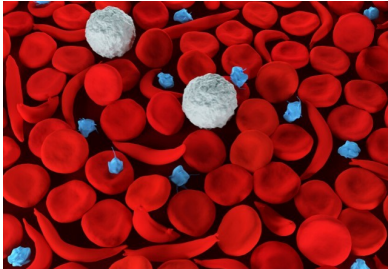


# Errant Gene Therapeutics' Gene Therapy Vector Shows Promise for Sickle Cell Disease and Thalassemia Patients



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The **abstract** submitted to the European Hematology Association by Dr. Simona Raso for a new analysis of the safety and efficacy of Errant Gene Therapeutics' vector, technically "human globin TNS9.3.55," shows promising results and potentially offers newfound hope for the 1.8 million worldwide who suffer from the rare diseases Sickle Cell Anemia and Thalassemia. The study analyzes the use of the EGT vector on three patients at Memorial Sloan Kettering from 2012 to 2015, with a five to seven-year follow-up.

Although a small investigational effort, two out of three patients examined had a sustained reduction in blood transfusions of 44%, and no unwanted clonal dominance was found. EGT emphasizes that reduced transfusions are a huge decrease in various risks to the patient. EGT notes that they made "the world's first commercial batch of gene therapy vector" in 2009, and this was used in the study. Today, making tweaks, which is commonplace, using anti-sicklers, insulators, enhancers, improved filtration, and other prep regimen drugs, EGT believes that the vector they produced in 2009 could meet, if not surpass, any competitor today at a far less cost for patients. Drug pricing is of paramount importance in many parts of the world.

Dr. Lucio Luzzatto, Professor of Haematology at Muhimbili University (Dar-es-Salaam, Tanzania) and former President of the FDA Gene Ethics Committee, says, "If things go well for EGT, I have a feeling many Sickle Cell and Thalassemia patients will have a new ray of hope."

EGT is in litigation with Bluebird Bio and Memorial Sloan Kettering Cancer Center concerning this vector. The trial is set for October 29, 2020.

*TrialSite News* has examined in reports such as "Family Man," "the optics" associated with big money, drugs, and research in a competitive context can look like a David vs. Goliath epic confrontation between different worlds, paradigms, and ways of being.

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