

### FDA Drug Approvals for 2023

Investors pay close attention to the FDA's approval process. The FDA's stance on innovation as it relates to safety and efficacy is crucial to any investment in therapeutic drug development. Innovation in drug development moves at the pace of the FDA. The table below lists novel FDA approvals in 2023 spanning innovation from gene therapy to antibodies to small molecule drugs.

## Table 2: Novel FDA Drug Approvals for 2023 (CDER)

Approval Date in 2023	Drug Name & Company	Drug Modality	Indication & Mechanism of Action
21-Dec	Wainua (eplontersen), Astra Zeneca & Ionis	Antisense	Polyneuropathy of hereditary transthyretin (TTR)-mediated amyloidosis, binds to TTR mRNA and reduces TTR protein
18-Dec	Filsuvez (birch triterpenes), Chiesi	Small Molecule	Mechanism of action not clear, promotes wound healing, reduces inflammation
5-Dec	Fabhalta (iptacopan), Novartis	Small Molecule	Paroxysmal nocturnal hemoglobinuria, complement Factor B inhibitor
27-Nov	Ogsiveo (nirogestat), SpringWorks Therapeutics	Small Molecule	Progressing desmoid tumors requiring systemic treatment, gamma secretase inhibitor that blocks proteolytic activation of the Notch receptor
16-Nov	Truqap (capivasertib), Astra Zeneca	Small Molecule	Breast cancer that meets certain disease criteria, kinase inhibitor of all 3 isoforms of AKT (AKT1, AKT2 and AKT3)
16-Nov	Ryzneuta (efbmalenograstim alfa-vuxw), Evive Biotech	Recombinant Fusion Protein	Neutropenia, fusion protein consisting of human G-CSF that acts on hematopoietic cells
15-Nov	Augtyro (reprotretinib), Bristol Myers	Small Molecule	ROS1-positive non-small cell lung cancer, inhibitor of proto-oncogene tyrosine-protein kinase ROS1 and of the tropomyosin receptor tyrosine kinases (TRKs)
15-Nov	Defencath (taurolidine, heparin), CorMedix	Small Molecule	Reduction of infection during hemodialysis, Taurolidine is an antimicrobial agent and heparin is an anticoagulant
8-Nov	Fruzaqla (fruquintinib), Takeda	Small Molecule	Refractory, metastatic colorectal cancer, kinase inhibitor of vascular endothelial growth factor receptors (VEGFR)-1/2/3
27-Oct	Loqtorzi (toripalimab-tpzi), Coherus Biosciences	Antibody	Recurrent or metastatic nasopharyngeal carcinoma, inhibits the PD-L1/PD-1 pathway on T cells
26-Oct	OmvoH (mirikizumab-mrkz), Eli Lilly	Antibody	Ulcerative colitis, IL-23 monoclonal antibody
26-Oct	Agamree (vamorolone), Santhera Pharma	Small Molecule	Duchenne muscular dystrophy, corticosteroid that acts through the glucocorticoid receptor to exert anti-inflammatory and immunosuppressive effects.
17-Oct	Bimzext (bimekizumab): UCB	Antibody	Moderate to severe plaque psoriasis, IL-17 mab (IL-17A and IL-17F)
17-Oct	Zilbrysq (zilucoplan), UCB	Synthetic peptide	Myasthenia gravis in adults who are anti-acetylcholine receptor (AChR) antibody positive, C5 complement inhibitor
12-Oct	Velsipity (etrasimod), Pfizer	Small Molecule	Moderate to severe ulcerative colitis, sphingosine 1-phosphate (S1P) receptor modulator
29-Sep	Rivfloza (nedosiran), Novo Nordisk	siRNA	Lowers urinary oxalate levels in primary hyperoxaluria type 1, hepatic lactate dehydrogenase-targeted siRNA
28-Sep	Pombiliti (cipaglicosidase alfa-ata), Amicus Therapeutics	Enzyme Replacement	Late-onset Pompe disease, GAA replacement (acid alpha glucosidase)
22-Sep	Exxua (gepirone), Fabre-Kramer Pharmaceuticals (private)	Small Molecule	Major depressive disorder, 5-HT1A receptor agonist
15-Sep	Ojjaara (momelotinib), GlaxoSmithKline	Small Molecule	Intermediate or high-risk myelofibrosis, JAK1/JAK2/ACVR1 inhibitor
8-Sep	Aphexda (motixafortide), BioLineRx	Synthetic peptide	Mobilize hematopoietic stem cells during autologous transplantation, CXCR4 antagonist
18-Aug	Veopoz (pozelimab-bbfg), Regeneron	Antibody	Patients 1 year and older with CD55-deficient protein-losing enteropathy (PLE), also known as CHAPLE disease, C5-targeted monoclonal antibody
16-Aug	Sohonos (palovarotene), Ipsen	Small Molecule	Reduction of heterotopic ossification in patients with fibrodysplasia ossificans progressiva, RAR $\gamma$ modulator
14-Aug	Elrefxio (elranatamab-bcmm), Pfizer	Bispecific Antibody	Relapsed or refractory multiple myeloma, BCMA $\times$ CD3 bispecific
9-Aug	Talvey (talquetamab-tgvs), Janssen (J&J)	Bispecific Antibody	Relapsed or refractory multiple myeloma, GPRC5D $\times$ CD3 bispecific
4-Aug	Izervay (avacincaptad pegol), Iveric Bio & Astellas	RNA Aptamer	Geographic atrophy secondary to age-related macular degeneration, C5-targeted aptamer
4-Aug	Zurzuvae (zuranolone), Sage Therapeutics & Biogen	Small Molecule	Postpartum depression, GABA $\alpha$ receptor modulator (gamma-aminobutyric acid receptor type A)
25-Jul	Xdemvy (lotilaner), Tarsus Pharma	Small Molecule	Demodex blepharitis, GABA-Cl channel inhibitor (GABA-gated chloride channel)
20-Jul	Vanflyta (quizartinib), Daiichi Sankyo	Small Molecule	Acute myeloid leukemia, FLT3 inhibitor
17-Jul	Beyfortus (nirsevimab-alip), Astra Zeneca	Antibody	Prevention respiratory syncytial virus (RSV) lower respiratory tract disease, F-protein-targeted monoclonal antibody
27-Jun	Ngenla (somatrogon-ghla), Pfizer & OPKO Health	Fusion protein	Growth failure due to inadequate secretion of endogenous growth hormone, GHRH receptor agonist
26-Jun	Rystiggo (rozanolixizumab-noli), UCB	Antibody	Myasthenia gravis in adults who are anti-acetylcholine receptor- or anti-muscle-specific tyrosine kinase antibody-positive, FcRn antagonist
23-Jun	Litfulo (ritlecitinib), Pfizer	Small Molecule	Severely patchy hair loss, JAK3 inhibitor
15-Jun	Columvi (glofitamab-gxmb), Genentech/Roche	Bispecific Antibody	Diffuse large B-cell lymphoma, not otherwise specified, or large B-cell lymphoma arising from follicular lymphoma, CD20 $\times$ CD3 bispecific
26-May	Inpefa (sotagliflozin), Lexicon Pharma	Small Molecule	Heart failure, SGLT1/2 inhibitor
23-May	Xacduro (sulbactam, durlobactam), Entasis Therapeutics	Small Molecule	Hospital-acquired bacterial pneumonia and ventilator-associated bacterial pneumonia, $\beta$ -Lactamase inhibitors
19-May	Epkinly (epcoritamab-bysp), AbbVie & Genmab	Bispecific Antibody	Relapsed or refractory diffuse large B-cell lymphoma (not otherwise specified) and high-grade B-cell lymphoma, CD20 $\times$ CD3
18-May	Miebo (perfluorhexyloctane), Bausch & Lomb	Small Molecule	Dry eye disease, perfluorohexyloctane, a semifluorinated alkane - exact mechanism of action not known
12-May	Veozah (fezolinetant), Astellas	Small Molecule	Moderate to severe hot flashes caused by menopause, NK3 receptor antagonist
9-May	Elfabrio (pegunigalsidase alfa-iwxj), Protalix Bio & Chiesi	Enzyme Replacement	Fabry disease, recombinant $\alpha$ -galactosidase enzyme replacement therapy
25-Apr	Qalsody (tofersen), Biogen	Antisense	Amyotrophic lateral sclerosis in adults who have a SOD1 gene mutation, SOD1 inhibitor
24-Mar	Joenja (lenilolisib), Pharming	Small Molecule	Phosphoinositide 3-kinase delta syndrome -genetic disease, PI3K $\delta$ inhibitor
22-Mar	Rezzayo (rezafungin), Cidara & Melinta Therapeutics	Small Molecule	Candidemia and invasive candidiasis, 1,3- $\beta$ -d-glucan synthase inhibitor
22-Mar	Zynyz (retifanlimab-dlwr), Incyte	Antibody	Metastatic or recurrent locally advanced Merkel cell carcinoma, PD1-targeted monoclonal antibody
10-Mar	Daybue (trofinetide), Acadia Pharmaceuticals	Small Molecule	Rett syndrome, IGF1 peptide analogue (insulin-like growth factor 1)
9-Mar	Zavzpret (zavegepant), BioHaven & Pfizer	Small Molecule	Migraine, GRP antagonist (calcitonin gene-related peptide)
28-Feb	Skyclarys (omaveloxolone), Biogen	Small Molecule	Friedrich's ataxia, NRF2 activator (nuclear factor erythroid 2-related factor 2)
17-Feb	Filspari (sparsentan), Travere Therapeutics	Small Molecule	Proteinuria in adults with primary immunoglobulin A nephropathy, AT1 and ETA antagonist
16-Feb	Lamzede (velmanase alfa-tycv): Chiesi	Enzyme Replacement	Reduce proteinuria in adults with primary immunoglobulin A nephropathy, Recombinant $\alpha$ -mannosidase
1-Feb	Jesduvroq (daprodustat), GlaxoSmithKline	Small Molecule	Anemia caused by chronic kidney disease for adults on dialysis, HIF-PH inhibitor (hypoxia inducible factor prolyl hydroxylase)
27-Jan	Orserdu (elacestrant), Stemline Therapeutics & Menarini	Small Molecule	ER-positive, HER2-negative, ESR1-mutated metastatic breast cancer, SERD (selective oestrogen receptor degrader)
27-Jan	Jaypirca (pirtobrutinib), Eli Lilly	Small Molecule	Refractory mantle cell lymphoma, BTK inhibitor
20-Jan	Brenzavvy (bexagliflozin), TheracosBio	Small Molecule	Type 2 diabetes mellitus, SGLT2 inhibitor
6-Jan	Leqembi (lecanemab-irmb), Eisai & Biogen	Antibody	Alzheimer's disease, Amyloid- $\beta$ -targeted monoclonal antibody

## Table 3: Novel FDA Drug Approvals for 2023 (CBER)

Approval Date in 2023	Drug Name & Company	Drug Modality	Indication & Mechanism of Action
8-Dec	Casgevvy (exagamglogene autotemcel), CRISPR Therapeutics & Vertex Pharmaceuticals	Cellular Gene Therapy/ Gene Editing - CRISPR	Sickle cell disease; CRISPR/CAS9 gene editing reduces BCL11A expression in CD34+ stem cells, leading to increased fetal hemoglobin (HbF) production
8-Dec	Lyfgenia (lovotibeglogene autotemcel), Bluebird Bio	Cellular Gene Therapy	Sickle cell disease (SCD), Lentiviral gene therapy - delivery of a functional version of a hemoglobin-producing gene in autologous CD34 stem cells
9-Nov	Adzyna (ADAMTS13, recombinant-krhn), Takeda	Enzyme Replacement	Congenital thrombotic thrombocytopenic purpura (CTTP), recombinant ADAMTS13 is a plasma zinc metalloprotease that regulates the activity of von Willebrand factor (VWF) reducing the platelet binding properties of VWF and its propensity to form microthrombi
10-Nov	IxchIQ (Chikungunya vaccine, live)	Live attenuated Vaccine	Chikungunya virus prevention, Immune response to live attenuated virus
20-Oct	Penbraya (Meningococcal Groups A/B/C/W/Y Vaccine), Pfizer	Polysaccharide conjugated to tetanus toxoid vaccine	Active immunization to prevent invasive disease caused by Neisseria meningitidis. PENBRAYA induces the production of bactericidal antibodies specific to the capsular polysaccharides of N. meningitidis serogroups A, C, W, and Y
21-Aug	Abrysvo (RSV vaccine), Pfizer	Recombinant protein-based vaccine	Active immunization of pregnant women and for those 60 and older for RSV infection prevention, Active immune response against RSV refusion F protein that protects against lower respiratory tract disease. And passive immunization via antibodies transferred via placenta
21-Jul	Balfaxar (human plasma-derived prothrombin complex concentrate), Octapharma	Human plasma-derived protein	Reversal of acquired coagulation factor deficiency induced by Vitamin K antagonist (VKA, e.g., warfarin) - provides a rapid increase in plasma levels of the vitamin K-dependent coagulation factors
20-Jul	Cyfundus (Anthrax Vaccine), Emergent Product Development	B. anthracis antigens	For post-exposure prophylaxis of Bacillus anthracis infection/exposure, induces antibodies against Bacillus anthracis antigens
29-Jun	Roctavian (valoctocogene roxaparvovec), BioMarin Pharmaceuticals	Gene Therapy	Severe hemophilia A (congenital factor VIII deficiency), adeno-associated virus vector-based gene therapy that induces functional copy of factor VIII
28-Jun	Lantidra (donisilecel-jujn), CellTrans, Inc.	Allogeneic cell therapy	Type 1 diabetes, allogeneic pancreatic islets that secrete insulin and other hormones
28-Jun	Elevedys (delandistrogene moxeparvovec-rokl), Sarepta Therapeutics, Roche, Chugai	Gene Therapy	Adeno-associated virus vector-based gene therapy for Duchenne muscular dystrophy (DMD), encodes the gene for micro-dystrophin protein and regulatory elements
19-May	Vyjuvek (beremagene geperpavec), Krystal Biotech, Inc.	Gene Therapy	Epidermolysis bullosa patients with mutation(s) in the collagen type VII alpha 1 chain (COL7A1) gene, topical application
3-May	Arexvy (RSV Vaccine), GlaxoSmithKline	Recombinant protein-based vaccine	Vaccine for lower respiratory tract disease caused by RSV in individuals 60 and older, recombinant respiratory syncytial virus glycoprotein F
26-Apr	Vowst (fecal microbiota spores, live-brpk) Seres Therapeutics	Spores in capsule	Prevent the recurrence of <i>Clostridioides difficile</i> infection. VOWST is manufactured from human fecal matter, mechanism of action not established
17-Apr	Omisirge (omidubicel-onlv), Gamida Cell Ltd.	Allogeneic cell therapy	Nicotinamide (NAM) modified allogeneic hematopoietic progenitor cell therapy for patients with hematologic malignancies who are planned for umbilical cord blood transplantation following myeloablative conditioning
22-Feb	Altuviiio (antihemophilic factor (recombinant), Fc-VWF-XTEN fusion protein-ehtl), Bioverativ Therapeutics & Sanofi	Fusion protein	Hemophilia A (congenital Factor VIII deficiency), fusion protein of human FVIII covalently fused to the Fc domain, replaces the missing coagulation factor VIII needed for effective hemostasis