Pharmaceuticals sector

Gene therapies test Europe's willingness to pay

Two $2m treatments launch this year but a reluctance to fork out could dim investor enthusiasm

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Sarah Neville, Global Pharmaceuticals Editor 12 HOURS AGO

The drive to cure deadly diseases is about to enter a new and demanding phase as the pharmaceutical industry prepares for the first time to test the appetite for hyper-expensive gene therapies in Europe.

Two treatments with price tags of about $2m will be by far the most costly drugs offered in the region. Not only are the fates of patients — and the outlook for the pharma groups that make the therapies — at stake. The willingness of traditionally cash-strapped European health systems to pay for these innovative treatments will affect future investment in the field.

In the vanguard is Zynteglo, a treatment for rare blood disorder Beta thalassaemia that is manufactured by Bluebird Bio. It launched in Germany this week, a prelude to a wider European expansion.

Also poised to enter the European market is Novartis's Zolgensma, a one-off treatment for the rare childhood disease spinal muscular atrophy, affecting up to 600 new babies a year in the EU. The Swiss drugmaker is likely to secure a positive opinion from the European Medicines Agency's committee for medicinal products for human use — generally the precursor to regulatory approval — early this year. A delay after a request for more information, apparently reflecting the unfamiliarity of the field to regulators, has not dented the company's confidence.

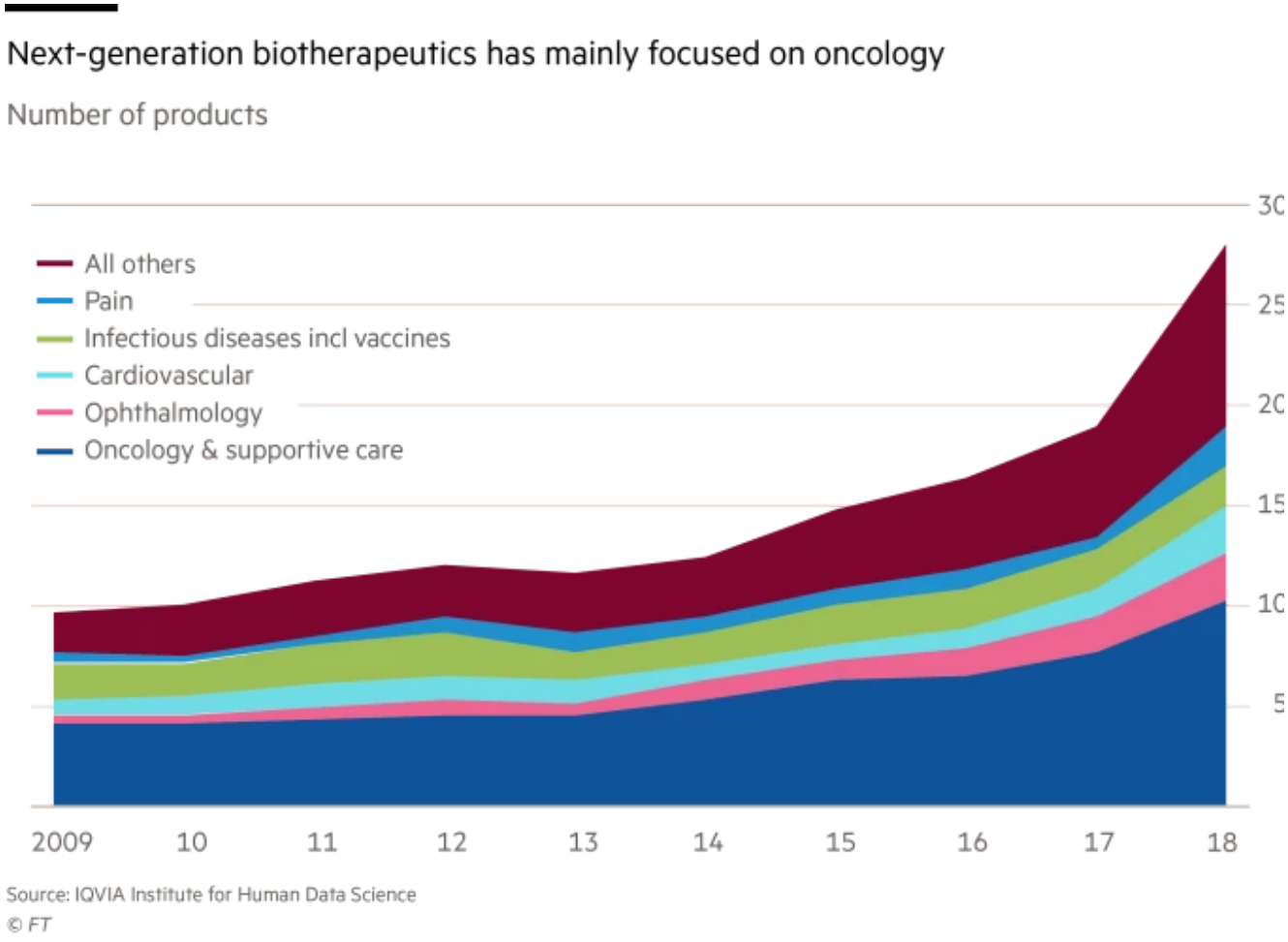
Both treatments have been designated orphan drugs by regulators, in recognition of the rarity of the conditions they treat.

"Next generation biotherapeutics" such as these make up less than 10 per cent of the late stage R&D pipeline, according to the IQVIA Institute for Human Data Science, which charts global

pharma trends. But the number more than doubled in the three years to 2018, underlining a growing sense of momentum in this area.

Last month Roche provided fresh evidence of the field's vitality when it announced the largest ever gene therapy licensing deal of its kind. It will pay Sarepta Therapeutics, a Massachusetts-based biotech, $750m cash up front for the rights to its Duchenne muscular dystrophy gene therapy outside the US, as well as making a $400m equity investment in the company.

In justifying their prices, both Novartis and Bluebird say that existing treatments, over a much longer span, mean healthcare systems already pay far higher bills than for their own one-off, curative therapies.



Yet if European health systems do not adopt the most pricey treatments widely, it could dim investors' enthusiasm for committing substantial funding to the field.

Brad Loncar, chief executive of Loncar Investments and a prominent life sciences investor, pointed out that European governments tended to push back much more heavily against high drug prices than insurers and other payers in the US because many have government-led health systems that impose a tight limit on spending.

A large government payer can also use its scale and reach to drive harder bargains.

Yet it was important the European launches of Zolgensma and Zynteglo went well "because those are very risky things to invest in" and investors needed to be reassured there was "a robust commercial market", added Mr Loncar.

Dave Lennon, president of AveXis, the Novartis subsidiary that makes Zolgensma, bristles when it is suggested that its price — thought to be the highest for a medicine — is the most remarkable thing about it. "I would hope the highest profile aspect of this treatment is that we save children's lives," he said.

Zolgensma's $2.1m price tag was "about half the price of the economic value we believe we're creating", said Mr Lennon, and, taking into account longer-term savings, it was "about half the price of typical rare disease products that are in the market".

We don't see yet a lot of resistance to covering the product for the appropriate patients

Dave Lennon, AveXis president

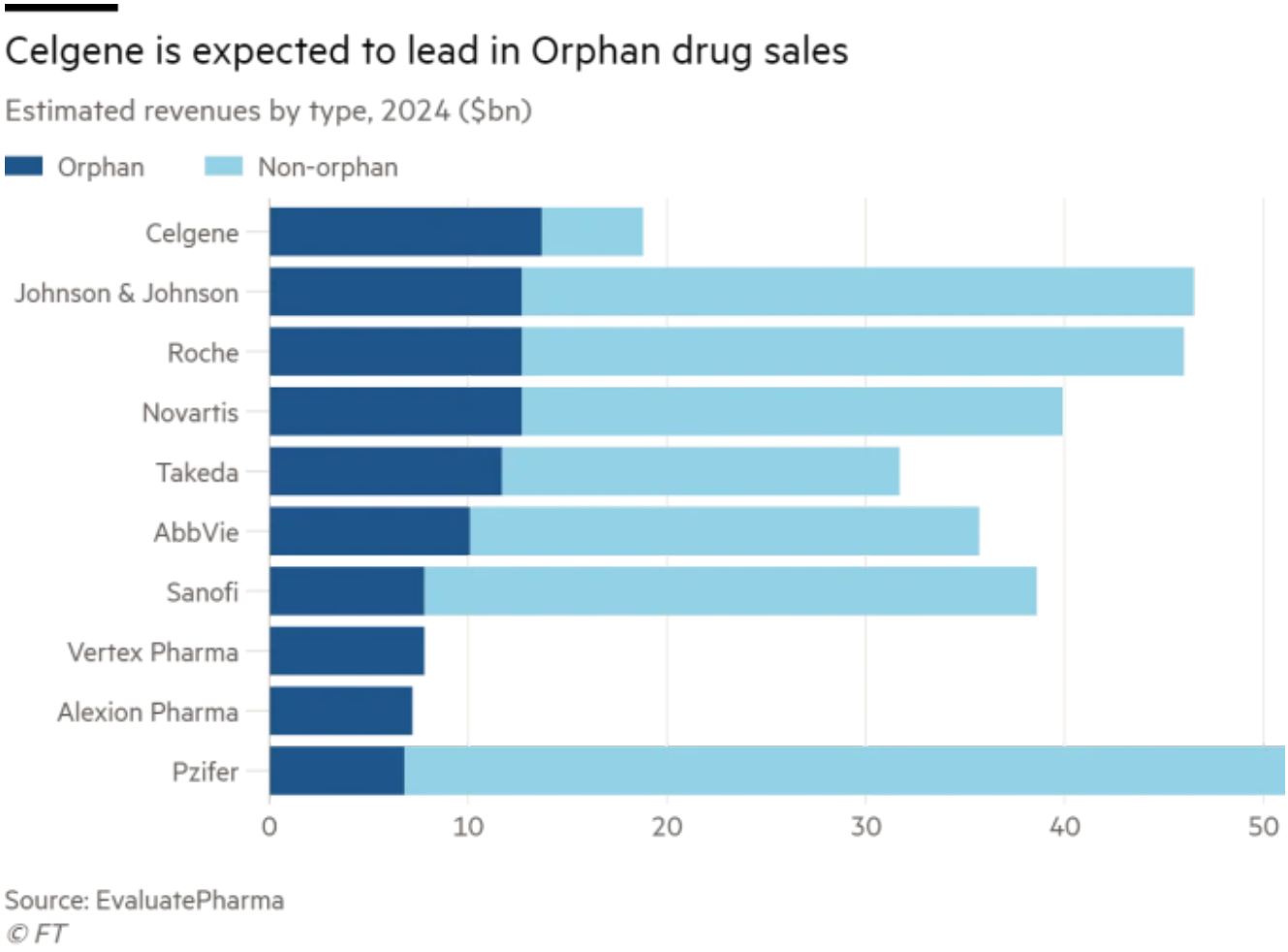
By the time it secured approval from US regulators last May, Novartis said it had spent more than $1bn on developing the drug. By the end of the third quarter of last year, it had netted revenues of $175m from US sales.

The company told investors in October that 90 per cent of commercially insured patients, and about 30 per cent of Medicaid patients, were covered to receive the drug in the US.

The question, however, is whether European health systems, particularly those with a well-established process for determining the cost-effectiveness of drugs, will be able to adapt their models to look beyond the immediate upfront cost and take into account longer-term savings.

Murray Aitken, executive director of the IQVIA Institute for Human Data Science, said: "European countries are a little inconsistent in terms of the extent to which they look at a broader healthcare and social cost budget." Many countries were still "trying to manage within a set envelope of a drug budget", an approach that was appropriate in the 20th century "but it's not at all in the 21st century", he argued.

Asked about the tenor of conversations Novartis was having with European authorities, Mr Lennon said: "When we've had discussions on value, obviously the same initial reaction can often come in terms of the price, but once we go through the health economic arguments, often there's a high level of acceptance and understanding of the opportunity that it provides to actually save the system money and lead to better outcomes for these children.



"So no one's going to stand up and say: 'We love your price.' But at the same time, we don't see yet a lot of resistance to covering the product for the appropriate patients."

Mr Lennon added that rare disease treatments "can often cost between $300,000 and $500,000 per year, and then if you just look at treating a kid say for maybe 10 years you're talking about $3m to $5m".

The starting point in talks about cost is "always to think how do we flexibly partner" with health systems, he said. "One of the ways we can do that is by spreading out payments over time, if that's of interest to a payer or an insurer."

Novartis added that several countries in Europe and the Middle East were in the process of agreeing "early access solutions", allowing some patients to receive Zolgensma even before a marketing authorisation had been granted. It declined to name the countries.

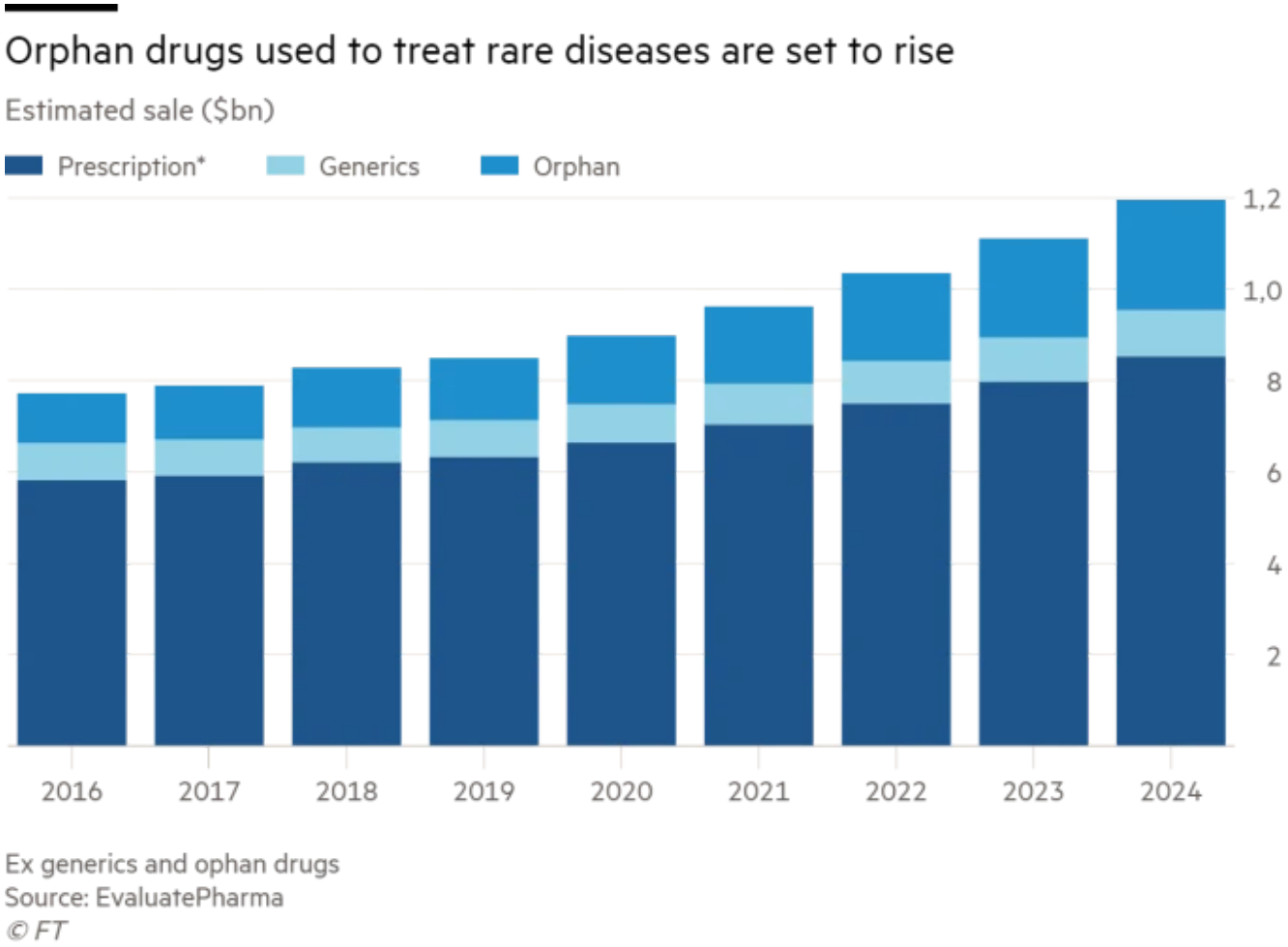
Unlike AveXis, which first launched Zolgensma in the US, Bluebird Bio has chosen to debut its product in Europe, where there is a concentration of Beta thalassaemia patients.

Up to about 9,000 people are thought to suffer from the condition in the four European countries which will be the first to receive the medicine, with about a further 4,000 in the rest of Europe. The potential patient population in the US is roughly 1,500, according to Bluebird.

The company announced on Monday that it had "entered into value-based payment agreements with multiple 'sickness funds', or statutory health insurance funds, in Germany to help ensure patients and their healthcare providers have access to Zynteglo".

However, reimbursement for the $1.8m drug is still not guaranteed in the long term. Bluebird has submitted a dossier to the Joint Federal Committee in Germany, which examines whether a drug is genuinely novel and offers greater benefit than comparable existing treatments. A favourable ruling will be required if the government is to agree to refund the cost of the drug.

Discussions are also under way about launching the drug in the UK, France and Italy, the company said.



Andrew Obenshain, until recently Bluebird's European chief, said payers would fund only the first year of treatment, paying a fifth of the overall price, unless it delivered "transfusion independence". Currently many patients are forced to undergo up to eight transfusions a year to manage their condition.

Describing the drug as "a one-time treatment for a lifetime of benefit", he added: "I think that frames up the conversation very differently with the payers."

Doug Danison, head of access, value and evidence strategy, said that even if patients were able to reduce the number of transfusions they required, "we're saying that hasn't met our bar and therefore they're not paying. So there's a certain amount of benefit that we're giving up there."

The negotiations it has held in Germany have helped to create a template that Bluebird, and potentially other companies, can use or adapt as these expensive but potentially transformative medicines become more prevalent.

"The sickness funds are being as creative as we are," Mr Danison added. "[They] are really coming to the table as well because they see that there's a future gene therapy here and they partnered with us to find a new payment model that works for them as well."