Tests suggest scientists achieved 1st 'in body' gene editing

By MARILYNN MARCHIONE Associated Press

Scientists think they have achieved the first gene editing inside the body, altering DNA in adults to try to treat a disease, although it's too soon to know if this will help.

Preliminary results suggest that two men with a rare disorder now have a corrective gene at very low levels, which may not be enough to make the therapy a success.

Still, it's a scientific milestone toward one day doctoring DNA to treat many diseases caused by faulty genes.

"This is a first step," said Dr. Joseph Muenzer of the University of North Carolina at Chapel Hill, who helped test the treatment. "It's just not potent enough.'

He gave the results today at a conference in Orlando, Florida, and has consulted for the therapy's maker, California-based Sangamo Therapeutics. Researchers are working on a stronger version of the treatment.

Gene editing is intended as a more precise way to do gene therapy, to disable a bad gene or supply a good one that's missing. Trying it in



AP Photo/Eric Risberg

Brian Madeux starts to receive the gene editing therapy for Hunter syndrome in 2017.

adults to treat diseases is not controversial and the DNA changes do not pass to future generations, unlike the recent case of a Chinese scientist who claims to have edited twin girls' genes when they were embryos.

Sangamo's studies involve men with Hunter or Hurler syndrome, diseases caused by a missing gene that makes an enzyme

to break down certain sugar compounds. Without it, sugars build up and damage organs, often killing people in their teens.

In 2017, Brian Madeux of Arizona became the first person to try it. Through an IV, he received many copies of a corrective gene and an editing tool called zinc finger nucleases to insert it into his DNA

Results on him and seven other Hunter patients, plus three with Hurler syndrome, suggest the treatment is safe, which was the main goal of these early experiments. Three problems - bronchitis, an irregular heartbeat and a hernia — were deemed due to the diseases, not the treatment.

Tissue samples showed evidence of gene editing at very low levels in two Hunter patients who were given a middle dose but not in one given a low dose. Tests are expected later this year on patients who received the highest dose and on Hurler patients.

Blood tests detected slightly higher levels of the missing enzyme in a few of the Hunter patients but none of them reached normal levels. One patient had a larger increase but also showed signs that his immune system might be attacking the therapy. He was treated for that and symptoms resolved.

More encouraging results were seen in Hurler patients - enzyme levels rose to normal in all three after treatment, tests on certain blood cells showed.

"This is very promising" for Hurler patients, said Dr. Paul Harmatz of UCSF Benioff Children's Hospital Oakland, who presented those results.

None of the patients with either disease showed a sustained decline in urine levels of the troubling sugar compounds, though, and some other tests also did not detect intended effects of the therapy.

The key test will be stopping the patients' weekly enzyme treatments to see if their bodies can now make enough of it on their own. Three have gone off treatments so far and one was recently advised to resume them because of fatigue and rising levels of the sugar compounds. The others have not been off long enough to know how they will fare.

"It looks like it's safe . that's a very positive sign," said one independent expert, Dr. Kiran Musunuru of the University of Pennsylvania. He called the early results promising but said "it's hard to be sure it's doing any good" until patients are studied longer.

"What they're trying to do with gene editing is very challenging," he said. "It's much harder to make a correction or insert a gene" than to disable one.

Dr. Tyler Reimschisel of Vanderbilt University agreed.

"It's not discouraging, it's just early and on a small amount of people," he said. "This is definitely a novel and innovative treatment" but it's not clear if it's going to help.

WORLD IN BRIEF

Associated Press

Democrats seek Green New Deal for climate change

WASHINGTON - Democrats including Rep. Alexandria Ocasio-Cortez of New York are calling for a Green New Deal intended to transform the U.S. economy to combat climate change and create thousands of jobs in renewable energy.

The freshman lawmaker and veteran Sen. Ed Markey of Massachusetts are teaming up on the plan, which aims to eliminate the U.S. carbon footprint by 2030

While setting lofty goals, the plan does not explicitly call for eliminating the use of fossil fuels such as oil and natural gas, a nod to pragmatism that may disappoint some of Ocasio-Cortez's strongest supporters.

Even so, their Green New Deal goes far beyond the Clean Power Plan proposed by President Barack Obama. President Donald Trump has scrapped Obama's plan, which imposed emissions limits on coal-



San Francisco firefighters battle a fire after a gas explosion.

San Francisco gas explosion shoots fire that burns buildings

Wednesday show the rocket was gone with what appears to be burn marks on its launch pad.

It wasn't immediately clear if the satellite, if launched, made it into orbit.

The U.S. alleges such launches defy a U.N. Security Council resolution calling on Iran to undertake no activity related to ballistic missiles capable of delivering nuclear weapons.

Dislike: Facebook will appeal German data-use ruling

BERLIN — German authorities ruled today that Facebook should not be allowed to use customer data from other apps and websites to help target advertisements shown on their Facebook pages without their explicit consent, saying it was exploiting its dominant position in social media.

Federal Cartel Office, or The Bundeskartellamt, said Facebook was guilty of "exploitative abuse" by forcing users to agree to allow it to collect data from other Facebook-owned services like WhatsApp and Instagram, as well as third-party websites through the "Like" or "Share" features. Facebook said it rejected the decision, and would appeal within the one-month frame before it becomes final. If the ruling is upheld, Facebook will be required to allow users to specifically approve data collected from other Facebook-owned sources and third-party websites be assigned to their accounts. The decision is not about Facebook's processing of data generated by its own site, which the Cartel Office acknowledged is the business model for data-based social networks like Facebook.

fired power plants, as a job-killer.

Images suggest Iran launched satellite

DUBAI, United Arab Emirates - Iran appears to have attempted a second satellite launch despite U.S. criticism that its space program helps the country develop ballistic missiles, satellite images released today suggest. Iran did not immediately acknowledge conducting such a launch.

The images show a rocket at the Imam Khomeini Space Center in Iran's Semnan province on Tuesday. Images from

SAN FRANCISCO — A gas explosion in a San Francisco neighborhood shot flames into the air for hours Wednesday and burned five buildings, sending panicked residents and workers fleeing into the streets.

It took utility crews more than two hours to put out the fire after private construction workers cut a natural gas line, igniting the towering flames. Authorities initially said five workers were missing, but the entire construction crew was found safe, and no other injuries were reported.

Officials evacuated several nearby buildings, including a medical clinic and apartment buildings. Vehicles on a busy street got rerouted as authorities cordoned off the bustling retail and residential neighborhood.

Pacific Gas & Electric is under heightened scrutiny over its natural gas pipelines after one exploded under a neighborhood south of San Francisco in 2010, killing eight people and wiping out a neighborhood in suburban San Bruno.

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