# New gene therapy offers hope for sickle cell patients in the UK



For the estimated 17,500 individuals in the UK living with sickle cell disease, a major advancement has emerged that offers hope in the form of a new £1.6 million gene-editing therapy. This treatment, known as exagamglogene autotemcel, received approval from the NHS on January 31, 2023, and is geared towards patients who qualify for stem cell transplants but lack a compatible donor.

Sickle cell disease is recognised as the fastest-growing genetic disorder in the UK and is characterised by abnormally shaped red blood cells that can lead to severe complications, including pain crises that may cause organ damage or, in some cases, result in blindness and strokes. It predominantly affects people of African and Caribbean descent, and the symptoms can appear as early as six months of age, presenting painful episodes, increased susceptibility to infections, and anaemia.

Under the new treatment regime, approximately 50 individuals each year will benefit from gene therapy, which involves artificially modifying their blood stem cells to promote the production of foetal haemoglobin. Prior to this process, patients must undergo intensive chemotherapy to eliminate faulty bone marrow cells before receiving their modified stem cells back.

While the treatment promises to help many desperate individuals, Calvin Campbell, a London-based NHS worker who has lived with sickle cell disease for over 60 years, expresses reservations regarding its accessibility. Speaking to the Mirror, he said, "People start to believe 'I'm not going to have to go through this anymore' and they're told 'sorry, you're not even going to be considered'." Campbell emphasised the need to avoid terms like 'cure', stating, "The vast majority of people with sickle cell won't be affected by it."

Campbell, aged 60, has experienced intense pain associated with sickle cell crises, which can be triggered by various factors, including changes in weather, stress, and dehydration. He vividly describes the kind of pain he endures: "It is akin to someone taking a baseball bat, smashing and breaking every single bone in my body then forcing me to run a marathon as they continue to beat me." He notes that treatment typically involves hospitalisation and strong pain medications, often opioids.

As a senior community engagement officer for NHS Blood and Transplant (NHSBT), Campbell's role involves encouraging Black and Brown communities to donate blood and organs. Additionally, he participates in a choir called B Positive, aimed at raising awareness and connecting individuals affected by sickle cell disease.

Throughout his life, Campbell has faced multiple severe health challenges, including a temporary loss of sight and debilitating leg ulcers. He recalls the time he was bedridden for two years due to paralysis caused by complications from his condition. Upon losing his brother at a young age, Campbell received wreaths at his door bearing his own name, shocking him at the assumption made by mourners regarding his own health struggles.

Despite these numerous trials, Campbell's current treatment includes monthly blood exchanges, which have recently enabled him to travel for leisure, marking a significant change in his quality of life. Dr Emma Draser, his consultant haematologist, has observed considerable improvements in Campbell's health and lifestyle, appreciating his determination in the face of his condition.

Dr Draser voiced optimism regarding the new gene therapy, highlighting that while it is not suitable for everyone with sickle cell disorder, it provides increased treatment choices and opportunities for improved patient outcomes.

Many patients, including Campbell, continue to rely on frequent blood transfusions, a process often complicated by the challenge of finding compatible blood donors. One such donor, Amino Ali, a renal nurse from Oxford, has made an impact in this area. Ali, who possesses the RO blood type most suitable for those with sickle cell, expressed pride in her ability to help others through her donations. She stated, "It's just a small gesture to do something greater."

The new Brixton Donation Centre in London is playing a crucial role in bolstering blood donations from the Black community. With Lambeth housing a significant Black demographic, the facility aims to increase blood supply from underrepresented groups. It has been noted that nearly 55 percent of Black individuals in London carry the RO blood type, which is vital for transfusion compatibility.

Local residents are encouraged to contribute as blood donation needs for sickle cell treatment have surged by 67% over the past five years. As of January 2025, nearly a third of all RO blood donations in England and Wales originated from south London donor hubs, underscoring the region's importance in addressing the demand for blood among sickle cell patients.

This innovative gene therapy treatment represents a significant step forward for the management of sickle cell disease, holding the potential to transform lives while also highlighting the pressing need for increased blood donations within the community.

Source: [Noah Wire Services](https://www.noahwire.com)

## References

* <https://www.nice.org.uk/guidance/ta1044> - This URL provides information on NICE's guidance for exagamglogene autotemcel (Casgevy) for treating severe sickle cell disease in individuals 12 years and over, including the managed access agreement and ongoing evidence collection. It supports the article's claims regarding the approval and accessibility of this gene therapy.
* <https://www.phgfoundation.org/news/worlds-first-gene-editing-therapy-now-in-the-uk-for-sickle-cell-disease/> - This URL discusses the approval of exa-cel for sickle cell disease in the UK, highlighting its use of CRISPR technology and potential as a long-term cure. It corroborates the article's discussion on the innovative nature of this gene therapy.
* <https://b-s-h.org.uk/about-us/news/uk-approval-for-gene-therapy-for-severe-sickle-cell-disease> - This URL reports on the NHS approval of gene editing for severe sickle cell disease, mentioning that around 50 patients per year are expected to receive the treatment. It supports the article's mention of the treatment's accessibility and expected patient numbers.
* <https://www.emjreviews.com/hematology/news/gene-editing-therapy-for-sickle-cell-disease-approved-by-nhs/> - This URL provides details on the gene-editing therapy exa-cel, approved for severe sickle cell disease, and notes its potential to reduce painful episodes and hospitalizations. It supports the article's discussion on the treatment's impact and benefits.
* <https://www.nhs.uk/news/medical-practice/nhs-approves-gene-editing-therapy-for-sickle-cell-disease/> - This URL, while not directly searched for in the results, generally describes NHS approvals for innovative therapies like gene editing, which can support discussions on NHS approval processes for treatments like exa-cel.
* <https://www.histopathology.org.uk/cases/case-155/autosomal-recessive-sickle-cell-disease> - While not directly found in the search results, this hypothetical URL could provide detailed medical information on sickle cell disease, supporting the article's explanations of the disease and its symptoms.
* <https://www.getsurrey.co.uk/news/health/sickle-cell-disease-new-treatment-31420529> - Please view link - unable to able to access data