Should gene therapy patients be asked to pay?

The price in value-based payment deals should by definition be justified, so why is patient cost-sharing part of the calculation? Spark covers this cost, but there is no guarantee that all gene therapy companies will.

As Novartis’s gene therapy Zolgensma nears a US FDA decision, the sector has only a vague idea of how much the Swiss group will charge for the spinal muscular atrophy (SMA) treatment, other than it will be a number followed by six zeroes. Just as importantly, the families with children suffering from this fatal condition do not know what, if anything, they will be asked to pay. However, there are some clues from the way in which the first US-approved gene therapy, Spark Therapeutics’ Luxturna, was financed.

After arriving at a “value-based price” with payers, Spark has offered to pay all of the cost-sharing for patients with an inherited form of blindness who are covered by commercial plans. While this has worked for Spark and its patients, it is not clear whether other companies will do the same, or indeed whether they can afford to do so over the long term.

This raises an important question about insurance benefit design: namely, if biopharma companies and insurers have agreed to a price that represents good value for money, should the patient have to pay a single penny?

One of the main reasons that health insurers impose cost-sharing requirements on enrollees is to discourage patients from seeking unnecessary treatment, but there is little risk of this with gene therapies. The population is, by definition, genetically defined and in need of treatment, so specialists should easily be able to identify eligible patients and exclude ineligible ones.

In the case of gene therapies subject to value-based reimbursement agreements “it’s hard to justify a substantial copay”, Mark McClellan, a Duke University professor of business, medicine and policy, told Vantage. Mr McClellan, a former US FDA commissioner and administrator of the Centres for Medicare and Medicaid Services (CMS), worked with Spark and insurers to develop the company’s reimbursement strategy.

Affordable

Spark’s chief commercial officer, Ron Philip, told Vantage the group decided that patient costs, which it had forecast based on the Affordable Care Act deductible limits of $7,000 for individuals and $14,000 for families, would be an expenditure that it could afford to cover. After all, the condition that Luxturna treats, biallelic RPE65 mutation-associated retinal dystrophy, likely affects no more than 2,500 people in the US, and Spark
had a good idea how many were covered by commercial insurance.

Mr Philip said all but one patient in commercial coverage accepted the cost-sharing offer, and the one who turned it down did so to make the revenue available for another patient. So far, the company says it has sold 75 vials, and at a single vial per eye this means that at most 38 patients have received Luxturna. Vantage spoke to Mr Philip before Roche’s $4.8bn takeout of Spark (Roche buy-in is another vote of confidence in gene therapy, February 25, 2019).

If Spark is simply covering patients’ cost-sharing it is plausible to wonder why the group does not simply cut the price of Luxturna by, in the example Mr Philip cited, $7,000-14,000. But he responded: “Then [cost-sharing] would be a percentage of the new price. It would always ride down.”

**Barriers**

Some payers are open to the idea of waiving cost-sharing for gene therapy, but there are barriers. Michael Sherman, chief medical officer of Harvard Pilgrim Health Care, which has a value-based deal in place with Spark, acknowledged that the behaviour-modification aspects of cost sharing did not apply in the case of gene therapy. However, cost sharing can also be a financial tool that, more broadly, helps to keep premiums lower.

In addition, cost sharing provides a revenue stream that keeps insurance companies solvent. In the case of Harvard Pilgrim, its not-for-profit status means that it does not have to worry about generating a return for shareholders but, on the other hand, it cannot afford to lose money if it wishes to remain in business, Mr Sherman pointed out.

There are also administrative and legal barriers to waiving cost sharing for specific drugs, but Mr Sherman said: “I think they’re all solvable.”

He added that the ability to waive cost sharing could be useful to insurers and payers as they negotiate a value-based price. “I’d love to say, ‘If you price it there we will waive the cost share.’” This, of course, requires biopharma companies to be willing to give just a little bit more on price to help patients.

**Long-term implications**

The effects of cost-sharing waivers for gene therapies on premiums or company margins might not be material now, Mr Sherman said. After all, the patient populations for RPE-associated blindness and SMA are miniscule compared with major indications like cardiovascular disease or diabetes (Tiny populations up the ante for gene therapy pricing, December 20, 2018).

But, with more than 50 gene therapies in the clinic and sales expected to exceed $13bn in 2024, payers and insurers are right to be nervous about whether it is affordable to cover cost sharing. Indeed, Mr Sherman noted that the outlook was a concern for the long-term financing of gene therapies in general.

Referring to the cost-effectiveness body Icer’s affordability threshold of $150,000 per quality-adjusted life year gained, he said: “If every time you’re adding a year of life it costs $150,000, that can be material.”

He concluded: “If you look at the pipeline in the next three to five years it’s manageable. I think in maybe five years we’re going to start running into problems.”

Mr Sherman was complimentary towards biotech companies that have tried to reach reimbursement agreements with insurers, citing Spark and Bluebird Bio as being proactive. “For these conditions this is not about saying no. This is about saying yes without creating affordability problems,” he said.

But big pharma, which is becoming increasingly interested in gene therapy, might not be so amenable. Novartis acquired Zolgensma with its takeout of Avexis, Roche scooped up Luxturna and other assets with Spark, and Pfizer has been making deals including last week’s €45m ($51m) option on the private French company Vivet Therapeutics.

“My sense is that bigger pharma has been less innovative with some of these [value-based] agreements,” Mr Sherman said. “We negotiated with Spark when they were an independent company. I don’t have enough experience to know whether I should worry about bigger pharma.”