The 21st Century Cures Act: Impact on FDA

FDA Presentation to STAMP

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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

PULL to the right SAFETY!
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

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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