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High doses of biotin in chronic progressive multiple sclerosis: a pilot study. Sedel F<sup>1</sup>, Papeix C<sup>2</sup>, Bellanger A<sup>3</sup>, Touitou V<sup>4</sup>, Lebrun-Frenay C<sup>5</sup>, Galanaud D<sup>6</sup>, Gout O<sup>7</sup>, Lyon-Caen O<sup>2</sup>, Tourbah A<sup>8</sup>.

#### **Abstract**

**BACKGROUND**: No drug has been found to have any impact on progressive multiple sclerosis (MS). Biotin is a vitamin acting as a coenzyme for carboxylases involved in key steps of energy metabolism and fatty acids synthesis. Among others, biotin activates acetylCoA carboxylase, a potentially rate-limiting enzyme in myelin synthesis.

**OBJECTIVES**: The aim of this pilot study is to assess the clinical efficacy and safety of high doses of biotin in patients suffering from progressive MS.

STUDY DESIGN: Uncontrolled, non-blinded proof of concept study

**METHODS**: 23 consecutive patients with primary and secondary progressive MS originated from three different French MS reference centers were treated with high doses of biotin (100-300mg/day) from 2 to 36 months (mean=9.2 months). Judgement criteria varied according to clinical presentations and included quantitative and qualitative measures.

**RESULTS:** In four patients with prominent visual impairment related to optic nerve injury, visual acuity improved significantly. Visual evoked potentials in two patients exhibited progressive reappearance of P100 waves, with normalization of latencies in one case. Proton magnetic resonance spectroscopy (H-MRS) in one case showed a progressive normalization of the Choline/Creatine ratio. One patient with left homonymous hemianopia kept on improving from 2 to 16 months following treatment's onset. Sixteen patients out of 18 (89%) with prominent spinal cord involvement were considered as improved as confirmed by blinded review of videotaped clinical examination in 9 cases. In all cases improvement was delayed from 2 to 8 months following treatment's onset.

**CONCLUSIONS:** These preliminary data suggest that high doses of biotin might have an impact on disability and progression in progressive MS. Two double-blind placebo-controlled trials are on going.

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KEYWORDS: Biotin; Multiple sclerosis; Optic neuritis; Progressive; Visual evoked potentials

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