Drugs, medical progress, and the road ahead

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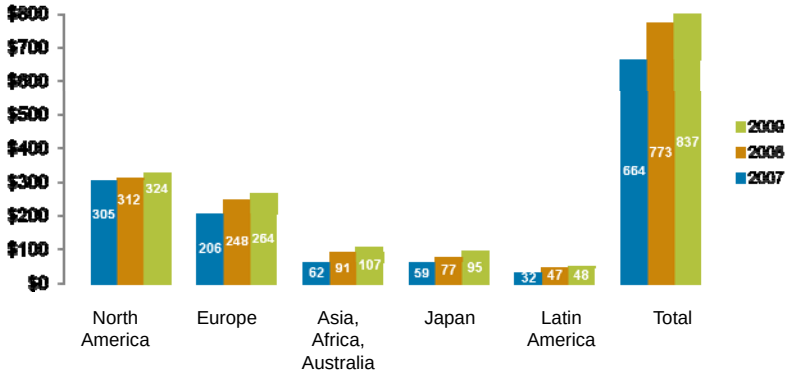
The Pharmaceutical Industry's Market

Global Pharmaceutical Sales, by Region (in billions $)



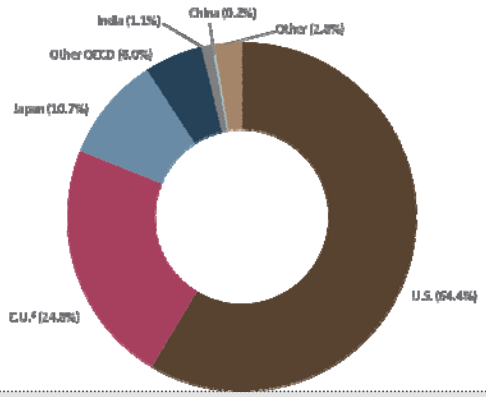
- Largest commercial health sector
- Global industry, consolidated players
- Highly regulated industry (FDA, Patent and Trademark Office)

Source: Standard & Poor's. Industry Surveys Healthcare: Pharmaceutics. November 25, 2010.
IMS Health Inc.

Global Biopharmaceutical Intellectual Property

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

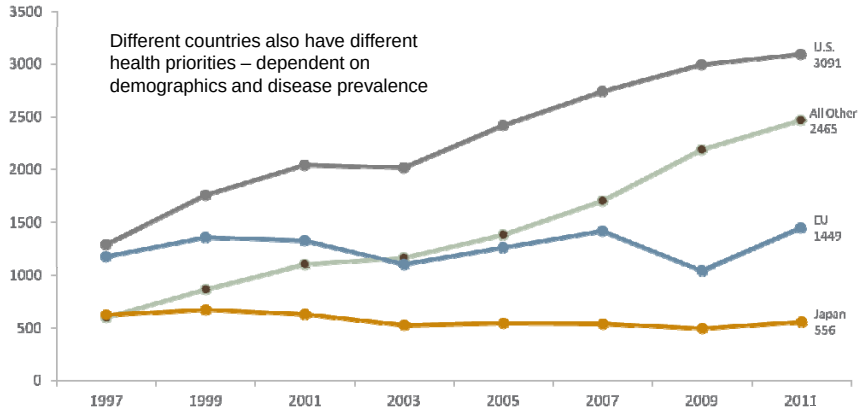
- *U.S. Biopharmaceutical Patents 1990–2002, by Location of Inventors*



Source: J.T. Macher and D.C. Mowrey[5]

Global Market: Development of Medicines by the Geographic Region

- *Number of Compounds in Development, by Geographic Region[4], 1997–2011*



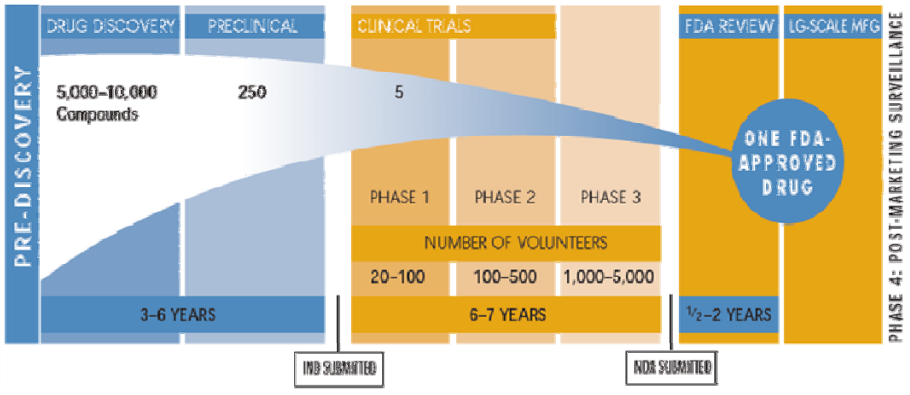
Source: Adis R&D Insight Database[5]

The Terminology of Drug Development

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

Source: PhRMA

The R&D Process Takes Time and Resources

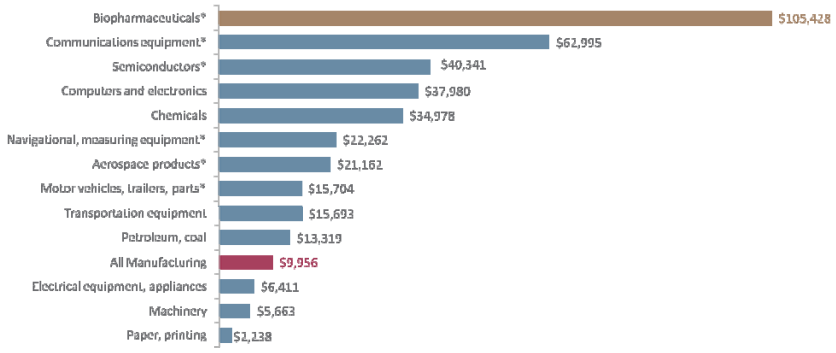


Source: Pharmaceutical Research and Manufacturers of America, Drug Discovery and Development: Understanding the R&D Process. http://www.phrma.org/research-development-process

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.

  - *R&D Expenditures per Employee, by Manufacturing Sub-sector and Industry, 2000–2007*

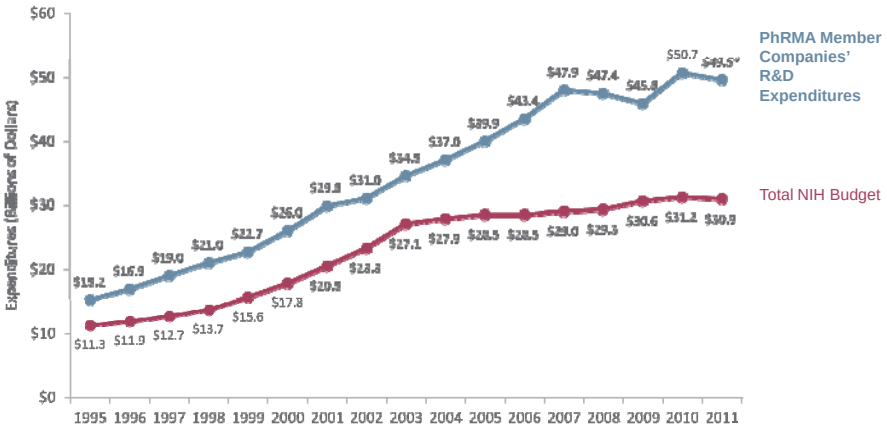


* Asterisks indicate manufacturing subsectors.

Source: N.D. Pham[1]

PhRMA Member Company and Public R&D Spending

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



*Estimated for CY 2011.

Source: PhRMA, NIH Office of Budget[10]

Global Leaders in Biotechnology Research

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

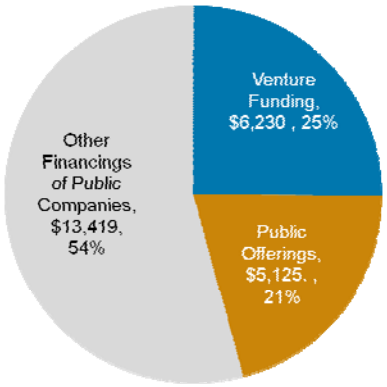
2008 Biotechnology Statistics*					
	USA	Europe	Asia/Pacific	Canada	**Total**
Annual R&D	$24B	$5B	$0.6B	$0.9B	**$30B**
Total Companies	1,450	1,600	760	400	**4,210**
Total Employees	140,000	65,000	15,000	6,000	**226,000**
Publicly Held Corporations	336	150	160	67	**693**

* 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[6]

Biotechnology: Who's providing the money?

Biotechnology Financing (in millions $)

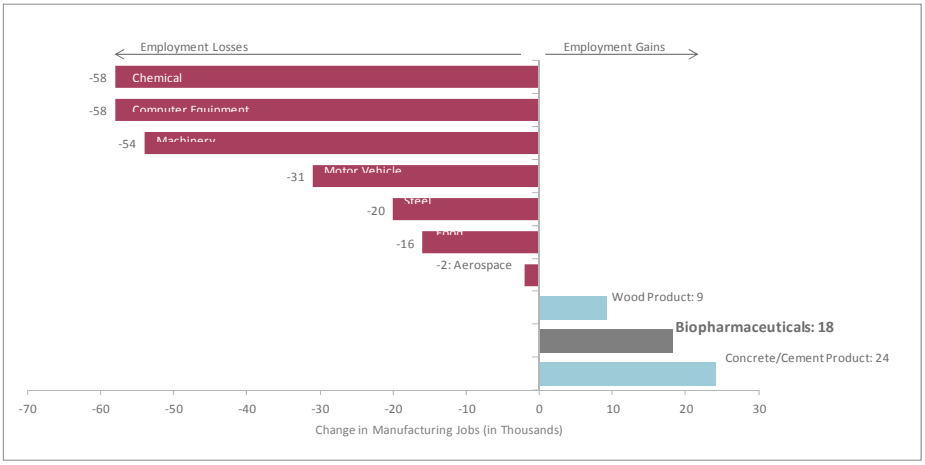


Total = $24,773.8 Million

Source: Biotechnology Industry Organization, Guide to Biotechnology 2008

Biopharmaceuticals are a Source of Projected Growth in US Manufacturing Jobs

*Projected Change in Employment from 2006 to 2018**

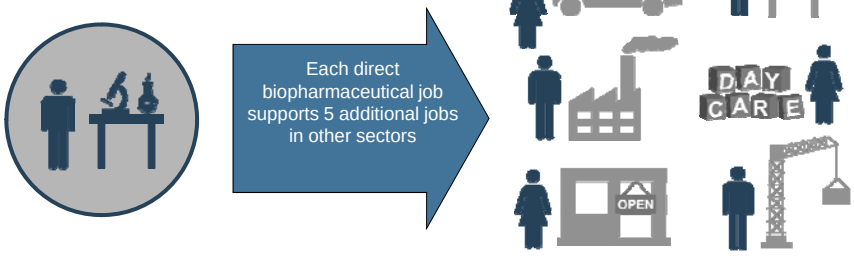


* Selected illustrative sectors. The government projects increases in manufacturing employment in only one fifth of the sectors or subsectors it defines.

Source: PhRMA, adapted from Bureau of Labor Statistics[3]

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.



Biopharma Jobs

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

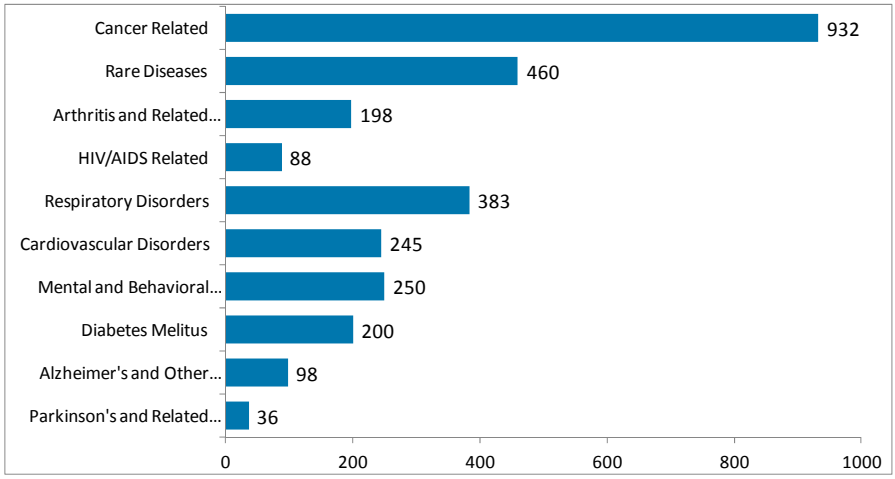
Total Jobs Supported

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

Source: Battelle Technology Partnership Practice[2]

In the US the greatest proportion of biopharmaceuticals in development are oncology-related

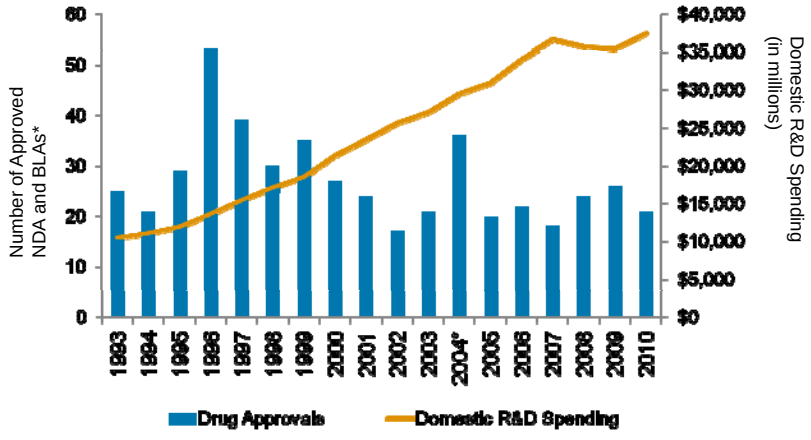
Biopharmaceuticals in Development by Therapeutic Category, 2009



*Reflects number of compounds in clinical trials or under review by the FDA for approval through New Drug Application (NDA) or Biologic License Application (BLA) pathways. Medicines with multiple indications may appear in more than one category but are counted only once for total (3,091).
Source: PhRMA

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

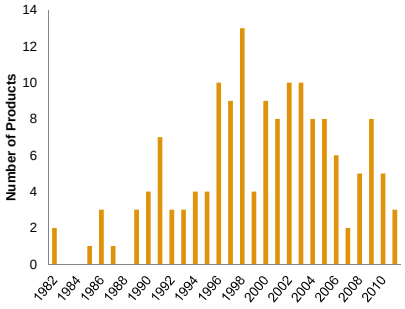
Research and Development Spending, and New Drug Approvals (NDAs) and New Biologic License Applications (BLAs) by the U.S. FDA, 1998-2008



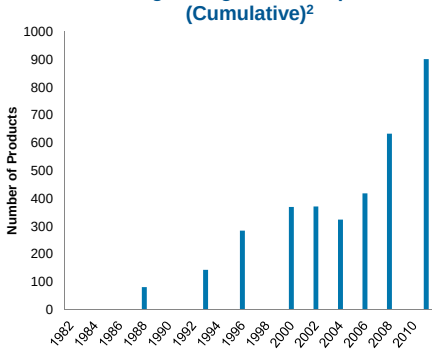
*Beginning in 2004, figures include BLAs.
Sources: PhRMA, Profile 2011, Pharmauetical Industry. http://www.phrma.org/sites/default/files/159/phrma_profile_2011_final.pdf
FDA, CDER Approval Times for Priority and Standard NMEs and New BLAs CY 1993 – 2008.
FDA, NMEs Approved by CDER.

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

Biologic Drug Approvals Per Year (NDA and BLA)[1]



Biologic Drugs in Development (Cumulative)[2]

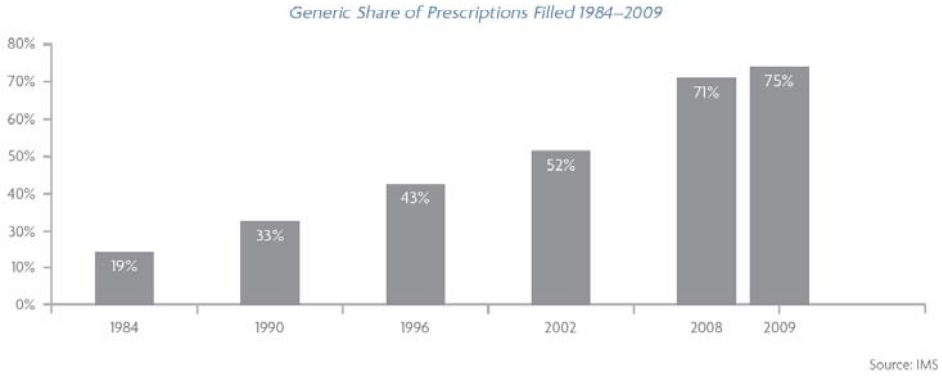


As drugs in development move through the pipeline, the expansion of the biologic drug market is expected to dramatically increase the total costs of biologics

1. FDA. "Drug and Biologic Approval Reports." http://www.fda.gov/Drugs/DevelopmentApprovalProcess/HowDrugsareDevelopedandApproved/DrugandBiologicApprovalReports/default.htm
2. PhARMA. "Biotechnology Research Promises to Bolster the Future of Medicine with More Than 900 Medicines and Vaccines in Development." 2011.

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)



http://www.phrma.org/sites/default/files/159/phrma_chart_pack.pdf

Source: PhRMA

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

Source include http://www.gphaonline.org/about-gpha/about-generics/facts

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…

* http://www.fda.gov/downloads/Drugs/GuidanceComplianceRegulatoryInformation/UCM216146.pdf

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?