

In China, a death following safety failures in a gene therapy trial

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In late July 2026, an article published in the journal *Science* revealed, more than a year after the event took place, the death of a six-year-old girl following a gene therapy trial in China. This therapy was based on new techniques of genetic modification (so-called new genomic techniques - NGT) and used viral vectors to deliver the gene modifying material to the target cells.



Le Pr. Zilong Qiu, qui a mené cette expérience de thérapie génique.

The current trend towards the rapid advancement of biotechnologies – particularly those involving transgenesis and mutagenesis in the agricultural and medical fields, using new tools such as CRISPR/Cas or RNA – has led *InfOGM* to examine various aspects of biomedical research. Here, we examine the specific case of a gene therapy carried out on a child in China, which was reported by the journal *Science* in July 2026.

This girl suffered from neurological disorders, particularly relating to speech and language, which were not life-threatening. The condition was caused by a mutation in a single gene: the CDH3 gene, which is expressed in certain nerve cells of the brain. This gene is involved in chromatin remodelling (one of the forms of DNA, when it is "*coiled up*"), which is essential for the proper transcription of DNA into messenger RNA (mRNA)ⁱⁱ.

For the doctor who carried out this trial, Qiu Zilong, at Xinhua Hospital in Shanghai, the aim was to "correct" this gene. However, as reported in the journal *Science*, the girl eventually died as a result of the treatment. Her death highlights scientific negligence, but also a toxic environment within certain medical circles, where the implementation of genetic "*innovation*" takes precedence over the survival of those being treated. This provides an opportunity for *Inf'OGM* to revisit two key issues: gene therapy and bioethics.

The biomedical context of preclinical studies

The syndrome from which the young patientⁱⁱⁱ suffered was highly likely to lead, in later life, to autistic behaviours, motor deficits and other disabilities, without affecting life expectancy. Hence the search for a treatment.

To understand the work of Professor Qiu Zilong's laboratory, we must go back in 2024, the year in which a scientific article^{iv} was submitted to the journal *Nature*, as reported by the newspaper *Le Monde*^v. This article, written by academics from the Shanghai Medical School, including Qiu Zilong, was finally published thirteen months later, in February 2026 – that is, after the child's death in March 2025. It demonstrated the proof of concept (feasibility) of experiments described primarily in mice, with the aim of eventually treating the syndrome in question in humans using gene therapy.

This trial, conducted on mice and macaques, was a preclinical study. It described how the use of a new form of the CRISPR/Cas9 tool (the "*adenine base editor, TeABE-1248*") can modify the deleterious CDH3 gene in the brain neurons of mice so as to "*correct*" it^{vi}.

In fact, the mice used in this study were "*humanised*", meaning that they had previously been genetically modified with a deleterious version of the CDH3 gene similar to the one carried by the girl. The modification caused the mice to become ill: they are experimental models.

The authors of the *Nature* article explain that, in their study, thanks to the "*gene editor*", the correction of the deleterious CDH3 gene restored normal neuronal development and normal behaviour in these mice. It is not known whether any other consequences were observed.

To deliver the genetic "*correction*" material – that is, the genetic construct of the "*base editor*" and the guide RNA – to the brain's nerve cells in the GM mice *in vivo*, the researchers use viruses known as AAVs, or adeno-associated viruses, which serve as vectors. These naturally occurring viruses, known to exist in primates^{vii}, are genetically modified and routinely used in gene therapy, as shown in a primatology paper from 2025^{viii}. In mice, some of these viruses can cross the blood-brain barrier and be carried via the cerebrospinal fluid to the brain, which is normally protected by this barrier.

According to the authors of the *Nature* article, the experimental therapy had therefore been successful in humanised mice. However, it is not known whether any off-target effects of the genetic modifications were observed. In the same *Nature* article^{ix}, experiments corresponding to the second stage of the mouse trials were replicated in four macaques. However, these are described only very briefly.

The authors state: "*To assess translational feasibility, we evaluated delivery and intein-mediated reconstitution of TeABE [the CRISPR tool used] in the NHPs [primate nervous system]. Dual AAV9 vectors encoding split-TeABE were intrathecally administered to promote broad CNS exposure [central nervous system]*". This injection allows direct access to the cerebrospinal fluid, as AAV viruses do not cross – or cross only minimally – the blood-brain barrier in primates, unlike in mice. In practice, the situation is complex and warrants further explanation.

On the one hand, this is because the "*base editor*" is very large; consequently, the genetic information was split into two parts and integrated into two different AAVs, which were injected simultaneously with the aim of ensuring that both viruses are expressed within the same cells throughout the brain. In the United States, a researcher specialising in this field admits to being "*skeptical that a dual vector approach would achieve high enough editing*" to treat humans^x.

Furthermore, only a small fraction of these viruses penetrate their target cells and stimulate the production of the basic components of the "*gene editor*". Thus, to repair nerve cells in primates, hundreds of thousands of billions of viruses are required! This is known to cause large-scale inflammatory reactions^{xi} that may impair the therapy's effectiveness and lead to serious side effects...

Researchers who are critical and cautious about their own work

Regarding the consequences of the mutation affecting the CDH3 gene, the authors of the study on mice and macaques agree that important data are lacking: "*further mechanistic studies are necessary to elucidate how the mutation affects the chromatin-remodelling functions of CHD3 and contributes to disease pathology*". They also acknowledge that a greater reduction in the side effects and off-target effects of this "*base editor*" will be necessary. This proves, if proof were needed, that the CRISPR/Cas tool, even in the form of a "*base editor*", is not as precise as its proponents claim.

Regarding the feasibility of using this 'basic editing' tool in macaques, the researchers report having observed "*robust editor expression and efficient reconstitution of the full-length complex (around 180 kDa), with [\"editor\"] reconstitution efficiencies of about 70% across the frontal cortex, occipital cortex and cerebellum*". This effectively means that the reconstitution efficiency of the "*gene editor*" is not 100 per cent, as would be expected in the medical field. They add, however, that "*compelling evidence for the efficacy of TeABE in the NHP [primate nervous system] brain, paving the way for the application of TeABE in clinical trials*". For them, this study represents "*a substantial advancement in gene therapy for brain disorders...*". Thus, these researchers are paving the way for the clinical (human) application of their research.

However, the conclusion of their article states that "*these models [mice and macaques] are valuable for evaluating phenotype improvements but fall short of providing guidance on clinical dosing or application in humans. Bridging the gap between preclinical research and clinical translation [of the CRISPR complex] remains a significant challenge*".

In response to this trial, experts have expressed serious concerns about the way in which the feasibility study was conducted in primates, particularly regarding the lack of rigour, as there were no control groups. "*It's completely unconvincing*", explained David Sanders, a biochemist at Purdue University (United States), who has studied this gene therapy^{xii}.

Unrecognised adverse effects and ethical guidelines not followed

A clinical trial (in humans) is listed on a specialist website^{xiii} and a review of the details reveals that the trial was abandoned at the end of July 2026. The dates confirm that this trial did indeed concern the young Chinese patient who died. The report reveals the cause of her death: thrombotic microangiopathy. This marked the start of a complex investigation, the details of which were revealed in the article published in *Science* at the end of July 2026^{xiv}.

With regard to the use of modified adeno-associated viruses (AAVs) in humans, the risks of death were far from negligible. The 2025 primatology document^{xv} states that there are still concerns regarding the safe use of high-dose viral therapies in humans. These relate to immune responses against AAVs and adverse effects, such as toxicity to the liver or the nervous system and... the onset of thrombotic microangiopathy!

Furthermore, contamination by substances used in the production process of viral vectors (AAV) is always a possibility^{xvi}. In these circumstances, how can we explain why the girl was given this type of gene therapy?

According to the article in *Le Monde*^{xvii}, *"this preclinical phase [Editor's note: as reported in the Nature article] clearly convinced the ethics committee at Xinhua Hospital to approve the move to the clinical trial: the administration of the treatment to [the patient]"*. This decision is all the more surprising given that, as *Le Monde* also points out, *"one factor, however, was not assessed by the committee: a toxicological study by PriMed, the company responsible for the trials on macaques^{xviii}, showing that the four primates studied exhibited liver damage, and one of them kidney damage"*. The incidence of damage is therefore 100 per cent among the four macaques.

Furthermore, the article in *Nature* is preceded by an editor's note dated 29 July 2026, five months after the article's publication: *"Readers are alerted that concerns have been raised regarding this article, including with the data presented. Further editorial action will be taken if appropriate as soon as the investigation into the concerns is complete and all parties have been given an opportunity to respond in full"*.

The article in *Science* that brought this matter to light states that it had *"stripped the paper [the original article published in Nature] of references to the family and its financial contributions"*, noting only that *"bridging the gap between preclinical research and clinical translation remains a significant challenge"*. According to *Science* and *Le Monde*, the patient's parents did indeed contribute financially to the clinical trial, to the tune of €756,000, through payments from donors, according to *Science*, and from foundations and companies involved, according to *Le Monde*^{xix}. The *Science* article states that, according to text messages shared with the family, *"Qiu also asked the couple to pay other members of the research team directly, through informal arrangements"*, arrangements which the parents found increasingly troubling.

Experts in genetics, virology and bioethics who have examined the details of the preclinical and clinical studies have expressed concern that Qiu and his team downplayed the risks of the clinical trial when describing them to the parents, overlooked safety signals in animal studies and proceeded with the injecting AAV vectors into the cerebrospinal fluid even though success was unlikely.

Following the young patient's death, the article in *Science* reveals that, *"according to official documents and accounts provided by the girl's parents, the hospital had allowed Qiu's experimental treatment to proceed under a regulatory provision that does not require approval from national regulators. After the child's death, the hospital paid a modest fine to a local health authority but Qiu was not publicly sanctioned"*.

The editor-in-chief of *Nature* also states that she was not informed of any other planned or ongoing human trials being conducted by this hospital^{xx}. In response to the parents of the deceased girl, she states that "*the ethical issues raised do not fall within [their] remit in terms of data integrity and that the matter should be dealt with by the university*".

We are therefore faced with a serious case of accountability for biomedical research in China and for the Chinese government. The secrecy surrounding the case for over a year is telling. The article in *Science* recalls that in 1999, the death of 18-year-old American Jesse Gelsinger following gene therapy for a condition of which he suffered only a "mild" form had significantly slowed down research in this field.

The motivations of the Chinese team that carried out this clinical trial on the girl are similar to those of He Jiankui, who, in 2018, secretly genetically modified three human embryos using the CRISPR/Cas tool. In both cases, the motivation is to be the first on the international stage in the field of human biotechnology, a source of coveted power and financial windfalls. Even if it means turning a blind eye to special favours and other ethical misconduct...

This is a matter of great importance to the international biomedical community and to us all. Indeed, another death in China following gene therapy was revealed a year later, on 6 August 2026^{xxi}. This second death, about which few details have been provided, has led the journal *Science* to raise questions "*about China's transparency and oversight of cutting-edge treatments*".

These sad incidents serve as a reminder that ethical guidelines must be strictly adhered to when medical treatments are still at the stage of holding promise, and that the information provided to patients and their families must be clear and comprehensive. It must also be guided by safety considerations.

i Brendan Borrell, Retraction Watch, [« A fatal reaction – A cutting-edge gene-editing trial in China went disastrously wrong. A family wants accountability »](#), *Science*, Vol. 393, Issue 6809, 23 July 2026.

ii For informations about chromatin, see:
Annick Bossu, [« Off-target effects of NGTs: CRISPR/Cas "fatigues chromatin" »](#), *Inf'OGM*, 29 June 2026.

iii It is the Snijders Blok-Campeau syndrom (SNIBCPS) : YBY In, [« Syndrome de Snijders Blok-Campeau »](#).

iv Yang, K., Li, WK., Geng, YX. *et al.*, [« In vivo base editing of Chd3 rescues behavioural abnormalities in mice »](#), *Nature*, Vol. **651**, pp. 785–795, 18 February 2026.

v Axel Favrot et Harold Thibault, [« La Chine cache la mort d'une enfant après une thérapie génique »](#), *Le Monde*, 5 août 2026, p. 19.

vi According to the authors, TeABE is more précise than Crispr/Cas9 and, cutting one DNA strand, has less undesired and off-target effects. For more informations, see:
Lingjie Jing, Mengyao Chen, Qunjun Yang, [« TeABE-mediated CHD3 correction: A novel therapeutic strategy for monogenic neurodevelopmental disorders »](#), *Genes & Diseases*, 13 June 2026.

vii Contributors of Wikipédia, [« Adeno-associated virus »](#), *Wikipedia, The Free Encyclopedia*.

[viii](#) Many informations in French in :

Cécile Gaston, Audrey Fayard, Noëlle Dufour *et al.*, « [Importance translationnelle des primates non humains pour l'étude de la réponse immunitaire dans les procédures de thérapie génique impliquant l'administration in vivo de vecteurs rAAV](#) », *Revue de primatologie*, Vol. 16, 2026.

[ix](#) Yang, K., Li, WK., Geng, YX. *et al.*, « [In vivo base editing of Chd3 rescues behavioural abnormalities in mice](#) », *Nature*, Vol. **651**, pp. 785–795, 18 February 2026.

[x](#) Brendan Borrell, Retraction Watch, « [A fatal reaction – A cutting-edge gene-editing trial in China went disastrously wrong. A family wants accountability](#) », *Science*, Vol. 393, Issue 6809, 23 July 2026.

[xi](#) *Ibid.*

[xii](#) *Ibid.*

[xiii](#) National Library of Medicine, « [Clinical Trial of the Dual Vector Base Editor for the Treatment of the CHD3-R1025W Mutation](#) », *ClinicalTrials.gov*, 30 July 2026.

[xiv](#) Brendan Borrell, Retraction Watch, « [A fatal reaction – A cutting-edge gene-editing trial in China went disastrously wrong. A family wants accountability](#) », *Science*, Vol. 393, Issue 6809, 23 July 2026.

[xv](#) Cécile Gaston, Audrey Fayard, Noëlle Dufour *et al.*, « [Importance translationnelle des primates non humains pour l'étude de la réponse immunitaire dans les procédures de thérapie génique impliquant l'administration in vivo de vecteurs rAAV](#) », *Revue de primatologie*, Vol. 16, 2026.

[xvi](#) John Sterling, « [Unlocking Opportunities in Upstream Viral Vector Manufacturing](#) », *Genetic Engineering & Biotechnology News*, 25 September 2024.

[xvii](#) Translation by *Inf'OGM*.

[xviii](#) Research structure under contract in the Sichuan province (China).

[xix](#) Axel Favrot et Harold Thibault, « La Chine cache la mort d'une enfant après une thérapie génique », *Le Monde*, 5 août 2026, p. 19.

[xx](#) *Ibid.*

[xxi](#) John Travis, « [Second gene-editing death in China disclosed after long delay](#) », *Science*, 6 August 2026.

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