U.S. FOOD & DRUG ADMINISTRATION

CENTER FOR DRUG EVALUATION AND RESEARCH

Advancing Health Through Innovation:

New Drug Therapy Approvals 2023

INNOVATION PREDICTABILITY ACCESS

January 2024

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Director's Message

Patrizia Cavazzoni, M.D.

Director, Center for Drug Evaluation and Research

Welcome to FDA's Center for Drug Evaluation and Research's (CDER) 13th annual report, *Advancing Health Through Innovation: New Drug Therapy Approvals*. This report highlights the role that CDER plays in bringing safe and effective drugs to patients and consumers.

2023 was an important year for public health. Perhaps most notably, after more than three years, the U.S. Department of Health and Human Services declaration of a Public Health Emergency (PHE) for COVID-19 ended in May. While the PHE has ended, CDER is continuing to review drug applications for the treatment of COVID-19 infections.To that point, CDER approved the first oral antiviral pill for COVID-19 in 2023 and is supporting further drug development in this space.

Our 2023 New Drug Therapy Approvals Report highlights CDER's "novel" drug approval actions — which are for therapies that have not previously been approved in the U.S. The report covers other important CDER actions as well, such as expanding the use or patient population of previously approved drugs. The report also describes drugs approved in new dosage forms or formulations.

I want to particularly point out our efforts in the opioid overdose space. In 2023, CDER took actions to make three opioid overdose reversal drugs available without a prescription, which can increase access to life-saving therapies. We also approved new dosage forms of drugs that reverse opioid overdose and treat opioid use disorder. Our efforts align with FDA's Overdose Prevention Framework, which aims to prevent drug overdose and reduce overdose-related death.

But our work is not confined to any one area of drug development, and our 2023 drug approvals collectively target a wide range of diseases and conditions. Many of the 2023 approvals are for patients with few or no treatment options, including those with rare diseases. Other drug approvals offer improvements in effectiveness, safety, or ease of use compared to standard-of-care therapies.

We approved the majority of the 2023 therapies on or before their goal dates, or congressionally authorized agreements with industry. We also approved most of these drugs in the U.S. before our regulatory counterparts did so in other countries.

In encouraging news for biosimilars, CDER approved five biosimilars, including three biosimilars for reference products that did not have a corresponding biosimilar. To date, CDER has approved 45 biosimilars for 14 reference products.

This report captures CDER's 2023 approvals. FDA's Center for Biologics Evaluation and Research (CBER) also approves important biologics and CDER and CBER may collaborate on approval actions. Please visit CBER's webpage for 2023 Biological Approvals for information on CBER's 2023 product approvals.

We hope this report showcases CDER's steadfast commitment to protecting and advancing patient care through the careful and vigorous review and approval of meaningful treatments.

Patrizia Cavazzoni, M.D.
Director, Center for Drug Evaluation and Research

Executive Summary

CDER approved a variety of safe and effective drug therapies in 2023. These approvals, spanning a broad scope of diseases and conditions, aim to help many people live better and potentially longer lives.

Innovation Across Medical Conditions

In 2023, we approved 55 new drugs never before approved or marketed in the U.S., known as “novel” drugs. We also made other important approval decisions, such as expanding the use or patient population of previously approved drugs.

The 2023 actions, both novel and other important drug approvals, focus on prevention, diagnosis, and treatment of diseases and conditions such as:

- Infectious diseases, including COVID-19, respiratory syncytial virus (RSV), hospital-acquired and ventilator-associated bacterial pneumonia, and HIV-1.
- Neurological conditions, such as amyotrophic lateral sclerosis (ALS), Alzheimer’s disease, and migraine.
- Opioid use, misuse, and abuse.
- Heart, blood, kidney, and endocrine diseases, including type 2 diabetes in pediatrics, types of anemia, pediatric hormone deficiency, and chronic weight management.
- Lung diseases, such as asthma and cystic fibrosis.
- Gastrointestinal conditions, including inflammatory bowel disease and pediatric functional constipation.

- Different types of cancers, such as colorectal, prostate, lung, and low-grade gliomas (tumors that start in the brain).
- Women's health, such as postpartum depression, hot flashes due to menopause, and nonprescription oral contraception.

New Drugs for Patients with Rare Diseases

Patients with rare diseases are often in critical need of new therapies, as these individuals generally have few or no existing treatment options. In 2023, 28 of 55, or 51% of our novel drug approvals received orphan drug designation because they target rare diseases, including:*

- Friedreich's ataxia, an inherited, degenerative disease that damages the nervous system.
- Candidemia and invasive candidiasis, which are serious and life-threatening fungal infections.
- Rett syndrome, a genetic, neurological disorder that affects brain development.
- CD55-deficient protein-losing enteropathy (CHAPLE disease), a genetic disease that affects the immune system.
- Paroxysmal nocturnal hemoglobinuria, a disease that causes red blood cells to break apart.
- Activated phosphoinositide 3-kinase delta, a genetic disorder that impairs the immune system.

In 2023, CDER also approved many therapies for rare cancers or tumors, including:

- Mantle cell lymphoma, an aggressive form of non-Hodgkin's lymphoma.
- Nasopharyngeal carcinoma, a rare head and neck cancer.
- Large B-cell lymphoma.
- Desmoid tumors, noncancerous growths in the connective tissue.

**Not all drugs for rare diseases necessarily receive orphan designation.*

Efficiencies in Bringing Therapies to Market

Our 2023 approvals demonstrate efficiencies in our review process, as shown by the following:

- **User Fee Goals Performance:** Of the 55 new drugs approved in 2023, CDER met or exceeded its Prescription Drug User Fee Act (PDUFA) goal dates for 49 of these approvals (89%). A number of novel drug approvals in 2023 were delayed due to COVID-19 related foreign travel restrictions, which hindered onsite inspections within the user fee review timeline.
- **First Cycle Approvals:** In 2023, CDER approved 46 of the 55 novel approvals (84%) on the first cycle. This differs from when CDER initially is unable to approve a drug because information in the application does not support approval. Subsequently, the sponsor resubmits the application with additional information, starting another review cycle that may lead to drug approval.
- **Approvals in U.S. Before Other Countries:** 35 of the 55 novel drugs approved in 2023 (64%) were first approved in the U.S.
- **Expedited Programs for Serious Conditions:** CDER has four broadly applicable programs to facilitate and expedite development and review of drugs for serious or life-threatening conditions: Fast Track designation, Breakthrough Therapy designation, Priority Review designation, and Accelerated Approval. In 2023, 36 of the 55 of CDER's novel drug approvals (65%) used one or more of these expedited programs, which helped bring new therapies to the market sooner.

CDER's Novel Drug Approvals of 2023

In 2023, CDER approved 55 novel drugs, either as new molecular entities (NMEs) under New Drug Applications (NDAs), or as new therapeutic biologics under Biologics License Applications (BLAs). The active ingredient(s) in a novel drug have not been approved in the U.S.

CDER's novel drug approvals for 2023 are listed alphabetically below by trade name.*

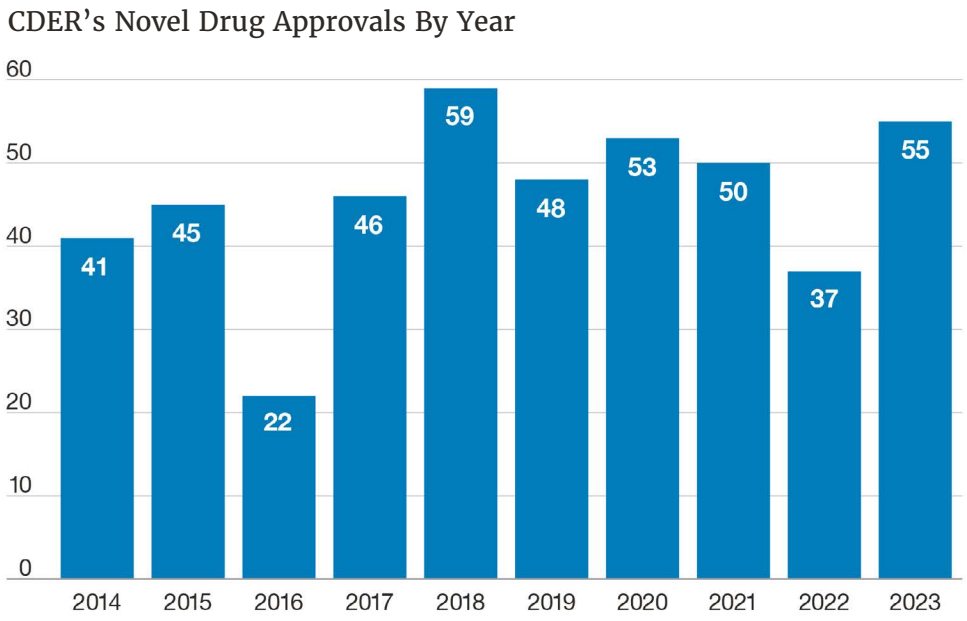
See Appendix B for a summary chart of designations for CDER's novel approvals.

Trade Name	Active Ingredient(s)
Agamree	vamorolone
Aphexda	motixafortide
Augtyro	repotrectinib
Beyfortus	nirsevimab-alip
Bimzelx	bimekizumab-bkzx
Brenzavvy	bexagliflozin
Columvi	glofitamab-gxbm
Daybue	trofinetide
Defencath	taurolidine, heparin
Elfabrio	pegunigalsidase alfa-iwxj
Elrexfio	elranatamab-bcmm
Epkinly	epcoritamab-bysp
Exxua	gepirone
Fabhalta	iptacopan
Filspari	sparsentan
Filsuvez	birch triterpenes
Fruzaqla	fruquintinib
Inpefa	sotagliflozin
Izervay	avacincaptad pegol
Jaypirca	pirtobrutinib
Jesduvroq	daprodustat
Joenja	leniolisib
Lamzede	velmanase alfa-tycv
Leqembi	lecanemab-irmb
Litfulo	ritlecitinib
Loqtorzi	toripalimab-tpzi
Miebo	perfluorohexyloctane
Ngenla	somatrogon-ghla
Ogsiveo	nirogacestat
Ojjaara	momelotinib
Omvoh	mirikizumab-mrkz
Orserdu	elacestrant
Paxlovid	nirmatrelvir, ritonavir (co-packaged)
Pombiliti	cipaglucosidase alfa-atga
Posluma	flotufolastat F 18
Qalsody	tofersen
Rezzayo	rezafungin
Rivfloza	nedosiran
Rystiggo	rozanolixizumab-noli
Ryzneuta	efbemalenograstim alfa-vuxw
Skyclarys	omaveloxolone
Sohonos	palovarotene
Talvey	talquetamab-tgvs
Truqap	capivasertib
Vanflyta	quizartinib
Velsipity	etrasimod
Veopoz	pozelimab-bbfg
Veozah	fezolinetant
Wainua	eplontersen
Xacduro	sulbactam, durlobactam (co-packaged)
Xdemvy	lotilaner
Zavzpret	zavegepant
Zilbrysq	zilucoplan
Zurzuvae	zuranolone
Zynyz	retifanlimab-dlwr

**This information is accurate as of December 31, 2023. In rare instances, CDER may need to change a drug's NME designation or the status of its application as a novel BLA. For instance, new information may become available that could lead to a reconsideration of the original designation or status. If CDER makes these types of changes, the agency intends to communicate the nature of, and the reason for, any revisions as appropriate.*

CDER's Annual Novel Drug Approvals: 2014–2023

The 10-year graph below shows that from 2014 through 2023, CDER has averaged about 46 novel drug approvals per year.



First-in-Class Drugs

CDER identified 20 of the 55 novel drugs approved (36%) in 2023 as first-in-class. These drugs have mechanisms of action different from those of existing therapies.

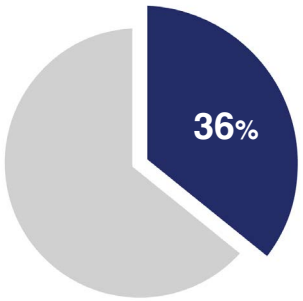
Novel drugs approved in 2023 that CDER identified as first-in-class were:

Daybue, Defencath, Fabhalta, Filspari, Filsuvez, Jesduvroq, Joenja, Lamzede, Miebo, Ogsiveo, Paxlovid, Qalsody, Rivfloza, Skyclarys, Sohonos, Talvey, Truqap, Veopoz, Veozah, Xdemvy

Notable examples of novel first-in-class approvals include:

- **Daybue (trofinetide)** oral solution as the first treatment for patients aged two years and older with Rett syndrome, a rare, genetic neurological disorder that affects brain development.
- **Jesduvroq (daprodustat)** tablets as the first oral treatment for anemia (decreased number of red blood cells) caused by chronic kidney disease for adults receiving dialysis for at least four months.
- **Miebo (perfluorohexyloctane)** ophthalmic solution to treat signs and symptoms of dry eye disease, which occurs when a patient's tears do not provide sufficient eye lubrication, leading to potential ocular (eye-related) inflammation and damage.
- **Paxlovid (nirmatrelvir and ritonavir, co-packaged for oral use)** tablets to treat mild-to-moderate COVID-19 in adults who are at high risk for progression to severe COVID-19, including hospitalization or death. Paxlovid is the first oral antiviral pill approved to treat COVID-19 in adults.

First-in-Class Drugs



CDER identified 20 out of the 55 novel drugs (36%) approved in 2023 as first-in-class.

- **Skyclarys (omaveloxolone)** capsules as the first treatment for Friedreich's ataxia, a rare, inherited, degenerative disease that damages the nervous system, characterized by impaired coordination and difficulty walking.
- **Talvey (talquetamab-tgvs)** injection to treat adults with refractory (treatment-resistant) or relapsed (recurring) multiple myeloma who have received other therapies. It was approved through the Accelerated Approval pathway.
- **Veozah (fezolinetant)** tablets to treat moderate to severe vasomotor symptoms, or hot flashes, due to menopause.
- **Xdemvy (lotilaner)** ophthalmic solution as the first drug to treat Demodex blepharitis, chronic eyelid inflammation caused by Demodex folliculorum, a microscopic mite.

Drugs for Rare Diseases

In 2023, 28 of CDER's 55 novel drug approvals (51%) received orphan drug designation because they target rare diseases (diseases that affect fewer than 200,000 people in the U.S.). Patients with rare diseases often have few or no drugs available to treat their conditions.

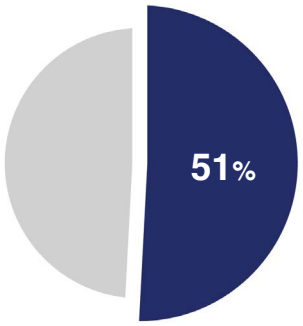
Novel drugs approved in 2023 with the orphan drug designation were:*

Agamree, Aphexda, Augtyro, Daybue, Elrexfio, Fabhalta, Filspari, Filsuvez, Jaypirca, Joenja, Lamzede, Loqtorzi, Ngenla, Ogsiveo, Ojjaara, Pombiliti, Qalsody, Rezzayo, Rivfloza, Rystiggo, Skyclarys, Sohonos, Talvey, Vanflyta, Veopoz, Wainua, Zilbrysq, Zynyz

Examples of novel approvals of 2023 for rare diseases include:

- **Fabhalta (iptacopan)** tablets as the first oral treatment for paroxysmal nocturnal hemoglobinuria, a disease that causes red blood cells to break apart.
- **Filspari (sparsentan)** tablets to reduce proteinuria (elevated protein in the urine) in adults with primary immunoglobulin A (IgA) nephropathy at risk of rapid disease progression. IgA nephropathy is a chronic kidney disease that can lead to kidney failure. Filspari was approved through the Accelerated Approval pathway.
- **Jaypirca (pirtobrutinib)** tablets to treat relapsed or refractory mantle cell lymphoma, an aggressive form of non-Hodgkin lymphoma, after receiving other therapies. Later in 2023, CDER approved Jaypirca to treat adults with chronic lymphocytic leukemia or small lymphocytic lymphoma after receiving other therapies. Both indications were approved through the Accelerated Approval pathway.
- **Joenja (leniolisib)** tablets as the first treatment for activated phosphoinositide 3-kinase delta syndrome (APDS) in patients 12 years and older. APDS is a genetic disorder that impairs the immune system.
- **Lamzede (velmanase alfa)** injection to treat non-central nervous system manifestations of alpha-mannosidosis, a rare genetic condition in which patients do not have the alpha-mannosidase enzyme. Symptoms include intellectual disability, hearing loss, weakened immune system, distinctive facial features, skeletal abnormalities, and muscle weakness.

Drugs for Rare Diseases



More than half (28) of the drugs CDER approved in 2023 received orphan drug designation.

- **Loqtorzi (toripalimab-tpzi)** infusion to treat recurrent or metastatic (spreading) nasopharyngeal carcinoma (NPC) when used together with or following other therapies. NPC is a rare head and neck cancer.
- **Ogsiveo (nirogacestat)** tablets to treat desmoid tumors, which are noncancerous growths in the connective tissue, such as the abdomen, arms, and legs.
- **Ojjaara (momelotinib)** tablets to treat myelofibrosis, a type of bone marrow cancer that causes scar tissue buildup in the bone marrow, disrupting the body's normal production of blood cells.
- **Rezzayo (rezafungin)** injection to treat candidemia and invasive candidiasis, which are serious and life-threatening fungal infections, in patients 18 years or older with limited or no alternative treatments.
- **Rystiggo (rozanolixizumab-noli)** injection for adults with the two most common subtypes of generalized myasthenia gravis, a chronic autoimmune neuromuscular condition that causes muscle weakness and severe fatigue.
- **Sohonos (palovarotene)** capsules to reduce the volume of new heterotopic ossification (bone formation outside the skeleton) in females eight years and older and males 10 years and older with fibrodysplasia ossificans progressiva, a disorder in which bones gradually form in muscle tissue and connective tissue.
- **Veopoz (pozelimab)** injection as the first drug to treat CHAPLE disease, a very rare genetic disease. CHAPLE disease affects the immune system and can be life-threatening.
- **Wainua (eplontersen)** injection to treat hereditary transthyretin-mediated amyloidosis, a genetic disorder that leads to organ and tissue dysfunction.

**Not all drugs for rare diseases necessarily receive orphan designation.*

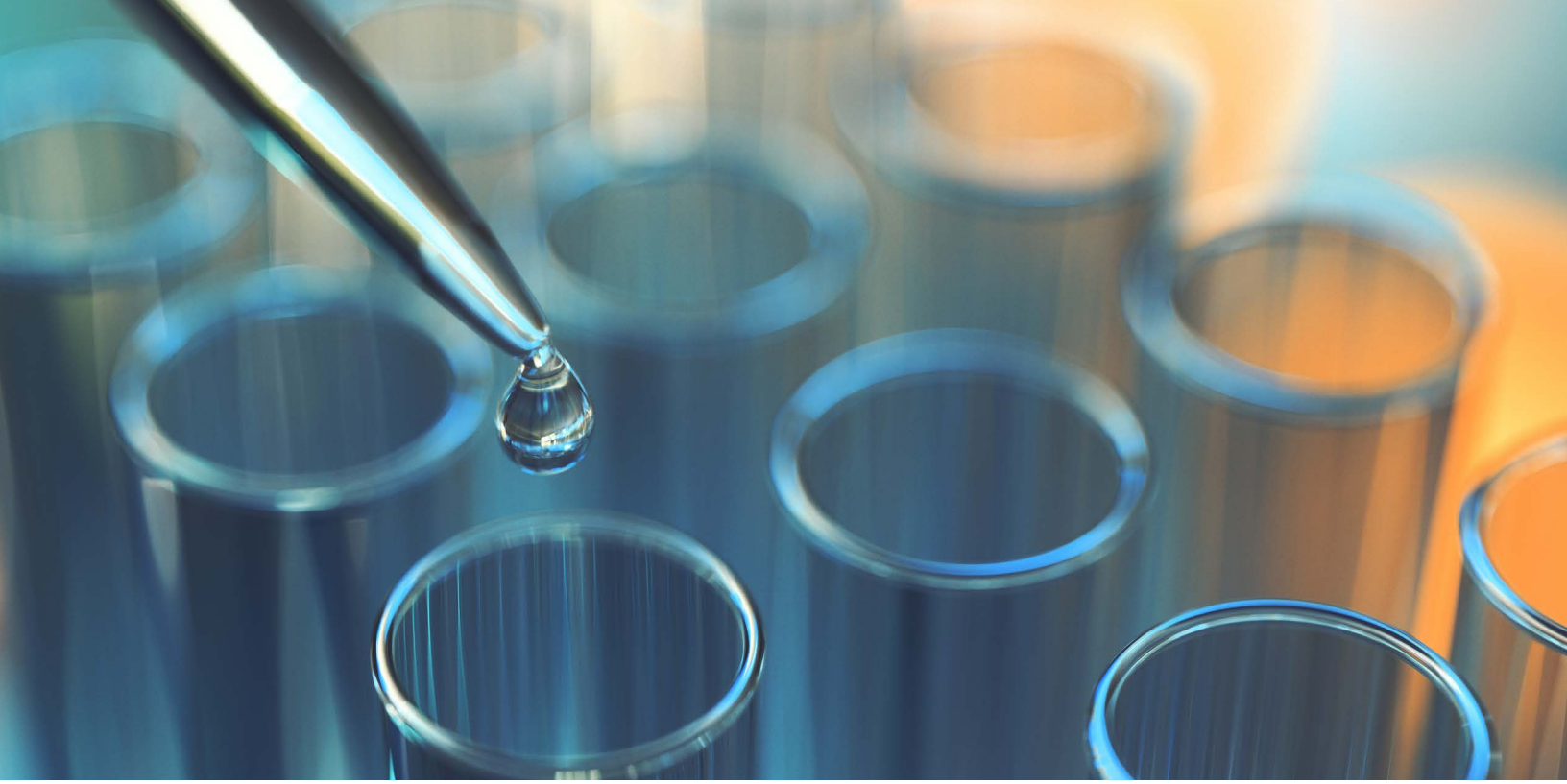
Other Novel Drug Approvals

In addition to the first-in-class and drugs for rare diseases, CDER approved these notable approvals in 2023:

- **Beyfortus (nirsevimab-alip)** injection as the first approved drug to prevent RSV lower respiratory tract disease in all (i.e., not only high-risk) babies born during or entering their first RSV season and certain high-risk children up to 24 months. While most patients experience mild cold-like symptoms, some infants, especially with their first RSV infection, develop lower respiratory tract disease such as pneumonia and bronchiolitis (swelling of the lungs' small airway passages).
- **Columvi (glofitamab-gxbm)** injection to treat types of relapsed or refractory diffuse large B-cell lymphoma (DLBCL) and high-grade B-cell lymphoma. DLBCL is marked by rapid tumor growth in the lymph nodes, spleen, liver, bone marrow, or other tissues and organs. Columvi was approved through the Accelerated Approval pathway.
- **Defencath (taurolidine and heparin)** catheter lock solution to reduce catheter-related bloodstream infections in adults with kidney failure who are receiving chronic hemodialysis (a procedure to purify the blood) through a central venous catheter.

- **Epkinly (epcoritamab-bysp)** injection to treat types of relapsed or refractory DLBCL and high-grade B-cell lymphoma. Epkinly was approved through the Accelerated Approval pathway.
- **Fruzaqla (fruquintinib)** capsules to treat refractory, metastatic colorectal cancer.
- **Izervay (avacincaptad pegol)** injection to treat geographic atrophy secondary to age-related macular degeneration (AMD). Geographic atrophy is an advanced form of AMD that leads to progressive and irreversible retinal cell loss and permanent vision loss.
- **Leqembi (lecanemab-irmb)** injection to treat Alzheimer's disease, a progressive neurologic disorder that is the most common cause of dementia. Leqembi was approved through the Accelerated Approval pathway and later converted to traditional approval after clinical benefit was confirmed, both in 2023.
- **Orserdu (elacestrant)** tablets to treat breast cancer with certain genetic features and disease progression after at least one line of endocrine therapy.
- **Xacduro (sulbactam and durlobactam)** injection to treat hospital-acquired bacterial pneumonia and ventilator-associated bacterial pneumonia caused by Acinetobacter baumannii-calcoaceticus complex.
- **Zavzpret (zavegepant)** nasal spray to treat acute migraine with or without aura (a migraine warning sign).
- **Zurzuvae (zuranolone)** tablets as the first oral medication to treat postpartum depression, a complex mix of physical, emotional, and behavioral changes that can occur around childbirth.

CDER approved the first oral medication for postpartum depression in 2023.



Innovation: Use of Expedited Development and Review Pathways

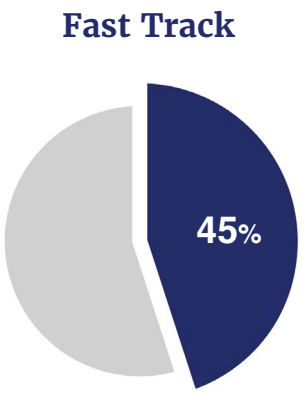
CDER used diverse regulatory approaches to enhance and expedite drug review in 2023. These approaches enable increased flexibility, efficiency, and interactions between CDER staff and drug developers. They often also allow shorter review times to speed the availability of new therapies to patients with serious conditions, especially in cases where there are no satisfactory alternatives, while preserving FDA's rigorous standards for safety and effectiveness.

Fast Track

CDER granted Fast Track status to 25 of the 55 novel drugs (45%) in 2023. Fast Track speeds development and review of new drugs and biologics by increasing the level of communication between FDA and drug developers and by enabling CDER to review portions of a drug application on a rolling basis.

Drugs granted Fast Track status were:

Agamree, Beyfortus, Columvi, Daybue, Defencath, Elfabrio, Elrexfio, Filsuvez, Fruzaqla, Izervay, Jaypirca, Lamzede, Leqembi, Ogsiveo, Orserdu, Paxlovid, Qalsody, Rezzayo, Skyclarys, Truqap, Vanflyta, Veopoz, Xacduro, Zurzuvae, Zynyz



CDER designated 25 of the 55 novel drugs (45%) as Fast Track.

Breakthrough Therapy

CDER designated 9 of the 55 novel drugs (16%) in 2023 as Breakthrough Therapies. A Breakthrough Therapy designation includes all the Fast Track program features and offers intensive FDA guidance during drug development, including involvement from senior managers.

Drugs designated with Breakthrough Therapy status were:

Elrexfio, Fabhalta, Izervay, Leqembi, Loqtorzi, Ogsiveo, Pombiliti, Rivfloza, Talvey

Priority Review

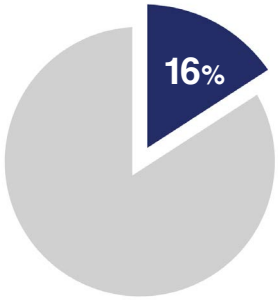
In 2023, 31 of the 55 novel drugs approved (56%) were designated Priority Review. A drug receives Priority Review if CDER determines that the drug treats a serious condition and, if approved, would provide a significant improvement in safety or effectiveness of the treatment, diagnosis, or prevention of the serious condition. Generally speaking, a Priority Review application is one on which CDER aims to take action within six months of filing (compared to a target date of 10 months under standard review).

(In some instances, sponsors may redeem a priority review voucher under CDER's Priority Review Voucher program. Such drugs are not included in the list below, and do not meet Priority Review criteria.)

Drugs designated Priority Review were:

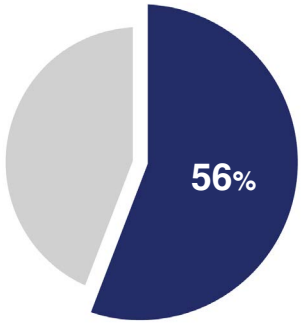
Augtyro, Columvi, Daybue, Defencath, Elfabrio, Elrexfio, Epkinly, Filspari, Filsuvez, Fruzaqla, Izervay, Jaypirca, Joenja, Lamzede, Leqembi, Loqtorzi, Ogsiveo, Orserdu, Paxlovid, Qalsody, Rezzayo, Rystiggo, Skyclarys, Sohonos, Talvey, Truqap, Vanflyta, Veopoz, Xacduro, Zurzuvae, Zynyz

Breakthrough Therapy



CDER identified 9 of the 55 novel drugs (16%) approved in 2023 as Breakthrough Therapies.

Priority Review



31 of the 55 drugs (56%) approved in 2023 were designated as Priority Review.

Accelerated Approval

CDER approved 9 of the 55 novel drugs (16%) in 2023 under the Accelerated Approval pathway. This program aims to bring drugs to market that can provide treatment for unmet medical needs on a faster timeline than would be possible following a traditional approval pathway. Accelerated Approval may be an option for a new drug intended to treat a serious condition that offers a meaningful advantage over available therapies. For drugs eligible to follow the Accelerated Approval pathway, a determination of safety and effectiveness may be made based not on measures of direct clinical benefit, but rather on one of two alternative endpoints: (1) a surrogate endpoint that is reasonably likely to predict clinical benefit; or (2) an intermediate clinical endpoint that is reasonably likely to predict clinical benefit. Use of such endpoints may enable the drug to be studied for a shorter treatment duration and receive Accelerated Approval based on these findings. Importantly, however, for products approved under the Accelerated Approval pathway, FDA requires post-approval studies designed to confirm clinical benefit, and, among other things, may withdraw an accelerated approval product from the market for failure to confirm clinical benefit.

The novel drugs approved via Accelerated Approval were:

Columvi, Elrexfio, Epkinly, Filspari, Jaypirca, Leqembi,* Qalsody, Talvey, Zynyz

**Was converted to traditional approval in 2023 as well.*

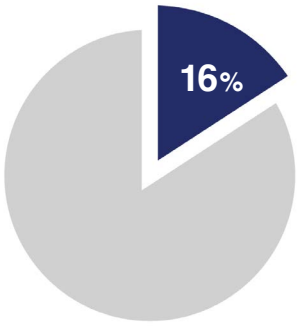
Overall Use of Expedited Development and Review Methods

Thirty-six of the 55 novel drug approvals of 2023 (65%) used one or more expedited programs, specifically Fast Track designation, Breakthrough Therapy designation, Priority Review designation, or Accelerated Approval.

Novel drugs approved in 2023 that used at least one expedited program were:

Agamree, Augtyro, Beyfortus, Columvi, Daybue, Defencath, Elfabrio, Elrexfio, Epkinly, Fabhalta, Filspari, Filsuvez, Fruzaqla, Izervay, Jaypirca, Joenja, Lamzede, Leqembi, Loqtorzi, Ogsiveo, Orserdu, Paxlovid, Pombiliti, Qalsody, Rezzayo, Rivfloza, Rystiggo, Skyclarys, Sohonos, Talvey, Truqap, Vanflyta, Veopoz, Xacduro, Zurzuvae, Zynyz

Accelerated Approval



CDER identified 9 of the 55 novel drugs (16%) as Accelerated Approvals.

CDER used at least 1 expedited program to speed approval of 65% of all novel drugs approved in 2023.

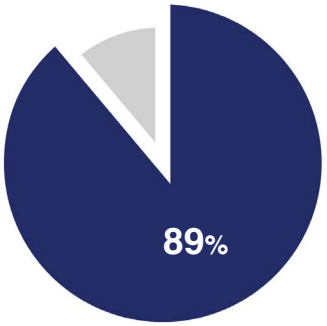
Predictability: Meeting PDUFA Goals

Under PDUFA, industry is assessed user fees that provide resources to CDER to expand capabilities for review activities. With PDUFA, applications are reviewed targeting specific timeframes. Throughout 2023, CDER met or exceeded the PDUFA goal date for taking action on 89% (49 of 55) of the novel drugs approved.

Novel drugs approved in 2023 on or before their PDUFA goal dates were:

Agamree, Aphexda, Augtyro, Beyfortus, Brenzavvy, Columvi, Daybue, Defencath, Elfabrio, Elrexfio, Epkinly, Exxua, Fabhalta, Filspari, Filsuvez, Fruzaqla, Inpefa, Izervay, Jaypirca, Jesduvroq, Joenja, Lamzede, Leqembi, Litfulo, Miebo, Ogsiveo, Ojjaara, Omvoh, Orserdu, Paxlovid, Posluma, Qalsody, Rezzayo, Rivfloza, Skyclarys, Sohonos, Talvey, Truqap, Vanflyta, Velsipity, Veopoz, Veozah, Wainua, Xacduro, Xdemvy, Zavzpret, Zilbrysq, Zurzuvae, Zynyz

Meeting PDUFA Goals



In 2023, 49 out of the 55 novel drugs (89%) were approved on or before their PDUFA goal date.

Access: First Cycle Approvals and First in U.S. Approvals

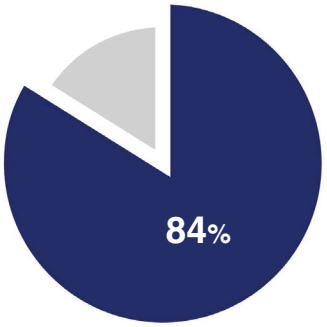
First Cycle Approvals

CDER approved 46 of the 55 novel drugs of 2023 (84%) on the "first cycle" of review. This high percentage is in part reflective of the extent to which CDER staff provide clarity to drug developers on the necessary study design elements and other data needed in the drug application to support a full and comprehensive drug assessment.

Novel drugs approved in 2023 on the first cycle were:

Agamree, Aphexda, Augtyro, Beyfortus, Brenzavvy, Columvi, Daybue, Elrexfio, Epkinly, Fabhalta, Filspari, Fruzaqla, Inpefa, Izervay, Jaypirca, Jesduvroq, Joenja, Lamzede, Leqembi, Litfulo, Miebo, Ogsiveo, Ojjaara, Orserdu, Paxlovid, Pombiliti, Posluma, Qalsody, Rezzayo, Rivfloza, Rystiggo, Ryzneuta, Skyclarys, Talvey, Truqap, Vanflyta, Velsipity, Veopoz, Veozah, Wainua, Xacduro, Xdemvy, Zavzpret, Zilbrysq, Zurzuvae, Zynyz

First Cycle Approvals



In 2023, CDER approved 46 of the 55 novel drugs (84%) on the first cycle.

Approval in the U.S. Before Other Countries

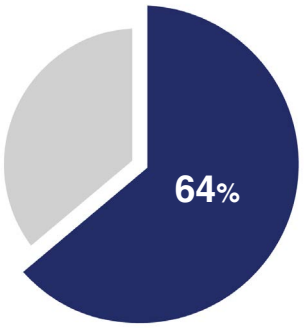
Thirty-five of the 55 novel drugs approved in 2023 (64%) were approved in the U.S. before any other country.

Novel drugs of 2023 approved first in the U.S. were:

Agamree, Aphexda, Augtyro, Brenzavvy, Daybue, Elrexfio, Epkinly, Exxua, Fabhalta, Filspari, Izervay, Jaypirca, Joenja, Leqembi, Litfulo, Ogsiveo, Ojjaara, Orserdu, Posluma, Qalsody, Rezzayo, Rivfloza, Rystiggo, Skyclarys, Talvey, Truqap, Velsipity, Veopoz, Veozah, Wainua, Xacduro, Xdemvy, Zavzpret, Zurzuvae, Zynyz

64% of the novel drugs approved in 2023 were approved in the U.S. before any other country.

First in the U.S.



35 of the 55 novel drugs approved in 2023 were first approved in the U.S.

New Uses of Approved Drugs

After CDER approves a new treatment, a drug sponsor may generate new data about the product that suggests an additional use. The drug sponsor may then submit an application to modify or expand the use of an approved drug based on this new data.

The products below are some 2023 CDER approvals for new uses or indications of an approved drug:

- **Ayvakit (avapritinib)** tablets were first approved in 2020 to treat types of gastrointestinal stromal tumors. Ayvakit was approved in 2023 for indolent systemic mastocytosis, a rare disorder that results in the buildup of too many mast cells (a type of white blood cell).
- **Eylea (aflibercept)** injection was originally approved in 2011 for neovascular (wet) age-related macular degeneration. In 2023, CDER approved Eylea to treat retinopathy of prematurity (ROP), an eye disease that can occur in babies born prematurely. In ROP, abnormal blood vessels grow in the retina and can lead to vision loss.
- **Ilaris (canakinumab)** injection was originally approved in 2009. In 2023, CDER approved Ilaris to treat gout flares in adults in whom certain other therapies are not advised. Gout is a type of arthritis characterized by sudden attacks of pain, swelling, and redness in one or more joints.
- **Jemperli (dostarlimab-gxly)** injection was initially approved in 2021. In 2023, CDER approved Jemperli for two endometrial cancer uses, as a single agent and in combination with other therapies. Endometrial cancer occurs in the tissues of the endometrium, which is the lining of the uterus.
- **Kevzara (sarilumab)** injection was first approved in 2017 for moderately to severely active rheumatoid arthritis. In 2023, CDER approved it for adults with polymyalgia rheumatica (PMR) who did not adequately respond to corticosteroids or who cannot tolerate corticosteroid taper (gradual dose reduction). PMR is an inflammatory disorder that causes muscle pain and stiffness around the shoulders and hips.
- **Keytruda (pembrolizumab)** injection was first approved in 2014. In 2023, CDER approved Keytruda for several new uses, including in combination with Padcev for patients with locally advanced or metastatic urothelial carcinoma who are not eligible for a type of chemotherapy; for types of non-small cell lung cancer as an adjuvant (after other therapies) therapy; and for early-stage non-small cell lung cancer as a neoadjuvant (before other therapies) and adjuvant treatment.
- **Linzess (linaclotide)** capsules were first approved in 2012. In 2023, CDER approved it as the first treatment for pediatric functional constipation in patients six to 17 years. Pediatric functional constipation occurs when patients have infrequent bowel movements with hard stools that can be difficult or painful to pass.
- **Lonsurf (tipiracil hydrochloride and trifluridine)** tablets were first approved in 2015. In 2023, CDER approved Lonsurf, alone or in combination with another therapy (bevacizumab), for patients with previously treated metastatic colorectal cancer.

- **Lynparza (olaparib)** tablets were first approved in 2014. In 2023, Lynparza was approved in combination with another therapy for adults with a type of prostate cancer that is metastatic and castration-resistant (i.e., keeps growing when the amount of testosterone is reduced to very low levels).
- **Padcev (enfortumab vedotin-ejfv)** injection was first approved in 2019 as a single agent. CDER approved Padcev in combination with Keytruda in 2023 to treat adults with locally advanced or metastatic urothelial cancer.
- **Polivy (polatuzumab vedotin-piiq)** injection was first approved in 2019 to treat adults with relapsed or refractory diffuse large B-cell lymphoma (DLBCL), not otherwise specified, after at least two therapies. In 2023, CDER expanded the indication for adults with previously untreated DLBCL, not otherwise specified, or high-grade B-cell lym-phoma that is considered low-intermediate risk or higher.
- **Rexulti (brexpiprazole)** tablets were initially approved in 2015 as an add-on therapy for major depressive disorder and as a treatment for schizophrenia. In 2023, CDER approved Rexulti as the first drug to treat agitation associated with dementia due to Alzheimer's disease.
- **Rinvoq (upadacitinib)** tablets were originally approved in 2019 for adults with moderately to severely active rheumatoid arthritis. In 2023, CDER approved Rinvoq for adults with moderately to severely active Crohn's disease with an inadequate response or intolerance to other therapies (specifically TNF blockers). Rinvoq is the first approved oral product for moderately to severely active Crohn's disease, a chronic disease that causes inflammation in the digestive tract.
- **Talzenna (talazoparib)** capsules were first approved in 2018 to treat types of breast cancer. In 2023, Talzenna was approved in combination with another therapy to treat a type of metastatic castration-resistant prostate cancer.
- **Tukysa (tucatinib)** tablets were first approved in 2020. In 2023, CDER approved Tukysa for a type of colorectal cancer that has progressed following certain chemotherapies.
- **Verzenio (abemaciclib)** tablets were initially approved in 2017. In 2023, CDER approved Verzenio with endocrine therapy (tamoxifen or an aromatase inhibitor) for a type of early breast cancer with a high risk of recurrence.

Approved Drugs Expanded for New Pediatric Populations

Section 505B of the Federal Food, Drug, and Cosmetic Act (FD&C Act) (often referred to by the legislation that originally created it, the Pediatric Research Equity Act, or PREA) and section 505A of the FD&C Act (often referred to by the legislation that originally created it, the Best Pharmaceuticals for Children Act, or BPCA) give CDER the authority to require (PREA) or request (BPCA) pediatric studies under certain circumstances. These two laws have been largely responsible for the inclusion of pediatric information in the labeling for many drugs.

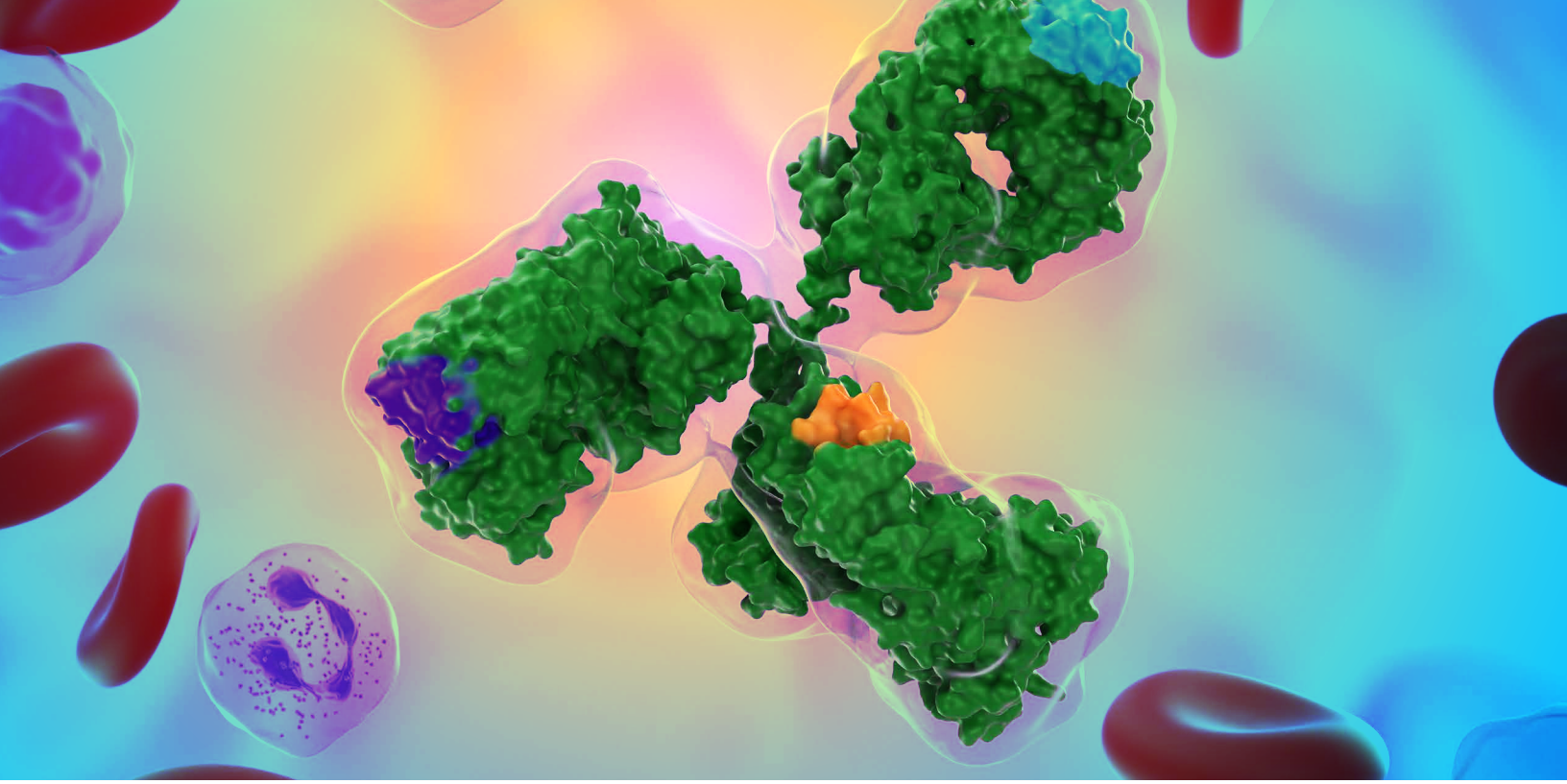
Upon drug approval, CDER may require pediatric studies of that drug under PREA. In response to that requirement, sponsors may submit new data to support the safe and effective use of the drug in the pediatric population studied. Sponsors submit this data in an application to expand the patient population. Under BPCA, sponsors may obtain additional marketing exclusivity for pediatric studies requested in a Pediatric Written Request.

The products below are examples of 2023 approvals for drugs expanded to include new pediatric populations:

- **Cresemba (isavuconazonium sulfate)** injection was initially approved in 2015 to treat invasive aspergillosis and invasive mucormycosis, which are fungal diseases. In 2023, CDER extended Cresemba's patient population to include patients one year and older.
- **Evkeeza (evinacumab-dgnb)** injection was initially approved in 2021 as an add-on to other therapies to treat patients 12 years and older with homozygous familial hypercholesterolemia, a genetic disease that causes extremely high cholesterol levels. Evkeeza's patient population was extended to individuals five years and older in 2023.
- **Jardiance (empagliflozin)** tablets were approved in 2014 as an addition to diet and exercise to improve blood sugar in adults with type 2 diabetes. In 2023, CDER expanded the patient population to children 10 years and older.
- **Livmarli (maralixibat)** oral solution was approved in 2021 to treat cholestatic pruritus (extreme itching) in patients one year and older with Alagille syndrome, a genetic disease affecting the liver, heart, kidneys, and other organs. In 2023, CDER expanded the patient population to babies three months and older.
- **Mekinist (trametinib)** tablets were first approved in 2013 and are now approved for various oncology indications in combination with Tafinlar [see page 19]. In 2023, the patient population was extended to one year and older.
- **Opdivo (nivolumab)** injection was originally approved in 2014 and has subsequently been approved for certain oncology indications. In 2023, CDER extended the patient population to 12 years and older for certain indications.
- **Revatio (sildenafil citrate)** tablets had been approved to treat a type of pulmonary arterial hypertension (PAH) in adults. In 2023, CDER expanded the population to include pediatric patients. PAH is a rare, progressive disorder characterized by high blood pressure in the lungs' arteries.

- **Rozlytrek (entrectinib)** capsules were first approved in 2019 for several oncology indications, including solid tumors with a NTRK gene fusion (where two genes form one hybrid gene) in patients 12 years and older. In 2023, CDER extended the patient population for this indication down to one month. CDER has also approved a new oral pellet formulation for Rozlytrek.
- **Synjardy (empagliflozin and metformin hydrochloride)** tablets were first approved in 2015 as an addition to diet and exercise to improve blood sugar in adults with type 2 diabetes whose disease meets certain criteria. CDER extended the patient population to children 10 years and older in 2023.
- **Tafinlar (dabrafenib)** tablets were first approved in 2013 and are now approved for various oncology indications in combination with Mekinist [see page 18]. In 2023, the patient population was extended to one year and older.
- **Takhzyro (lanadelumab-flyo)** injection was initially approved in 2018 to prevent attacks of hereditary angioedema, a disorder characterized by recurrent episodes of severe swelling, in patients 12 years and older. CDER expanded the patient population to two years and older in 2023.
- **Trikafta (elexacaftor, tezacaftor, and ivacaftor)** tablets were first approved in 2019 to treat cystic fibrosis in patients 12 years and older. In 2023, CDER extended the patient population down to two years.
- **Triumeq Pd (abacavir, dolutegravir, and lamivudine)** tablets for oral suspension were first approved in 2022 to treat HIV-1 infection in adults and children who weigh at least 10 kg. In 2023, the pediatric population was extended down to children who are at least three months old and weigh at least six kg.
- **Yervoy (ipilimumab)** injection was first approved in 2011. In 2023, CDER expanded the population to include patients 12 years and older to treat unresectable or metastatic melanoma in combination with Opdivo [see page 18].
- **Zinplava (bezlotoxumab)** injection was initially approved in 2016 to reduce recurrence of Clostridioides difficile infection (CDI) in patients 18 years or older receiving antibacterial drug treatment at a high risk for recurrence. In 2023, CDER expanded the patient population to children one year and older. CDI is a bacteria-caused infection that results in colon inflammation.

In 2023, CDER approved a new class of medicines to treat pediatric type 2 diabetes.



Biosimilar Approvals

The biosimilar pathway is an abbreviated approval pathway for biologics that are highly similar to and have no clinically meaningful differences from an FDA-approved biological reference product. This pathway was established to provide more treatment options, increase patient access, and potentially reduce the cost of therapies through competition.

In 2023, CDER approved five new biosimilars for five reference products, including three reference products that did not have a corresponding biosimilar. Several drugs were approved as interchangeable biosimilars, which are biosimilars that may be substituted for the reference product at the pharmacy similar to how generics are substituted, subject to state law.

In 2023, CDER approved 5 biosimilars for 5 reference products.

- **Avzivi (bevacizumab-tnjn)** injection was approved to treat types of metastatic colorectal cancer and hepatocellular carcinoma *(reference product: Avastin)*.
- **Tofidence (tocilizumab-bavi)** injection was approved to treat types of arthritis, including rheumatoid arthritis, polyarticular juvenile idiopathic arthritis, and systemic juvenile idiopathic arthritis *(reference product: Actemra)*.
- **Tyruko (natalizumab-sztn)** injection was approved as a monotherapy (a single therapy) to treat relapsing forms of multiple sclerosis and to induce and maintain a clinical response in adults with moderately to severely active Crohn's disease whose condition meets certain criteria *(reference product: Tysabri)*.
- **Wezlana (ustekinumab-auub)** injection was approved to treat plaque psoriasis and psoriatic arthritis in patients six years and older, and Crohn's disease and ulcerative colitis in adults *(reference product: Stelara)*. Wezlana was approved as an interchangeable biosimilar.

- **Yuflyma (adalimumab-aaty)** injection was approved to treat a variety of inflammatory conditions *(reference product: Humira)*.

In 2023, CDER also approved notable changes to the following biosimilars:

- **Abrilada (adalimumab-afzb)** injection was originally approved in 2019 for several inflammatory conditions. In 2023, CDER approved it as an interchangeable biosimilar *(reference product: Humira)*.
- **Byoovis (ranibizumab-nuna)** injection was initially approved in 2021 to treat certain eye conditions. In 2023, CDER approved it as an interchangeable biosimilar *(reference product: Lucentis)*.

CDER has approved a total of 45 biosimilars for 14 different reference products since 2015. This includes at least one biosimilar for each of these top selling biologics in the U.S.: nine biosimilars to Humira; six biosimilars to Neulasta; five biosimilars to Herceptin and Avastin; four biosimilars to Remicade; three biosimilars to Rituxan and Neupogen; two biosimilars to Lantus, Enbrel, and Lucentis; and one biosimilar to Epogen/Procit, Actemra, Stelara, and Tysabri. Multiple biosimilars for an approved reference product can enhance competition, which may lead to reduced costs for both patients and our health care system.

Other Important Approvals

New formulations of approved drugs can offer significant therapeutic advances. Similarly, new dosage forms (such as from a capsule to a chewable tablet for those unable to swallow pills) can help increase adherence, make sure patients take the proper dose, and improve quality of life for patients who must use the medication on a prolonged basis. Making a drug available as an over-the-counter product can also increase patient access to therapies. Below are examples of new formulations, new dosage forms, over-the-counter actions, and other notable approval actions of 2023:

- **Airsupra (albuterol and budesonide)** inhalation aerosol for as-needed treatment or prevention of bronchoconstriction (tightening of lung airways) and to reduce the risk of asthma attacks in adults. Airsupra is a combination product of two approved active ingredients, albuterol and budesonide.
- **Akeega (niraparib and abiraterone acetate)** tablets to treat a type of prostate cancer that meets certain disease criteria, together with prednisone (a steroid). Akeega is a combination product of two approved active ingredients, niraparib and abiraterone.
- **Brixadi (buprenorphine)** extended-release injection to treat moderate-to-severe opioid use disorder. Brixadi is now approved in both weekly and monthly under-the-skin injectable formulations at varying doses, including lower doses that may be appropriate for those who do not tolerate higher doses of extended-release buprenorphine.
- **Entyvio (vedolizumab)** injection was approved in 2014 for intravenous (into the vein) administration to treat moderately to severely active ulcerative colitis and Crohn's disease in adults. In 2023, CDER approved Entyvio as an under-the-skin injection to treat moderately to severely active ulcerative colitis in certain adults. This will allow patients to self-administer the medication after training, avoiding needing to go to an infusion center.
- **Hepzato (melphalan hydrochloride)** injection. Melphalan, the active ingredient, has been approved for different oncology indications. In 2023, CDER approved Hepzato with the same active ingredient to treat liver metastases (metastatic growths) in certain patients with uveal melanoma, a rare cancer that develops in a part of the eye.
- **Lampit (nifurtimox)** tablets were converted from Accelerated Approval to full approval for use in pediatric patients from birth to younger than 18 years to treat Chagas disease (American Trypanosomiasis) caused by the Trypanosoma cruzi parasite. Chagas disease can cause heart, digestive, and neurological problems and may be life-threatening.
- **Lodoco (colchicine)** tablets. Lodoco, with the previously approved active ingredient colchicine, was approved in 2023 to reduce the risk of certain cardiovascular events in adults with established atherosclerotic disease (thickening of the arteries) or with multiple risk factors for cardiovascular disease.
- **Mydcombi (tropicamide and phenylephrine hydrochloride)** ophthalmic spray, a combination of two approved active ingredients, was approved in 2023 to induce mydriasis (pupil dilation) for diagnostic procedures and in other conditions.

In 2023, CDER converted Lampit tablets from Accelerated Approval to full approval to treat Chagas disease.

- **Narcan (naloxone hydrochloride)** nasal spray had been approved as a prescription drug to reverse the effects of opioid overdose. In 2023, CDER approved Narcan as a nonprescription drug.
- **Opill (norgestrel)** tablets had been approved as a prescription drug to prevent pregnancy. In 2023, CDER approved Opill as the first nonprescription daily oral contraceptive.
- **Opvee (nalmefene hydrochloride)** nasal spray was approved in a new dosage form in 2023 to reverse the effects of opioid overdose. Nalmefene hydrochloride (the active ingredient) had been approved as an injection drug.
- **Prevymis (letermovir)** tablets and injection were first approved in 2017 to prevent cytomegalovirus (CMV) infection and disease in adults at high risk for CMV who received an allogeneic (from a donor) hematopoietic stem cell transplant. In 2023, CDER approved Prevymis for the same indication in adults at high risk for CMV who received a kidney transplant.
- **RiVive (naloxone hydrochloride)** nasal spray was approved in 2023 as a nonprescription drug to reverse the effects of opioid overdose. Its active ingredient, naloxone hydrochloride, had been approved for prescription use.
- **Ryzumi (phentolamine)** ophthalmic solution was approved in 2023 to treat pharmacologically induced mydriasis (eye dilation). CDER had previously approved its active ingredient, phentolamine.
- **Syfovre (pegcetacoplan)** injection. Pegcetacoplan, the active ingredient, was first approved as an under-the-skin injection in 2021. In 2023, CDER approved Syfovre for intravitreal use (a shot directly into the eye) to treat geographic atrophy resulting from AMD.
- **Technegas (kit for preparation of technetium Tc 99m labeled carbon)** inhalation aerosol was approved as a radioactive diagnostic agent for visualization of lung ventilation and evaluation of pulmonary embolism (a blockage in the pulmonary arteries), when paired with perfusion imaging. Other dosage forms of technetium Tc 99m have been previously approved.
- **Zepbound (tirzepatide)** injection. In 2023, CDER approved Zepbound, with the previously approved active ingredient tirzepatide, for chronic weight management, in addition to a reduced-calorie diet and increased physical activity, in adults with obesity or overweight and at least one weight-related comorbid condition.

Please note that all drugs carry risks and patients should review the drug labeling and consult with their health care professional to determine their preferred course of treatment.

Conclusion

Reviewing a drug application — whether for a novel drug or a supplemental approval — is a collaborative, well-coordinated process that involves scientific, regulatory, and policy experts from throughout CDER and sometimes other parts of the agency. For each application, we perform a very careful and diligent analysis of safety and effectiveness data, including a benefit-risk analysis that factors in the severity of the disease or condition, the currently available treatment options, and the intended patient population. If the therapy meets the standard for approval, we must reach agreement on the indication, labeling, safety issues, and other considerations.

We often consult outside scientific experts, patients and patient advocates, industry representatives, academics, and other community members who are involved in drug development and review. Each of these parties has their unique expertise and perspective, and we consider their viewpoints. We take our regulatory decision-making seriously, because we know our decisions affect the health and well-being of patients and consumers nationwide.

Appendix A: CDER's Novel Approvals of 2023
(in alphabetical order)

For information about vaccines, allergenic products, blood and blood products, cellular and gene therapy products, go to the 2023 Biologics License Application Approvals.

Approval Date	Proprietary Name	Active Ingredient(s)	Summary of FDA-approved use on approval date (see Drugs@FDA for complete indication)	Dosage Form
10/26/2023	Agamree	vamorolone	To treat Duchenne muscular dystrophy	Oral suspension
9/8/2023	Aphexda	motixafortide	To use with filgrastim to mobilize hematopoietic stem cells to peripheral blood for collection and subsequent autologous transplantation in patients with multiple myeloma	Injection
11/15/2023	Augtyro	repotrectinib	To treat ROS1-positive non-small cell lung cancer	Capsule
7/17/2023	Beyfortus	nirsevimab-alip	To prevent respiratory syncytial virus (RSV) lower respiratory tract disease	Injection
10/17/2023	Bimzelx	bimekizumab-bkzx	To treat moderate-to-severe plaque psoriasis	Injection
1/20/2023	Brenzavvy	bexagliflozin	To improve glycemic control in type 2 diabetes mellitus as an adjunct to diet and exercise	Tablet
6/15/2023	Columvi	glofitamab-gxbm	To treat diffuse large B-cell lymphoma, not otherwise specified or large B-cell lymphoma arising from follicular lymphoma, after two or more lines of systemic therapy	Injection
3/10/2023	Daybue	trofinetide	To treat Rett syndrome	Oral solution
11/15/2023	Defencath	taurolidine, heparin	To prevent catheter-related bloodstream infection in patients on dialysis using a central venous catheter	Catheter lock solution
5/9/2023	Elfabrio	pegunigalsidase alfa-iwxj	To treat confirmed Fabry disease	Injection
8/14/2023	Elrexfio	elranatamab-bcmm	To treat relapsed or refractory multiple myeloma after at least four lines of therapy	Injection
5/19/2023	Epkinly	epcoritamab-bysp	To treat relapsed or refractory diffuse large B-cell lymphoma, not otherwise specified, and high-grade B-cell lymphoma after two or more lines of systemic therapy	Injection
9/22/2023	Exxua	gepirone	To treat major depressive disorder	Tablet

Appendix A (continued)

Approval Date	Proprietary Name	Active Ingredient(s)	Summary of FDA–approved use on approval date (see Drugs@FDA for complete indication)	Dosage Form
12/5/2023	Fabhalta	iptacopan	To treat paroxysmal nocturnal hemoglobinuria	Capsule
2/17/2023	Filspari	sparsentan	To reduce proteinuria in primary immunoglobulin A nephropathy at risk of rapid disease progression	Tablet
12/18/2023	Filsuvez	birch triterpenes	To treat wounds associated with dystrophic and junctional epidermolysis bullosa	Gel
11/8/2023	Fruzaqla	fruquintinib	To treat refractory metastatic colorectal cancer	Capsule
5/26/2023	Inpefa	sotagliflozin	To reduce the risk of cardiovascular death, hospitalization for heart failure, and urgent heart failure visits	Tablet
8/4/2023	Izervay	avacincaptad pegol	To treat geographic atrophy secondary to age-related macular degeneration	Intravitreal solution
1/27/2023	Jaypirca	pirtobrutinib	To treat relapsed or refractory mantle cell lymphoma after at least two lines of systemic therapy, including a BTK inhibitor	Tablet
2/1/2023	Jesduvroq	daprodustat	To treat anemia caused by chronic kidney disease	Tablet
3/24/2023	Joenja	leniolisib	To treat activated phosphoinositide 3-kinase delta syndrome	Tablet
2/16/2023	Lamzede	velmanase alfa-tycv	To treat non-central nervous system manifestations of alpha-mannosidosis	Injection
1/6/2023	Leqembi	lecanemab-irmb	To treat Alzheimer's disease	Injection
6/23/2023	Litfulo	ritlecitinib	To treat severe alopecia areata	Capsule
10/27/2023	Loqtorzi	toripalimab-tpzi	To treat recurrent or metastatic nasopharyngeal carcinoma with or following other therapies	Injection
5/18/2023	Miebo	perfluorohexyloctane	To treat signs and symptoms of dry eye disease	Ophthalmic solution
6/27/2023	Ngenla	somatrogon-ghla	To treat growth failure due to inadequate secretion of endogenous growth hormone	Injection
11/27/2023	Ogsiveo	nirogacestat	To treat desmoid tumors	Tablet
9/15/2023	Ojjaara	momelotinib	To treat intermediate or high-risk myelofibrosis	Tablet
10/26/2023	Omvoh	mirikizumab-mrkz	To treat ulcerative colitis	Injection

Appendix A (continued)

Approval Date	Proprietary Name	Active Ingredient(s)	Summary of FDA-approved use on approval date (see Drugs@FDA for complete indication)	Dosage Form
1/27/2023	Orserdu	elacestrant	To treat certain types of advanced or metastatic breast cancer with disease progression following at least one line of endocrine therapy	Tablet
5/25/2023	Paxlovid	nirmatrelvir, ritonavir (co-packaged)	To treat mild-to-moderate COVID-19 at high risk for progression to severe COVID-19	Tablet
9/28/2023	Pombiliti	cipaglucosidase alfa-atga	To treat late-onset Pompe disease with miglustat	Injection
5/25/2023	Posluma	flotufolastat F 18	To use with positron emission tomography for prostate cancer imaging	Injection
4/25/2023	Qalsody	tofersen	To treat a form of amyotrophic lateral sclerosis	Injection
3/22/2023	Rezzayo	rezafungin	To treat candidemia and invasive candidiasis	Injection
9/29/2023	Rivfloza	nedosiran	To lower urinary oxalate levels in primary hyperoxaluria type 1 and relatively preserved kidney function	Injection
6/26/2023	Rystiggo	rozanolixizumab-noli	To treat generalized myasthenia gravis	Injection
11/16/2023	Ryzneuta	efbemalenograstim alfa-vuxw	To decrease the incidence of infection, as manifested by febrile neutropenia, associated with myelosuppressive anti-cancer drugs	Injection
2/28/2023	Skyclarys	omaveloxolone	To treat Friedreich's ataxia	Capsule
8/16/2023	Sohonos	palovarotene	To reduce the volume of new heterotopic ossification in fibrodysplasia ossificans progressiva	Capsule
8/9/2023	Talvey	talquetamab-tgvs	To treat relapsed or refractory multiple myeloma after at least four therapies	Injection
11/16/2023	Truqap	capivasertib	To treat breast cancer that meets certain disease criteria	Tablet
7/20/2023	Vanflyta	quizartinib	To use as part of a treatment regimen for newly diagnosed acute myeloid leukemia that meets certain criteria	Tablet
10/12/2023	Velsipity	etrasimod	To treat moderately to severely active ulcerative colitis	Tablet
8/18/2023	Veopoz	pozelimab-bbfg	To treat CD55-deficient protein-losing enteropathy (PLE) (i.e., CHAPLE disease)	Injection

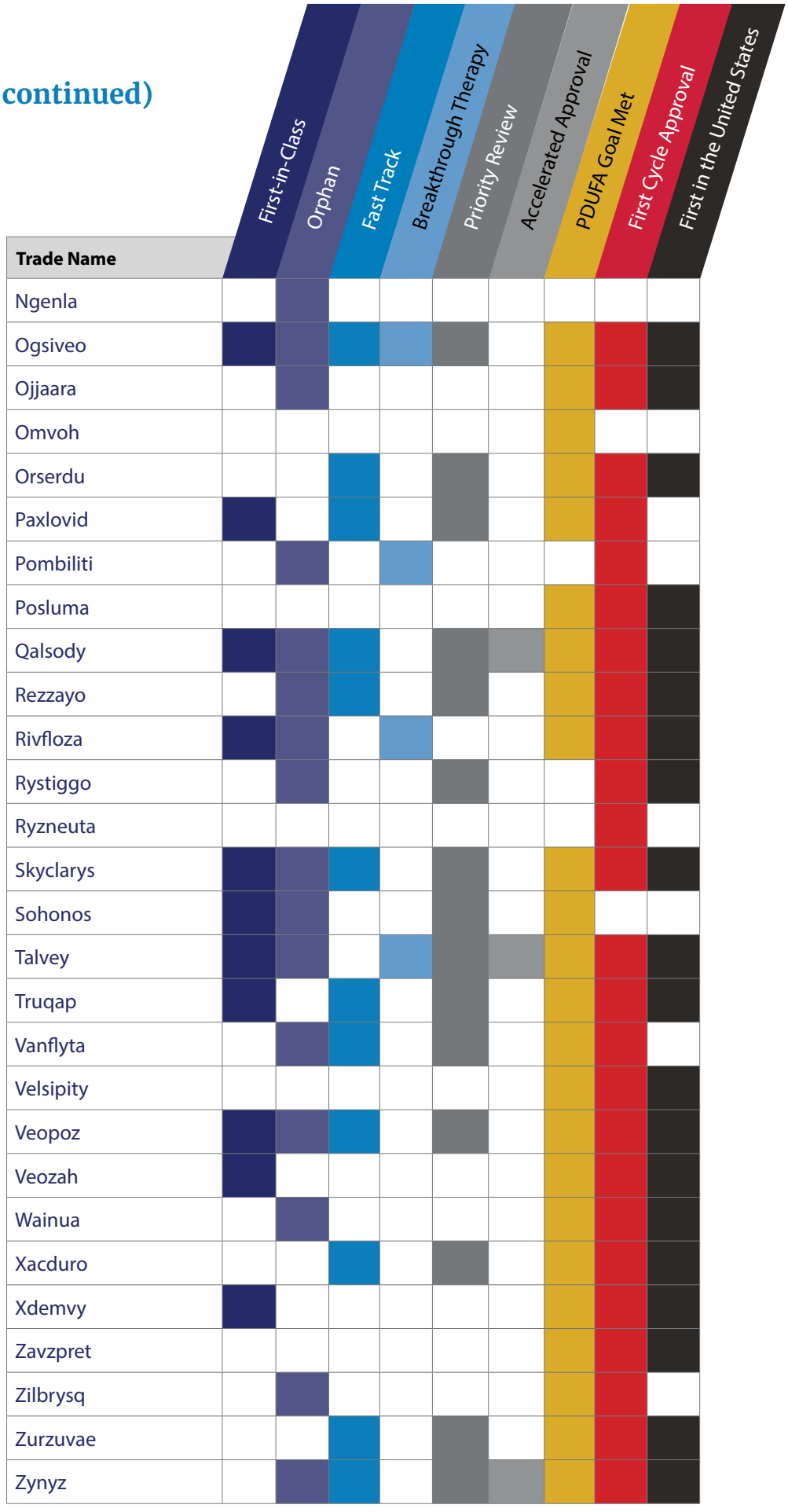
Appendix A (continued)

Approval Date	Proprietary Name	Active Ingredient(s)	Summary of FDA-approved use on approval date (see Drugs@FDA for complete indication)	Dosage Form
5/12/2023	Veozah	fezolinetant	To treat moderate to severe hot flashes caused by menopause	Tablet
12/21/2023	Wainua	eplontersen	To treat hereditary transthyretin-mediated amyloidosis	Injection
5/23/2023	Xacduro	sulbactam, durlobactam (co-packaged)	To treat hospital-acquired bacterial pneumonia and ventilator-associated bacterial pneumonia caused by susceptible isolates of Acinetobacter baumannii-calcoaceticus complex	Injection
7/24/2023	Xdemvy	lotilaner	To treat Demodex blepharitis	Ophthalmic solution
3/9/2023	Zavzpret	zavegepant	To treat migraine	Nasal spray
10/17/2023	Zilbrysq	zilucoplan	To treat generalized myasthenia gravis	Injection
8/4/2023	Zurzuvae	zuranolone	To treat postpartum depression	Capsule
3/22/2023	Zynyz	retifanlimab-dlwr	To treat metastatic or recurrent locally advanced Merkel cell carcinoma	Injection

Appendix B: Novel Drug Designations
(in alphabetical order)

Trade Name
Agamree
Aphexda
Augtyro
Beyfortus
Bimzelx
Brenzavvy
Columvi
Daybue
Defencath
Elfabrio
Elrexfio
Epkinly
Exxua
Fabhalta
Filspari
Filsuvez
Fruzaqla
Inpefa
Izervay
Jaypirca
Jesduvroq
Joenja
Lamzede
Leqembi
Litfulo
Loqtorzi
Miebo

Appendix B (continued)



Trade Name
Ngenla
Ogsiveo
Ojjaara
Omvoh
Orserdu
Paxlovid
Pombiliti
Posluma
Qalsody
Rezzayo
Rivfloza
Rystiggo
Ryzneuta
Skyclarys
Sohonos
Talvey
Truqap
Vanflyta
Velsipity
Veopoz
Veozah
Wainua
Xacduro
Xdemvy
Zavzpret
Zilbrysq
Zurzuvae
Zynyz

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