CENTER FOR
Science IN THE
Public Interest

Merrill Goozner

Director

Integrity in Science Project

Innovation in Biomedicine

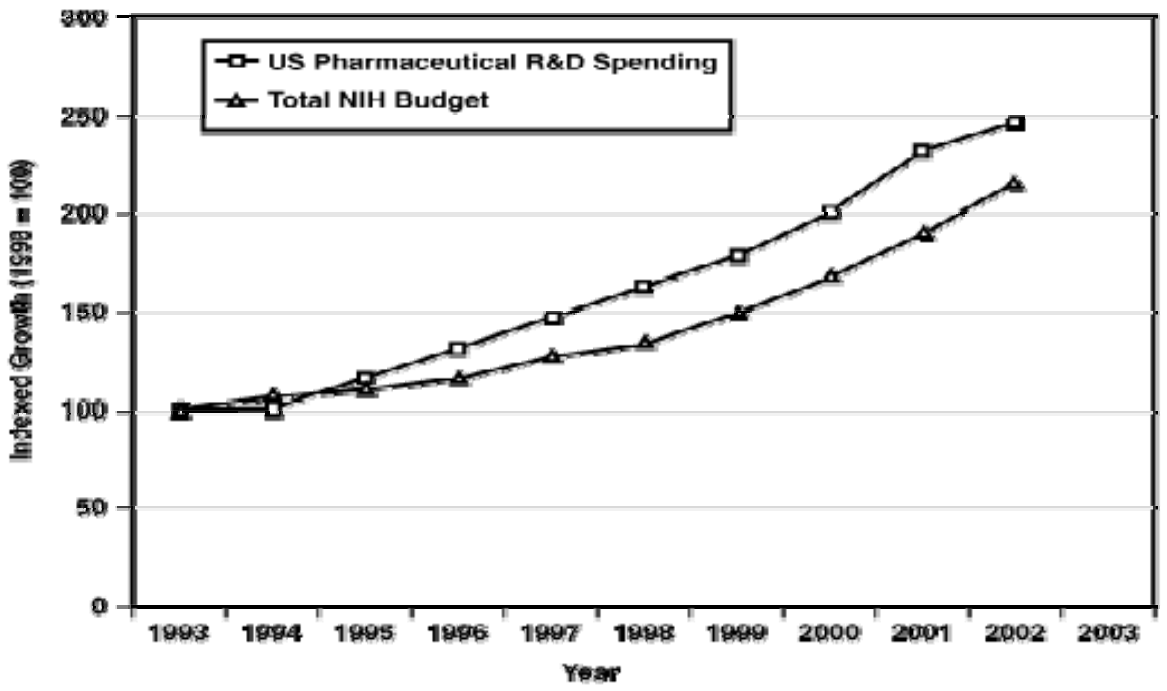
Can Stem Cell Research Lead the Way to Affordability?

The problem

- Under current system, new therapies, no matter how marginally effective, come to market at the highest prices.
  - Avastin
  - Celebrex/Vioxx/Bextra
- Health care systems can no longer afford it.
  - 15 percent of GDP; projected to rise to 20 percent
  - Aging populations worldwide

Innovation or Stagnation?

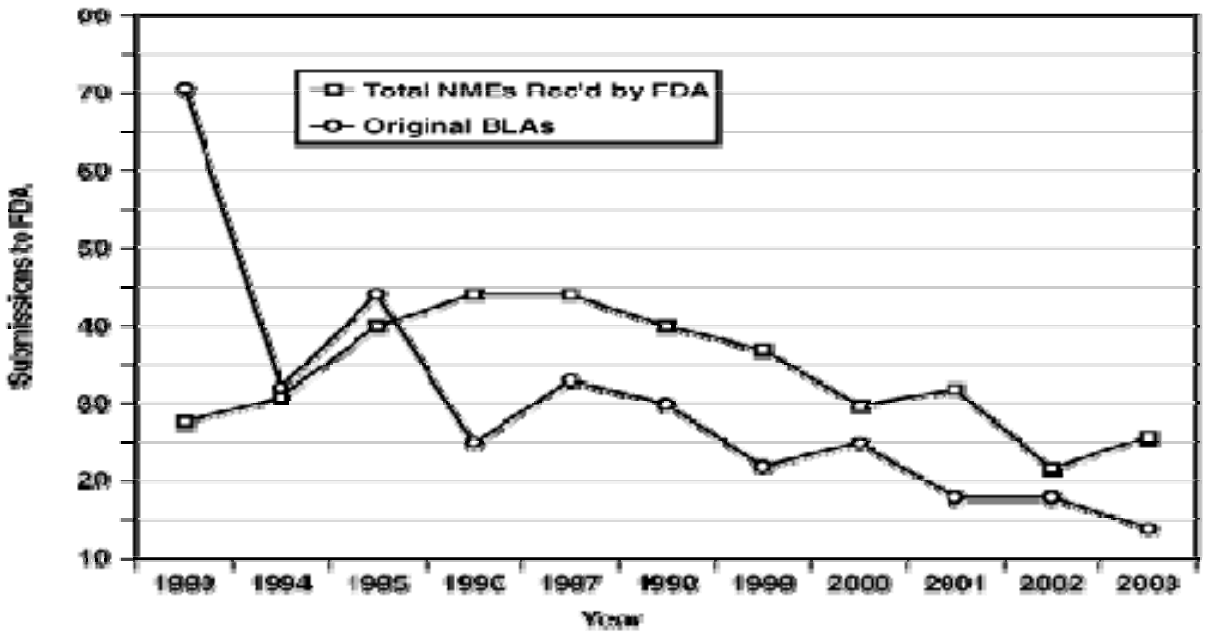
Figure 1: 10-Year Trends in Biomedical Research Spending



The figure shows 10-year trends in biomedical research spending as reflected by the NIH budget (Budget of the United States Government, appendix, FY 1993-2003) and by pharmaceutical companies' research and development (R&D) investment (PAREXEL's Pharmaceutical R&D Statistical Sourcebook 2002/2003).

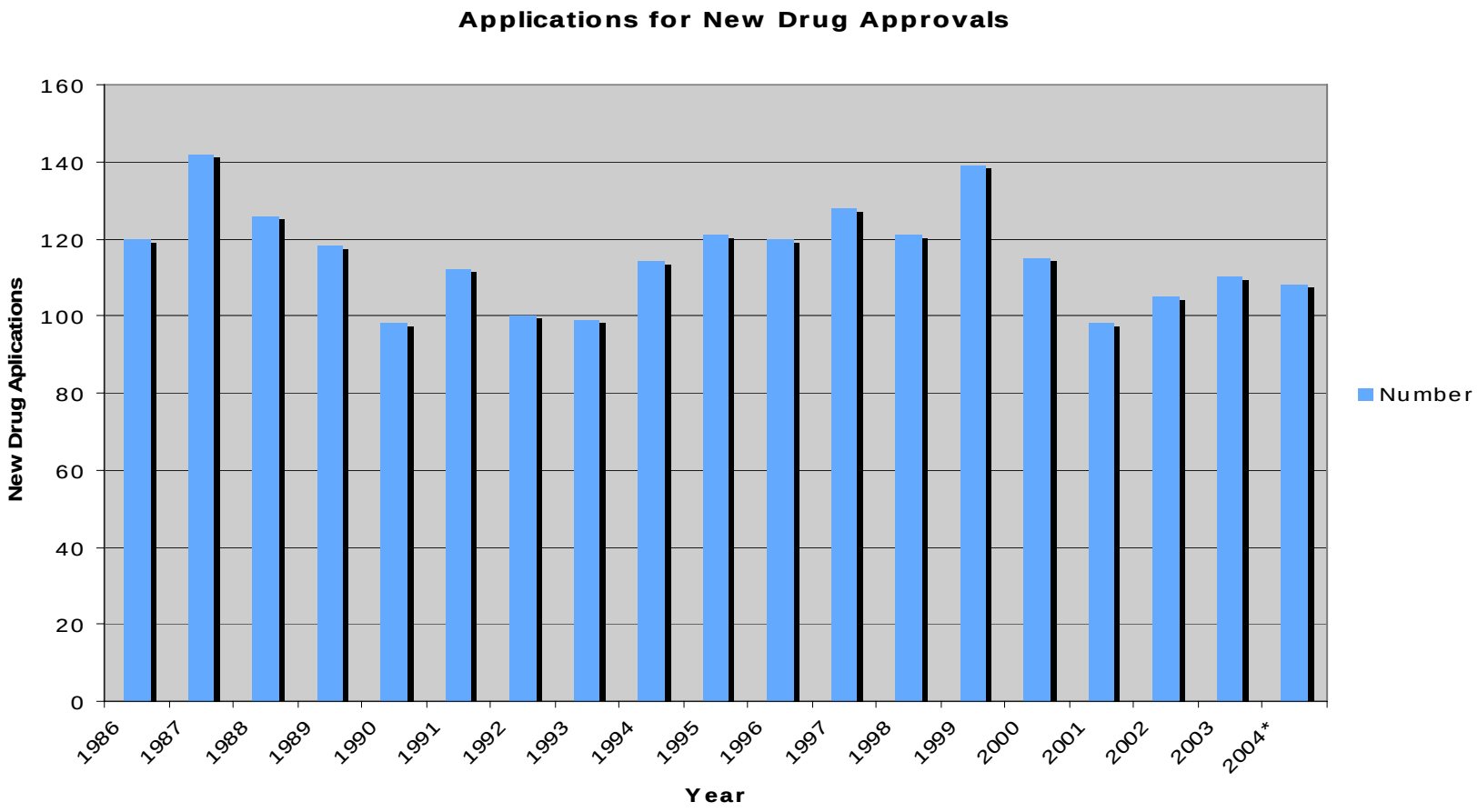
Innovation or Stagnation?

Figure 2: 10-Year Trends in Major Drug and Biological Product Submissions to FDA

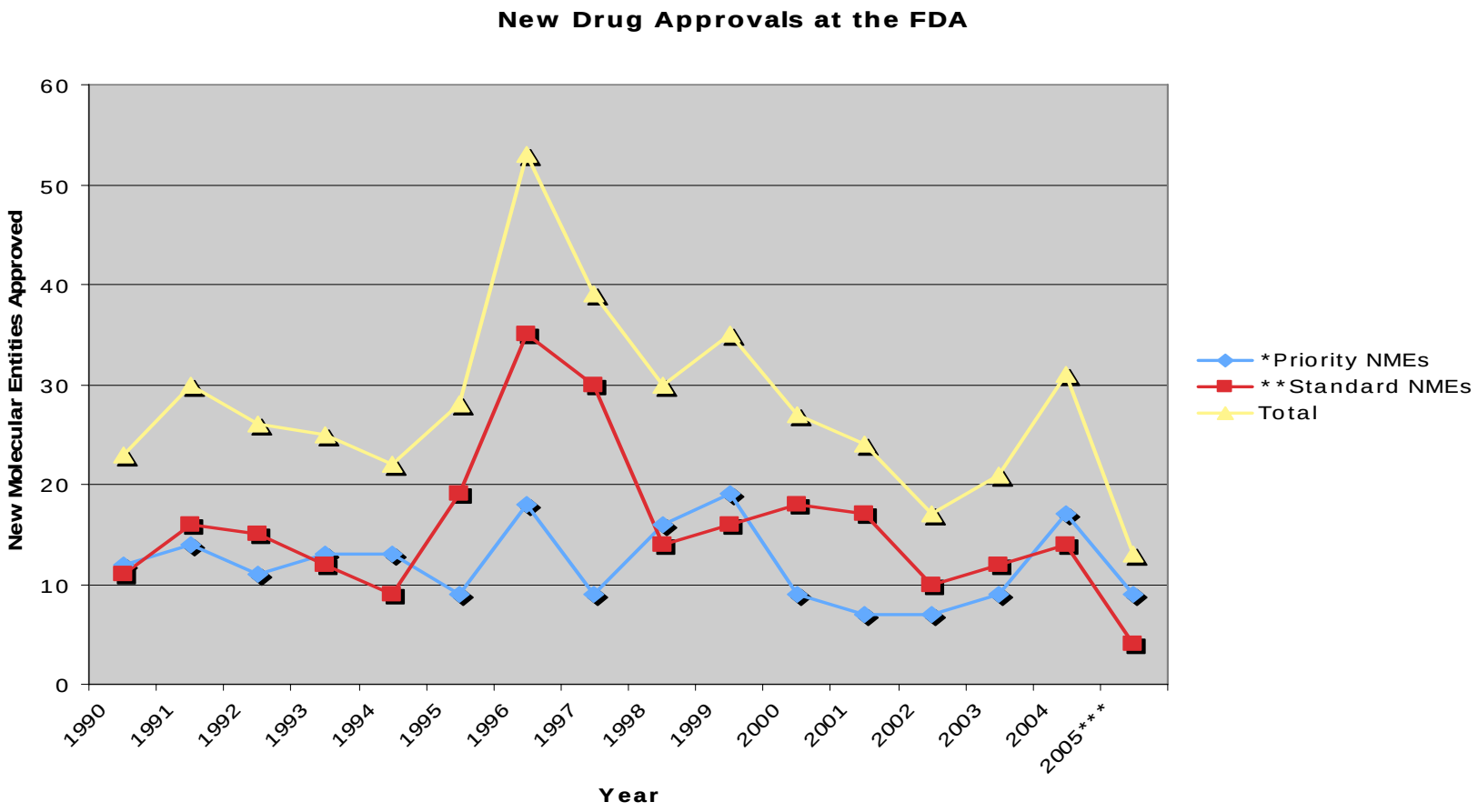


The figure shows the number of submissions of new molecular entities (NMEs) — drugs with a novel chemical structure — and the number of biologics license application (BLA) submissions to FDA over a 10-year period. Similar trends have been observed at regulatory agencies worldwide.

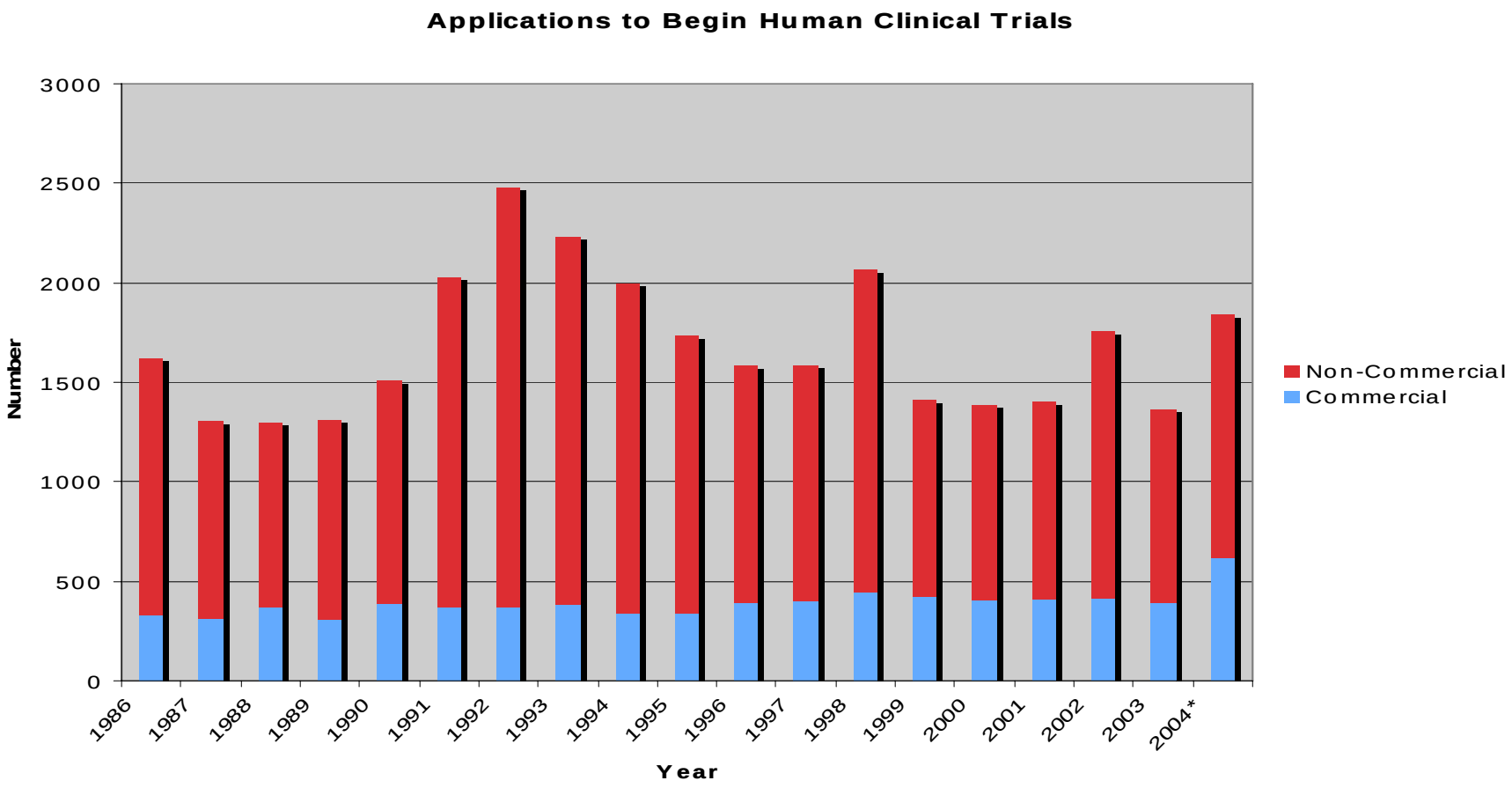
Applications for New Drug Approvals



New Drug Approvals at the FDA



Applications to Begin Human Clinical Trials



Possible explanations

- Low hanging fruit gone
- Major Innovations are rare
- Corporate focus on “me-too” drugs to maintain cash flow and profits
- Regulatory bottlenecks
- Decline of government targeted research campaigns like AIDS and cancer
- IP system

Patents

- Incentives are well-known
  - Provides researchers with incentives to commercialize inventions and knowledge
  - Attracts risk capital
- Disincentives
  - Silo mentality versus collaboration
  - Patent thickets

Patent thickets

- 18,000 stem cell patents worldwide, two-thirds in U.S.
- Seminal patents exist with more to come
  - Thomson, Gearhart, Hwang/Schatten
- Transaction costs and psychic roadblocks

A potential solution

- The goal: To break down barriers to research while retaining incentives of existing patent system

Open source patent pool

- A requirement of government-funded research
  - California Stem Cell initiative, the first major targeted research campaign in over a decade
- Can serve as one-stop shop for securing licenses
- Can enforce common ethical standards and help create regulatory standards
- Can leverage cooperation from existing or outside patent holders
- Can facilitate collaboration
- The biomedical difference: give flexibility to grant exclusive license for final, most costly clinical trials

Eyes on the Prize

- Substitute prize system for monopoly patent rights
  - Value of prize based on medical value, size of patient population, difficulty in developing cure, and capital investment required to achieve results.
  - Divided among patent holders using either mandatory arbitration or audited expenditures
  - Creates an innovation market separate from health care finance system
  - Generic production and tax exempt prize financing facilities lowest possible cost for health care system

Pool/Prize consistent with existing IP system

- Inventors and institutions retain IP ownership and returns
- For academic institutions, prize can be shared with inventor with remainder invested in research and education
- Prize also encourages tech transfer offices to pursue use
- Should be sufficiently large to attract private sector, especially if division among rights holders based on investment

States – the laboratories of democracy

- By combining a patent pool, an open-source model of IP development, and a shared prize system for developing stem cell therapies, California can point the way to a new medical innovation system for the 21st century.
- This can be adapted by the federal government or any country grappling with how to afford the future medical breakthroughs demanded by their ill and aging populations.