The Future of Regulatory Science at FDA

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"Study the past if you would define the future."

Confucius

Regulatory Science

(FDA definition)

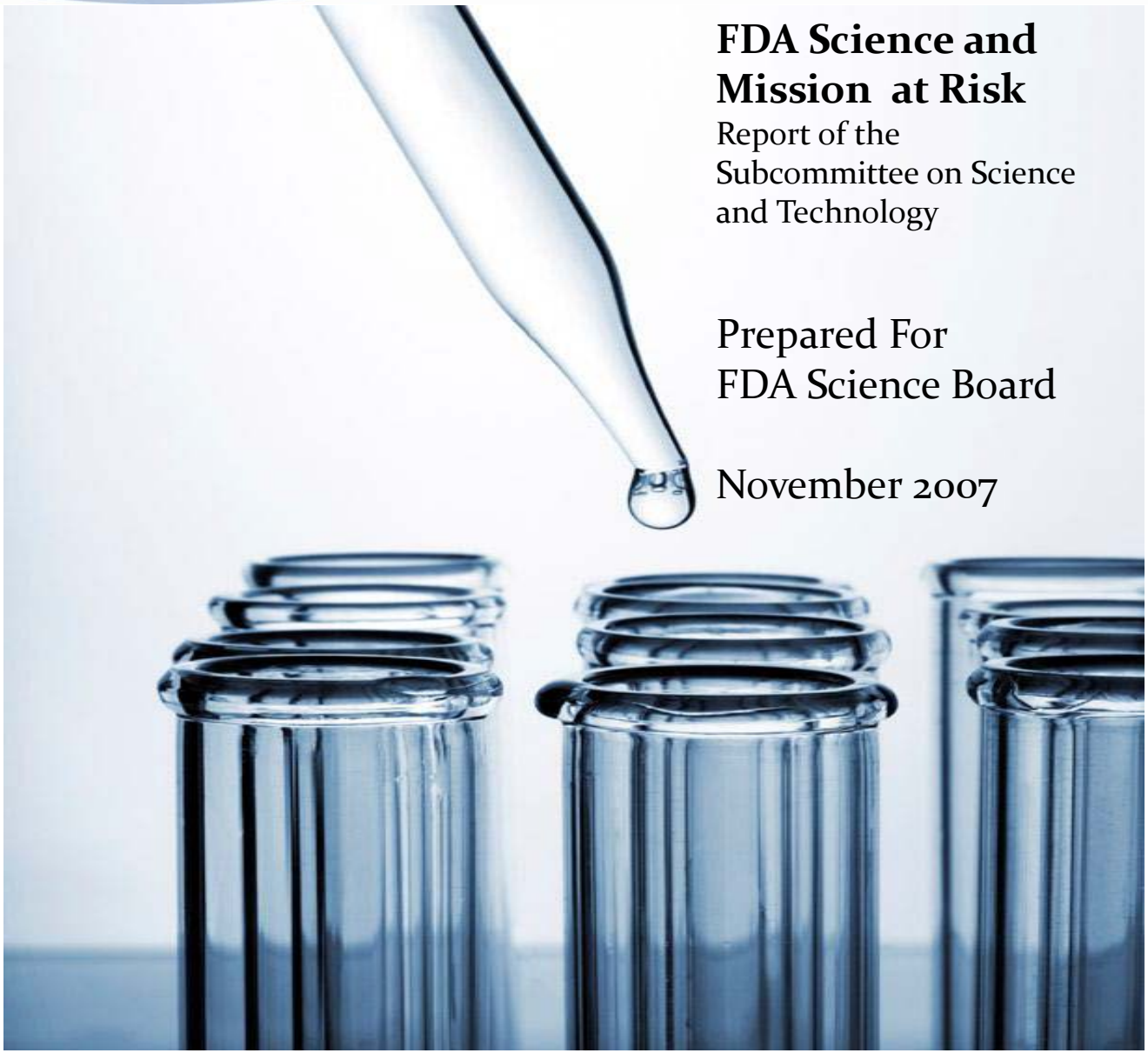
The science of developing new tools, standards, and approaches to assess the safety, efficacy, quality, and performance of FDA-regulated products.

"If you don't know where you're going, any road will get you there."

Lewis Carroll

"If you don't know where you're going, you'll wind up somewhere else."

Yogi Berra


FDA Science and Mission at Risk
Report of the Subcommittee on Science and Technology
Prepared For FDA Science Board
November 2007

Science and Mission at Risk 2007 Report

- Raised serious concerns regarding the health of the science enterprise at FDA
- Causes identified:
  - *Chronic underfunding*
  - *Loss of scientific expertise*
  - *Increasing legislative mandates/responsibilities without corresponding resources to support them*

Additional Legislative Responsibilities Since 2007 Report

- FDA Amendments Act (FDAAA 2007)
- Tobacco Control Act (2009)
- Affordable Care Act (2010)
  - Title VII - generics
- Food Safety Modernization Act (FSMA 2011)
- FDA Safety and Innovation Act (FDASIA 2012)
- Drug Quality and Security Act (DQSA 2013)
- 21st Century Cures and Senate Innovation Act (2015)

Science and Mission at Risk 2007 Report

- Major recommendations:
  - *Realign science organization to better manage/ coordinate emerging science*
  - *Strengthen the science base*
  - *Enhance the scientific workforce*

Science Organization at FDA

- **Cross cutting coordination and science promotion improved**
  - **Office of Chief Scientist established**
    - **Office of Regulatory Science and Innovation**
    - **Office of Scientific and Professional Development**
    - **Office of Countermeasures and Emerging Threats**
    - **Office of Health Informatics**
  - **Senior Science Council**

Cross-Cutting Regulatory Science Programs

- Centers of Excellence in Regulatory Science & Innovation (CERSI)

- Challenge Grants
- Broad Agency Announcement
- Medical Countermeasures Initiative

Cross Cutting Regulatory Science Programs

- Medical Countermeasures Initiative
- Nanotechnology Core Facilities
- Highly Integrated Virtual Environment (HIVE)
- Shared scientific resources

- Modernize toxicology to enhance product safety
- Stimulate innovation in clinical evaluations and personalized medicine
- Support new approaches to improve product manufacturing and quality
- Ensure FDA readiness to evaluate innovative technologies
- Harness diverse data through information sciences
- Implement a prevention-focused food safety system
- Develop medical countermeasures
- Strengthen social and behavioral sciences
- Strengthen global product safety

Regulatory Science at FDA

- Important to note all agency components also have program-specific regulatory science initiatives
- Includes intramural and extramural support
- Centers of Excellence
- Critical Path Initiative

Training Programs

FDA Food and Drug Administration
COMMISSIONER'S
Fellowship Program
Protecting Public Health Through Science and Regulation

IOTF
Interagency Oncology Task Force
Joint Fellowship Program

ORISE
OAK RIDGE INSTITUTE FOR SCIENCE AND EDUCATION

Service Fellowships

Facilities

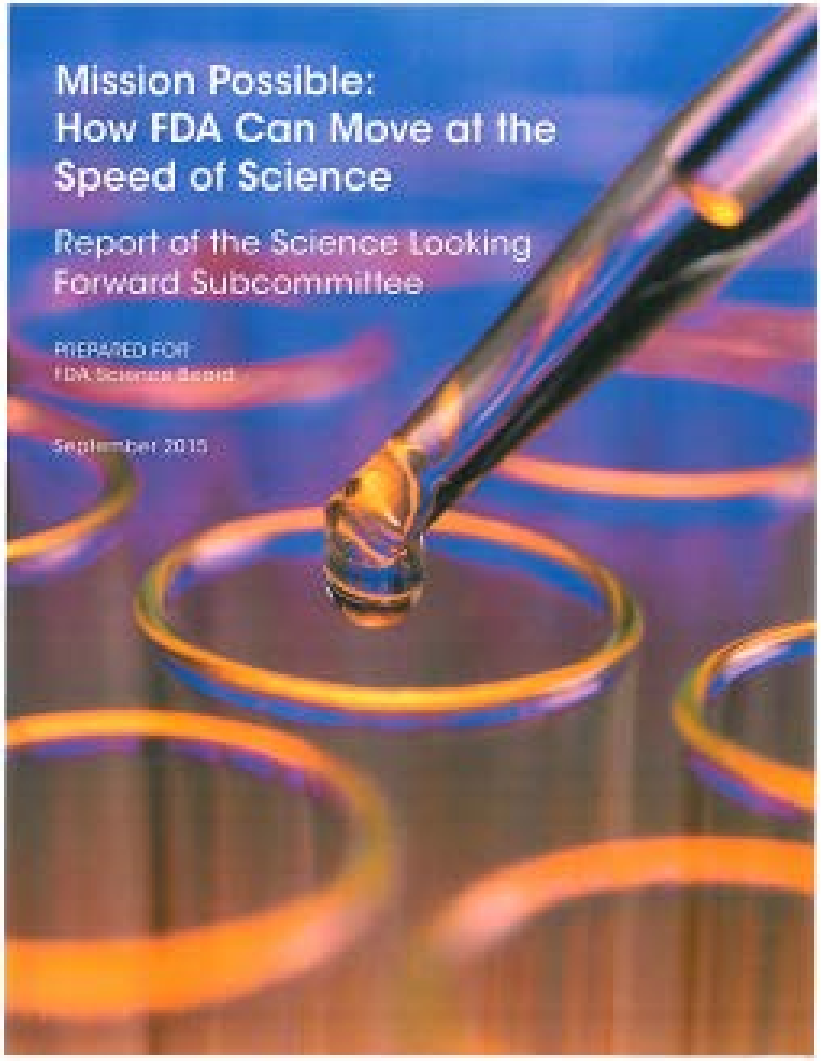

FDA
U.S. Department of
Health and Human Services
Food and Drug Administration

Science Board Updated Review

- Late 2013, Science Board requested to review progress since previous report
- Specific questions:
  - **Whether changes that FDA has made to its regulatory science programs have been a success**
  - **Whether FDA should consider any other programmatic changes**
  - **What opportunities, strategies, and frameworks for collaboration, will best advance FDA's mission**
  - **Whether FDA has taken sufficient steps to strengthen the scientific workforce & whether additional steps may be necessary**

- Outlines current status of regulatory science at FDA
- Details of current programs
- Examples of activities in each regulatory science priority thematic area

http://www.fda.gov/downloads/ScienceResearch/AboutScienceResearchatFDA/UCM456328.pdf



Progress Since 2007

"The responsiveness of FDA to the Mission at Risk report and those responsible for overseeing its work has been extensive, transformative, and laudable. Many substantive changes have been made in FDA's organization, authorities, and programs that significantly address issues identified in 2007."

Report Recommendations

- Medical Product Innovation
  - Facilitate biomarker & surrogate endpoint qualification
  - Clinical trial efficiency
    - Master protocols
    - Clinical trial networks
  - Data mining & analytic tools
    - Full deployment of Sentinel; expand to devices

Report Recommendations

- Product quality
  - Genomics
    - medical product quality and safety
    - Foodborne and other outbreak investigation
  - Analytic methods for nanotechnology
  - Novel technologies for lab & field analytics

Report Recommendations

- Modernizing toxicology
  - Pluripotent stem cells for toxicity evaluation
  - Bioimaging for neurotoxicity
  - Mathematical modeling of systems
  - *In silico* modeling for susceptibility to toxicity
  - "Organ-on-a-chip" technology

Report Recommendations

- Expand extramural collaborations
  - Specific mention of Reagan-Udall Foundation
- Recruitment of "21st Century Workforce"
- *Resources to match responsibilities*

Overarching Regulatory Science Priority for Medical Products

*Study and support development of innovative ways to streamline/supplement/speed medical product availability **without** negative impact on the FDA gold standard of product efficacy and safety.*

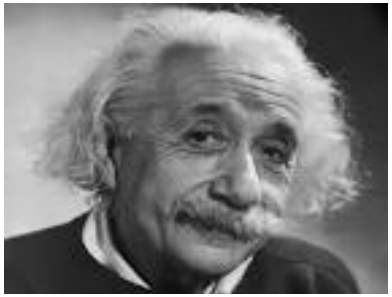
Additional Priority Areas

- Methods development for use of real world data (esp. pre-market)
- Adaptive/innovative clinical trial design
- Patient-focused drug development
- Social and behavioral science

GLOBAL SUMMIT REGULATORY SCIENCE 2015

- 5th GSRS in Parma Italy
- Focus area was bioinformatics
- 26 countries & 150 participants
- Increasing opportunities for regulatory science internationally

"We cannot solve our problems with the same thinking we used when we created them."



Albert Einstein