

The National Institutes of Hope

Francis S. Collins, M.D., Ph.D.
Director, National Institutes of Health

Demystifying Medicine

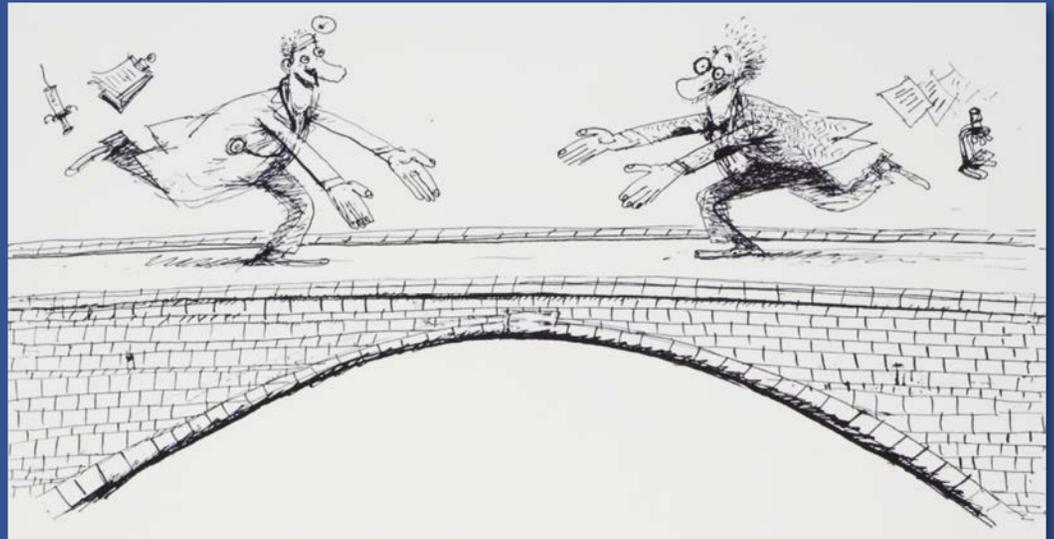
May 8, 2018



Improving Lab-Clinic Translations: *Central to Biomedicine's Success*

“For the past half century, advances in biology and technology have occurred logarithmically; however, their application to understanding and treating human disease has proceeded only arithmetically, creating an increasing gap.”

~Win Arias



YALE MEDICINE

Alumni Bulletin of the School of Medicine

Fall/Winter 1991-1992

Fall/Winter 1991-1992

“When the combination of clinical medicine and basic research does work, the benefit to society is spectacular. There is a cost, in dollars, in time demands on personal life, in trying to wear both hats and sometimes feeling as if neither one fits very well. But surely those of us who have the opportunity to experience both the intensely personal challenge of patient care, and the exultation of scientific advance, are among the luckiest of all human beings.”

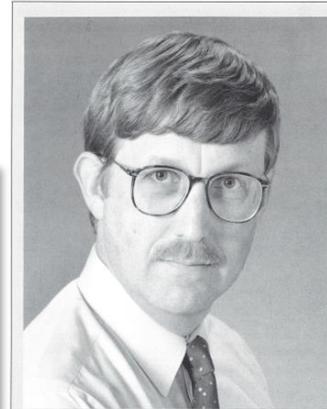
PHYSICIAN-SCIENTISTS: A VANISHING BREED

by Francis S. Collins, M.D., Ph.D.

“The physician-scientist is an ‘endangered species.’”

An article bearing this disturbing title appeared 12 years ago in the *New England Journal of Medicine*. It was written by Jim Wyngaarden, who subsequently became head of the National Institutes of Health (NIH). In the intervening years, matters have gotten even worse — a decreasing number of physicians are playing a meaningful role in basic science.

To define my terms, I refer to a physician-scientist as someone who runs a laboratory and who has funding from a grant-



Dr. Francis S. Collins

Francis S. Collins, M.D., Ph.D.:
Award-winning Geneticist

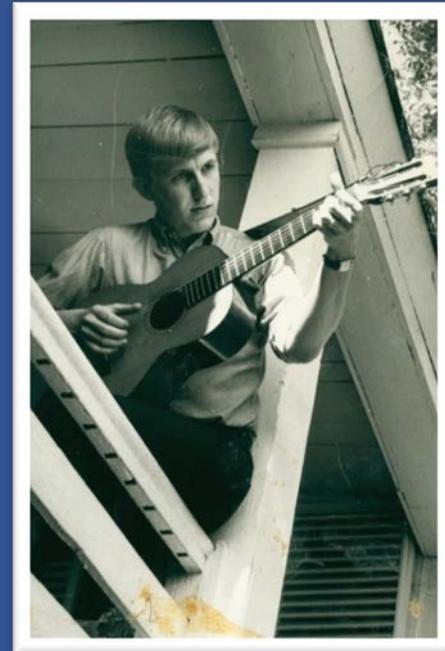
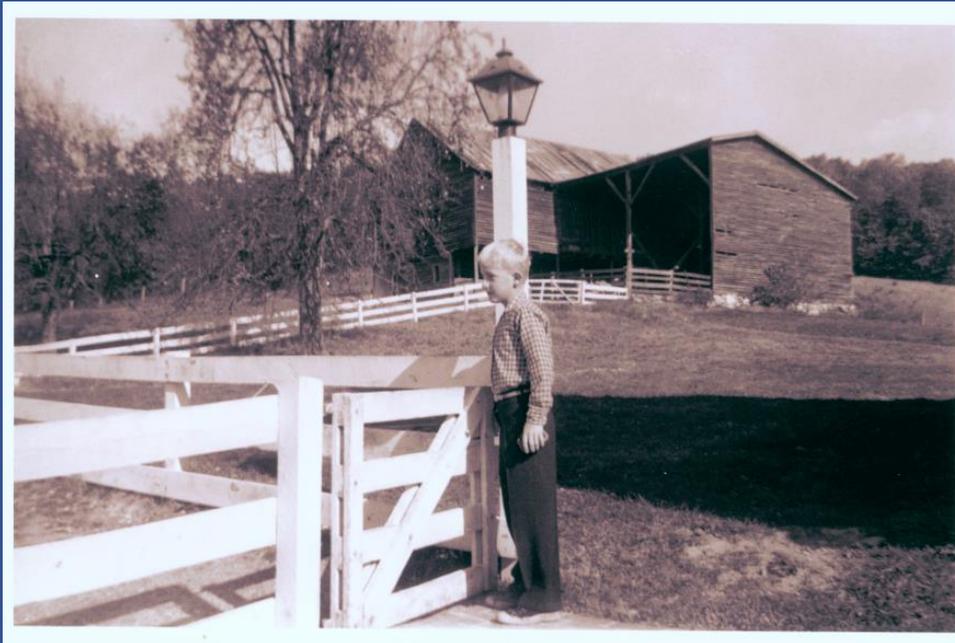
Dr. Francis S. Collins played a leading role in the discovery of the cystic fibrosis gene, a contribution that won him the 1990 Paul di-Sant'Agnesse Distinguished Achievement Award from the Cystic Fibrosis Foundation. A year later, his research group identified the gene for neurofibromatosis. A professor of internal medicine and human genetics at the University of Michigan, he also is an investigator with the Howard Hughes Medical Institute. In 1974, he received his Ph.D. degree in physical chemistry from Yale and in 1977, his M.D. degree from the University of North Carolina. He returned to Yale for a post-doctoral fellowship in human genetics after completing his internship and residency at North Carolina Memorial Hospital.

family trying to stir through these new developments. I'll return to this point later.

YALE MEDICINE Fall/Winter 1991-92

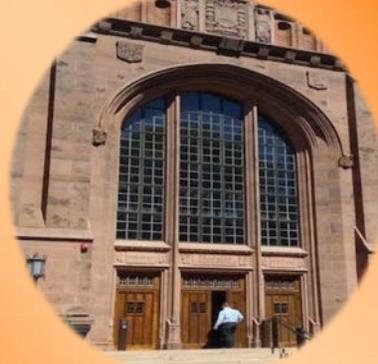
This article was based on an address Dr. Collins delivered to University of Michigan medical school graduates at the Honors Convocation, May 1990.

My Path to Medical Research





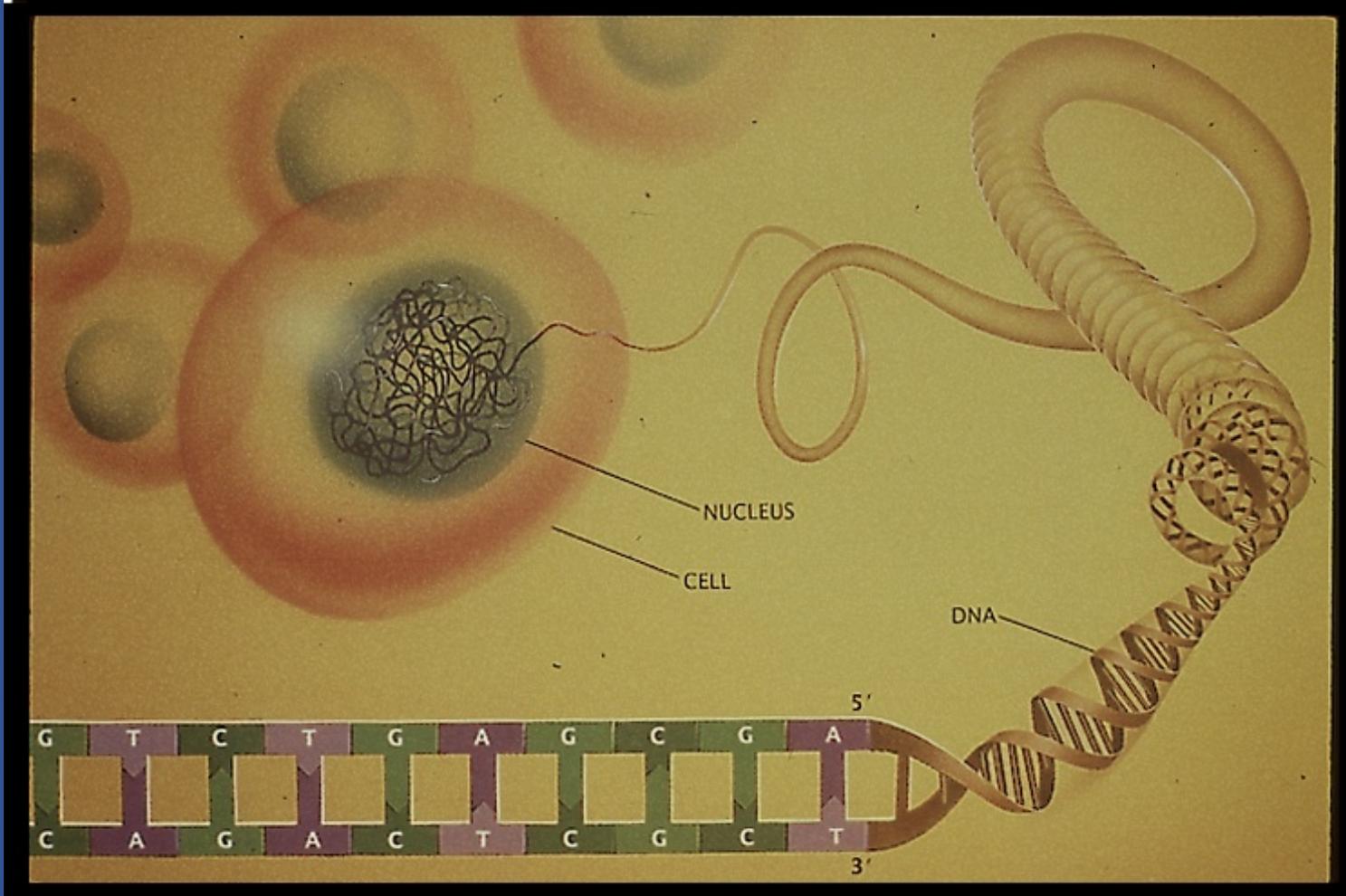
UNIVERSITY
of VIRGINIA



Yale



*PhD Thesis: Semiclassical
theory of vibrationally
inelastic scattering, with
application to $H^+ + H_2$ (1974)*





UNIVERSITY
of VIRGINIA




Yale
Yale University
School of Medicine



 | UNC
SCHOOL OF MEDICINE

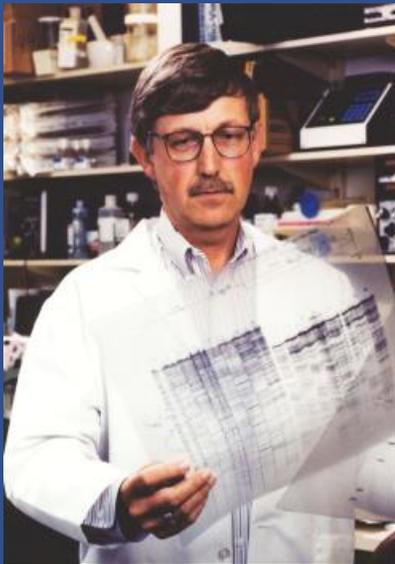


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MICHIGAN

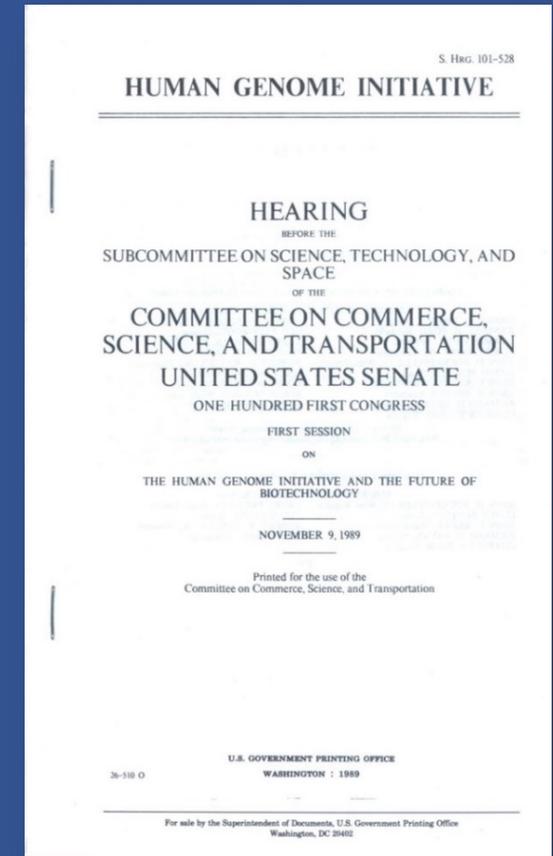
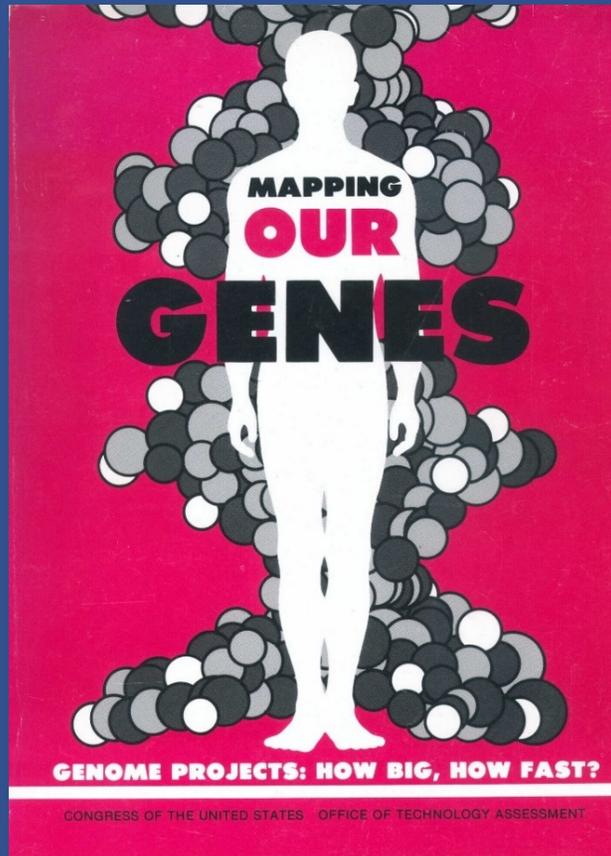
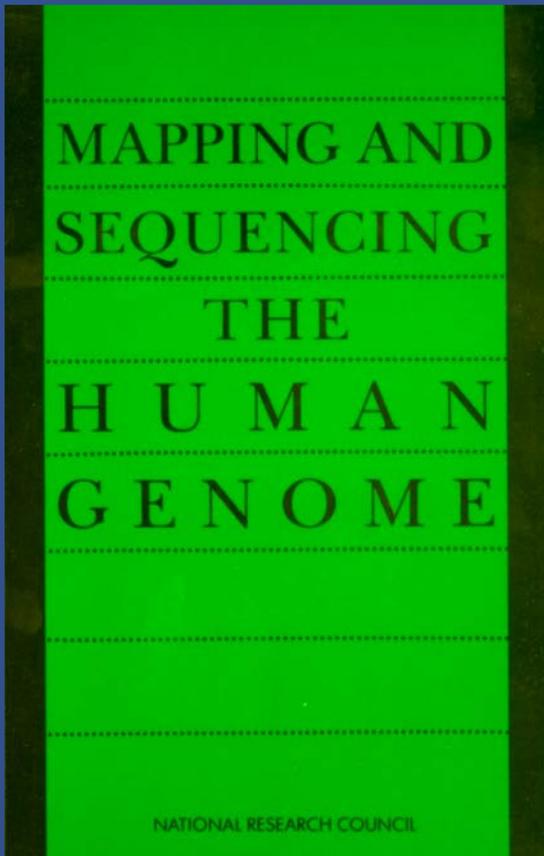


“Gene Hunter”

- Cystic fibrosis
- Huntington’s disease
- Neurofibromatosis
- Hutchinson-Gilford Progeria Syndrome



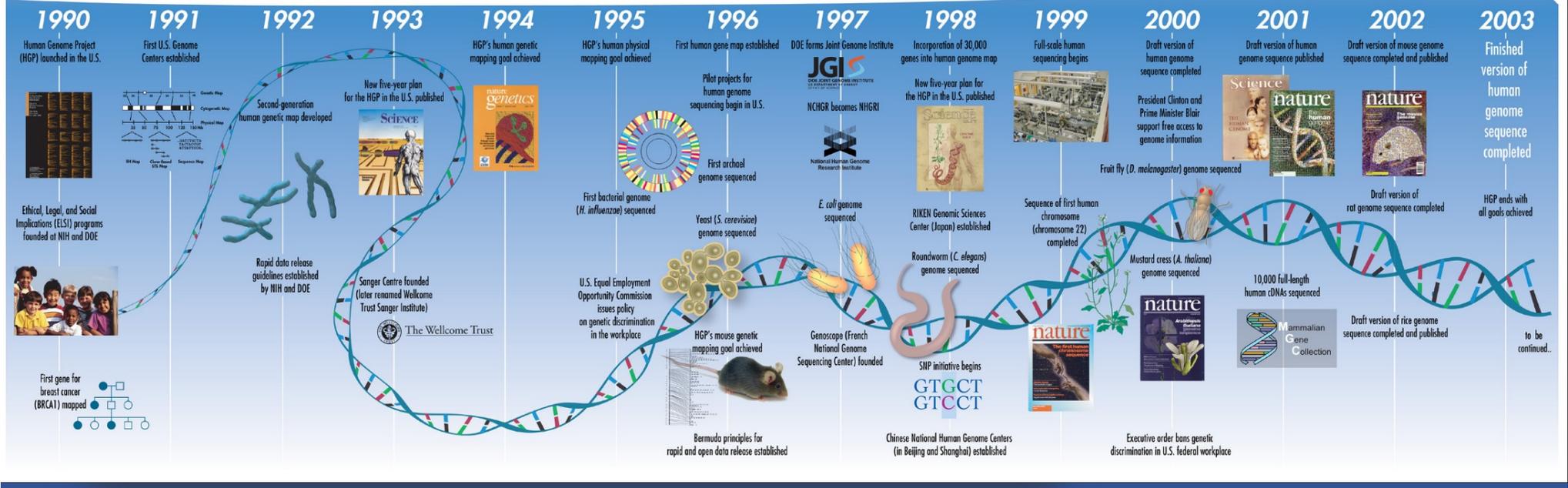
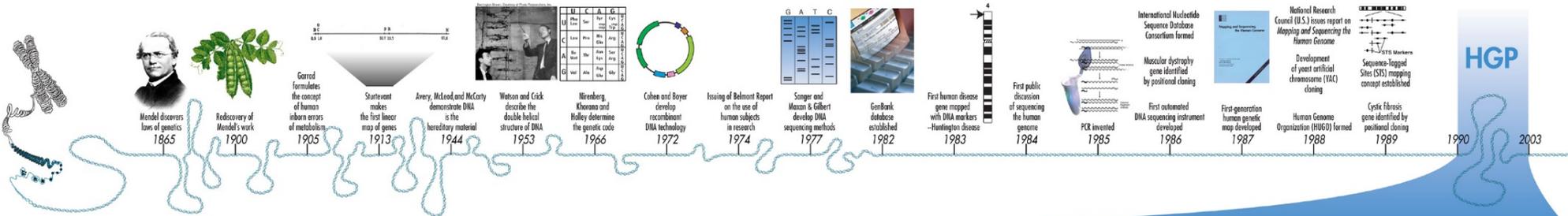
Gene Hunting: A Better Way?



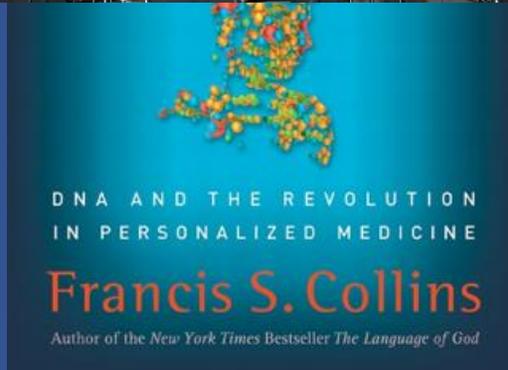
An Unexpected Call to Public Service: NIH (1993)



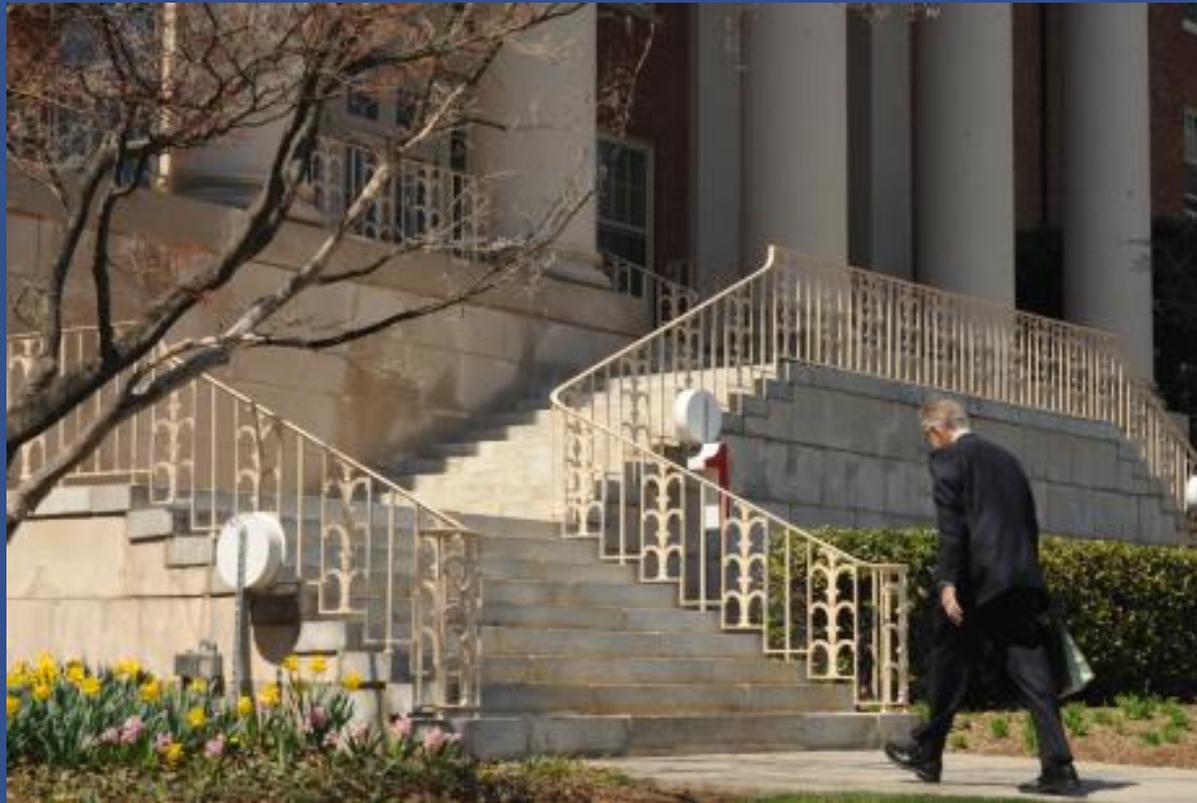
Leading the Human Genome Project



Took a Break from Public Service



Returned to NIH as Director in 2009



NIH: Steward of Medical and Behavioral Research for the United States



“Science in pursuit of **fundamental knowledge** about the nature and behavior of living systems ... and the **application of that knowledge** to extend healthy life and reduce illness and disability.”



NIH History



- **1887:** Dr. Joseph Kinyoun established “Laboratory of Hygiene,” Marine Hospital, Staten Island, NY
 - Became lab of U.S. Public Health Service
- **1930:** Ransdell Act: Hygienic Laboratory became National Institute (singular) of Health
- **1937:** National Cancer Institute established with sponsorship by every U.S. Senator
- **1940:** President Franklin D. Roosevelt dedicated buildings and grounds of Bethesda campus
- **1944:** Federal Funding Law authorized NIH grants program; clinical research
- **1948:** New Institutes → National Institutes of Health

NIH Today

- Conducts research in its own laboratories
- Supports research of non-Federal scientists
 - In universities, medical schools, hospitals, and research institutions throughout United States and overseas
- Helps train research investigators
- Fosters communication of medical information
- 153 NIH-supported researchers have become Nobel Laureates*



* As of 10/04/2017

NIH Clinical Center: The “House of Hope”

- Construction began in 1948 ... opened in 1953
- Today: world’s largest clinical research center
- Admits patients as part of clinical studies
 - Patients can be referred to a study by their physician or can self-refer

— Currently conducting ~1,500 clinical studies

- Winner of the 2011 Lasker-Bloomberg Public Service Award



NIH Clinical Center: Discovery's *First In Human*



Debut: August 4, 2017



NIH's Impact on U.S. Health and Medicine

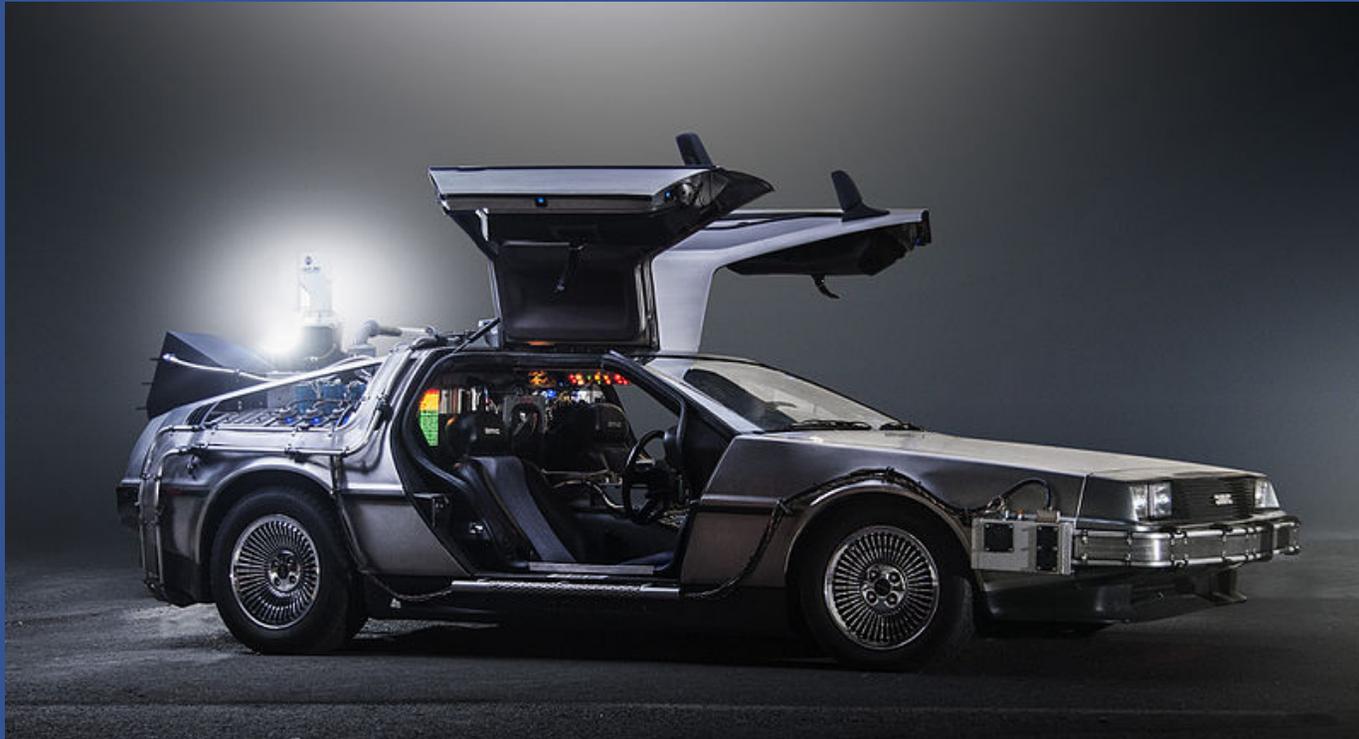
U.S. Life Expectancy



- Cardiovascular disease death rates have fallen more than 70% in the last 60 years
- Cancer death rates now falling more than 1% per year; each 1% drop saves ~\$500 billion
- HIV therapies enable people in their 20s to live to age 70+

Hope Through Biomedicine

Traveling Into the Future....

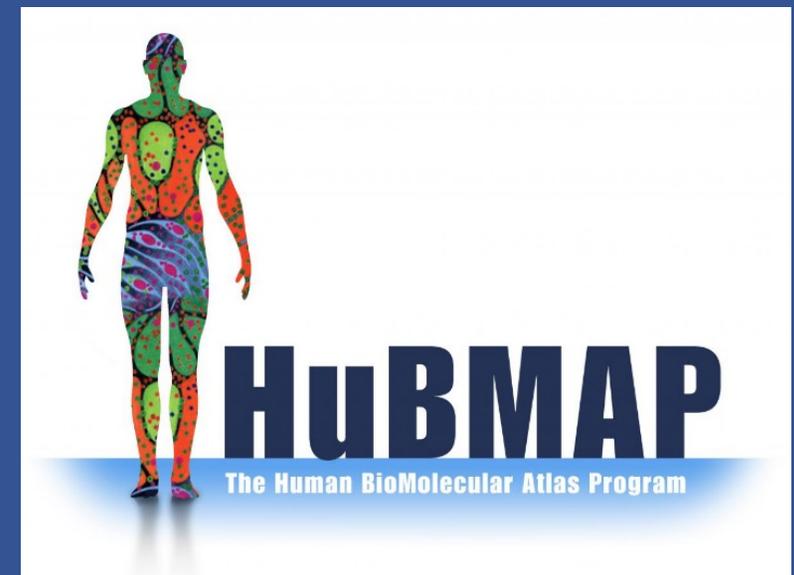


10 Biomedical Advances in 10 Years

In 10 Years, We Will Have...

Dramatically advanced our analysis of individual human cells

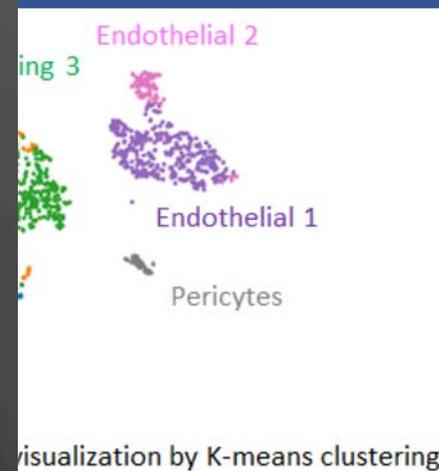
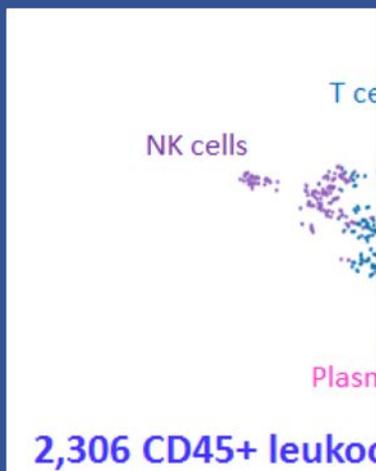
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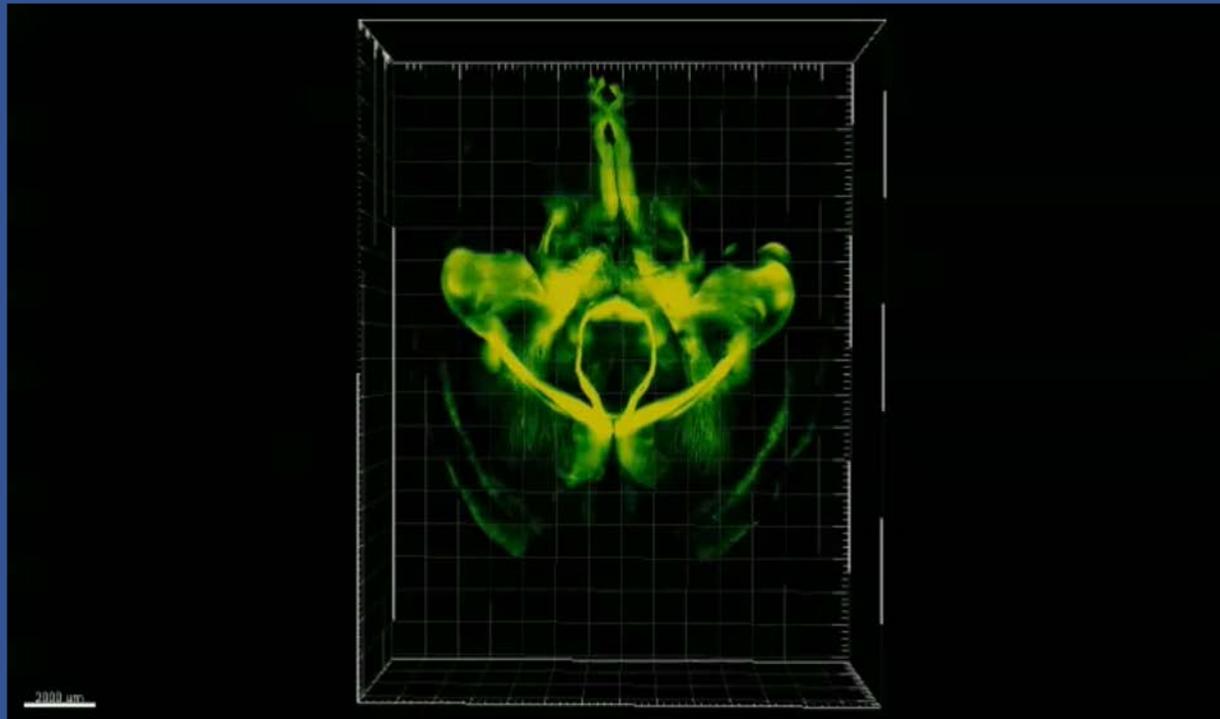


Increasing our understanding of many disorders, including autoimmune conditions like rheumatoid arthritis

In 10 Years, We Will Have...

Developed tools to identify new brain cell types, circuits

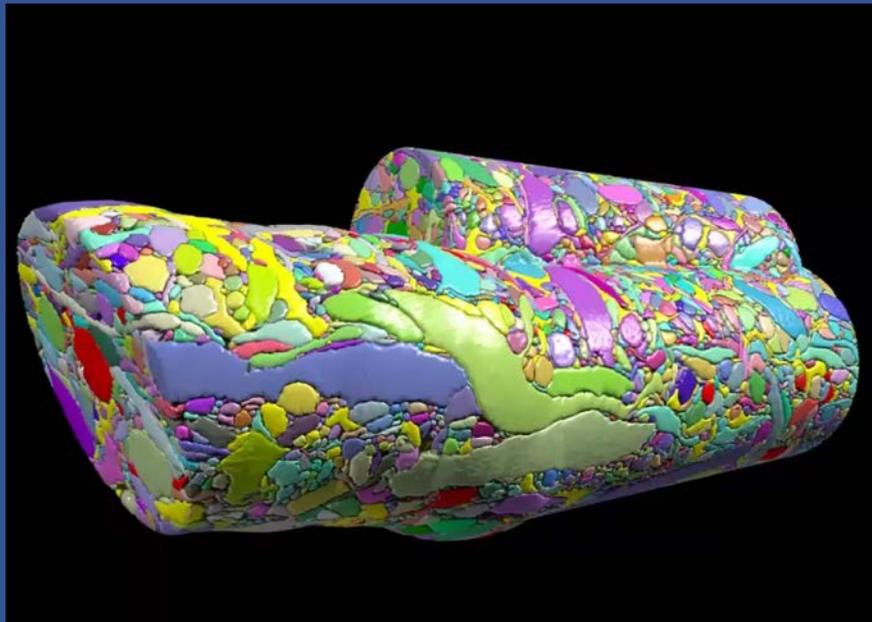
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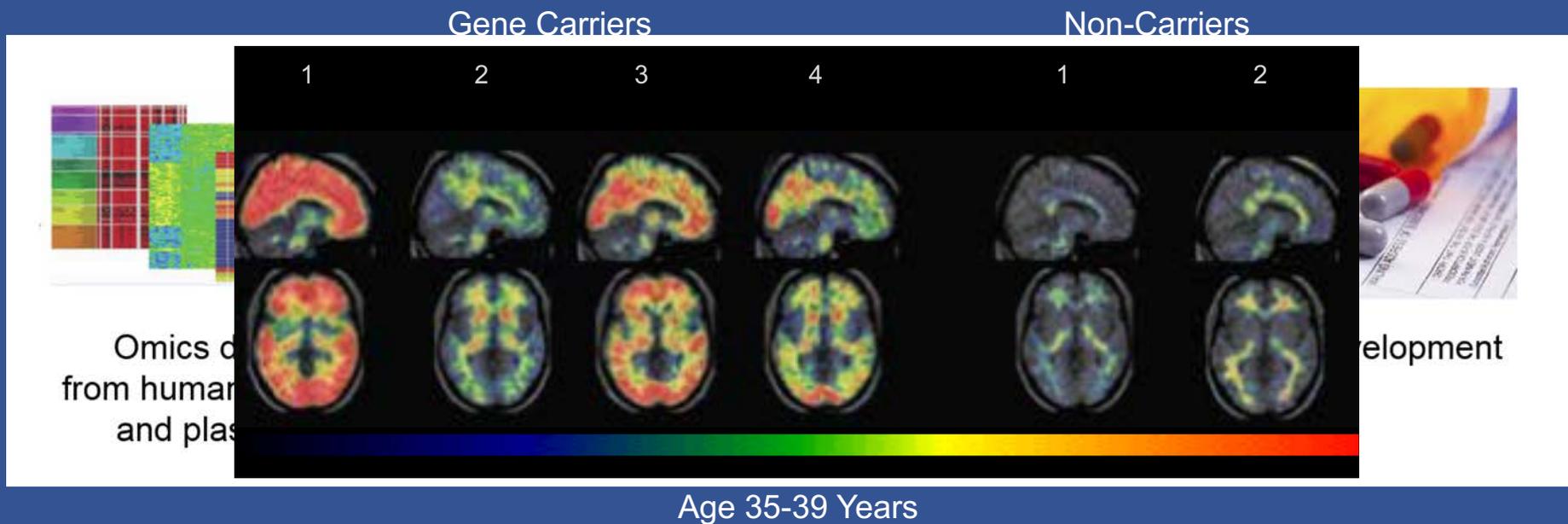
2



Improving diagnosis, treatment, and prevention of autism, schizophrenia, Parkinson's, other neurological conditions

In 10 Years, We Will Have...

Implemented targeted therapies for Alzheimer's *before* signs appear



Slowing or even preventing the disease

In 10 Years, We Will Have...

4

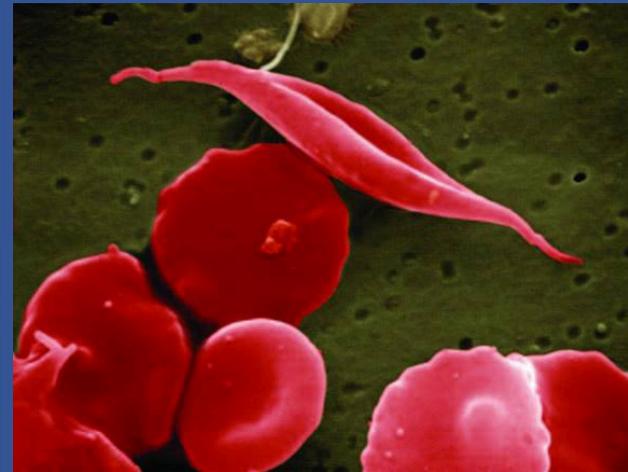
Gene therapy for rare diseases like Spinal Muscular Atrophy (SMA)



Preventing loss of motor function from SMA and other related inherited diseases

In 10 Years, We Will Have...

Harnessed the power of CRISPR-Cas and other gene editing tools

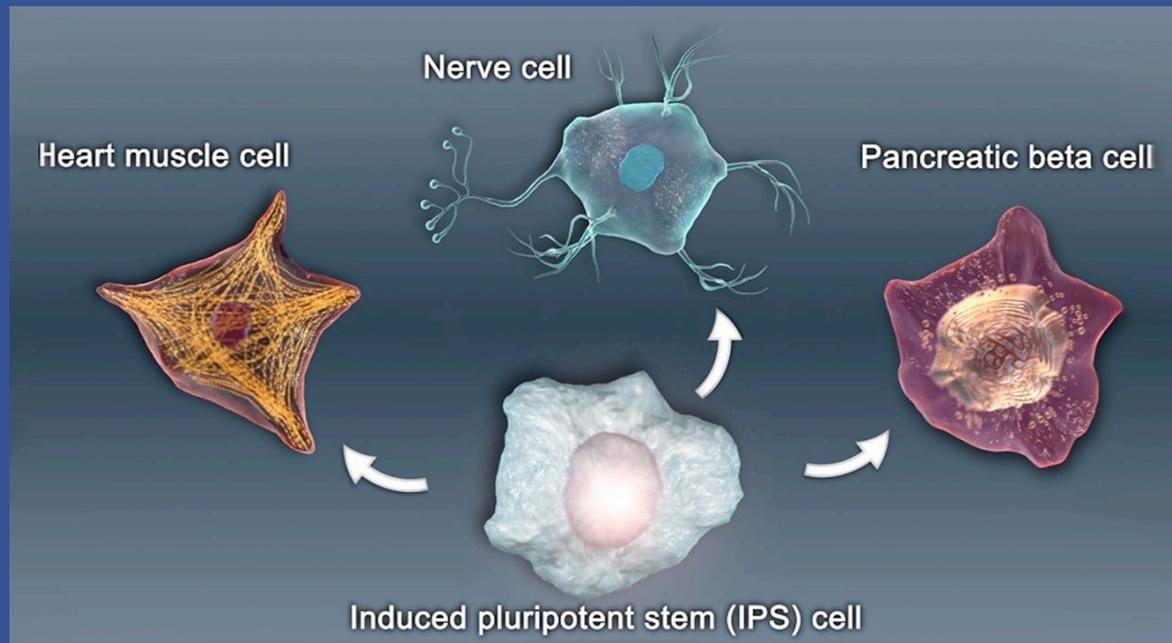


Curing Sickle Cell Disease first – and then many other rare diseases

In 10 Years, We Will Have...

6

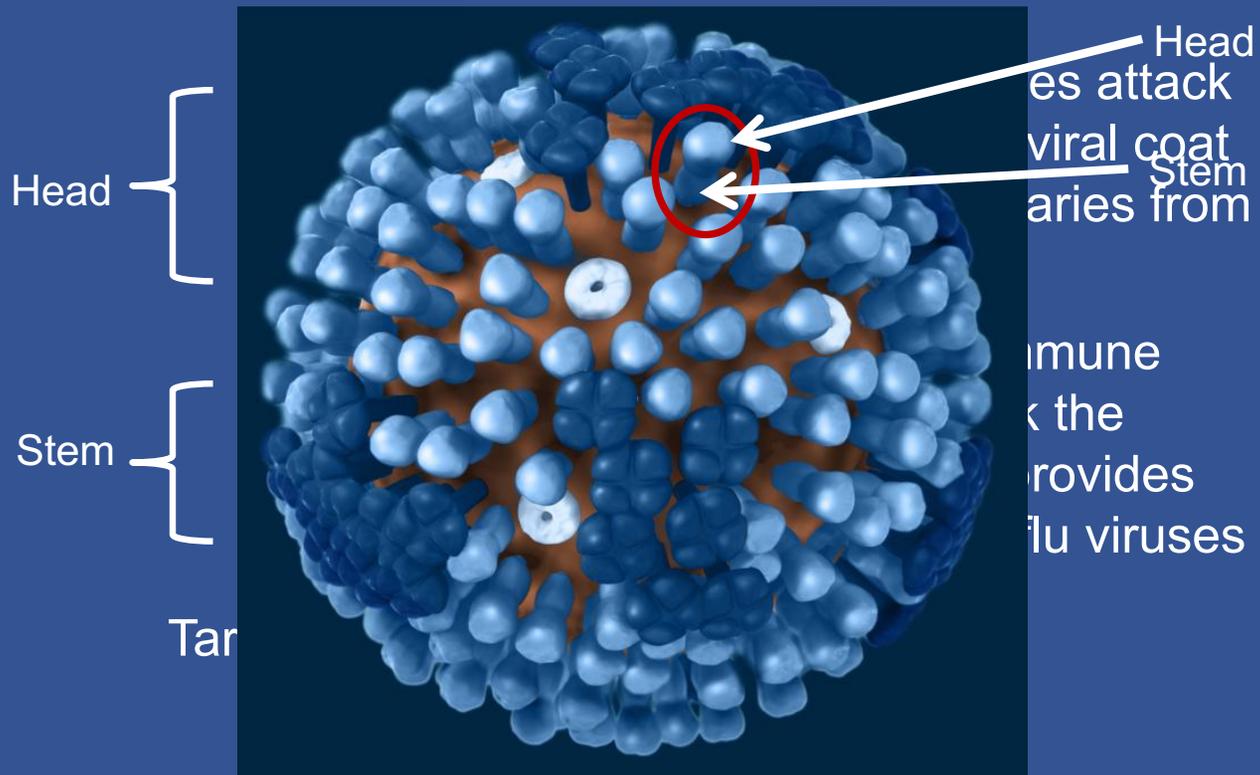
Deepened our understanding and application of iPS cells



Allowing us to build "YOU on a biochip" and generate matched transplantable organs

In 10 Years, We Will Have...

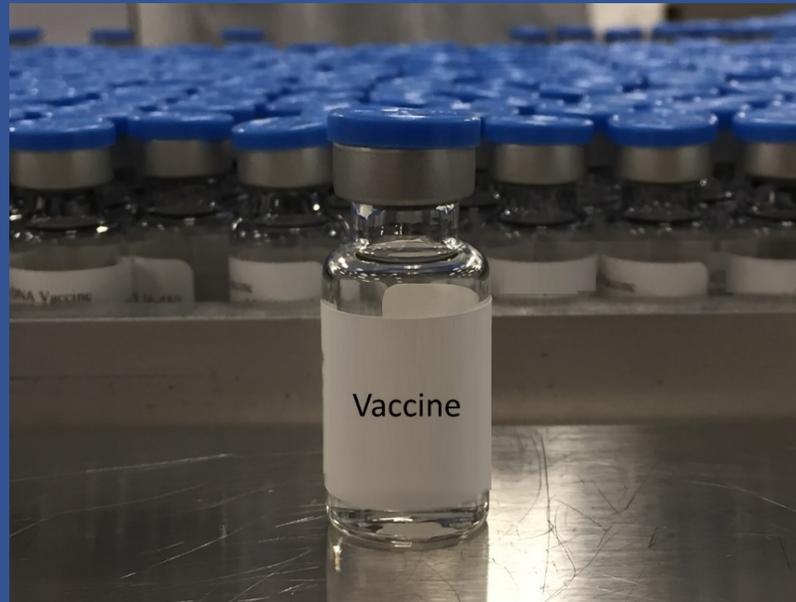
Created a universal influenza vaccine



In 10 Years, We Will Have...

7

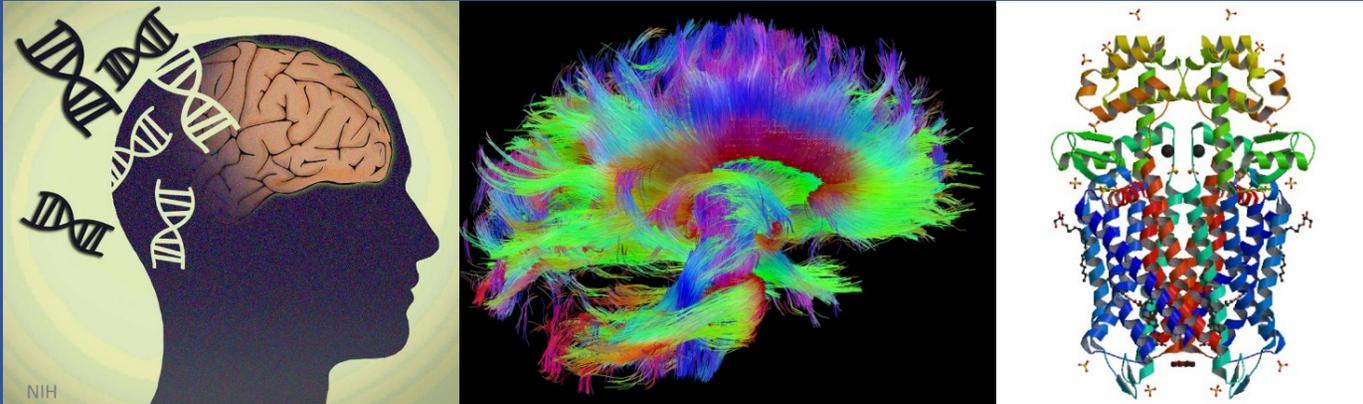
Created a universal influenza vaccine



Protecting against all strains of flu, seasonal and pandemic

In 10 Years, We Will Have...

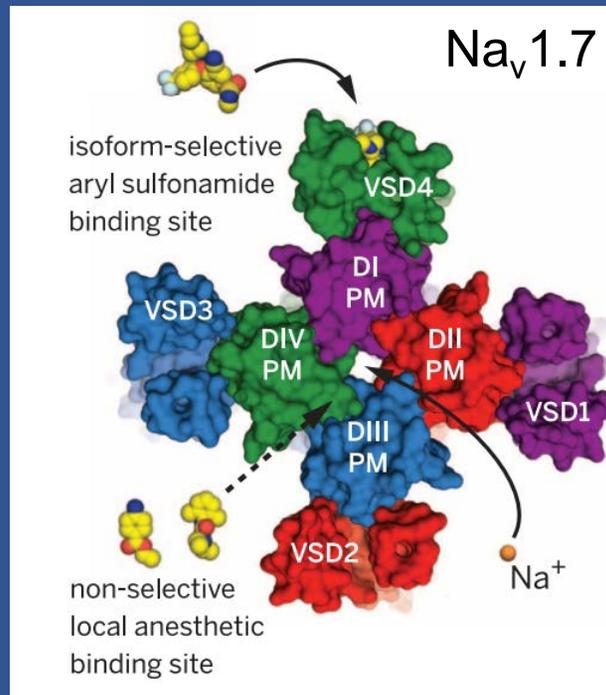
Deployed genomics, neuroscience, structural biology to uncover new targets for addiction prevention, treatment of pain



In 10 Years, We Will Have...

8

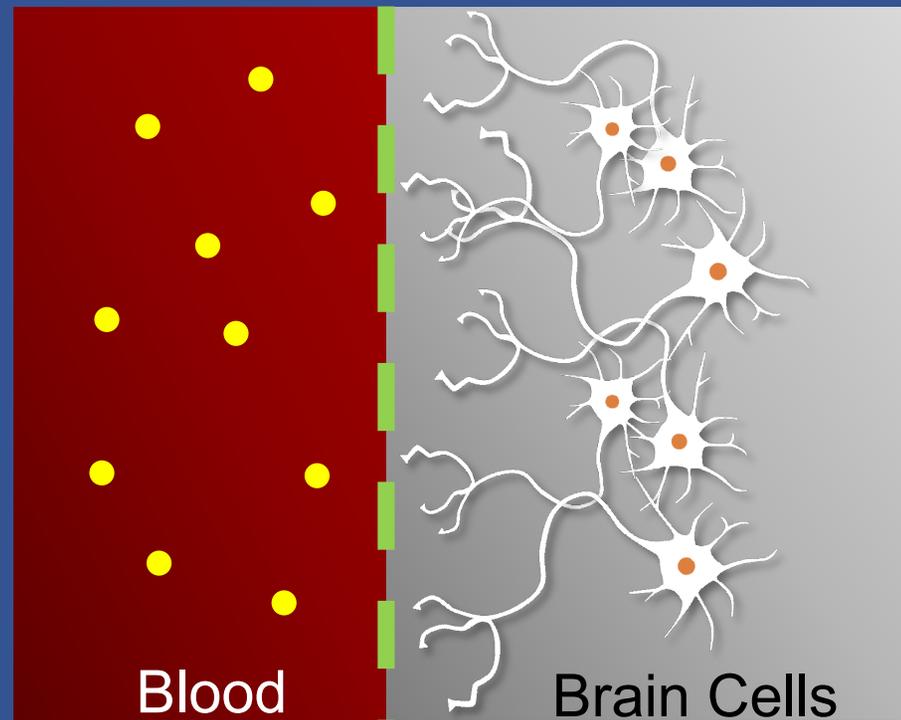
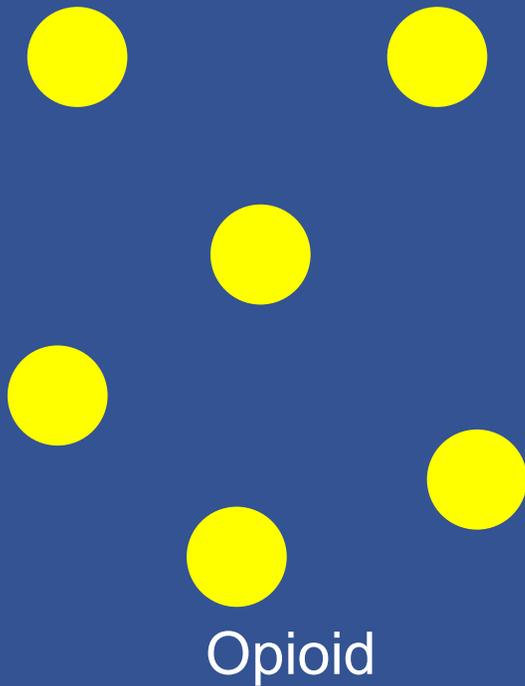
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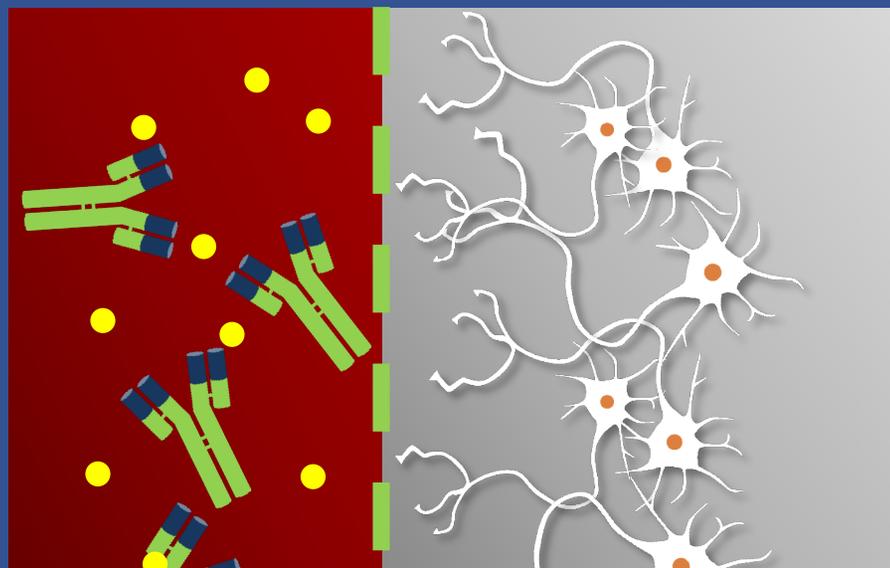
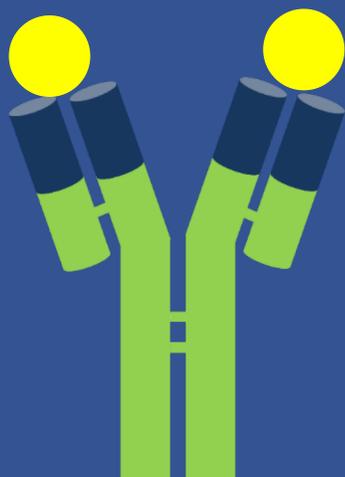


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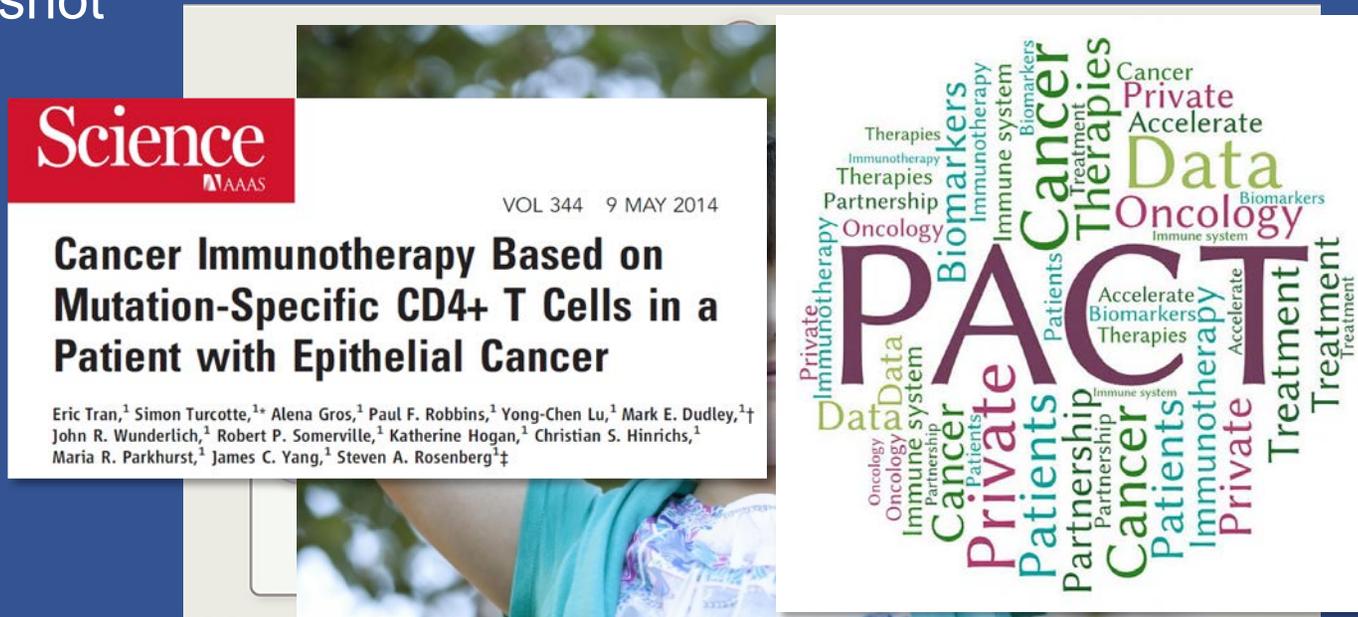
Opioid



Strategies that will end the opioid crisis – and lessen patients' pain

In 10 Years, We Will Have...

Accelerated immunotherapy, other advances through the Cancer Moonshot



Expanding the reach of immunotherapy to provide cures for solid tumors

In 10 Years, We Will Have...

10

Actualized the potential of precision medicine through the *All of Us* Research Program



Patient Partnerships



Electronic Health Records



Technologies



Genomics



Data Science

In 10 Years, We Will Have...

10

Actualized the potential of precision medicine through the *All of Us* Research Program



*Advancing individualized prevention, treatment, and care –
and YOU TOO can be One in a Million!*

In 10 Years, We Will Have...

10

Actualized the potential of precision medicine through the *All of Us* Research Program



USA TODAY

Pay it forward: Join with All of Us Research Program to build a healthier future

Alex M. Azar II and Francis S. Collins Published 7:00 a.m. ET May 7, 2018

By signing up for All of Us, you will help accelerate the growth of precision medicine. We will be able to deliver better health for every American.



www.joinallofus.org



Hope in every sphere
of life is a privilege that
attaches to action.
No action, no hope.

~ Peter Levi





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