

Change and Challenge in Drug Development: What will it take?

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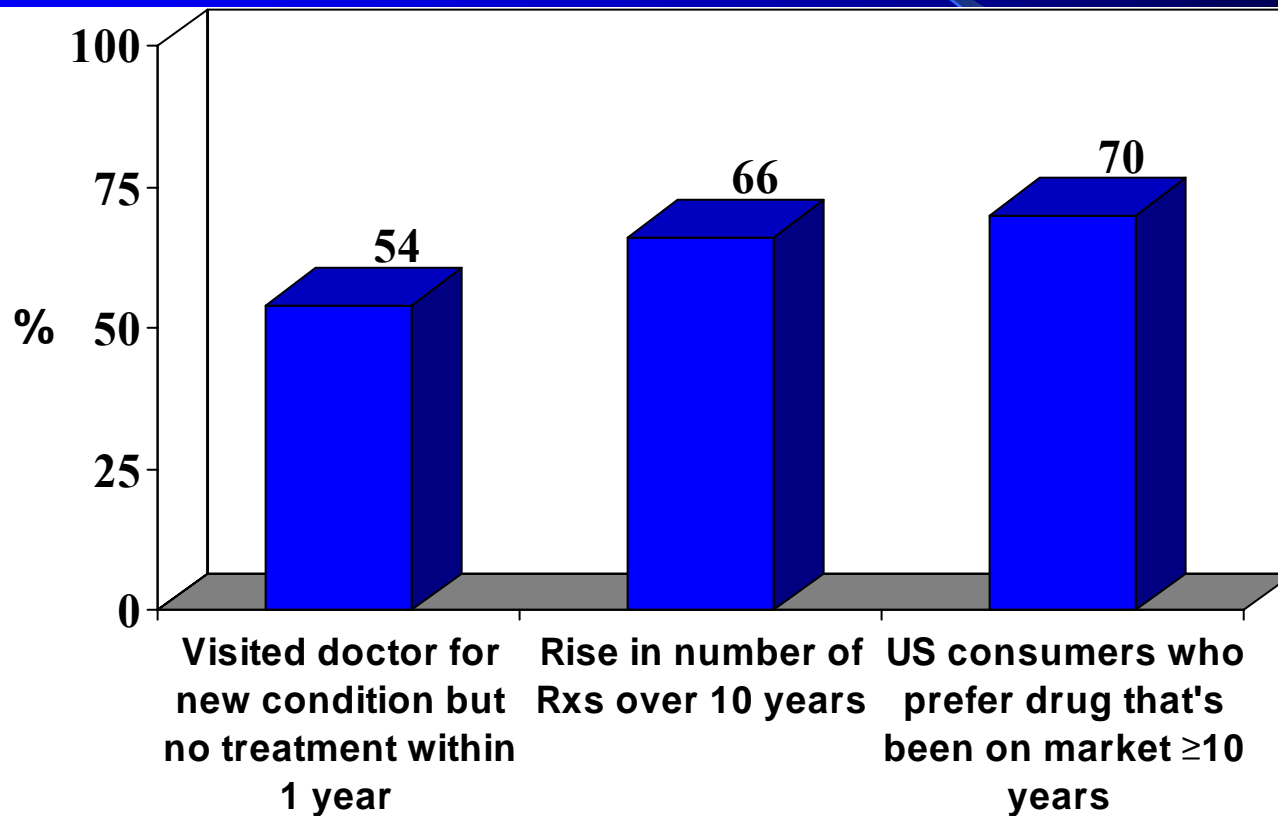
PhRMA Briefing:

Future of Innovation

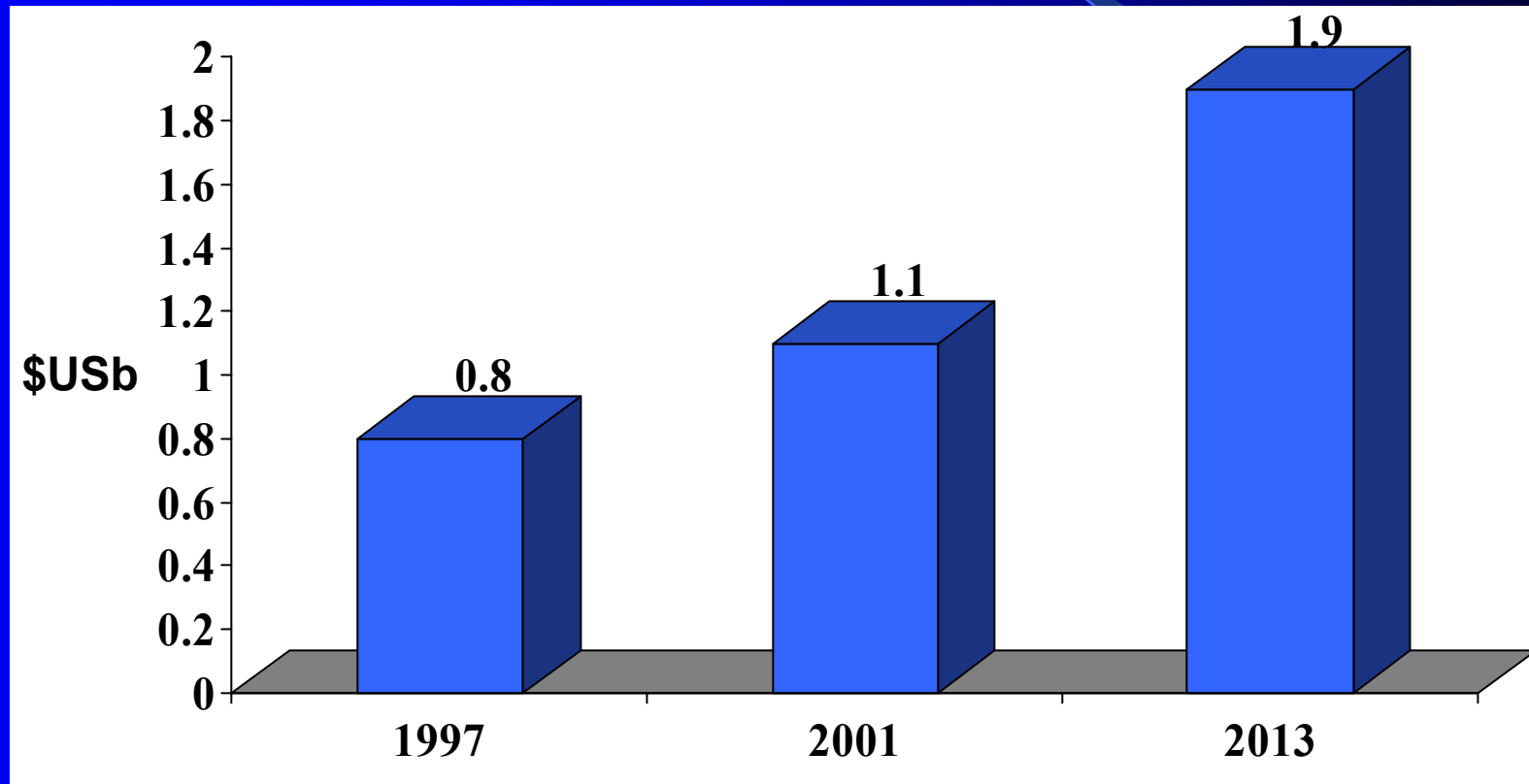
May 24th, 2006

Washington DC

Are We Under, Over, or “Well”-Medicated?

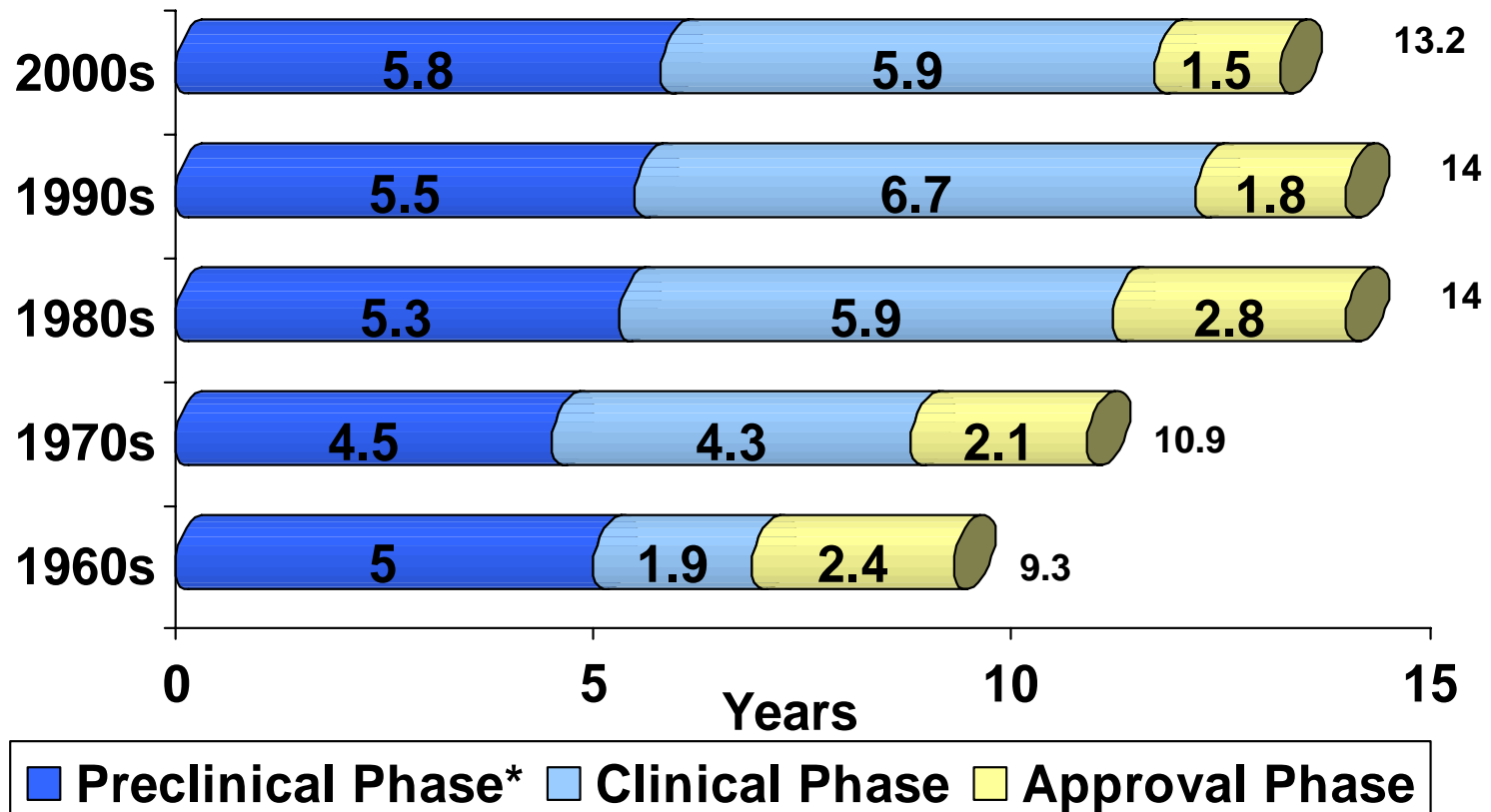


Basic Challenges Remain: Cost of New Drug Development



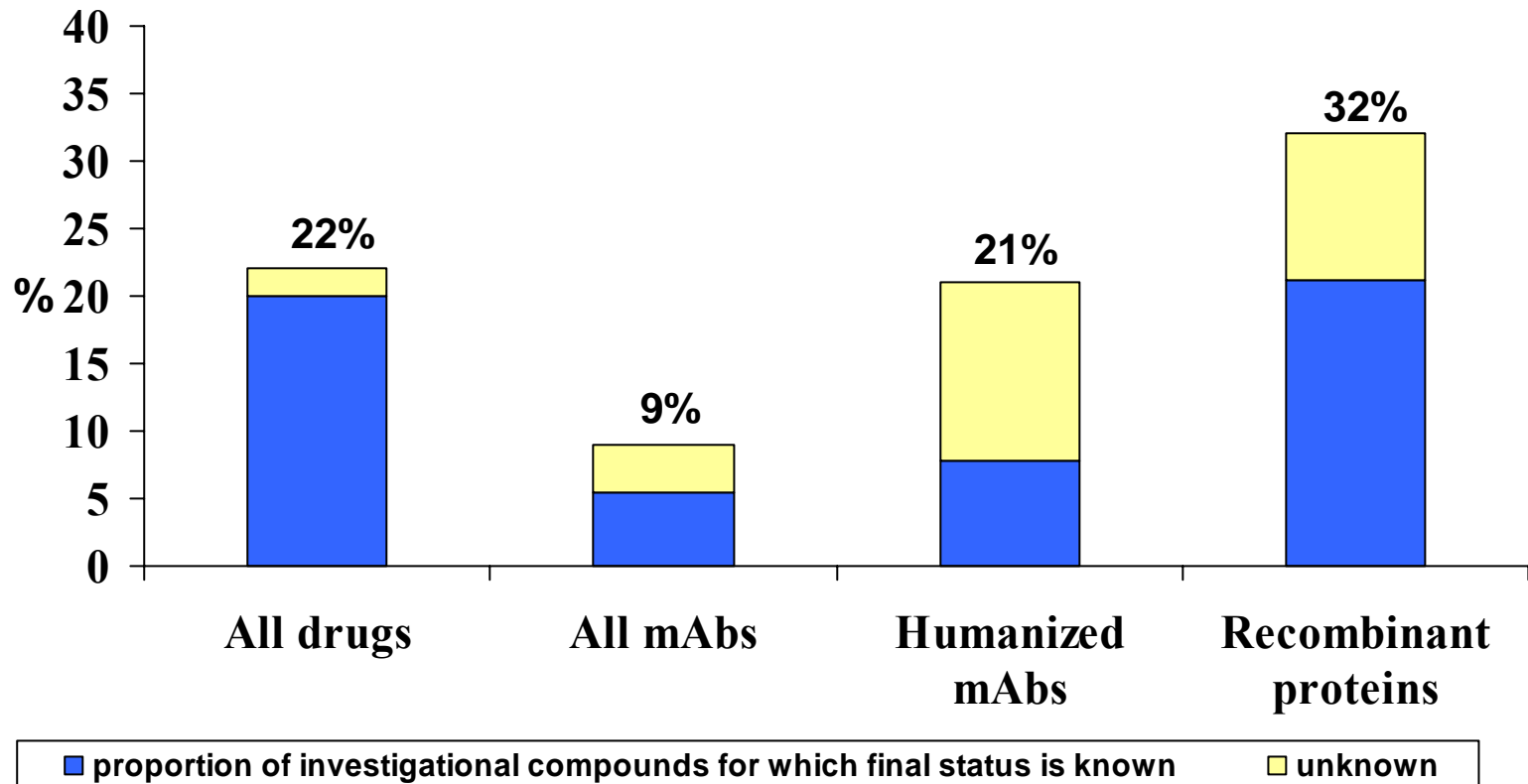
Source: Tufts CSDD, 2003

New Drug Development Times in US Spanning Five Decades, 1963-2005



*Preclinical phase=synthesis to IND filing

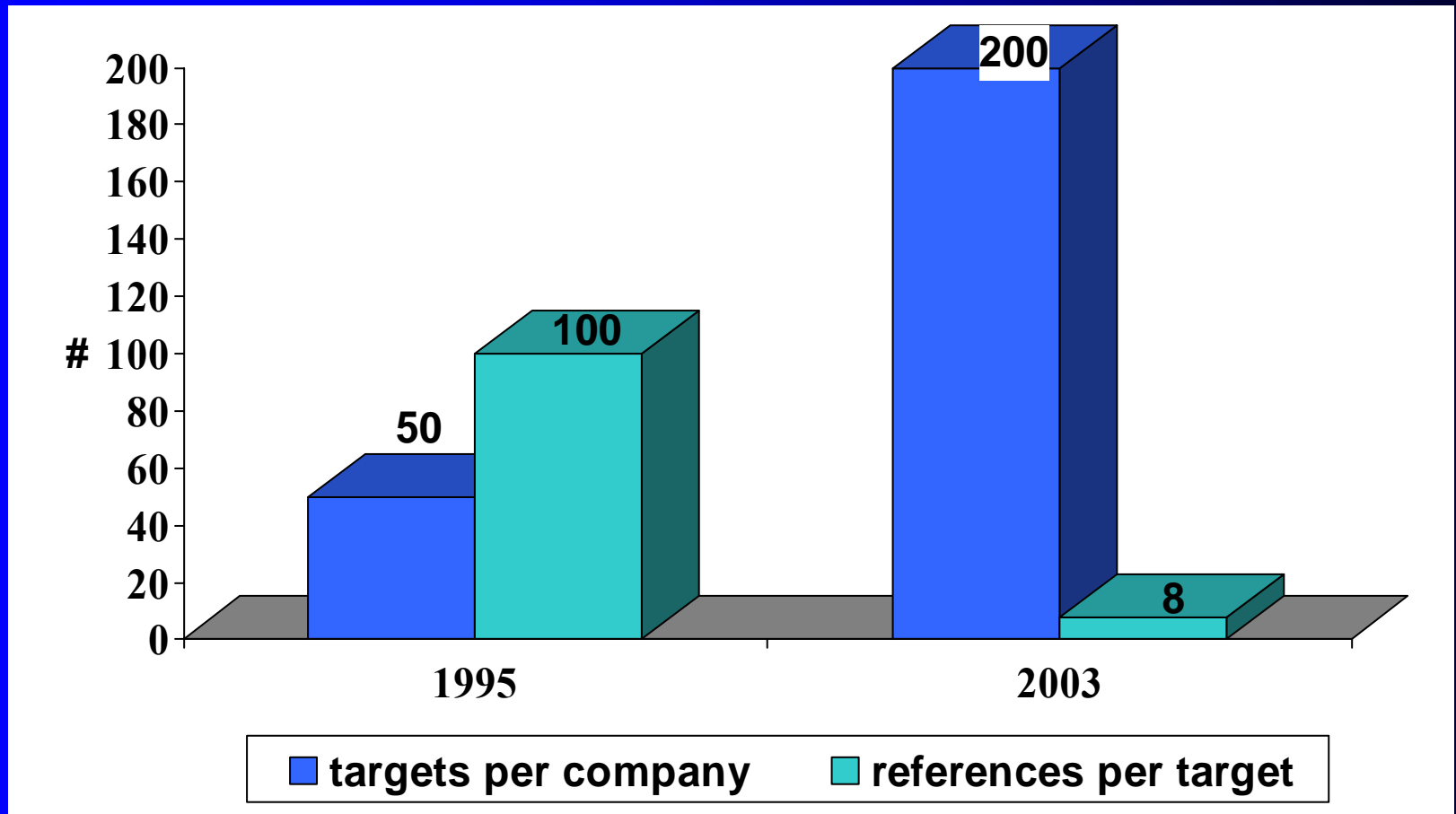
US Success Rates for “New” Drugs & Biologicals



What Do the Metrics Tell Us!

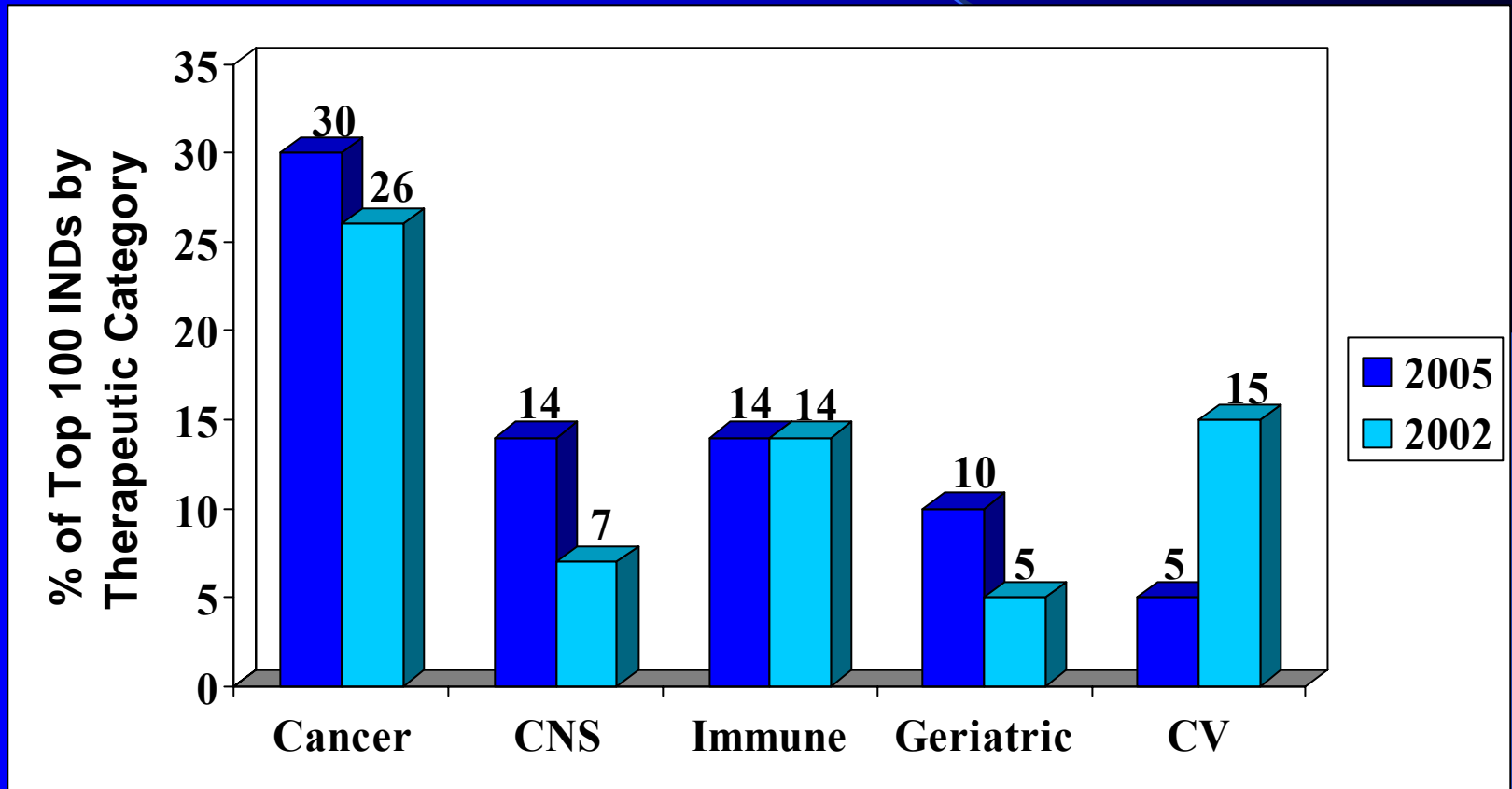
	\$100 million	\$200 million
Time reduction	18.9%	41.3%
Clinical success rate	25.2%-25.6%	30.4%-31.7%
Out-of-pocket preclinical cost reduction	29.8%	59.6%

Problems in the Process!

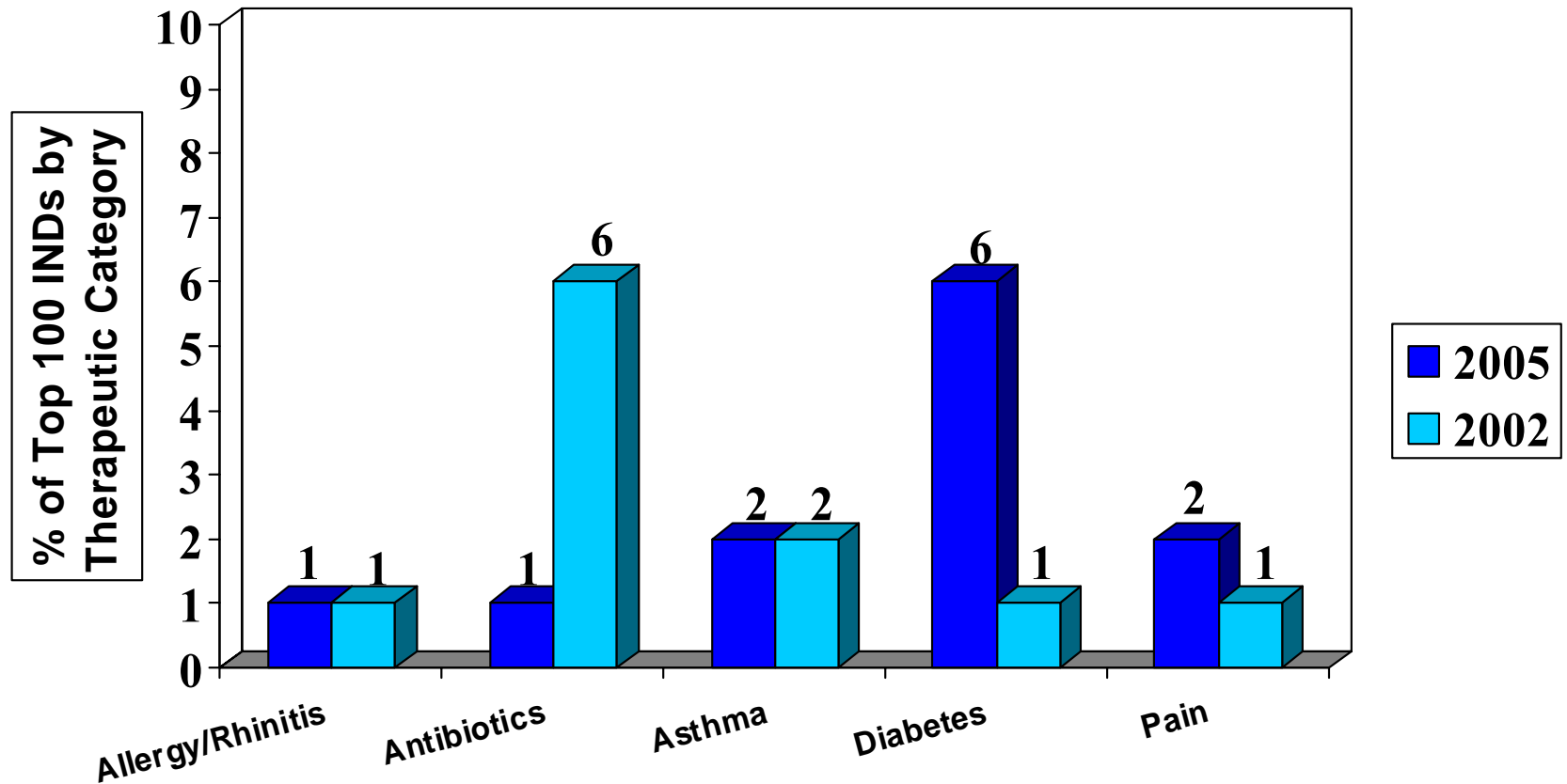


- The R&D Process Is Different Today Because the Starting Point Is Different! (Lehman & McKinsey 2001)

Problems With Products



Are Some Needs Not Being Met?



Problems With The Paradigm

- Chemistry vs. Biology
- Classic vs. Large-scale approach
- Product vs. Platform
- Blockbuster vs. Portfolio
- Focus inward vs. Focus outward

Paradigm: Practical vs Philosophical

- Address *practical* problem: every market, model, and molecule exploited vs. evolution into small, specialized, science-driven, and smart (i.e., microdosing, SCTs, PGx, paperless, etc.)
- Address *philosophical* problem: “Let’s find something that will sell good” vs. “If it’s a good drug, we’ll find a way to sell it”

Innovation Threshold Issues

- Basic research- What's druggable
- Discovery- Validate targets + biomarkers
- Pre-clinical- Animal versus modeling
- Clinical- New trial design (e.g. Adaptive CTs, exploratory INDs, dosing, attrition)
- Manufacturing- Technology turnover
- Regulatory Review- Pre vs Post approval safety

Approaches to Innovation: Scope

- Specific or Limited
 - Orphan Drug Act (ODA)
 - Biomarkers
 - Small + Medium Sized Entities
- General or Comprehensive
 - Critical Path, EMEA Road to 2010
 - Innovative Medicines Initiative, NIH Roadmap

Function

- Structural- Network, consortia, clusters, et cetera
- Regulatory Policy- ICH
- Science/Technology- Basic Plan, Japan
- IT (data sharing, online inventories, etc)- NIH Roadmap
- Organizational- Private, public, PPP, etc- MVI
- Public Health Imperatives- ODA
- Training/Education- IMI

Mode of Action

- Feedback Loop- Critical Path, UK National Cancer Plan
- Create new policy, organization, etc- ODA
- Revise, add resources to existing programs- EMEA 2010
- Create new business model- NRDOs

Phase/Bottleneck

- Basic research- High volume, but low yield (HapMap)
- Discovery- Too many choices, too little money (Rapid access to NCI discovery resources i.e. RAND)
- Development- Not enough choice (Critical Path)
- Delivery- Best choice or prioritize? (NICE)

Conclusions

- Increasing success rates is most realistic near-term target for improving efficiency of development
- Industry, academia, and regulators need to change process and product mix, which will affect paradigm
- New technology is driver of change with help from regulators and legislators as well as new business and R&D collaboration models