NEWS DIGEST

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Remap

How will governments face the high cost of medical miracles?

Have you read the NY Times article published early this year?

A Mother's plight:

Suhellen Oliveira Da Silva was six months pregnant when she found out that the child she was carrying had the same disease that had left her firstborn paralyzed. But this time, there was a treatment that could make a world of difference. This baby could live a normal life.

The problem was the price: **the treatment cost the equivalent of 1.7 million dollars** and the public health system in Brazil, where the family lives, refused to pay for it.

So da Silva took her case to court — and won. A judge ruled that the government had to buy the therapy for her youngest son, Levi. Today, 2-year-old Levi talks, claps and crawls, things his 10-year-old brother Lorenzo can't do.

The treatment, Zolgensma, a single-dose infusion, is among the first in a new class of gene therapies that offer enormous promise for people with fatal or debilitating diseases—at astronomical prices. Its maker, the pharmaceutical company Novartis, has negotiated deals with national health systems and insurers so that the drug is covered in many wealthy countries.

With Zolgensma, which treats a **rare genetic disorder** known as spinal muscular atrophy, or SMA, experiencing a slowdown in sales, Novartis is pushing for broad coverage in middle-income countries like Brazil, where public health systems are often underfunded. It has become a test of whether such therapies can gain wide coverage around the world.

The impact:

After more than 100 successful lawsuits that forced the Brazilian public health system to pay for the treatment, the government announced in December that it would start covering Zolgensma for babies with more severe cases of SMA later this year. The government has agreed to pay the equivalent of around $1 million for each treatment.

At a hearing on the issue of coverage, one congresswoman, Adriana Ventura, expressed sympathy for families seeking treatment, but said, "We also can't be irresponsible and pass something that's not sustainable in the long run." She added that the concern is that "to give to one, you have to take the basics away from millions of people."

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An analysis by a researcher at Brazil's drug regulatory agency found that court-ordered spending in the first 14 months of Zolgensma's availability in Brazil **they could have solved more than 4 million covid-19 vaccines.**

Among the most expensive therapies are gene therapies that show promise to transform inherited disorders with a single dose. Zolgensma's 2019 US list price of $2.1 million, believed to be the most expensive when it was established, has since been exceeded four times, and many more treatments are on the horizon that are anticipated to be just as expensive.

Worldwide ramifications:

In Europe, a product approved for a deadly neurological disorder known as metachromatic leukodystrophy has received list prices of up to $3.9 million.

Last year, Germany's healthcare system agreed to pay $2.6 million at a discount.

In the United States, biotech company Bluebird Bio set prices last year of $2.8 million when it won approval to treat an inherited blood disorder called beta thalassemia and $3 million to treat a deadly neurological condition known as cerebral adrenoleukodystrophy. When European systems refused to pay what Bluebird was asking, it withdrew the products from the continent.

Record prices for gene therapies have largely escaped criticism of other industry pricing decisions. The sentiment reflects how powerful many of the therapies are and their unique position as single-dose treatments. In some cases, such a therapy can replace chronic treatments that would otherwise be administered for the rest of the patient's life at a much higher cumulative cost.

A numbers game:

Until now, Novartis has booked $3.7 billion in revenue from Zolgensma, charging different prices in different places. The treatment has not become a mega-success in part because so few patients are eligible for it. And sales have started to decline.

Middle-income countries like Brazil could bring many more patients. Novartis has already gained coverage for Zolgensma in Russia, Egypt and, most recently, Argentina. It continues in negotiations in more than 10 countries, including Ecuador.

Brazil's experience with Zolgensma shows the challenges that the breathtaking prices of these therapies will pose for governments and insurers with limited budgets. Those challenges are poised to multiply in the next few years as more such treatments become available for larger groups of patients.

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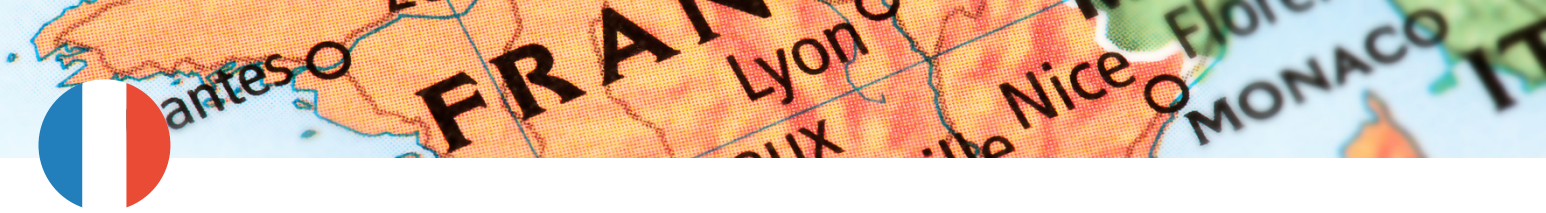


CEPs Annual Report indicates reduced time to market access

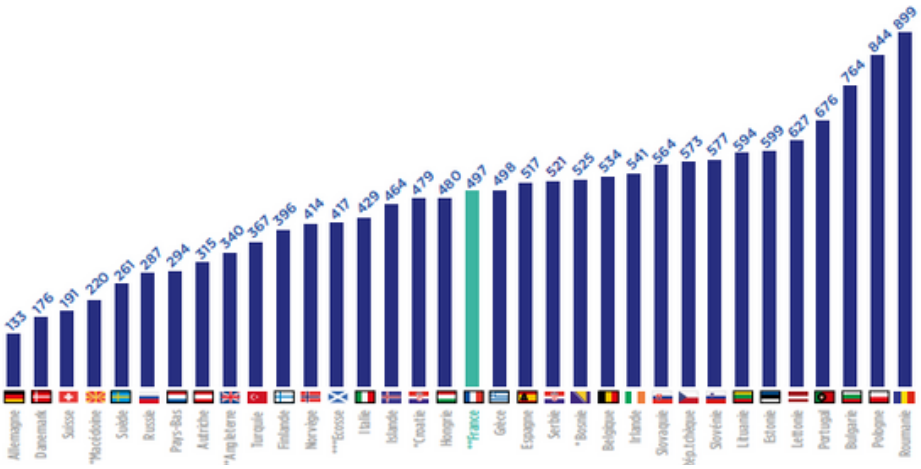
- 497 days average time* to market access for reimbursable medicines in France (period 2017-2020). This has reduced from 527 days in the period 2016-2019.

*time between obtaining the Marketing Authorisation and the publication of prices in the Official Journal

- In July 2021, France initiated a reform to its early access system to facilitate patient access to innovation. 71 early access requests have already been assessed by HAS (July '21-July '22), and more than half were evaluated favourably.
  - 497 days includes products under the ATU system, for which price negotiation is usually longer; if you consider these are directly available, the average time is 240 days.

- The 2001 EU directive sets the target for access time at 180 days. Access times across the EU rarely meet this target although they have reduced overall.

- France remains in 18th place in Europe for its time to market access (Figure 1).



- Time to market access is a sum of the delays of the drug evaluation, price negotiation and publication in the Official Journal, which accounts for 39% of the average time to market or 63% for hospital medicines.

Spain's Ministry of Health copies NICE, and asks pharma's for their economic evaluations

The Ministry of Health seems convinced that it does not have sufficient resources to prepare adequate economic evaluations of the new medicines that are marketed in Spain. For this reason, and following in the footsteps of other European countries including the UK, it will ask pharmaceutical companies to submit their economic evaluation studies to be reviewed later, something that requires less public resources than *ad hoc* preparation.

"We are going to copy the NICE and we are going to ask the laboratories for their economic evaluations and we will make a critical reading," said Carlos Martín Saborido, advisory member of the General Directorate of Common Portfolio of Services of the SNS and Pharmacy.

This indicates that very soon the holders of the marketing authorization will begin to be asked for these pharmacoeconomics studies. In addition, the Ministry is working on a manual, a guide that includes the characteristics that these evaluations must have, which will then be analysed through published checklists to verify that it has been "faithful to the methodology". With that, the advisory member considers that "transparency will be gained".

This new approach to the incorporation of economic evaluations into decision-making will also allow all new drugs, "100%", emphasizes Martín Saborido, that are marketed in five years will have their economic evaluation.

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The FDA's Biosimilar User Fee Amendments III (BsUFA III)

Biosimilars are FDA-approved biologic medications that are highly similar to, and have no clinically meaningful differences from existing FDA-approved biologics, called reference products. Biosimilars are safe and effective treatment options for many illnesses such as chronic skin and bowel disease, arthritis, kidney conditions and cancer.

An interchangeable biosimilar is a biosimilar that meets additional requirements and may be substituted for the reference product at a pharmacy, depending on the state law.

The Biosimilar User Fee Amendments (BsUFA III) for fiscal years 2023-2027 facilitate the development of safe and effective biosimilars and interchangeable biosimilars for the American public by supporting FDA review of biosimilar submissions and biosimilar regulatory science.

The overall objective of this work is to increase the efficiency and predictability of biosimilar development to help reduce the cost and time of development. This ultimately can **increase availability and access to much needed biological treatment options for patients**.

The fee rates were published in the Federal Register (FR) on 7th October 2022.

User Fee Type		FY 2022	FY 2023
Biosimilar Biological Product Development (BPD) Fee	Initial BPD	$ 57,184	$ 47,325
	Annual BPD	$ 57,184	$ 47,325
	Reactivation	$ 114,368	$ 94,650
Application Fee	Clinical Data Required	$ 1,746,745	$ 1,746,745
	Clinical Data not Required	$ 873,373	$ 873,373
Program Fee		$ 304,162	$ 304,162

As of the 13th February 2023, CDER and CBER have begun accepting meeting requests for in-person, face-to-face industry meetings (with a hybrid component), starting with Type A, BPD Type 1, and Type X meeting requests.

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We always welcome your thoughts and opinions on the topics raised here.

If you'd like to share anything or hear how we can support you in getting your product to market, email Paul and Graham, managing directors, today at: contact@remapconsulting.com.

Paul Craddy

MANAGING DIRECTOR
& FOUNDER

Graham Foxon

MANAGING DIRECTOR
& FOUNDER

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