

## ETHICAL CONCERNS ABOUT IN UTERO GENE EDITING FOR PEOPLE WITH HEMOPHILIA

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**Background & Objectives:** Hemophilia is an inherited lifelong debilitating disease for which current treatment is non-curative. While gene therapy, and gene editing are explored as potential curative alternatives, there is a need to evaluate the bioethical concerns that they implicate. In this study, we explored the ethical concerns about these new technologies in patients with hemophilia and their relatives in order to bring up important questions before the technologies are implemented.

**Method (s) and Results:** This qualitative study used semi-structured interviews on 22 patients with hemophilia A or B and their relatives across the U.S. Grounded theory was used to analyze the data. We had 21 participants between 18 and 67 years old, 19 of them had severe hemophilia. The themes about Genetic identity and Trustworthy Science were brought up when the participants talked about gene therapy (GT) and gene editing (GE). However, gene editing only brought up quotes about Respect for Persons (eugenics and the right for child of self-determination) and Transnational Cooperation to control the use of gene editing.

**Conclusions (Significance and Impact of the Study):** Ethical concerns are common in the hemophilia community regarding gene therapy and editing. While the results of clinical trials from gene modification therapies will clarify most concerns, gene editing elicits more ethical concerns compared to gene therapy and is less likely to be accepted among the community.

**Conflict of interest disclosure:** The authors disclose no conflicts of interest during the execution of this study.

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