25 million wholesale cost reflects the value the treatment has for patients and families.

No doubt, MLD is awful. The nerve disorder strikes toddlers, quickly robbing them of their ability to speak and walk. Around half die, the others live on in a vegetative state causing crushing burdens for families.

But it’s also incredibly rare, affecting only around 40 kids a year in the U.S.

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It means even at this price, selling the newest DNA treatment could be a shaky business. “Gene therapies have struggled commercially—and I wouldn’t expect Lenmeldy to buck that trend,” says Maxx Chatsko, founder of Solt DB, which gathers data about biotech products.

Call it the curse of being the world’s most expensive drug.

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