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Financing Innovation

Gerard Anderson, PhD
Professor and Director of
Johns Hopkins Drug Access and Affordability Initiative

Protecting Health, Saving Lives—*Millions at a Time*

The Politics of R&D

- While we can probably agree that innovation benefits society, a related question is are we financing innovation correctly?
- While economists know that R&D are sunk costs and should not be used to justify the high cost of a drug or a price increase, the pharmaceutical lobby often uses R&D as a justification for its high prices and price increases
- Need to examine the relationship between R&D spending and innovation

What is the Cost of R&D

- Tufts Center for Study of Drug Development quantified the R&D costs over time:
  - $ 231 million in 1991
  - $ 802 million in 2003
  - $2580 million in 2014
  - Real cost increases averaged 7.9% per year
- Why is the R&D cost increasing so much faster than inflation?

Cost of Capital

- 40% of the $2.6 billion is the cost of capital according to the Tufts study
- The 2014 Tufts study estimated the cost of capital at 10.5%
- The direct cost of developing a new drug is closer to $1.3-$1.6 billion
  - Cost reflects many failures

How is the $1.3- $1.6 billion spent?

- Ome concern is that some of this money is not actually spent on research
- Is the money being spent on scientists and equipment?
- Unfortunately, how R&D dollars are spent is proprietary information
- Last month in my testimony I requested the House Oversight Committee to review how drug companies are spending research dollars

The Model of How R&D is Financed Is Changing

- No longer is all of the R&D being done within the drug company
- New model
  - Initial research done in academic medical centers using NIH funds
  - some VC money funds phase 1 and 2 clinical trials
  - pharmaceutical company purchases the research during phase 3, completes the phase 3 trials, and markets the drug
- Is this new model more efficient in producing innovation?

Gilead and Solvaldi - An Example

- Emory researchers conducted the basic science and early testing using NIH funds
- Venture capital supported the next round
- NIH and VC each put in approximately $200 million
- Gilead purchased the company (Pharmasset) for $ 10 billion
- Gilead doubled the price Pharmasset was going to charge to recoup its $10 billion investment

Incentives To Clinical Researchers

- Did the possibility to earn $10 billion influence the type of research the academics chose?
- Have we created a bidding war for promising drugs
- Is this the most cost effective way to develop new drugs?
- You need to pay a premium for the research that fails – but how much is needed to motivate researchers?

Policy Questions

- How much of the purchase price for the R&D for the drug should be tax deductible?
- Is internal development or external purchasing of R&D more cost effective?
- Should the government get a price reduction when government funds helped develop the drug?

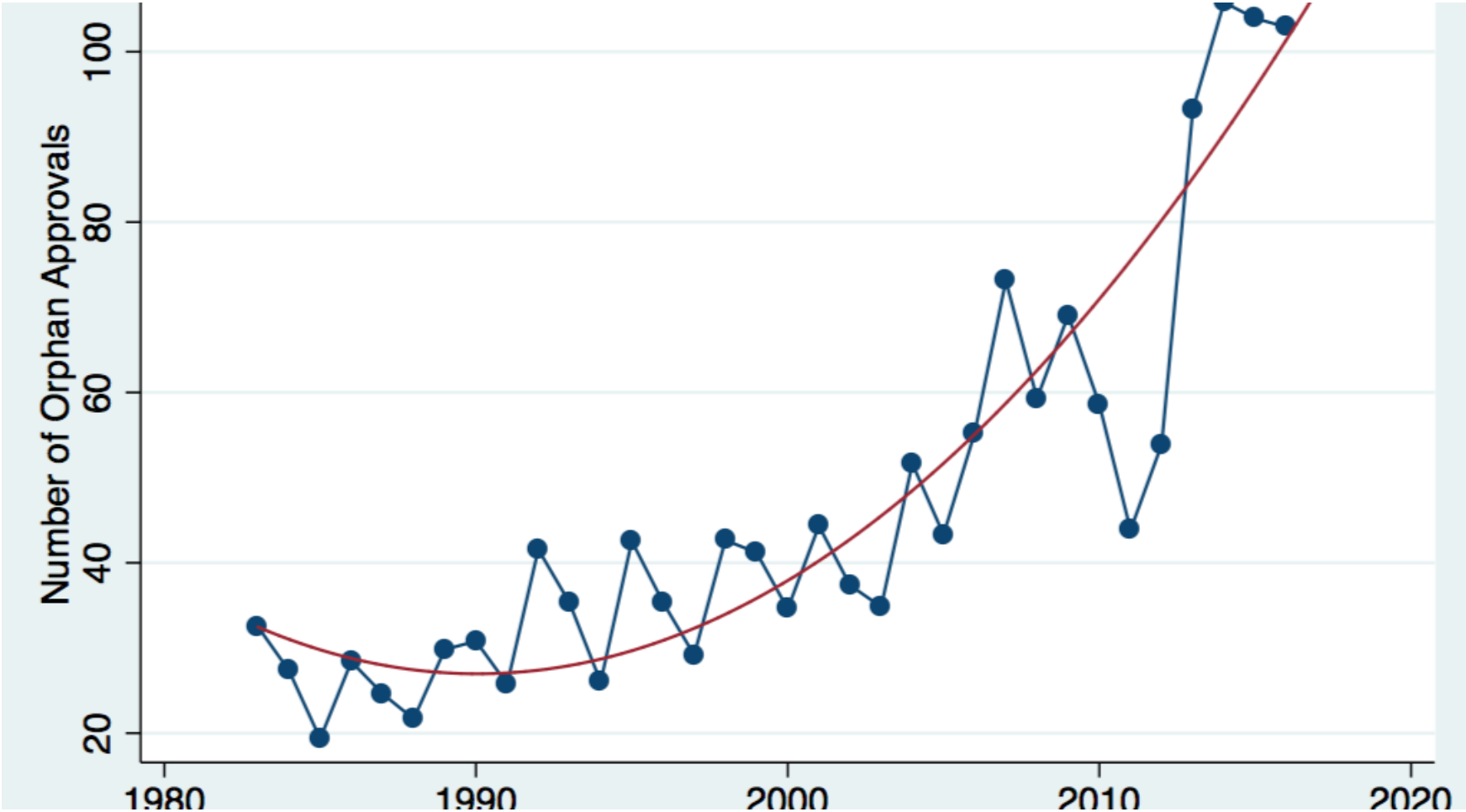
Bayh-Dole

- Bayh-Dole allows the government to lower the price if the government has invested in the drug development
- 5 requests to NIH to use Bayh-Dole; none granted
- What would be the effect on university and industry relationships ? Will it affect use of NIH research?
- What is the appropriate return on the NIH investment?

A Case Study In Innovation - Orphan Drugs

- There are 5000 rare diseases but only 5% have drugs to treat the disease
- What is the best way to motivate research in rare diseases?
- Do we need to revise the Hatch Waxman law?
- According to Hatch Waxman, rare diseases have less than 200,000 potential patients
- Current Government Incentives for orphan diseases
  - 25% tax credit (used to be 50%)
  - Additional market exclusivity period

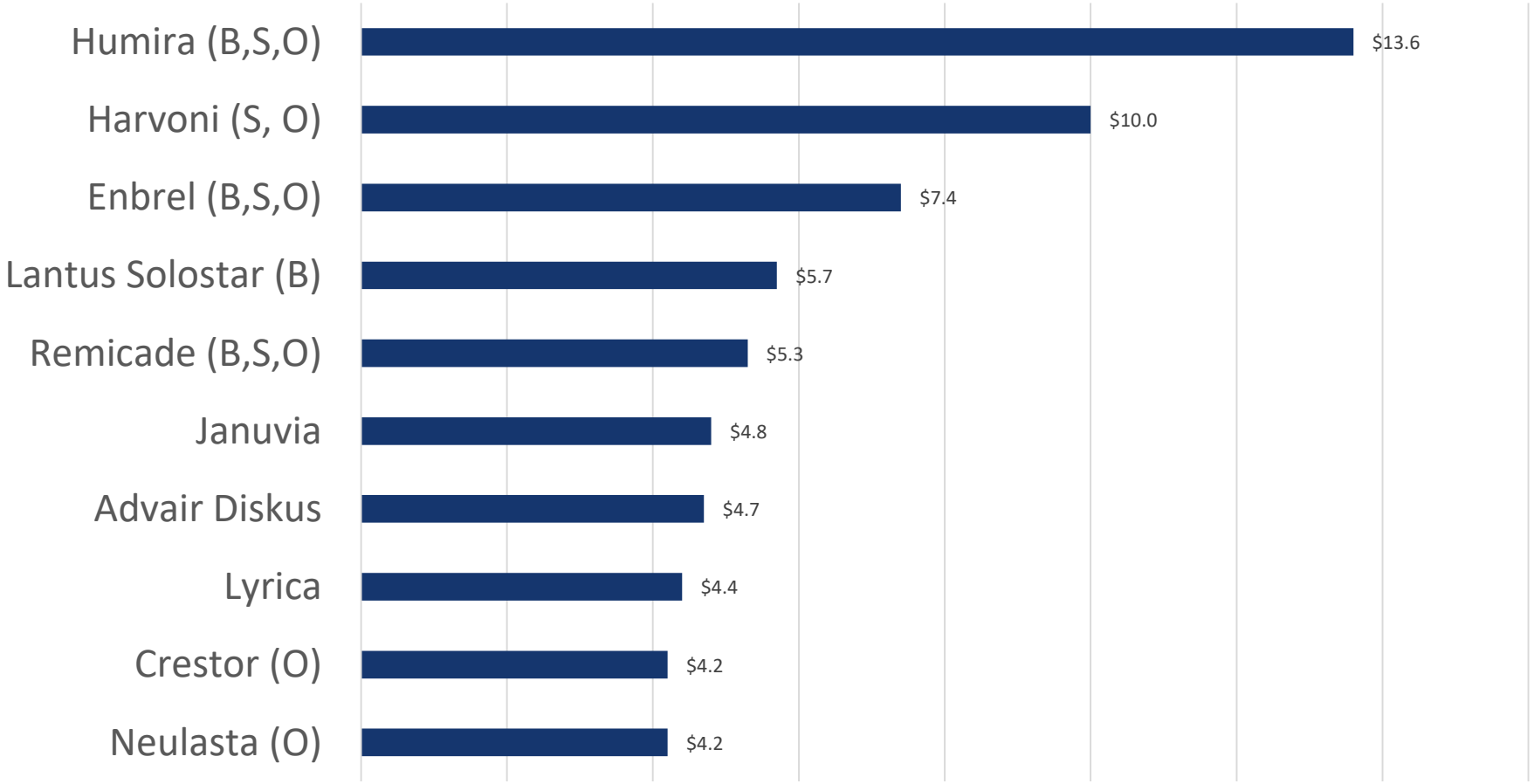
Success – Growing Number of Orphan Drug Approvals 1980-2018



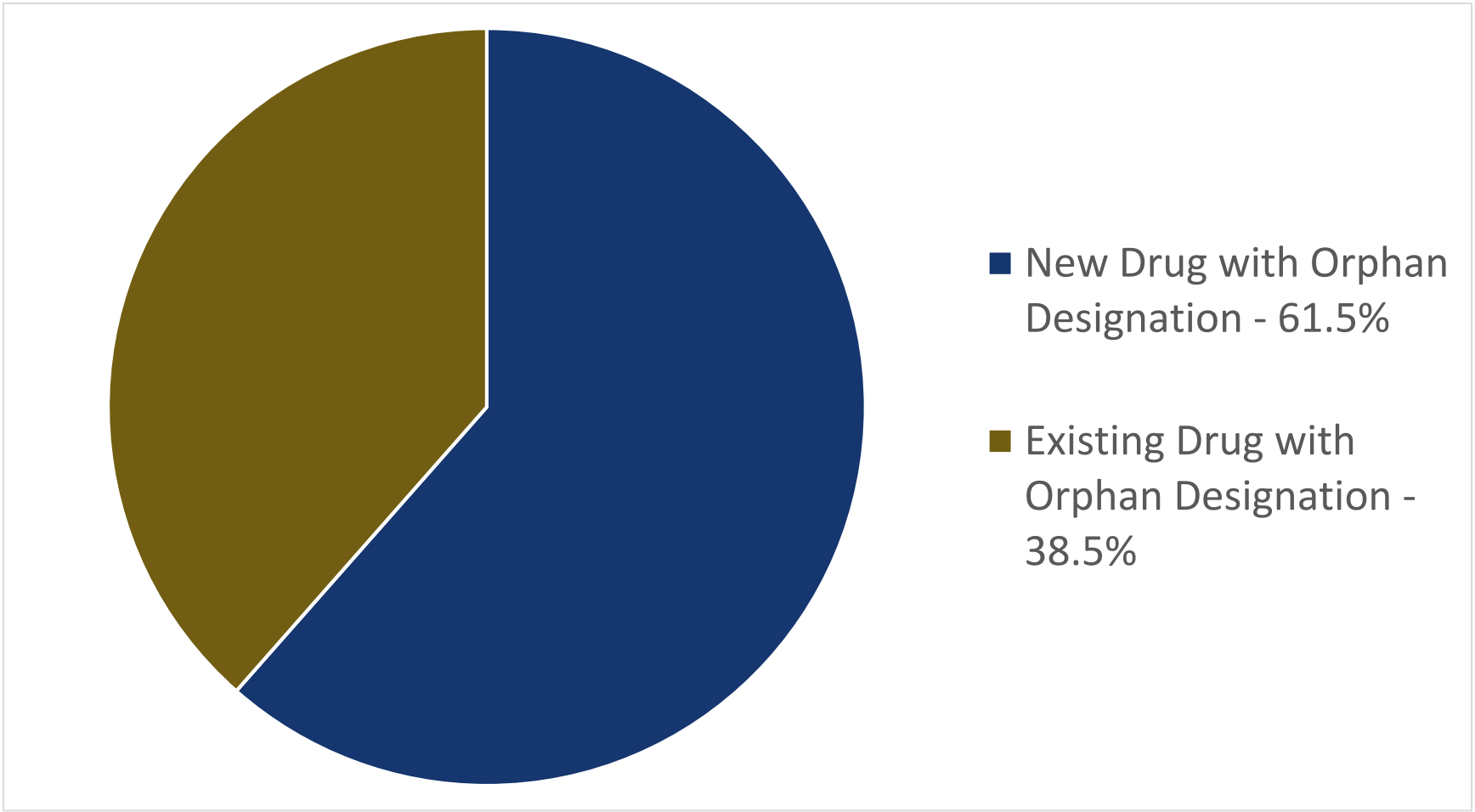
However

- Still have many disease without drugs. Are additional incentives needed?
- At same time, many drugs with orphan approvals also are blockbuster drugs
- 6 out of top 10 best selling drugs in Medicare also have orphan designations

Top Ten Drugs in US by Spending (2016 in $ billions)



Orphan Drug Approvals for New and Existing Drugs



Cost of Clinical Trial

- Averages $19 million with wide variation (1)
- The more effective the drug the less the cost for the clinical trial
  - Fewer patients and shorter duration needed for approval

1. Moore, Thomas J., Hanzhe Zhang, Gerard Anderson, and G. Caleb Alexander. "Estimated costs of pivotal trials for novel therapeutic agents approved by the US Food and Drug Administration, 2015-2016." *JAMA internal medicine* 178, no. 11 (2018): 1451-1457

Significant Return on Investment

- For a drug that has already been developed
  - Only $19 million spent on clinical trial
  - Potential for $ 2 billion in additional revenue
    - PBM typically puts only one drug on formulary
    - Concern about malpractice if give the generic version of drug to a patient that has orphan status
- Argument that need to make a significant profit on blockbuster drugs to support innovation
- But is excess profits from orphan designation the best way to encourage innovation?

Thank You