Where to from here? Achieving the vision

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This is a transcript of the presentation by Sharon Caris in the final plenary at the 20th Australian Conference on Haemophilia, VWD and Rare Bleeding Disorders. You can watch Sharon's presentation on the Conference portal at https://haemophilia.delegateconnect.co

A conference like this is just the right time for us to think, plan and to look forward.

We've covered a range of issues affecting the bleeding disorders community over the last couple of days of the Conference and looked at what's new and the changes we might need to make; but this is all on the back of COVID-19, and I think that alone will likely have implications for health care priorities and costs going forward in Australia for many years, and it will affect all of us. We need to take this into account when we're thinking about treatment requirements, new therapies and what we ask of our governments to supply.

I think what stands out for me today is that we shouldn't put all our eggs in one basket. We don't just need 'this treatment product' or 'that one'. There are many things, at many levels, to be done to achieve our vision for best practice care and treatment for everyone living with a bleeding disorder in Australia, and to support their families and their carers and treating health professionals as well. Nor can we forget that the World Federation of Hemophilia estimates that approximately 70% of the world's bleeding disorders community is either not yet diagnosed or adequately treated.

The first extended half-life product was registered in Australia in 2014, just before we were hosting the World Federation of Hemophilia Congress in Melbourne. We were hearing some of the promising results of clinical trials of other products, and just what could be achieved from these new products. But it has taken a few more years for these important new treatments to be funded and available for everyone who could benefit from them, even though there was evidence of significantly better health outcomes and improved quality of life. And since then, of course, more new treatment products have come into the mix.

There are many people at the Conference who are amazed by what can be achieved now with what we know as 'the new treatments' after such a reliance on conventional therapies for the last 30 or 40 years or so. Of course, standard clotting factor concentrates have been and are still important, but there's a great relief that several new treatment products are now available here.

As a community, I think we do have to decide what we're really aiming for. We've been hearing about other therapies around the corner. Some of these new haemophilia treatments are very close to market, while others are further away, and still need to be adequately tested for their safety and effectiveness. Some might be preferred. Time will tell.

We also need to make sure that von Willebrand disease and other rare factor deficiencies and inherited platelet disorders are addressed as well so that people living with these conditions have the best treatment and care.

It's not a straightforward thing to be able to access a new treatment in Australia and our experience over the past seven years has been frustrating. Some new treatments were approved for use by the Therapeutic Goods Administration for four years or more, so they had been assessed as safe and effective, yet funding and access to them was delayed because of various bureaucratic assessment processes, including necessary funding evaluations.

The principles and processes for approving and funding new treatments could be improved. There are several layers to this, and some changes will be needed to address some of the problems. HFA and other stakeholders are concerned about this and there are opportunities to contribute to solutions at a structural level, and HFA will contribute the experiences of our community. We have already been able to make submissions and participate in stakeholder discussions with governments in the hope that patients as experts, and their treaters, who have

the actual treatment experience are more involved at critical times in decision-making.

So, I do think the processes for assessment and funding can be better informed, so that access is more timely and there are fewer bureaucratic delays. This isn't to under-emphasize, though, the importance of critical assessments about the relative costs, benefits and value for money of these new products against others, that needs to be addressed for all medicines and healthcare services.

There isn't an unlimited pot of money, and we want governments to get the best bang for their buck in our space. We've recently had input to discussions around health technology assessment and how to contribute the experiences of patients and what they think are the most important outcomes to achieve from their treatment. We want to see more patient involvement so patients can demonstrate what is important to them. We have just heard from Shauna, Claude and Alan about their views and hopes for their futures, and just in the space of those three minutes you would agree, this was inspiring and very much of relevance to some of these evaluation processes. Most importantly, we want to see patient involvement so they can explain what is most important and the value of new therapies from their point of view and HFA will be working to ensure this input is taken into account at critical times.

We also want the Australian environment to encourage industry to retain interest and investment in the Australian market and to continue to invest in clinical trials here. So again, at a different level, we want to see barriers removed for industry that will enable them to bring their new treatment products to Australians.

We already have a strong national framework for bleeding disorders in Australia, and this is reflected in the national treatment guidelines for haemophilia. The legislation to establish the National Blood Authority in 2003 created a new system for the purchase and supply of blood and blood products and an agreement by Australian governments to share the cost of funding for blood and blood products. This is very important because so many practical aspects of our treatment and care crosses jurisdictions; treatment for bleeding disorders is more than just clotting factor replacement therapy, it is also about the care and treatment provided by Haemophilia Treatment Centres, and this is in the remit of state/territory governments. Both federal and state/territory governments have a stake in the Australian framework for care.

All governments need to be involved and engaged to make sure that we have the services needed. In addition to clotting factor, Haemophilia Treatment Centres also need to be adequately resourced to provide comprehensive care, which is a critical component of the national framework. It's all there in the framework. We need to make sure that it's understood and embedded in each of the decisions that follow.

I think it is also really important to have the concept of innovation included in the legislation that underpins all of this, so that there is a priority for considering the benefit to all stakeholders of new therapies as these might not only contribute to improved health outcomes for patients but save money as well. We need to embed a priority for innovation and best practice care and treatment as a principle into policy and the decision making that flows from this.

We've heard about potential different models for Haemophilia Treatment Centres, and how care might be provided in the future. The fundamental principles can be built into the 'hub and spoke' approach referred to as one example of a different structure for care to be delivered, and there are probably different concepts that can be considered as well across Australia, including the more recent experience of telehealth. We live in a big country, and comprehensive care doesn't need to be thrown out in any new models for healthcare delivery; it can actually create the very strength and success of new models. But we do need to make sure services are funded adequately so they are able to provide the care and treatment that's needed.

HFA will of course, continue to work with the stakeholder community. We will play our part to represent the community and this includes working closely with our state and territory haemophilia member foundations who are critical to this, as well as with medical and nursing specialists, allied health professionals, governments and industry to achieve our goals.

Most central to success will be how we ensure that the people living with a bleeding disorder are front and centre and that their needs and experience guides all of this.

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