Institute of Medicine Workshop: Regulatory Science

Success Requires Solving for the Larger Context

Theodore F. Reiss, M.D.
Key Question

• Can regulatory science be addressed by itself?

• Is addressing broader issues necessary for success?

*Need to address broader issues*
Important Environment Issues

- Transformational trends in pharma
  - Understanding biologic pathways is increasingly challenging
  - Identifying targets is incrementally difficult
    - Optimize population specific benefit /risk
  - Expenses rapidly increasing
    - Downsizing
  - Price pressures

- Public sector
  - Funds shrinking
  - Increased focus on discovery and development

*These trends suggest collaborative solutions for a new educational, broader scientific, and funding environment*
The Relationship Between Development and Regulatory Science

• Same coin, different sides (best practice)
  – Design and regulation of development (bench to bedside) programs
    • Public health objective focused
  – Result of pharma / regulatory dynamic
    • Cross disciplines / institutions
    • Scientific integration results in greater effectiveness
    • Evolved together

• Different from present academic paradigm
  – Deep, not broad
  – Level of certainty in decision making
    • Benefit/risk
  – Neither needed nor valued in present academic culture
Foundational Principles

• Addressing needs piecemeal will limit impact
• Vision for more integrated biomedical science environment
  – Align trends, systems thinking shapes structure
  – Efficiently and effectively advance health of the public
  – Pragmatic, evolutionary framework
  – Needs jump starting and commitment
  – Collaborative: university, government, and pharma

• University value and promote translational/ development/ regulatory thinking
  – Knowledge generation to application
  – Academic model before hyper specialization
  – Better integration, better foundation for regulatory performance

• Efficient and Robust
  – Leverage the structures already in motion: CTSAs/ NCATS
  – Basis for development, methodological research, education and teaching
Across Disciplines

Across translational stages

Across institutions

Present-Future Scientific Model
Model: Integrating Best Practices

- University centered
  - Dept. of “bench to bedside” science
  - Small, critical mass core faculty
  - Expertise: development/regulatory/translational know how
    - Center for collaborative efforts – facilitate translational projects
      - Internally: departments/centers
      - Externally: pharma, regulatory agencies, foundations
  - Education: center for teaching
    - Translational/development/regulatory science
    - Team/collaboration/leadership
  - Research: collaboratively identify gaps in development/regulatory
    - Initiate and collaborate on projects
- Linked across institutions through CTSA mechanisms into Virtual Institute of Drug Development
  - Academic or collaboration with pharma/foundations
  - Self sustaining version of IMI initiative
Gaps and Barriers

• Lack of broad university exposure and comfort with:
  – Expanded scientific values/world view
    • Broad vs. deep
    • Health solutions orientation vs. discipline only
  – Cognitive framework
    • Knowledge only vs. benefit/risk based on significant, consistent evidence
  – Broader pool of biomedical science stakeholders
    • Brake down of trust: polarization
    • University vs. regulatory vs. pharma: COI
    • Pharma does not trust university: lack of world view and thought process

• Translational, development, regulatory projects are generally not rewarded in university

• Teaching/ training/ research programs scattered
  – Not coordinated towards common objectives
Critical Requirements for Sustainable Progress in Development/Regulatory Science

• University: value and reward
  – Foster a culture for integrated research, career paths
  – Researchers and educators for translational/discovery and development/regulatory sciences
  – Educate selected leaders to think both deeply and broadly

• Industry: comfort with government and university partnership
  – Scientifically and operationally

• All: Promote collaborative scientific culture
  – Balanced, rationalize COI guidelines
  – Culture of a shared scientific purpose
Approaches to Funding Derive from Model

• **Concerted initiative**
  – Initiation and baseline funding from NIH through CTSA/NCAT
    • Not FDA’s mission
    • Universities: matching infrastructure funds

• **Balance portfolio of NIH funding initiatives towards discovery/ development**
  – Critical incentive

• **Other funds necessary**
  • Virtual Development Institute

• **Pharma(small to large)/foundations/venture collaborate with institute scientifically and operationally**
  – Revenue above costs fund research and education
    • The price of discovery and development is education and translational/development/regulatory science research
  – Other revenue alternates (user fees)
    • Approval tax on drugs
    • Revenue Tax (>1B sales/year)
Summary: Regulatory Science

• Success requires solving for the broader issues
  – Concerted collaborative effort necessary
  – Across university, government, industry
  – Need to form a broader scientific culture
  – University based architecture for translational, development, regulatory science and education

• Must be self sustaining
  – Virtual development institute