Submission to Pharmac on Proposal to fund Emicizumab for severe Haemophillia A

To: consult@pharmac.govt.nz
Date of Submission: 6 July 2023
Submitted by: Rare Disorders NZ
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Declaration of interest

Rare Disorders NZ works with clinicians, researchers, allied health professionals, academia, government and industry to achieve better outcomes for people with a rare disorder in New Zealand and their whānau. We are funded by grants, donations, fundraising events, Pharma roundtable and a small Te Whatu Ora contract. This submission is in response to Pharmac's proposal to fund Emicizumab for severe Haemophillia A.

Rare Disorders NZ

Rare Disorders New Zealand is the respected voice of rare disorders in Aotearoa. We are the national peak body organisation, supporting the 300 000 New Zealanders with rare disorders and the people who care for them. We help those affected by rare disorders navigate the healthcare system, find information and resources, and connect with support groups specific to their condition.

We proudly advocate for public health policy and a future healthcare system that works for those with rare disorders – using a strong and unified voice to collaborate with Government, clinicians, researchers, and industry experts, to promote diagnosis, treatment, services, and research.

Our vision is for New Zealand to become a country where people living with a rare disorder are fully recognised and supported with equitable access to health and social care.

Our submission

Rare Disorders NZ supports the proposal to widen access to emicizumab, brand name Hemlibra, for the treatment of severe Haemophilia A without FVIII inhibitors, subject to eligibility criteria. As stated by the proposal, this would mean that any person with severe Haemophilia A, defined as endogenous factor VIII activity less than or equal to 2%, would have access to funded emicizumab, regardless of FVIII inhibitor titre.

Rare Disorders NZ welcomes this huge step towards meeting internationally recognised standards of care for people with severe Haemophillia A. We request the proposal is approved with no further delay.

Support for the submission by the Haemophillia Foundation of New Zealand

Rare Disorders NZ also endorses the submission written by Deon York, Chair, Haemophillia Foundation of New Zealand, copied below.

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HFNZ

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4 July 2023

Te Pātaka Whaioranga – Pharmac

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Kia ora koutou,

The Haemophilia Foundation of New Zealand (HFNZ) strongly endorse access to emicizumab being widened to people with severe haemophilia A without inhibitors, subject to eligibility criteria. As stated by the proposal, this would mean that any person with severe haemophilia A, defined as endogenous factor VIII activity less than or equal to 2%, would have access to funded emicizumab, regardless of FVIII inhibitor titre.

We applauded the PTAC recommendation in May 2021 that this therapy be funded with a high priority, due to the health need of people with this condition, the suitability compared to current treatment and the benefits to the individual and their whānau. PTAC also noted the benefits to the health system through a reduction in bleeding events. In November 2021, the haematology subcommittee also recommended some amendments to the proposal.

We support the details of the proposal released on June 22, 2023:

Initial application – (Severe Haemophilia A with or without FVIII inhibitors) only from a haematologist. Approvals valid without further renewal unless notified for applications meeting the following criteria:

Both:

1. *Patient has severe congenital haemophilia A with a severe bleeding phenotype (endogenous factor VIII activity less than or equal to 2%); and*
2. *Emicizumab is to be administered at a dose of no greater than 3 mg/kg weekly for 4 weeks followed by the equivalent of 1.5 mg/kg weekly.*

Haemophilia is a life-long and debilitating condition. With appropriate clinician and patient management, individuals can live long lives, but these are not lived without pain or challenge. People with haemophilia depend on factor replacement therapy and the relationship with medication is as life-long as the condition itself. While funded replacement therapy exists, these no longer represent best practice, and this is no longer an acceptable position for members of HFNZ. This proposal corrects this and represents the most significant change to care and treatment for people with severe haemophilia A for nearly 25 years.

Members of HFNZ are elated by the proposal and convey heartfelt appreciation towards this proposed recommendation.

Nāku noa, nā,

Deon York
Chair, Haemophilia Foundation of New Zealand

ENDS

Rare Disorders NZ

6 July 2023