

Mei's Story: Investigation Reveals Unreported Fatal Outcome After In Vivo Base Editing of *CHD3*



In March 2025, a six-year-old girl received the first reported in vivo base-editing treatment directed at the human CNS. The girl, given the pseudonym “Mei” — meaning “beautiful” in Chinese — had [Snijders Blok-Campeau](#) syndrome, an extremely rare genetic condition associated with mutations in the *CHD3* gene. Her condition was not life-threatening, and she was on the milder side of the disease spectrum. Tragically, a week after receiving the experimental treatment, she died.

Her death was not made public at the time, and lead researcher Zilong Qiu [later published](#) related animal studies in the journal *Nature* (1). The paper did not mention her death or her parents’ financial contributions to the development of the therapy, stating only that “bridging the gap between pre-clinical research and clinical translation remains a significant challenge.”

The story remained hidden until her parents, who asked to be called Jason and Linda, decided to tell their story to [Science and Retraction Watch](#) “to prevent more children from suffering” and their “blood and tears flowing in vain.”

Mei's story

Mei seemed a little clumsier than other toddlers, and at four, she didn’t draw or write as well as other students in her kindergarten. She still spoke in simple sentences and used training chopsticks, but speech and occupational therapy were helping.

In March 2023, Mei was diagnosed with global developmental delay, a term used when a child takes longer to reach certain milestones. Specialists said that some of her behaviors were associated with autism.

After a brain MRI, chromosome analysis, and a test panel for genes associated with neurodevelopment all came back normal, a detailed sequencing of Mei's DNA revealed a defect in a gene called *CHD3* that affected how DNA strands inside her cells were wound into chromosomes. It also influenced how other genes, such as those involved in early brain development, were expressed.

The condition associated with the *CHD3* mutation is called Snijders Blok-Campeau syndrome and affects only 237 individuals worldwide, though this is likely underrepresented due to the ultra rare nature. Those with the mutation have a normal life expectancy but may have symptoms like a slightly large head and speech and language issues, and around two-thirds have intellectual deficits. Moderate to severe cases may include being nonverbal, experiencing seizures and heart problems, and having fluid-filled voids in their heads.

Mei's condition was on the milder side of the spectrum, but her parents continued to worry that the gap between her and her peers would only grow with each grade and that she might never be able to live independently.

Her mom, Linda, quit her job so she could focus completely on her only child. They took Mei to speech and occupational therapists and got her special education support. Although she was transferred to another kindergarten to repeat a year, her parents said most of the other parents were unaware of her condition.

A bespoke treatment

When Linda and Jason joined a private WeChat group where parents of children with autism and similar conditions shared advice, they heard about Zilong Qiu, an expert in neurodevelopmental disorders who was working on a gene therapy for [Rett syndrome](#), a neurological condition that can cause severe impairments in language and coordination. After the therapy's recent patent in China, Qiu founded Lanqui Xintu Gene Technology, which planned to bring the therapy into the clinic for testing. Parents in the chat followed Qiu's work closely, as he was one of several researchers worldwide pushing to use base editors for custom treatments for children with rare diseases. A parent in the group had attended one of Qiu's lectures about his work and other efforts and posted slides in the chat.

The following week Jason emailed Qiu about his daughter's condition and told him they knew gene therapy could be expensive but were willing and able to bear the costs.

"Watching our child suffer every day is the most painful ordeal for us as parents," he wrote.

According to recordings and communications provided to [Science and Retraction Watch](#), Qiu told the parents it could take up to two years to develop the treatment and test it in mice and monkeys, and there was no guarantee it would be effective. Getting the trial approved by the hospital's ethics board would also be difficult because Mei's condition was not life-threatening.

"The psychological cost of doing this is very high for me," [Linda told Qiu](#). "But I am willing to try this because you have proven to be very trustworthy."

Jason and Linda began wiring payments to a foundation affiliated with another university, which would support one of Qiu's former students who was designing the base editor. Their money went to other organizations as well, and Qiu also asked them to pay other members of the research team directly through informal arrangements.

In April 2024, Jason told one of the researchers that he was at a financial breaking point after discovering the production cost of clinical-grade viruses would exceed \$250,000. Altogether, the family put \$860,000 toward developing the therapy, using their savings and money from relatives.

By the end of 2024, Qiu's team had engineered mice to have a human version of the *CHD3* gene carrying Mei's mutation, R1025W, which results in a protein with the amino acid tryptophan where there should be an arginine.

When researchers repaired the mutation, the mice developed normally. Base editors like this use a modified version of CRISPR's DNA-cutting enzyme, but unlike traditional CRISPR editing, which breaks both strands of DNA's double helix to make an edit, base editors only nick a single strand. The treatment was designed to rewrite the mutated gene in Mei's neurons, restoring the needed DNA base so she could make a vital protein. However, there is no published evidence establishing that the treatment edited enough of Mei's neurons to produce a meaningful recovery of CHD3 protein, nor is there evidence indicating that on-target gene editing caused her death.

Mei's treatment happened around the same time another base-editing therapy made headlines. In early 2025, [KJ Muldoon](#), an infant with a life-threatening metabolic disorder, had received his first intravenous infusion of a bespoke base-editing treatment at the Children's Hospital of Philadelphia. While Baby KJ's base editor was delivered into his liver via tiny nonviral fatty particles, Mei's treatment utilized a viral vector administered into cerebrospinal fluid to reach her brain.

Researchers chose to package genes encoding the editor's enzyme and guide RNA into adeno-associated viruses (AAVs), a common gene therapy delivery vehicle. However, it can trigger a severe immune response, especially at high doses. Only a small fraction of these viruses enter their target cells and trigger production of the base-editing components, so fixing enough brain cells would require injecting Mei with trillions of viruses.

The base editor itself was too large to fit inside a single AAV, so researchers had to split its genetic instructions between two AAV9 vectors, which were injected simultaneously into Mei's spinal canal. The approach allowed the oversized editor to be delivered, but it came with a tradeoff: because both vectors needed to reach the same cells and reconstitute the editor, the treatment required a high number of viral particles. High-dose AAV therapies have been associated in other programs with serious systemic toxicities, including complement-mediated thrombotic microangiopathy (TMA) and thrombocytopenia. Thus, even though the AAV9 vectors were delivered into the CNS to bypass the blood-brain barrier, the dose and potential for systemic exposure remained important safety considerations.

Warning signs dismissed

In January, Qiu sent Jason and Linda a manuscript he had submitted to Nature showing his lab's preclinical work on Mei's mutation over the previous 18 months. It included the parents' genetic data and acknowledged their support.

The manuscript also included images of brain slices from two macaques that had received Mei's potential therapy via AAV9. Imaging stains suggested the editor had made it into almost every neuron in the area where *CHD3* is most strongly expressed. The results helped convince the hospital ethics committee to approve the clinical trial on January 2, 2025.

Experts who later assessed the figure, however, expressed concerns about the quality of the primate proof of concept, and believe Nature should request raw data and images from the authors. The original submitted paper had no contemporaneous control samples demonstrating that the stain selectively highlighted the gene editor, which one expert said meant there was no confidence that the image wasn't mostly background staining.

Additionally, about a month before Mei's treatment, the primate toxicology study found that all four monkeys that received the therapy, at both low and high doses, had moderate to severe liver damage. One monkey that received a higher dose also showed kidney damage. [Thrombotic microangiopathy](#) (TMA), a potentially fatal condition where small blood clots form in tiny blood vessels, is a known reaction to AAV gene therapy and could have caused the monkey's kidney damage. However, because the relevant data was not collected during the first month after the monkey's infusion, it is impossible to know the exact cause.

The hospital ethics committee did not review the final report from the primate safety study before approving Mei's trial. According to [Science and Retraction Watch's investigation](#), Qiu explained the findings to Jason and Linda but appeared unconcerned about their importance.

In February, when the family arrived at Xinhua Hospital to sign the consent form and have tests done on Mei's blood, the document listed TMA as a possible outcome requiring immediate treatment. The doctor overseeing the clinical side of the study, Yongguo Yu, told the parents the biggest risk was if Mei had antibodies to the viral vector, but otherwise "the data shows it's relatively safe."

Tests showed that Mei did not have antibodies to the vector. To help reduce her immune response, she would receive the steroid prednisone. Many gene-therapy protocols use more potent immunosuppressive drugs than prednisone alone. But because this was an investigator-initiated trial rather than a commercially sponsored trial overseen by China's National Medical Products Administration (NMPA), the treatment was subject to a different regulatory framework. While investigator-initiated research can have important benefits, in Mei's case, decisions regarding immunosuppression, preclinical toxicology, and serious adverse-event reporting were not subject to the same regulatory oversight as they would have been in the commercial drug-development pathway — a difference that proved consequential.

The informed consent form also stated that "participation in this study will not incur any additional costs to you. All expenses will be borne by the sponsor Lanqui Xintu Gene Technology." Qiu explained this contradiction by telling Jason and Linda that the money they had paid meant they had a stake in the company. However, regardless of the type of trial, it is illegal under Chinese law to charge for an unproven therapy.

A devastating outcome

The day before Mei's procedure on March 23, 2025, the family shared a hospital room with another little girl. When her roommate was crying, Mei handed her one of her favorite stuffed animals. Her father described her as someone who liked to give things to other kids.

The next day, her spinal canal was filled with a high-titer / high-dose dual-AAV9 infusion into CSF as she underwent the procedure. Three days later, Mei developed a fever. Qiu assured Jason and Linda it was a common AAV reaction and that her condition would improve soon. But Mei didn't get better.

She wasn't urinating, signaling that her kidneys were seriously injured. Her fluid intake had to be reduced, making her extremely thirsty, and her blood platelet levels were dangerously low. These were among the complications the consent form had warned her parents about, but nowhere did it state they could end in death. Nor, according to her parents, had Qiu or the other doctors warned them of the fatal risk.

Mei was taken to the intensive care unit. Before entering the ward, she told her mom she wanted to go home. Jason and Linda sat in the waiting room with Mei's grandparents for the next 24 hours. Qiu joined them after hearing about Mei's state and was with the family the following morning when they were told Mei had died.

The hospital's ethics board, the same one that had approved the treatment, later concluded her death was "definitely related" to the treatment. Her cause of death was thrombotic microangiopathy.

The hospital was fined \$3,600 in September 2025 by the district health department for failing to adequately oversee the trial and failing to register it as commercially sponsored research in the national database.

A trial published but a story hidden

In the weeks following Mei's death, Qiu expressed his sadness to the family but refused to speculate about the cause. Jason and Linda told [Science and Retraction Watch](#) that they asked him to retract the Nature paper because they worried it might mislead researchers and other families with similarly affected children. Although Qiu initially agreed, he eventually stopped replying.

The parents couldn't bear returning to their old apartment, where all of Mei's possessions still sat, so they moved into a hotel and then a new apartment. They stopped replying to and seeing some of their closest friends, not knowing what to say. They blamed themselves for not asking more questions.

Almost a year after Mei's death, they learned that Qiu's study had been published in Nature. The section about the family's genetic data was no longer present, and the sentence thanking them for their participation and support had been removed.

While the paper was still about base-editing therapy for Mei's mutation, the data showing how the treatment worked in the monkeys' brains was new. Control samples had been added, though they were produced at a different time than the treated samples and used different microscope settings, which experts said makes comparisons difficult. The mouse experiments had also been redone. Descriptions of the kidney and liver problems observed in the primate safety study were omitted.

The paper was well received, with scientists calling the results promising and Chinese state media reports describing the work as a ray of hope for families.

When Jason and Linda saw families in their WeChat group saying they would reach out to Qiu about their own children, they filed a complaint with Qiu's university dean, demanding an investigation and the

retraction of the Nature paper. The university later told the parents it was taking no action against Qiu and that the Nature paper was unrelated to the experiments they had funded.

Nature said it had been unaware of Mei's death during review and publication. Victoria Aranda, Nature's deputy editor, wrote that the ethical issues raised by the parents "fall outside our purview in terms of data integrity" and should be handled by the university. Nature later posted an editor's note on data concerns; that note, not the earlier reply to the family, is the journal's current public stance (see below).

In a later statement responding to questions from Science and Retraction Watch, the journal said it had also been unaware of the issues or sanctions the hospital faced before publishing the paper. Francesca Cesari, Nature's chief editor for biological, clinical, and social sciences, said that had relevant information been disclosed during peer review, it would have been thoroughly considered in the decision-making process. Other experts who reviewed the paper said Nature should request and analyze the raw data and image files and that the study's sources of funding should be fully disclosed.

What Mei's case means for gene-editing research

The case has been compared to earlier Chinese gene-editing scandals, particularly He Jiankui's 2018 [CRISPR-baby incident](#), and has raised questions about oversight of investigator-led trials, informed consent, transparency around adverse events, and the governance of experimental gene-editing therapies. Despite China tightening some rules following He Jiankui's experiment, sociologists and ethicists argue that a gap remains between formal regulations and actual practice, especially around adverse-event reporting.

After Mei's death became public, the Chinese Society of Gene & Cell Therapy (CSGCT) said the case underscored the need for robust preclinical evidence, rigorous ethical governance, transparency, and timely reporting of serious adverse events. It pointed to China's new national framework for investigator-initiated research, [State Council Order No. 818](#), as an effort to strengthen oversight and patient protection.

The story, however, did not end with those statements. Following the July 2026 Science/Retraction Watch investigation, Shanghai Jiao Tong University School of Medicine announced a comprehensive inquiry on July 26th. The school said it took the reports "very seriously," established a special working group to investigate both Qiu's Nature paper and the Xinhua Hospital clinical study, and said "serious action" would follow the findings. As of September 2026, no public report, sanction, or personnel action has been announced.

On July 29th, Nature added a note flagging concerns about the data in the February 2026 preclinical paper. As of September 2026, the paper remains published and citable, and Nature has not issued a correction that adds the human death, family funding, or the primate liver and kidney injury. An editor's note states further action will follow an investigation and an opportunity for all parties to respond.

The ClinicalTrials.gov record was later updated to state that the sole participant died on March 31, 2025, of treatment-related thrombotic microangiopathy (TMA). By July 30, 2026, China Daily reported that the record was marked terminated and said the death was "assessed as definitely related to the study drug." The only publicly reported penalty remains the September 2025 local fine of about \$3,600 against Xinhua

Hospital. No on-record scientific rebuttal from Qiu or Yongguo Yu has appeared in the follow-up Nature News or Science coverage, and no confirmed suspension of either has been reported.

By September 2026, the case was also being considered alongside a second, separately disclosed pediatric gene-editing death (2) in China and in the context of the country's Order 818 rules for investigator-initiated advanced therapy research. Those developments have broadened the oversight debate, but they do not by themselves resolve the outstanding factual questions surrounding Mei's dosing, the primate toxicology file, or the integrity of the published images. Until the university and the journal publish their findings, much remains unknown, and descriptions of the matter as "resolved," "covered up with no aftermath," or "officially retracted" would be inaccurate.

This real-life story is not meant to implicate specific research hospitals or investigators, or investigator-initiated research in general, but rather to emphasize the critical necessity for timely and transparent communication within the scientific community and the incredible importance of preclinical safety studies. Enthusiasm for innovation cannot replace caution when signals present themselves in well-designed, thorough preclinical safety investigations.

References:

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