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



¹ALEH, Israeli Hemophilia Association, Sheba Medical Center, Ramat Gan, Israel; ²National Hemophilia Center, Sheba Medical Center, Ramat Gan, Israel





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

1. Life before the clinical trial

- Two patients received prophylactic treatment since childhood while two patients received prophylaxis only in adulthood
- In childhood, two patients were not allowed by their parents to play sports, participate in school trips or other activities where injury might be a risk
- All patients had hemophilia-related symptoms and shared the physical difficulties and mental struggles they faced growing up and in adulthood
- It was important for all patients to live a normal life along with hemophilia

3. Decision to participate in the clinical trial

- Three patients shared their participation with their families; the fourth patient consulted with his partner and family before deciding to take part
- All patients spoke about the challenges of ongoing health problems, their desire to stop factor VIII (FVIII) replacement therapy and wish for their situation not to decline further
- They also factored in the state of the Israeli health system and the limited chance of GT being available in the future (due to cost)
- Although they expressed concerns about the likelihood of success and potential side effects, all patients ultimately decided to participate in the clinical trial in the hope of avoiding disabilities and improving their health

I was cut by a knife at work – I was waiting for a lot of blood to come out and suddenly it stopped, a miracle!

I'm going to bed without pain, getting up in the morning and nothing hurts

I started running

I can hold my baby with both hands

I don't think so much about whether to do an activity or not

5. Life after the clinical trial

I go to the gym

this – great

I don't limp

every day and I don't inject.
If they give me 10 years like

2. Getting into the clinical trial

- Each patient met the study criteria (NCT03370913): ≥18 years of age, no liver disease, no pre-existing adeno-associated virus 5 antibodies
- To better understand the GT clinical trial process, two patients sought further information independently whereas two patients were satisfied with the information provided by the study team

4. The first year of the clinical trial

- Despite responding well to GT, all patients were initially anxious about fluctuating FVIII levels and the impact of uncertain coagulation levels on their daily life
- After GT, all patients required corticosteroid therapy, which contributed to physical changes and emotional swings during the first year
- Two patients sought emotional support for their mood changes the same two who were more active in seeking information initially
- Although physical and psychological support were available, the patients felt unable to access them and indicated that more proactive assistance from the healthcare professionals (HCPs) would have been beneficial

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

