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# Many Firsts Among The Drugs That Launched In 2023

by Jessica Merrill

Drug launches in 2023 included some groundbreaking innovations, but that didn't necessarily translate to quick commercial success.

It was a year of innovation for new drugs. The number of drugs that reached the US market in 2023 rebounded from 2022 and many of them were notable advances. Among them were the first vaccines and treatments for respiratory syncytial virus (RSV), the first treatments for the advanced eye disease geographic atrophy, and the first gene therapies for hemophilia A and Duchenne muscular dystrophy (DMD).

The year kicked off with the US Food and Drug Administration's accelerated approval of a second anti-amyloid treatment for Alzheimer's disease, *Eisai Co., Ltd.*/*Biogen, Inc.*'s Leqembi (lecanemab) in January, but it became the first to secure full approval from the FDA later in the year, and thus the first to be granted reimbursement from the US Centers for Medicare & Medicaid Services (CMS). (Also see "*Leqembi Treatment Infrastructure: If Medicare Pays For It, They Will Come*" - Scrip, 7 Jul, 2023.)

The year ended with two other important firsts in sickle cell disease: the simultaneous FDA approval of the first gene therapy, *bluebird bio*'s Lyfgenia (lovotibeglogene autotemcel), and the first ever CRISPR gene-edited medicine, *Vertex Pharmaceuticals Incorporated*/*CRISPR Therapeutics AG*'s Casgevy (exagamglogene-autotemcel). (Also see "*Vertex/CRISPR Nab First-Ever Gene Editing FDA Nod, Overshadow Bluebird's Same-Day Win*" - Scrip, 9 Dec, 2023.) Those approvals came in December and therefore, the drugs have not effectively launched in 2023.

Altogether, FDA's Center for Drug Evaluation and Research (CDER) has approved 55 new molecular entities in 2023 and the Center for Biologic Evaluation and Research (CBER) approved a record-breaking 17 novel agents. The number of novel drugs approved by the FDA marks a return to high approval trends after a lower number of approvals last year. Around 40 new drugs

launched in the US in 2022, the fewest since 2016. (Also see "*Fewer Drug Launches In 2022, But Standouts Among Them*" - Scrip, 3 Jan, 2023.) *(See table below.)*

Not all of the drugs approved by FDA in a given year necessarily launch during that year. Drugs that are approved late in 2023 effectively launch in 2024. In other cases, launches are delayed. For example, *Revance Therapeutics, Inc.*'s botulinum toxin Daxxify was approved in September 2022 as a new competitor to Botox, but the company held out on a full launch until 2023. (Also see "*Revance's Daxxify Is Approved As Rival To Botox With Longer Efficacy*" - Scrip, 8 Sep, 2022.)

Despite the number of drugs approved and interesting innovations, commercial success remained elusive for many of the new launches. There were few standouts when it came to early sales trajectories.

"We're seeing the compounded impact of payer and health systems cracking down a bit from the perspective of trying to control budget impact ... combined with, in some cases, the compellingness of the evidence package," ZS Associates principal Bill Coyle said in an interview.

"Then, I think you've just got the health care system's capacity, as well as physicians and providers, to take on new information, new data, apply it into practice, get it to the right people, knowing that they'll benefit," he added. "There's just so much information, so many products and so much lack of capacity in the health care system. I think those things piece together to make launches challenging."

EY Americas industry markets leader-health sciences and wellness Arda Ural said the trend is one that has been going on for several years, pointing out that more than 70% of drug launches in the last 12 years have not met external analyst expectations.

## RSV Vaccines Stand Out

The RSV vaccines from *Pfizer Inc.* and *GSK plc*, Abrysvo and Arexvy, respectively, were approved by the FDA in May but launched in the fall for the RSV season, following recommendations for use by the Centers for Disease Control and Prevention (CDC).

The products were outliers, generating impressive revenues in their first quarter on the market, driven by inventory stocking, but also vaccinations as older adults sought to protect themselves against multiple respiratory illnesses, including influenza and COVID-19.

GSK's Arexvy outpaced its rival, with an early lead in the market. Arexvy generated £709m ($806m) in the third quarter, while Pfizer's Abrysvo generated $375m. Arexvy exceeded launch expectations and is on track to be a blockbuster in its first year on the market, GSK said during its third quarter sales and earnings call.

The company has big expectations for that trajectory to continue. “There’s a long runway for Arexvy in the US, where, during the quarter, we vaccinated more than 1.4 million adults of the 83 million at risk,” chief commercial officer Luke Miels said at the time. “We remain confident in our peak sales being greater than £3bn.”

Both Arexvy and Abrysvo were standouts versus other launch trajectories in 2023, including in comparison to an RSV treatment from *Sanofi* and *AstraZeneca PLC* that launched as a protective antibody approach for infants, Beyfortus (nirsevimab), which nonetheless generated a strong €137 in sales in its first quarter on the market. *(Editor's Note: This story has been updated to accurately report Beyfortus revenues)*

Revenues for only a handful of drugs that launched this year were broken out in the first nine months of 2023, generally a sign that sales were not material to a company’s top line. It’s not unusual for a large pharmaceutical company not to break out sales of a drug in the first year, but if a launch is off to blockbuster-sized trajectory, it tends to be the kind of news that drug makers like to highlight.

One other strong launch came from a smaller player, *Apellis Pharmaceuticals, Inc.*, with its launch of Syfovre (pegcetacoplan), though it was not without hiccups. Apellis was the first to market with a treatment for geographic atrophy, which occurs at the advanced stage of age-related macular degeneration (AMD).

The C3 inhibitor got off to a strong start, generating $161m following its approval in February through the third quarter. The market is large and untapped, and GA is considered a blockbuster-sized indication. (Also see "*Apellis Set For Blockbuster Success With Syfovre, The First Approved Geographic Atrophy Drug*" - Scrip, 20 Feb, 2023.) But, the launch hit a snag in July when cases of occlusive retinal vasculitis emerged in the real-world setting. (Also see "*Panic Not, Say Analysts As Apellis’s Syfovre Launch Is Beset By Retinal Vasculitis Events*" - Scrip, 18 Jul, 2023.)

Apellis regained its footing in the third quarter as the company – and providers – came to better understand the benefit/risk profile of Syfovre, but by then *Astellas Pharma, Inc.* had launched a competing product, the C5 inhibitor Izervay (avacincaptad pegol), which it acquired in the $5.9bn purchase of *Iveric Bio* in May. (Also see "*Panic Not, Say Analysts As Apellis’s Syfovre Launch Is Beset By Retinal Vasculitis Events*" - Scrip, 18 Jul, 2023.) Izervay launched in August and has not had as much time on the market, so it is hard to compare how the two drugs are competing against one another.

## Wait And See

*Eli Lilly and Company*’s Zepbound (tirzepatide) is all but destined to be a mega-blockbuster, but the GLP-1/GIP agonist was only FDA-approved for obesity in November and launched in December. It simply hasn’t had time to live up to investors’ enormous expectations – yet. (Also

see "*Lilly's Zepbound: The Biggest-Selling Drug In The World, Ever?*" - Scrip, 15 Nov, 2023.)

Tirzepatide was approved in 2022 under a different brand name, Mounjaro, for type 2 diabetes, where it has become a mega-blockbuster and has presumably been used off label for obesity. Mounjaro generated $2.96bn in the first nine months of 2023, positioning the drug as one of the best launches of all time.

"The opportunity is essentially unbound in many ways, just because of the prevalence of the condition and the positive knock-on effects of many people getting to healthy weight and staying that way," Coyle said of the potential for new obesity drugs.

The strength of anti-obesity medicines is fueling renewed interest in the cardio-metabolic space more generally.

"I think it's actually one of the bright spots this year, from my perspective, is a bit of a renaissance in cardiovascular focus," Coyle said.

Given the high unmet need in Alzheimer's and pent-up demand for a disease-modifying treatments, Eisai and Biogen's Leqembi is another drug that could grow into a big-selling blockbuster, but the launch trajectory in Alzheimer's has not developed anything like it was expected to. The challenging diagnosis, administration and reimbursement environment have diminished expectations for a splashy launch, even after the US government agreed to cover the treatment's costs.

Eisai records revenues for Leqembi and reported, in November, revenues of ¥400m ($2.7m) in the first half of its fiscal year. The company told investors it expects to make ¥10bn ($66.5m) in revenues from Leqembi in the current fiscal year, which ends in March.

## New Gene Therapies Off To Slow Start

CBER's 16 approvals included three gene therapies and the first CRISPR gene edited therapeutic, making it a banner year for gene therapies. The commercial success of this new wave of gene therapies could pave the way for future products.

Nonetheless, it remains early days for gene therapies in the commercial realm, and it will take time for the market to develop.

"The industry needs to build muscle memory. That's not going to be overnight," EY's Ural said of the complex market for gene therapies. "I think the next three years will be when we will figure this out as an industry and then it becomes a more muscle memory."

Two of the approvals – Lyfgenia and Casgevy in sickle cell disease – came in December and have

not had time to launch. But *Sarepta Therapeutics, Inc.*s Elevidys (delandistrogene moxeparvovec) for DMD and *BioMarin Pharmaceutical Inc.*'s Roctavian (valoctocogene roxaparvovec) for hemophilia A were both approved in June and are early test cases.

The launches have gotten off to a slow start, as was expected given the novelty of the new treatments, questions about their durability, the complex reimbursement environment for the pricey therapies and administration challenges. Neither Elevidys nor Roctavian have the kind of time on the market that is needed to determine their long-term trajectory, but both face challenges. Roctavian is entering a competitive market dominated by well understood therapeutics like Roche's Hemlibra (emicizumab), while Elevidys enters a market desperate for new treatment options but with mixed clinical trial data.

Following the accelerated approval of Elevidys in June, Sarepta's Phase III confirmatory trial failed to meet the primary endpoint in October, raising questions about how restricted use of the therapy might be in the future. It's currently only approved for boys four to five years of age, but the confirmatory trial was expected to broaden that range. Nonetheless, demand for the treatment is high. Third quarter Elevidys sales surpassed analyst expectations, with the gene therapy generating $69.1m. (Also see "*A Strong Launch For Sarepta's Elevidys, But Future Hinges On FDA Decision*" - Scrip, 2 Nov, 2023.)

The launch of Roctavian has gotten off to a slower start, but the market is a competitive one dominated by well understood treatments. During the company's third quarter sales and earnings call, BioMarin confirmed that two patients had been treated with commercial product in Europe, where it was approved ahead of the US.

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