Prescription Drug User Fee Act (PDUFA) Webinar

PDUFA Webinar Outline

1. **PDUFA Program Overview**
   Theresa M. Mullin, PhD
   Associate Director for Planning and Informatics (CDER)
2. **PDUFA Activities in Drug Development**
   Robert Yetter, PhD
   Associate Director for Review Management (CBER)
3. **FDA Drug Review in PDUFA IV**
   John K. Jenkins, MD
   Director, Office of New Drugs (CDER)

Pre-PDUFA – timeliness of drug review was a big concern

- **Problem**
  - FDA was understaffed; drug review was slow and unpredictable
  - Median approval time for a standard application was nearly two years
- **PDUFA**
  - User fees added resources for more review staff to eliminate the backlog of overdue applications and improve review timeliness
  - FDA agreed to meet specific performance goals
  - Under the program FDA is provided funding through user fees to assure that appropriate resources are brought to the task of review.
- **Result**
  - A streamlined, more predictable process
  - Reduced review and approval times

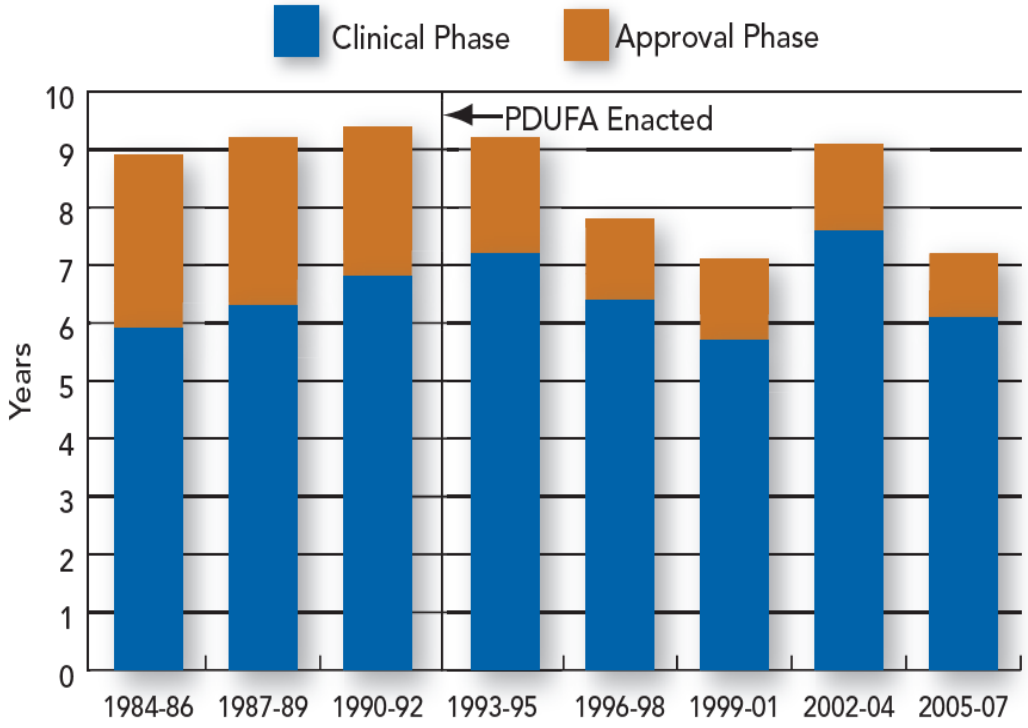
Since 1992, PDUFA has expanded in fee funding and performance commitments

- **PDUFA I (FY 1993-1997)**
  - Funding for pre-market review; eliminate NDA/BLA backlog
  - Introduce performance goals to shorten review time for standard and priority NDAs/BLAs, efficacy and manufacturing supplements, and resubmitted applications
- **PDUFA II (FY 1998-2002)**
  - Significant increase in goal commitments – meeting requests, dispute resolution, review of special protocol assessments (SPAs)
  - Small increase in fee funding

PDUFAs III and IV expanded fee funding and performance commitments

- **PDUFA III (FY 2003-2007)**
  - Significant base increase in fee funding and inclusion of workload adjuster for fee calculations
  - Expanded FDA-sponsor interactions during 1st review cycle – Good Review Management Principles and Practices (GRMPs)
  - New but limited support for post-market risk management
- **PDUFA IV (FY 2008-2012)**
  - Further increase in base fee funding and enhancement of workload adjuster
  - Congress increased fee funding for drug safety
  - New guidances for innovative clinical trial design
  - FDAAA Titles IV, V and IX added new process requirements

PDUFA's Effect – Review and approval time has dropped nearly 60%



Source: "Outlook 2010." Tufts Center for the Study of Drug Development. Tufts University, 2010.

Clinical development timelines have dropped by about 10% since PDUFA was enacted.

PDUFA has significantly increased patient access to new drugs and biologics

- **From FY1993-2009, FDA approved over 1,000 NDAs and 100 BLAs, including:**
  - Over 90 new cancer drugs
  - 139 drugs for metabolic and endocrine disorders
  - 125 anti-infective drugs
  - 138 drugs for neurologic and psychiatric disorders
  - 106 drugs for cardiovascular and renal disease

PDUFA – How Does It Work?

- Fee funds are added to appropriated funds and are intended to increase staffing and other resources to speed and enhance review process
- User fees pay for services that directly benefit fee payers
- As part of the PDUFA program, FDA has made performance commitments
  - Performance commitments are focused on improving the process of human drug review
  - FDA commitments do not include review policy issues

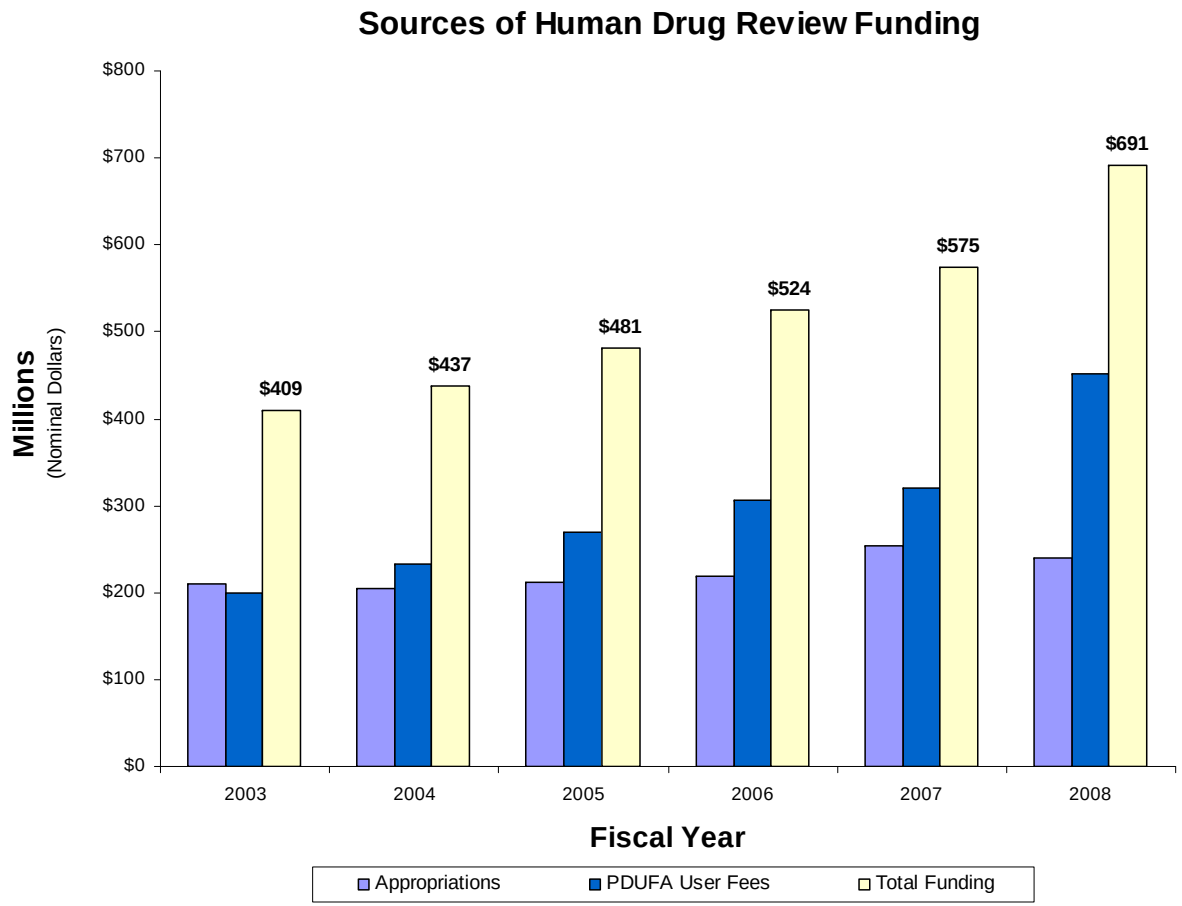
PDUFA Fee structure

- Total fee amounts are collected equally from 3 sources
- Fee revenue amounts for FY 2010

Application fees	$189,736,000
Product fees	$189,736,000
Establishment fees	$189,736,000
Total	$569,207,000

- Statutory criteria for collecting and spending fees
  - FDA's overall Salaries and Expenses Appropriation (excluding user fees) must meet or exceed FDA's overall FY 1997 Salaries and Expenses Appropriation
  - The amount of user fees collected in each year must be specified in Appropriation Acts
  - FDA must use a specified minimum amount of appropriated funds for the review of human drug applications [to which user fee funds are added].

User fees fund a major share of human drug review program costs



FY2008 - 65% of human drug review funding now from user fees.

PDUFA V Introduces new reauthorization requirements

- **Congress directed FDA to consult a wide range of stakeholders prior to the next reauthorization**
  - Scientific and academic experts
  - Health care professionals
  - Patient and consumer advocacy groups
  - Regulated industry
- **Reauthorization discussions will begin with public input**
  - Public meeting and written docket submissions
- **Process will include negotiations with industry and regular meetings with patients and consumer groups to continue discussions of their views**
- **After negotiations are finished, FDA will publish minutes of negotiation meetings on our Website and we will hold a public meeting on the proposed recommendations**
- **FDA will consider the public views and comments and revise recommendations as necessary**

Anticipated Timeline

Some of the key activities include:

- **2010**
  - April: Public meeting, open public docket for written comments
  - May: Analyze docket comments
  - June—December: Hold discussions with industry and other public stakeholders
- **2011**
  - January: Target completion of industry and other stakeholder discussions
  - February – September: Administration review of proposed draft recommendations
  - September – November: Public review of proposed draft recommendations
  - December: Administration review and clearance of final proposed recommendations
- **2012**
  - January 15: Transmit final recommendations to Congress

PDUFA Reauthorization process is strengthened by public input

- The PDUFA program is strengthened by the opportunity for public input
- The PDUFA-funded process of human drug review now includes both pre-market review and post-market drug safety
- The PDUFA stakeholder discussions are not about policy, but FDA is very interested in hearing the public's views on how the process could be further improved

Next – PDUFA Activities in Drug Development