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
“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.”
My Path to Medical Research
PhD Thesis: Semiclassical theory of vibrationally inelastic scattering, with application to $\text{H}^+ + \text{H}_2$ (1974)
“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

- 2,500 scientists
- 20 research institutions
- 6 different countries
- 13 years
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

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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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…

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...

Harnessing 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…

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

- Natural antibodies attack the head of the viral coat protein, which varies from year to year.
- Teaching the immune system to attack the invariant stem provides immunity to all flu viruses.
In 10 Years, We Will Have...

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
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In 10 Years, We Will Have...

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...

Actualized the potential of precision medicine through the *All of Us* Research Program
In 10 Years, We Will Have…

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…

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

[USA TODAY Article: Pay it forward: Join with All of Us Research Program to build a healthier future](https://www.usatoday.com/story/health/2016/05/07/all-of-us-precision-medicine-research/84761084/)

*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](http://www.joinallofus.org)
Hope in every sphere of life is a privilege that attaches to action. No action, no hope.

~ Peter Levi