PDUFA VII Public Meeting

July 23, 2020

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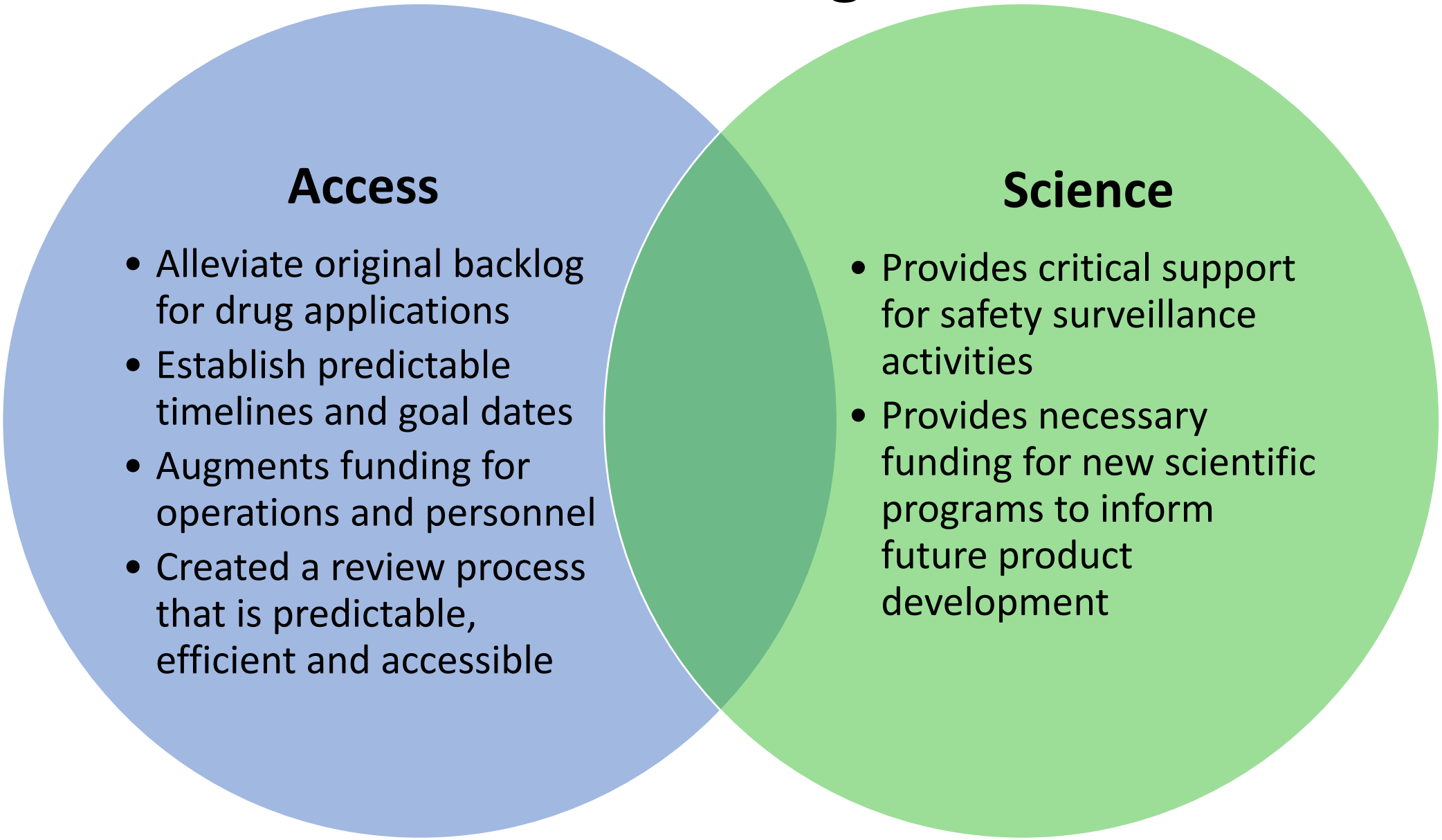
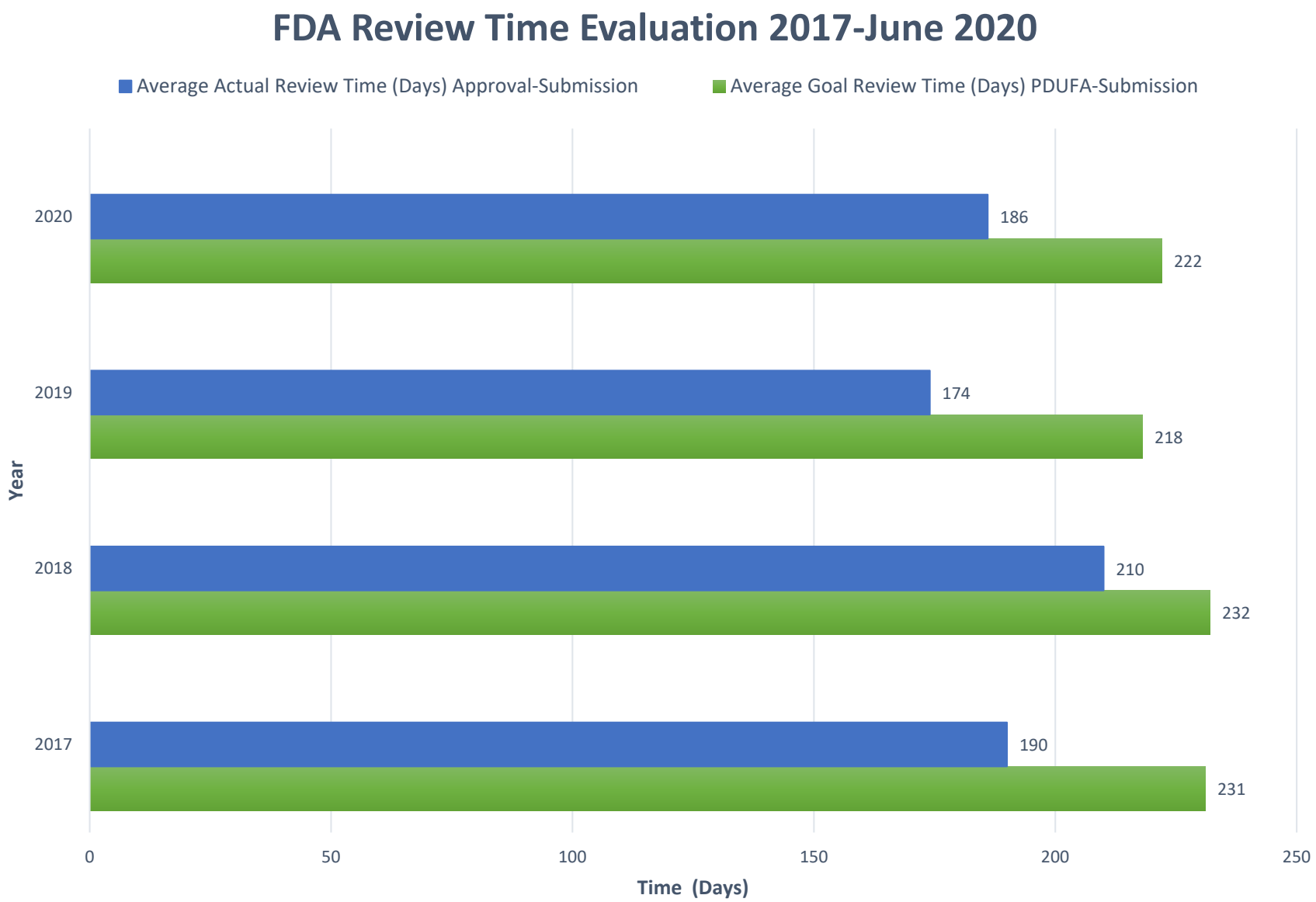
FRIENDS of CANCER RESEARCH

Benefits of the PDUFA Program



PDUFA Program Impact

Case Study: Oncology



Standard Review = 10mo (~300d)

Priority Review = 6mo (~180d)

Key Components of PDUFA VI

Advance the Role of Patients and Their Experiences

- PDUFA VI allowed FDA to facilitate public workshops, develop guidance documents, strengthen internal capacity, and establish new methods for clinical outcomes assessments and patient reported outcome measures.
  - ❖ Includes internal and externally led Patient Focused Drug Development meetings, new guidance documents, and pilot projects

Support the Continued Success of the Breakthrough Therapy Designation

- PDUFA VI provided critical resources to allow the successful Breakthrough Therapy Designation to continue to facilitate rapid access to highly promising new medicines for patients suffering from serious diseases.
  - ❖ FDA has approved 158 breakthrough therapy designated products with 372 total designations granted
  - ❖ Including ~100 designations granted since the implementation of PDUFA VI

Promote Qualification and Use of Drug Development Tools

- PDUFA VI enables processes in which new biomarkers and other drug development tools can be accurately assessed and ensure their appropriate use.
  - ❖ Holds promise for validation of new tools, animal models, and modernized approaches to development

Enhance the Development and Use of High-Quality Real-World Evidence

- PDUFA VI allowed collaboration between FDA and other stakeholders to identify limitations and explore different opportunities for the use of data collected from post-market experience with a drug
  - ❖ Development of the FDA RWE Framework and numerous other pilot projects to inform the use of RWE in different use cases

Can we learn from COVID-19?

Clinical trial design considerations may be needed

- Modifications in enrollment if stratification is needed
- Different statistical approaches to account for differences in populations may be needed
- Eligibility criteria may be affected

Expansion of process improvements designed to expedite the launch of new studies

- Shortened IRB reviews
- Preplanned modifications and amendments

Increased utilization of master protocols

- Improve the efficiency of launching trials and transition toward a research-network type approach

Routine adoption of remote services and more decentralized trials

- Remote consultations
- Sending oral medication directly to patients
- May make it easier for more patients to participate in clinical trials

Key Areas to Consider for PDUFA VII

Efficiency Pilots to Explore & Expand

- Real-Time Review
- Global Coordination

Cell and Gene Therapies

- Increasing number of therapies in the pipeline (~400) will require robust review

Manufacturing Innovation & Readiness

- Harness technological advancements to optimize drug and biologic manufacturing processes

Biomarkers / Diagnostics

- Growing number of biomarker-selected drugs would benefit from continued alignment of development processes and regulatory requirements

Individualized Drug Development

- Increased ability to identify genetic abnormalities has led to an increased capability for developing and testing potential interventions on an individual basis

Continue Progress on Real World Evidence

- Support for post-market safety surveillance and product evaluation over time