



# Monitoring the Progress of Clinical Trials

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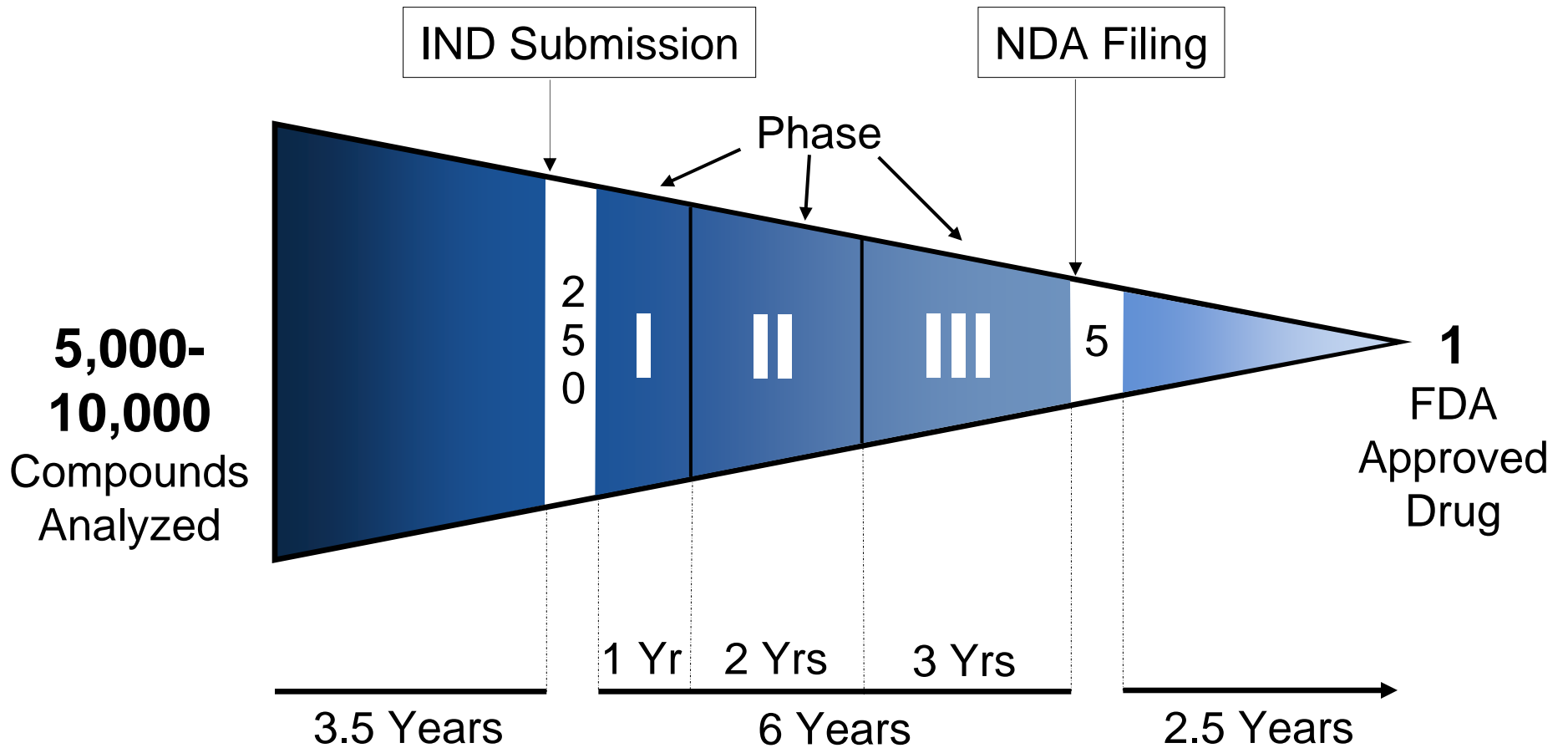
# Overview

- Clinical Trials and Drug Approval Process: Cost and Probability of Success
- Market Perspectives: How Do We Fund R&D?
- Monitoring Progress of Clinical Trials
- Influencing Research and Development: Patient Advocacy

# Overview

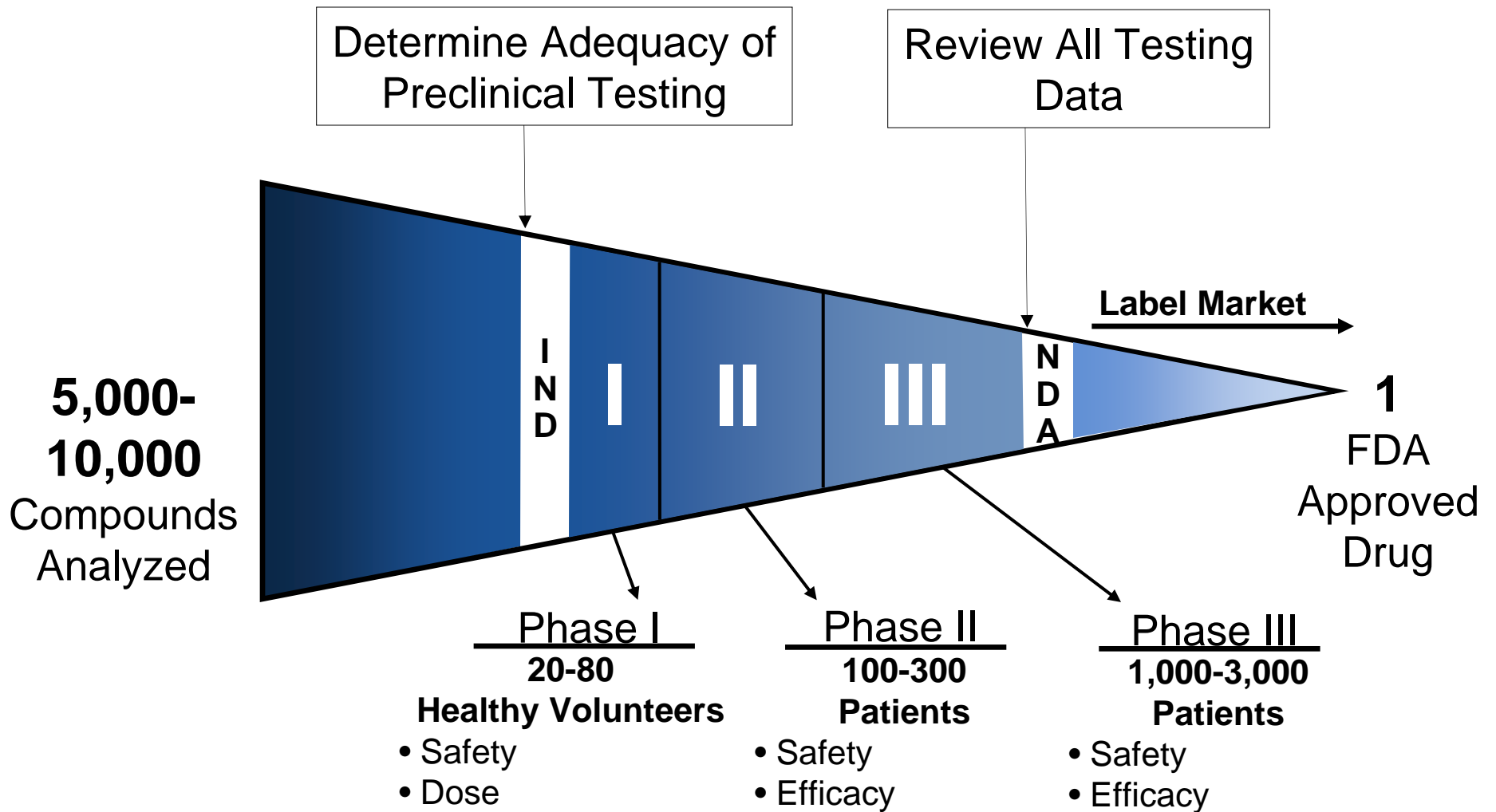
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# Drug Approval Process: Timing



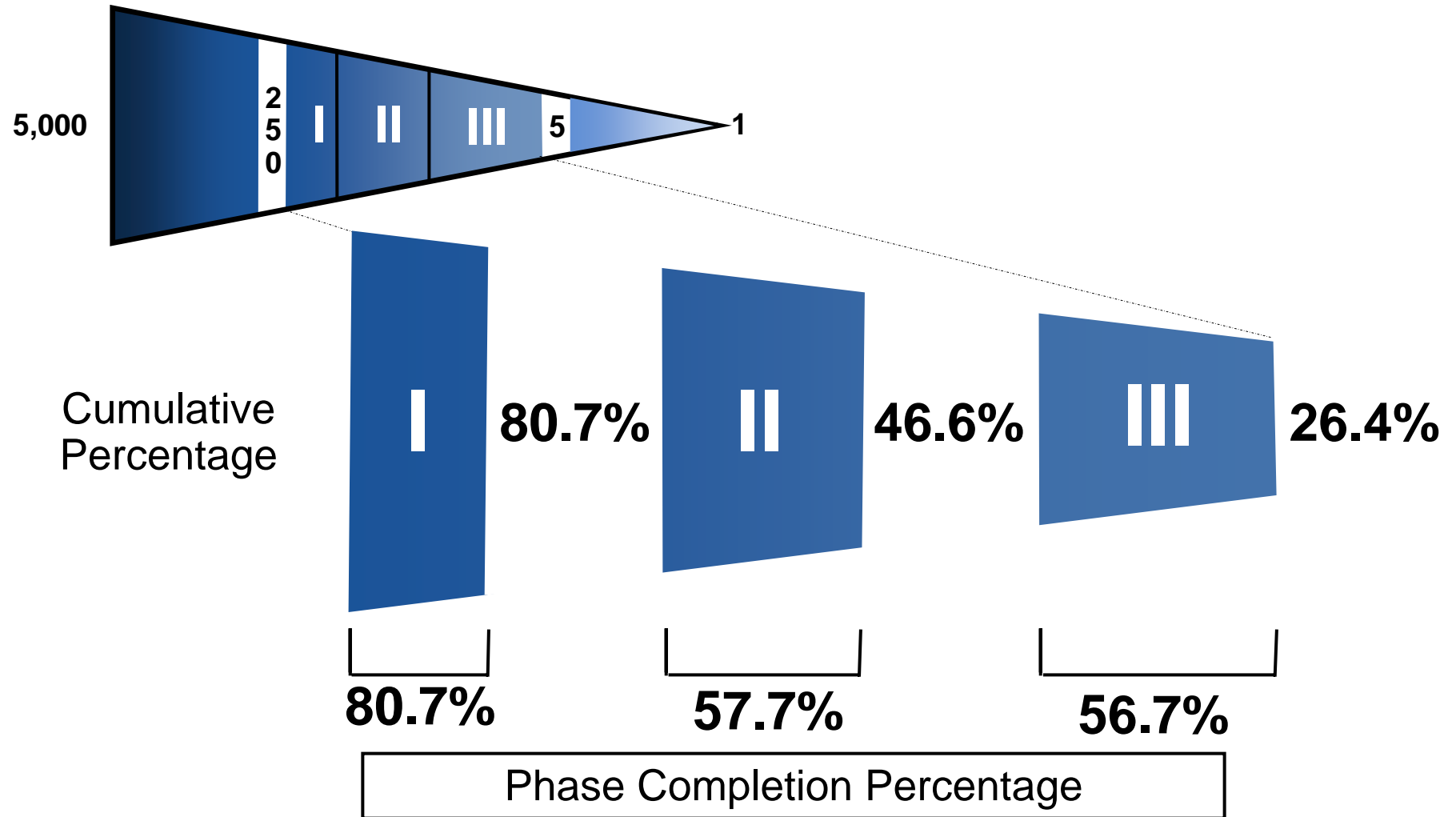
Source: Pharmaceutical Manufacturers Association "Phases of Product Development"

# Drug Approval Process: Beginning to End



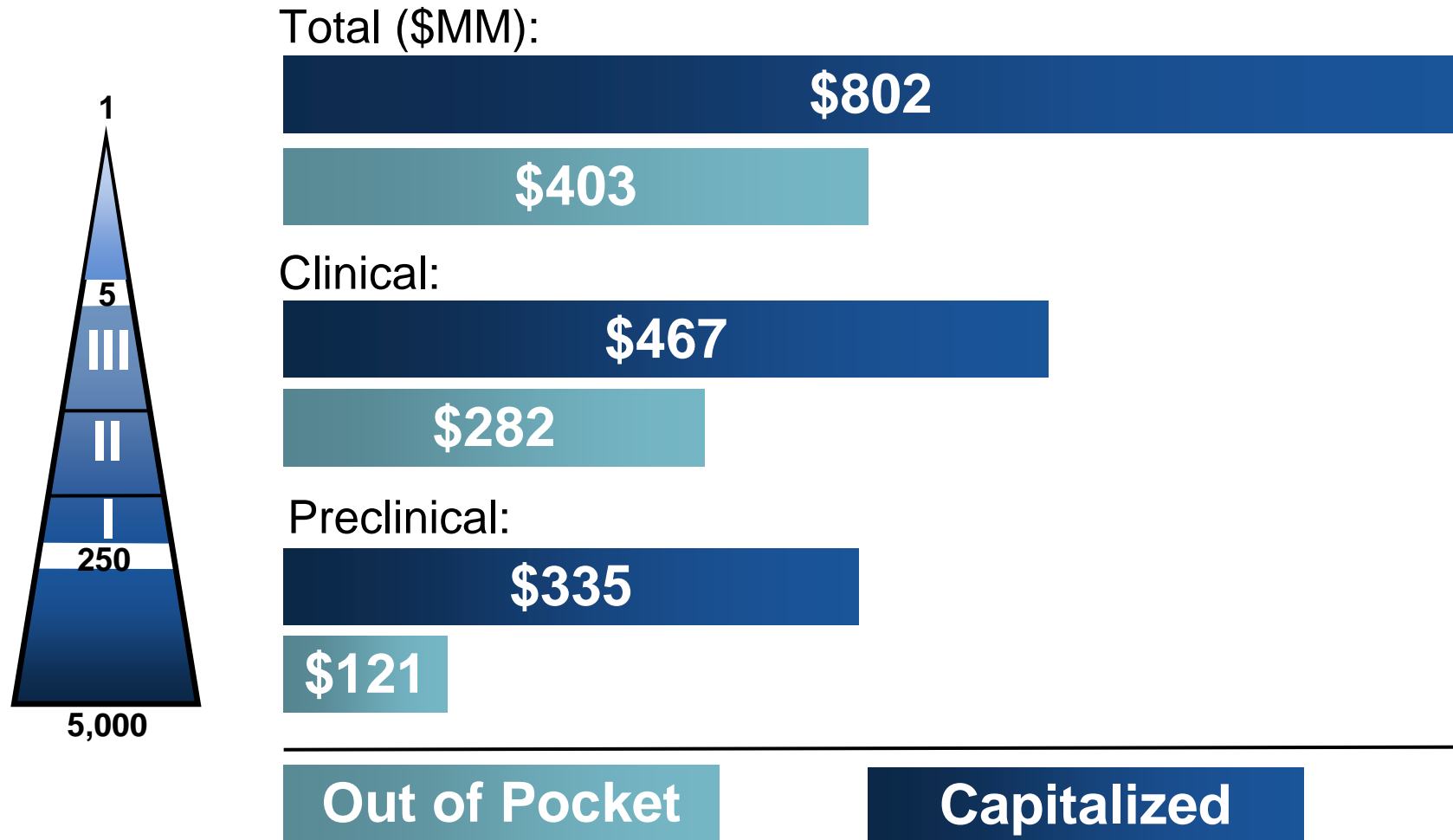
Source: Pharmaceutical Manufacturers Association "Phases of Product Development"

# Drug Approval Process: Probability of Success



Source: Metz et al "White Paper" p. 7

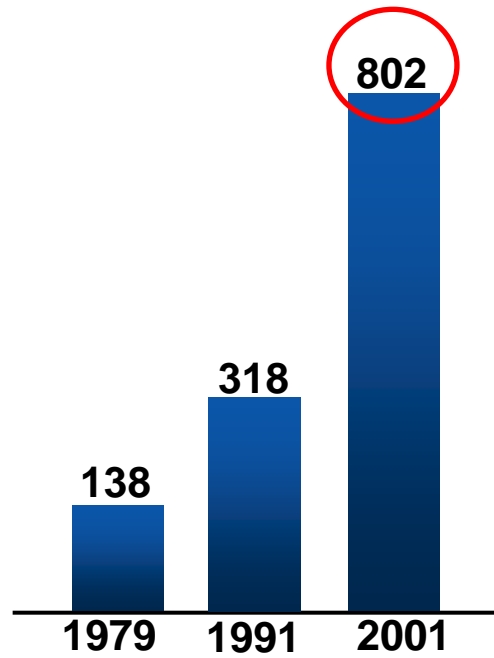
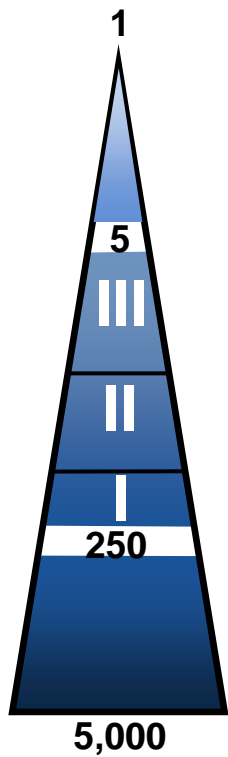
# Drug Approval Process: Costs



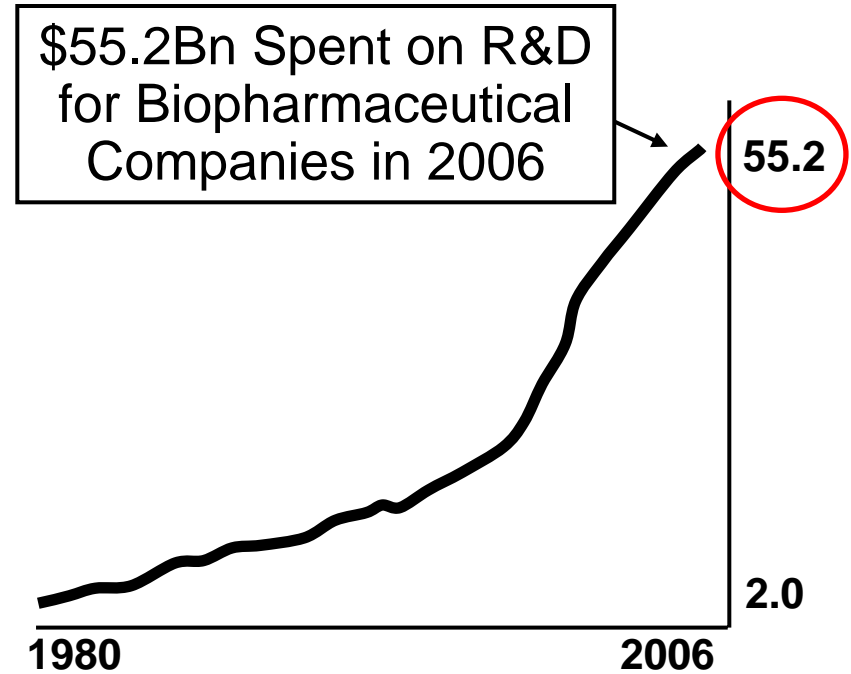
Source: DiMasi, Hansen, Grabowski, "The price of innovation" 2002

# Production Cost Over Time

## Tier 1 Biotech



Rising Drug Costs<sup>(1)</sup>



Increasing R&D Expenditures<sup>(2)</sup>

Source:

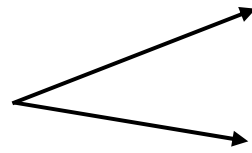
(1) DiMasi, Hansen, Grabowski, "The price of innovation," 2002

(2) PhRMA, "Profile2007," 2007



# Drug Approval Process: FDA Over Time

High Profile  
Safety Concerns



**VIOXX**



**Ketek**  
TELITHROMYCIN tablets

Removing Products  
From Market



**Zelnorm**

Rejections Based  
on Safety Concerns



**ARCOXIA**  
(etoricoxib)

Increased Push for  
Phase 4 Trials



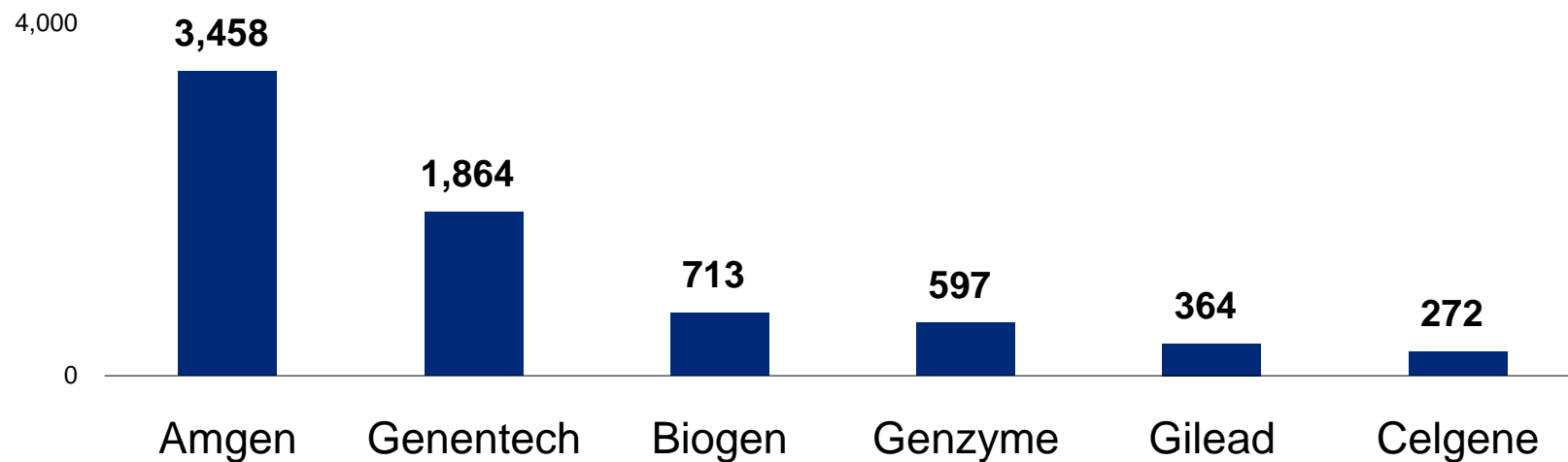
**Phase IV**

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# Market Perspective: R&D Spend

## Tier 1 Biotech



**Market Cap  
(\$Bn)**

**\$68.4**

**\$81.1**

**\$17.6**

**\$17.6**

**\$37.6**

**\$22.8**

**Rank**

**#2**

**#1**

**#6**

**#5**

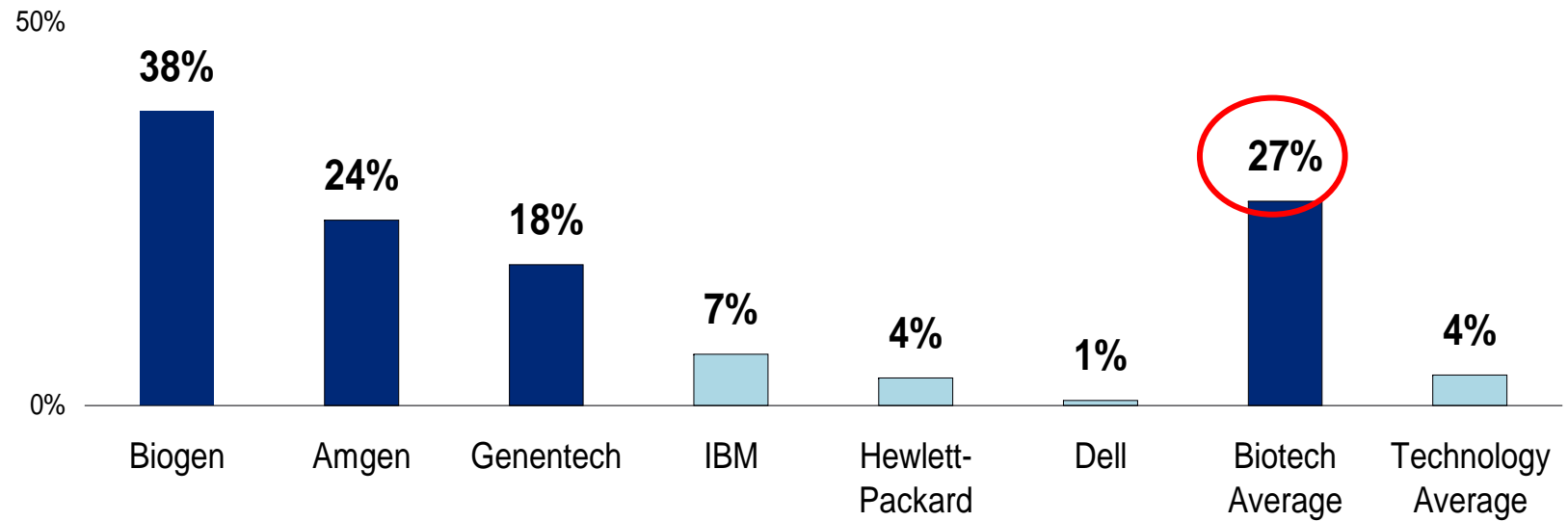
**#3**

**#4**

Source Wall Street Research, CapIQ

# Market Perspective: R&D Spend

## R&D as a Percentage of LTM Revenue

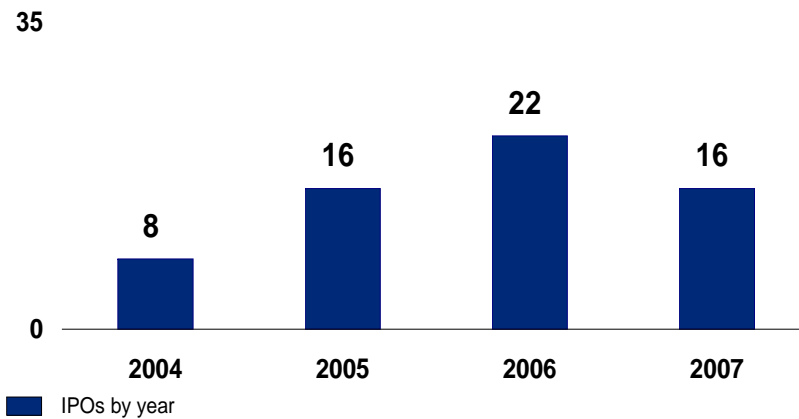


### Comparison Analysis: Biotech vs. Technology

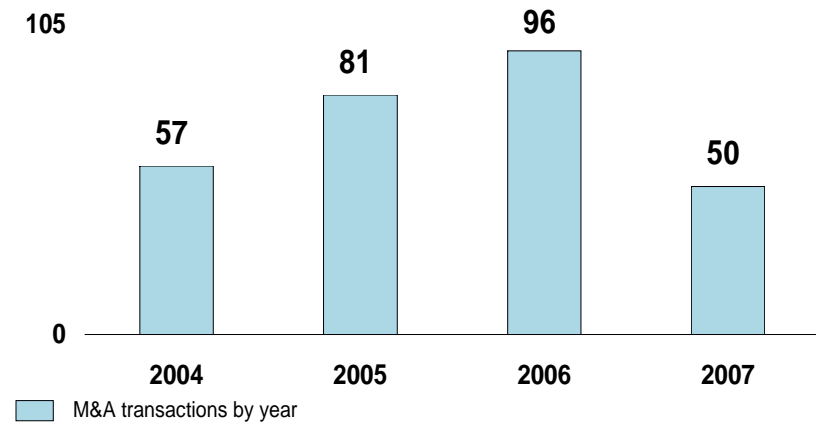
Source Morgan Stanley Research

# Investors and Competitors Willing to Support R&D

## Recent Investment and M&A Activity



Biotech and Emerging Pharma  
IPOs  
Since 2004



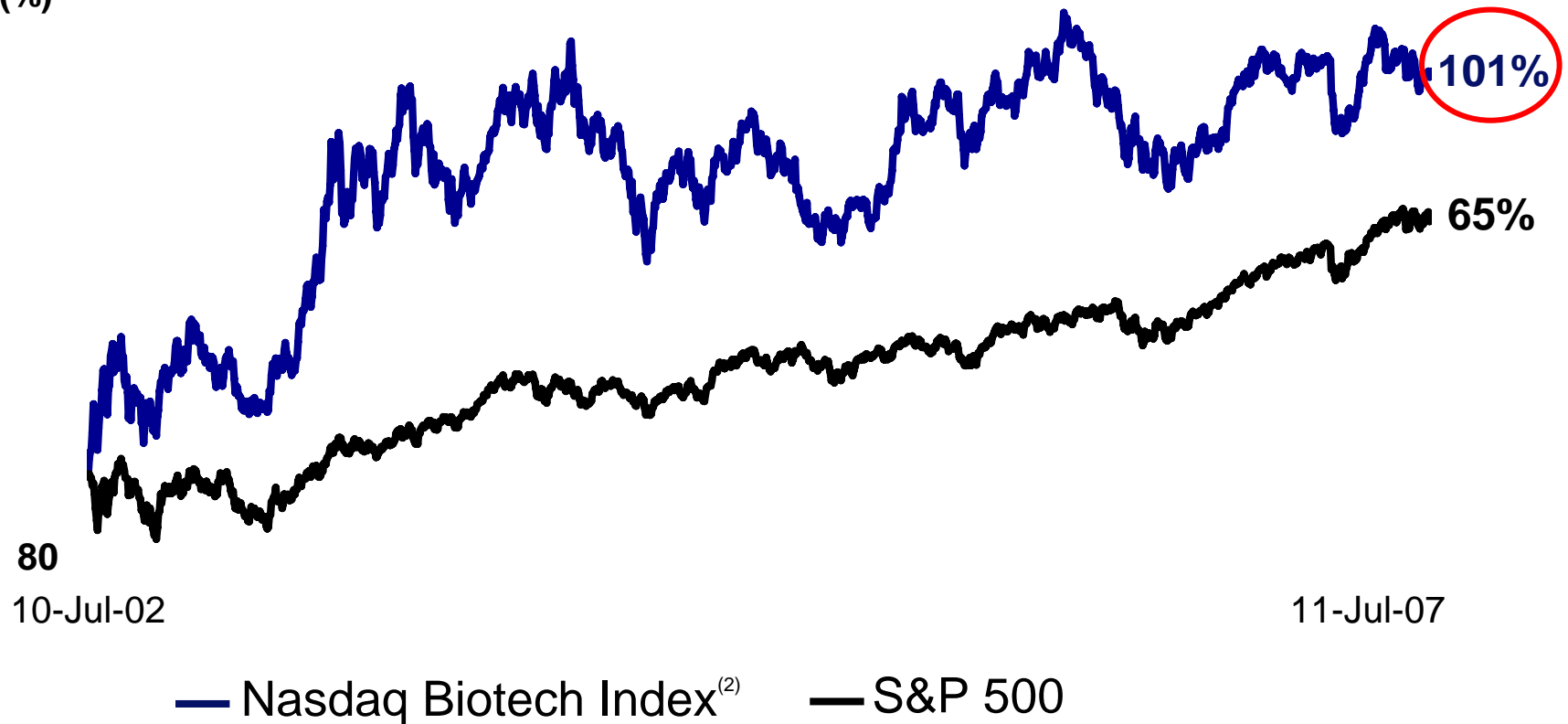
Biotech and Emerging Pharma  
M&A Transactions  
Since 2004<sup>(1)</sup>

Source: Morgan Stanley Research  
Notes: (1) Based on transactions greater than \$100MM

# Market Perspective: Investor Return

## 5 Year Indexed Price Performance Analysis

(%)



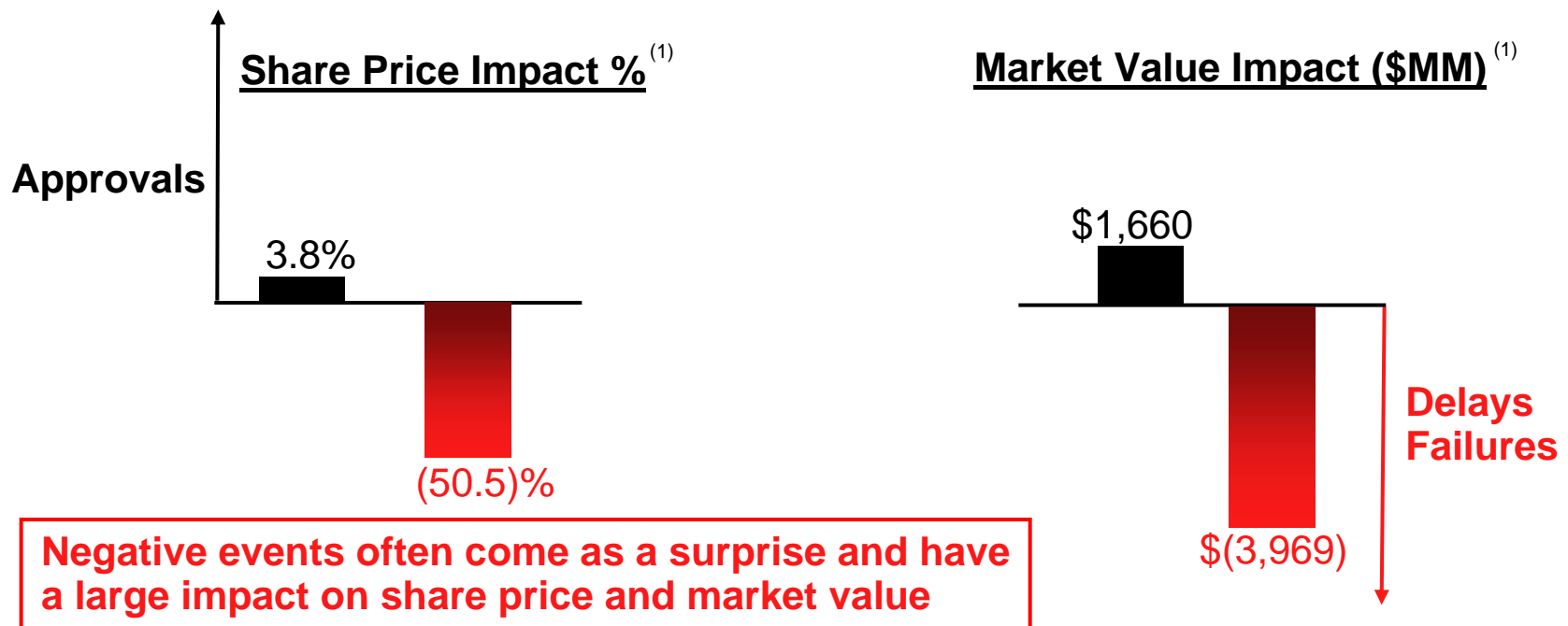
Source: FactSet  
Notes: (1) As of Jul 11, 2007  
(2) Nasdaq Biotech Index

Morgan Stanley

# Market Perspective: Investor Return

## Impact of Positive and Negative Events

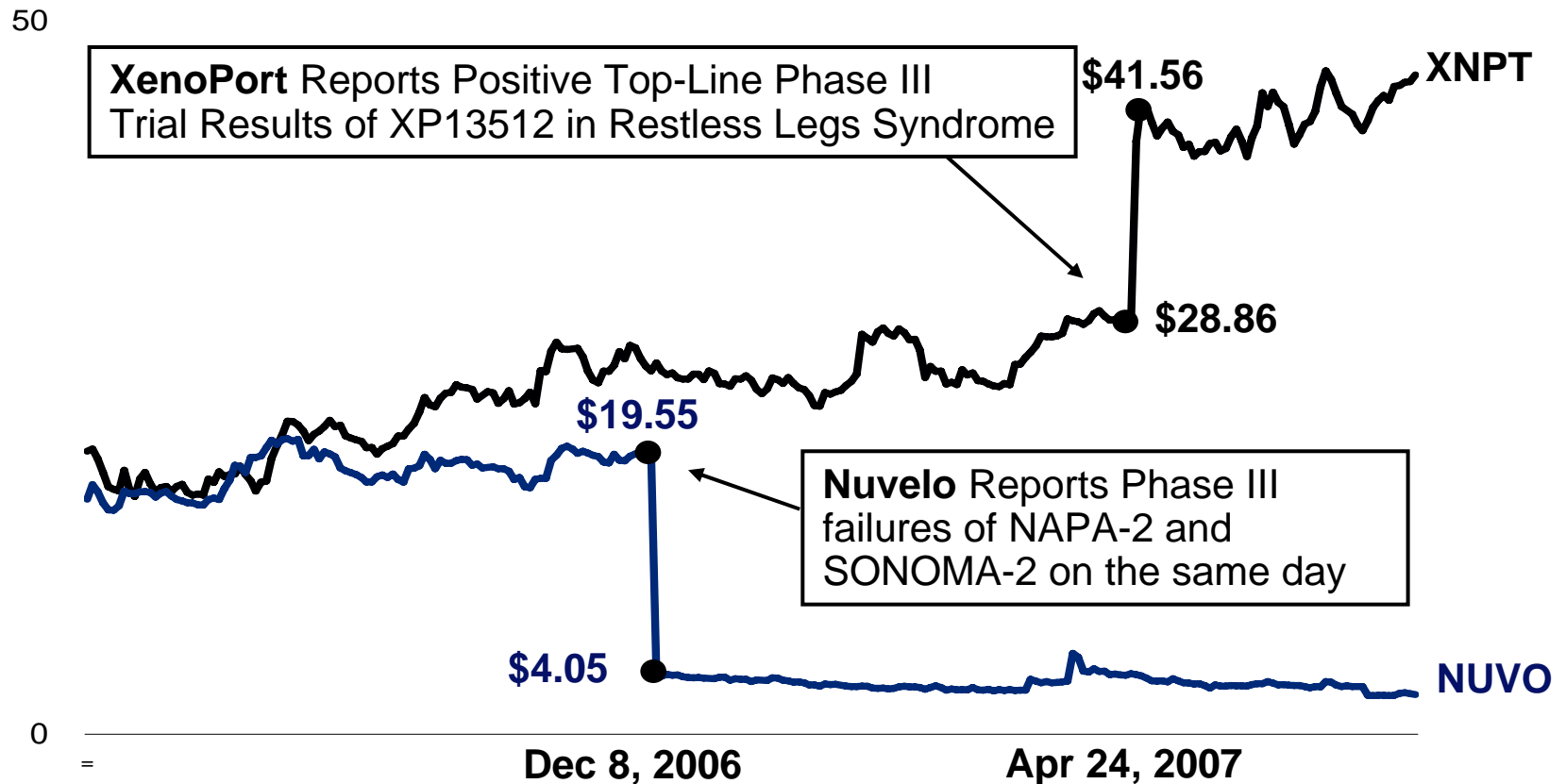
Positive events are factored into valuations and do not drive share price or market value much



Source: Wall Street Research, CapIQ  
Notes: (1) Values from selected high profile events

# Case Study: Value Creation and Destruction by Event

## XenoPort vs. Nuvelo

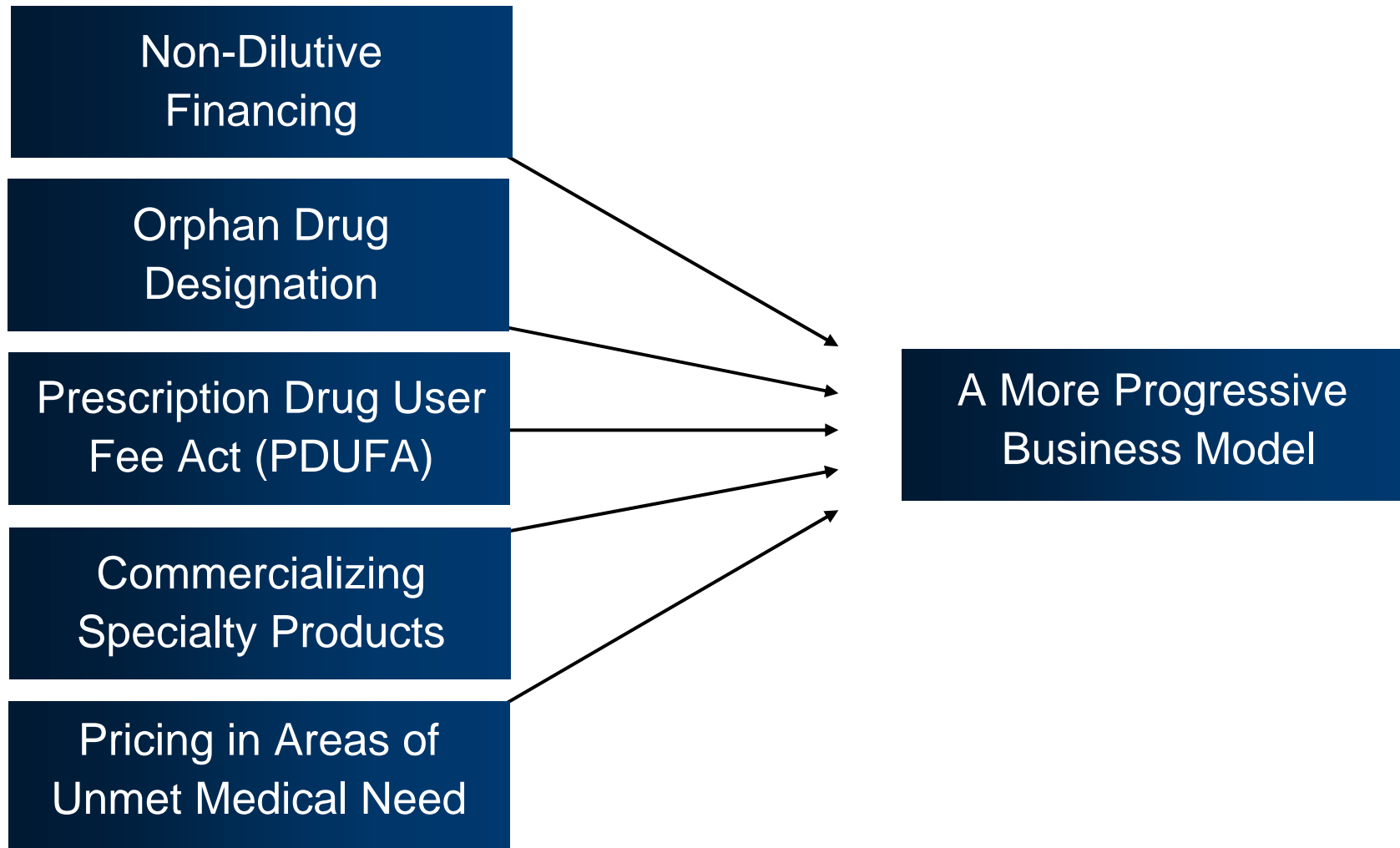


Source: FactSet  
Notes: (1) As of Jul 11, 2007

Morgan Stanley



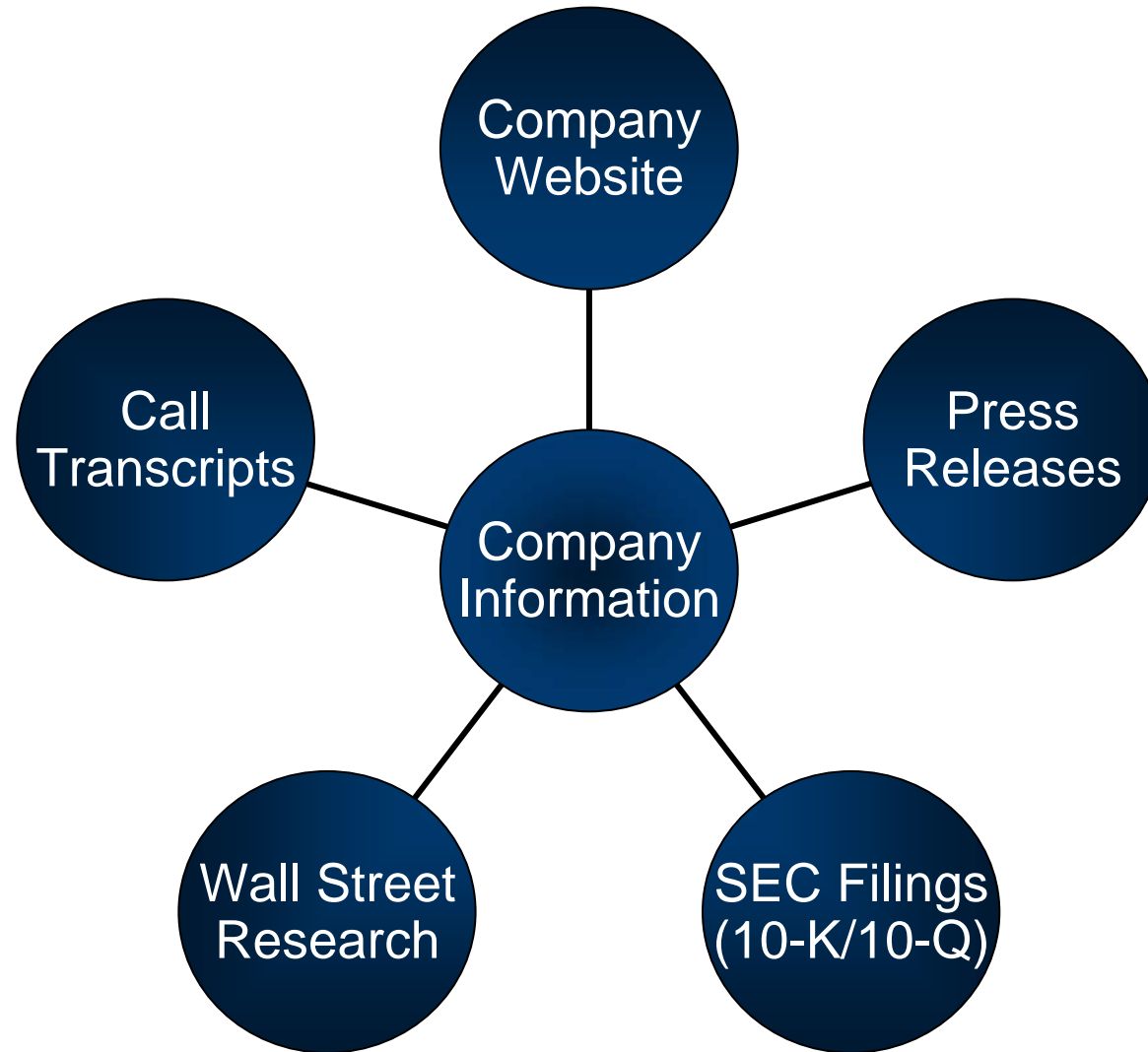
# Changing the Equation: Business Model



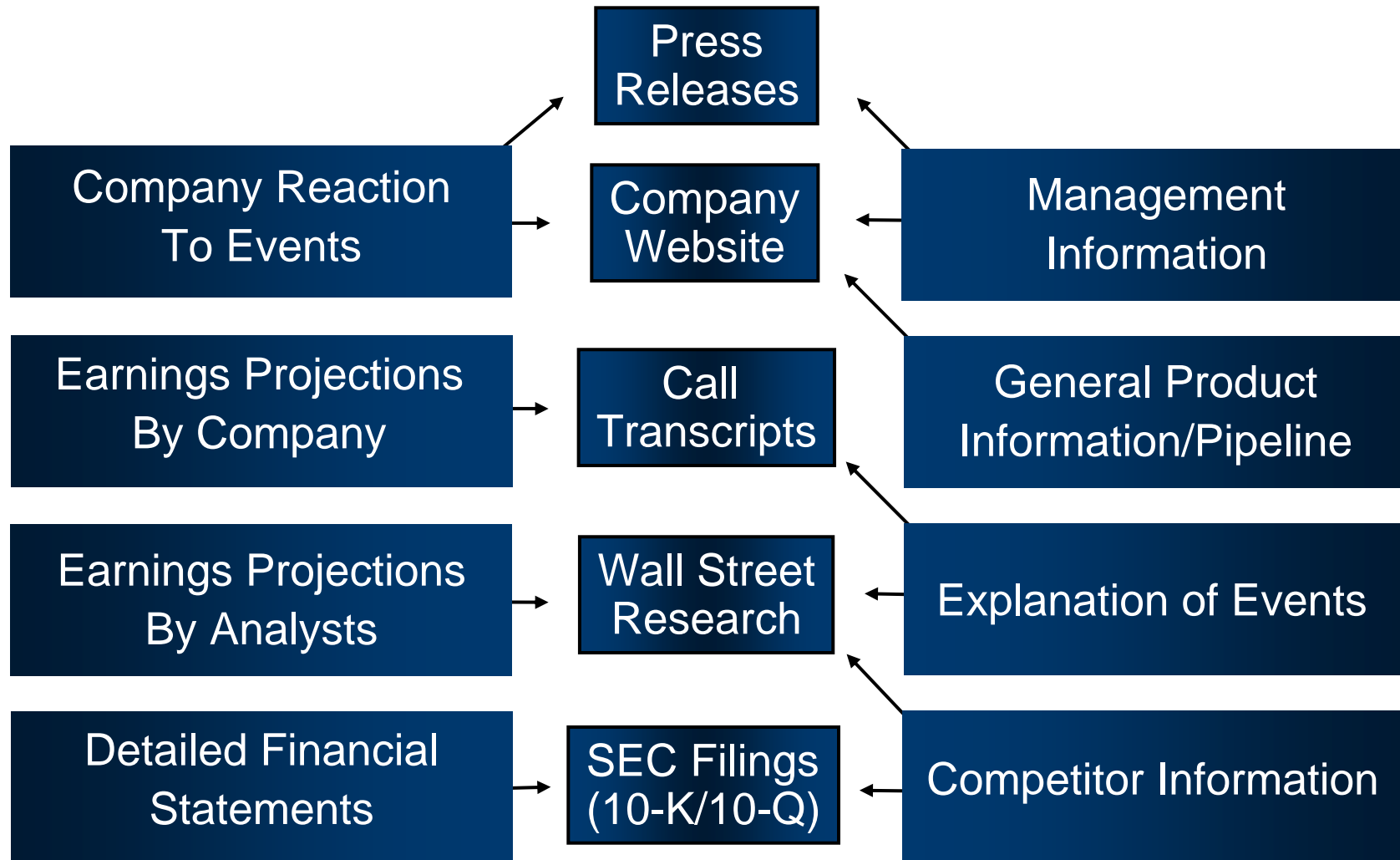
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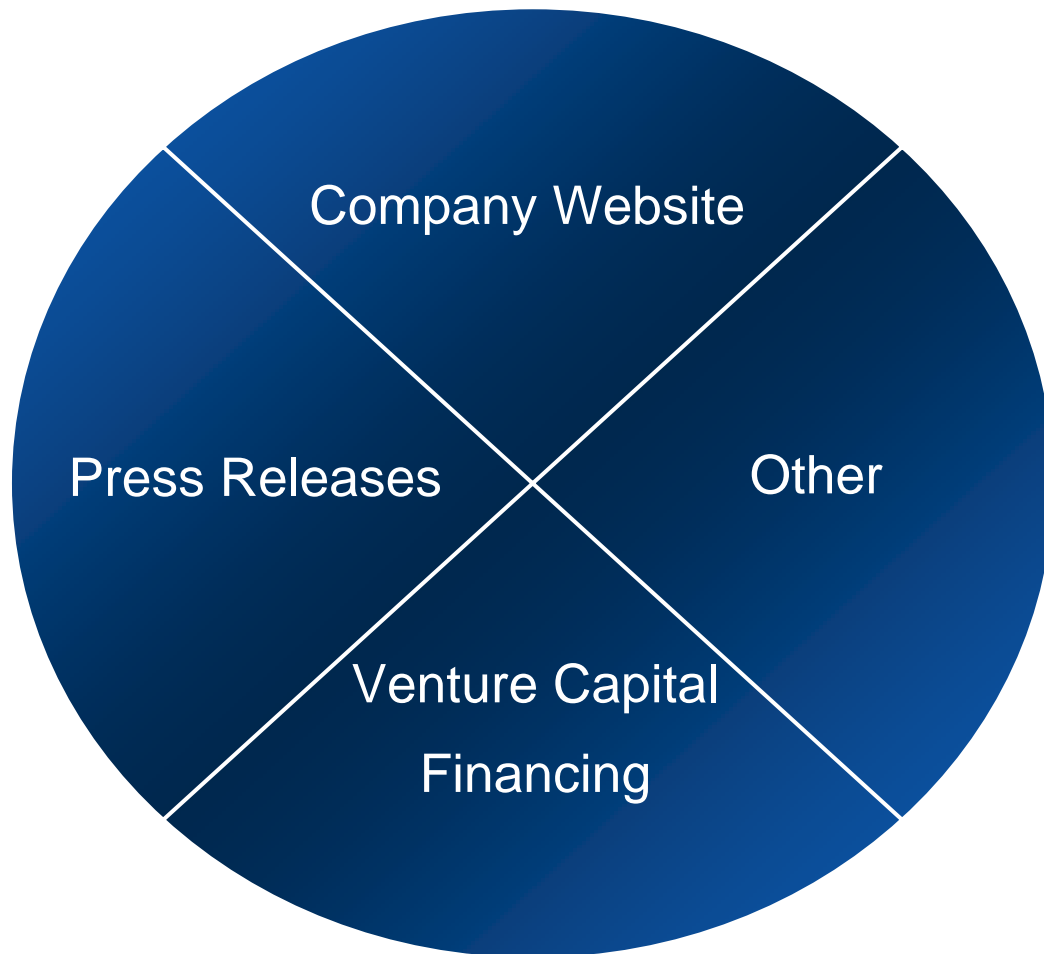
# Monitoring A Public Company



# Monitoring A Public Company



# Monitoring A Private Company



- ClinicalTrials.gov
- Patient Advocacy Websites
- Journal Articles  
(e.g. NEJM, Nature Medicine)

# Most Importantly: Talk to Your Physician

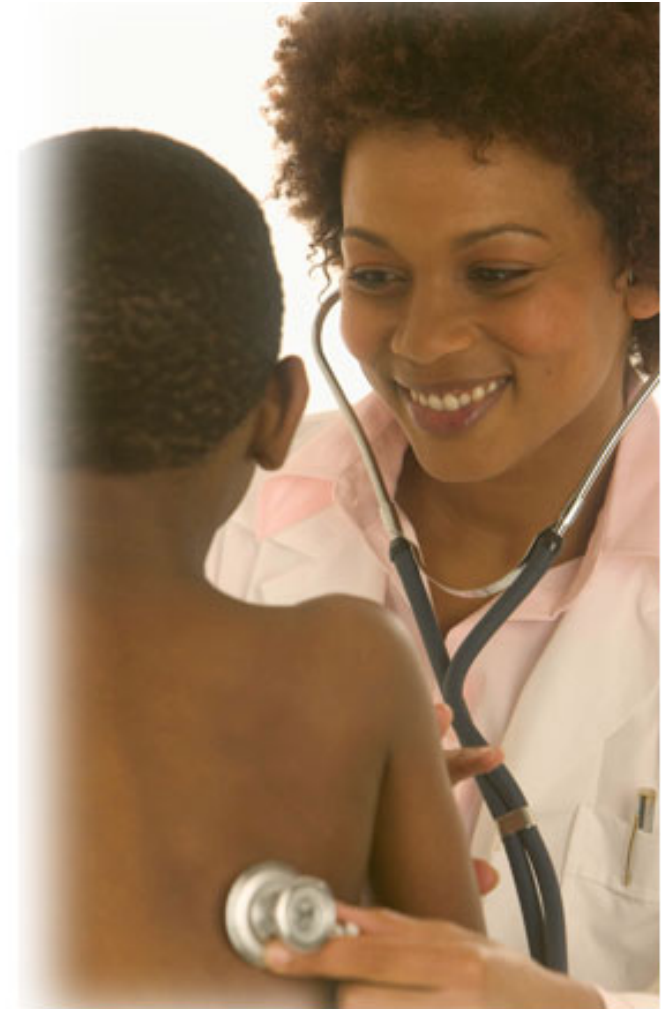
Product  
Efficacy

New Drugs Coming  
To Market

Generic  
Alternatives

Enrolling in  
Clinical Trials

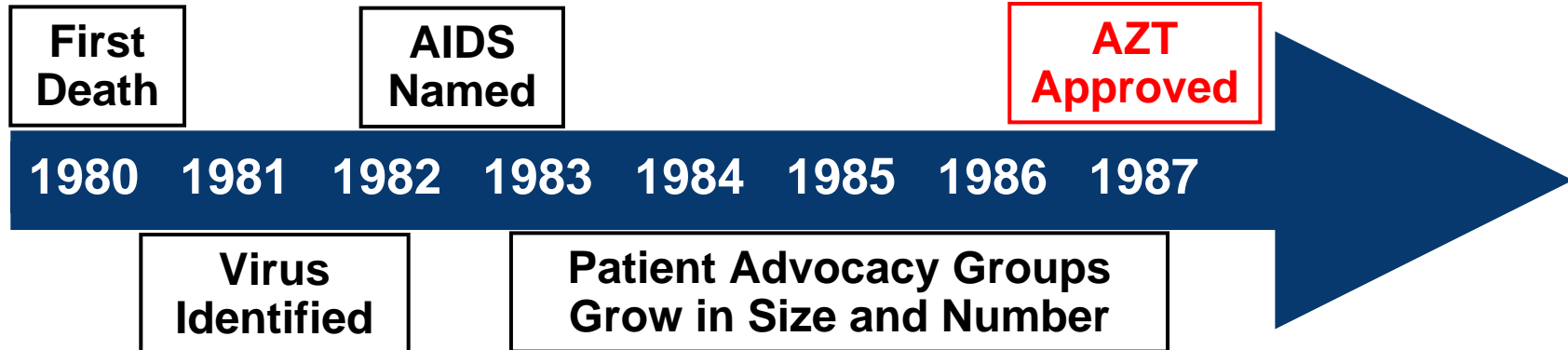
Safety  
Concerns



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# Influencing R&D: The Importance of Patient Advocacy



Source: [www.scribd.com](http://www.scribd.com), "The History of AZT" 2007



# Influencing R&D: The Importance of Patient Advocacy



Time from IND filing to approval:

Average

8-10 Years

AZT

Fewer than 3 Years

Source: [www.scribd.com](http://www.scribd.com), "The History of AZT" 2007

# What Can You Do?

## Tier 1 Biotech

### Activism

#### Government Support

- Gain Political Support
- Influence FDA

#### Fundraising for Research

- Individual Donations
- Institutional Donations
- Patient Advocacy Groups

#### Financing New Drugs

- Orphan Drug Designation
- Non-Dilutive Financing



# Monitoring the Progress of Clinical Trials

## Q&A

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