Ensuring Continued U.S. Biopharmaceutical Competitiveness

Alpha-1 Foundation Virtual Annual Conference

Stephen Ezell
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Information Technology and Innovation Foundation (ITIF)

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Today's Presentation

1 How the U.S. Became World's Life-sciences Innovation Leader

2 Rebutting U.S. Life-sciences Industry Criticisms

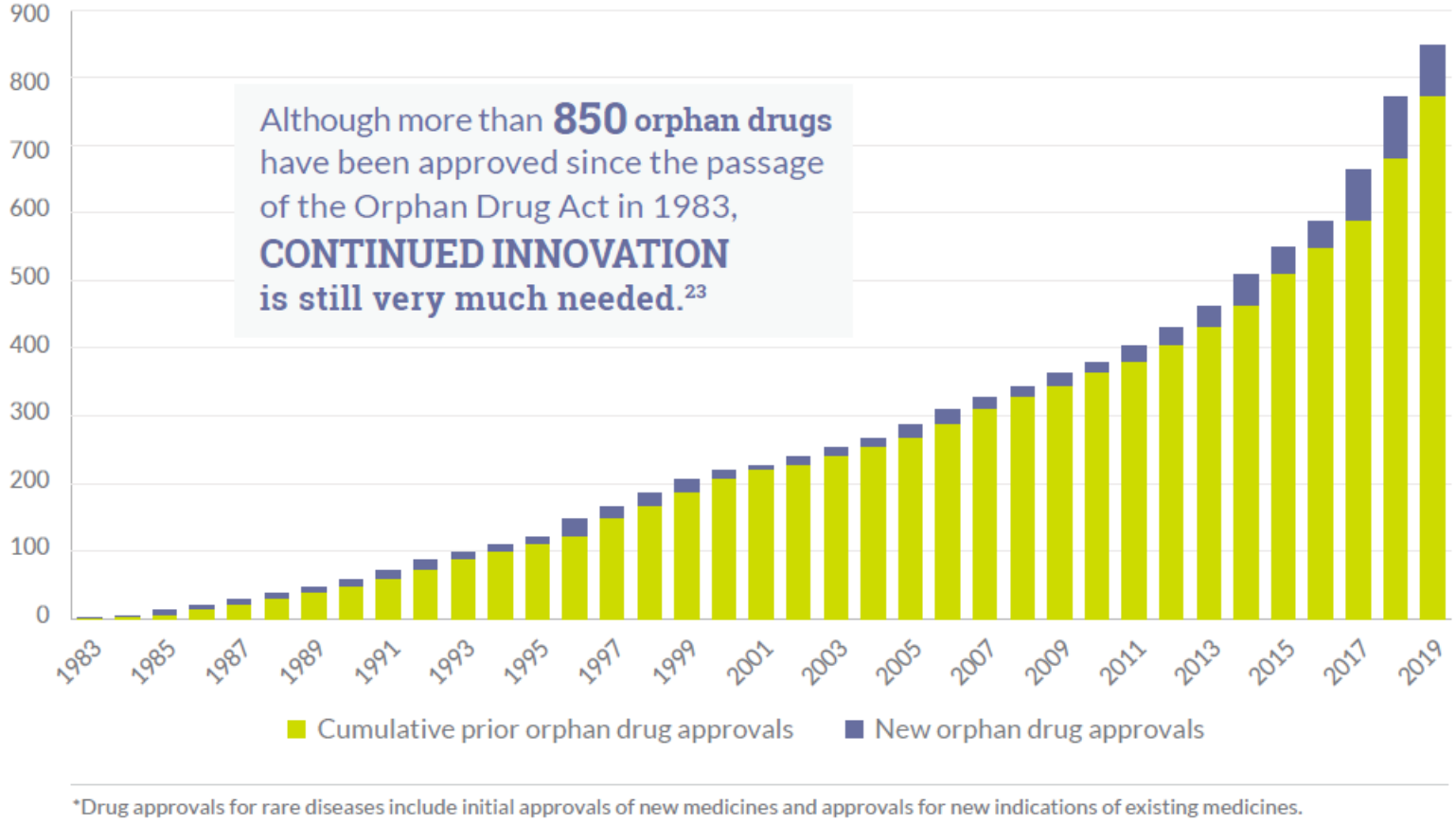
3 Policy Recommendations for Continued U.S. Leadership

United States Leads the World In New Drug Development

8,000 Medicines Under Development Globally

United States Leads the World In New Drug Development

Over 850 Drug Approvals for Rare Diseases Since 1983

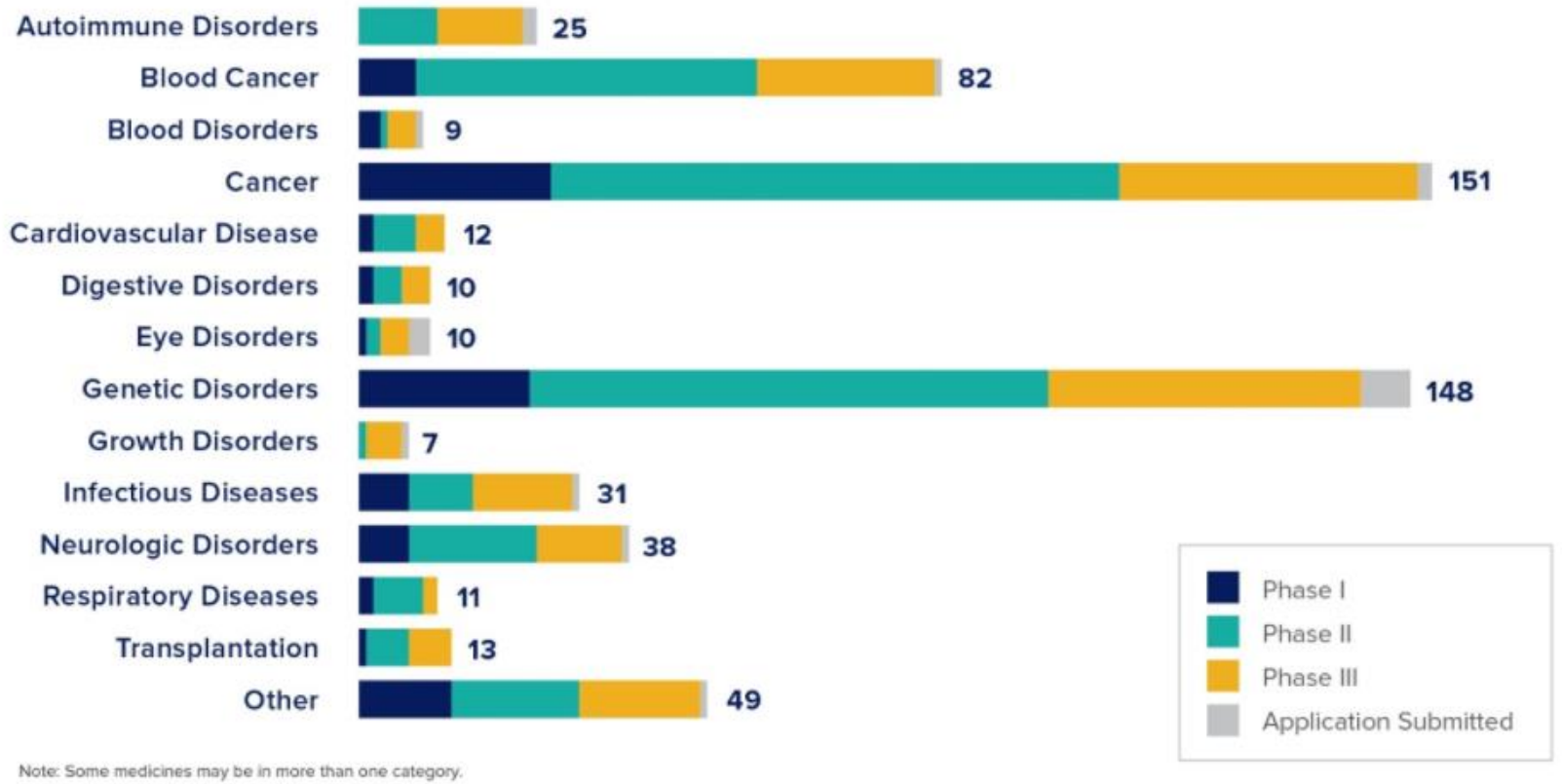


*Drug approvals for rare diseases include initial approvals of new medicines and approvals for new indications of existing medicines.

Source: PhRMA, "Biopharmaceuticals in Perspective, 2020 Chart Pack"

United States Leads the World In New Drug Development

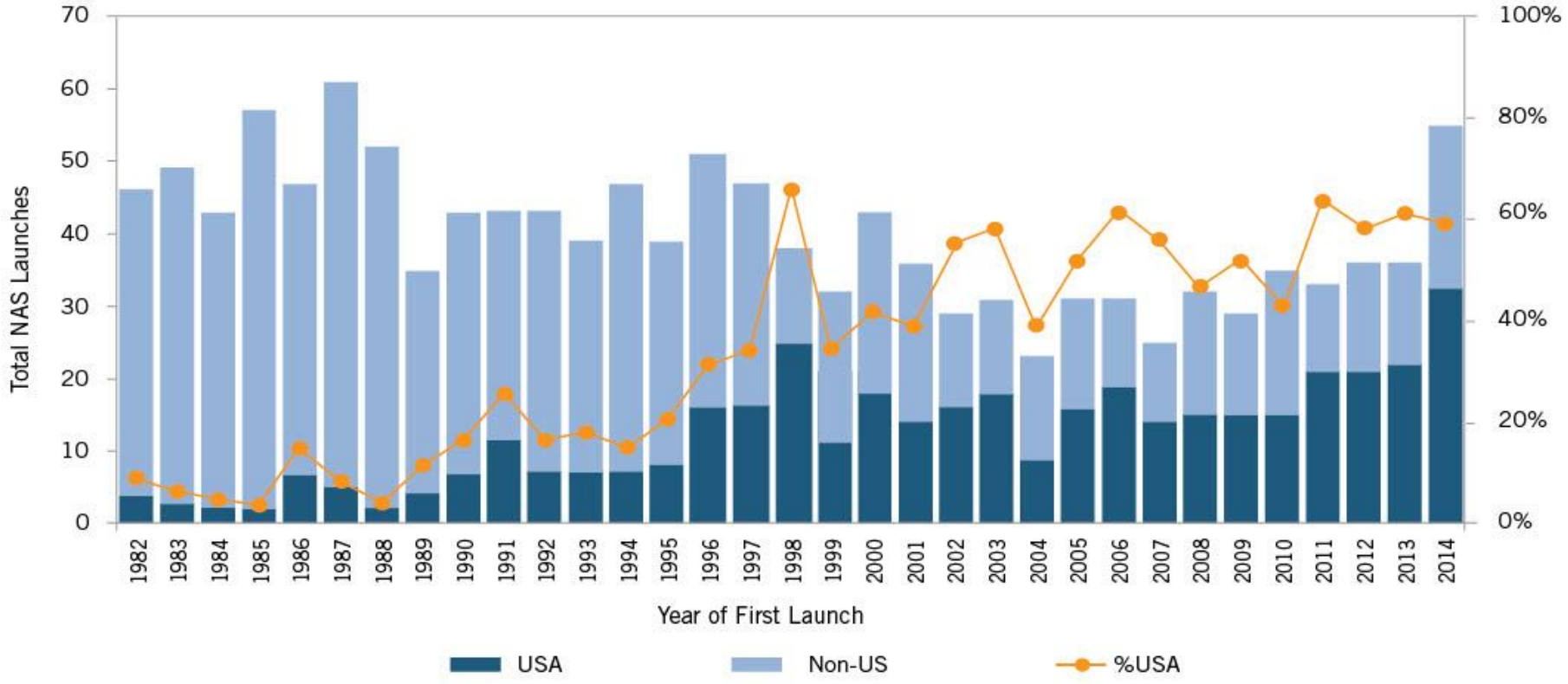
Medicines in Development for Rare Diseases



Source: PhRMA, "Medicines in Development for Rare Diseases"

But U.S. Life-sciences Leadership A Recent Phenomenon

U.S. Share of New Active Substances (NAS) Launched First on World Market



Source: John K. Jenkins, M.D., "CDER New Drug Review: 2015 Update

United States Leads the World In New Drugs Developed

New Chemical or Biological Entities: By Number and By Share GDP ($ Trillions)

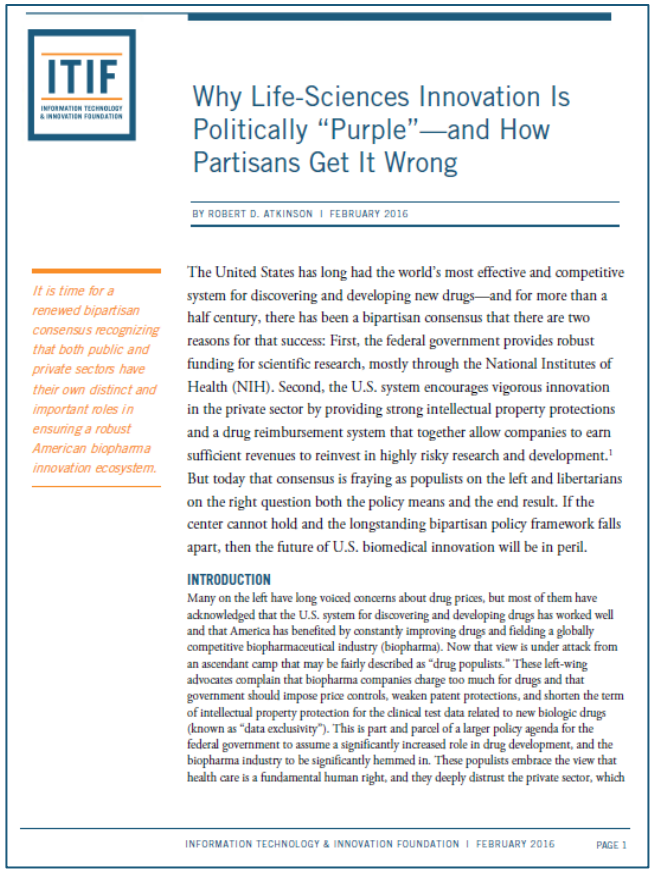
Region	1999–2003	2004–2008	2009–2013	2014–2018	Total: 2009–2018
Europe	62	47	66	67	133
U.S.	73	67	64	125	189
Japan	28	16	26	34	50
Other	8	14	23	41	64

Region	1999–2003	2004–2008	2009–2013	2014–2018	Total: 2009–2018
Europe	1.53	0.70	0.88	0.91	0.90
U.S.	1.38	0.98	0.82	1.32	1.10
Japan	1.25	0.68	0.91	1.42	0.95
Other	0.14	0.13	0.13	0.20	0.17

Source: ITIF, "Ensuring U.S. Biopharmaceutical Competitiveness"; EFPIA, ""The Pharmaceutical Industry in Figures, Key Data 2019"

Keys to U.S. Life-Sciences Innovation Leadership

1. Robust public/private investment in biomedical research.
2. Aggressive incentives to encourage investment.
3. Effective regulatory/drug approval system (PDUFA).
4. Robust intellectual property rights & protections.
5. Pricing/reimbursement system allowing innovators to earn sufficient revenues.



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Why Life-Sciences Innovation Is Politically "Purple"—and How Partisans Get It Wrong

BY ROBERT D. ATKINSON | FEBRUARY 2016

It is time for a renewed bipartisan consensus recognizing that both public and private sectors have their own distinct and important roles in ensuring a robust American biopharma innovation ecosystem.

The United States has long had the world's most effective and competitive system for discovering and developing new drugs—and for more than a half century, there has been a bipartisan consensus that there are two reasons for that success: First, the federal government provides robust funding for scientific research, mostly through the National Institutes of Health (NIH). Second, the U.S. system encourages vigorous innovation in the private sector by providing strong intellectual property protections and a drug reimbursement system that together allow companies to earn sufficient revenues to reinvest in highly risky research and development.[1] But today that consensus is fraying as populists on the left and libertarians on the right question both the policy means and the end result. If the center cannot hold and the longstanding bipartisan policy framework falls apart, then the future of U.S. biomedical innovation will be in peril.

INTRODUCTION

Many on the left have long voiced concerns about drug prices, but most of them have acknowledged that the U.S. system for discovering and developing drugs has worked well and that America has benefited by constantly improving drugs and fielding a globally competitive biopharmaceutical industry (biopharma). Now that view is under attack from an ascendant camp that may be fairly described as "drug populists." These left-wing advocates complain that biopharma companies charge too much for drugs and that government should impose price controls, weaken patent protections, and shorten the term of intellectual property protection for the clinical test data related to new biologic drugs (known as "data exclusivity"). This is part and parcel of a larger policy agenda for the federal government to assume a significantly increased role in drug development, and the biopharma industry to be significantly hemmed in. These populists embrace the view that health care is a fundamental human right, and they deeply distrust the private sector, which

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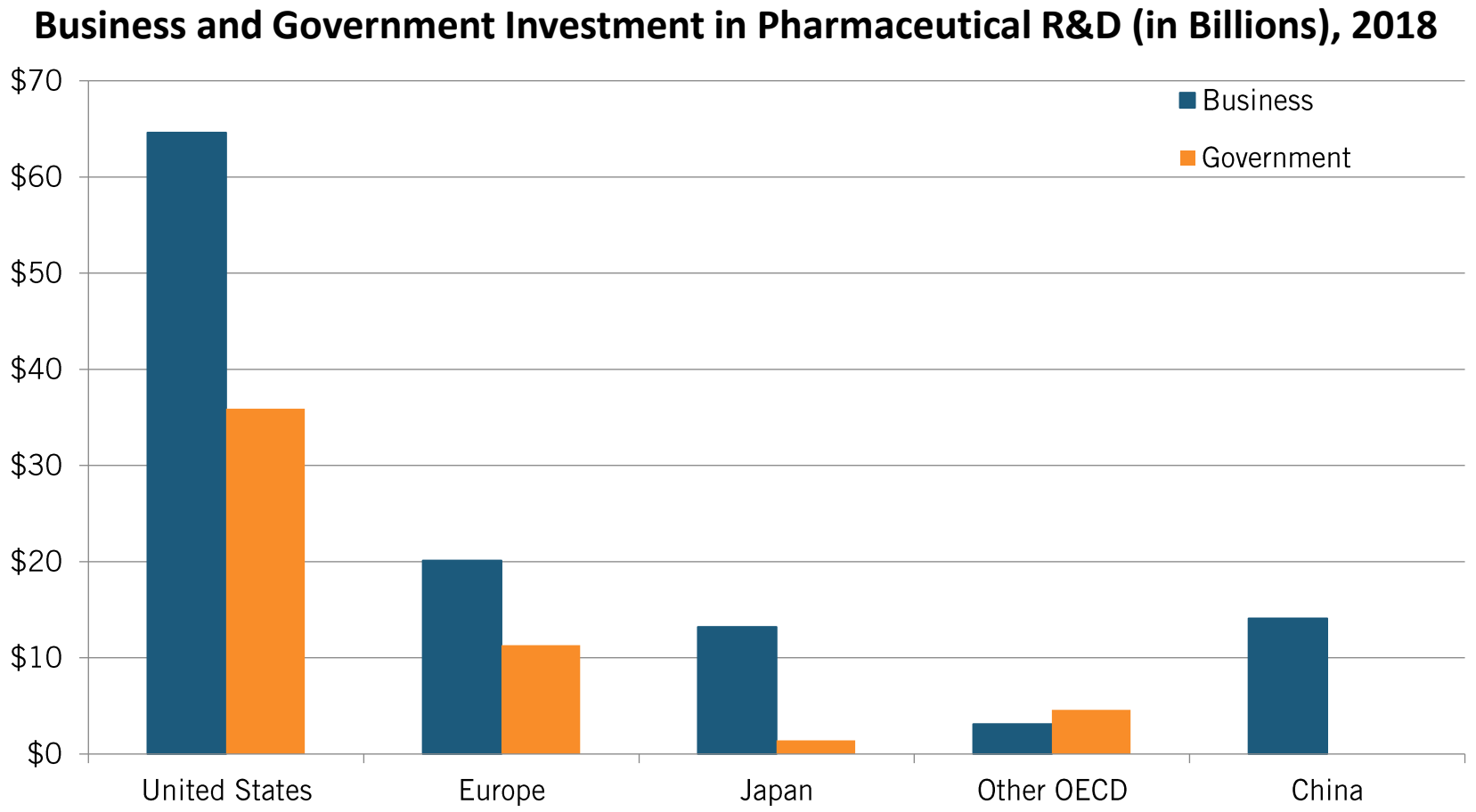
Source: ITIF, "Why Life-Sciences Innovation is "Politically Purple"—And How Partisans Get It Wrong"

United States Leads World in Biopharmaceutical R&D



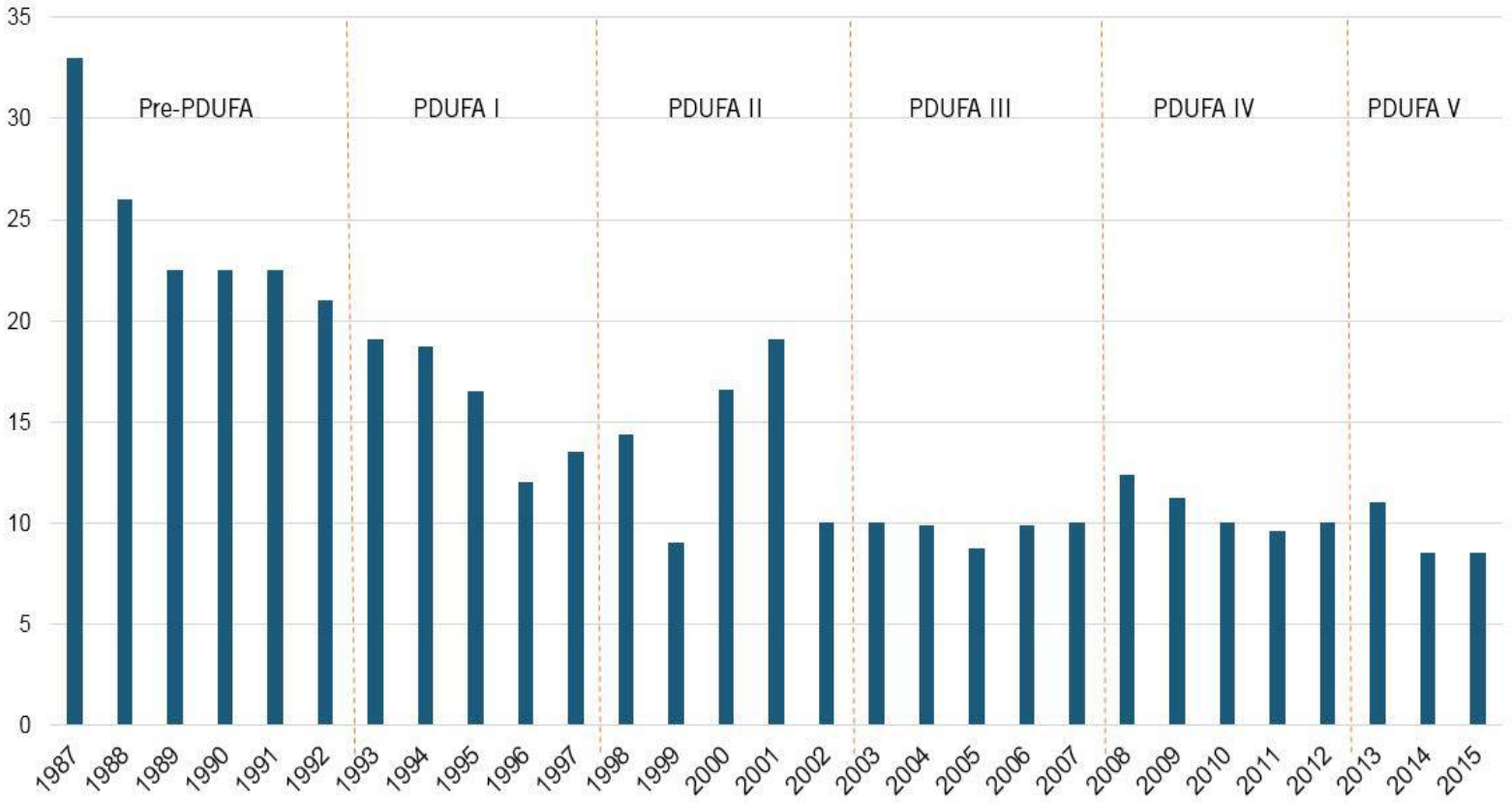
Source: ITIF, "Ensuring U.S. Biopharmaceutical Competitiveness"

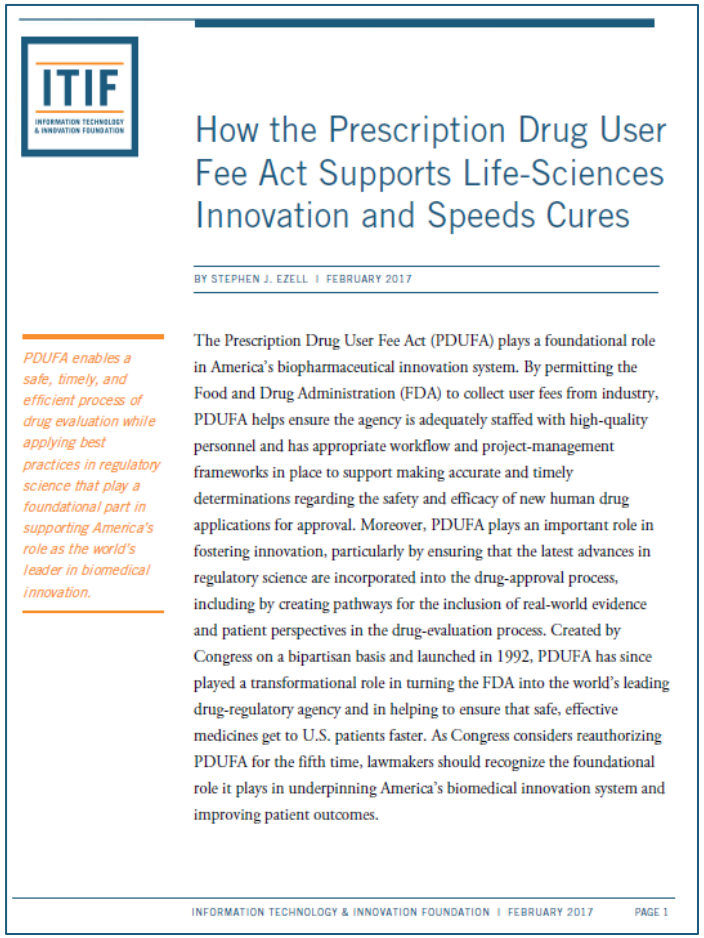
Aggressive Measures to Incent Life-sciences Innovation

- In 1981, U.S. the world's first country to introduce R&E tax credit.
  - Over 40 U.S. states now offer R&E tax credits as well.
- In 1983, U.S. introduced the Orphan Drug Tax Credit.
  - From 1983-2018, provided 50% credit for clinical testing costs (now 25%).
  - Has led to approvals for over 850 products treating over 250 rare diseases.

An Effective Regulatory/Drug Approval System

Median Approval Times for New Medicines, Months (CDER NME NDAs/BLAs)





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How the Prescription Drug User Fee Act Supports Life-Sciences Innovation and Speeds Cures

BY STEPHEN J. EZELL | FEBRUARY 2017

PDUFA enables a safe, timely, and efficient process of drug evaluation while applying best practices in regulatory science that play a foundational part in supporting America's role as the world's leader in biomedical innovation.

The Prescription Drug User Fee Act (PDUFA) plays a foundational role in America's biopharmaceutical innovation system. By permitting the Food and Drug Administration (FDA) to collect user fees from industry, PDUFA helps ensure the agency is adequately staffed with high-quality personnel and has appropriate workflow and project-management frameworks in place to support making accurate and timely determinations regarding the safety and efficacy of new human drug applications for approval. Moreover, PDUFA plays an important role in fostering innovation, particularly by ensuring that the latest advances in regulatory science are incorporated into the drug-approval process, including by creating pathways for the inclusion of real-world evidence and patient perspectives in the drug-evaluation process. Created by Congress on a bipartisan basis and launched in 1992, PDUFA has since played a transformational role in turning the FDA into the world's leading drug-regulatory agency and in helping to ensure that safe, effective medicines get to U.S. patients faster. As Congress considers reauthorizing PDUFA for the fifth time, lawmakers should recognize the foundational role it plays in underpinning America's biomedical innovation system and improving patient outcomes.

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Source: John K. Jenkins, M.D., "CDER New Drug Review: 2015 Update; ITIF, "How the Prescription Drug User Fee Act Supports Life-sciences Innovation and Speeds Cures"

Robust IP Rights Essential for Life-sciences Innovation

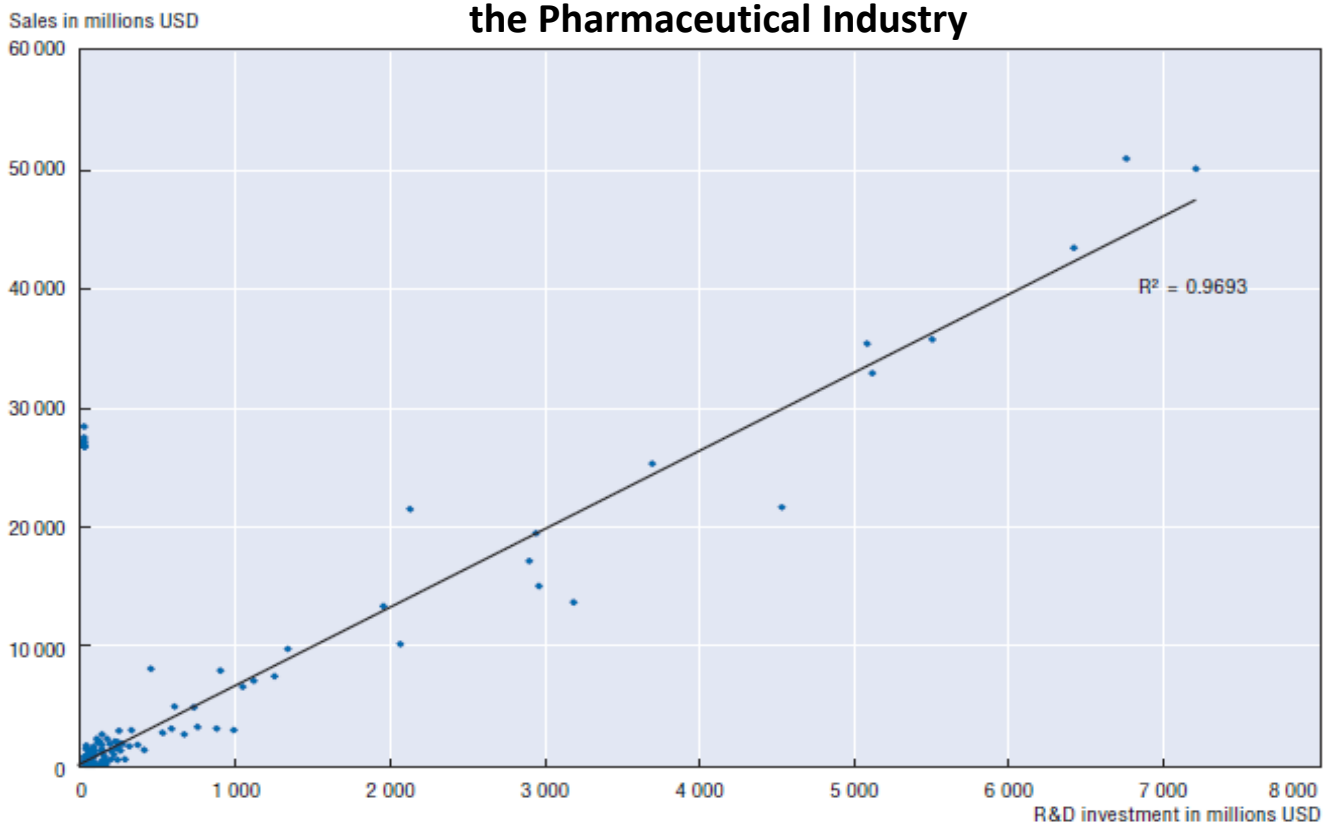
- Robust IP rights incent investment in expensive, risky, lengthy innovation.
  - Takes 12-14 years to bring new drugs to market at a cost of $2.5 billion.
- IP constitutes as much as 80% of a life-sciences company's value.

- Bayh-Dole Act allows licensing of IP resulting from federally funded research.
- Requires clear standards for patentability and subject-matter eligibility.

Reasonable Profits Are Vital to Biopharmaceutical Innovation

- OECD: "There exists a high degree of correlation between pharmaceutical sales revenues and R&D expenditures."
- Every $2.5 billion of additional revenue leads to a new drug approval.
- CBO: Price controls would reduce the number of new drugs 3-5% over the next decade.

Relationship Between Sales and R&D Expenditures in the Pharmaceutical Industry



Sources: OECD, "Pharmaceutical Pricing Policies in a Global Market"; Dubois, "Market size and pharmaceutical innovation";
CBO: "Effects of Drug Price Negotiation Stemming From Title 1 of H.R. 3, the Lower Drug Costs Now Act of 2019, on Spending and Revenues Related to Part D of Medicare"

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Rebutting Criticisms Against the U.S. Life-sciences Industry

1. The industry has become extremely concentrated.
2. Companies are cutting R&D to boost profits.
3. Drug innovation has stalled.
4. Drug prices have grown abnormally and rapidly.
5. The government plays the lead in drug development.


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Five Fatal Flaws in Rep. Katie Porter's Indictment of the U.S. Drug Industry

ROBERT D. ATKINSON AND STEPHEN EZELL | MAY 2021

In her sensationally titled report, "Killer Profits: How Big Pharma Takeovers Destroy Innovation and Harm Patients," the deputy chair of the Congressional Progressive Caucus issued an ideologically inspired jeremiad grounded in assertions that are easily refuted with data.

KEY TAKEAWAYS

- Drug industry concentration has increased only modestly. The top eight firms increased their market share from 54 percent in 2002 to 58 percent in 2017, a ratio viewed by antitrust experts as unconcentrated.
- Large drug firms are not disinvesting in R&D to boost profits. In fact, the industry's R&D-to-sales ratio has increased and is now the world's most R&D-intensive industry.
- Contrary to the Porter report's claim that new drug innovation is declining, FDA data shows that it has been increasing.
- Contrary to the report's claim that drug prices are increasing dramatically and that the industry is earning excessive profits, the data show otherwise.
- Contrary to the assertion that the government is largely to thank for breakthrough drug development, patent and investment data show that private biopharma firms devote far more capital to develop and bring drugs to market than government.
- Efforts to paint America's private-sector-led drug-development system as failing are misleading and serve mainly to build the case for radical change that would undermine the country's capacity to drive drug innovation and create jobs.
- The private-sector-led system—with a healthy mix of large, midsized, and start-up firms, plus government funding for basic science—is working well.

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Source: ITIF, "Five Fatal Flaws in Rep. Katie Porter's Indictment of the U.S. Drug Industry"

U.S. Life-sciences Industry Isn't Inordinately Concentrated

- In 2006, the top 10 drug producers accounted for 56% of global industry sales, which fell to 43% by 2019.
- The critical "C4" ratio increased only slightly, from 36% to 43%, from 2002 to 2017; the "C8" from 54% to 58%.

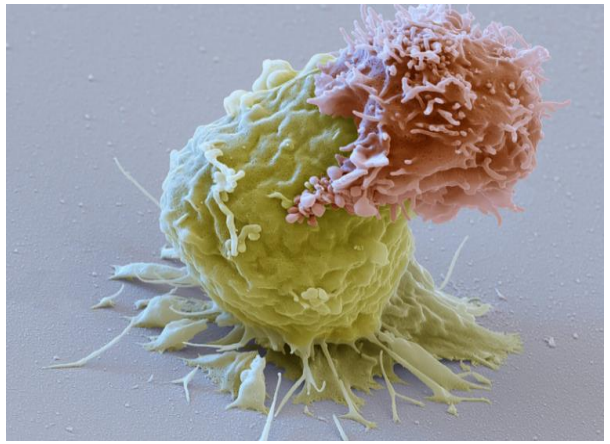
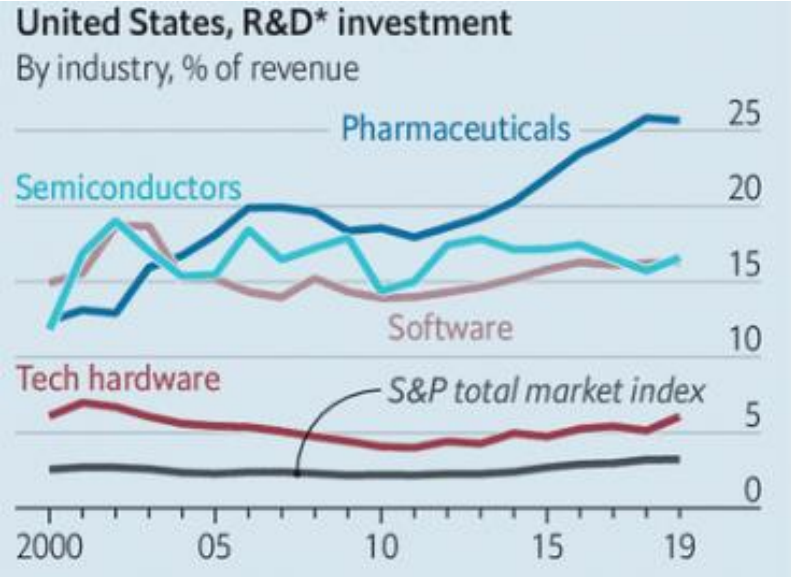


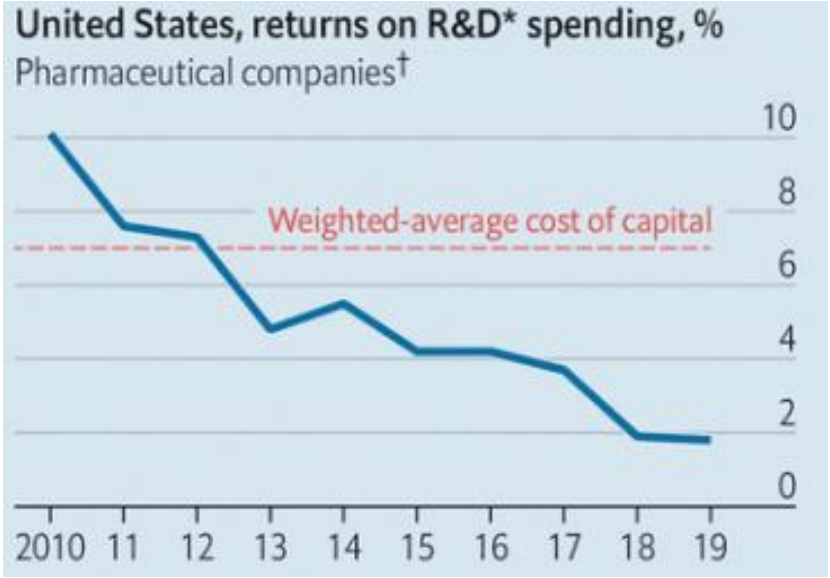
Image of a CAR-T cell (reddish) attacking a leukemia cell (green).

Source: ITIF, "Five Fatal Flaws in Rep. Katie Porter's Indictment of the U.S. Drug Industry"

Companies Aren't Cutting R&D to Boost Shareholder Profits

- In 2018, the R&D intensity of the 8-largest firms was 25%
- In 2016, the top 20 firms accounting for 66.5% of global sales accounted for 64% of R&D investment.

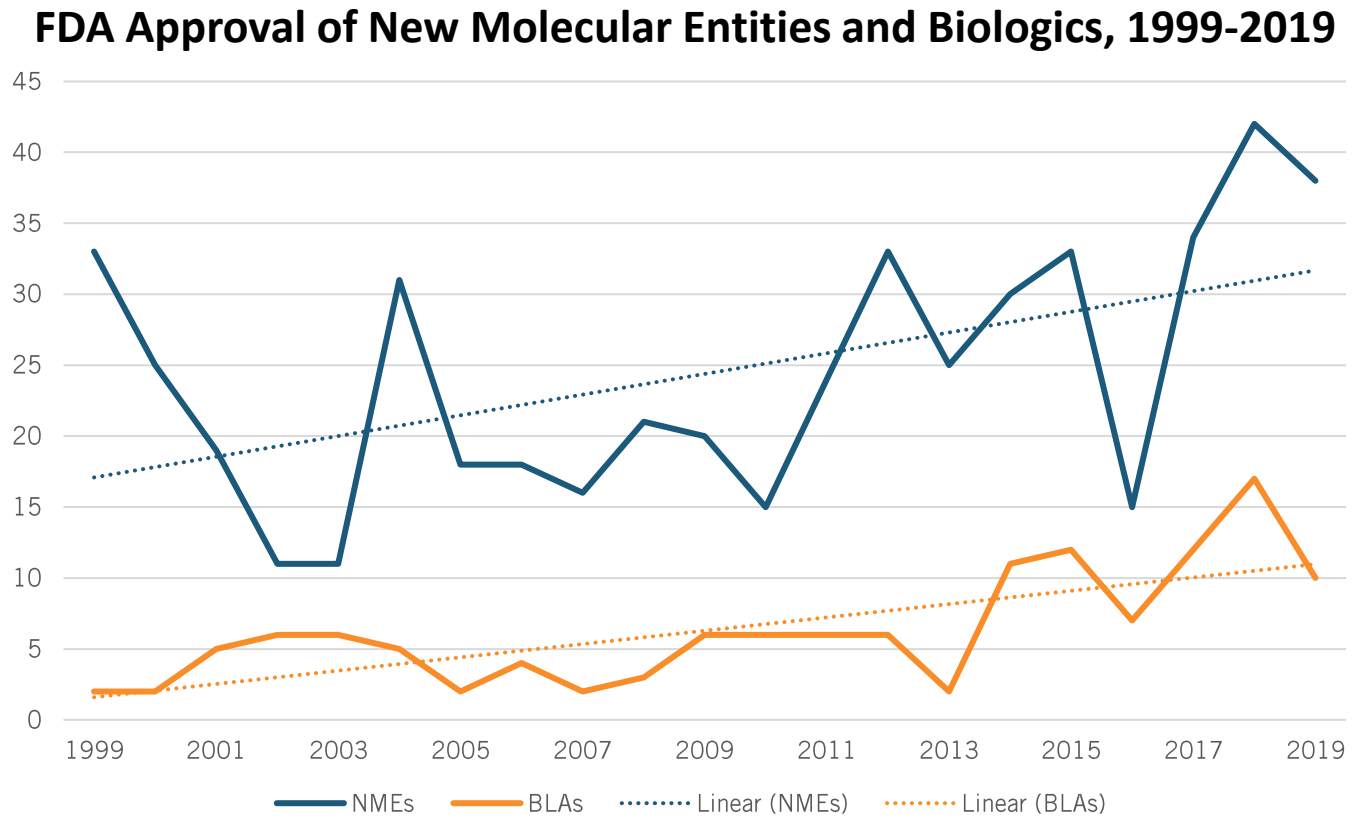




Source: ITIF, "Five Fatal Flaws in Rep. Katie Porter's Indictment of the U.S. Drug Industry"; *The Economist*, "Less Bang for the Buck"

Drug Innovation Hasn't Stalled; It Has Accelerated

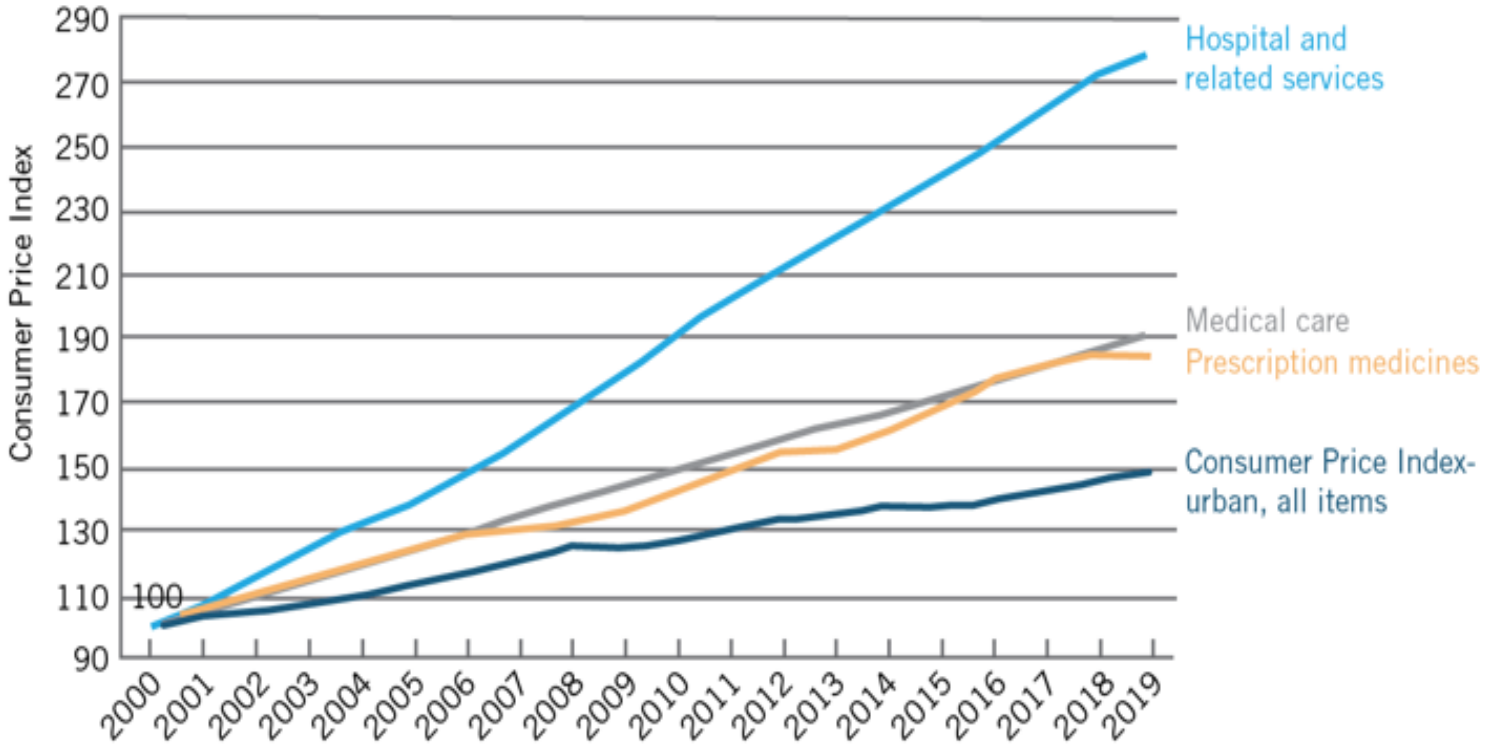
- Number of new drugs approved by the FDA doubled over past decade.



Source: ITIF, "Five Fatal Flaws in Rep. Katie Porter's Indictment of the U.S. Drug Industry"

Drug Prices Aren't Rising Exorbitantly or Disproportionately

Average Price Levels, Select Goods and Services, 2000-2019



U.S. Patented Drug Prices, % Change YoY

Source: ITIF, "Five Fatal Flaws in Rep. Katie Porter's Indictment of the U.S. Drug Industry"; *The Economist*, "Less Bang for the Buck"

Public and Private Sector R&D Investments Complementary

- NIH-funded basic life-sciences research, such as understanding cellular processes identifying novel biomarkers, creates a platform for innovation.
- Each $1 of NIH support for basic research leads to an increase of private medical research of roughly 32 cents.
- Biotechnology companies invest $100 in development for every $1 the government invests in research that leads to an innovation.

Sources: ITIF, "Five Fatal Flaws in Rep. Katie Porter's Indictment of the U.S. Drug Industry";
Dr. Everett Ehrlich, "An Economic Engine: NIH Research, Employment, and the Future of the Medical Innovation Sector"
Sabarni K. Chatterjee and Mark L. Rohrbaugh, "NIH Inventions Translate Into Drugs and Biologics With High Public Health Impact"

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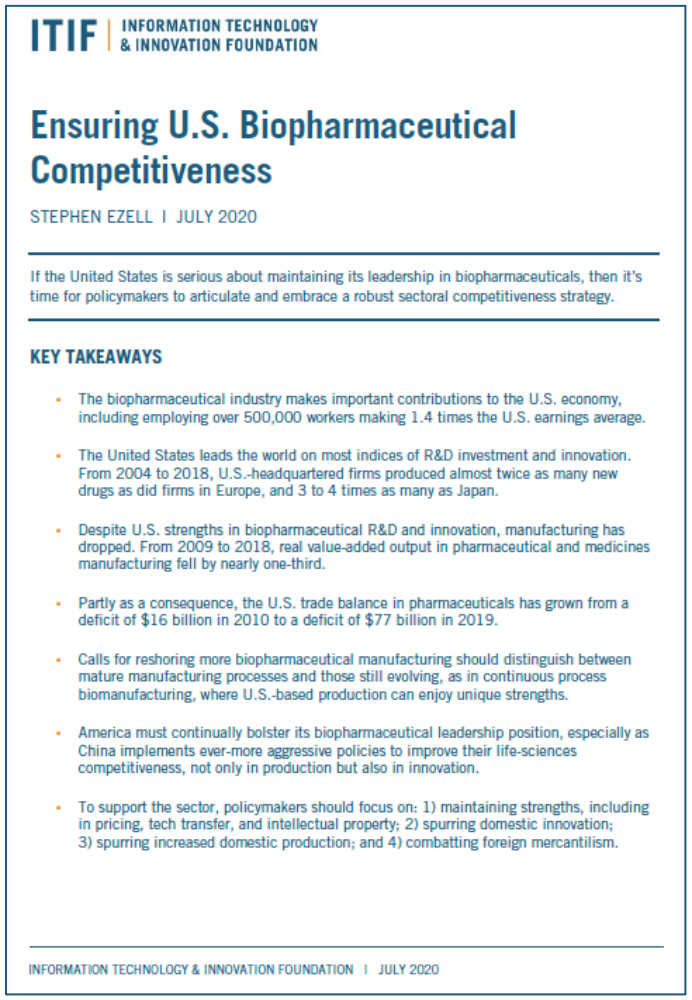
Ensuring U.S. Biopharmaceutical Competitiveness

- Articulate a robust national biopharmaceutical competitiveness strategy.
- Increase NIH funding by at least $12 billion, to at least $50 billion annually.



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Ensuring U.S. Biopharmaceutical Competitiveness

STEPHEN EZELL I JULY 2020

If the United States is serious about maintaining its leadership in biopharmaceuticals, then it's time for policymakers to articulate and embrace a robust sectoral competitiveness strategy.

KEY TAKEAWAYS

- The biopharmaceutical industry makes important contributions to the U.S. economy, including employing over 500,000 workers making 1.4 times the U.S. earnings average.
- The United States leads the world on most indices of R&D investment and innovation. From 2004 to 2018, U.S.-headquartered firms produced almost twice as many new drugs as did firms in Europe, and 3 to 4 times as many as Japan.
- Despite U.S. strengths in biopharmaceutical R&D and innovation, manufacturing has dropped. From 2009 to 2018, real value-added output in pharmaceutical and medicines manufacturing fell by nearly one-third.
- Partly as a consequence, the U.S. trade balance in pharmaceuticals has grown from a deficit of $16 billion in 2010 to a deficit of $77 billion in 2019.
- Calls for reshoring more biopharmaceutical manufacturing should distinguish between mature manufacturing processes and those still evolving, as in continuous process biomanufacturing, where U.S.-based production can enjoy unique strengths.
- America must continually bolster its biopharmaceutical leadership position, especially as China implements ever-more aggressive policies to improve their life-sciences competitiveness, not only in production but also in innovation.
- To support the sector, policymakers should focus on: 1) maintaining strengths, including in pricing, tech transfer, and intellectual property; 2) spurring domestic innovation; 3) spurring increased domestic production; and 4) combatting foreign mercantilism.

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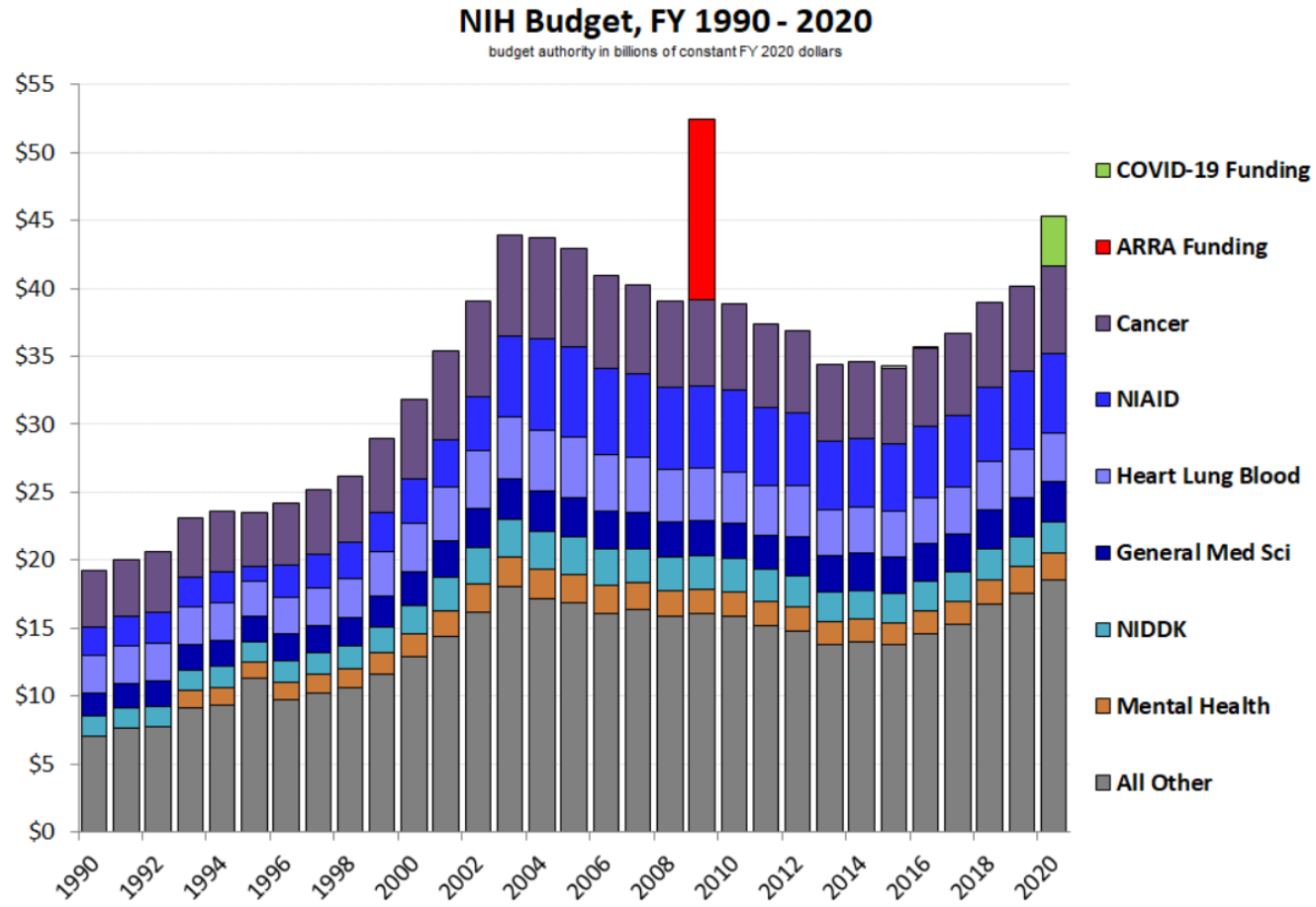
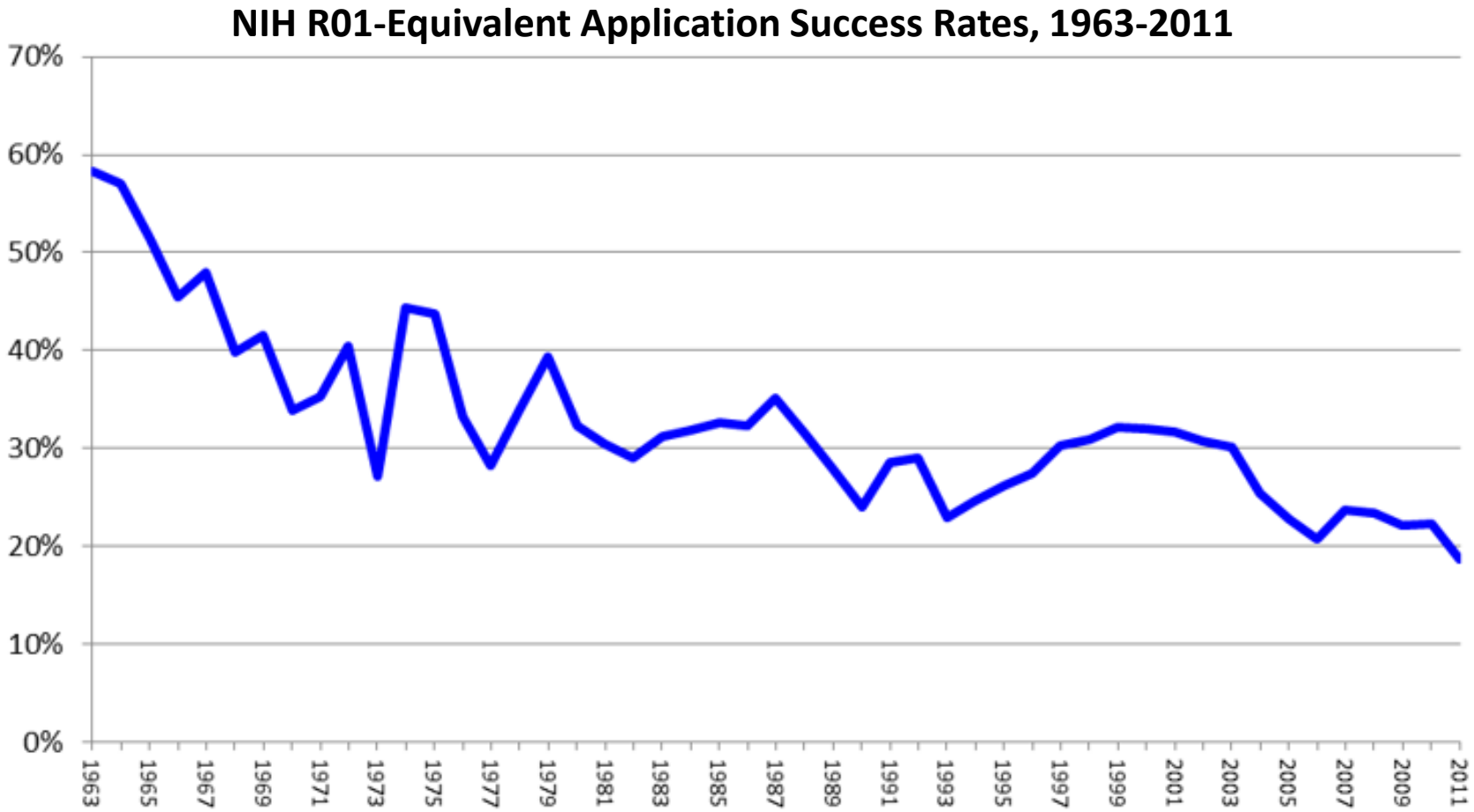
Source: ITIF, "Ensuring U.S. Biopharmaceutical Competitiveness"

NIH Funding Stagnant Since 2002



Source: AAAS, "Historical Trends in Federal R&D," https://www.aaas.org/programs/r-d-budget-and-policy/historical-trends-federal-rd

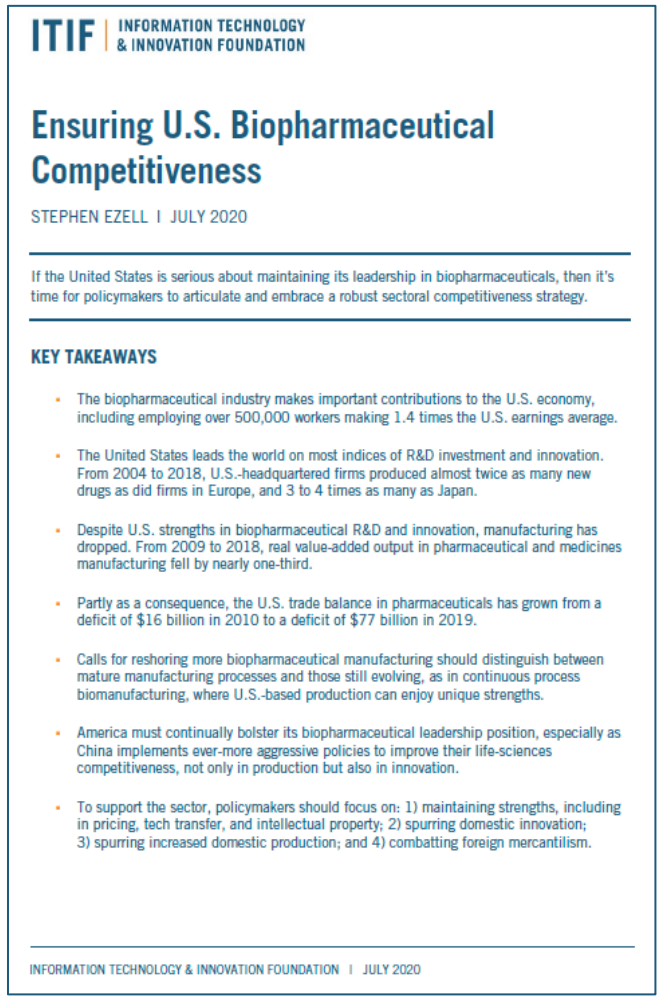
Fewer and Fewer PI Grants Being Funded



Source: ITIF, "Leadership in Decline: Assessing U.S. International Competitiveness in Biomedical Research"

Ensuring U.S. Biopharmaceutical Competitiveness

- Articulate a robust national biopharmaceutical competitiveness strategy.
- Increase NIH funding by at least $12 billion, to at least $50 billion annually.
- Restore the orphan drug tax credit to 50%.
- Refrain from introducing drug price control schemes.
- Refrain from applying Bayh-Dole "march-in" rights regarding drug pricing.



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Source: ITIF, "Ensuring U.S. Biopharmaceutical Competitiveness";

Ensuring U.S. Biopharmaceutical Competitiveness

- Align drug approval/orphan drug designation with CMS's reimbursement procedures.
- Ensure the United States retains a strong environment for conducting clinical trials.
- Invest more in industry-university partnerships focused on biomedical innovation (i.e., I/UCRCs).

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Reforming Regulation to Drive International Competitiveness

BY JOSEPH V. KENNEDY | MARCH 2015

Regulatory reform focused on traded sector industries can substantially reduce costs and boost competitiveness while maintaining, or even increasing, social benefits.

Without faster economic growth, America will be unable to deliver on the implicit promise of high employment and increased living standards that underlies our social contact. Unfortunately, many economists are starting to worry whether the economy has entered a period of secular stagnation. One promising antidote to this problem is regulatory reform. Poor regulation is especially damaging when applied to industries that face international competition. Unlike firms in other industries that face little global competition, these companies are more likely to move their production to jurisdictions where the cost of regulation is lower. Failing that, they may find themselves losing global market share to less burdened rivals in other nations. In either case, the U.S. economy suffers.

INTRODUCTION

Regulatory reform focused on traded sector industries can substantially reduce the costs that agencies impose on these industries and boost their competitiveness while maintaining, or even increasing, the social benefits. This paper analyzes some of the general policy issues associated with regulatory reform. It first looks at the regulatory process to show why it is unreasonable to expect that regulation will always maximize social welfare. In fact, it is not unreasonable to expect that some regulations will become significantly out of date or that the regulatory process imposes significant costs. The paper then looks at case studies in three areas—medical devices, aircraft production, and export controls of high-tech productions—in which regulation affects the competitiveness of specific industries that face

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Conclusion: Balancing the Innovation vs. Cost Dilemma

"I would guess that one can buy today, at rock bottom generic prices, a set of small-molecule drugs that has greater medical utility than the entire set available to anyone, anywhere, at any price in 1995."

Jack Scannell,
Oxford CASMI

"Nearly all the generic medicine chest was created by firms who invested in R&D to win future profits that they tried pretty hard to maximize; short-term financial gain building a long-term common good."

Thank You!

Stephen Ezell | sezell@itif.org | 202.465.2984