

A randomized controlled trial on corticosteroid therapy for HTLV-1-associated myelopathy/tropical spastic paraparesis in Japan (HAMLET-P trial)

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Background/Purpose:

Corticosteroids are most commonly used to treat HTLV-1-associated myelopathy/tropical spastic paraparesis (HAM/TSP). However, their clinical efficacy has not been tested in randomized clinical trials.

Approach:

This multicenter, randomized controlled trial included 8 and 30 HAM/TSP patients with rapidly and slowly progressing walking disability, respectively. Rapid progressors were assigned (1:1) to receive or not receive a 3-day course of intravenous methylprednisolone in addition to oral prednisolone therapy. Meanwhile, slow progressors were assigned (1:1) to receive oral prednisolone or placebo. The primary outcomes were a composite of ≥ 1 -grade improvement in the Osame Motor Disability Score (OMDS) or $\geq 30\%$ improvement in the 10-meter walking time (10mWT) at week-2 for rapid progressors and changes from baseline in 10mWT at week-24 for slow progressors.

Outcomes/Impact:

In the rapid progressor trial, all four patients receiving intravenous methylprednisolone but none of those receiving oral prednisolone alone experienced improvement in OMDS. One patient in each treatment arm experienced improvement in 10mWT. Therefore, four and one patients with and without intravenous methylprednisolone achieved the primary outcome, respectively ($p = 0.14$). In slow progressor trial, the median changes from baseline in 10mWT were -13.8% (95% CI: -20.1—-7.1; $p < 0.001$) and -6.0% (95% CI: -12.8—1.3; $p = 0.10$) with prednisolone and placebo, respectively (p for between-group difference = 0.12).

Innovation and Significance:

Whereas statistical significance was not reached for the primary efficacy endpoints due to the small sample size, the data overall indicate the benefit of corticosteroid therapy in patients with HAM/TSP.

Disclosure of Interest Statement:

None.