Concept Elicitation Interviews to Refine a Conceptual Model of the Patient Experience of Amyotrophic Lateral Sclerosis (ALS)

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Background

- Amyotrophic lateral sclerosis (ALS) is a rare, incurable disease that affects motor neurons and causes progressive muscle weakness, disability, and eventually death.[1, 2]
- A previously conducted targeted review on ALS patient experiences carried out by Sanofi, identified core concepts that led to the development of a disease-specific conceptual model (CM).[3]

Objective

- To explore the signs, symptoms, and impacts of ALS by conducting qualitative interviews with people living with ALS and refine the CM for ALS.

Methods

- This study was a cross-sectional, non-interventional, qualitative study comprising of concept elicitation interviews of adult participants with a clinical diagnosis of ALS.
- Interviews were conducted by trained interviewers using a semi-structured interview guide, allowing for flexibility in interview schedules to cope with speech impairment (dysarthria).
- Interview transcripts were analysed by directed content methods using ATLAS.ti software version 9 by trained researchers.
- Data collection and analysis were completed when conceptual saturation was achieved.

Results

- Fifteen adult participants were interviewed.
  - The mean (standard deviation) age of participants was 53.5 (10.11) years, and the majority were females (67%) (**Figure 1**).
- Twenty-two signs/symptoms were reported by participants, mostly spontaneously (**Figure 2**).
- The most frequently reported signs/symptoms of ALS were physical weakness (n = 12/15, 80.0%), changes in speech/ difficulty speaking (n = 11/15, 73.3%), respiratory/breathing issues (n = 7/15, 46.7%), fatigue/tiredness (n = 7/15, 46.7%), and decrease in fine and gross motor control (n = 7/15, 46.7%).

Figure 1. Demographic Characteristics

Patients, N = 15

Age, years

Mean (standard deviation): 53.5 (10.11)

Sex, n (%)

Male: 5 (33.3)
Female: 10 (67)

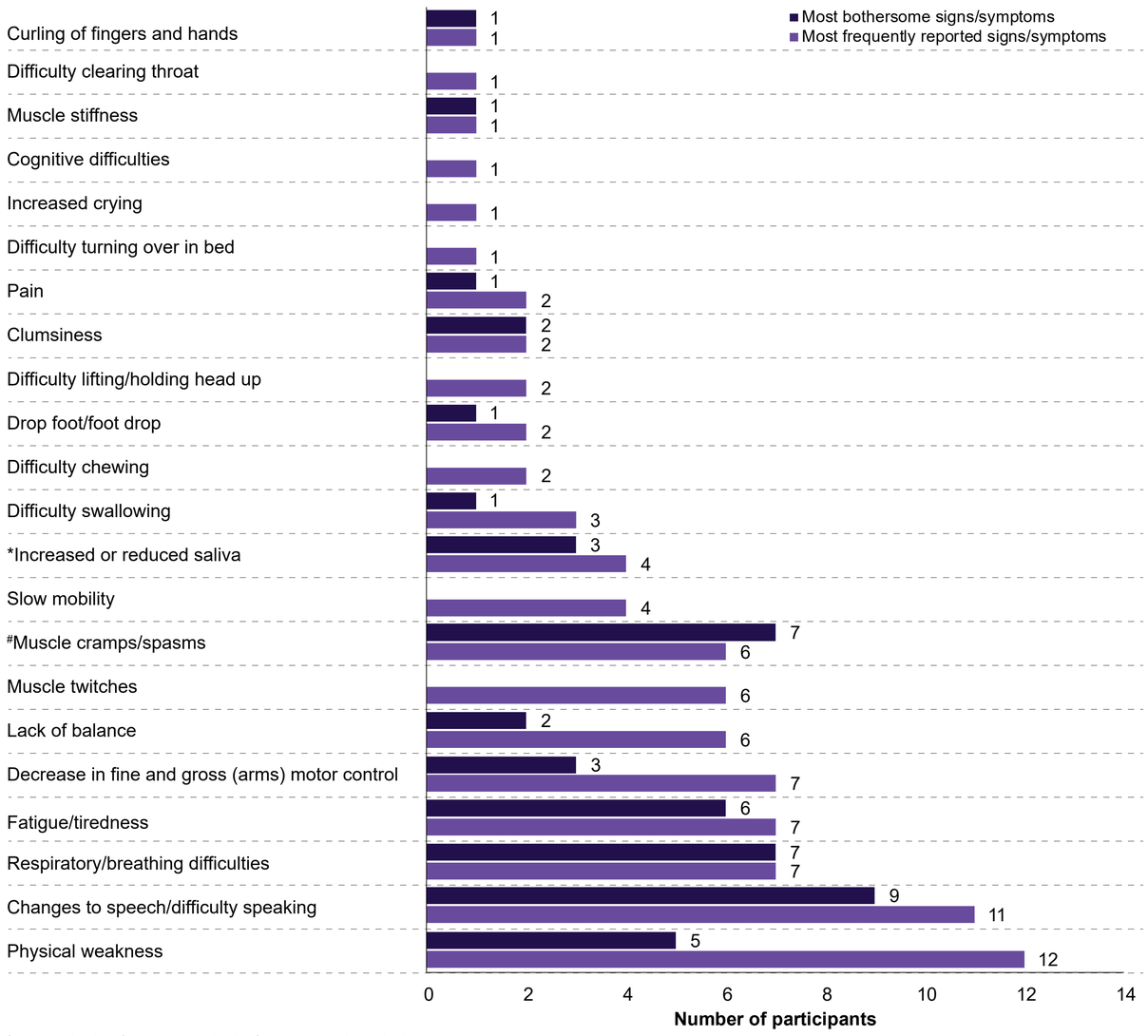
Race, n (%)

White: 9 (60)
Hispanic: 3 (20)
Native Hawaiian/Other Pacific Islander: 2 (13.3)
Prefer not to say: 1 (6.7)

Highest level of education, n (%)

High school diploma or equivalent: 3 (20)
Associate's degree: 6 (4)
Bachelor's degree: 4 (26.7)
Graduate/post-graduate degree: 2 (13.3)

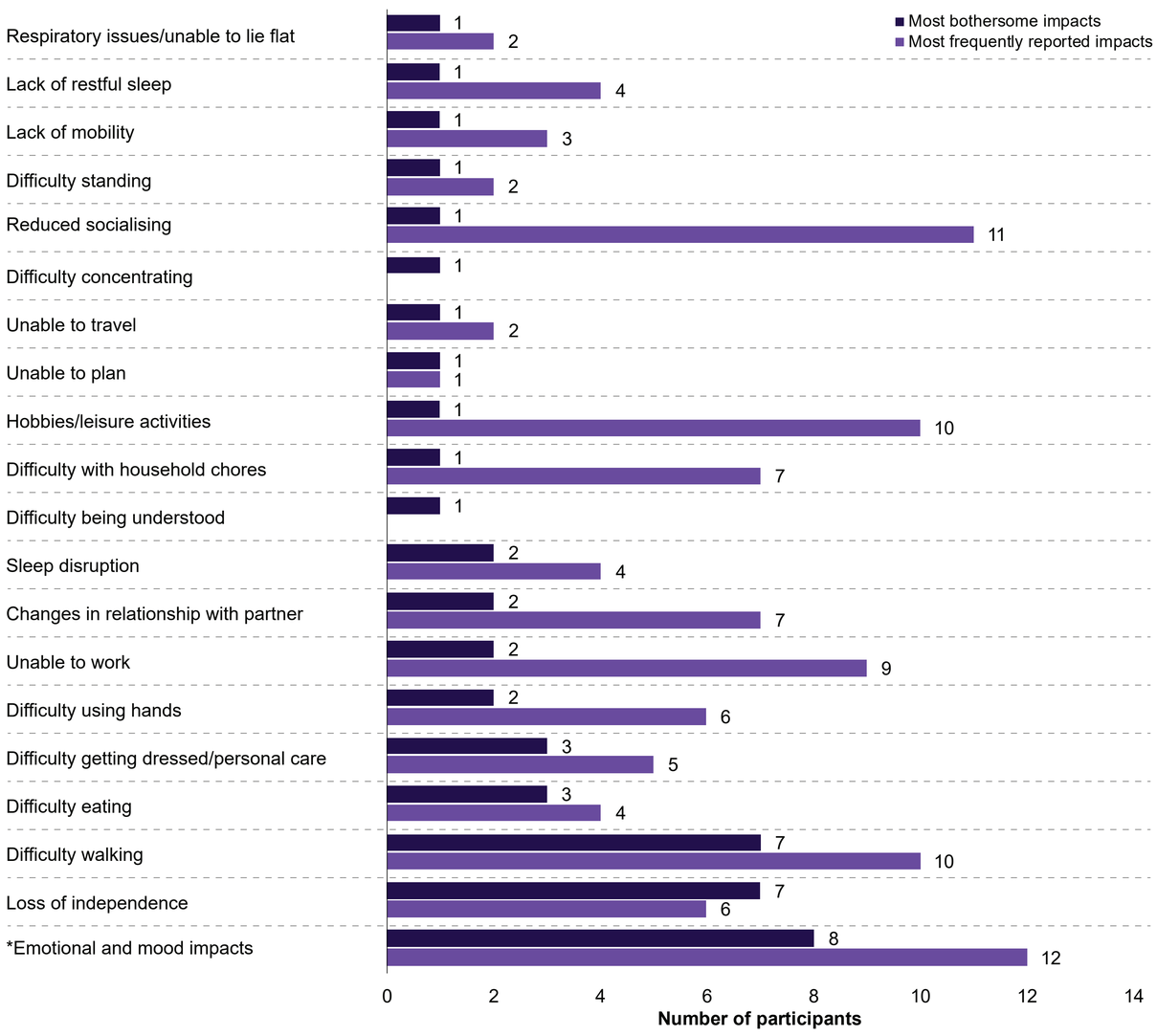
Figure 2. Most frequently reported and the most burdensome signs/symptoms of amyotrophic lateral sclerosis



*increased saliva (not decreased saliva) was reported as a bothersome symptom
#most bothersome value represents muscle cramps, spasms and twitches

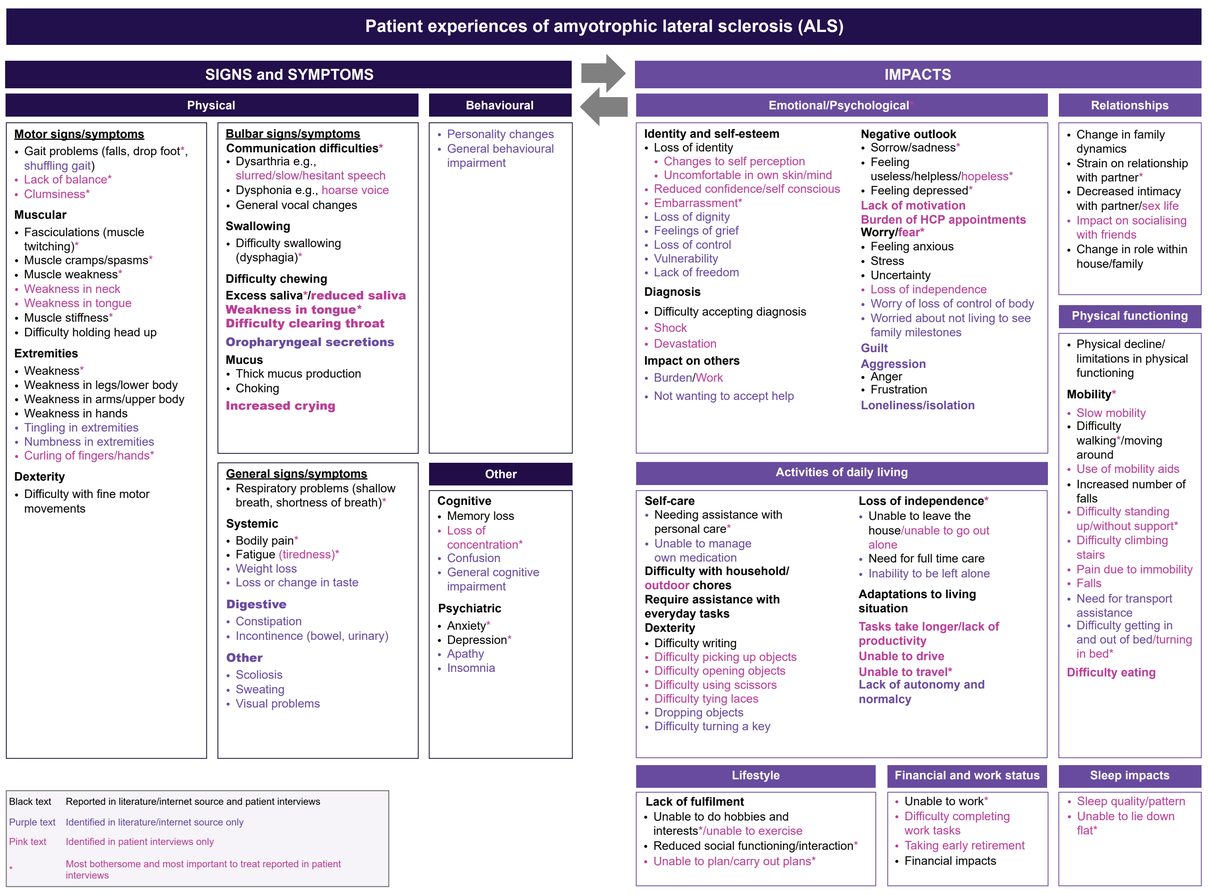
- The most bothersome symptoms frequently reported by participants included changes in speech and talking, muscle spasms, twitches and cramps, breathing difficulties, and fatigue/exhaustion/lack of energy (**Figure 2**).
- ALS affected physical functioning of participants including difficulty walking (n = 10/15), difficulty climbing stairs (n = 5/15), falls (n = 4/15), difficulty eating (n = 4/15), requiring use of mobility aids (n = 4/15), reduced/loss of mobility (n = 3/15), difficulty standing up without support (n = 2/15), pain due to immobility (n = 1/15), and increased need for rest during the day (n = 1/15).
- Participants experienced disrupted sleep (n = 4/15), unable to sleep lying flat (n = 2/15), difficulty moving in bed (n=1/15), and increased need for sleep (n = 1/15).
- Participants reported that ALS impacted many aspects of their daily activities such as, difficulties completing indoor/outdoor chores (n = 7/15), various impacts of decreased fine motor control (n = 6/15), need for assistance with everyday tasks (n = 6/15), loss of independence (n = 6/15), difficulty with personal care (n = 5/15), reduced productivity (n = 4/15), difficulty leaving the house (n = 3/15), difficulty with travelling (n = 2/15), unable to drive (n = 2/15), and adaptations to living situation (n = 2/15).
- ALS impacted emotional and psychological wellbeing of participants, including fear/worry/anxiety (n = 12/15), depression/sadness (n = 8/15), reduced/loss of independence (n = 6/15), frustration/anger (n = 4/15), reduced self-confidence (n = 4/15), lack of motivation (n = 2/15), shock at diagnosis (n = 2/15), and stress (n = 2/15).
- Participants reported considerable impacts on various aspects of lifestyle such as, socialising (n = 11/15), hobbies/leisure activities (n = 10/15), exercise (n = 8/15), and planning activities (n = 1/15).
- ALS impacted relationship with family (n = 8/15), partner (n = 7/15), and friends (n = 4/15).
- Participants also reported that ALS had caused impacts on their ability to work. This included being unable to work (n = 9/15), having trouble with completing work tasks (n = 3/15), and taking early retirement due to ALS (n = 2/15).
- The most frequently reported bothersome impacts included emotional and mood impacts (n = 8/15, 53.3%), loss of independence (n = 7/15, 46.7%), and difficulty in walking (n = 7/15, 46.7%) (**Figure 3**).
- A conceptual model, drafted after a review of existing literature/online blogs/forums was updated following patient interviews (**Figure 4**).

Figure 3. Most frequently reported and the most bothersome impacts of amyotrophic lateral sclerosis



*frequently reported impacts include fear/worry/anxiety, while the most bothersome impacts include depression, embarrassment, anxiety, feeling hopeless, and worry/concern

Figure 4. Updated conceptual model with concepts reported in patient interviews



Conclusions

- **ALS is a debilitating and rapidly progressing disease with high unmet need and devastating impacts on all aspects of patients' lives with severe impacts on physical and emotional wellbeing.**
- **The conceptual model emerging from this study can be used to support the choice of existing disease-specific instruments in clinical studies.**

REFERENCES

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DISCLOSURES

- Tamara Al-zubeidi, Harriet Makin: Employees of Clarivate and may hold shares or share options.
- Charles Minor, Ekaterina Smolkina, Natalia Hakimi-Hawken: Employees of Sanofi and may hold stock or stock options.

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