

Many readouts of phase 2 and phase 3 studies, pipelines are moving ahead.

HE Advisory team

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US Food and Drug Administration (FDA)

The FDA made the following decisions:

1. Abecma (**idecabtagene vicleucel**, BMS) was approved for the treatment of adult patients with relapsed or refractory multiple myeloma after two or more prior lines of therapy. [BMS press release](#).
2. Accelerated approval was granted to Enhertu (**fam-trastuzumab deruxtecan-nxki**, Daiichi Sankyo) for adult patients with unresectable or metastatic HER2-positive (IHC3+) solid tumors. [FDA press release](#).
3. Zevtera (**ceftobiprole medocartil sodium for injection**, Basilea) was approved for the treatment of adults with Staphylococcus aureus bloodstream infections (bacteremia). [Basilea press release](#).
4. Voydeya (**danicopan**, AstraZeneca) was approved as an add-on therapy for people with paroxysmal nocturnal hemoglobinuria. [AstraZeneca press release](#).
5. Fasentra (**benralizumab**, AstraZeneca) received approval for an indication extension as add-on maintenance treatment for patients with severe asthma aged 6 to 11 with an eosinophilic phenotype. [AstraZeneca's press release](#).
6. Orphan Drug Designation was granted to **CAN-2409** (Candel Therapeutics) for the treatment of pancreatic cancer. [Candel press release](#).
7. Dovato (**Dolutegravir/Lamivudine**, ViiV Healthcare) received an additional indication for the treatment of HIV-1 infection in adolescents 12 years of age and older. [ViiV Healthcare press release](#).

European Medicine Agency

The EMA's Pharmacovigilance Risk Assessment Committee (PRAC) had their monthly meeting last week.

The PRAC found that available evidence does not support a causal association between Glucagon-Like Peptide-1 receptor agonists (GLP-1) – dulaglutide, exenatide, liraglutide, lixisenatide and semaglutide, and suicidal or self-injurious thoughts or actions. See the [PRAC meeting highlights](#).

New data from phase 2 or phase 3 studies

1. Here are the updates on Phase 2 and Phase 3 clinical trials with published data in the last 2 weeks, between April 01 and April 14, 2024:
2. Pfizer announced positive results from the Phase 3 study of their **RSV vaccine**, Abrysvo, in adults aged 18 to 59 with an increased respiratory syncytial virus (RSV) disease risk. Pfizer said it plans to ask regulators to broaden the approval of its vaccine for RSV after the trial met its goals. [Pfizer press release](#).
3. Ionis Pharmaceuticals announced that **olezarsen** met the primary endpoint in its Phase 3 study in adults with familial chylomicronemia syndrome (FCS). Olezarsen could be the first FDA-approved drug for FCS. [Ionis press release](#).
4. Regeneron Pharmaceuticals reported positive results from the Phase 1/2 trial of its investigational bispecific antibody, **linvoseltamab**, in patients with relapsed/refractory multiple myeloma. . [Regeneron press release](#).
5. Geneos Therapeutics published positive results from the Phase 1/2 study of **GNOS-PV02**, a DNA plasmid-encoded personalized therapeutic cancer vaccine in combination with DNA plasmid-encoded IL-12 (a cytokine adjuvant) and pembrolizumab for advanced hepatocellular carcinoma (HCC). [Geneos press release](#).
6. YS Biopharma reported positive interim outcomes from the Phase 3 trial of their PIKA Rabies Vaccine. The **PIKA Rabies Vaccine** met its primary endpoints. [YS Bio press release](#).
7. Amylyx Pharmaceuticals announced positive interim results from the Phase 2 trial of Relyvrio (**AMX0035**) in patients with Wolfram syndrome. This comes just one week after Amylyx pulled Relyvrio from the market for amyotrophic lateral sclerosis (ALS). [Amylyx press release](#).
8. Teva Pharmaceuticals shared positive data from a Phase 3 study of Ajovy (**fremanezumab**) for the prevention of migraines in Chinese adult patients. [Teva press release](#).
9. Allay Therapeutics reported positive data from the Phase 2b study of its lead **ATX101** for post-surgical pain relief. [Allay press release](#).
10. Akeso Biopharma shared positive interim data from the Phase 3 trial of **cadonilimab** (PD-1/CTLA-4 BsAb) and xelox (capecitabine plus oxaliplatin) for unresectable locally advanced or metastatic gastric or gastroesophageal junction adenocarcinoma. The results were presented in the form of an oral presentation at the 2024 American Association for Cancer Research (AACR). [News coverage](#).
11. Novartis announced they will pause enrolment in certain clinical trials of their breast cancer drug **Kisqali** to address regulators' concerns about impurities related to nitrosamines. The enrolment suspension will allow Novartis to adjust its manufacturing in response. [Novartis press release](#).

12. Gritstone announced positive preliminary data from the Phase 2/3 study of Granite (**neoantigen cancer vaccine**) in front-line metastatic microsatellite stable colorectal cancer. However, the biological marker used to assess treatment response was uninformative. [Gritstone press release](#).
13. Roivant announced that **brepocitinib** met both the primary and secondary endpoints in its Phase 2 study in patients with non-infectious uveitis. [Roivant press release](#).
14. LENZ Therapeutics reported positive data from the Phase 3 study of two investigational formulations of **aceclidine**, LNZ100 and LNZ101, for presbyopia treatment. An NDA is planned to be filed late 2024. [LENZ press release](#).
15. BMS shared positive results from the Phase 3 trial of KarXT (**xanomeline-trospium**) in adults with schizophrenia. . [BMS press release](#).
16. Zeposia (**ozanimod**, BMS) did not meet its primary endpoint in its Phase 3 study in patients with modal of its vaccine for RSV after the trial met its goals. [Pfizer press release](#).

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