

## **Stakeholder views and attitudes towards prenatal gene therapy for sickle cell disease**

Makhoul L, Thomas I, Oteng-Ntim E, Nicolaides KH, Shangaris P, Lewis C  
King's College London, London, United Kingdom

### **Objective**

Sickle cell disease (SCD) is a severe, progressive and life-threatening disease caused by a single gene mutation. Despite its high prevalence, most treatments focus solely on symptomatic management. With the development of non-invasive prenatal diagnostic tests, detecting SCD from 10 weeks gestation has become easier, allowing for in-utero intervention, specifically gene therapy. This would introduce the corrected version of the gene to the fetus, thus curing the disease before birth. Given that this is a novel therapy, we wanted to explore stakeholder views towards this as a treatment option.

### **Methods**

To date, we have conducted 11 one-to-one semi-structured qualitative interviews via Zoom with stakeholders, including SCD patients and healthcare professionals who work with SCD patients. Interviews were digitally recorded, transcribed verbatim and analysed using inductive content analysis.

### **Results**

All participants were excited about gene therapy for SCD and were enthusiastic about a potential cure. Treatment before birth was considered advantageous, especially if the participant had SCD or had experience with family or friends. Common concerns were procedure safety and the likelihood of miscarriage, efficacy, and short/long-term side effects of gene therapy on the fetus. Complexities arose surrounding religion and culture and whether gene therapy would be widely accepted. In addition, particular emphasis was placed on clear information for parents to make an informed decision, as well as the need for psychological and practical support before, during and after. Nevertheless, 100% of participants stated that they would sign up for gene therapy if offered.

### **Conclusion**

Stakeholders support prenatal therapy for SCD and welcome this much-awaited advancement in management. Indeed, a positive opinion from stakeholders is critically important for the ongoing development of this therapy and this is reassuring. Moving forward, precise information and dialogue with religious/cultural principals is essential, as well as providing counselling and support for couples throughout.