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Global Healthy Living Foundation Australia and CreakyJoints Australia

Submission to support the listing of tofacitinib (Xeljanz®) on the PBS for the treatment of juvenile idiopathic arthritis

For consideration at the March 2023 PBAC Meeting

These comments are submitted by [CreakyJoints Australia](#) and [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.

CreakyJoints Australia/GHLF Australia

CreakyJoints Australia provides information, daily living tips and resources for Australians with any form of arthritis and related conditions and we advocate on their behalf. Most of our members and online followers are adults but many have had arthritis since childhood. The older members of this cohort had fewer treatment options (such as gold injections, methotrexate and prednisolone) when they were young than children and adolescents have now. Therefore, they are more likely to have severely damaged joints, joint replacements and high levels of disability than those who began advanced treatment at an early age.

Over the years, we have heard many personal stories from this cohort about the pain, stigma and other challenges they faced growing up with arthritis. We believe the insights we have gained from them are very relevant for this submission as many of the challenges still exist today.

JIA diagnosis challenges

The [Australian and New Zealand Childhood Arthritis Risk factor Identification Study \(ANZ CLARITY\)](#) states that around 6000 Australian children have a form of childhood arthritis, yet JIA symptoms (especially in very young children) often go unrecognised due to a lack of community awareness of the condition. Many are unaware that children can even have arthritis, so parents, carers and even some health professionals dismiss early symptoms or attribute them to other causes such as falls, growing pains or viral conditions.

Children often have to endure extensive and prolonged pain before being diagnosed with JIA. They can also experience high levels of anxiety and frustration which can be compounded if adults don't believe them or if they are non-verbal. Some develop JIA so young they can't remember life any other way.

Delays to diagnosis can often be adversely affected by difficulties accessing rheumatologists, especially paediatric rheumatologists or even nurses or general practitioners with experience of JIA. Access difficulties can be caused by geography, finance, cultural or language barriers or by a lack of suitably qualified health professionals in the field of rheumatology.

JIA symptom, treatment and social challenges

Children with chronic conditions such as JIA face multiple challenges that healthy children their age are spared. For example, they can spend lots of time away from school, attending medical and allied health appointments or having extended periods in hospital — not to mention having countless blood tests and scans or periods of neutrophilia. As a result, they often spend a lot of time on their own instead of having fun with their friends.

Unfortunately, they also deal with social stigma from their peers because they are seen (or feel as though they are seen) as being different, attention-seeking or whinging. It is usually very helpful for children with JIA to meet other children with similar conditions but opportunities to do so are few and far between.

Children can become traumatised from so much pain, stress and treatment experiences (such as injections) which needs to be addressed early. Many children (especially young ones) rely on an adult to give them their oral or biologic medications which can be daunting for the child, the person caring for them and others watching the medication being administered. This can also make the child feel as though they have no control over the situation or their bodies.

It is not only the children with JIA that are affected by it. Having a child with arthritis in the family can also be difficult for parents, siblings and other close family and friends due to the amount of time and attention that child needs. Parents may also experience lost earnings due to their need to reduce their hours or stop work to be a carer for their child.

In the past, there were few government or community support services available to families. That is changing, especially with the introduction of the NDIS but there is still much to be done in this area.

Children with JIA risk developing comorbidities such as eye or lung problems. Years of prednisolone use can also lead to conditions such as osteoporosis or type 2 diabetes so families and treating doctors must continuously balance the benefits of treatment now against the risk of such outcomes in the future.

Sometimes, children with JIA go into remission for extended periods or even years. It can be shocking and hard for them to cope if/when their arthritis comes back.

As children with JIA reach 18, they have to transition from paediatric to adult health care systems. This can be daunting as they often need to see different doctors and take more control of their own medical needs. This comes at a time when they usually want to do all the things other teenagers do, like go out to parties or on dates, but struggle to do so. They often do what they can to hide their condition in order to feel more socially accepted.

Many of the adults in our community who had arthritis as a child needed joint replacements by the time they were early adults because most of the treatments they had access to did not slow the progression

of the disease. Some in this category have needed joint revision surgery in their 40s/50s as the artificial joints they originally had were not designed to last more than roughly 15 to 20 years. Many are now worried about needing further revisions in the future as they often have less natural bone left to support new artificial joints.

An issue that they faced, that isn't often spoken about, is the difficulty many had as young adults forming and maintaining close relationships, including sexual relationships. Some had issues with partners who did not understand their condition and treated them poorly. For example, some were told they were too needy or boring. Others already had significant joint damage and found sex very uncomfortable but didn't know what to do about it.

Some people had hip replacements in their early 20s and spoke of how this affected their sexual relationships and family planning dreams. Some women made the decision to not have children. The factors that influenced their decision included (but were not limited to) concerns about:

- The stress this would place on their body during pregnancy and childbirth.
- Possibly having to stop or change their arthritis treatments before and during pregnancy.
- Their ability to safely care for a child if their hands were too sore or weak to hold them or if they were too fatigued to manage the child's daily needs.
- Their potential to pass on the genetic predisposition for autoimmune conditions to their child.

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

CreakyJoints Australia/GHLF Australia

These days, children with JIA can still be underdiagnosed or misdiagnosed. However, those who are diagnosed correctly now have access to biologic medications to slow disease progression and manage symptoms better. This makes them far less likely than children of earlier generations to have severe joint damage in the long term.

The lack of paediatric rheumatologists, nurses and GPs with experience in paediatric rheumatology is still an issue for some families. This looks like it will still be the case in Australia for years to come.

Today's children have more treatment options available, including conventional synthetic disease-modifying antirheumatic drugs such as methotrexate and other oral therapies including prednisolone, anti-inflammatories and pain killers. Fortunately, they can now access the following biologic medications:

- Adalimumab (subcutaneous injection)
- Etanercept (subcutaneous injection)
- Tocilizumab (subcutaneous injection or IV infusion)

All advanced treatments for JIA currently involve needles. If the child has a needle phobia, their family has to make the difficult choice between forcing the child to have the treatment or not pursuing that option and missing out on the benefits the child would gain from being on a biologic.

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

CreakyJoints Australia/GHLF Australia

CreakyJoints Australia first became aware of the JAK inhibitor, tofacitinib, when it was in the process of being approved for use in Australia for adults with rheumatoid arthritis. Since then, we've seen more and more of our patient community using it, especially as it became available for psoriatic arthritis as well.

Like all medications, it works better for some people than for others. Generally speaking, though, the results and feedback we've seen have been overwhelmingly positive.

One of the biggest advantages JAK inhibitors have over biologics is that they are administered orally rather than via injection. For many, taking daily tablets is far more convenient than using biologics. They don't need to be refrigerated so that makes travelling easier as well.

As we understand it, JAK inhibitors are less likely than biologics to cause digestive side effects (such as nausea) as they do not pass through the digestive system. Some people get allergic reactions from biologics, especially around the injection site. As JAK inhibitors are taken orally, the risk of them causing an allergic reaction is much lower than for biologics.

We believe the introduction of tofacitinib will be a game-changer for today's children with JIA and their families as it will be the only advanced treatment available to them that doesn't involve needles.

Adults with arthritis in their hands often prefer oral medications than self-administered biologics as injecting devices can be uncomfortable or impossible to use with sore or swollen hands. Older children who are able to manage their own medications will likely find this a big advantage as well.

Having the option of trying a JAK inhibitor can help those children who have not had an adequate response to biologics.

Finally, JAK inhibitors cost less to produce than biologics so they are a more cost-effective option for the Government to supply through the PBS. While this does not affect the amount consumers pay at pharmacy level, it does mean the Government has more funds available to use for other medications or research.

Comments from our community

As a child of the age of about 5, I used to hide at The Children's Hospital when I saw people wearing white coats as I thought they were going to give me needles.

I think this would be a game changer for children with arthritis. They already have enough to deal with. If this medication was available it would help a lot.

Shirani Wright

I am a 39-year-old female and have had juvenile arthritis since I was 10.

I would have loved to be on something like Xeljanz when I was a child. A tablet instead of an injection would have been so helpful! Injections as an adult are fairly easy, but as a child they were a lot harder, and I used to dread each week, counting down to the big day.

Injections also made travel hard, having to bring Esky bags and swap the ice multiple times on the way. Traveling by plane was even harder. You couldn't go to the UK in one day, you had to break it up into a few flights. A tablet version of a biologic would have made traveling much easier.

I had Xeljanz as an adult for a couple of years and it was quite effective for me. I had to stop due to a side effect, but up until then I was happy with it!

Fiona Naughtin

“I was diagnosed with JIA 40 years ago when I was 12. My options for medications were very limited back then, so unfortunately I now live with irreversible musculoskeletal damage, with many years of complications due to ongoing prosthetic joint infections.

I have been on a number of biologics, which have helped to stop more damage occurring, and know from my fellow patient friends how effective they are in helping them live a relatively normal life. Having another option like Xeljanz available to children is so important, particularly in tablet form. Children need to be diagnosed earlier than they are and get onto medications such as this so they can lead a normal life.”

Naomi Creek

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

CreakyJoints Australia/GHLF Australia

All medications have side-effects and those that suppress the immune system can make people more at risk of developing serious infections than otherwise healthy people. However, we believe that risk is about the same for all advanced medications for autoimmune arthritis, regardless of delivery form.

JAK inhibitors are known to occasionally lead to side effects such as heart or lung issues in adults. Also, biologics have been on the market much longer than JAK inhibitors so there is still a lot we don't know about the long-term safety profiles for adults let alone for children.

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

CreakyJoints Australia/GHLF Australia

We have no further comments.

Q6: We are considering revising the consultation survey for future PBAC consultation rounds, along with providing additional guidance. Are there any suggestions you would like us to consider as part of this process?

CreakyJoints Australia/GHLF Australia

It would be helpful to have the option to provide input for more than one medicine in a single submission. This would be especially beneficial in cases where similar amendments are proposed for multiple medicines in one disease area.

Source

Murdoch Children's Research Institute: Australian and New Zealand Childhood Arthritis Risk Factor Identification Study (ANZ CLARITY). <https://www.mcri.edu.au/research/projects/clarity>