

Emerging Therapeutic Options for Sick Cell Anemia: Enhancing the Current Predominant Treatment

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May 1, 2023

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Dear Editor,

We have read the article entitled "Trends in blood transfusion, hydroxyurea use, and iron overload among children with sickle cell disease enrolled in Medicaid, 2004–2019" by Amy et al. [1] with great interest and appreciate the effort put into the research. However, upon reviewing the manuscript, we have identified a few ideas that we believe are worth discussing to enhance the overall quality of the article. The study's results indicate that hydroxyurea is effective in reducing hospitalization rates, inpatient days, pain events, ER visits, episodes of acute chest syndrome, and transfusions, and improving hemoglobin levels. The research also demonstrates that hydroxyurea has a greater impact on younger children than older ones and that the change in Hb F levels varies in different studies. Despite some limitations, the study provides valuable information on the effectiveness of hydroxyurea in treating Sick Cell Anemia (SCA) in real-life settings.

To enhance the current predominant treatment, additional therapeutic choices such as l-glutamine, crizanlizumab, and voxelotor have become accessible in addition to hydroxyurea. Hematopoietic Stem Cell Transplantation (HSCT) with fully matched donors is regarded the most effective treatment for SCD patients who are unresponsive to hydroxyurea. [2]

In addition, an ongoing phase 1-2 trial of LentiGlobin gene therapy for Sick Cell Disease involves transplanting autologous hematopoietic stem cells transduced with a lentiviral vector carrying a modified -globin gene, resulting in the synthesis of anti-sickling hemoglobin. The study included 35 individuals who had at least four severe vaso-occlusive episodes within the previous 24 months. Engraftment happened in all patients, with the majority of red cells continuing to produce HbAT87Q, resulting in less hemolysis and complete remission of severe vaso-occlusive disease. [3]

In conclusion, the article by Amy et al. provides valuable insights into the effectiveness of hydroxyurea in treating sickle cell anemia in real-life settings. However, with the emergence of additional therapeutic options such as l-glutamine, crizanlizumab, and voxelotor, as well as hematopoietic stem cell transplantation and gene therapy, the current predominant treatment for sickle cell anemia can be enhanced. These new treatments have shown promising results in reducing the frequency and severity of vaso-occlusive episodes and improving overall outcomes for patients with sickle cell disease. Future research should focus on evaluating the long-term efficacy and safety of these therapies and identifying the most appropriate treatment options for different patient populations.

References:

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