Evaluate Vantage

January 22, 2018

Biotech gets creative about the pay cheque

Madeleine Armstrong

Amid a backlash over the high prices of advanced therapies, biotech companies are coming up with new payment models.

The price of cell and gene therapies was one of the hottest topics at the JP Morgan healthcare conference earlier this month. And Sarepta came out swinging, insisting that it was not part of the problem, and defending high prices for potentially once-and-done approaches.

Still, many other companies have decided that discretion is the better part of valour, and have taken a placatory stance. But most now agree that, without a creative stance on how treatment is paid for, gene therapy might have a limited future as an investment proposition.

Sarepta put the blame for recent bad publicity about drug pricing squarely on "non-innovators". The company's chief executive, Doug Ingram, refused to name names, but a spate of January price increases for ageing drugs will have escaped nobody's attention (*Few storms for biopharma to weather in 2017, January 3, 2018*).

"[Gene therapy] isn't actually where the healthcare system is creaking," he told *EP Vantage* in an interview on the sidelines of the JP Morgan meeting. "I think we need to get a better handle on price increases in areas that are non-innovative."

Ironic

"I find it a little ironic that this is the major focus of talk about healthcare cost containment," he said. "Spark comes out with a fascinating gene therapy programme, and everyone wants to focus on how they price it; the truth is that it will have a minimal impact on the healthcare system."

Sarepta itself is working on several gene therapies for Duchenne muscular dystrophy, and its two most advanced programmes, targeting micro-dystrophin and GALGT2, should yield preliminary results from their first-in-human studies in mid-2018.

Mr Ingram's attitude is perhaps not surprising given that, for Sarepta investors to part with their hard-earned cash, they must have faith that rare disease therapies can continue to command high prices. But he maintained that, while healthcare costs are an important topic, payers should not throw the baby out with the bathwater. "Whatever fix we have, we want to make sure that it rewards innovation," he stated.

Risk sharing

Still, the trend towards outcomes-based pricing – something Mr Ingram conceded "probably has its place" – looks like it is here to stay if comments from other groups presenting at JP Morgan are anything to go by.

Spark defended its $850,000 price tag for the first US-approved bona fide gene therapy, Luxturna, highlighting its outcomes-based rebate arrangement with the insurer Harvard Pilgrim (*Snippet roundup: Spark sets record price tag and Biontech brings in big bucks, January 5, 2018*). This could pave the way for similar agreements with other payers, with which Spark said it is in advanced discussions.

A few months ago, in launching Kymriah, the world's first CAR-T therapy, Novartis announced a no-remission-no-pay pricing model. Glaxosmithkline has risk-sharing agreements in EU countries where its gene therapy Strimvelis is available.

According to Celgene's chief executive, Mark Alles, these kinds of deals might soon become the norm. "I think we're getting into an environment where you can expect risk-based pricing models," he told attendees at the group's JP Morgan breakout session, just after praising the rise of innovative reimbursement approaches alongside scientific innovation. He was talking specifically on the topic of CAR-T therapy, where Celgene yesterday increased its bet by buying its partner Juno outright.

Annuity payments?

Meanwhile Emil Kakkis, chief executive of the rare disease specialist Ultragenyx, spoke in favour of multiple years of "annuity-type payments" rather than a one-off sum for gene therapy. "This more than $1m per injection thing – the PR looks bad," he admitted during the group's breakout session.

Smaller, regular payments would give a bigger overall return than $1m per patient, he reckoned, "but spread out over time, which is better for the company, better for the health system, and also you can structure it so that if treatment stops working payment can be stopped."

Overall Mr Kakkis seemed optimistic, insisting: "If you treat patients and do great things for them, there's always a way to get it paid for."

Not everyone agrees that gene therapy holds the key to all diseases. Sanofi's purchase of the haemophilia player Bioverativ was a bet on the continued dominance of traditional factor-replacement products (*Sanofi bets against haemophilia gene therapy, January 22, 2018*).

The next few years should show whether cell and gene therapy can become commercially successful and justify the billions spent on CAR-T developers like Kite and Juno. One thing is already becoming clear: to live up to expectations gene therapy players will need to get creative about how they get paid.

To contact the writer of this story email Madeleine Armstrong in London at madeleinea@epvantage.com or follow @ByMadeleineA on Twitter