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.”

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 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.

The 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.

Safe treatment
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. None of the patients with either disease showed a sustained decline in urine levels of the 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. “It’s not discouraging, it’s just early and on a small amount of people,” Dr. Tyler Reimschisel of Vanderbilt University said.