Multiple Sclerosis Research Australia
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PBAC Submission – July 2019 Meeting

For: New strength of fingolimod capsules for patients with RRMS who weigh 40kg or less, including paediatric patients.

MS Research Australia is writing to support the inclusion of a new strength of fingolimod capsules on the Pharmaceutical Benefits Scheme (PBS) for people with relapsing remitting MS who weigh 40kg or less, including those with paediatric 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 inclusion of the additional dosage of fingolimod 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 those under 40kg including children with MS. 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.

In the phase 3 PARADIGMS trial, 10 to 17 years of age with relapsing MS were given oral fingolimod at a dose of 0.5 mg per day or 0.25 mg per day for patients with a body weight of ≤40 kg or intramuscular interferon beta-1a at a dose of 30 µg per week for up to 2 years. In this trial fingolimod was associated with a lower rate of relapse and less accumulation of lesions on MRI over a 2-year period than interferon beta-1a but was associated with a higher rate of serious adverse events (Chitnis T et al., N Engl J Med 2018 379(11):1017-1027). Other data also suggests that fingolimod significantly reduces annualised relapse rates compared to placebo or interferon

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beta-1a in young adults (Gärtner J et al., Mult Scler J Exp Transl Clin 2018 4(2):2055217318778610) and that the magnitude of the treatment effect, in terms of the percentage reduction in the annualised relapse rate, may be inversely proportional to patient age, meaning younger adults may particularly benefit from treatment with fingolimod.

As such, MS Research Australia supports the inclusion of the new strength of fingolimod on the PBS to ensure affordable access for patients who require it. MS Research Australia supports any proven treatments 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.