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Abstract Title: #P04.269 - Pilot study of monthly pulse adrenocorticotropic hormone (ACTH) or methylprednisolone as an add-on therapy to beta-interferons for long-term treatment of multiple sclerosis

Press Release Title: Can Hormone Help Treat Multiple Sclerosis Long-Term?

Objective: This single-center, examiner-blinded pilot study evaluated the efficacy and safety of pulse adrenocorticotropic hormone (ACTH) treatment added to beta-interferon in breakthrough multiple sclerosis (MS) compared with pulse methylprednisolone (MP).

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Background: ACTH may have immune-modulating mechanisms beyond steroidogenesis that are relevant to the MS disease course. Although ACTH gel is approved to treat MS relapses, its use as pulse therapy is less known.

Designs/Methods: MS patients receiving ongoing beta-interferon treatment were eligible if they had Expanded Disability Status Scale (EDSS) scores of 3.0-6.5 and ≥1 relapse or new T2 or Gadolinium-enhanced lesion within the previous year. Patients were randomly assigned to open-label ACTH (80 units IM once/day x 3 consecutive days) or MP (1 gram IV x 1 dose) monthly for 12 months, with assessments every 3 months for 15 months. Outcomes included relapse rate (primary), EDSS, MS Functional Composite, and MS Quality of Life.

Results: The study included 23 patients (ACTH: n=12, mean±SD EDSS 4.6±1.5; MP: n=11, mean±SD EDSS 4.6±1.3). Over 15 months, the cumulative number of relapses/patient was 0.08 (95% CI: 0.01-0.54) with ACTH and 0.80 (95% CI: 0.36-1.75) with MP (risk ratio [MP vs ACTH]: 9.56 [95% CI: 1.23-74.6; P=0.03]). The cumulative number of psychiatric episodes/patient was greater with MP (0.55 [95% CI: 0.12-2.6]) than with ACTH (0 episodes; P<0.0001). The urinary tract infection cumulative incidence rate with MP was 0.65/patient and with ACTH was 0.16/patient (P=0.25). Mixed effect modeling showed no difference between groups in trajectory slopes of EDSS over time, but significantly stronger (P=0.03) improvement in Mental Health Inventory for ACTH (slope: 0.95/month [P=0.02]) compared with MP (slope: 0.29/month [P=0.32]).

Conclusions: These data suggest a potential benefit of ACTH pulse therapy in breakthrough MS with more favorable relapse and psychiatric side effect profiles. Further studies, including randomized controlled trials are needed to validate these findings.
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