Implications for Managed Care Pharmacy from the FDA Reauthorization Act of 2017

August 15, 2017

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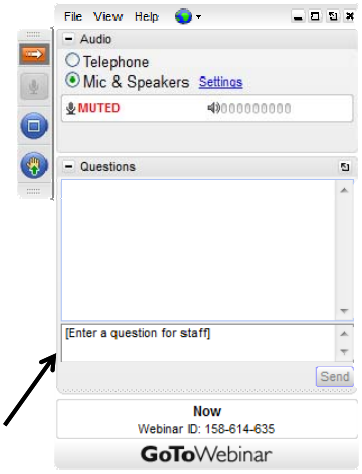
How to Ask A Question



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Agenda

What is FDARA & Why Does it Matter to AMCP?

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The User Fee Reauthorization Process

Key Provisions & Implications to Managed Care Pharmacy

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Question & Answer

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What is FDARA & Why Does It Matter to AMCP?

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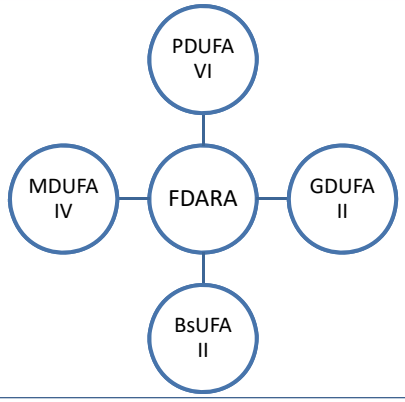
What is FDARA?

- H.R. 2430 – The Food & Drug Administration Reauthorization Act (FDARA) of 2017
  - Revises and extends the user-fee programs for prescription drugs, medical devices, generic drugs, and biosimilar biological products through 2022

What is FDARA?



"The user fee programs – GDUFA, PDUFA, BsUFA, MDUFA – may look and sound like wonky acronyms, but they are critical to patients, drug and device manufacturers, and the millions of Americans who work to deliver new treatments and cures to Americans."

– E&C Subcommittee on Health Chairman Rep. Michael C. Burgess, M.D. (R-TX) and Vice Chairman Brett Guthrie (R-KY)

History of the User Fee Acts & Success To Date

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History of the User Fee Acts

- 1980's – Pre-PDUFA
  - Major backlogs
    - Average review time for NDA or BLA was greater than 29 months[1]
    - "Drug lag" – products approved in Europe years prior to approval in US[2]
    - Estimated that a one-month approval delay cost manufacturers an average of $10 million per month[3]
    - Delays in patient access to novel therapies
  - Understaffed FDA
  - AIDS epidemic

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History of the User Fee Acts

- 1992 – PDUFA I[4]
  - Goal - to expedite the drug approval process by increasing FDA funding for reviewing applications
  - Established three fees:
    - Application review fees
    - Establishment fees
    - Product fees
  - Established performance goals for the FDA
    - Review 90% of priority applications within 6 months
    - Review 90% of standard applications within 12 months

History of the User Fee Acts

- 1997 – PDUFA II[5]
  - Established tighter performance goals for the FDA
    - Review 90% of priority applications within 6 months
    - Review 90% of standard applications within 10 months
  - Established additional interaction requirements between the FDA and manufacturers during drug development
  - Established scientific advisory committees
  - Expanded use of fees to Investigational New Drug (IND) applications

History of the User Fee Acts

- 2002 – PDUFA III[6]
  - Expanded ability of FDA to use fees on post-market and pre-clinical activities
  - Established first-cycle preliminary reviews
  - Empowered biotech reviews
  - Established the Medical Device User Fee Act (MDUFA)

History of the User Fee Acts

- 2007 – PDUFA IV[7]
  - Established the REMS program
  - Addressed conflicts of interest for advisory committee members

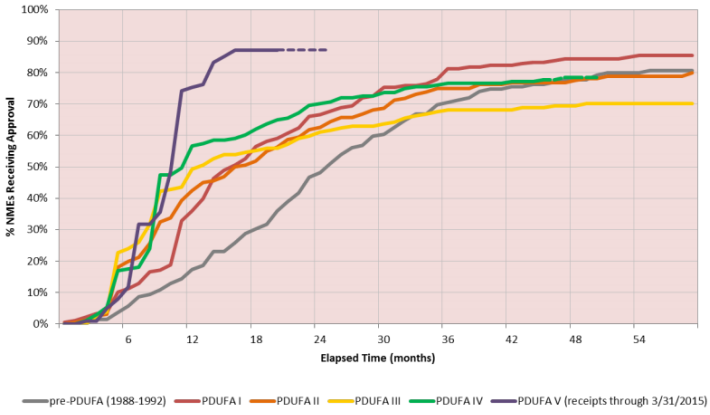
History of the User Fee Acts

- 2012 – PDUFA V[8]
  - Established breakthrough therapy designation
  - Established the Patient Focused Drug Development program
  - Extended market exclusivity for pediatric products
  - Established the Generic Drug User Fee Act (GDUFA) and Biosimilar User Fee Act (BsUFA)

Success of the User Fee Acts[9]

CDER New Molecular Entity Approval Rates by PDUFA Cohort

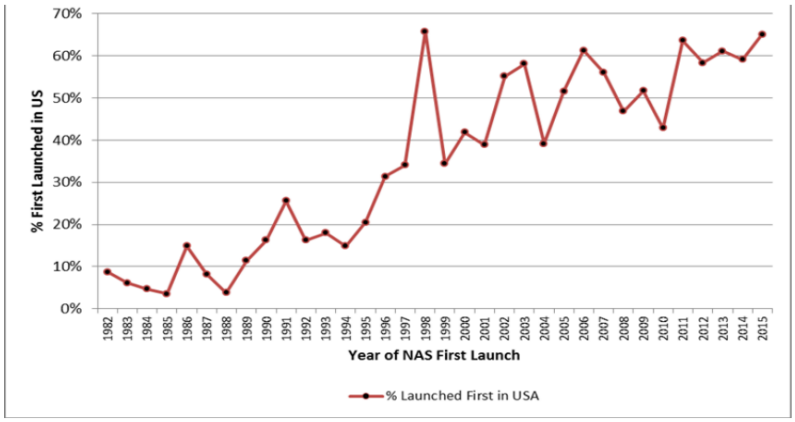
FDA



* PDUFA V estimates based on 77 NMEs submitted in FY 2013 – mid FY 2015 (it is too early to estimate performance for later submissions)
Projection estimates account for actions to date and elapsed time to date for non-approvals
Data as of 9/30/16

Success of the User Fee Acts[9]

USA Share of New Active Substances Launched on World Market

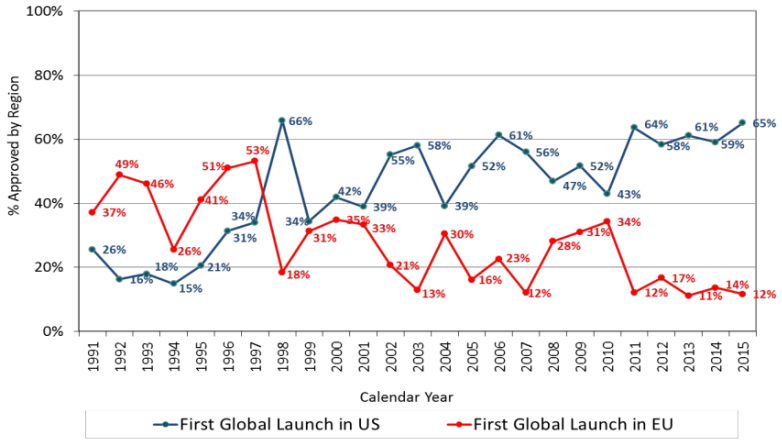


Data as of 11/30/2016

Source: *Scrip Magazine* (1982 - 2006), *Pharmaprojects/Citeline* **Pharma R&D Annual Review** (2007 - 2016)

Success of the User Fee Acts[9]

Global New Active Substance First Launches in the USA and EU: 1991 – 2015



Source: *Scrip Magazine* (1992 - 2006), *Pharmaprojects/Citeline* **Pharma R&D Annual Review** (2007 - 2016)

The User Fee Reauthorization Process

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The User Fee Reauthorization Process[10]

	SEP 2015 – MAR 2016 **FDA-Industry Discussions**	APR 2016 – SEP 2016 **Clearance Process**	OCT 2016 – SEP 2017 **Legislative Process**
5/13/15 Federal Register notice for initial public meeting **7/15/15** Public meeting **8/15/15** Docket closes; FDA analyzes comments	**SEP 2015** Initiation of FDA/Industry technical negotiations And FDA/Stakeholder consultation meetings **MAR 2016** Finalization of draft PDUFA VI Performance Goals Letter	**APR 2016** Submit draft PDUFA VI Performance Goals Letter for HHS clearance **JUN 2016** HHS clearance **JUN 2016** Submit draft PDUFA VI Performance Goals Letterer for OMB clearance **SEP 2016** OMB clearance **SEP 2016** Federal Register notice for final public meeting	**OCT/NOV 2016** Final public meeting **1/15/17** Transmit Proposed PDUFA VI Performance Goals Letter to Congress **9/30/17** PDUFA V Expires

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The User Fee Reauthorization Process

- Challenges to the passage of FDARA
  - Change in administration
  - Presidential budget blueprint
  - "Clean PDUFA"
  - Delayed timelines
  - Focus on health care reform

The User Fee Reauthorization Process

- Amendments considered
  - Importation
  - Right to try
  - Opioids
  - Off-label communications
  - Drug pricing
  - Competitive generic therapies

The User Fee Reauthorization Process

- 7.12.2017 - House passed FDARA by voice vote
- 8.3.2017 – Senate passed FDARA without amendments 94-1
- 8.7.2017 – Presented to President

Key Provisions & Implications for Managed Care Pharmacy

Key Provisions – PDUFA VI

- Enhancing Use of Real-World Evidence for Use in Regulatory Decision-Making
  - FDA commits to convening public workshops to understand issues related to RWE, initiating pilot programs, and publishing draft guidance on how RWE can contribute to the assessment of safety and effectiveness in regulatory submissions by the end of FY 2021

Key Provisions – PDUFA VI

- Enhancing the Incorporation of the Patient's Voice in Drug Development and Decision-Making
  - The FDA commits to strengthening staff capacity to focus on this issue and publish a series of draft guidance documents to allow for meaningful assessment of PROs

Key Provisions – PDUFA VI

- Advancing Postmarketing Drug Safety Evaluation Through Expansion of the Sentinel System and Integration into FDA Pharmacovigilance Activities
  - FDA commits to augmenting the quality and quantity of data available via Sentinel through expansion of data sources, enhanced communication with sponsors, and evaluation of additional ways for sponsors and the public to conduct safety surveillance

Key Provisions – PDUFA VI

- Advancing Modern Drug Development
  - Enhances the FDA's biomarker qualification pathway
  - Facilitates the use of innovative clinical trial approaches
  - Advances model-informed drug development (MIDD)
  - Enhances capacity to review complex innovative designs
  - Supports the breakthrough therapy designation pathway

Key Provisions – GDUFA II

- Addresses small business concerns

- Creates a competitive generic therapy pathway
- Studies opportunities to improve the number of first-cycle approvals for generic drug applications

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The Future of the User Fee Acts

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The Future of the User Fee Acts

- Are the user fees here to stay?
- Potential for additional user fee acts in the future?
  - OTC User Fee Act (ODUFA)

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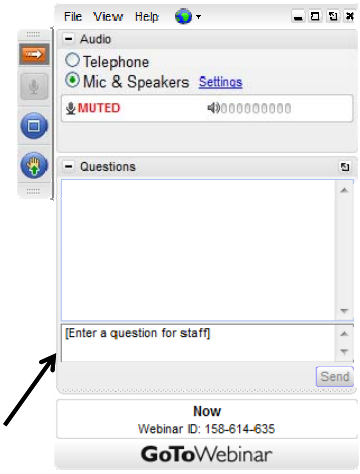
Questions & Answers

How to Ask A Question



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