

# Development and Adaptation of the ALSFRS-R for Remote Use

- Amyotrophic lateral sclerosis (ALS) is a progressive, neurodegenerative disorder
- In people living with ALS, disease severity and level of function are commonly measured in the clinic using a standardized, 12-item questionnaire known as the Amyotrophic Lateral Sclerosis Functional Rating Scale-Revised (ALSFRS-R) (Cedarbaum, 1999)
- RTI International is a nonprofit research institute based in North Carolina collaborating with the Food and Drug Administration (FDA) on a study to develop remote administration modalities for the ALSFRS-R
  - Decrease burden on patients
  - Improve clinical trial access and efficiency
- A Patient Committee (PC) has been developed to inform the project based on lived experiences of ALS, consisting of:
  - 3 persons living with ALS
  - 3 care partners of persons living with ALS
  - 1 neurologist
  - 1 measurement expert
- Funds for this contract were appropriated by Congress to the National Institutes of Health and the FDA for the Accelerating Access to Critical Therapies (ACT) for ALS Act

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This contract was funded by **FDA's Rare Neurodegenerative Disease Grant Program**. The grant program was created under the Accelerating Access to Critical Therapies (ACT) for ALS Act. Grants or contracts may be awarded to public and private entities to cover the costs of research and development of interventions intended to prevent, diagnose, mitigate, treat, or cure ALS and other rare neurodegenerative diseases in adults and children.

**RTI International** is an independent, not for profit research institute dedicated to improving the human condition.

# Our PC Collaborators

Persons with ALS	Care partners of persons with ALS	Neurologist	Psychometrician
<p>Albert Koo <i>Malaysia</i></p> <p>Angelique van der Lit <i>The Netherlands</i></p> <p>Bruce Virgo <i>Scotland</i></p>	<p>Ashley Lee <i>USA</i></p> <p>Wendy Wilson <i>USA</i></p> <p>Jessica Mabe <i>Colombia</i></p>	<p>Terry Heiman-Patterson <i>Lewis Katz School of Medicine at Temple University, USA</i></p>	<p>Lori McLeod <i>USA</i></p>

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## Research Objectives

- Identify facilitators and barriers for the collection of ALSFRS-R assessments to guide selection of remote modalities
- Adapt and evaluate remote-use modes of the ALSFRS-R tool to support regulatory decision-making and potential labeling claims
- Create standardized, remote access versions of the tool that will help facilitate decentralized, patient-centric trials that would reduce patient burden
- Commitment to recruit a diverse group of participants with regard to sex, gender, race, ethnicity, age, educational level, socioeconomic background, ALS severity scores, and geographical diversity (i.e., urban, suburban, or rural)
- Include testing of two-remote versions: phone and video, to acknowledge a widening a digital divide
- Disseminate results

## PC Selected Key Areas of Project Significance

### **Accessibility Challenges:**

- To care
  - Deserts of care
- To clinical trials and other research opportunities
  - Will new ALSFRS-R modalities lead to other aspects of care that could be provided at home? Such as other remote assessments, for example, spirometry...

## PC Selected Key Areas of Project Significance

### **Standard of care:**

- Recognizing differences among standards of care; could development of these ALSFRS-R remote modalities have an influence?
- Across regions
  - Member countries (PC members are spread across Asia, Europe, and North and South America) influence use of the ALSFRS-R

## PC Selected Key Areas of Project Significance

### Standardization:

- Implementing best practices for use of the ALSFRS-R *within* our research and then as an *outcome* of our research
  - ALSFRS-R modalities
    - Manuals/training tools
    - Appropriate cross-cultural adaptation of the manuals in the future
- Potential needs for feasibility/comparability studies in other countries or regions
  - Ability to use Zoom or similar web-based platform
  - Availability of internet access and practicalities of global implementation of remote assessments
  - Importance of a low-tech option

## PC Selected Key Areas of Project Significance

### Project framework:

- Incorporation of the PC to guide the research effort
  - *“Nothing about us, without us.”*
- Opportunity to share the lived experience of ALS to assist the study’s mission
  - *“Truth telling” and supporting of each other while having a shared agenda*
- Importance of using this work to demonstrate strong collaboration of regulators partnering with a patient group