

Patients' personal insights following a gene therapy clinical trial for hemophilia A

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Introduction

- Gene therapy (GT) is a novel therapeutic approach requiring education for clinicians and patients to aid treatment decision-making
- Reports of patient experiences during GT clinical trials provide valuable insights to support and prepare future trial participants
- Here, we conducted qualitative interviews with patients who received GT for hemophilia A in the clinical trial setting to better understand their perceptions of this treatment approach

Conclusions

- Patients considered GT to have benefited their lives, with all participants reporting significant improvements in their condition, mood and overall quality of life
- However, participants highlighted the need for more structured education and psychological, social and physical support at all stages of the process than is currently available

Methods

Retrospective semi-structured interviews to understand the decision-making process to participate in the Phase 3 study of valoctocogene roxaparvovec in hemophilia A, and life before and after treatment

Interviews were conducted with four male patients with severe hemophilia A (aged 23–37 years) who participated in the GT clinical trial at the Sheba Medical Center, Ramat Gan, Israel

Transcription of interviews, identification of common themes and qualitative analyses were completed by the lead author as part of her position at ALEH, Israeli Hemophilia Association

Results



Key insights emerging from the interviews

Patients' expectations of HCPs

It is important HCPs prepare patients for the physical and emotional challenges associated with any new drug but especially GT, by helping them understand the changes and fluctuations that are likely to occur, particularly in the first year after treatment

Need for structured support

Proactive support from allied HCPs including physiotherapists, social workers and psychologists would greatly assist patients during the first year after GT when they feel most vulnerable, along with support from patient associations/groups

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