



**FDA** U.S. FOOD & DRUG  
ADMINISTRATION

ADVANCING HEALTH  
THROUGH INNOVATION:  
**NEW DRUG THERAPY  
APPROVALS 2020**



IMPACT \ INNOVATION \ PREDICTABILITY \ ACCESS  
FDA's Center for Drug Evaluation and Research



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## Director's Message



**Patrizia Cavazzoni, M.D.**

Acting Director, Center for Drug  
Evaluation and Research

Welcome to the FDA's Center for Drug Evaluation and Research's (CDER's) annual report, *Advancing Health Through Innovation: New Drug Therapy Approvals*, representing our tenth consecutive year of reporting our Center's notable new drug approvals, and our role in bringing innovative new drug therapies that are safe and effective to patients in need.

Throughout the year, the COVID-19 pandemic presented unprecedented challenges to our entire staff. Despite these hardships, we have approved many new therapies that will advance health for the American public. This includes the first FDA-approved drug for the treatment of patients with COVID-19.

As with previous years, this year's notable new drug therapies include a variety of novel drugs --- those never before approved or marketed in the United States. Novel drugs often represent important new therapies for advancing patient care. This is now our fourth consecutive year of publishing an expanded version of the report that goes beyond a discussion of novel approvals and includes an overview of additional notable approvals --- for instance, new approvals for uses of already FDA-approved drugs. You will find, as in past years, that many important advances in drug therapy approved in 2020 use an already FDA-approved drug to treat a new disease beyond that for which it was originally approved or to treat a new population of patients, such as children.

Our report emphasizes some of the many innovative ways in which we were able to evaluate safety and efficacy for these new therapies, as well as key regulatory tools we used to enhance our efficiency and expedite the review and approval of applications. This report also summarizes the year's biosimilar approvals. Biosimilars have great potential for both patients and the entire health care system. As patents and exclusivity protections for biologics expire in the United States, we can expect many more biosimilars to be submitted for approval. More biosimilar products on the market means greater competition that may lead to increased access to therapies and lower costs to patients.

The decisions we made on these approvals were generally completed by or before their goal dates as defined by Congressionally approved agreements with industry (referred to as user fee programs). Most were approved in the United States before any other country in the world.

Our work to review and approve new therapies is meaningful to the extent that we can also ensure their safety. All newly approved products were required to meet our rigorous premarket safety standards --- and they will all be part of a strong postmarket safety surveillance system that monitors how they perform after they are more widely used by larger patient populations. We will summarize our safety activities in a different report.

Keep in mind that this report focuses on CDER approvals. FDA's Center for Biologics Evaluation and Research (CBER) also approves many important therapies to advance and protect the health of the American public. For more information, please visit CBER's [web page](#) for 2020 Biological Approvals. We trust this report will continue to promote greater understanding of the many ways CDER works to support innovation and improve treatments for patients.





## 2020: Another Strong Year for Innovation and Advances

In 2020, despite severe challenges from the COVID-19 pandemic, the FDA's Center for Drug Evaluation and Research (CDER) approved many new drug therapies to help a wide range of patients suffering from many different medical conditions to gain new hope for improved quality of life, and in some cases, improved chances of surviving life-threatening illnesses. Many of these new therapies were approved for patients with rare diseases, which, as shown below, span a broad spectrum of disease categories.

### Infectious Disease

In 2020, CDER approved the first medication in the U.S. for the treatment of patients with **COVID-19** (hospitalized adults and adolescents). For patients living with **HIV-1**, CDER approved the first new drug in a new class of drugs to treat adults with HIV-1 who have tried multiple HIV-1 medications and whose infection cannot be successfully treated with other therapies because of resistance, intolerance, or safety considerations. We also approved a new tablet for oral suspension formulation of an already

FDA-approved drug, given in combination with other HIV-1 drugs, that will help ensure earlier treatment for many pediatric patients --- an important part of HIV therapy. We also approved the first FDA-approved **flu prevention** medication that is taken as a single dose in pill form when a person is exposed to a household contact. This past year also saw CDER expand approval of a **chronic hepatitis C** drug to include treatment for a larger population of patients than those treated under the original approval. CDER also approved two new treatments for patients with **hospital-acquired bacterial pneumonia and ventilator-associated bacterial pneumonia**.

Approvals of note for patients with rare infections in the U.S. include, 1) a new drug for the treatment of patients with **severe malaria**, 2) two new drugs approved for the treatment of patients infected with **Ebola virus**, and, 3) a new therapy for **Chagas disease**, a rare parasitic disease that can cause congestive heart failure: the first FDA-approved therapy to treat *pediatric* patients younger than two years of age.

## Neurology

For patients with **Parkinson's disease**, we approved a new therapy to use during "off" episodes when their regular medication is not working. Additionally, CDER approved a new therapy for certain patients suffering from **migraine** headaches. CDER also approved two notable new therapies to help patients with forms of **multiple sclerosis**. Additionally, CDER approved a new therapy to start and maintain **sedation** in adults undergoing short (less than 30 minute) medical procedures.

Approvals of note for patients with rare neurological diseases include, 1) the first orally-administered drug to treat patients with **spinal muscular atrophy**, a disease that diminishes physical strength by affecting nerve cells in the spinal cord, taking away the ability to walk, eat, or breathe, 2) a new drug for certain patients with **Duchenne muscular dystrophy**, a genetic disorder, usually occurring in young boys, in which alterations of a protein called dystrophin leads to progressive muscle degeneration and weakness, 3) a new therapy to treat patients with seizures associated with **tuberous sclerosis complex**, a rare, multi-system genetic disease that causes non-cancerous tumors to grow in the brain and on other vital organs such as the kidneys, heart, eyes, lungs, and skin, 4) a new therapy to help prevent seizures in certain pediatric patients with the seizure disorder, **Dravet syndrome**, and, 5) two new drugs to treat patients with **neuromyelitis optica spectrum disorder**, a rare disease of the central nervous system that primarily attacks the optic nerves and spinal cord leading to blindness and paralysis.

## Autoimmune, Inflammatory, and Lung Diseases

In 2020, CDER approved two new therapies for the treatment of **polyarticular juvenile idiopathic arthritis**, a type of juvenile arthritis (arthritis in children) that causes inflammation in five or more joints within the first six months of the disease. CDER also approved a new therapy for pediatric patients with **ulcerative colitis**, a chronic disease of the large intestine, in which the lining of the colon becomes inflamed and develops open sores, or ulcers. Additionally, CDER approved a new therapy to treat patients with **chronic fibrosing interstitial lung diseases (ILD) with a progressive phenotype** (trait) --- the first FDA-approved treatment for this group of fibrosing lung diseases that worsen over time.

Notable approvals for patients with rare diseases include, 1) the first FDA-approved therapy to treat the rare disease **lupus nephritis**, a serious and sometimes fatal complication of lupus caused by inflammation and dysfunction of the kidneys, 2) a new approval for the treatment of active **Still's disease**, including **adult-onset Still's disease (AOSD)**, a rare type of inflammatory arthritis that affects the entire body. This is the first FDA-approved therapy for AOSD, 3) a new drug to treat patients with **hereditary angioedema** a rare disorder characterized by recurrent episodes of severe swelling (angioedema), most commonly in the limbs, face, intestinal tract, and airway, and, 4) two new treatments for patients with a condition called, **deficiency of interleukin-1 receptor antagonist**, a very rare genetic inflammatory condition resembling an infection throughout the body or a bone infection that happens in newborns during the first days of life.

## Heart, Blood, Kidney, and Endocrine Diseases

CDER approved a new therapy to help raise excessively low levels of phosphate in the blood (induced by a specific type of tumor) in certain patients with **osteomalacia** (softening of the bones). We also approved a new therapy to reduce the risk of cardiovascular death and hospitalization for adult patients with a type of **heart failure**. Additionally, CDER approved two different new types of already FDA-approved insulins to improve glycemic control in certain adults and children with **diabetes**, which may help expand patient access and market competition for insulin products in the United States. CDER also approved a new drug for adults with **growth hormone deficiency**, the first of its kind that can be injected weekly, rather than daily.

Approvals of note for patients with rare diseases include, 1) a new therapy to treat patients with **hypereosinophilic syndrome**, a group of rare blood disorders that occur when the blood contains too many eosinophils (white blood cells that normally play an important role in the immune system), which eventually damage organs, 2) a new drug for the treatment of obesity and the control of hunger associated with **pro-opiomelanocortin deficiency**, a rare disorder that causes severe obesity that begins at an early age, 3) a new drug for the treatment of certain patients with **Cushing's disease**, a condition in which the body makes too much cortisol, a hormone related to stress response, and, 4) a new drug for the treatment of adult and pediatric patients with **primary hyperoxaluria type 1**, the most common and severe type among various forms of this condition. It is a rare disorder that mainly affects the kidneys, which results from buildup of a substance called oxalate, which, absent this disease, is normally filtered by the kidneys and excreted in the urine.

## Cancers

CDER approved many new therapies in 2020 for a wide array of cancers, particularly **lung cancers**, for which we approved two notable new treatments for patients with small cell lung cancer and nine to treat various forms of non-small cell lung cancer, two of which were also approved to treat patients with certain types of **thyroid cancer**. We also approved four new notable therapies to treat patients with various forms of **breast cancer**. For patients with other forms of cancer CDER approved, 1) two new treatments for certain patients with **urothelial cancer**, which is a cancer that begins in cells called urothelial cells in the urinary system, 2) a new first-line treatment for patients with a type of **colorectal cancer** that has spread to other parts of the body or for which surgery is not an option, 3) two new therapies for the treatment of certain patients with forms of **prostate cancer**, and, 4) a new approval for the treatment of certain patients with **bladder cancer** who do not respond favorably to first-line treatment.

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CDER's drug therapy approvals of 2020 will help advance patient care in many new areas.

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CDER also approved a variety of new therapies to treat patients with **rare cancers**. These include 1) three notable new treatments for certain patients with **multiple myeloma**, a rare cancer that forms in white blood cells called plasma cells, which fight infections, 2) a new drug for the treatment of patients with high-risk refractory or relapsed **neuroblastoma**, a very rare type of cancerous tumor, often found in the adrenal glands, that almost always affects children, 3) a new drug in tablet form approved for treating patients with the rare blood cancers, **myelodysplastic syndromes** and **chronic myelomonocytic leukemia**, many of whom will benefit from taking self-administered tablets instead of having to go the health care facility for injections of other treatments, 4) two new drugs to treat certain patients with **gastrointestinal stromal tumor**, a rare type of cancer that starts in specialized nerve cells located in the walls of the digestive system, 5) a new drug for the treatment of certain patients who have **epithelioid sarcoma**, a rare, slow-growing type of cancer that often begins in the soft tissue under the skin of a finger, hand, forearm, lower leg, or foot, 6) a new drug for treating certain patients with a specific type of **metastatic cholangiocarcinoma** (bile duct cancer), 7) a new drug for the treatment of certain pediatric patients, two years of age and older, with **neurofibromatosis type 1**, a genetic disorder of the nervous system causing tumors to grow on nerves --- the first drug approved by the FDA to treat this debilitating, progressive, and often disfiguring rare disease that typically begins early in life, and, 8) a new use for two already FDA-approved drugs to be used in combination to treat patients with **mesothelioma**, a type of cancer caused by inhaling asbestos fibers.

### Other Advances and Other Rare Diseases

In other advances of 2020, CDER approved a new combination of already FDA-approved drugs for the management of **heavy menstrual bleeding associated with fibroids** (a kind of tumor typically non-cancerous) in premenopausal women. Additionally, 2020 saw the first FDA-approved therapy for certain pediatric patients with **neurogenic detrusor over-activity**, a bladder dysfunction frequently observed in patients with multiple sclerosis and spinal cord injury.

Other notable approvals for patients with rare diseases include, 1) a new drug to treat certain patients with **Hutchinson-Gilford Progeria Syndrome** and **progeroid laminopathies**, rare conditions caused by certain genetic mutations that lead to premature aging, and, 2) a new drug for the treatment of **thyroid eye disease**, a rare condition where the muscles and fatty tissues behind the eye become inflamed, causing the eyes to be pushed forward and bulge outwards.

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CDER's drug therapy approvals of 2020 will help many patients with rare diseases.

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To help detect and diagnose disease, CDER approved three new positron emission tomography (PET) imaging agents, 1) a diagnostic agent to help evaluate patients for **Alzheimer's disease**, 2) a new diagnostic agent for finding tumors called **somatostatin receptor positive neuroendocrine tumors**, which are cancerous and can arise anywhere in the body, and, 3) a PET imaging agent for detection and localization of **prostate cancer**, a significant advance that offers a more accurate imaging option for patients with primary or recurrent prostatic cancer.

## CDER's Drug Therapy Approvals of 2020

In 2020, CDER approved a wide variety of drug therapies to improve the health of the American public, including:

- **Novel drugs**, which are often among the more innovative products in the marketplace, and help advance clinical care by providing therapies never before marketed in the U.S.;
- **New and expanded uses** for already FDA-approved drugs;
- **Biosimilars**, which are highly similar to already FDA-approved therapeutic biological products. These approvals add consumer choice and spur marketplace competition;
- **New formulations** or new manufacturers of already FDA-approved products that can provide advantages over original products, such as being able to take the drug on an empty stomach instead of with food, and;
- **New dosage forms** that can add value to already FDA-approved drugs, such as chewable tablets for patients unable to swallow pills.

This report summarizes these approvals and highlights examples, emphasizing those approvals that offer new and innovative treatments to patients in need.

**As with all FDA-approved products, the new drug therapies discussed in this report have risks. For more information about these drugs and for complete risk information, see the drugs' approval letters and FDA-approved labeling at [Drugs@FDA](https://www.fda.gov/drugs@fda).**

## Novel Drugs

Novel drugs are often innovative products that serve previously unmet medical needs or otherwise significantly help to advance patient treatments. The active ingredient or ingredients in a novel drug have never before been approved in the United States. This report lists all of CDER's novel drug approvals of 2020 and also discusses those that CDER considers notable advances. In 2020, CDER approved 53 novel drugs, either as new molecular entities (NMEs) under New Drug Applications (NDAs), or as new therapeutic biologics under Biologics License Applications (BLAs).

### CDER's Novel Drug Approvals of 2020

CDER's novel drug approvals for 2020 are listed alphabetically below by trade name.\* See [Appendix A](#) in this report or visit online, [CDER's Novel Drug Approvals for 2020](#), for the non-proprietary names, dosage forms, and what each drug is used for.

artesanate**	Evryydi	Margenza	Qinlock	Uplizna
Ayvakit	Ga 68 PSMA-11**	Monjuvi	Retevmo	Veklury
Barhemsys	Gavreto	Nexletol	Rukobia	Viltepsa
Blenrep	Gemtesa	Nurtec ODT	Sarclisa	Vyepti
Byfavo	Imcivree	Olinvyk	Sogroya	Winlevi
Cerianna	Inmazed	Ongentys	Tabrecta	Xeglyze
Danyelza	Inqovi	Orgovyx	Tauvid	Zeposia
Detectnet	Isturisa	Orladeyo	Tazverik	Zepzelca
Dojolvi	Klisyri	Oxlumo	Tepezza	Zokinvy
Ebanga	Koselugo	Pemazyre	Trodelyv	
Enspryng	Lampit	Pizensy	Tukysa	

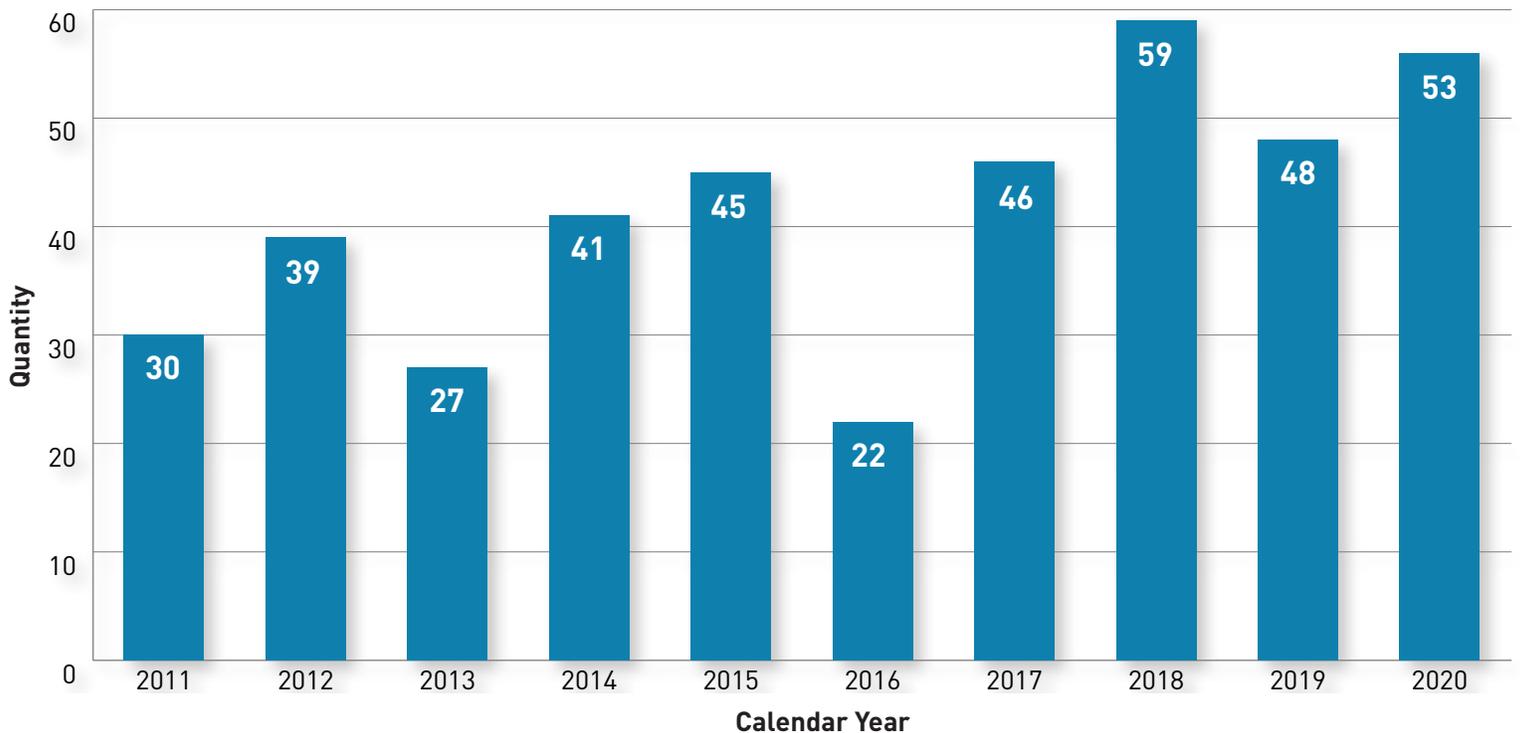
\* This information is accurate as of December 31, 2020. In rare instances, it may be necessary for FDA to change a drug's NME designation or the status of its application as a novel BLA. For instance, new information may become available which could lead to a reconsideration of the original designation or status. If changes must be made to a drug's designation or the status of an application as a novel BLA, the agency intends to communicate the nature of, and the reason for, any revisions as appropriate.

\*\* Product approved with no trade name



## CDER's Annual Novel Drug Approvals: 2011 - 2020

In 2020, CDER approved 53 novel drugs. The 10-year graph below shows that from 2011 through 2019, CDER has averaged about 40 novel drug approvals per year



## Impact of Novel Drug Approvals

Many of the novel drugs CDER approved in 2020 are notable for their potential positive impact and unique contributions to quality medical care and patient treatment.

### First-in-Class

CDER identified 21 of the 53 novel drugs approved in 2020 (40%) as first-in-class, which is one indicator of the drug's potential for strong positive impact on the health of the American people. These drugs often have mechanisms of action different from those of existing therapies.

#### Novel drugs approved in 2020 that FDA identified as first-in-class

**were:** Blenrep, Enspryng, Ga 68 PSMA-11, Imcivree, Inmazeb, Isturisa, Klisyri, Koselugo, Monjuvi, Nexletol, Oxlumo, Rukobia, Tauvid, Tazverik, Tepezza, Trodelvy, Uplizna, Veklury, Winlevi, Xeglyze, and Zokinvy.

#### Examples of notable First-in-Class novel approvals for 2020 include:

**Rukobia (fostemsavir)**, tablets, a new type of antiretroviral medication for adults living with **HIV-1** who have tried multiple HIV-1 medications and whose HIV-1 infection cannot be successfully treated with other therapies because of resistance, intolerance, or safety considerations. This approval marks a new class of antiretroviral medications that may benefit patients who have run out of HIV-1 treatment options.

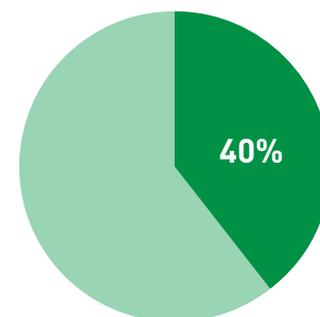
**Koselugo (selumetinib)**, capsules, for the treatment of certain pediatric patients, two years of age and older, with **neurofibromatosis type 1**, a genetic disorder of the nervous system causing tumors to grow on nerves. This is the first drug approved by the FDA to treat this debilitating, progressive, and often disfiguring disease that typically begins early in life.

### Drugs for Rare Diseases

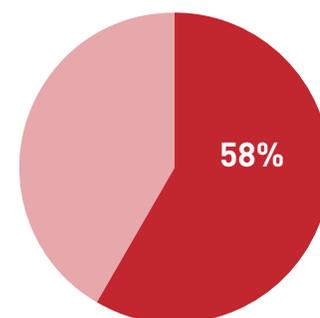
In 2020, 31 of CDER's 53 novel drug approvals, (58%) were approved to treat rare or "orphan" diseases that affect 200,000 or fewer Americans. Patients with rare diseases often have few or no drugs available to treat their conditions.

#### Novel drugs approved in 2020 with the orphan drug designation were:

artesanate, Ayvakit, Blenrep, Danyelza, Detectnet, Dojolvi, Ebanga, Enspryng, Evrysdi, Gavreto, Imcivree, Inmazeb, Inqovi, Isturisa, Koselugo, Lampit, Monjuvi, Orladeyo, Oxlumo, Pemazyre, Qinlock, Retevmo, Sarcilisa, Tabracta, Tazverik, Tepezza, Tukysa, Uplizna, Viltepso, Zepzelca, and Zokinvy.



CDER identified 21 of the 53 (40%) novel drugs approved in 2020 as first-in-class



31 of CDER's 53 novel drug approvals, (58%) were approved to treat rare or "orphan" diseases

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Patients with rare diseases often have few or no drugs available to treat their conditions.

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In 2020, CDER approved a new drug for the treatment of patients with severe malaria.

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**Notable examples of novel approvals of 2020 that advance the care of patients with rare diseases include:**

- **Evrysdi (risdiplam)**, powder for oral solution, to treat patients two months of age and older with **spinal muscular atrophy (SMA)**, a rare and often fatal genetic disease affecting muscle strength and movement. This is the third FDA-approved therapy for SMA, but the only one approved that is taken orally.
- **Lampit (nifurtimox)**, tablets, an accelerated approval (see p. 22), the first therapy approved by FDA to treat pediatric patients with **Chagas disease** younger than two years of age (from birth to younger than 18 years of age and weighing at least 2.5 kilograms). Chagas disease is a rare parasitic disease which, if left untreated, can cause congestive heart failure.
- **Orladeyo (berotralstat)**, capsules, to treat patients with **hereditary angioedema** a rare disorder characterized by recurrent episodes of severe swelling (angioedema), most commonly in the limbs, face, intestinal tract, and airway.

**Other Notable Novel Drug Approvals: Advances in Patient Care Across a Broad Range of Diseases**

In addition to the noteworthy first-in-class and orphan-designated drugs mentioned above, the 2020 novel drug field also includes these notable examples --- approved for the first time in the U.S., and likely to significantly improve the care of patients with the conditions noted below:

- **artesunate (approved with no trade name)**, injection, to treat **severe malaria** in adult and pediatric patients. Prior to this approval, there had been no FDA-approved drug for treatment of severe malaria in the U.S. since the marketing of quinidine was discontinued by the manufacturer in March 2019. Approximately 2,000 cases of malaria are diagnosed in the U.S. each year, with 300 of those infected having severe disease. Most people diagnosed with malaria in the U.S. acquire it during travel to countries with malaria.
- **Ayvakit (avapritinib)** [see also Qinlock, below], tablets, for the treatment of adults with unresectable (unable to be removed with surgery) or metastatic (when cancer cells spread to other parts of the body) **gastrointestinal stromal tumor (GIST)** – a type of tumor that occurs in the gastrointestinal tract, most commonly in the stomach or small intestine – harboring a platelet-derived growth factor receptor alpha (PDGFRA) exon 18 mutation. This approval includes GIST that harbors a PDGFRA D842V mutation, which is the most common exon 18 mutation. GIST harboring a PDGFRA exon 18 mutation does not respond to standard therapies for GIST. This approval provides patients with the first drug specifically approved for GIST harboring this mutation.

- **Blenrep (belantamab mafodotin)** [See also Sarclisa, below], injection, an accelerated approval (see p. 22), as a monotherapy treatment (not combined with other treatments) for certain adult patients with relapsed and refractory **multiple myeloma**, a cancer of plasma cells. Normal plasma cells are found in the bone marrow and are an important part of the immune system.
- **Byfavo (remimazolam)**, injection, to start and maintain **sedation** in adults undergoing short (less than 30 minute) medical procedures.
- **Danylza (naxitamab-gqgk)**, injection, an accelerated approval (see p. 22), for the treatment of patients with high-risk refractory or relapsed **neuroblastoma**, a very rare type of cancerous tumor, often found in the adrenal glands, that almost always affects children.
- **Detectnet (copper dotatate Cu-64)**, a positron emission tomography (PET) imaging agent using a novel radionuclide (Cu64) for finding and assessing tumors called **somatostatin receptor positive neuroendocrine tumors**, which are cancerous and can arise anywhere in the body.
- **Ebanga (ansuvimab-zykl)** [see also, Inmazed, below], injection, for the treatment of patients with Zaire **Ebola virus** infection.
- **Enspryng (satralizumab-mwge)** [see also, Uplizna, below], injection, for the treatment of certain patients with **neuromyelitis optica spectrum disorder**, a rare, autoimmune disease of the central nervous system that primarily attacks the optic nerves and spinal cord, leading to blindness and paralysis. Following the approval of Soliris (eculizumab) in 2019 and Uplizna (inebilizumab-cdon) earlier in 2020, this was the third approval in a very short time for a disease that previously had no approved treatments.
- **Ga 68 PSMA-11 (approved with no trade name)**, a positron emission tomography (PET) imaging agent for detection and localization of **prostate cancer**, a significant advance that offers a more accurate imaging option for patients with prostatic cancer, one of the leading causes of cancer mortality in the U.S. In addition to its use in patients with biochemically recurrent prostate cancer, this is the first PET imaging agent to be indicated in patients with primary prostatic cancer.
- **Gavreto (pralsetinib)** [see also Retevmo, below], capsules, an accelerated approval (see p. 22), to treat patients with a type of **non-small cell lung cancer** caused by a chromosomal abnormality in a gene called the RET (rearranged during transfection) gene. This drug was approved later in the year to treat certain patients with **thyroid cancer**.
- **Imcivree (setmelanotide)**, injection, for the treatment of obesity and the control of hunger associated with **pro-opiomelanocortin deficiency**, a rare disorder that causes severe obesity that begins at an early age.




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In 2020, CDER approved two new drugs approved for the treatment of certain patients infected with Ebola virus.

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In 2020, CDER approved two new therapies for neuromyelitis optica spectrum disorder.

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## CDER approved a new drug with a new way of treating Cushing's disease.

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## CDER approved a new injectable opioid pain reliever that will be safely administered in hospitals or other controlled clinical settings.

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- **Inmazeb (atoltivimab, maftivimab, odesivimab-ebgn)**, [See also, Ebanga, above], injection, for the treatment of patients with **Zaire Ebola virus** infection.
- **Inqovi (decitabine and cedazuridine)**, tablets, for treatment of adult patients with the blood cancers, **myelodysplastic syndromes (MDS)** or **chronic myelomonocytic leukemia**. Previously approved treatments for MDS are only administered intravenously in a health care facility. The oral tablet form represents an important advance for patients with MDS, who can now self-administer the drug without the need to travel to a health care facility for their medicine.
- **Isturisa (osilodrostat)**, tablets, for adults with **Cushing's disease** who either cannot undergo pituitary gland surgery or have undergone the surgery but still have the disease. This is the first FDA-approved drug to directly address this cortisol overproduction by blocking the enzyme known as 11-beta-hydroxylase and preventing cortisol production. Cushing's disease is caused by a pituitary tumor that releases too much of a hormone called adrenocorticotropin, which stimulates the adrenal gland to produce an excessive amount of cortisol.
- **Margenza (margetuximab-cmkb)**, injection, to treat certain patients who have **HER2-positive breast cancer**.
- **Nurtec ODT (rimegepant)**, tablets, for treatment of acute **migraine** with or without aura in adults. Migraine is a type of headache that, in addition to pain, can be associated with nausea, vomiting, and sensitivity to light or sound. Some patients may experience an aura, which may consist of a temporary visual disturbance, or other symptoms shortly before the onset of the headache.
- **Olinvyk (oliceridine)**, injection, an opioid medication indicated in adult patients for the management of **moderate to severe acute pain** severe enough to require an intravenous opioid pain reliever and for whom alternative treatments are inadequate. This product will only be used for short-term intravenous use in hospitals or other controlled clinical settings (for inpatient and outpatient procedures) as a substitute for current IV opioids. It will not be available for at-home use.
- **Ongentys (opicapone)**, capsules, used to treat patients with **Parkinson's disease** who are having "off" episodes while taking drugs (levodopa and carbidopa) to treat this condition. An "off" episode is a time when a patient's medications are not working well, leading to an increase in Parkinson's symptoms, such as tremor and difficulty walking.
- **Orgovyx (relugolix)**, [see also Lynparza under New Uses], tablets, for the treatment of certain patients with **prostate cancer**.

- **Oxlumo (lumasiran)**, injection, for the treatment of adult and pediatric patients with primary **hyperoxaluria type 1**, the most common and severe type among various forms of this condition. It is a rare disorder that mainly affects the kidneys, and results from buildup of a substance called oxalate, which, absent this disease, is normally filtered by the kidneys and excreted in the urine.
- **Pemazyre (pemigatinib)**, tablets, an accelerated approval (see p. 22), for the treatment of adults with certain types of previously treated, **advanced cholangiocarcinoma**, a rare form of cancer that forms in bile ducts.
- **Qinlock (ripretinib)** [see also Ayvakit, above], tablets, the first new drug specifically approved as a fourth-line treatment for advanced **gastrointestinal stromal tumor (GIST)**, a type of tumor that originates in the gastrointestinal tract. This drug is approved for adult patients who have received prior treatment with three or more kinase inhibitor therapies, including imatinib. Despite the progress that has been made over the past 20 years in developing treatments for GIST, including four FDA-approved therapies – imatinib in 2002, sunitinib in 2006, regorafenib in 2013, and avapritinib earlier this year – some patients do not respond to treatment and their tumors continue to progress. This approval provides a new treatment option for patients who have exhausted all FDA-approved therapies for GIST.
- **Retevmo (selpercatinib)** [see also Gavreto, above], capsules, an accelerated approval (see p. 22), to treat three types of cancers – **non-small cell lung cancer, medullary thyroid cancer, and other types of thyroid cancers** – in patients whose tumors have an alteration (mutation or fusion) in a specific gene (the RET or rearranged during transfection gene).
- **Sarclisa (isatuximab-irfc)** [see also Blenrep, above], injection, approved in combination with pomalidomide and dexamethasone for the treatment of certain adult patients with **multiple myeloma** who have received at least two prior therapies. Sarclisa is a CD38-directed cytolytic antibody that works by helping certain cells in the immune system attack multiple myeloma cancer cells. While there is no cure for multiple myeloma, Sarclisa is now another CD38-directed treatment option added to the list of FDA-approved treatments of patients with multiple myeloma who have progressive disease after previous therapies. Multiple myeloma is a form of blood cancer that occurs in infection-fighting plasma cells (a type of white blood cell) found in the bone marrow.

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CDER approved new therapies for rare cancers, including multiple myeloma, neuroblastoma, chronic myelomonocytic leukemia, myelodysplastic syndromes, gastrointestinal stromal tumor, epithelioid sarcoma, and metastatic cholangiocarcinoma.

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CDER approved a new diagnostic agent to help clinicians evaluate patients for Alzheimer's disease.

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In 2020, CDER approved a new drug for thyroid eye disease.

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- **Sogroya (somapacitan-beco)**, injection, a recombinant human growth hormone for the treatment of adults with **growth hormone deficiency**. It is the first of its kind approved by CDER that can be injected weekly instead of daily.
- **Tabrecta (capmatinib)**, tablets, an accelerated approval (see p. 22), for the treatment of adult patients with **non-small cell lung cancer** (NSCLC) that has spread to other parts of the body. This is the first FDA-approved therapy specifically for the treatment of patients with non-small cell lung cancer whose tumors have mutations that lead to mesenchymal-epithelial transition (MET) exon 14 skipping. NSCLC is a disease in which malignant cancer cells form in the tissues of the lung. It is the most common type of lung cancer with up to 90 percent of all lung carcinomas falling into the non-small cell category.
- **Tauvid (flortaucipir F18)** injection, the first drug used to help image a distinctive characteristic of **Alzheimer's disease** in the brain called tau pathology. Tauvid is a radioactive diagnostic approved for positron emission tomography (PET) imaging of the brain to estimate the density and distribution of aggregated tau neurofibrillary tangles, a primary marker of Alzheimer's disease.
- **Tazverik (tazemetostat)**, tablets, an accelerated approval (see p. 22), for the treatment of patients aged 16 years and older with metastatic (when cancer cells spread to other parts of the body) or locally advanced (when cancer has grown outside the organ it started in, but has not yet spread to distant parts of the body) **epithelioid sarcoma** not eligible for complete resection (surgically removing all of a tissue, structure, or organ). Epithelioid sarcoma is a rare sub-type of soft tissue sarcoma that often occurs in young adults. It accounts for less than one percent of all soft tissue sarcomas. Until this approval, there were no treatment options specifically for this disease. Tazverik was also approved in 2020 to treat certain adult patients with **follicular lymphoma**, a type of non-Hodgkin lymphoma, which is a group of related cancers that affect the lymphatic system.
- **Tepezza (teprotumumab-trbw)**, injection, an accelerated approval (see p. 22), for the treatment of adults with **thyroid eye disease**, a rare condition where the muscles and fatty tissues behind the eye become inflamed, causing the eyes to be pushed forward and bulge outwards (proptosis). This approval represents the first drug approved for the treatment of thyroid eye disease. This treatment has the potential to alter the course of the disease, potentially sparing patients from needing multiple invasive surgeries by providing an alternative, non-surgical treatment option.

- **Trodelyv (sacituzumab govitecan-hziy)**, injection, an accelerated approval (see p. 22), for the treatment of certain adult patients with triple-negative breast cancer that has spread to other parts of the body. Metastatic **triple-negative breast cancer** is an aggressive form of breast cancer with limited treatment options. Chemotherapy has been the mainstay of treatment. This approval represents a new targeted therapy for patients living with this aggressive malignancy. Approximately two of every 10 breast cancer diagnoses worldwide are triple-negative. Triple-negative breast cancer tests negative for estrogen receptors, progesterone receptors, and human epidermal growth factor receptor 2 (HER2) protein. Therefore, triple-negative breast cancer does not respond to hormonal therapy medicines or medicines that target HER2.
- **Tukysa (tucatinib)**, tablets, for the treatment of certain adult patients with advanced unresectable or metastatic **HER2-positive breast cancer**. HER2 is a growth-promoting protein on the outside of all breast cells. Breast cancer cells with higher than normal levels of HER2 are called HER2-positive. These cancers tend to grow and spread faster than other breast cancers. Tukysa was approved under the FDA's [Project Orbis](#), which provides a framework for concurrent submission and review of oncology products among international partners.
- **Uplizna (inebilizumab-cdon)** [see also Enspryng, above], injection, for the treatment of patients with **neuromyelitis optica spectrum disorder**, a rare, autoimmune disease of the central nervous system that primarily attacks the optic nerves and spinal cord, leading to blindness and paralysis. Following the approval of Soliris (eculizumab) in 2019, this was only the second FDA approval in a very short time for a disease that previously had no approved treatments. Enspryng (satralizumab-mwge) (see above) followed later in the year as a third approval to treat this condition.
- **Veklury (remdesivir)**, injection, the first medication in the U.S. for the treatment of patients with **COVID-19** (hospitalized adults and adolescents).
- **Viltepso (viltolarsen)**, injection, an accelerated approval (see p. 22), for the treatment of **Duchenne muscular dystrophy** (DMD) in patients who have a confirmed mutation of the DMD gene that is amenable to exon 53 skipping. DMD is a severe type of muscular dystrophy that primarily affects boys. Muscle weakness usually begins around the age of four and worsens quickly.

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CDER approved a new drug for certain patients with Duchenne muscular dystrophy.

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## CDER approved eleven notable new therapies for various forms of lung cancer in 2020.

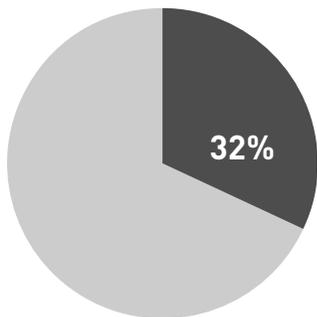
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## CDER approved the first medication in the U.S. for the treatment of certain patients with COVID-19

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CDER designated 17 of the 53 novel drugs (32%) of 2020 as Fast Track

- **Zeposia (ozanimod)**, capsules, for the treatment of adults with relapsing forms of **multiple sclerosis** (MS). Multiple sclerosis is a disease of the brain and spinal cord in which the immune system attacks the protective covering of nerve fibers and causes communication problems between the brain and the rest of the body. In relapsing forms of MS, patients have episodes of worsening function (relapses) followed by recovery periods. Patients can also experience an increase in the underlying disability, particularly as the disease progresses.
- **Zepzelca (lurbinectedin)**, injection, an accelerated approval (see p. 22), to treat certain patients with **small-cell lung cancer**, a fast-growing type of lung cancer that spreads much more quickly than non-small cell lung cancer, which is more common.
- **Zokinvy (lonafarnib)**, capsules, to treat certain patients with **Hutchinson-Gilford Progeria Syndrome** and **progeroid laminopathies**, rare conditions caused by certain genetic mutations that lead to premature aging.

## Innovation: Frequent Use of Expedited Development and Review Pathways

CDER used several regulatory pathways to enhance efficiency and expedite the development and approval of novel drugs in 2020. These pathways use a range of approaches, including more interactions between CDER staff and drug developers, greater program design flexibility, and shortened timelines for review of applications.

### Fast Track

Fast Track-designated drugs have the potential to address unmet medical needs. CDER designated 17 of the 53 novel drugs (32%) in 2020 as Fast Track. Fast Track speeds new drug development and review by increasing the level of communication between FDA and drug developers, and by enabling CDER to review portions of a drug application ahead of the submission of the complete application.

**Drugs designated with Fast Track status were:** artesunate, Ayvakit, Detectnet, Dojolvi, Enspryng, Evrysdi, Margenza, Monjuvi, Olinvyk, Orladeyo, Qinlock, Rukobia, Tepezza, Trodelvy, Tukysa, Veklury, and Viltepso.

## Breakthrough Therapy

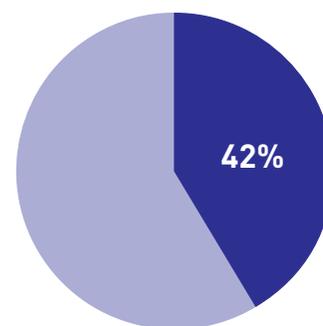
Breakthrough therapies are drugs for serious or life-threatening diseases for which there is unmet medical need and for which there is preliminary clinical evidence demonstrating that the drug may result in substantial improvement on a clinically significant endpoint (usually an endpoint that reflects how the patient feels, functions or survives) over other available therapies. CDER designated 22 of the 53 novel drugs (42%) in 2020 as breakthrough therapies. A breakthrough therapy designation includes all the Fast Track program features, as well as more intensive FDA guidance on an efficient drug development program. Breakthrough therapy designation is designed to help shorten the development time of potentially important new therapies.

**Drugs designated with Breakthrough therapy status were:** artesunate, Ayvakit, Blenrep, Danyelza, Ebanga, Enspryng, Gavreto, Imcivree, Inmazeb, Koselugo, Monjuvi, Oxlumo, Pemazyre, Qinlock, Retevmo, Rukobia, Tavegra, Tepezza, Trodelvy, Tukysa, Uplizna, and Zokinvy.

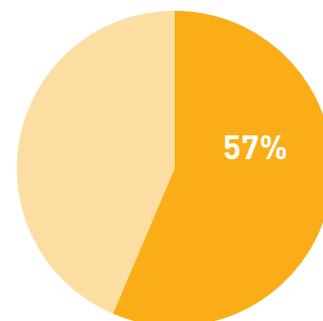
## Priority Review

A drug receives a Priority Review if CDER determines that the drug could potentially provide a significant advance in medical care. The drug is reviewed in an expedited time line: within eight months instead of the standard 12 months. Thirty of the 53 novel drugs approved in 2020 (57%) were designated Priority Review. *Note, in some instances, priority review is assigned as a result of the sponsor redeeming a voucher for priority review under CDER's Priority Review Voucher program, which may mean the drug does not potentially provide a significant advance. Such drugs are not included in the list below.*

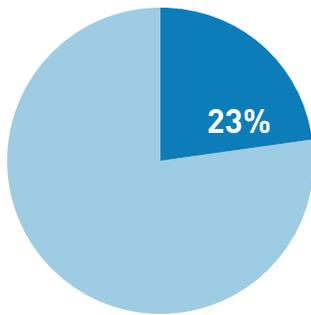
**Drugs designated Priority Review were:** artesunate, Ayvakit, Blenrep, Danyelza, Detectnet, Ebanga, Evrysdi, Gavreto, Imcivree, Inmazeb, Inqovi, Koselugo, Lampit, Monjuvi, Orgovyx, Oxlumo, Pemazyre, Qinlock, Retevmo, Rukobia, Tavegra, Tauvid, Tazverik, Tepezza, Trodelvy, Tukysa, Veklury, Viltepso, Zepzelca, and Zokinvy.



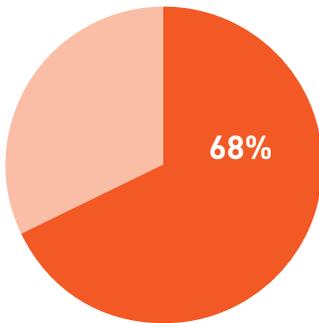
**CDER designated 22 of the 53 novel drugs (42%) of 2020 as breakthrough therapies**



**30 of the 53 novel drugs approved in 2020 (57%) were designated Priority Review**



**CDER approved 12 of the 53 novel drugs approved in 2020 under the Accelerated Approval Program**



**36 of the 53 novel drug approvals of 2020 (68%) were designated in one or more expedited categories of Fast Track, Breakthrough, Priority Review, and/or Accelerated Approval**

## Accelerated Approval

The Accelerated Approval program allows FDA more flexibility in what endpoints can be used to approve a drug that offers a benefit over current treatments for a serious or life-threatening illness. These accelerated approval endpoints may include ones that show benefits over a shorter duration of treatment (where longer-term demonstration of benefit is needed for full approval) or are considered as “reasonably likely” to predict an important clinical benefit. Subsequent confirmatory trials must be conducted to support full approval. CDER approved 12 of the 53 novel drugs (23%) in 2020 under the Accelerated Approval program. The application of accelerated approval brings drugs that can provide important advances to patients sooner than with traditional approvals.

**The Novel drug approved in 2020 that received the Accelerated Approval designation were:** Blenrep, Danyelza, Gavreto, Lampit, Monjuvi, Pemazyre, Retevmo, Tabrecta, Tazverik, Trodelvy, Viltepso, and Zepzelca.

## Overall Use of Expedited Development and Review Methods

Thirty-six of the 53 novel drug approvals of 2020 (68%) were designated in one or more expedited categories of Fast Track, Breakthrough, Priority Review, and/or Accelerated Approval.

**Novel drugs approved in 2020 using at least one expedited approval method were:** artesunate, Ayvakit, Blenrep, Danyelza, Detectnet, Dojolvi, Ebanga, Enspryng, Evrysdi, Gavreto, Imcivree, Inmazeb, Inqovi, Koselugo, Lampit, Margenza, Monjuvi, Olinvyk, Orgovyx, Orladeyo, Oxlumo, Pemazyre, Qinlock, Retevmo, Rukobia, Tabrecta, Tauvid, Tazverik, Tepezza, Trodelvy, Tukysa, Uplizna, Veklury, Viltepso, Zepzelca, and Zokinvy.

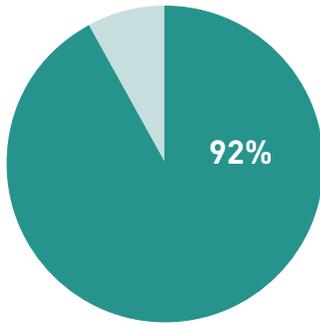
**CDER used at least one expedited development and review method to speed approval for 68% of all novel drugs approved in 2020.**



### **Predictability: Meeting PDUFA Goals**

Under the Prescription Drug User Fee Act (PDUFA), sponsors are assessed user fees that provide FDA with the additional resources needed to maintain an efficient and effective review process. Throughout the year, CDER met or exceeded almost every PDUFA goal date for application review agreed to with the pharmaceutical industry and approved by Congress. In 2020, CDER met its PDUFA goal dates for 100% of the novel drugs approved (53 of 53).

**CDER met its PDUFA Goal Date for 100% of the novel drugs approved in 2020.**



**CDER approved 49 of the 53 novel drugs of 2020 (92%) on the “first cycle”**

## Access: First Cycle Approval and Approvals Compared to Other Countries

### First Cycle Approval

CDER approved 49 of the 53 novel drugs of 2020 (92%) on the “first cycle” of review, meaning without a “complete response” letter from FDA that requires re-submission with additional information, resulting in a delay to approval. From 2011 through 2019, CDER approved 357 novel drugs, of which 304 (85%) were approved on the first cycle. This high proportion of first-cycle approval reflects the extent to which CDER staff and drug developers work together to ensure that the studies supporting approval are well designed and that the application contains the information CDER needs to be able to fully review, and if appropriate, approve an application.

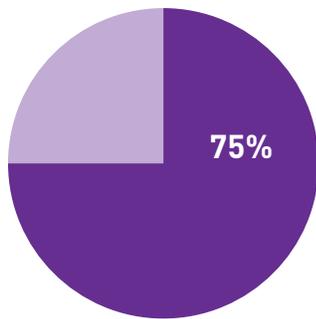
**First cycle approval prevents delays in bringing valuable new therapies to market.**

**Novel drugs approved in 2020 on the first cycle were:** artesunate, Ayvakit, Blenrep, Byfavo, Cerianna, Danyelza, Detectnet, Dojolvi, Ebanga, Enspryng, Evrysdi, Ga 68 PSMA-11, Gavreto, Gemtesa, Imcivree, Inmazeb, Inqovi, Isturisa, Klisyri, Koselugo, Lampit, Margenza, Monjuvi, Nexletol, Nurtec ODT, Ongentys, Orgovyx, Orladeyo, Oxlumo, Pemazyre, Pizensy, Qinlock, Retevmo, Rukobia, Sarclisa, Sogroya, Tabrecta, Tauvid, Tazverik, Tepezza, Tukysa, Uplizna, Veklury, Viltepso, Vyepti, Winlevi, Zeposia, Zepzelca, and Zokinvy.

### Approval in the U.S. Before Other Countries

Although regulatory processes differ widely between FDA and those of regulatory agencies in other countries, 40 of the 53 novel drugs approved in 2020 (75%) were approved in the U.S. before receiving approval in any other country.

**75% of the novel drugs approved in 2020 were approved in the U.S. before any other country.**



**40 of the 53 novel drugs approved in 2020 (75%) were approved in the U.S. before receiving approval in any other country**

**Novel drugs of 2020 approved first in the U.S. were:** Ayvakit, Blenrep, Danyelza, Detectnet, Dojolvi, Ebanga, Evrysdi, Ga 68 PSMA-11, Gavreto, Imcivree, Inmazeb, Inqovi, Klisyri, Koselugo, Margenza, Monjuvi, Nexletol, Nurtec ODT, Olinvyk, Orladeyo, Pemazyre, Qinlock, Retevmo, Rukobia, Sarclisa, Sogroya, Tabrecta, Tauvid, Tazverik, Tepezza, Trodelvy, Tukysa, Uplizna, Veklury, Vyepti, Winlevi, Xeglyze, Zeposia, Zepzelca, and Zokinvy.

**See Appendix B for a summary chart of designations for CDER’s novel drug approvals.**

## 2020's Novel Drug Approvals

### Expedited Review Pathway Usage

#### Fast Track

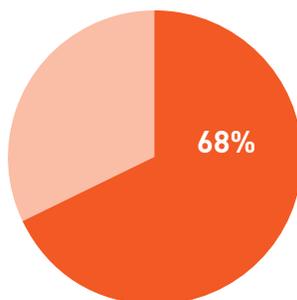
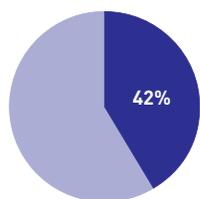
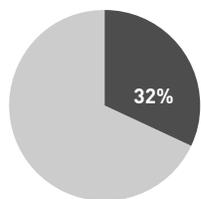
(17 of 53)

artesunate	Orladeyo
Ayvakit	Qinlock
Detectnet	Rukobia
Dojolvi	Tepezza
Enspryng	Trodelvy
Evrysdi	Tukysa
Margenza	Veklury
Monjuvi	Viltepso
Olinvyk	

#### Breakthrough Therapy

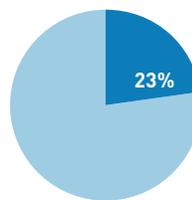
(22 of 53)

artesunate	Oxlumo
Ayvakit	Pemazyre
Blenrep	Qinlock
Danyelza	Retevmo
Ebanga	Rukobia
Enspryng	Tabrecta
Gavreto	Tepezza,
Imcivree	Trodelvy
Inmazeb	Tukysa
Koselugo	Uplizna
Monjuvi	Zokinvy



**Used One or More Expedited Pathway**  
(36 of 53)

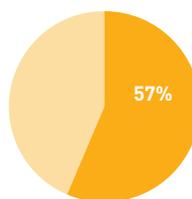
artesunate	Inqovi	Rukobia
Ayvakit	Koselugo	Tabrecta
Blenrep	Lampit	Tauvid
Danyelza	Margenza	Tazverik
Detectnet	Monjuvi	Tepezza
Dojolvi	Olinvyk	Trodelvy
Ebanga	Orgovyx	Tukysa
Enspryng	Orladeyo	Uplizna
Evrysdi	Oxlumo	Veklury
Gavreto	Pemazyre	Viltepso
Imcivree	Qinlock	Zepzelca
Inmazeb	Retevmo	Zokinvy



#### Accelerated Approval

(12 of 53)

Blenrep	Retevmo
Danyelza	Tabrecta
Gavreto	Tazverik
Lampit	Trodelvy
Monjuvi	Viltepso
Pemazyre	Zepzelca

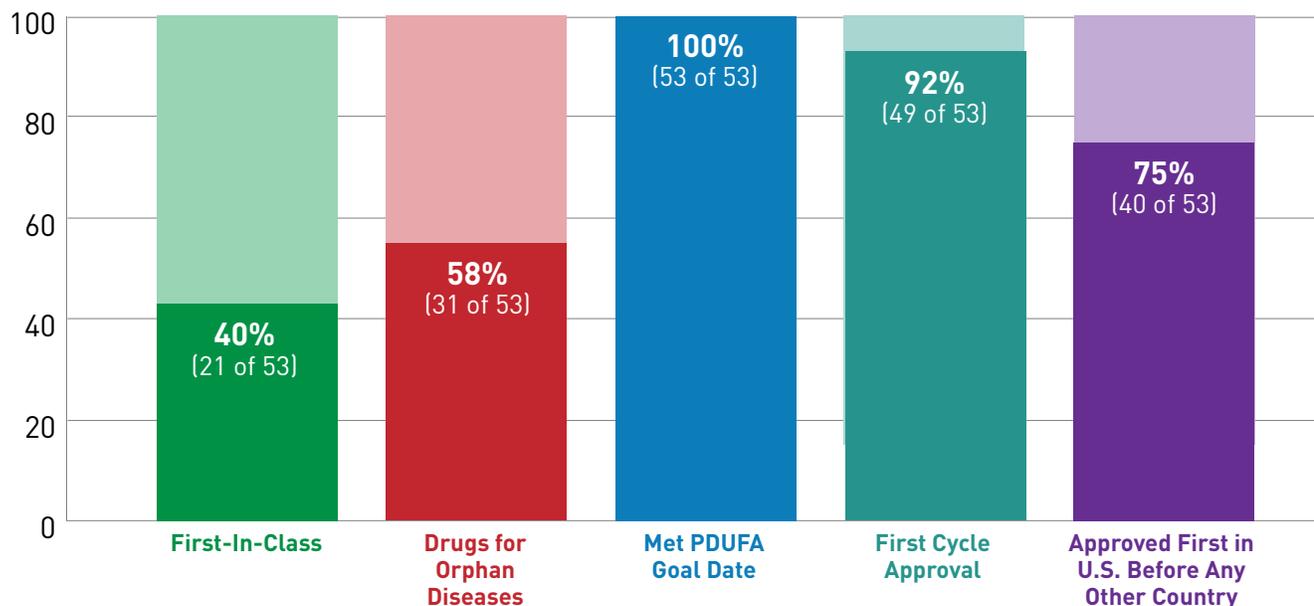


#### Priority Review

(30 of 53)

artesunate	Oxlumo
Ayvakit	Pemazyre
Blenrep	Qinlock
Danyelza	Retevmo
Detectnet	Rukobia
Ebanga	Tabrecta
Evrysdi	Tauvid
Gavreto	Tazverik
Imcivree	Tepezza
Inmazeb	Trodelvy
Inqovi	Tukysa
Koselugo	Veklury
Lampit	Viltepso
Monjuvi	Zepzelca
Orgovyx	Zokinvy

### Other Key Measures





## New and Expanded Uses of Already FDA-Approved Drugs

After CDER approves a new drug, it is not uncommon for a manufacturer to submit an application with new data that demonstrate safety and effectiveness of the same product for an additional purpose or for use in a different population of patients. Applications to modify the use of an already-approved drug or to expand its use to other patients are known as “efficacy supplements.”

### New Uses

The products below are some notable approvals of 2020 for new uses of an already-FDA-approved drug:

- **Arcalyst (rilonacept)**, [see also, Kineret, below], injection, originally approved in 2008 to treat patients with different forms of cryopyrin-associated periodic syndrome, which is a rare hereditary inflammatory disorder with symptoms that can include HIV-1-like rash, fatigue, headache, fever, pain and swelling in joints, and red eyes. In 2020, this drug was also approved for the treatment of patients with **deficiency of interleukin-1 receptor antagonist**, a very rare genetic inflammatory condition resembling an infection throughout the body or a bone infection that happens in newborns during the first days of life.
- **Bavencio (avelumab)**, [see also Jelmyto under New Formulations], injection, originally approved in 2017 for the treatment of certain patients with metastatic Merkel cell carcinoma (a rare type of skin cancer), this drug was approved in 2020 for the maintenance treatment of certain patients with locally advanced or metastatic **urothelial carcinoma** (a type of bladder cancer).

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CDER approved a new therapy to treat lupus nephritis, a serious and sometimes fatal complication of lupus.

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- **Benlysta (belimumab)**, injection, originally approved in 2011 to treat certain patients with systemic lupus erythematosus, the most common form of lupus, which can cause severe fatigue and joint pain. In 2020, this drug's approval was expanded to include treatment of patients with **lupus nephritis**, a serious and sometimes fatal complication of lupus caused by inflammation in the kidneys, making them unable to properly remove waste from the blood or control the amount of fluids in the body. This is the first FDA-approved therapy to treat lupus nephritis.
- **Cyramza (ramucirumab)** and Tarceva (erlotinib), injection and tablets respectively, approved as first-line treatment for patients with metastatic **non-small cell lung cancer** (NSCLC) whose tumors have epidermal growth factor receptor (EGFR) exon 19 deletions or exon 21 (L858R) mutations. Cyramza was originally approved in 2014 for the treatment of certain patients with either of two types of stomach cancer following a form of chemotherapy. Tarceva was originally approved in 2004 for treatment of locally advanced or metastatic NSCLC after failure of at least one prior chemotherapy regimen.
- **Crysvita (burosumab-twza)**, injection, first approved by CDER in 2018 for the treatment of patients with X-linked hypophosphatemia, an inherited disorder characterized by low levels of phosphate in the blood. In 2020, CDER expanded approval of this drug to include treatment of certain patients with **tumor-induced osteomalacia** (TIO) a rare and debilitating disease in which tumors in the body release a substance which lowers phosphate levels and weakens bones. This is the first drug approved by the FDA for TIO.
- **Epidiolex (cannabidiol)**, oral solution, originally approved by CDER in 2018 for the treatment of seizures associated with two rare and severe forms of epilepsy, Lennox-Gastaut syndrome and Dravet syndrome. In 2020, it was approved for the treatment of patients one year of age and older with **seizures associated with tuberous sclerosis complex** (TSC). This is only the second FDA approval of a drug for the treatment of seizures associated with TSC, following approval in 2018 of Afinitor Disperz (everolimus) for this use.
- **Farxiga (dapagliflozin)**, tablets, originally approved by CDER in 2014 as an adjunct to diet and exercise to improve glycemic control (blood sugar) in adults with type 2 diabetes. It was approved in 2020 to reduce the risk of cardiovascular death and hospitalization for **heart failure** --- specifically, New York Heart Association's functional class II-IV heart failure with reduced ejection fraction. Heart failure occurs when the heart does not pump enough blood to support the body's needs, and this type of heart failure happens when the heart's main pumping chamber, the left ventricle, is weakened. This is the first in this particular drug class, sodium-glucose co-transporter 2 (SGLT2) inhibitors, to be approved to treat adults with this type of heart failure.

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**CDER approved the first new therapy to help patients with tumor-induced osteomalacia (weakened bones).**

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**CDER approved a new therapy to reduce the risk of cardiovascular death and hospitalization for adult patients with a type of heart failure.**

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**CDER approved a new therapy for colorectal cancer that eliminates the need for chemotherapy.**

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- **Fetroja (cefiderocol)** [see also Recarbrio, below], injection, originally approved by CDER in 2019 to treat certain patients 18 years of age or older who have complicated urinary tract infections, was approved in 2020 to treat patients 18 years of age and older who have **hospital-acquired bacterial pneumonia/ventilator-associated bacterial pneumonia** (HABP/VABP) caused by a variety of specific microorganisms. HABP/VABP is a type of pneumonia that occurs in hospitalized patients and can cause symptoms such as fever, chills, cough, chest pain and increased oxygen requirements.
- **Imfinzi (durvalumab)**, injection, in combination with certain types of chemotherapy for the first-line treatment of adult patients with extensive-stage **small cell lung cancer**. This drug was originally approved by CDER in 2017 for the treatment of certain patients with locally advanced or metastatic urothelial carcinoma (a type of bladder cancer).
- **Inlyta (axitinib)**, tablets, originally approved in 2012 for the treatment of advanced renal cell carcinoma (a type of kidney cancer) only after failure of one prior systemic therapy (known a second-line treatment), this drug was approved in 2020 to be used in combination with certain other cancer drugs for the first-line treatment (i.e., without the need to try another treatment first) of patients with advanced **renal cell carcinoma**.
- **Keytruda (pembrolizumab)**, injection, first approved by CDER in 2014 to treat certain patients with melanoma (a skin cancer) and subsequently approved for many other uses, including several in 2020. Of note in 2020, this drug was approved to treat patients with a type of inoperable **colorectal cancer** called microsatellite instability-high or mismatch repair deficient. This is the first immunotherapy approved for this patient population as a first-line treatment which does not include chemotherapy. Also in 2020, this drug was approved for the treatment of certain patients with a type of bladder cancer unresponsive to treatment with Bacillus Calmette-Guerin (BCG) who are ineligible for or have elected not to undergo surgery. BCG is often used to treat patients with bladder cancer. This approval provides an important option for patients whose condition does not respond favorably to BCG treatment.
- **Kineret (anakinra)** [see also, Arcalyst, above], injection, first approved by CDER in 2001 to treat certain patients with active rheumatoid arthritis. It was approved in 2020, for the treatment of patients with **deficiency of interleukin-1 receptor antagonist**, a very rare genetic inflammatory condition resembling an infection throughout the body or a bone infection that happens in newborns during the first days of life.

- **Kesimpta (ofatumumab)** [see also Zeposia under Novel Approvals], injection, for the treatment of certain patients with relapsing forms of **multiple sclerosis** (MS). Ofatumumab was originally approved by CDER in 2009 --- under the trade name, Arzerra --- to treat certain patients with chronic lymphocytic leukemia. Its additional approval as Kesimpta to treat relapsing forms of MS is shared with many other FDA-approved therapies --- but Kesimpta is a subcutaneous (under the skin) patient-injected B-cell depletion agent --- the first such agent approved for relapsing forms of MS to be used without a trip to an infusion center. The other approved B-cell depletion treatment, Ocrevus (ocrelizumab), requires infusion over several hours in an outpatient setting.
- **Lynparza (olaparib)** [see also Orgovyx under Novel approvals], tablets, originally approved in capsule form in 2014 to treat certain patients with a type of ovarian cancer, this drug was approved in 2020 to treat certain patients with a form of **prostate cancer**.
- **Nucala (mepolizumab)**, injection, originally approved by CDER in 2015 as an add-on maintenance treatment of certain patients with severe asthma, and for eosinophilic granulomatosis with polyangiitis, a condition characterized by asthma, high levels of eosinophils (a type of white blood cell important in the immune system), and inflammation of small- to medium-sized blood vessels. It was approved in 2020 to treat patients with **hypereosinophilic syndrome**, a group of rare blood disorders that occur when the blood contains too many eosinophils, which eventually damage organs. This is the first approval for this disease in more than 13 years.
- **Opdivo (nivolumab)** and **Yervoy (ipilimumab)**, both injectable products, approved in 2020 to be used in combination with each other as a first-line treatment for adults with **malignant pleural mesothelioma** (MPM) which cannot be removed by surgery. This is the first drug treatment approved by the FDA for mesothelioma in 16 years (following approval of pemetrexed in 2004). MPM is a life-threatening cancer of the lungs' lining caused by inhaling asbestos fibers. In 2020, these same two drugs were also approved for use in combination to treat patients with **non-small cell lung cancer** (NSCLC). The combination was also approved in 2020 for use with certain types of chemotherapy to treat patients with NSCLC. Opdivo was originally approved by CDER in 2014 for treatment of certain patients with metastatic squamous non-small cell lung cancer. Yervoy was first approved by CDER in 2011 for the treatment of patients with unresectable or metastatic melanoma.

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## CDER approved two new treatments for patients with hospital-acquired bacterial pneumonia and ventilator-associated bacterial pneumon

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- **Ofev (nintedanib)**, capsules, to treat patients with range of **chronic fibrosing interstitial lung diseases** (ILD) with a progressive phenotype, a potentially life-threatening disease that includes lung scarring and rapid disease progression. Ofev was originally approved in 2014 to treat patients with idiopathic pulmonary fibrosis, a serious chronic lung disease that causes lung scarring and progressive difficulty breathing. In 2019, CDER also approved this drug to treat patients with systemic sclerosis-associated ILD (SSc-ILD), a subclass of ILD.
- **Otezla (apremilast)**, tablets, first approved by CDER in 2014 for adult patients with active psoriatic arthritis. In 2020 CDER expanded the approval of this drug to include treatment of patients with **scalp psoriasis**, a skin disorder of the scalp that makes raised, reddish, often scaly patches and can spread to the forehead, back of the neck, or behind and inside the ears.
- **Ozempic (semaglutide)**, injection, originally approved by CDER in 2017 as an adjunct to diet and exercise to improve glycemic control (blood sugar) in adults with type 2 diabetes. In 2020, it was approved to **reduce the risk of major adverse cardiovascular events** (cardiovascular death, non-fatal myocardial infarction or non-fatal stroke) in adults with type 2 diabetes and established cardiovascular disease.
- **Recarbrio (imipenem-cilastatin and relebactam)** [see also, Fetroja, above], injection, first approved by CDER in 2019 to treat certain patients with complicated urinary tract infections and complicated intra-abdominal infections. It was approved in 2020 to treat adult patients with **hospital-acquired bacterial pneumonia** and ventilator-associated bacterial pneumonia, a type of pneumonia that occurs in hospitalized patients and can cause symptoms such as fever, chills, cough, chest pain and increased oxygen requirements.
- **Simponi Aria (golimumab)** [see also, Xeljanz, below], injection, originally approved in 2013 for the treatment of certain adult patients with rheumatoid arthritis. It was approved in 2020 to also treat patients with **polyarticular juvenile idiopathic arthritis**, a type of juvenile arthritis (arthritis in children) that causes inflammation in five or more joints within the first six months of the disease.

- **Tecentriq (atezolizumab)**, injection, originally approved by CDER in 2016 to treat certain patients with metastatic urothelial carcinoma (a type of bladder cancer), this drug was approved in 2020 for three additional uses, 1) to treat certain patients with unresectable or metastatic BRAF V600 mutation positive **melanoma** (a type of skin cancer), 2) to treat certain patients with a type of **liver cancer**, and, 3) to treat certain patients with **non-small cell lung cancer**.
- **Trulicity (dulaglutide)**, injection, originally approved by CDER in 2017 as an adjunct to diet and exercise to improve glycemic control (blood sugar) in adults with type 2 diabetes. In 2020, it was approved to **reduce the risk of major adverse cardiovascular events** (cardiovascular death, non-fatal myocardial infarction or non-fatal stroke) in adults with type 2 diabetes **with or without established cardiovascular disease**. This is the first type 2 diabetes medicine approved by CDER to reduce cardiovascular events in adults with or without established cardiovascular disease.
- **Xeljanz (tofacitinib)** [see also, Simponi Aria, above], tablets, first approved by CDER in 2012 to treat certain patients with rheumatoid arthritis. In 2020 CDER expanded this drug's approval to include treatment of patients with **polyarticular juvenile idiopathic arthritis** a type of juvenile arthritis (arthritis in children) that causes inflammation in five or more joints within the first 6 months of the disease.
- **Xofluza (baloxavir marboxil)**, tablets, first approved by CDER in 2018 for the treatment of uncomplicated flu within 48 hours of symptoms in otherwise healthy patients 12 years of age and older. In 2020, it was approved for use in a single dose for **influenza prophylaxis** (flu prevention) in persons exposed to a household contact with influenza. This is the first FDA-approved flu prevention medication taken as a single dose in pill form.

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CDER approved two new therapies to treat a type of arthritis in children.

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## New Populations

The products listed below are notable approvals in 2020 of an already-approved drug for use in an expanded population of patients:

- **Alunbrig (brigatinib)**, tablets. Following original approval as an NME in 2017 for treatment of patients with anaplastic lymphoma kinase (ALK)-positive metastatic **non-small cell lung cancer** who have progressed on or are intolerant to crizotinib, CDER approved this drug for a broader patient population in 2020 by removing the stipulation that the patient have progressed or be intolerant to crizotinib.
- **Dupixent (dupilumab)**, injection, originally approved by CDER in 2017 for the treatment of certain adult patients with **atopic dermatitis**, often called eczema, a chronic skin condition characterized by dry, itchy skin. It was approved in 2020 for the treatment of this same condition of for patients aged six years and older.
- **Epclusa (sofosbuvir and velpatasvir)**, tablets, originally approved by CDER treatment of certain adult patients with **chronic hepatitis C** virus (HCV) genotypes 1, 2, 3, 4, 5, and 6. In 2020, it was approved to treat children ages six years and older or weighing at least 37 pounds (17 kilograms) with any of the six HCV genotypes—or strains—without cirrhosis

(liver disease) or with mild cirrhosis. Epclusa in combination with ribavirin is indicated for the treatment of pediatric patients six years and older or weighing at least 37 pounds with severe cirrhosis. HCV is a viral disease that causes inflammation of the liver that can lead to diminished liver function or liver failure. This is now the first hepatitis C drug approved to treat all six strains in children.

- **Ilaris (canakinumab)**, injection, first approved by CDER in 2009 cryopyrin-associated periodic syndromes a rare hereditary inflammatory disorder with symptoms that can include HIV-1-like rash, fatigue, headache, fever, pain and swelling in joints, and red eyes. In 2013 it was also approved to treat patients with systemic juvenile idiopathic arthritis (SJIA), a rare disease that causes body-wide swelling in joints. It was approved in 2020 for the treatment of **active Still's disease**, a rare type of inflammatory arthritis that features fevers, rash and joint pain, and **adult-onset Still's disease (AOSD)** a rare and serious autoinflammatory disease of unknown origin and its symptoms are similar to SJIA. This is the first FDA-approved drug to treat patients with AOSD.
- **Lialda (mesalamine)**, tablets, first approved by CDER in 2007 as a new dosage form of mesalamine, a drug originally approved in 1987 as an enema for patients with ulcerative colitis. Lialda's approval in 2007 was for the induction of remission in certain adult patients with **ulcerative colitis**. It was approved in 2020 for this same use in children. Ulcerative colitis is a chronic disease of the large intestine, in which the lining of the colon becomes inflamed and develops open sores, or ulcers. Symptoms range from mild to severe and include rectal bleeding, bloody diarrhea, abdominal cramps, and pain. Patients with ulcerative are at increased risk of developing colon cancer.
- **Taltz (ixekizumab)**, injection, originally approved by CDER in 2016 for the treatment of adult patients with moderate-to-severe **plaque psoriasis** who are candidates for systemic therapy or phototherapy. In 2020 CDER expanded the approval of this drug for the same use in children aged six years or older. Psoriasis is a skin disease that causes red, itchy scaly patches, most commonly on the knees, elbows, trunk and scalp. Plaque psoriasis is the most common form of psoriasis and appears as raised, red patches covered with a silvery-white buildup of dead skin cells.
- **Tagrisso (osimertinib)**, tablets, originally approved in 2015 to treat certain patients with metastatic epidermal growth factor receptor (EGFR) T790M mutation-positive-**non-small cell lung cancer** who have progressed on or after EGFR TKI (tyrosine kinase inhibitor) therapy, CDER expanded this drug's approved use to a larger group of patients by enabling patients to use this drug as adjuvant therapy (treatment to prevent recurrence after initial treatment).

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**CDER approved a new therapy for pediatric patients with ulcerative colitis.**

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## Additional Approvals

In addition to the many notable novel drug and efficacy supplement approvals of 2020, CDER approved a variety of other therapies. Among these are **biosimilars**, and **new formulations, manufacturers, combinations, or dosage forms** of already FDA-approved drugs, as well as others. Below are notable examples of these various types of approvals.

### Biosimilars

An FDA-approved biosimilar is a biological product that is highly similar to and has no clinically meaningful differences in terms of safety, purity and potency (safety and effectiveness) from an already FDA-approved product, called the reference product. Biological products are highly complex, and often used to treat patients with serious and life-threatening conditions. The law allowing FDA to approve biosimilars was designed to create competition, increase patient access, and potentially reduce cost of important therapies.

#### In 2020, CDER approved:

- A biosimilar to Rituxan (rituximab), **Riabni** (rituximab-arrx), approved to treat patients with non-Hodgkin lymphoma (cancer that starts in the lymphatic system), chronic lymphocytic leukemia (a type of cancer of the blood and bone marrow), rheumatoid arthritis, granulomatosis with polyangiitis and microscopic polyangiitis (disorders involving inflammation of blood vessels), and, pemphigus vulgaris (a rare, severe disease in which blisters break out on the skin, lining of the mouth, and other mucous membranes).
- A biosimilar to Humira (adalimumab), **Hulio** (adalimumab-fkjp), for the treatment of rheumatoid arthritis, juvenile idiopathic arthritis (4 years and older), psoriatic arthritis, ankylosing spondylitis, adult Crohn's disease, ulcerative colitis and plaque psoriasis.
- A biosimilar to Neulasta (pegfilgrastim), **Nyvepria** (pegfilgrastim-apgf), approved to decrease the incidence of infection by treating **neutropenia** (a lack of certain white blood cells) caused by cancer chemotherapy.

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**Biosimilars expand treatment options and bring competition to the U.S. marketplace.**

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CDER has now approved a total of 29 biosimilars for 9 different reference products since 2015. This includes at least one biosimilar for each of these top selling biological drugs in the U.S. Humira, Rituxan, Enbrel, Herceptin, Avastin, Remicade, and Neulasta. One reference product (Humira), now has six biosimilars. One reference product (Herceptin) now has five biosimilars. Two reference products (Remicade and Neulasta) have four biosimilars. One reference product (Rituxan) has three biosimilars. Three reference products (Avastin, Enbrel, and Neupogen) have two biosimilars. One reference product (Epogen/Procrit) has one biosimilar. Multiple biosimilars for

an FDA-approved reference product can strengthen market competition. An increase in market competition may lead to significantly reduced costs for both patients and our healthcare system.

## New Formulations and Other Notable Approvals

A new formulation of a drug is one in which the product's active ingredient is already FDA-approved. New formulations of already-approved drugs can offer significant advances in therapy. Below are **notable new formulations** as well as other notable non-novel drug approvals of 2020, including, but not limited to, those with a **new combination of active ingredients** or a **new manufacturer** of an already FDA-approved drug.

- **Darzalex Faspro (daratumumab and hyaluronidase-fihj)**, subcutaneous (under the skin) injection, approved in 2020 to treat certain patients with **multiple myeloma**; it is injected under the skin by a healthcare provider as an abdominal injection that takes a few minutes. This new approval represents a significant advantage over the original formulation of Darzalex (daratumumab without hyaluronidase-fihj), which requires an intravenous (into-the-vein) infusion given over several hours.
- **Jelmyto (mitomycin)** [see also, Bavencio, under New Uses], powder, a new formulation of mitomycin, a drug approved by CDER for decades as a combination therapy with other drugs to treat patients with cancer of the stomach and pancreas. It was approved by CDER in 2020 to treat low-grade upper tract **urothelial cancer**, a disease affecting the lining of the urinary system.
- **Lyumjev (insulin lispro-aabc)**, injection, evaluated under an expedited review program via comparison to the FDA-approved insulin lispro innovator product, Humalog, which was approved by CDER in 1996 to improve glycemic control (blood sugar) in adult and pediatric patients with **type 1 diabetes** or adult patients with **type 2 diabetes**, this product was approved by CDER in 2020 for the same uses. Multiple versions of this type of insulin may promote market competition and enhanced patient access.
- **Oriahn (elagolix, estradiol, and, norethindrone)**, capsules, a combination of three already FDA-approved products used separately, or sometimes in combination, for a variety of women's health issues (e.g., uterus pain, menopause symptoms, osteoporosis, birth control, and hormonal imbalances), this drug was approved in 2020 as the first non-surgical treatment for the management of **heavy menstrual bleeding associated with fibroids** (a kind of tumor that is typically non-cancerous) in premenopausal women.

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## CDER approved four new notable therapies for various forms of breast cancer in 2020.

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- **Phesgo (pertuzumab, trastuzumab, and, hyaluronidase-zzxf)**, subcutaneous (SQ, under the skin) injection, a combination of two already FDA-approved drugs used to treat certain patients with breast cancer and another FDA-approved drug that helps injected medicine disperse through the body, this drug was approved in 2020 to treat adult patients with early HER2-positive breast cancer, and **HER2-positive breast cancer** that has spread to other parts of the body. With this new SQ administration, this approval offers an outpatient option for patients to receive trastuzumab and pertuzumab.
- **Semglee (insulin glargine)**, injection, evaluated under an expedited review program via comparison to the FDA-approved insulin glargine innovator product, Lantus, which was approved by CDER in 2000 to improve glycemic control (blood sugar) in adult and pediatric patients with **type 1 diabetes** or adult patients with **type 2 diabetes**. This drug was approved by CDER in 2020 for the same uses. Multiple versions of this type of insulin may promote market competition and enhanced patient access.

### New Dosage Forms

New dosage forms for already FDA-approved drugs can improve patient health by helping to increase patient adherence to therapy, ensure a proper dose is taken, and improve quality of life for patients who must use the medication on a prolonged basis. Notable approvals in this category include:

- **Fintepla (fenfluramine)**, oral solution, to help prevent seizures in certain pediatric patients with **Dravet syndrome**, a severe form of epilepsy characterized by prolonged seizures. Patients often develop Dravet syndrome before age one. This drug is a needed treatment because many patients do not respond well to other FDA-approved therapies. Fenfluramine was previously approved by the FDA in tablet form for the treatment of obesity but was removed from the market for causing adverse cardiac (heart) side effects.
- **Gimoti (metoclopramide)**, nasal spray, a new formulation of metoclopramide, originally approved as an injectable medication in 1979 and in tablet form in 1980, under the trade name Reglan, to treat gastroparesis, a condition that affects the stomach muscles and prevents proper stomach emptying. It was approved in nasal spray form in 2020 for the relief of symptoms in adults with acute and recurrent **diabetic gastroparesis**, a type of gastroparesis caused by diabetes.

- **sotalol hydrochloride**, intravenous (IV) injection, originally approved as an oral tablet in 1992 under the trade name Betapace --- and subsequently approved for marketing by other manufacturers as tablets and an oral solution, sotalol is approved by the FDA to control different forms of **irregular heartbeat**. In 2020 it was approved as an injection for patients who are unable to take the drug orally. Prior to this approval, initiating sotalol in new patients would involve a hospital stay of three days in order to monitor patients. The new approval allows for initiation with sotalol IV followed by a transition to oral sotalol --- without hospitalization.
- **Tivicay PD (dolutegravir)**, tablets for oral suspension, approved in 2020 to treat **HIV-1 infection** in pediatric patients at least four weeks old and weighing at least three kilograms (kg) (6.61 pounds) in combination with other antiretroviral treatments. Dolutegravir was originally approved by CDER in 2013 to treat HIV-1 infection in adults and children 12 years of age and older and weighing at least 40 kg. This approval expands the patient population approved to use this drug to include much younger and smaller children --- a significant advance; for babies and young children with HIV, getting treatment early is very important as HIV can progress more quickly in children than adults.
- **VESIcare LS (solifenacin succinate)**, oral suspension, originally approved by CDER in 2004 in tablet form for the treatment certain adult patients with overactive bladder. It was approved in 2020 as an oral suspension for the treatment of children two years of age and older with **neurogenic detrusor overactivity** (NDO), a form of bladder dysfunction related to neurological impairment. This is the first FDA-approved treatment for NDO patients as young as two years of age.

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**CDER Approved a new HIV-1 therapy that will help ensure earlier treatment for many babies and pediatric patients.**

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**As with all FDA-approved products, the new drug therapies discussed in this report have risks. For more information about these drugs and for complete risk information, see the drugs' approval letters and FDA-approved labeling at [Drugs@FDA](mailto:Drugs@FDA).**



## Conclusion

Drug review and approval challenges in 2020 included overcoming difficulties in completing facility inspections, conducting remote advisory committee meetings, and facilitating staff working from home under less-than-ideal circumstances, among many others. Despite these challenges from the COVID-19 public health emergency, CDER approved many new drug therapies for patients in need. CDER's staff consists of individuals with a range of expertise, including physicians, safety evaluators, chemists, biologists, biostatisticians, nurses, pharmacists, pharmacologists, epidemiologists, legal and regulatory experts, and many more. They work together to bring safe and effective drug therapies to the American public as efficiently as possible.

These therapies come in the form of novel drugs never before marketed in the United States, other new drugs that add important medical value, already FDA-approved products approved for new uses and for administration to new populations of patients, and new dosage forms of products designed to offer advantages over earlier versions.

More important than the quantity of the new therapies is their medical value and the important new roles these drugs are serving to advance patient care.

Also noteworthy is the efficiency with which these drugs were reviewed and approved. CDER used a variety of expedited development and regulatory review tools to help speed these drugs to market.

In all cases, CDER maintains its rigorous standards for demonstration of safety and efficacy while striving for efficiency of review of applications for new drug therapies.



Our drug therapy approvals of 2020 will help many patients in need for years to come. However, CDER's mission goes well beyond critically reviewing the safety and efficacy of drug applications we receive from industry. We also look to advance the science and technology that can lead to future innovative drugs --- many of which may not yet even be conceived. We are working to develop more innovative and efficient approaches for the development and study of the drug therapies that will emerge from these technological advances.

Although our regulatory work extends to many scientific, clinical, and technological areas, we cannot accomplish all that is necessary on our own. CDER works collaboratively with a wide range of stakeholders across the medical community, including academia, industry, patients and their caregivers, patient advocacy groups, state and other federal agencies, and more. Listening is an important component of our work. We strive to ensure that we understand the needs of our key constituencies and that we are providing the most benefit for patients and the strongest possibilities for improved public health in America.

**Although not a comprehensive compilation of our approvals for the year, this report serves to provide a wide variety of valuable examples of the many ways CDER approves new drug therapies to enhance patient health.**

## Appendix A:

### CDER's Novel Approvals of 2020 (In alphabetical order)

For information about vaccines, allergenic products, blood and blood products, cellular and gene therapy products, go to [2020 Biological License Application Approvals](#).

Approval Date	Trade Name	Active Ingredient(s)	Summary of FDA-approved use on approval date (see Drugs@FDA for complete indication)	Dosage Form
05/26/20		artesunate	To treat severe malaria	Injection
01/09/20	Ayvakit	avapritinib	To treat unresectable or metastatic gastrointestinal stromal tumor	Tablet
02/26/20	Barhemsys	amisulpride	To help prevent nausea and vomiting after surgery	Injection
08/05/20	Blenrep	belantamab mafodotin-blmf	To treat relapsed or refractory multiple myeloma	Injection
07/02/20	Byfavo	remimazolam	For procedural sedation	Injection
05/20/20	Cerianna	fluoroestradiol F 18	Diagnostic imaging agent for certain patients with breast cancer	Injection
11/25/20	Danyelza	naxitamab-ggqk	To treat refractory or relapsed high-risk neuroblastoma	Injection
09/03/20	Detectnet	copper Cu 64 dotatate	Diagnostic imaging agent for certain types of neuroendocrine tumors	Injection
06/30/20	Dojolvi	triheptanoin	To treat molecularly confirmed long-chain fatty acid oxidation disorders	Oral Liquid
12/21/20	Ebanga	ansuvimab-zykl	To treat Ebola virus	Injection
08/14/20	Enspryng	satralizumab-mwge	Neuromyelitis optica spectrum disorder	Injection
08/07/20	Evrysdi	risdiplam	To treat spinal muscular atrophy	Powder for Oral Solution
12/01/20		gallium Ga 68 PSMA-11	Diagnostic imaging agent for certain lesions in men with prostate cancer	Injection
09/04/20	Gavreto	pralsetinib	To treat non-small lung cancer	Capsule
12/23/20	Gemtesa	vibegron	Treatment of overactive bladder	Tablet
11/25/20	Imcivree	setmelanotide	To treat obesity and the control of hunger associated with specific enzyme deficiencies including proopiomelanocortin deficiency	Injection
10/14/20	Inmazeb	atoltivimab, maftivimab, odesivimab-ebgn	To treat Ebola virus	Injection
07/07/20	Inqovi	cedazuridine, decitabine	To treat myelodysplastic syndromes	Tablet
03/06/20	Isturisa	osilodrostat	To treat Cushing's disease who either cannot undergo pituitary gland surgery or have undergone the surgery but still have the disease	Tablet
12/14/20	Klisyri	tirbanibulin	Actinic keratosis of the face or scalp	Ointment
04/10/20	Koselugo	selumetinib	To treat neurofibromatosis type 1	Capsule
08/06/20	Lampit	nifurtimox	To treat Chagas disease in certain pediatric patients younger than age 18	Tablet
12/16/20	Margenza	margetuximab-cmkb	Metastatic HER2-positive breast cancer	Injection
07/31/20	Monjuvi	tafasitamab-cxix	To treat relapsed or refractory diffuse large B-cell lymphoma	Injection
02/21/20	Nexletol	bempedoic acid	To treat adults with heterozygous familial hypercholesterolemia or established atherosclerotic cardiovascular disease who require additional lowering of LDL-C	Tablet

Approval Date	Trade Name	Active Ingredient(s)	Summary of FDA-approved use on approval date (see Drugs@FDA for complete indication)	Dosage Form
02/27/20	Nurtec ODT	rimegepant	To treat migraine	Oral Disintegrating Tablet
08/07/20	Olinvyk	oliceridine	To manage severe acute pain in certain adults	Injection
04/24/20	Ongentys	opicapone	To treat patients with Parkinson's disease experiencing "off" episodes	Capsule
12/18/20	Orgovyx	relugolix	Advanced prostate cancer	Tablet
12/03/2020	Orladeyo	berotralstat	Prophylaxis for attacks of hereditary angioedema	Capsule
11/23/20	Oxlumo	lumasiran	To treat primary hyperoxaluria type 1	Injection
04/17/20	Pemazyre	pemigatinib	To treat locally advanced or metastatic cholangiocarcinoma	Tablet
02/12/20	Pizensy	lactitol	To treat chronic idiopathic constipation	Powder for Oral Solution
05/15/20	Qinlock	ripretinib	To treat advanced gastrointestinal stromal tumor	Tablet
05/08/20	Retevmo	selpercatinib	To treat metastatic non-small cell lung cancer and thyroid cancers	Capsule
07/02/20	Rukobia	fostemsavir	To treat HIV-1	Tablet
03/02/20	Sarclisa	isatuximab-irfc	To treat refractory multiple myeloma	Injection
08/28/20	Sogroya	somapacitan-beco	Growth hormone deficiency	Injection
05/06/20	Tabrecta	capmatinib	To treat metastatic non-small cell lung cancer	Tablet
05/28/20	Tauvid	flortaucipir F 18	Diagnostic agent for patients being evaluated for Alzheimer's disease	Injection
01/23/20	Tazverik	tazemetostat	To treat metastatic or locally advanced epithelioid sarcoma	Tablet
01/21/20	Tepezza	teprotumumab-trbw	To treat thyroid eye disease	Injection
04/22/20	Trodelyv	sacituzumab govitecan-hziy	To treat metastatic triple-negative breast cancer	Injection
04/17/20	Tukysa	tucatinib	To treat advanced unresectable or metastatic HER2-positive breast cancer	Tablet
06/11/20	Uplizna	inebilizumab-cdon	To treat neuromyelitis optica spectrum disorder	Injection
10/22/20	Veklury	remdesivir	To treat COVID-19	Injection
08/12/20	Viltepso	viltolarsen	Duchenne muscular dystrophy	Injection
02/21/20	Vyepti	eptinezumab-jjmr	For the preventive treatment of migraine	Injection
08/26/20	Winlevi	clascoterone	To treat acne vulgaris	Cream
07/24/20	Xeglyze	abametapir	To treat head lice	Lotion
03/25/20	Zeposia	ozanimod	To treat relapsing forms of multiple sclerosis	Capsule
06/15/20	Zepzelca	lurbinectedin	To treat metastatic small cell lung cancer	Injection
11/20/20	Zokinvy	lonafarnib	To reduce the risk of death due to rare genetic diseases that cause premature aging	Capsule

## Appendix B: Novel Drug Designation Summary (In alphabetical order)

Trade Name	First-in-Class	Orphan Designation	Fast Track	Breakthrough	Priority	Accelerated Approval	Met. PDUFA Goal	First Cycle Approval	First in the United States
artesanate									
Ayvakit									
Barhemsys									
Blenrep									
Byfavo									
Cerianna									
Danyelza									
Detectnet									
Dojolvi									
Ebanga									
Enspryng									
Evrysdi									
Ga 68 PSMA-11									
Gavreto									
Gemtesa									
Imcivree									
Inmazed									
Inqovi									
Isturisa									
Klisyri									
Koselugo									
Lampit									
Margenza									
Monjuvi									
Nexletol									
Nurtec ODT									
Olinvyk									

Trade Name	First-in-Class	Orphan Designation	Fast Track	Breakthrough	Priority	Accelerated Approval	Met PDUFA Goal	First Cycle Approval	First in the United States
Ongentys									
Orgovyx									
Orladeyo									
Oxlumo									
Pemazyre									
Pizensy									
Qinlock									
Retevmo									
Rukobia									
Sarclisa									
Sogroya									
Tabrecta									
Tauvid									
Tazverik									
Tepezza									
Trodelyv									
Tukysa									
Uplizna									
Veklury									
Viltepso									
Vyepti									
Winlevi									
Xeglyze									
Zeposia									
Zepzelca									
Zokinvy									



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