Appendix 1

Potential Future Arrangements for Imported Plasma and Recombinant Products

Summary of Stakeholder Responses

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.
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.
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.
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.

	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.
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.

	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.
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.

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.
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.
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.