Ensuring Continued U.S. Biopharmaceutical Competitiveness

Alpha-1 Foundation Virtual Annual Conference

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Information Technology and Innovation Foundation (ITIF)

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About ITIF

- The world’s leading science and technology policy think tank.
- Supports policies driving global, innovation-based economic growth.
- Focuses on a host of issues at the intersection of technology innovation and public policy across several sectors:
  - Innovation and competitiveness
  - IT and data
  - Telecommunications
  - Trade and globalization
  - Clean energy, manufacturing, life sciences, and ag biotech
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

<table>
<thead>
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<th>Condition</th>
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<tbody>
<tr>
<td>Cancers</td>
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<tr>
<td>Heart Disease &amp; Stroke</td>
<td>190</td>
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<tr>
<td>Alzheimer's Disease</td>
<td>77</td>
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<tr>
<td>Autoimmune Diseases</td>
<td>311</td>
</tr>
<tr>
<td>Diabetes</td>
<td>171</td>
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<tr>
<td>Mental Health Disorders</td>
<td>135</td>
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<td>Rare Diseases</td>
<td>566</td>
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<tr>
<td>Neurological Disorders</td>
<td>420</td>
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</table>
United States Leads the World In New Drug Development

Over 850 Drug Approvals for Rare Diseases Since 1983

Although more than 850 orphan drugs have been approved since the passage of the Orphan Drug Act in 1983, continued innovation is still very much needed.²³

*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

<table>
<thead>
<tr>
<th>Category</th>
<th>Phase I</th>
<th>Phase II</th>
<th>Phase III</th>
<th>Application Submitted</th>
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<tr>
<td>Blood Cancer</td>
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<tr>
<td>Blood Disorders</td>
<td>12</td>
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<tr>
<td>Cancer</td>
<td>10</td>
<td>10</td>
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<td>Cardiovascular Disease</td>
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<td>Eye Disorders</td>
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<tr>
<td>Genetic Disorders</td>
<td>7</td>
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<td>31</td>
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<tr>
<td>Growth Disorders</td>
<td>11</td>
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<tr>
<td>Infectious Diseases</td>
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<td>Neurologic Disorders</td>
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<td>Respiratory Diseases</td>
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<tr>
<td>Transplantation</td>
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<tr>
<td>Other</td>
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<td></td>
<td></td>
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</tr>
</tbody>
</table>

Note: Some medicines may be in more than one category.

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

# United States Leads the World in New Drugs Developed

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

<table>
<thead>
<tr>
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<td>U.S.</td>
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<td>Japan</td>
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<td>16</td>
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<td>Other</td>
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<td>14</td>
<td>23</td>
<td>41</td>
<td>64</td>
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</tbody>
</table>

<table>
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<th></th>
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</thead>
<tbody>
<tr>
<td>Europe</td>
<td>1.53</td>
<td>0.70</td>
<td>0.88</td>
<td>0.91</td>
<td>0.90</td>
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<tr>
<td>U.S.</td>
<td>1.38</td>
<td>0.98</td>
<td>0.82</td>
<td>1.32</td>
<td>1.10</td>
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<tr>
<td>Japan</td>
<td>1.25</td>
<td>0.68</td>
<td>0.91</td>
<td>1.42</td>
<td>0.95</td>
</tr>
<tr>
<td>Other</td>
<td>0.14</td>
<td>0.13</td>
<td>0.13</td>
<td>0.20</td>
<td>0.17</td>
</tr>
</tbody>
</table>

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.

Source: ITIF, “Why Life-Sciences Innovation is “Politically Purple”—And How Partisans Get It Wrong”
United States Leads World in Biopharmaceutical R&D

Business and Government Investment in Pharmaceutical R&D (in Billions), 2018

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)

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.

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.

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%.

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.

Drug Innovation Hasn’t Stalled; It Has Accelerated

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

Drug Prices Aren’t Rising Exorbitantly or Disproportionately

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

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.

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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.

Source: ITIF, “Ensuring U.S. Biopharmaceutical Competitiveness”
NIH Funding Stagnant Since 2002

Fewer and Fewer PI Grants Being Funded

NIH R01-Equivalent Application Success Rates, 1963-2011

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.

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).
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.”

“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.”

Jack Scannell, Oxford CASMI
Thank You!

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