Breaking the sickle: Ugandan who rewrote medical history

After 35 years of consistent pain, Allan Byamukama became one of the few individuals in the world cured of sickle cell disease through gene therapy.

BY BEATRICE NAKIBUUKA

Born in Uganda and diagnosed with sickle cell disease as an infant, Byamukama grew up frequently in and out of hospitals, missing school, sitting on the sidelines while other children played, and learning early that his body followed different rules.

Sickle cell disease is a genetic blood disorder passed down through families. It affects haemoglobin, the protein in red blood cells responsible for carrying oxygen throughout the body. In people with this condition, red blood cells become hard, sticky, and curved like a sickle, blocking blood flow and depriving organs of oxygen. The result is episodes of severe pain known as crises.

In Uganda, the disease is common. Thousands of children are born with sickle cell disease each year, and many do not survive to adulthood. Those who do often face repeated hospital visits, chronic pain, anaemia, infections, and organ damage.

Byamukama began showing signs of the disease at just five months old. His eyes turned yellow, his fingers and toes swelled painfully, and he cried constantly. Tests at a local hospital confirmed his parents' fears.

As he grew older, he quickly realised he was different. His mother forbade him from playing football with other children, fearing that too much exertion would trigger a crisis. Long hospital stays meant missed lessons and fractured friendships. Pain would arrive without warning, sometimes every few weeks and other times after months of calm.

When a crisis struck, his parents rushed him to the hospital for strong painkillers.

"I am thankful to them for never giving up on me," Byamukama says. "They did everything they could with what was available."

The limits of care at home

For the first 25 years of his life, Byamukama managed sickle cell disease in Uganda. As many patients, his treatment focused on alleviating symptoms rather than preventing them. Painkillers, blood transfusions, and careful monitoring helped him survive, but they did not stop the disease from tightening its hold.

As he grew older, complications increased. Sickle cell disease can shorten life expectancy by two to three decades and raises the risk of stroke, heart problems, and organ failure. For Byamukama, it also meant witnessing his dreams fade as his body weakened.

In 2015, at the age of 28, he moved to the United States, joining family in the hope that better healthcare would bring relief. He settled in Massachusetts, continued his education, and began working with a nonprofit organisation. He also started seeing a haematologist regularly.

For a time, things seemed manageable. However, the pain crises gradually became more frequent and severe. At his worst, he found himself visiting the emergency room every few days. In March 2025 alone, he went to the emergency room 42 times.

"It felt like my life was slipping away," he says. "I was studying and working, but always planning around pain."

A new option emerges

It was during a clinic visit at Massachusetts General Hospital's Comprehensive Sickle Cell Disease Treatment Centre that Byamukama first learnt about gene therapy. At the time, it was still experimental and awaiting approval from the United States Food and Drug Administration (FDA).

"When my doctor mentioned it, I did not hesitate," he recalls. "If there was even a chance it could change my life, I wanted it," he says.

In December 2023, the FDA approved a gene therapy called Casgevy for sickle cell disease. Massachusetts General Hospital was among the first centres authorized to offer the treatment. Byamukama was identified as a strong candidate because his disease was severe, other therapies had failed, and his quality of life was rapidly declining.

Dr Sharl Azar, the medical director of the sickle cell clinic, remembers the urgency of his situation. His frequent hospitalisations and escalating pain raised concerns about his long-term survival. Equally important, she adds, was his determination: "He was deeply invested in trying to live a normal life."

Inside the science of hope

Gene therapy for sickle cell disease is not a simple injection; it is a long, intense, and risky process that can take about a year.

Doctors first collect the patient's own stem cells, cells that produce blood, during several hospital stays. The cells are sent to a laboratory, where scientists edit them using advanced gene-editing techniques. The goal is to reactivate fetal haemoglobin, which does not sickle and can protect red blood cells from damage.

Once the edited cells are ready, the patient undergoes intensive chemotherapy to destroy the existing bone marrow and make room for the new cells. The edited stem cells are then transplanted back into the body. Recovery involves a lengthy hospital stay and close monitoring.

"It is truly a team effort," Dr Azar says. Haematologists, transplant specialists, nurses, social workers, and patient navigators all play vital roles. In Byamukama's case, Dr Richard Newcomb led the transplant process, while Dr Azar managed sickle cell-related complications.

A turning point

When Byamukama completed his treatment, the results were almost immediate. Blood tests revealed that his haemoglobin levels were rising and that his red blood cells were living longer. Most importantly, the painful crises he had experienced stopped.

Weeks turned into months without a single visit to the emergency room. For the first time in his life, he could plan for the future without fear. "I once asked God for just one pain-free year before I died," he says. "But He gave me so much more."

By August 2025, doctors declared him free of sickle cell disease. He became the first Ugandan to be cured through gene therapy and only the 15th person worldwide to receive the FDA-approved treatment. He is also the second Ugandan ever cured of sickle cell disease, following seven-year-old Miriam Mulumba, who underwent a bone marrow transplant in 2008.

"I am not cured in the sense that nothing can ever go wrong," he adds. "But the disease no longer controls my life."

The cost of a miracle

Byamukama's story is inspiring but highlights a painful reality. The treatment cost $2.1 million (about Shs7.5 billion) and was covered by insurance in the United States. Such a figure is unimaginable for most families in Uganda and far exceeds the public health budget.

"This would have been impossible at home," he admits. "That is the hardest part of this story."

Many Ugandan patients continue to manage sickle cell pain with basic painkillers such as paracetamol. Access to specialised care is limited, especially outside major cities. Misconceptions and stigma persist, leading some families to hide affected children or turn to traditional remedies.

Gene therapy remains out of reach for most Ugandans today, but it illustrates what is scientifically possible. For Byamukama, the future feels open for the first time.

"I thought I would not live past 18," he says. "Now I am 35 and healthy," he adds.

His journey from a sickly infant in Uganda to a global medical milestone is extraordinary and serves as a reminder that medical miracles matter most when they are accessible to all.

FREQUENTLY ASKED QUESTIONS

What are the benefits of sickle cell gene therapy?
Gene therapy may reduce or stop red blood cells from sickling, helping them live longer and flow better. This can lead to fewer pain crises, less fatigue, reduced hemolysis, and improved quality of life, including emotional and physical wellbeing.

Why is chemotherapy used in gene therapy?
Chemotherapy prepares the bone marrow to receive the modified stem cells. While necessary, it carries risks such as infections, hair loss, mouth sores, and infertility.

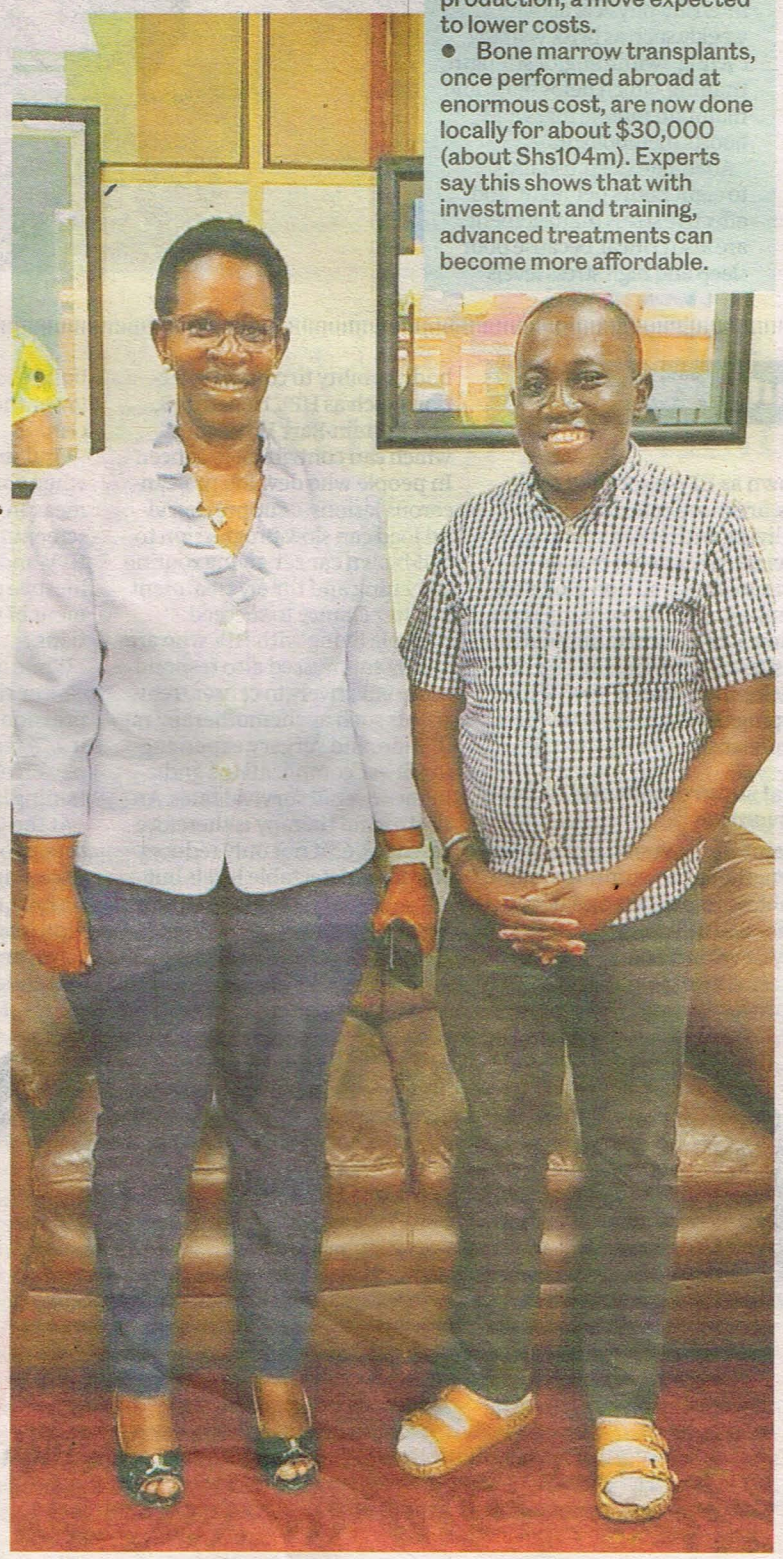
Can I have children after gene therapy?
Chemotherapy may affect fertility, so fertility preservation options should be discussed before treatment.

Can I still pass the sickle cell gene to my children?
Yes. Gene therapy does not change sperm or egg DNA, so the sickle cell gene can still be inherited.

What are the risks of gene therapy?
Risks include chemotherapy side effects, possible treatment failure, unknown long-term effects, and a small but uncertain cancer risk.

SIGNS OF PROGRESS

- Despite these challenges, there are signs of progress. Newborn screening for sickle cell disease is now free in all government hospitals. Adults can also test their status at public facilities at no cost, and premarital screening is encouraged.
- Hydroxyurea, a drug that reduces pain crises and complications, is becoming more widely available. In October 2025, the Minister of Health announced that a Ugandan pharmaceutical company will soon begin local production, a move expected to lower costs.
- Bone marrow transplants, once performed abroad at enormous cost, are now done locally for about $30,000 (about Shs104m). Experts say this shows that with investment and training, advanced treatments can become more affordable.



Allan Byamukama poses for a photo with Diana Atwine, the Ministry of Health permanent secretary. PHOTO/COURTESY