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Importance of Regulatory Science

Driving Biomedical Innovation by Advancing Regulatory Science at FDA

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Innovation is Linked to Ecosystem and Partnerships

FDA-regulated products account for 25 cents of every consumer dollar spent in the U.S.

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Why do we need Regulatory Science?

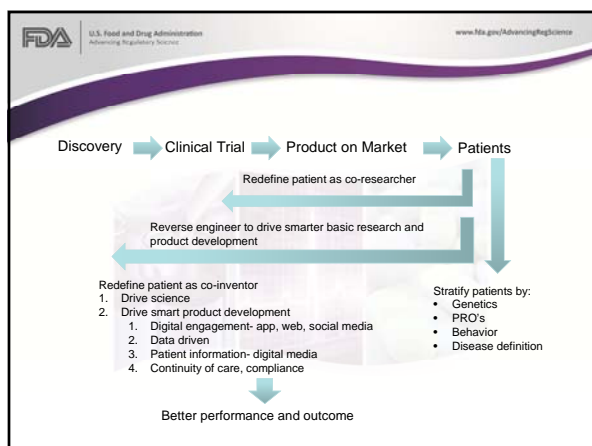
- Major investments and advances in basic sciences are not fully translating into products to benefit patients
- Product development is increasingly costly, success rates remain low, many uncertainties exist
- Development/evaluation tools and approaches have neither kept pace with nor incorporated emerging technologies
- Economic health of innovative biotech and medical product industry at risk

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FDA's Role

- Bringing together stakeholders to identify and overcome the challenges ahead
- Implementing reforms that adapt to the changing scientific and technological landscape
- Assuring modern, streamlined regulatory pathways



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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 for Emerging Technologies
- Harness Diverse Data through Information Sciences to Improve Health Outcomes
- Enable a Prevention Focused Food Safety System
- Facilitate Development of MCMs to Protect US and Global Health and Security
- Strengthen Social and Behavioral Science to Help Consumers and Patients Make Informed Decisions

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Snapshot of Science Activities at FDA

- **Scientific Publications (FDA-wide)**
2012 over 1,300 publications, several in Proc Natl Acad Sci, Science, Nature Reviews Genetics, Nature Biotech etc.
- **Collaborations (examples)**
> 30 Government---CDC, USDA, NIH, NOAA, USGS, EPA etc
> 90 Academic --- Univ. of Maryland, Virginia Tech, Mich. State, Howard Univ., Purdue, Univ. of Florida, UC Davis, Hopkins and other US & international centers
- **Guidance Documents**
76 new guidance since 2011 for drugs
>35 for biologics, >35 for medical devices, and 20 for food
- **Infrastructures**
high throughput sequencing and analysis
IT (high performance computing environment, bioinformatics)
- **Workgroups, Consortia, and Committees**

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Goals of Collaboration, cont.

Harness Diverse Data through Information Science to Improve Medical Products Development

- Develop standards for data collection and submission
 - Electronic data
 - The use of EHR in patient recruitment and selection
- FDA, CDISC and CP Institute are working together on data standards models
- Analyze large-scale clinical and pre-clinical data sets
- FDA is actively developing scientific computing systems to support scientists and reviewers
- Examples: Amalga, HIVE, Science Enclave, CHIO cloud

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Goals of Collaboration

- **Address the mission critical challenges, including advancing regulatory science in the priority areas as defined in the FDA Strategic Plan.**
- **Promote, develop and earn trust and a culture of collaboration between stakeholders**
- **Reduce average pharma product development "Cycle Time" from 9 years to 5 years in next 10 years**

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Opportunities for Collaboration

- Data collection, standardization and sharing
- Develop and refine clinical trial designs, endpoints, and analysis methods
- Harmonize clinical trial requirements and regulations (inter-agencies and US/EU)
- Invest in basic science to improve prediction of efficacy and toxicity
- Basic science research to advance regulatory science
- Create new assessment tools for emerging technologies (genomics, organ on chip). Clinical outcome related validation of these DDT through access to data/evidence.
- Invest in building a stronger scientific infrastructure and workforce training
- Identify and qualify biomarkers (drug efficacy and toxicity, and patient selection)
- Work with other government agencies (like NIH/NCATS) to create innovative pathways (faster, cheaper) to drug discovery and development

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Goals of Collaboration, cont.

- Build evaluative standards, tools, and measures
- Identify gaps (FDA strategic Plan, Driving Biomedical Innovation)
- Harmonize clinical trial requirements and regulations (inter-agencies and US/EU)
- Educate healthcare workforce on regulatory science
- Enhance information technology infrastructure development and data mining
- Develop standards for data collection and submission
 - Electronic data
 - The use of EHR in patient recruitment and selection

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Advancing Regulatory Science Website

<http://www.fda.gov/ScienceResearch/SpecialTopics/RegulatoryScience/default.htm>



THANK YOU