Congress – a Foundation perspective

Haemophilia Foundation Australia President Gavin Finkelstein spoke with HFA about his experiences at the WFH 2024 World Congress.

Natashia Coco, HFA Executive Director, and Gavin Finkelstein

What was important to you about Congress overall?

It's always important to get the worldwide community together.

It's good to see what is happening in other countries who are similar to the way we operate in Australia, like the UK, Canada and Scandinavian countries. Are they doing anything innovative that is working that we can try to replicate here? How are they managing the range of new treatment products that are coming, and making them available to their patient group? How is shared decision-making working for them – are they using the WFH Shared Decision Making tool or other alternatives?

I enjoyed catching up with my international colleagues. When you look at it, their issues are very similar to what we are experiencing here. There's a range of new treatment products coming to market that may have benefits for different members of the community. Making sure choice is available to both treaters and patients is a common concern – to choose the product that will provide the best outcome and experience for an individual. In some cases, it might be they feel more comfortable with device they use to administer it, or that it is sub-cutaneous delivery rather than intravenous; or the length of time between administration and increasing the trough level with their factor levels. These days we are seeing a smoothing of the trough level line, which is pretty exciting.

Congress is also an update on what's happening worldwide: with women, clinical trials, gene therapy, you name it.

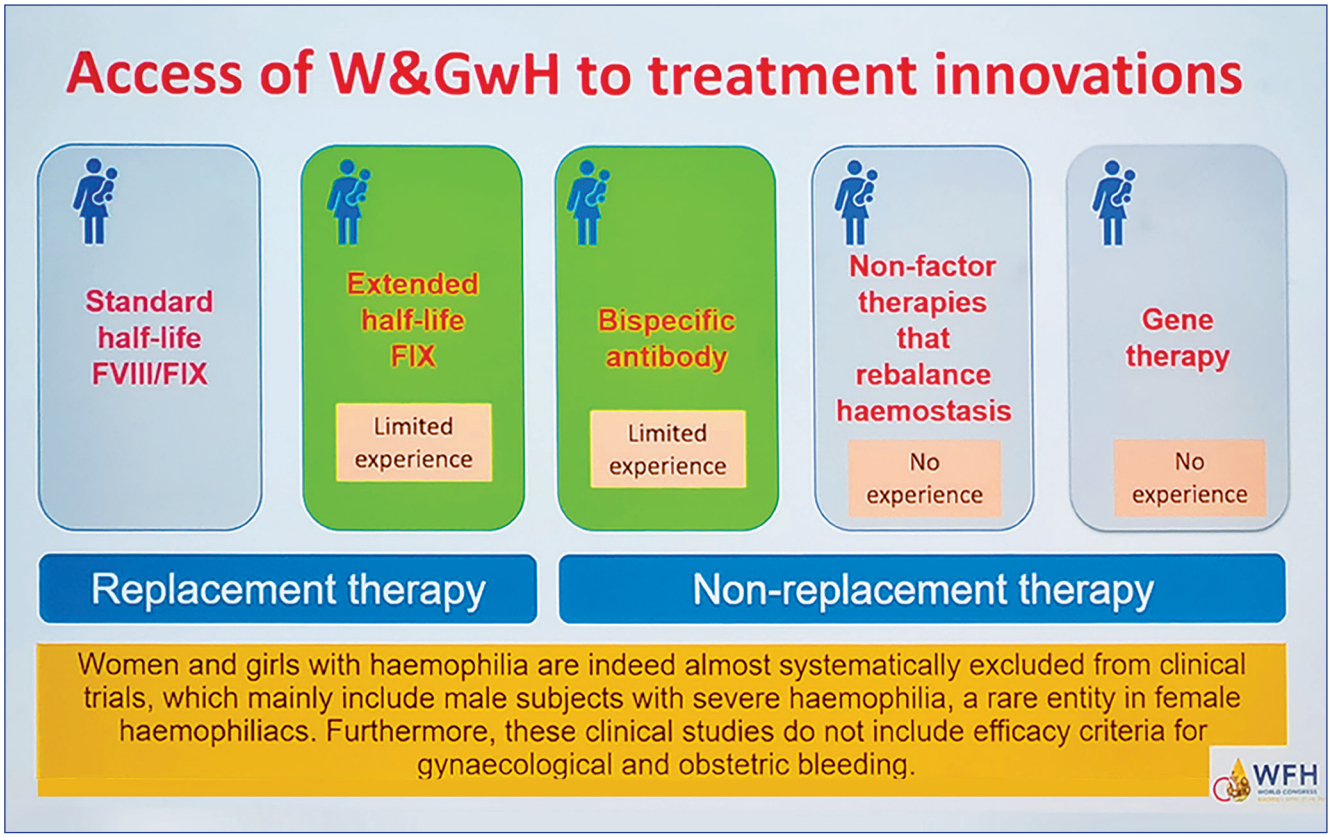
Which sessions stood out for you?

The women's sessions were a highlight. There is still a way to go. Things are moving quickly, but not quickly enough, as the women said in the sessions.

There were two aspects that stood out for me.

Clinical trials need to include women, including gene therapy clinical trials. It's an inequitable situation if women are excluded and this is long overdue. In the past including women has been seen as too complicated, but now researchers are seeing the benefits and, I believe, just need to get their parameters and doses correct.

There are women with severe forms of a range of bleeding disorders, including haemophilia, and there are women who are appropriate for prophylaxis and emerging therapies and need to be considered. This needs to be a primary thought when developing clinical trials, rather than



secondary, and it will help to avoid any issues in the future. It's a big change in the way research into treatment is looked at, but an important one.

Gene therapy was another issue where there is still a way to go. I don't know that a large proportion of the community will want to access gene therapy at the moment, so it is important that the efficacy of gene therapy does improve over time. Opportunities for further doses in the future are also crucial. We need to take into account that some people are not eligible because of AAV immunity and that gene therapy fails for some people. To be realistic, in the next few years many people will still choose to use factor and non-factor treatments rather than taking on gene therapy.

What was your take home message from Congress?

While it has been exciting to see the progress with gene therapy, it is important to recognise the range of very good therapies becoming available to people with haemophilia.

It was also the first time I had heard of women being included in clinical trials and that was a really positive message. Women are a big part of the affected community. Research and development at pharmaceutical companies will want to take this seriously, maybe with different arms of clinical trials to test parameters, doses etc.

More imaginative approaches to treatment for von Willebrand disease (VWD) was also clearly on the agenda, including alternatives to plasma-derived products. For example, I was interested to hear about existing products for people with factor VIII (8) deficiency being used to treat VWD. There do need to be more treatment options for people with type 3 VWD on prophylaxis, particularly.

On the world platform, we are always happy to be involved with our regional neighbours, but Congress highlighted how valuable it is to connect to countries with similar health systems as well, both around treatment and around managing the challenges of Foundation development. For example, succession planning and dealing with treatment advocacy into the future are common issues – and how you encourage younger community members to understand the importance of being involved with their local Foundation. It was great to talk with my international NMO colleagues and I'm looking forward to our ongoing discussions.