21st Century Cures Act
Section 1002 Work Plan Review

FDA Science Board Meeting
May 9, 2017

Overview

- Overview of the 21st Century Cures Act (Cures Act) Section 1002 Work Plan Review
  - Background of the Cures Act
  - Section 1002 and the Innovation Funds
  - Charge to the Science Board

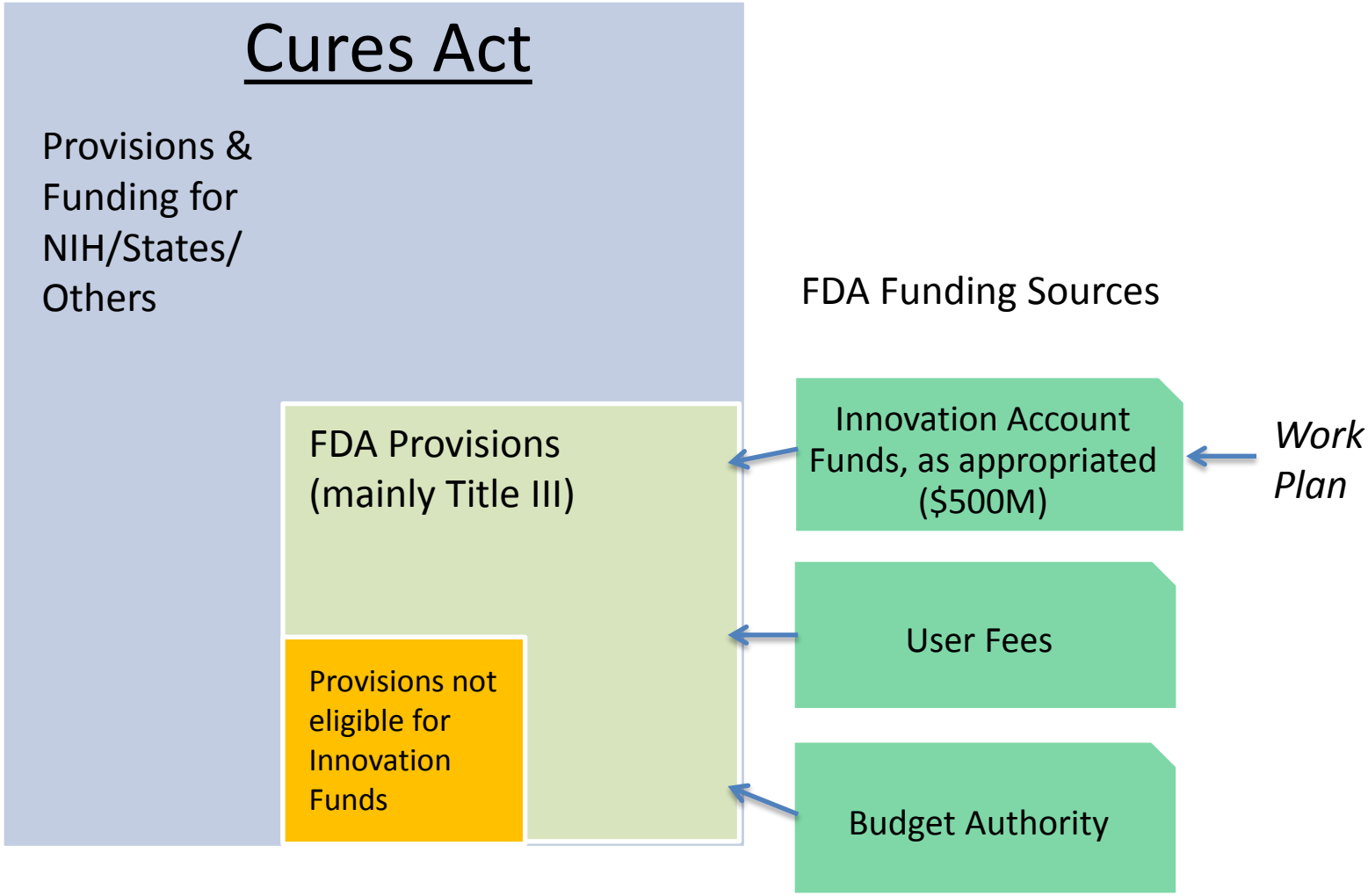
- Examples of Significant Provisions
  - Patient Engagement
  - Drug Development Tools
  - Regenerative Medicine

Background

- Cures Act was signed into law on December 13, 2016
- Authorizes $500 M to be appropriated to FDA over nine fiscal years (subject to annual appropriations) to carry out specific medical product development innovation activities in Title III of the Cures Act, including:
  - Patient-Focused Drug Development
  - Advancing New Drug Therapies
  - Modern Trial Design and Evidence Development
  - Patient Access to Therapies and Information
    - Combination Products
  - Antimicrobial Innovation and Stewardship
  - Medical Device Innovations
    - Breakthrough Devices
    - Least Burdensome Device Review
  - Improving Scientific Expertise and Outreach at FDA

Cures Act Funding

Conceptual diagram; relative box sizes are not to scale



Title I, Section 1002

- Sec. 1002 – Develop work plan for how FDA will allocate the $500 M Innovation Account funds over 9 fiscal years (2017-2025)
  - Limit allocations to eligible activities authorized to be funded (Title III, subtitles A-F and Section 3073)
  - Seek recommendations from FDA Science Board (SB) on work plan and proposed allocation of funds
  - Consider FDA SB recommendations and submit work plan to Congress within 180 days of enactment
  - Submit annual reports with updates of activities conducted using Innovation Account funds

FDA Evaluated Provisions Eligible for Innovation Funds

- FDA prioritized activities based on the following criteria:
  - present the greatest opportunity for FDA to foster innovation and integrate advances in biological sciences, engineering, information technology, and data science, to most directly enhance the Agency's product review tools and processes.
  - address the greatest need for scientific modernization.
  - have the most immediate impact on delivery of services to patients, the medical product industry, academia, and health professionals.
  - other funds may not be available.

Charge to the FDA Science Board

- FDA requests that the Science Board review the proposed work plan and provide recommendations for FDA's consideration.

- Questions for the FDA Science Board:
  - Are the criteria used by FDA to prioritize the proposed allocation of funds appropriate?
  - Are the proposed activities reasonably likely to contribute to successful achievement of the Cures Act requirements?

Next Steps

- FDA will consider the Science Board's recommendations in finalizing the work plan.
- FDA will submit the work plan to HHS and OMB for final clearance.
- FDA will submit to Congress the work plan along with the Science Board's recommendations.

FDA Patient-Focused Drug Development (PFDD) Helped Lay Foundation for Cures Act PFDD

- PFDD began as part of FDA PDUFA V (2012-2017) commitment
  - Develop a more systematic way of gathering patient perspective on their condition and available treatment options to inform B-R assessment
  - Conduct 20 public meetings each focused on a specific disease area
  - Produce **Voice of the Patient** reports* to faithfully capture patient input
    - 21 PFDD meetings conducted by FDA as of May 2017
- Key Learnings of PDUFA V:
  - **Patients with chronic serious disease are experts** on what it is like to live with their condition. Their "chief complaints" may not be factored into drug development and data collection plans. More guidance is needed.
- Proposal for PDUFA VI PFDD (2018-2022) thus includes:
  - Developing methods and approaches to bridge from PFDD meetings to fit-for-purpose tools to collect meaningful patient input that can be incorporated into regulatory review—via series of public workshops and **issuing series of guidance documents**
  - Providing a repository of publicly available tools and reference documents

* https://www.fda.gov/forindustry/userfees/prescriptiondruguserfee/ucm368342.htm

Cures Act Title III Subtitle A
Patient-Focused Drug Development (PFDD)

Section 3001: Patient Experience Data

- Following the approval of an NDA/BLA submitted after June 12, 2017, make public a brief statement regarding the patient experience data and related information, if any, submitted and reviewed as part of such application.

Section 3002: PFDD Guidance -- to address the following

1. Methodological approaches for collection of patient experience data to ensure data are relevant, objective, accurate and **representative of the intended population**, including methods to collect **meaningful patient input throughout drug development** and methodological considerations for data collection, reporting, management, and analysis;
2. Methodological approaches to develop and **identify what is most important to patients** with respect to **burden of disease, burden of treatment**, and the **benefits and risks** in the management of the patient's disease;
3. Approaches to identifying and developing **methods to measure impacts to patients** that will help **facilitate collection of patient experience data in clinical trials**;
4. Methodologies, standards, and technologies to **collect and analyze clinical outcome assessments for purposes of regulatory decision-making**;

Cures Act Title III Subtitle A
Patient-Focused Drug Development (PFDD)

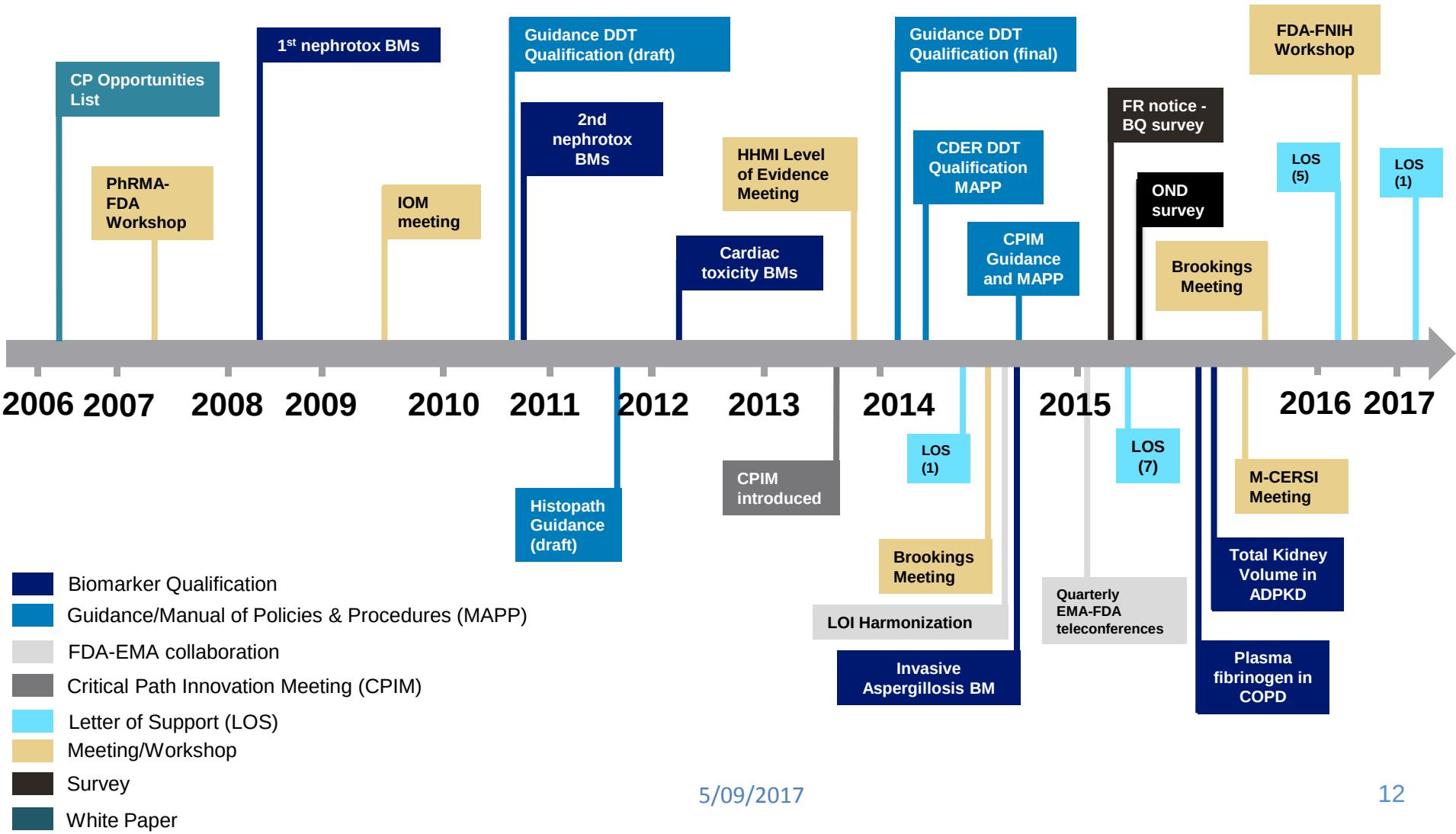
Section 3002: PFDD Guidance --contd.

5. **How** a person seeking **to develop and submit proposed draft guidance** relating to patient experience data **for consideration by FDA** may submit such proposed draft guidance to the Secretary;
6. **Format and content required for submissions** under this section to the Secretary, including with respect to the information described in paragraph (1);
7. **How FDA intends to respond** to submissions of information described in paragraph (1), if applicable, including timeframe; and
8. **How FDA anticipates using** relevant patient experience data and related information **to inform regulatory decision-making**

Section 3002 requires FDA to develop a **Plan** to issue draft and final versions of one or more guidance documents to cover these 8 areas over a period of 5 years

- FDA's Plan is nearing completion
- FDA's Plan integrates planned work and aligns timelines to address significant overlap of Cures Act requirement and PDUFA VI commitments

FDA Laid the Foundation for Qualification of Drug Development Tools



Cures Act
Subtitle B-Advancing Drug Therapies
Section 3011-Qualification of DDTs

- Applies to biomarkers and clinical outcome assessments
- Qualification means that a drug development tool and its proposed context of use can be relied upon to have a specific interpretation and application in drug development and regulatory review
- Establish process for qualification of DDTs
- Develop guidance that
  - Provides conceptual framework describing appropriate standards and scientific approaches to support the development of biomarkers
  - Delineates qualification process
- Hold public meeting to describe and solicit public input regarding the qualification process
- Issue public report on the qualification processes
- Publicly post information on qualification submission status

Regenerative Medicine Provisions in Cures Act

- Section 3033
  - Accelerated Approval for Regenerative Advanced Therapies
- Section 3034
  - Guidance Regarding Devices Used in the Recovery, Isolation, or Delivery of Regenerative Advanced Therapies
- Section 3035
  - Report on Regenerative Advanced Therapies
- Section 3036
  - Standards for Regenerative Medicine and Regenerative Advanced Therapies

Regenerative Medicine Advanced Therapy (RMAT) Designation

- Designation program to expedite development and review of Regenerative Medicine Advanced Therapies (RMATs)
- Regenerative Medicine Advanced Therapies are:
  - Regenerative medicine therapies (including cell therapies, tissue engineering products, human cell and tissue products)
  - For a serious or life-threatening disease or condition, and
  - Preliminary clinical evidence indicates that the drug has potential to address an unmet medical need in that disease or condition

Website with information about process:
http://www.fda.gov/BiologicsBloodVaccines/CellularGeneTherapyProducts/ucm537670.htm

Regenerative Medicine Standards Development

- FDA will facilitate the development of standards to help foster the development, evaluation, and review of regenerative medicine advanced therapies
  - Coordinate and prioritize the development of standards and consensus definitions of terms in consultation with NIST and other stakeholders
  - Identify opportunities for the development of laboratory regulatory science research and documentary standards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