Reauthorization of the Prescription Drug User Fee Act Public Meeting

Biotechnology Innovation Organization

PDUFVA VII Priorities – Patient-Centric & Effective

- COVID-19 Lessons Learned
- Hiring & Resource Management Accountability
- Modernize Regulatory Evidence Generation
- Innovative Review Approaches
- Complex Innovative Trials Designs
- Patient-Centric Drug Review
- Advancing Digital Health Technologies
- Data & Technology Infrastructure Modernization
- **CBER Resourcing & Review Management (Gene/Cell Therapies)**
- **Improving FDA/Sponsor Interactions**
- **NME Milestones/Post-Market Requirements**
- **Biologic Manufacturing**

Biotechnology Innovation Organization

- BIO represents more than 1,000 biotechnology companies, academic institutions, state biotechnology centers, and related organizations across the United States and in more than 30 other nations.

- BIO member companies range from small start-ups and companies with one or only a few FDA-approved products, to some of the largest biopharmaceutical companies in the world.

- These companies share a commitment to bring innovation to patients and to work at the cutting edge of technology to achieve that goal. They also share an unwavering dedication to improving health and healthcare in the face of a very high risk of failure.

The PDUFA Program Has Been An Overwhelming Success For Innovation, Access, and Public Health

- For the past 25+ years the PDUFA program has lowered review time, increased engagement between industry and sponsors, advanced regulatory science and patient centric drug development & review that has resulted in more timely access to better treatments for patients and improved the public health all without compromising FDA's high standards for safety and efficacy.

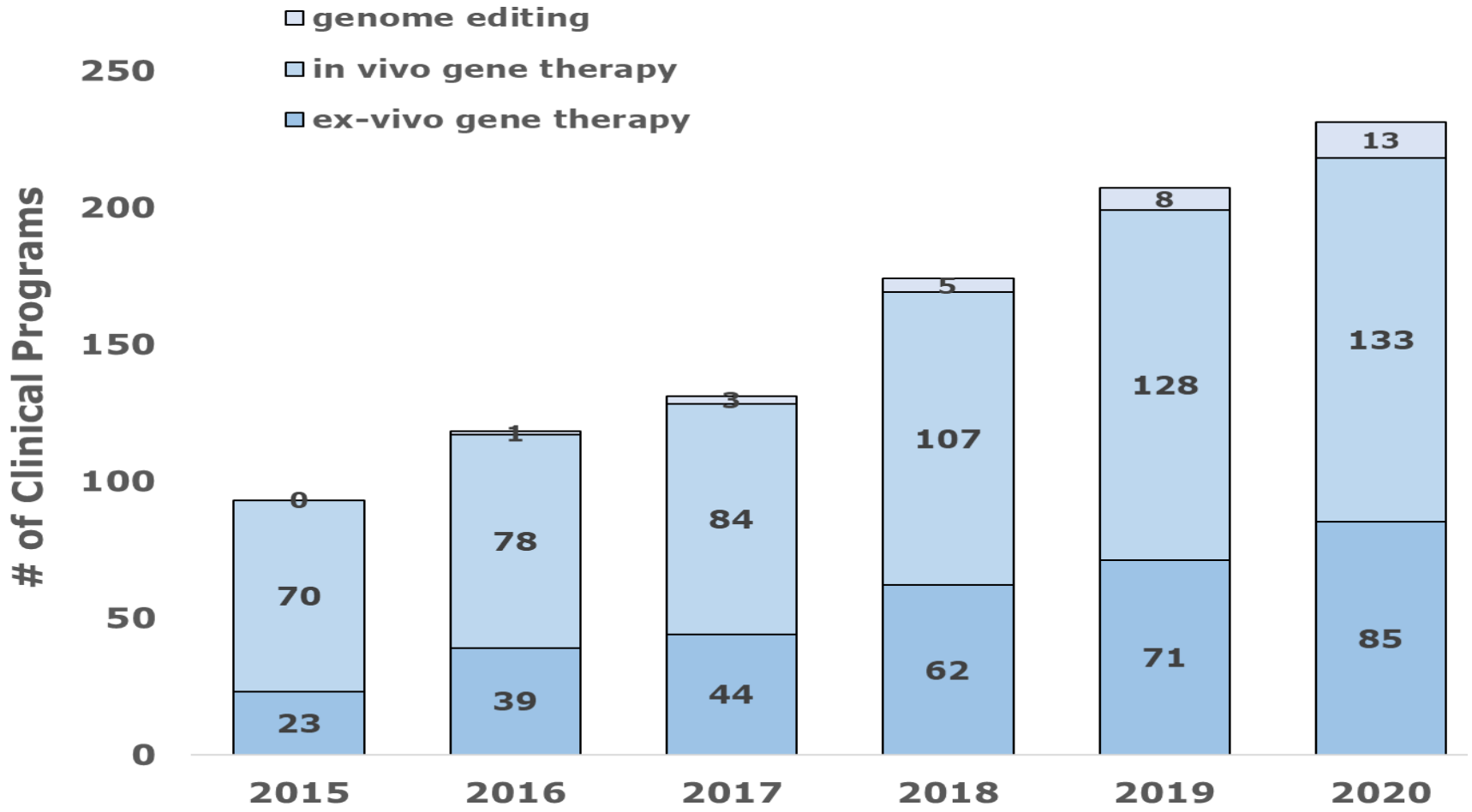
- It will be important to look at potential new areas where the program may benefit patients and the drug development & regulatory ecosystem in PDUFA VII.

- BIO looks forward to working collaboratively with FDA to ensure a timely reauthorization of this important program.

COVID-19 Response

- FDA has played a critical role in helping the country and the world respond to the COVID-19 pandemic. BIO and the biopharmaceutical industry has worked diligently with regulators and other government agencies to develop therapeutic treatments for COVID-19.
- Industry and FDA are utilizing novel approaches to digital technologies, decentralized trials, novel mechanisms to capture outcomes, facilities inspections, and supply chain security to spur innovation and inform rapid regulatory decision-making.
- PDUFA VII provides an opportunity to build upon these efforts to further enable novel approaches that have demonstrated their effectiveness and applicability to innovative drug development beyond the emergency declaration.
- BIO supports the FDA's third-party assessment of the COVID-19 response and looks forward to working with the FDA to develop new best practices & processes that are more patient-centric and effective across all therapeutic areas.

Promoting Gene Therapies and Advanced Biologics



Data Source: Informa Biomedtracker

Promoting Gene Therapies and Advanced Biologics

- The increasing wave of gene therapy products entering the pipeline threatens to outstrip CBER resources unless proactive steps are taken to ensure adequate resourcing and optimal review management practices.
- In order to ensure FDA has the resources and expertise to drive optimization of development and review processes, considerations include:
  - Provide resources and support for regulatory review and approval to advance the field, provide regulatory certainty to application review, and ultimately expand patient access to therapies for serious diseases and conditions.
  - Further clarify expedited pathway evidentiary standards.
  - Advance and standardize gene therapy technologies and manufacturing processes.

Enhancing Scientific Dialogue Between FDA and Sponsors

- Appropriate communication and early/effective engagement between the FDA and Sponsors is integral to ensuring that data on safety and efficacy is sufficient for regulatory approval.

- The increased use of innovative approaches in drug development has resulted in a greater need for earlier and more frequent interaction.

Enhancing Scientific Dialogue Between FDA and Sponsors

- Industry and FDA should work together to enable iterative, timely, and effective scientific dialogue and engagement planning related to the development and review of drug or biological drug products regulated by CDER and CBER. Considerations include:
  - Analyzing and establishing processes and best practices to improve efficiency and effectiveness of FDA-Sponsor meetings.
  - Developing an FDA-Sponsor communication plan early on in drug development and review to identify the most appropriate times for FDA-Industry engagement.
  - Establishing a mechanism for Sponsors to receive efficient follow-up and obtain answers to clarifying questions following milestone meetings to ensure clarity and improve efficiency.

Optimizing the New Molecular Entity (NME) Review Model

- In PDUFA V, FDA established the NME Review Model, which has been a great success and has led to improved performance and more first cycle approvals.
- Enhancements to the NME Review Model will ensure a more efficient and timelier review and approval of NMEs/BLAs, including:
  - Predictable communication around key issues such as labeling, post-marketing requirements (PMRs)/post-marketing commitments (PMC), and pediatric study plans prior to approval to allow FDA and Sponsors foresight to address any potential issues.
  - Establishing a mechanism for Sponsors and the Agency to re-discuss and/or re-evaluate the feasibility and/or need for a particular existing PMR/PMC as new information becomes available.

Enabling Modern Manufacturing, Quality, and Inspection Methods and Processes

- Manufacturing and quality play a vital role in the drug development process and ensuring timely access to medicines for patients. The following elements will enhance the review of drug and biologic manufacturing and enable innovative technologies:
  - Earlier communication for manufacturing supplement review to increase the efficiency and effectiveness of the first cycle review of these important submissions.
  - Facilitating the use of innovative manufacturing technologies for both products in development (new) and those that are commercially available.
  - Ensuring effective and quality manufacturing of gene and cell therapies.
  - Enabling risk-based approaches to preapproval/license inspections to create a more efficient and risk-based inspection paradigm.

Thank You

- BIO appreciates the opportunity to comment on the PDUFA VII Performance Goals and Procedures, Fiscal Years 2023 Through 2027.

- We look forward to continuing to work with FDA and other stakeholders to ensure the successful achievement of these goals.