

FDA's Critical Path Research Initiative & Intro to the CBER Research Program

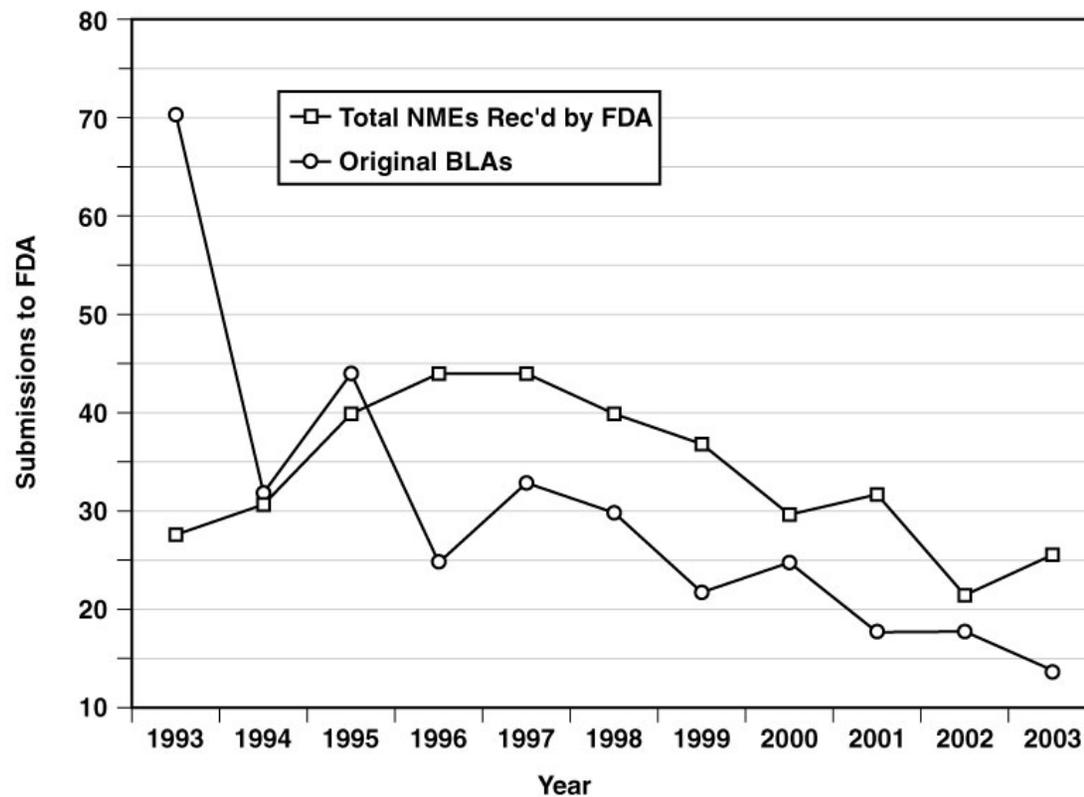


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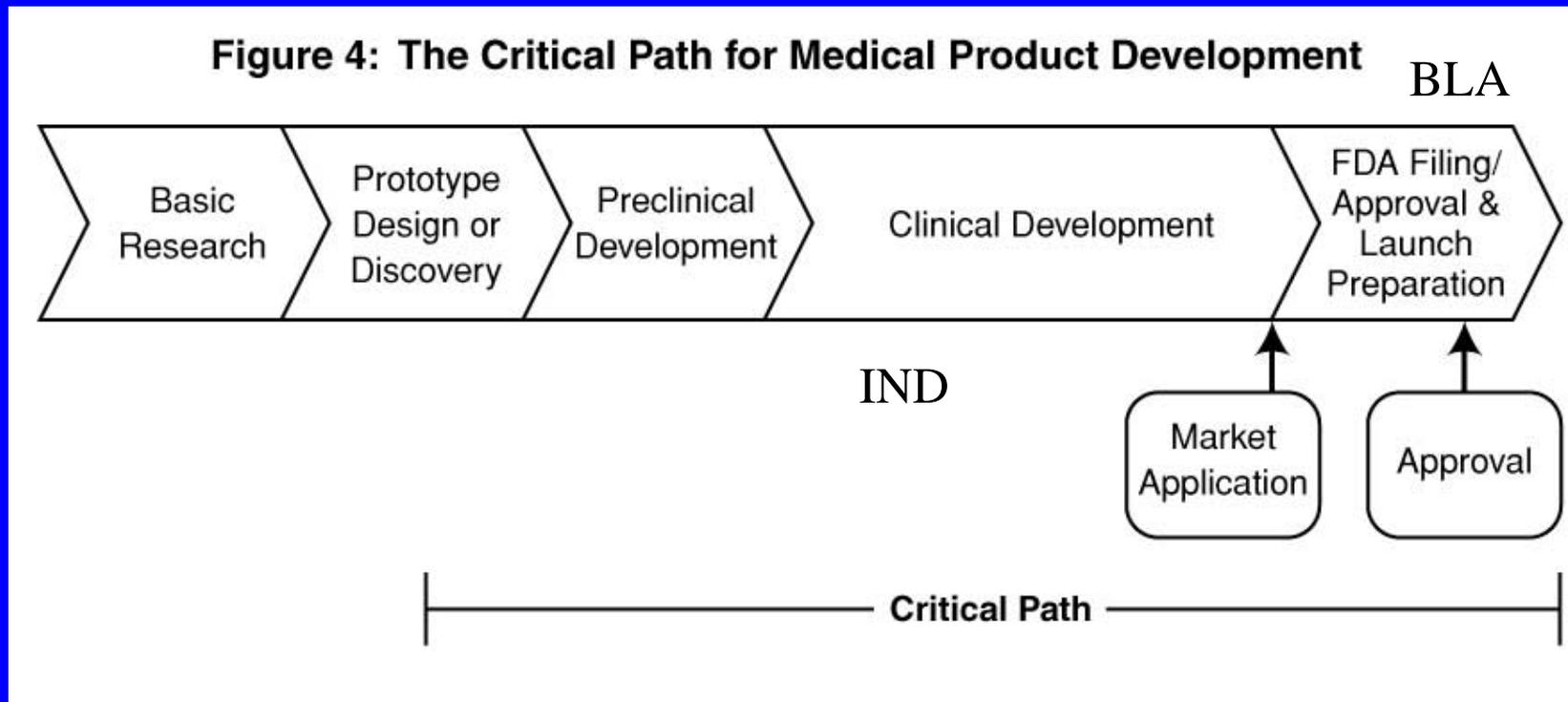


Despite basic biomedical research investment going up.....

Figure 2: 10-Year Trends in Major Drug and Biological Product Submissions to FDA



Attending to the Critical Path for medical product development



FDA Critical Path Research Initiative
www.fda.gov/oc/initiatives/criticalpath.htm

- Identify, focus upon and manage to regulatory & scientific opportunities to improve product development process and availability
 - Potency/effectiveness/standards
 - Safety
 - Consistency/manufacturing/quality
- Needed policy and guidance
- Preserve a science based FDA



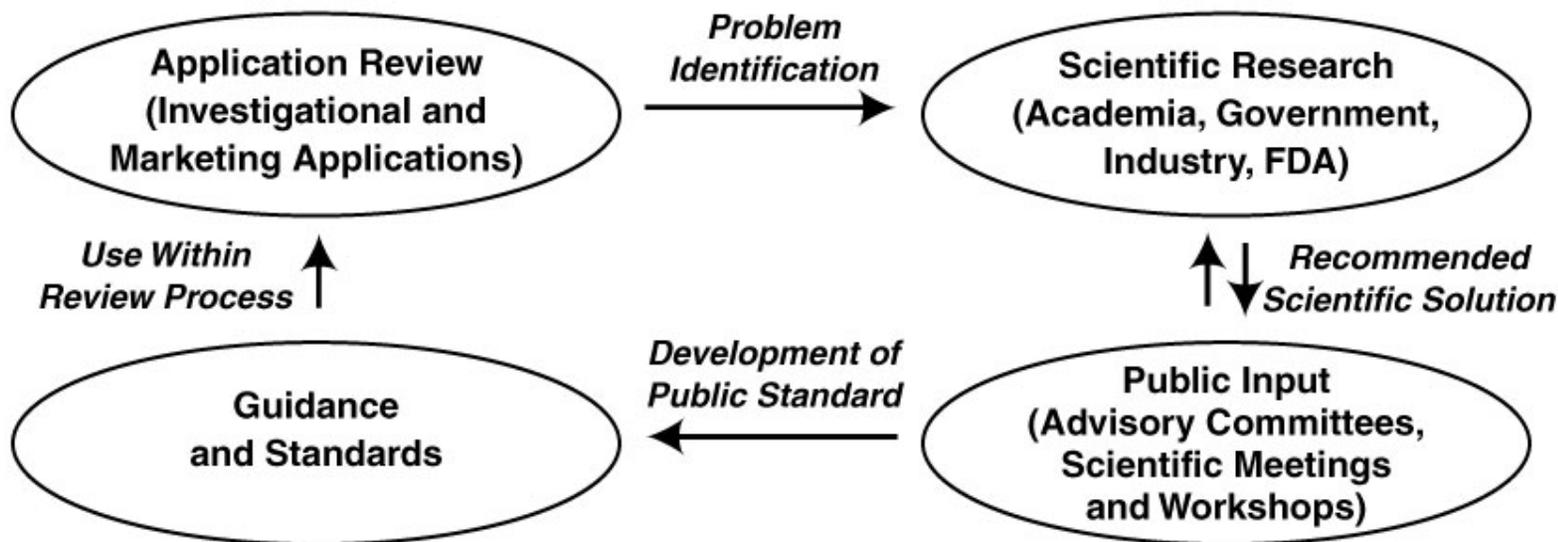
Why FDA?

- Unique perspective of the Agency vis-a-vis leads to a valuable role in convening and coordinating Critical Path Research
- Combination of FDA intramural, FDA intramural/extramural collaborations, and extramural research efforts



Integral Role of Research to Inform Policy of Product Evaluation

Figure 8: Problem Identification and Resolution During the FDA Product Review Process



Subcommittee for the External Review of CBER Research, 2/98

- *“The Researcher/Reviewer Model is essential to providing CBER with top-level expertise in a regulatory culture.”*
- Working closely with CBER Regulatory Scientists and Clinical Review Scientists to perform high quality evaluation of novel biological products



Multitasking at the FDA: Research Supports Regulatory Mission

- Research Programs organized by Product Offices
- CBER researchers are fully integrated into the regulatory process (~50% average time) = “Researcher-Regulator” model
 - Review INDs and BLAs
 - Development of Policy and Guidance Documents
 - Meeting with Sponsors and Advisory Committees
 - Participation in Pre-license and Biennial Inspections
 - Evaluation of Adverse Drug Reactions and Risk Assessment
 - Performing research relevant to product evaluation of safety, efficacy, manufacturing: Developing/evaluating scientific tools & knowledge



Mission Relevance of Research Programs

- Hundreds of Biologics Licensing Applications and Investigational New Drug Applications directly supported by research programs
- CBER research in the public domain supports development of safe and effective biologics across entire product classes



Managing Research Programs at CBER

- Evaluation of past achievements and future plans
 - External Laboratory/Res-Reg Site Visits: Four year cycle
 - Internal Management reviews: Yearly cycle using Annual *Research Program* reporting: E.g., Publications, Regulatory Policy/Guidances, Invited talks, Research QA/QC
- Office Research Site Visit conducted FY06
- Developing cross-Office Coordinated Research Expertise Teams
- CBER researchers provided with intramural support
 - Must compete for select sources of extramural funding to support research programs

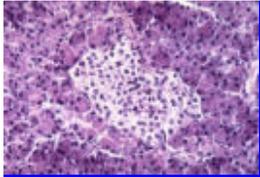


CDER Research: Supporting Innovation



- WNV blood donor screening advances: enhanced IND NAT testing = 1000+ units detected
- New tests and standards for biologic products: HIV, hepatitis, blood typing, blood cross-matching, IGIV immune globulin, α -1 proteinase, thrombin, WNV
- New safety evaluations: HBOC oxidative toxicity; prion detection and removal





Examples of CBER Critical Path Investment Opportunities

- New assays, standards, biomarkers, surrogates for complex biologics safety, efficacy and quality
- Methods & validation of pathogen inactivation for blood, plasma, tissues and other products
- Multipathogen and rapid detection methodologies
- Improving longevity/storage of blood and tissues
- Enhanced clinical trial design/analysis



CBER: INNOVATIVE TECHNOLOGY ADVANCING PUBLIC HEALTH

- We are proud of our staff and our role in public health, biodefense, product safety and availability.
- New technologies need innovative and interactive regulation, new models, standards and assays.
- Expertise and partnerships essential.
- We welcome your input.



• **Contact me: carbonek@cber.fda.gov or 301-827-0372**

