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The European Union Pushes for Major Pharma Reforms

(Source: An article by Zoey Becker for Fierce Pharma)

In a sweeping list of proposals, the European Commission is addressing several shortfalls afflicting the US$148 billion European pharmaceutical industry. The proposed updates represent the most significant revisions to the EU's pharma legislation in 20 years with the intent of making medicines "more available, accessible and affordable," the Commission announced in a recent statement.

However, industry groups and executives have already voiced concerns over the proposed legislation. One of the biggest changes would make for a leaner drug authorization process. Under this proposal, only two committees of the European Medicines Agency would review efficacy and safety data during drug reviews. Other committees, such as the existing orphan, pediatric and advanced therapy groups, would be scrapped.

Officials also aim to speed up the review process under the new framework. Currently, the average time between a drug's submission and authorization in Europe is 400 days. If the legislation is implemented, the European Medicines Agency (EMA) will have 180 days to assess medicines for authorization. Then, to approve EMA-recommended drugs, the commission will have 46 days instead of the previous standard of 67. For drugs of "major public health interest" the EMA would be locked into even stricter guidelines.

As for the issue of pricing and access, the rules provide a host of changes. Included in these changes is a proposed shortening of the period of regulatory exclusivity from 10 years to eight for most medicines. Companies could extend their period of regulatory exclusivity if they launch their product in all EU member states, address diseases with unmet need, conduct comparative trials or launch drugs that treat multiple diseases.

The proposals have already received pushback from the pharma industry. The European Federation of Pharmaceutical Industries and Associations (EFPIA) believes the legislation "manages to undermine research and development in Europe while failing to address access to medicines for patients," Director General Nathalie Moll said in a statement. She added, "The approach set out in the pharmaceutical legislation, penalizing innovation if a medicine is not available in all member states within two years is fundamentally flawed and represents an impossible target for companies."

The European Commission further aims to boost transparency around R&D funding. The new rules would require companies to disclose research funding from public authorities or publicly funded organizations on public websites. This, in turn, could lower drug prices by giving EU member states a negotiating edge during pricing talks, according to the Commission.

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In Brief...

- **McKesson Corporation** announced Q4 and full year financial results for 2023, with Q4 revenues of US$68.9 billion (an increase of 4% year-over-year) and full year revenues of US$276.7 billion, a 5% increase over 2022. 2023 Earnings per diluted share from continuing operations increased US$17.79 to US$25.05 and fiscal 2024 adjusted earnings per diluted share guidance was a range of US$26.10 to US$26.90, a 1% to 4% increase compared to 2023. 'Our strong fourth quarter represented continued operating momentum and execution against our company priorities. For the full year, McKesson reported performance across all business segments above our long-term targets. Our results reflect the commitment of our employees and their dedication to perform for our customers, patients, communities and shareholders," said *Brian Tyler*, Chief Executive Officer.
- **Amazon** subsidiary **Pillpack** reported a cybersecurity attack affecting the accounts of nearly 20,000 customers. An unauthorized individual used customer emails and passwords to log into PillPack customer accounts, over 3,000 of which contained prescription information. An internal investigation found that the breach took place between April 2 and April 6, although suspicious log-in attempts were detected on April 3.
- The **U.S. Food and Drug Administration (FDA)** announced the supplemental approval of *Rexulti (brexpiprazole)* oral tablets for the treatment of agitation associated with dementia due to Alzheimer's disease. This is the first FDA-approved treatment option for this indication. Patients with dementia often have behavioral and psychological disturbances. Agitation is among the most persistent, complex, stressful and costly aspects of care among patients with these symptoms.
- Two years after the launch of **CVS Clinical Trial Services**, the **CVS Health** announced it was shutting down the business.

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The Most Expensive Drugs in the U.S. in 2023

(Source: An article by Fraiser Kansteiner, Angus Liu, Eric Sagonowsky and Kevin Dunleavy for FiercePharma)

As lawmakers in the U.S. look for ways to cut drug costs, gene therapy prices keep setting records. This can be attributed to these innovative drugs' potential as one-time, curative treatments – a factor that has prompted drugmakers to attach significant price tags on these therapeutics. While the high cost of gene therapies has not always been viewed positively in other parts of the world, these groundbreaking medicines have found a foothold in the U.S. market.

Many of the drugs on the list – which focuses specifically on FDA-approved medicines – will be well known to those who keep tabs on drug pricing. Last year, Bluebird, plus CSL Behring and uniQure, secured three gene therapy approvals in quick

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European Union (cont.)

Meanwhile, supply issues have been squeezing the pharmaceutical market worldwide. To combat the problem in the EU, the legislation stipulates that companies would have to set shortage prevention measures/plans for all medicines. Additionally, the Commission would designate certain drugs as "critical" and closely monitor their supplies.

Ahead of the proposal's release, GSK CEO Emma Walmsley warned on the company's first quarter earning conference call that the EU must "regulate for growth and competitiveness." Pressure to weaken market exclusivity protections could cause companies to shy away from researching and launching meds in Europe, she added.

It remains to be seen how the proposals will advance through the Commission's regulatory process. The next step is deliberations in the European Parliament and Council, which will begin "as soon as possible," the Commission said.

The talks in Europe come after the U.S. Congress and the current U.S. administration passed the Inflation Reduction Act in the U.S. last year. The law provides for new Medicare drug pricing negotiating powers in 2026 and allows for penalties by the government for raising prices faster than the rate of inflation, as well as other changes. Additionally, U.S. lawmakers are contemplating more pharmaceutical regulations in the future.

Most Expensive (cont.)...

succession with pricing records creeping up each time. From Bluebird's thalassemia therapy *Zynteglo* at US$2.8 million for a one-time dose to CSL and uniQure's US$3.5 million hemophilia B treatment *Hemgenix*, the three newest gene therapies to make their mark in the U.S. have quickly skyrocketed to the top of the industry's most expensive products.

Elsewhere, Novartis' established gene therapy *Zolgensma* continues to rank among the top of the list of most expensive drugs.

In addition to gene therapies, orphan drugs such as Eiger BioPharmaceuticals' *Zokinvy* (the first drug approved in the U.S. for Hutchinson-Gilford progeria syndrome and processing-deficient progeroid laminopathies) comes with a high price tag and costs approximately US$1.7 million a year. Unlike gene therapies, it isn't meant to be a cure.

Other cost drugs outside the gene therapy arena include Y-mAbs' *Danyelza* for relapsed or refractory high-risk neuroblastoma, Chiesi's leptin deficiency drug *Myalept* and Immunocore's T-cell receptor therapy for uveal melanoma called *Kimmtrak*.

Last year's passage of the U.S. Inflation Reduction Act (IRA) marked some of the first tangible movements in drug pricing reform in years, and lawmakers are committed to continue increased scrutiny.

At the same time, public and political attention on drug costs has prompted certain drugmakers to take matters into their own hands. Earlier this year, a trio of diabetes majors—Eli Lilly, Novo Nordisk and Sanofi—separately cut the prices of many of their insulin products and tweaked their value programs for patients.

But while those moves covered old insulins, many of the industry's new products continue to break pricing records. And that's not likely to change in the years to come.

Top 10 Expensive Drugs in the U.S.

Name	Company	Disease	Cost/Dose (US$)
Hemgenix	CSL Behring, uniQure	Hemophilia B	3,500,000
Skysona	Bluebird Bio	Cerebraladreno-leukodystrophy	3,000,000
Zynteglo	Bluebird Bio	Thalassemia	2,800,000
Zolgensma	Novartis	Spinal muscular atrophy	2,250,000
Mylept	Chiesi Farmaceutici	Leptin deficiency	1,260,000
Zokinvy	Eiger BioPharmaceuticals	Hutchinson-Gilford progeria syndrom	1,070,000
Danyelza	Y-mAbs Therapeutics	Relapsed/refractory high-risk neuroblastoma	1,010,000
Kimmtrak	Immunocore	Uveal melanoma	975,000
Luxturna	Spark Therapeutics	Biallelic RPE65-mediated inherited retinal disease	850,000
Folotyn	Acrotech Biopharma	Relapsed/refractory peripheral T-cell lymphoma	842,585

In Brief (cont.)

The company issued a statement, saying "We continually evaluate our portfolio of assets to ensure they are aligned with our long-term strategic priorities. As a result, we're winding down our Clinical Trial Services business in a phased way, with a full exit expected by December 31, 2024.

- Japan has set a new goal for biosimilar promotion. By the end of 2029, the country aims to increase the ratio of APIs with 80%-plus biosimilar replacement rates on a volume basis to at least 60% of all APIs that have biosimilar versions available. The new target was unveiled by Japan's health ministry at a working group meeting of the Council on Economic and Fiscal Policy in April. The ministry will further examine effective ways to fuel biosimilar use for individual APIs after conducting a fact-finding survey.
- **Eli Lilly's** weight loss candidate, *tirzepatide*, has scored its second pivotal trial win with impressive results. Marketed as the Type 2 diabetes treatment, *Mounjaro*, *tirzepatide* helped diabetic patients who are obese or overweight lose up to 15.7% of their body weight in a Phase 3 SURMOUNT-trial and performed significantly better than a placebo on various weight loss and metabolic measurements. The company said it will wrap an FDA rolling submission for the drug in adults with obesity or who are overweight in the coming weeks and approval could come as soon as late 2023.
- **Roche** announced the launch of **The Institute of Human Biology** which will focus on advancing research in the field of human model systems such as organoids. The institute aims to accelerate drug discovery and development by improving the understanding of how organs function and how diseases develop. The ultimate goal is to help bring medicines to patients faster and more efficiently.

(Sources: Company Press Releases, Drug Store News, FierceBioTech, Pharma Japan, Scrip Intelligence, and World Pharma News)