Regulatory Science: Snapshot of a Work in Progress - Opportunities and Pillars for Implementation

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Regulatory Science: Overview

> ***"While the world of drug discovery and development has undergone revolutionary change, shifting from cellular to molecular and gene-based approaches, FDA's evaluation methods have remained largely unchanged over the last half century."*** Science and Mission at Risk; FDA Science Board, 2007

– Regulatory science: unique, neglected

- Science integral to all we do and quality and integrity of our decision-making
- FDA is acting on a vision for collaborative regulatory science to *promote & protect* health
- *Promote*: help bridge the gap ("valley of death") between basic science/proof of principle and actual products for patients and public health
- *Protect*: public health and safety
- High interest among multiple stakeholders

Regulatory Science

- Develop, assess and provide tools, methods, models, standards, guidance, pathways to evaluate product safety, efficacy and quality
  - Laboratory, population, clinical, manufacturing and behavioral sciences and scientists
  - FDA can see what works and what does not across multiple products and stakeholders
  - Unlike work typically performed in academia or by individual sponsors and becomes available to all
- Opportunity to engage scientifically throughout the development and evaluation process to *catalyze and enable change and success, both internally and externally*

Draft Scientific Emphasis Areas

- Transforming product development
  - *Vision: Product development is radically different – agile and adaptive as new information is collected, bringing clinical and biologic information together to identify population subgroups and individuals who will uniquely benefit, and bringing products to patients more rapidly, efficiently and safely*
  - Examples: personalized medicine, diagnostics, biomarkers and other unique outcomes (e.g. imaging, patient reported), innovative clinical trail designs, combination/multiple interventions

Draft Emphasis Areas: cont.

– Products for unmet public health needs

- *Vision: The potential of innovation is realized to prevent and treat disease and reduce suffering globally, mitigate pandemics, and respond to emerging infectious disease threats and bioterrorism. Nimble, rapid generation of effective countermeasures protects health and stability.*
- Examples: Products for global diseases and markets, emerging threats, pandemics and national security. Platform technologies for vaccines, drugs and diagnostics for rapid mobilization for new threats and for dual uses.

Draft Emphasis Areas: cont.

- Regenerative medicine
  - *Vision: Stem cells, tissues and tissue engineered and combination products are successfully brought to bear against serious diseases and to repair and replace damaged organs and tissues.*
  - Examples: new treatments for diabetes, cardiac and neurodegenerative diseases

Draft Emphasis Areas: cont.

- Modernizing toxicology, product characterization, and pathogen and contaminant detection and assessment
  - Vision: *Advances in life science and engineering transform product science, better identifying and predicting what is safe and what is harmful*
  - Examples: in vitro toxicology, product characterization/complex biologics, rapid accurate detection of pathogens and contaminants in food and medical products, environmental/chemical hazard assessment

Draft Emphasis Areas: cont.

- Informatics enhancing safety, health outcomes and transformation of health care
  - *Vision: Vast amounts of clinical, health care and biological data are brought together and harnessed to speed product development and improve patient safety, outcomes and the effectiveness of health care*
  - Examples: monitoring of safety using health care and public health data (allowing earlier approvals and faster recognition of problems), utilization of health care and community settings to better characterize and optimize outcomes, including enabling clinical trials in practice settings

Regulatory Science at FDA: Office of the Chief Scientist; Major Pillars in Implementation

- *Leadership to Strengthen and Support Science and Promote Innovation at FDA*
  - e.g. cross-center working groups, coordination, scientific guidance, shared resources and infrastructure, scientific computing and tools
  - key role of new Office for Science and Innovation
- *Scientific Excellence and Professional Development*
  - e.g. scientific exchanges, agreements and access to local universities, NIH etc.

Major Pillars in Implementation

- *Recruitment and retention of outstanding scientists*
  - E.g. Commissioner's and other Fellowship programs, of newly independent scientists
- *Mission critical applied research*
  - e.g. program specific activities, Chief Scientist's challenge grants
  - FDA/NIH and other research partnerships
- *Collaboration, partnerships and transparency*
  - E.g. Critical Path, Centers of Excellence in Regulatory Science (CERS), joint appointments with academia, partnerships with NIH, CDC, DARPA, NIST, NTP etc.
  - Input of internal and external advisors/public, including senior agency leadership/Centers, Science Board

Thanks

- ***Science is key to the quality, independence and integrity of FDA decisions***
- ***Applied regulatory science must be strengthened to enable innovation and to soundly protect and promote health***
- ***Resources & implementation strategy and plans being developed***
- ***Outreach, collaboration, and continuing input essential***

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