



Operation of the Prescription Drug User Fee Program

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What Is PDUFA Paying For?

- ◆ “Process of Review of New Human Drugs”
- ◆ Covers drugs and biologics
- ◆ “Process” Definition describes “covered activities”
 - Activities eligible for support from fee program

"Process" Activities: Investigational Phase

- ◆ Decide whether safety data and safeguards are adequate to start human trials
- ◆ Set standards for safety evaluation at each stage of development (e.g., what testing is done)
- ◆ Set requirements for dose finding
- ◆ Set standards for trial designs and endpoints for approval for any given indication

"Process" Activities: Investigational Phase

- ◆ Interact with and advise developers on trial design, safety monitoring, trial endpoints to avoid unnecessary trials and exposure of volunteers

- ◆ Review adverse events occurring in clinical trials; place clinical holds or trial modifications

- ◆ Oversee clinical research (BiMo Program)
 - IRB inspections and standards/policy
 - Inspect clinical trials and set standards for trial conduct (GCPs)

"Process" Activities: Market Application Phase

- ◆ Review manufacture, controls and testing
 - Inspect plants
 - Review packaging

- ◆ Review animal toxicology
 - Reproductive toxicity
 - Carcinogenicity

- ◆ Review clinical data
 - Safety data (including drug name)
 - Efficacy data

- ◆ Review and agree upon drug label

"Process" Activities: Peri-approval Phase

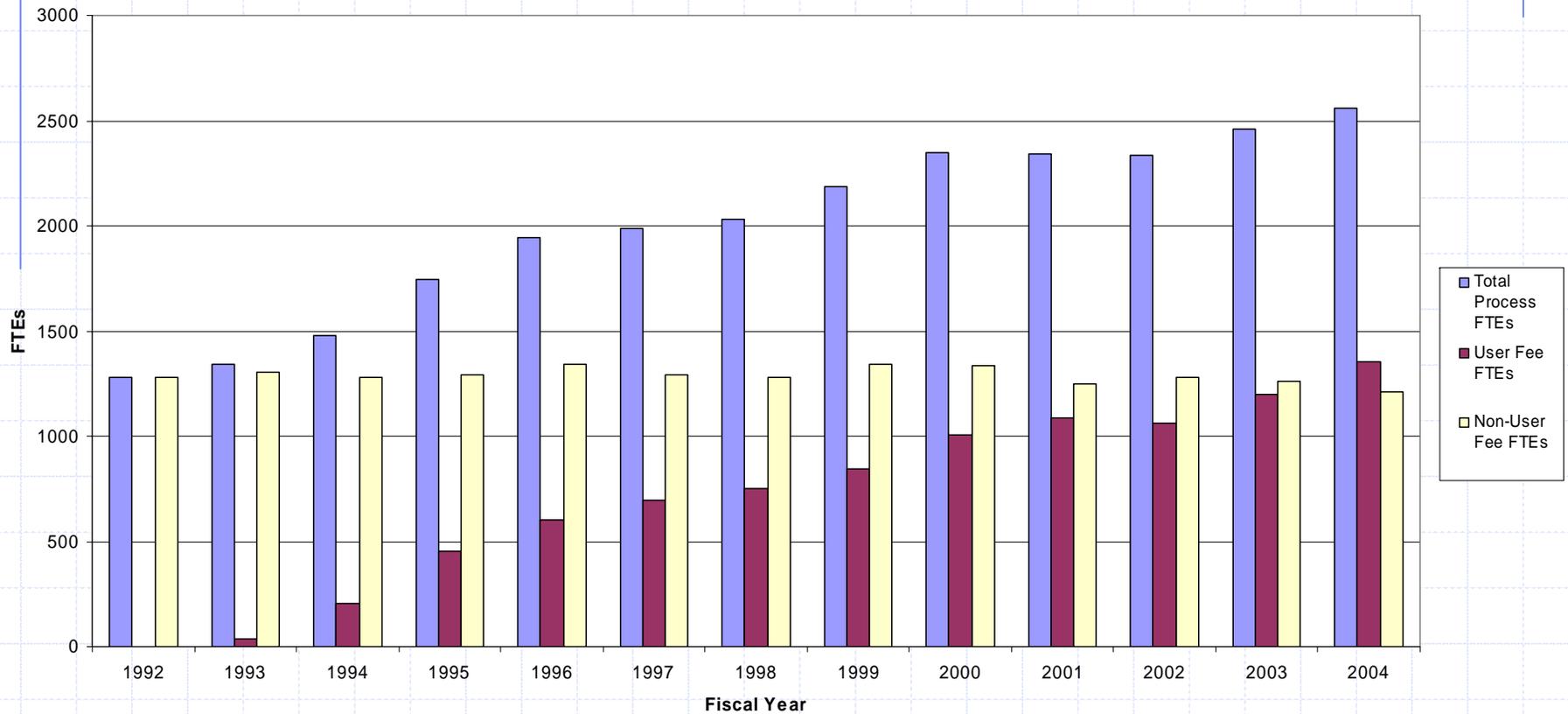
- ◆ Hold public Advisory Committee Meetings
- ◆ Evaluate risk management plans
- ◆ Agree on Phase 4 commitments
- ◆ Review Phase 4 studies & results

Question: Which of These Activities DON'T We Want FDA to Do?

- ◆ Wide support for FDA carrying out each of these activities
 - Most stakeholders calling for more FDA work in their area of interest—i.e. drug safety, improving development process for products for unmet medical needs, oversight of clinical trials
 - Each of these activities require adequate scientific staff with appropriate support (IT and staff)

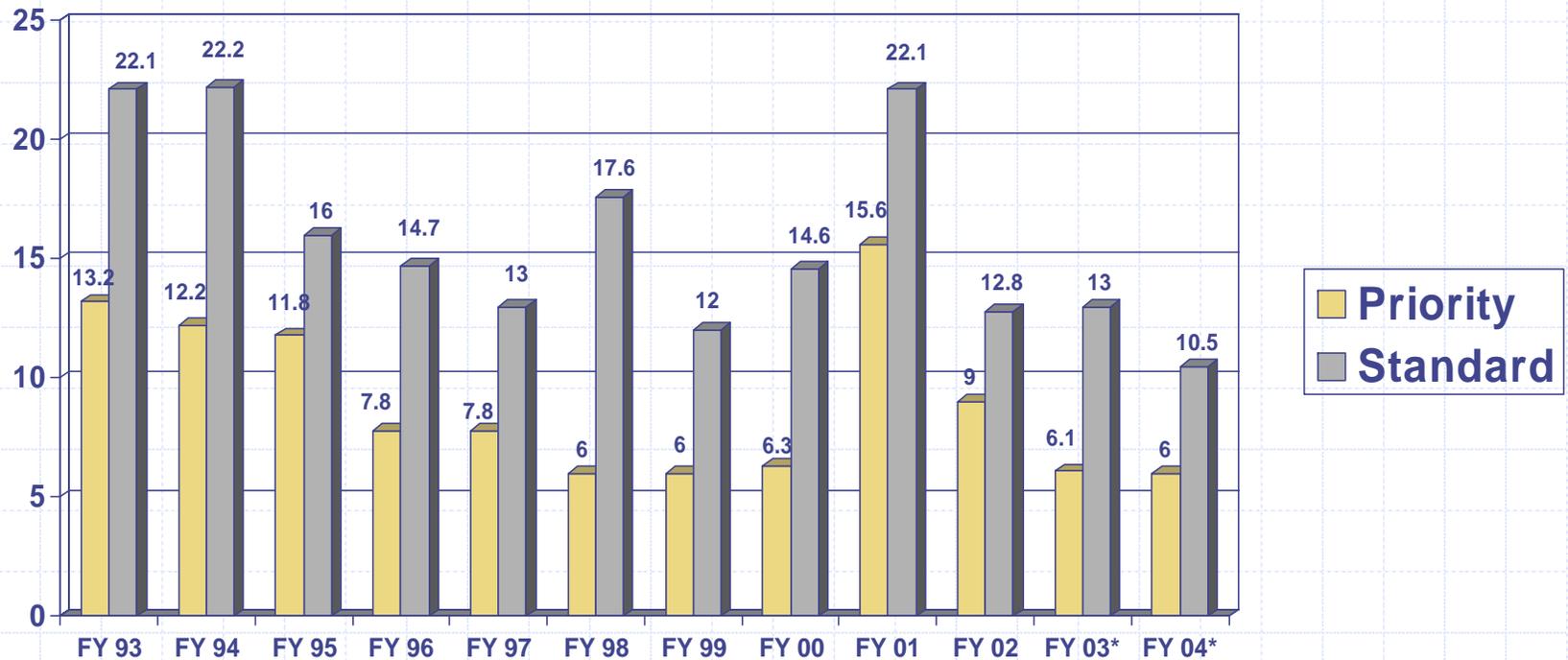
PDUFA Fees Enabled FDA to Increase Staffing & Systems for Drug Review Process

History of PDUFA Total Process and User Fee Funded FTEs



With More Staff & Better Managed Process FDA Reduced Review Times

Median Approval Time for NDAs and BLAs by Year of Receipt (months)



Result: Significant Increase in Patient Access to New Drugs and Biologics

- ◆ Since 1993 FDA has approved 999 NDAs and 96 BLAs

More Staff and Better Managed Process Have Resulted in Increased FDA Application Scrutiny and Better Articulation of FDA Standards

- ◆ Reviewer Guidance – Conducting a Clinical Safety Review of a New Product Application and Preparing a Report on the Review (85 pages)
- ◆ Review Templates in each discipline
- ◆ Large number of Guidances on important topics – e.g., dose response, etc.
- ◆ Reorganization of review function (e.g., cancer review, manufacturing review)

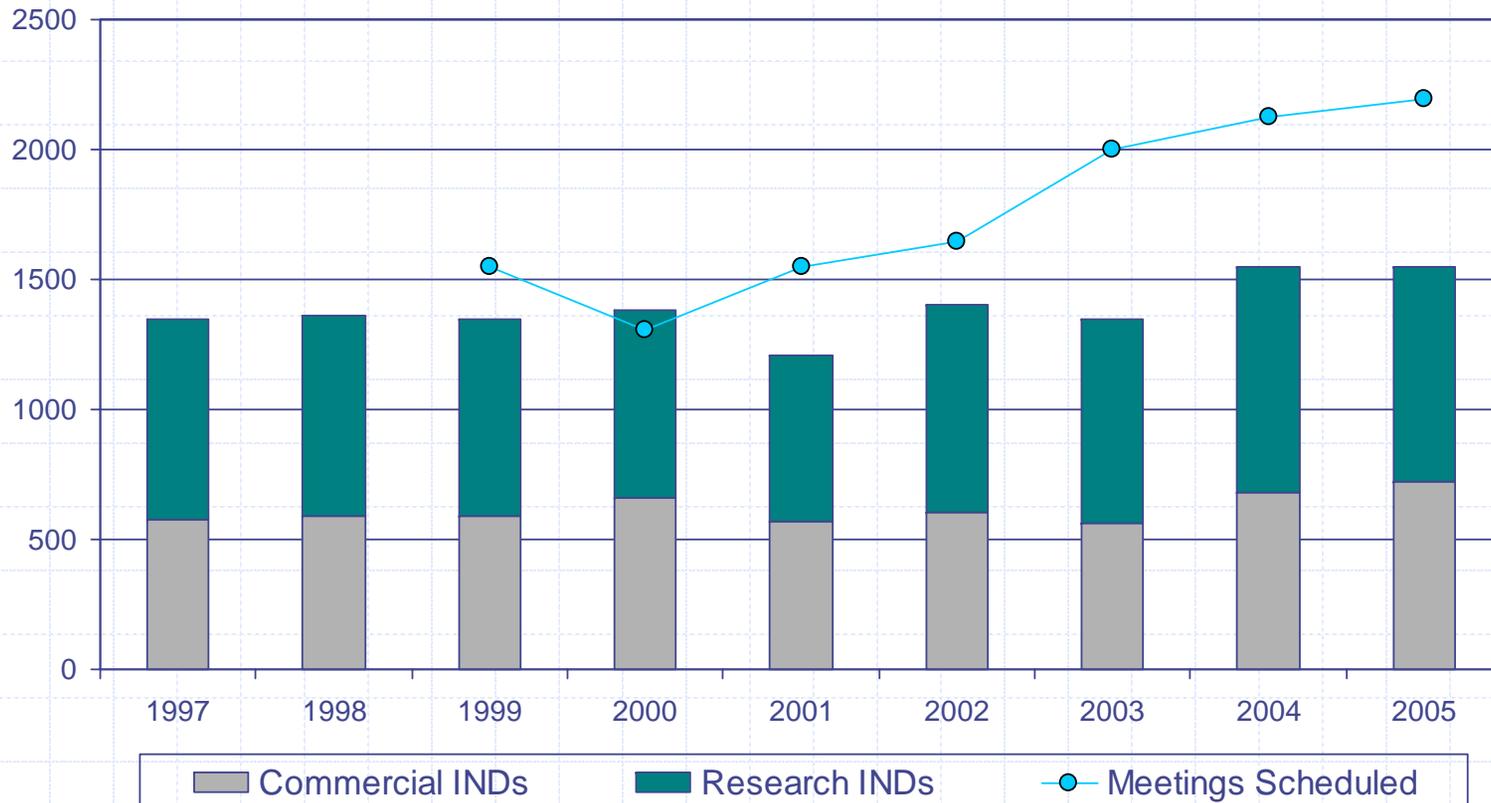
PDUFA Has Also Provided Value for Industry

- ◆ New molecular entity (NME) review reduced by average of 12.4 months under PDUFA
- ◆ Reducing NME review by 12 months saves a company \$30M in R&D costs
 - In FY04 FDA approved 33 NMEs and new BLAs resulting in estimated industry saving \$1.2B in FY04
 - In FY04 industry paid \$246M in user fees
- ◆ Recent survey by MIT researchers found that consultation with FDA during development is highly valued, e.g.,
 - 70% surveyed willing to pay \$100k-\$500K for additional communications w/ FDA in Phase I

Current Challenges for PDUFA Program

◆ Workload

Fee Adjuster for Workload Does Not Reflect Growth in Non-application Work

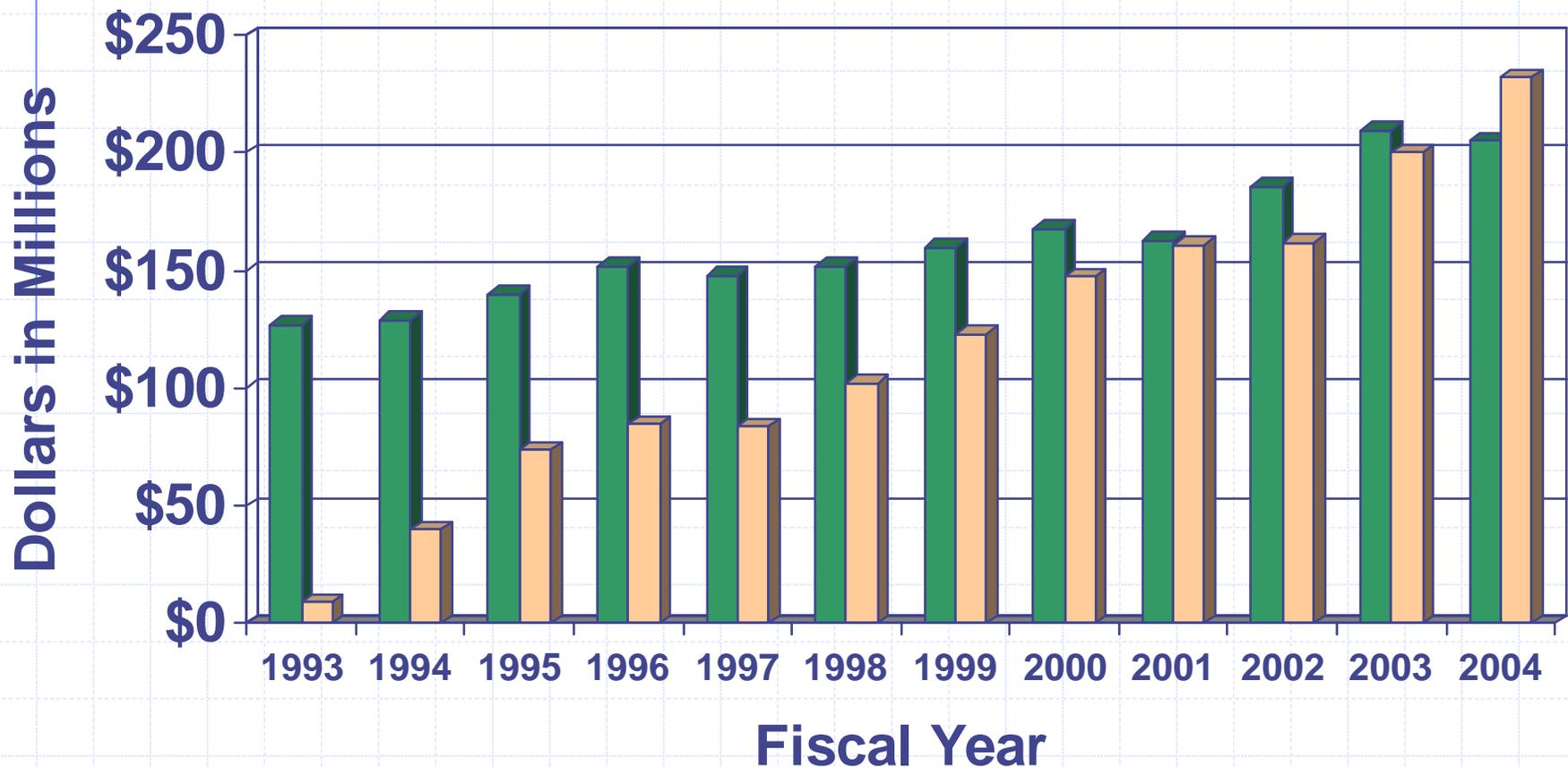


Additional Challenges for FDA in Drug Review Process

- ◆ IT Infrastructure and Bioinformatics
- ◆ DTC Advertising Regulation
- ◆ Oversight of Clinical Trials
- ◆ Preparing for the impact of new science –
Critical Path

Fees Now Outpace Appropriated Funds

Trend in Spending by Source of Funds



■ Appropriations ■ Fees