Federal Perspectives on Clinical Regulatory Research Innovations

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“Please note that some of the views presented do not necessarily reflect those of the Food and Drug Administration.”
Problems Facing the Biopharmaceutical Industry

- Struggling to meet increasing demands on R&D investments
- Declining levels of productivity/innovation
- Loss of revenue due to patent expirations
- Three dozen drugs lost patent protection from 2007-2010

Problems Facing the Biopharmaceutical Industry-
Driving Towards Stratified Medicine

• Deficiencies in the economics of the blockbuster business model
  – 70% of approved drugs do not meet or match their R and D cost
  – Lower efficacy levels (40%-60% for most blockbuster drugs)

• Successes in stratified medicine
  – Genetech’s Herceptin
  – Novartis’ Gleevec

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
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
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
Questions?

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