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Global Healthy Living Foundation Australia

Submission to support the Section 100 listing of teprotumumab (Tepezza®) for active moderate to severe thyroid eye disease

For consideration at the March 2025 PBAC Meeting

These comments are submitted by Global Healthy Living Foundation Australia (GHLF Australia) on behalf of our patient community. We appreciate the opportunity to provide this submission.

PBAC public consultation survey

Q1: Please outline your experience with the medical/health condition

About GHLF Australia

GHLF Australia is a not-for-profit organisation founded in 2015. GHLF Australia is part of the US-based Global Healthy Living Foundation (GHLF), a non-profit organisation whose mission is to improve the quality of life for people with chronic illness.

Established in 2014 and incorporated in 2016, GHLF Australia is the parent organisation of CreakyJoints Australia, the vibrant online patient community for autoimmune and inflammatory arthritis patients and their families throughout Australia.

Drawing on nearly two decades of foundational knowledge, success and the reputation of GHLF and CreakyJoints, GHLF Australia aims to localise, mobilise and engage the Australian patient and caregiver community and to provide education, advocacy and research for better health outcomes.

Our organisation connects patients with current and relevant condition-specific information and support across a range of conditions, using a diverse set of digital platforms.

About thyroid eye disease

Thyroid Eye Disease (TED) is a little-known autoimmune condition that can lead to decreased quality of life, permanent disfigurement and vision loss in some people. Common symptoms include:

- Bulging eyes (proptosis)
- Eye pain or eye pressure
- Retracted eyelids
- Dry, gritty eyes
- Watery, teary eyes (epiphora)

- Puffy eyes or eyelids (periorbital oedema)
- Inflamed eyelids (blepharitis)
- Red eyes or eyelids or bloodshot eyes
- Light sensitivity (photophobia)
- Blurry vision
- Misaligned or crossed eyes (strabismus).

There are two distinct phases of TED, the acute or active phase and the chronic or inactive phase. The acute phase occurs suddenly and can last up to three years. In the chronic phase, symptoms may improve, but the appearance of proptosis can remain.

While symptoms such as pain, inflammation and double vision can be managed through limited treatment options or surgery, there are currently no disease-modifying treatments approved specifically for TED in Australia. At GHLF Australia, we want to help change all that.

Our experience with thyroid eye disease

As a global organisation working across a range of autoimmune diseases, we are acutely aware of the variations in the availability of medicines and resources for patients in different parts of the world. When we recognised the unique challenges faced by Australians living with thyroid eye disease (TED) — a rare and debilitating condition — we were compelled to step in and offer greater support and resources to this underserved community.

In Australia, there is no official registry or dedicated patient organisation for TED, and the information available is largely written for health professionals, leaving patients and caregivers without the tools and guidance they need. To address this, we developed the ***Thyroid Eye Disease Education and Advocacy Hub***, designed to empower Australians living with TED by providing comprehensive, patient-friendly information and practical support.

The hub includes:

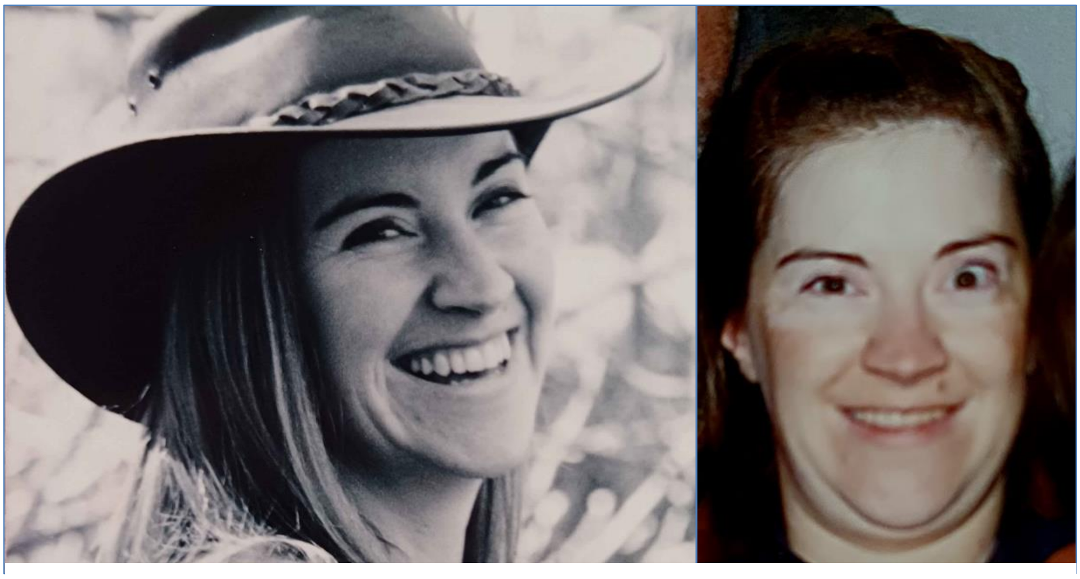
- In-depth, reliable information on TED, its symptoms and current treatment options.
- Practical advice for managing symptoms and building a healthcare team.
- Acknowledgement of and support for the emotional and mental health impacts of living with TED.
- Guidance on how to advocate for access to new treatments in Australia.

Understanding the lived experiences of TED patients was central to our work. Through social media campaigns and connections with health networks, we reached out to people living with the condition and listened to their stories.

We hosted a virtual “meet and greet” with patients like Mary, Marion and Graham, who shared their personal journeys and perspectives. Veronica, who connected with us through our outreach, allowed us to feature her story in an article on our website and in social media video reels. At the time of writing, we believe at least two of the patients we've spoken with will write their own submissions for teprotumumab.

The insights we gathered from TED patients were further enriched by the ***Through My Eyes: Understanding the Impact of Thyroid Eye Disease on Australians*** booklet, which highlighted the voices and challenges of this community.

By creating resources, amplifying patient stories, and advocating for equitable access to life-changing treatments, we aim to fill the gap in support for Australians with TED and help ensure they have access to the care and medicines they need.



Veronica before and after her thyroid eye disease symptoms first appeared.

Q2: How is the medical/health condition currently treated?

Points for individual consumers to consider

- *What is the effect of your current treatment on your health condition?*
- *Are there any symptoms which cannot be controlled with the current treatment?*
- *What side-effects have you experienced with current treatments? Are these manageable?*
- *Do you have any issues accessing your current treatment? (For example, where or how it is given, how it is funded, whether you fit the criteria to qualify for access.)*

Please provide your comments.

Access to care

One of the biggest barriers to care for Australian TED patients is a lack of awareness of the condition, even in the health sector.

Early symptoms of TED can include gritty, dry, red or inflamed eyes. These can be misdiagnosed as dry eyes or infections by GPs, optometrists, hospital staff and other health professionals. Even when eye bulging and crossed eyes become apparent, clinicians and patients themselves are often none the wiser.

TED cannot currently be diagnosed via a specific laboratory test or symptoms. Instead, doctors need to conduct several tests to help eliminate other possibilities and see what is happening within and around the person's eyes.

Effective TED treatment can relieve not only symptoms but underlying inflammation that can cause other serious problems over time. Therefore, it is important for patients to get an accurate diagnosis and see an ophthalmologist early in their disease journey. However, Australia has a shortage of ophthalmologists and many of the ones we have don't have a lot of experience with TED. Access to knowledgeable care providers is even harder in regional, rural and remote areas.

Current TED treatments

Topical treatments and lifestyle changes only help with mild TED symptoms or in conjunction with medical treatments.

As there are currently no medicines in Australia specifically designed to target the parts of the immune system that affect TED disease progression, the only medical treatment options we have either suppress the whole immune system or only work on managing TED symptoms.

It is worth noting that people respond to medications differently and some people might not get a response at all.

Steroid therapy

Steroids are used for a wide range of conditions, including autoimmune diseases, asthma, allergies and tumours. Steroids are effective for treating inflammation and soft tissue symptoms but are often not effective in addressing double vision and eye protrusion.

Those with active vision-threatening disease will likely be given a weekly or fortnightly infusion of steroids (intravenous methylprednisolone or IVMP) at a high dose with a total of 12 infusions. These are administered by an appropriately trained health professional.

Over the course of treatment, the patient's responses will be monitored closely with the benefits weighed up against the severity of any side effects. If symptoms have improved enough to reach key milestones, patients will complete the course of treatment and their symptoms will continue to be monitored. They may need another course of steroid infusions or non-medical treatments if their symptoms return.

As with all forms of steroid therapy, IVMP can cause many, and sometimes severe, side effects including (but not limited to) diabetes, osteoporosis, high blood pressure, gastric issues, weight gain and sleeplessness especially if used at high doses or over long periods.

In our interview with Veronica, she told us she is managing a roller coaster of side effects from IVMP, including having trouble sleeping, increased fatigue, mood changes, digestive upsets and having a red, flushed face the day after her infusion. All of these have a negative effect on her quality of life.

"Because steroids cause a whole lot of other kinds of things you don't want, it's been really important for me to take the lead in negotiating with my endocrinologist and my ophthalmologist around how's that going to be managed and asking lots of assertive questions… gathering information so that I felt confident the decision I was making was the best one.

I guess there was an option to do nothing, but the risk of that with serious thyroid eye disease is of the muscle compressing the optic nerve and causing vision loss or blindness. And because it's not the eye that can't see, it's the nerve that won't let the message through. It's not repairable. It can't be treated if it gets to that stage. And it was certainly not something I wanted to risk." **— Veronica**

Oral immunotherapies

If IVMP alone has not worked well enough, some TED patients might be offered an immunotherapy medicine. Oral immunotherapy medicines commonly used for TED include mycophenolate, cyclosporin and azathioprine.

These work by suppressing the immune system and reducing inflammation. They can slow or even stop disease progression whereas the treatments mentioned above only treat symptoms. Immunotherapy can be used instead of or as well as steroids.

However, immunotherapy can have many uncomfortable side effects similar to those caused by IVMP. Taking immunotherapy medicines also increases patients' risk of infections and skin cancer.

Biologics

Biologics are a form of immunotherapy treatment administered directly into the bloodstream. The two biologics currently available for Australians with TED are tocilizumab and rituximab. Yet, these were not developed specifically for TED and are only used in specific cases where all other treatments have failed.

Non-medicinal treatments

There are a few non-medicinal treatments used for people with thyroid conditions in Australia. However, radiotherapy laser treatment and radioactive iodine are not appropriate for TED patients as they can aggravate the condition.

Thyroidectomy surgery is sometimes used to help moderate TED symptoms, but it is invasive, does not prevent TED from occurring or reoccurring and it can also have other health consequences.

Two patients we spoke with were offered a thyroidectomy as a treatment for their thyroid eye disease but were given no guarantees it would help their condition. This left them feeling uncertain about their treatment options and their future. Marion developed TED after a thyroidectomy.

Corrective eye surgery is also a very invasive procedure that can only minimise the structural damage caused by TED.

Overall, surgeries can be expensive and are associated with increased anxiety, depression and often painful recovery.

"I decided not to pursue any treatments for my condition. My ophthalmologist mentioned that the only available option at the time was a minor surgery. After my thyroidectomy, my calcium levels dropped drastically, and I didn't want to risk another invasive procedure that could lead to more side effects. The thought of undergoing eye surgery and potentially facing further complications made me hesitant, so I chose not to proceed with any treatment." **— Marion**

Living with TED

The TED patients we spoke with helped us gain excellent insights into what it's like to live with TED symptoms and treatments. We believe it is just as important for the PBAC to be aware of their perspectives as it is to learn about the condition and current treatments.

The physical and mental effects of the visible symptoms are among the ones that impact TED patients the most. Not only are they in pain from the inflammation, irritation and eye pressure, they are also embarrassed about their bulging and misaligned eyes.

This stigma and loss of self-esteem prevents many people with TED from going out and can have a negative effect on their relationships. They can also actively avoid mirrors or having their photo taken. Many use dark glasses or hats to hide their appearance, which also helps manage their sensitivity to glare.

"The way TED changed my face completely smashed my self-confidence. Friends and dating were all put on hold. I went into periods of dark depression. I can understand why people would want to take their own lives over this." **— Steve (from *Through My Eyes: Understanding the impact of Thyroid Eye Disease (TED) on Australians*)**

Shaking and palpitations were early symptoms mentioned by several people we spoke with.

While the impacts on vision are less visible to others, they have a profound effect on the people experiencing TED. Double vision and eye fatigue make it difficult to do day-to-day activities like reading a computer screen, driving, cooking, caring for others or pursuing their hobbies.

For some, that also means not being able to do the work they normally do. That can mean they have to change jobs or stop working altogether, which can have a big financial impact and their future plans.

Underlying all that is often the fear of side effects of medical or surgical treatment for moderate to severe cases or permanent disfigurement or vision loss if the treatment doesn't work or they don't pursue it.

"I had to think about what's worse, the current situation or what might happen in the future. It felt like a gamble, but I had to trust my team, find a balance, go step by step and focus on what I could control." **— Veronica**

Finally, all the people we spoke with mentioned various impacts of dealing with the health system itself.

Some had significant delays to diagnosis or were misdiagnosed, so their symptoms were very active by the time they received appropriate care. All were frustrated by the limited range of treatment options and the need to "wait and see" how their symptoms progressed before knowing if they could access more proactive treatments.

Some mentioned appointment fatigue, especially from untrained or under-trained clinicians and endless or repeated tests and phone calls. All agreed a significant challenge is navigating a health system that functions in silos. They just want their health professionals to communicate smoothly with each other.

Q3: What do you see as the advantages of this proposed medicine, in particular for those with the medical condition and/or family and carers?

Points for individual consumers to consider

- *What are the specific positive impacts that you hope this treatment will have on your health condition? (for example, reducing pain)*
- *What impact would you like it to have on your quality of life? (for example, enabling you to return to work)*
- *If you have used this medicine what was your experience? What changed for you?*
- *Are there advantages in the way the medicine is delivered? (For example, where it is delivered (for example, home, GP, hospital), or how it is given (for example, tablets rather than injection))*

Please provide your comments.

We believe one of the biggest advantages of teprotumumab is having it available as a first-line treatment for people with active TED. Treating the underlying condition and calming the inflammatory response before it has time to cause permanent eye damage will be a game-changer for Australian TED patients in many ways, as it had been for those in the US.

Not only can it help many patients avoid the side effects of IVMP and oral immunotherapy medicines, it appears to have a lower risk-to-benefit ratio when compared with existing treatments. Equally as important, it can reduce their likelihood of needing corrective surgery, living with permanent eye disfigurement or developing secondary conditions.

"Access to a new form of TED treatment would undoubtedly be beneficial. As a TED patient with limited access to eye specialists and professional guidance, I believe introducing an alternative option would be a game-changer for those of us hesitant to undergo invasive surgery." **— Marion**

The fact that teprotumumab is a biologic agent allows clinicians to monitor specific diagnostic markers and optimise treatment regimes. Patients and clinicians will also have more treatment options to choose from so treatment plans can be even more tailored to individual needs.

Those benefits alone will likely make a very positive difference to their mental health, ability to work and overall quality of life.

Other benefits include the fact that it only involves eight infusions rather than 12. That means four fewer trips to an infusion centre, four fewer days that need time allocated for the appointment, four fewer days of travel expenses and four fewer days of stress. It also means a reduction in treatment costs for the government.

Q4: What do you see as the main disadvantages of this proposed medicine?

Points for individual consumers to consider

- *Are there disadvantages in how you can access the medicine, for example whether you meet the criteria, where it is delivered (for example, home, GP, hospital), or how it is given (for example, tablets rather than injection)?*
- *Have you heard of any side effects from this medicine? Do you consider these to be manageable?*
- *What side effects would stop you from taking this medicine?*
- *If you have used this medicine, what did you consider to be the disadvantages?*

Please provide your comments.

One potential disadvantage of teprotumumab relates to the lack of professional expertise in treating TED itself. Clinicians less experienced in treating TED may be less inclined to offer new treatments until they have more training and/or further research is done into the efficacy and safety of teprotumumab.

GHLF Australia intends to make a positive impact in addressing these unmet needs by creating a range of online resources for TED clinicians and patients.

Teprotumumab is delivered as an IV infusion so it will have the same access barriers as IVMP. That is, there are not enough people trained in such infusions and patients will still have to travel to infusion centres. Those problems are amplified for those living in regional, rural and remote areas of the country. In an ideal world, teprotumumab would be administered via subcutaneous injection as this would make it far more convenient and accessible for patients.

Eligibility will be limited to people with active moderate to severe TED as this is the cohort studied in the clinical trials. Those with milder symptoms or whose TED is in the stable chronic phase could potentially still benefit from teprotumumab, but more research needs to be done with these cohorts.

We believe more research needs to be done on the possible side effects of teprotumumab. These include muscle cramps or spasms, nausea, hair loss, diarrhoea, feeling tired, high blood sugar, hearing problems, taste changes, headache, dry skin, weight loss, nail problems and changes in menstruation.

More research is also needed to help us gain a better understanding of:

- The effect on TED symptoms in the long term after teprotumumab treatment is finished.
- The likelihood of interactions with medicines commonly used by TED patients.
- The effect of teprotumumab on comorbid conditions, including IBD, diabetes and other hormonal or autoimmune conditions.

For example, Marion told us she uses thyroxine to regulate her hormones after a thyroidectomy. She is hesitant about the effects teprotumumab might have on thyroxine and her hormone levels. She knows she would need to work very closely with her endocrinologist to manage such interactions.

Q5: Please provide any additional comments you would like the PBAC to consider.

Although outside the scope of the PBAC March 2025 meeting, we would like to acknowledge the issues caused by the inadequacy of care for people with rare conditions and people from regional, rural and remote communities.

All the TED patients we spoke with stressed that optimal management of TED needs a multidisciplinary team approach, including endocrinologists, ophthalmologists, optometrists, GPs, pharmacists, infusion nurses, ED departments, rheumatologists and surgeons.

Veronica noted that trying to coordinate her care with so many different health professionals across all levels of the system is incredibly stressful and tiring, even though she believes she has high levels of health and computer literacy. We agree with her that such challenges would be even worse for people from CALD backgrounds or with some forms of disability. She suggested a dedicated case manager for people with complex chronic conditions would help to address this.

The PBAC committee members may like to read our article ***Living with thyroid eye disease: Veronica's story*** at https://ghlf.org.au/thyroid-eye-disease/living-with-thyroid-eye-disease-veronicas-story.

Members can also watch Veronica's video reels ***Thyroid Eye Disease (TED): Awareness, Treatment & Patient Stories – YouTube*** at https://www.youtube.com/playlist?list=PLf0iG26PiXMscVTzRIekMQhS3kn7Ebilo.

Finally, members may like to visit our ***Thyroid Eye Disease Education and Advocacy Hub*** at https://ghlf.org.au/thyroideyedisease.

Q6: If you have any suggestions on ways to improve this survey please provide these.

We like this survey a lot because it is makes it accessible to the public, asks patient-centred questions and conveys a deep desire to understand lived experiences with a disease.

While we do not have suggestions to improve the survey, we would support the work of the PBAC or the Health Department in promoting the availability of the survey to the general public to encourage people to participate further in the process.