

ALS ACCELERATING TREATMENTS AND IMPROVING QUALITY OF LIFE

- Two things are critically in need of improvement from the perspective of the patient community:
 - A quicker and more efficient clinical trial process that produces quicker results and greater certainty in evaluating drug/treatment efficacy.
 - An infrastructure that combines the results of all the various observational studies and is available to all researchers, physicians, scientists and drug/treatment developers for use in filling disease knowledge gaps so that when a patient contributes data, they know that it will produce actionable results that change the disease state and not just interesting journal articles

It is possible to tackle both improvement opportunities at the same time

ALS: Accelerating Treatments and Improving Quality of Life

The desire to contribute v. be observed in the patient community with respect to the clinic experience,

Importance of the NINDS Coordinating Center RFP and AMP-ALS and seeing that these efforts are fully funded.

Developing the infrastructure so that anyone diagnosed can immediately begin to contribute

Three Critical Knowledge Gaps That Can Be Filled

- The need for diagnostic biomarkers and biomarkers of disease progression and survival that can serve as clinical trial surrogate endpoints
- Understanding the genetic and environmental risk factors defining so-called sporadic ALS to better understand ALS subgroups – a true Genome Wide Association Study
- Developing more reliable disease progression predictive algorithms for use in clinical trial design

Potential Benefits of Filling These Knowledge Gaps

- Diminishing disease heterogeneity effects in assessing clinical trial results. Recruiting from the heart of the bell curve and eventually having a patient-by-patient control using predictive algorithm results.
- Supplementing ALSFRS-R as a single trial endpoint in order to reduce the effect of its drawbacks (e.g. floor effect; subjectivity; necessary trial length to see a result).
- Using the GWAS data to narrow drug targeting, quicken drug development and streamline clinical trial design in the so-called sporadic ALS population (90% of patients) – BIOGEN Biib005 example

Key Recommendations

- 1. A single database with broad clinic network participation for recording periodic clinic observations that integrates multiple types of data (genetic testing results, periodic biomarker tracking through biofluid collection, electronic health records and changes in medications and medical interventions etc.) and is broadly searchable and sufficiently powered with participants so reliable statistical correlations can be discerned.
- 2. Follow participating patients throughout the entire disease process even using home (visiting nurse, para-med services etc.) and telehealth capabilities for those no longer attending clinic.