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A pilot study on the use of prednisolone-encapsulated liposomes for the treatment of moderate-to-severe Graves' orbitopathy with reduced systemic steroid exposure.

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PURPOSE: To demonstrate that long-circulating PEGylated liposomal prednisolone is a safe and effective therapy in patients with active moderate-to-severe Graves' orbitopathy.

METHODS: Open-label, proof-of-concept, multicentre pilot study. Ten patients with moderate-to-severe Graves's orbitopathy, who were euthyroid for at least three months. Long-circulating PEGylated liposomal prednisolone 150 mg was administered intravenously twice, with 2-week interval. Total follow-up was 12 months, with visits at baseline, week 2, 6, 13, 26 and 52. Physical, laboratory and ophthalmological examinations were performed. Response to treatment was defined as a reduction in Clinical Activity Score by ≥2 points; palpebral aperture by ≥3 mm; soft tissue signs by ≥2 grades; exophthalmos by ≥2 mm; and motility by >8 degrees or improvement in diplopia score. A response was sustained when equally observed at weeks 6 and 13.

RESULTS: One patient achieved a sustained response according to the predetermined definition. All patients showed a decrease in Clinical Activity Score after one infusion, with a mean decrease of two points. The Clinical Activity Score was ≤1 at week 52 for all patients. Improvement was also observed in the soft tissue signs. Most of the adverse events were mild and of a transient nature. Two patients required further treatment with intravenous methylprednisolone.

CONCLUSION: This pilot study showed a positive effect of long-circulating PEGylated liposomal prednisolone on the Clinical Activity Score in patients with moderate-to-severe Graves's orbitopathy, resulting in fewer hospital visits and possibly less glucocorticoid-related side-effects.