Fabry support group Australia

Senate Finance and Public Administration Committees
PO Box 6100
Parliament House
Canberra ACT 2600
Australia

21st July 2011

**RE: The Government's administration of the Pharmaceutical Benefits Scheme**

Dear Committees,

The Fabry Support Group of Australia (FSGA) welcomes the opportunity to provide comment on the Government's administration of the Pharmaceutical Benefits Scheme (PBC).

Fabry disease is a rare, progressive, genetic, fatal condition that requires specific life saving treatments. Such life saving therapies don't meet the cost effectiveness under the PBS and are referred by the PBAC for funding under the Life Saving Drugs Program. Two Fabry treatments have been funded under the LSDP since 2004 after being referred by the PBAC in 2002. This program has been under review since 2008 and continues to be reviewed.

As health consumers representing a National rare disease organisation currently receiving funding for 2 life saving therapies, we would like to highlight the following points for consideration and inclusion in your administration committees. With particular reference to the following:

1) **The deferral of listing medicines on the PBS that have been recommended by the Pharmaceutical Benefits Advisory Committee.**

   - It is our understanding that traditionally, PBS medicines which cost the government less than $10 million per year in total would be listed once PBAC approval and following price negotiation being around 3 to 4 months after PBAC. However drugs costing more than $10 million would be considered by Cabinet (with exceptions such as Viagra and nicotine patches, usually approved but only once Cabinet would hold them up in order to save money.

   - **This is a similar situation with the life saving drugs listed on the current LSDP program which have always gone via Cabinet facing delays before being approved.**

   Here are the listing times for all medicines on the LSDP since it was formed post Cerezyme®.

| | |
|---|---|
| Fabrazyme® | PBAC referred Dec 2002, funded in July 2004 – 19 months |
| Replagal® | PBAC 1st submission Sep 2002 (rejected)<br>PBAC 2nd submission Sep 2003 (rejected & referred),<br>funded in July 2004 - 22 months |
| Aldurazyme® | PBAC referred Nov 2004, funded July 2007 – 32 months |

| | |
|---|---|
| Naglazyme® | PBAC referred July 2007, funded July 2008 – 12 months |
| Elaprase® | PBAC referred Nov 2007, funded December 2008 – 13 months |
| Myozyme® | PBAC referred July 2008, funded February 2010 – 19 months |
| Soliris® | PBAC referred (first time) March 2009, funded Jan 2011 – 22 months |

**This comes to an average of 19.5 months to list a product on the LSDP!**

- It appears that this process is already a lengthy process for not only companies but patients with life threatening conditions such as Fabry disease. People who have a rare life threatening condition depending on such drugs do not need such delays with their life saving treatments! Patients with rare conditions don't have time on their side and they do die! They need these 'life saving' drugs!

- But the announcement in February that ALL drugs (not just high cost) would go through Cabinet and 7 have been deferred until budget circumstances permitted is simply ludicrous, unethical and unacceptable. This was unprecedented because many of them would not have previously gone through Cabinet and because Cabinet almost never deferred or rejected drug listings.

- As well as the current Enzyme Replacement Therapy (ERT) funded by the LSDP, most Fabry patients receive a range of PBS listed medications for the ongoing management of their Fabry health and most of these medications are both life saving and dependent medications.

**a) Any Consequences for patients of such deferrals;**

- The Government claims that they have always had the right to not list medicines and that they are sticking to their commitment on Cabinet review within 6 months.
- But you are now dealing with ALL drugs and also this is not a guarantee of funding! To justify the decision by claiming that the deferrals would not have much impact since the products were not life-saving and that an alternative existed is also outrageous!
- Most doctors need to use a range of medicines to treat the same condition.

  For example: a patient who has high blood pressure a doctor normally prescribes a drug and see if this is a 'good fit' for the patient. But if not, moves to another and continues to do this until they find a suitable drug for their patient. If you are not giving choice for doctors to do this, then you are putting the patient at risk as well as their treating doctor! How can the doctor treat their patient adequately without the availability of medications? Based on cost?
- The obvious consequence for patients of such deferrals surely does not need to be explained. Patients receiving medications NEED them and rely on them. This is both unethical and dangerous. You are putting both patients and the treating doctors in a very unstable and life threatening situation.

  How can the Government be confident in its decision knowing that patients' health is at risk?

**d) Impacts on the future availability of medicines in the Australian market due to such deferrals;**

- It seems that really the government is being fiscally responsible and subjecting drug spending to the same scrutiny as all other spending. This is somewhat misleading as the drugs have already gone through the PBAC!
- Surely the PBAC sets a far greater, higher standard for showing cost/benefit than say education or defence spending?
- The deferral will make it very difficult for companies who have treatments and medicines in the pipe line in clinical trials in order to attract investment.

**h) Compliance with the intent of the Memorandum of Understanding signed with Medicines Australia in May last year**

- It is understood that the government committed in its Memorandum of Understanding with the pharmaceutical industry last year to make better efforts in listing new medicines within 6 months. But there are a lot of examples where LSDP medicines have been delayed significantly, and this has not been without consequence for patients as listed earlier in point a) on average the current listed drugs 19.5 months before funding via LSDP! See point 1) current funded life saving drugs list on the LSDP.

**i) Other relevant matter**

The following statement:
*"**Importantly, high-cost innovative medicines that treat serious or life-threatening conditions continue to be listed."***
This is a false statement! It is a fact that Fabrazyme® for newly diagnosed Fabry patients is not listed in the 2011/2012 budget! Myozyme® for late onset Pompe is also not currently listed and has been rejected over 5 times! There are many life saving treatments that are not listed under the LSDP but listed in other regions.

FSGA has made a submission to; 'Comments on the draft LSDP Submission Guidelines.' In this submission the following points have been raised;

1. **Protection of current funded treatments listed on the LSDP**.
FSGA would like to be reassured that unless there is conclusive evidence that suggests a life saving therapy does not do what it was thought that it did at the time it was funded and approved, that they remain listed and continue to receive funding as life saving therapies for Fabry patients in Australia.

2. **Support for alternative therapies.**
There needs to be support for alternative therapies to ERT for conditions where ERT is already funded and where there is shown to be disease stabilisation with the alternative therapy.

3. **The "Process for Government Approval of Funding" section in the Administrative Requirements paper**
The wording of this section could allow the government to not list or delay PBAC referred drugs because of competing financial priorities.

- Is there a framework for assessing these priorities?
- Is there an explanation of what might be more important than life saving medicines?
- Is there a commitment to any particular timeframe?

For the **new administrative requirements** for the LSDP, we're concerned about the following statement on p4:

*"The timeframes for a Government decision are dependent on the business of the day, and the Government's priorities at that time. The Government has a number of competing priorities at any given time and these need to be weighed up when considering the total approval time for a drug after a positive PBAC recommendation."*

It is important that the government understands the impact on patients with these kinds of delays. Once a product has been deemed "life-saving", the obvious issue is that any delay with a life saving drug has consequences to patients. The government needs to make a pretty good case for why other competing priorities take precedence. It is understood that the government committed in its Memorandum of Understanding with the pharmaceutical industry last year to make better efforts in listing new medicines within 6 months. But there are a lot of examples where LSDP medicines have been delayed significantly, and this has not been without consequence for patients as listed earlier in point a) on average the current listed drugs 19.5 months before funding via LSDP.

Thank you for considering the views expressed in this submission on behalf of the Australian Fabry community.

Kindest Regards,

Megan Fookes
President
Fabry Support Group Australia Inc.