Advances in Biotech Innovation

AMIIF – Semana de Innovacion
Mexico City
March 30, 2017
What is BIO

- World’s Largest Biotechnology Organization
  - About 1000 members, from start ups to large MNCs
  - **Most** are small companies
  - Members from 35 countries
  - Host BIO International Convention annually – c. 16,000 participants. June 19-22, San Diego

- Covers all three sectors or biotech: Biopharma, Agriculture, industrial/environmental. Common elements:
  - All three use same technologies
  - All involved in addressing some of the most important issues confronting mankind
Serve as Voice of Global Innovative Biotech Sector

Define and advance policies best practices that promote global biotech innovation (regulatory, IP, financial)

- Promote partnerships among parties engaged in biotech research and commercialization (the biotech “ecosystem”)
Impact: Innovation Saves Lives

www.bio.org/innovation

5-year survival rates increases

Survival is increasing dramatically for many forms of cancer: since 1975, 5-year survival rates went up 21% for breast cancer; 50% for prostate cancer; 36% for colon cancer; 54% for lung cancer.¹

- Breast cancer: 21%
- Prostate cancer: 50%
- Colon cancer: 36%
- Lung cancer: 54%

Innovative medicines save millions of lives every year and are transforming how we treat and cure disease.

- HIV/AIDS death rates have decreased 85% ... since 1995
- Heart disease death rates have decreased 30% ... from 2001 to 2011
- Cancer death rates have decreased 22% ... since 1991
In the United States, the Congressional Budget Office (CBO) credits each $1 of additional spending on medicines with $.20 reduction in other healthcare expenses.

**Economic Benefits of Innovation**

- Gains in cancer survival are worth nearly $2 trillion, with a majority of that savings going to patients, families and our economy as a whole.\(^5\)
### Biotech on the Horizon: What’s in the Pipeline?

<table>
<thead>
<tr>
<th>Selected Diseases</th>
<th>Medicines in Development*</th>
</tr>
</thead>
<tbody>
<tr>
<td>Cancers</td>
<td>1,813</td>
</tr>
<tr>
<td>Cardiovascular disorders</td>
<td>599</td>
</tr>
<tr>
<td>Diabetes</td>
<td>475</td>
</tr>
<tr>
<td>HIV/AIDS</td>
<td>159</td>
</tr>
<tr>
<td>Immunological disorders</td>
<td>1,120</td>
</tr>
<tr>
<td>Infectious diseases</td>
<td>1,256</td>
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<tr>
<td>Mental health disorders</td>
<td>511</td>
</tr>
<tr>
<td>Neurological disorders</td>
<td>1,329</td>
</tr>
</tbody>
</table>

New Drug Approvals
(US FDA/CDER)

Source: FDA.gov, EvaluatePharma, BIO Industry Analysis, January 2016

Small Molecule

Biologic

CDER BLA Approvals

Global R&D Spending ($B)

$30B NIH

January 2016
Clinical Phase Success Rates

- Phase I: 63%
- Phase II: 31%
- Phase III: 58%
- NDA/BLA: 85%

All Diseases, All Modalities
Phase Success Rates & Likelihood of Approval from Phase I

- Phase I: 63%
- Phase II: 31%
- Phase III: 58%
- NDA/BLA: 85%
- Phase I to Approval: 9.6%
Likelihood of Approval from Phase I By Disease

*includes IBS, but not IBD (in autoimmune)
Success by Type of Product

Likelihood of Approval from Phase I

- Biomarker Programs: 25.9%
- Rare Disease: 25.3%
- Biologics: 11.5%
- All Diseases: 9.6%
- Chronic, High Prevalence: 8.7%
- NME, Small Molecule: 6.2%
Global BioPharma Pipeline
- 5,393 clinical programs
- 70% from small companies

38% Partnered

3706

2318 Unpartnered

1402 Partnered

Small Company

1673

Large Company

Source: BIO Industry Analysis, BioMedTracker, June 2016
Financing Innovation

- Angels, Incubators
- Family Offices
- Patient Groups / Foundations/
- Gov SBIR/BARDA
- Venture Capital/Corporate VC
- R&D Collaborations with Pharma/Biotech
- IPOs
- Follow-On Offerings

Preclinical | Phase I | Phase II | Phase III | Market
---|---|---|---|---

$1.1 B** | $3.7 B | $3.5 B | $1.0 B | $2.7 B
US Investment into Biotech 2006-2015

Source: Factset, BIO Industry Analysis, October 2016
New Company Formation by Country

![Bar chart showing the number of startups by country in 2014. The chart indicates that the US had 61 A rounds, followed by the UK with 14.](image)

**Figure 1** Number of startups by country in 2014. Source: BCIQ: BioCentury Online Intelligence.

*Source: Nature Biotechnology, Vol 33, No 1, Jan 2015*
Cost of Innovation

Cost of research and development

The average amount spent to bring a new prescription drug to market more than doubled since it was last estimated 11 years ago.

NOTE: All figures are inflation adjusted to 2013 dollars.

SOURCE: Tufts Center for the Study of Drug Development  DAVID BUTLER/GLOBE STAFF
Clinical Trials

1–2 years

Direct Cost: ~$1-2 B Capitalized
Cost: $2.6 B*

FDA Approval

1 FDA Review

Ph III

6-9 years

Ph II

2

Ph I

6

3-5 years

Discovery

1000s molecules

In vitro testing

100

In vivo testing

10

Hypothesis

Cost of Innovation

*Tufts 2014, BIO Industry Analysis, January 2016
Vast majority of the companies working on biotech innovations are small, pre-revenue enterprises that do not earn a profit.

Only 2 of every 10 drugs on the market ever earn back enough money to match the costs of R&D and regulatory approval process before their patent expires.
Can drug development be done more efficiently, i.e., faster, less costly and higher success rates?
- Will require researchers, industry and governments working together to solve

How will societies ensure that the incentives for innovation are sufficient? Can societies take a longer term view (the business model is long term) of the benefits

Can the “start-up culture” – of spreading risk among many small developers – be replicated globally
- Indeed, can the ecosystem of innovation become more globalized – what policies will it take to advance that goal
Thank you

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