Million-dollar gene therapies offer salvation for many patients but pose financial challenges for government-funded health care systems

Over the past decade, wealthy countries have devoted a growing share of their pharmaceutical budgets to paying for expensive drugs that treat a tiny fraction of their citizens. Their spending on so-called specialty drugs, which treat rare and often serious diseases and can come with enormous price tags, now represents about half of their drug expenditures. And it is expected to keep growing.

“The situation simply isn’t sustainable,” said Ruth Lopert, a health economist at George Washington University. “Countries simply aren’t going to be able to afford these products, at the prices that are being asked.”

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Record-setting price tags for gene therapies have largely escaped the criticism that has followed other industry pricing decisions. The sentiment reflects just how powerful many of the gene therapies are — doctors sometimes go as far as to call them cures — and their unique position as one-time treatments. Such a therapy has just one chance to earn money, and in some cases can replace chronic treatments that would otherwise be given for the rest of a patient’s life at a much higher cumulative cost.

Still, for middle-income countries, “if benefits of these therapies are immediate in terms of health but the potential savings happen in the future, that math may not work for them,” said Rena Conti, a health economist at the Questrom School of Business at Boston University.

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