

Special Roles of Regulatory Authorities in Enhancing Medical Science Innovation

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Status Quo - Product Development

- **4 out of 5 (80%) potential products that start *clinical* development fail to make it to market**
- **50% of drugs that undergo *Phase 3* trials don't make it to the market:**
 - **turn out to be too unsafe or not effective enough for marketing;**
 - **the product cannot ultimately be commercially scaled-up; or**
 - **the economics of the product don't support continued development**
- **Only 15-20% that do make it to market meet various definitions of "innovative"**



What is Wrong?

- This is *failure of prediction* on a large, no longer sustainable or affordable scale



What is Wrong?

- **High Product Failure Rate Fuels Escalating Development Costs**
- **Cost of bringing a successful novel drug to market estimated at US\$800M – *including the amortized costs of all the product failures***
- **High costs drive focus on “blockbuster” drugs with widespread chronic use in economically developed economies – only way to recover overall development costs**



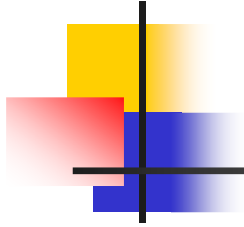
What is Wrong?

- **Decreased focus on:**
 - **Curative interventions,**
 - **Preventative interventions,**
 - **Rare or less common diseases,**
 - **Individualization of therapy,**
 - **Diseases of developing economies**
- **Decreased focus on innovation in the broader sense of the word**



Bottom Line

- **Although rate of discovery rising, fueled by investment in biomedical science (genomics, proteomics, nanotechnology, bioinformatics), there is a serious bottleneck between the laboratory and the bedside in product development**



- **Three Areas Where Regulators and Government Research Institutions Can Positively Impact Innovation**



(1) Actively Champion “Critical Path” Research: Key to Improving Prediction

- **Coordinate and Engage in the science necessary to evaluate and predict safety and efficacy earlier, and to enable consistent commercial manufacture**
 - **Different from the science that generates the new idea for a drug, biologic, or device.**
 - **Science of “development” / not “discovery”**
 - **Better choice of products to take into late development**



Regulators' Unique Perspective

- **Regulators have a unique perspective on the problem because of their access to the data surrounding so many of the failures – often data not in the public domain**



Critical Path Science Is Underdeveloped

- **Falls outside traditional areas of academic research and federal funding.**
- **When industry creates critical path tools, typically applicable to their specific products and not shared with others in industry**



Actively Champion “Critical Path” Research: Key to Improving Prediction

- **CP research is complementary to basic and translational research, but results in the creation of new validated tools for more predictive new product development.**
- **New assays; relevant biomarkers; animal, *in vitro* and *in silico* models for safety & efficacy testing; 21st century manufacturing quality control and assurance systems**



The Path Forward

- **Identify/prioritize the most severe product development problems and areas that provide the greatest opportunity -- solicit input from wide variety of sources. Recently completed.**
- **Construct a national Critical Path Opportunities List and publicize it. Presently being finalized.**
- **Re-focus FDA and NIH research - See NIH Roadmap**
- **Requested Congressional funding of top priority research projects to develop and validate these new tools**



(2) Enforce and Incentive-ize Meaningful, Appropriate Market Protection

- **Given risks, innovators must know that successful development will result in a reasonable period of return on investment**
- **Confidence in generics**
 - **Incentives for good behavior in this market;**
 - **Incentives for more efficient, validated standards for demonstrating equivalence**
- **Counterfeits**



(3) Create Incentives When Marketplace Does Not

- **Orphans – human and animal**
- **Pediatrics**
- **Terrorism Countermeasures**

- **Public policy solutions that improve access to new technologies by providing appropriate non-market incentives**



THANK YOU!
