Incentivizing Innovation for Rare Disease Treatment Development

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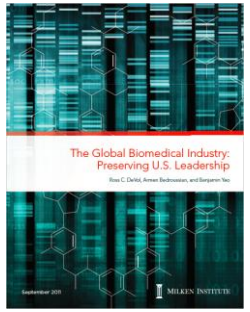
U.S. World Leader in Life-Sciences Innovation

Table 2: New chemical entities
By headquarter country of inventing firm

	1971-1980		1981-1990		1991-2000		2001-2010	
Country	**NCEs**	**% total**	**NCEs**	**% total**	**NCEs**	**% total**	**NCEs**	**% total**
U.S.	157	31	145	32	75	42	111	57
France	98	19	37	8	10	6	11	6
Germany	96	20	67	15	24	13	12	6
Japan	75	15	130	29	16	9	18	9
Switzerland	53	10	48	11	26	14	26	13
U.K.	29	6	29	6	29	16	16	8
Total NCEs	508		456		180		194	



Source: DeVol, Bedroussian, Benjamin Yeo, ***The Global Biomedical Industry: Preserving U.S. Leadership***

The Need for Innovation in Rare Diseases

- In 2015, 47% of novel new drug approvals were for rare diseases.
- Yet still, 95% of rare diseases lack a single FDA-approved treatment.

Source: PhRMA, ***Medicines in Development for Rare Diseases, 2016***

Why America Leads in Life-Sciences Innovation

Bipartisan policy framework with four key pillars:

1. Robust public & private science investment
   - @$30B annually in NIH scientific research.
   - @$70B annually private-sector drug R&D.

2. Incentives to stimulate private-sector research, e.g., Orphan Drug Tax Credit.

3. Robust intellectual property protections.

4. Effective drug approval system (PDUFA)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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, *While Life Sciences Innovation is Politically "Purple"-And How Partisans Get it Wrong*

Why Robust IP Rights Are Vital for Innovation

1. Biopharmaceutical industry America's most R&D-intense.

2. Drug development is risky, uncertain, and expensive. Firms must be able to recoup upfront R&D and development costs.

3. Innovators depend upon the profits from one generation of innovation to finance investment in the next.

4. Generics markets fundamentally depend on the success of upstream innovators.

Key Biopharmaceutical IP Elements

Patents: A property right protecting the underlying molecule, chemical compound or formulation, or biologics mfg. process.

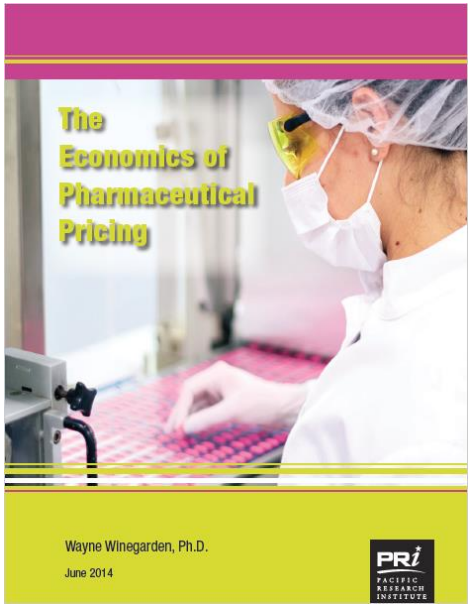
Orphan Drug Exclusivity: Granted to drugs approved to treat rare diseases; precludes FDA from approving any other application for the same drug for same disease for 7 years.

New Chemical Exclusivity (NCE): Provides 5 years marketing exclusivity for most pharmaceutical drugs.

Data Exclusivity: Protects the clinical trial data that validates the safety and efficacy of the biologic drug. During this period, competitors can't use innovator's data, but may submit own.

The Exclusivity Period Matters Greatly

- "Exclusivity rights are key to enabling innovative drug manufacturers to recoup both R&D and production costs."
- "The exclusivity part of the pricing process exists to encourage the process of developing new medicines that address untreated diseases."



Policy Recommendations

1. Continue to support robust federal investment in life-sciences R&D.
2. Expand incentives for orphan drugs research.
    - OPEN Act: Provide six month extended exclusivity for any FDA-drug repurposed for a new rare disease indication.
3. Make the tax code more supportive of high-risk R&D: expand the R&D tax credit & institute an innovation box.

Thank You

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