Incentivizing Innovation for Rare Disease Treatment Development

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VP, Global Innovation Policy

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

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<tr>
<td>Total NCEs</td>
<td>508</td>
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<td>456</td>
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<td>180</td>
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<td>194</td>
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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)

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.

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.

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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