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**PBAC Submission – July 2019 Meeting**

**To request the removal of age restrictions from the existing listing for the treatment of RRMS to permit use of natalizumab in all ages.**

MS Research Australia is writing to support the removal of the age restriction of natalizumab on the Pharmaceutical Benefits Scheme (PBS), to permit use for people of all ages with relapsing remitting MS.

As the largest national not-for-profit organisation dedicated to funding MS discoveries and coordinating MS research in Australia, we are proud to advocate on behalf of people affected by this disease. One area of particular importance to MS Research Australia and the MS community, is the affordable availability of treatments that have been shown to be effective in clinical trials to reduce the impact of MS.

The removal of the age restriction of natalizumab on list of PBS supported treatments for MS is vital to maximising the availability of evidence based treatments for people with MS and provide affordable access for all those who need it, regardless of their age. The heterogeneous nature of MS means no single disease modifying treatment is likely to be effective for all cases of MS. Therefore, it is vital that there is an extensive arsenal of available treatments to allow optimal treatment for as many patients as possible. Suboptimal treatment can lead to MS relapses causing irreparable damage to the central nervous system leading to an increased burden on the healthcare system and a further reduction in the quality of life of patients and their families.

MS most commonly strikes during young adulthood, but up to 5% of diagnoses occur in children, including some as young as two years of age. Since clinical trials in children can be challenging, there is a lack of information about the safety and side effects of MS medications in children. However, in practice, children with MS are often treated "off-label" with adult MS medications, so approvals for affordable medications that could apply to children are welcome.

A number of observational studies have examined treatment of highly active MS in children. In one study of 144 children with highly active MS, it was found natalizumab performed equally or better than in adult patients [1]. In two other studies, one in 20 children and another in nine children demonstrated that natalizumab may be safe and effective against MS in paediatric patients with breakthrough disease who require a second line therapy [2,3]. An Italian registry study of people under 18 treated with natalizumab identified 101 patients and found that natalizumab was safe, well tolerated and very efficacious in the large majority of patients and supported the use of this medication in subjects with paediatric MS and an aggressive course [4].

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A recent large observational study that looked at overall treatment use in children with MS or clinically isolated syndrome (a precursor to MS), including natalizumab, with MS found no new side effects were seen in the children compared to the adult experience with these MS medications and the rates of known side effects were similar to those seen in adults. None of the children in the study developed progressive multifocal leukoencephalopathy (PML), a brain infection which can be a rare side-effect of some MS medications. It was slightly more common for children with MS to stop using the newer generation therapies than seen in the adult MS population, but the reasons for this were unclear [5].

As such, MS Research Australia supports the removal of the age restriction on natalizumab on the PBS to ensure affordable access for patients who require it. MS Research Australia supports any proven treatments that have been approved by the TGA that will reduce the frequency of disabling relapses and improve the quality of life of people with MS. This in turn will affect those around them – their family members and carers. MS Research Australia appreciates the opportunity to make this submission and applauds the Committee for seeking the views of patients and the wider community as part of the process of considering new MS treatments for inclusion on the PBS.

**References**

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