

How to pay for gene therapies in developing nations



[Madeleine Armstrong](#)



With rich countries like the US finding it hard to fund gene therapies, it is worth asking whether these projects will ever reach patients in developing countries. And if they do how will companies cope?

Gene therapies are hot right now, with their developers taking aim at everything from haemophilia to rare eye diseases. While many groups are looking at diseases prevalent in rich nations, some are also targeting disorders largely seen in poorer areas, such as sickle cell disease.

Those companies developing projects that could prove valuable in the developing world might soon come under pressure to provide these cheaply. That might be possible for big groups like Novartis, but gene therapy is dominated by smaller players, which could struggle to sustain such a loss leader.

Suggested strategies for broadening the reach of gene therapy into poorer nations include two-tier pricing, flying some patients to better-off countries for treatment, and the development of more convenient, off-the-shelf options. Each of these comes with its own issues, however.

Discrepancies in healthcare between rich and poor nations are nothing new, but the potentially curative nature of many gene therapies heightens the moral conundrum that companies will face if and when these projects get to market.

“What makes gene therapies different is that someone is going to be saying: I can fix this in your child forever and yet you can’t get it because it costs too much,” Arthur Caplan, a specialist in bioethics at the NYU School of Medicine, told *Vantage*.

“I fear what’s going to happen is we’ll see treatments rolled out in the US, and the poor [countries] will go without until ways are found to drive the cost down,” he continued. “Is it fair? No. Is it likely? Yes.”

Two tiers

One way of driving down costs could be a two-tier pricing system, with companies providing gene therapies for a much lower cost in developing nations. This solution was proposed by Bayer’s chief medical officer, Mike Devoy, on the sidelines of the Accessibility to High Value Medicines Symposium in London earlier this year.

“I think the industry recognises it has a responsibility that goes beyond just the richest countries,” he told

Vantage. “Bayer will be looking at how much tiered pricing can play a role in ensuring wider access.”

Still, this has not always worked out in the past, with HIV medicines earmarked for Africa being diverted to Europe, for example.

And Mr Devoy conceded: “If you tier pricing based on ability to pay that has to be respected by the richer countries that are going to be paying higher prices.”

Meanwhile, Mr Caplan was scathing about the two-tier idea. “Having a two-tier price system has worked for drugs and some vaccines, but I don’t think it’s going to work for gene therapy,” he said. “It’s is too expensive. You’d have to drop the price by a factor of 10.”

As for whether this would be possible for the current gene therapy players, he added. “The providers are generally small companies, not giant pharmaceuticals where they can say: it’s going to cost us a billion dollars but we’ll eat it.”

Logistics

Even if the price were low enough, logistical concerns could make gene therapy unfeasible for developing countries. “There are often technical issues like refrigeration and having a hospital that can do IV administration and monitor and handle adverse events – that costs money, too,” Mr Caplan said.

This means that autologous candidates, which make up much of the first wave of gene therapies, probably would not be suitable for the developing world. The most advanced sickle cell gene therapy candidate, Bluebird Bio’s Lentiglobin, can take up to three weeks to produce from a patient’s own cells.

An allogeneic gene therapy that could be delivered quickly could be ideal for lower-income countries. However, such a candidate is a long way from reality, and the other sickle cell gene therapies in human trials are also autologous.

Bluebird has another autologous candidate, known as BCL11a shRNA^{miR}, which yielded data from one patient at [last year’s Ash meeting](#). Others include [Sangamo/Sanofi’s BIVV003](#) and [Aruvant’s RVT-1801](#). At least the latter is designed to be used with a less intensive conditioning regimen, which could reduce adverse events and time in hospital, and therefore costs, so it is hoped that this could be more suited to resource-poor areas.

Another potential approach to getting gene therapies to patients in poorer countries would involve flying them to western nations for treatment, Mr Caplan said. But this would only work in very rare conditions and would barely make a dent in more common disorders like sickle cell disease. “There are thousands and thousands of sickle cell disease cases in sub-Saharan Africa. You’re not going to handle that by charity.”

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