



## Key Topics

- Macro View of Healthcare Landscape
- Macro View of Biotechnology Sector Organization
- Things That Have Worked Well
- Challenges & Barriers

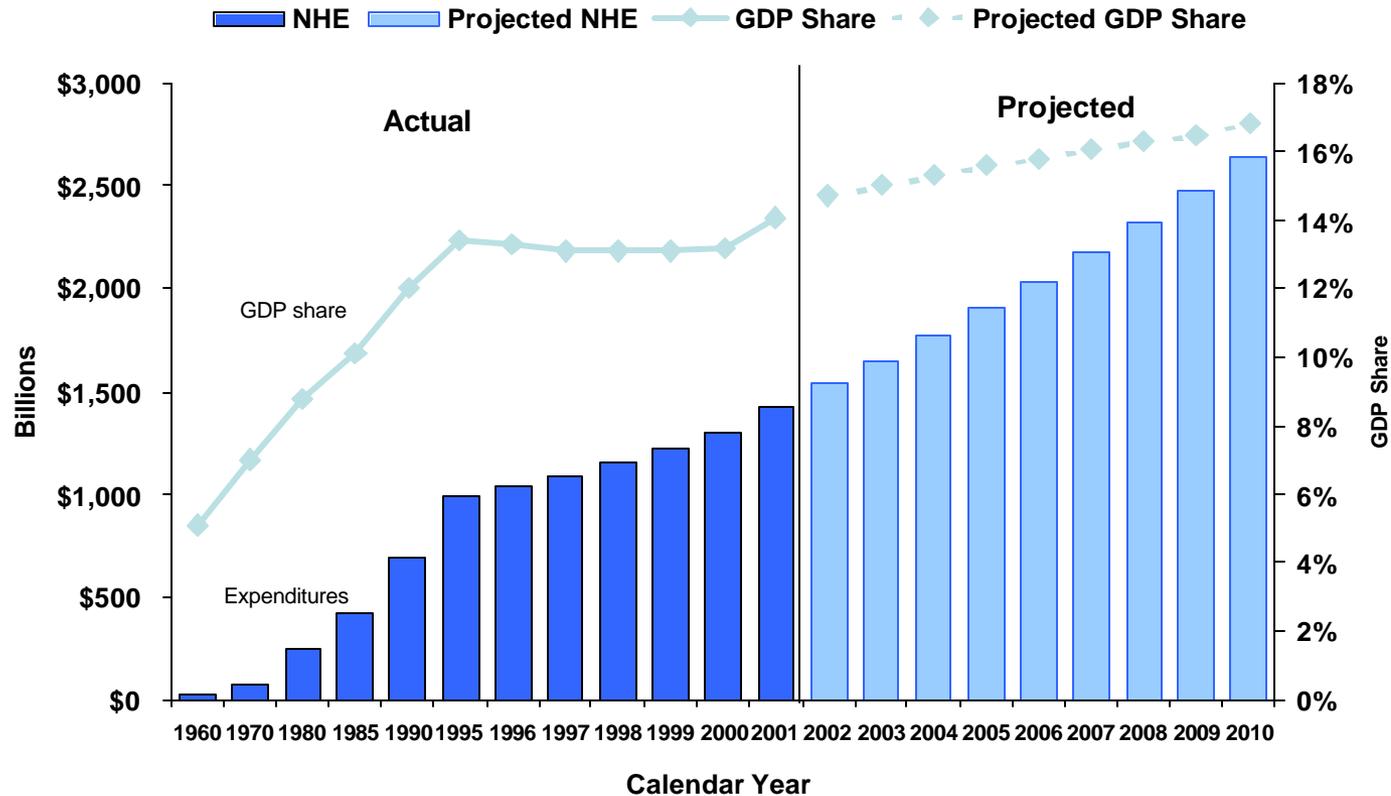


# The Macro View

*Important Trends in Healthcare*

# National Healthcare Expenditures Increasing

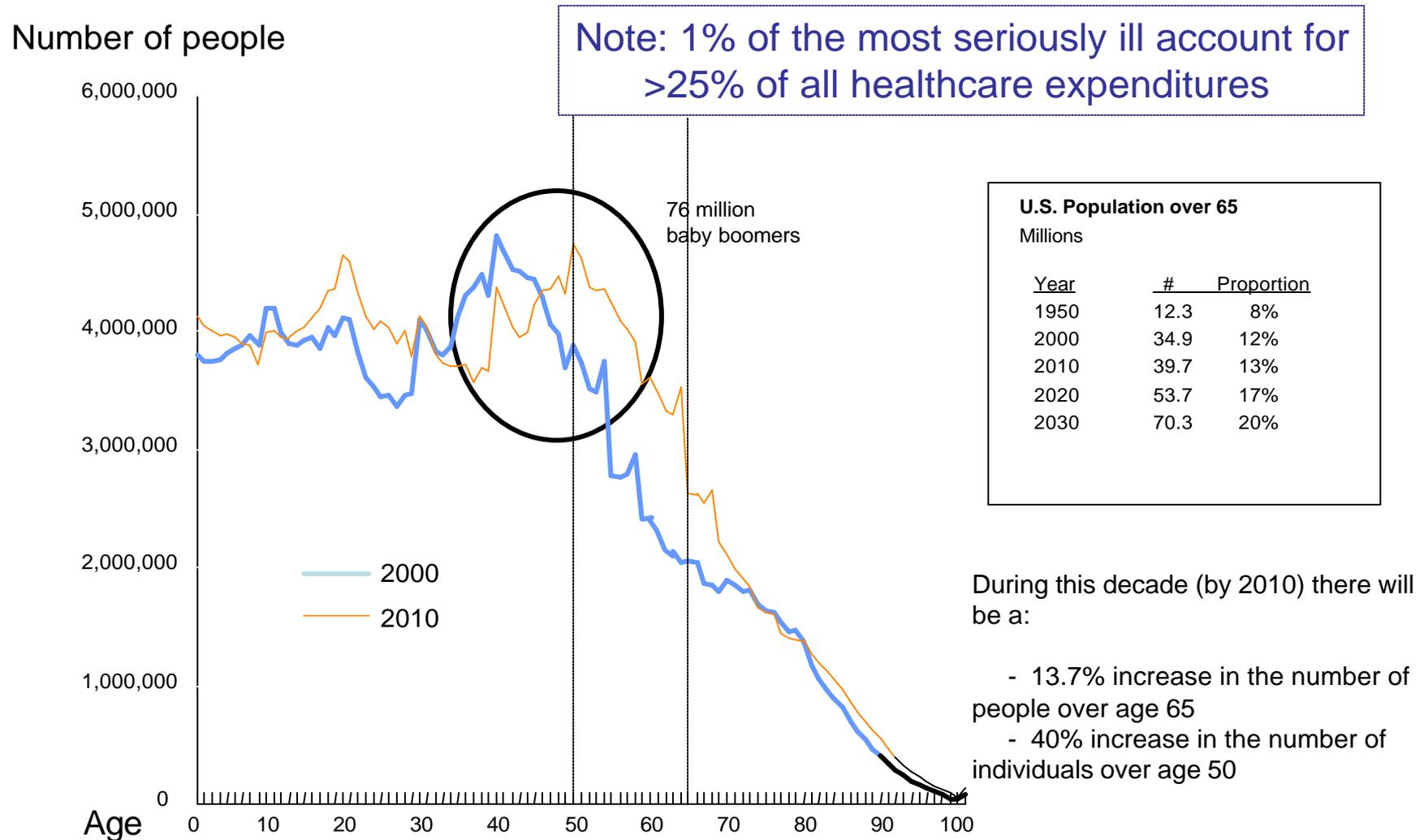
Aggregate National Health Expenditures, Share of GDP  
1960-2010



Source: CMS, Office of the Actuary, National Health Statistics Group; BioEnterprise

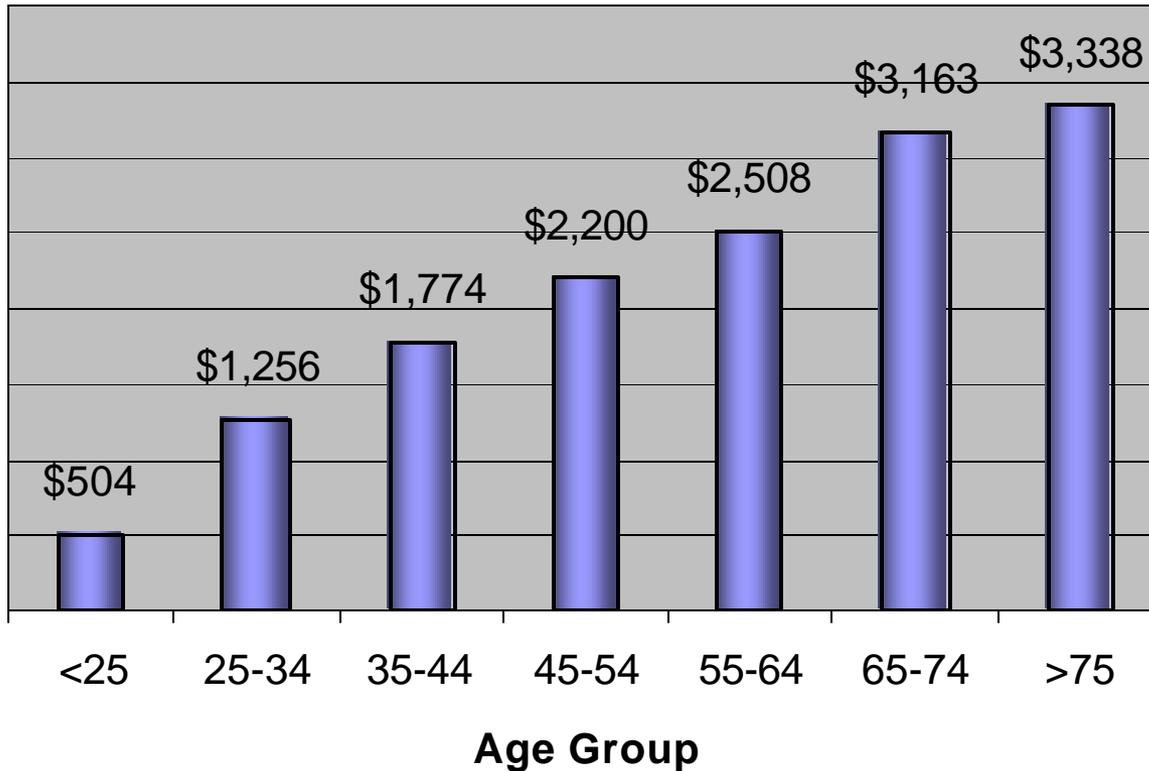
# Shifting Population Demographics are a Major Factor

*Largest "At risk" population will substantially increase in the coming decades*



# Health Care Expenditures are Significantly Greater for Older Americans

Average Annual U.S. Per Capita Healthcare Expenditures by Age – 1985-2000



## Key drivers in cost increases

- Increased incidence, impact of chronic disease
  - Heart disease
  - Cancer
  - Stroke
  - Pulmonary disease
  - Diabetes
  - Osteoporosis
- Significant burden associated with seriously ill (1% of most seriously ill account for >25% total healthcare expenditures)

Source: 2002 Statistical Abstract, Bureau of Labor Statistics; BioEnterprise



# Biotechnology Sector Organization (and some key macro trends)

# Snapshot of the Biotechnology Industry



- Since 1980...
  - Industry has grown from inception to roughly 1,500 biotech companies
  - Dozens of new medicines successfully developed to treat human disease
  - Nearly 400 additional candidate medicines in clinical trials
- The impact is more than just dollars and sense
  - Improvement to quality of life enormous, but hard to quantify
  - Improvements in human health have a major impact on our national economy by increasing the ability of many to contribute to society
- Most of the industry action is “on the coast”
  - California is home to over half the biotechnology industry
  - A few select “hot spots” are home to most of the rest of the industry
    - Boston
    - North Carolina
    - Maryland
    - New Jersey
    - New York

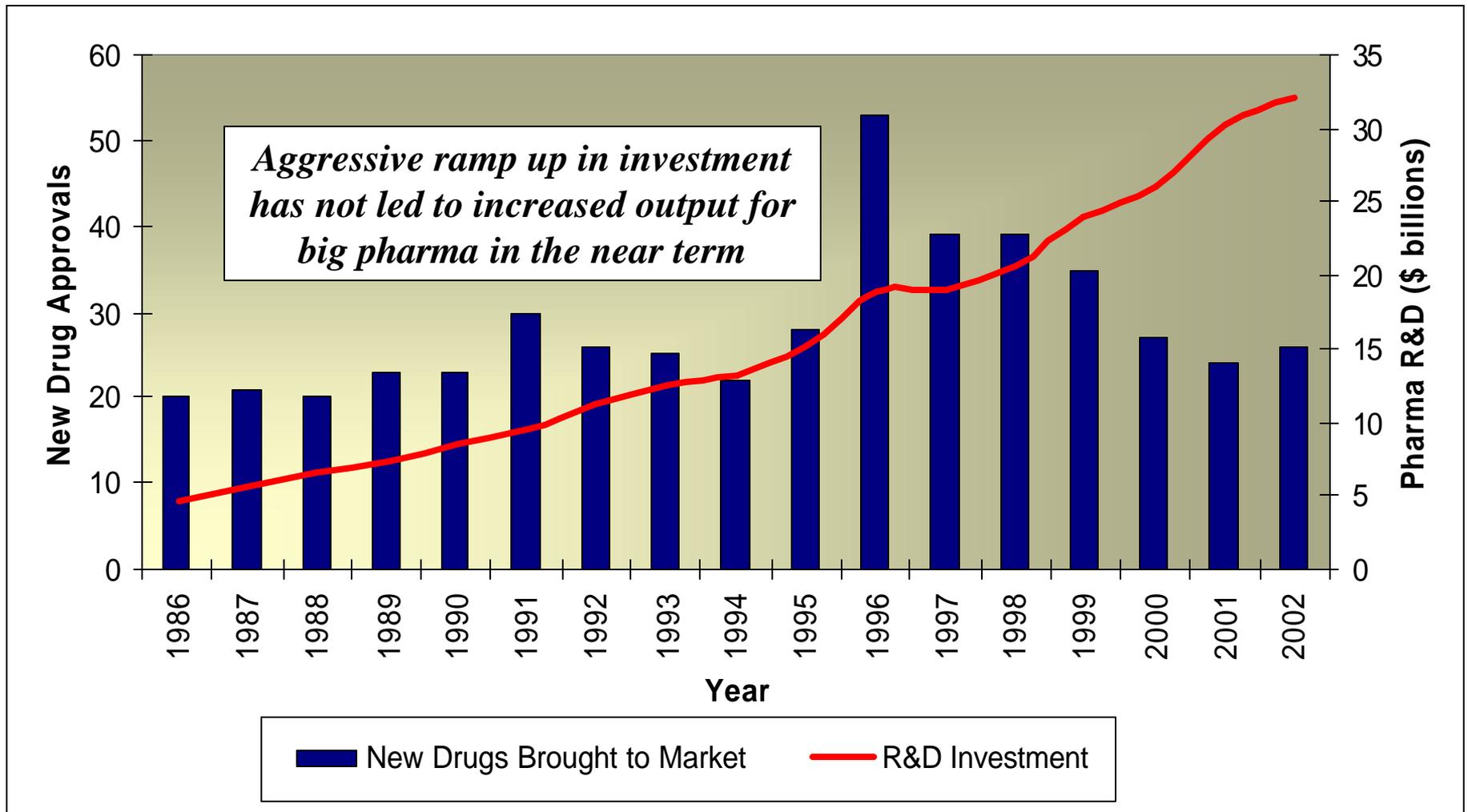
# Key Patents are Expiring on Big Pharma Products

Brand Name	Marketer	2002 World-wide Sales (\$ in millions)	Brand Name	Marketer	2002 World-wide Sales (\$ in millions)
2002			2005		
Neurontin	Pfizer	2,269	Prilosec	Astra-Zeneca	4,623
Claritin	Schering-Plough	1,802	Prevacid	Tap	3,157
Augmentin	GSK	1,787	Zolof	Pfizer	2,742
Intron A	Schering-Plough	1,500	Pravachol	BMS	2,266
Zestril	Astra-Zeneca	877	Zithromax	BMS	1,516
	Total	8,235	Biaxin	Pfizer	1,112
2003			Zofran	Abbott	1,062
Cipro	Bayer	1,334	Zoladex	Novartis	794
Singulair	Merck	1,505		Total	17,272
Flovent	GSK	1,174	2006		
Flonase	GSK	801	Zocor	Merck	5,580
Engerix	GSK	725	Paxil	GSK	3,083
	Total	5,539	Fosamax	Merck	2,250
2004			Pravachol	BMS	2,266
Lovenox	Aventis	1,478		Total	13,179
Diflucan	Pfizer	1,112	2007		
Lupron	Tap	876	Norvasc	Pfizer	3,846
Lamisil	Novartis	874	Risperdal	Janssen	2,146
Paraplatin	BMS	727	Lupron	TAP	1,300
Xenical	Roche	490	Imitrex	GSK	1,197
	Total	5,557	Lamisil	Novartis	874
				Total	9,363

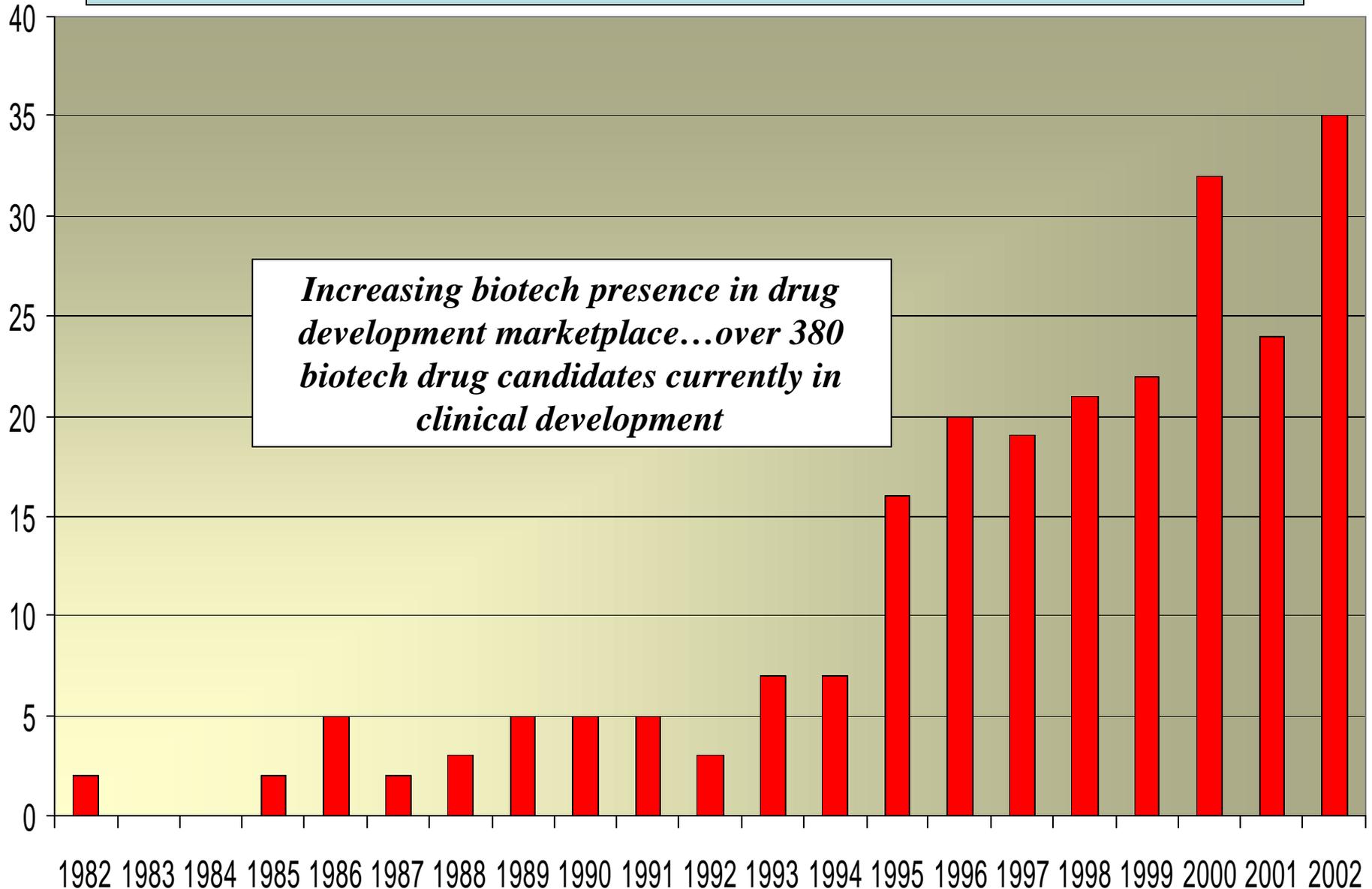
- Revenue losses of over \$60 Billion expected by 2007...resulting price erosion from patent expiration and generic competition directly benefits consumers
- To put this in perspective the top 50 prescription drugs in 2002 brought in revenues of just over \$100 Billion

# Pharmaceutical Companies Are Facing a Major Productivity Gap

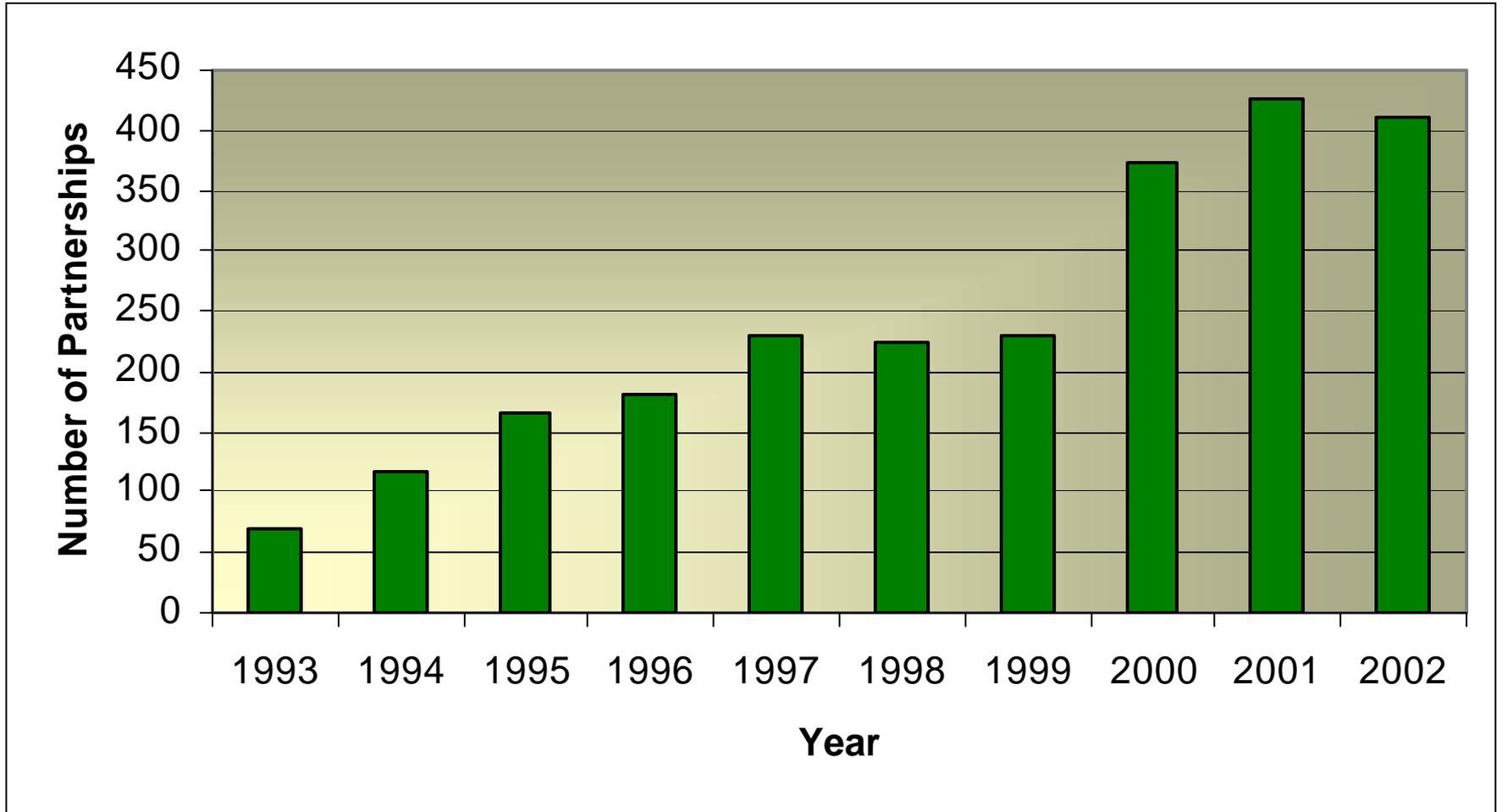
*As Big Pharma spends more on internal R&D, it is seeing fewer NDA's*



## In Contrast... Biotech Product Approvals Are Increasing



# Number of Biotech-Pharma Collaborations Increasing as Pharma Tries to Fill the Pipeline Gap





## Some Things That Have Worked Well

# Things That Have Worked Well\*

- Technology Transfer
  - Earliest roots stem from linking tech transfer to national economic growth from studies in the 1940's
  - Bayh-Dole Act enacted in 1980
- Key NIH Funded/Sponsored/Managed Initiatives
  - Primary focus on basic study of human biology, disease – not drug development
  - Enormous successes in many areas (Example: Human Genome Project)
  - Best with appropriate oversight and when some competition/cooperation between public and private sector exists
- Programs That Provide Early Capital for Development of High Impact Technologies
  - Examples: SBIR, ATP, other programs that seek to leverage federal, state, and private capital
- Incentives for Investment of Capital into the Private Sector
  - The biotechnology industry is critically dependent on venture/investment capital
  - Initiatives that increase the pool of investment capital enable fundamental advances in the healthcare system
  - Over 1,500 companies that are working to develop treatments or cures for almost every conceivable major form of human disease, and many orphan diseases

Note: Doesn't mean things that they have worked perfectly - commitment to a "Culture of Continuous Improvement" is required (building on successes, iteratively addressing shortcomings)



# Things That Have Worked Well\*

- One Example: Stem Cell Research
  - Just a few short years ago that embryonic and adult stem cells were first identified
  - Prior to the current administration, there was NO POLICY in place to support work in this area, no national focus/effort
  - Funding to support stem cell research (both federal, state, and private) has grown dramatically in the last 3 years...but everyone recognizes we still have a long way to go
  - Many ethical (and other) issues to consider...no simple answers
- Potential Impact of Stem Cell Research on National Healthcare
  - Regenerative medicine will reshape medicine as we know it, enabling treatment of diseases, conditions that today are virtually impossible to treat effectively
  - It's about more than just the stem cells – it's also about identification of factors, compounds that promote better tissue regeneration and repair
  - Will impact many clinical areas, including: cardiovascular disease, diabetes, orthopedics, oncology, chronic diseases of aging, others

# An Emerging Success: Creation of National Centers for Stem Cells And Regenerative Medicine

- Early Example in Ohio: [Center for Stem Cell and Regenerative Medicine Research](#)
- Five Founders (Commercial & Non-profit Institutional Alliance):
  - Athersys
  - The Cleveland Clinic
  - University Hospitals Health Systems
  - Case Western Reserve University
  - BioEnterprise
  - Additional collaborators (Arteriocyte)
- \$19.4 million Ohio state grant (ODOD) to launch CSCRM institute (Third Frontier Initiative)
- Multiple adult stem cell technologies in development: MAPCs, cord blood stem cell therapies, others
  - First efforts expected to enter clinical trials in 2005
- Additional Linkages Formed Between Institute and NIH, Leading Disease Foundations
  - Athersys-NHLBI (Cardiovascular Disease, Peripheral Vascular Disease)
  - Athersys-JDRF (Type I Diabetes)



# Challenges & Barriers

# Major Challenges to the Biotechnology Industry

- Exogenous Threats to Investment Capital Formation/Implementation
  - Two forms of investment capital:
    - (1) **portfolio capital** (transfer of shares in already public companies – creates wealth and liquidity for investors, but does not directly provide capital for growth;
    - (2) **growth capital** (e.g. venture capital, capital accessed through IPO or secondary offerings)
  - In 2002 biotech investment funds decreased by over \$3 Billion; in 2003 decreased by over \$1 Billion...effect seen predominantly in growth capital
- Law of Unintended Consequences (Political Form)
  - **Example**: proposing vaguely defined transformational changes in national healthcare system...without considering what this will do to capital markets
  - **Consequence**: Dramatic reduction in capital investment in biotech industry...requiring several years to achieve clarity
  - **Example**: proposing fundamental changes to intellectual property law...without considering what this will do to capital markets
  - **Consequence**: Billions in market value lost overnight as a result of poorly considered statements, and lack of instant/strong clarification

# Major Challenges to the Biotechnology Industry

- Drug Reimportation/Unrestricted Drug Importation Legislation
  - Everyone wants lower drug prices...even companies would prefer faster, less expensive development in exchange for lower prices
  - Foreign Governments Use Price Controls as a Mechanism to control their healthcare costs...they unfairly leverage our national investment
  - **Consequence:** Drug counterfeiting is already surging...nobody takes responsibility or assumes liability for counterfeit products, or resultant damage to patients...many times damage not immediately visible
  - **Consequence:** Price controls artificially limit economic efficiency and company profitability...for what other industry would Congress/the public willingly accept foreign imposed price controls?
  - **Consequence:** Reduced economic efficiency means less attractive returns to investors, and reduced capital flows into sector...or even worse, could literally cause a massive outflow of investment capital
  - **Consequence:** Significant reduction in growth capital means substantial reduction in innovation and delayed/reduced commercialization of breakthrough products
  - **Suggestion:** Instead of legislating price controls which limit profitability, restrict innovation and investment - why not incentivize companies to negotiate price reductions, e.g. through extensions of effective patent life?

# Major Challenges to the Biotechnology Industry

- Inefficiencies in Capital Markets
  - Example: Overconcentration of growth capital in a few select locations in the U.S. (e.g. Venture Capital industry in Bay Area)
  - Enormous pool of potential growth capital (e.g. pension funds) allocated to growth capital managers located largely in handful of locations in U.S. (California, Boston, New York etc.)
  - **Consequence:** Investment capital (primarily venture funding) is deployed in regions proximal to those financial centers
  - **Consequence:** Hundreds of billions of dollars “leave” midwest states (like Ohio) to be managed and invested elsewhere (e.g. California, Boston)
  - **Consequence:** Economic infrastructure in states like Ohio continues to erode, while coastal economies are continually reinvented
  - **Suggestion:** To strengthen and diversify our national economy, why not create financial incentives for states to invest more growth capital in themselves?

# Major Challenges to the Biotechnology Industry

- Inefficient Linkage Between Public and Privately Funded Research
  - Example: Human Genome Project
  - Competition (as opposed to tangible cooperation) between publicly funded and private sector initiatives
  - **Consequence:** Redundant investment of billions of dollars in competing initiatives
  - **Consequence:** IP confusion, inefficient technology transfer
  - **Suggestion:** Create stronger incentives for industry to work together with NIH, and other institutions receiving federal funding
  - **Suggestion:** Make it easier for private sector to access federal funding if there is a demonstrable/quantifiable public health benefit

# Major Challenges to the Biotechnology Industry

- Regulatory Landscape

- Proper leadership, management has a major positive impact
- PDUFA has been a success...regulatory clarity and efficiency is in everyone's best interest
- FDA needs to be adequately funded to function properly...but also held accountable, incentivized to move efficiently
  
- **Suggestion:** Create stronger incentives for industry to work more efficiently with the FDA
  
- **Suggestion:** Create incentives for companies to develop products that are not only better, but also demonstrably more cost effective (not necessarily under the purview of the FDA)