Gene Editing - We Can, But Should We?

A deeper look at the ethics of scientific breakthroughs.

Stories of scientists manipulating genes have flooded news channels lately. With them come a steady stream of speculation on the potential miracles — and disasters — of what is perhaps the most revolutionary biological breakthrough of the past two decades. Now, most people have formed an opinion about gene editing — without truly knowing what it is or how it works.

For some, gene editing is a welcomed medical technique that could help eradicate many diseases.

For others, it’s simply dangerous, immoral, a method that in the wrong hands can spell big trouble for mankind.

“This is not a new debate,” says Rosario Isasi, an expert in comparative law and ethics regarding genomics with the University of Miami Health System. “For the past 40 years or more, we’ve been asking ourselves the question: What should we do in our quest to offer relief from human suffering?”

Our ability to use genetic technologies as a “fix,” she adds, revives an age-old dilemma. “Just because we can do it doesn’t mean we should.”

Gene editing technology allows scientists to delete, insert, or replace the DNA in a gene. This is a big deal because DNA — that double helix deoxyribonucleic acid that looks like a twisted ladder — is where all our genetic information is stored. Proteins found there govern how our body functions and develops. Hence, being able to
change that information in any way has many consequences, both physical and ethical ones.

Most people’s familiarity with gene-editing involves the more recent technology of CRISPR-Cas9. CRISPR stands for clustered regularly interspaced short palindromic repeats. Originally a defense mechanism in certain kinds of bacteria, scientists have been able to engineer an enzyme named Cas9 to act like molecular scissors and cut strands of DNA. The first scientific paper on CRISPR was published in early 2013, and since then the method has used bacterial enzymes to target and cut specific sections of DNA. UHealth researcher Dr. Jonathan Schatz and his team of scientists, for example, have employed CRISPR to “cut and paste” specific chromosomal changes in blood stem cells. These cells are then transplanted into mice to eventually cause lymphoma tumors for researchers to study.

CRISPR techniques also have been used on crops and on mosquitoes. But before CRISPR, there were lesser-known genome editing tools. The oldest gene-editing method is zinc finger nuclease (ZFN) technology. Developed in the 1990s, ZFNs can cut an original DNA sequence entirely or remove and then insert a new DNA sequence in place of the old. Another gene-editing technology is known as TALEN, or transcription activator-like effector nuclease. Like ZFN, it can delete targeted DNA sequences or cut and paste new ones.

The discovery of CRISPR changed the landscape, however, because CRISPR “is more flexible, more precise and simpler to use than the other technologies,” says Isasi. “It’s also more affordable, so it has opened the field” of genome editing.

And therein lies the rub.

**How will the limitless potential of CRISPER be applied?**
Will it be used only to alter disease-causing genes or will it serve to enhance genes to create super creatures — including humans? Or worse: will it fall into the hands of terrorists who could then unleash a biological attack?

“This is another example of how science is a click or two away from ethics and government,” explains Professor Ken Goodman, founder and director of the Institute for Bioethics and Health Policy at the University of Miami Miller School of Medicine. In other words, experimenting with new technologies continues to surpass the speed with which we can figure out the consequences.

Much of the ongoing debate about gene editing has centered on the difference between two types of cells. Germline gene editing involves changing genes that will be inherited. The name comes from germ cells, which include egg and sperm cells that combine to form an embryo. Somatic cell editing, on the other hand, involves altering genes in cells that aren’t related to reproduction and therefore do not impact offspring. While the U.S. and many others countries restrict germline editing, this doesn’t mean scientists aren’t pushing ethical boundaries elsewhere.

Last year, a Chinese scientist revealed that he had use the technology to make a pair of twin girls resistant to HIV. The announcement shocked the world because it has been an accepted tenet that this technique is not ready to be used on humans.

“This is certainly off the books and it erodes confidence in the scientific enterprise,” Goodman notes.

In reaction to this development in China, the National Institutes of Health and top science and ethics experts from around the world called for an international five-year moratorium on editing human germlines. Shortly thereafter, a World Health Organization advisory committee recommended that a central registry be established for both somatic (non-heritable) and germline genetic cell research. The
committee also suggested that grant funders require researchers to register before they receive any money and that scientific journals refuse to publish studies or researchers who aren’t willing to participate in this global registry.

Both Isasi and Goodman point out that already established guidelines can be applied to gene editing, some of which were developed for other groundbreaking research, such as embryonic stem cell investigations. Goodman adds that these regulations can be summed up in this way: Reducing illness and suffering is a good thing. Enhancement, however, is another matter altogether.

“If we’re doing an experiment with humans, we have to get consent from the individual,” Goodman says. “This didn’t happen [with the Chinese twins] or technically with the generations that will inherit this change. You cannot get consent from people who don’t exist yet.”

Both UHealth ethicists also agree that hitting the pause button is absolutely essential in order to further study the potential — and possibly unintended — consequences of altering the genome.

“We cannot move forward until we have the clinical data that quantifies with certainty what the risks and benefits are,” Isasi says. While there won’t be 100 percent agreement, scientists must establish a “minimally acceptable threshold” that, if not legally binding, is at least morally binding, she says.

There is no current global authority with the power to punish rule-breakers. She is hopeful, however.

Back in 2017, the American Society of Human Genetics (ASHG), along with 10 other organizations from around the world, issued a position statement recommending against “genome editing that culminates in human pregnancy,” or germline cell
editing. While supporting in vitro studies of human embryos and gametes, it did so only “with appropriate oversight and consent from donors” and “to facilitate research on the possible future clinical applications.” of genome editing.

The global community has come together on other issues that were important to the wellbeing of all, Isasi says, including regulations on nuclear proliferation and a ban on land mines.

“We have a responsibility to the kind of society we want to leave future generations,” she says.

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