U.S. FOOD & DRUG ADMINISTRATION

The 21st Century Cures Act: Impact on FDA

FDA Presentation to STAMP

27 June 2017

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Disclaimer

The views expressed in this presentation are my own and do not reflect official FDA policy.

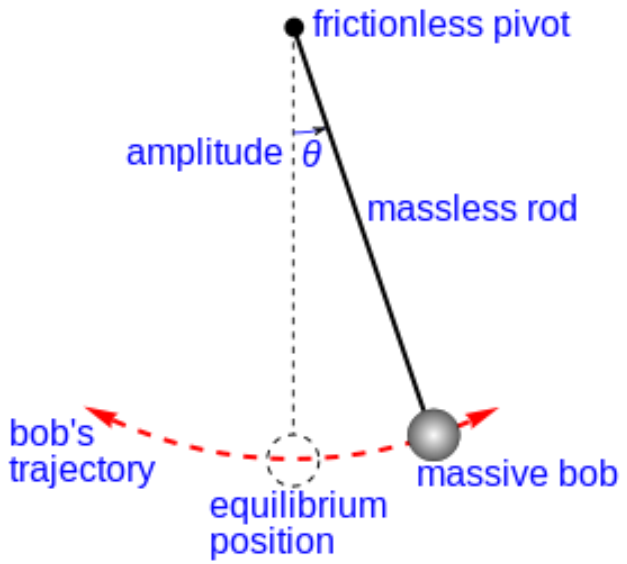
Legislation and Drug Regulation: Context

Society sets expectations

Legislation responds

Regulation ensues

Drug development evolves



Landmarks in US Drug Regulation

- 1906 – Pure Food and Drug Act
- 1938 – Food Drug & Cosmetic Act
- 1962 – Kefauver-Harris Amendment

Modern Landmark Legislation

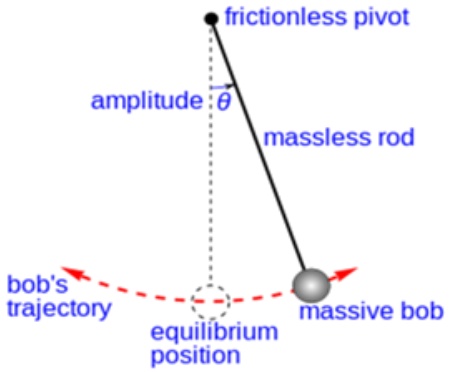
2007 – FDA Amendments Act (FDAAA) and PDUFA IV

- Longstanding fear that FDA regulation and practice favored industry came to fore with large public health concerns
  - anti-depressants/suicidality
  - VIOXX cardiovascular safety
- New FDA responsibility and authority to manage the full "life cycle" of drugs

Modern Landmark Legislation (continued)

2012 FDA Safety and Innovations Act FDASIA and PDUFA V

- Old debate resurrected: FDA pace of drug approval
  - 1990s - too slow (PDUFA I)
  - 2007 - too fast (FDAAA) – need more attention to safety
- 2012 concerns
  - Economy…jobs…innovation…FDA reform
  - Along with rising voices of **patient advocates** pressing for **more and more early access** and pathways for rapid market access
- Focus
  - Innovation: Breakthrough
  - Stakeholder Engagement: Patient Focused Drug Development



21st Century Cures Omnibus….scope and money

- Broad public health concerns
- Drug discovery and innovation
- Mental health and substance abuse disorders treatment, prevention, access
- Ensuring children have access to mental health resources
- Medical devices
- State opioid abuse programs
- Patient privacy
- Pediatric research
- Patient focused drug development
- Rare diseases
- Biomarker and other tools development
- Antimicrobial resistance
- Regenerative therapies
- Vaccines
- Medical countermeasures

2016 21st Century Cures Act: Basics

- 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
- FDA submitted the workplan to Congress on June 9, 2017.

Cures: Components

- Title I: Innovation Projects and State Responses to Opioid Abuse
  - NIH: Precision Medicine, BRAIN, and Cancer Moonshot Initiatives
  - FDA: $500 MM for projects under Title III
- Title II: Discovery
  - Targeted largely toward NIH
  - Precision Medicine Initiative
  - FDA: data gaps for pregnant and lactating women, improvement to CT database
- Title III: Development
  - The FDA slice

Title III

Expansive, far reaching, pushing innovation

- **Patient Experience Data**
- **Patient-Focused Drug Development Guidance**
- **Streamlining Patient Input**
- **Report on Patient Experience Drug Development**
- **Qualification of Drug Development Tools**
- Targeted Drugs for Rare Diseases
- Reauthorization of Program to Encourage Treatments for Rare Pediatric Dis.
- GAO Study of Priority Review Voucher Programs
- Amendments to the Orphan Drug Grants
- Grants for Studying Continuous Drug Manufacturing
- **Novel Clinical Trial Designs**
- **Real World Evidence**
- Reducing administrative burden for researchers
- **Summary Level Review**
- **Expanded Access Policy**
- Accelerated Approval for **Regenerative Advanced Therapies**
- Guidance Regarding Devices Used in the Recovery, Isolation, or Delivery of Regenerative Advanced Therapies
- Report on Regenerative Advanced Therapies*
- Standards for Regenerative Medicine and Regenerative Advanced Therapies
- Health Care Economic Information
- **Combination Product** Innovation
- Antimicrobial Resistance Monitoring
- Limited Population Pathway

More Title III

- Prescribing Authority
- Susceptibility Test Interpretive Criteria for Microorganisms; Antimicrobial Susceptibility Testing Devices
- **Breakthrough Devices**
- Humanitarian Device Exemption
- Recognition of Standards
- Certain Class I and Class II Devices
- Classification Panels
- Institutional Review Board Flexibility
- CLIA Waiver Improvements
- Least Burdensome Device Review
- Cleaning Instructions and Validation Data Requirement
- Clarifying Medical Software Regulation
- Silvio O. Conte Senior Biomedical Research and Biomedical Product Assessment Service
- Hiring Authority for Scientific, Technical, and Professional Personnel
- **Establishment of Food and Drug Administration Intercenter Institutes**
- Scientific Engagement
- Drug Surveillance
- Reagan-Udall Foundation for the Food and Drug Administration
- Medical Countermeasures Guidelines
- Clarifying BARDA Contracting Authority
- Countermeasure Budget Plan
- Streamlining Project Bioshield Procurement
- And much, much more……

IIIA: Patient-Focused Drug Development

All this talk about patient focus needs something to show for it

- Patient Experience Data
  - NDA reviews must include a brief statement regarding the patient experience data and related information, if any, submitted and reviewed
- Patient-Focused Drug Dev. Guidance
  - Develop plan to issue draft and final guidance documents regarding collection of patient experience data; the first of these drafts must issue within 18 months

IIIB: Advancing New Drug Therapies

Biomarkers and other tools must work for patients, but within reason and FDA resources

- Qualification of Drug Development Tools
  - Establish a process for submission of letter of intent to develop a tool, a qualification plan, and a qualification package for review
  - FDA can prioritize qualification work based on severity of disease and alternatives available
  - Describes how tools can be used to support approval or licensure
  - Transparency – FDA will post information about what is being reviewed

IIIC: Modern Trial Design and Evidence Development

Time to put FDA learnings in writing for others

- Novel Clinical Trial Designs
  - FDA to issue of guidance on complex adaptive and other novel trial designs following a public meeting
- Real World Evidence
  - "Program" to be established to evaluate use of real world evidence for new indication or fulfilling post approval commitments within 2 years and requires issuance of guidance within 5 years

IIID: Patient access to therapies and information

- Summary Level Review
  - FDA can rely on data summaries for supplemental application
- Expanded Access Policy
  - One “call out” to industry
  - Manufacturers with investigational drugs for serious diseases or conditions must make available their expanded access policies

Reflections the Act

- Public money invested in key innovation areas, with accountability
- Government agencies must work together – FDA Centers, too
- You must share what you have learned with public/industry in key areas
  - Innovative methods, special need areas, qualification programs
  - All this talk about patient focus needs to be pulled together

- FDA needs to go faster? No
- Industry needs tighter regulation? No
- No truly novel programs suggested
  - Focus is on more use or expansion of existing tools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