

## 2019: the year gene therapy came of age

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Victoria Gray's blood was drawn so that doctors could get to the cause of her illness—stem cells from her bone marrow that were making deformed red blood cells

In the summer, a mother in Nashville with a seemingly incurable genetic disorder finally found an end to her suffering—by editing her genome.

Victoria Gray's recovery from [sickle cell disease](#), which had caused her painful seizures, came in a year of breakthroughs in one of the hottest areas of medical research—gene therapy.

"I have hoped for a cure since I was about 11," the 34-year-old told AFP in an email.

"Since I received the new cells, I have been able to enjoy more time with my family without worrying about pain or an out-of-the-blue emergency."

Over several weeks, Gray's blood was drawn so doctors could get to the cause of her illness—stem cells from her bone marrow that were making deformed red blood cells.

The stem cells were sent to a Scottish laboratory, where their DNA was modified using Crispr/Cas9—pronounced "Crisper"—a new tool informally known as molecular "scissors."

The genetically edited cells were transfused back into Gray's veins and bone marrow. A month later, she was producing normal blood cells.

Medics warn that caution is necessary but, theoretically, she has been cured.

"This is one patient. This is early results. We need to see how it works out in other patients," said her doctor, Haydar Frangoul, at the Sarah Cannon Research Institute in Nashville.



DNA from Gray's stem cells was modified at a Scottish laboratory, using Crispr/Cas9—pronounced "Crisper"—a new tool informally known as molecular "scissors"

"But these results are really exciting."

In Germany, a 19-year-old woman was treated with a similar method for a different blood disease, beta thalassemia. She had previously needed 16 blood transfusions per year.

Nine months later, she is completely free of that burden.

For decades, the DNA of living organisms such as corn and salmon has been modified.

But Crispr, invented in 2012, made gene editing more widely accessible. It is much simpler than preceding technology, cheaper and easy to use in small labs.

The technique has given new impetus to the perennial debate over the wisdom of humanity manipulating life itself.

"It's all developing very quickly," said French geneticist Emmanuelle Charpentier, one of Crispr's inventors and the cofounder of Crispr Therapeutics, the biotech company conducting the clinical trials involving Gray and the German patient.



Crispr made gene editing more widely accessible - but has given new impetus to the perennial debate over the wisdom of humanity manipulating life

## Cures

Crispr is the latest breakthrough in a year of great strides in gene therapy, a medical adventure started three decades ago, when the first TV telethons were raising money for children with muscular dystrophy.

Scientists practising the technique insert a normal gene into cells containing a defective gene.

It does the work the original could not—such as making normal [red blood cells](#), in Victoria's case, or making tumor-killing super white blood [cells](#) for a cancer patient.

Crispr goes even further: instead of adding a gene, the tool edits the genome itself.

After decades of research and clinical trials on a genetic fix to genetic disorders, 2019 saw a historic milestone: approval to bring to market the first gene therapies for a neuromuscular disease in the US and a blood disease in the European Union.

They join several other gene therapies—bringing the total to eight—approved in recent years to treat certain cancers and an inherited blindness.





Scientific director of the Telethon Serge Braun poses for a photograph after a press conference to present the Telethon 2018, the annual fundraising for medical research, at the Myology Institute in Paris on December 3, 2018

Serge Braun, the scientific director of the French Muscular Dystrophy Association, sees 2019 as a turning point that will lead to a medical revolution.

"Twenty-five, 30 years, that's the time it had to take," he told AFP from Paris.

"It took a generation for gene therapy to become a reality. Now, it's only going to go faster."

Just outside Washington, at the National Institutes of Health (NIH), researchers are also celebrating a "breakthrough period."

"We have hit an inflection point," said Carrie Wolinetz, NIH's associate director for science policy.

These therapies are exorbitantly expensive, however, costing up to \$2 million—meaning patients face grueling negotiations with their insurance companies.

They also involve a complex regimen of procedures that are only available in wealthy countries.



French geneticist Emmanuelle Charpentier, one of Crispr's inventors, doesn't believe in the more dystopian scenarios that have been predicted for gene therapy

Gray spent months in hospital getting blood drawn, undergoing chemotherapy, having edited [stem cells](#) reintroduced via transfusion—and fighting a general infection.

"You cannot do this in a community hospital close to home," said her doctor.

However, the number of approved gene therapies will increase to about 40 by 2022, according to MIT researchers.

They will mostly target cancers and diseases that affect muscles, the eyes and the nervous system.

## **Bioterrorism**

Another problem with Crispr is that its relative simplicity has triggered the imaginations of rogue practitioners who don't necessarily share the medical ethics of Western medicine.

Last year in China, scientist He Jiankui triggered an international scandal—and his excommunication from the scientific community—when he used Crispr to create what he called the first gene-edited humans.





Chinese scientist He Jiankui, pictured in 2018, used Crispr to create what he called the first gene-edited humans

The biophysicist said he had altered the DNA of human embryos that became twin girls Lulu and Nana.

His goal was to create a mutation that would prevent the girls from contracting HIV, even though there was no specific reason to put them through the process.

"That technology is not safe," said Kiran Musunuru, a genetics professor at the University of Pennsylvania, explaining that the Crispr "scissors" often cut next to the targeted gene, causing unexpected mutations.

"It's very easy to do if you don't care about the consequences," Musunuru added.

Despite the ethical pitfalls, restraint seems mainly to have prevailed so far.

The community is keeping a close eye on Russia, where biologist Denis Rebrikov has said he wants to use Crispr to help deaf parents have children without the disability.

There is also the temptation to genetically edit entire animal species—malaria-causing mosquitoes in Burkina Faso or mice hosting ticks that carry Lyme disease in the US.



A picture taken at the French National Institute of Agricultural Research (INRA) in Maison-Alfort, on July 20, 2016 shows a tick, whose bite can transmit the Lyme disease

The researchers in charge of those projects are advancing carefully, however, fully aware of the unpredictability of chain reactions on the ecosystem.

Charpentier doesn't believe in the more dystopian scenarios predicted for [gene therapy](#), including American "biohackers" injecting themselves with Crispr technology bought online.

"Not everyone is a biologist or scientist," she said.

And the possibility of military hijacking to create soldier-killing viruses or bacteria that would ravage enemies' crops?

Charpentier thinks that technology generally tends to be used for the better.

"I'm a bacteriologist—we've been talking about bioterrorism for years," she said. "Nothing has ever happened."

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