Overview of FDA Support for Innovation

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Disclosure Statement

I have no financial relationships with proprietary entities that produce health care goods and services

The opinions and information in this presentation are my own and do not necessarily reflect the views and policies of the FDA

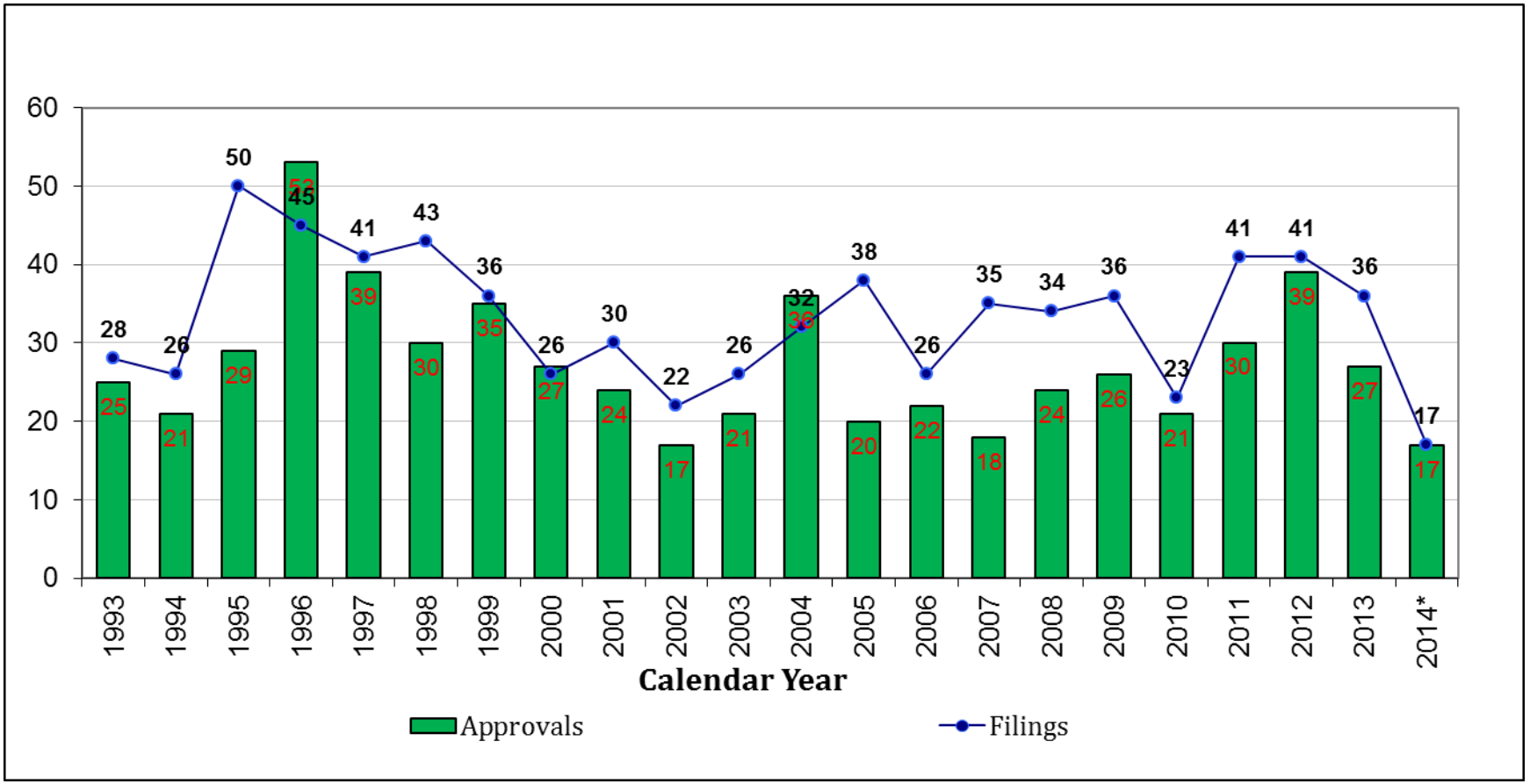
Outline

- Importance of innovation for TBI
- FDA role in supporting innovation
- Power of consortiums in innovation

FDA Challenge

- Patients and Caregivers want:
  - Rapid access to safe and effective new drugs
  - Better information about how to use these drugs after approval
- Inefficient medical product development:
  - Is failing to keep pace with the new scientific discoveries
  - Is delaying access to new innovations and limit information on appropriate use of approved drugs

CDER NME NDAs/BLAs† Filings and Approvals



Data as of 6/30/2014

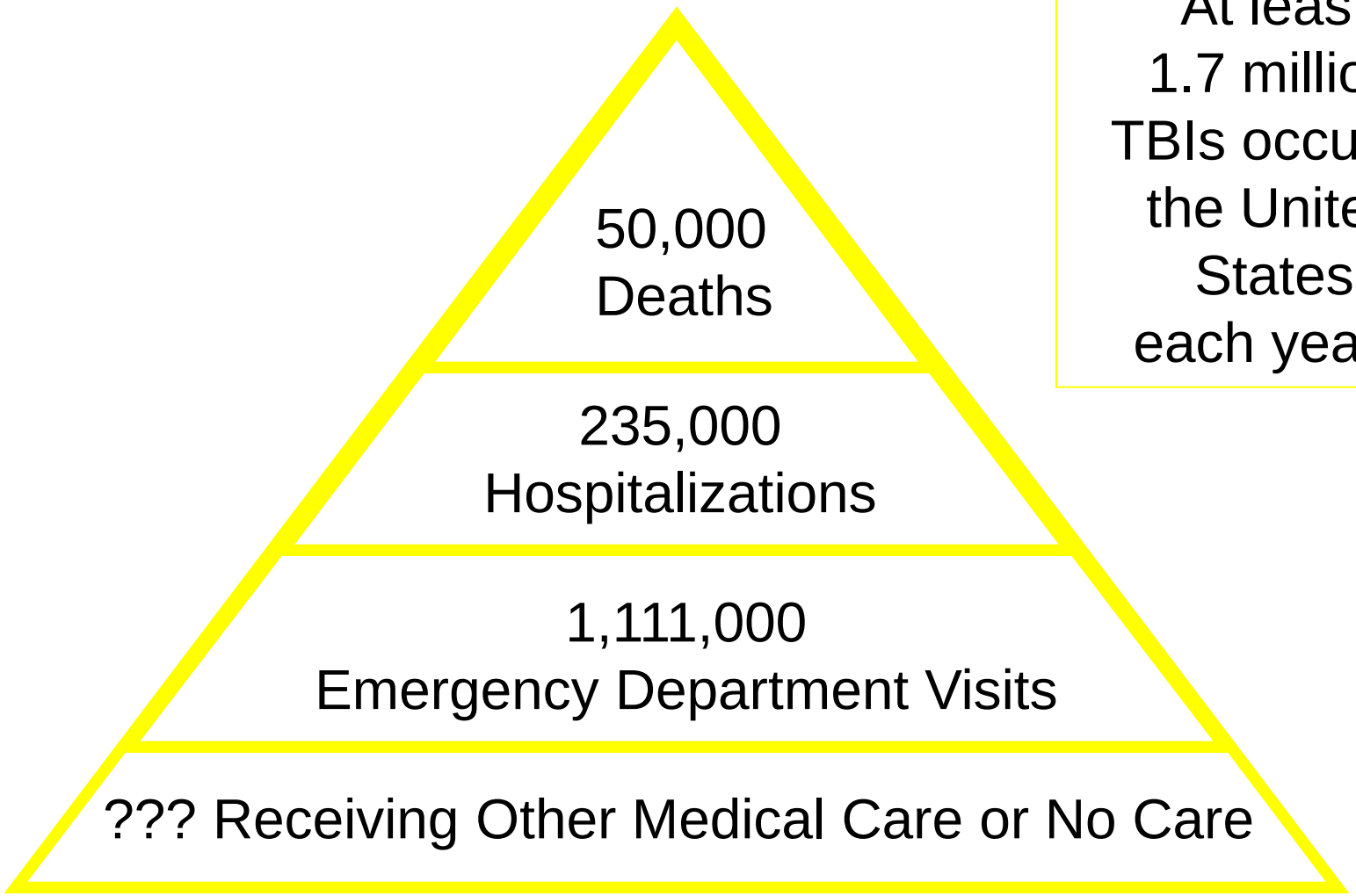
† Multiple applications pertaining to a single new molecular/biologic entity (e.g. single ingredient and combinations) are only counted once. Therefore, the numbers represented here for CY14 filings are not indicative of workload in the PDUFA V Program.

† Original BLAs that do not contain a new active ingredient are excluded

*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.

Importance of Innovation in Treatments for Traumatic Brain Injury (TBI)

Challenge of TBI in the United States



At least 1.7 million TBIs occur in the United States each year.*

Challenge of TBI (cont)

- TBI is a complex condition (not an ‘event’)*
- New tools promise better differentiation of patients and responses to treatment
- Traditional classification schemes are based on symptoms and may be insensitive to mechanistic targeting using new imaging and diagnostic tools.
- Data standards needed

*--Manley and Maas, JAMA (2013) 310:473

FDA Role in Supporting Innovation

FDA's Role In The Science of Drug Development

- **Develop infrastructure and tools for product development (not focus on development of specific products but rather areas of need)**
- **Encourage collaborative efforts among government, academia, industry, and patient groups**
- Develop relevant data standards and regulations
- Build support for relevant academic science
- Create opportunities to share existing knowledge and databases

Critical Targets: Drug Development Tools (DDTs)

Gains for Use of DDTs

- High potential to reinvigorate drug development and improve efficiency of development
  - Earlier information about benefits and risks
  - Consistent data collection across studies
  - Reduce the need for clinical data

Challenges to Developing DDTs

- Time, money, people….
- Progress needs
  - Focus on science that will make a difference
    - Multiple views can be taken into account
  - Process that works
    - Mechanism to support a balanced collection and review of available data
    - Mechanism to support appropriate transparency
    - Example: CDER DDT Qualification Process
  - Champion
    - Collaboration….

Critical Additional Element of Success: Collaboration

Power of Collaboration

- It's the most efficient game in town
  - Multiple stakeholders with multiple needs
    - No single company, university, or governmental agency will have sufficient resources, expertise, or information bas to undertake the work.
  - Builds consensus, expanding use
  - Many examples of success of collaboration
    - PCAST report calls for it,
    - IOM is applying it, work on clinical trials certification
    - FDA is applying it in a variety of situations

Power of Collaboration (cont)

- FDA has experience in appropriate ways for government to partner…
  - Transparent, open, inclusive, rigorous
  - Results broadly applicable, maximally transparent, for maximum value
- FDA is highly supportive of groups looking to form collaborations to support innovation

Conclusion: Dr. Woodcock's Advice

- "…MS community needs to build on current foundations by applying creativity to the development of modern outcome assessments that will ignite innovation in MS treatments and ultimately improve the lives of MS patients and their families."*

• Woodcock, J and Rowzee, A.M., Multiple sclerosis outcome assessment consortium: bringing the community together to shape the future of multiple sclerosis drug development. *Therapeutic Innovation and Regulatory Science* (September, 2013).


WARNING
CHALLENGES
AHEAD