



***Creating the Path for  
Innovative New Therapies***

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**President, The Critical Path Institute**



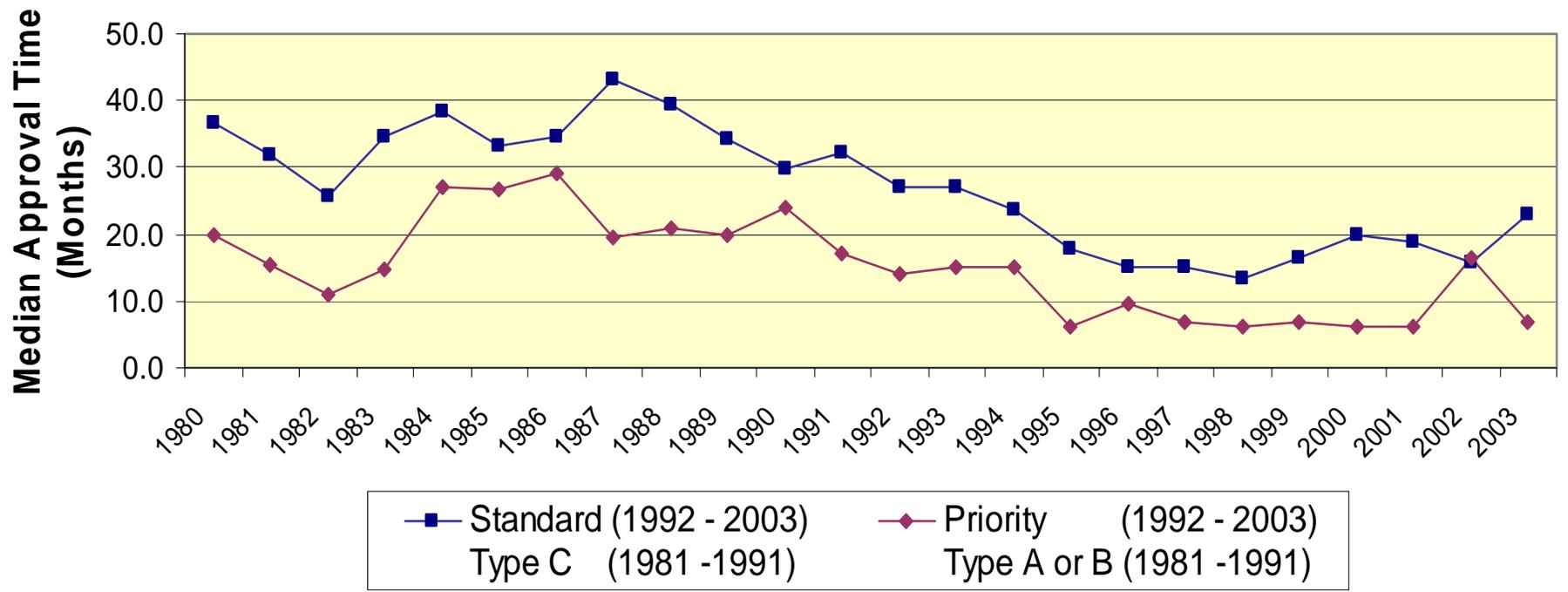
# Goals

- 1. Overview of the PDUFA Impact**
- 2. Critical Path Initiative**
- 3. Purpose of the Critical Path Institute**
- 4. A model for new relationships**
- 5. Proposal for a PDUFA initiative**



# FDA Review Efficiency Has Increased...

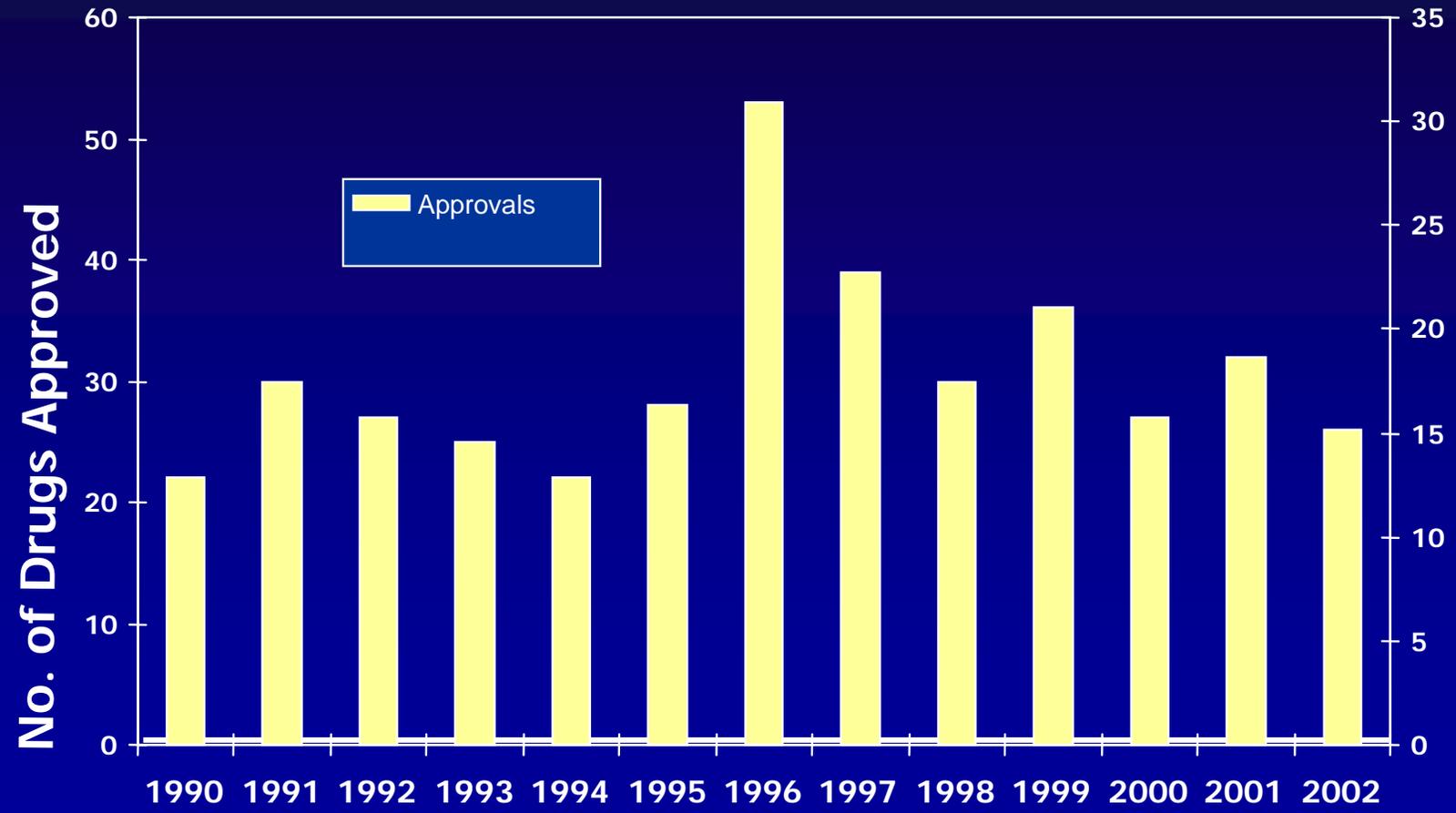
### CDER NME Median Approval Times by Calendar Year



\* Prior to 1992, therapeutic gain was classified as type A, B, or C (defined below). Starting in 1992 Priority and Standard designation was used to represent therapeutic potential for new drug approvals.



# Product Approvals Increased Transiently...

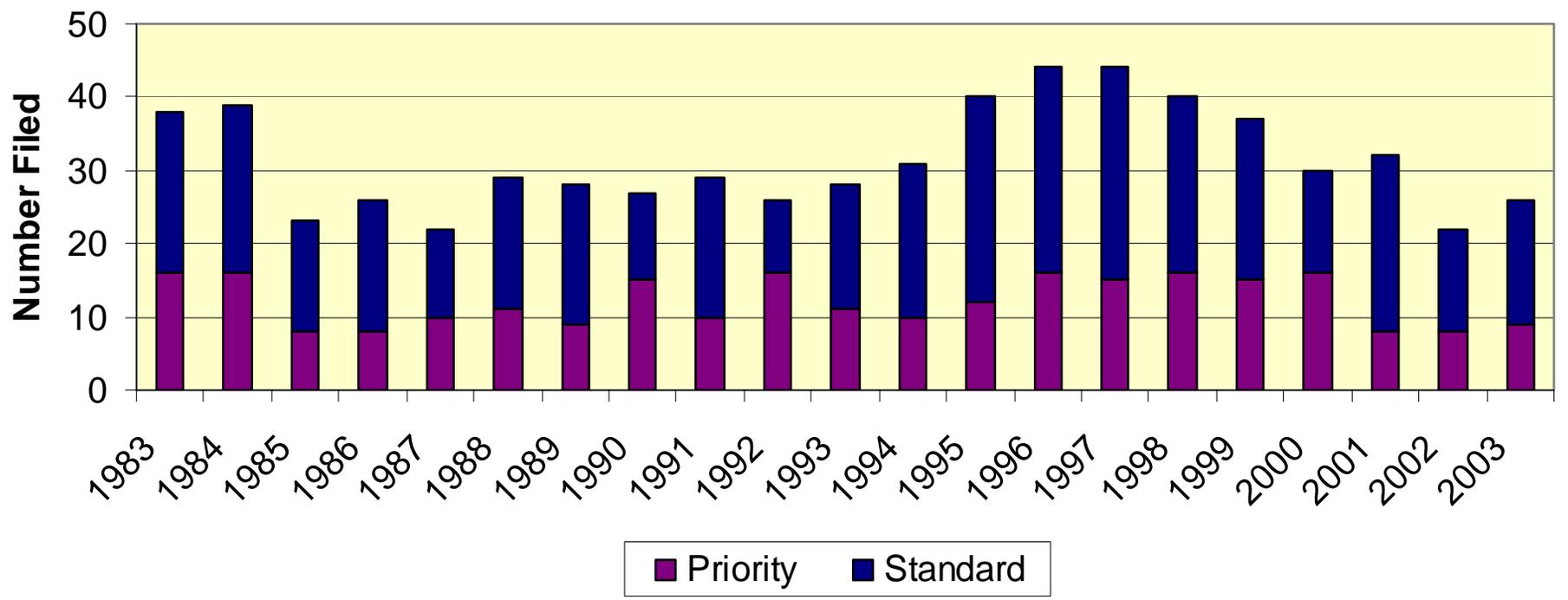


Sources: Washington Analysis, LLC and PhRMA for 1990-2000; PhRMA website for 2001-2



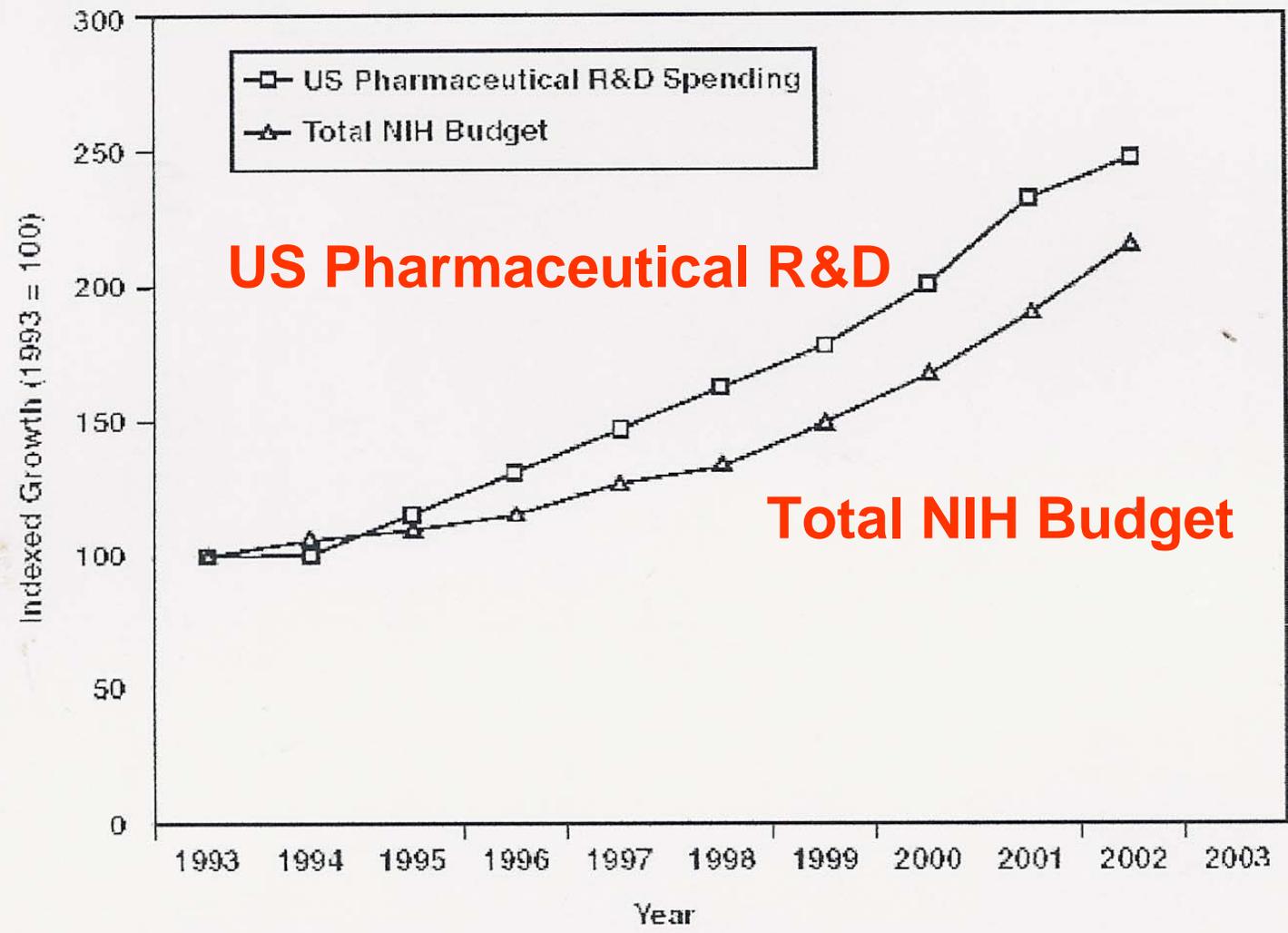
# New Drug Submissions Increased Transiently...

## NMEs Filed by Fiscal Year

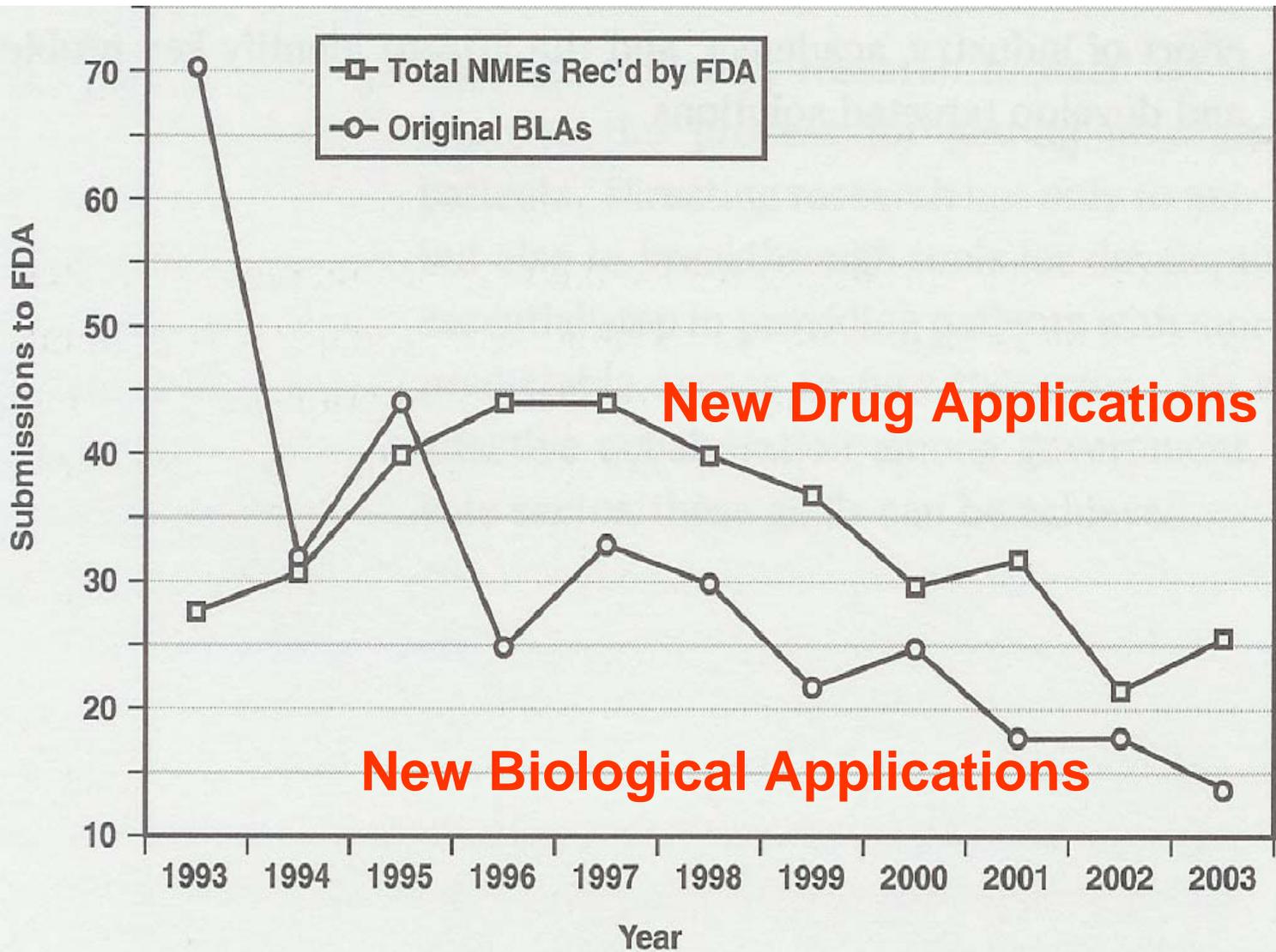


\* for NMEs submitted prior to 1992, type A and type B applications are counted as Priority review and type C applications are counted as Standard review.

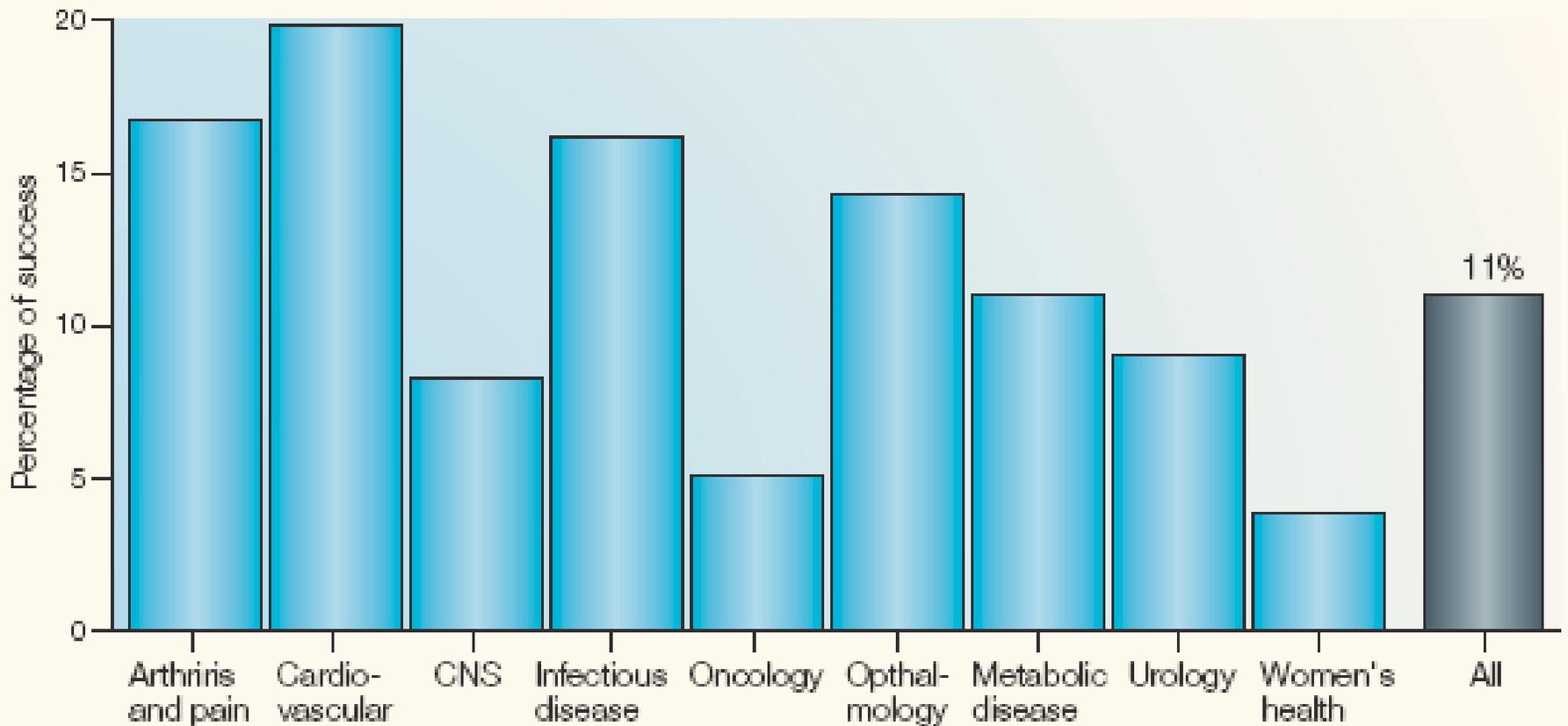
# 10 year Trend in Biomedical R&D Spending



## 10 year Trend in New Applications to FDA



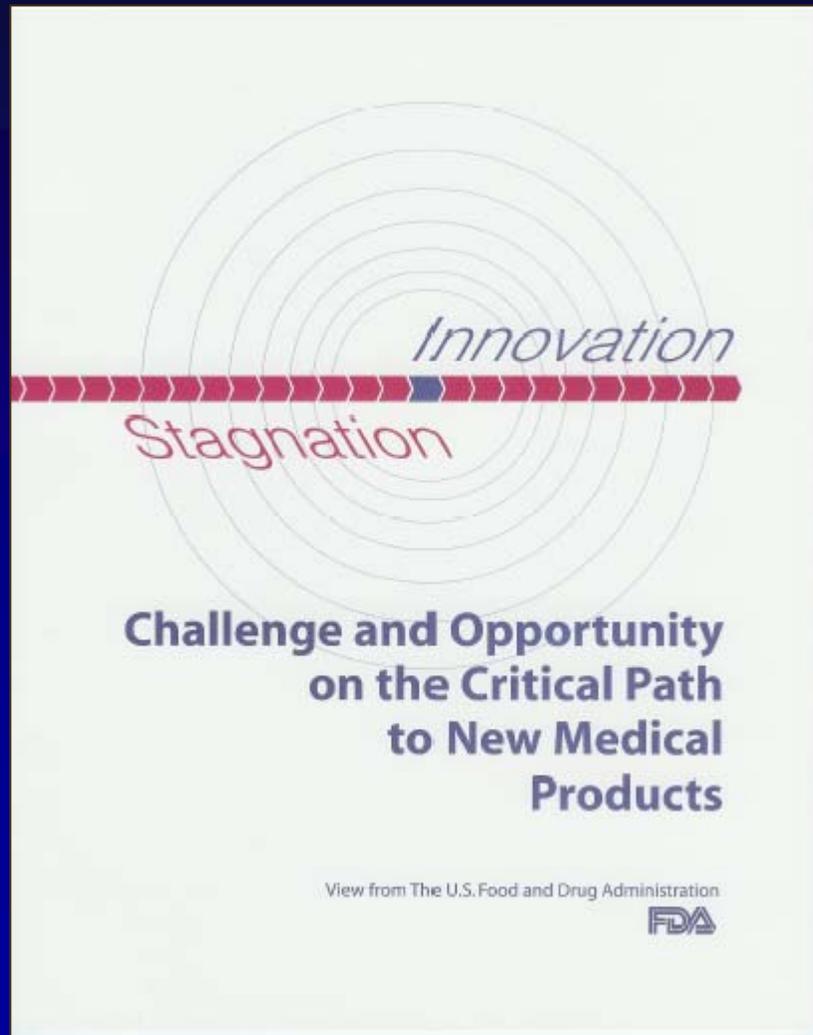
# Success Rates Remain Low



**Nature Reviews: Drug Discovery, 3 (8): 711, 2004**

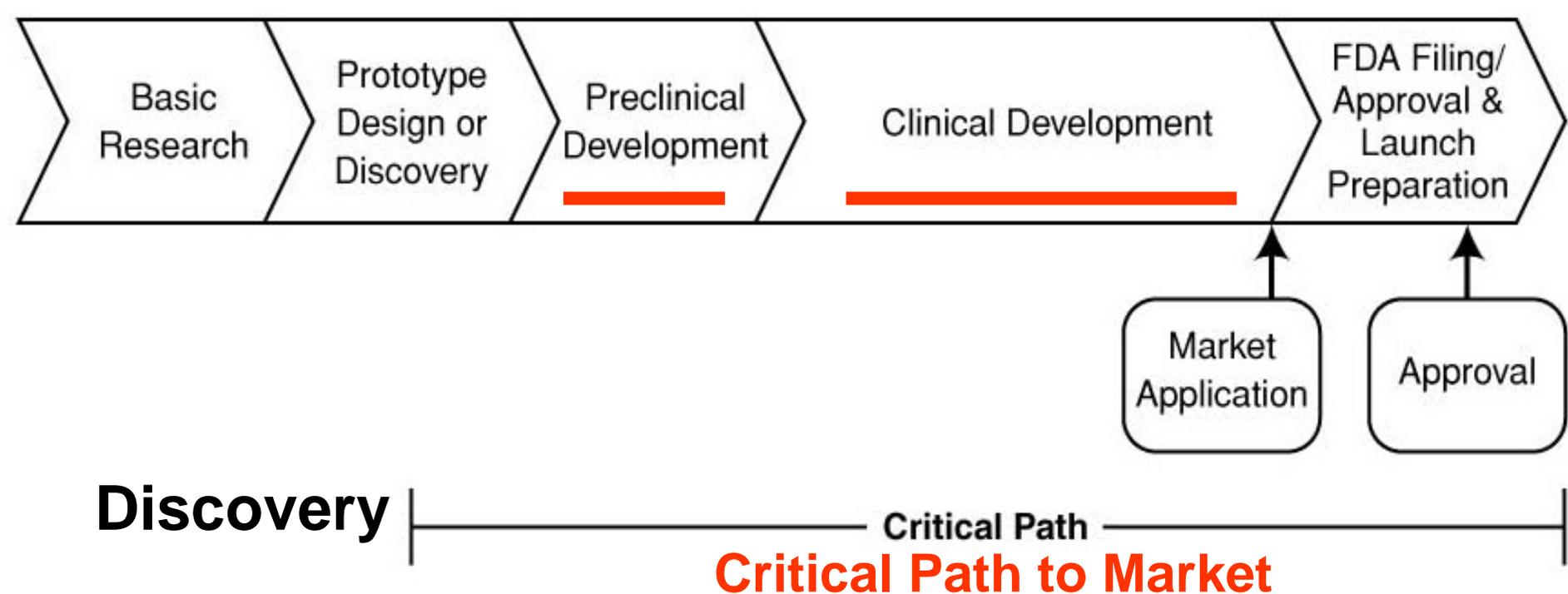


# The Critical Path Initiative





# CPI calls for Innovations and Collaboration in the Development Phases





# Response to CPI

Overwhelming support:

Industry (Internal CPI task forces)

NIH Collaborations (NCI, NHGRI)

The Critical Path Institute

Academia

**CDDS and JETS at UCSF**

**MIT Center for Biomed. Innovation**

**ISIS at Indiana Univ.**

**ECG Warehouse at Duke Univ.**

**NIPTE (11 univ. manufacturing partners)**



# Why “Critical Path Institute”





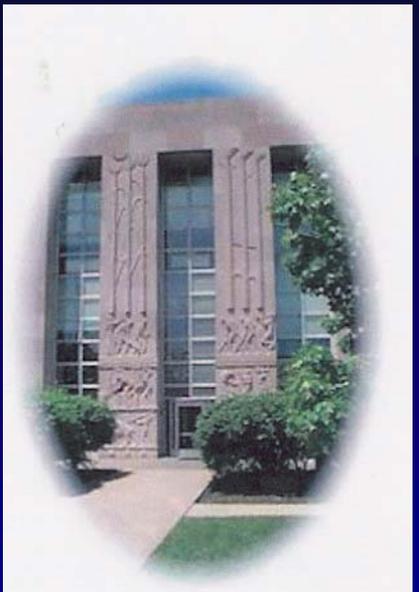
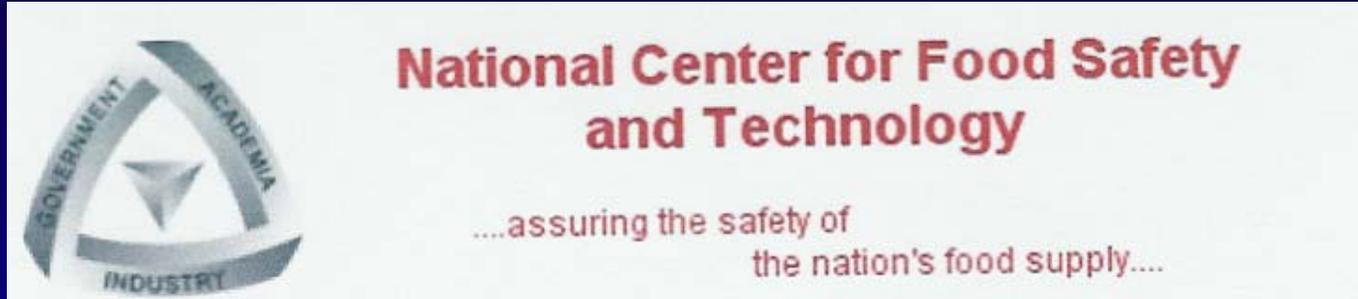
# C-Path Vision and Mission

A non-profit, publicly-funded Institute that serves as a “neutral ground” for scientists from the FDA, academia and the pharmaceutical industry to accelerate the development of safe medical products.

C-Path will develop tools not drugs

# Neutral Ground

A proven concept.....



**Moffett Center, U. Illinois, Chicago**

The NCFST is a unique research consortium composed of scientists from academia, the FDA and food-related industries. The Center provides **a neutral ground** where industry, academia and the FDA scientists address food safety issues.



# C-Path Programs

[www.C-Path.org](http://www.C-Path.org)

**FASTER**

**SMARTER**

**SAFER**



# **Toxicogenomic Cross-Validation Consortia**

- **Pharmaceutical companies have created innovative tests to predict drug toxicity**
- **Data from these tests cannot be submitted as evidence of safety for new drugs because they have not been “independently validated”**
- **Companies will disclose their methods so they can test and validate one another’s methods**
- **Focus areas: liver, kidney, muscle, nerve**
- **C-Path will gather the data and submit to FDA**



# Toxicogenomic Cross-Validation Consortia

- FDA
- SRI
- UA
- TGen
- Pharmaceutical Consortium
  - Company
    - “A”
    - “B”
    - “C”
    - “D”
    - “E”
    - “F”



# C-Path: "Neutral Territory"

## Basic Principles:

- Publicly funded, no direct funding from medical product companies
- Industry consortia funding is possible with transparency and oversight
  - Project Specific consortia funding
  - Oversight Board
    - FDA
    - Industry
    - Consumer/Patient Representatives

# Major Impediments for CPI

- **Lack of funding for FDA participation**
- **Lack of funding for method development and validation**
- **Lack of process to prioritize and coordinate CPI activities**
- **Lack of “laboratory” for testing new methods, biomarkers, etc**



# **A Proposal to Advance Drug Development:**

## **1. FDA Funding for CPI**

- **Small percent increase in PFUFA fees for FDA/CPI**
- **Match from Congressional appropriations**

# **A Proposal to Advance Drug Development:**

## **2. Funding for Methods Development/Validation**

- Industry consortia operating with FDA advisors on “neutral ground”**
- PDUFA grants/contracts for work mutually agreed upon by CPI Steering Committee**

# **A Proposal to Advance Drug Development:**

## **3. CPI Steering Committee**

### **Moffett Center Model**

- FDA representatives**
- Industry representatives**
- Consumer/Patient reps**
- Independent scientists/experts**



# **A Proposal to Advance Drug Development:**

## **4. CPI Testing Environment**

- **Life threatening illnesses**
- **Orphan drug development  
(Congressionally mandated to  
assist in development)**
- **Personalized medicine**



# Summary

**The regulators and the regulated need “neutral ground” where they can work together to improve the process of drug development.**

**PDUFA could be the catalyst for change.**

Thank You!

