#### Exceptional Opportunities in Biomedical Research

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

**Director, National Institutes of Health** 

Worcester Regional Research Bureau Annual Meeting







# 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:

#### World's Largest Supporter of Biomedical Research

- Conducts research in its own laboratories
- Supports research of non-Federal scientists
  - Universities, medical schools, hospitals, research institutions, etc.
- Helps train research investigators
- Fosters communication of medical information
- 158 NIH-supported researchers have become Nobel Laureates

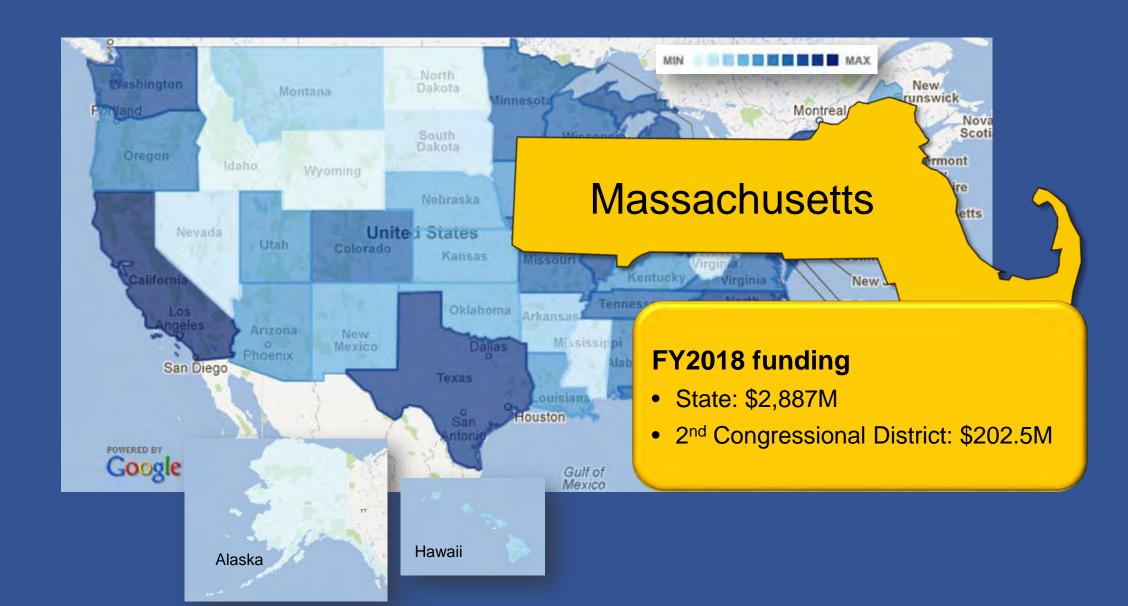
#### 2019 Nobel Prize in Physiology or Medicine







#### NIH Funds Scientists Across U.S.



#### 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
  - Dropping by ~26% from 1991 to 2015
- HIV therapies enable people in their 20s to live to age 70+

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#### **Economic Value of NIH-Supported Research**

- NIH funding supports >433,000 high quality jobs across the US
  - Producing ~\$74 billion in new economic activity
- Every \$1 of NIH investment → ↑ in R&D investment:
  - Basic science → \$8.38 ↑ in 8 years
  - Public clinical research → \$2.35 ↑ after 3 years
- Example: Human Genome Project (1990–2003)
  - Cost: \$3.8B (on time & under budget!)
  - 1988–2012: →  $\sim$ \$1 trillion  $\uparrow$  in economic growth
    - 178-fold return on investment



# Hope Through Biomedicine Traveling Into the Future....



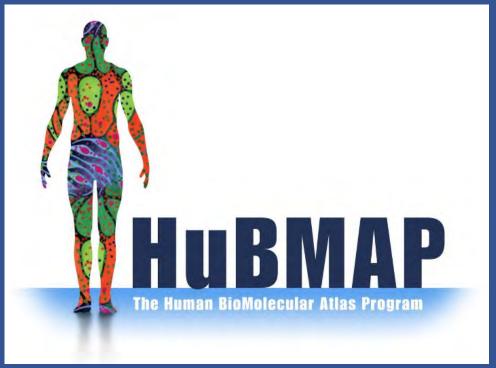
10 Biomedical Advances in 10 Years

# 1

#### In 10 Years, We Will Have...

Dramatically advanced our analysis of individual human cells





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Dramatically advanced our analysis of individual human cells

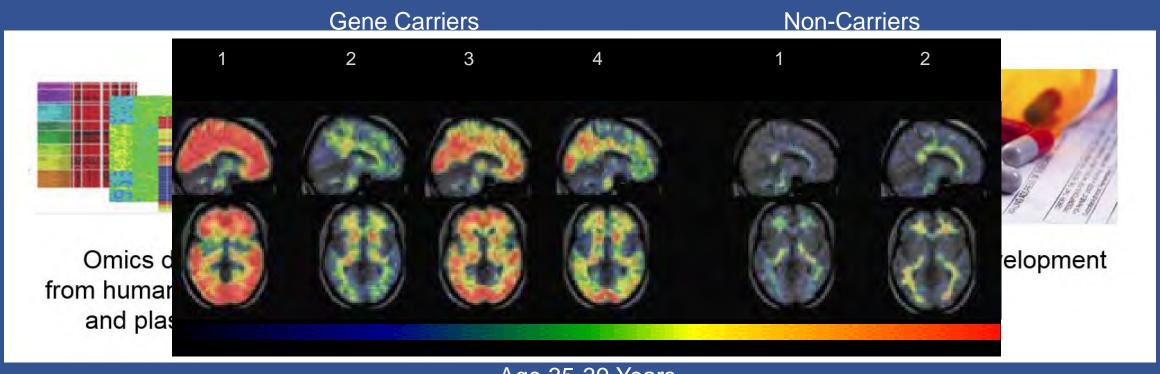


Increasing our understanding of many disorders, including autoimmune conditions like rheumatoid arthritis

Developed tools to identify new brain cell types, circuits

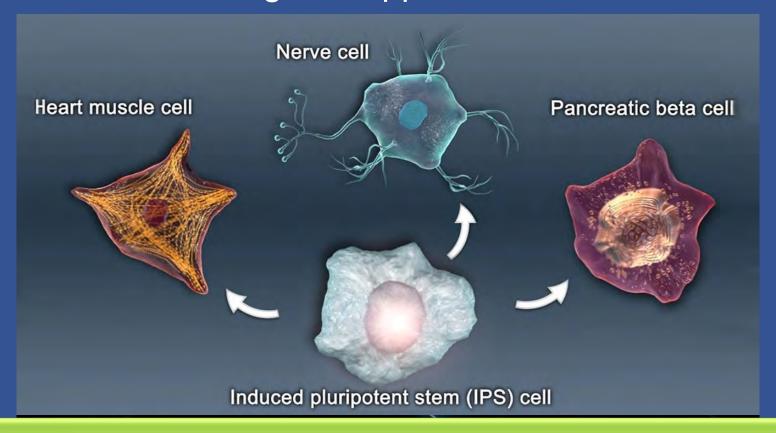
Improving diagnosis, treatment, and prevention of autism, schizophrenia, Parkinson's, other neurological conditions

Implemented targeted therapies for Alzheimer's before signs appear



Age 35-39 Years

Deepened our understanding and application of iPS cells



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

#### Expanded our concept of our "self" to include our microbiome

- broadens possibilities for study of humans First-gen DNA and microbes sequencing
- NIH launches Human Microbiome Project
- Sequences 3,000 reference strains from human body
- Microbiome Project deepens understanding of microbiome-associated

 International Human conditions

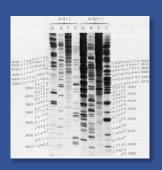
1970s

1990s

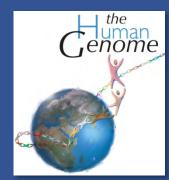
DNA sequencing

2008-2013

2014-2018

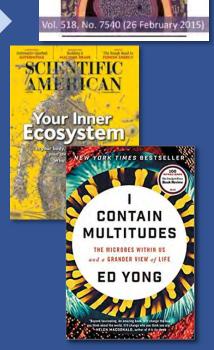












Innovations®



Expanded our concept of our "self" to include our microbiome



Posted on July 14th, 2015 by Dr Francis Collin

#### **NIH** Director's Blog

Fundamental Knowledge of Microbes Shedding New Light on Human Health

Posted on May 29th, 2019 by Dr. Francis Collins



Targeting the Microbiome to Treat Malnutrition

earch in biolog impossible to most always



#### NIH Director's Blog

You Won't Believe What's In These Pills

Posted on October 21st, 2014 by Dr. Francis Collins



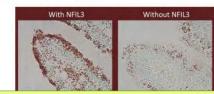
3 ohmann lab

um difficile or more commonly "C. diff" is a nasty bacterium that claims the lives of 14,000 at at risk are people with conditions requiring prolonged use of antibiotics, which have the wiping out the natural good bacteria in the colon—thus allowing bad bugs like C. diff to ned. In many folks, C. diff infect



Protein Links Gut Microbes, Biological Clocks, and Weight Gain

Posted on September 12th, 2017 by Dr. Francis Collins



Some 'Hospital-Acquired' Infections Traced to Patient's Own

Posted on October 23rd, 2018 by Dr. Francis Collins

Microbiome

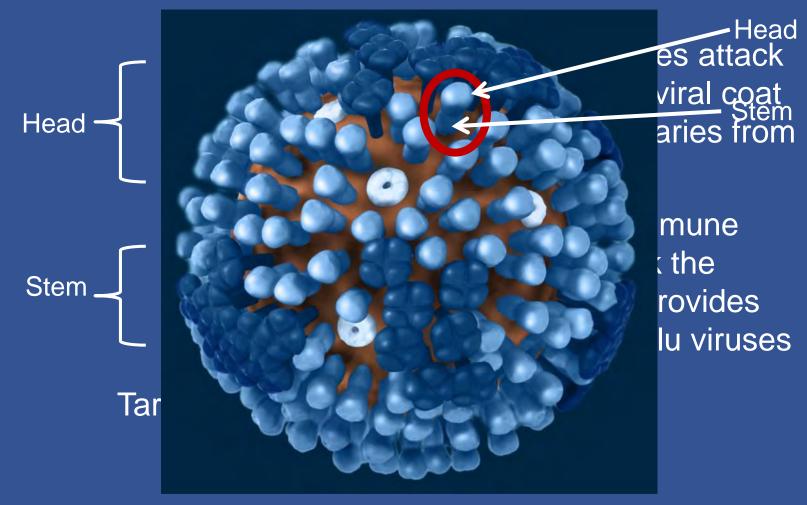


urce of a hospital-acquired bloodstream infect , Palo Alto, CA

people develop potentially life-threatening s with microbes lurking on medical equipme ly that is often true. But now an NIH-funded quired' infections may actually stem from a

With profound implications for our approaches to sickness and health

Created a universal influenza vaccine

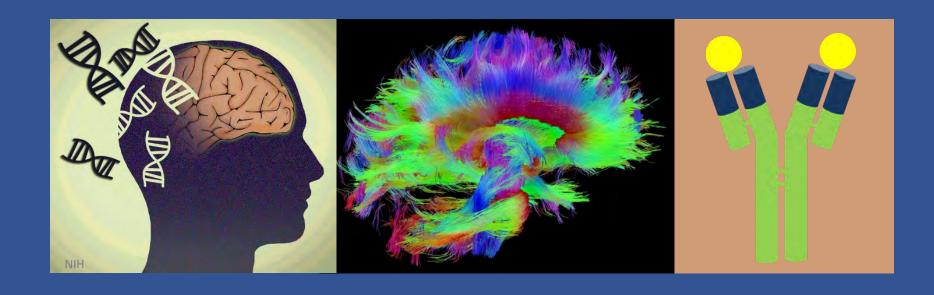


Created a universal influenza vaccine



Protecting against all strains of flu, seasonal and pandemic

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

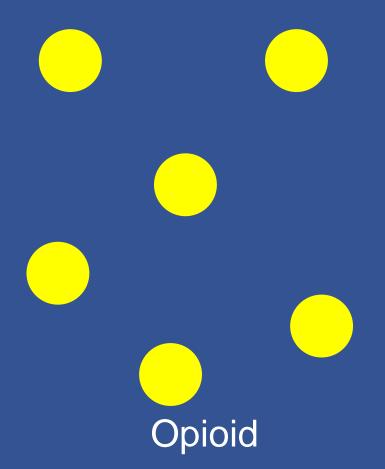


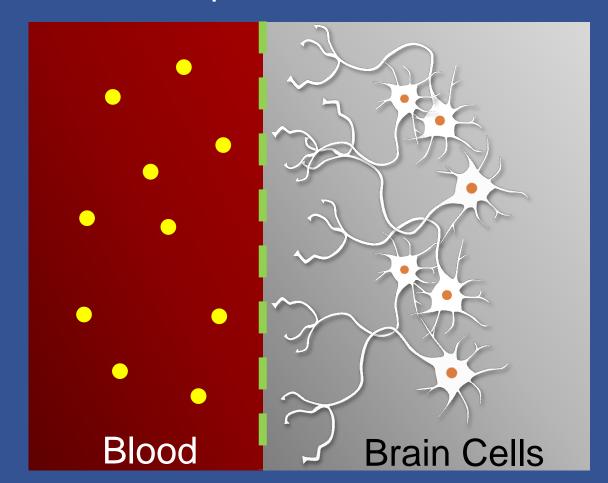
Deployed genomics, neuroscience, structural biology to uncover new targets for addiction prevention, treatment of pain



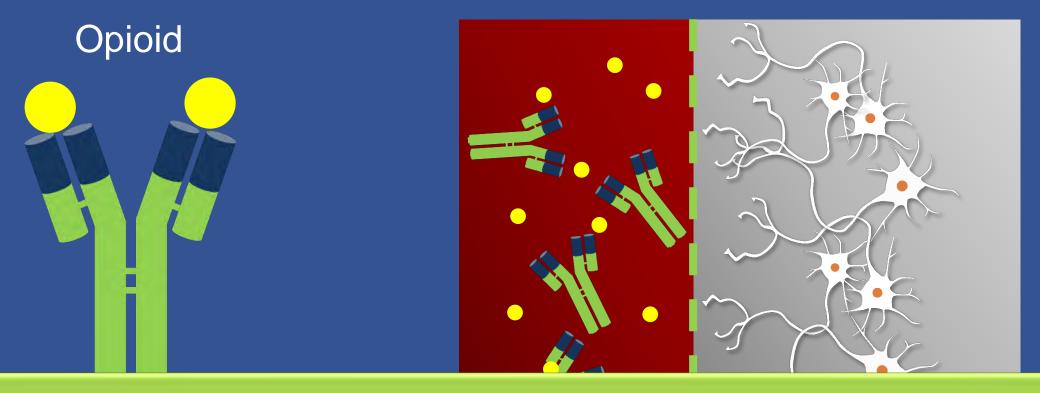
- NIH-led research initiative to:
  - Improve prevention and treatment of opioid misuse, addiction
  - Enhance pain management
- ~\$945M (FY19) in research funding
  - ~375 awards in 41 states
- Coordinated effort across sectors: from government agencies to local communities

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





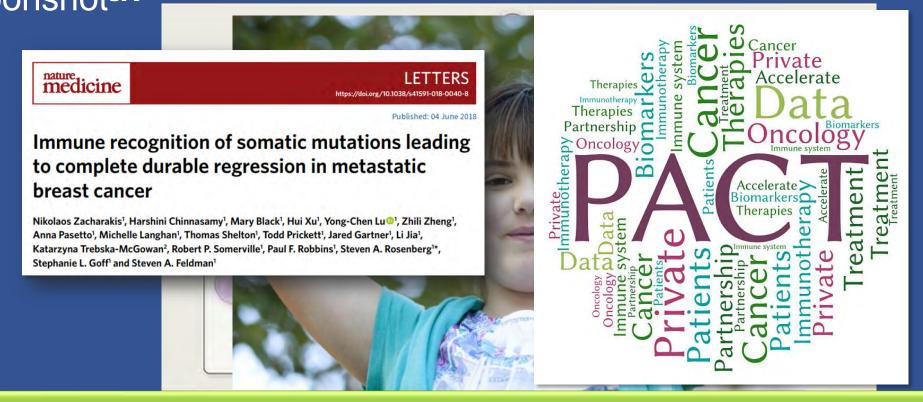
Deployed genomics, neuroscience, structural biology to uncover new targets for addiction prevention, treatment of pain



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

8

Accelerated immunotherapy, other advances through the Cancer Moonshot<sup>SM</sup>



Expanding the reach of immunotherapy to provide cures for solid tumors

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



Patient Partnerships



Technologies



Electronic Health Records



Genomics



Data Science

9

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



Actualized the potential of precision medicine through the All of Us

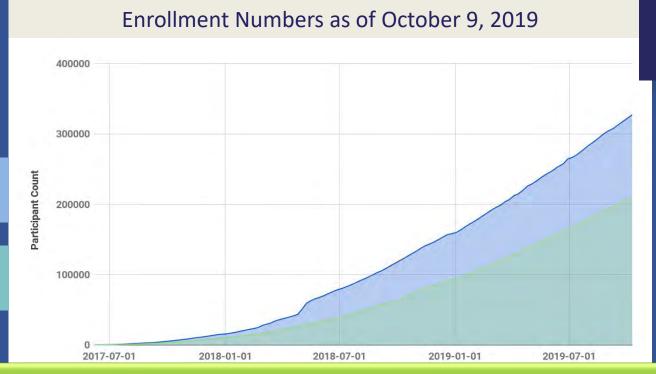
Research Program

326,976

Started enrollment

209,518

Completed all steps

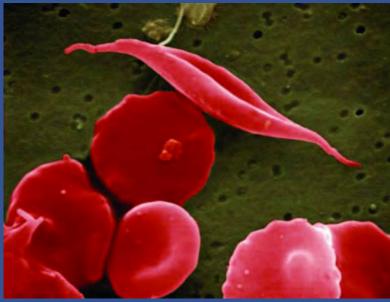


www.joinallofus.org

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

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





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



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



### Gene Therapy for Sickle Cell Disease







# COULD GENE THERAPY CURE SICKLE CELL ANEMIA?

An NIH clinical trial is ushering in a genetic revolution as an innovative type of gene therapy is used to attempt to cure sickle cell anemia.

MAR 10

JONATHAN LAPOOK



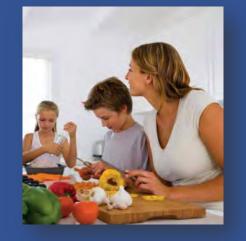


Offering effective cures for all individuals with SCD











Turning Discovery Into Health www.nih.gov/hope

directorsblog.nih.gov

