

A CONVERSATION WITH FRANCIS COLLINS DIRECTOR, NATIONAL INSTITUTES OF HEALTH

Dr. Francis Collins is a physician-geneticist noted for his landmark discoveries of disease genes and his leadership of the international Human Genome Project, which culminated in April 2003 with the completion of a finished sequence of the human DNA instruction book. He served as director of the National Human Genome Research Institute at NIH from 1993–2008. He was appointed NIH Director by President Barack Obama in 2009 and selected by President Donald Trump in 2017 to continue to serve in this role.

Below is an excerpt from the conversation with Dr. Collins edited for length.

Can you talk about some of the things that are on the cusp of being possible today because of biomedical research that maybe even five or ten years ago we wouldn't have imagined possible?

This is the part of my job that's so fun — looking across the landscape of biomedical research at what's becoming possible — so I'll give you three.

First, [The BRAIN Initiative](#). The idea is that, with the development of new technologies, we might be able to figure out how the brain actually works — how those circuits in our 86-billion-neuron brain are able to do amazing things. By 2025, we aim to have uncovered some profoundly significant features of how the brain works. For instance, how does a memory get laid down? How do you retrieve it? I believe we'll be seeing these kinds of things emerging in the next seven or eight years.

Second, I'm very excited about the potential of new genetic opportunities to treat illnesses that seemed out of reach — particularly, gene editing with the advance called [CRISPR-Cas](#). With this system, we can go in and fix a single letter in the DNA code. I'm most excited about the application of this to sickle cell disease. As a post-doc, I worked on sickle cell disease. Back then, it was very uncertain whether there would be a treatment in my lifetime. But look at where we are now.

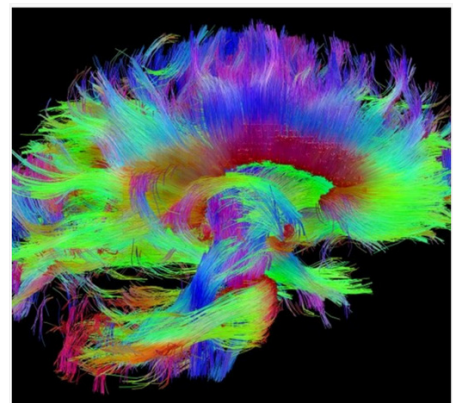
CONTINUED ON BACK >



BRAIN INITIATIVE

Aims to develop a detailed understanding of the 86 billion neurons in the human brain. Alzheimer's disease alone has an estimated economic cost of \$259 billion this year and may exceed \$1.1 trillion by 2050. The hope is to develop tools and technologies to identify someone at risk for Alzheimer's or other mental diseases, and use such "advance warning" to develop effective ways of disease prevention.

NIH



FRANCIS COLLINS

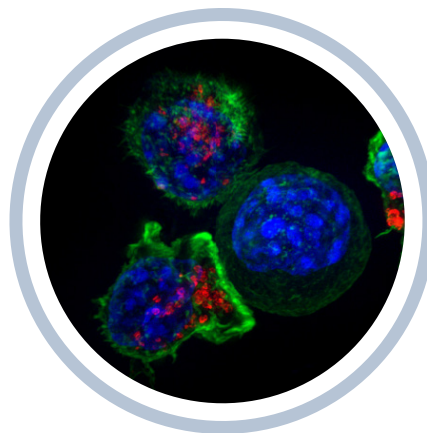
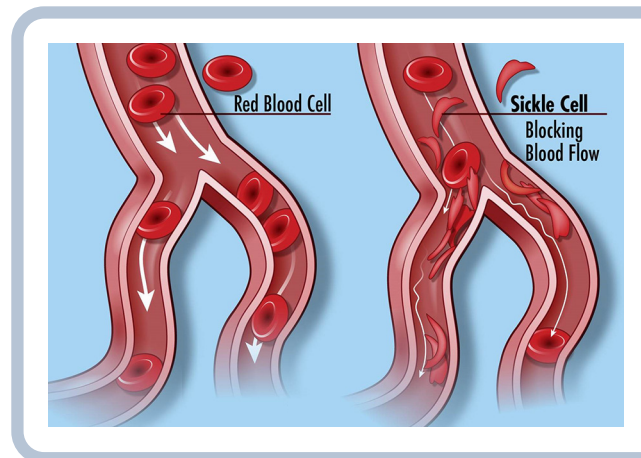
CONTINUED

We know a lot about this disease. We know it affects the bone marrow. We know how to take bone marrow cells out of the body and work on them. So here's the strategy: take bone marrow from somebody with sickle cell disease, purify those hematopoietic stem cells, utilize the magic of CRISPR-Cas to fix that mutation, expand those cells, make some room in the bone marrow by giving somebody a moderate ablation protocol, and then give them back their now cured, no longer sickle cell, red cells. These are the patient's cells. It's not a situation where one would need immunosuppression for the rest of their life. I think this is going to work in the next five years. I think we're going to cure this disease — not have a treatment that sort of works, but a cure.

Third, because it's so breathtaking in its sweep, is cancer immunotherapy. And, particularly, that this approach might also work for those solid tumors that have seemed resistant to [immunotherapy](#) — pancreatic cancer, prostate, breast, ovarian cancer, brain cancers. Recent developments, especially by [Steve Rosenberg](#) here at NIH, make me optimistic that that pathway is beginning to take shape. And what a phenomenal set of advances that could be for people who have metastatic disease, for a solid tumor, like pancreatic cancer, to have the chance of being not just treated but cured.

The entire conversation with Dr. Collins, covering the All of Us research initiative, NIH's work to combat the opioid epidemic, and new advances made possible by biomedical research, is available for listening and downloading at www.unitedformedicalresearch.org/amazing-things.

PHOTOS COURTESY OF NATIONAL INSTITUTES OF HEALTH



United for Medical Research has undertaken the Amazing Things Podcasts because America's investment in medical research — through the National Institutes of Health — is making amazing things possible. Listen to the full conversation with Dr. Collins at www.unitedformedicalresearch.org/amazing-things.



www.unitedformedicalresearch.org