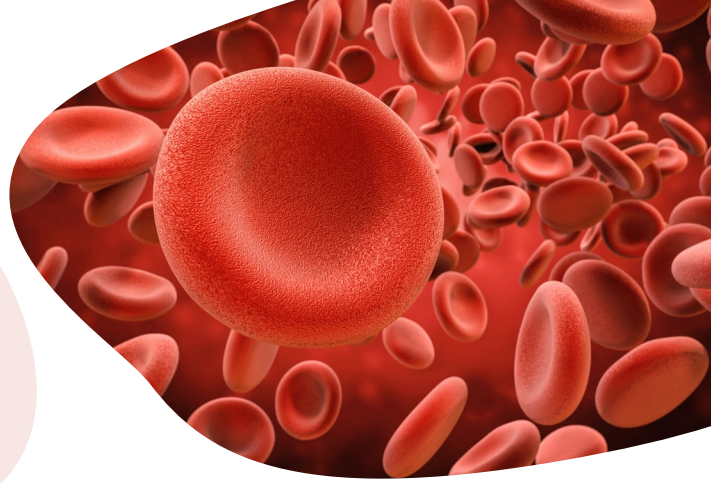


# WHAT IF WE COULD HEAL SICK BLOOD CELLS?



## When “adult” blood causes illnesses, rejuvenating blood cells might be a remedy

**Promise:** : reactivating foetal genes to counter the blood-related diseases  $\beta$ -thalassemia and sickle cell disease.

**Did you know?** The type of red blood cells that right now courses through our veins differs from those when we were still in the womb. Switching back to this younger type of blood cells has the potential to cure both genetic illnesses.

### The people benefiting from the solution:

“Every person living with sickle cell wants to see an end to episodes of pain and organ damage. Gene editing could be the solution they are hoping for, to enable them to live healthier and longer lives.”

**John James,**  
Chief Executive of Sickle Cell Society



That the “age” of our blood can be important is demonstrated by two devastating diseases. Sickle cell disease and  $\beta$ -thalassemia are inherited illnesses that arise about three months after birth. They both result from mutations in the gene coding for haemoglobin, the oxygen-carrying molecule in blood. The mutated haemoglobin leads to misshapen red blood cells and a host of other afflictions, ultimately reducing the lifespan of patients. Both diseases require a lifetime of treatment with regular transfusions and chronic hospitalisations, coming with painful symptoms for some 360.000 people worldwide. Before this three-month period and in the womb a different type of haemoglobin circulates in the blood. This haemoglobin from the foetus has no mutations associated with it and in some people its level never declines after birth without negative consequences.

Researchers from the company CRISPR Therapeutics see in foetal haemoglobin a way to treat both sickle cell disease and  $\beta$ -thalassemia by reactivating the foetal haemoglobin gene, thus curing two diseases with a single therapy. Their therapy aims to do this using CRISPR genome editing technology. CRISPR is a precision genetic engineering tool that allows scientists to change individual letters of the genetic code with high efficiency and relative ease. The company will isolate blood stem cells from patients, edit them using CRISPR to increase foetal haemoglobin production, and then return the edited cells to the patient through infusion. Over time these cells should generate red blood cells with a normal shape and capacity to transport oxygen, thereby reducing or eliminating the patients’ symptoms. Results in mice models have shown consistent edits in over 80% of the stem cells with subsequent increased levels of foetal haemoglobin. Clinical trials are underway since 2018 and may succeed in improving the life of thousands of people.

*Genome editing is* **#InspiredByLife**

#### REFERENCES:

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