Strengthening a Workforce for Innovative Regulatory Science in Therapeutics Development: An Institute of Medicine Workshop

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“Please note that some of the views presented do not necessarily reflect those of the Food and Drug Administration.”
The Valley of Death
What is regulatory science?

• The application of basic science to the development and utilization of new tools, standards, and approaches for the assessment of medical product efficacy, safety, and quality

• The critical bridge between basic scientific research discoveries and new marketed, medical products
Why do we need regulatory science?

• Major investments and advances in basic sciences are not fully translating into products to benefit patients

• Product development is increasingly costly, success rates remain low, many uncertainties exist

• Development/evaluation tools and approaches have neither kept pace with nor incorporated emerging technologies

• Economic health of innovative biotech and medical product industry at risk
Regulatory Science, Innovation and Critical Path Updates

- FDA Science Strategic Plan
  - Final draft completed, released – presentation and discussion later today
- Regulatory Science targeted RFA’s issued– awards to be made in upcoming days
  - Advancing Regulatory Science through Novel Biomarker Research and Science Based Technologies
  - Innovation in Development and Qualification of Alternative Testing Methods for Reproductive Toxicology
    - Workshop planned on validation of new approaches
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Advancing Regulatory Science at FDA: Strategic Plan

Released August 19, 2011
VISION STATEMENT

“FDA will advance regulatory science to speed innovation, improve regulatory decision-making, and get safe and effective products to people in need. 21st Century regulatory science will be a driving force as FDA works with diverse partners to promote and promote the health of our nation and the global community”
Purpose

• Identify priority opportunity areas of regulatory science essential to the success of medical product innovation and FDA’s public health mission

• Develop/use the 21st century regulatory science tools and approaches needed for development and evaluation of 21st century products

• Promote innovation through targeted and collaborative approaches to regulatory science that enable new technologies and product development

• Build FDA’s scientific capacity, infrastructure, culture and collaborations, including through scientific and professional development of FDA’s scientists
Eight (8) Priority Areas

• Modernize Toxicology to Enhance Safety
• Stimulate Innovation in Clinical Evaluation & Personalized Medicine
• Support new Approaches to Improve Product Manufacturing and Quality
• Ensure FDA Readiness to Evaluate Emerging Technologies
• Harness Diverse Data through Information Sciences to Improve Health Outcomes
• Enable a Prevention Focused Food Safety System
• Facilitate Development of Medical Countermeasures to Protect US and Global Health and Security
• Strengthen Social and Behavioral Science to Help Consumers and Professionals Make Informed Decisions
1. Modernize Toxicology to Enhance Product Safety

- Develop better models of human adverse response
- Identify and evaluate biomarkers and endpoints that can be used in non-clinical and clinical evaluations
- Use and develop computational methods and in silico modeling
2. Stimulate Innovation in Clinical Evaluations and Personalized Medicine to Improve Product Development and Patient Outcomes

- Develop and refine clinical trial designs, endpoints and analysis methods
- Leverage existing and future clinical trial data
- Identify and qualify biomarkers and study endpoints
- Increase the accuracy and consistency, and reduce inter-platform variability of analytical methods to measure biomarkers
- Develop a virtual physiologic patient
3. Support New Approaches to Improve Product Manufacturing and Quality

- Enable development and evaluation of novel and improved manufacturing methods
- Develop new analytical methods
- Reduce risk of microbial contamination of products
4. Ensure FDA Readiness to Evaluate Innovative Emerging Technologies

- Stimulate development of innovative medical products while concurrently developing novel assessment tools and methodologies
- Develop assessment tools for novel therapies
- Assure safe and effective medical innovation
- Coordinate regulatory science for emerging technology product areas
5. Harness Diverse Data through Information Sciences to Improve Health Outcomes

- Enhance information technology infrastructure development and data mining
- Develop and apply simulation models for product life cycles, risk assessment, and other regulatory science uses
- Analyze large scale clinical and preclinical data sets
- Incorporate knowledge from FDA regulatory files into a database integrating a broad array of data types
- Develop new data sources and innovative analytical methods and approaches
The Vision- FDA Science Enclave

- FDA has been working towards an electronic approach to acquire, receive, and analyze study data
  - FDA is working with CDISC standards to support our current regulatory and research activities. What we learn from this will help to inform our long term information database model.
  - FDA will working with Standards Organizations and other Government Agencies to define and design the next generation information database model, set of standards and scientific enclave to enhance our regulatory and research activities.

- Electronic capture of study data is vital to integrate pre-marketing study data and post-marketing safety data

- FDA is working towards development of a scientific computing environment to support research and a development for our data
Science Enclave and Data Repository: Central to the Vision

- An enterprise initiative to improve FDA’s management of scientific data about regulated products and improve regulatory decision-making

- Establish data architecture and science enclave to facilitate integration of scientific data – across studies, within studies, combine with outside data, enable collaborations

- Create structured scientific data repositories that support the acquisition, validation, integration, and extraction of data from the increasingly large and complex datasets received by the Agency

- Make use of enhanced analytical tools and techniques that enable scientists, and ultimately reviewers to search, model, and analyze data to enable personalized medicine and to conduct better safety and efficacy analyses
Scientific Computing Goals

• Institute a regulated product information data warehouse for exploration
  – Electronically acquire, validate, integrate, and extract standardized, structured scientific data
  – Synthesize information across product applications, across trials, with biomarker data, across classes of products, and across product lifecycle
    • For example, new nephrotoxicity biomarker approved in one area could be used to inform a different product area
    • Ingredient found unsafe or component found defective may be found in other product areas (e.g., combination products, kits, inactive ingredients)

• Transform the regulatory review and decision process
  – Transition to interactive, electronic reviews
    • Support quantitative decision-making to assess safety and effectiveness throughout a product’s life cycle (e.g., data mining to detect possible safety signal)
    • Leverage analysis tools across product areas improving consistency and efficiency
  – Provide springboard to environment of the future that enables
    • Enriched scientific interpretations that integrate latest domain knowledge
    • Advanced analytics (e.g., virtual clinical trials, disease models)
6. Implement a New Prevention-Focused Food Safety System to Protect Public Health

- Establish and implement centralized planning and performance measurement processes
- Improve information sharing internally and externally
- Maintain mission critical science capabilities
- Cultivate expert institutional knowledge
7. Facilitate Development of Medical Countermeasures to Protect Against Threats to U.S. and Global Health and Security

- Develop, characterize, and qualify animal models for MCM development
- Modernize tools to evaluate MCM product safety, efficacy, and quality
- Develop and qualify biomarkers of diseases or conditions
- Enhance emergency communication
8. Strengthen Social and Behavioral Science to Help Consumers and Professionals Make Informed Decisions about Regulated Products

- Know the audience
- Reach the audience
- Ensure audience understanding
- Evaluate the effectiveness of communication about regulated products
Key Implementation Components: Collaboration, Professional Development

• Goals: leverage expertise, resources, enhance culture of collaboration, promote scientific and career development

• Partnerships with Government Agencies
• Staff Scientific Training and Professional Development and exchanges
• Direct Funding Mechanisms
• Public-Private Partnerships
New Initiative: Centers of Excellence

• RFA issued by OCS, and applications now under review, for new National Capitol Region Center for Excellence in Regulatory Science and Innovation (CERSI)
  – “to advance the field of regulatory science (including laboratory, population, behavioral, and manufacturing sciences) and the Critical Path Initiative toward more effective and efficient product development and evaluation. CERSI efforts will focus on promoting innovation in support of the development and evaluation of safe and effective products through training, applied collaborative science, professional development and scientific exchanges.”
  – Three components planned for FDA support (up to $1 million) to academic institution(s)
    • Regulatory science collaborative research, focused on FDA Priority areas
    • Training and scientific exchanges (bi-directional)
    • Core dedicated infrastructure to support the above
Centers of Excellence: cont.

- MOU with State of Arkansas for Center of Excellence in Regulatory Science
  - Signed this August: Opportunity to builds on longstanding relationships with state and its educational institutions
  - Includes commitment to collaboration in science and training
  - Commitment to focus on the safety assessment of FDA regulated products that contain nanomaterials.
  - Collaborative development of a degree and certificate program in Regulatory Science in the School of Public Health, University of Arkansas
Workforce for Innovative Regulatory Science

• FDA Workforce over 13,800 (as of Sept 11)
  – Interdisciplinary Scientists
    • Toxicologist
    • Microbiologist
    • Biologist
    • Chemist
  – Pharmacist
  – Medical Officers
  – Engineers
  – Statistical/Math Stat
  – Database/Information Technology
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FDA Organizational Components & Working Groups

- SISCA (Center Directors)
- Foods Program Science and Research Steering Committee
- Office of the Chief Scientist
- Office of External Affairs
- Office of Minority Health
- Office of Women’s Health
- Science and Innovation
- Senior Advisory Council
- Senior Science Council
- Scientific Computing Board
Questions?

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