

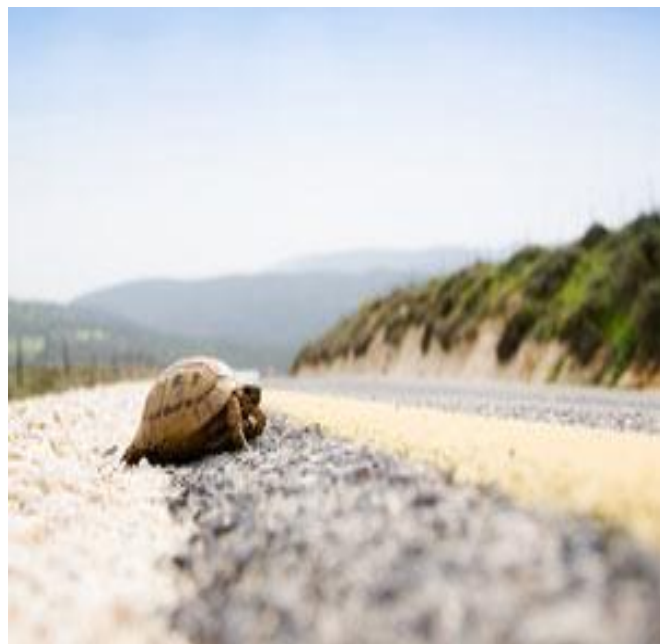
ECONOMIC LANDSCAPE FOR ORPHAN DRUGS: HOW THE PICTURE HAS CHANGED!

Christopher-Paul Milne, DVM, MPH, JD
Director of Research
Center for the Study of Drug Development
Tufts University Medical School, Boston, USA

Symposium on Rare Disease Study Design
Children's National Medical Center
GWU, Washington, DC
29-30 April 2013

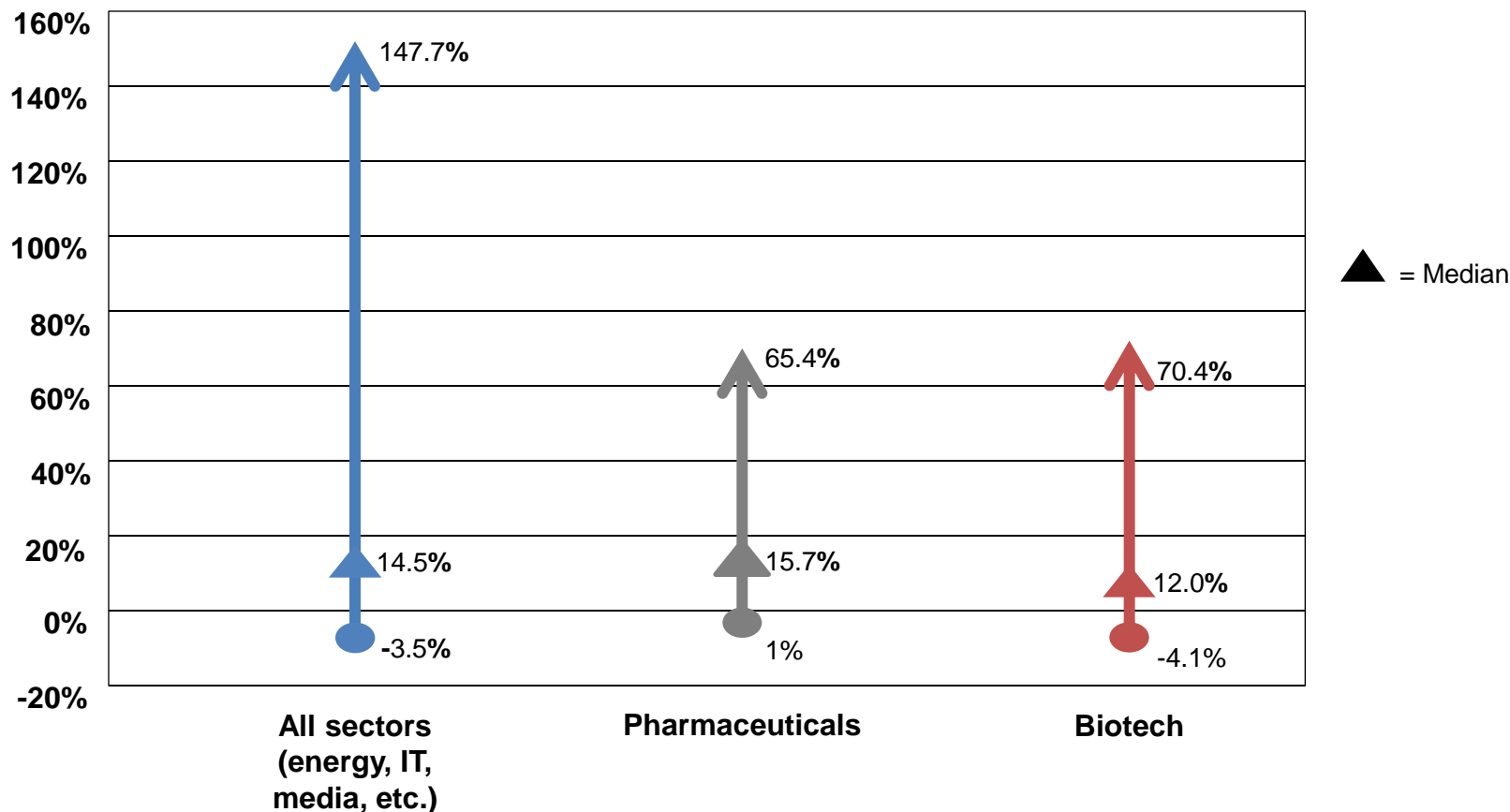
Orphan R&D: Why the Paradigm Shift?

From This.....to This!



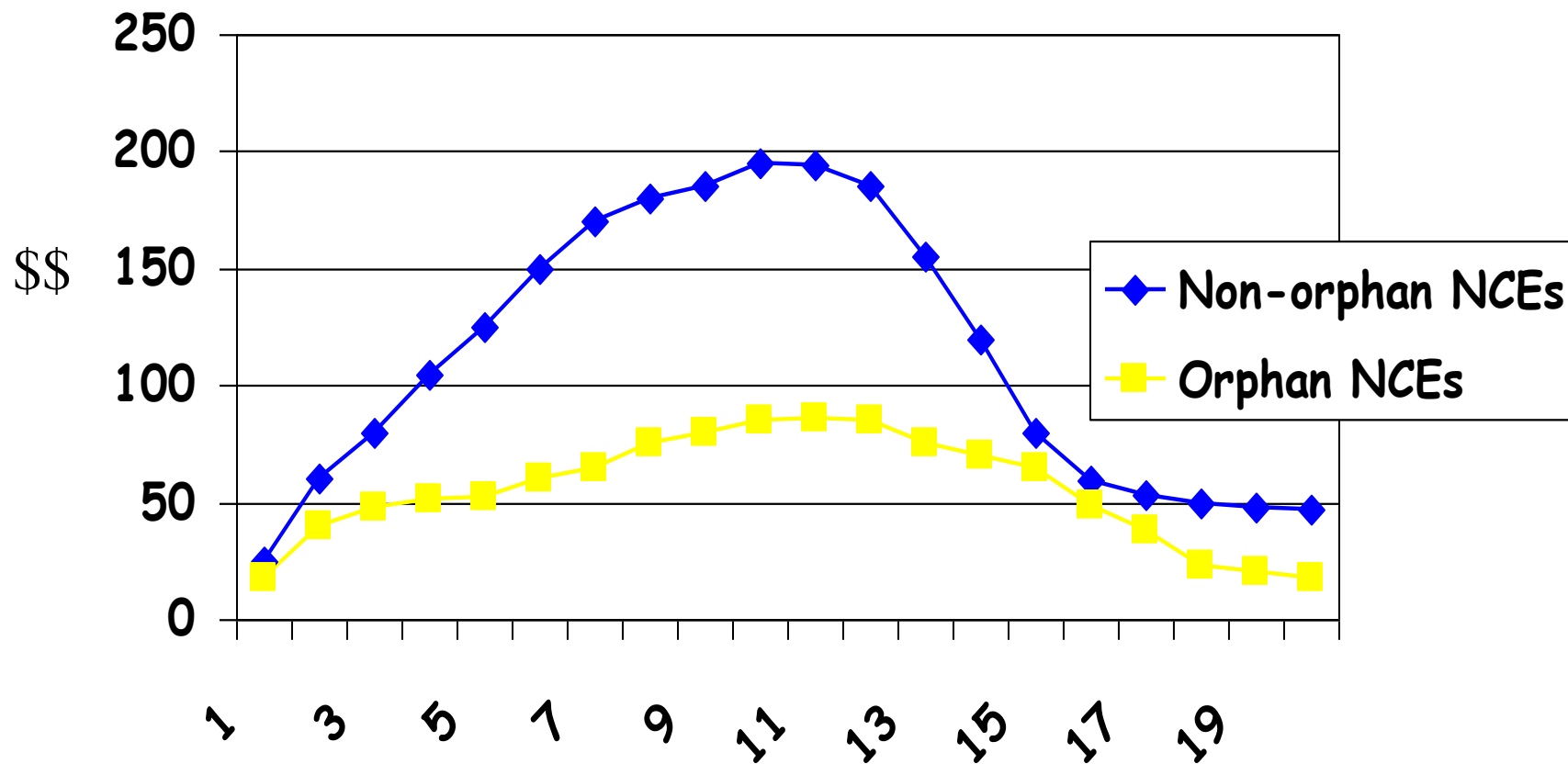
Evolving BioPharma Paradigm, Sticking to the Bottom Line!

(Internal Rates of Return for Venture Capital Investment, 1997 to 2010)



Source: Biocentury (October 10, 2011); 19(42):A3

“Old” Model of the Orphan Drug Market!



Years since launch for early 1990s cohort
by annual sales in millions of dollars

Source: Pharmacoconomics 2000; 18 Suppl.1

“New” Model: Reverse Blockbuster Pyramid!



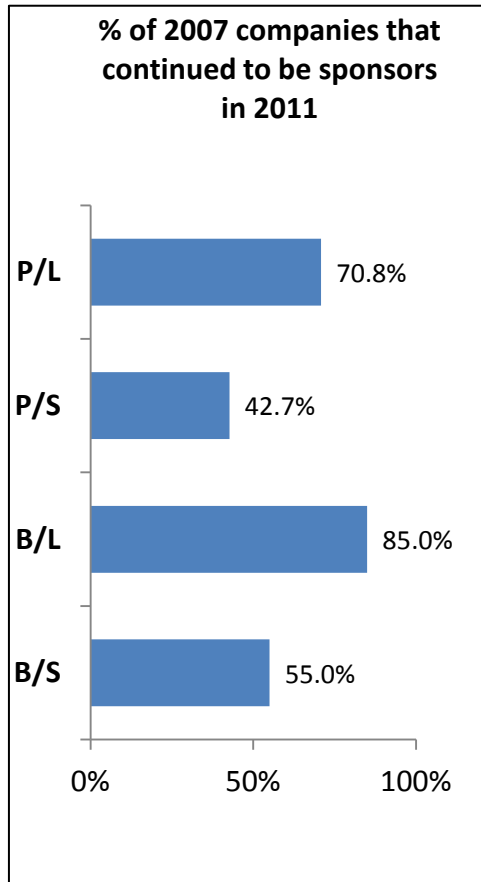
WILLINGNESS-TO-PAY FOR ORPHAN DRUGS!

❖ **Customer-focused brand value reduces price elasticity through the following customer-specific effects:**

- **orphan drug effect (low total budget costs)**
- **unique value effect (no substitutes, high unmet need)**
- **total benefit effect (efficacy, safety, tolerability, etc.)**
- **total expenditure effect (cost offsets including reduction of hospitalizations and complications)**
- **shared cost effect (risk-sharing arrangements)**

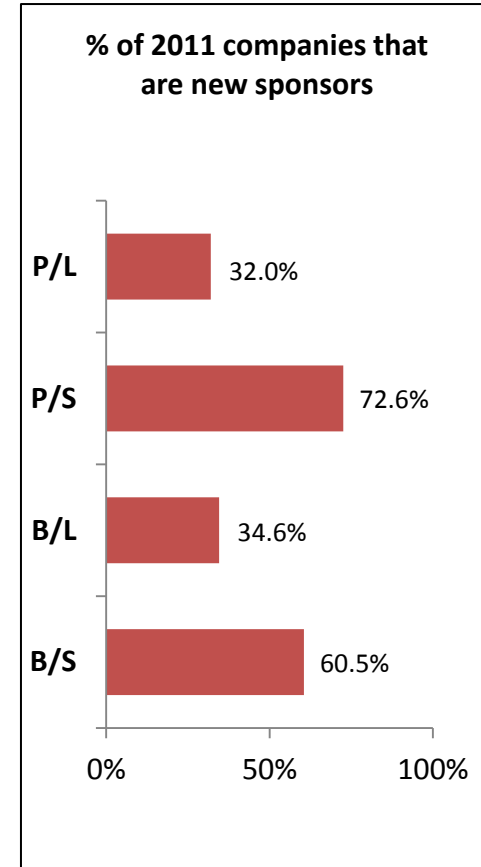
(Source: Simon, Health Affairs, 2006)

Despite Improving Landscape, Valley of Death Still a Prominent Feature Confronting “Emerging” Sponsors

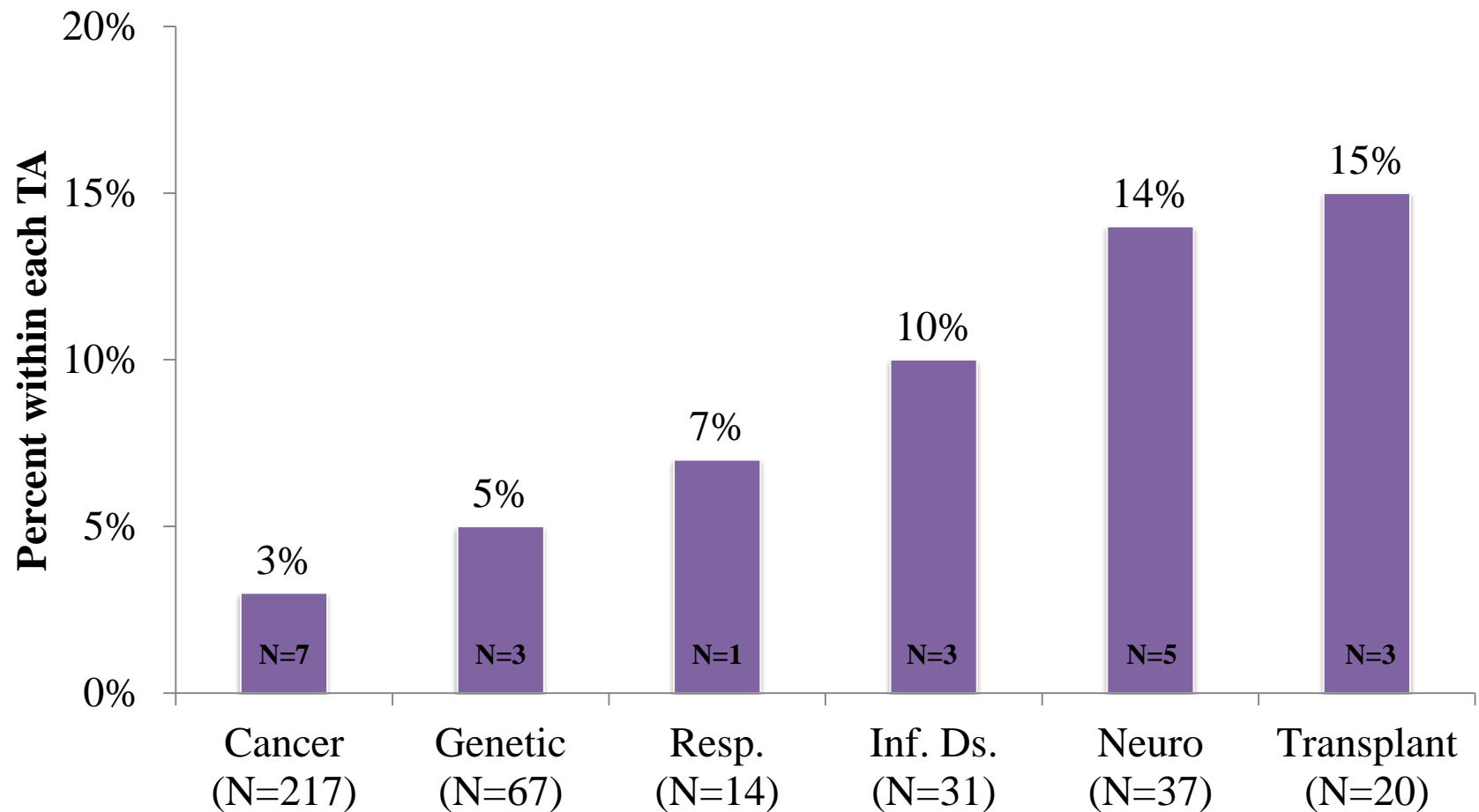


- 60 companies worldwide with specific focus on orphan R&D (Ernst & Young 2011)

- Top 10 pharma companies accounted for half of pharma sponsored approvals; top 10 biotech companies accounted for one-quarter of biotech approvals up to 2009 (Wellman-Labadie & Zhou 2010)

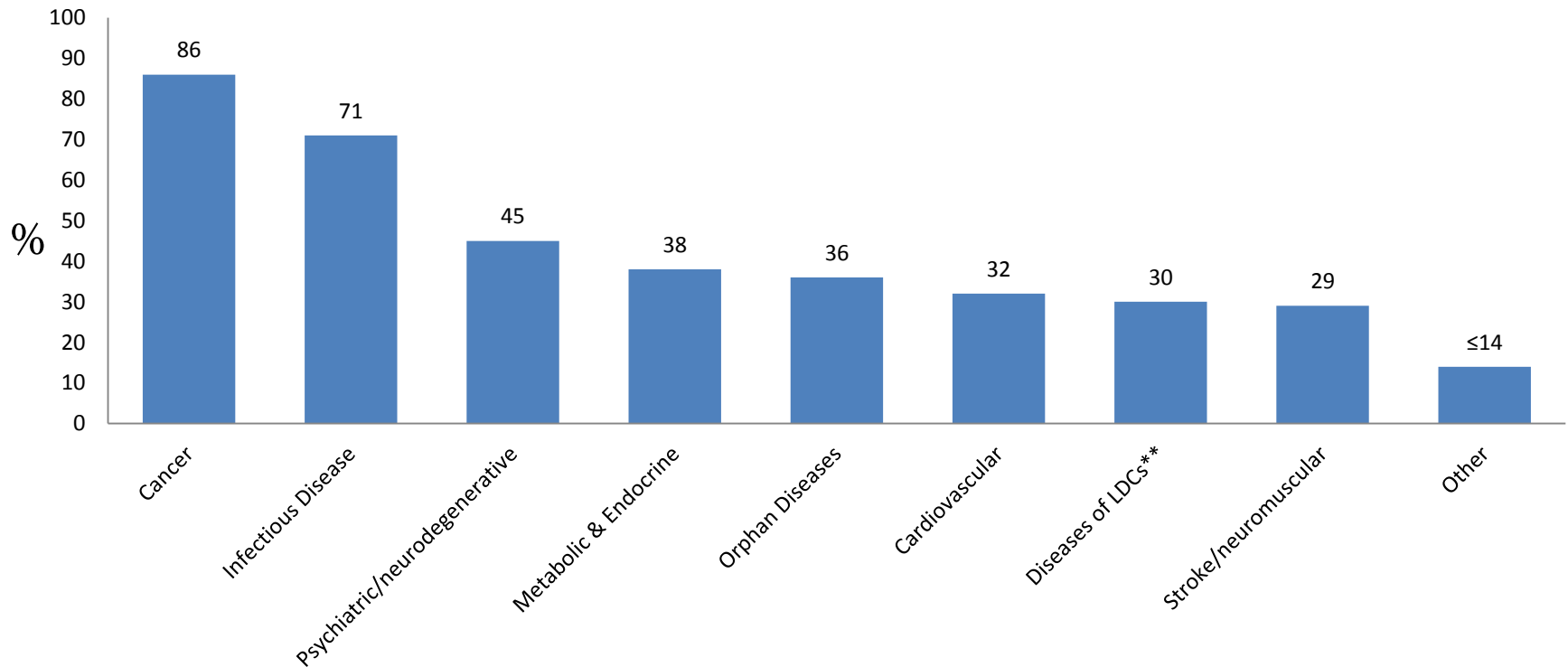


Will Partnering Be the Way Out? If So, How Soon? (Total: 23/460 or only 5%)



Source: Derived from PhRMA Rare Disease Report 2011

How Much Will Academia Help?



Therapeutic area focus among academic drug discovery centers

Source: Frye, S., Crosby, M., Edwards, T., & Juliano, R. (2011). US academic drug discovery. *Nat Rev Drug Discov*, 10(6), 409-410. doi:10.1038/nrd3462; ** LDCs = less developed countries



Facing the Future...It's All About Attitude!



- **Orphan markets – thousands of diseases, millions of patients (with relatively few therapies on the market) – are boundless but resources are not!**
- **Tension between payers focused on meeting broad population needs and future of health care focused on patient-centric tailoring of therapy to individual disease states: specialty + targeted + personalized = orphan!**
- **Partnering is key for overcoming what each sector lacks in terms of expertise, goodwill, experience, and resources, but relationships can be stormy, and changing R&D paradigm, regulatory environment, payer criteria and healthcare priorities make basis for selection as uncertain as shifting seas!**