# New Drug Molecules and Gene Therapies Approval

**July 25, 2024**

***Product:*** Sun Pharma's Leqselvi

***Indication:*** Alopecia Areata

Adult patients with severe alopecia areata, an autoimmune disease that leads to hair loss, have a new treatment option after the FDA approved Sun Pharma's Leqselvi on Thursday. An oral JAK inhibitor, Leqselvi interrupts the pathways thought to contribute to hair loss in severe alopecia areata, according to Sun.

**July 24, 2024**

***Product:*** BioMarin's Brineura

***Indication:*** Batten disease

BioMarin secured an expanded approval Wednesday for Brineura, its treatment for the genetic condition called neuronal ceroid lipofuscinosis type 2 (CLN2) disease. The treatment, which was previously indicated only for symptomatic children 3 years of age and older with late infantile CLN2 disease, also known as Batten disease, can now be given to diagnosed children of all ages, regardless of whether they are symptomatic or presymptomatic.

**July 9, 2024**

***Product:*** Arcutis Biotherapeutics' Zoryve

***Indication:*** Atopic dermatitis

Arcutis Biotherapeutics won its third indication in two years for Zoryve, as the FDA green lit the topical cream to treat adults and children six years and older with atopic dermatitis. Arcutis said Zoryve will be available through wholesalers and dermatology pharmacies by the end of July.

**June 26, 2024**

***Product:*** Verona's Ohtuvayre

***Indication:*** Chronic obstructive pulmonary disease

Patients with chronic obstructive pulmonary disease (COPD) have their first inhaled treatment with a new mechanism of action in more than 20 years after the FDA greenlit Verona Pharma's Ohtuvayre. A selective dual inhibitor of the PDE3 and PDE4 enzymes, Ohtuvayre produces both bronchodilator and non-steroidal anti-inflammatory effects.

**June 26, 2024**

***Product:*** AbbVie and Genmab's Epkinly

***Indication:*** Follicular lymphoma

AbbVie and Genmab's bispecific antibody Epkinly can now be used to treat patients with relapsed or refractory follicular lymphoma. The FDA approval makes Epkinly the first T cell-engaging bispecific antibody for the subcutaneous treatment of relapsed or refractory follicular lymphoma, according to the partners.

**June 22, 2024**

***Product:*** Bristol Myers Squibb's Krazati

***Indication:*** Colorectal cancer

Bristol Myers Squibb's oncology franchise is celebrating another approval after the FDA greenlit Krazati in combination with cetuximab for patients with previously treated KRASG12C-mutated locally advanced or metastatic colorectal cancer. The approval is the second for Krazati, which is also FDA-authorized to treat KRASG12C-mutated locally advanced or metastatic non-small cell lung cancer.

**June 18, 2024**

***Product:*** AbbVie's Skyrizi

***Indication:*** Ulcerative colitis

AbbVie's blockbuster therapy Skyrizi now has further reach after the FDA greenlit the IL-23 antagonist for the treatment of moderately to severely active ulcerative colitis. Skyrizi is the first drug of its class approved for both ulcerative colitis and Crohn's disease, according to the company's announcement.

**June 14, 2024**

***Product:*** Amgen's Blincyto

***Indication:*** B-ALL

Patients with an aggressive blood cancer have a new treatment option after the FDA approved Amgen's Blincyto for CD19-positive Philadelphia chromosome-negative B-cell precursor acute lymphoblastic leukemia (B-ALL) in the consolidation phase.

**June 13, 2024**

***Product:*** BMS's Augtyro

***Indication:*** NTRK-positive, locally advanced or metastatic solid tumors

Thursday, the FDA granted accelerated approval to Bristol Myers Squibb's tyrosine kinase inhibitor (TKI) Augtyro for the treatment of NTRK-positive, locally advanced or metastatic solid tumors. Augtyro can now be used in patients aged 12 years and older whose disease has progressed after initial treatment as well as patients with no satisfactory alternative therapies and those who are likely to suffer from "severe morbidity" due to surgical resection, according to BMS.

**June 10, 2024**

***Product:*** Ipsen and Genfit's Iqirvo

***Indication:*** Primary biliary cholangitis

Monday, the FDA approved the first new drug in nearly a decade for primary biliary cholangitis: Ipsen and Genfit's Iqirvo. A rare liver disease, PBC affects around 100,000 people in the U.S. and can lead to liver failure. Iqirvo is intended to be used in combination with ursodeoxycholic acid (UDCA) in adult patients who have an inadequate response to UDCA, or as monotherapy in patients unable to tolerate UDCA.

**June 10, 2024**

***Product:*** Almirall's Klisyri

***Indication:*** Actinic keratosis

Dermatology company Almirall secured expanded approval of Klisyri for larger actinic keratosis-affected areas of the face or scalp. Klisyri can now be used to treat lesions up to 100 $cm^2$ caused by the pre-cancerous dermatological condition, after safety and tolerability profiles were consistent with original pivotal trial results. The new authorization for Klisyri, a microtubule inhibitor ointment, increases dosing for surface area treatment from up to 25 $cm^2$ to up to 100 $cm^2$, according to the company's press release.

**May 30, 2024**

***Product:*** BMS's Breyanzi

***Indication:*** Mantle Cell Lymphoma

After winning approval earlier this month in follicular lymphoma, Bristol Myers Squibb's Breyanzi got the FDA nod for another indication on Thursday: relapsed or refractory mantle cell lymphoma (MCL). Specifically, Breyanzi is approved for patients with MCL who have received at least two prior lines of systemic therapy, including a Bruton tyrosine kinase inhibitor.

**May 29, 2024**

***Product:*** Eli Lilly's Retevmo

***Indication:*** RET-altered pediatric cancers

Eli Lilly won accelerated approval Wednesday for Retevmo to treat pediatric patients two years and older with RET-positive thyroid cancers and other solid tumors that carry the mutation. Retevmo is the first drug in the class available for children under 12 years of age, *Pharmaphorum* reported.

**May 29, 2024**

***Product:*** Tris Pharma's Onyda XR

***Indication:*** Attention deficit hyperactivity disorder

Wednesday, the FDA greenlit Tris Pharma's Onyda XR as the first non-stimulant medication for attention deficit hyperactivity disorder (ADHD) with a liquid formulation and nighttime dosing, according to the company. Onyda XR is a reformulation of clonidine hydrochloride, which was first approved by the FDA in 1974 to treat high blood pressure. Clonidine was approved for ADHD in 2010 under the brand name Kapvay, which is owned by Shionogi.

**May 29, 2024**

***Product:*** Teva's Austedo XR

***Indication:*** Tardive dyskinesia and Huntington's disease chorea

People with tardive dyskinesia and Huntington's disease chorea have a streamlined treatment option after the FDA approved a new one-pill-a-day version of Teva's Austedo XR. The newly approved formulation "offers more flexibility with the most once-daily doses of any vesicular monoamine transporter 2 (VMAT2) inhibitor," for these conditions, according to Teva's press release.

**May 28, 2024**

***Product:*** Amgen's Bkemv

***Indication:*** Paroxysmal nocturnal hemoglobinuria and atypical hemolytic uremic syndrome

AstraZeneca's rare disease drug Soliris now has a biosimilar on the market after the FDA greenlit Amgen's Bkemv to treat paroxysmal nocturnal hemoglobinuria (PNH) and atypical hemolytic uremic syndrome (aHUS). Bkemv was granted the FDA's interchangeability designation, which allows it to be used in place of the branded reference product without needing to change the prescription.

**April 29, 2024**

***Product:*** Pfizer and Genmab's Tivdak

***Indication:*** Cervical cancer

The FDA has converted the accelerated approval of Pfizer and Genmab's Tivdak into a full nod for recurrent or metastatic cervical cancer that has progressed on or after chemotherapy.

**April 26, 2024**

***Product:*** Pfizer's Beqvez

***Indication:*** Hemophilia B

People with moderate to severe hemophilia B have a new, one-time treatment option Friday, as the FDA approved Pfizer's Beqvez. The gene therapy is specifically approved for those patients who currently use factor IX (FIX) prophylaxis therapy, or have current or historical life-threatening hemorrhage, or repeated, serious spontaneous bleeding episodes and do not have neutralizing antibodies to the adeno-associated virus serotype Rh74var (AAVRh74var) capsid.

**April 22, 2024**

***Product:*** Abeona Therapeutics' prademagene zamikeracel

***Indication:*** Recessive dystrophic epidermolysis bullosa

Chemistry, manufacturing and controls (CMC) issues led the FDA to reject Abeona Therapeutics' investigational gene-corrected cell therapy prademagene zamikeracel (pz-cel) in recessive dystrophic epidermolysis bullosa, a rare disorder that causes painful blistering and erosion of the skin.

**April 22, 2024**

***Product:*** ImmunityBio's Anktiva

***Indication:*** Non-muscle invasive bladder cancer

A large number of bladder cancer patients will have a new treatment option as the FDA on Monday greenlit ImmunityBio's Anktiva, a first-in-class IL-15 superagonist for non-muscle invasive bladder cancer—which accounts for approximately 80% of new cases, according to the company.

**April 18, 2024**

***Product:*** Genentech's Alecensa

***Indication:*** Non-small cell lung cancer

The FDA approved Genentech's Alecensa Thursday as the first adjuvant treatment for patients with early-stage, anaplastic lymphoma kinase (ALK)-positive non-small cell lung cancer who have undergone tumor resection.

**April 18, 2024**

***Product:*** Takeda's Entyvio

***Indication:*** Crohn's disease

The FDA on Thursday approved a subcutaneous (SC) form of Takeda's Entyvio for maintenance therapy in adults with moderately to severely active Crohn's

disease (CD) after induction therapy with intravenous Entyvio.

**April 11, 2024**

***Product:*** AstraZeneca's Fasnera

***Indication:*** Asthma

AstraZeneca won a label expansion Thursday for Fasnera, a subcutaneous IL-5 receptor blocker, for the add-on maintenance treatment of severe eosinophilic asthma in kids 6 to 11 years of age.

**April 8, 2024**

***Product:*** Supernus Pharmaceuticals' SPN-830

***Indication:*** Parkinson's disease

Supernus Pharmaceuticals failed, for the second time, to secure approval for its drug-device combo SPN-830, which is intended for the continuous treatment of motor fluctuations in Parkinson's disease.

**April 1, 2024**

***Product:*** AstraZeneca's Voydeya

***Indication:*** Paroxysmal nocturnal haemoglobinuria

AstraZeneca kicked off April with a win for its rare disease portfolio as the FDA approved Voydeya, an oral, first-in-class factor D inhibitor to treat a subset of patients with paroxysmal nocturnal haemoglobinuria (PNH).

**March 28, 2024**

**Product:** Akebia's Vafseo

**Indication:** Anemia due to chronic kidney disease

After a lengthy regulatory battle, the FDA approved Akebia Therapeutics' oral treatment vadadustat. The drug, which will be sold under the name Vafseo, will treat adults with anemia due to chronic kidney disease who have been on dialysis for at least three months.

**March 22, 2024**

***Product:*** AbbVie's Elahere

***Indication:*** Certain ovarian, fallopian tube and primary peritoneal cancers

Friday, the FDA granted AbbVie's Elahere full approval for patients with certain ovarian, fallopian tube and primary peritoneal cancers. The nod, which converts a November 2022 accelerated approval, makes Elahere "the first and only antibody-drug conjugate (ADC) approved in the U.S." for ovarian cancer, Roopal Thakkar, AbbVie's CMO of global therapeutics, said in a statement.

**March 22, 2024**

***Product:*** Italfarmaco/ITF's Duvyzat

***Indication:*** Duchenne muscular dystrophy

Duchenne muscular dystrophy (DMD) patients have their third new treatment option in nine months after the FDA approved Italfarmaco's Duvyzat, a nonsteroidal oral drug to be sold by newly incorporated U.S. subsidiary ITF Therapeutics. Duvyzat (givinostat) is the first nonsteroidal drug approved to treat patients with all genetic variants of DMD, according to the FDA's announcement.

**March 20, 2024**

***Product:*** Idorsia's Tryvio

***Indication:*** Hypertension

Wednesday, the FDA greenlit Idorsia Pharmaceuticals' Tryvio for the treatment of hypertension to reduce blood pressure in adults who are not seeing adequate control on other drugs. Tryvio, which targets the endothelin pathway for hypertension, is intended to be taken alongside other antihypertensive drugs. It is the first FDA-approved medicine in its class for hypertension, according to Idorsia's announcement.

**March 18, 2024**

***Product:*** Orchard Therapeutics' Lenmeldy

***Indication:*** Metachromatic leukodystrophy

Monday, the FDA gave a glimmer of hope to families of children with metachromatic leukodystrophy (MLD), a rare genetic disease affecting the brain and nervous system, with the approval of Orchard Therapeutics' Lenmeldy.

**March 14, 2024**

***Product:*** Madrigal's Rezdiffra

***Indication:*** MASH

In one of the year's most highly anticipated FDA decisions, Madrigal Pharmaceuticals won approval Thursday for resmetirom—henceforth to be known as Rezdiffra—as the first-ever treatment for metabolic dysfunction-associated steatohepatitis (MASH), formerly noncirrhotic non-alcoholic steatohepatitis (NASH).

**March 14, 2024**

***Product:*** BeiGene's Tevimbra

***Indication:*** Esophageal squamous cell carcinoma

Also coming through on a jam-packed Thursday for the FDA was a nod for BeiGene's PD-1 blocker tislelizumab—which will carry the brand name Tevimbra—in unresectable or metastatic esophageal squamous cell carcinoma.

**References: www.biospace.com/biospace-fda-decision-tracker**

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