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CONGRESS: AN INTERVIEW WITH GAVIN FINKELSTEIN

Gavin Finkelstein, HFA President, spoke with Suzanne O'Callaghan about his experiences at the WFH Global National Member Organisation (GNMO) Training and the World Congress.

Suzanne: Where was GNMO Training held?

GAVIN: Training was at the Golden Jubilee Conference Hotel, in a lovely setting on the banks of the river Clyde, near the Glasgow airport. We had our own areas at the hotel and it was a great way to keep everyone together away from distractions. It is a big opportunity to reconnect with old friends, meet new people, and get fresh views, but also there were long meal breaks and time in the evening so that you could sit and have a concentrated chat with people.

Suzanne: What was different about GNMO Training this year?

GAVIN: From the beginning, there was excitement about gene therapy. There was a presentation from a young English guy who had had gene therapy, who was very positive about his experience and happy to discuss it afterwards. He talked about what the particular type of gene therapy really entailed – the weeks of steroid injections, and going to hospital multiple times a week for blood tests. As he pointed out, he still has factor levels that are 30-40% of normal, but even so, that has made a big difference to his life.

This is the first time you can talk about a 'cure' for haemophilia, which makes for a really different attitude. It is a bit like when the hepatitis specialists introduced the word 'cure' to hep C – the word highlights how much the health outcomes have improved. But there are still a few significant issues to deal with and the therapy needs to be fine-tuned. For example, around 30% of people with haemophilia have antibodies to the viral vector they have used for transporting the gene within the body in the clinical trials we have been hearing about, so they have to look into other vectors for the people this treatment doesn't suit.

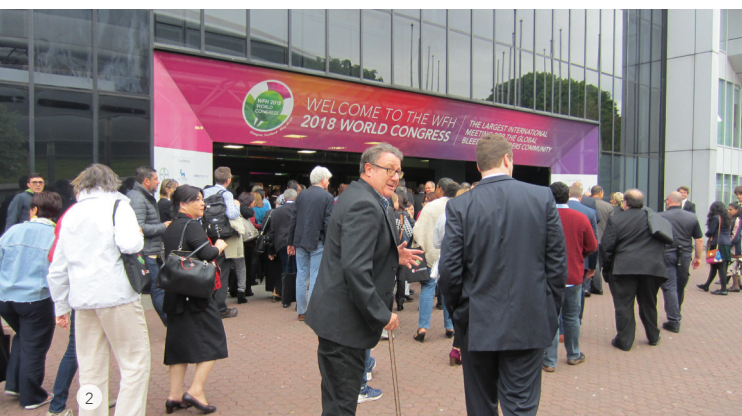
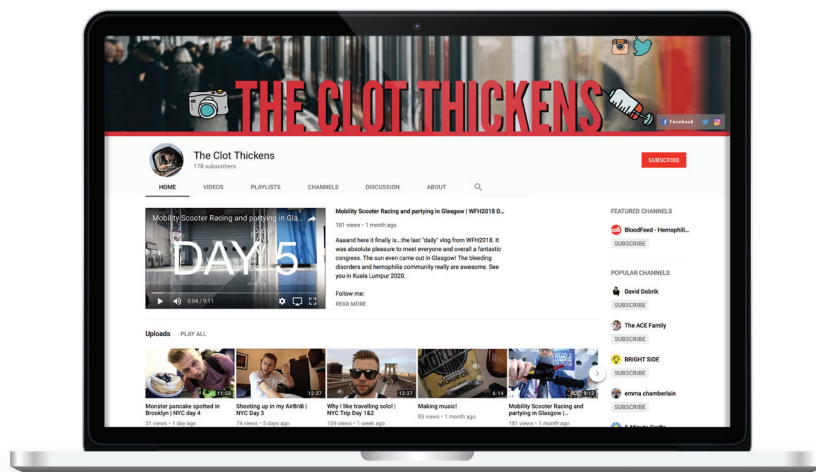
The question now becomes how countries about the world – both well-resourced and developing - would be able to access gene therapy in an equitable way. We discussed this in advocacy workshops and how it could be managed in the health system, covering the realities of health economics. It is going to be an interesting situation.

I found it quite stimulating to meet youth delegates from other countries who are very active in haemophilia awareness and peer support on social media, telling their story positively and responsibly. Two young guys in particular were very engaging as community advocates: David Braun from Brazil, who is on YouTube as David Braun, and Luke Pembroke from the UK, with his YouTube vlog, **The Clot Thickens**. The humour made it quite memorable - Luke talked about how he played soccer as a goalie, not that he was a good player! But it meant he could participate in something he loved doing. The camaraderie among the young people was good to see.

I had spent quite a bit of time talking with people from other countries at GNMO Training and getting to know them and their situation. The photos and videos in their presentations at Training also captured your interest and helped you to understand what they were describing. It's important to make these contacts when you have the opportunity, then you know them and have a good relationship to build on later in international meetings or when you are emailing each other. There were delegates from more than 130 countries and it was an opportunity to get to know a large number of people well in a way that you can't in Congress.

1. The Clot Thickens YouTube channel
2. Gavin entering the Scottish Event Centre
3. Prof Alok Srivastava explaining the emergence of gene therapy

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Suzanne: Did any sessions stand out for you?

GAVIN: Gene therapy was prominent, as I have mentioned. But there was a good dedicated session on VWD. I spoke in the session on education and awareness in Australia, where there was a lot of stimulating discussion. More women present with symptoms of VWD than men, and with the different symptoms and types there is a really broad spectrum of experiences, in comparison to haemophilia, where experiences can be quite similar depending on severity. It is important to be inclusive. We discussed some key messages for awareness and how to articulate them: once you are diagnosed, and have a treatment plan to manage your symptoms, you can get on and live your life; VWD doesn't define you as a person.

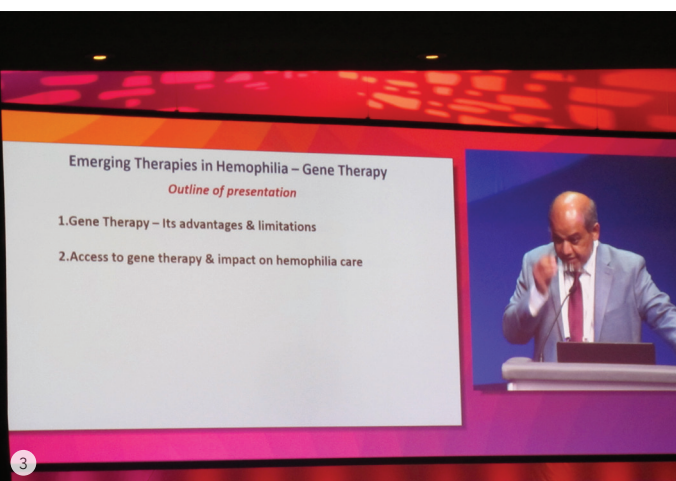
It is a massive issue for the developing world that VWD is rarely considered as a cause of bleeding, and this problem was also reinforced in Congress sessions. You can't have the same education and awareness campaign across the world: in some communities there are specific cultural issues and significant stigma and discrimination, which means that people are reluctant to admit that they have a bleeding problem; and some countries lack resources for diagnosis and treatment. We looked at starting with what can be done with general awareness - because VWD is more prevalent than haemophilia. Attitudes and knowledge really need to change – both among health professionals and the community.

Another issue raised was the complexities of bleeding disorders: medical presenters explained that an individual may have multiple disorders, not just a bleeding disorder, and unravelling what the medical issues are and treating them effectively is really important. We are now moving to individual treatment plans, not one size fits all.

I found the ethical issues with supplying EHLs [extended-half-life factor treatments] to resource-poor countries on a humanitarian basis very interesting. They only receive

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the factor on a short-term basis. What happens when their annual treatment quota runs out and there is no more treatment product in the country? There has to be a better way to supply so that treatment can be maintained for the individual for the full year. It's a difficult thing.

I think also that we need to be vigilant about gene therapy in developing countries to make sure safety and efficacy standards don't slip – they are very vulnerable to cost-benefit approaches.

Speaking of cost-benefits, everyone is very excited about the positive impact on the improved quality of life and better health outcomes of several new products, but they present a challenge with current funding evaluation models. We want to be sure governments take into account the most important outcomes and potential long term savings. This is particularly important with new therapies like Hemlibra® (emicizumab), where we have seen very good results with inhibitors.

I noticed very few sessions about HIV and hep C now. People are doing so well with treatment that the discussion is more about lifestyle and wellbeing. It's great to see.

Suzanne: What was the most memorable aspect of the World Congress?

GAVIN: The emphasis on new treatments has really changed the landscape. Because this is a global community, other questions were raised, such as is there a place for low-dose prophylaxis, if this is the only treatment available. In India they have been looking at this for some years now.

You can see the impact of changing treatment approaches on the types of concerns that came up for discussion. One example: now that there is a new generation that has grown up with prophylaxis, there are new issues for people who technically have a severe bleeding disorder, but whose experience is similar to someone with a mild disorder. This involves quite

significant problems, such as recognising a bleed when you have never had a bleed in your life. They need education to identify it and deal with it, rather than leaving it to somehow resolve itself. You still have a chronic medical condition and need to take it into account and need to be able to manage it through the different stages of your life.

Suzanne: What did you like about the social aspects of Congress?

GAVIN: Glasgow is a really easy place to get around, with good comfortable venues and food. Social functions were a really nice time to catch up with delegates, talk to people you know and meet new ones. There was a lot of camaraderie and everyone was so accepting and enjoying sharing experiences.

Suzanne: What were the take home messages?

GAVIN: It was exciting to hear that gene therapy is within our grasp and achievable. We had presentations from people who are actually receiving it and it is clearly a great thing for the future. News of this is getting out into the general media in the wider community; for example, there was a recent article about gene therapy in the New York Times. Obviously there are still issues about affordability and accessibility – and of course, gene therapy has to be safe and effective in the first place.

Suzanne: Why do you think the World Congress is important?

GAVIN: Congress brings different parts of the global bleeding disorders community together. This is a forum where we have a voice. It is an important opportunity to get the current information, to see what is happening in the world, and how other countries do things. It is a time when you engage with your peers, and learn from them. ■

Gavin Finkelstein was funded by HFA to attend the WFH World Congress.