**Appendix 1**

# Potential Future Arrangements for Imported Plasma and Recombinant Products

Summary of Stakeholder Responses

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| **Item** | **Stakeholder response** |
|  | **Product demand** |
| 1. | There may be an increase in demand for clotting factor products resulting from:* an ageing population;
* pharmaceutical trials; and
* people born overseas with severe haemophilia arriving in Australia.
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| 2. | There are a number of potential new products in development that may impact on future use of FVIII and FIX products. |
| 3. | The Extended Half-life (EHL) Product markets for rFVIII and rFIX are developing.  |
| 4. | There are potentially novel non-clotting factor treatments for the treatment of haemophilia in development. These products are in the early to mid-stages of assessment in clinical trials. |
| 5. | Demand for EHL products is expected to increase, based on anecdotal experiences of clinicians and patients who have reported:* improved quality of life;
* increased ability to participate in the normal activities of daily living; and
* improved patient confidence regarding the effectiveness of their prophylactic therapy.
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| 6. | The introduction of EHLs may decrease overall use in terms of infusions and consumption, although available evidence is considered to be low to moderate including:* the lack of direct comparison on clinical outcomes;
* inconsistent use of the type of estimate (e.g. means and medians across studies); and
* lack of estimates of variance.
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| 7. | Respondents also indicated the following uncertainties concerning EHL products:* limited real world published data;
* cost and price challenges; and
* differences in products and clinical utility.
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|  | **Product range and choice** |
| 8. | Product choice is important of offer best practice clinical practice given:* the risk of inhibitor development; and
* patient tolerance to some products.
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| 9. | Importance of having a range of Products if there are substantial differences in effectiveness, safety and reliability of supply. |
| 10. | Clinical professional and representative groups, patients and careers consider the inclusion of EHLs is priority in terms of patient benefits and potential reduction in health care costs. |
| 11. | Supplying products with administrative devices that are suitable for use by all patients including patients with patients with variable degrees of dexterity is seen as very important. |
| 12. | The range of vial sizes currently available is considered adequate and should be continued to minimise product wastage, especially in treating paediatric patients. |
|  | **Change in product brands** |
| 13. | The transition of patients from one product to another, involves a significant amount of clinician time to:* manage patient reviews and concerns;
* update hospital protocols and education material; and
* inventory management issues.

Education on new administration devices is also time consuming. |
| 14. | Appropriate planning and communication is required when changing products for health care professionals and patients |
| 15. | Patients are extremely reluctant to change brands once they have found one that suits their needs. There is a high level of psychological stress and anxiety that comes with changing products. |
| 16. | It is important for Suppliers to be prepared and have support materials and resources available prior the transition. |
| 17. | There is administration issues experienced when changing patients on home delivery. |
| 18. | A longer transition period for future transition processes would be beneficial. |
|  | **Ordering and delivery of products** |
| 19. | Ordering arrangements vary between jurisdictions and centres. |
| 20. | Current ordering and delivery arrangements are considered to be adequate. |
| 21. | Clinicians and patients strongly support home delivery of products.  |
| 22. | Consideration should be given to a review home delivery of product to patients with a view to:* improving this service; and
* reducing the cost of the service.
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|  | **Product supply** |
| 23. | Education and training resources should be available from suppliers in both print and electronic formats. |
| 24. | Education and training materials must be available prior the commencement of any transition process. Material should be consistent with national treatment guidelines rather than developed for other global distribution.  |
| 25. | A range of relevant and appropriate support material should be available for products during the term of product supply.  |
| 28. | The feedback facility to product suppliers is not well known within the clinical community. |
| 29. | Patients should be involved with the evaluation of product administration sets for future product procurements. |
|  | **New Products** |
| 30. | There is an strong awareness of a number of new product variants including:* Standard Half Life (SHL) and EHL factor concentrates;
* bypassing therapies to treat patients with inhibitors;
* other coagulation products; and
* Gene therapy products.
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| 31. | The EHL Product markets for rFVIII and rFIX are developing. |
| 32. | EHL should be considered by governments and a matter of urgency and made available to patients. |
| 33. | The benefits associated with EHL products include:* fewer infusions;
* reduced severe bleeds;
* better joint health;
* reduction in surgeries;
* reduced hepatisation;
* less damage to veins;
* less interruption to work/school;
* greater physical wellbeing;
* increased trough levels; and
* improved quality of life.
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| 34. | The following additional comments were made about EHL products:* EHL products can be tailored to the bleeding patterns of individuals, and outcomes carefully monitored and evaluated for their impact on health, productivity, quality of life.
* There is a potential to have high up-front costs with a transition to EHL products.
* There are significant differences in the half-life between EHL products.
* Some EHLs appear to have high dosing rates per kilogram.
* More information is required on EHL utilisation in the surgery setting.
* Not all patients are expected to transition to EHL products, as a result a moderated access program could be implemented.
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| 35. | New products with a subcutaneous route of infusion could significantly improve the following:* compliance;
* reduced need for support and education in relation to product administration; and
* reduced need for consumables and hospital admissions.
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| 36. | There is potential for unexpected adverse events with products in clinical trials which may impact on the availability of products reaching the marketplace.  |
| 37. | There is a view that the strongest evidence will come from real life experience and the data collected in an ongoing way after these products are funded. |