SOUTHWESTERN MEDICAL

# PERSPECTIVES

2019 A PUBLICATION OF SOUTHWESTERN MEDICAL FOUNDATION

# THE CRISPR REVOLUTION

## The gene-editing tool behind an unprecedented launch of hopes and dreams for humanity

A T
C G

# Dr. Francis S. COLLINS

Dr. Collins, a physician-geneticist noted for his landmark discoveries of disease genes and his leadership of the international Human Genome Project, is the Director of the National Institutes of Health (NIH). In that role, he oversees the work of the world's largest supporter of biomedical research.

## TEAMING BOLDNESS WITH RESPONSIBILITY

Scientists have identified the molecular causes of nearly 6,500 human diseases, yet treatments currently exist for only about 500. Within the next 10 years, biomedical researchers, disease advocates, and clinical experts will seek to realize the promise of new technologies to treat or even cure many conditions that once seemed out of reach. Particularly exciting in this arena is the potential of CRISPR genome-editing systems. Yet, as research moves boldly forward in this fast-paced field, it is imperative that our pursuit of cures also proceeds responsibly.

> "Among NIH's highest priorities is research aimed at using somatic cell genome editing to treat or cure life-threatening inherited disorders."

The promise of gene editing is great. But at the National Institutes of Health (NIH), the world's leading public supporter of biomedical research, we are among the many who think that, for the time being, the only CRISPR therapeutic strategies that should be pursued in humans involve somatic cell genome editing. In this approach, genetic material is edited only in relevant tissues without the risk of passing those changes on to future offspring. Among NIH's highest priorities is research aimed at using somatic cell genome editing to treat or cure life-threatening inherited disorders, such as sickle cell disease and muscular dystrophy.

Germline genome editing is quite a different story: NIH strongly opposes the use of genome editing in heritable cells in humans for the foreseeable future. We contend that society is not ready – and potentially may never be ready – for this approach, which will irreversibly alter the human DNA instruction book in ways that will have consequences for future generations of humankind.

Based on this concern, the NIH supports recent calls for an international moratorium on the clinical use of human germline genome editing. A moratorium period of five years or more could give scientific, economic, and thought leaders around the globe an opportunity to engage in serious discussions about the safety, ethical, philosophical, and theological concerns raised by this application of CRISPR technology. It is a debate that, while difficult, we simply cannot afford to postpone. The consequences of failing to provide an international moratorium are highlighted by the recent CRISPR editing experiments in human embryos that took place in China. Should such epic scientific misadventures continue to proceed, a technology with enormous potential for the prevention and treatment of disease could become overshadowed by justifiable public outrage, fear, and disgust.

As with many other emerging technologies, it will take much thoughtful deliberation by all sectors of society to weigh CRISPR's exceedingly bright promise for cure of cancer and genetic diseases against its potentially devastating pitfalls when applied to the germline. Yet, we are confident that the biomedical research community will rise to the challenge by standing up for what is both bold and ethical on behalf of the hundreds of millions of people all around the world who are still awaiting cures.