# CDER New Drugs Program: 2018 Update

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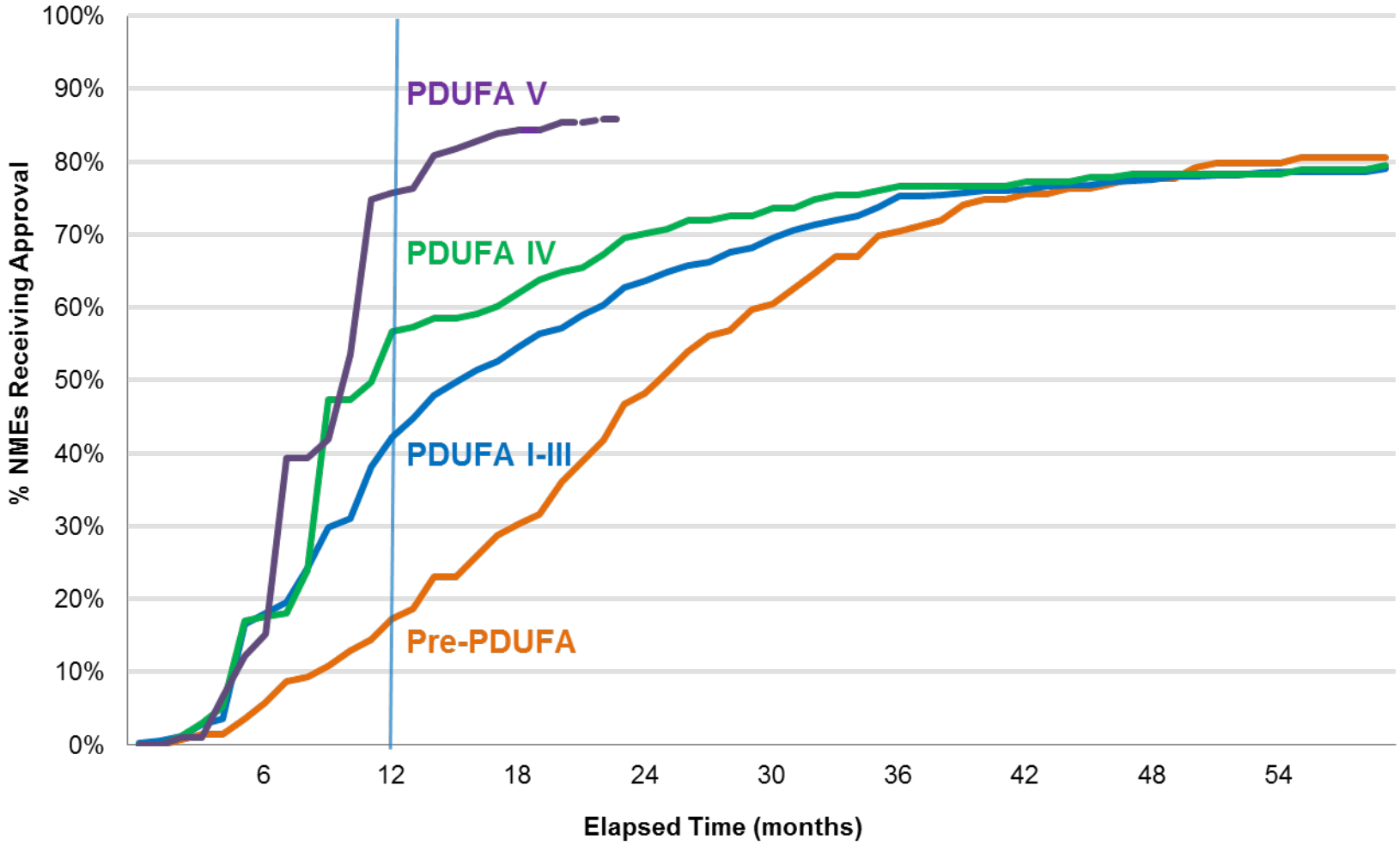
# Housekeeping

- Data and analyses presented to reflect latest information, although usual QC for official FDA reports has not occurred. Presentation content should be considered *preliminary*.
- Pay close attention to fiscal year (FY), calendar year (CY), or academic year (AY) and cut-off dates on data presentations; denominators are important too!
- Talented staff at FDA provide the data and analyses for this talk each year. Special thanks and acknowledgement to:
  - Nader Qassim, Nancy Maizel, and Reza Kazemi-Tabriz in CDER's Office of Program and Strategic Analysis
  - Mike Lanthier in the Office of the Commissioner

# Topics to be covered

- New drug review process efficiency: a historical look and changes in PDUFA VI
- New drug activity in 2018: approvals, workload, international comparisons, and profiling the 2018 class of NMEs/BLAs
- Development phase activity: IND workload, the breakthrough program, meeting workload and changes in PDUFA VI
- A look ahead to 2019

# CDER New Molecular Entity Approval Rates by PDUFA Cohort




Projection estimates account for actions to date and elapsed time to date for non-approvals

Data as of 11/30/18

# New Drug Activity in 2018

- In CY 2018 so far*, CDER has approved 55 NMEs, including 31 orphan drugs
  - 42 Priority Reviewed NME approvals, more than double the recent 5-year average of 20 priority approvals per year
  - For the first time ever, the majority of NMEs approved are orphan drugs to treat rare diseases
  - 2018 has a unique blend of therapeutic areas, quantity of approvals is not driven by oncology indications as in the past
- U.S. continues to lead the world in first approval of NMEs
- Several Notable Approvals, including:
  - Epidiolex (Cannabidiol) – Cannabinoid Approval
  - Erleada (Apalutamide) – Novel Endpoint
  - Lucemyra (Lofexidine Hydrochloride) - Opioid Withdrawal
  - TPOXX (tecovirimat) – treat small pox and address the risk of bioterrorism

* This information is accurate as of November 30th, 2018. In rare instances, it may be necessary for FDA to change a drug's new molecular entity (NME) designation or the status of its application as a new biologics license application (BLA). This note applies to all references to NME/Original BLAs in this presentation.

# Notable Approvals: Not Only Quantity but Quality for 2018

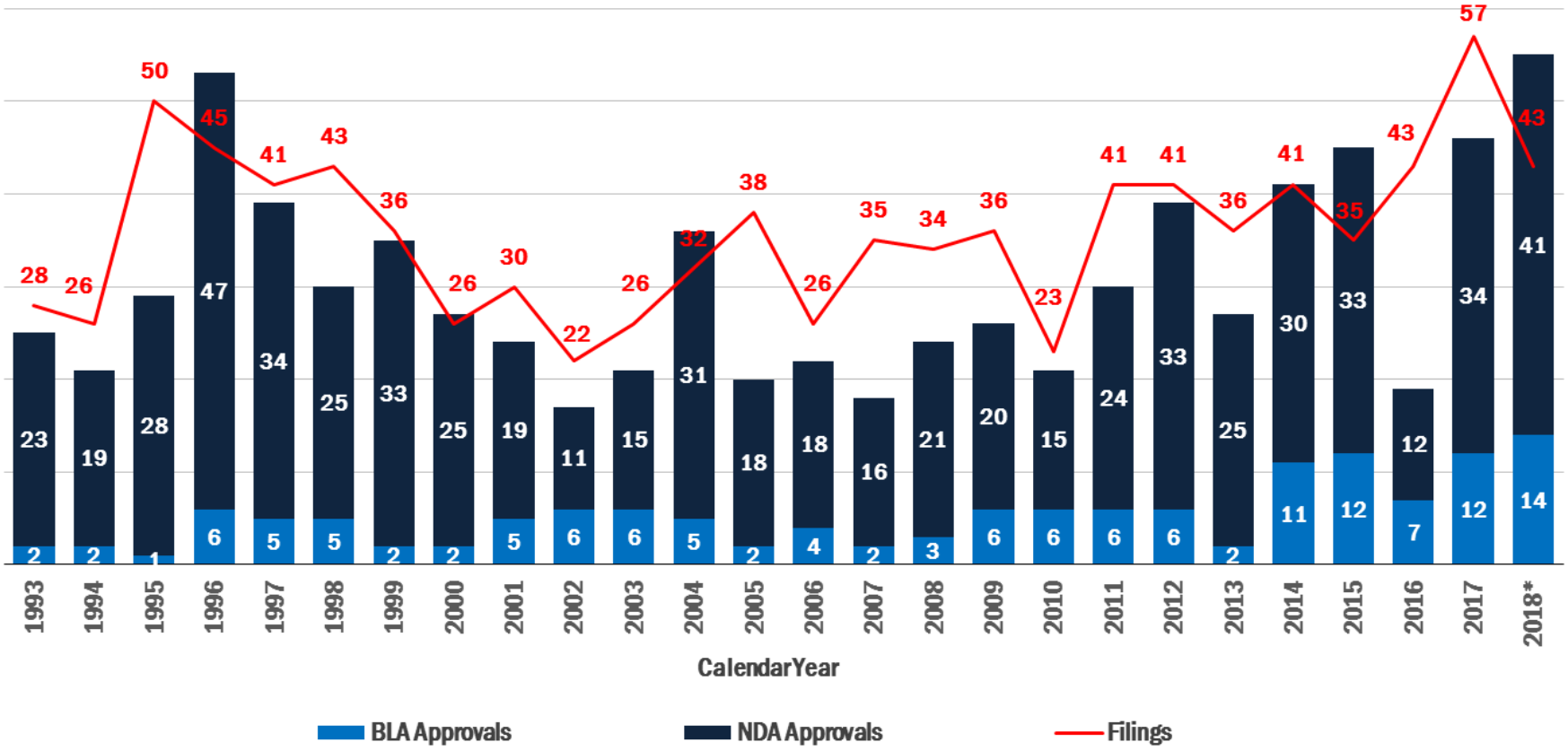
**Epidiolex (cannabidiol)**: for the treatment of seizures associated with two rare and severe forms of epilepsy, Lennox-Gastaut syndrome and Dravet syndrome, for patients two years of age and older. This is the first FDA-approved drug that contains a purified drug substance derived from marijuana. It is also the first FDA approval of a drug for the treatment of patients with Dravet syndrome.

**Erleada (Apalutamide):** for the treatment of patients with prostate cancer that has not spread (non-metastatic), but that continues to grow despite treatment with hormone therapy (castration-resistant). This is the first FDA-approved treatment for non-metastatic, castration-resistant prostate cancer using novel clinical trial endpoint.

**Lucemyra (Lofexidine Hydrochloride)** : for the non-opioid treatment for management of opioid withdrawal symptoms in adults.

**TPOXX (tecovirimat):** the first drug with an indication for treatment of smallpox. Though the World Health Organization declared smallpox eradicated in 1980, there have been longstanding concerns that smallpox could be used as a bioweapon.

# CDER NME NDAs/BLAs†
# Filings and Approvals by CY as of 11/30/18

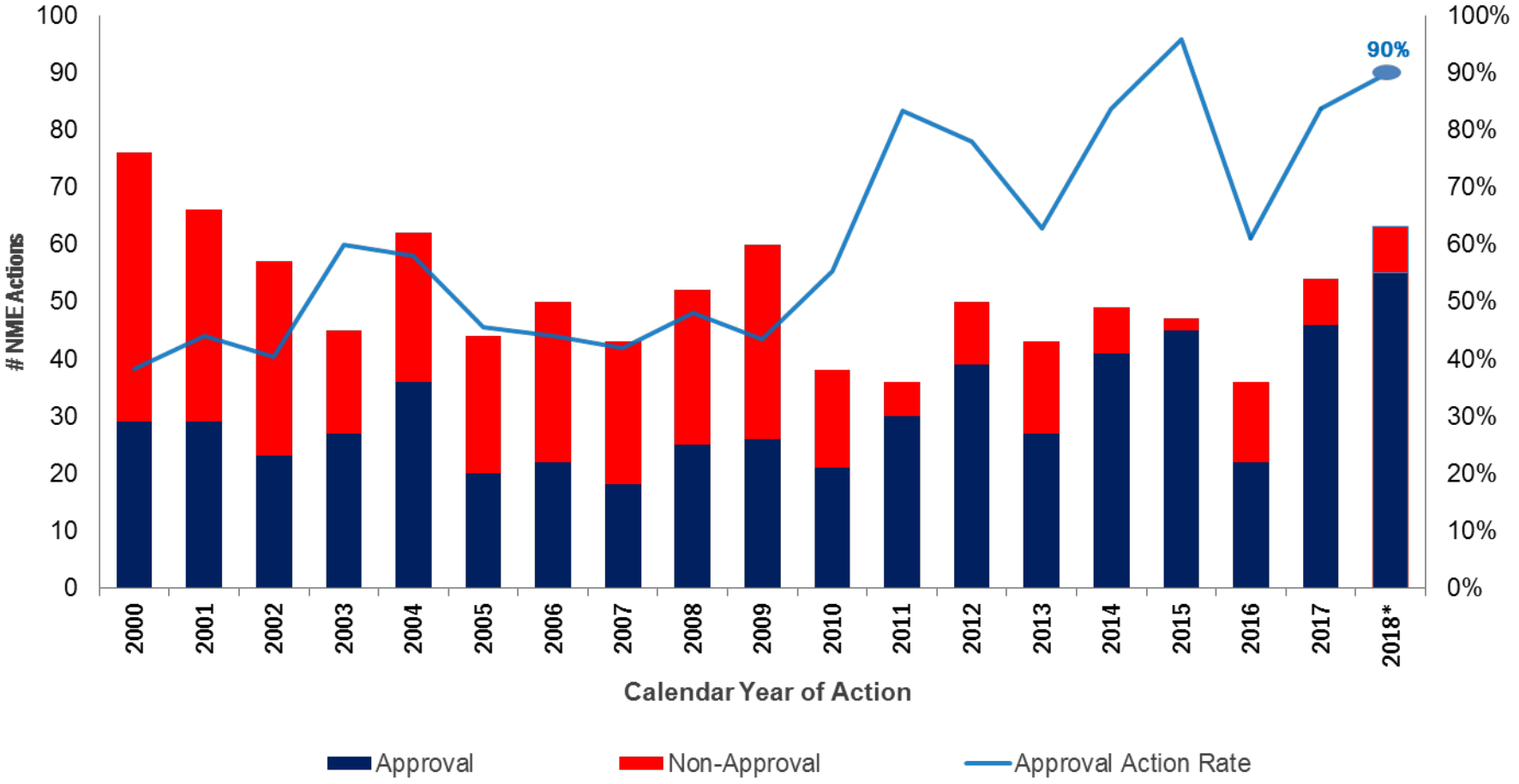



† Multiple applications pertaining to a single new molecular/biologic entity are only counted once. Original BLAs that do not contain a new active ingredient are excluded.

* This information is accurate as of November 30th, 2018. In rare instances, it may be necessary for FDA to change a drug's new molecular entity (NME) designation or the status of its application as a new biologics license application (BLA). This note applies to all references to NME/Original BLAs in this presentation.

Since applications are received and filed throughout a calendar year, the filed applications in a given calendar year do not necessarily correspond to an approval in the same calendar year. Certain applications are within their 60-day filing review period and may not be filed upon completion of the review.

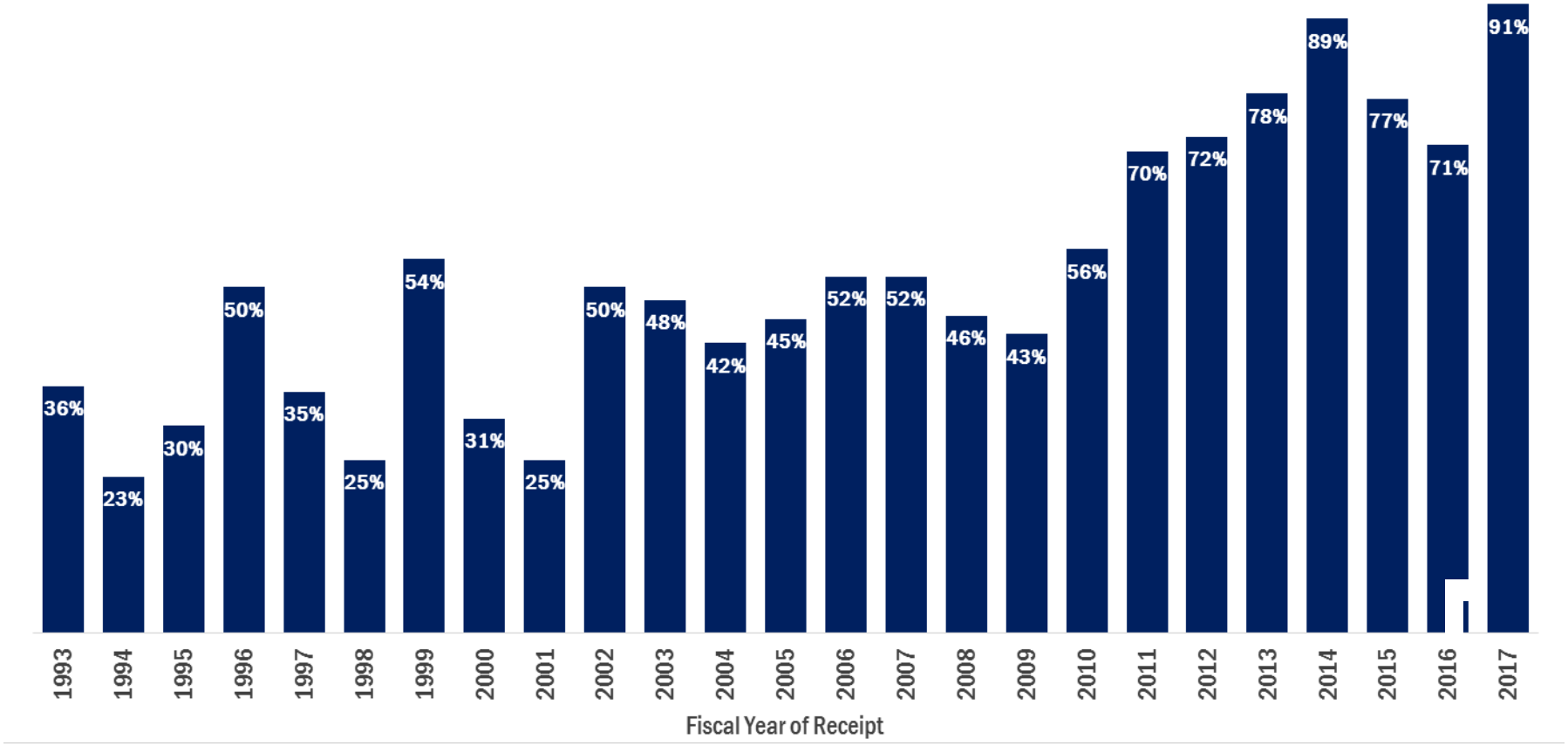
# NME Actions and Approvals by CY




*Data as of 11/30/2018

Includes discrete actions on a given date for an active ingredient which, if approved, would constitute a new molecular entity. Actions for original submissions and resubmissions as well as actions for new BLAs are included. Multiple actions which occur on the same date for multiple dosage forms or indications are counted as a single regulatory action.

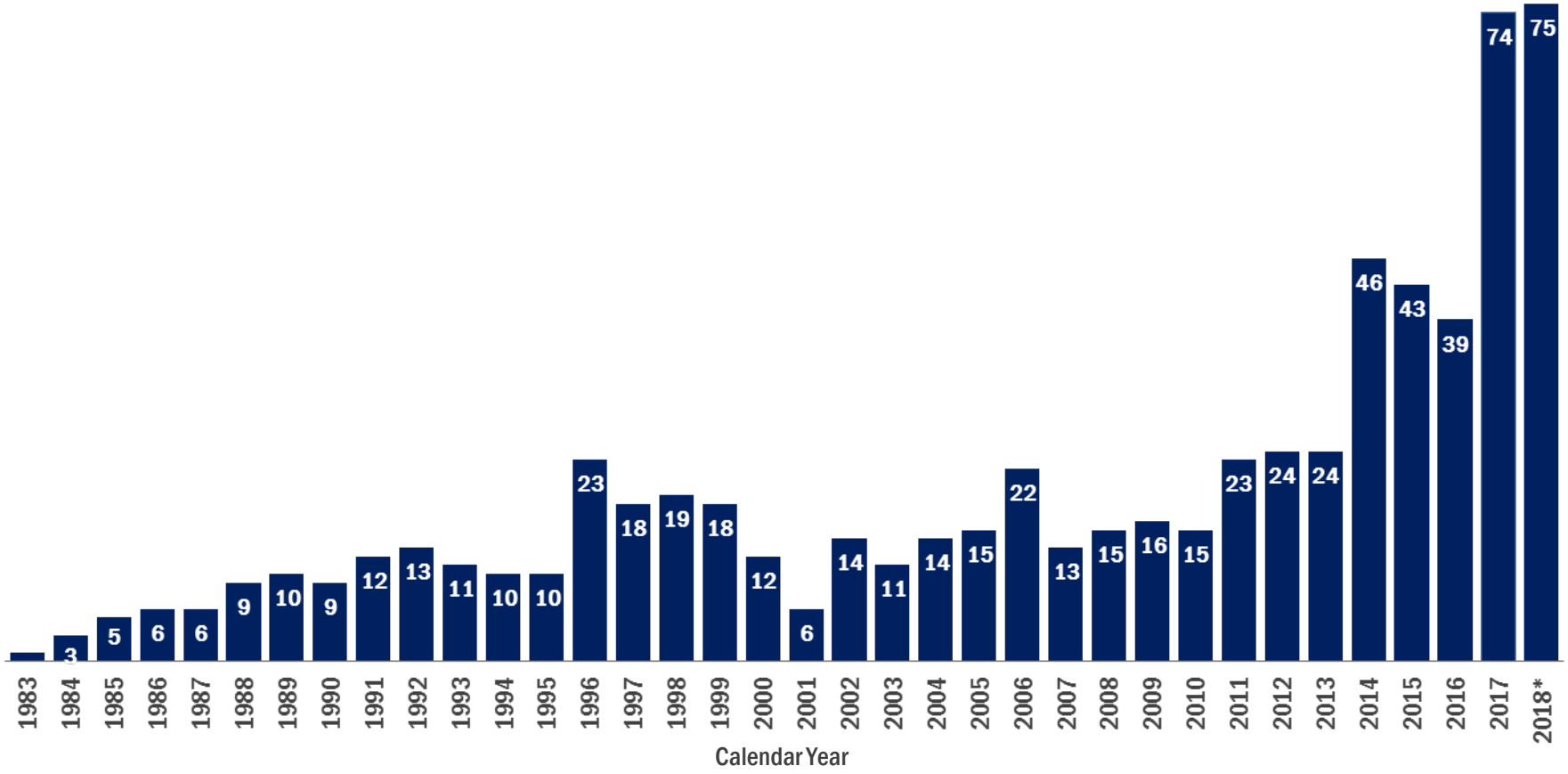
# CDER NME NDAs/BLAs†
# First Action Approval Rate by FY




Data as of 11/30/2018

† Multiple applications pertaining to a single new molecular/biologic entity (e.g., single ingredient and combinations) are only counted once. Therefore, the numbers represented here for filings are not indicative of workload in the PDUFA V Program. Original BLAs that do not contain a new active ingredient are excluded.

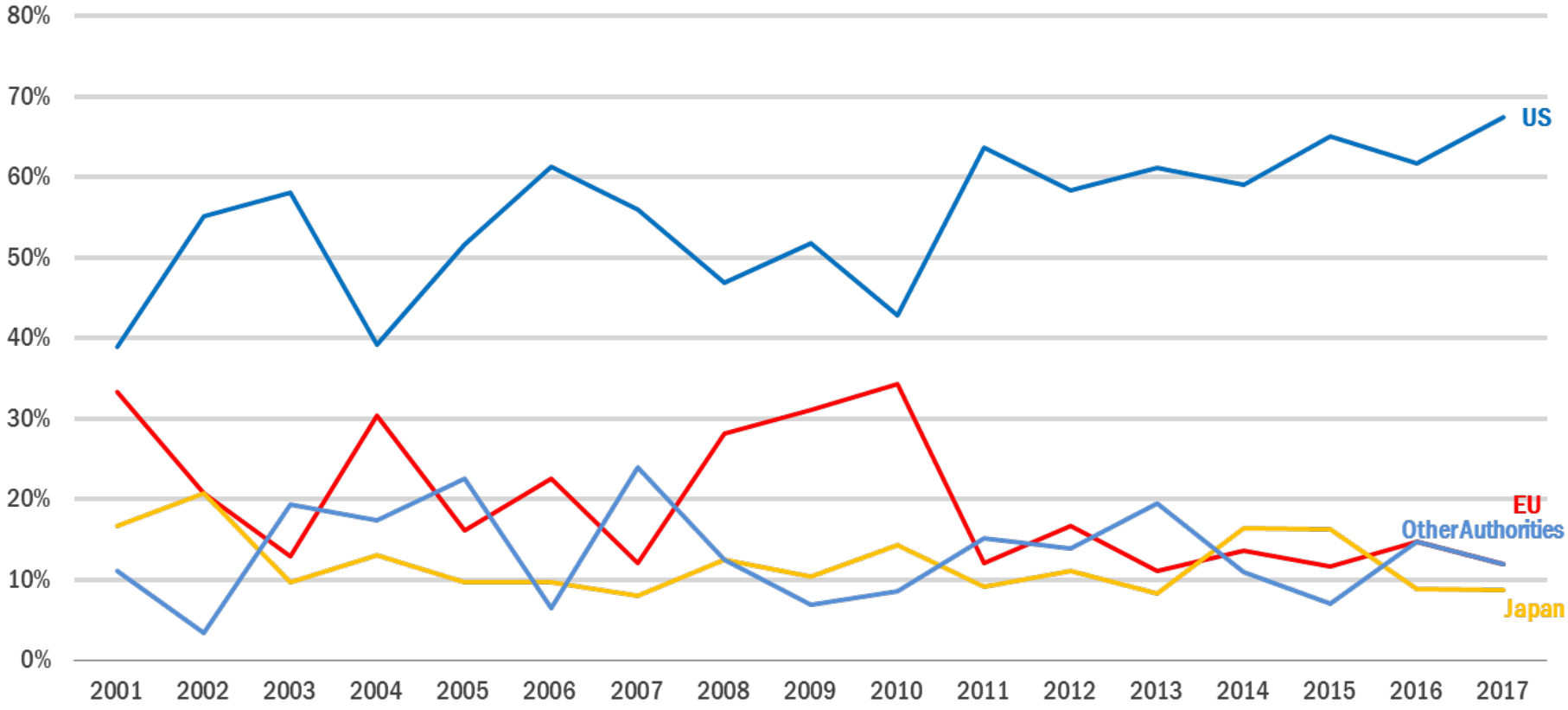
# CDER Approved Orphan Indications for all NDAs and BLAs† by CY




† Includes Efficacy Supplements

* Data as of 11/30/2018

# USA Share of New Active Substances Launched on World Market Remains High




Data as of 11/30/2018

Source: Scrip Magazine (1982 - 2006), Pharmaprojects/Citeline Pharma R&D Annual Review (2007 - 2017)

# Snapshot of CY 2018
# NME NDAs/BLAs† Drug Approvals (1/2)

| Trade Name | Met PDUFA Goal Date* | Approved on First Cycle | First in Class | Approved First in the U.S. | Breakthrough Therapy | Priority Approval | Fast Track | Accelerated Approval | Orphan Drug | QIDP |
|---|---|---|---|---|---|---|---|---|---|---|
| LUTATHERA | | | | | | | | | | |
| BIKTARVY | | | | | | | | | | |
| SYMDEKO | | | | | | | | | | |
| ERLEADA | | | | | | | | | | |
| TROGARZO | | | | | | | | | | |
| ILUMYA | | | | | | | | | | |
| TAVALISSE | | | | | | | | | | |
| CRYSVITA | | | | | | | | | | |
| AKYNZEO | | | | | | | | | | |
| LUCEMYRA | | | | | | | | | | |
| AIMOVIG | | | | | | | | | | |
| LOKELMA | | | | | | | | | | |
| DOPTELET | | | | | | | | | | |
| PALYNZIQ | | | | | | | | | | |
| OLUMIANT | | | | | | | | | | |
| MOXIDECTIN | | | | | | | | | | |
| EPIDIOLEX | | | | | | | | | | |
| ZEMDRI | | | | | | | | | | |
| MEKTOVI | | | | | | | | | | |
| BRAFTOVI | | | | | | | | | | |
| TPOXX | | | | | | | | | | |
| TIBSOVO | | | | | | | | | | |
| KRINTAFEL | | | | | | | | | | |
| ORILISSA | | | | | | | | | | |
| OMEGAVEN | | | | | | | | | | |
| MULPLETA | | | | | | | | | | |

Data as of 11/30/2018

† Multiple submissions pertaining to a single new molecular/biologic entity (e.g., single ingredient and combinations) are only counted once. Therefore, the numbers are not indicative of workload in the PDUFA V Program. Original BLAs that do not contain a new active ingredient are excluded.

* A PDUFA Goal Date is marked as met if the NME is acted upon within its approval cycle due date.

# Snapshot of CY 2018
# NME NDAs/BLAs† Drug Approvals (2/2)

| Trade Name | Met PDUFA Goal Date* | Approved on First Cycle | First in Class | Approved First in the U.S. | Breakthrough Therapy | Priority Approval | Fast Track | Accelerated Approval | Orphan Drug | QIDP |
|---|---|---|---|---|---|---|---|---|---|---|
| POTELIGEO | | | | | | | | | | |
| ONPATTRO | | | | | | | | | | |
| ANNOVERA | | | | | | | | | | |
| GALAFOLD | | | | | | | | | | |
| DIACOMIT | | | | | | | | | | |
| OXERVATE | | | | | | | | | | |
| TAKHZYRO | | | | | | | | | | |
| XERAVA | | | | | | | | | | |
| PIFELTRO | | | | | | | | | | |
| LUMOXITI | | | | | | | | | | |
| AJOVY | | | | | | | | | | |
| COPIKTRA | | | | | | | | | | |
| EMGALITY | | | | | | | | | | |
| VIZIMPRO | | | | | | | | | | |
| LIBTAYO | | | | | | | | | | |
| SEYSARA | | | | | | | | | | |
| NUZYRA | | | | | | | | | | |
| REVCOVI | | | | | | | | | | |
| TEGSEDI | | | | | | | | | | |
| TALZENNA | | | | | | | | | | |
| XOFLUZA | | | | | | | | | | |
| LORBRENA | | | | | | | | | | |
| YUPELRI | | | | | | | | | | |
| AEMCOLO | | | | | | | | | | |
| GAMIFANT | | | | | | | | | | |
| DAURISMO | | | | | | | | | | |
| VITRAKVI | | | | | | | | | | |
| FIRDAPSE | | | | | | | | | | |
| XOSPATA | | | | | | | | | | |

Data as of 11/30/2018

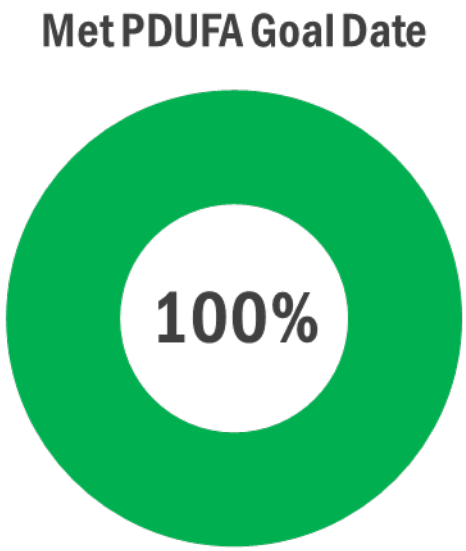
† Multiple submissions pertaining to a single new molecular/biologic entity (e.g., single ingredient and combinations) are only counted once. Therefore, the numbers are not indicative of workload in the PDUFA V Program. Original BLAs that do not contain a new active ingredient are excluded.

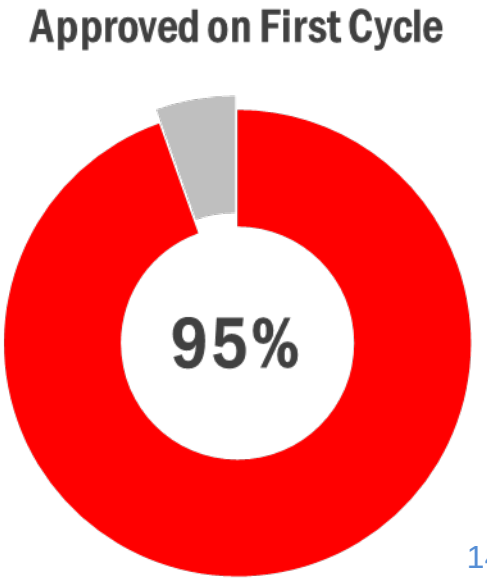
* A PDUFA Goal Date is marked as met if the NME is acted upon within its approval cycle due date.

# In CY 2018, CDER Continued To Ensure The Efficiency Of First Cycle Review

- All of the (100%) NMEs/BLAs approved to date in 2018 met their PDUFA goal dates

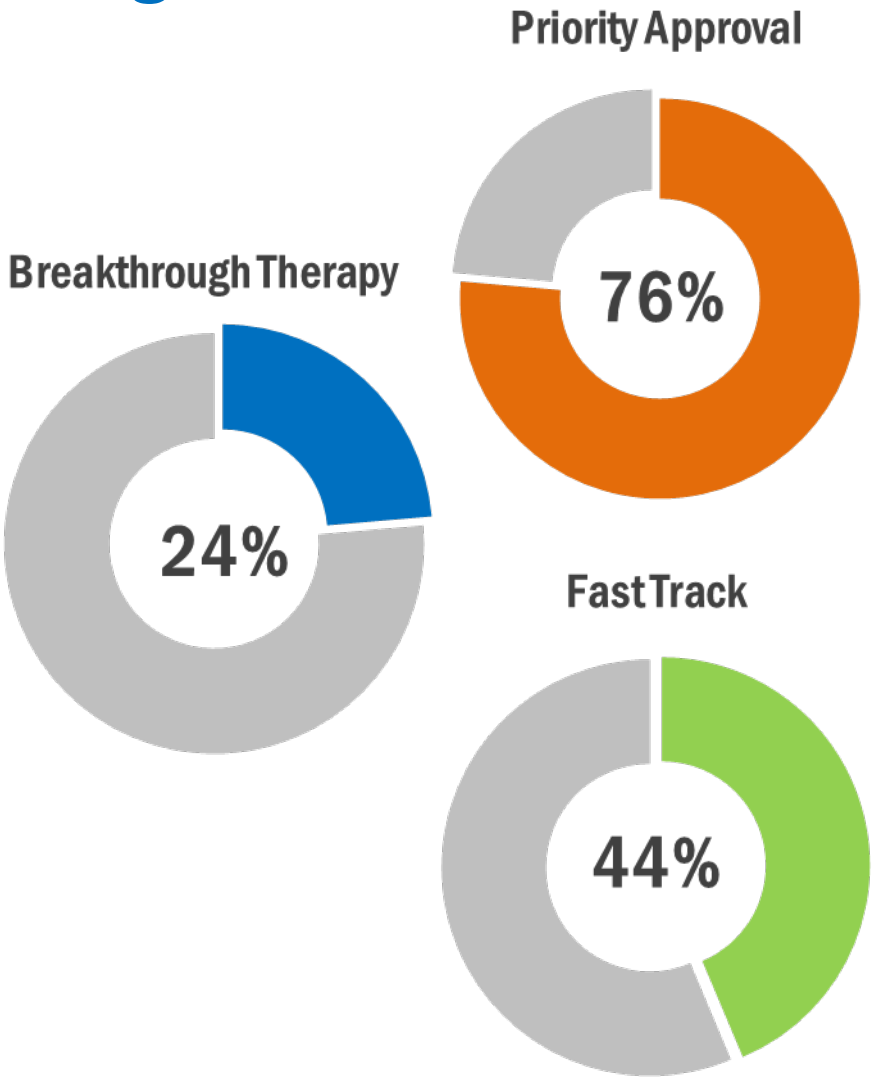
- All but three (95%) of the drugs approved to date in 2018 were approved in the first review cycle



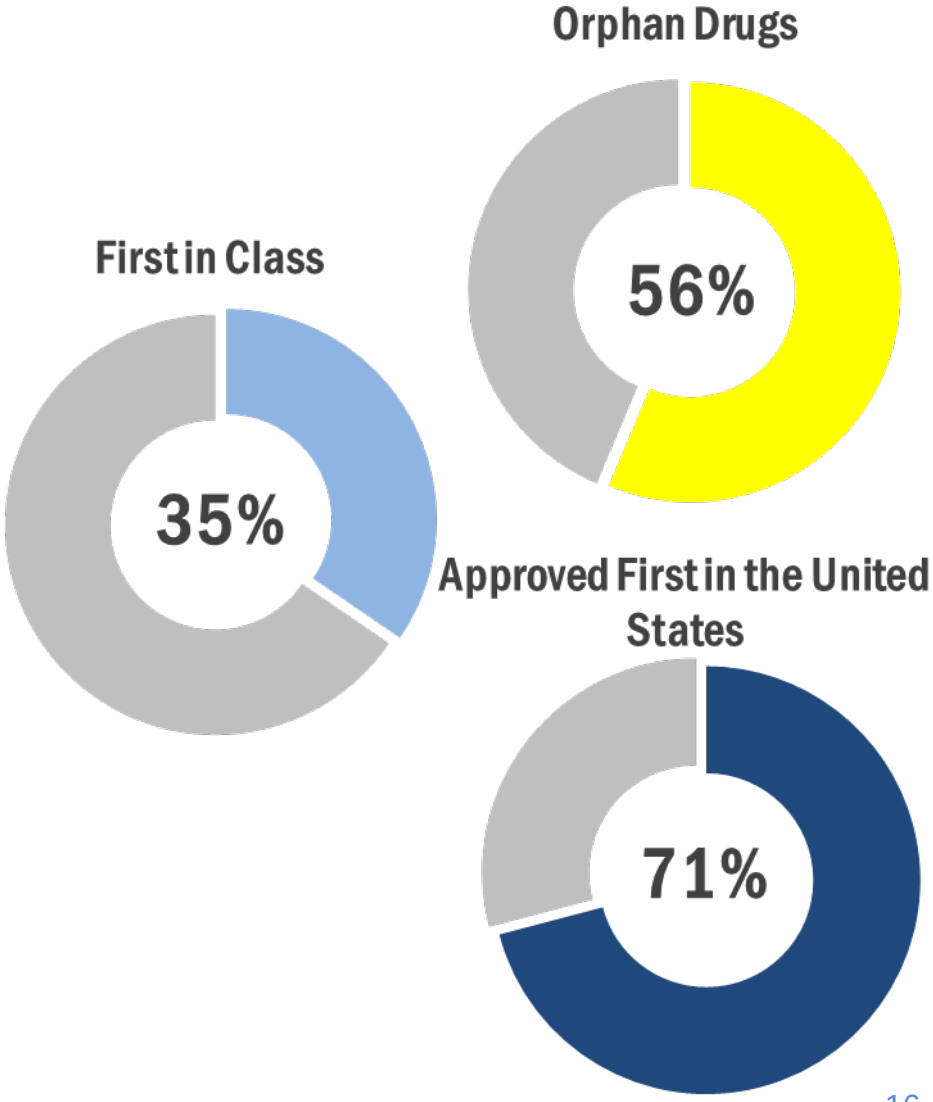
# Utilization of Expedited Development and Review Programs Remained High in 2018

- Over three – quarters (76%) of the drugs approved to date in 2018 were approved under Priority Review
- Almost one out of four (24%) of the drugs approved to date in 2018 received Breakthrough Therapy designation
- About four out of ten (44%) of the drugs approved to date in 2018 received Fast Track designation

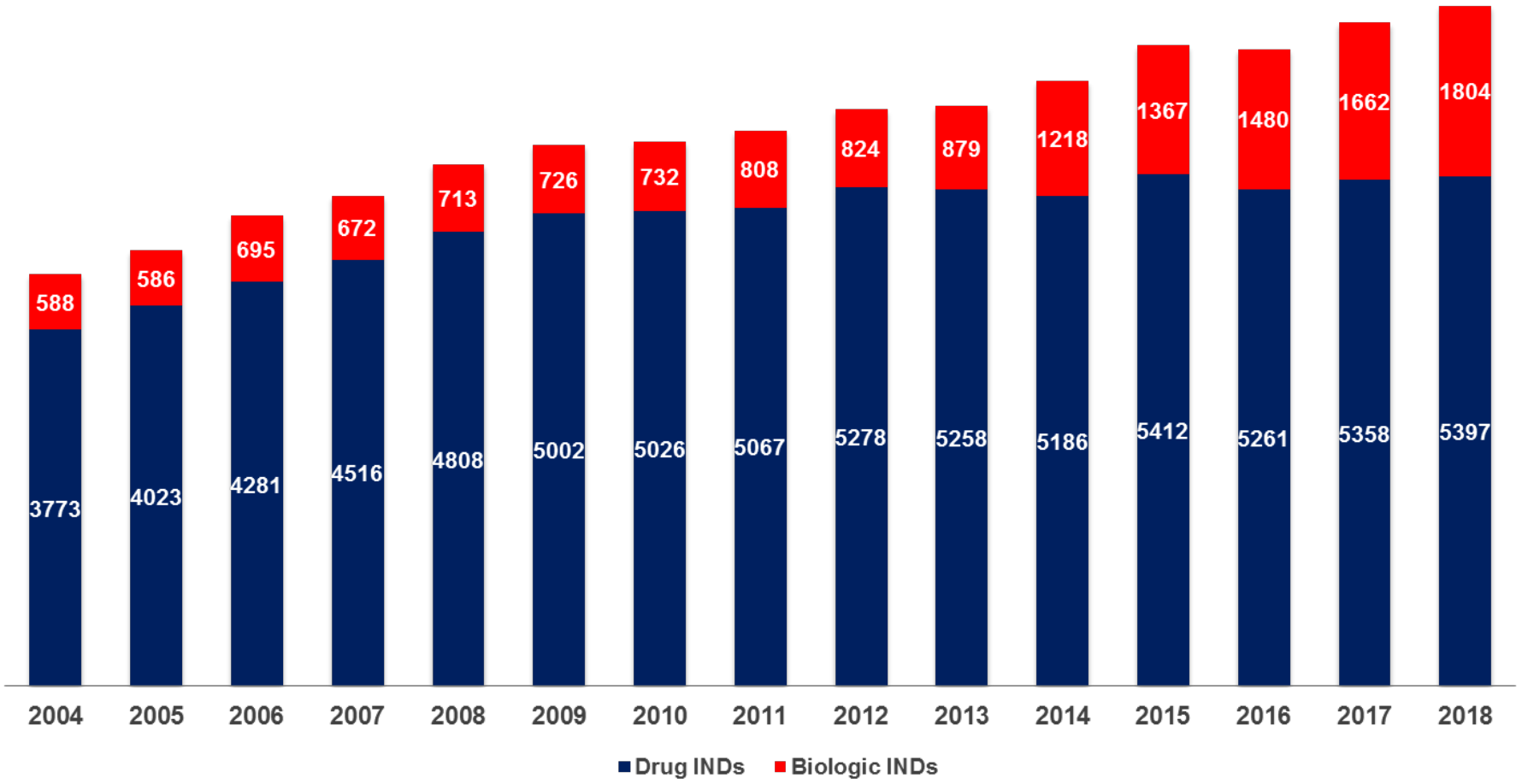


# 2018 Continues A Strong Track Record For Drug Innovation

- Over half (56%) of the drugs approved to date in 2018 are orphan drugs
- About a third (35%) of the drugs approved to date in 2018 are the first in their class
- Almost three – quarters (71%) of the drugs approved to date in 2018 were first approved in the U.S.



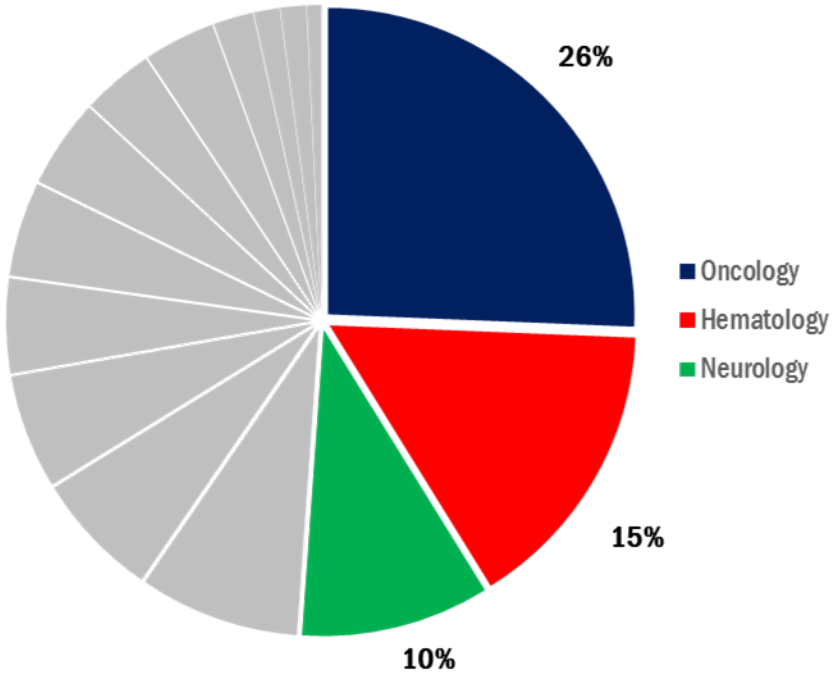
# Development Phase Work Continued to Grow in 2018




Data are from the PDUFA Workload Adjuster and represent a 12 month period of July 1st - June 30th

# CDER Breakthrough Therapy Requests by Division

**Oncology, Hematology, and Neurology account for over 50% of Breakthrough Requests.**




663 Requests since BT program inception in July 2012

## Some notable conditions include:

- Pheochromocytoma and Paraganglioma
- Hemophagocytic Lymphohistiocytosis (HLH)
- Lambert Eaton Myasthenic Syndrome (LEMS)

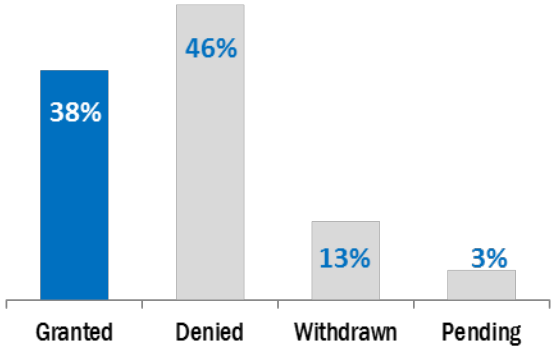
Data as of 11/30/2018

# CDER Breakthrough Therapy Grants by Division

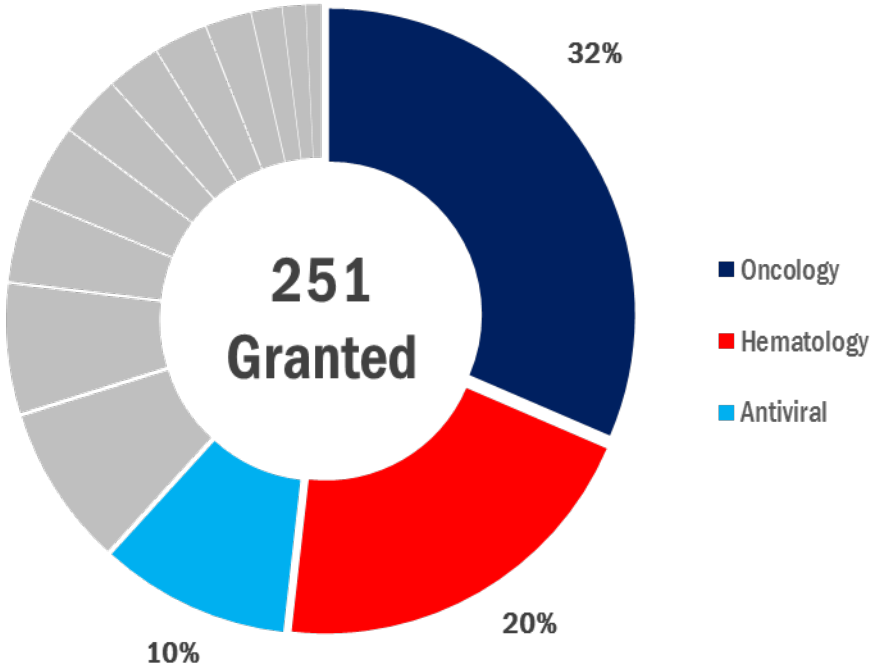
**Of 663 BT Requests CDER issues a BT Grant about**

**38%**

**of the time**

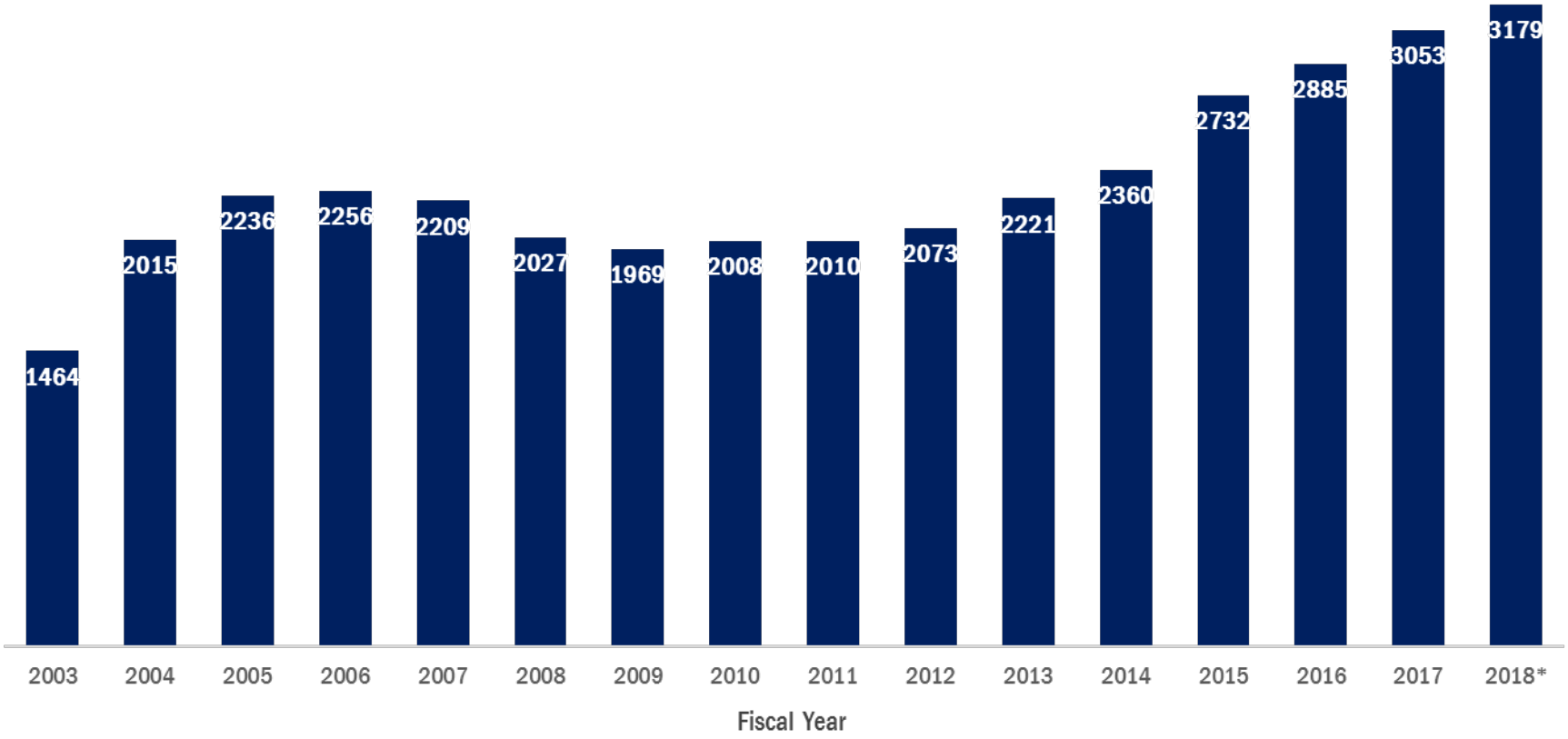



**Oncology, Hematology, and Antiviral account for the majority of Breakthrough Grants.**




Data as of 11/30/2018

# CDER PDUFA
# Formal Meeting Requests by FY




Data as of 9/30/2018

BLAs were transferred to CDER in FY2004

*2018 Data is preliminary

# Looking ahead to 2019

- FDARA Section 903 Real Time Reporting:
  - focuses on streamlining and improving consistency in performance reporting and requires the FDA to provide Real Time reporting related to the process for the review of human drugs and biologics, medical devices, generic drugs, and biosimilar biological product.
  - Report contains data on the number and title draft and final guidance; and the number and titles of public meetings held on topics related to the process for the review of human drug applications.

  https://www.fda.gov/RegulatoryInformation/LawsEnforcedbyFDA/SignificantAmendmentstotheFDCAct/FDARA/ucm598050.htm
- Continued implementation of PDUFA VI agreements, other aspects of FDARA and 21st Century Cures
- Continued ongoing critical evaluation of new drug regulatory program operations to ensure that we can meet program demands *and* our public health obligations to the American people
- Keep up with CDER NME Approvals and our year end report:
  - https://www.fda.gov/drugs/developmentapprovalprocess/druginnovation/ucm592464.htm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