

# Haemophilia treatments survey

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What information does our community want about new haemophilia treatments? And for those who are affected, what would you like haemophilia treatment to achieve for you, or your child or partner, now and in the future?

In December 2022 and January 2023 Haemophilia Foundation Australia (HFA) conducted a community survey to find this out.

We are developing fact sheets on new haemophilia treatments and the answers to these questions will be very valuable to help us to know what to cover. They will also assist us with our other work – for example, contributing to the ideas when putting together

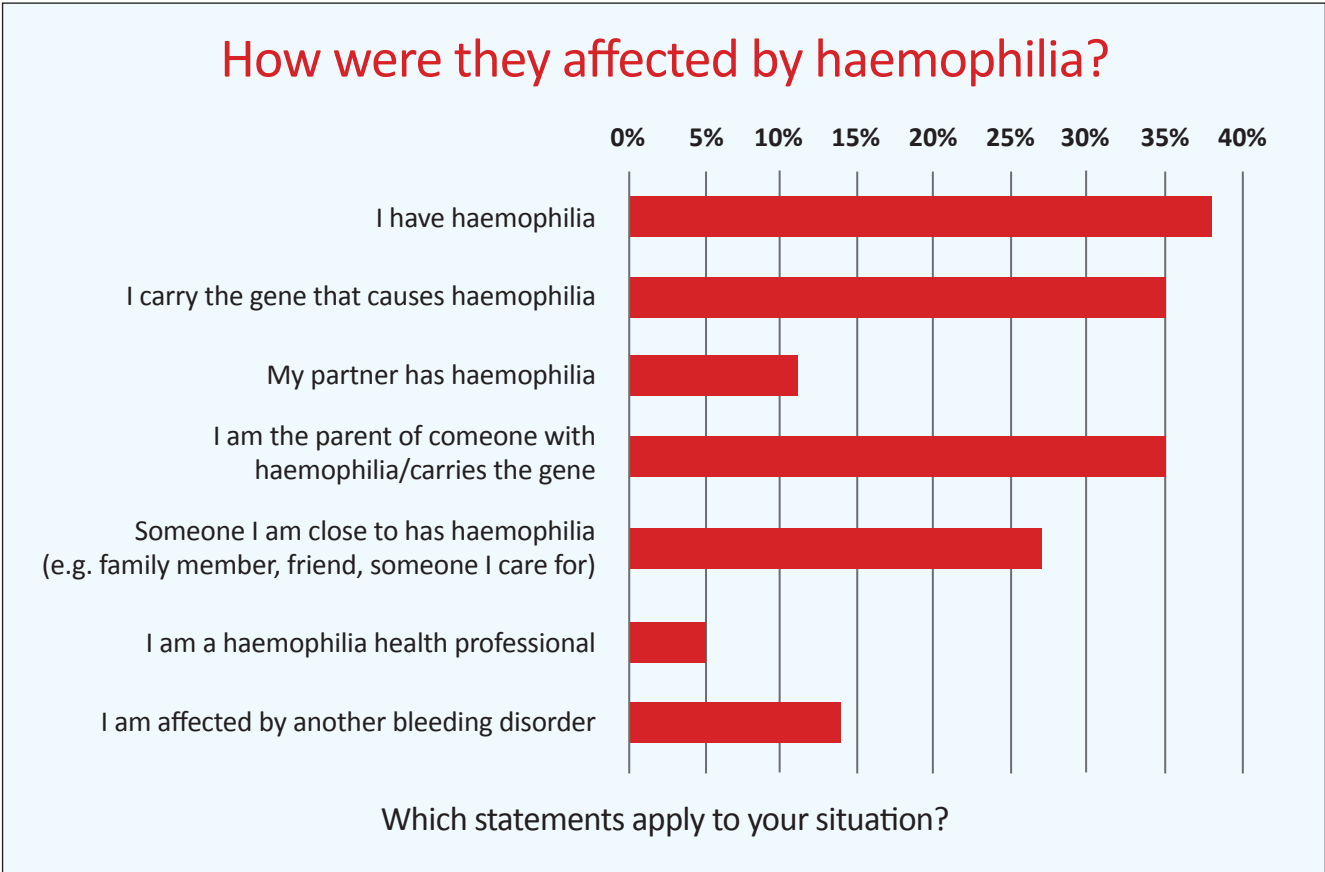
sessions for the upcoming Australian Conference and adding to our sources of information to understand what is important to our community when we undertake advocacy around haemophilia treatment.

## WHO COMPLETED THE SURVEY?

37 people (17 female/20 male) completed the survey.

All age groups from 18 years and over were represented. 38% were 31-44 years old.

68% lived in a capital city; 16% in the rural/urban fringe of a capital city; 8% from a regional/rural remote area; 8% were from overseas.



## EHLS AND NON-FACTOR THERAPIES

Asked about fact sheets on extend half-life (EHL) and non-factor therapies (eg, emicizumab/Hemlibra®), survey respondents thought they should answer questions about:

- What are they; how do they work
- Benefits/outcomes (short and long-term)
- Risks/side effects/safety
- How administered/dosing schedules
- Eligibility (who is it for?)/access to treatment/costs?
- How to work out - is this treatment for me?
- Difference to standard therapy
- Transitioning experiences
- What needs to be managed differently (eg bleeds, surgery)
- Understanding a personalised treatment plan
- Storing the treatment product.

Some also had other questions, including information on treatments in the pipeline and the policy on treating babies with prophylaxis before their first bleed.

## GENE THERAPY

HFA has already published information on gene therapy for haemophilia, but we asked what questions they would like covered in a short fact sheet. Their responses included:

- What the terms mean; how it works
- Types of gene therapy for haemophilia
- Benefits/outcomes
- Risks/Side-effects/safety
- Gene therapy procedure
- Availability/eligibility (who is it for?)
- How long does the effect last?
- AAV immunity and other options
- Options if it is not successful
- Other treatments needed for bleeding, surgery?
- What follow-up is required
- What has changed since the HFA booklet was published in 2019?
- Case studies/personal stories about experience
- Having children after gene therapy.

Some were also interested in the potential for gene therapies for other bleeding disorders.



## PREFERRED TREATMENT OUTCOMES NOW AND IN THE FUTURE

With new and innovative haemophilia treatments becoming available, it's important to understand what people with haemophilia and their parents and partners would like to see as outcomes of the treatment. What would result in a better quality of life for them?

When asked what they would like haemophilia treatment to achieve for them NOW, people completing the survey gave simple and practical responses. Their answers related closely to the types of benefits they could expect from current new therapies:

- Reducing symptoms, preventing bleeds, stable factor level
- Normal life, participating in activities, travel, sport
- Easy administration
- Fewer doses
- No or minimal side-effects
- Safe - no viruses
- Healthy joints
- Not so many hospital visits/stays
- Less pain
- Access to new treatments, including gene therapy.

One person was also hoping for a cure now.

'Stop the bleeding into joints so my kids can enjoy a NORMAL life.'

'Easy, non-intensive delivery. Treatment that reduces all symptoms and prevents bleeds. Low frequency dosing.'



## PREFERRED TREATMENT OUTCOMES IN THE FUTURE

When asked what they would like their treatment to achieve IN THE FUTURE, people responding to the survey had an opportunity to give their wish list and some of their answers were quite different:

- Cure
- Prevent passing haemophilia on to children
- Normal healthy life, travel
- No needles/injections/infusions
- Oral treatment
- No bleeds
- Normal factor levels
- No viruses or side-effects, including inhibitors
- Access to gene therapy
- Store at room temperature, longer shelf life
- Longer effect in the body
- Reduce treatment costs.

‘A cure would be incredible!’

‘Find a measure that can prevent carriers from transferring to their offspring.’

‘No needles. No bleeds.’

HFA’s vision is for active, independent and fulfilling lives for people in our bleeding disorders community. For many years our community members have had low expectations of their haemophilia treatments and it is exciting to see their vision of the future now that they can see the potential with new treatments.

## WHERE TO NEXT?

We are currently working on fact sheets about new haemophilia treatments that will be available on our website. We have also included the survey results in our discussions about the 2023 Conference program. More about the fact sheets and Conference soon!

Our thanks to everyone who completed the survey.

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