

From Hope to Cures: The Value of Biopharmaceutical Innovation



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Tara Ryan Vice President, State Government Advocacy



HIV/AIDS: Then & Now



HIV / AIDS PATIENTS WERE SUFFERING AND DYING

In the early years of the AIDS epidemic before ART was available, the median survival after an AIDS diagnosis was measured in weeks to months and patient care was confined to diagnosing and treating a complex array of opportunistic infections and AIDS-related types of cancer.

The New York Times

September 15, 1989

AIDS TREATMENT COSTS PUT AT \$5 BILLION A YEAR

"We have got to get our act together now because the medical system is going to be crushed in two years"

Daniel Hoth

Director of the Division of AIDS National Institute for Allergy and Infectious Diseases

The Washington Post

June 13, 1988

THE BIG DEAL ABOUT AIDS

public health experts
estimate the AIDS deal
will reach total annual
costs of \$66.5 billion by
1991 - a figure that could
bankrupt our health care
system.



ONGOING INNOVATION HAS BROUGHT DOWN THE DEATH RATE BY 83%. LIVES ARE DRAMATICALLY LONGER AND BETTER

In stark contrast to the early and mid-1980s, if a person aged 20 years is newly infected with HIV today and guideline recommended therapy is initiated, researchers can predict by using mathematical modeling that this person will live at least an additional 50 years—that is, a close-to-normal life expectancy.

Remember HIV?.... thanks to a wave of new discoveries that came both from academic centers and the pharmaceutical industry, the HIV crisis was transformed into a stable condition which is managed very differently by society where good drugs are available. They are controlling the disease, and society has been saving an enormous amount of money as a result of these innovative drugs by providing better care out of hospitals.

Hervé Hoppenot CEO, Incyte

We used to think HIV costs would overwhelm us....but we figured it out and let drug development progress.

Ira Klein, M.D.
M.B.A., FACP, Aetna

Medicines Are Transforming the Treatment of Many Diseases

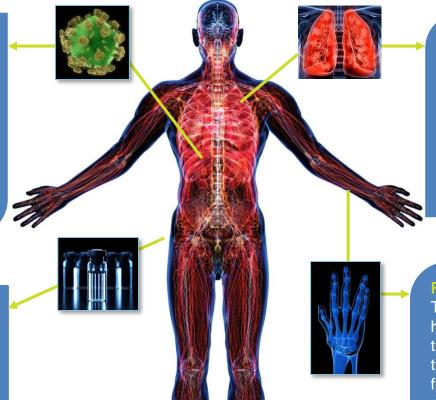
HIV/AIDS

In the last 2 decades, advances in treatment have contributed to a nearly 85% decline in death rates and transformed the disease from an acute, fatal illness to a chronic condition.



Cancer

New therapies have contributed to a nearly 22% decline in cancer deaths since the 1990s. Today, 2 out of 3 people diagnosed with cancer survive at least 5 years.



Cystic Fibrosis

New treatments targeting both the symptoms and root cause of the disease have contributed to improvements in quality of life for patients as well as increases in life expectancy.

Rheumatoid Arthritis (RA)

Therapeutic advances have transformed the RA treatment paradigm over the last 20 years, shifting from a focus on symptom management to now aiming for slowed disease progression and even disease remission.



The Challenge of Developing New Treatments & Cures



On average, it takes more than

10 Years for a new medicine to go through the entire research and development process.

of drug candidates that enter clinical testing are APPROVED FOR USE BY PATIENTS.



AND THE AVERAGE COST \$2.6 Billion to develop a new medicine has increased to



new medicines have been approved by the FDA since 2000.

medicines are in clinical development around the world today.

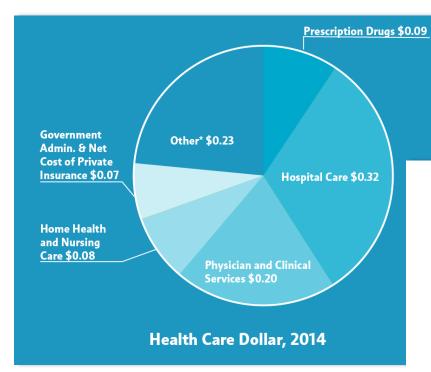


Yet spending on retail prescription medicines accounts for just

OF U.S. HEALTH CARE SPENDING.

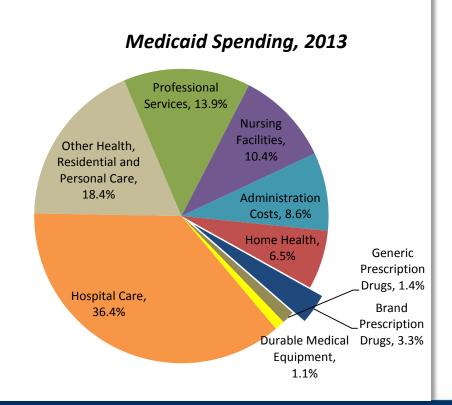


Retail Spending on Prescription Medicines Is a Small Share of Health Care Spending



US Health Care Spending

US Medicaid Spending



Curbing Health Care Costs

If all non-adherent patients with diabetes were to become adherent,

341,000



699,000 emergency room visits

COULD BE AVOIDED ANNUALLY, RESULTING IN NEARLY \$5 BILLION OF MEDICAL SAVINGS.



As more hepatitis C patients receive the newest generation of medicines with

cure rates of more than 90%,

WE CAN EXPECT TO SEE A DECLINE IN THE COST OF TREATING PATIENTS.

Average health care costs for a patient battling liver cancer

\$112K



Average cost for a patient who may need a liver transplant

\$500K

THE NEED FOR CONTINUED DEVELOPMENT OF NEW TREATMENTS IS CRITICAL.

In the fight against Alzheimer's disease, a new medicine that **DELAYS THE ONSET OF THE DISEASE BY FIVE YEARS WOULD SAVE** \$367 billion in health services.



National Foundation for Women Legislators Conference

Oklahoma City, OK September 12, 2015



BIOPHARMACEUTICAL RESEARCH CONTINUUM

NIH - Basic foundational research for biomedical innovation

Academia -Basic and applied research in the early stages of drug development Preclinical Stage Biopharmaceutical Companies -Preclinical proof of concept research Emerging &
Large Clinical Stage
Biopharmaceutical
Companies Phase I, II and III
Clinical
Development

Patient
Advocacy
Organizations Patient education
and support of
clinical trials

FDA Approves
biopharmaceuticals
to ensure safety
and efficacy

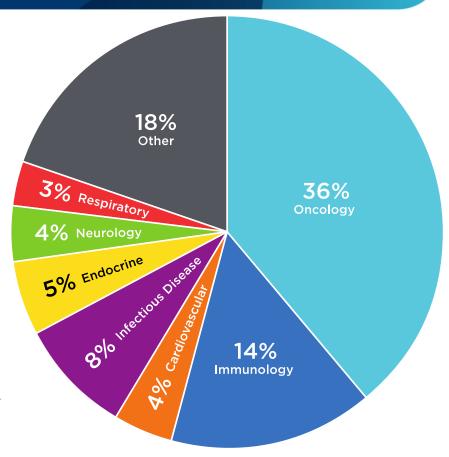
The entire continuum is interconnected and any policy change to one segment will inevitably impact the entire continuum



EMERGING COMPANY CLINICAL PIPELINE

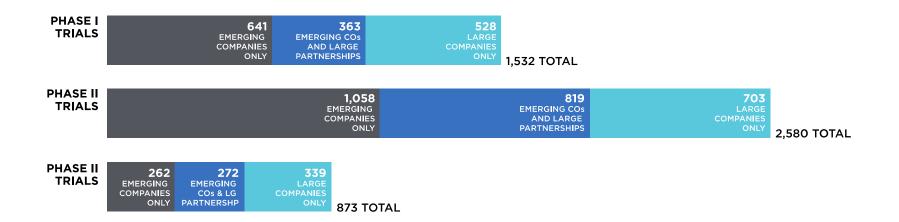
- Oncology accounts for the highest percentage of clinical pipeline and accounts for the highest level of funding from VCS, IPOS & FOPOS.
- The pipeline for orphan (rare diseases) in emerging companies contains 484 clinical trials programs, of that 44% are in partnership with other companies.

Source: Emerging Therapeutic Company Investment and Deal Trends 2005-2014, Biotechnology Industry Organization (BIO), June 2015





EMERGING COMPANY CLINICAL PIPELINE



- 69% of global pharmaceutical pipeline are with emerging companies
- 43% of that pipeline is in partnerships with other biopharmaceutical companies

Source: Emerging Therapeutic Company Investment and Deal Trends 2005-2014, Biotechnology Industry Organization (BIO), June 2015



EMERGING COMPANIES: NEED FOR FINANCING

Seed

Series A

Series B

Series C,D,E,... IPO or Sale

\$0.5-3.0MM

- Write business plan
- Acquire rights to technology / product

\$5-20MM

- Conduct initial discovery
- Animal testing and early human studies

\$25-50MM

- Proof of concept
- Early clinical trials

\$50-100MM+

- Pivotal studies (Phase II-III)
- NDA/MAA submission
- Commercialization

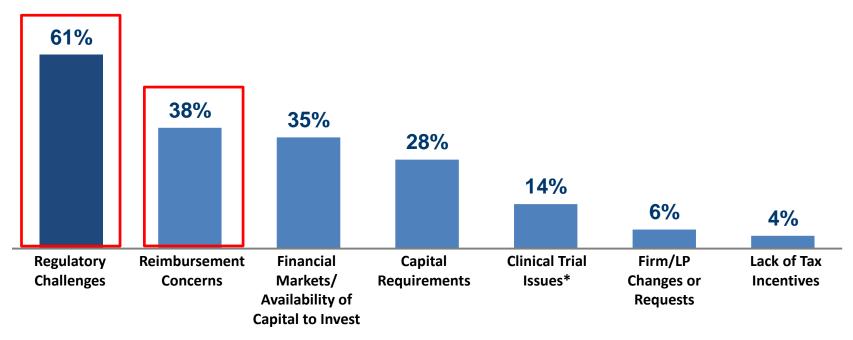
Must Demonstrate Progress To Raise Next Round Of Money



VENTURE CAPITALISTS: REASONS FOR NOT IINVESTING

NVCA Survey of Life Sciences Venture Capital Investors

% of Respondents Factors Cited as Having Highest Impact



^{*} Unrelated to Regulatory Challenges.





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Patient Engagement

Identify When to Engage



Determine Purpose for Engagement

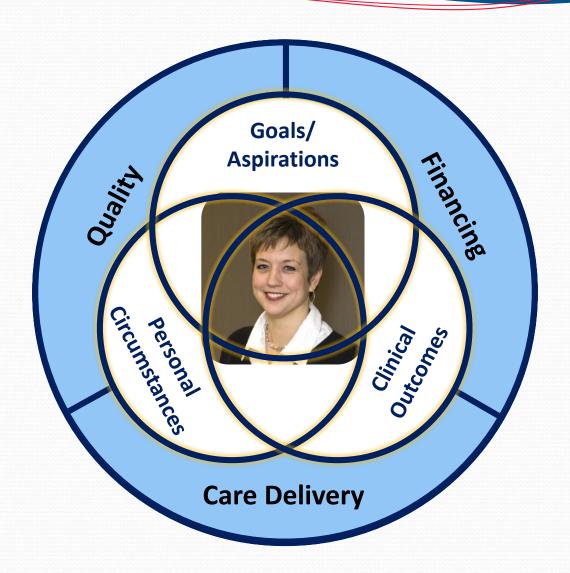


Decide on Method(s) for Engagement

Consider Who to Engage













Marc Boutin Chief Executive Officer National Health Council

mboutin@nhcouncil.org