Interim results from an open-label, single-center, hybrid-virtual 12-month trial of a Lunasin regimen for patients with Amyotrophic Lateral Sclerosis (BW28)

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Background

Lunasin, a soy peptide that may alter histone acetylation, has been associated with an ALS reversal. We recently finished enrollment in a patient-centric pilot trial of Lunasin. The hypotheses we are testing were described at last year’s meeting. Here we present our interim results as of 11/1/16.

Methods

This a widely inclusive, largely virtual, open label trial of a supplement regimen we refer to as Lunasin. Participants make 3 visits to Duke (Screening/Baseline, Month 1 and Month 12). During these they are taught to measure ALSFRS-R and weight, to use the PatientsLikeMe website (PLM), and we draw blood to assess pharmacodynamic biomarkers. At months 2-11, participants make “virtual visits” measuring their own ALSFRS-R score and weight, and logging onto PLM to record it as well as perceived efficacy, compliance, adverse events and changes in concomitant medications. Participants are compared to matched historical controls from PLM, generated using pre-treatment ALSFRS-R progression rates, as previously described (Nature Biotech 2011;29:411).

Enrollment & Retention

We enrolled 50 participants in 5.5 months for an enrollment rate of 9.1 participants per site per month. Participants are primarily white (94%) males (58%) and have a mean age of 60 and disease duration of 4 years. Eleven participants have dropped out of the study, 4 due to adverse events, 4 due to study burdens or perceived lack of efficacy, 3 due to deaths (assessed as unrelated to study).

Adherence

Participant ratings of treatment adherence. Coordinator-obtained measures of compliance will be reported in late 2017 after study completion.

Data Density

Number of participants reaching various time points, and the percentage that are compliant with outcome measure entry.

Safety & Tolerability

SAE and AE thus far (all unexpected).

Participant ratings of side effect severity this far.

ALSFRS Agreement

Agreement between coordinator and participant-obtained ALSFRS-R scores at month 1. Another comparison of scores will be made at 12 months.

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We thank the many ALS patients and families who contributed funds to support this study, especially the Larry Vance Hughes ALS Foundation. We thank Reliv for donating the Lunasin products.

Perceived Burdens and Efficacy

Participant ratings of burdens (left) and efficacy (right) this far. Additional measures will be obtained at the end of the trial in late 2017.