PNHSAA PNH Support Association of Australia Inc.

Linda Charlton
PRESIDENT

30 March 2010

Committee Secretary
Senate Community Affairs References Committee
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Parliament House
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Dear Sir/Madam,

Submission regarding Inquiry into Consumer Access to Pharmaceutical Benefits

I write on behalf of our members and other Australian sufferers of Paroxysmal Nocturnal Haemoglobinuria (PNH), a very rare and potentially fatal bone marrow disease.

PNH is an acquired stem cell mutation that causes high levels of hemolysis, resulting at times in serious and life-threatening complications. Further, it leaves sufferers with chronic fatigue, pain and a compromised immune system, together with long-term risk of organ damage, stroke, thrombosis and increased mortality. Thirty five percent (35%) of PNH patients die within 5 years of diagnosis.

I refer to the terms of the reference of the above inquiry and respond below specifically to items (a), (d) and (g).

(a) The impact of new therapeutic groups on consumer access to existing PBS drugs, vaccines and future drugs, particularly high cost drugs

PNH sufferers constitute a very small therapeutic group of approximately 70 identified in Australia today, of which at least half would be considered by experts to be at high risk of further complication and death (i.e. the patients at high risk of death within 5 years as described above). At present, the only cure for PNH is a bone marrow transplant which is prohibitively expensive and in most cases not indicated due to its high morbidity and mortality rates.

Most patients are treated with supportive therapies to treat progressive symptoms as they occur – blood transfusions to replace destroyed red blood cells and corticosteroids to slow hemolysis. Various other drugs and supplements are prescribed to patients to assist coping with pain, fatigue and other symptoms of the disease, as well as vaccines to prevent serious infections. Many patients are also prescribed anticoagulants to prevent any or further blood clots, which is especially important given that thrombosis is the leading cause of death of PNH patients.

These treatments result in numerous hospital admissions per patient, both as in- and outpatients, generally for life.

An alternative therapy with almost 100% efficacy in terms of returning red blood cells to normal functioning was approved by the Therapeutic Goods Administration in February 2009, a monoclonal antibody called Eculizumab. This drug is a high cost, ultra-orphan drug and funding through the PBS was rejected on cost-effectiveness grounds but referred by the PBAC to Government for subsidisation under the Life Savings Drug Program in April 2009. A funding decision has not yet been made, however approximately one-third of diagnosed PNH patients are receiving the treatment through the manufacturer's compassionate access scheme, with at least one-third more expected to fulfil the proposed criteria and receive treatment if funding is approved in 2010.

For those receiving the treatment in Australia and countless others internationally, quality of life has improved considerably with reduced pain, fatigue and a lower thrombotic risk.

Should this therapy become available, its high cost is greatly outweighed by the benefits to patients in enabling them to lead fuller, more productive lives. Research has shown that the risk of thrombosis is reduced by up to 85% and as symptoms are minimised or alleviated, there will be less reliance upon other PBS drugs and the healthcare system as a whole by this therapeutic group. Access to the treatment by this group will not prejudice access by other consumers to existing PBS drugs and vaccines.

Eculizumab is approved as an orphan drug and funded in USA, Belgium, Denmark, France, Germany, the Netherlands, Italy, Spain, Finland, Austria and the UK. It is currently under final stage review for 2010 funding in Japan, Canada, Taiwan, Ireland, South Korea and Switzerland.

It is also our understanding from consult with the manufacturer (Alexion) that the company has set a precedent for industry in offering upfront to government significant discounts, budget capping and other financial risk sharing arrangements via the PBAC and LSDP to increase certainty and budget sustainability. We have been assured by the manufacturer that these measures are intended to ensure no patient deemed to be at high risk by clinical experts will go without treatment whilst Government will have absolute budget certainty going forward with an approved program.

(d) consultation undertaken in the development of new therapeutic groups

I believe it is important for the consultation process to be more widely advertised to ensure that all stakeholders have the opportunity to submit responses. I am only aware of a call for submissions through the Federal Health Departments website regarding upcoming decisions of the PBAC in relation to specific drugs. It would be preferable for consultations to be undertaken prior to that point in the process and indeed for patient awareness groups, such as this Association, to be given the opportunity to view the proposed criteria of therapeutic groups where relevant and advertisement of same through a more accessible channel.

(g) the process and timing of consideration by Cabinet of high cost drugs and vaccines

By far the most frustrating aspect of the approval and funding process of Eculizumab is the lack of certainty of the process and timing of Cabinet's consideration and funding approval of the drug.

We have received a groundswell of support from MPs and Senators across the country who have urged the Health Minister to consider the matter with urgency, however despite assurances by the Health Minister that the Government will endeavour to ensure that the process is resolved in a timely manner, there is no set procedure or timescale of which I am aware against which to measure this promise.

This is particularly concerning when already there has been a delay of some 14 months since the treatment was approved by the TGA, 12 months since the PBAC recommended Government consider funding under the LSDP and it has been more than 9 months since I wrote to the Health Minister and other MPs and

Senators about this life threatening disease. With 35% of patients dying within 5 years of diagnosis, this delay represents almost 25% of a sufferer's possible life expectancy without treatment.

We are aware that the consideration of high cost drugs and vaccines is complicated, involving many factors and negotiations between the relevant parties, however I believe it would be beneficial to all concerned parties to have a set procedure and timeline to provide some assurances to all those involved and in particular to patients who are awaiting possible treatment.

We have attached a disease information flyer for inclusion in this submission.

Thank you for consideration of this submission. Should you wish to discuss, please do not hesitate to contact me on the number below.

Yours sincerely,

Linda Charlton
President
PNH Support Association of Australia Inc

Please note our new telephone and postal address, our email remains the same:
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