Drugs, medical progress, and the road ahead

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The Pharmaceutical Industry’s Market

Global Pharmaceutical Sales, by Region (in billions $)

- Largest commercial health sector
- Global industry, consolidated players
- Highly regulated industry (FDA, Patent and Trademark Office)

IMS Health Inc.
Global Biopharmaceutical Intellectual Property

- The intellectual property related to more than half of new medicines resides in the U.S.

  - U.S. Biopharmaceutical Patents 1990–2002, by Location of Inventors

![Pie chart showing the distribution of U.S. biopharmaceutical patents by location of inventors.]

Source: J.T. Macher and D.C. Mowrey

Global Market: Development of Medicines by the Geographic Region

- Number of Compounds in Development, by Geographic Region, 1997–2011

Different countries also have different health priorities – dependent on demographics and disease prevalence

![Line chart showing the number of compounds in development by geographic region from 1997 to 2011.]

Source: Adis R&D Insight Database
### The Terminology of Drug Development

#### Clinical Trials

<table>
<thead>
<tr>
<th></th>
<th>Discovery/ Preclinical Testing</th>
<th>Phase I</th>
<th>Phase II</th>
<th>Phase III</th>
<th>FDA</th>
<th>Phase IV</th>
</tr>
</thead>
<tbody>
<tr>
<td>Years</td>
<td>6.5</td>
<td>1.5</td>
<td>2</td>
<td>3.5</td>
<td>1.5</td>
<td>Review approval</td>
</tr>
<tr>
<td>Test Population</td>
<td>Laboratory and animal studies</td>
<td>20 to 100 healthy volunteers</td>
<td>100 to 500 patient volunteers</td>
<td>1,000 to 5,000 patient volunteers</td>
<td>File IND at FDA</td>
<td>Additional post-marketing testing required by FDA</td>
</tr>
<tr>
<td>Purpose</td>
<td>Assess safety, biological activity and formulations</td>
<td>Determine safety and dosage</td>
<td>Evaluate effectiveness, look for side effects</td>
<td>Confirm effectiveness, monitor adverse reactions from long-term use</td>
<td>File NDA at FDA</td>
<td></td>
</tr>
<tr>
<td>Success Rate</td>
<td>5,000 compounds evaluated</td>
<td>5 enter trials</td>
<td></td>
<td></td>
<td>1 approved</td>
<td></td>
</tr>
</tbody>
</table>

Source: PhRMA

### The R&D Process Takes Time and Resources

![Diagram of the R&D Process](image)

The biopharmaceutical sector is the most R&D-intensive in the US

- Biopharmaceutical companies invested more than ten times the amount of R&D per employee than manufacturing industries overall.

![](chart)


PhRMA Member Company and Public R&D Spending

PhRMA Member Company R&D and NIH Operating Budget: 1995–2011

![](chart)

*Estimated for CY 2011.
Global Leaders in Biotechnology Research

- U.S. biotechnology firms account for 80% of the world’s research

<table>
<thead>
<tr>
<th>2008 Biotechnology Statistics*</th>
</tr>
</thead>
<tbody>
<tr>
<td>USA</td>
</tr>
<tr>
<td>Annual R&amp;D</td>
</tr>
<tr>
<td>Total Companies</td>
</tr>
<tr>
<td>Total Employees</td>
</tr>
<tr>
<td>Publicly Held Corporations</td>
</tr>
</tbody>
</table>

* Biotechnology companies are defined as those whose primary activity is to use biological processes to develop health care products, and other companies whose primary activity is to supply health biotechnology companies with technology-based research products.

Source: Burrill and Company

Biotechnology: Who’s providing the money?

- Biotechnology Financing (in millions $)
  - Venture Funding: $6.23B, 25%
  - Public Offerings: $5.725B, 21%
  - Other Financings of Public Companies: $13.419B, 54%

Total = $24,773.8 Million

Biopharmaceuticals are a Source of Projected Growth in US Manufacturing Jobs

Projected Change in Employment from 2006 to 2018*

<table>
<thead>
<tr>
<th>Sector</th>
<th>Employment Losses</th>
<th>Employment Gains</th>
</tr>
</thead>
<tbody>
<tr>
<td>Chemical</td>
<td>-58</td>
<td></td>
</tr>
<tr>
<td>Computer Equipment</td>
<td>-58</td>
<td></td>
</tr>
<tr>
<td>Maintenance</td>
<td>-54</td>
<td></td>
</tr>
<tr>
<td>Motor Vehicle</td>
<td>-31</td>
<td></td>
</tr>
<tr>
<td>Aerospace</td>
<td>-15</td>
<td></td>
</tr>
<tr>
<td>Biopharmaceuticals: 18</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Concrete/Cement Product: 24</td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

Change in Manufacturing Jobs (in Thousands)

* Selected illustrative sectors. The government projects increases in manufacturing employment in only one fifth of the sectors or subsectors it defines.

Source: PhRMA, adapted from Bureau of Labor Statistics

The Ripple Effect of High-Value Biopharmaceutical Jobs

- The biopharmaceutical sector supported 4 million jobs across the economy in 2009, including about 3.3 million in other sectors.

Each direct biopharmaceutical job supports 5 additional jobs in other sectors

Biopharma Jobs
More than 650,000 Jobs in the U.S. Biopharmaceutical Sector

Total Jobs Supported
4 million total U.S. Jobs Supported by the Biopharmaceutical Sector

Source: Battelle Technology Partnership Practice
In the US the greatest proportion of biopharmaceuticals in development are oncology-related

Biopharmaceuticals in Development by Therapeutic Category, 2009

- Cancer Related: 932
- Rare Diseases: 460
- Arthritis and Related: 198
- HIV/AIDS Related: 88
- Respiratory Disorders: 383
- Cardiovascular Disorders: 245
- Mental and Behavioral: 250
- Diabetes Mellitus: 200
- Alzheimer’s and Other: 98
- Parkinson’s and Related: 36

- Other

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While companies have spent more on research, this has not led to more drug approvals

Research and Development Spending, and New Drug Approvals (NDAs) and New Biologic License Applications (BLAs) by the U.S. FDA, 1998-2008

- Drug Approvals
- Domestic R&D Spending

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Biologics are now a major component of the originator pipeline, but approvals lag behind investments

![Biologic Drug Approvals Per Year (NDA and BLA)](chart1)

![Biologic Drugs in Development (Cumulative)](chart2)

**As drugs in development move through the pipeline, the expansion of the biologic drug market is expected to dramatically increase the total costs of biologics**


Generic drugs took a while to take off after the enactment of Hatch Waxman in 1984, but now market penetration is rapid

**But by 2008, generics accounted for 14/15 most commonly prescribed meds, and 13/15 of those most commonly used by Medicare Part D beneficiaries (seniors pharmacy benefits)**

![Generic Share of Prescriptions Filled 1984–2009](chart3)

Source: IMS

Source: PhRMA

http://www.phrma.org/sites/default/files/159/phrma_chart_pack.pdf
Generic Small Molecule Drugs have Created Savings When Patents Expire

- Generic medicines account for 69% of all prescriptions dispensed in the United States, and only 16% of all dollars spent on prescriptions. (source: IMS Health)
- Brand sales of $228 billion compared to Generic sales of $58.5 billion (2007), and have saved consumers and the American health care system $931 billion over the last 10 years.
- In 2010, generics saved $158 billion — a savings of more than $3 billion every week.
- 10,072 of the 12,751 drugs listed in the FDA's Orange Book have generic counterparts (source: FDA, MedAd News).

Similar hopes and expectations are being applied to biologics, which, through the chance of history, did not have an explicit regulatory pathway in the US until 23 March 2010

Will Biosimilars Be the New Generics in the US Market?

- Congress included Title VII, Biologics Price Competition and Innovation Act (BPCIA)* in the Patient Protection and Affordable Care Act because of the expectations for savings, as well as the hope for expanded access.
  - Using the new 351(k) pathway, applications can be for biosimilars and also for interchangeable biosimilars
- Legislation was enacted 23Mar2010, and the new biosimilars pathway was available the day of enactment
- While FDA has held ~21 PreIND meetings (~35 meeting requests for 11 reference products) there have as yet been no applications filed for biosimilars in the US
- Draft guidances were published mid-Feb12, another FDA Public Meeting 11May12, transparent and on-going discussions; user fees negotiated and proposed (BsUFA) in parallel
- Payors/reimbursers are waiting…

Concluding Questions

- Can we sustain the pace of investment?
- Can we lower the cost of development?
- Will we settle for having to erode benefits in order to broaden coverage to more people?
- Will mounting budget woes and the cost of services erode investment in innovation?