BERKELEY, California: In this photo provided by UC Berkeley Public Affairs, taken June 20, 2014 Jennifer Doudna, right, and her lab manager, Kai Hong, work in her laboratory .

MANHATTAN, Kansas: Meat scientist Bob Danler prepares a sample of ground beef for testing at GreatO Premium Foods.—AP photos

Gene editing: Research spurs debate over promise vs ethics

WASHINGTON: The hottest tool in biology has scientists using words like revolutionary as they describe the long-term potential: wiping out certain mosquitoes that carry malaria, treating genetic diseases like sickle-cell, preventing babies from inheriting a life-threatening disorder. It may sound sci-fi, but research into genome editing is booming. So is a debate about its boundaries, what's safe and what's ethical to try in the quest to fight disease. Does the promise warrant experimenting with human embryos? Researchers in China already have, and they're poised to in Britain.

Should we change people's genes in a way that passes traits to future generations? Beyond medicine, what about the environmental effects if, say, altered mosquitoes escape before we know how to use them? "We need to try to get the balance right," said University of California, Berkeley, biochemist Jennifer Doudna. She helped develop new gene-editing technology and hears from desperate families, but urges caution in how it's eventually used in people.

The US National Academies of Science, Engineering and Medicine will bring international scientists, ethicists and regulators together in December to start determining that balance. The biggest debate is whether it ever will be appropriate to alter human heredity by editing an embryo's genes. "This isn't a conversation on a cloud," but something that families battling devastating rare diseases may want, Dr George Daley of Boston Children's Hospital told specialists meeting this week to plan the ethics summit. "There will be a drive to move this forward."

Experimental treatment

Laboratories worldwide are embracing a technology to precisely edit genes inside living cells - turning them off or on, repairing or modifying them - like a biological version of cut-and-paste software. Researchers are building stronger immune cells, fighting muscular dystrophy in mice, and growing human-like organs in pigs for possible transplant. Biotech companies have raised millions to develop therapies for sickle cell disease and other disorders. The technique has a wonky name - CRISPR-Cas9 - and a humble beginning.

Doudna was studying how bacteria recognize and disable viral invaders, using a protein she calls "a genetic scalpel" to slice DNA. That system turned out to be programmable, she reported in 2012, letting scientists target virtually any gene in many species using a tailored CRISPR recipe. There are older methods to edit genes, including one that led to an experimental treatment for the AIDS virus, but the CRISPR technique is faster and cheaper, and allows altering of multiple genes simultaneously.

"It's transforming almost every aspect of biology right now," said National Institutes of Health genomics specialist Shawn Burgess. CRISPR's biggest use has nothing to do with human embryos. Scientists are engineering animals with human-like disorders more easily than ever before, to learn to fix genes gone awry and test potential drugs. Engineering rodents to harbor autism-related genes once took a year. It takes weeks with CRISPR, said bioengineer Feng Zhang of the Broad Institute at MIT and Harvard, who also helped develop, and patented, the CRISPR technique. (Doudna's university is challenging the patent.)

Provide a cure

A peek inside an NIH lab shows how it works. Researchers inject a CRISPR-guided molecule into microscopic mouse embryos, to cause a gene mutation that a doctor suspects of causing a patient's mysterious disorder. The embryos will be implanted into female mice that wake up from the procedure in warm blankets to a treat of fresh oranges. How the resulting mouse babies fare will help determine the gene defect's role. Experts predict the first attempt to treat people will be for blood-related diseases such as sickle cell, caused by a single gene defect that's easy to reach. The idea is to use CRISPR in a way similar to a bone marrow transplant, but to correct someone's own blood-producing cells rather than implanting donated ones.

"It's like a race. Will the research provide a cure while we're still alive?" asked Robert Rosen of Chicago, who has one of a group of rare bone marrow abnormalities that can lead to leukemia or other life-threatening conditions. He co-founded the MPN Research Foundation, which has begun funding some CRISPR-related studies. So why the controversy? CRISPR made headlines last spring when Chinese scientists reported the first-known attempt to edit human embryos, working with unusable fertility clinic leftovers. They aimed to correct a deadly disease-causing gene but it worked in only a few embryos and others developed unintended mutations, raising fears of fixing one disease only to cause another.

If ever deemed safe enough to try in pregnancy, that type of gene change could be passed on to later generations. Then there are questions about designer babies, altered for other reasons than preventing disease. In the US, the NIH has said it won't fund such research in human embryos. In Britain, regulators are considering researchers' request to gene-edit human embryos - in lab dishes only - for a very different reason, to study early development. Medicine aside, another issue is environmental: altering insects or plants in a way that ensures they pass genetic changes through wild populations as they reproduce.—AP

Ebola's persistence in survivors fuels concerns over future risks

Virus can hide in body parts

LONDON: Ebola virus can hide in parts of the body such as eyes, breasts and testicles long after leaving the bloodstream raises questions about whether the disease can ever be beaten. Virologists said yesterday's case of a Scottish nurse, Pauline Cafferkey, who had recovered from Ebola but is now suffering complications adds to signs that the virus is a long-term health risk and can lead to a "post-Ebola syndrome".

"Over the past few years there has been mounting evidence of mental and physical health problems in Ebola survivors that can last for years after the virus is cleared from the bloodstream," said Ben Neuman, an Ebola expert and lecturer in virology at Britain's University of Reading. "The newly discovered twist on this post-Ebola syndrome is that in some cases the health problems - often including damage to the eyes and joints - are caused by live Ebola virus growing in fluids in some of the less accessible compartments of the body."

Ebola, one of the deadliest viruses known in humans, infected 28,000 people and killed more than 11,300 of them in an unprecedented outbreak in West Africa which was declared in March 2014 and is only now coming under control.

Partly because of the vast numbers involved in the epidemic, which centred on Guinea, Sierra Leone and Liberia, infectious disease experts say we are learning more every day about Ebola from cases such as Cafferkey's and thousands more survivors. Ebola experts said in August that around half of Ebola survivors in West Africa were already reporting suffering from chronic problems, including serious joint pain and eye inflammation that can lead to blindness.

Large virus reservoir?

"Due to the sheer scale of this outbreak compared to previous ones we are going to see aspects of Ebola virus infection that we have not observed before," said Julian Hiscox, a professor of infection and global health at Britain's Liverpool University.

He was concerned that Ebola's persistence in survivors, who have no obvious symptoms of Ebola infection and so are often living and working normally and not kept in isolation as a symptomatic patient would be, means they are "a potential reservoir of the virus".

"It's why men who have had Ebola and recovered are advised to abstain or wear condoms," he noted. The World Health Organization's advice is that all male survivors should be tested three months after the onset of symptoms and then monthly until they know they have no risk of passing on the virus through their semen. John Edmunds, an expert at the London School of Hygiene and Tropical Medicine, said that while the risk of transmission from survivors harboring the virus in their eye fluids and other organs "appears to be very low", it still warrants attention.

"With so many survivors in West Africa now, there is a risk that further outbreaks can be triggered, which is why authorities have to remain very vigilant," he said. Cafferkey, a 39-year-old nurse, was back in hospital in London yesterday with doctors saying she would be treated in isolation as a precautionary measure. The hospital said in statement it had "identified a small number of close contacts ... that we will be following up as a precaution", but added: "The risk to the general public remains low." Cafferkey was the first person to be diagnosed with Ebola on British soil and was originally discharged in January after seemingly making a full recovery. Neuman said the likelihood of survivors spreading Ebola depends on how much of the virus is present in the blood. In Cafferkey's case, he said, "if her body was able to control the virus once, the chances are she can do it twice."—Reuters