Zoom link:
LIVE POSTER SESSION
9/30 5:30p EDT

Advancing NurOwn® for ALS: Phase 3 Clinical Trial Design

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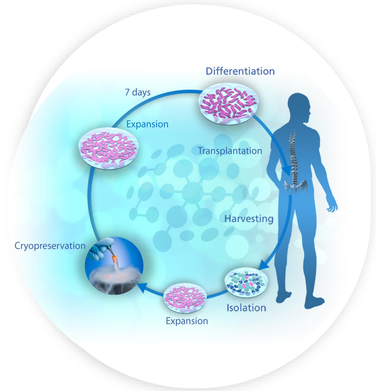
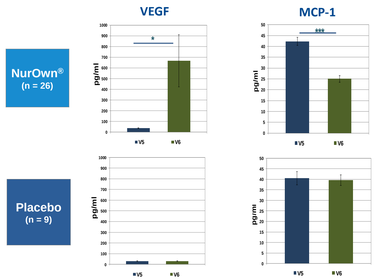
NurOwn Autologous Stem Cell Therapy[1]



- Single bone marrow harvest creates several years of therapy with patient's own cells
- No animal proteins, antibiotics, genetic modifications, viral vectors used in manufacturing process
- Reproducible functional effects observed in vitro and in vivo[2-4]

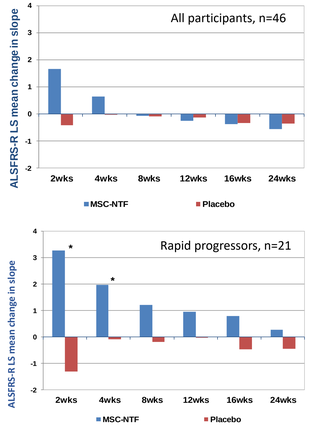
NurOwn treatment results in changes in CSF biomarker levels[5]

Single NurOwn treatment shows statistically significant changes in biomarkers and inflammatory markers measured in participant CSF as compared to placebo.



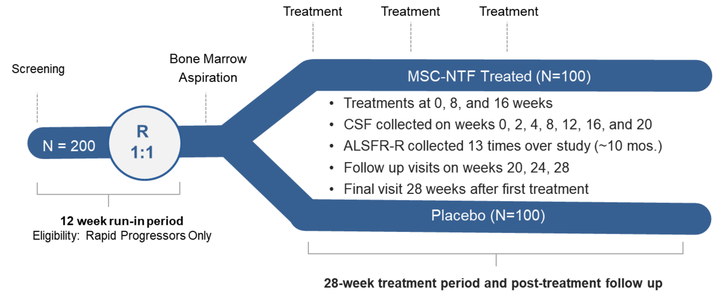
NurOwn treatment shows preliminary signs of clinical efficacy[5]

Single NurOwn treatment slowed the rate of disease progression by improving the ALSFRS-R LS mean change in slope compared to placebo.



NurOwn ALS Phase 3 Trial Design

- Placebo-controlled, randomized, double-blind Phase 3 trial
- Trial fully enrolled, dosing complete
- Topline data expected 4Q 2020



Primary Endpoint

Responder analysis change in the rate of decline in ALSFRS-R over 28 wks

- Allows for longitudinal view into disease progression, applies a threshold to define clinically meaningful changes, statistical model takes into account important covariates known to influence progression

Secondary Endpoints

- Safety
- Percentage of participants with disease progression halted or improved
- ALSFRS-R change from baseline
- Combined analysis of Function and Survival
- Slow vital capacity
- Tracheostomy-free and overall survival
- CSF/Biomarkers

ALS Functional Scale Analyses

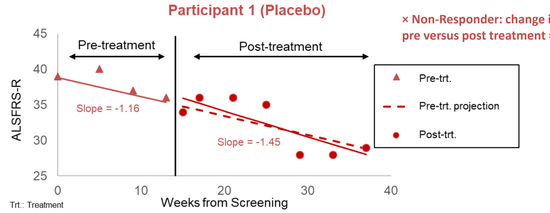
Analyses of slopes:

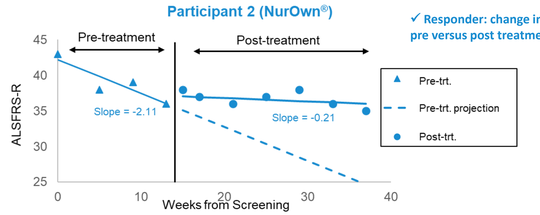
- Each participant's ALSFRS-R data fit with linear regression model, providing estimate of disease progression over time
- Responder = participant with a ≥1.25 points/month improvement in ALSFRS-R post-treatment slope compared to pre-treatment slope

Analyses of change in ALSFRS-R scores over time:

- Individual change scores over time calculated over treatment period and combined to form average treatment change over time
- Average treatment change scores analyzed using a longitudinal model, allowing comparison of magnitude of change between treatments

Illustration of Responder Analysis





Conclusions

NurOwn Phase 3 trial is rigorous, adequately powered, and designed to detect clinically meaningful treatment effect of autologous stem cell therapy in ALS patients. Primary and secondary endpoints will generate relevant evidence to assess the safety and efficacy of NurOwn in ALS.

Contact

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