BIOBUSINESS BRIEFS

MARKET WATCH

FDA new drug approvals in Q3 2022

The FDA approved 12 novel medicines in the third quarter of the year (TABLE 1), an uptick in activity after a slow first half. This apparent acceleration is a recent phenomenon, however, with no first-time green lights awarded in July, and only two in August. The balance came in September, and many will be hoping that this pace is maintained for the rest of the year.

The slowdown can partly be blamed on pandemic-related travel restrictions that prevented agency staff from inspecting overseas manufacturing facilities, which caused some decisions to be deferred or complete response letters to be issued. Other potential reasons include staff shortages at the FDA and a tightening of standards in the wake of last year's controversial approval of Biogen's Aduhelm (aducanumab) for Alzheimer disease.

At least one recent approval does not indicate a stricter regulator, however: Amylyx's amyotrophic lateral sclerosis treatment Relyvrio (sodium phenylbutyrate and taurursodiol), for which the FDA seemed to go out of its way to get the drug onto the market. After an initial advisory committee in March voted against the drug's effectiveness, the agency called a second hearing to review new cuts of data. Holding a second panel in the same review period is highly unusual, and the hearing itself was remarkable. It witnessed the FDA extract a public commitment from Amylyx executives that the company would withdraw Relyvrio from the market if an ongoing confirmatory pivotal trial failed.

While the Relyvrio approval might be the quarter's most notable decision, the green light for Bristol Myers Squibb's Sotyktu (deucravacitinib) probably has the most commercial significance, with forecasted sales of more than US$1 billion by 2026. The drug became the first TYK2 inhibitor to reach the market, and arrived with a cleaner-than-expected label in terms of safety. Sotyktu is considered the most effective oral option for psoriasis, and Bristol Myers Squibb has big label expansion plans in other autoimmune conditions.

The third quarter also saw bluebird bio get its two lead gene therapies across the line. Zynteglo (betibeglogene autotemcel) became the first gene therapy for severe β-thalassaemia — a rare blood clotting disorder — while Skysona (elivaldogene autotemcel) gained approval in cerebral adrenoleukodystrophy, an even rarer brain disease.

Also in the rare disease area, Boehringer Ingelheim's Spevigo (spesolimab) — a monoclonal antibody that targets the interleukin-36 receptor — won approval to treat generalized pustular psoriasis flares. It is being developed in wider settings including hidradenitis suppurativa and Crohn's disease.

Finally, Revance received approval for its long-acting Botox rival, Daxxify (daxibotulinumtoxin A), almost two years later than anticipated owing to a decision deferral due to the pandemic and then a complete response letter prompted by manufacturing deficiencies. However, the delay was still shorter than that for Terlivaz (terlipressin); various groups have tried to get this drug for hepatorenal syndrome to the US market for over a decade. The FDA was finally convinced in September, albeit insisting on a label with a black box warning of serious or fatal respiratory failure.

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Competing interests
The author declares no competing interests.

Table 1 | **Selected FDA new drug approvals in Q3 2022**

Date	Drug (brand name; company)	Properties	Indication	2026 global sales forecast
17 Aug	Betibeglogene autotemcel (Zynteglo; bluebird bio)	$CD34^+$ cells transduced with the $\beta^{A\text{-}T87Q}$-*globin* gene	β-Thalassaemia	$272 million
30 Aug	Olipudase alfa (Xenpozyme; Sanofi)	Recombinant human acid sphingomyelinase	Non-CNS manifestations of acid sphingomyelinase deficiency	$279 million
1 Sep	Spesolimab (Spevigo; Boehringer Ingelheim)	IL-36R-targeted antibody	Generalized pustular psoriasis flares	NA
7 Sep	Daxibotulinumtoxin A (Daxxify; Revance Therapeutics)	nAChR antagonist; SNAP25 inhibitor	Moderate to severe glabellar lines	$424 million
8 Sep	Deucravacitinib (Sotyktu; Bristol Myers Squibb)	TYK2 inhibitor	Moderate to severe psoriasis	$1,120 million
9 Sep	Eflapegrastim (Rolvedon; Spectrum Pharmaceuticals)	G-CSF receptor agonist	Chemotherapy-induced neutropenia	$235 million
14 Sep	Terlipressin (Terlivaz; Mallinckrodt Pharmaceuticals)	Vasopressin receptor agonist	Hepatorenal syndrome	$91 million
14 Sep	Futibatinib (Lytgobi; Otsuka Holdings)	FGFR antagonist	Cholangiocarcinoma harbouring *FGFR2* rearrangements	$54 million
16 Sep	Elivaldogene autotemcel (Skysona; bluebird bio)	$CD34^+$ cells transduced with the *ABCD1* gene	Cerebral adrenoleukodystrophy	$13 million
21 Sep	Gadopiclenol (Elucirem; Guerbet)	Gadolinium-based contrast agent for MRI	Detection and visualisation of lesions with abnormal vascularity	NA
26 Sep	Omidenepag isopropyl (Omlonti; Santen Pharmaceutical/UBE Corporation)	Selective prostaglandin E2 receptor agonist	Elevated intraocular pressure in glaucoma or ocular hypertension	$121 million
29 Sep	Sodium phenylbutyrate and taurursodiol (Relyvrio; Amylyx Pharmaceuticals)	Unknown mechanism of action	Amyotrophic lateral sclerosis	$1,095 million

CNS, central nervous system; FGFR; fibroblast growth factor receptor; G-CSF, granulocyte colony stimulating factor; IL-36R; interleukin 36 receptor; MRI; magnetic resonance imaging; NA, not available; nAChR; nicotinic acetylcholine receptor; SNAP25, synaptosomal-associated protein 25; TYK2; tyrosine kinase 2. Source: EvaluatePharma October 2022, Evaluate Ltd.