METHODS

Patient Screening Criteria and Study Design

Patient Disposition and Demographics

RESULTS

Efficacy: Primary endpoint UHDRS-TMS was not met but secondary endpoint % change in caudate volume loss was met

Efficacy: Exploratory Endpoints: no treatment effects in rater-dependent outcome measures, effects shown for Q-Motor

References


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CONCLUSIONS

In this placebo-controlled study of patients with early HD, laquinimod treatment showed no evidence of improved rater-dependent clinical outcomes, whereas laquinimod treatment demonstrated a statistically significant reduction in volume loss in caudate for the 1 mg dose, and nominally significant reduction in volume loss in caudate for the 0.5 mg dose and in whole brain and white matter for both doses.

Q-Motor measures suggest a nominal effect of laquinimod on motor coordination congruent with less decline in motor signs based on progression signals known from studies such as TRACK-HD [4] and PRIDE-HD [5].

Jointly, the treatment effects on MRI brain volume and Q-Motor measures suggest a central effect of laquinimod in LEGATO-HD of unknown clinical significance.

The lack of clinical effect could be due to possible confounders such as the relatively short treatment period of 52 weeks and rater biases in clinical scales.

Analysis of MRS regarding neuronal integrity and astrocytosis and PET regarding neuroinflammation could further elucidate the nature of changes observed in the brain.

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