Abstracts

Articles appearing in the September 2017 issue

Predicting long-term disability outcomes in patients with MS treated with teriflunomide in TEMSO

Objective To predict long-term disability outcomes in TEMSO core (NCT00134563) and extension (NCT00803049) studies in patients with relapsing forms of multiple sclerosis treated with teriflunomide.

Methods A post hoc analysis was conducted in a subgroup of patients who received teriflunomide in the core study, had MRI and clinical relapse assessments at months 12 (n = 552) and 18, and entered the extension. Patients were allocated risk scores for disability worsening (DW) after 1 year of teriflunomide treatment—0 = low risk, 1 = intermediate risk, and 2–3 = high risk—based on the occurrence of relapses (0 to ≥2) or active (new and enlarging) T2-weighted (T2W) lesions (≤3 or >3) after the 1-year MRI. Patients in the intermediate-risk group were reclassified as responders or nonresponders (low or high risk) according to relapses and T2W lesions on the 18-month MRI. Long-term risk (7 years) of DW was assessed by Kaplan-Meier survival curves.

Results In patients with a score of 2–3, the risk of 12-week–confirmed DW over 7 years was significantly higher vs those with a score of 0 (hazard ratio [HR] 1.96, $p = 0.0044$). Patients reclassified as high risk at month 18 (18.6%) had a significantly higher risk of DW vs those in the low-risk group (81.4%; HR 1.92; $p = 0.0004$).

Conclusions Over 80% of patients receiving teriflunomide were classified as low risk (responders) and had a significantly lower risk of DW than those at increased risk (nonresponders) over 7 years of follow-up in TEMSO. Close monitoring of relapses and active T2W lesions after short-term teriflunomide treatment predicts a differential rate of subsequent DW in the long term.

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High-dose cyclophosphamide without stem cell rescue in immune-mediated necrotizing myopathies

Objective To describe the experience managing treatment-refractory immune-mediated necrotizing myopathies (IMNM) with high-dose cyclophosphamide (HiCy) therapy.

Methods Five patients with severe refractory IMNM who were treated with HiCy without stem cell rescue were identified. Their medical records were reviewed to assess demographic, clinical, and histologic characteristics as well as response to therapy.

Results Three patients with anti–signal recognition particle (SRP) and 2 patients with anti-HMG-CoA reductase autoantibodies were included. The mean follow-up time after HiCy therapy was 37 ± 28 months. Two patients demonstrated substantial response, evidenced by improved muscle strength and decreased muscle enzymes after HiCy therapy; both of these patients were anti-SRP positive. Four patients experienced febrile neutropenia after HiCy therapy, one of which required a prolonged intensive care unit stay for infectious complications, from which they eventually recovered.

Conclusions These data suggest that HiCy therapy without stem cell rescue may be considered as an alternative for the treatment of refractory IMNM.

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