

Australian Haemophilia Nurses' Group

Response to

Review of Australia's Plasma Fractionation Arrangements

11/4/2006

**Response compiled by:
Megan Walsh & Anne Jackson
Co- Chairs Australian Haemophilia Nurses' Group**

The Australian Haemophilia Nurses' Group welcomes the opportunity to participate in the consultative review process. We believe that the Australian Bleeding Disorders community should have access to the best practice and treatment available, based on current research and evidence.

The group believes the supply of plasma products to patients first and foremost should be safe, adequate, secure, equitable and cost effective. The terms of reference have been examined for the review of Australia's Plasma fractionation arrangements. This submission has addressed only the questions the group has relevant experience and knowledge of.

Examine the projected demand for plasma products over the next ten years and the relationship between demand trends and the requirements on supply of plasma fractionation services

1 .Demand trends for plasma products

1.2 *Comment on current and projected clinical treatment and usage trends over the next ten years.*

At this present time there exists no recombinant product available to treat the von Willebrands community. Thus patients, who have Von Willebrands disease particularly type II or type III, require factor replacement with a plasma derived factor VIII product Biostate produced by CSL Bioplasma. The current use for this patient group will be maintained and including increased use for new diagnosis the anticipated trend over the next ten years would not see a significant increase in the use of this product for treatment.

A significant use of the plasma derived FV111 product has been the Haemophilia A community but since the increased access to recombinant products the demand from this group has significantly decreased. With the transfer from plasma derived to recombinant products for current patients and all newly diagnosed patients being commenced on recombinant products it is anticipated that the demand would be small and accessed only by those in particular circumstances, but not exclusive, such as:

- Tolerisation of inhibitor – Plasma derived product is used as a treatment option for the eradication (tolerisation) of inhibitors. This treatment uses large doses of plasma product often for greater than 12 months in order to try and eliminate the inhibitor. The patient then may elect or be recommended to remain on plasma derived factor VIII product. Research coming out of Europe suggests a greater chance of success in eliminating the Factor VIII inhibitors using plasma derived VIII products for tolerisation of the inhibitor.
- A very small number of patients who after genetic testing have been found to have mutations of their factor VIII gene, which are associated with a high risk of developing an inhibitor to factor VIII, are choosing on advice to remain on plasma derived factor VIII until there is more research into the use of recombinant products.

It would be envisaged neither of these issues should have a marked effect on the demand for Plasma derived Factor VIII product over the next 10 years.

MONOFIX is the plasma derived Factor IX replacement manufactured by CSL Bioplasma. Currently all newly diagnosed patients are commenced on the recombinant Factor IX product, Benefix, manufactured by Wyeth. Some patients have elected to remain on Monofix (plasma derived Factor IX), as the product is well regarded in the Haemophilia community as having a good safety profile and being efficacious. A very small number of patients who had previously been on MonoFIX and then swapped to the recombinant product, have chosen to swap back to the plasma derived Monofix stating they felt they had better resolution of their bleeding episodes on Monofix. Although some patients have elected to use MonoFIX as their product of choice it is not anticipated that the current demand will increase. The long term trend for Monofix would be seen as a decreasing need for the product as all newly diagnosed Haemophilia B patients will be commenced on Recombinant Factor IX.

Factor XI deficient patients only have access to overseas plasma products via the Special Access Scheme which is limited to one produced from British plasma BPL 's Factor XI or the French product Hemeleven. Only a very small number of patients require the product each year for surgical procedures.

Plasma Derived factor VII is also only available from overseas plasma suppliers but there is a very insignificant demand for this. Factor VII patients requiring Factor VII replacement only require a very small amount and thus it would be seen as a safer alternative to use the recombinant factor VII product.

Fibrogammin is also sourced from overseas supplier and manufactured from overseas plasma. There is very low usage of this product and not anticipated that the demand would increase over the next ten years.

1.3 *What demand trends are likely to arise for plasma products in Australia over the next ten years?*

The greatest increase in use will be in the IVIg market.

1.4 *What are the current and future supply requirements for Australian Plasma products?*

Currently there is decreasing demand for plasma derived Factor VIII and Factor IX products, but it is envisaged that there will always be a need for these products to be available to patients who for what ever reason are unable to use Recombinant products.

Identify appropriate requirements to be met by producers of plasma products or suppliers of fractionation services to ensure safety, quality and efficacy of such products or services. These requirements shall not create unnecessary obstacles to trade

2. Regulatory requirements

Identify the issues as a result of any increase in competition for the provision of plasma fractionation services for Australia and indicate how these issues could best be dealt with through future procurement arrangements

3. Increasing competition

3.1 *What are the options for increasing competition in plasma fractionation services in Australia and how could they be implemented?*

The options would be to allow a second company to establish a plasma fractionation plant in Australia. An alternative would be to send the Australian plasma off shore for fractionation to a company who has been awarded a tender after complying with all safety, transportation, packaging and cost issues.

3.4 *What is security of supply and how could Australia ensure security of supply if it increased competition in plasma fractionation services?*

Security of supply would be seen as the provision of enough products to meet all demands. Australia could ensure security of supply by negotiating to be a preferential recipient. If there was a problem with the plant and the product could not be processed for whatever reason, it could be negotiated that the company would have to organize for another plant to process the plasma or source appropriate overseas plasma to fill the short fall.

3.6 *How could Australia maintain safe, high quality, efficacious plasma products while increasing competition?*

By having one organisation collecting the starting plasma and having the plasma fractionated by more than one fractionator in Australia according to Australian Regulations controlling safety, quality and efficacy of the end product

3.7 *What risk mitigation and security measures would need to be implemented to ensure safety, quality, efficacy and security of supply of plasma products if competition was increased?.*

The company had adequate insurance both covering manufacture / manufacturing problems of the product and transportation of the product if it was to be fractionated outside Australia. That several different batches be manufactured each time and brought into the country to cover any loss of product, if a batch did not pass quality assurance. It would have to ensure that the product if manufactured overseas was brought into the country with an adequate shelf life to reduce wastage. It would be essential that a process for stock rotation by the manufacturer be in place.

3.8 *What potential implications for the collection of the plasma starting pool for fractionation which may arise from options for increasing competition?*

Management of a small pool of Donors would be an issue. Currently some of the starting plasma supplied by ARCBS is produced, as a result of by products from red cell or platelet concentrate production. Would the starting plasma still be collected by one organisation and then distributed to the fractionating organizations? If both fractionators were required to collect their own plasma there would be implications for the fresh blood supply because there is only a small donor group.

3.9 *Which organization would best be best placed to maintain the national reserve if Australia increased competition for plasma fractionation services?*

Australian Red Cross Blood Service

3.10 *Would the role or obligations of the National Blood Authority need to change if there was increased competition for plasma fractionation services?*

The role would need to encompass tender /contact negotiations and contract management and funding of plasma fractionation

Conclusion

Patients receiving plasma products feel very vulnerable. Many have lived through both the HIV and the Hep c era. Many Haemophilia/Von Willebrands patients who received plasma products during the late 1980's early 1990's are Hepatitis C positive. Patients on plasma products still live in fear of the "next virus". For them safety of the product is of paramount importance. Coupled with that is the trace ability of the product, should there be a problem with a batch or a Donor at a later date. The product should be able to be tracked and traced right from manufacturer to patient.

The product should be manufactured according to Australian standards. If the starting plasma is to be shipped off shore for fractionation there should be adequate checks that the plasma will be stored and shipped correctly. Ongoing monitoring of this would be paramount. When it comes back in the country it should meet Australian guidelines for safety, and efficacy and it should have an reasonable shelf life for use .The company producing the product should provide adequate support for their product and be responsive to consumer needs.

The plasma fractionation market in Australia does not appear to be a large market and it would have to be examined to see if it was viable to have more than one fractionator in the market, and the affect that this would have on the Donor pool.

Two plasma fractionators would alleviate some of the risk mitigation, but having said that if one plant went down, there would conceivably be some reliance on overseas plasma until the other plant could increase their output to service all requirements.