



impact ALS
EUROPE

IMPACT ALS EUROPE

A European Survey of People living with ALS

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Source of Funding

- Cytokinetics
- Biogen
- Ionis

Disclosures

- Katie Stenson is an employee of and may hold stock and/or stock options in Biogen
- Craig Wakeford is an employee of and may hold stock and/or stock options in Biogen
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REVIEW OF SURVEY METHODOLOGY

REVIEW OF GOALS OF IMPACT ALS Europe



Quantify and improve understanding of the functional burden of disease in ALS to ensure that the patient and caregiver voice is included in the development and evaluation of new therapies

BY



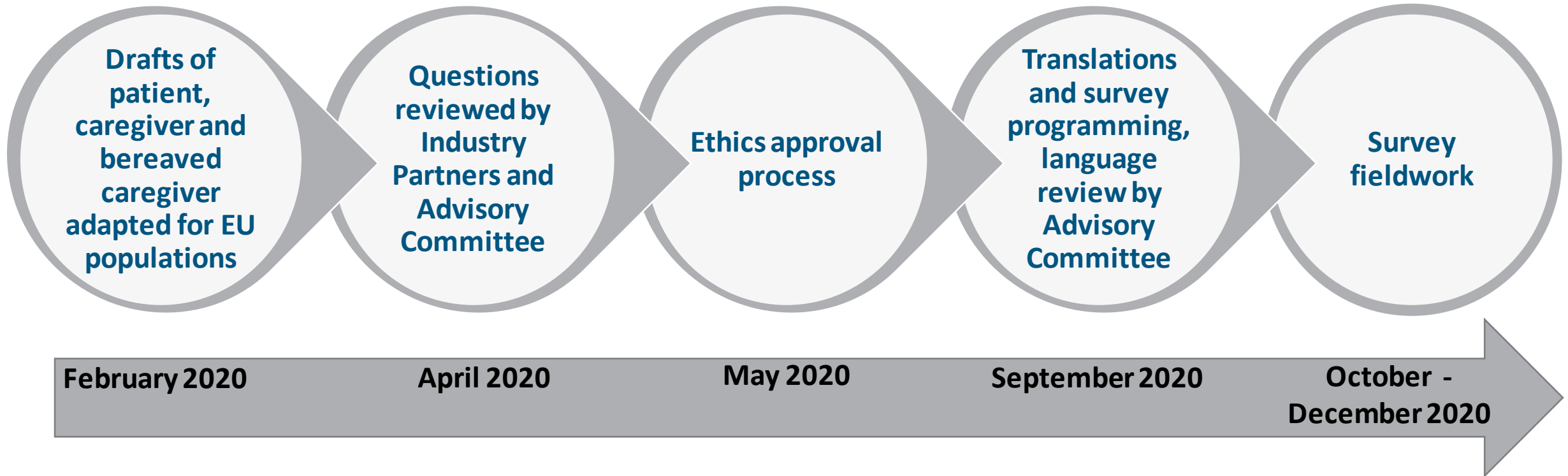
Delivering a robust evidence base for the burden of ALS from the perspective of the patient and caregiver

WHILE



Ensuring a scientifically rigorous approach to facilitate publication and overall impact in the context of regulatory review of new therapeutic options

SURVEY DEVELOPMENT PROCESS



SURVEY QUESTION DEVELOPMENT PROCESS

Patient Survey

(36 main questions, plus follow-up questions depending on answers given)

- Module 1: ALS Diagnosis and Care
- Module 2: Living with ALS
- Module 3: Approaches to Treating ALS
- Module 4: Background Demographics

Caregiver Survey

(55 main questions, plus follow-up questions depending on answers given)

- Module 1: ALS Diagnosis and Care
- Module 2: Living with ALS
- Module 3: Approaches to Treating ALS
- Module 4: Background Demographics
- Module 5: Background and Burden as a Caregiver

Bereaved caregiver survey

(59 main questions, plus follow-up questions depending on answers given)

- Module 1: ALS Diagnosis and Care
- Module 2: Living with ALS
- Module 3: Approaches to Treating ALS
- Module 4: Background Demographics
- Module 5: Background and Burden as a Caregiver

SURVEY RECRUITMENT

- Recruitment materials developed for IMPACT ALS Europe
- Channels for recruiting included voluntary ALS organisation websites, including:
 - Irish Motor Neurone Disease Association (IMNDA)
 - Motor Neurone Disease Association (MNDA)
 - ALS Centrum Nederland
 - Filière Nationale de Santé SLA et autres Maladies rares du Neurone Moteur (FILSLAN)
 - ALS Liga
 - Deutsche Gesellschaft für Muskelkranke e.V. (DGM)
 - Associazione Italiana Sclerosi Laterale Amiotrofica (AISLA ONLUS)
 - Fundación Luzón
 - Fundación Miquel Valls
 - Karolinska ALS Center
 - ENCALS (European Network for the Cure of ALS)
 - European Organisation for Professionals and Patients with ALS (EUALS)
- Survey was launched online on 15th October 2020 and closed 31st December 2020.



**Are you an ALS/MND patient or caregiver?
We need your help!**

Take a survey to help guide drug development and services for amyotrophic lateral sclerosis (ALS)/motor neurone disease (MND)

Please access this important survey at:
<https://cutt.ly/IMPACT-ALS-Survey>



In an effort to include your perspective to help guide the development of new drugs and services for ALS, we are conducting a survey of patients with ALS and of caregivers. This initiative, titled IMPACT-ALS (Investigating and Measuring Patient and Caregiver Trends about ALS), is a research collaboration between industry, European ALS doctors and ALS charities.



The survey is intended to identify the burden of ALS and concerns during the course of the disease, preferences for treatment, perceptions of patients and caregivers. Results from this survey are planned to be published in peer-reviewed journals and other channels. The results of the survey will be accessible to all participants.

The survey will be available from 13/10/2020 to 20/11/2020

- ✓ The survey should take approximately 30–45 minutes to complete
- ✓ Responses are completely anonymous

If you have any questions or difficulty while completing the survey, please contact:
ALS_Survey_Europe@apothecom.com

The information that you provide in this survey will:

- Be anonymised. You will not be identifiable
- Be used solely for the purpose of research studies relating to ALS treatments and experiences of ALS patients and caregivers
- Improve future experiences for other ALS patients and caregivers
- Be shared with companies who have helped to fund the study
- Not be shared with companies if it is personal information, and you will not be contacted by anybody from industry about the information you have provided if you participate in this study





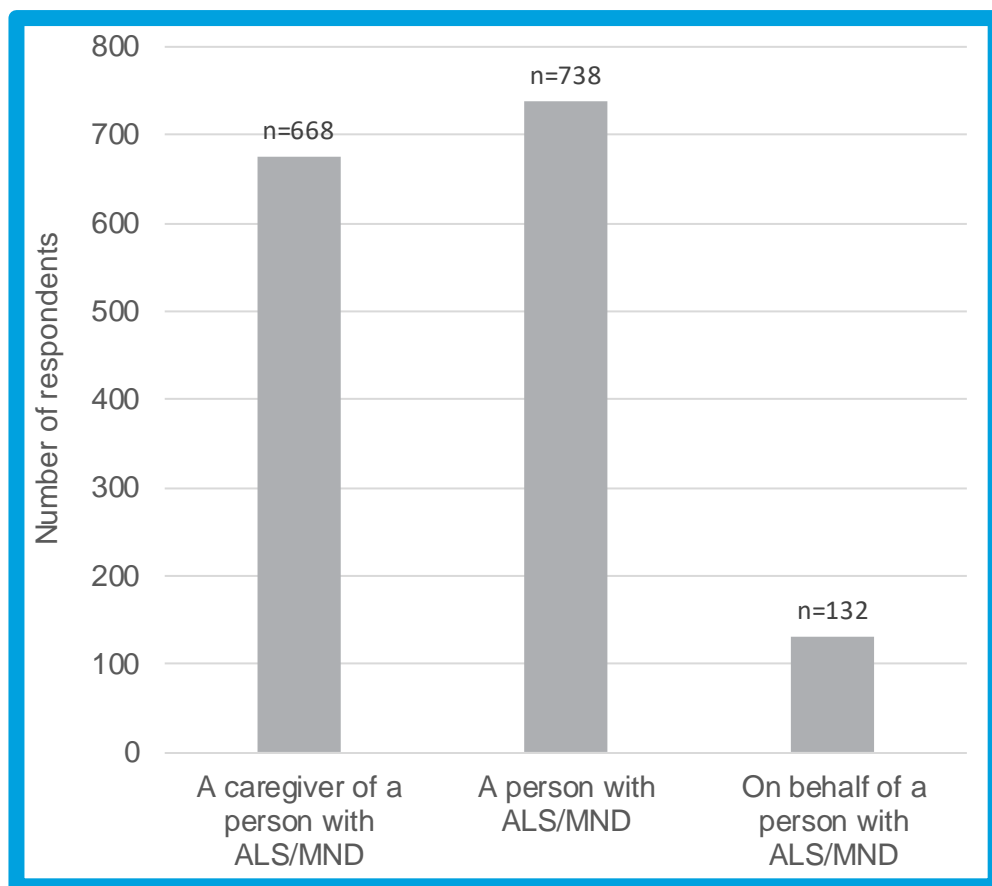
SURVEY RESULTS



DEMOGRAPHICS & DISEASE CHARACTERISTICS

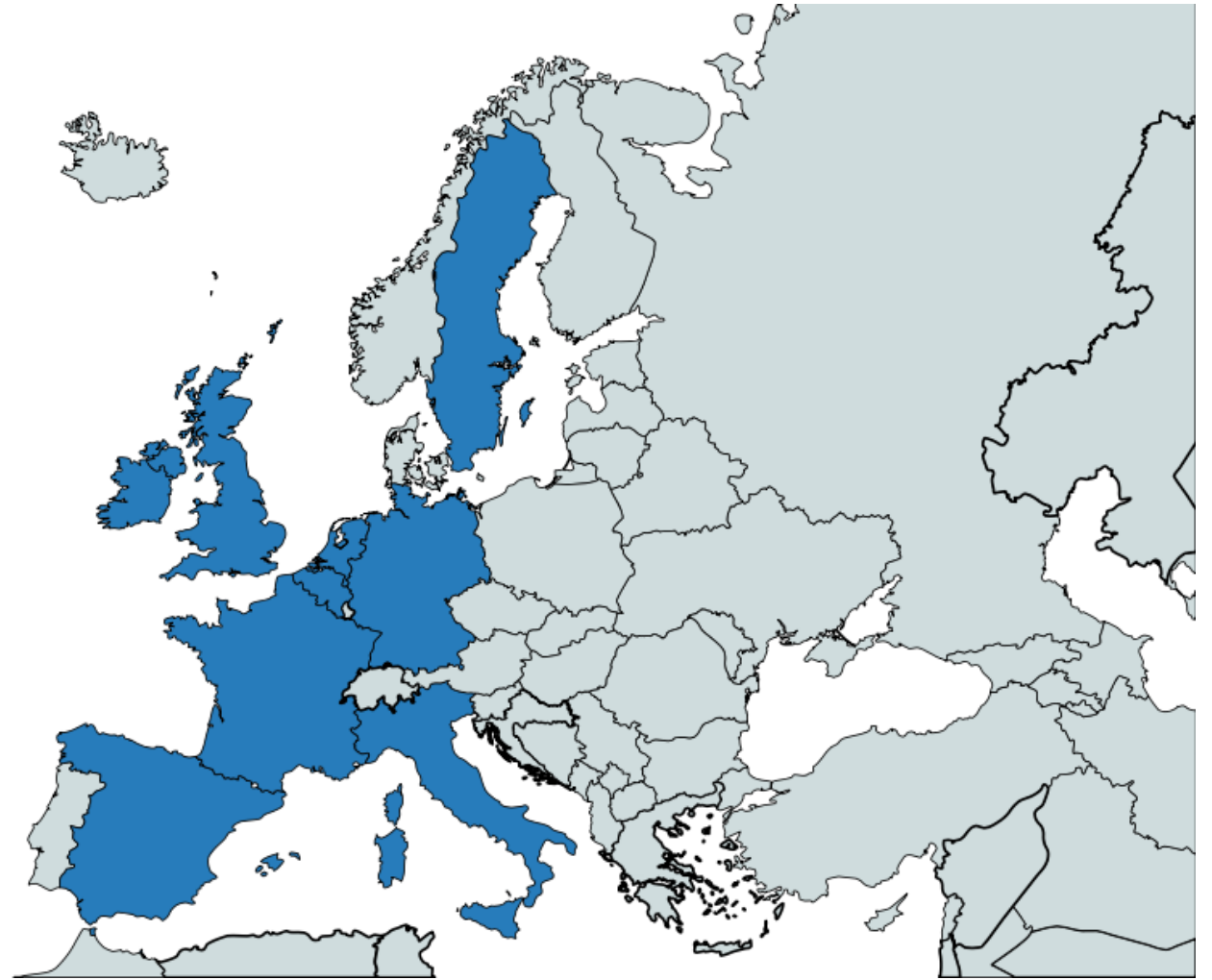
IMPACT ALS Modules 1 & 4

SURVEY RESPONDENTS



PARTICIPANT GROUP	TOTAL
Patients	870
Caregivers	450
Bereaved Caregivers	218
Overall Total	1538

Participating European Countries



RESPONDENT DEMOGRAPHICS BY SUBGROUPS

	Patient (n=870)	Caregiver (n=450)	Bereaved caregivers (n=218)
Respondent age groups, n (%)			
25-34	5 (0.6)	3 (0.7)	0 (0.0)
35-44	41 (4.7)	14 (3.1)	6 (2.8)
45-54	92 (10.6)	50 (11.1)	20 (9.2)
55-64	189 (21.7)	79 (17.6)	42 (19.2)
65-74	147 (16.9)	92 (20.4)	52 (23.9)
75 and older	45 (5.2)	37 (8.2)	24 (11.0)
Not answered	351 (40.3)	175 (38.9)	74 (33.9)
Sex, n (%)			
Male	298 (34.3)	145 (32.2)	71 (32.6)
Female	221 (25.4)	130 (28.9)	73 (33.5)
Not answered	351 (40.3)	175 (38.9)	74 (33.9)

	Patient (n=870)	Caregiver (n=450)	Bereaved caregivers (n=218)
Current country of residence, n (%)			
Belgium	16 (1.8)	2 (0.4)	2 (0.9)
France	75 (8.6)	21 (4.7)	26 (11.9)
Germany	11 (1.3)	9 (2.0)	5 (2.3)
Ireland	24 (2.8)	20 (4.4)	14 (6.4)
Italy	254 (29.2)	158 (35.1)	15 (6.9)
Netherlands	63 (7.2)	14 (3.1)	4 (1.8)
Spain	30 (3.4)	32 (7.1)	63 (28.9)
Sweden	11 (1.3)	6 (1.3)	8 (3.7)
United Kingdom	22 (2.5)	9 (2.0)	3 (1.4)
Other	13(14)	4(.08)	4(1.9)
Not answered	351 (40.3)	175 (38.9)	74 (33.9)

INDIVIDUAL COUNTRY DEMOGRAPHICS

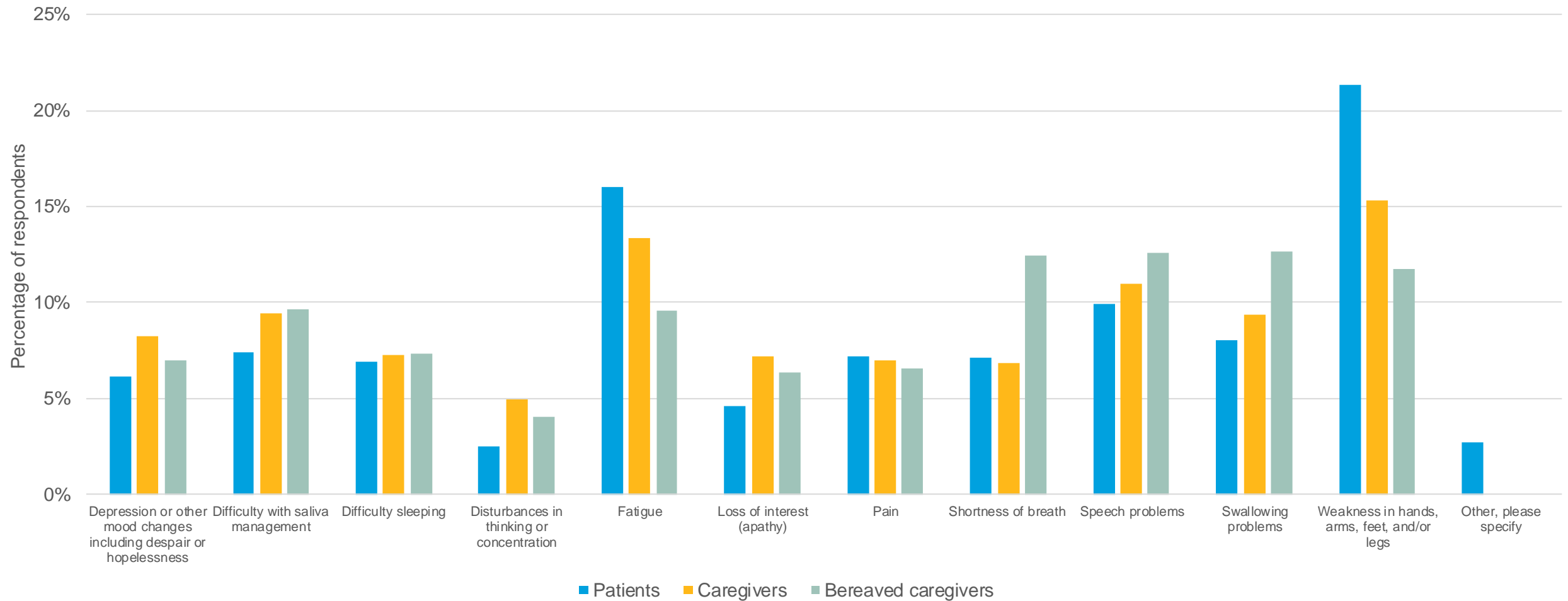
Demographics	OVERALL (n=1538)	Belgium (n=20)	France (n=122)	Germany (n=25)	Ireland (n=58)	Italy (n=427)	Netherlands (n=81)	Spain (n=125)	Sweden (n=25)	UK (n=34)
Respondent age groups, n (%)										
25-34	8 (0.5)	2 (10.0)	1 (0.8)	1 (4.0)	1 (1.7)	1 (0.2)	1 (1.2)	1 (0.8)	0 (0.0)	0 (0.0)
35-44	61 (4.0)	1 (5.0)	10 (8.2)	3 (12.0)	4 (6.9)	28 (6.6)	1 (1.2)	10 (8.0)	3 (12.0)	1 (2.9)
45-54	162 (10.5)	2 (10.0)	16 (13.1)	8 (32.0)	6 (10.3)	76 (17.8)	11 (13.6)	25 (20.0)	7 (28.0)	6 (17.6)
55-64	310 (20.2)	8 (40.0)	40 (32.8)	8 (32.0)	14 (24.1)	144 (33.7)	31 (38.3)	40 (32.0)	5 (20.0)	11 (32.4)
65-74	291 (18.9)	7 (35.0)	42 (34.4)	3 (12.0)	23 (39.7)	130 (30.4)	27 (33.3)	34 (27.2)	8 (32.0)	11 (32.4)
75 and older	106 (6.9)	0 (0.0)	13 (10.7)	2 (8.0)	10 (17.2)	48 (11.2)	10 (12.3)	15 (12.0)	2 (8.0)	5 (14.7)
Sex, n (%)										
Male	514 (54.8)	16 (80.0)	68 (55.7)	11 (44.0)	35 (60.3)	234 (54.8)	47 (58.0)	58 (46.4)	10 (40.0)	22 (64.7)
Female	424 (45.2)	4 (20.0)	54 (44.3)	14 (56.0)	23 (39.7)	193 (45.2)	34 (42.0)	67 (53.6)	15 (60.0)	12 (35.3)
Employment status										
Full-time	89 (11.2)	1 (6.3)	8 (10.7)	4 (20)	10 (22.7)	41 (16.1)	1 (1.3)	1 (3.3)	2 (18.2)	5 (22.7)
Part-time	110 (13.8)	0 (0)	4 (5.3)	1 (5)	7 (15.9)	17 (6.7)	10 (13)	1 (3.3)	1 (9.1)	0 (0)
On disability	148 (18.6)	3 (18.8)	11 (14.7)	3 (15)	4 (9.1)	61 (24)	17 (22.1)	17 (56.7)	4 (36.4)	3 (13.6)
Retired	235 (29.6)	6 (37.5)	45 (60)	7 (35)	12 (27.3)	104 (40.9)	25 (32.5)	8 (26.7)	4 (36.4)	14 (63.6)
Unemployed	141 (17.8)	0 (0)	0 (0)	3 (15)	7 (15.9)	10 (3.9)	6 (7.8)	1 (3.3)	0 (0)	0 (0)
Other	71 (8.9)	6 (37.5)	7 (9.3)	2 (10)	4 (9.1)	21 (8.3)	18 (23.4)	2 (6.7)	0 (0)	0 (0)



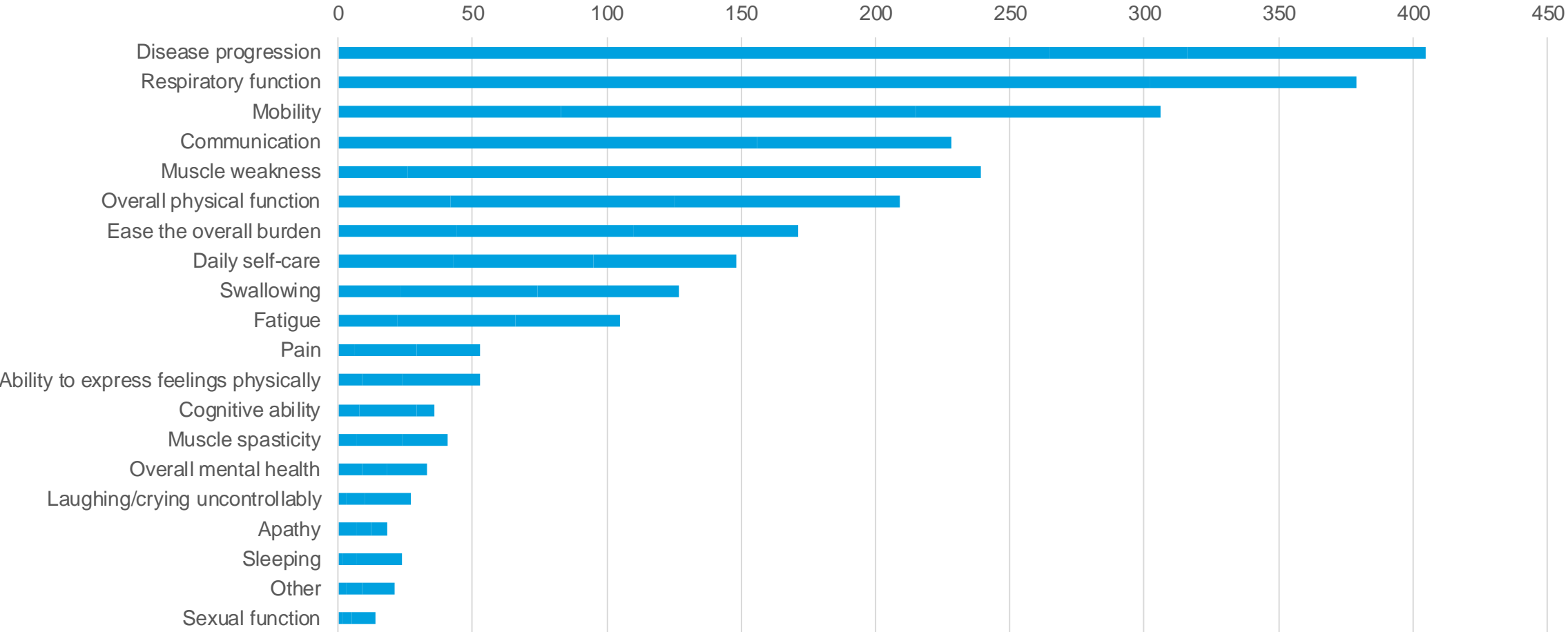
LIVING WITH ALS

IMPACT ALS Module 2

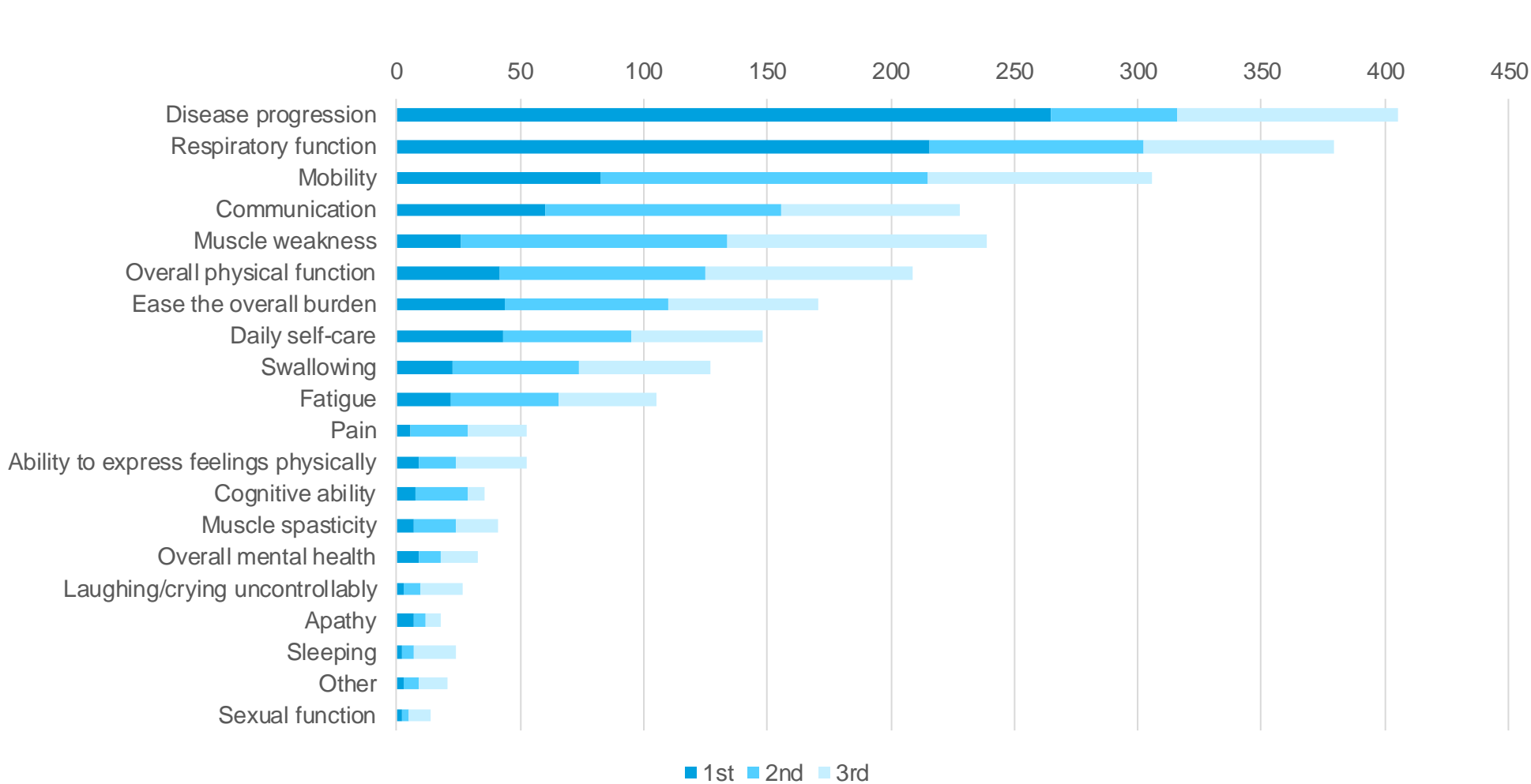
SYMPTOMS OF ALS IN THE PAST 2 WEEKS



PREFERRED SYMPTOMS FOR TREATMENT TO IMPACT

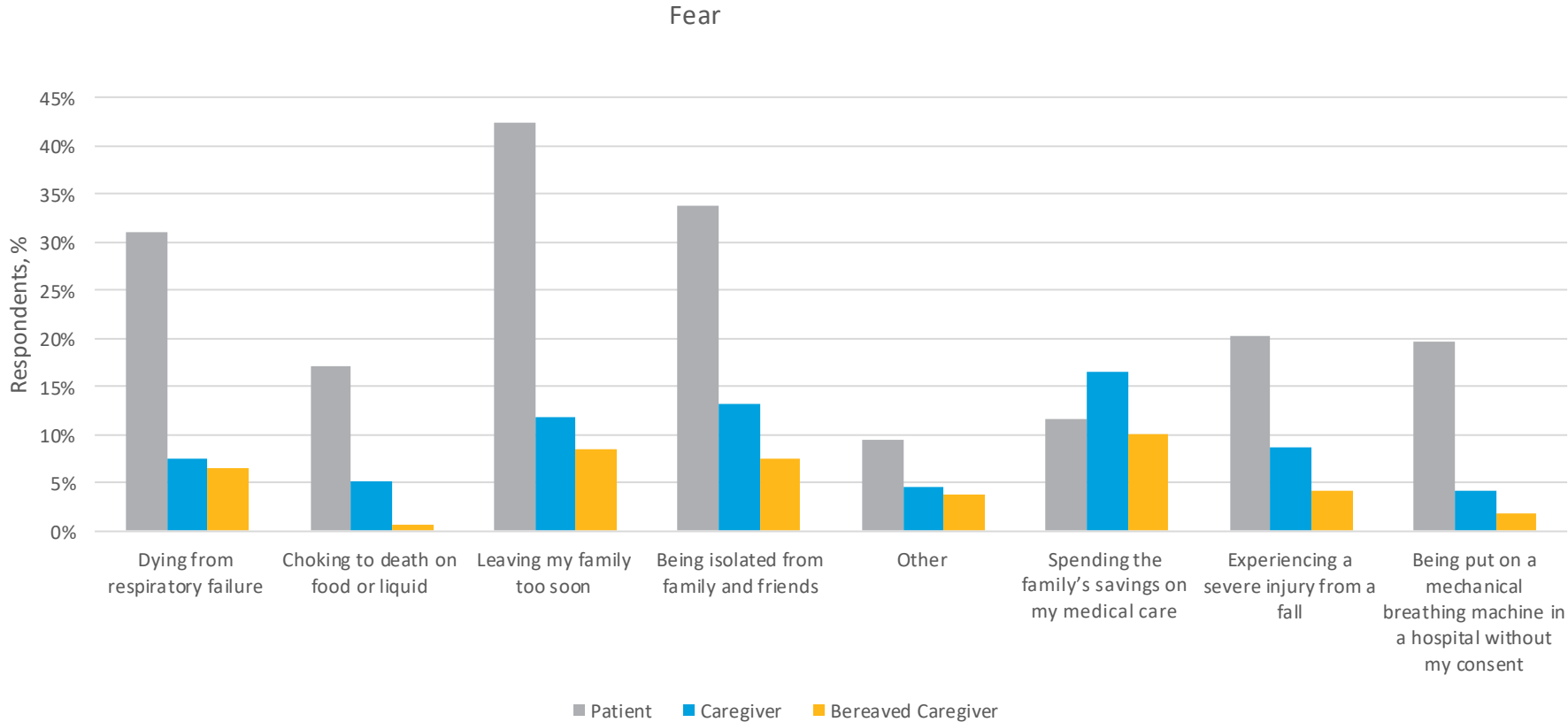
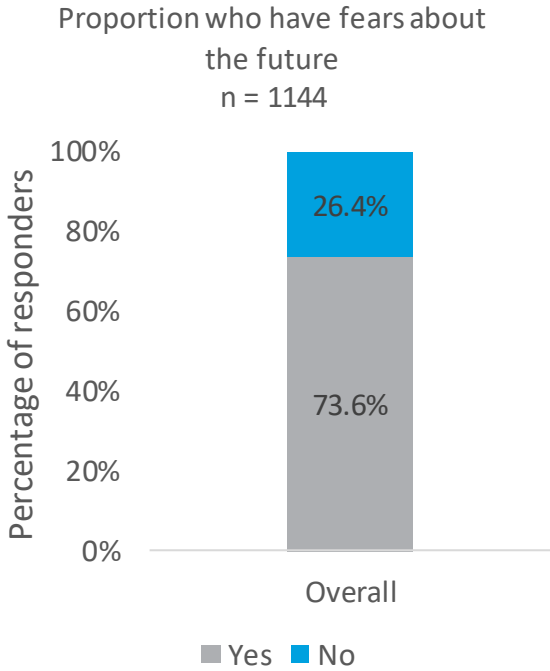


PREFERRED SYMPTOMS FOR TREATMENT TO IMPACT



Impairment	Mean rank
Disease progression	1.57
Respiratory function	1.79
Mobility	2.51
Communication	2.90
Muscle weakness	3.02
Overall physical function	3.07
Ease the overall burden	3.20
Daily self-care	3.29
Swallowing	3.45
Fatigue	3.52
Pain	3.78
Ability to express feelings physically	3.79
Cognitive ability	3.82
Muscle spasticity	3.82
Overall mental health	3.85
Laughing/crying uncontrollably	3.90
Apathy	3.91
Sleeping	3.92
Other	3.92
Sexual function	3.95

FUTURE FEARS OF ALS PATIENTS



FUTURE FEARS: OTHER

Losing independence/autonomy

Being a burden on family

Death/Pain

Uncertainty of disease course

Partner leaving

Exclusion from social activities

Not being allowed to drive

Feelings of burden to others

Losing independence

Death

Suffering

COVID-19

Disease affecting children

No fears

The future

Isolation due to the pandemic

Not seeing children grow up

Being unable to communicate

Not seeing your family grow up

Losing autonomy

Being a burden

Dying

Being immobile

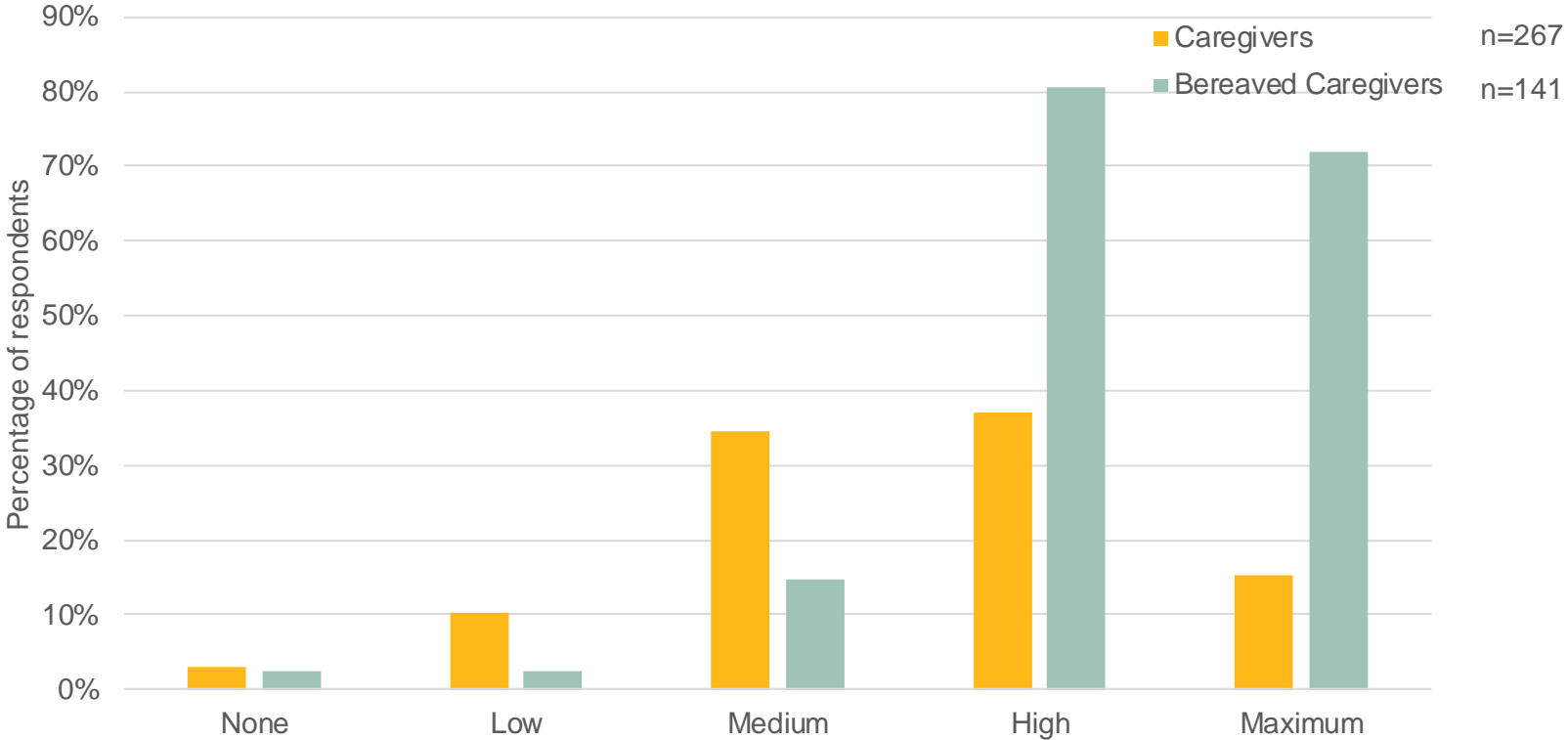
Aggressive/invasive therapy

Suffering

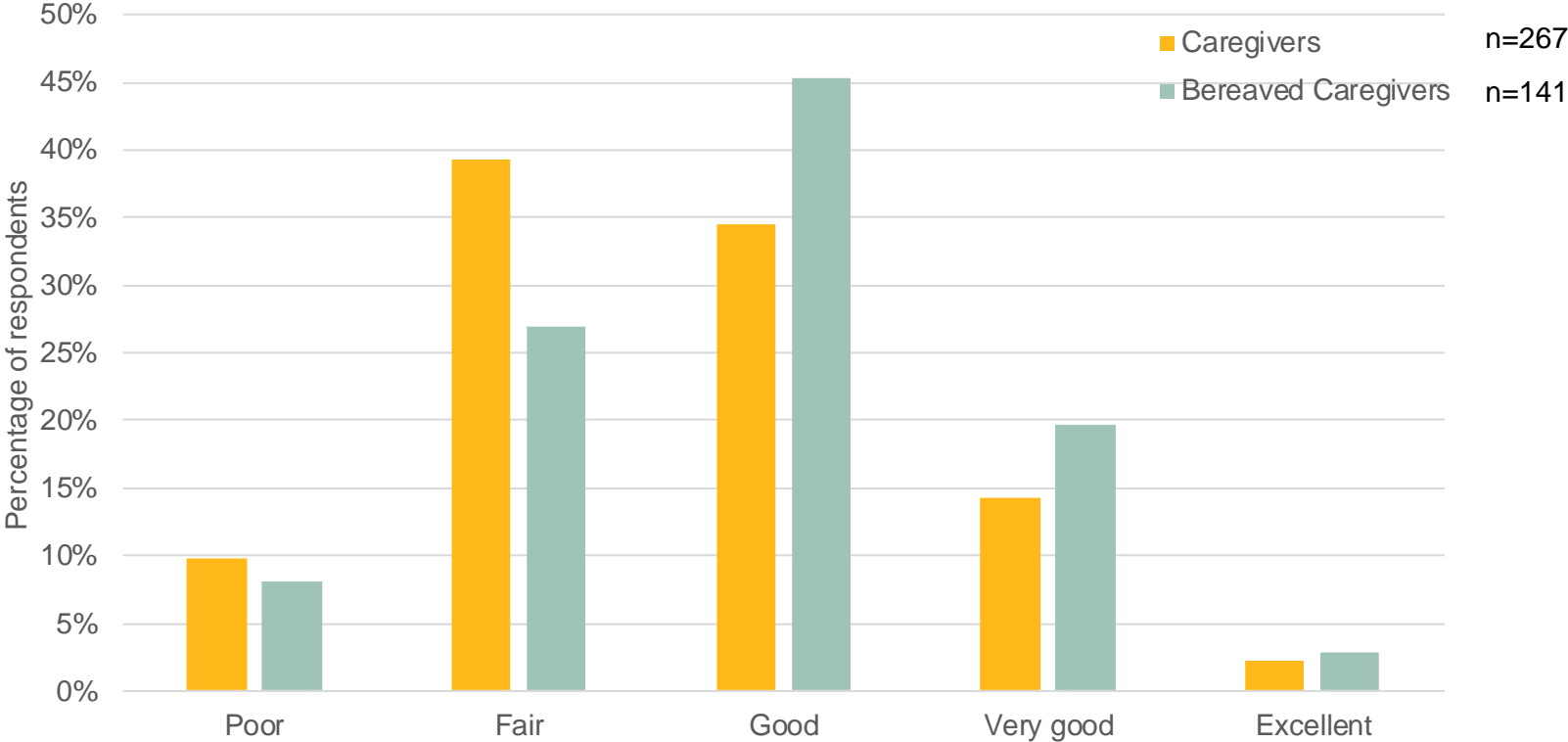
Being useless

- Patients
- Caregivers
- Bereaved caregivers

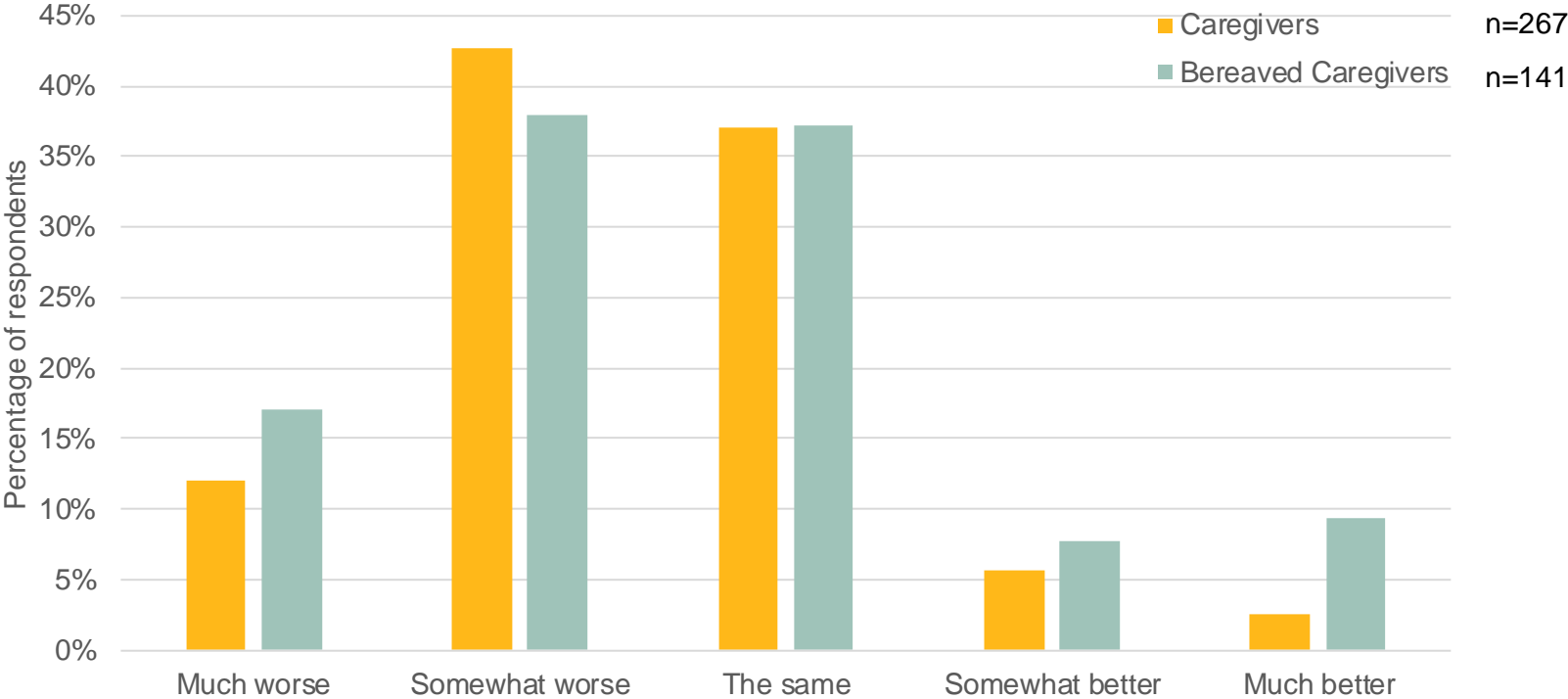
CAREGIVER STRESS LEVELS IN THE LAST 2 WEEKS



CAREGIVERS' CURRENT HEALTH STATUS



CAREGIVER HEALTH STATUS; CURRENT VERSUS PREVIOUS



CONCLUSIONS AND NEXT STEPS





**THANKS FOR YOUR
ATTENTION**

Questions and discussion