MDUFA V Reauthorization

Public Meeting

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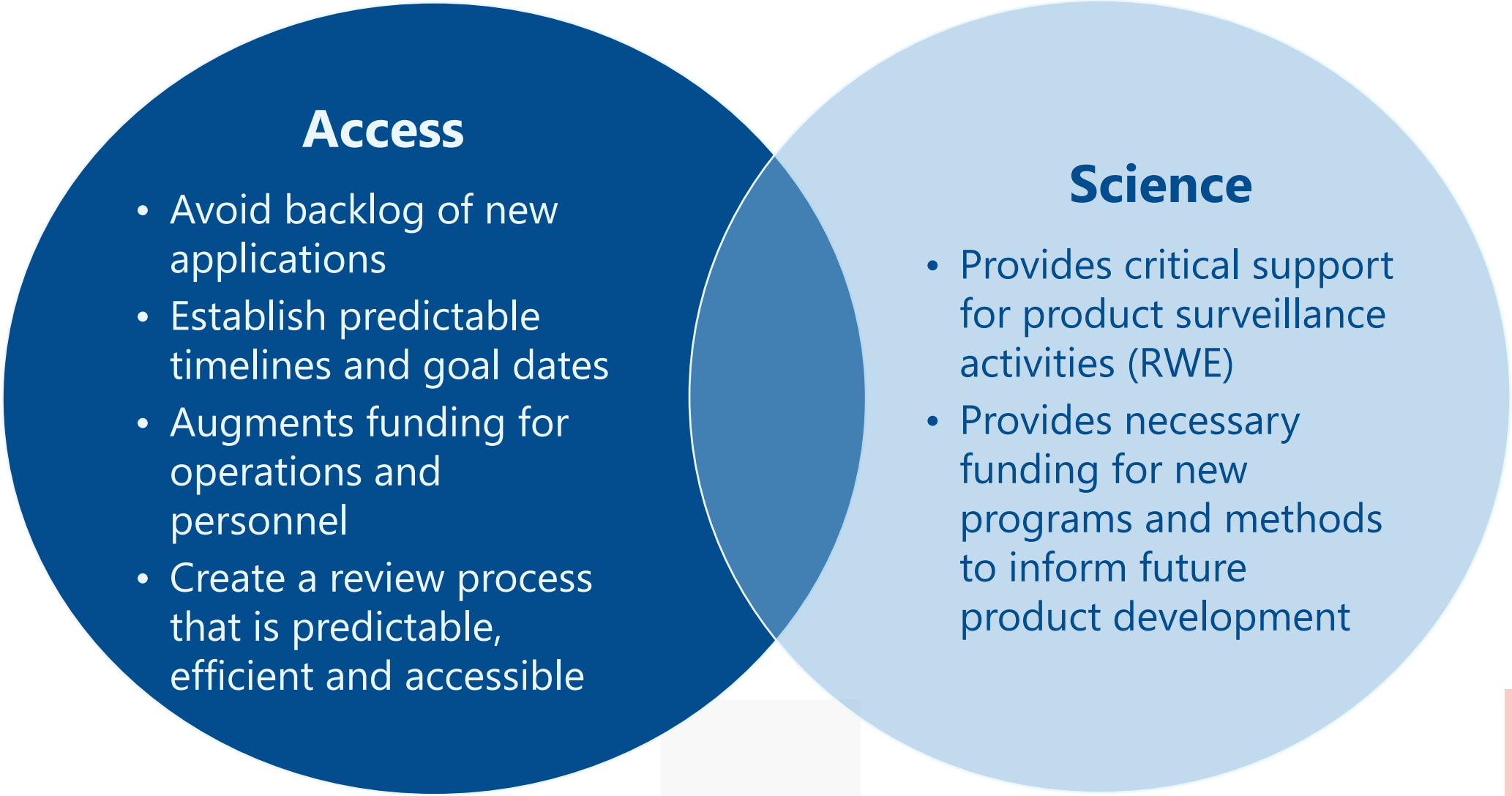
Benefits of the user fee programs



MDUFA IV - Key Programs

- **Patient Engagement**
  - Create mechanisms to obtain information about patient's experiences with technologies and underlying conditions
  - Enhance methods for measuring patient's experiences
- **Real-World Evidence**
  - Develop methodology and standards for use of RWE for identifying new uses and monitoring for malfunctions
- **Digital Health**
  - Explore the role that digital technologies may play in pre-market review
  - Consider pathway by which software could be reviewed

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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

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

Post-market performance data can provide important information about products

- Particularly in instances where pre-market processes are expedited, and initial data may be limited

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Considerations for MDUFA V

- Expand the scope and capabilities for active surveillance and use of real-world evidence to help balance patients benefits/risks
  - Help strengthen medical product safety net and allow additional pre-market flexibility
  - Support emerging technologies and accelerate the ability to efficiently address unmet medical needs
- Develop and implement digital health strategies across FDA
  - Including aspects of digital tool performance as well as clinical metrics and standards for including in clinical trials
- Sufficient support for processes associated with Breakthrough Technologies

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Considerations for MDUFA V – Oversight of DX Tests

- As of 2019, over 30 FDA approved drugs have been developed with a biomarker test associated with its use

- Diagnostic tests can be developed and sold to labs for use (FDA regulated) or they can be developed by the lab in which they are intended to be used in (not historically FDA regulated)

- ***Case Study***: Molecular testing has become a standard of care for NSCLC patients. Based on these tests - >80% of patients that test positive for an actionable biomarker (EGFR, ALK) receive the associated targeted therapy. However, ~30% of patients rely on test that are not approved/reviewed by the FDA

- This MDUFA reauthorizations provides the opportunity to identify the appropriate support for Congressional efforts to ensure that all tests have equal quality performance assurances prior to their use no matter where they are developed.

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Medical Device Regulation - 2027

A global and continuous approach, over product life cycles, to foster efficient transition between concept to patient access to balanced surveillance to informing future discovery