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Long term daily nomlabofusp administration of nomlabofusp in patients with Friedreich's ataxia: Interim observations from an ongoing open label study

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Background and Objectives

Friedreich's ataxia (FRDA) is a neurodegenerative disease resulting from a deficiency in frataxin (FXN). Nomlabofusp is a recombinant fusion protein being developed to increase FXN levels in adults and children with FRDA. Interim data from an ongoing open label study were reviewed to assess changes in FXN concentrations, clinical measures, and safety in patients with FRDA receiving long term nomlabofusp treatment.

Methods

Enrolled subjects received daily administration of nomlabofusp. FXN concentrations and clinical outcomes (e.g. mFARS, FARS_ADL, 9HPT) were measured at baseline and every three months. Safety was assessed monthly either during in-person or remote study visits.

Results

Data from 41 subjects who received at least 1 dose of nomlabofusp were reviewed. Subjects received nomlabofusp treatment for up to 2 years. Mean age at screening and symptom onset were 29.0 and 12.4 years, respectively. Mean baseline results for mFARS, FARS_ADL, and 9HPT were 55.0 points, 17.1 points, and 93.8 seconds, respectively. Median baseline buccal and skin FXN levels were 18-20% of healthy controls. FXN levels increased through Month 6 and then plateaued. Skin FXN concentrations in almost all subjects at Months 6 and 12 were within the range expected in heterozygous carriers without phenotypic expression of disease. Mean clinical outcome values were at or below baseline at all timepoints through 2 years. Mild-moderate injection site reactions were the most commonly reported adverse events. Anaphylaxis was observed in some subjects; almost all cases occurred in subjects exposed to nomlabofusp during a prior study. Adverse event reports decreased after the first 3 months of treatment.

Discussion and Conclusion

In patients with FRDA, daily nomlabofusp resulted in increased FXN levels with concomitant improvements in clinical outcomes that were consistent across measures and sustained out to 2 years. There is a risk of anaphylaxis during early treatment, particularly in patients with prior nomlabofusp exposure. Long term treatment with nomlabofusp appeared to be well tolerated.