# Article information:

Base editing of hematopoietic stem cells rescues sickle cell disease in mice - PMC
<https://www.ncbi.nlm.nih.gov/pmc/articles/PMC8266759/>

# Article summary:

1. Researchers used a custom adenine base editor to convert the sickle cell disease allele to a non-pathogenic variant in hematopoietic stem and progenitor cells (HSPCs) from SCD patients.

2. Ex vivo delivery of the base editor resulted in 80% conversion of the sickle cell allele to the non-pathogenic variant.

3. Transplantation of edited HSPCs into mice showed durable editing, with reduced sickling and rescue of hematologic parameters, suggesting a potential one-time autologous treatment for SCD.

# Article rating:

Appears strongly imbalanced: The article is written in a biased or one-sided way, and the information it provides is not trustworthy enough to be considered a reliable source. You should consult other sources to find reliable information on the presented issues.

# Article analysis:

The article titled "Base editing of hematopoietic stem cells rescues sickle cell disease in mice" discusses a study that explores the use of base editing to treat sickle cell disease (SCD) in mice. The authors claim that they were able to convert the SCD allele to a non-pathogenic variant using a custom adenine base editor. They also state that this approach resulted in durable editing and rescue of hematologic parameters in mice.

One potential bias in this article is the lack of discussion on potential risks and limitations of base editing. While the authors briefly mention that base editing avoids p53 activation and larger deletions observed with Cas9 nuclease treatment, they do not thoroughly explore other potential risks associated with base editing, such as off-target effects or unintended consequences of genomic modifications.

Additionally, the article does not provide a balanced view by presenting both sides of the argument regarding the efficacy and safety of base editing for treating SCD. It primarily focuses on the positive outcomes of the study without discussing any potential drawbacks or limitations. This one-sided reporting could give readers an incomplete understanding of the topic.

Furthermore, there are some unsupported claims in the article. For example, the authors state that their findings suggest a "one-time autologous treatment for SCD." However, this claim is not supported by sufficient evidence from human studies or clinical trials. It is important to note that this study was conducted in mice, and extrapolating these results to humans may not be accurate.

The article also lacks exploration of counterarguments or alternative approaches to treating SCD. While it briefly mentions other experimental therapies, it does not discuss their potential advantages or disadvantages compared to base editing. This omission limits the reader's ability to critically evaluate the presented approach.

Overall, this article appears to have promotional content as it focuses on highlighting positive outcomes without adequately addressing potential risks or limitations. It also lacks a comprehensive analysis of alternative approaches and counterarguments. Therefore, readers should approach the information presented with caution and seek additional sources to gain a more balanced understanding of the topic.

# Topics for further research:

* Potential risks and limitations of base editing in gene therapy
* Off-target effects of base editing in genetic modification
* Unintended consequences of genomic modifications in base editing
* Efficacy and safety of base editing for treating sickle cell disease
* Alternative approaches to treating sickle cell disease
* Comparison of different experimental therapies for sickle cell disease

# Report location:

<https://www.fullpicture.app/item/b74f7e442a5243c2191d6b15348269d5>