CONSULTATION

PHARMAC
Pharmaceutical Management Agency

26 September 2013

Proposal relating to the listing of haemophilia treatments

PHARMAC is seeking feedback on a proposal to list a number of haemophilia treatments in Section B and Section H of the Pharmaceutical Schedule.

In summary, this proposal would result in the following:

- The listing of the following haemophilia treatments in Section B (Community Pharmaceuticals) of the Pharmaceutical Schedule:
    - Recombinant blood factors (VIIa, VIII and IX); and
    - Factor eight inhibitor bypassing agent (FEIBA).

  Funded access to these treatments would continue to be via the National Haemophilia Management Group (NHMG) and the Haemophilia Treaters Group (HTG).

  and

- The listing of factor eight inhibitor bypassing agent (FEIBA) in Part II of Section H of the Pharmaceutical Schedule (Hospital Pharmaceuticals) (which, for the avoidance of doubt, already lists recombinant blood factors (VIIa, VIII and IX).

Feedback sought

PHARMAC welcomes feedback on this proposal. To provide feedback, please submit it in writing by **Thursday, 10 October 2013** to:

Sue Anne Yee
Therapeutic Group Manager
PHARMAC
PO Box 10 254
Wellington 6143

Email: sueanne.yee@pharmac.govt.nz

Fax: 04 460 4995

All feedback received before the closing date will be considered by PHARMAC's Board (or its delegate) prior to making a decision on this proposal.

Feedback we receive is subject to the Official Information Act 1982 (OIA) and we will consider any request to have information withheld in accordance with our obligations under the OIA. Anyone providing feedback, whether on their own account or on behalf of an organisation, and whether in a personal or professional capacity, should be aware that the content of their feedback and their identity may need to be disclosed in response to an OIA request.

We are not able to treat any part of your feedback as confidential unless you specifically request that we do, and then only to the extent permissible under the OIA and other relevant

Level 9, 40 Mercer Street, PO Box 10-254, Wellington 6143, New Zealand
Phone: 64 4 460 4990 - Fax: 64 4 460 4995 - www.pharmac.govt.nz

laws and requirements. If you would like us to withhold any commercially sensitive, confidential proprietary, or personal information included in your submission, please clearly state this in your submission and identify the relevant sections of your submission that you would like it withheld. PHARMAC will give due consideration to any such request

Details of the proposal

Schedule listing change

- The following pharmaceuticals, used in the treatment of haemophilia, would be listed in Section B of the Pharmaceutical Schedule (Community Pharmaceuticals) from 1 December 2013:
    - Recombinant factor VIIa (Novoseven RT);
    - Recombinant factor VIII (Advate, Kogenate FS and Xyntha);
    - Recombinant factor IX (BeneFIX); and
    - Factor eight inhibitor bypassing agent (FEIBA).

- Recombinant blood factors (VIIa, VIII and IX) and FEIBA would be listed Xpharm - which means that pharmacies cannot claim subsidy because PHARMAC has made alternative distribution arrangements. The current system for supply of these treatments to patients would not change as a result of this proposal.

- To maintain consistency between Sections B (community) and H (hospital) of the Pharmaceutical Schedule, Rule 2 in Part I of Section H of the Pharmaceutical Schedule would be amended as follows (addition in bold) from 1 December 2013:

> 2. Hospital Pharmaceuticals
>
> 2.1 Section H Part II contains the list of Hospital Pharmaceuticals that must be funded by DHB Hospitals. Section H Part II does not currently encompass the following categories of pharmaceuticals:
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> a) Medical Devices;
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> b) whole or fractionated blood products **unless specifically listed in Section H Part II**;
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> c) diagnostic products which have an ex vivo use, such as pregnancy tests and reagents;
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> d) disinfectants and sterilising products, except those that are to be used in or on a patient;
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> e) foods and probiotics;
>
> f) radioactive materials;
>
> g) medical gases; and
>
> h) parenteral nutrition.
>
> Subject to rule 2.2, the funding of pharmaceuticals identified in a) – h) above is a decision for individual DHB Hospitals.

- The listing of factor eight inhibitor bypassing agent (FEIBA) would be shifted from Part III of Section H (Optional Pharmaceuticals) to Part II of Section H (Hospital Pharmaceuticals) of the Pharmaceutical Schedule from 1 December 2013.

- Recombinant blood factors (VIIa, VIII and IX) and FEIBA would be listed in Section B of the Pharmaceutical Schedule with the following eligibility criteria:

For patients with haemophilia, whose treatment is managed by the Haemophilia Treaters Group in conjunction with the National Haemophilia Management Group.

- Recombinant blood factors (VIIa, VIII and IX) and FEIBA would be listed in Part II of Section H of the Pharmaceutical Schedule with the following access criteria:

  When used in the treatment of haemophilia, treatment is managed by the Haemophilia Treaters Group in conjunction with the National Haemophilia Management Group.

The role of the NHMG and the HTG

- The NHMG and HTG would each continue to have a role in determining patients funded access to haemophilia treatments listed in the Pharmaceutical Schedule, whether for in-hospital or in-community use. The NHMG would continue to oversee the expenditure for recombinant blood factors and FEIBA.

- PHARMAC would work closely with the NHMG to determine the appropriate annual funding provision (from the Combined Pharmaceutical Budget) for these treatments, commencing with funding for the full year ending 30 June 2014.

- In exceptional circumstances, for instance when there is a need to address clinical situations such as acute bleeding episodes, surgery or tolerisations, the NHMG would have discretion to spend up to an agreed annual funding provision. PHARMAC would decide whether to approve funds above the annual funding provision.

- The existing role of the NHMG and the HTG in relation to management of expenditure on related health services (such as physiotherapy) and other plasma-derived products for haemophilia on behalf of District Health Boards would be unaffected by this proposal.

Assessment of new treatments

- New haemophilia treatments, which may become available in the future, would be assessed by PHARMAC for funding through the same processes currently applying to other pharmaceuticals (see http://www.pharmac.health.nz/medicines/how-medicines-are-funded).

- In considering applications for funding of new haemophilia treatments, PHARMAC could seek clinical advice from the HTG, the Pharmacology and Therapeutics Advisory Committee (PTAC) and/or the Haematology Subcommittee of PTAC.

Background

PHARMAC is responsible for the funding of community pharmaceuticals, vaccines, and hospital cancer medicines. This includes assessing, prioritising and managing expenditure within the available funding (the Combined Pharmaceutical Budget). We anticipate taking on full budget management of all hospital medicines and medical devices in the next few years.

From 1 July 2013 the Hospital Medicines List in Section H came into effect. This is the list of medicines that may be used in public hospitals. It includes some treatments for haemophilia, a condition that affects the blood's clotting ability due to a deficiency in blood clotting factors. Haemophilia A is associated with Factor VIII deficiency and Haemophilia B is associated with Factor IX deficiency. There are an estimated 400-600 people living with haemophilia in New Zealand.

Treatment for haemophilia depends on how severe a patient's haemophilia is. The main treatment for haemophilia involves 'replacement therapy' which is the administration of the clotting factor that the body is missing. People can receive this treatment in hospital but most of them are able to administer it themselves at home. Replacement therapy is used to treat a bleed, but it can also be given prophylactically on a regular basis to prevent bleeding.

Replacement blood clotting factors can be:

- plasma-derived (produced from pooled human donor plasma and processed to produce a clotting factor concentrate that is pure); or
- recombinant products (made in a lab with recombinant DNA technology. These do not come from donated blood).

A complication of haemophilia treatment is the development of an inhibitor, which usually occurs shortly after replacement therapy has been initiated. The inhibitors are antibodies directed against the specific deficient factor. Recombinant factor VIIa and factor eight inhibitor bypassing agent (FEIBA) are treatments used in the treatment and management of patients with inhibitors.

The NHMG was established in 2006 and is responsible, on behalf of 20 District Health Boards, for management of haemophilia treatments in New Zealand, which includes both recombinant and plasma derived products. The HTG is made up of clinicians who are involved in the clinical management of patients with haemophilia in New Zealand, and it collaborates closely with the NHMG.

PHARMAC has been involved in the procurement (negotiating national contracts) of haemophilia treatments since 2007, working in close collaboration with the NHMG and HTG.