

# Is Cancer Treatment A Stalking Horse For The Future Of Health Care?

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Deputy Chief Medical Officer  
October 22, 2018



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## Is Cancer A Stalking Horse For The Next Generation Of Health Care?

### Burkitt's Tumor as a Stalking Horse for Leukemia

Joseph H. Burchenal, MD



The *Oxford Universal Dictionary* defines a stalking horse as "a horse trained to allow a fowler to conceal himself behind it or under its coverings in order to get within easy range of the game without alarming it." Thus the title and its definition are meant to suggest that a careful study of Burkitt's tumor may provide a useful approach to the eventual control of acute leukemia.

JAMA, Nov 27, 1972 • Vol 222, No 9

Lasker Awards 1165

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### The National Cancer Act, December 23, 1971



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### How Times Have Changed...

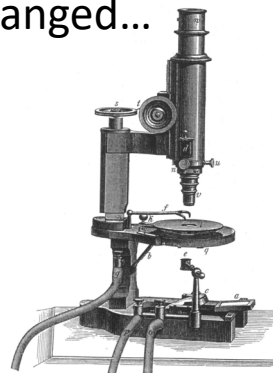
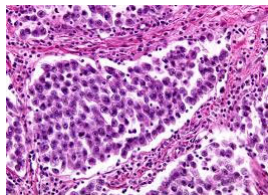
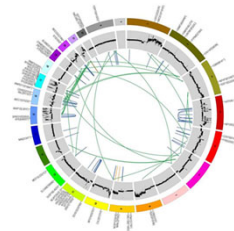


Fig. 20.



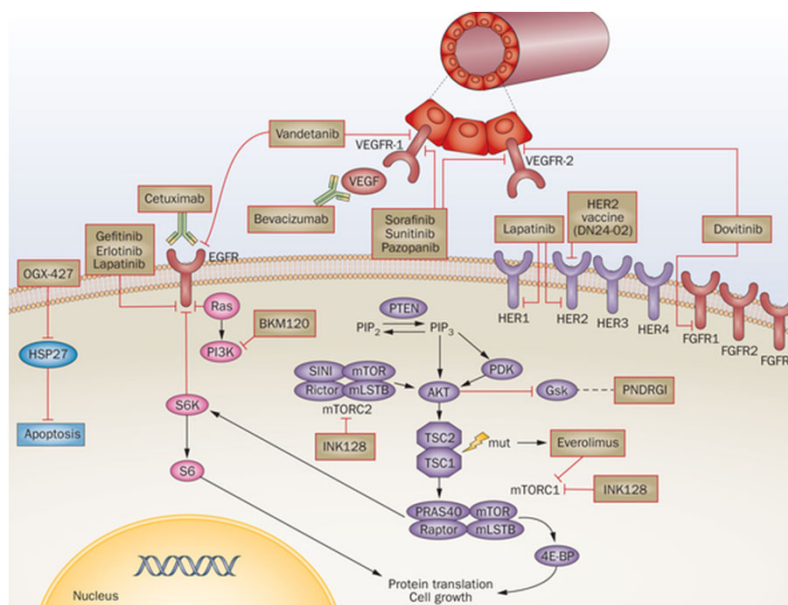
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## It Didn't Happen Overnight...



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Expert perspective, insight and discussion

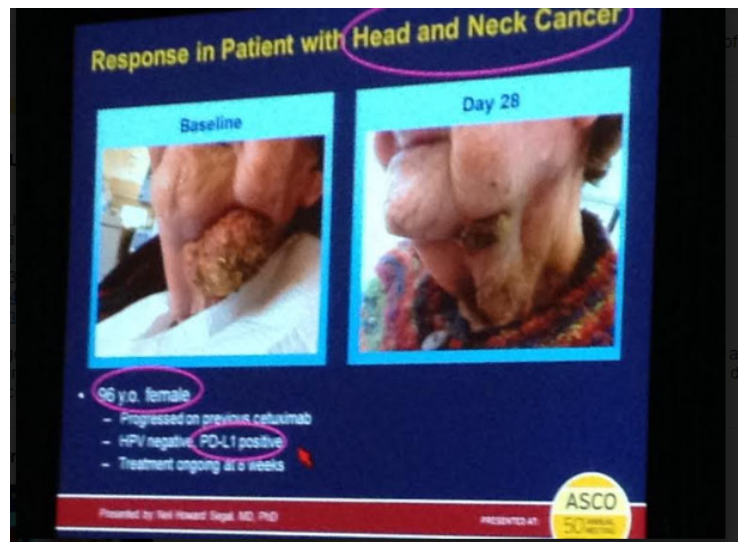
As noted by some of the speakers at the IOM meeting, we are facing “pharmageddon”: a situation where the investments required to bring a drug to market are becoming so great that the folks who normally fund those opportunities (read that: pharmaceutical companies and venture capitalists) are becoming very wary of putting their money on the table to do the research necessary to take an idea from the lab to the bedside and see it through to success in the clinic.

April 2, 2012

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Annual meeting ASCO June 3, 2014


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NBC NEWS

HEALTH  
MAY 18 2016, 5:43 PM ET

## Cancer Drug Keytruda Keeps Some Patients Alive For 3 Years

by MAGGIE SENEVIRATNE



► Cancer Drug Used by Pres. Carter Shows Signs of Being a Breakthrough 2:22

The cancer drug that former president Jimmy Carter says made his melanoma seemingly disappear has helped about 40 percent of similar patients survive for as long as three years, oncologists said Wednesday.

The drug, called Keytruda, takes a new approach to treating cancer by stopping tumor cells from cloaking themselves against the normal, healthy immune system response.

The drugs must be infused and they are pricey. Keytruda costs about \$12,500 a month, or \$150,000 a year.

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## US Health Expenditures In 2017

# \$3.5 Trillion

“CMS projected that healthcare spending will on average rise 5.5 percent annually from 2017 to 2026 and will comprise 19.7 percent of the U.S. economy in 2026, up from 17.9 percent in 2016. By 2026, health spending is projected to reach \$5.7 trillion.”

REUTERS

HEALTH NEWS    FEBRUARY 14, 2018 / 4:07 PM

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## Indirect Costs



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Stanford MEDICINE

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Peter Bach on drug pricing: "A system so broken even a child could manipulate it"

Sep 26 2015  
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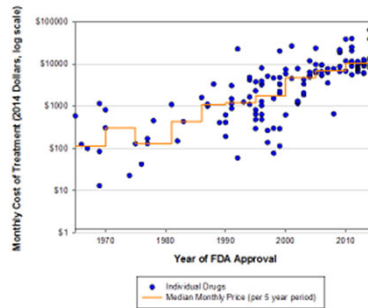
A meditation at the start of medical school

The power of learning by experience as a clerkship student

I'm not suicidal But we should talk

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Monthly and Median Costs of Cancer Drugs at the Time of FDA Approval  
1965-2015



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## GILEAD SCIENCES SETS PRICE FOR YESCARTA, CAR-T LYMPHOMA DRUG APPROVED BY FDA AT \$373,000

FDA AT \$373,000

Published 6:05 PM ET Wed, 18 Oct 2017

**REUTERS**

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## Loxo Oncology Announces Positive Top-Line Results from Independent Review Committee Assessment of Larotrectinib Dataset

Wed October 18, 2017 6:00 AM | GlobeNewswire | About LOXO

~ 75% Overall Response Rate by Independent Review; 80% Overall Response Rate by Investigator Assessment ~

STAMFORD, Conn., Oct. 18, 2017 (GLOBE NEWSWIRE) -- Loxo Oncology, Inc. (Nasdaq:LOXO), a biopharmaceutical company innovating the development of highly selective medicines for patients with genetically defined cancers, today announced top-line overall response rate (ORR) results from the independent review committee assessment of the larotrectinib dataset. The full dataset is

The screenshot shows a Genomeweb article. The header includes the Genomeweb logo and navigation links: Business & Policy, Technology, Research, Diagnostics, Disease Areas, Applied Markets, and Resources. The article title is "Loxo Oncology to Seek FDA Approval for Pan-Cancer Drug in Patients with TRK Fusions" with a sub-headline "Jun 03, 2017 | Turna Ray". A "Premium" tag is visible. The article text states: "CHICAGO -- Loxo Oncology will seek US Food and Drug Administration approval for larotrectinib as a treatment for patients with TRK fusion-positive tumors after three-quarters of participants in a combined analysis responded to the drug." A quote follows: "TRK fusions occur in between 1,500 and 5,000 cancer patients per year, comprising 1 to 3 percent of cancer cases. 'This study, due to the rarity of TRK fusions, required patients to travel from around the world,' Hyman said. 'It really was a heroic effort on the part of the patients, many of whom flew transcontinentally, monthly to be in this study.'" A "Breaking News" sidebar on the right lists: "Luminex Receives FDA Clearance for Aries Group A Strip Assay", "New Statistical Model Identifies Rare Tumor Suppressing Genes", and "Zymo Research Inks Distribution Deal With VWR".

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## Is \$475,000 Too High a Price for Novartis's 'Historic' Cancer Gene Therapy?

By Sy Mukherjee August 31, 2017

On Wednesday, Swiss pharmaceutical giant Novartis made history as the first company to win Food and Drug Administration (FDA) approval for a groundbreaking new type of cancer treatment known as CAR-T. This technology harnesses the power of patients' very immune cells—which are extracted from them, reengineered in a lab, and then pumped back into the body—to kill aggressive blood cancers. The treatment, named Kymriah, was hailed by doctors and the life sciences community as a major advance in medicine and a boon to children and young adults with a certain form of leukemia (the group for whom the gene therapy is approved). The FDA itself called the approval a "historic action."

"It's really transformative," as Dr. Kevin J. Curran, a pediatric oncologist at the Memorial Sloan Kettering Cancer Center, told *Fortune* in an interview. "It's shown a massive response rate in people with these cancers. It's given hope to patients and parents. If other treatments fail, we can tell them, we have this new weapon in our arsenal that teaches your cells to fight cancer."

FORTUNE



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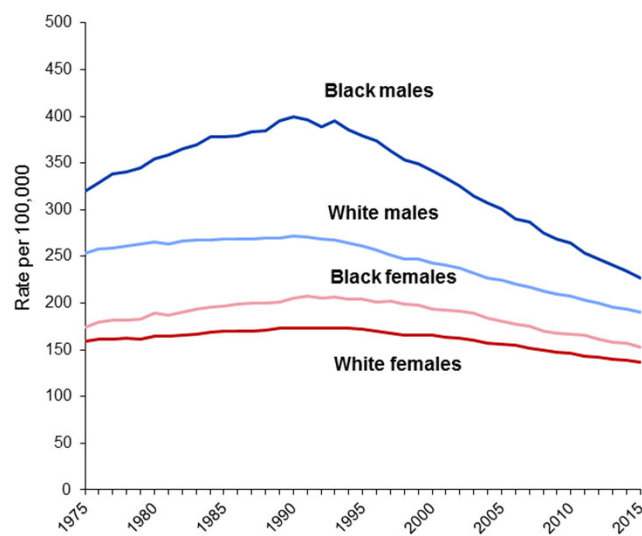
# Next?



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**Trends in Cancer Death Rates\* by Sex and Race, US, 1975-2015**



\*Age-adjusted to the 2000 US standard population.  
Source: National Center for Health Statistics, Centers for Disease Control and Prevention, 2017.

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# From Evolution to Revolution: The Funnel Effect



## Historical Perspectives on the Management of Hypertension

Marvin Moser MD

First published: August 2006 Full publication history

DOI: 10.1111/j.1524-6175.2006.05836.x View/save citation

Cited by (CrossRef): 18 articles Check for updates Citation tools



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View Issue TOC  
Volume 8, Issue s8  
August 2006  
Pages 15–20

### Abstract

*As late as the 1950s, elevated blood pressure was considered by many expert physicians to be necessary for the adequate perfusion of vital organs. Although the morbidity and mortality risks of hypertension were known at that time to insurance companies, which often refused life insurance policies to people with high blood pressure, there was a lag in the recognition of the dangers of hypertension in the medical community, following the pioneering efforts of researchers who began to treat patients with malignant hypertension, the results of clinical trials and population studies, and the availability of effective antihypertensive agents. Hypertension management improved rapidly. This review traces the history of hypertension management from the 1940s, when President Franklin Delano Roosevelt died of a cerebrovascular accident—a result of uncontrolled hypertension—to today, when a large number of patients, even those with less severe hypertension, are being treated successfully, with*

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**FINANCING AROUND A HEALTH CARE BOTTLENECK**

A NEW MIT SLOAN COURSE WILL APPLY ALTERNATIVE FUNDING TECHNIQUES TO NEW DRUG DEVELOPMENT.

By JH Murrell | October 13, 2012

MIT Sloan professor Andrew W. Lo

What good is discovering a potential cure for cancer if there's no money to make a happen? Scientists understand more about diseases now than ever before, says MIT Sloan professor Andrew W. Lo, but investment in drug development has stalled at a critical moment. Lo, the director of the MIT Laboratory for Financial Engineering, believes a little creativity will go a long way when it comes to delivering new treatments and cures to patients.

## Commercializing biomedical research through securitization techniques

Jose-Maria Fernandez, Roger M Stein & Andrew W Lo

Affiliations | Contributions | Corresponding author

Nature Biotechnology 30, 964–975 (2012) | doi:10.1038/nbt.2374

Published online 30 September 2012

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### Abstract

Abstract · References · Author information · Supplementary information

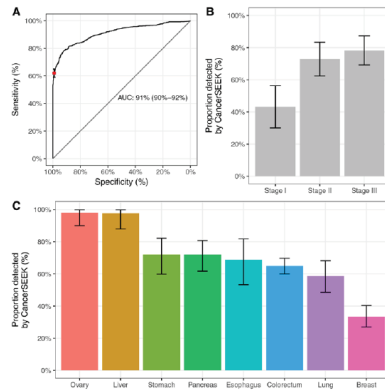
Biomedical innovation has become riskier, more expensive and more difficult to finance with traditional sources such as private and public equity. Here we propose a financial structure in which a large number of biomedical programs at various stages of development are funded by a single entity to substantially reduce the portfolio's risk. The portfolio entity can finance its activities by issuing debt, a critical advantage because a much larger pool of capital is available for investment in debt versus equity. By employing financial engineering techniques such as securitization, it can raise even greater amounts of more-patient capital. In a simulation using historical data for new molecular entities in oncology from 1990 to 2011, we find that megafunds of \$5–15 billion may yield average investment returns of 8.9–11.4% for equity holders and 5–8% for 'research-backed obligation' holders, which are lower than typical venture-capital hurdle rates but attractive to pension funds, insurance companies and other large institutional investors.

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## Detection and localization of surgically resectable cancers with a multi-analyte blood test

Joshua D. Cohen,<sup>1,2,3,4,5,6,7</sup> Lu Li,<sup>4</sup> Yuxuan Wang,<sup>1,2,3,4,5</sup> Christopher Thoburn,<sup>4</sup> Bahman Afshari,<sup>1</sup> Ludmila Dantova,<sup>4</sup> Christopher Donville,<sup>1,2,3,4,5</sup> Ammar A. Javed,<sup>4</sup> Fay Wong,<sup>1,2,3,4,5</sup> Austin Mattox,<sup>1,2,3,4,5</sup> Ralph H. Hruban,<sup>1,2,3</sup> Christopher L. Wolfgang,<sup>1,2,3</sup> Michael G. Goggins,<sup>1,2,3,4,5,6,7,8</sup> Marco Dai Mollo,<sup>4</sup> Tian-Li Wang,<sup>1,2</sup> Richard Roden,<sup>1,2</sup> Alison P. Klein,<sup>1,2,3,4</sup> Janine Prisk,<sup>1,2,3,4</sup> Lisa Doherty,<sup>1,2,3,4</sup> Joy Schaefer,<sup>1,2,3,4</sup> Natalie Silliman,<sup>1,2,3,4</sup> Maria Popoli,<sup>1,2,3,4,5</sup> Joshua T. Vogelstein,<sup>1,2</sup> James D. Browne,<sup>1,2</sup> Robert E. Schoen,<sup>1,2,3</sup> Randall E. Brand,<sup>1,2</sup> Jeanne Tse,<sup>1,2,3,4,5,6,7,8,9,10,11</sup> Peter Gibbs,<sup>1,2,3,4,5,6,7,8,9,10,11</sup> Hui-Li Wong,<sup>1,2</sup> Aaron S. Mansfield,<sup>1,2</sup> Jin Jen,<sup>1,2</sup> Samir M. Hanash,<sup>1,2</sup> Massimo Falconi,<sup>1,2</sup> Peter J. Allen,<sup>1,2</sup> Shihlin Zhou,<sup>1,2,3,4</sup> Chetan Bettegowda,<sup>1,2,3,4,5</sup> Luis Diaz,<sup>1,2,3,4</sup> Cristian Tomasetti,<sup>1,2,3,4</sup> Kenneth W. Kinzler,<sup>1,2,3,4</sup> Bert Vogelstein,<sup>1,2,3,4,5</sup> Anne Marie Lennon,<sup>1,2,3,4,5,6,7,8,9,10,11</sup> Nickolas Panadourides<sup>1,2,3,4</sup>



**Fig. 2. Performance of CancerSEEK.** (A) Receiver operator characteristic (ROC) curve for CancerSEEK. The red point on the curve indicates the test's average performance (62%) at > 99% specificity. Error bars represent 95% confidence intervals for sensitivity and specificity at this particular point. The median performance among the 8 cancer types assessed was 70%, as noted in the main text. (B) Sensitivity of CancerSEEK by stage. Bars represent the median sensitivity of the eight cancer types and error bars represent standard errors of the median. (C) Sensitivity of CancerSEEK by tumor type. Error bars represent 95% confidence intervals.

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## The Options/Solutions (?)

- Value based pricing
- Outcomes based pricing
- Indication based pricing
- Transparency
- Regulatory/legislative
- Innovation
- The true price of drug development—and risk?
- The economics of small markets: the 1% (or less) drugs
- One time lifesaving/life altering treatments
- Investment/novel payment mechanisms

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## The Ultimate Question

How can we assure ALL patients, families and caregivers that they are receiving the optimal treatment under the right circumstances at a price that is affordable?

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Thank You!

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