ITP Australia Ltd
ACN 625 253 394

11/9 Talbot Street
PO Box 130
Coorparoo QLD 4151

☎ 0409 611 545
E info@ITPAustralia.org.au
W ITPAustralia.org.au

Tuesday, 3 November 2020

Committee Secretariat
PO Box 6021
Parliament House
CANBERRA
Canberra ACT 2600

Dear Committee Secretariat,

Parliamentary Submission: Inquiry into the approval processes for new drugs and novel medical technologies in Australia

Thank you for the opportunity to make a submission to the House of Representatives Standing Committee on Health, Aged Care and Sport.

This submission has been developed with a focus on ways to improve the accessibility of new therapies and novel medical technologies specifically for patients with ***Immune Thrombocytopenia*** (ITP), a rare autoimmune disease where patients experience a low platelet count as their immune system attacks their platelets.

Herein we outline the need to alter the eligibility criteria for Australian patients to access treatments under the Pharmaceutical Benefits Scheme (PBS), in line with international research and treatment protocols in other countries including United States of America, the United Kingdom and across Europe.

We thank the Minister for Health and Minister Assisting the Prime Minister for the Public Service and Cabinet, the Hon Greg Hunt MP for recognising the need to improve access to new drugs, treatments and novel medical technologies. ITP Australia also acknowledges the important and pioneering work done to develop the National Strategic Action Plan for Rare Diseases, published in February 2020.

We trust that this submission is useful and informative for the consideration by the Committee and we are happy to elaborate or discuss any elements of the content as needed.

Kind regards
Danielle Boyle
CEO, ITP Australia Ltd

INTRODUCTION

ITP Australia Ltd is a patient organisation focusing on patients with Immune Thrombocytopenia (ITP) and their caregivers. ITP Australia is a member of Rare Voices Australia and an active member of the ITP International Alliance, a global group of international patient associations.

ITP Australia was launched in early 2018 and has been working to offer patients and their support teams much needed Australia-focused information. Engagement with our community over the past three years demonstrated to ITP Australia that patients needed a voice, and we started to provide support and advocacy for patients, projecting their voices with key influencers and stakeholders including clinicians, pharmaceutical companies and government.

This submission includes a number of patient-focused insights.

ITP Australia will be focusing on the following Terms of Reference (TOR) in this submission.

- TOR 1 - *The range of new drugs and emerging novel medical technologies in development in Australia and globally, including areas of innovation where there is an interface between drugs and novel therapies;*
- TOR 2 - *Incentives to research, develop and commercialise new drugs and novel medical technologies for conditions where there is an unmet need, in particular orphan, personalised drugs and off-patent that could be repurposed and used to treat new conditions;*
- TOR 4 - *Without compromising the assessment of safety, quality, efficacy or cost-effectiveness, whether the approval process for new drugs and novel medical technologies, could be made more efficient, including through greater use of international approval processes, greater alignment of registration and reimbursement processes or post market assessment.*

This submission will be focusing on Thrombopoietin-receptor agonists (TPO-RAs), with a primary focus on Eltrombopag.

- TPO-RAs include:
    - Eltrombopag, commercially known as Revolade
    - Romiplostim, commercially known as Nplate

SUBMISSION

Currently, the Pharmaceutical Benefits Scheme (PBS) provides ITP patients in Australia with access to TPO-RAs under the following criteria:

- The condition of Immune Thrombocytopenia (ITP) must be classified as chronic in the patient
- The patient must have undergone the following therapies:
    - Splenectomy – or there is a contraindication to splenectomy
    - Intravenous immunoglobulin (IVIg)
    - Corticosteroids
- The patient must have a platelet count of less than 20×10^9 /L
-

Given the significant learnings developed through medical research and trials and especially in the lessor studied rare diseases sector, an acceptable review period for TPO-Ras and current patient use criteria has been exceeded. Since TPO-RAs were first registered with the Therapeutic Goods Administration (TGA) in July 2010 and submitted to the Pharmaceutical Benefits Advisory Committee (PBAC) for consideration in July 2013, there has been no change to the criteria for patients to receive these treatments.

As identified in the National Strategic Action Plan for Rare Diseases (2.4.3), people living with rare diseases, including Immune Thrombocytopenia (ITP), are to have equitable access to medicines with demonstrated clinical benefits for rare disease, and regardless of the growing evidence (see Appendix) around the efficacy of TPO-RAs for ITP patients, there has been no change to the criteria to access these treatments in Australia. In addition, the SPRITE Clinical Trial for the efficacy of Eltrombopag in Australia showed positive results for patients receiving TPO-Ras - prior to undergoing a splenectomy.

The main criteria change requested by ITP Australia is the requirement that ITP patients undergo a splenectomy. The splenectomy is an invasive and irreversible treatment, with an approximate 60% rate of placing the patient into remission (not a cure). There are no tests to identify which ITP patients will respond successfully to a splenectomy and further, of those patients that do successfully go into remission there is no guarantee of lifelong remission.

In addition, TPO-RAs have a reduced daily impact on the ITP patient (see the Patient Perspective) when compared with other first and second line treatments including corticosteroids and Intravenous immunoglobulin (IVIg) both of which are required courses of treatment under the PBS criteria above.

Recently published international treatment guidelines support TPO-Ras as a second line treatment, including:

- the International Consensus Report for the Diagnosis, Treatment and Management of ITP;
- the American Society of Hematology (ASH) Treatment Guidelines; and
- the European Haematology Association (EHA) Treatment Guidelines

Reference to this research can only assist in expediting the process to making TPO-RAs available to ITP patients as a second line treatment.

Key to the ITP Australia submission is the acknowledgement that this change will have several positive outcomes for ITP patients, including:

- Improved patient Quality of Life (QoL)
- Reduced incidents of active bleeding
- Reduced financial and (direct/in-person) burden on the Public Health Network (PHN)
- Reduced financial burden to patients and their caregivers

PATIENT PERSPECTIVE: Danielle Boyle

I was diagnosed in July 2015 upon presenting to the emergency department at my local hospital with chest pains. During the investigation phase of my visit, it was identified that I have a low platelet count and could potentially have Immune Thrombocytopenia. This was a chance diagnosis.

I returned to my local hospital for further review and was admitted into the oncology ward, where I physically looked healthy and well, however my platelet count continued to fall.

I underwent a high dose of corticosteroids (Prednisone) while an in-patient and was monitored daily until my platelet count had returned to what was considered a 'safe' platelet count of 30×10^9 /L.

While I underwent this treatment, the side effects that I developed included mental health issues, weight gain, swollen face (moon face), insomnia and highly erratic mood swings. After a number of weeks, I was then placed on the SPRITE Clinical Trial (see Appendix) where I was administered a TPO-RA (Eltrombopag) and started to taper off the corticosteroids.

Having access to the clinical trial and this treatment early in my patient journey gave me three years of minimal symptoms of ITP and it improved my quality of life as it reduced the daily impact of my rare disease.

Unfortunately, due to the current criteria, many patients do not get the opportunity that I had in receiving this treatment early as part of their journey. Having to remove their spleen, regardless of there being no guarantee that it will work, has life changing implications for these patients and their families.

As mentioned in the submission, many patients do not wish to have their spleen removed as this will have lifelong general health impacts. And if this line of treatment does not work, then the patient now has two diseases to consider as well as the greater impact on their daily life.

Patients without a spleen have to undergo additional immunisations and take prophylactic antibiotics for at least three years post-surgery, while some patients require antibiotics for the rest of their life. These patients, in addition to their rare disease, are at greater risk of infections, especially pneumococcal infection plus other bacterial infections, resulting in greater burden on the PHN, as well as the patient's quality of life.

I have spoken with hundreds of patients over the last three years including frontline workers (health professionals and teachers) who are exposed to the general public regularly, ITP patients with young children and parents of young ITP patients. All of these people are exposing themselves to greater health risks caused by having had their spleen removed before all treatment options were available to them.

RECOMMENDATIONS:

ITP Australia's recommendations for this Parliamentary Inquiry include:

1. Regularly review the criteria required to access current drugs and treatments, including TPO-RAs for ITP patients, to ensure that more rare disease patients receive access to appropriate and more effective treatments within a shorter timeframe.

2. Utilise evidence from reputable international agencies to support review processes and make regular changes to the criteria of current and emerging drugs and treatments, thereby improving the access to appropriate and more effective drugs and treatments for patients with rare diseases, include ITP patients.

3. Review the way the PBAC makes decisions on the availability of treatments for rare diseases, including ITP. This includes a restructure of the economics of treatments to include not just the immediate costs associated with a drug or treatment, but to include the lifelong economics of a drug or treatment, especially for those rare diseases like ITP that require long term and lifetime treatments.

4. Ensure that the patient voice is heard and considered when making decisions and approving drugs and treatments as they ultimately impact patients. This includes, but is not limited to, working with rare disease organisations and consulting effectively on patient criteria.

These recommendations are aligned with the National Strategic Action Plan into Rare Disease, published in February 2020.

APPENDIX

1. National Strategic Action Plan for Rare Diseases
    a. Pillar 2: Care and Support
        i. Priority 2.4: Enable all Australians to have equitable access to the best available health technologies
            1. Action 2.4.3 – Ensure people living with a rare disease have equitable access to medicines with demonstrated clinical benefits for a rare disease, including those that are already funded for another condition.
                a. Ensure funding and reimbursement in the outlined in the recently updated International Consensus Report into the diagnosis, treatment and management of ITP amongst patients, the treatment protocol for TPORAs have been elevated ABOVE that of a splenectomy, which forms part of the current Australian requirements to accessing these treatments through the PBS.

2. SPRITE Clinical Trial:
    a. A dose finding trial for TPO-RA treatment Eltrombopag (commercially known as Revolade) to understand its efficacy for refractory patients with Immune Thrombocytopenia BEFORE splenectomy.
        i. Registration number - ACTRN12613000721707
        ii. Universal Trial Number (UTN) U1111-1144-4732
        iii. Trial Acronym – SPRITE
        iv. Enrolment Period – 15/7/2013 – 9/2/2016
        v. Completion Date – 7/8/2018
        vi. Further information - https://www.anzctr.org.au/Trial/Registration/TrialReview.aspx?id=364423

3. Spleen Australia Current International Access to TPORAs:
    Medical Recommendations for A splenetic adult patients - https://spleen.org.au/wp-content/uploads/2020/03/RECOMMENDATIONS_Spleen_Registry.pdf

4. International Consensus Report Current International Access to TPORAs:
    a. International Consensus Report into the Diagnosis, Treatment and Management of Immune Thrombocytopenia – Reviewed and published in December 2019 - https://ashpublications.org/bloodadvances/article/3/22/3780/428877/Updated-international-consensus-report-on-the
    b. American Society of Hematology (ASH) Guidelines for ITP – Reviewed and published in December 2019 – https://ashpublications.org/bloodadvances/article/3/23/3829/429213/American-Society-of-Hematology-2019-guidelines-for

5. Current International Access to TPO-RAs:

Country/Region	Approving Organisation	Initial approval	2nd line treatment Approved (pre-splenectomy)
United States of America	Food and Drug Administration (FDA)	2008	tbc
United Kingdom	National Institute for Health and Care Excellence (NICE)	2013	2018
Europe	The European Medicines Agency (EMA)	2010	2018
Brazil	Brazilian Health Surveillance Agency (ANVISA)	2010	2019
Israel	The Pharmaceutical Division, Ministry of Health	2010	2019
Argentina	The National Administration of Drugs, Foods, and Medical Devices (ANMAT)	2010	2018