

Dawn of a Post-Venture Era: The New Face of Innovation & Translational Research

**BIO 2017 Annual Meeting
June 2017**

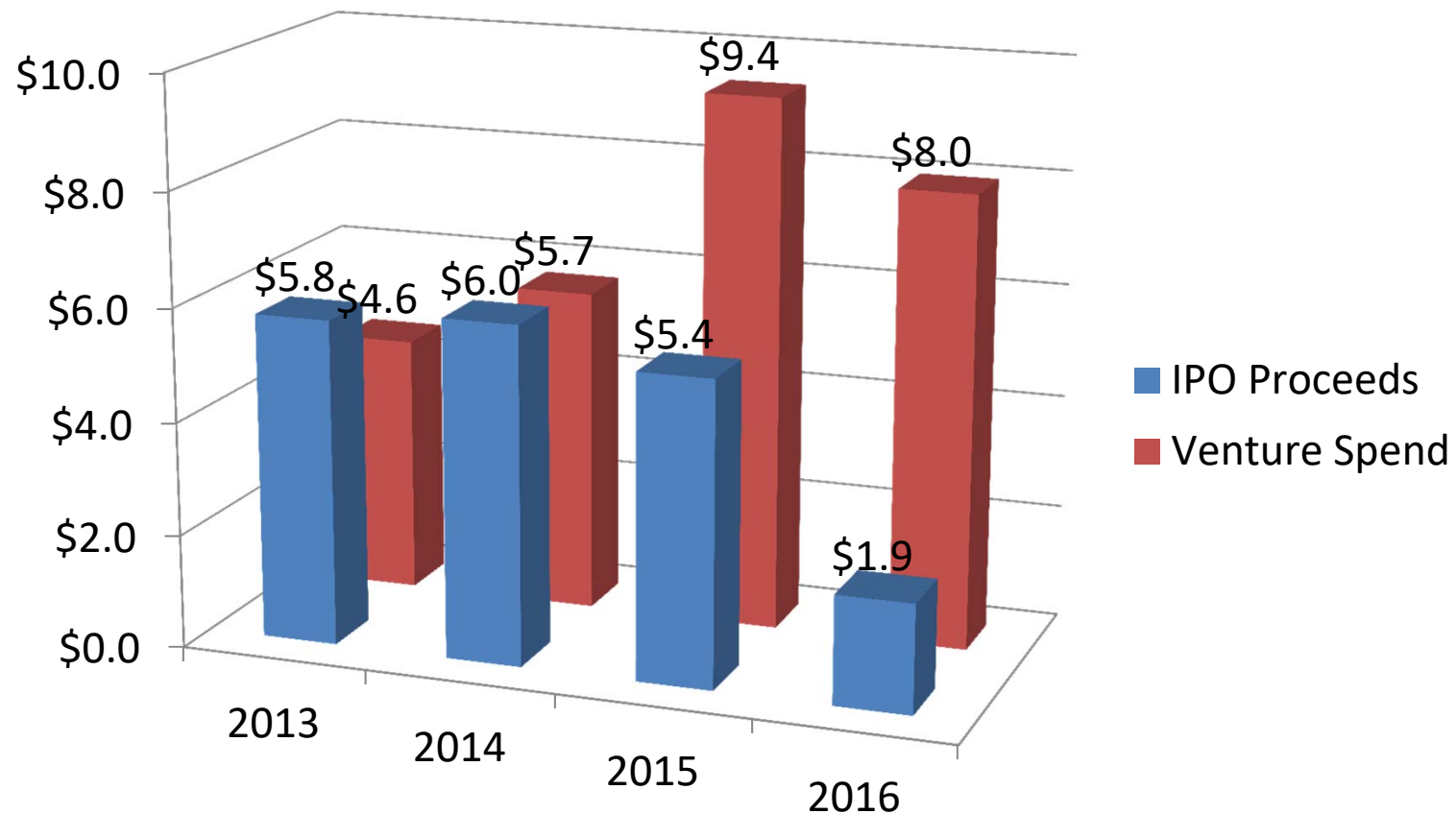
Mark G. Edwards
Managing Director



The New Face of Innovation & Translational Research

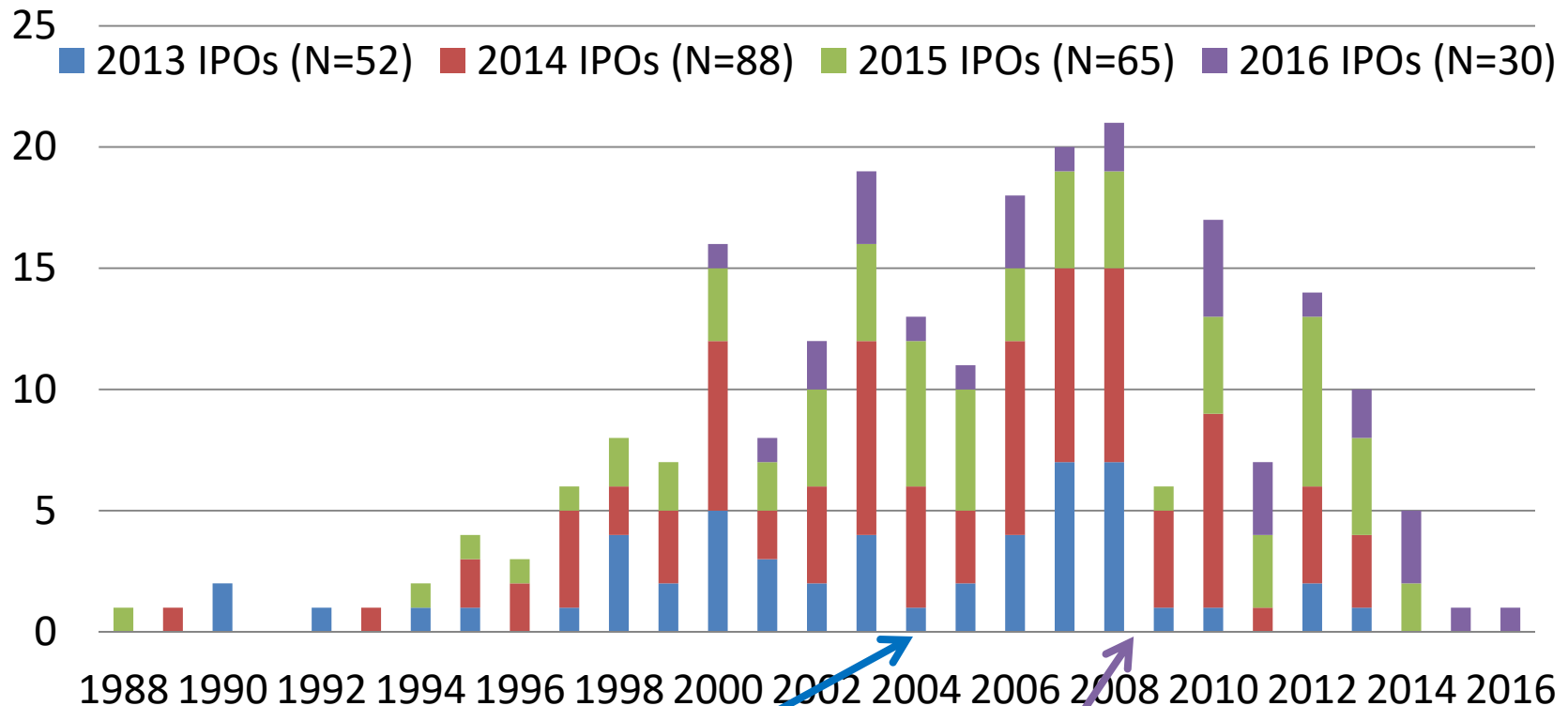
- A Quick “Recap” of Biopharma’s Financial Landscape
- Funding Translational Research: The Road Less Traveled
- An Early Trailblazer – The Cystic Fibrosis Foundation
- Can Research Institutions Thrive in a Post-Venture Era?

Financing for Biopharma has Been Strong in Recent Years, with \$19.1B in IPO Proceeds & \$27.6B in Venture Spend



235 Biotechs Went Public in US From 2013-16, 50% More than over the Previous Decade

Number of IPO Companies by Founding Year

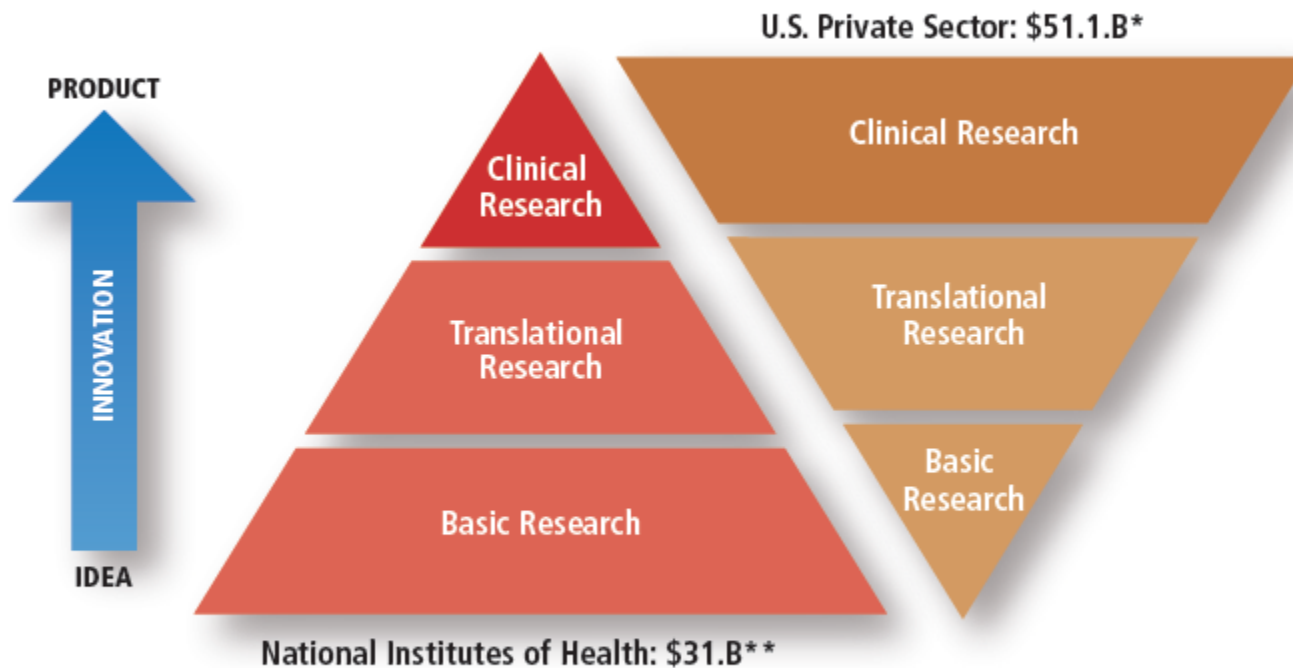


2013 IPOs
Median Year
Founded

2016 IPOs
Median Year
Founded

Industry Maintains that Public & Private Investments in Biopharma are Highly Complementary

Roles of NIH and Private Sector in Biomedical Research

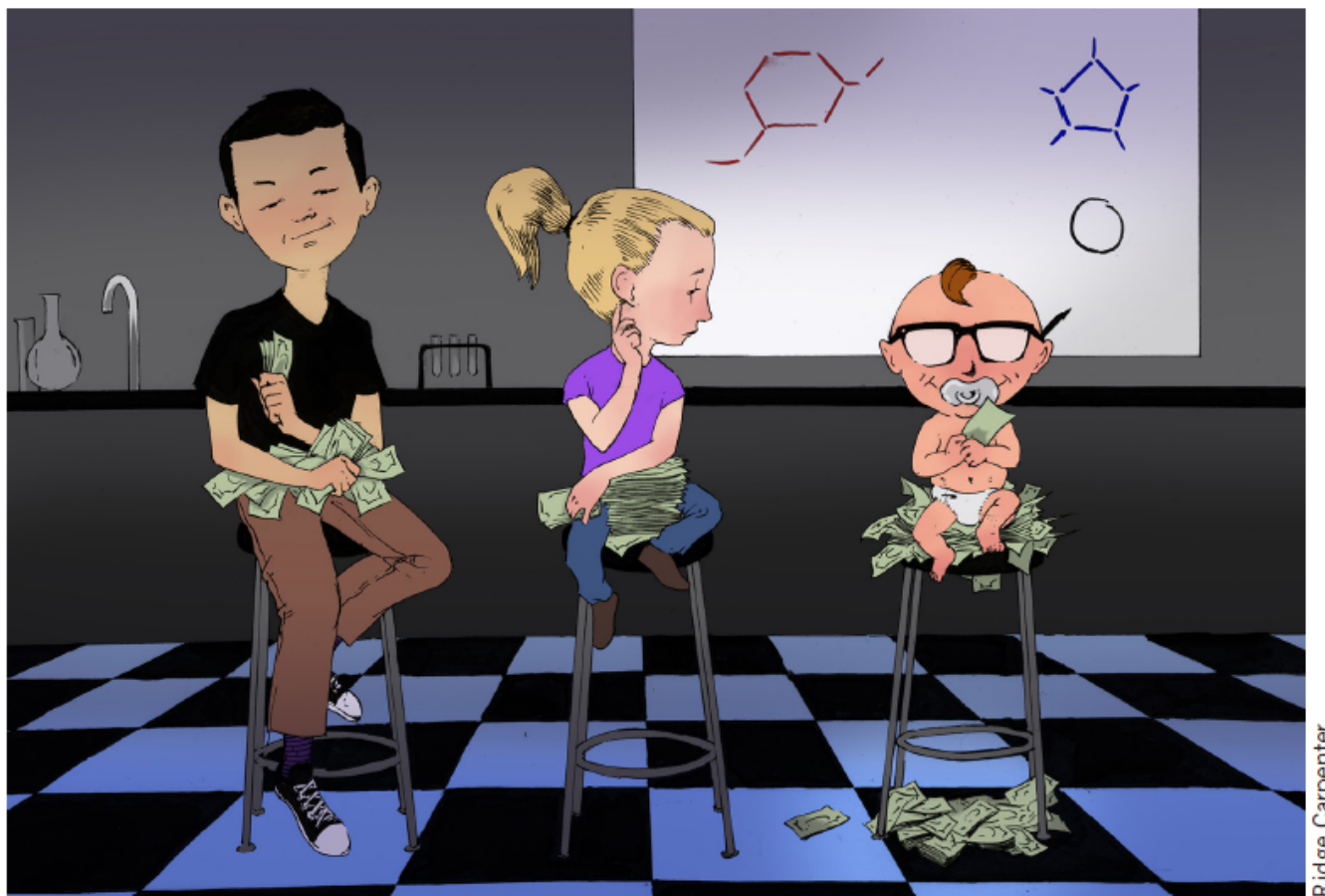


* Batelle analysis for PhRMA: <http://www.pharma.org/sites/default/files/pdf2014economic%20futures-report.pdf>

** National Institute of Health Office of Budget; http://report.nih.gov/categorical_spending.aspx

Source: Public and Private Sector Contributions to the Research & Development of the Most Transformational Drugs of the Last 25 Years, Tufts Jan 2015

Recent Venture Dollars Went to Late Stage & Start-up Companies, But Not Much to Companies in the Middle



In 2016, the youngest companies received the most money.

Source: Biotech's wellspring – a survey of the health of the private sector in 2016, Nature Biotechnology, May 2017

Late Stage and First Round Companies Received Far More From Venture than Companies in the Middle Stages

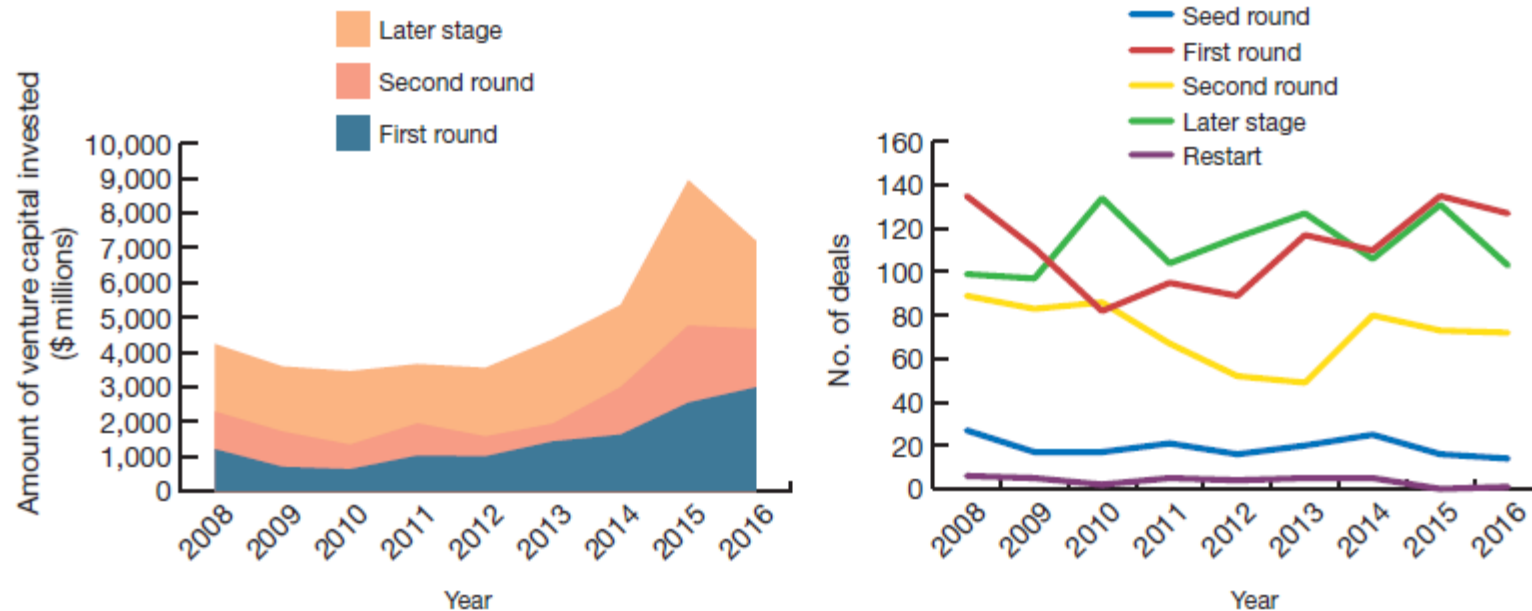


Figure 2 Venture capital investment by stage, number of deals by stage and total invested. Source: Dow Jones VentureSource.

Source: Biotech's wellspring – a survey of the health of the private sector in 2016, Nature Biotechnology, May 2017

Average Biotech Venture Rounds Have Doubled in Size Over the Past Five Years

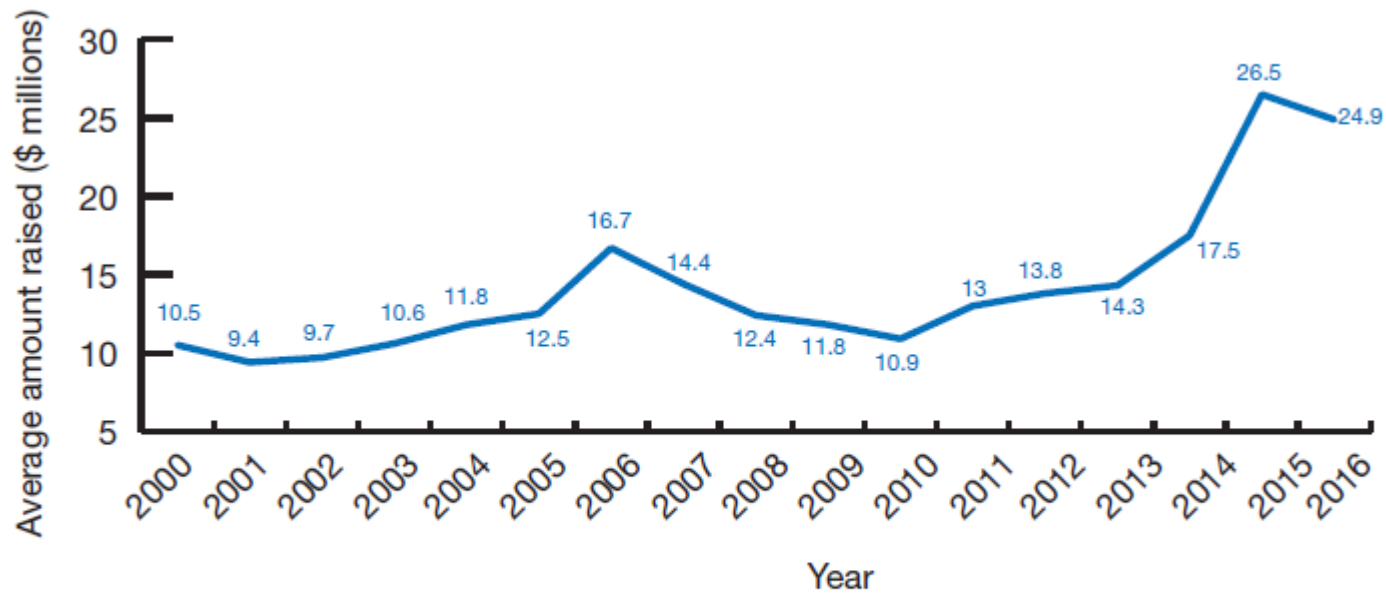
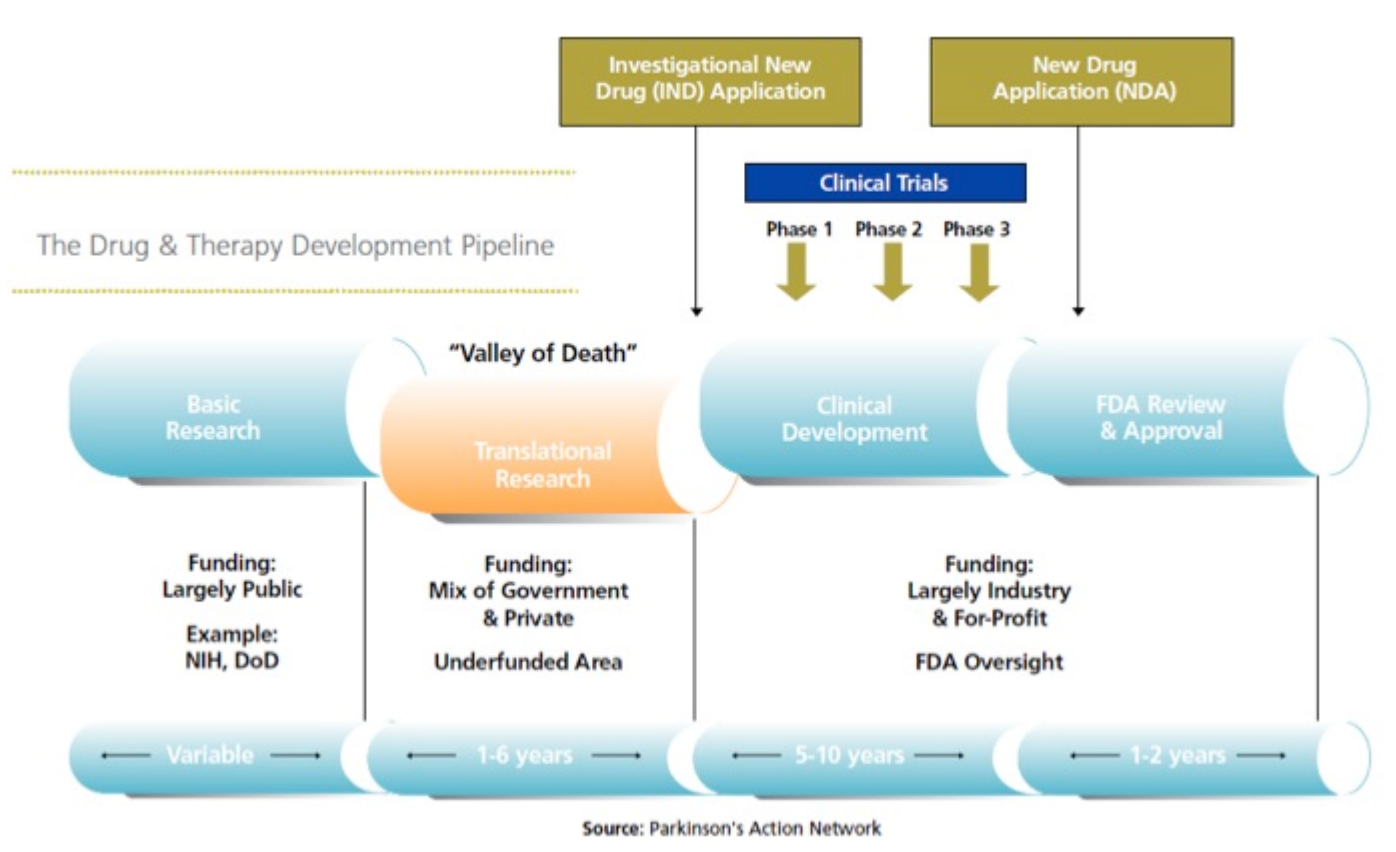


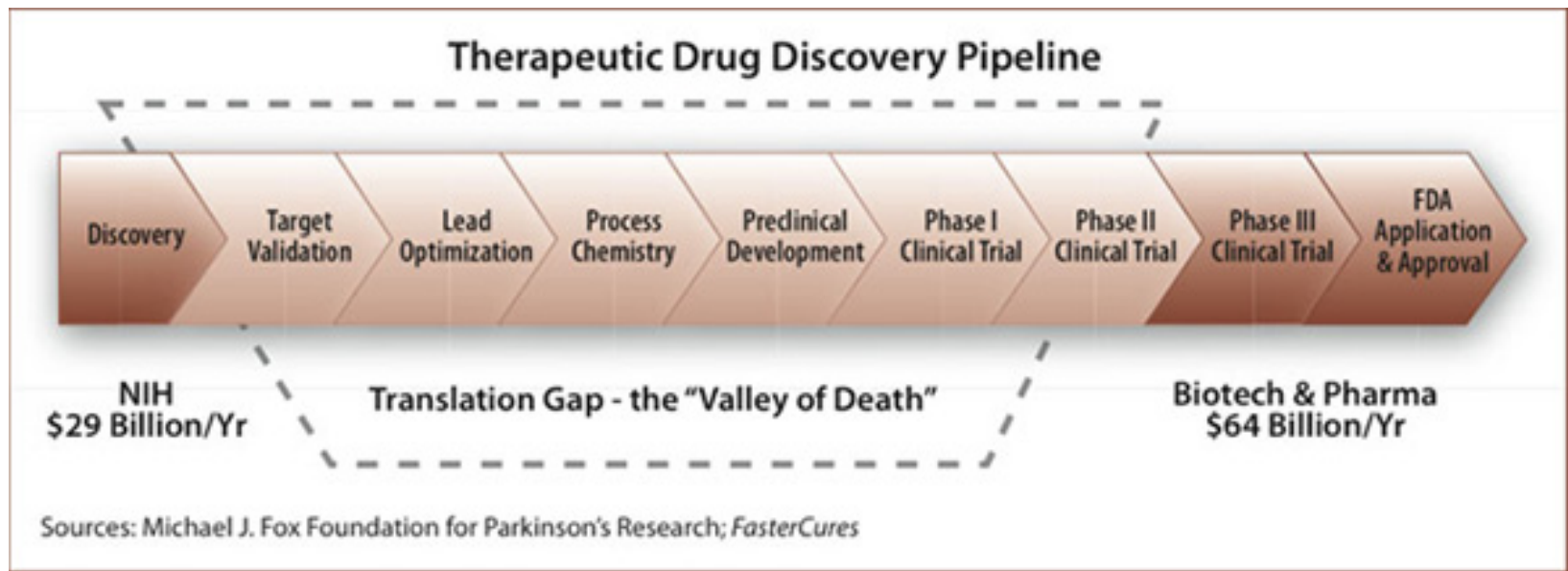
Figure 5 Global average amount invested per biotech venture capital round. Source: Dow Jones VentureSource.

Source: Biotech's wellspring – a survey of the health of the private sector in 2016, Nature Biotechnology, May 2017

Insufficient Funding of Translational Research as been Dubbed “The Valley of Death”



What Work Needs to Be Done to Get Through the Valley?



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Funding Translational Research: The Road Less Traveled

- Over the past three decades, 82% of Biopharma Licenses with Research Institutions have been Early Stage:
 - Discovery (34%)
 - Lead (18%)
 - Early Preclinical (30%)

- Median License Terms have been Generally Static:
 - \$80K Upfront
 - \$720K Milestones
 - 3.5% Royalty
 - 20-25% of Sublicense Revenue
 - 1 in 10 Licenses with Equity

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An Early TrailBlazer: Cystic Fibrosis Foundation Therapeutics

- CFFT has Sponsored Five Early Stage CF Projects:
 - Vertex (2000) \$17.4M + \$5.5M milestones – 6% royalty
 - SGX (2001) \$17.1M + \$6M milestones – 3% royalty
 - Altus (2001) \$25M – \$100M less value of warrant rec'd
 - Epix (2005) \$29.7M + \$7M milestones – 6 to 10%
 - Epirus (2006) \$13.8M + \$5M milestones – 6.5 to 10.5%

- CFFT also Funded Three Early Stage CF Human Clinicals:
 - Inspire (2002) \$2M – 6x funds + \$4M sales milestone
 - AmpliPhi (2003) \$1.7M – 4.5x funds + \$4M sales
 - Harbor (2003) \$1.7M – 6x funds + \$4M sales milestone

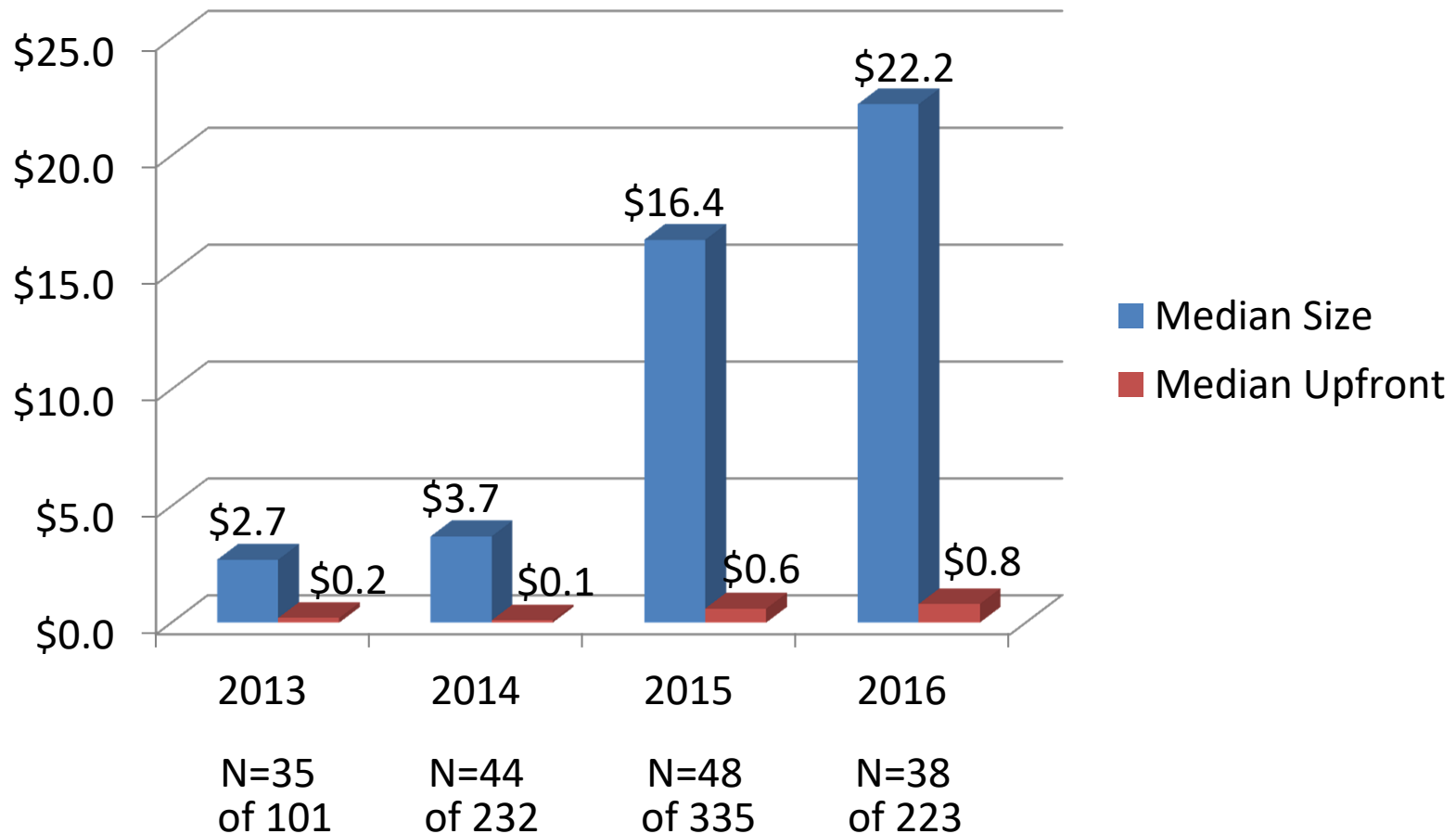
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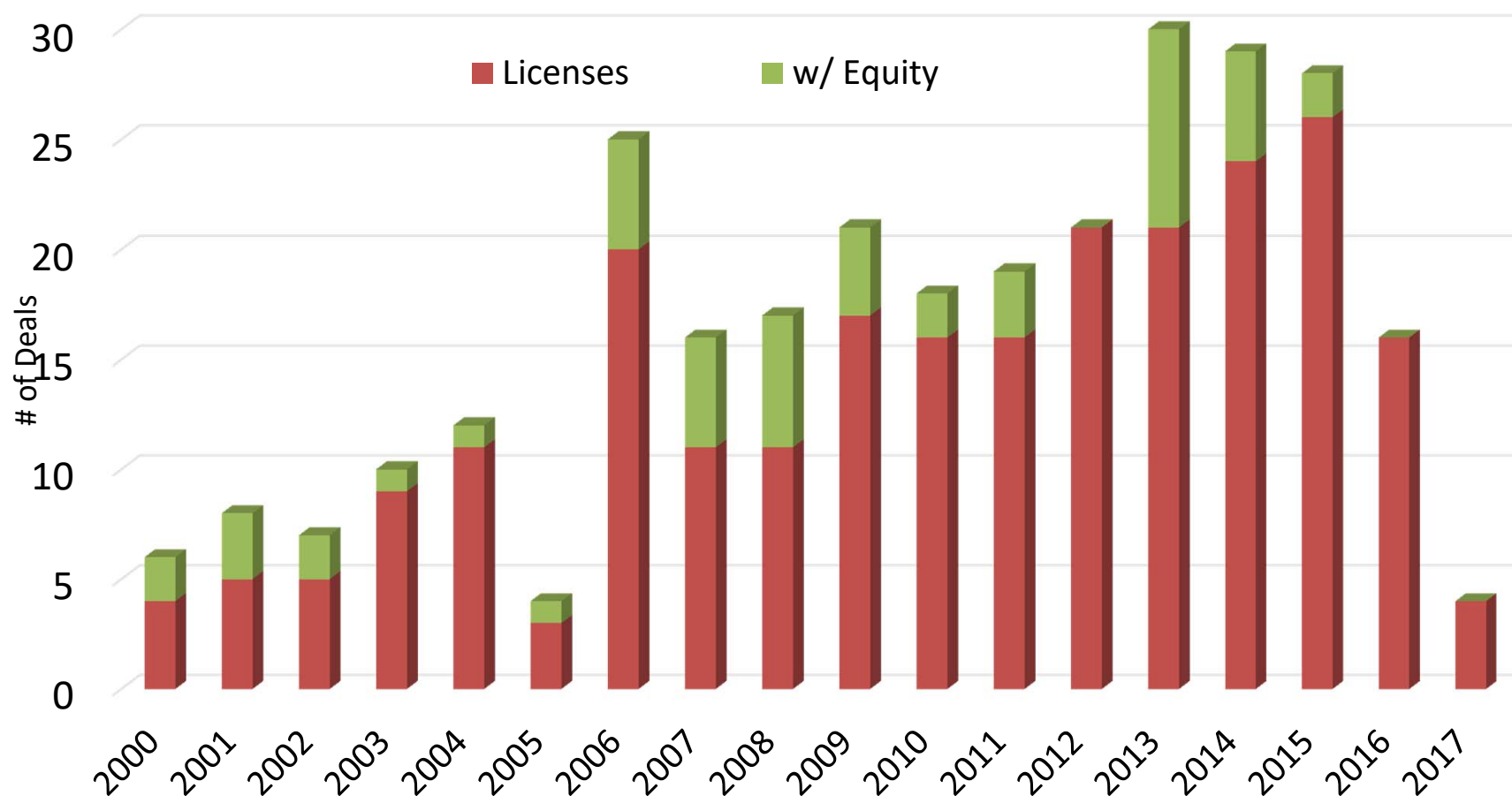
Funding Translational Research: Novel Approaches & New Players

- Case Study – Aeglea BioTherapeutics / CPRIT (6/15)
 - \$19.8M to fund FIH trials for AA depletion drug
 - Repay funding to 4x cap; 0.5% above cap
 - 3% to \$500M, 4% to \$1B, 5% > \$1B cum rev
- Other Sources of Funding:
 - Alliance Revenues – Direct & Downstream
 - Royalty Monetization
 - SBIR/STIR
 - CIRM, Wellcome Trust, Parker, BioHub
 - Venture Philanthropy:
 - Program Related Investments (PRIs)
 - Demand Dividend

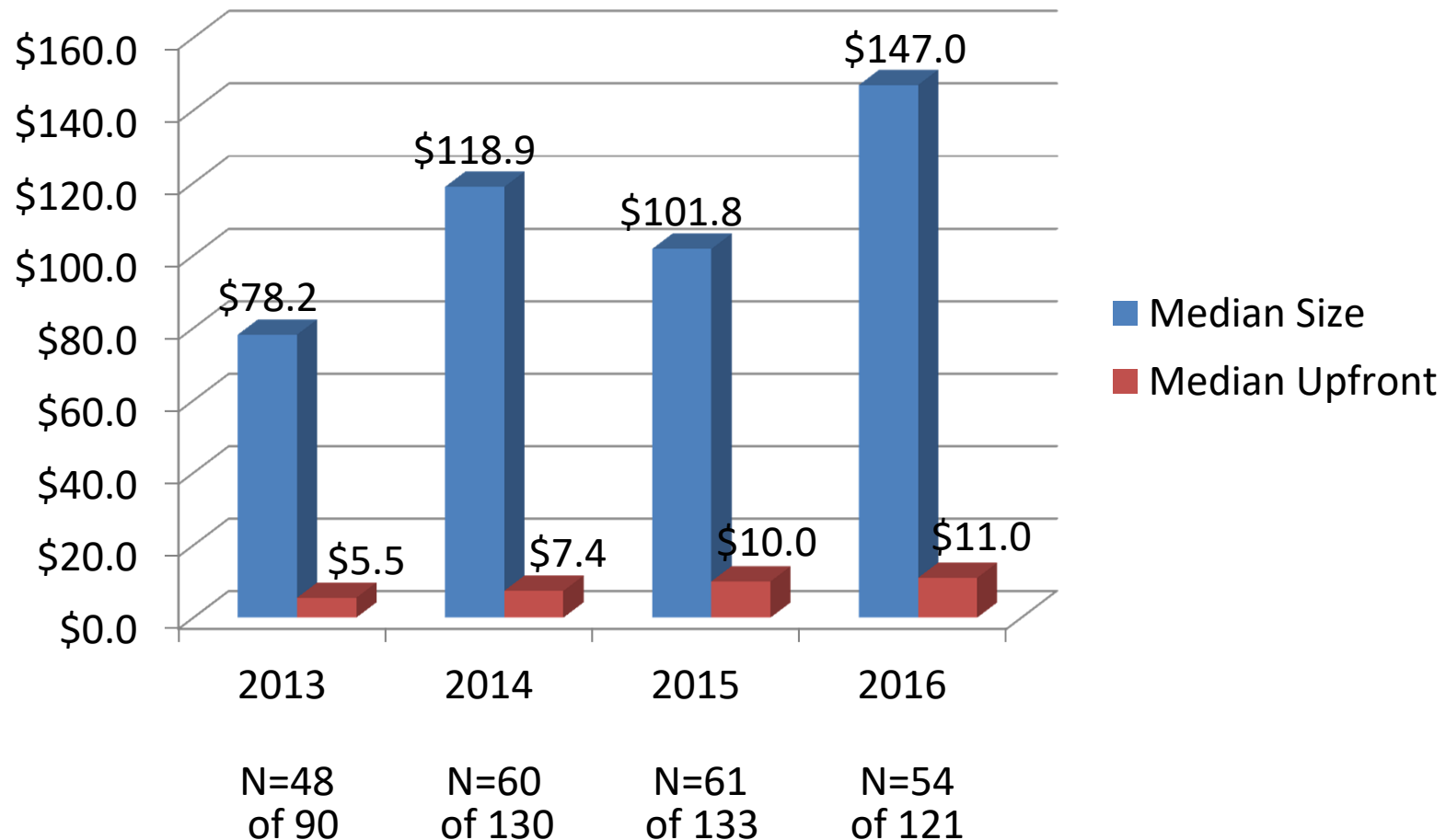
Research Institutions Have Benefited from Recent Increases in Disclosed Upstream License Terms



There are 240 University Licenses with Members of the Biotech IPO Class of 2013-16, & 20% have Equity



Research Institutions Should Benefit Substantially from Recent Increases in IPO Class of 2013-16 Commercialization Alliances

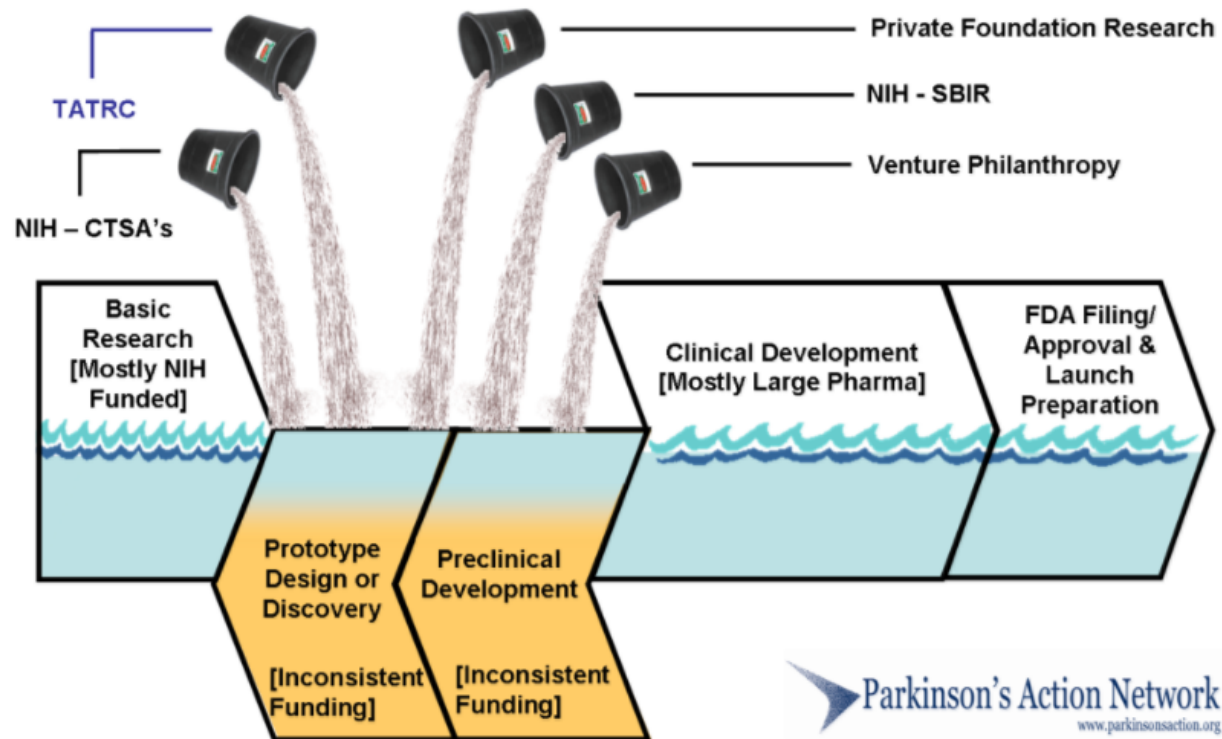


Royalty Monetization: A Potential Source of Translational Research Funding

- \$7.1 Billion has been raised via Royalty Monetization:
 - CFFT/Vertex (Kalydeco) -- \$3.7 Billion (6% royalty)
 - UCLA/Medivation (Xtandi) -- \$1.1 Billion (4% royalty)
 - Northwestern/Pfizer (Lyrica) -- \$990M (6% royalty)
 - NYU/Centocor (Remicade) -- \$650M (6% royalty)
 - CHOP/Merck (RotaTeq) -- \$182M
 - Dana Farber/Multiple (PD-L1) -- \$168M
 - MRC/Merck (Keytruda) -- \$150M
 - Children's Boston/Celgene (Revlimid) -- \$132M (1%)
 - Penn/Aegerion (Juxtapid) -- \$55M (5-8% royalty)

Who Will Fill the Translational Research Funding Gap?

The Development Pipeline and Valley of Death Various Solutions



Parkinson's Action Network
www.parkinsonsaction.org