

# **Stimulating Innovation In Medical Technologies**

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# **Innovation and the Pharma Industry**

**Pharmaceutical research has historically been extremely productive**

- **Between 1993 and 2003, FDA approved more than 300 new drugs and vaccines for over 150 different conditions**
- **More commercial INDs active in 2002 than a decade before**
- **More than 1,000 medicines now in development**
- **New medicines have changed the standard of care for many diseases, such as HIV infection**

# Emerging Trend is Less Positive<sup>1</sup>

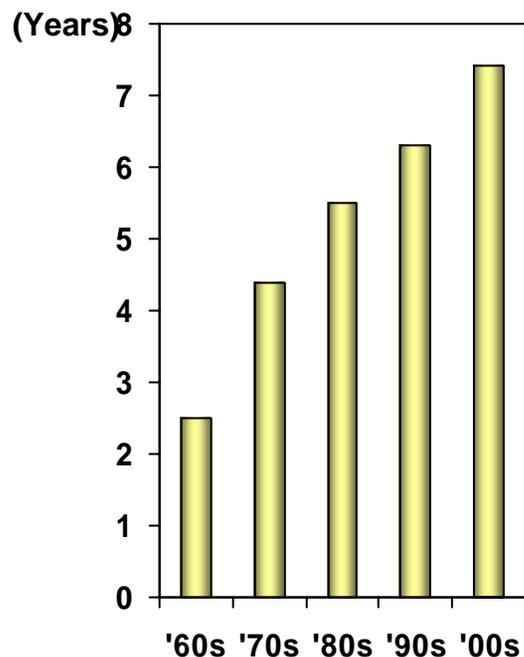
**Despite huge successes of the past decade, scientific and regulatory barriers to innovation have grown**

- **Costs of innovation are enormous, and climbing**
  - **10-15 year R&D effort**
  - **\$1.1 billion investment**
- **Output is declining in spite of this**

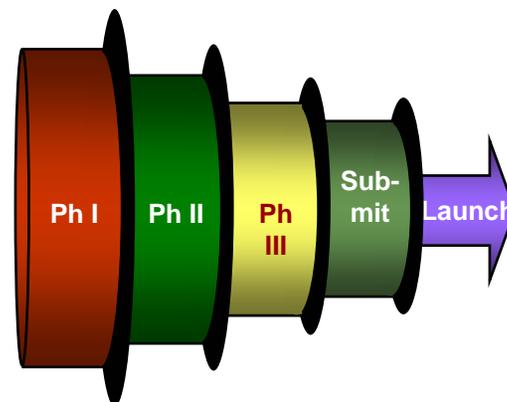
# Attrition Trends and Development Times are Deteriorating

## Clinical timelines increasing

Mean clinical development time<sup>(1)</sup>



## Success rates deteriorating<sup>(2)</sup>



Period	Ph I	Ph II	Ph III	Submit	Launch	Cumulative success rate
1994–1997	5.6	3.8	1.8	1.2	1	18%
1998–2000	11.7	6.9	1.9	1.1	1	9%

Cumulative success rate

Modeled clinical development costs also rising from \$176M per successful candidate in '96-'99 time period to \$340M in '00-'03 time period

(1) Tufts Center for Study of Drug Development, DiMasi; E. Schmidt and R. Wong, Nature Reviews, December 2003; CMR data / BCG analysis

(2) CMR data; BCG analysis

# Even Extraordinary Phase III Success Cannot Compensate For Increase In Phase I-II Attrition

Scenario	Success Rates (%)				
	Phase I	Phase II	Phase III	Approval	Cumulative
1996 – 1999 (Actual)	69	46	66	86	18
2000 – 2003 (Actual)	59	28	56	93	9

If Phase I success rates remain at the new lower levels while Phase II recovers, Phase III success rates will have to improve to 71% in order to return to the historical cumulative success rate of 18%:

Reduced Phase I success rates:	59	46	71	93	18
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If both Phase I & II success rates remain at their new lower levels, Phase III success rates would have to improve to an unattainable 117% in order to generate historical cumulative success rate of 18%:

Reduced Phase I & II success rates:	59	28	117	93	18
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# Evolution In Competitive And Regulatory Environment Driving Declining Performance

## Industry Trends

### Market pressures

Blockbuster drug classes more crowded

Payers reticent to pay for drugs with incremental benefits

Less unmet need in highest opportunity diseases<sup>(1)</sup>

### Regulatory pressures

Increase in scientific knowledge about drug safety

Evolving risk-benefit threshold

New requirements, e.g. pediatric, LFT, QT,

Market place more competitive and demanding increasing differentiation

Regulatory burden increasing and more variable across TAs/diseases

## Implications

Increasingly difficult to differentiate products

Increase in pipeline novelty and shift in disease mix

Sponsors pursue more risky development strategies

Increasing clinical trial costs

Increasing FDA variability across Divisions

Burden higher due to uncertainty around FDA expectations/ requirements

(1) e.g., depression, hypertension, high cholesterol, schizophrenia

# **Need a Systematic, Solution-Based Approach to Drivers of this Decline**

**PhRMA welcomes HHS Innovation Initiative**

# What Can HHS Do?

**Fully support FDA's Critical Path Initiative**

**Facilitate identification and validation of biomarkers and surrogate endpoints**

**Encourage NIH basic research into disease mechanism of action**

**Facilitate research and reimbursement for primary prevention products**

**Support payment policies that encourage continued innovation**

**Educate the public and policymakers about the need for innovation-friendly public policies**

# **Support FDA's Critical Path Initiative**

**FDA is uniquely positioned to understand and address the scientific and regulatory hurdles associated with drug development**

## **FDA's Critical Path Initiative:**

- Should be the primary focus of HHS efforts to spur innovation;**
- Should be adequately funded; and**
- Should be coordinated with other important initiatives, such as the NIH Roadmap**

# **Facilitate Biomarker Research**

**Increased use of biomarkers and surrogate endpoints could streamline clinical testing and approval pathways for many drugs**

**Collaboration between FDA, NIH and industry could facilitate biomarker research**

**There needs to be close coordination between FDA, NIH and industry to ensure that:**

- Appropriate candidates are chosen; and**
- Research meets scientific and regulatory needs**

**Collaborative model unclear, but PhRMA is willing to help explore this concept further**

# **Basic Research By NIH**

**There is a need for basic research into the mechanisms of disease progression for many debilitating diseases (eg osteoarthritis)**

**Basic research could spur development of innovative treatments by identifying new targets**

**NIH is perfectly situated to fund and/or conduct this type of basic research (and already conducts much of it)**

**NIH should expand its current basic research programs and ensure that there is good coordination with FDA and industry**

# **Primary Prevention Products**

**Prevention products often are the most cost-effective treatments**

**Significant regulatory, reimbursement and other barriers often impede research and development of prevention products**

**HHS should clarify and streamline the approval process for prevention products, including increased use of surrogate endpoints**

**HHS should encourage reimbursement policies that support effective delivery of prevention products**

# **Establish Payment Policies That Support Innovation**

**Current payment policies are a significant barrier to diffusion of innovation and access to recommended care**

**Government payment policies should:**

- **Value choice and competition in healthcare;**
- **Recognize the importance of incremental innovation;**
- **Improve the timeliness and openness of coverage, coding and payment decisions; and**
- **Keep pace with innovation in technology (eg Personalized Medicine) and healthcare delivery (eg disease management programs)**

# **Education on Innovation**

**Innovation is not guaranteed; it requires appropriate public policies**

**There is a need to educate the public and policymakers about the difficult, risky and uncertain nature of the drug discovery process**

**HHS can play an important role in educating the public and policymakers in this area**

**Such education will help ensure that the US retains and encourages policies that support continued leadership in medical innovation**