Resources, Priorities, and Long Term Vision for the FDA

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Commissioner of Food and Drugs

Alliance for a Stronger FDA

April 19th, 2016
The Big Picture

- Knowledge revolution impacting every mission of FDA

- Biological sciences
  - Agriculture
  - Medicine

- Engineering

- Information sciences
  - Computation
  - Analysis
  - Democratization
Preparing for the Future: Examples from Past Few Weeks

- Next generation sequencing
- Wearable devices that monitor and provide decision support for serious conditions
- Targeted precision therapies in combinations
- Organ replacement, stem and cell therapy
- The pregnant woman and fetal pair in therapeutics
- E Cigarettes
- The Animal Rule
- Monitoring the safety of the food supply
- Determining when advertising is false and/or misleading
Program Level: $2.0B to $4.7B (136%)

- Budget Authority: $1.6B to $2.7B (73%)
- User Fees: $433M to $2.0B (365%)

User fees do not support work on:
- Counterfeit drugs
- Dietary supplements
- Compounding policy and enforcement
- Nutrition
- Tissues
- Whole blood or a blood component for transfusion
- Allergenic extract products
- Cosmetics
FDA’s Regulatory Scope: 20 cents of every GDP dollar
FDA Mission

FDA is responsible for protecting the public health by assuring the safety, efficacy, and security of human and veterinary drugs, biological products, medical devices, our nation’s food supply, cosmetics, and products that emit radiation.
FDA Mission

FDA is also responsible for advancing the public health by helping to speed innovations that make medicines more effective, safer, and more affordable and by helping the public get the accurate, science-based information they need to use medicines and foods to maintain and improve their health. FDA also has responsibility for regulating the manufacturing, marketing and distribution of tobacco products to protect the public health and to reduce tobacco use by minors.
FDA Mission

Finally, FDA plays a significant role in the Nation’s counterterrorism capability. FDA fulfills this responsibility by ensuring the security of the food supply and by fostering development of medical products to respond to deliberate and naturally emerging public health threats.
FDA Priorities

- **FDA Workforce and Recruitment**
- **Evidence Generation**
  - FSMA
  - Opioids
  - Precision Medicine
  - Deeming and Nicotine
  - Antimicrobial Resistance
  - Compounding
- **Biomarkers, Surrogates, Clinical Outcome Assessment**
- **Program Alignment**
- **Laboratory Developed Tests**
- **Patient Focused Development**
- **Interagency Coordination**
- **Combination Products**
- **Labeling & Patient/Consumer Communication**
- Communications about FDA and its mission and activities
FDA Workforce

- Hiring
- Professionalism
- Workplace Environment
- Interface with people and knowledge in outside world
Evidence Based Practice

- EBP is the integration of clinical expertise, patient values, and the best research evidence into the decision making process for patient care.

- Clinical expertise refers to the clinician’s cumulated experience, education and clinical skills.

- The patient brings to the encounter his or her own personal preferences and unique concerns, expectations, and values.

- The best research evidence is usually found in clinically relevant research that has been conducted using sound methodology.

  - (Sackett D, 2002)
Generating Evidence to Inform Decisions

1. FDA Critical Path
2. NIH Roadmap
3. Data Standards
4. Network Information
5. Empirical Ethics
6. Priorities and Processes
7. Inclusiveness
8. Use for Feedback on Priorities
9. Conflict of Interest Management
10. Evaluation of Speed and Fluency
11. Pay for Performance
12. Transparency to Consumers

Discovery Science → Early Translational Steps → Clinical Trials → Clinical Practice Guidelines → Measurement and Education → Outcomes → Performance Measures → Discovery Science
Which Treatment is Best for Whom?
High-Quality Evidence is Scarce; < 15% of Guideline Recommendations Supported by High Quality Evidence

Scientific Evidence Underlying the ACC/AHA Clinical Practice Guidelines

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Judith M. Kramer, MD, MS
Robert M. Califf, MD
Sidney C. Smith Jr, MD

**Context**  The joint cardiovascular practice guidelines of the American College of Cardiology (ACC) and the American Heart Association (AHA) have become important documents for guiding cardiology practice and establishing benchmarks for quality of care.

**Objective**  To describe the evolution of recommendations in ACC/AHA cardiovascular guidelines and the distribution of recommendations across classes of recommendations and levels of evidence.

**Data Sources and Study Selection**  Data from all ACC/AHA practice guidelines issued from 1984 to September 2008 were abstracted by personnel in the ACC Science and Quality Division. Fifty-three guidelines on 22 topics, including a total of 7196 recommendations, were abstracted.

- Tricoci P et al.: JAMA 2009;301:831-41
Sentinel: Distributed Data Networks
(Over 150,000,000 people included)

FDA, The Sentinel Initiative
July 2010
FDA Safety and Innovation Act

- Section 1136 of FDASIA (Jul 9, 2012) amended the FD&C Act by adding new section which addresses electronic submissions.

- Starting 24 months after final guidance for a specific submission type, Sponsors must use the standards defined in the data standards catalog (for submissions for NDAs, ANDAs, and BLAs)

- Guidance document for Submissions Under Section 745A(a):
  - Draft published February 2014
  - Final publication December 17, 2014
The U.S. Precision Medicine Initiative
"Precision Medicine refers to the tailoring of medical treatment to the individual characteristics of each patient. It does not literally mean the creation of drugs or medical devices that are unique to a patient, but rather the ability to classify individuals into subpopulations that differ in their susceptibility to a particular disease, in the biology and/or prognosis of those diseases they may develop, or in their response to a specific treatment”

National Research Council 2011
Precision Medicine

“Preventive or therapeutic interventions can then be concentrated on those who will benefit, sparing expense and side effects for those who will not. Although the term ‘Personalized Medicine’ is also used to convey this meaning, that term is sometimes misinterpreted as implying that unique treatments can be designed for each individual.”
Success of Precision Medicine Requires:

• **Safe and accurate diagnostic tests** that reliably identify individual variation

• **Learning health systems** that enable researchers and clinicians to learn from and inform the patient experience

• **Development of targeted therapies** that are more efficacious or have less deleterious side effects for specific individuals

• **Updated research and regulatory policies** that catalyze the development of new treatments while protecting patients
FDA – A Long History of Enabling Precision Medicine

- 23 companion diagnostics cleared or approved
- 50 biomarkers used in targeting 147 approved drugs*
  - Cystic Fibrosis, Cancer, Cholesterol, Psychiatric,
    Pulmonary, Infectious Diseases, etc.
- More than 60 approved/cleared human nucleic acid
  based tests**
- More than 24 Guidances issued since 2005
Precision Medicine Initiative - FDA

Vision: Implement new regulatory policies to promote research and accelerate the translation of precision medicine technologies into treatments that **benefit patients**.

- Near Term: Implement standards and shared resources that will enable the development of knowledge for research and patient decision making

- Longer Term: Implement standards-based regulation of diagnostic tests that will ensure that the tests patients receive provide accurate, reproducible, and meaningful results
Precision Medicine Initiative: Modernizing FDA Regulation of Genomic Laboratory Tests

Traditional testing

Next generation sequencing
Precision Medicine Initiative

• FDA will develop a new approach for evaluating Next Generation Sequencing tests
• A new approach will facilitate the generation of knowledge while ensuring that the tests are accurate and reliable.
Patient Partnerships

Technologies

Data Science

EHRs

Genomics
A Modern National Evidence Generation System (EvGen)

Node
Nodes are Clusters of Accessible Data
Drug Safety and Knowledge about Real World of Drug Use
National Evaluation System for healthcare Technology

National Device Evaluation System

Coordinating Center
Which Treatment is Better?

PCORnet

Coordinating Center
Vice President’s Cancer Moonshot

Cancer Moonshot (Mandatory Resources) +$75.0M/5 years

FDA will:

• Establish an integrated Oncology Center of Excellence to evaluate products for screening, prevention, diagnosis, and treatment of cancer

• Work closely with the National Institutes of Health and other Federal partners

Results:

• Streamlined development of cancer fighting vaccines and advanced diagnostics for early screening and detection

• Expedited approval of novel medical products
# FY 2016 Enacted Budget

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<thead>
<tr>
<th>Budget Authority (BA)</th>
<th>User Fees (UF)</th>
<th>Total</th>
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+$240.1M (BA: +$132.3M / UF: +$107.8M) above FY 2015 Enacted

- **BA Increases:**
  - Food Safety Modernization +$104.5M
  - FDASIA Implementation +$5.0M
  - Combating Antibiotic Resistant Bacteria +$8.7M
  - Precision Medicine +$2.4M
  - Orphan Product Development Grants Program +$2.5M
  - Sunscreen +$0.7M
  - Foreign High Risk Inspections +$5.0M
  - Rent and Facilities +$11.0M
  - Program Reductions -$7.5M

- **Transfer to HHS Office of Inspector General (non-add)** -$1.5M
## FY 2017 President’s Budget

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+ $358.3M (BA: +$14.6M / UF: +$268.7M / MR: +$75M) above FY 2016 Enacted

- **Budget Authority:** +$14.6M
  - Food Safety +$18.4M
  - Medical Product Safety +$3.2M
  - Infrastructure and Offsets -$7.0M

- **Cancer Moonshot (Mandatory Resources)** +$75.0M

- **Current Law User Fees** +$66.4M

- **Proposed User Fees** +$202.3M
FDA Appreciates Your Support and Suggestions!