P1778
The real-world effectiveness and safety of cyclophosphamide in progressive multiple sclerosis: A single-centre retrospective cohort study

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Introduction: Therapeutic options for people with progressive multiple sclerosis (PMS) remain limited, with currently approved disease-modifying treatments applicable to only a subset of this population. Cyclophosphamide (CYC) is often used off-label, yet real-world data on its safety and effectiveness remain scarce.
Objectives/Aims: This study evaluated CYC's long-term effectiveness and safety in a real-world cohort of people with PMS.
Methods: This single-centre, retrospective cohort study included all patients with primary (PPMS) or secondary PMS (SPMS) who received at least one treatment cycle of intravenous CYC at Noorderhart, Rehabilitation and MS Centre in Pelt, Belgium. A treatment cycle consisted of four to six CYC infusions. Follow-up included maintenance therapy following CYC treatment. Effectiveness was assessed using the expanded disability status scale and neurologist-reported clinical stability. Safety was evaluated based on adverse event reporting. Kaplan-Meier analysis was used to estimate progression-free survival over time.
Results: A total of 169 patients (median age 60 [IQR 53–68]; 93 female, 76 male; 45 PPMS, 124 SPMS) were included. Most patients (72.8%) received a single cycle of CYC treatment, while 23.1% underwent two cycles, and 4.1% received three cycles. Over a median follow-up of 7.6 years [range 1.3–14.3], 27.2% of patients remained stable or improved during the observation period. Clinical stability or improvement was observed in 78.7% (± 3.2 SD) of patients at one year, 55.1% (± 3.9 SD) at three years, and 26.2% (± 3.7 SD) at five years following initiation of the first treatment cycle. No significant differences in outcomes were observed based on MS subtype, prior treatment exposure, or number of CYC cycles. Adverse events occurred in 48.5% of patients, most frequently nausea (16.0%), fatigue (15.4%), and urinary tract infections (12.4%).
Conclusion: CYC was associated with clinical stability or improvement in a substantial proportion of PMS patients in a real-world setting. The treatment was generally well tolerated, with a manageable safety profile. These findings support the potential utility of CYC as a therapeutic option for people with PMS and underscore the need for further prospective studies.

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