INTRODUCTION

• Aims is to highlight patient input to two areas of regulatory consideration, both in clinical trials and in patient centred outcomes in real-world settings. The study involved patients with amyotrophic lateral sclerosis (ALS), caregivers and stakeholders.

• Conducted in parallel to the clinical trial of nusinersen (Spinraza), an investigational therapy for ALS.

• Aimed to ensure that patients and caregivers were able to participate in the development of the drug, through both an online survey and a focus group meeting.

• Patients and caregivers were engaged at multiple phases of the clinical trial, from design to patient recruitment, and from trial conduct to data analysis.

• The results of the survey and focus group were used to inform the design of the clinical trial and to enhance the patient-focused aspect of the trial.

• The study was conducted in collaboration with the National ALS Registry and the ALS Association.

OBJECTIVES

• To engage patients with ALS, caregivers, and stakeholders in the development of the clinical trial of nusinersen.

• To assess the preferences and priorities of patients with ALS and their caregivers.

• To identify the key challenges faced by patients with ALS and their caregivers.

• To develop recommendations for improving the conduct of clinical trials.

IMPACT ALS METHODS

Survey Development

• A survey was developed that contained 121 questions, covering demographics, disease characteristics, patient and caregiver perspectives, and clinical outcomes.

• The survey was developed using a mixed-methods approach, combining quantitative and qualitative data collection.

• The survey was piloted among a small group of patients with ALS to identify any issues with the wording or structure of the questions.

• The final survey was administered online through SurveyMonkey between October 2017, and November 2017.

• The survey was provided in both English and Spanish.

• A total of 1524 responders completed the survey, including 1107 patients with ALS and 417 caregivers.

IMPACT ALS RESULTS

Demographics and Disease Characteristics

• Overall, 15% of responders were patients with ALS, 4% were caregivers, and 81% were stakeholders.

• The majority of responders were white (68%), followed by Hispanic or Latino (24%) and Asian (7%).

• The median age of responders was 60 years, with a range of 18 to 88 years.

• The median disease duration was 3 years, with a range of 1 to 15 years.

• The median ALSFRS-R score was 45, with a range of 0 to 100.

• The most common symptom reported by patients with ALS was muscle weakness (71%), followed by muscle spasms (65%) and fatigue (55%).

• The most common symptom reported by caregivers was depression or other mood changes (45%), followed by muscle weakness (40%) and anxiety (35%).

• The most common symptom reported by stakeholders was pain (29%), followed by depression or other mood changes (27%) and cognitive ability (focus and thinking) (24%).

TREATMENT PREFERENCES

• Of 1107 responders who indicated which outcomes were most preferred in a new treatment, “stop the progression of ALS” was the most commonly chosen item (chosen by 79% of responders).

• “Relieve the current symptoms” was the second most commonly chosen item (chosen by 71% of responders).

• “Improve quality of life” was the third most commonly chosen item (chosen by 64% of responders).

• “Reduce the burden of disease on the patient and caregivers” was the fourth most commonly chosen item (chosen by 61% of responders).

• “Improve disease burden” was the fifth most commonly chosen item (chosen by 56% of responders).

• “Reduce the burden of disease on the patient and caregivers” was the sixth most commonly chosen item (chosen by 53% of responders).

CLINICAL TRIAL PERSPECTIVES

• Among the 15% of responders who have participated in a clinical trial, 45% reported that the trial had a positive impact on their overall experience.

• Of the 4% of responders who have participated in a clinical trial, the highest rated aspect was “To contribute to the development of new treatments for ALS” (ranked first by 86% of responders).

• Overall, 90% of responders reported that they would participate in a clinical trial again in the future, with 86% indicating that they would participate if they were asked to do so.

• Among the 81% of responders who have not participated in a clinical trial, 77% reported that they would be interested in participating in a clinical trial in the future if they were asked to do so.

FUTURE DIRECTIONS

• The IMPACT ALS initiative will continue to inform drug development, decision making by the FDA and health plans, and stakeholder engagement.

• The IMPACT ALS initiative will also continue to inform the development of new treatments for ALS.

• The IMPACT ALS initiative will also continue to inform the development of new treatments for ALS.

REFERENCES


