Drug group Spark to charge $850,000 for blindness gene therapy

Luxturna is most expensive treatment on market and price raises affordability questions

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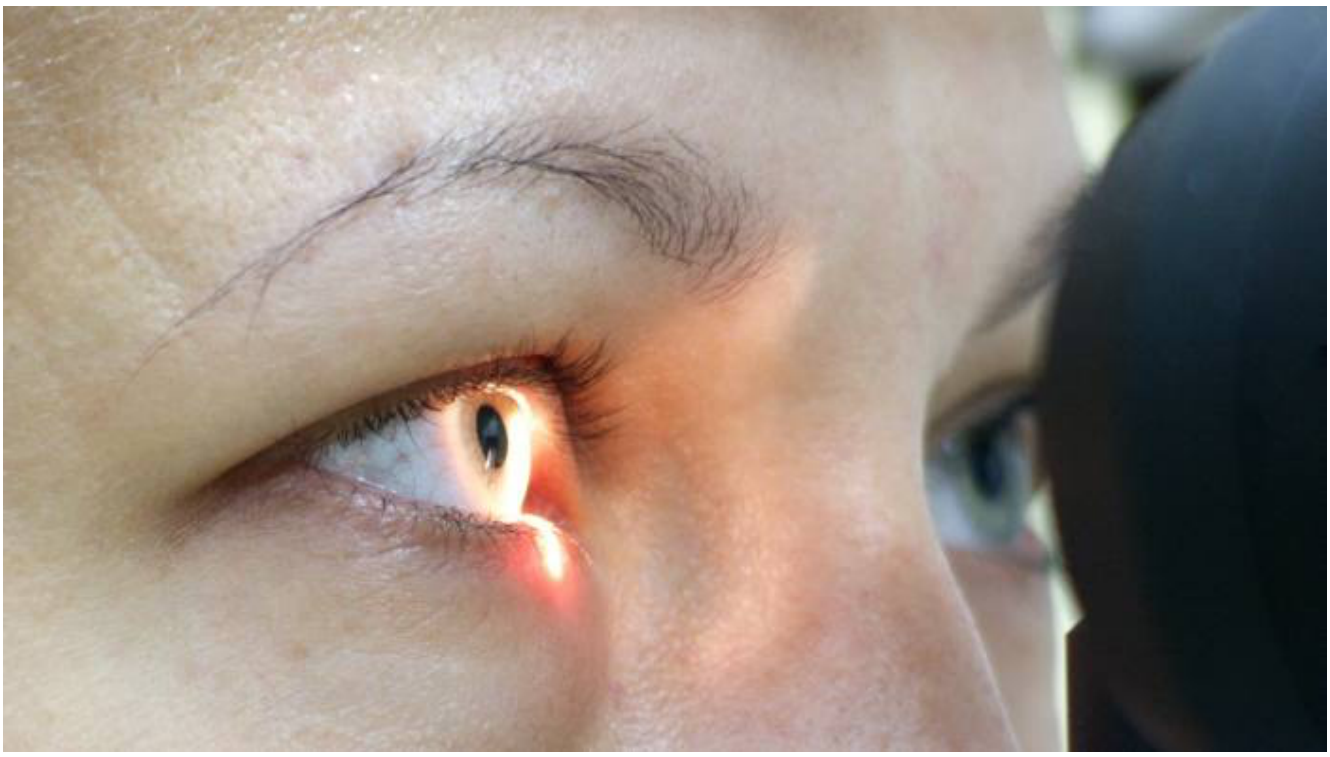

Spark says it will share the risk of the gene therapy failing by paying some health insurers a rebate linked to whether the treatment worked

David Crow in New York

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Spark Therapeutics says it will charge $850,000 for its new gene therapy for blindness, making it the most expensive drug on the market and kick-starting a debate about the affordability of pioneering treatments.

Jeff Marrazzo, Spark chief executive, said the company would charge $425,000 for each eye for Luxturna, which recently became the first gene therapy to be approved by the US Food and Drug Administration.

Even at $850,000, Luxturna is lower than the $1m pricetag the company had been hinting at just a few weeks ago. In an interview last month, Mr Marrazzo said Spark believed "the value of a therapy like this is in excess of $1m".

Spark announced the price for Luxturna as the UK's drug costs watchdog cleared its most expensive treatment ever: a €594,000 gene therapy made by GlaxoSmithKline for children with "bubble boy disease".

Unlike traditional drugs, which tend to be taken for months or years at a time, these gene therapies are intended to be one-off treatments that tackle a disease at its source, repairing faulty DNA so the body can fix itself.

Spark's Luxturna, for instance, involves inserting a functioning copy of a missing gene directly into a patient's eye, where it encourages the body to produce a protein essential for sight. In clinical trials, the medicine restored eyesight in some people with severe visual impairment.

Although Luxturna is intended for a relatively rare type of inherited blindness, several companies are developing similar therapies for more common illnesses. Spark and others are targeting haemophilia while others are focused on other blood disorders.

In the case of GSK's drug, the National Institute for Health and Care Excellence (NICE) said it had approved the one-off procedure for children with adenosine deaminase deficiency, an ultra-rare immune disease that leaves infants vulnerable to infection, forcing them to live in isolation or a "bubble".

Around three babies a year are born with the condition each year in the UK. GSK's London-listed shares added 1.8 per cent on Wednesday.

The advent of single-time gene therapies — and of new cancer cell therapies from Novartis and Gilead that are also administered once — has prompted a debate over how much drugmakers should charge for scientific breakthroughs and whether society can afford them.

Spark also announced what it described as "first of their kind" programmes designed to help employers, the government and patients manage the cost of Luxturna.

The group said it would share the risk of the treatment failing by paying some health insurers a rebate linked to whether the treatment worked in the first 30 to 90 days, and also to whether it was still effective after 30 months.

Harvard Pilgrim, a non-profit healthcare group, which sells health plans in New England, has signed up to the scheme, and Spark said it was in "active discussions with other commercial insurers".

"This shows we stand behind the product's efficacy and the long-term durability of a single dose," said Mr Marrazzo.

Spark said it had also designed a scheme that would allow the drug to be sold directly to insurers or special pharmacies, rather than hospitals or treatment centres, which tend to charge big mark-ups that can prove costly in the case of expensive drugs such as Luxturna.

Express Scripts, a large pharmacy benefits manager that negotiates drug prices on behalf of employers and insurers, said it would work with Spark on implementing such a scheme.

Steve Miller, chief medical officer at Express Scripts, said: "For one-time therapies like Luxturna, we believe non-traditional payment and distribution models are needed."

Spark said that government regulations meant it could not offer to sell the drug under instalment plans that would allow insurers and patients to bear

the cost over time.

However, the biotech group said it was in discussions about designing a pilot instalment scheme with the US Centers for Medicare and Medicaid Services, the federal agency that administers taxpayer-funded healthcare for people on low incomes.

If successful, the pilot could act as a prelude to regulatory changes that might make instalment plans for medicines more common.