

NOVEL DRUGS 2016 SUMMARY

Infographic Source Material

<http://www.fda.gov/Drugs/DevelopmentApprovalProcess/DrugInnovation/ucm534863.htm>

22

In 2016, the FDA's Center for Drug Evaluation and Research (CDER) approved 22 novel drugs.



Novel drugs are innovative products that serve previously unmet medical needs or otherwise significantly help to advance patient care and public health.

30

From 2007 through 2015 CDER averaged about 30 novel drug approvals per year.

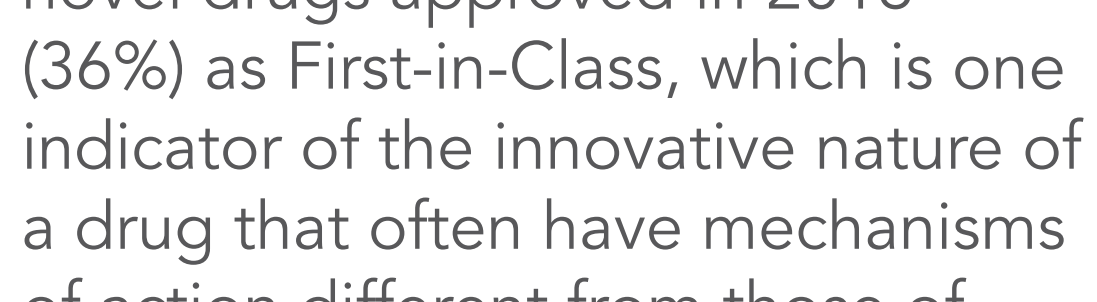
Adlyxin
Anthem
Axumin
Briviact
Cinqair
Defitelio
Epclusa
Eucrisa
Exondys 51
Lartruvo
Netspot

Nuplazid
Ocaliva
Rubraca
Spinraza
Taltz
Tecentriq
Venclexta
Xiidra
Zepatier
Zinbryta
Zinplava

IMPACT

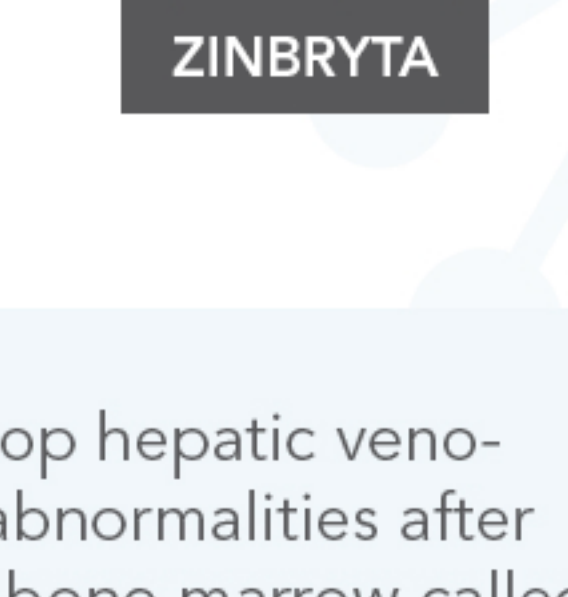
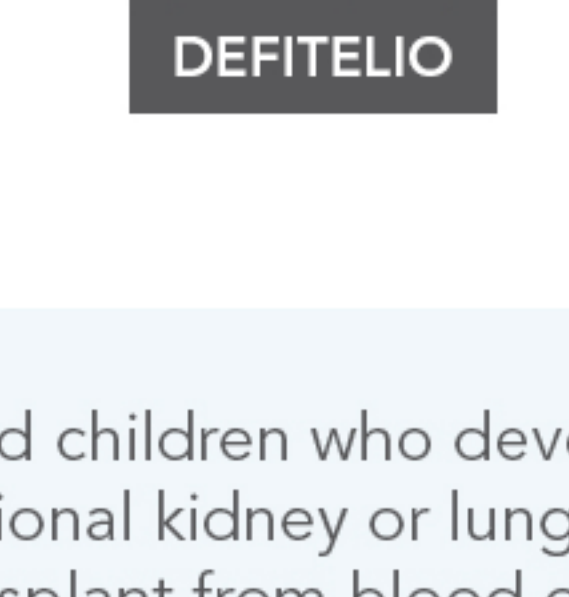
Many of the 22 novel drugs CDER approved in 2016 are notable for their potential positive impact and unique contributions to quality medical care and public health.

First-in-Class

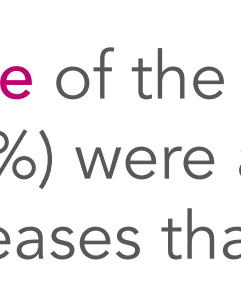


Noteworthy First-in-Class products include:

CDER identified eight of the 22 novel drugs approved in 2016 (36%) as First-in-Class, which is one indicator of the innovative nature of a drug that often have mechanisms of action different from those of existing therapies.

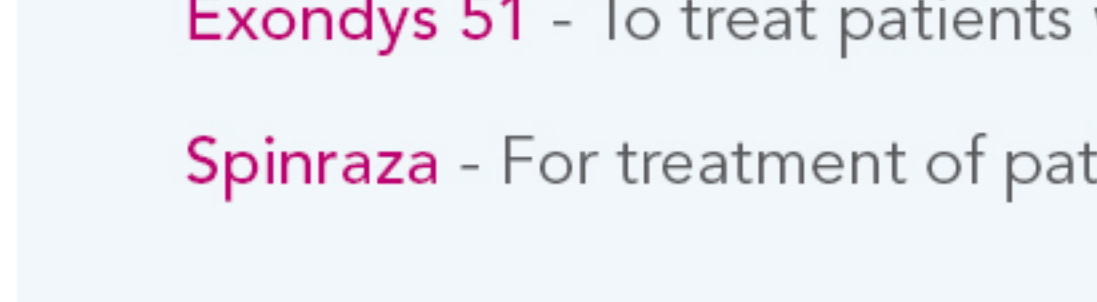


Defitelio - To treat adults and children who develop hepatic veno-occlusive disease with additional kidney or lung abnormalities after they receive a stem cell transplant from blood or bone marrow called hematopoietic stem cell transplantation



Zinbryta - To treat multiple sclerosis

Drugs for Rare Diseases



200,000
OR
FEWER
AMERICANS

Nine of the 22 novel drugs approved in 2016 (41%) were approved to treat rare or "orphan" diseases that affect 200,000 or fewer Americans.

Noteworthy examples of drugs to treat rare diseases among the 2016 novel drugs include:

Exondys 51 - To treat patients with Duchenne muscular dystrophy

Spinraza - For treatment of patients with spinal muscular atrophy

Other Noteworthy Novel Drugs of 2016

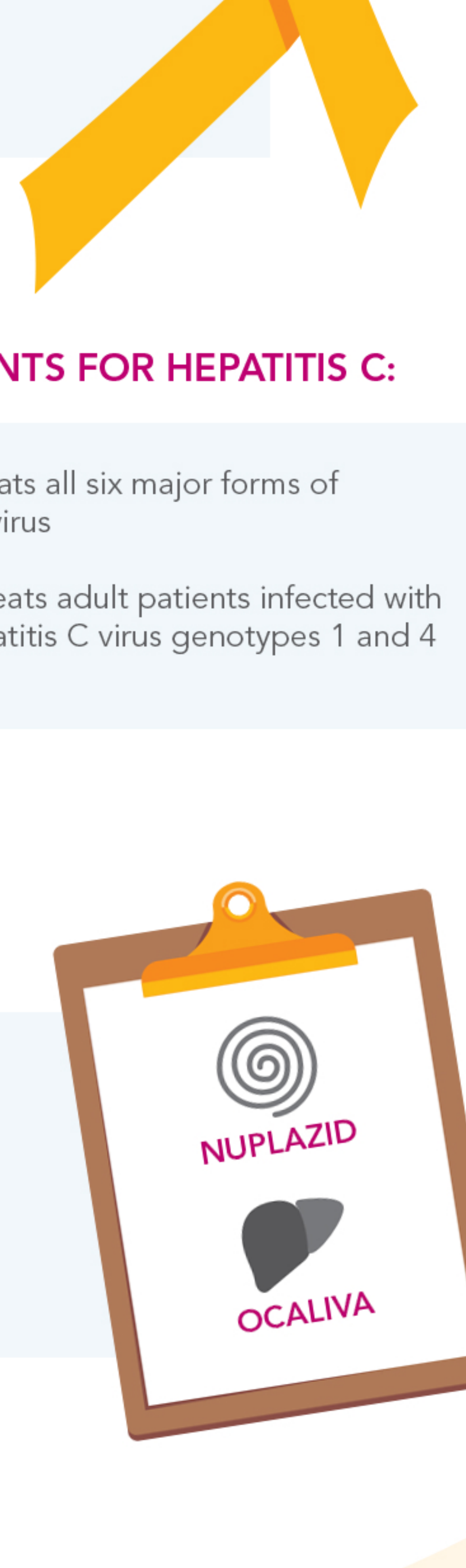
CANCER THERAPIES:

Lartruvo: Treats patients with a form of cancer called soft tissue sarcoma

Rubraca: Treats women with ovarian cancer

Tecentriq: Treats patients with the most common type of bladder cancer (urothelial carcinoma)

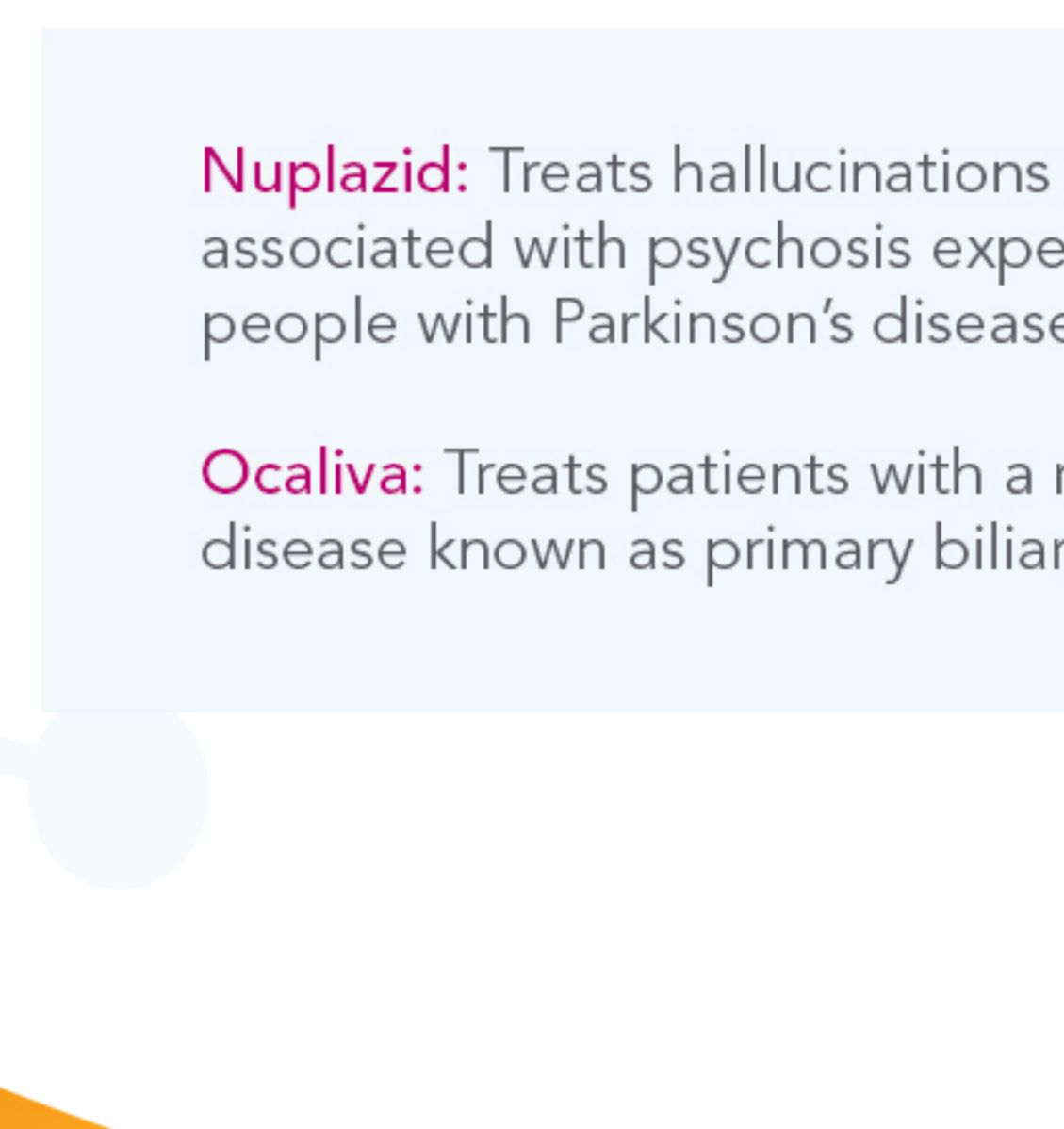
Venclexta: For certain patients with chronic lymphocytic leukemia



TREATMENTS FOR HEPATITIS C:

Epclusa: Treats all six major forms of hepatitis C virus

Zepatier: Treats adult patients infected with chronic hepatitis C virus genotypes 1 and 4



ADDITIONAL NOTEWORTHY APPROVALS INCLUDE:

Nuplazid: Treats hallucinations and delusions associated with psychosis experienced by some people with Parkinson's disease

Ocaliva: Treats patients with a rare chronic liver disease known as primary biliary cirrhosis



INNOVATION

CDER used a number of regulatory methods to expedite the development and approval of novel drugs in 2016, including:

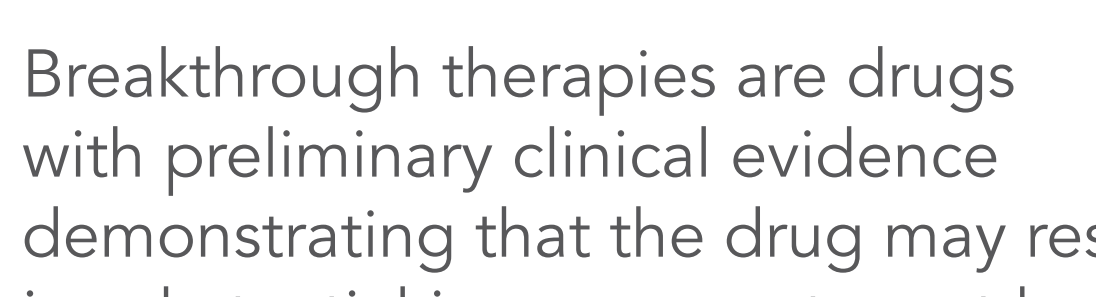
Fast Track

- Fast Track speeds new drug development and review.
- These drugs have the potential to address unmet medical needs.

Anthem
Defitelio
Epclusa
Exondys 51

Lartruvo
Ocaliva
Spinraza
Zinplava

Eight of the 2016 novel drugs (36%) were designated by CDER as Fast Track.



Breakthrough

- Breakthrough status is designed to help shorten the development time of a potential new 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 over other available therapies.

Epclusa
Lartruvo
Nuplazid
Rubraca

Tecentriq
Venclexta
Zepatier

CDER designated seven of the 2016 novel drugs (32%) as Breakthrough therapies.



Priority Review

- The drug is reviewed within six months instead of the standard 10 months.
- These drugs could potentially provide a significant advance in medical care.

Axumin
Defitelio
Epclusa
Exondys 51
Lartruvo

Netspot
Nuplazid
Ocaliva
Rubraca
Spinraza

Tecentriq
Venclexta
Xiidra
Zepatier
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Fifteen of the 2016 novel drugs (68%) were designated Priority Review.



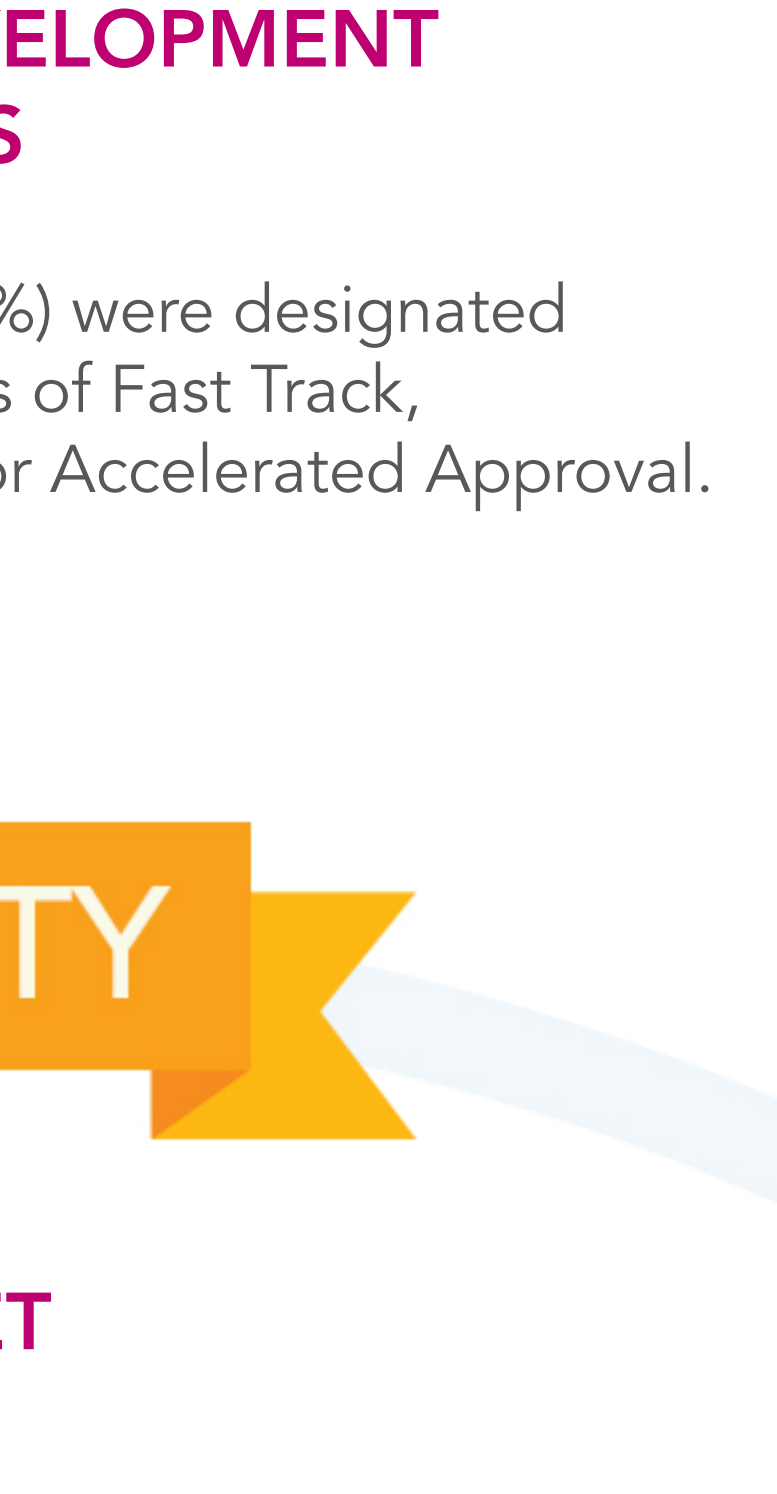
Accelerated Approval

- The Accelerated Approval program allows for early approval of a drug for serious or life-threatening illness that offers a benefit over current treatments.
- Once Accelerated Approval is granted, the drug must undergo additional testing to confirm that benefit.

Exondys 51
Lartruvo
Ocaliva

Rubraca
Tecentriq
Venclexta

CDER approved six of the 2016 novel drugs (27%) under the Accelerated Approval program.



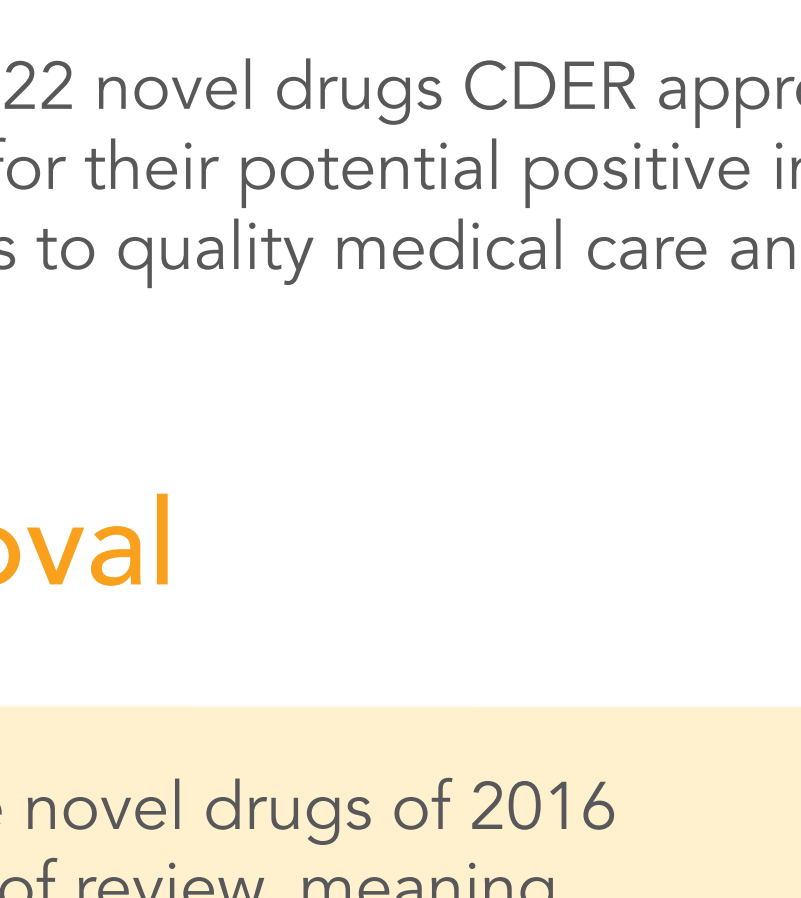
OVERALL USE OF EXPEDITED DEVELOPMENT AND REVIEW METHODS

73%

Sixteen of the 2016 novel drugs (73%) were designated in one or more expedited categories of Fast Track, Breakthrough, Priority Review, and/or Accelerated Approval.

PREDICTABILITY

PDUFU GOAL DATES MET



In 2016, CDER met its PDUFU goal dates for 95% of the novel drugs approved in 2016.

ACCESS

Many of the 22 novel drugs CDER approved in 2016 are notable for their potential positive impact and unique contributions to quality medical care and public health.

First Cycle Approval

CDER approved 21 of the novel drugs of 2016 (95%) on the "first cycle" of review, meaning without requests for additional information that would delay approval and lead to another cycle of review.



Approval in the United States Before Other Countries

19 of the 22 novel drugs approved in 2016 (86%) were approved in the United States before receiving approval in any other country.



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