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Ms Jeanette Radcliffe
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3 March 2017

Dear Secretary Jeanette Radcliffe

Thank you for the opportunity to comment on the *Therapeutic Goods Amendment (2016 Measures No. 1) Bill 2016*.

We note that the Bill is intended to support the implementation of eight key recommendations of the Expert Panel Review of Medicines and Medical Devices Regulation and would like to make some overall comments in relation to these recommendations.

HAEMOPHILIA FOUNDATION AUSTRALIA

Haemophilia Foundation Australia (HFA) is a not for profit organisation which represents people with haemophilia, von Willebrand disorder and other rare inherited bleeding disorders in Australia. It is the national peak organization for bleeding disorders and provides advocacy, education and support to the bleeding disorders community and promotes research relating to bleeding disorders.

PEOPLE WITH BLEEDING DISORDERS IN AUSTRALIA

There are more than 5600 people who have been diagnosed with inherited bleeding disorders in Australia. As a result of their bleeding disorder, the blood clotting process does not work properly. Without treatment they can bleed for longer than normal, usually internally, into joints, muscles and organs; and some bleeding episodes can be life- or limb-threatening. Over time these bleeding episodes can damage muscles and joints, leading to complications such as severe arthritis and the need for joint replacements.

Treatment for inherited bleeding disorders often requires medicines that replace, stimulate the generation of or mimic the function of the clotting factor or platelet that is missing or not working properly. This includes replacement therapy derived from human plasma and/or genetically engineered recombinant clotting factors and related treatments. In the future this may also include treatments such as gene therapy.

Some Australian adults with bleeding disorders live with blood borne viruses, including HIV and hepatitis C due to treatment with infected blood clotting products before safe blood screening tests and viral inactivation procedures were introduced in Australia in the 1990s or from products sourced from and/or used overseas. The manufacture of blood products in Australia, such as plasma factor concentrates, is now carefully regulated and monitored to make sure they are as safe as possible from infections that can be transmitted by blood. The risk of new infections from using human blood products is now thought to be extremely low. However, it cannot be entirely excluded, and relies on constant vigilance by blood bankers, manufacturers and regulatory authorities.

There is also a 20-30% lifetime risk of the development of inhibitors in people with haemophilia treated with currently available clotting factor replacement therapy. Inhibitors are antibodies that prevent clotting and make bleeding difficult to treat. Fortunately, for some patients these resolve naturally or with immune tolerance therapy. But for the others, current treatment product options are limited, with a short half-life and inherent risks of thrombosis, and these people face a lifetime of a complex interaction of difficult and costly

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clinical management, frequent hospitalisations for uncontrolled life and organ threatening bleeding and serious psychosocial issues for themselves, their families and carers.

NEW MEDICINES AND DEVICES

We note that this Bill proposes a regulation-making power to support the implementation of new pathways for the approval of new medicines and medical devices in Australia), "to facilitate quicker access by patients to new medicines and devices that have significant advantages over existing treatments."

Currently available treatments for bleeding disorders usually involve injecting the clotting factor replacement therapy into a vein, sometimes around three to four times a week. The burden of this therapy to the person with the bleeding disorder can be substantial, resulting in issues such as vein problems and pain, adherence difficulties, depression, problems with managing school, work, sport, travel and other recreational activities, stigma and discrimination, and impact on their partner, family and caregivers. As mentioned above, a significant number of people will also develop inhibitors to this treatment.

New therapies are currently in development or coming to market that address some of these issues, while at the same time improving clinical results relating to bleeding episodes. In the future, experimental treatments such as gene therapy may also prove to be effective and safe and enable people with bleeding disorders to improve their factor levels to a point where they have very few bleeding episodes.

Having a process to fast-track access to new treatments like these would be of great benefit to people with bleeding disorders in Australia, not only for improved quality of life but also for their clinical benefit in preventing bleeding episodes, each one of which may be life- or limb-threatening. Too often there are tragic episodes in our small community where a community member has died from a significant bleed like a head bleed, or has lost the use of a limb, with all of the repercussions for living and working in the future.

With many of these new therapies, access is often available in other similar countries such as Canada, the USA and United Kingdom where approval has already been completed, while Australians with bleeding disorders may wait years for the same therapy to be approved in Australia, and subsequently funded.

From the perspective of an organisation like HFA that represents the individual with a chronic health condition and their partner and family, any fast-tracking of a life-saving medicine that can improve their chances of survival or reduce their burden of disease is invaluable. Significant improvements to quality of life also make an enormous difference to an individual and having access to these therapies is often life-changing.

SAFETY

We note that the Bill is conscious of safety issues and risk in the amendments that are proposed and welcome this approach.

While new clotting factor replacement products, by their nature, are tested for safety through clinical trials in the bleeding disorders population, Haemophilia Foundation Australia urges that policy around regulatory requirements at a broader level take safety for coagulation disorders into account, as this is often overlooked, especially in complementary therapies. We also ask that careful consideration be given to any policies that have the potential to reduce or relax regulatory requirements in relation to the safety implications for blood products manufactured in Australia or overseas.

Yours sincerely

Sharon Caris
Executive Director