



Drug Repurposing in the Context of Drug Development

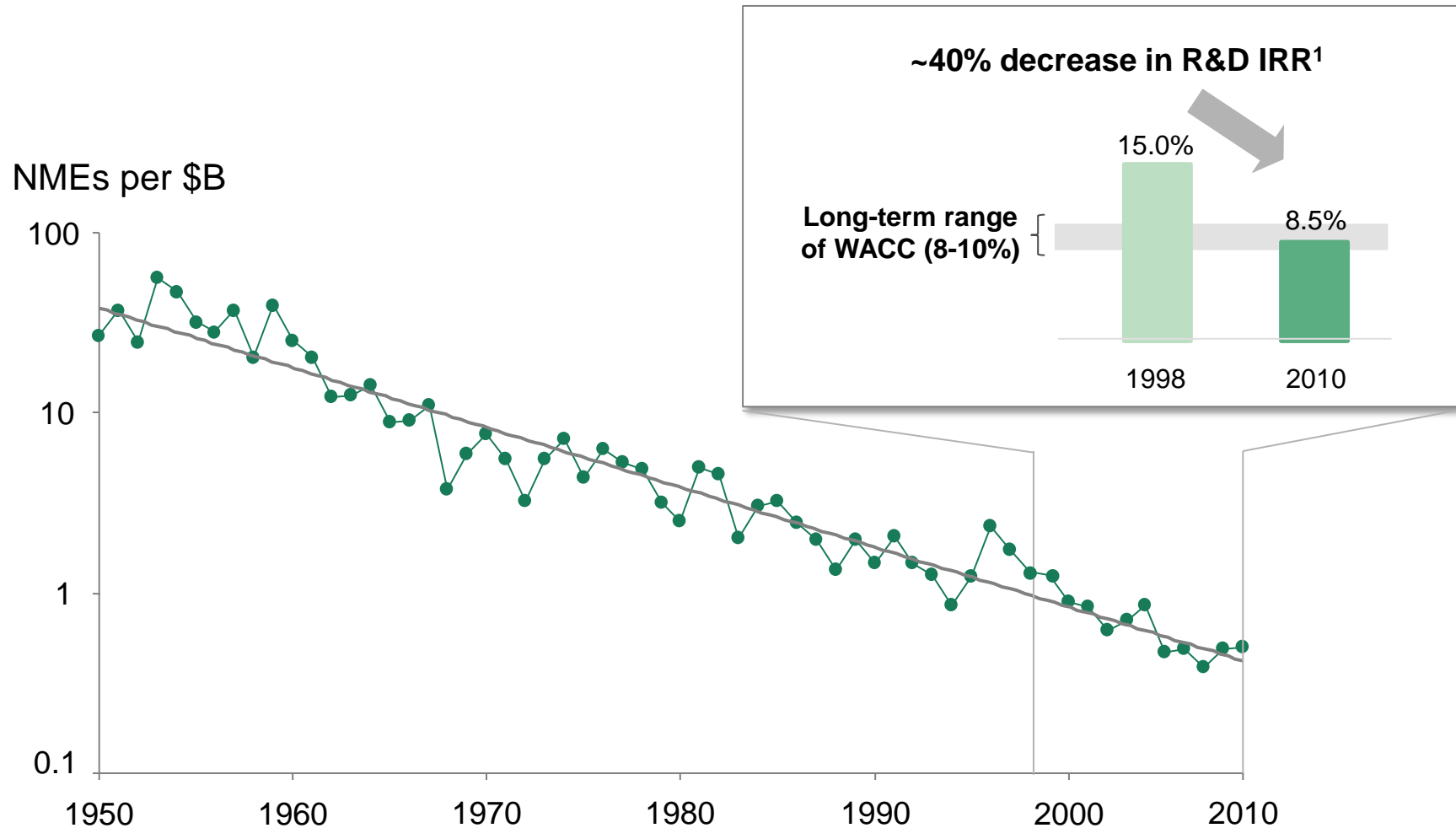
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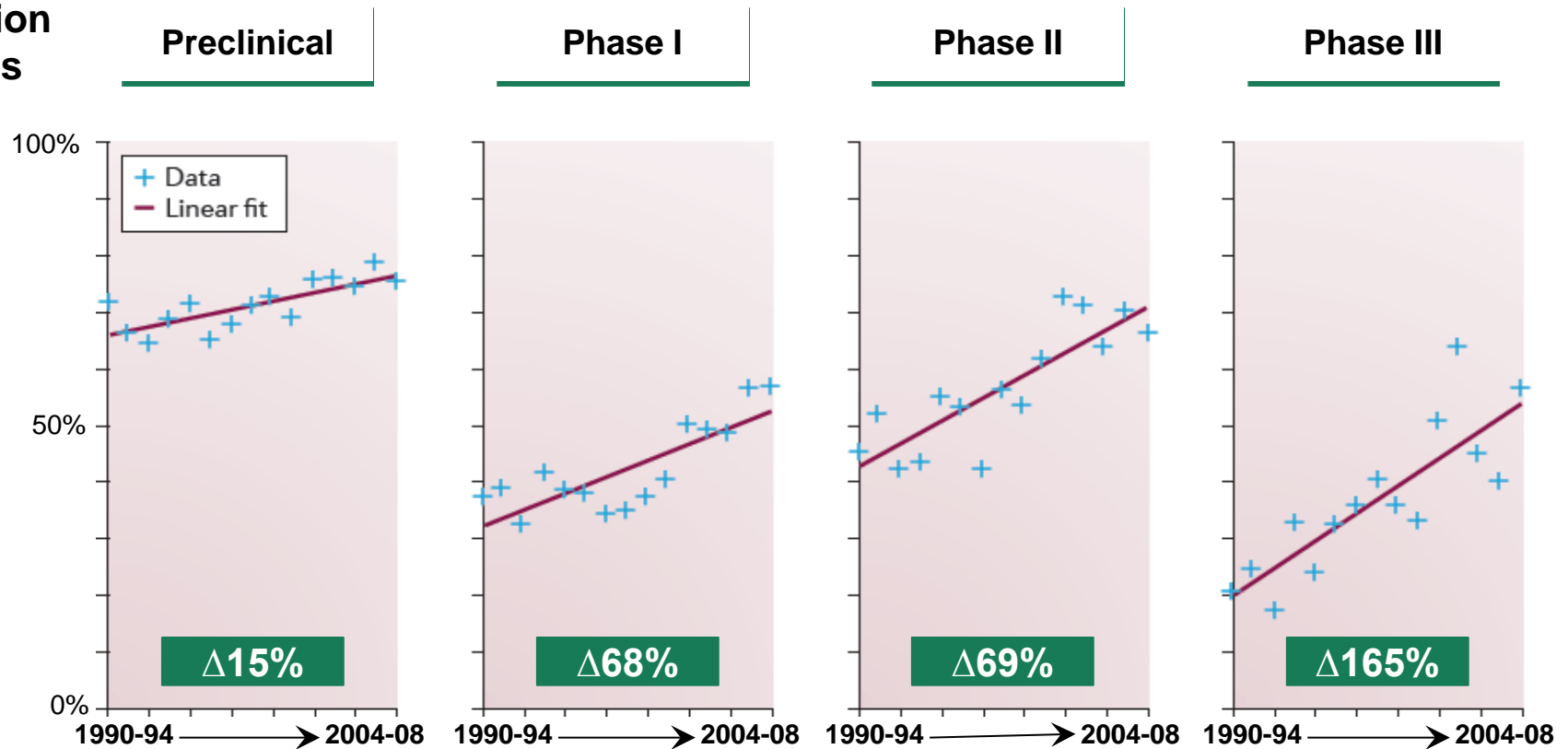
Productivity decline



1. Decrease in ROI driven by 54% decrease in PTRS, 30% increase in costs, partially offset by 25% increase in operating margin from COGS and SG&A savings
 Note: R&D costs are estimated from PhRMA annual survey 2009; NMEs are the total number of small molecule and biologic approvals by the FDA. Based on approved NMEs from 2002-2011 and R&D spend from 1998-2007, discount rate = 11%
 Source: Bernstein Research "The Long View – R&D Productivity" (September 30, 2010), and BCG model of R&D economics

Proximate cause: cost of failure

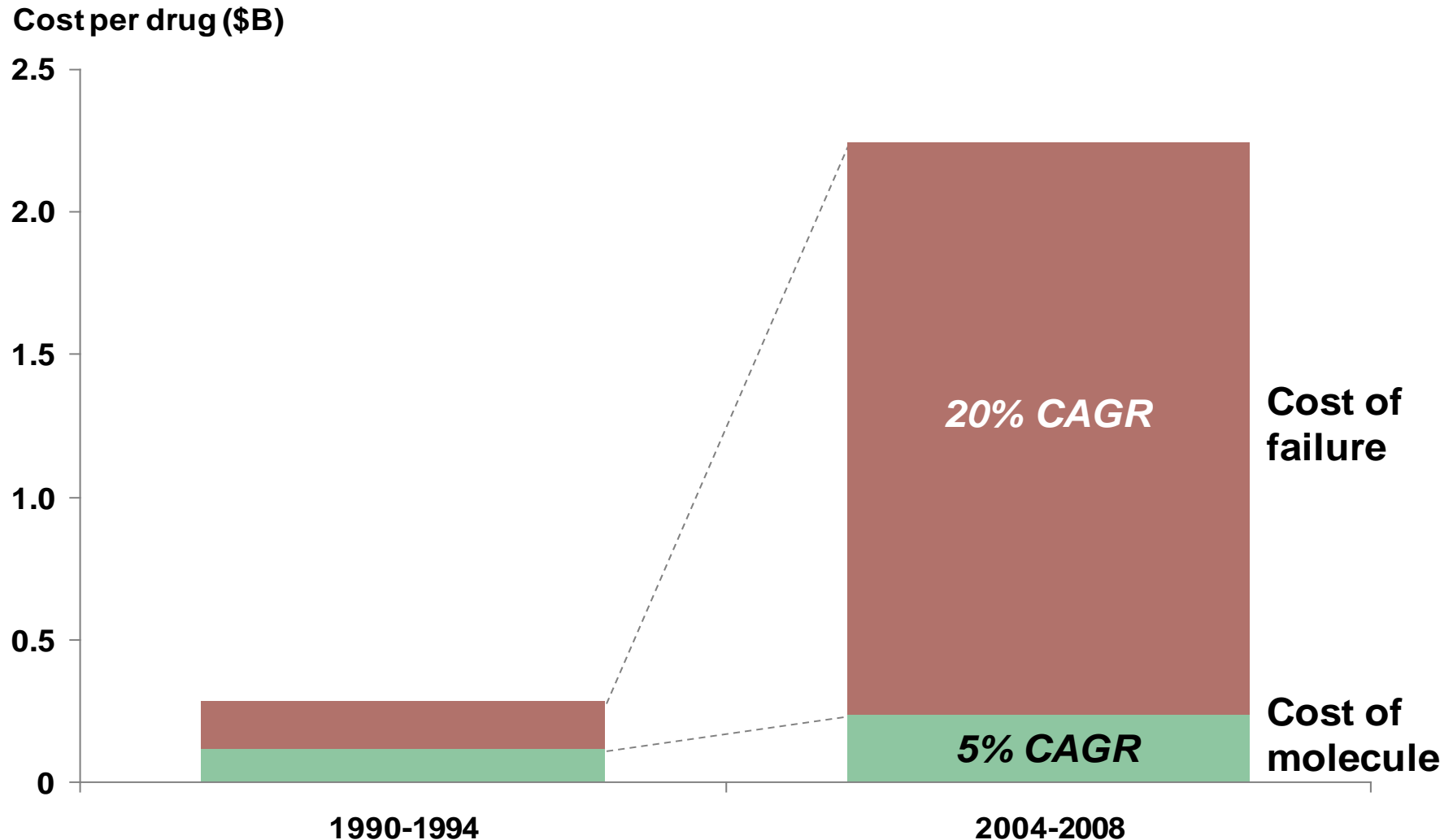
Attrition rates



Note: All costs capitalized, in 2006 USD

Sources: Pammoli et. al (June 2011) "The productivity crisis in pharmaceutical R&D" Nature Reviews Drug Discovery; Parexel statistical sourcebooks, PharmaProjects, BCG pharmaceutical cost of development model, BCG analysis

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The strategy: kill your losers



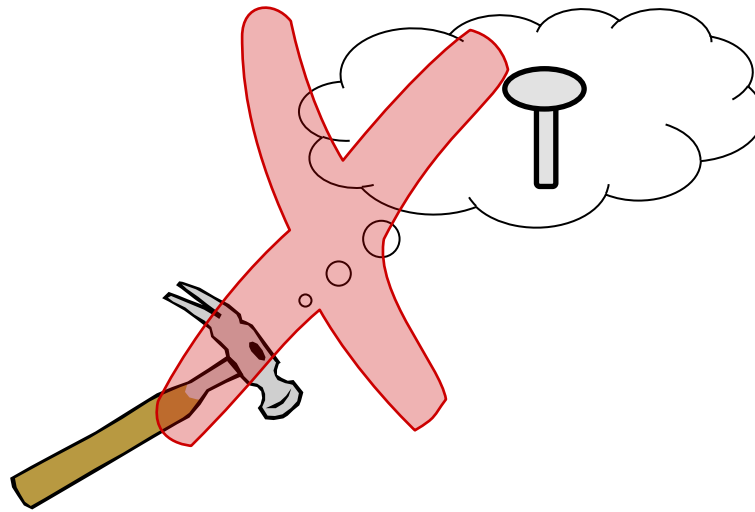
The fourfold pattern: good money after bad

	Gain	Loss
High Probability	95% chance to win \$10K RISK AVERSE	95% chance to lose \$10K RISK SEEKING
Low Probability	5% chance to win \$10K RISK SEEKING	5% chance to lose \$10K RISK AVERSE

Many unfortunate human situations unfold in the top right cell. We take gambles to keep slim hopes alive... likely to lead to even greater losses

A simple heuristic... part one

Hammer Seeking Nail

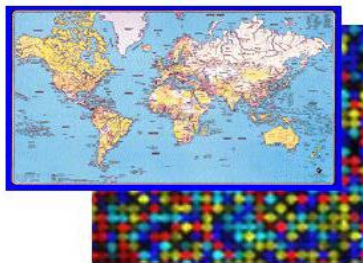


May be 'good money after bad'

At the same time, significant reason for optimism

Genome-wide capabilities

(ENCyclopedia Of DNA Elements)



Transcriptome Reference Sets (MRT Project)

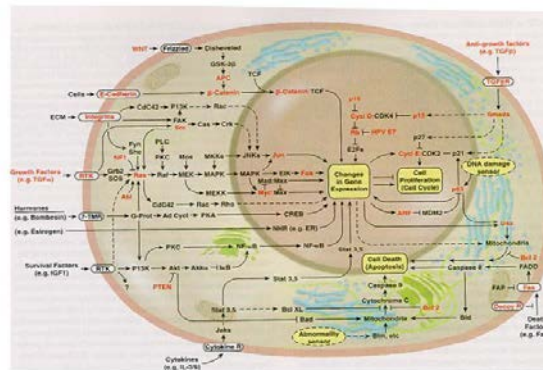


KO mice genome-wide (KOMP)

**>1,200 GWAS;
400 hits on the cusp**

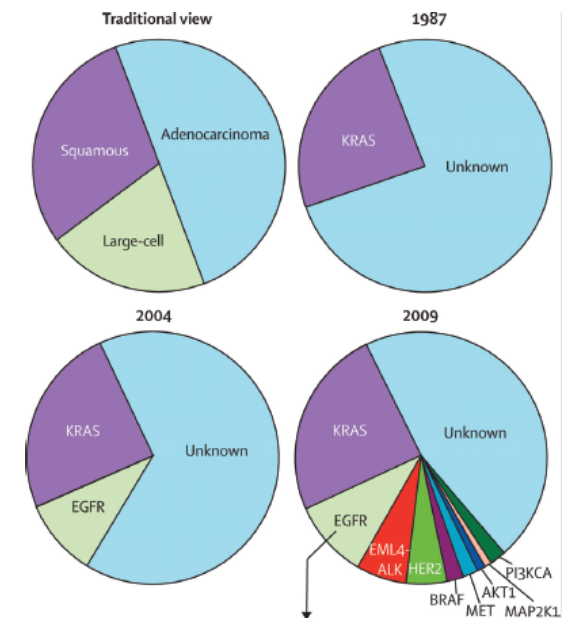
Deeper understanding of pathways...

Cancer pathways



...and targets

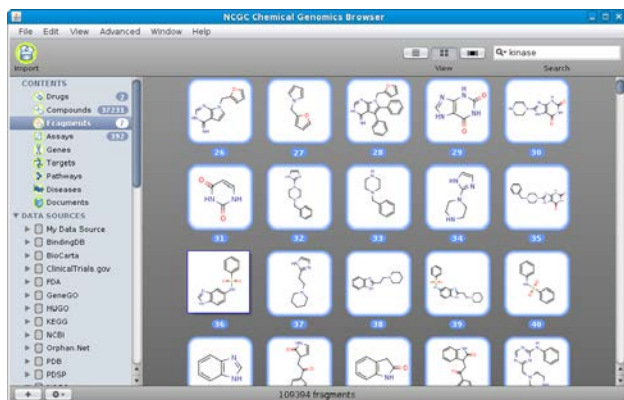
NSCLC targets



Sources: Christopher Austin, NHGRI; Francis Collins (2011) Target Validation: Joint NIH-Industry Workshop; Hanahan & Weinberg (2000); National Research Council of the National Academies (2011) *Toward Precision Medicine*

Tools coming together

NPC NCATS Pharmaceutical Collection



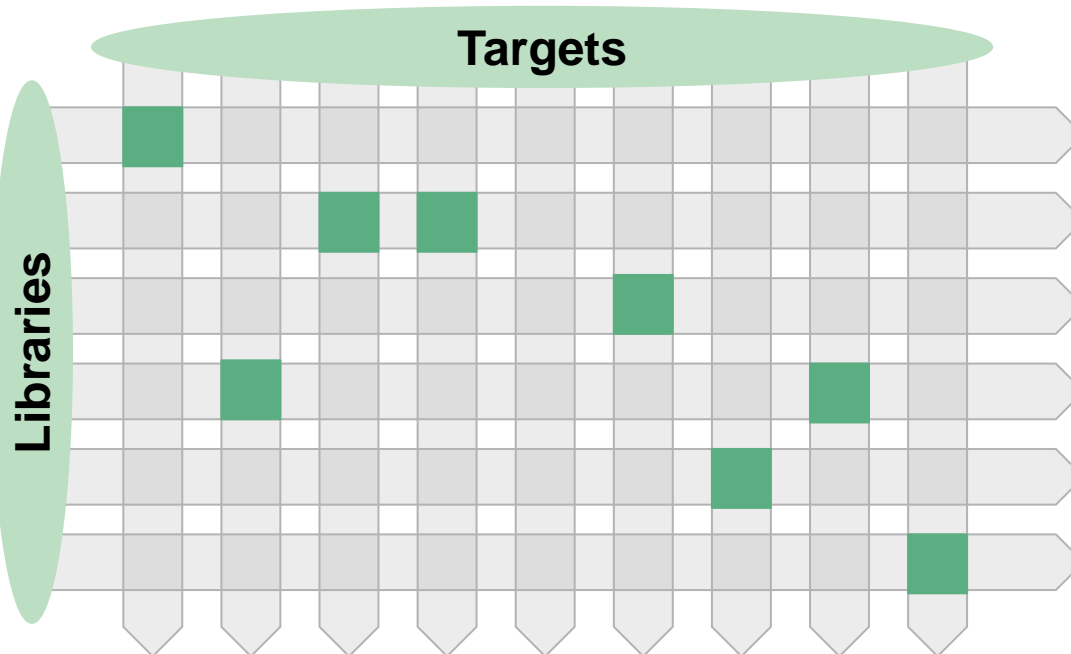
Drug Discovery Assays

PD²

Phenotypic Drug Discovery

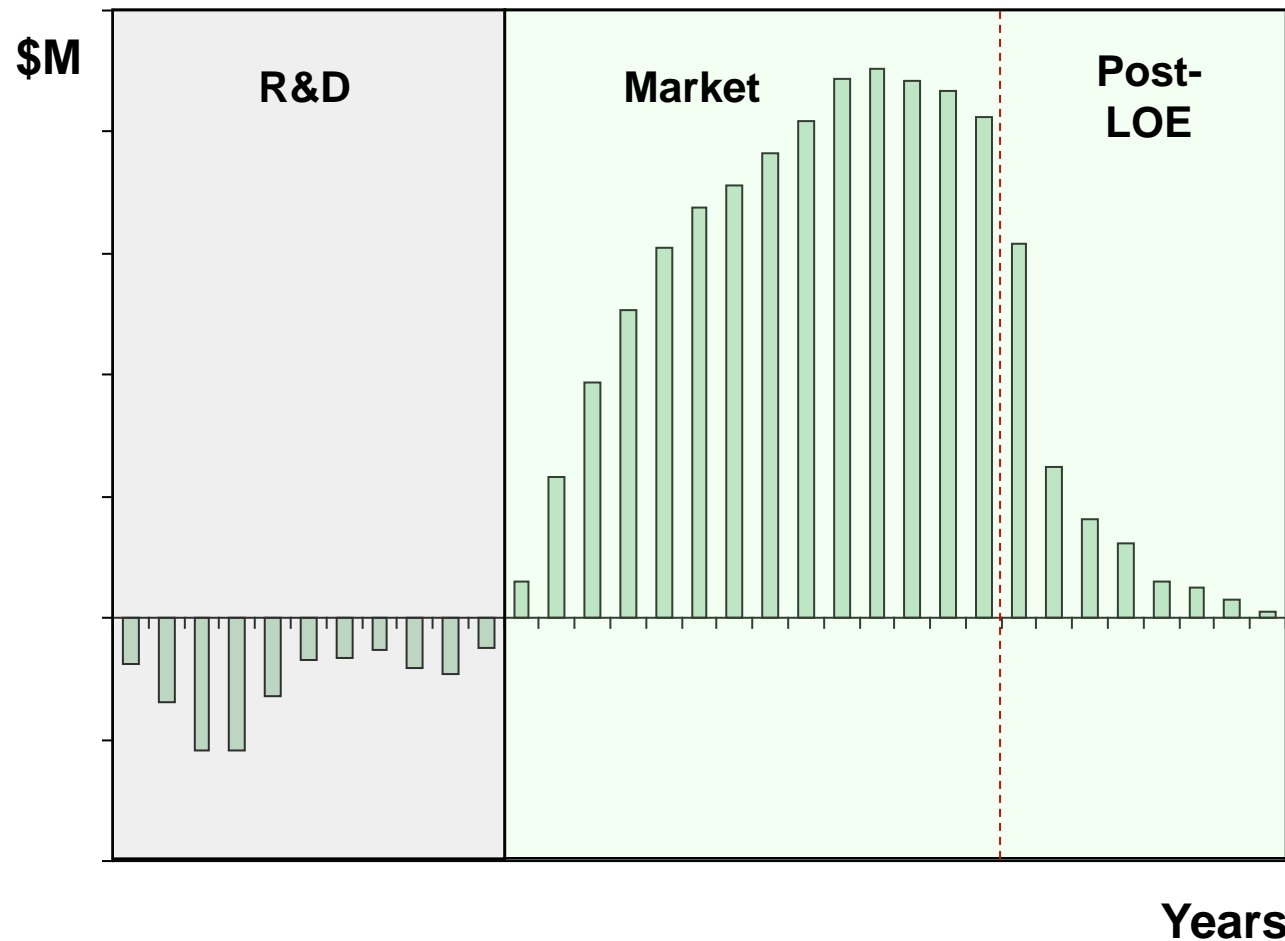
TargetD²

Target Drug Discovery



Reasons why biopharma would fail to invest

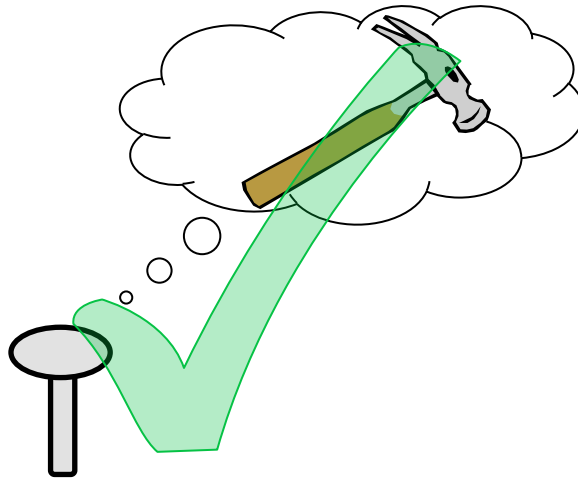
Illustrative Pharmaceutical Cash Flow Curve



- 1 Near patent expiry
- 2 Past patent expiry
- 3 Not your molecule
- 4 Not patentable

A simple heuristic... part two

Nail Seeking Hammer



May be valuable (to patients)

Some example companies



Revenue: \$20M



Revenue: \$2,000M



Revenue: \$4,000M

**Crowd-sourcing platforms
(eg, Innocentive)**

Source: Company websites, Orbis

2013-06-24 IOM Workshop-Michael Ringel BCG.pptx

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Draft—for discussion only

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Some example government and consortium efforts



**Biomedical Catalyst:
Developmental Pathway
Funding Scheme**



Medicines Chest

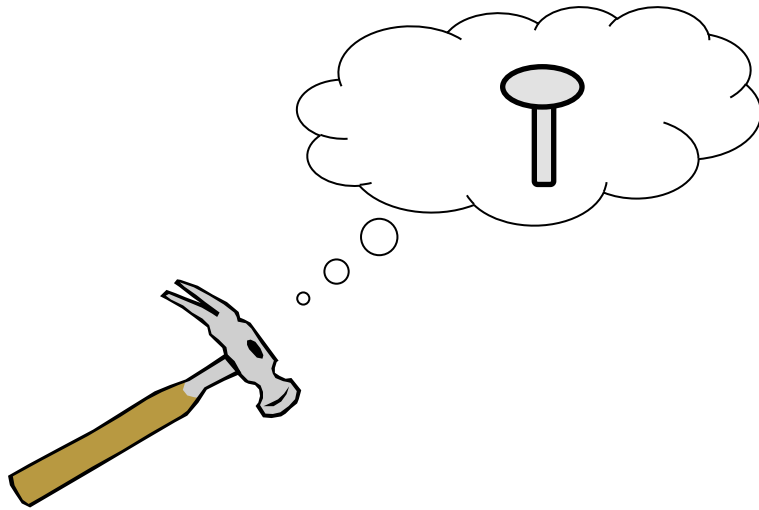


**Rare Disease
Repurposing Database**



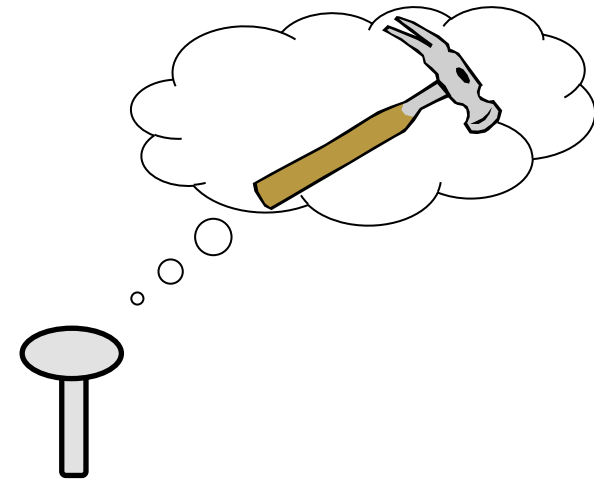
A simple heuristic

Hammer Seeking Nail



More likely to be 'good money after bad'

Nail Seeking Hammer



More likely to be valuable

But of course... it depends on the specific situation