Center for Drug Evaluation and Research
Food and Drug Administration
U.S. Department of Health and Human Services

IMPACT • INNOVATION • PREDICTABILITY • ACCESS

2011 NOVEL NEW DRUGS

JANUARY, 2012

CDER's 2011 NMEs

30 novel new drugs approved in 2011

In Calendar Year 2011, FDA's Center for Drug Evaluation and Research (CDER) approved 30 novel new medicines, known as new molecular entities (NMEs).* This includes applications for both New Drug Applications (NDAs) and Biologics License Applications (BLAs).

The clear bars in the chart to the right indicate the number of NMEs approved by CDER in each year since 2002. CDER approved 30 NMEs in 2011, representing the second highest total in the past ten years, except 2004, in which the Center approved 36 NMEs. In terms of recent comparison, for the ten-year period from 2002 through 2011, CDER has averaged about 24 NME approvals per year.

Novel Drug Approvals Remain Steady

The number of NMEs approved over time, however, has not been substantially increasing. This is due primarily to the fact that the number of applications for NMEs has also not been increasing.

The red dots in the graph to the right indicate the number of new NDA and BLA applications CDER has received over the last ten years for NMEs. From 2002 through 2010 CDER filed an average of about 30 applications for NMEs per year. Although all applications submitted in 2011 were not accepted for filing as of 12/31/11, CDER projects about 39 for 2011, roughly on par with numbers from recent years such as 35 in 2007, 34 in 2008, and 36 in 2009.

In 2011 CDER approved **30 NMEs***

Since 2002 CDER has averaged **about 24 NME** approvals per year.

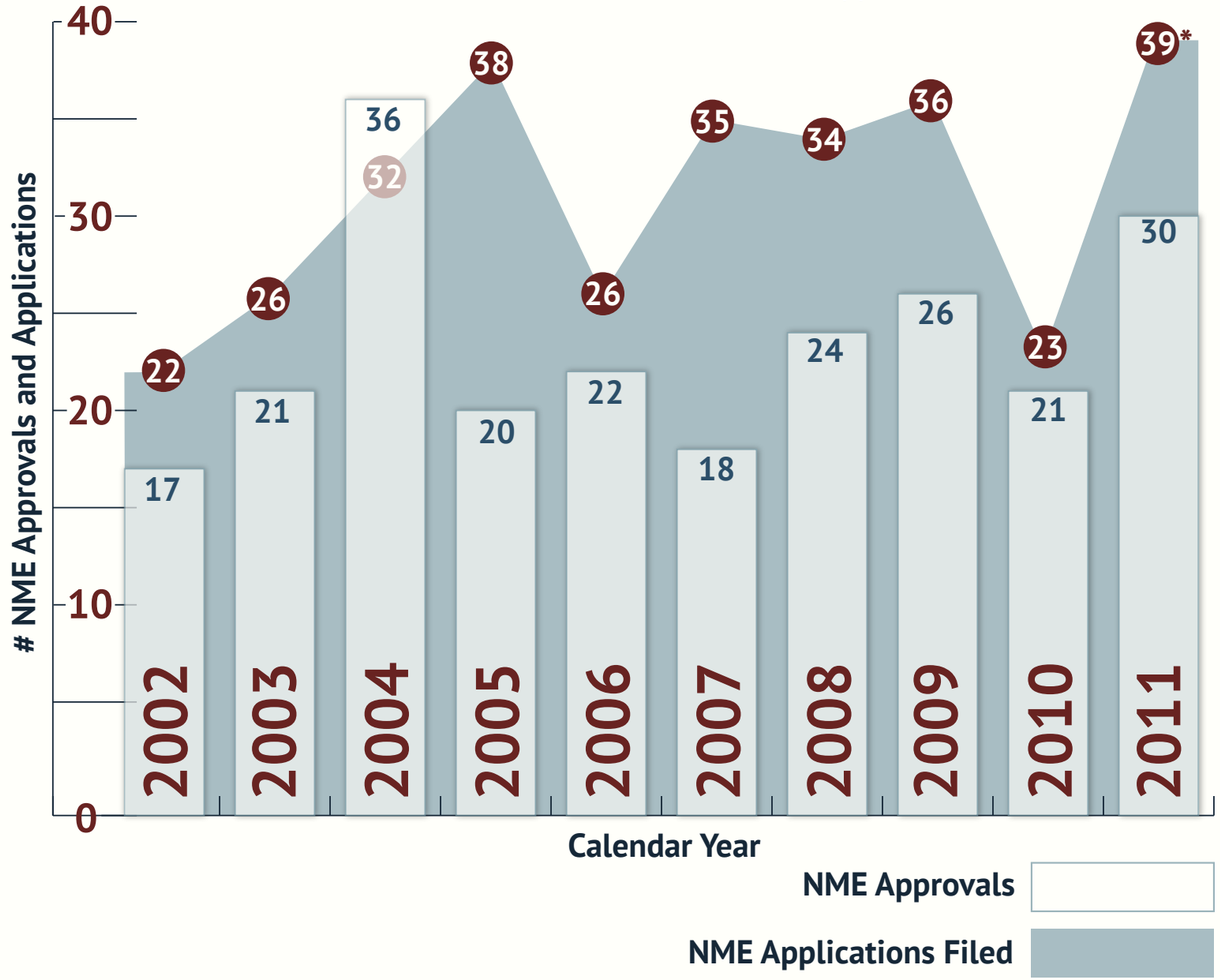
The NMEs of 2011: see pages 14 & 15 for what these drugs are used for.									
Adcetris	Arcapta	Benlysta	Brilinta	Caprelsa	Daliresp	Datscan	Dificid	Edarbi	Edurant
Eylea	Erwinaze	Ferriprox	Firazyr	Gadavist	Horizant	Incivik	Jakafi	Natroba	Nulojix
Onfi	Potiga	Tradjenta	Victrelis	Viibryd	Xalkori	Xarelto	Yervoy	Zelboraf	Zytiga

*For more details about the individual NMEs, see pages 14 & 15.

Ten Year Historic Comparison

The 30 NMEs approved in 2011 is the **highest total** in seven years.

From 2002 through 2010, CDER filed an average of 30 applications for NMEs.



*The final number of NME Applications filed in 2011 is projected, pending final validation of the data and dependent outcome of 12 applications submitted in late 2011.

For more details about the individual NMEs, see pages 14 & 15.

IMPACT

Impact on Public Health

Although the 30 new molecular entities approved by FDA's Center for Drug Evaluation and Research (CDER) in 2011 represent the most since 2004, these NMEs are more notable for their potential positive impact on quality care and public health.

First-in-Class Drugs

Twelve of the 30 NMEs approved in 2011 (40%) were identified as **First-in-Class**, meaning drugs which, for example, use a new and unique mechanism of action for treating a medical condition.

- Adcetris
- Benlysta
- Daliresp
- Firazyr
- Jakafi
- Nulojix
- Potiga
- Victrelis
- Xalkori
- Yervoy
- Zelboraf
- Zytiga

Particularly noteworthy first-in-class products include Benlysta, the first new drug approved to treat lupus in over 50 years, Adcetris, the first new drug to treat Hodgkin Lymphoma in over 30 years, and Yervoy, the first drug to clearly demonstrate extending life for patients with late stage melanoma.

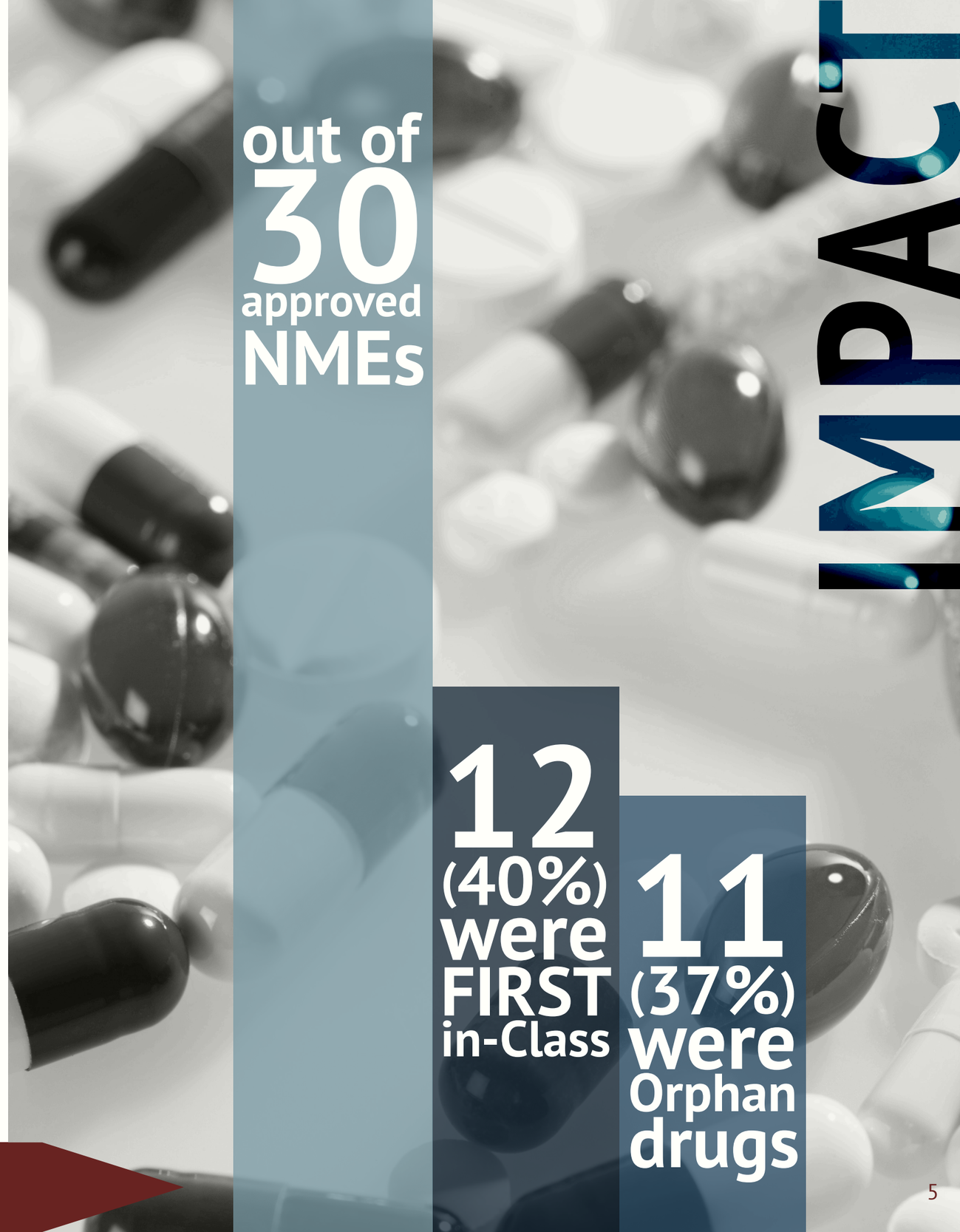
Orphan Drugs

Eleven of the 30 NMEs of 2011 (37%) were new drugs approved to treat rare or "**orphan**" diseases that affect 200,000 or fewer Americans. This is significant because patients with rare diseases often have few or no drug treatment options.

- Adcetris
- Caprelsa
- Erwinaze
- Ferriprox
- Firazyr
- Jakafi
- Nulojix
- Onfi
- Xalkori
- Yervoy
- Zelboraf

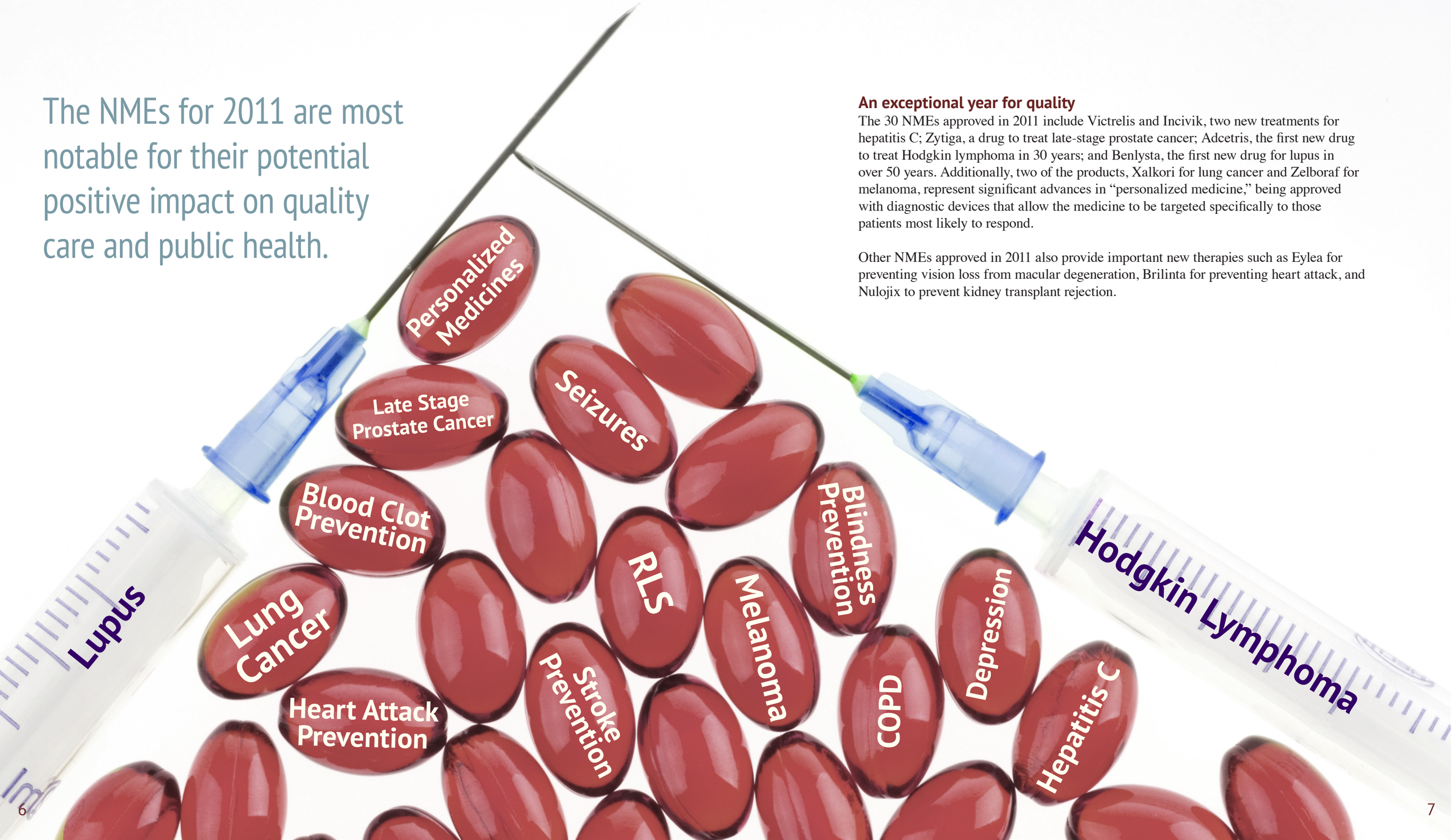
Examples of rare diseases that now have new effective treatment options include myelofibrosis (Jakafi), hereditary angioedema (Firazyr), and Lennox-Gastaut syndrome (Onfi).

For more details about the individual NMEs, see pages 14 & 15.



Impact on Public Health

The NMEs for 2011 are most notable for their potential positive impact on quality care and public health.



An exceptional year for quality

The 30 NMEs approved in 2011 include Victrelis and Incivik, two new treatments for hepatitis C; Zytiga, a drug to treat late-stage prostate cancer; Adcetris, the first new drug to treat Hodgkin lymphoma in 30 years; and Benlysta, the first new drug for lupus in over 50 years. Additionally, two of the products, Xalkori for lung cancer and Zelboraf for melanoma, represent significant advances in "personalized medicine," being approved with diagnostic devices that allow the medicine to be targeted specifically to those patients most likely to respond.

Other NMEs approved in 2011 also provide important new therapies such as Eylea for preventing vision loss from macular degeneration, Brilinta for preventing heart attack, and Nulojix to prevent kidney transplant rejection.

INNOVATION

Innovative methods for expediting NMEs to market

Many of the 30 NMEs of 2011 approved by CDER are notable for the regulatory methods CDER used to expedite the approval process. Particularly noteworthy examples of drugs approved rapidly are Zytiga (prostate cancer), approved in 4.2 months, Zelboraf (melanoma), approved in 3.6 months, Xalkori (lung cancer), approved in 4.9 months, and Adcetris (two types of lymphoma), approved in 5.7 months.

Priority Review

Fifteen of the 30 NMEs were designated **Priority Review** (50%), in which CDER determines the drug to potentially provide a significant advance in medical care and sets a target to review the drug within six months instead of the standard 10 months.

- Adcetris
- Benlysta
- Caprelsa
- Datscan
- Dificid
- Erwinaze
- Eylea
- Firazyr
- Incivek
- Jakafi
- Victrelis
- Xalkori
- Yervoy
- Zelboraf
- Zytiga

Fast Track

Fourteen of the 30 NMEs approved in 2011 (47%) were designated **Fast Track** approvals, meaning drugs that can treat unmet medical needs; this is the highest number of Fast Track approvals since the program began in 1998. Fast Track speeds new drug reviews; for instance, by increasing the level of communication FDA allocates to developers and by enabling developers to use a "rolling review" process such that CDER can review portions of an application ahead of the submission of the full application.

- Adcetris
- Benlysta
- Caprelsa
- Dificid
- Erwinaze
- Ferriprox
- Firazyr
- Incivek
- Jakafi
- Nulojix
- Victrelis
- Xalkori
- Yervoy
- Zelboraf

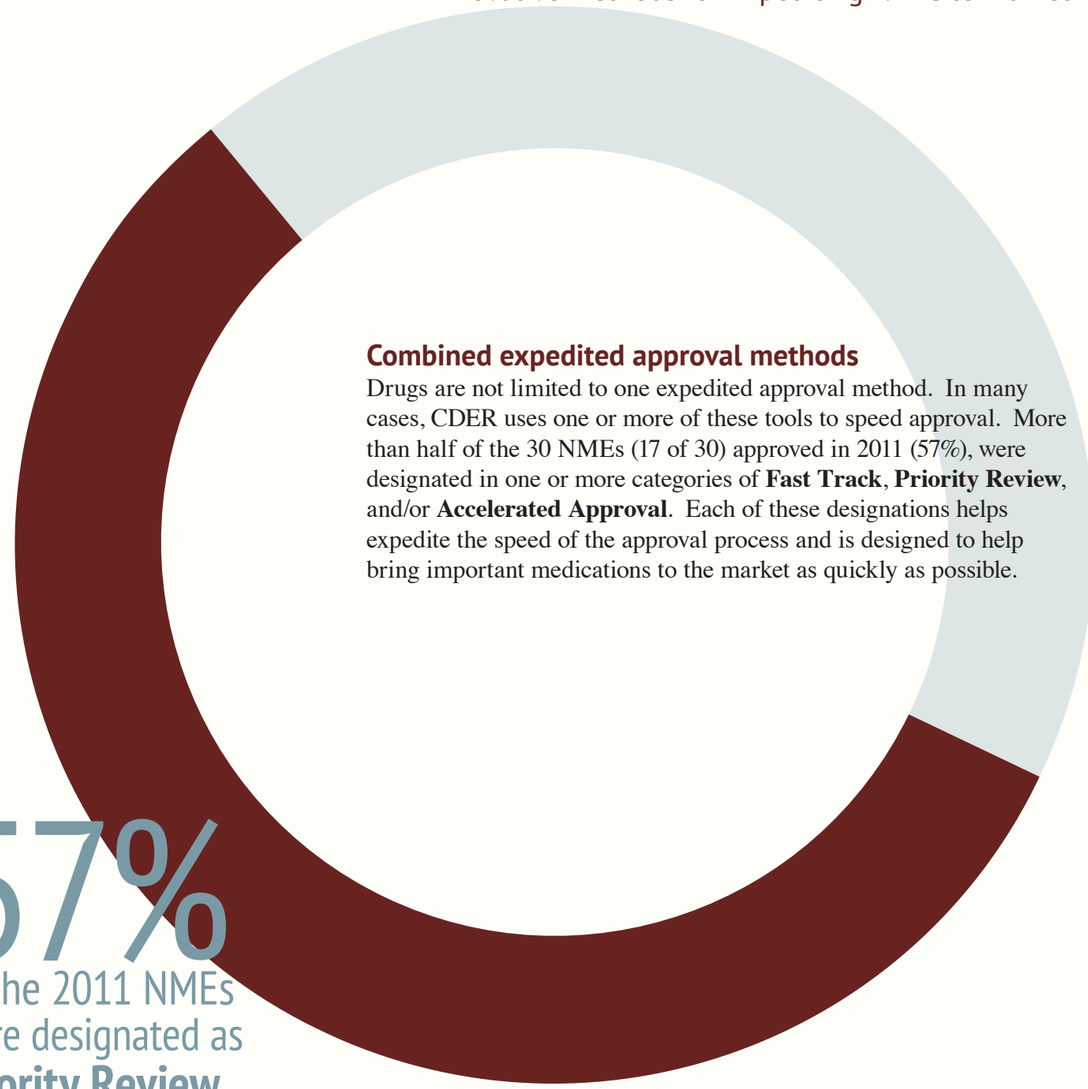
Accelerated Approval

Three of the 30 NMEs approved in 2011 (10%) were approved under FDA's **Accelerated Approval** program, which allows early approval of a drug for serious or life-threatening illness that offers a benefit over current treatments. This approval is based on a "surrogate endpoint" (e.g., a laboratory measure) or other clinical measure that FDA considers reasonably likely to predict clinical benefit. After this approval, the drug must undergo additional testing to confirm that benefit-this speeds the availability of the drug.

- Adcetris
- Ferriprox
- Xalkori

For more details about the individual NMEs, see pages 14 & 15.

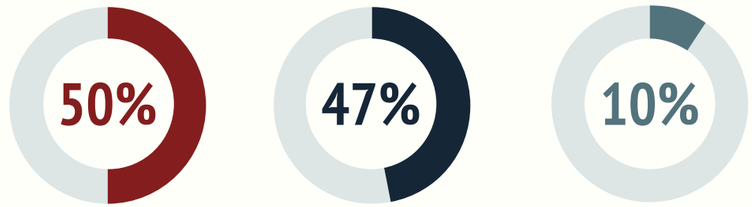
Innovative Methods for Expediting NMEs to market



Combined expedited approval methods

Drugs are not limited to one expedited approval method. In many cases, CDER uses one or more of these tools to speed approval. More than half of the 30 NMEs (17 of 30) approved in 2011 (57%), were designated in one or more categories of **Fast Track**, **Priority Review**, and/or **Accelerated Approval**. Each of these designations helps expedite the speed of the approval process and is designed to help bring important medications to the market as quickly as possible.

57% of the 2011 NMEs were designated as **Priority Review, Fast Track, or Accelerated Approval**



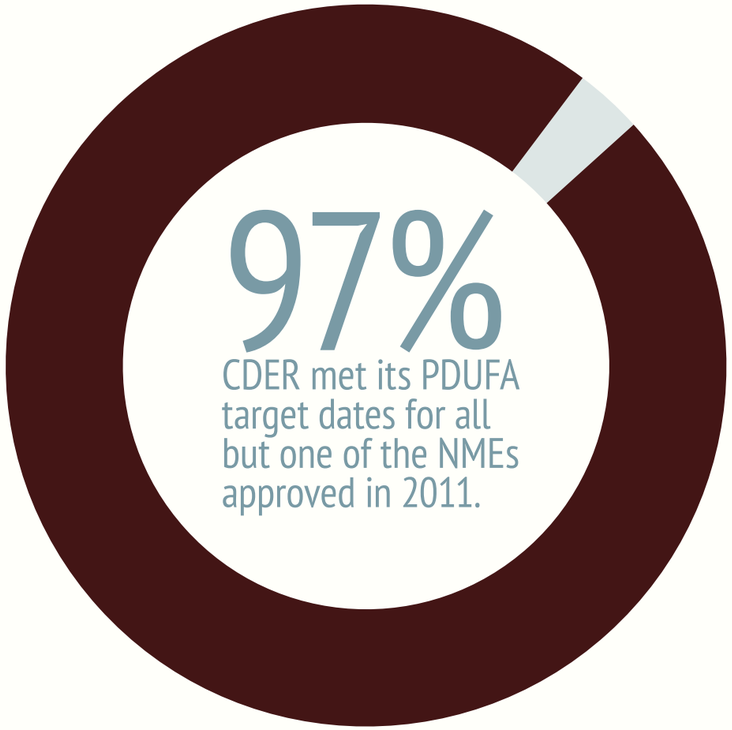
For more details about the individual NMEs, see pages 14 & 15.

PREDICTABILITY

Met PDUFA Target Dates

Under the Prescription Drug User Fee Act (PDUFA), the pharmaceutical industry provides FDA with funding to help ensure the agency conducts reviews for new products within targeted time frames.

Throughout the year, CDER was able to meet or exceed **PDUFA target dates** for application review agreed to with the pharmaceutical industry and approved by Congress. CDER met its PDUFA target dates for all but one (97%) of the NMEs approved in 2011.



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ACCESS

First Cycle Approval

Adcetris, Benlysta, Caprelsa, Dificid, Edarbi, Edurant, Erwinaze, Eylea, Gadavist, Incivek, Jakafi, Onfi, Tradjenta, Victrelis, Viibryd, Xalkori, Yervoy, Zelboraf, Zytiga

CDER approved most drugs (19 of 30) on the "**first cycle**" of review (63%), meaning without requests for additional information that would delay approval and lead to another cycle of review.

First Cycle Approval of Priority Review Drugs

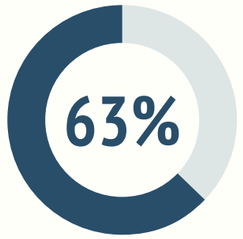
Adcetris, Benlysta, Caprelsa, Dificid, Erwinaze, Eylea, Incivek, Jakafi, Victrelis, Yervoy, Xalkori, Zelboraf, Zytiga

Of the 15 NMEs approved in 2011 designated for Priority Review, 13 (87%) were approved on the first cycle. This is particularly important because Priority Review drugs have the potential to serve as significant medical advances in health care. With faster review and approval results these products become available to patients sooner.

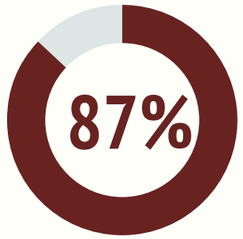
Approved First in the U.S.

Adcetris, Benlysta, Caprelsa, Dificid, Edarbi, Edurant, Eylea, Horizant, Incivek, Jakafi, Natroba, Nulojix, Tradjenta, Victrelis, Viibryd, Xalkori, Yervoy, Zelboraf, Zytiga

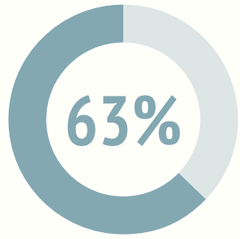
Comparing approval to other countries offers another measure of approval efficiency. Although regulatory processes differ widely between FDA and those of regulatory agencies in other countries, over half (19 of 30) of the NMEs approved in 2011 (63%) were approved first in the U.S. before any other country worldwide.



First Cycle Approval



First Cycle Approval of Priority Review Drugs



Approved first in the U.S.

For more details about the individual NMEs, see pages 14 & 15.

OVERVIEW

This document represents a broad overview of CDER approvals of new molecular entities (NMEs) for calendar year 2011.

Although the number of NMEs approved in 2011 is higher than those in the past seven years, the quantity of NMEs being approved by CDER over the past decade has not been substantially increasing. A key reason is that the number of applications filed for NMEs has also not been substantially increasing.

More important than the quantity of new drugs approved in 2011, is the quality of the new drugs and the important new roles they are serving to advance medical care.

Also noteworthy is the speed and predictability with which most of these drugs were reviewed and approved. CDER used a variety of "expedited approval" regulatory tools to speed these drugs to market.

In all cases of expedited approval, CDER does not compromise safety in the approval process.

More important than the quantity of new drugs approved in 2011, is the quality of the new drugs and the important new roles they are serving to advance medical care.

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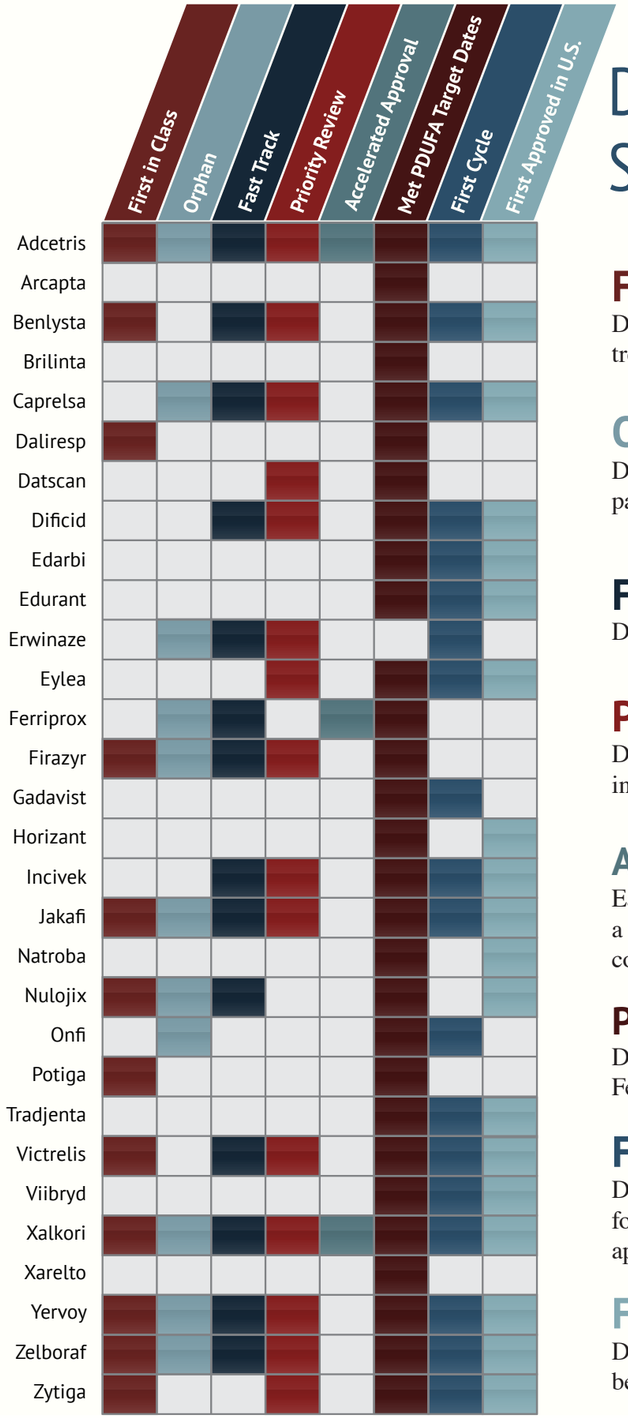

DRUG DESIGNATION SUMMARY

First-in-Class
Drugs with a new and unique mechanism for treating a medical condition

Orphan Drugs
Drugs approved for small populations of patients with rare diseases

Fast Track
Drugs that can treat unmet medical needs

Priority Review
Drugs with a target review of 6 months instead of 10 months

Accelerated Approval
Early approval based on markers that predict a reasonable benefit, with more testing to confirm clinical benefit after approval

PDUFA Target Dates
Drugs that met the Prescription Drug User Fee Act target dates for review

First Cycle
Drugs that were approved without request for additional information that would delay approval and lead to another cycle of review

First Approved in U.S.
Drugs that were approved first in the U.S. before any other country worldwide

THE NMEs OF 2011

Drug Name	Active Ingredient	Date	What it's used for
Eylea	aflibercept	11/18	To treat wet (neovascular) age-related macular degeneration (AMD), a leading cause of vision loss and blindness in Americans ages 60 and older.
Erwinaze	asparaginase Erwinia chrysanthemi	11/18	For patients with acute lymphoblastic leukemia (ALL), who have developed an allergy (hypersensitivity) to E. coli derived asparaginase and pegapargase chemotherapy drugs used to treat ALL.
Jakafi	ruxolitinib	11/16	To treat patients with the bone marrow disease myelofibrosis.
Onfi	clobazam	10/24	For use as an adjunctive (add-on) treatment for seizures associated with Lennox-Gastaut syndrome in adults and children 2 years of age and older.
Ferriprox	deferiprone	10/14	Iron overload from blood transfusions in patients with thalassemia (genetic disorder causing anemia), who had an inadequate response to chelation therapy.
Xalkori	crizotinib	08/26	Certain patients with late-stage (locally advanced or metastatic), non-small cell lung cancers who express the abnormal anaplastic lymphoma kinase gene.
Firazyr	icatibant	08/25	For the treatment of acute attacks of a rare condition called hereditary angioedema (HAE) in people ages 18 years and older.
Adcetris	brentuximab vedotin	08/19	Hodgkin lymphoma and ALCL (systemic anaplastic large cell lymphoma).
Zelboraf	vemurafenib	08/17	To treat patients with late-stage (metastatic) or unresectable (cannot be removed by surgery) melanoma, the most dangerous type of skin cancer.
Brilinta	ticagrelor	07/20	To reduce cardiovascular death and heart attack in patients with acute coronary syndromes (ACS).
Xarelto	rivaroxaban	07/01	To reduce the risk of blood clots, deep vein thrombosis (DVT), and pulmonary embolism (PE) following knee or hip replacement surgery.
Arcapta Neohaler	indacaterol inhalation powder	07/01	For the long term, once-daily maintenance bronchodilator treatment of airflow obstruction in people with chronic obstructive pulmonary disease (COPD) including chronic bronchitis and/or emphysema.
Nulojix	belatacept	06/15	To prevent acute rejection in adult patients who have had a kidney transplant.
Potiga	ezogabine	06/10	An add-on medication to treat seizures associated with epilepsy in adults.

To view this full summary online please visit the CDER web page spotlight on Drug Innovation:
http://www.fda.gov/Drugs/DevelopmentApprovalProcess/DrugInnovation/ucm285554.htm

CY 2011 New Molecular Entities (NMEs)

Drug Name	Active Ingredient	Date	What it's used for
Dificid	fidaxomicin	05/27	For the treatment of *Clostridium difficile*-associated diarrhea (CDAD).
Incivek	telaprevir	05/23	To treat certain adults with chronic hepatitis C infection.
Edurant	rilpivirine	05/20	Treatment of HIV-1 infection in adults who have never taken HIV therapy.
Victrelis	boceprevir	05/13	To treat certain adults with chronic hepatitis C.
Tradjenta	linagliptin	05/02	Addition to diet and exercise to improve glycemic control in adults with type 2 diabetes mellitus.
Zytiga	abiraterone acetate	04/28	In combination with prednisone to treat patients with late-stage (metastatic) castration-resistant prostate cancer who have received docetaxel (chemotherapy).
Caprelsa	vandetanib	04/06	To treat adult patients with late-stage (metastatic) medullary thyroid cancer, ineligible for surgery who have disease that is growing or causing symptoms.
Horizant	gabapentin enacarbil	04/06	A once-daily treatment for moderate-to-severe restless legs syndrome (RLS).
Yervoy	ipilimumab	03/25	Late-stage (metastatic) melanoma, the most dangerous type of skin cancer.
Gadavist	gadobutrol	03/14	Magnetic resonance imaging (MRI) of the central nervous system.
Benlysta	belimumab	03/10	To treat patients with active, autoantibody-positive lupus (systemic lupus erythematosus) who are receiving standard therapy, including corticosteroids, antimalarials, immunosuppressives, and nonsteroidal anti-inflammatory drugs.
Daliresp	roflumilast	02/28	To decrease the frequency of flare-ups (exacerbations) or worsening of symptoms from severe chronic obstructive pulmonary disease (COPD).
Edarbi	azilsartan medoxomil	02/25	To treat high blood pressure (hypertension) in adults.
Viibryd	vilazodone HCl	01/21	To treat major depressive disorder in adults.
Natroba	spinosad	01/18	For the treatment of head lice infestation in patients ages 4 years and older.
Datscan	ioflupane i-123	01/14	An imaging drug used to assist in the evaluation of adult patients with suspected Parkinsonian syndromes (PS).

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