Further developments with antisense treatment for myasthenia gravis

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Abstract:

We present further developments in the study of the antisense oligonucleotide EN101. Ongoing in vitro and in vivo studies demonstrate that EN101 is a TLR9-specific ligand that can suppress pro-inflammatory functions and shift nuclear factor kappa B (NF-κB) from the pro-inflammatory canonical pathway to the anti-inflammatory alternative pathway, which results in decreases acetylcholinesterase (AChE) activity. Preliminary results of a double-blinded phase II cross-over study compared 10, 20, and 40 mg EN101 administered to patients with myasthenia gravis. Patients were randomly assigned to one of three treatment groups in weeks 1, 3, and 5 and received their pretreatment dose of pyridostigmine in weeks 2 and 4. Thus far, all doses show a decrease in QMG scores, with a greater response to higher doses.

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