Evaluate Vantage

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Sarepta and biopharma want to take, but how much will they give back?

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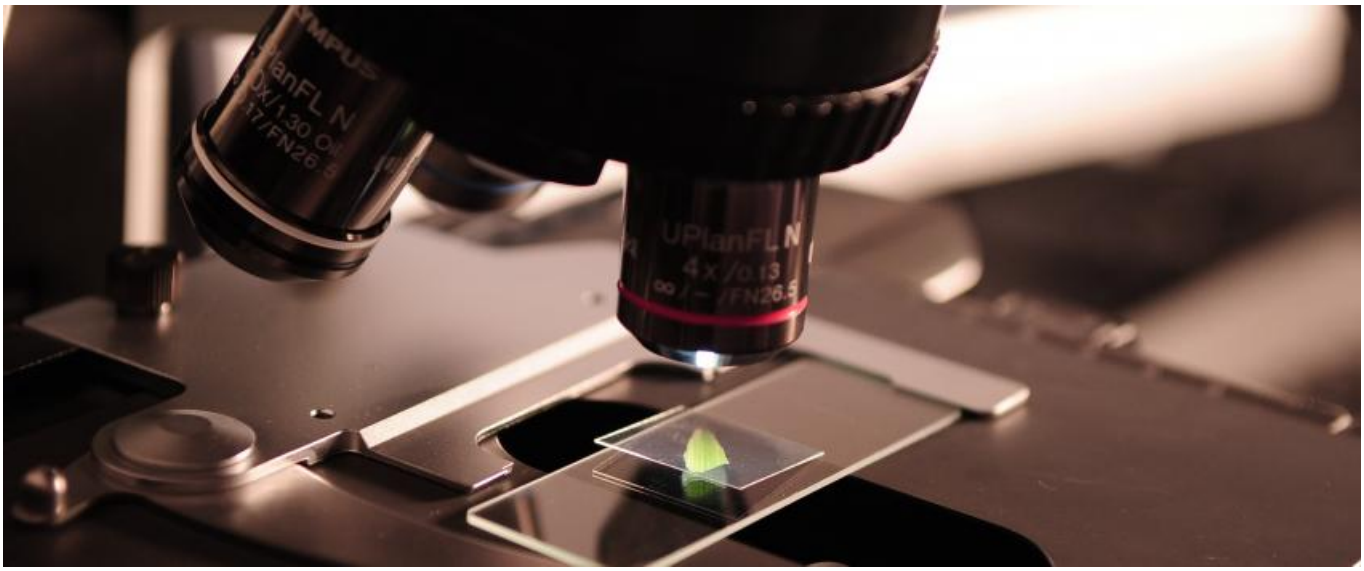

Biopharma companies have benefited from public funding for their early research, so do they have the right to charge high prices for these projects?

Sarepta has benefited from a US ecosystem that offers government support for basic research, financial incentives for R&D and legal protection from competition. As the group could soon have a life-changing gene therapy on the market it must be challenged to come up with a price that does not stretch the finances of the families it likes to spotlight in its public relations.

However, if past gene therapies are anything to go by, Sarepta's Duchenne muscular dystrophy treatment will cost above $1m. And, as the therapy uses vector numbers higher than ever before, the company might try to make a case for an even higher price to wring out a profit – something that will ratchet up the pressure to reciprocate on its end of the social contract.

If the price issue gains traction Sarepta's margins could be squeezed severely. Indeed, few appreciate the costs and logistical problems in dosing AAV vectors in quantities used in Sarepta's trial: so far data on up to 1014vg/kg have been presented, and the trial aims to go up to 2x1014vg/kg (*Vantage point – The $100,000 problem gene therapy companies would rather not mention, June 25, 2018*).

Economic protection

Sarepta has already come under fire for the $300,000 a year it charges for its marketed DMD product, Exondys 51.

But it is by no means unique here; all biopharma groups benefit from the protection from competition offered by US FDA regulation and intellectual property law, along with taxpayer-funded research through public universities and the NIH, all the while pocketing the profits.

There is a parallel here with CAR-T therapies brought to market by Novartis and Gilead. These are expensive and complex cell therapies, and much of the early development work – before biopharma bought in – originated at the NCI, a government body, though in Novartis's case the route taken was somewhat circuitous.

David Mitchell, founder and president of Patients for Affordable Drugs, has argued that there is a special obligation on companies that develop drugs funded largely by the taxpayer (*CAR-TCR Summit: Industry and patients clash on cell therapy cost, September 8, 2017*). But, in spite of the outcry, the cost of CAR-T therapies

has not come down.

Sarepta makes for an interesting case study, and already has form with its previous DMD drug, the now-marketed exon-skipping agent Exondys 51. The science behind Exondys 51 was developed at public research universities like the University of Western Australia, Imperial College London and Leiden University in the Netherlands – all countries where Exondys is not approved – and the Western Australia team received NIH funding.

On the regulatory side, Sarepta got a lucky break when US FDA officials overlooked its modest efficacy in increasing dystrophin levels. An advisory committee had recommended against Exondys 51, and a top-level FDA reviewer described it as an "elegant placebo" – and approved it. This surprise decision enabled a massive fund raising.

Alongside this, a list price of $300,000 a year makes Exondys 51 an expensive proposition even for families with good health insurance. The average co-insurance for speciality drugs was 27% in 2017, according to the Kaiser Family Foundation and Health Research and Education Trust annual survey, meaning at the list price of $25,000 a month the average family would be facing $6,750 a month in drug costs until it reached its plan's out of pocket maximum.

Sarepta has a programme that helps defray patients' out-of-pocket costs, but it does not disclose how much it spends on this. Product revenues of $155m in 2017 were reported net of discounts and patient assistance.

Public support

Meanwhile, the gene therapy asset that Sarepta highlighted at its investor R&D day last week is another example of how the group is benefiting from public goodwill: it grew out of work by Dr Jerry Mendell, an Ohio State University professor practising at Nationwide Children's Hospital in Columbus *(Sarepta investors party like it's 2015, June 19, 2018).*

Like the University of South Australia and Imperial College London, Ohio State is a publicly supported research institution. Professor Mendell has received $2m in NIH funding to test gene therapy in DMD.

The Nationwide hospital is a not-for-profit institution that has serving the public as part of its mission. Its status means that it pays no taxes on its positive margins, which amounted to $307m on patient revenue of $1.3bn in 2016; it had a cash reserve of $224m.

And, to help further its educational and research work, it receives add-on payments from its Ohio Medicaid fees – Ohio spent $100m across all teaching hospitals in the state in 2015.

Sarepta also got a boost from charitable money in the form of funding for the gene therapy trial from the Parent Project Muscular Dystrophy (PPMD), to the tune of $2.2m, along with DMD families and foundations. The PPMD grant "was a key component to help expand and accelerate this opportunity", Sarepta said in a press release when the FDA granted permission to begin dosing in patients.

Bold

After Sarepta received so much public benefit, asking patients to pick up tens of thousands of dollars in costs seems bold. And families could be footing an even bigger bill if the group's gene therapy reaches the market.

There is an argument that the once-and-done nature of gene therapy justifies a higher price tag, as the cost could ultimately be similar to or even lower than that for chronic therapy over a long period of time.

But the nature of gene therapy pricing has many discussing the idea of a mortgage or annuity-type financing structure, which suggests that families could be asked to carry additional debt as the price for having of a genetic mutation.

True, the financial, regulatory and legal advantages received by Sarepta and the rest of biopharma have built a robust sector delivering life-changing and extending medications at a fast pace. But this innovation is increasingly being priced out of reach of even those with health insurance.

Biopharma has been responsive to its investors, who want to see high prices and maximised profits. But companies now need to start living up to their end of the social contract and ensure that their products are affordable to as many patients as possible.

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