Scientists, ethicists tackle gene editing's ethics, promise

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Nobel laureate David Baltimore of CalTech speaks to reporters at the National Academy of Sciences international summit on the safety and ethics of human gene editing, Tuesday, Dec. 1, 2015, in Washington. Alternating the promise of cures for intractable diseases with anxiety about designer babies and eugenics, hundreds of scientists and ethicists from around the world began debating the boundaries of a revolutionary technology to edit the human genetic code. (AP Photo/Susan Walsh)

A hot new tool to edit the human genetic code has a big wow factor: the promise of long-sought cures for intractable diseases. But depending on how it's used, that same tool could alter human heredity.

The debate has brought hundreds of scientists and ethicists from 20 countries to a highly unusual, three-day meeting in Washington on the ethics of human gene editing.

"We could be on the cusp of a new era in human history," Nobel laureate David Baltimore of the California Institute of Technology said Tuesday in opening the international summit to examine what he called "deep and disturbing questions."

"The overriding question is when, if ever, would we want to use gene editing to change human inheritance?" he said.

That question gained urgency after Chinese researchers made the first attempt at altering genes in human embryos, a laboratory experiment that didn't work well but did raise the prospect of one day modifying genes in a way that goes far beyond helping one sick person—it also could pass those alterations on to future generations.

"That really does raise the issue of, how do we use this technology in a responsible fashion," said molecular biologist Jennifer Doudna of the University of California, Berkeley, who helped pioneer the most-used gene-editing tool. Her calls for debate on its implications and boundaries led to this week's gathering, a step that could eventually lead to research recommendations.

At issue are tools to precisely edit genes inside living cells, finding specific sections of DNA to slice and repair or replace much like a biological version of cut-and-paste software. There are a few methods but one with the wonky name CRISPR-Cas9 is so fast, cheap and simple

for biologists to use that research is booming.

The potential is huge: Scientists are engineering animals with humanlike disorders to unravel the gene defects that fuel them. They're developing treatments for muscular dystrophy, sickle cell disease, cancer and HIV. They're trying to grow transplantable human organs inside pigs. They're even hatching mutant mosquitoes designed to be incapable of spreading malaria, and exploring ways to wipe out invasive species.

University at California Berkeley biochemist Jennifer Doudna speaks at the National Academy of Sciences international summit on the safety and ethics of human gene editing, Tuesday, Dec. 1, 2015, Washington. Alternating the promise of cures for intractable diseases with anxiety about designer babies and eugenics, hundreds of scientists and ethicists from around the world began debating the boundaries of a revolutionary technology to edit the human genetic code. (AP Photo/Susan Walsh)

One hurdle is safety. While the CRISPR tool is pretty precise, it sometimes cuts the wrong section of DNA. Tuesday, CRISPR pioneer Feng Zhang of the Broad Institute at MIT and Harvard reported Tuesday tweaking the tool's molecular scissors to significantly lower chances of off-target editing errors—an improvement that could have implications both for developing therapies and for germline research.

"The reality is, it will be years until this is turned into some kind of a therapy," Doudna cautioned reporters.

Yet many scientists said it's not too early to consider the biggest ethical quandary, that performing what's called germline editing, manipulating reproductive cells—sperm, eggs or embryos—could spread gene changes to future generations.

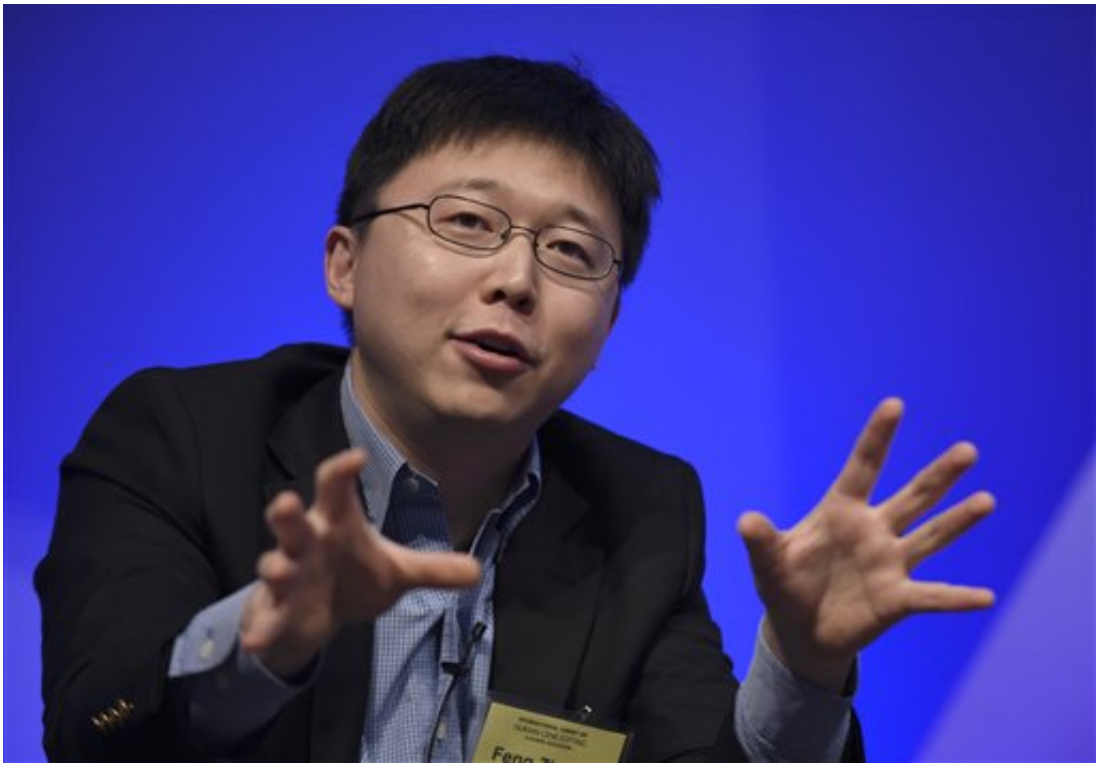
In the U.S., germline editing for clinical use—meaning for pregnancy—"is a line that should not be crossed at this time," John Holdren of the White House Office of Science and Technology Policy said Tuesday.

Last spring's experiment in China highlighted that ethics aside, embryo editing wasn't anywhere near ready for real-world use, because those off-target edits risked fixing one problem only to create another.

But there's controversy over whether and how to continue laboratory experiments to see if it eventually could work. And just as fraudulent stem cell clinics already lure desperate patients, there's worry about misuse of gene-editing techniques before they're proven.

Around the world, laws and guidelines vary widely about what germline, or hereditary, research is allowed. Some ban any research; some allow only lab research but not pregnancies; some have no policies. In the U.S., the National Institutes of Health won't fund germline research but

private funding is allowed.



Feng Zhang of the Broad Institute of MIT participates in a panel discussion at the National Academy of Sciences international summit on the safety and ethics of human gene editing, Tuesday, Dec. 1, 2015, in Washington. Alternating the promise of cures for intractable diseases with anxiety about designer babies and eugenics, hundreds of scientists and ethicists from around the world began debating the boundaries of a revolutionary technology to edit the human genetic code. (AP Photo/Susan Walsh)

What one country attempts "will have consequences in others," the White House's Holdren noted.

It's not just about editing embryos. At the University of Pittsburgh, Dr. Kyle Orwig is exploring treatments for male infertility that could alter

sperm-producing cells that don't do their job.

Critics note there are other ways to halt transmission of inherited disease. Already, couples undergo in vitro fertilization and have the resulting embryos tested for the family's problem gene before deciding which to have implanted, noted Marcy Darnovsky of the Center for Genetics and Society advocacy group.

Allowing gene editing for medical reasons would open the door to designer babies with cosmetic changes, too, she added.

"It would alter future human societies, perhaps profoundly so," Darnovsky said Tuesday.

Pre-testing of embryos doesn't solve the problem for all families with devastating rare diseases, said Dr. George Daley of Boston Children's Hospital, recounting families that have dozens of embryos created through IVF to come up with one or two usable ones.

"Is it more ethical to edit embryos, or to screen a lot of embryos and throw them away? I don't know the answer," Doudna said.

More information: Human gene-editing initiative: bit.ly/1YDu8OU