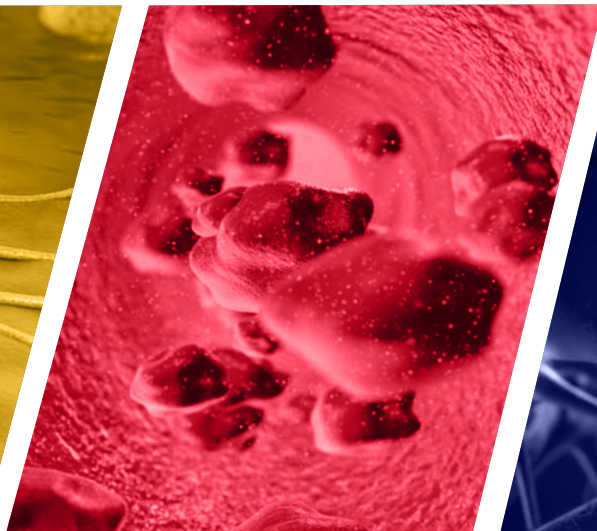
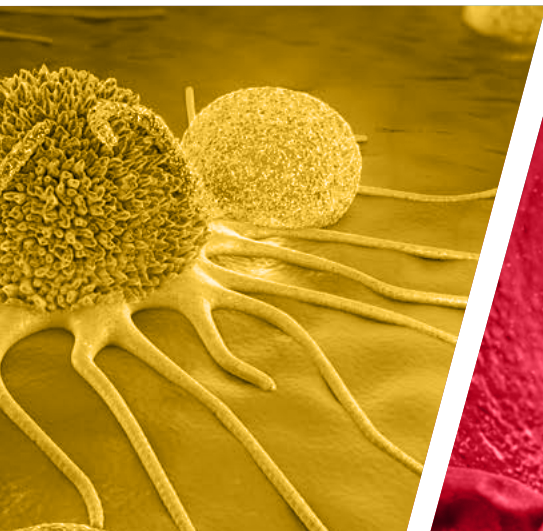




**U.S. FOOD & DRUG
ADMINISTRATION**

CENTER FOR DRUG EVALUATION AND RESEARCH
ADVANCING HEALTH
THROUGH INNOVATION
**2017 NEW DRUG
THERAPY APPROVALS**

Impact | Innovation | Predictability | Access | Engagement



January 2018
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Table of Contents

Introduction	5
2017: A Year of Innovation and Advances	6
CDER’s Drug Therapy Approvals of 2017	8
Novel Drugs	8
Impact of Novel Drug Approvals	10
Innovation: Frequent Use of Expedited Development and Review Pathways	13
Predictability: Meeting PDUFA Goals	14
Access: First Cycle Approval and Approvals Compared to Other Countries	15
New and Expanded Uses of Already FDA-Approved Drugs	17
New Uses	17
New Populations.....	20
Additional Approvals	21
Biosimilars	21
New Formulations	22
Other New Formulations and Notable Approvals	23
New Dosage Forms.....	26
Ensuring Access to CDER-Regulated Products in an Emergency.....	27
External Engagement	29
Medical and Scientific Engagement.....	29
The Voice of the Patient	31
Conclusion	32
Appendix A: Drug Designation Summary.....	34



Introduction

Welcome to our new report, *Advancing Health through Innovation: New Drug Approvals and Other Drug Therapy Advances of 2017*. Every year since 2012, FDA's Center for Drug Evaluation and Research (CDER) has issued our *Novel Drugs* summary, a report that helps illustrate CDER's role in bringing innovative new drug therapies that are safe and effective to patients in need. This year, we have expanded the report to provide even more valuable information.

Novel drugs can represent important new therapies for advancing patient care. As you will see in this report, 2017 was no exception. There is, however, more to the story. Our report this year also highlights new approvals for drugs that are not novel, yet offer significant clinical advances that provide important medical value. As in other years, many important advances in 2017 are approvals to use an already FDA-approved drug for a new purpose or to treat a new population of patients, such as children.

Our report also emphasizes the many innovative regulatory tools CDER uses to enhance our efficiency and expedite the review and approval of drug therapies never marketed in the United States. It is important to recognize that the decisions we made on these approvals were completed by or before their goal dates as defined by Congressionally-approved agreements with industry (referred to as user fee programs), and that most were approved in the United States before any other country in the world.

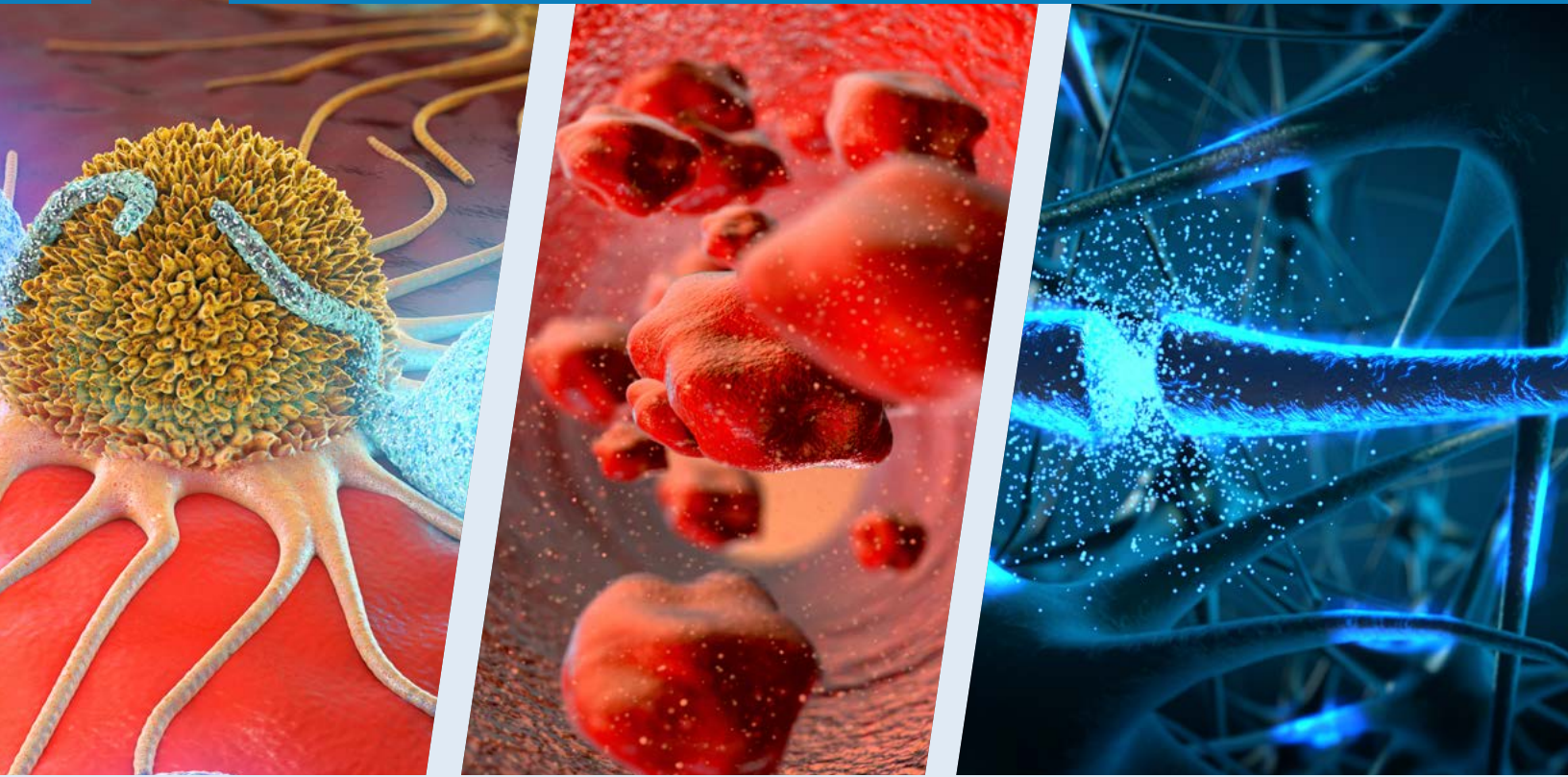
CDER also played an active role in FDA's response to the severe hurricanes of 2017 by maintaining communications with pharmaceutical manufacturers, identifying potential shortages of key medical products and working closely with manufacturers to address these shortages, conducting expedited reviews, and utilizing enforcement discretion to mitigate drug shortages of medically necessary products.

Importantly, CDER cannot effectively evaluate the safety and effectiveness of all of these new drug therapies without a great deal of help from a wide range of stakeholders throughout manufacturing, scientific, medical, and patient-focused organizations. We take the opportunity in this report to highlight some of the many public-private partnerships and consortia CDER leads or participates in to support innovation and improve health care. The role of the patient in drug development and approval is becoming increasingly vital. Our report also shares some of the important progress FDA has made in incorporating the patient perspective into our decision-making.

We hope our new report provides a deeper understanding of the *many* ways CDER works to support innovation and improve treatments for patients.



Janet Woodcock, M.D.
Director, Center for Drug
Evaluation and Research



2017: A Year of Innovation and Advances

In 2017, FDA's Center for Drug Evaluation and Research's (CDER's) new drug therapy approvals helped a wide range of patients suffering from many different medical conditions—from rare disorders to common diseases—to gain new hope for improved quality of life, and in some cases, improved chances of surviving life-threatening illnesses.

Rare Diseases

Among many other new approvals to help patients with rare diseases, CDER approved the first new treatment for patients with **sickle cell disease** in almost 20 years and the first-ever non-blood product to treat patients with **hemophilia A with inhibitors**. For the first time, a treatment is available for adults diagnosed with **giant cell arteritis**, a rare condition that results in inflammation of blood vessels. CDER also approved a new treatment for the rare condition known as **Batten disease**, which can cause seizures, dementia, and a variety of other debilitating symptoms.

Many new treatment options
for patients in need.

Infectious Diseases

We approved a new antibiotic to treat certain types of **serious skin infections**, and another to treat **complicated urinary tract infections**, including kidney infections. We also approved two new treatments for certain patients with **chronic hepatitis C**; a new drug to help prevent **cytomegalovirus infection** in patients who have received a bone marrow transplant; and, the first therapy in the United States to treat **Chagas disease**, a rare parasitic disease which, after years of infection, can cause serious heart illness.

Neurological Disorders

Last year was a particularly productive year for approving new therapies for patients with neurological disorders. CDER approved new therapies to treat patients with **tardive dyskinesia**, a frequent side effect of psychiatric medications, **myasthenia gravis**, a rare neuromuscular disease, and new treatments for **Duchenne muscular dystrophy**, for certain forms of **multiple sclerosis**, for **amyotrophic lateral sclerosis** (often called Lou Gehrig's disease), and for **Parkinson's disease**.

Cancer Therapies

2017 was also another strong year for making new cancer therapies available to patients in need. Among others, we approved new therapies for certain patients with **acute lymphoblastic leukemia**; **Merkel cell carcinoma**; certain forms of **relapsed or refractory acute myeloid leukemia**; certain forms of **lymphoma**; **recurrent epithelial ovarian, fallopian tube, or primary peritoneal cancer**; and specific forms of **liver, breast, and colorectal cancer**. We also approved the first cancer treatment based on a genetic feature of a cancer rather than the location of the body where the tumor originated.

Other Advances

Also in 2017, CDER approved a new therapy for decreasing heart risk for patients with **diabetes**, a new drug to treat adults with moderate-to-severe **eczema** and three therapies to treat patients with moderate-to-severe **plaque psoriasis**. Two of these were for adults and one was for adolescents. We also approved the first drug in the United States with a sensor embedded in the pill that records that the medication was taken. And in response to the devastation inflicted by the 2017 Hurricane Season, CDER worked with pharmaceutical manufacturers with facilities in affected areas to address potential drug shortages.

CDER's drug therapy approvals of 2017 will help a wide range of patients suffering from many different diseases.

CDER's Drug Therapy Approvals of 2017

In 2017, 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/or help advance clinical care by providing therapies never before marketed in the United States;
- **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 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 approved by CDER discussed in this report are associated with risks.

For more information about these drugs and for complete risk information, see the drugs' approval letters and FDA-approved labeling at [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 2017 and also discusses those that CDER considers notable advances. In 2017, CDER approved 46 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 2017

CDER's novel drug approvals for 2017 are listed below.* See [CDER's Novel Drug Approvals for 2017](#) on the FDA website for the approval dates, non-proprietary names, and what each drug is used for.

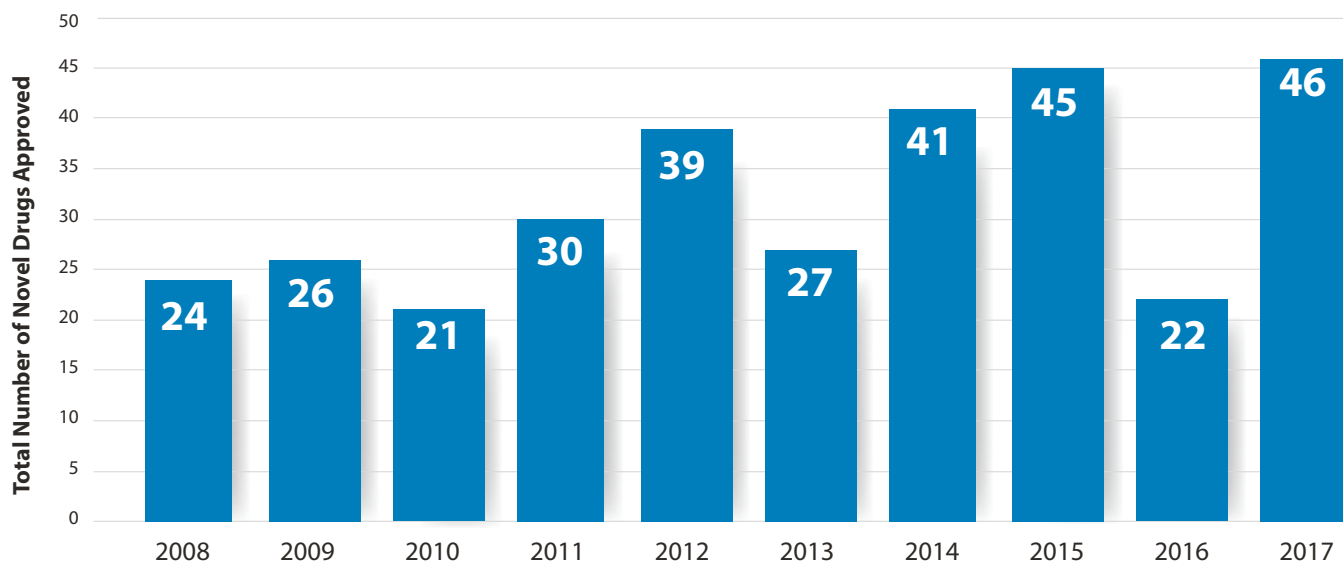
Aliqopa	Brineura	Imfinzi	Ocrevus	Solosec	Vosevi
Alunbrig	Calquence	Ingrezza	Ozempic	Steglatro	Vyzulta
Austedo	Dupixent	Kevzara	Parsabiv	Symproic	Xadago
Bavencio	Emflaza	Kisqali	Prevymis	Tremfya	Xermelo
Baxdela	Fasenra	Macrilen	Radicava	Trulance	Xepi
benznidazole**	Giapreza	Mavyret	Rhopressa	Tymlos	Zejula
Besponsa	Hemlibra	Mepsevii	Rydapt	Vabomere	
Bevyxxa	Idhifa	Nerlynx	Siliq	Verzenio	

* This information is accurate as of December 31, 2017. 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: 2008 - 2017

In 2017, CDER approved 46 novel drugs. The ten-year graph below shows that from 2008 through 2016, CDER has averaged about 31 novel drug approvals per year.



Drugs designated First-in-Class and those approved to treat patients with rare diseases often have a strong positive impact on patient health.

Impact of Novel Drug Approvals

Many of the novel drugs CDER approved in 2017 are notable for their potential positive impact and unique contributions to quality medical care and patient treatment.

First-in-Class

CDER identified 15 of the 46 novel drugs approved in 2017 (33%) 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 2017 identified as first in class by FDA were: Besponsa, Brineura, Dupixent, Emflaza, Giapreza, Hemlibra, Idhifa, Macrilen, Mepsevii, Ocrevus, Prevyimis, Radicava, Rhopressa, Rydapt, and Xermelo.

Examples of notable novel First-in-Class approvals for 2017 include:

- **Dupixent (dupilumab)** to treat adults with **moderate-to-severe eczema** (atopic dermatitis), and;
- **Ocrevus (ocrelizumab)** to treat adults with relapsing forms of **multiple sclerosis** and primary progressive multiple sclerosis.

Drugs for Rare Diseases

In 2017, 18 of CDER's 46 novel drugs (39%) 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 2017 with the orphan drug designation were: Aliqopa, Alunbrig, Austedo, Bavencio, benznidazole, Besponsa, Brineura, Calquence, Emflaza, Hemlibra, Idhifa, Macrilen, Mepsevii, Prevyimis, Radicava, Rydapt, Xermelo, and Zejula.

Examples of drugs that advance the care of patients with rare diseases approved in 2017 include:

- **Brineura (cerliponase alfa)**, a treatment for a specific form of **Batten disease**, a rare disease that can cause progressive neurological impairments, including seizures, visual problems/blindness, personality and behavior changes, dementia, and loss of the ability to walk, talk, and communicate, and;
- **Hemlibra (emicizumab)**, for the prevention of bleeding or to reduce the frequency of bleeding episodes in patients with **hemophilia A** who have developed antibodies called Factor VIII inhibitors. This is the first non-blood product approved for this condition.

Other 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 2017 novel drug field also includes these notable examples --- approved for the first time in the United States, and likely to significantly improve the care of patients with the diseases noted below:

A wide range of notable novel approvals in 2017 helps to enhance public health in the United States.

- **Aliqopa (copanlisib)** to treat adults with **relapsed follicular lymphoma** --- a slow-growing type of non-Hodgkin lymphoma (a cancer of the lymph system) --- who have received at least two prior systemic therapies;
- **Bavencio (avelumab)** for the treatment of patients 12 years and older with a rare and aggressive form of cancer called **metastatic Merkel cell carcinoma**, including those who have not received prior chemotherapy;
- **Benznidazole** to treat children ages 2 to 12 years with **Chagas disease**, a parasitic infection that can cause serious heart illness after years of infection, and can also affect swallowing and digestion. This is the first treatment approved in the United States for this rare disease;
- **Besponsa (inotuzumab ozogamicin)** for the treatment of adults with a type of cancer of the blood called **relapsed or refractory B-cell precursor acute lymphoblastic leukemia**;
- **Calquence (acalabrutinib)** to treat adults with **mantle cell lymphoma** who have received at least one prior therapy. Mantle cell lymphoma is a particularly aggressive cancer;
- **Emflaza (deflazacort)** to treat patients age 5 years and older with **Duchenne muscular dystrophy**, a rare genetic disorder that causes progressive muscle deterioration and weakness;
- **Giapreza (angiotensin II)**, for the treatment of **hypotension in adults with distributive or vasodilatory shock** (dangerously low blood pressure despite adequate heart function) whose blood pressure remains low despite receiving fluids and treatment with drugs called vasopressors;
- **Idhifa (enasidenib)** for the treatment of adults with **relapsed or refractory acute myeloid leukemia**, a form of blood cancer, who have a specific genetic mutation;
- **Ingrezza (valbenazine)** to treat adults with **tardive dyskinesia**, a side effect of some antipsychotic medications whereby patients can experience uncontrollable stiff, jerky movements of their face and body, and other uncontrolled movements such as eye-blinking, sticking out the tongue, and arm-waving;
- **Mavyret (glecaprevir and pibrentasvir)** to treat adults with chronic **hepatitis C virus** genotypes 1-6 without cirrhosis (liver disease) or with mild cirrhosis, including patients with moderate to severe kidney disease, as well as those who are on hemodialysis;
- **Mepsevii (vestronidase alfa-vjbk)** to treat patients with Sly syndrome or **mucopepolysaccharidosis type 7** – a rare genetic disorder where an enzyme deficiency results in skeletal abnormalities, developmental delay, enlarged liver and spleen, and narrowed airways, which can lead to respiratory infections;

- **Nerlynx (neratinib)** for the extended adjuvant treatment --- a form of therapy administered after an initial treatment to further lower the risk of the cancer coming back --- of early-stage, **human epidermal growth factor receptor 2 (HER2)-positive breast cancer**;
- **Prevymis (letermovir)** for prevention of an infection called **cytomegalovirus (CMV)** in patients who are receiving a bone marrow transplant. CMV disease can cause serious health issues in these patients;
- **Radicava (edaravone)** to treat patients with **amyotrophic lateral sclerosis**, commonly referred to as Lou Gehrig's disease, a rare disease that attacks and kills the nerve cells that control voluntary muscles;
- **Rydapt (midostaurin)** to treat adults newly diagnosed with a form of blood cancer known as **acute myeloid leukemia** who have a specific genetic mutation called FLT3, in combination with chemotherapy;
- **Siliq (brodalumab)** to treat adults with moderate-to-severe **plaque psoriasis**, a chronic disorder in which the body's immune system sends out faulty signals that speed growth of skin cells that then accumulate, causing red, flaky patches that can appear anywhere on the body;
- **Symproic (naldemedine)** for the treatment of **opioid-induced constipation** in adults with chronic non-cancer pain;
- **Tremfya (guselkumab)** for the treatment of adults with moderate-to-severe **plaque psoriasis**;
- **Trulance (plecanatide)** to treat adults with **chronic idiopathic constipation**, which is a persistent condition of constipation due to unknown origin;
- **Vabomere (vaborbactam and meropenem)** for treatment of adults with **complicated urinary tract infections**, including pyelonephritis (kidney infection) caused by bacteria;
- **Verzenio (abemaciclib)** to treat adults who have **hormone receptor (HR)-positive, HER2-negative advanced or metastatic breast cancer** that has progressed after taking therapy that alters a patient's hormones (endocrine therapy);
- **Vosevi (sofosbuvir/velpatasvir/voxilaprevir)** to treat adults with **chronic hepatitis C virus** genotypes 1-6 without cirrhosis (liver disease) or with mild cirrhosis;
- **Xadago (safinamide)** as an add-on treatment for patients with **Parkinson's disease** who are currently taking levodopa/carbidopa and experiencing "off" episodes;
- **Xermelo (telotristat ethyl)** combined with somatostatin analog (SSA) therapy to treat adults with **carcinoid syndrome diarrhea** that SSA therapy alone has inadequately controlled, and;
- **Zejula (niraparib)** for the maintenance treatment (intended to delay cancer growth) of adults with recurrent epithelial **ovarian, fallopian tube or primary peritoneal cancer**, whose tumors have completely or partially shrunk (complete or partial response, respectively) in response to platinum-based chemotherapy.

Novel drugs are often innovative products that serve previously unmet medical needs.

Innovation: Frequent Use of Expedited Development and Review Pathways

CDER used several regulatory pathways to expedite the development and approval of novel drugs in 2017. These pathways utilize a range of approaches that can enhance development efficiency and shorten timelines; these approaches can include 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. Eighteen of the 46 2017 novel drugs (39%) were designated by CDER as Fast Track. Fast Track speeds new drug development and review, for instance, 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: Aliqopa, Bavencio, Baxdela, Bevyxxa, Emflaza, Idhifa, Ingrezza, Mavyret, Mepsevii, Ocrevus, Prevyomis, Rydapt, Solosec, Vabomere, Verzenio, Vosevi, Xermelo, and Zejula.

Breakthrough Therapy

Breakthrough therapies are drugs with preliminary clinical evidence demonstrating that the drug may result in substantial improvement on at least one clinically significant endpoint (e.g., study result) over other available therapies for serious or life-threatening diseases for which there is unmet medical need. CDER designated 17 of the 2017 novel drugs (37%) 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 a potential new therapy.

Drugs designated with Breakthrough therapy status were: Alunbrig, Bavencio, Besponsa, Brineura, Calquence, Dupixent, Hemlibra, Imfinzi, Ingrezza, Kisqali, Mavyret, Ocrevus, Prevyomis, Rydapt, Verzenio, Vosevi, and Zejula.

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 within six months instead of the standard 10 months. Twenty-eight of the 46 novel drugs approved in 2017 (61%) 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: Aliqopa, Alunbrig, Bavencio, Baxdela*, benznidazole, Besponsa, Bevyxxa, Brineura, Calquence, Dupixent, Emflaza, Giapreza, Hemlibra, Idhifa, Imfinzi, Ingrezza, Kisqali, Mavyret, Mepsevii, Ocrevus, Prevyomis, Rydapt, Solosec*, Vabomere*, Verzenio, Vosevi, Xermelo, and Zejula.

* Baxdela, Solosec, and Vabomere received Priority Review as Qualified Infectious Disease Products (QIDPs) as authorized by the Generating Antibiotics Incentives Now Act (GAIN Act), which provides incentives to help bring new antibiotics and other antimicrobials to market. These products may or may not have otherwise received the *priority review* designation.

CDER used at least one expedited development and review method to speed approval for

61%

of all novel drugs approved in 2017.

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 that an important clinical benefit will be seen. Subsequent confirmatory trials must be conducted to support full approval. CDER approved six of the 2017 novel drugs (13%) under the Accelerated Approval program. The application of accelerated approval brings drugs that can provide important advances to patients sooner than with traditional approvals.

Novel drugs approved in 2017 that received the Accelerated Approval designation were: Aliqopa, Alunbrig, Bavencio, benznidazole, Calquence, and Imfinzi.

Overall Use of Expedited Development and Review Methods

Twenty-eight of the 2017 novel drug approvals (61%) were designated in one or more expedited categories of Fast Track, Breakthrough, Priority Review, and/or Accelerated Approval.

Novel drugs approved in 2017 using at least one expedited approval method were: Aliqopa, Alunbrig, Bavencio, Baxdela, benznidazole, Besponsa, Bevyxxa, Brineura, Calquence, Dupixent, Emflaza, Giapreza, Hemlibra, Idhifa, Imfinzi, Ingrezza, Kisqali, Mavyret, Mepsevii, Ocrevus, Prevymis, Rydapt, Solosec, Vabomere, Verzenio, Vosevi, Xermelo, and Zejula.

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 every PDUFA goal date for application review agreed to with the pharmaceutical industry and approved by Congress. In 2017, CDER met its PDUFA goal dates for 100% of the novel drugs approved (46 of 46).

CDER met its target date for

100%

of 2017's novel drug approvals.

Access: First Cycle Approval and Approvals Compared to Other Countries

First Cycle Approval

CDER approved 39 of the 46 novel drugs of 2017 (85%) on the “first cycle” of review, meaning without requests for additional information that would delay approval and lead to another cycle of review. From 2011 through 2016, CDER approved 204 novel drugs, of which 166 (81%) were approved on the first cycle. The rate for 2017 is consistent with this average. This high proportion of first cycle approval reflects the extensive discussions between CDER staff and drug developers that go on during drug development. This helps to assure that the application contains the information CDER needs to be able to fully review—and if appropriate—approve an application.

Novel drugs approved in 2017 on the first cycle were: Aliqopa, Alunbrig, Bavencio, Baxdela, benznidazole, Besponsa, Bevyxxa, Brineura, Calquence, Dupixent, Emflaza, Fasenra, Giapreza, Hemlibra, Idhifa, Imfinzi, Ingrezza, Kisqali, Mavyret, Mepsevii, Nerlynx, Ocrevus, Ozempic, Prevymis, Radicava, Rhopressa, Rydapt, Siliq, Solosec, Steglatro, Symproic, Tremfya, Trulance, Tymlos, Vabomere, Verzenio, Vosevi, Xermelo, and Zejula.

Approval in the United States Before Other Countries

Although regulatory processes differ widely between FDA and those of regulatory agencies in other countries, 36 of the 46 novel drugs approved in 2017 (78%) were approved in the United States before receiving approval in any other country.

Novel drugs of 2017 approved first in the United States were: Aliqopa, Alunbrig, Austedo, Bavencio, Baxdela, Bevyxxa, Brineura, Calquence, Dupixent, Fasenra, Giapreza, Hemlibra, Idhifa, Imfinzi, Ingrezza, Kisqali, Macrilen, Mavyret, Mepsevii, Nerlynx, Ocrevus, Ozempic, Prevymis, Rhopressa, Rydapt, Steglatro, Symproic, Tremfya, Trulance, Tymlos, Vabomere, Verzenio, Vosevi, Vyzulta, Xermelo, and Zejula.

78%

of the novel drugs approved in 2017 were approved first in the United States.

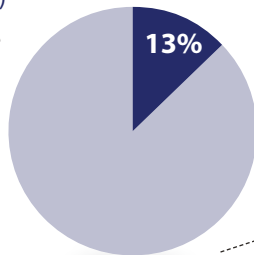
See [Appendix A](#) for a summary chart of designations for CDER’s novel drug approvals.

2017's Novel Drug Approvals

Expedited Review Pathway Usage

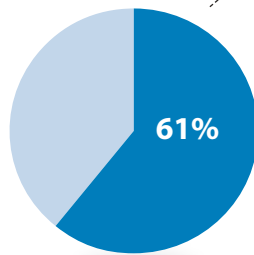
Accelerated Approval (6 of 46)

- Aliqopa
- Alunbrig
- Bavencio
- benznidazole
- Calquence
- Imfinzi



Priority Review (28 of 46)

- Aliqopa
- Alunbrig
- Bavencio
- Baxdela
- benznidazole
- Besponsa
- Bevyxxa
- Brineura
- Calquence
- Dupixent
- Emflaza
- Giapreza
- Hemlibra
- Idhifa
- Imfinzi
- Ingrezza
- Kisqali
- Mavyret
- Mepsevii
- Ocrevus
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- Solosec
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- Zejula

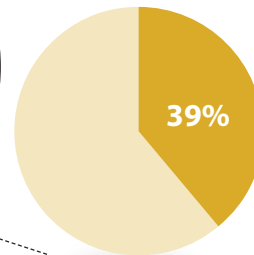


Used One or More Expedited Pathway (28 of 46)

- Aliqopa
- Alunbrig
- Bavencio
- Baxdela
- benznidazole
- Besponsa
- Bevyxxa
- Brineura
- Calquence
- Dupixent
- Emflaza
- Giapreza
- Hemlibra
- Idhifa
- Imfinzi
- Ingrezza
- Kisqali
- Mavyret
- Mepsevii
- Ocrevus
- Prevmis
- Rydapt
- Solosec
- Vabomere
- Verzenio
- Vosevi
- Xermelo
- Zejula

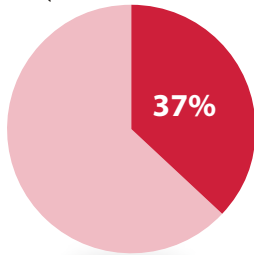
Fast Track (18 of 46)

- Aliqopa
- Bavencio
- Baxdela
- Bevyxxa
- Emflaza
- Idhifa
- Ingrezza
- Mavyret
- Mepsevii
- Ocrevus
- Prevmis
- Rydapt
- Solosec
- Vabomere
- Verzenio
- Vosevi
- Xermelo
- Zejula

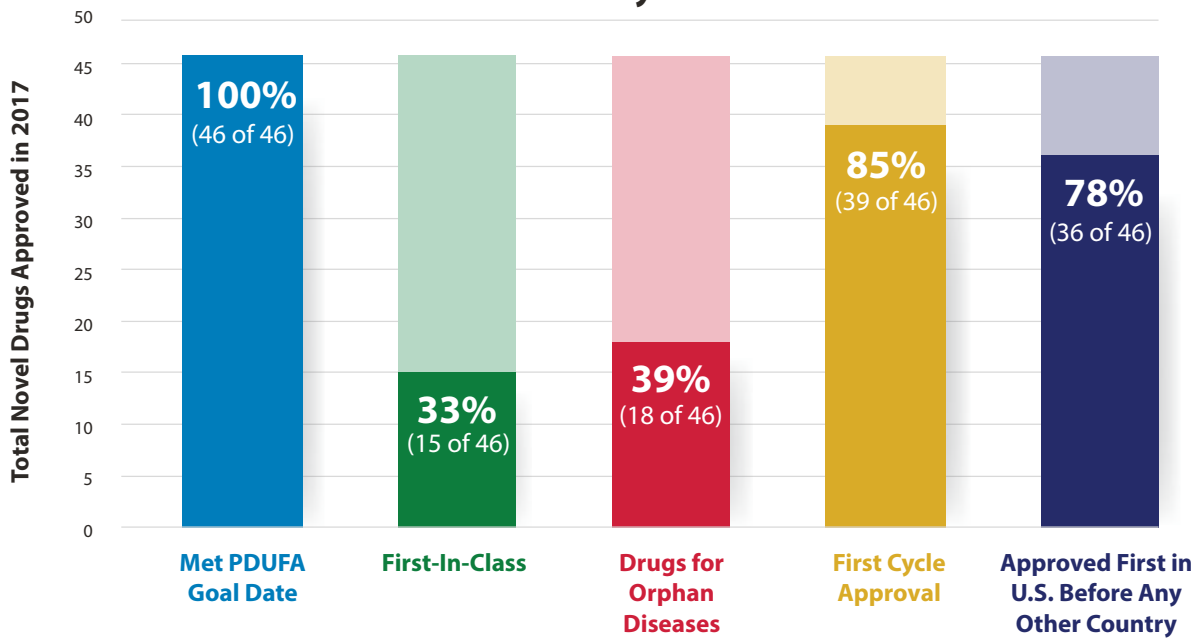


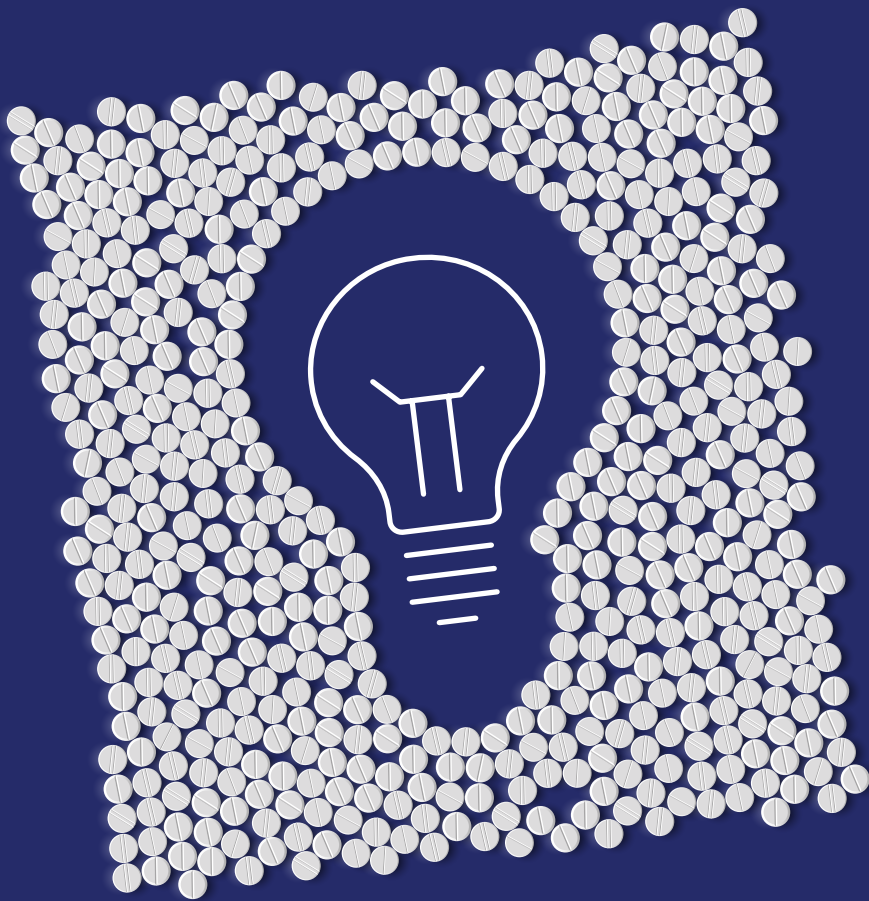
Breakthrough Therapy (17 of 46)

- Alunbrig
- Bavencio
- Besponsa
- Brineura
- Calquence
- Dupixent
- Imfinzi
- Hemlibra
- Ingrezza
- Kisqali
- Mavyret
- Ocrevus
- Prevmis
- Rydapt
- Verzenio
- Vosevi
- Zejula



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.

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 in a category of supplemental applications known as “efficacy supplements.”

New Uses

The products below are some notable approvals of 2017 for new uses of an already-FDA-approved drug:

- **Actemra (tocilizumab)**, originally approved in 2010 to treat patients with rheumatoid arthritis. It was approved in May 2017 for a new use to treat adults diagnosed with **giant cell arteritis**, a form of vasculitis, which is a group of disorders that results in inflammation of blood vessels. This is the first FDA-approved therapy specifically for this type of vasculitis. In August 2017, Actemra was also approved to treat patients 2 years of age and older with severe or life-threatening complications of **cytokine release syndrome**, a condition related to a reaction caused by a treatment called chimeric antigen receptor (CAR) T cell therapy;
- **Dysport (abobotulinumtoxinA)**, first approved by FDA in 2009 to treat adults with cervical dystonia (torticollis) and wrinkles between the eyebrows. It was approved in 2017 to treat **muscle spasticity** in adults;
- **Imbruvica (ibrutinib)**, originally approved in 2013 to treat patients with mantle cell lymphoma, a rare and aggressive blood cancer. In 2017, FDA approved the drug for treatment of adults with **chronic graft versus host disease (cGVHD)** after one or more treatments --- typically corticosteroids to suppress their immune system --- have failed. This was the first therapy approved by FDA specifically to treat cGVHD, a rare and life-threatening condition that can occur after an allogeneic (bone marrow or peripheral blood

cells) transplant. Also of note, in 2017, CDER expanded the approved use of Imbruvica as the first FDA-approved treatment for certain patients with a blood cancer called **marginal zone lymphoma**;

- **Nucala (mepolizumab)**, originally approved in 2015 for use with other asthma medicines for the maintenance treatment of asthma in patients age 12 years and older. It was approved in 2017 to treat patients with **eosinophilic granulomatosis with polyangiitis (EGPA)**, formerly known as Churg-Strauss, an extremely rare disease for which there are only about two to five new cases a year per one million people. EGPA is caused by inflammation (swelling) that occurs in certain types of cells in blood or in tissues, which causes injury to organ systems. The most commonly involved areas are the lungs, nose, sinuses, skin, joints, nerves, intestinal tract, heart, and kidneys. This is the first FDA-approved therapy specifically to treat EGPA;
- **Opdivo (nivolumab)**, originally approved in 2014 to treat patients with unresectable (cannot be removed by surgery) or metastatic (advanced) form of skin cancer called melanoma who no longer respond to other drugs. Since that approval, it has been approved for a variety of other uses related to cancer therapy. Notably, in 2017, Opdivo was approved for the treatment of certain patients with **hepatocellular carcinoma**, a type of liver cancer;
- **Keytruda (pembrolizumab)**, originally approved in 2014 to treat patients with advanced or unresectable melanoma who are no longer responding to other drugs. Throughout 2014-2017, it was approved for many new uses to treat patients with various forms of cancer. In May 2017, Keytruda was approved to treat patients whose cancers have a **specific genetic feature** (biomarker). This was the first time FDA has approved a cancer treatment based on a common biomarker rather than the location in the body where the tumor originated. In 2017, CDER also expanded its approved use to include treatment of patients with **refractory classical Hodgkin lymphoma**, a particularly difficult-to-treat type of Hodgkin lymphoma in which the cancer returns after treatment. In 2017, Keytruda was also approved to treat certain patients with recurrent, locally advanced or metastatic forms of stomach cancer called **gastric or gastro-esophageal junction adenocarcinoma**;
- **Revlimid (lenalidomide)**, originally approved in 2005 to treat certain patients who receive blood transfusions who have a form of anemia called transfusion dependent anemia. It was approved in 2017 as maintenance treatment for patients with **multiple myeloma** (cancer of blood plasma cells) following

2017 marked the
**first time FDA has approved
a cancer treatment
based on a common
biomarker**

rather than the location of the body
where the tumor originated.

a specific type of stem cell transplant intended to increase a patient's ability to produce new blood cells. This is the first FDA-approved maintenance treatment for multiple myeloma;

- **Soliris** (eculizumab), first approved in 2007 to prevent the breakdown of red blood cells in people with the rare disease known as paroxysmal nocturnal hemoglobinuria, and subsequently also approved in 2011 to treat patients with another rare disease called atypical hemolytic uremic syndrome, a chronic blood disorder. It was approved in 2017 to treat patients with **myasthenia gravis**, a rare neuromuscular disease;
- **Somatuline Depot** (lanreotide) injection, originally approved in 2007 to treat acromegalic patients --- those with excessive growth hormone in their body. In 2017, it was approved for the treatment of adults with **carcinoid syndrome** --- a neurochemical imbalance in the body caused by a cancerous tumor which can result in diarrhea and skin flushing. Patients taking the drug required fewer days on their rescue short-acting somatostatin analogs;
- **Stelara** (ustekinumab), first approved in 2009 to treat adults with plaque psoriasis. It was approved in 2017 for the treatment of adults with moderately to severely active **Crohn's disease** who have failed or were intolerant of other systemic treatments (see also Stelara under New Populations);
- **Sutent** (sunitinib malate), first approved in 2006 to treat certain patients with gastrointestinal stromal tumors and advanced renal cell carcinoma (both are forms of cancer). In 2017, it was approved for the new use of the adjuvant treatment of adults with **renal cell carcinoma**, a type of kidney cancer, who are at a high risk of the cancer returning after a kidney has been removed. Adjuvant treatment is a form of therapy that is taken after surgery to lower the risk of the cancer returning. This new approval fills an unmet medical need as it is the first adjuvant treatment approved for patients with renal cell carcinoma;
- **Stivarga** (regorafenib), originally approved in 2012 to treat patients with a certain form of colorectal cancer. It was approved for a new use in April 2017 to include treatment of patients with **hepatocellular carcinoma** (liver cancer) who have been previously treated with the drug sorafenib. This was the first new FDA-approved treatment for liver cancer in almost a decade;
- **Victoza** (liraglutide), originally approved in 2010 to treat patients with type 2 diabetes. In 2017, CDER expanded the approved use of the drug to include treatment for **reducing risks of heart attack, stroke and cardiovascular deaths in patients with type 2 diabetes**, and;

In 2017,
CDER approved the
**first treatment for
liver cancer in
almost a decade.**

Expanded approval can bring treatment options to a much larger population of patients, such as children with cystic fibrosis.

- **Zelboraf** (vemurafenib), first approved in 2011 to treat certain patients with melanoma whose malignant cells have a specific genetic mutation known as BRAF V600. In 2017 it was approved for the treatment of certain adults with **Erdheim-Chester Disease** (ECD), a rare cancer of the blood. Zelboraf is approved for this condition for patients whose malignant cells harbor the BRAF V600 mutation. This the first FDA-approved treatment for ECD.

New Populations

The products listed below are notable approvals in 2017 of an already-approved drug for use in an expanded population of patients:

- **Kalydeco** (ivacaftor), originally approved in 2012 to treat a narrow population of pediatric patients with **cystic fibrosis** (CF), was approved in 2017 to treat new populations of children with one of 23 additional rare mutations. Kalydeco is now indicated for 33 CF mutations, up from 10 previously;
- **Sovaldi** (sofosbuvir) and **Harvoni** (ledipasvir), first approved in 2013 and 2014 respectively, were approved only to treat adults infected with the **hepatitis C virus** (HCV). In 2017, both drugs were approved to also treat children with HCV, and;
- **Stelara** (ustekinumab), approved first in 2009 to treat adults with plaque psoriasis. It was approved in 2017 to treat adolescent patients 12 years or older, with moderate to severe **plaque psoriasis** --- only the second systemic biologic therapy approved for pediatric patients with psoriasis (see also Stelara in New Uses).

Additional Approvals

In addition to the many notable novel drug and efficacy supplement approvals of 2017, CDER also approved a variety of other therapies. Among these are **biosimilars** and drugs with **new formulations, manufacturers, combinations, or dosage forms** of already FDA-approved drugs, as well as others. Below discusses notable examples of these various types of approvals.

Biosimilars

An FDA-approved biosimilar 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 biological 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 consumer choice, and support greater access to important therapies.

In 2017, CDER approved five new biosimilars:

- **Cyltezo (adalimumab-adbm)**, biosimilar to Humira (adalimumab), approved for the treatment of patients with rheumatoid arthritis, juvenile idiopathic arthritis, ankylosing spondylitis, psoriatic arthritis, Crohn's disease, ulcerative colitis, and plaque psoriasis;
- **Ixifi (infliximab-qbtx)**, biosimilar to Remicade (infliximab), which can be used to treat patients with rheumatoid arthritis, psoriatic arthritis, ankylosing spondylitis, Crohn's disease, plaque psoriasis, and ulcerative colitis;
- **Mvasi (bevacizumab-awwb)**, biosimilar to Avastin (bevacizumab), used in the treatment of multiple types of cancer, including treatment for certain patients with metastatic colorectal cancer, non-squamous non-small cell lung cancer, glioblastoma, metastatic renal cell carcinoma, and cervical cancer;
- **Ogivri (trastuzumab-dkst)** biosimilar to Herceptin (trastuzumab), for the treatment of patients with breast or metastatic stomach cancer (gastric or gastroesophageal junction adenocarcinoma) whose tumors overexpress the HER2 gene, and;
- **Renflexis (infliximab-abda)**, also biosimilar to Remicade (infliximab) (see Ixifi above.).

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

Many of CDER's new drug approvals are not novel drugs, but still offer important medical value to patients in need.

The first biosimilar in the United States, **Zarxio** (filgrastim-sndz), was approved in 2015 as a biosimilar to the brand name product, Neupogen (filgrastim), which helps patients make white blood cells after being depleted by a cancer treatment. In 2016, CDER approved three more biosimilars: **Inflectra** (infliximab-dyyb), biosimilar to Remicade (see Ixifi and Renflexis above); **Erelzi** (etanercept-szss), biosimilar to Enbrel, which treats autoimmune diseases, such as rheumatoid arthritis, by interfering with a substance in the body called tumor necrosis factor; and **Amjevita** (adalimumab-atto) biosimilar to Humira (see Cyltezo above).

Multiple biosimilars for each FDA-approved reference product can make market competition even stronger. An increase in market competition may lead to significantly reduced costs for both patients and our healthcare system. The biopharmaceutical industry has shown great interest in developing these products, and CDER expects many more applications for biosimilars to be submitted. There are currently nine FDA-approved biosimilars, but there are many more in the development pipeline.

New Formulations

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.

Opioids with Properties Intended to Deter Abuse

CDER approved three new formulations of already approved opioid pain medications in 2017-- oxycodone, hydrocodone, and morphine sulfate. These new formulations have **properties that are intended to deter abuse of these highly addictive medications**. Although these products are formulated to make abuse more difficult, they cannot prevent abuse by all routes, nor are they expected to prevent or reduce addiction. They joined seven other products approved previously by FDA with properties intended to deter abuse. Given our nation's ongoing opioid epidemic, such new formulations have the potential to improve public health. These three new approvals for 2017 are:

- **Arymo ER (morphine sulfate extended-release tablets)**, designed with inactive ingredients that make the tablet more difficult to manipulate for misuse and abuse, and thereby deter intravenous abuse. Arymo ER is the third morphine sulfate product approved by CDER with properties designed to deter abuse --- following the 2009 approval of Embeda and the 2015 approval of Morphabond;

New opioid approvals of 2017 may help to reduce abuse and contribute to efforts to address our nation's opioid epidemic.

- **Roxybond (oxycodone immediate-release tablets)**, the first FDA-approved **immediate-release** opioid with properties designed to deter abuse by the intravenous and intranasal (snorting) routes. To date, CDER has approved ten opioid products with properties intended to deter abuse, but all except Roxybond are extended release/long acting (ER/LA) preparations, and;
- **Vantrela ER (hydrocodone extended-release tablets)**, formulated with properties intended to make the tablet more difficult to manipulate for misuse and abuse, and deter abuse by the oral, intranasal (snorting) and intravenous routes. Vantrela ER is the second ER/LA hydrocodone product with properties intended to deter abuse, after the 2014 approval of Hysingla (hydrocodone ER).

All approved products with properties intended to deter abuse must conduct post-approval epidemiologic studies to determine whether the abuse-deterrent properties are actually having an effect on the rates of abuse in the community.

Other New Formulations and Notable Approvals

Below includes **other notable new formulations** as well as other notable non-novel drug approvals of 2017, including, but not limited to, those with a **new combination of active ingredients** or a **new manufacturer** of an already FDA-approved drug.

- **Abilify MyCite (aripiprazole tablets with sensor)**. Abilify was first approved by FDA in 2002, in tablet form, to treat patients with schizophrenia. It was subsequently approved to treat certain patients with bipolar I disorder and as adjunctive treatment of major depressive disorder. Generic versions have also since been approved and aripiprazole is available in a variety of dosage forms: tablet, oral solution, and injectable, including extended release injections. In 2017, Abilify MyCite was approved as a tablet that contains an **electronic sensor** that allows the patient to track whether he or she has taken the medication via a smartphone or the cloud. Patients can also allow their caregivers or physician to access the information through a web-based portal.
- **Admelog (insulin lispro injection)**, a short-acting insulin to help control blood glucose in patients 3 years and older with **type 1 diabetes mellitus** and adults with **type 2 diabetes mellitus**. It was approved through an abbreviated pathway known as (505)(b)(2), which allows



In 2017, CDER approved a tablet with an electronic sensor that allows the patient to track whether he or she has taken the medication.

In 2017, CDER approved the first treatment for patients with sickle cell disease in almost 20 years.

a company to rely on FDA's previous finding of safety and effectiveness of already approved products if the company has provided evidence that its product will not differ substantially from the approved product. This regulatory pathway may enable approval of therapies in shorter amounts of time to help increase consumer choice, create market competition, and possibly decrease cost. This approval relied, in part, on comparison to the already FDA-approved product, Humalog (insulin lispro injection). Admelog is the first insulin lispro product and the second insulin product approved via the 505(b)(2) pathway, following the 2016 approval of Basalglar (insulin glargine), approved based on comparison to the already FDA-approved product, Lantus (insulin glargine).

- **Endari (L-glutamine oral powder)**, the first treatment approved for patients with **sickle cell disease** in almost 20 years. Prior to this approval, L-glutamine was approved as a prescription drug in 2004 to treat certain patients with short bowel syndrome. It has also been marketed as a dietary supplement and used for treatments that include digestive issues (e.g., stomach ulcers, ulcerative colitis, Crohn's disease), depression, moodiness, irritability, anxiety, insomnia, and enhancing exercise performance;
- **Juluca (rilpivirine and dolutegravir)**, a combination of two FDA-approved drugs, each used in the treatment of certain patients with HIV. It was approved in 2017 as a complete regimen for the treatment of certain adults with **HIV-1 infection**. This is the first complete regimen containing only two HIV-1 drugs and neither is a nucleoside reverse transcriptase inhibitor (NRTI). Long-term NRTI use is associated with some degree of kidney, bone, and cardiovascular toxicities;
- **Mylotarg (gemtuzumab ozogamicin)**, approved in 2017 for the treatment of adults with newly diagnosed **acute myeloid leukemia** whose tumors express the CD33 antigen (CD33-positive AML) and the treatment of patients aged 2 years and older with CD33-positive AML who have experienced a relapse or who have not responded to initial treatment (refractory). Mylotarg originally received accelerated approval in May 2000 as a stand-alone treatment for older patients with CD33-positive AML who had experienced a relapse, but was voluntarily withdrawn from the market after subsequent confirmatory trials failed to verify clinical benefit and demonstrated safety concerns, including a high number of early deaths. This new approval in 2017 was based on

careful review of a new dosing regimen, which has shown that the benefits of this treatment outweigh the risk;

- **Noctiva (desmopressin)**, the first approval of desmopressin to treat adults who wake up at least twice a night to urinate because they overproduce urine, a condition called **nocturnal polyuria**. Desmopressin was previously approved by FDA for a different manufacturer to treat diabetes insipidus and a variety of blood disorders;
- **Rituxan Hycela (rituximab and hyaluronidase human)**, a new combination of two already FDA-approved drugs. It was approved in 2017 for the treatment of certain patients with **various forms of lymphoma** (a type of blood cancer). A dose of Rituxan Hycela can be administered in just a few minutes compared to hours for a similar product, Rituxan (rituximab), originally approved in 1997;
- **Sublocade (buprenorphine)**, the first once-monthly injectable buprenorphine product to treat certain patients with **moderate-to-severe opioid use disorder**. It is injected by a healthcare professional under the patient's skin in the abdominal region and provides sustained plasma levels of buprenorphine over the dosing interval. Buprenorphine was originally approved in 1981 and has been used in a variety of other formulations to help patients fighting opioid addiction;
- **Symjepi (epinephrine)**, a product approved for a new manufacturer that enables a patient to self-inject epinephrine to counteract a dangerous **allergic reaction**. There are other types of epinephrine products that are intended for this use. This new product adds another choice for patients and increases competition;
- **Tepadina (thiotepa)**, a new formulation of the drug thiotepa, a product approved by FDA since 1959 to treat patients with a variety of cancers. Tepadina was approved in 2017 for **reducing the risk of graft rejection** when used in conjunction with certain other drugs to prepare for a stem cell transplant. It is used to increase red blood cell production, and;
- **Vyxeos (cytarabine and daunorubicin)**, a combination of two already FDA-approved drugs, approved for the treatment of adults with two types of **acute myeloid leukemia (AML)**: newly-diagnosed therapy-related t-AML or AML with myelodysplasia-related changes. This is the first approved treatment specifically for patients with certain types of high-risk AML.

In 2017, CDER approved the first once-monthly injectable buprenorphine product to help patients fighting opioid addiction.

Approval for a new manufacturer of self-injectable epinephrine adds another choice for patients and increases competition.

New dosage forms can ensure a proper dose, help patients adhere to treatment, and improve quality of life.

As with all FDA-approved products, the new drug therapies approved by CDER discussed in this report are associated with risks. **For more information about these drugs and for complete risk information, see the drugs' approval letters and FDA-approved labeling at Drugs@FDA.**

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:

- **Esbriet (pirfenidone)** 534 mg. and 801 mg. tablets, new dosage forms of a 267 mg. capsule originally approved in 2014 as the first new drug in the United States to treat patients with **idiopathic pulmonary fibrosis**. A typical dose of the originally approved product is two or three 267 mg. capsules three times daily. The higher strength tablets enable taking fewer tablets daily;
- **Gocovri (amantadine extended release capsule)**, approved by FDA in 2017 as a new dosage form of the already FDA-approved drug amantadine, which has long been approved to treat and prevent respiratory infections caused by the influenza A virus, as well as to treat symptoms of Parkinson's disease and similar conditions, such as stiffness and shaking. Gocovri is specifically approved for the treatment of **dyskinesia** in certain patients with Parkinson's disease. It is the only FDA-approved medication for this use. Dyskinesia is a category of movement disorders that are characterized by involuntary muscle movements and diminished voluntary movements;
- **Mydayis (mixed salts of a single-entity amphetamine)**, a new amphetamine dosage form designed to enable once-daily dosing and provide all day control for **attention deficit hyperactivity disorder**;
- **Norvir (ritonavir)** oral powder, a new dosage form of the tablet and oral solution forms of Norvir, an HIV protease inhibitor approved in 1996 to be used in combination with other antiretroviral agents for the treatment of **HIV-1 infection**. The oral powder formulation can be mixed in soft foods and does not contain ethanol or propylene glycol and therefore may be a more palatable and safer alternative for children.



Ensuring Access to CDER-Regulated Products in an Emergency

CDER's drug therapy approvals discussed above were all conducted under carefully planned and executed timelines --- with a goal of bringing safe and effective new therapies to the American public as soon as possible. But, when emergencies strike, CDER is flexible with our use of enforcement discretion to help ensure access to all needed medications for the protection of public health.

Hurricane Season 2017 caused widespread devastation to states along the Gulf Coast, U.S. Caribbean island territories, and Caribbean island nations. Well before Hurricanes Harvey, Irma, Jose, and Maria made landfall, FDA was preparing for the impact on FDA-regulated products by assessing types and locations of facilities in the paths of these hurricanes and the effects of potential product shortages.

When Hurricane Maria struck Puerto Rico, the storm caused a significant loss of manufacturing capability for many CDER-regulated drug products made on the island. CDER staff worked tirelessly to help these manufacturers mitigate potential drug shortages, using expedited reviews and enforcement discretion for product importation, which helped facilitate availability of needed medications.

CDER's responses during hurricane season of 2017 underscore the importance of emergency access to FDA-approved medical products to protect public health.

Sodium chloride-based and glucose-based solutions for intravenous infusion are critical for mixing drugs appropriately for use in hospitals and medical clinics. A major manufacturer of these solutions, Baxter Healthcare Corporation, lost much of its production capability for these products after Maria struck. In response, CDER expedited review of information on other Baxter manufacturing sites around the world, and concluded that unapproved Baxter solutions could be imported and distributed under enforcement discretion. CDER also allowed certain waivers for approved drugs made by another company.

These steps helped to maintain supplies of important products for treatment of serious conditions, such as cancers and autoimmune diseases, and for prevention of organ rejection in kidney transplant patients. Hurricane damage interrupted supplies of medical oxygen and nitrogen, prompting CDER's ongoing efforts with several companies to help maintain supply.

Until generators could be turned on, the loss of electricity at manufacturing plants in Puerto Rico exposed some drug products to temperatures and humidity outside of their approved storage conditions. Through interactions with certain companies, CDER concluded that their manufacturing can resume in Puerto Rico when power is restored or at other manufacturing sites. Although the products made at locations experiencing power outages are not in shortage, CDER will review plans for determining if product quality was affected.

CDER's efforts after the hurricane damage helped to maintain supplies of important products for treatment of serious conditions.



External Engagement

CDER collaborates with a wide variety of stakeholders including medical and scientific organizations and patient advocacy groups, as well as patients, and caregivers, to support innovation and advance public health.

Medical and Scientific Engagement

CDER's work with other government agencies, global organizations, academia, industry, and patient advocacy groups helps to develop and advance the science needed to bring innovative new drug therapies to the marketplace. CDER often facilitates the creation of partnerships and collaborations, and advises on specific scientific projects. Through FDA's [Critical Path Initiative \(CPI\)](#), CDER has initiated scores of scientific collaborations including the following:

- **Improving drug testing by advancing the science of clinical trials**, such as our work with:
 - [The Clinical Trials Transformation Initiative \(CTTI\)](#);
- **Enhancing the scientific techniques used to evaluate a drug's safety and efficacy**, such as our work with:
 - [The Biomarkers Consortium \(BC\)](#), and;
 - [The Global Pediatric Clinical Trials Network Pre-Launch Consortium \(PTC\)](#).

- **Studying specific diseases and conditions where new therapies are particularly important for public health**, such as our work with:
 - [The Accelerating Medicines Partnership \(AMP\)](#)
 - [The Analgesic Clinical Trial Translations, Innovations Opportunities and Networks Initiative \(ACTION\)](#)
 - [The Coalition Against Major Disease Consortium \(CAMD\)](#)
 - [The Critical Path for Parkinson's \(CPP\)](#)
 - [The Critical Path to TB Drug Regimens Consortium \(CPTR\)](#)
 - [The Duchenne Regulatory Science Consortium \(D-RSC\)](#)
 - [The International Neonatal Consortium](#)
 - [Kidney Health Initiative \(KHI\)](#)
 - [The Multiple Sclerosis Outcome Assessments Consortium \(MSOAC\)](#), and
 - [SmartTots](#).
- **Developing common methods or standards, so scientists can better work together**, such as our work with:
 - [The Coalition For Accelerating Standards and Therapies \(CFAST\)](#).
- **Improving drug development by predicting safety earlier in product development**, such as our work with:
 - [The Cardiac Safety Research Consortium](#)
 - [The HESI Cardiac Safety Technical Committee](#), and
 - [The Predictive Safety Testing Consortium \(PSTC\)](#).
- **Developing technology to assure quality in manufacturing and the drug supply chain**, such as our work with:
 - [The National Institute for Pharmaceutical Technology and Education \(NIPTE\)](#)
 - [The Product Quality Research Institute \(PQRI\)](#), and
 - [RX-360](#).
- **Gaining information from the patient perspective**, such as our work with:
 - [The Patient Reported Outcome Consortium \(PRO\)](#), and
 - [Electronic Patient-Reported Outcome \(ePRO\) Consortium](#).

CDER also regularly engages with stakeholders via Critical Path Innovation Meetings to address issues in drug development. These engagements spur development of tools such as animal- or computer-based predictive models, biomarkers for safety and effectiveness, and new clinical methods.

These meetings have become a valuable tool by which CDER, industry, academia, patient advocacy groups, and other government entities can communicate early in the drug development process to improve efficiency and success.

For more information on how CDER uses science to advance innovation, please see [Regulatory Science in Action](#) on the FDA website.

The Voice of the Patient

CDER's external engagement also extends to our most important stakeholders --- patients. Drug development and FDA's review process benefit when we can systematically obtain patients' experiences, perspectives, needs, and priorities about their condition and available treatment options.

People living with a condition are uniquely positioned to inform our understanding of the therapeutic context for drug development and evaluation. Therefore, FDA is increasingly involving the patient in many of our regulatory activities and decision-making processes.

Since 2013, our Patient Focused Drug Development (PFDD) initiative has been helping FDA to better understand the needs of patients and how they think and feel about new drug development. During this time, we have conducted 24 [meetings](#) with patients, caregivers, and patient advocacy groups.

The meetings have focused on disease areas for which there is a particular need, in the context of drug development and evaluation, to better understand patient perspective on their condition and treatments. In 2017, we conducted meetings to engage patients with [sarcopenia](#), [autism](#), [alopecia areata](#), and [hereditary angioedema](#).

For each meeting, we publish a detailed [Voice of the Patient](#) report, summarizing what we have learned. Completion of these meetings was part of FDA's commitment under the [fifth authorization of the Prescription Drug User Fee Act](#) (PDUFA V).

We recognize that there are many more disease areas to address. To help expand the benefits of FDA's PFDD initiative, FDA encourages patient organizations to identify and organize patient-focused collaborations to generate public input on other disease areas, using the process established through PFDD as a model. FDA recommends that patient organizations who are interested in conducting an [externally-led PFDD meeting](#) send FDA a Letter of Intent.

As we work to enhance patient-focused drug development and regulatory decision-making, CDER recognizes the need for systematic, methodologically sound approaches to collecting patient input in such a way that the data can further inform regulatory decision-making. An important step is to provide [guidance](#) to patient communities, researchers, and drug developers on pragmatic and sound strategies, pathways, and methods to gather and use patient input.

Patient involvement has become an integral part of the drug development process.



Conclusion

CDER's staff consists of individuals with a range of different areas of expertise, including physicians, safety evaluators, chemists, biologists, biostatisticians, nurses, pharmacists, pharmacologists, epidemiologists, legal and regulatory experts, and many more --- working 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 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. Further, as illustrated by our response during the hurricane season of 2017, we expedited reviews and used importation under enforcement discretion to facilitate access to needed medications.

Although not a comprehensive compilation of all 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.

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

Our drug therapy approvals of 2017 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 efficient and innovative approaches for evaluating the safety and efficacy of the drug therapies that industry will develop with these new 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 has become an important component of this 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.

Of note, CDER works to assure that all of our approvals provide value to the American public.

This report is not intended to be a comprehensive compilation of all of CDER's approvals, but rather to offer a wide variety of valuable examples of the many ways CDER acts to enhance the health of the American people.

Appendix A: Drug Designation Summary

Approval Date	Trade Name	Active Ingredient	Designation									Dosage Form		
			First-in-Class	Orphan	Fast Track	Breakthrough	Priority	Accelerated Approval	Met PDUFA Goal	First Cycle Approval	First in the United States			
1/19/2017	Trulance	plecanatide												Tablet
2/7/2017	Parsabiv	etelcalcetide												Injection
2/9/2017	Emflaza	deflazacort												Tablet, Oral Solution
2/15/2017	Siliq	brodalumab												Injection
2/28/2017	Xermelo	telotristat ethyl												Tablet
3/13/2017	Kisqali	ribociclib												Tablet
3/21/2017	Xadago	safinamide												Tablet
3/23/2017	Bavencio	avelumab												Injection
3/23/2017	Symproic	naldemedine												Tablet
3/27/2017	Zejula	niraparib												Capsule
3/28/2017	Dupixent	dupilumab												Injection
3/28/2017	Ocrevus	ocrelizumab												Injection
4/3/2017	Austedo	deutetrabenazine												Tablet
4/11/2017	Ingrezza	valbenazine tosylate												Capsule
4/27/2017	Brineura	cerliponase alfa												Injection
4/28/2017	Alunbrig	brigatinib												Tablet
4/28/2017	Rydapt	midostaurin												Capsule
4/28/2017	Tymlos	abaloparatide												Injection
5/1/2017	Imfinzi	durvalumab												Injection
5/5/2017	Radicava	edaravone												Injection
5/22/2017	Kevzara	sarilumab												Injection
6/19/2017	Baxdela	delafloxacin												Tablet, Injection
6/23/2017	Bevyxxa	betrixaban												Capsule

Approval Date	Trade Name	Active Ingredient										Dosage Form			
			First-in-Class	Orphan	Fast Track	Breakthrough	Priority	Accelerated Approval	Met PDUFA Goal	First Cycle Approval	First in the United States				
7/13/2017	Tremfya	guselkumab												Injection	
7/17/2017	Nerlynx	neratinib maleate													Tablet
7/18/2017	Vosevi	voxilaprevir; velpatasvir; sofosbuvir													Tablet
8/1/2017	Idhifa	enasidenib													Tablet
8/3/2017	Mavyret	glecaprevir; pibrentasvir													Tablet
8/17/2017	Besponsa	inotuzumab ozogamicin													Injection
8/29/2017	benznidazole	benznidazole													Tablet
8/29/2017	Vabomere	vaborbactam; meropenem													Injection
9/14/2017	Aliqopa	copanlisib hydrochloride													Injection
9/15/2017	Solosec	secnidazole													Granules
9/28/2017	Verzenio	abemaciclib													Tablet
10/31/2017	Calquence	acalabrutinib													Capsule
11/2/2017	Vyzulta	latanoprostene bunod													Ophthalmic Solution
11/8/2017	Prevymis	letermovir													Tablet
11/14/2017	Fasenra	benralizumab													Injection
11/15/2017	Mepsevii	vestronidase alfa-vjbc													Injection
11/16/2017	Hemlibra	emicizumab													Injection
12/5/2017	Ozempic	semaglutide													Injection
12/11/2017	Xepi	ozenoxacin													Cream
12/18/2017	Rhopressa	netarsudil													Ophthalmic Solution
12/19/2017	Steglatro	ertugliflozin													Tablet
12/20/2017	Macrilen	macimorelin acetate													Oral Solution
12/21/2017	Giapreza	angiotensin II													Injection

