PERSONAL EXPERIENCE EXPECTATIONS KNOWLEDGE

PEEK

www.cc-dr.org

CARDIAC AMYLOIDOSIS + OTHER FORMS OF AMYLOIDOSIS

AUSTRALIAN STUDY 2020

Centre for Community-Driven Research

Section 1

Introduction
&
Methodology

This is where you can read about the PEEK methodology, information about the recruitment period and approach.

There is also a review of other mixed methodology studies in the field to identify the position of the PEEK study in the context of other literature. PEEK studies are often the largest of their kind in depth and participant numbers.

Full Report
Summary of Results
Infographic
Slide Set
Introduction & Methodology
Demographics
Symptoms & Diagnosis
Decision-making
Treatment
Communication & Information
Care & Support
Quality of Life
Expectations & Messages to Decision-Makers
Advice from current patients to patients in the future
Discussion
Where to from here?

The Centre for Community-Driven Research team for this study included:

Catherine Holliday
Anne Holliday
Klair Bayley
Josephine Keneally
Eve Houghton
Jenny Hutton
Lara Baker

Thank you to each and every person that participated in this PEEK study and partners that supported the project including Pfizer, The Australian Amyloidosis Network and Leukaemia Foundation, and all of the health professionals across Australia that also helped to spread the word about this PEEK study.

Position of this PEEK study

In this PEEK study, 28 people diagnosed with amyloidosis, and eight carers to people with amyloidosis throughout Australia participated in the study that included a qualitative structured interview and quantitative questionnaire. This study in ATTR is therefore the largest mixed methods study reported in an Australian population, and it includes the most patient interviews worldwide. In addition, PEEK is a comprehensive study covering all aspects of disease experience from symptoms, diagnosis, treatment, healthcare communication, information provision, care and support, quality of life, and future treatment and care expectations.

A comparison of studies is available in Section 1.

A search was conducted in Pubmed (June 24, 2020) to identify ATTR or AL amyloidosis quality of life or patient experience studies of adults that had been conducted in the past ten years in worldwide (Table 1.1). Meta-analysis studies, studies conducted in developing countries, and studies of less than five participants were excluded.

There were 32 studies identified that collected patient self-reported data. There were three studies using qualitative methods of between 10 and 18 participants, and 28 studies using quantitative methods of between 10 and 1,739 participants, and one mixed methods study of 10 interviewed participants with 341 participants completing questionnaires. There were eight international studies, eight in the USA, six in Portugal, two in France, two in the UK, and one each in, Japan[2] and Sweden. One international study included participants from Australia.

There were ten drug trials, five studies focused on quality of life, three nutrition studies, two studies each focused on distress, liver transplants, disease characterisation, and a single study each focused on anxiety and depression, coping strategies, diagnosis, education, genetic screening, pharyngo-laryngeal involvement, stigma, and urinary tract dysfunction.

Section 2

Demographics

The demographic section is where you can find information about the study cohort. This is also where the sub-groups used throughout the study are described and where the baseline health of the cohort is described, using the SF36 questionnaire.

36 Participants

High SEIFA
25

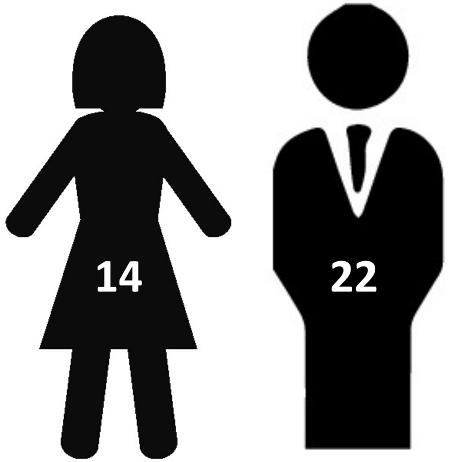
Mid to low SEIFA
11

14 University
14 Trade/High school
(Participants with amyloidosis only)

27 Metropolitan

9 Regional/Remote

8 Carers
28 Amyloidosis



30 hours & 39 minutes of interviews

Average interview time of 59 minutes*

*Average based on n=31 participants who undertook phone interviews

Formal name	Definition	n=36	Percent
Participant type	ATTR-Cardiac	18	50.00
	All-cardiac	25	69.44
	AL Amyloidosis	10	27.78
	Carer	8	22.22
Gender	Male	22	61.11
	Female	14	38.89
Age	Aged 55 to 64	8	22.86
	Aged 65 to 74	19	54.29
	Aged 75 or older	8	22.86
Education (n=28)	Highest qualification trade or high school	14	50.00
	University qualification	14	50.00
Location	Major city by AGSG Remoteness 2016	27	75.00
	Inner/outer regional by AGSG	9	25.00
Socio-Economic Indexes for Areas (SEIFA)	SEIFA decile 1-6	11	30.56
	SEIFA decile 7-10	25	69.44

Calculations are presented for each of the sub-groups throughout the full report.

Qualitative: Where there is a variation between a sub-group and the general cohort this has been highlighted in red for less than 10% variation, and green for more than 10% variation. These variation tables can be viewed in the full report.

Quantitative: Where there is a statistically significant p-value variation, where $p<0.05$, these have been included.

In this study, participant numbers are assigned by their diagnosis type. For example a carer would appear as 001CA while someone diagnosed with AL Amyloidosis would appear as 001AL.

Sub-groups

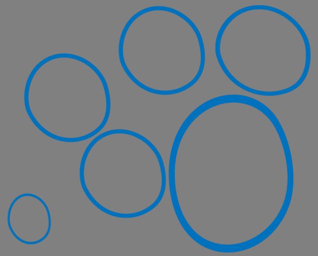
SF36 scale (n=36)	Mean	SD	Median	IQR	Possible range	Quintile
Physical functioning	53.47	31.82	52.50	57.50	0 to 100	3
Role functioning/physical	37.50	43.30	25.00	100.00	0 to 100	2
Role functioning/emotional	62.04	41.52	66.67	66.67	0 to 100	4
Energy/Fatigue*	43.33	25.41	45.00	35.00	0 to 100	3
Emotional well-being	72.44	17.44	76.00	20.00	0 to 100	4
Social functioning	60.76	28.99	62.50	40.63	0 to 100	4
Pain*	59.58	24.39	55.00	32.50	0 to 100	3
General health*	46.81	22.46	45.00	41.25	0 to 100	3
Health change	40.28	24.11	37.50	25.00	0 to 100	2

*Normal distribution, use mean and SD as central measure. Possible range 0-100

The “**SF36 Role functioning/physical"** scale measures how physical health interferes with work or other activities. On average, physical health of the participants in this study interfered quite a bit with work or other activities.

The **“SF36 Health change”** scale measures health now compared to a year ago. On average, the participants in this study had somewhat worse now, than a year ago.

Section 3



Symptoms & Diagnosis

The symptoms and diagnosis section is where you can find information about experience of symptoms before diagnosis, as well as the symptoms that led to the diagnosis. There is information about the diagnostic tests that were performed, where the tests were conducted and the time from tests to diagnosis. In this section, there is also information about the participant's understanding of their condition and their understanding of their prognosis.

Symptom	All participants	
	n=28	Percent
Short of breath	17	60.71
Palpitations	10	35.71
Chest pain	2	7.14
Fatigue	18	64.29
Sleep problems	8	28.57
Carpal tunnel	11	39.29
Swollen feet/ankles	6	21.43
Limb weakness	16	57.14
Eye floaters	7	25.00
Lightheaded	16	57.14
Decrease appetite	10	35.71
Bloating	7	25.00
Diarrhea/constipation	11	39.29
Nausea	2	7.14
Weight loss	13	46.43
Swollen tongue	3	10.71
Skin changes	5	17.86
Other	10	35.71

Number of symptoms per participant	All participants	
	n=28	Percent
No symptoms	2	7.14
1 to 2	3	10.71
3 to 4	6	21.43
5 to 6	3	10.71
7 to 8	5	17.86
9 to 10	5	17.86
11 or more	4	14.29

All symptoms experienced before diagnosis & number of symptoms

Symptom	Number (n=28)	Percent	Quality of life			
			Mean	SD	Median	IQR
Short of breath	17	60.71	4.00	1.70	4.00	2.00
Palpitations	8	28.57	3.63	1.51	3.00	2.25
Chest pain	1	3.57	1.00	0.00	1.00	0.00
Fatigue	18	64.29	3.33	1.33	3.00	1.75
Sleep problems	8	28.57	2.63	1.19	3.00	1.50
Carpal tunnel	11	39.29	3.82	1.47	4.00	2.00
Swollen feet/ankles	6	21.43	2.33	1.03	2.00	0.75
Limb weakness	16	57.14	3.25	1.44	3.00	3.00
Eye floaters	7	25.00	3.43	1.72	3.00	2.00
Lightheaded	23	82.14	3.70	1.46	4.00	2.00
Decrease appetite	10	35.71	2.90	1.10	3.00	0.75
Bloating	7	25.00	3.14	1.46	3.00	1.50
Diarrhea/constipation	11	39.29	3.45	1.69	3.00	2.50
Nausea	2	7.14	2.00	1.41	2.00	1.00
Weight loss	13	46.43	3.08	1.38	3.00	2.00
Swollen tongue	2	7.14	2.00	0.00	2.00	0.00
Skin changes	4	14.29	2.50	0.58	2.50	1.00

Quality of life was rated on a Likert scale from 1 to 7, where 1 is “Life was very distressing” and 7 is “Life was great” (Table 3.2, Figure 3.2). The median quality of life was between 1.00 and 4.00, for all of the symptoms listed in the questionnaire, this is in the “Life was very distressing” to “Life was average” range.

Quality of Life while experiencing symptoms

What were the symptoms that led to your diagnosis?

22% EXCESSIVE WEIGHT LOSS
19% BREATHLESSNESS
11% TIREDNESS/FATIGUE
11% SPECIFIC PHYSICAL SENSATION E.G. NUMBNESS

Loss of weight, about 20 kilos and have regained about 4 kilos of that over the treatment time, et cetera. It's a net loss of 15 kilos to date. Participant 001AL

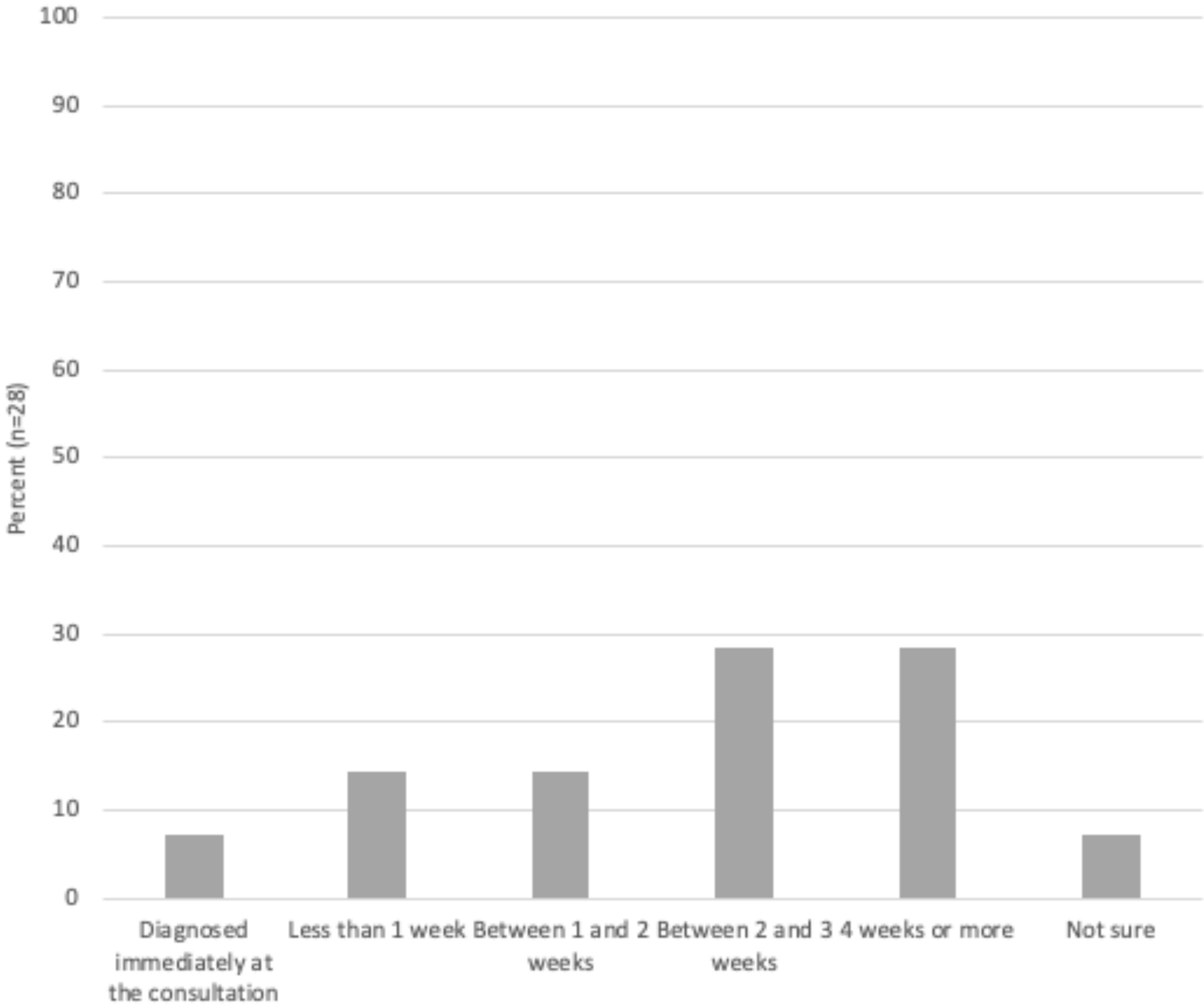
Then I started to have weight loss. Unexplained weight loss. I was eating but I was just-- I wasn't exercising any more that what I would normally. In fact, I'd cut back because I was feeling fatigued and I'd lost interest. Participant 001ATR

Then in 2008, in about eight or nine months I lost about 30, 35 kilos. After a series of blood tests, my GP looked at me one day and said, 'Oh, I think you better go and see a haematologist.' Participant 002ALX

Time from diagnostic test to diagnosis	Number (n=28)	Percent
Diagnosed immediately at the consultation	2	7.14
Less than 1 week	4	14.29
Between 1 and 2 weeks	4	14.29
Between 2 and 3 weeks	8	28.57
4 weeks or more	8	28.57
Not sure	2	7.14

Out of pocket expenses diagnostic tests	Number (n=28)	Percent
$0	8	28.57
$100 - $500	2	7.14
$500 to $1000	4	14.29
More than $1000	2	7.14
Unsure	12	42.86

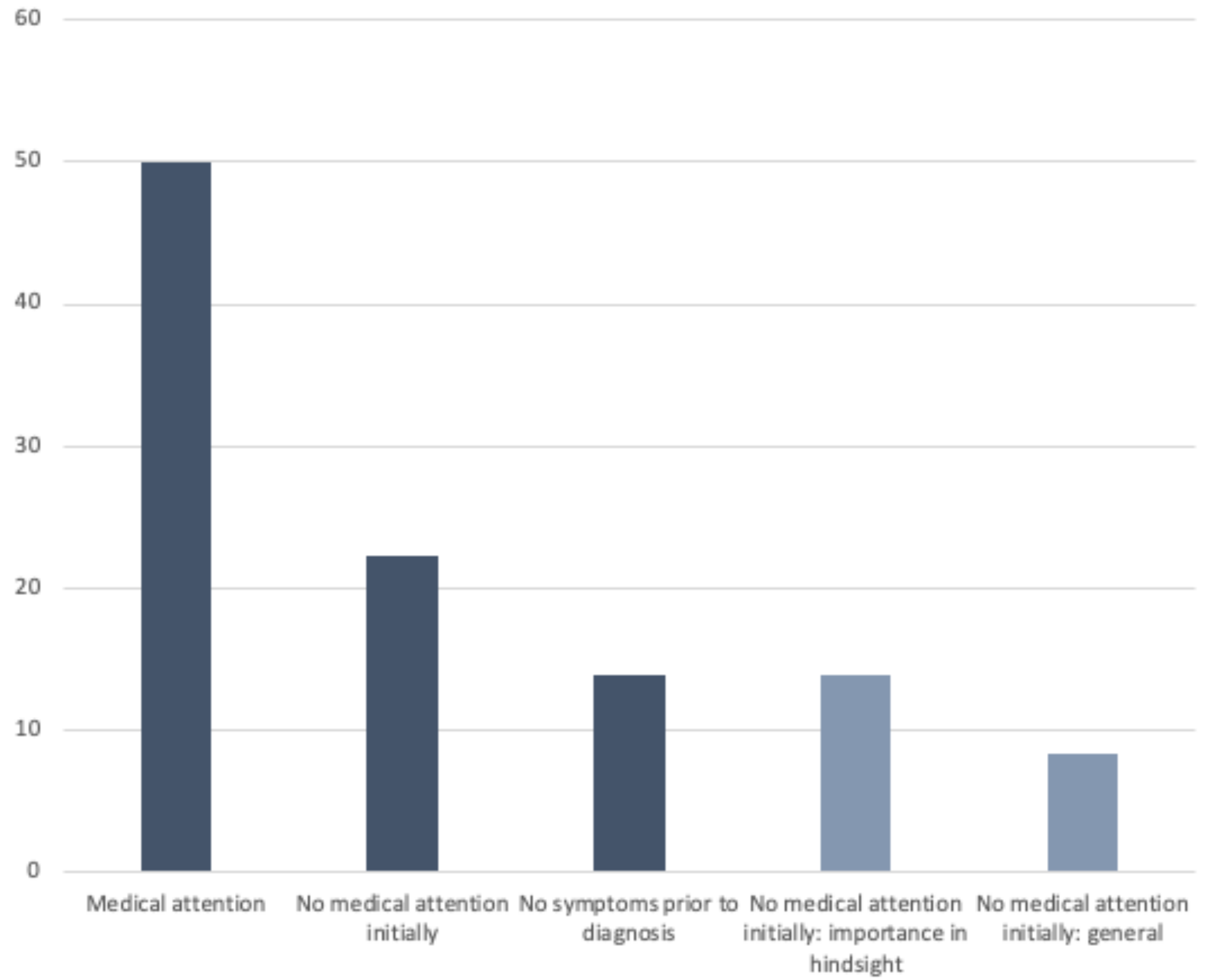
Cost of diagnosis: burden	Number (n=20)	Percent
Not at all significant	7	35.00
Slightly significant	5	25.00
Somewhat significant	5	25.00
Moderately significant	3	15.00
Extremely significant	0	0.00



Centre for Community-Driven Research

Time from tests to diagnosis & costs

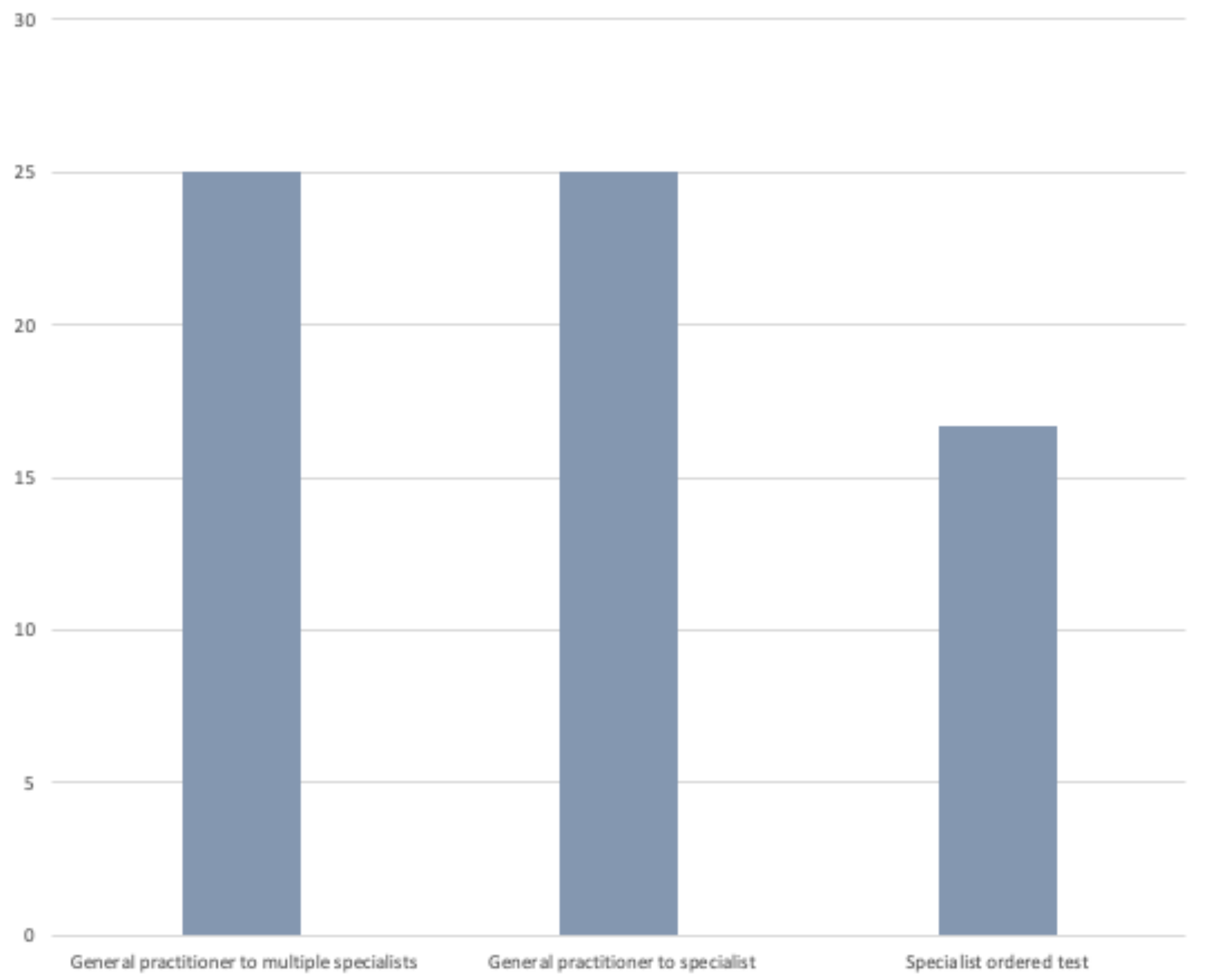
Symptoms leading to diagnosis (% all)



The principal symptom was just shortness of breath and sort of a gripping thirst the whole way up for the first sort of ten, fifteen minutes. Very unusual. I consulted my GP here in LOCATION REGIONAL and he says, "Oh, you might have diabetes or some other renal condition" and sent me for a bunch of tests, but anyway no diabetes. Participant 004AL

First time I started to notice something wasn't right was in August 2017, when I was getting out of breath. As a result of that, everyone thought I was having a heart attack, so they sent me to a cardiologist. Participant 004ATR

It all just started to keep building up and I knew there was something wrong, but I couldn't get to where they would understand what I was saying. The GP kept fobbing me off…I was eventually sent to a neurologist and he picked up. Participant 005AL



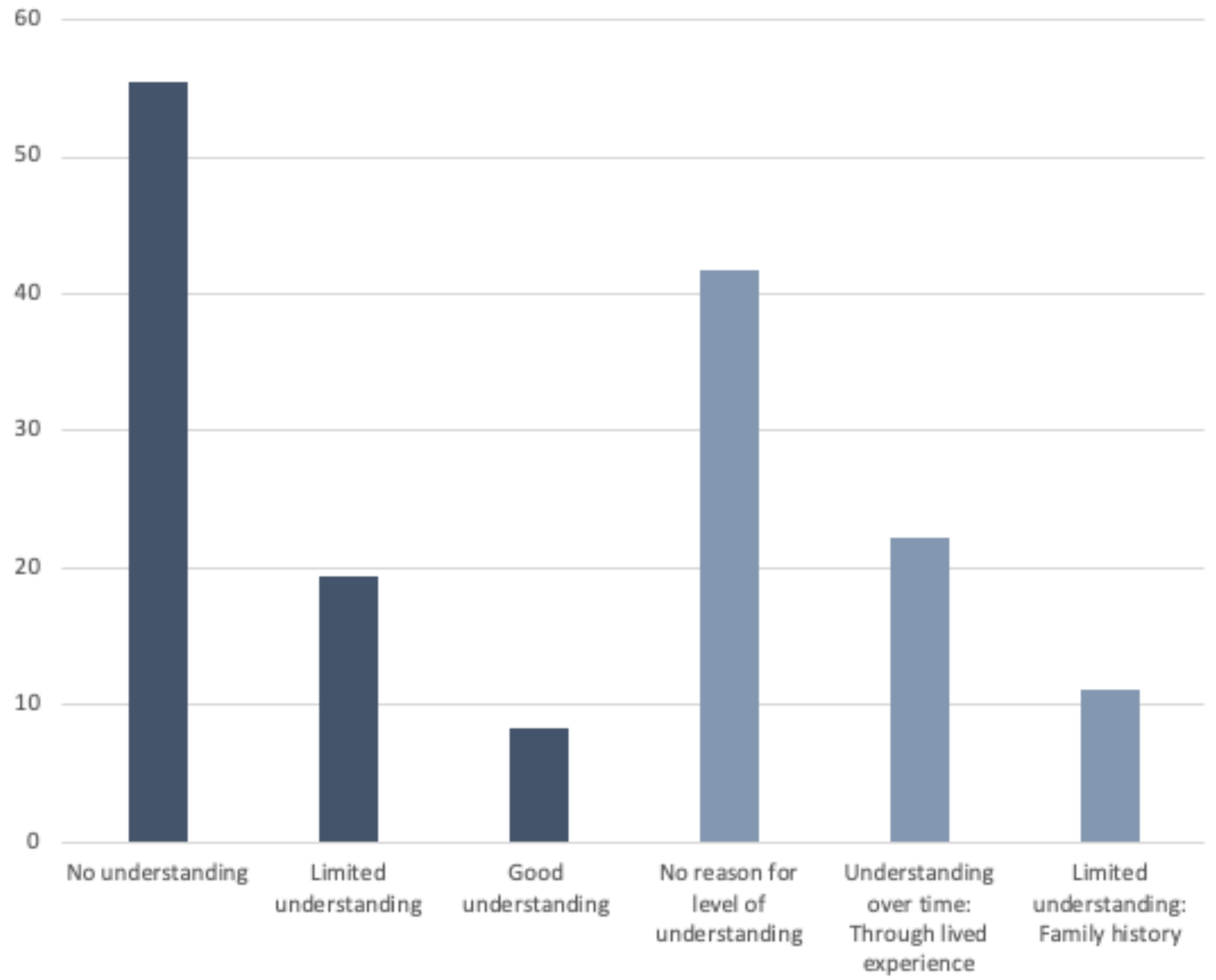
After my second visit to emergency, my GP said, "There's got to be something wrong here. We'll have to do some more tests to find out." …He sent me off to a kidney specialist…NAME KIDNEY SPECIALIST knew what was wrong but he didn't specifically tell me. He said, "I'm going to send you off to a haematologist and he will explain it in more detail." That's sort of when I was told exactly what it was, after lots of tests, of course. Participant 001ALX

I was sent to a physician, and the physician had me have some blood tests which it included the light chain numbers. Hadn't been ever done to me before, and it was then that they found that I had an imbalance between the kappa and the lambda blood chains, and then I was sent to haematologist-oncologist who did a bone marrow biopsy. Participant 003AL

Symptoms leading to diagnosis (% all)

Centre for Community-Driven Research

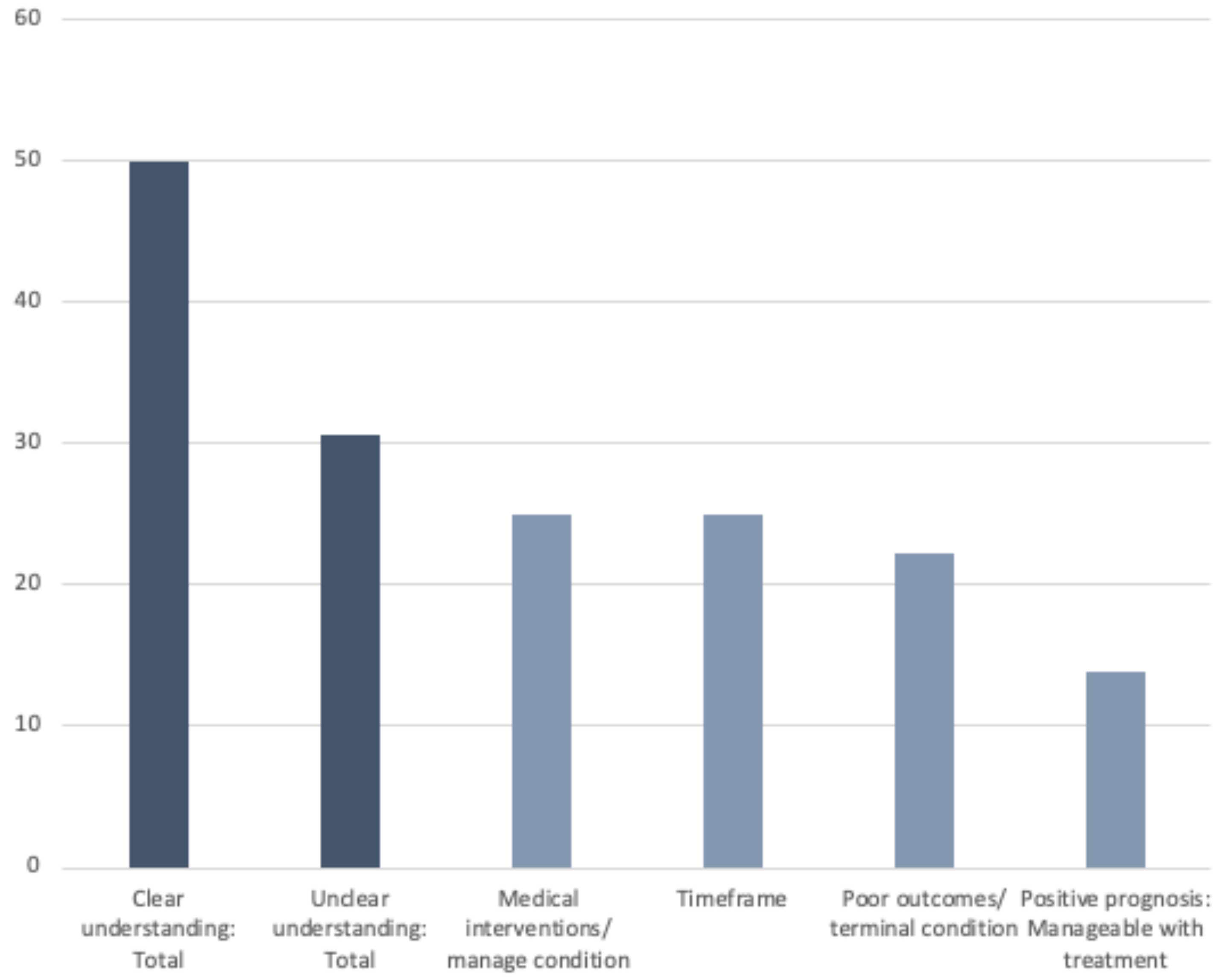
Understanding of disease at diagnosis (% all)



Nothing. Initially nothing. I just went on a steep learning curve. Participant 004AL

I had never heard of it and I thought we were quite well-read and quite knowledgeable people. I had never heard of amyloidosis, so that put me as AN ACADEMIC that put me in the fast track of having to find out as much about this as I possibly could. Participant 001CA

Nothing at all. Nothing at all. NAME HUSBAND probably told you, I think he did tell you this, that the haematologists we saw at the beginning was very abrupt, very non-empathetic basically said, "Oh, yes, it's this. You better get your affairs in order and take off your bucket list," and that's it. We saw our future go from somewhere in the distance straight up in front of our faces, then we both came home and got onto the internet. Participant 002CA



I think pretty good. I think it's pretty good, the treatment has got the light chain down to just a little bit above normal, the high range of normal. It's gone from being through the roof down to high range normal. Participant 004ATR

They did an ultrasound recently-- not recently, probably six months ago, and they said that it didn't seem to be much worse, and the heart didn't seem to be much worse than what it was. I'm just taking the medication apparently with the specialist in the amyloid clinic in LOCATION METROPOLITAIN, they had agreed that I can't take anything else or do anything else at this point in time until some new medication comes along. Participant 008ATR

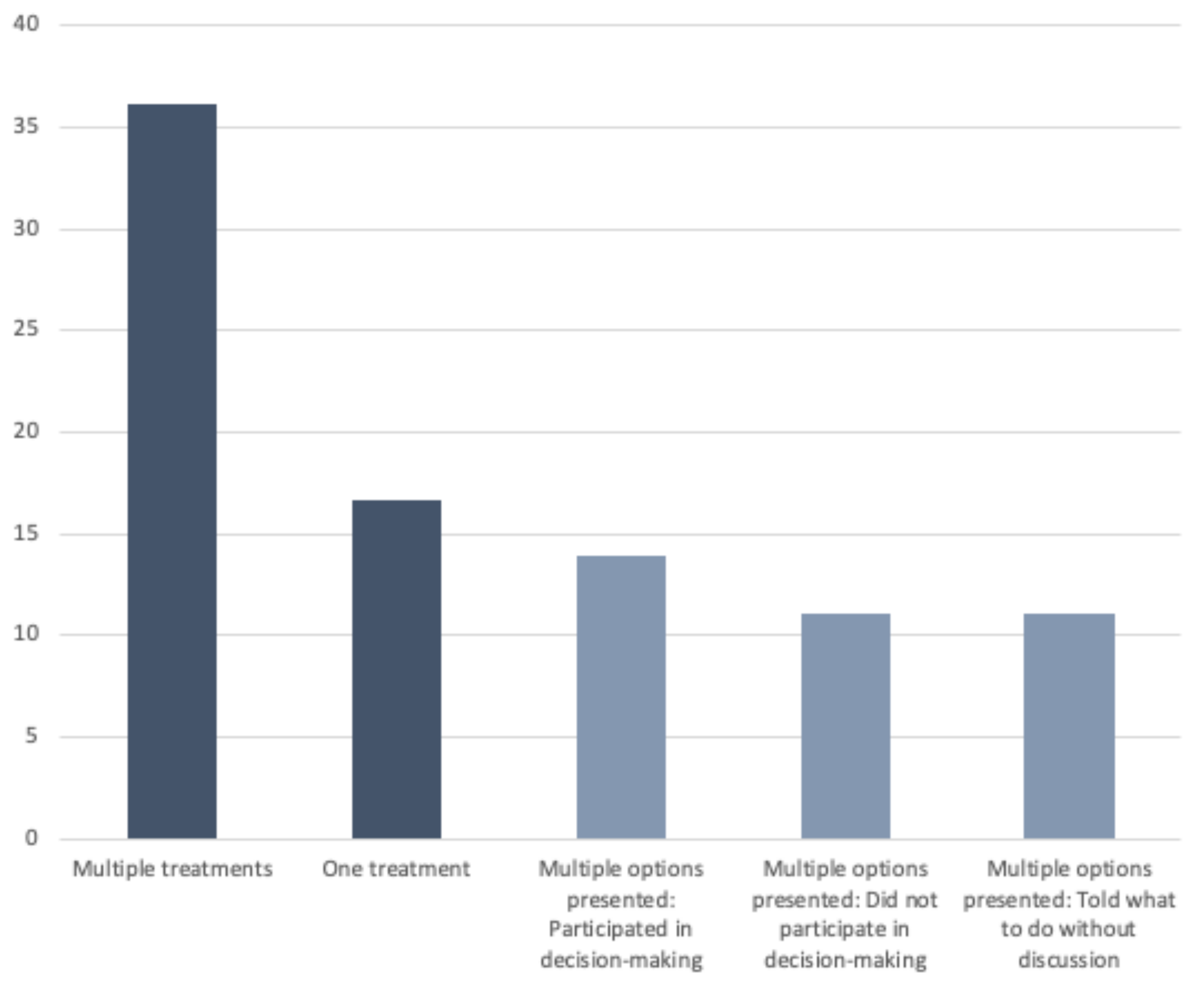
They say that I am in, haematological remission which means my light chains have stayed down and that's over a year since I finished my chemotherapy. Participant 012ATR

Understanding of prognosis (% all)

Section 4

Decision Making

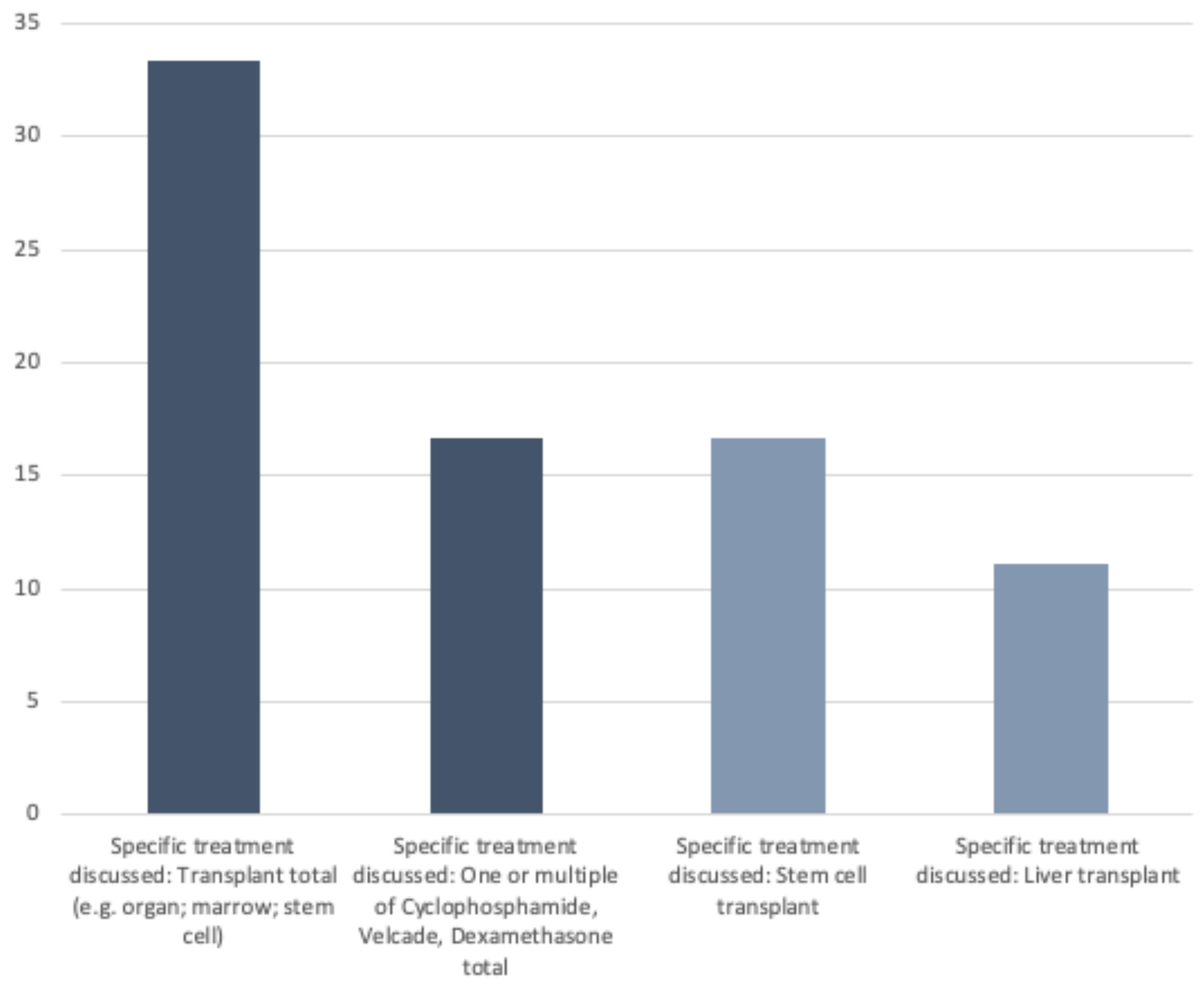
In the decision making section, you can find information about the conversations that were had about treatment options at diagnosis, what is important to people when they make decisions about treatment, and whether decision-making changes over time.



Well, I was in his hands. He told me what he went through which was generally, oral chemo, et cetera. That was it. Then suddenly, well, that didn't work. On one particular visit, "We got to do plan B," and then he explained to me all about the stem cell transplants. Participant 001ALX

He did suggest that a bone marrow transplant could probably fix the problem, but the pre-treatment would probably kill me. We decided not to go with that. Apart from that, he didn't really talk much about it. He put me on to oral chemo and dexamethasone on the grounds that my system at that stage was so bad that he didn't think I could've taken intravenous chemo. Participant 2ALX

Discussions about treatment (% all)



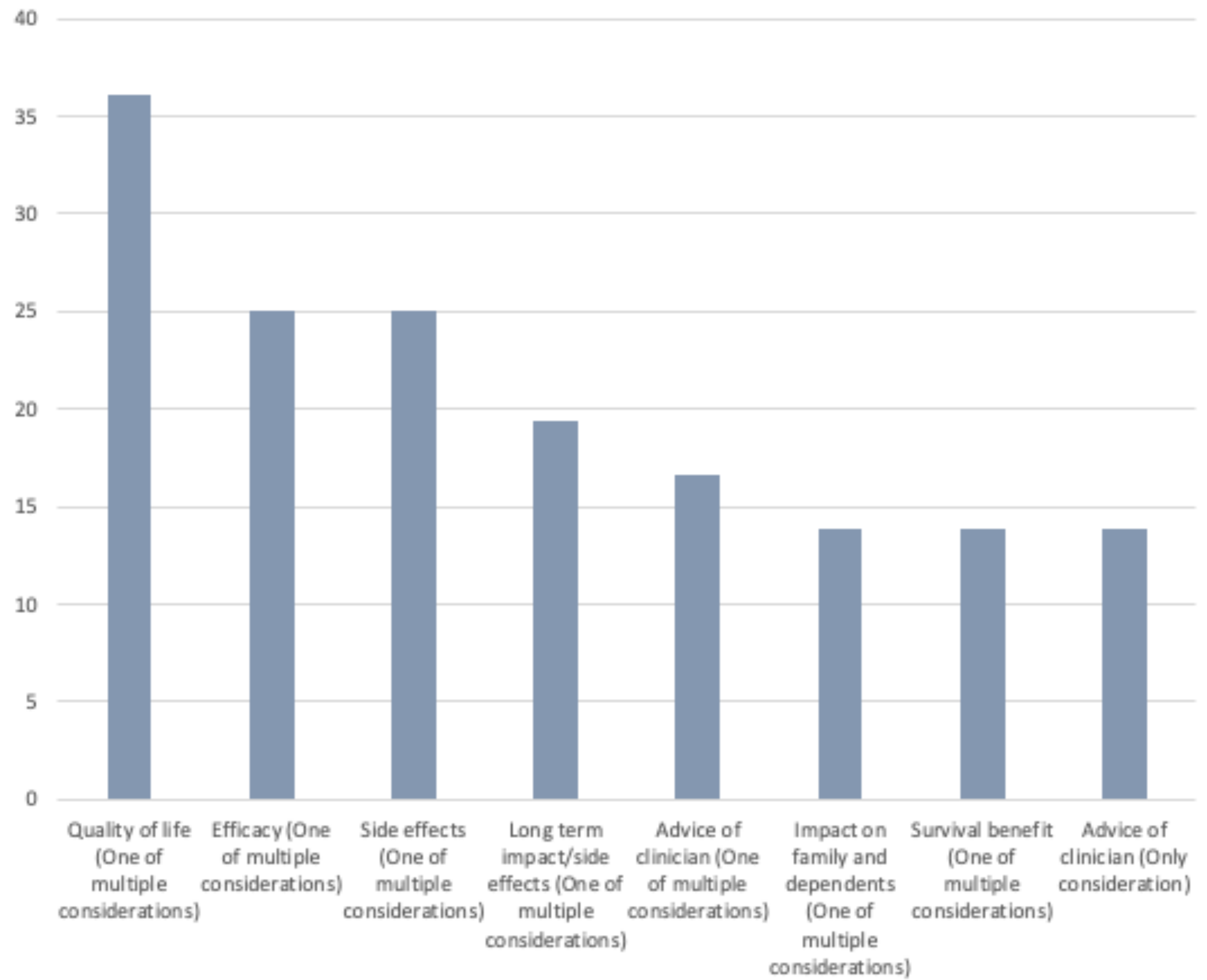
He put me on that combination just for a couple of months just to check that they were affecting the free light chains, and they did, and then we just scheduled the stem cell transplant. Participant 002AL

The second time, as I was put on that treatment, I was assessed for a liver and a heart transplant because this protein is produced by the liver. It produces trans-direction and it converts it into this amyloid. One treatment or intervention was to-- And the heart, I should say, is the key organ. Participant 016ATR

The discussions was basically, "No, there's nothing there that you can do. Nothing at all." I had my lab biopsy done the same day as the guy who ended up, that same day, being diagnosed with AL type. He had heart transplants and chemo. He's had a full stem cell transplant. Now he's set up about five years of extra life that he can live for. Participant 013ATR

Discussions about treatment (% all)

Considerations with treatment (% all)

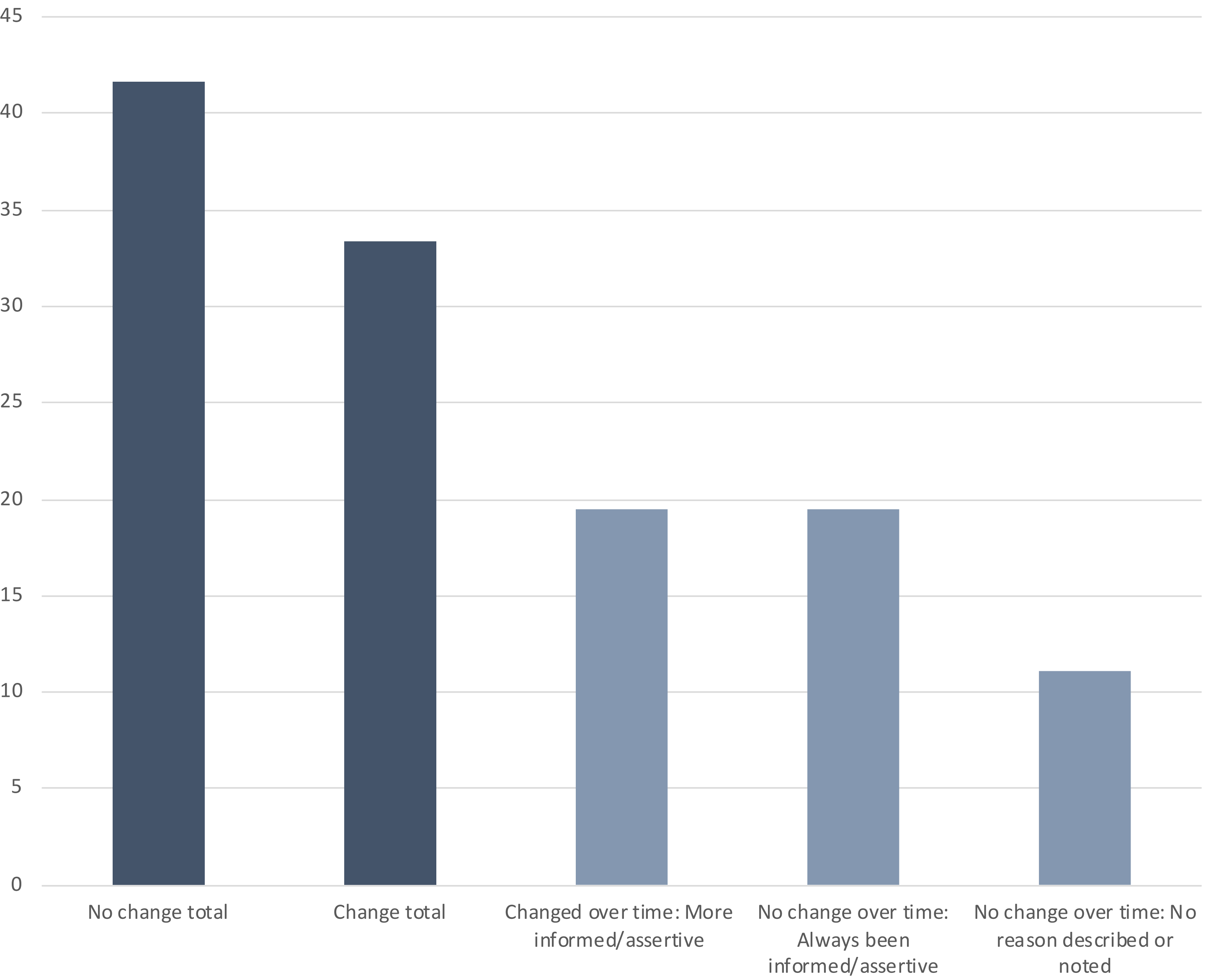


My decisions about treatment are pretty much based on the quality of life and the effect of the treatment. That's probably the same thing, isn't it? I mean, quality of life and the treatment and what the actual amyloidosis does. Participant 001AL

Quality of life, as we get further along in the journey, the quality of life balance I think is really important. A few times, he's been losing track now because we've just gone back on chemo again as of last Friday. Participant 002CA

I take a logical approach being in PROFESSION. I list the benefits and the risks and what the outcome. The outcome is to live longer. The outcome is to live with the quality of life and not be restricted to our bed or our medication and the ongoing care. Participant 002ATR

Centre for Community-Driven Research



I think I probably make decisions in the same kind of way because I've always wanted to be informed, I guess. I like to make informed choices and weigh things up. I like to understand what the treatments are doing and how they work, but that's probably just assigned to me really. Participant 002AL

As much as the same way as I've always done. Amyloidosis isn't the first health scare I had. Participant 002ALX

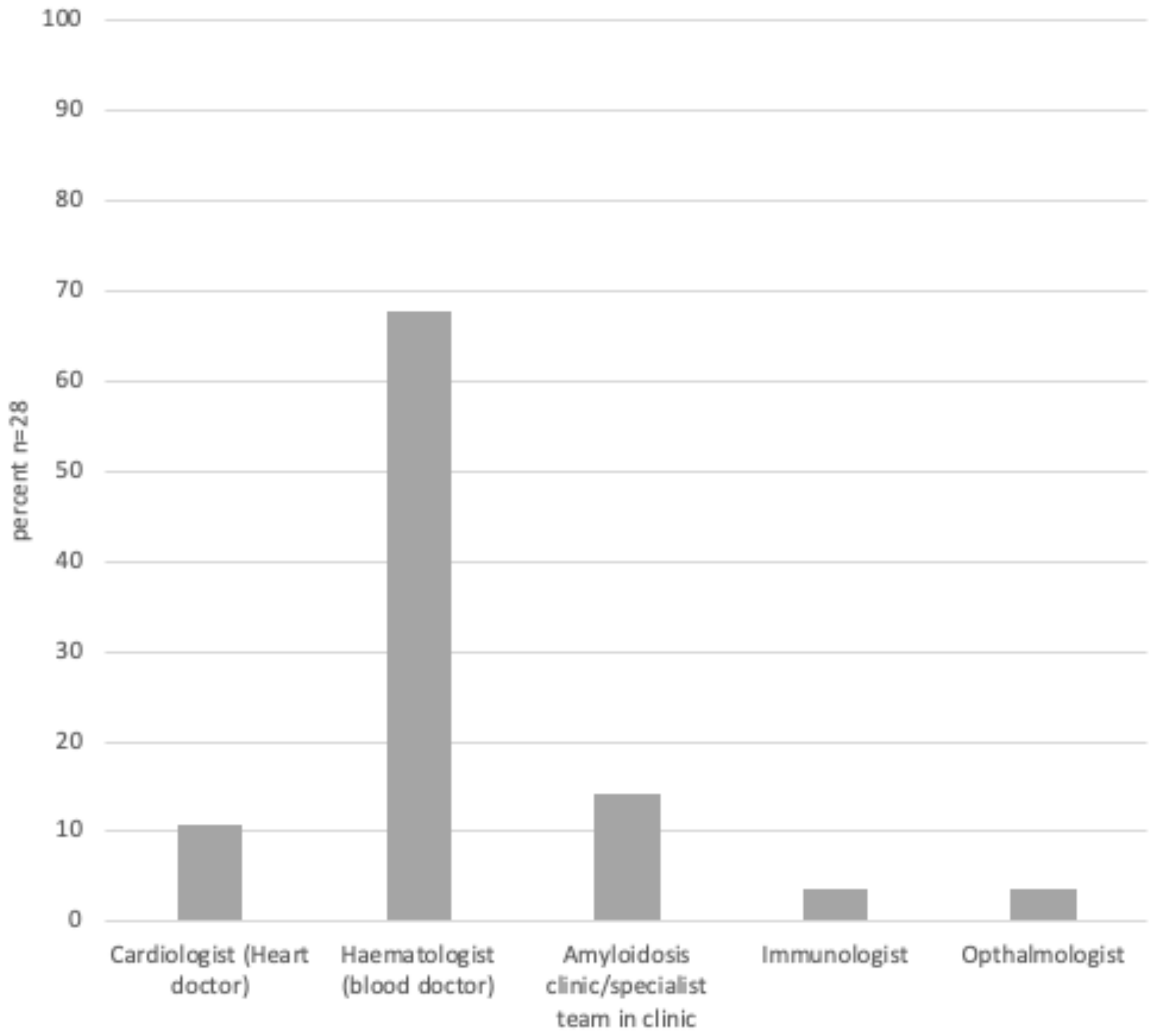
Oh, pretty well the same. NAME HUSBAND has always been a very independent person, was late marrying, late becoming a father. I think one of the biggest impacts of this disease was all of a sudden, he had no control. I don't know whether he would say that. I don't think he would say that, but he'd always been in control of his life. Participant 002CA

Does decision-making change over time (% all)

Section 5

Treatment

The treatment section is one of the largest sections in each study. Information is available in relation to the types of treatments people have experienced as well as rating quality of life and effectiveness for each treatment. There is information about the main providers of treatment, access to health professionals and information about which part of the health system participants were treated in. There is information about affordability of healthcare, any reduced income for the patient or their family, an additional costs or changes to employment. There is then also detailed information about the treatments that the participants have experienced, whether they had any side effects, their description of mild side effects and severe side effects, and any complementary therapies that they may have used. Information in relation to access to allied health is also available. Information about what needs to change to feel like treatment is effective, and adherence to treatment is also presented. In this section, there is also information on whether discussions about clinical trials were held with clinicians, and for those who have been on a trial, what their experience was like.



Treated with respect during experience in the health system?

Yes
86% (n=31)

Yes, with the exception of the odd occasion
14% (n=5)

No
0% (n=0)

Centre for Community-Driven Research

Main provider of treatment & respect shown

Healthcare professional	Number	Percent
General Practitioner	28	100.00
Cardiologist (Heart doctor)	26	92.86
Haematologist (blood doctor)	24	85.71
Pharmacist	14	50.00
Gastroenterologist	12	42.86
Neurologist (nerve doctor)	10	35.71
Nephrologist (kidney doctor)	8	28.57
Dietician/nutritionist	7	25.00
Chiropractor	6	21.43
Exercise physiologist	5	17.86
Physiotherapist	5	17.86
Psychologist	3	10.71
Specialist nurse or Care coordination nurse (in addition to general ward nurse)	3	10.71
Occupational therapist	2	7.14
Osteopath	2	7.14
Social worker	2	7.14
Complementary therapist	2	7.14
Counsellor	1	3.57
Genetic Counsellor	1	3.57
Immunologists	1	3.57
Ophthalmologist	1	3.57
Podiatrist	1	3.57
Urologist	1	3.57
Weight loss specialist	1	3.57

Access to healthcare professionals

Health services and insurance	Response	Number (n=28)	Percent
Private health insurance	No	5	17.86
	Yes	23	82.14
Asked whether you want to be treated as a public or private patient	No	23	82.14
	Yes	5	17.86
Asked whether you had private health insurance	No	15	53.57
	Yes	13	46.43
Throughout your treatment in hospital, have you most been treated as a public or a private patient	Equally as a public and private patient	4	14.29
	I'm not sure	2	7.14
	Private patient	11	39.29
	Public patient	11	39.29
Which hospital system have you primarily been treated in	Both public and private	9	32.14
	Private	6	21.43
	Public	13	46.43

Delay or cancel healthcare appointments due to affordability	Number (n=28)	Percent
Never	23	82.14
Rarely	4	14.29
Sometimes	1	3.57
Often	0	0.00
Very often	0	0.00

Did not fill prescriptions due to cost	Number (n=28)	Percent
Never	27	96.43
Rarely	0	0.00
Sometimes	1	3.57
Often	0	0.00
Very often	0	0.00

Difficult to pay for basic necessities such as housing, food, electricity	Number (n=28)	Percent
Never	23	82.14
Rarely	2	7.14
Sometimes	2	7.14
Often	0	0.00
Very often	1	3.57

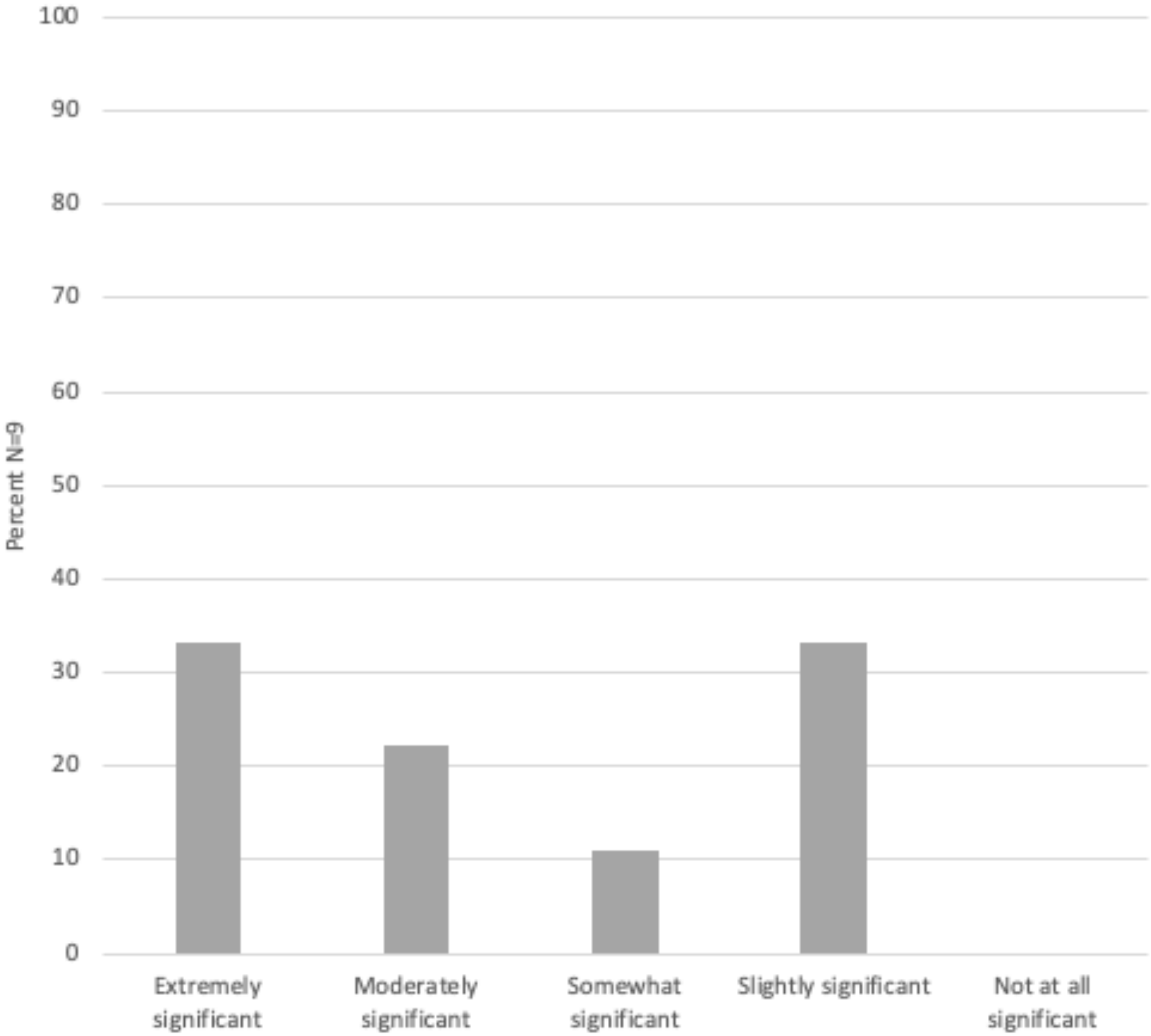
As a result of condition, had to pay for additional carers for self or family	Number (n=28)	Percent
No	26	92.86
Yes	2	7.14

Affordability of healthcare

Reduced income (self or family)	Number (n=28)	Percent
No	19	67.86
Yes	9	32.14

Burden of reduced income	Number (n=9)	Percent
Extremely significant	3	33.33
Moderately significant	2	22.22
Somewhat significant	1	11.11
Slightly significant	3	33.33
Not at all significant	0	0.00

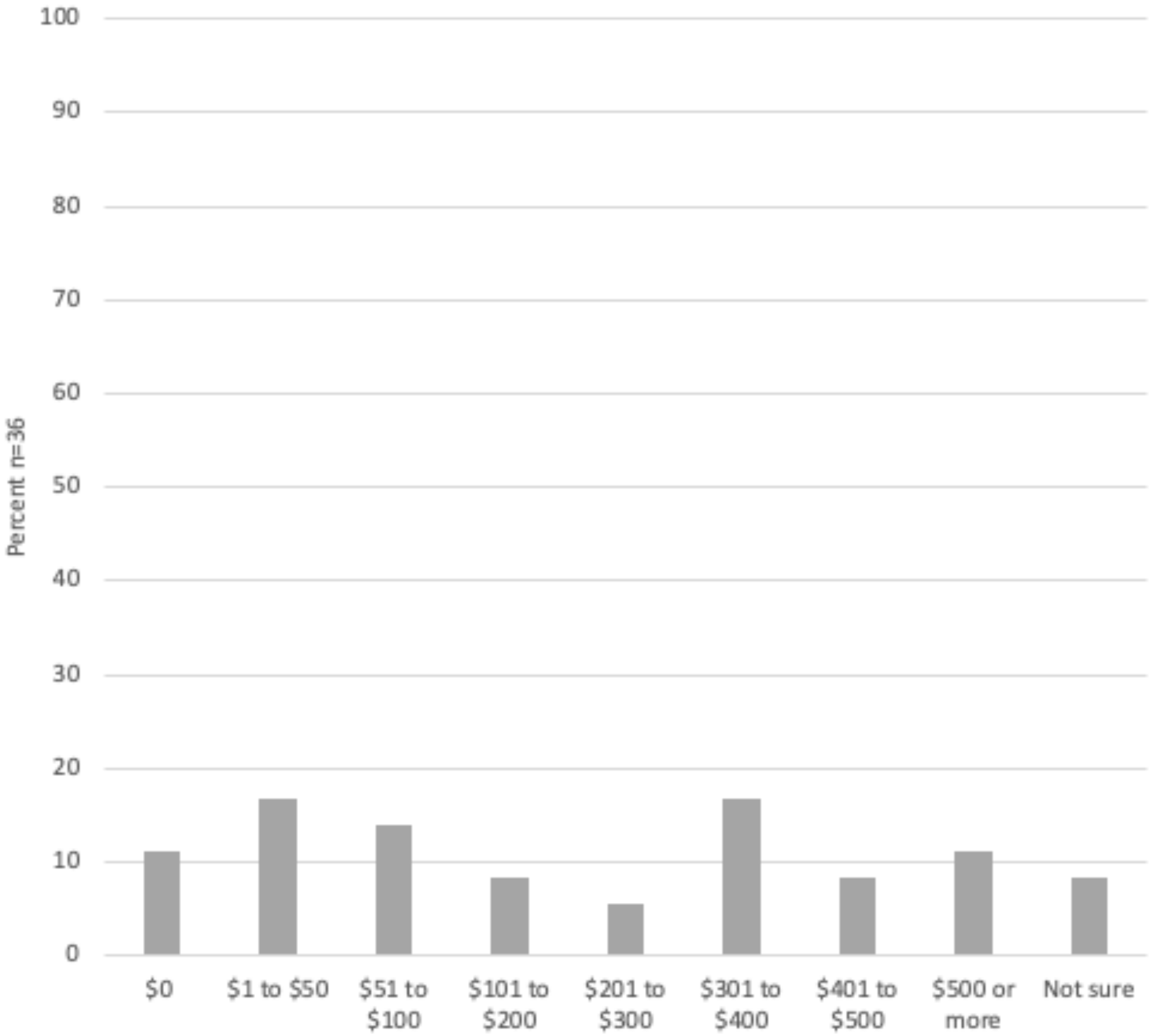
Estimated monthly loss of income	Number (n=13)	Percent
$1 to $100	1	7.69
$1000 to $2000	3	23.08
$2001 to $5000	4	30.77
More than $5001	3	23.08
Not sure	2	15.38



Impact on income

Estimated monthly out of pocket expenses	Number (n=36)	Percent
$0	4	11.11
$1 to $50	6	16.67
$51 to $100	5	13.89
$101 to $200	3	8.33
$201 to $300	2	5.56
$301 to $400	6	16.67
$401 to $500	3	8.33
$500 or more	4	11.11
Not sure	3	8.33

Estimated monthly out of pocket expenses	Number (n=28)	Percent
Extremely significant	2	7.14
Moderately significant	2	7.14
Somewhat significant	5	17.86
Slightly significant	7	25.00
Not at all significant	12	42.86



Cost of disease

Changes in work status due to condition	Number (n=36)	Percent
My work status has not changed	8	22.22
I was retired or did not have a job	18	50.00
I have had to quit my job	6	16.67
I have reduced the number of hours that I work	4	11.11
I have taken leave from work without pay	1	2.78
I have taken leave from work with pay	2	5.56
I have accessed my Superannuation early due to my condition	1	2.78

Changes to partner/main carer work status due to condition	Number (n=28)	Percent
I do not have a partner/main carer	3	10.71
My partner/main carer was retired or did not have a job when I was diagnosed	13	46.43
The employment status of my partner/main carer status has not changed since I was diagnosed	7	25.00
My partner/main carer had to quit their job	3	10.71
My partner/main carer reduced the number of hours that they work	2	7.14
My partner/main carer took leave from work with pay	1	3.57

Changes in work status

Treatment	Number	Percent	Status	Median QOL	Median effectiveness
Tafamidis	2	11.11	Stopped early (1) Treatment ongoing (1)	3 Life was a little distressing	2 Somewhat effective
Patisiran	1	5.56	Treatment ongoing (1)	3 Life was a little distressing	4 Effective
Diflunisal	5	27.78	Treatment ongoing (4) Completed as planned (1)	4 Life was average	3 Moderately effective
Doxycycline	7	38.89	Treatment ongoing (5) Completed as planned (1) Not specified (1)	3 Life was a little distressing	4 Effective
Loop-acting diuretics (e.g. bumetanide, etacrynic acid, furosemide)	8	44.44	Treatment ongoing (6) Completed as planned (1) Not Started yet (1)	3.5 Life was a little distressing - average	4 Effective
Potassium-sparing diuretics (e.g. spironolactone, amiloride)	2	11.11	Treatment ongoing (4) Completed as planned (1	2.5 Life was distressing - a little distressing	4 Effective

ATTR treatment

Treatment summary	Tafamidis	Patisiran	Diflunisal	Doxycycline	Loop-acting diuretics	Potassium-sparing diuretics
Number (n=18)	2	1	n=5	n = 7	n=8	n=2
Percent	11.11	5.56	27.78	38.89	44.44	11.11
Treatment status	Stopped early (1) Treatment ongoing (1)	Treatment ongoing (1)	Treatment ongoing (4) Completed as planned (1)	Treatment ongoing (5) Completed as planned (1) Not specified (1)	Treatment ongoing (6) Completed as planned (1) Not Started yet (1)	Treatment ongoing (4) Completed as planned (1)
Median quality of life	3 Life was a little distressing	3 Life was a little distressing	4 Life was average	3 Life was a little distressing	3.5 Life was a little distressing - average	2.5 Life was distressing - a little distressing
Median effectiveness	2 Somewhat effective	4 Effective	3 Moderately effective	4 Effective	4 Effective	4 Effective
No side effects	2	1	4	1	3	0
Gas/bloating	0	0	1	0	0	0
Loss of appetite or taste sensation	0	0	0	4	0	0
Difficulty or pain when swallowing	0	0	0	2	0	0
Diarrhoea	0	0	0	1	0	0
Oral	0	0	0	1	0	0
Sore mouth or tongue	0	0	0	1	0	0
Tooth discolouration, changes in tooth enamel	0	0	0	1	0	0
Hives	0	0	0	1	0	0
Fatigue	0	0	0	1	1	1
Nail changes	0	0	0	1	0	0
Sensitivity to the sun	0	0	0	3	0	0
Nausea and vomiting	0	0	0	1	0	0
Feeling faint or dizzy, especially on standing up	0	0	0	0	4	2
Thirst	0	0	0	0	3	2
Rash	0	0	0	0	1	0
Diarrhoea	0	0	0	0	1	0
Low blood potassium	0	0	0	0	1	1
Headache	0	0	0	0	1	1
Increased cholesterol	0	0	0	0	1	1

ATTR treatment

Treatment	Number	Percent	Status	Median QOL	Median effectiveness
Melphalan and Dexamethasone	5	50.00	Treatment ongoing (2) Treatment stopped early (1) Treatment completed as planned (2)	2 Life was distressing	4 Effective
Cyclophosphamide, Thalidomide and Dexamethasone	4	40.00	Treatment ongoing (1) Treatment stopped early (2) Treatment completed as planned (1)	3 Life was a little distressing	2.5 Somewhat to moderately effective
Lenalidomide and Dexamethasone	3	30.00	Treatment ongoing (1) Treatment stopped early (2)	2 Life was distressing	2 Somewhat effective
Melphalan, Bortezomib, and Dexamethasone	1	10.00	Yes, completed as planned (1)	2 Life was distressing	3 Moderately effective
Pomalidomide and Dexamethasone	1	10.00	Treatment ongoing (1)	5 Life was good	4 Effective
Bortezomib, Cyclophosphamide, Dexamethasone	5	50.00	Treatment ongoing (2) Treatment stopped early (1) Treatment completed as planned (2)	3 Life was a little distressing	3 Moderately effective
Dexamethasone and Rituximab	1	10.00	Treatment stopped early (1)	2 Life was distressing	2 Somewhat effective
Autologous stem cell	2	20.00	Treatment completed as planned (2)	2.5 Life was distressing to a little distressing	5 Very effective

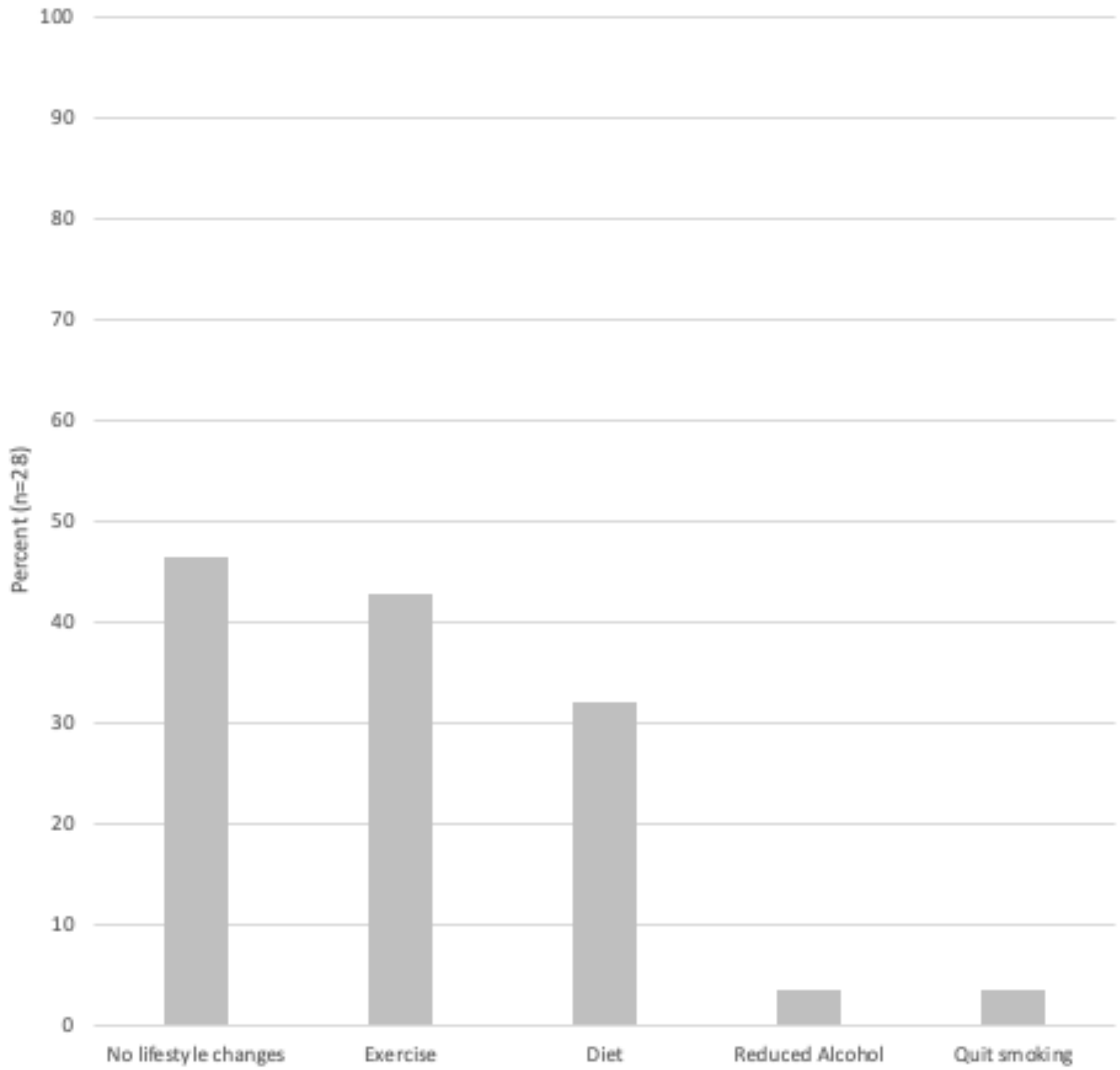
AL treatment

Treatment summary	Melphalan and Dexamethasone	Cyclophosphamide, Thalidomide and Dexamethasone	Lenalidomide and Dexamethasone	Melphalan, Bortezomib, and Dexamethasone	Pomalidomide and Dexamethasone	Bortezomib, Cyclophosphamide, Dexamethasone	Dexamethasone and Rituximab	Autologous stem cell
Number (n=10)	5	4	3	1	1	5	1	2
Percent	50.00	40.00	30.00	10.00	10.00	50.00	10.00	20.00
Treatment status	Ongoing (2) Stopped early (1) Completed as planned (2)	Ongoing (1) Stopped early (2) Completed as planned (1)	Ongoing (1) Stopped early (2)	Completed as planned (1)	Ongoing (1)	Ongoing (2) Stopped early (1) Completed as planned (2)	Stopped early (1)	Completed as planned (2)
Median quality of life	2 Life was distressing	3 Life was a little distressing	2 Life was distressing	2 Life was distressing	5 Life was good	3 Life was a little distressing	2 Life was distressing	2.5 Life was distressing to a little distressing
Median effectiveness	4 Effective	2.5 Somewhat to moderately effective	2 Somewhat effective	3 moderately effective	4 Effective	3 moderately effective	2 Somewhat effective	5 Very effective
No side effects	0	0	0	0	0	0	0	0
Infection risk/neutropenia	4	1	3	0	1	3	1	1
Fatigue	4	3	3	1	0	5	1	2
Joint or muscle pain	3	1	2	1	0	2	0	0
Low platelets	1	1	1	1	0	1	0	0
Hair loss,	2	1	1	1	0	2	0	1
Anaemia	2	1	2	1	0	2	0	0
Mood swings	3	3	2	1	0	2	1	0
Swelling in your hands and feet	1	2	1	0	0	1	0	0
Trouble sleeping	3	1	1	1	0	2	1	0
Constipation	2	1	3	0	1	4	1	0
Numbness or tingling in fingers and toes	3	1	1	1	0	2	0	0
Dizziness or light-headed	4	2	2	1	0	3	0	0
Skin rash	1	1	2	1	0	2	0	0
Changes in taste and smell	3	1	2	1	0	0	0	0
Fever or chills	2	0	1	1	0	1	0	0
Nausea or vomiting	2	1	1	1	0	0	0	1
Headache	2	1	1	1	0	1	0	0
Diarrhoea	1	0	0	1	0	0	0	0
Heartburn	1	0	1	0	0	1	0	0
Loss of appetite	0	0	0	0	0	0	0	2

AL treatment

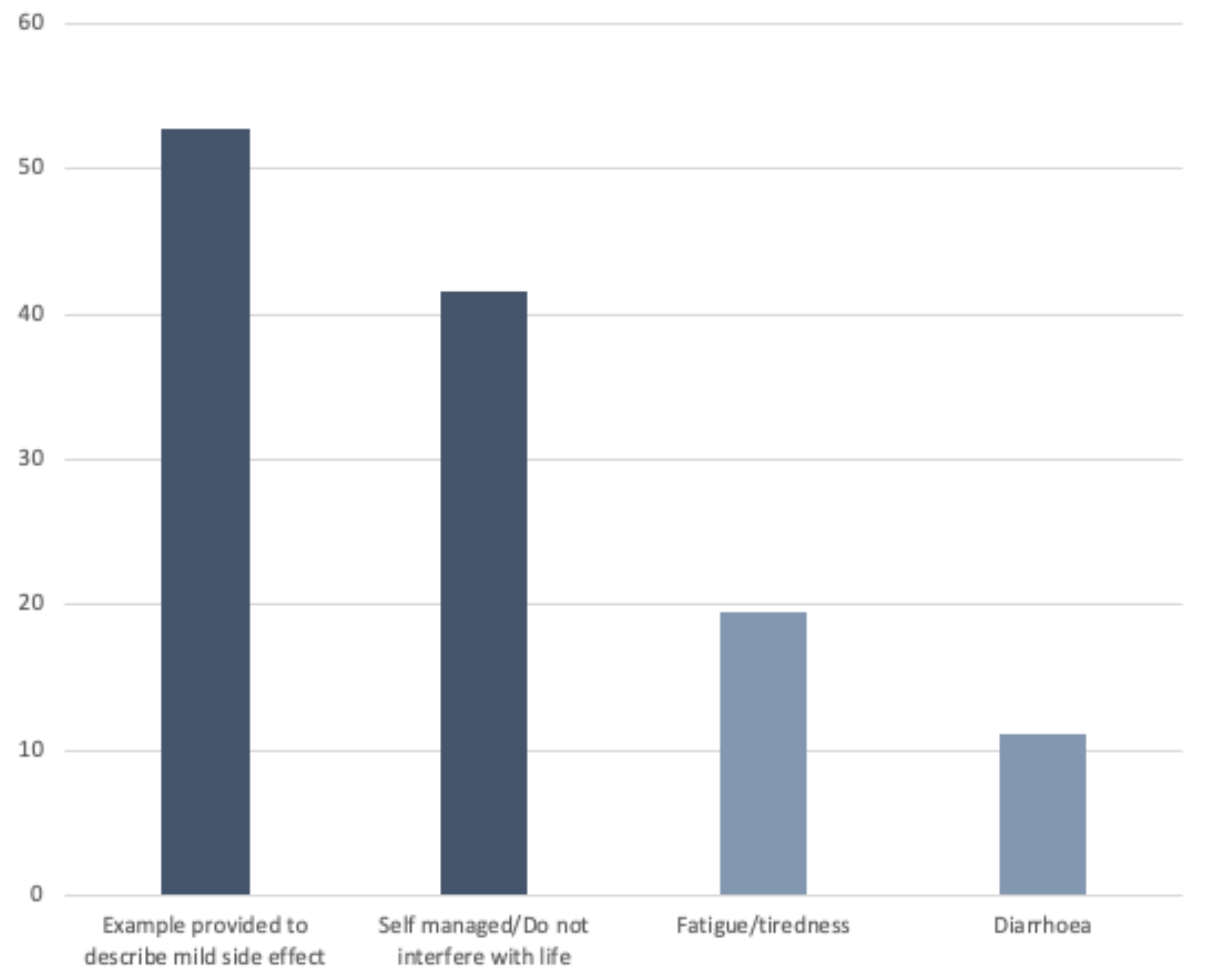
Surgery overview	Detail	Number
Number had surgery		5
Number of surgeries per participant	1 surgery	4
	4 or more	1
Type of surgery	Liver transplant	1
	Pacemaker	2
	Defibrillator fitted	1
	Carpal tunnel surgery	1

Lifestyle changes	Number (n=28)	Percent
No lifestyle changes	13	46.43
Exercise	12	42.86
Diet	9	32.14
Reduced Alcohol	1	3.57
Quit smoking	1	3.57



Lifestyle changes

Complementary therapies	Massage therapy	Acupuncture	Exercise	Supplements	Mindfulness or relaxation	Homeopathy	Naturopathy	Diet (fluid intake steady/limited salt intake (ATTR only n=18)	Daily weigh (ATTR only n=18)
Number (n=28)	6	2	18	13	7	1	1	8	9
Percent	21.43	7.14	64.29	46.43	25.00	3.57	3.57	44.44	50.00
Median quality of life	3.5 Life was a little distressing to average	4.5 Life was average to good	4.5 Life was average to good	3 Life was a little distressing	4 Life was average	4 Life was average	5 Life was good	3.5 Life was a little distressing to average	4 Life was average
Median effectiveness	4 Effective	3.5 Moderately effective to effective	3 Moderately effective	2 Somewhat effective	3 Moderately effective	2 Somewhat effective	2 Somewhat effective	3 Moderately effective	3 Moderately effective

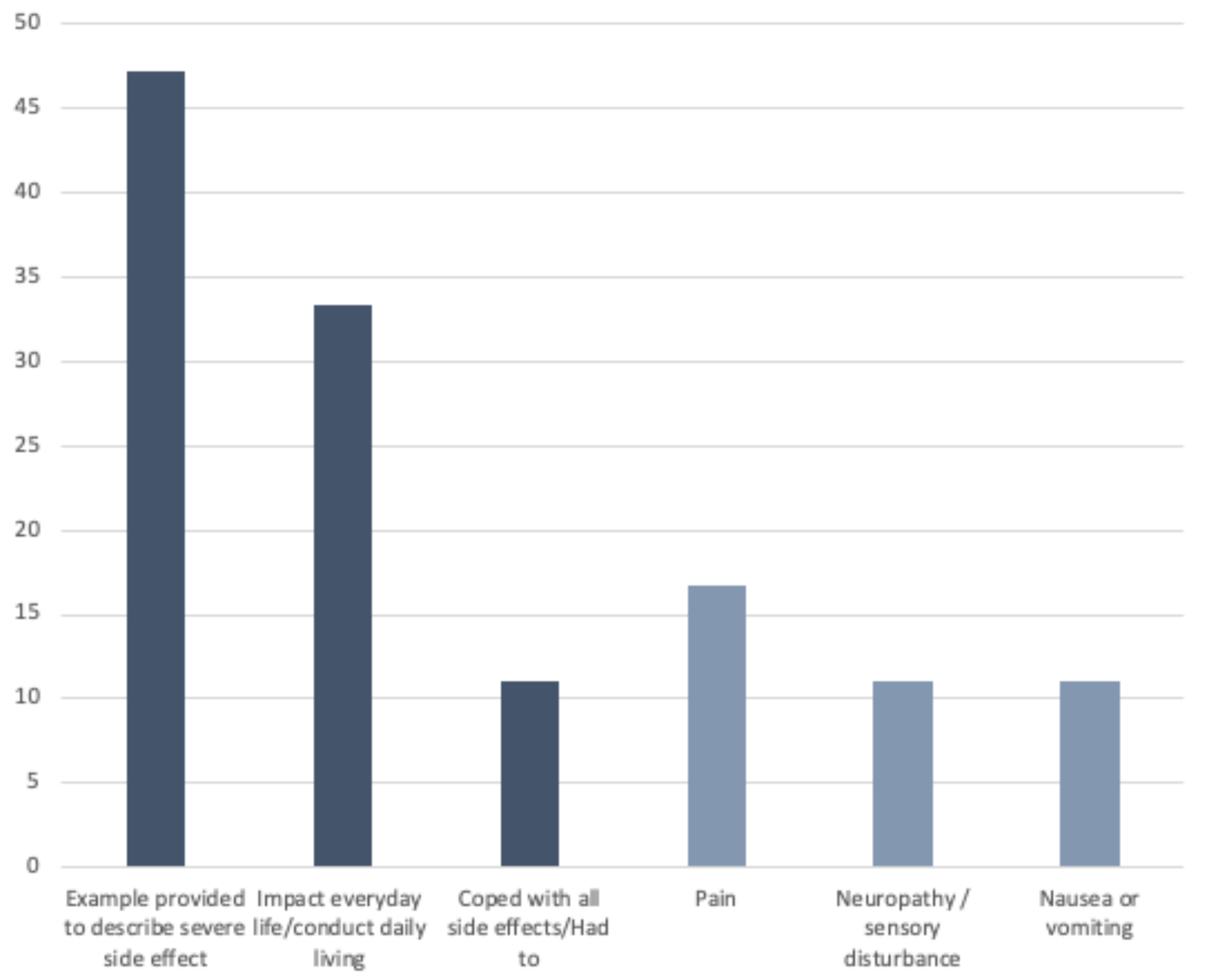


Mild side effects is probably tiredness because as soon as you lie down, as a mild side effect immediately, you feel better and I think with that, it has a bit to do with the blood pressure which then goes up and then I might sleep for half an hour or an hour or something and you get up and it's okay. I'd say in the mild effects, I'd say there's fatigue, a little bit of tiredness. Participant 001AL

A headache maybe, a bit of nausea, maybe a bit of constipation, just something that you wouldn't normally have in everyday living basically whereas for severe ones would be like absolutely ill, really, really ill. Participant 001ATR

It's a bit like the dizziness from one of those drugs as I've-- Combined with the heart, you know you just can't leap up out of the chair. There's no way in the world that you're going to go walking up a mountain or those sort of things. Participant 003ATR

Description of mild side effects (% all)



Severe? Well, I have had quite severe gut pain, and knotty gut and sitting on the toilet for an hour and those sort of things. I have-- What do you call it? The skin-- paraesthesia and that's on the chest. Again, that ranges from mild to sometimes quite severe in the sense that it's like jabs. It feels like jabs in the chest, but I've had that virtually all the time. It's like an itchy and stabbing skin thing. Participant 001AL

He was on, I mentioned, dexamethasone. At one stage, I was ready to divorce him because it actually changed his personality. Participant 003CA

When they get to a joint like a knee, mainly my knees, where the arteries and veins narrow, they dam up, and I got the most tremendous pain in my knees, hospitalised, couldn't move, couldn't stand up, couldn't do anything. That was severe pain. Participant 005AL

Description of severe side effects (% all)

Descriptions of specific side effects (Mild)

Just means that by mid-afternoon I have tendency to curl up in a ball on the couch and go to sleep. Yes. Participant 004ATR

For me, for example, a mild side effect is I can walk up a flight of stairs but when I get to the top, I'll just literally stop for two or three seconds and then carry on doing what I'm doing. Participant 015ATR

Well, as you described, things that I can cope with, day-to-day living, limitations on what I can do, tiredness. Participant 006AL

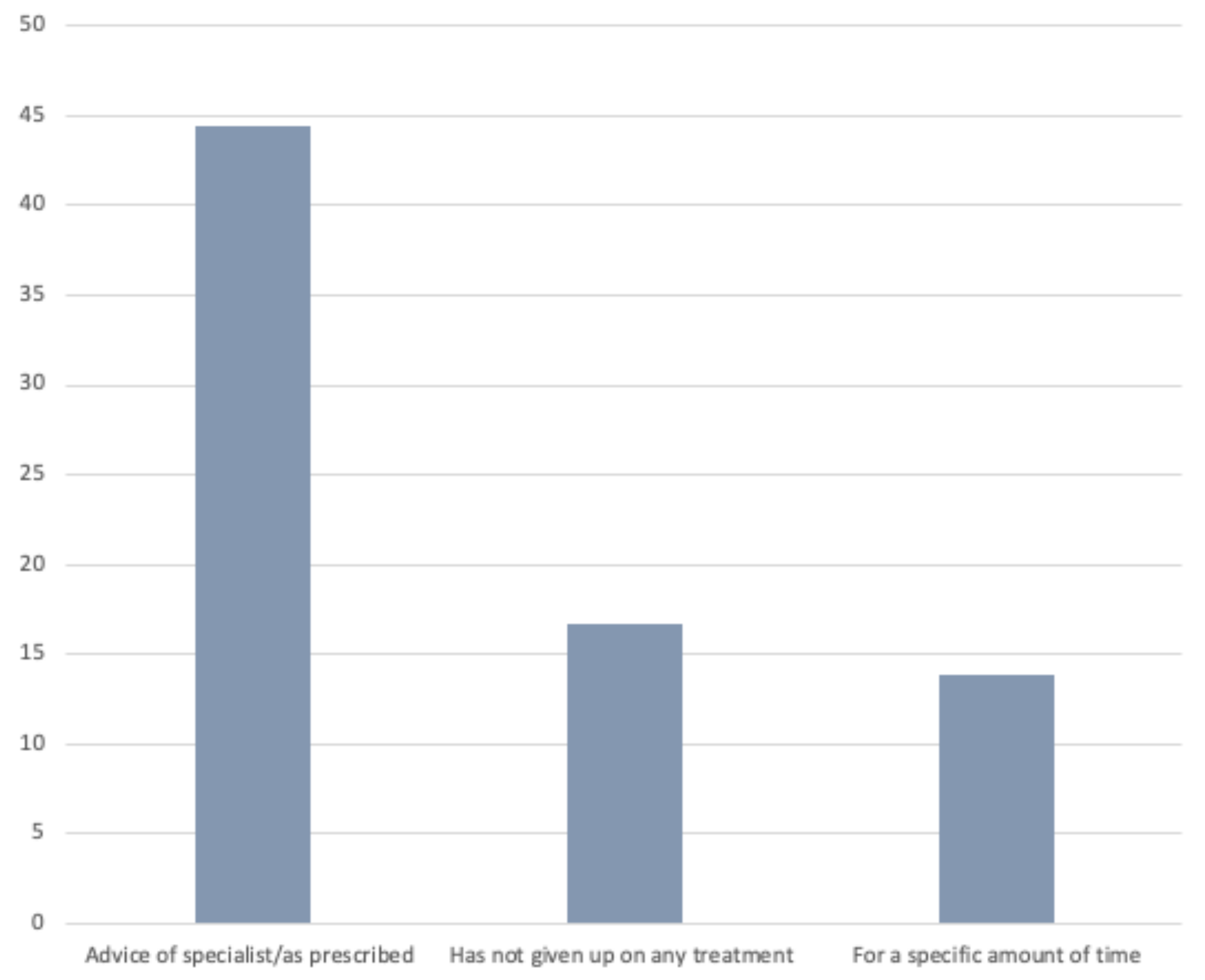
Descriptions of specific side effects (Severe)

That might mean seeking help whether it's for like the mental health side of things or relief for sickness or diarrhoea or pain. Participant 002AL

Side effects like very bad pain in your body, various places. Participant 003AL

The severe side effects where it definitely compromises your life to some degree. That would be painful or it compromised a particular bodily function, so pain plus loss of function. Participant 004AL

Side effect descriptions



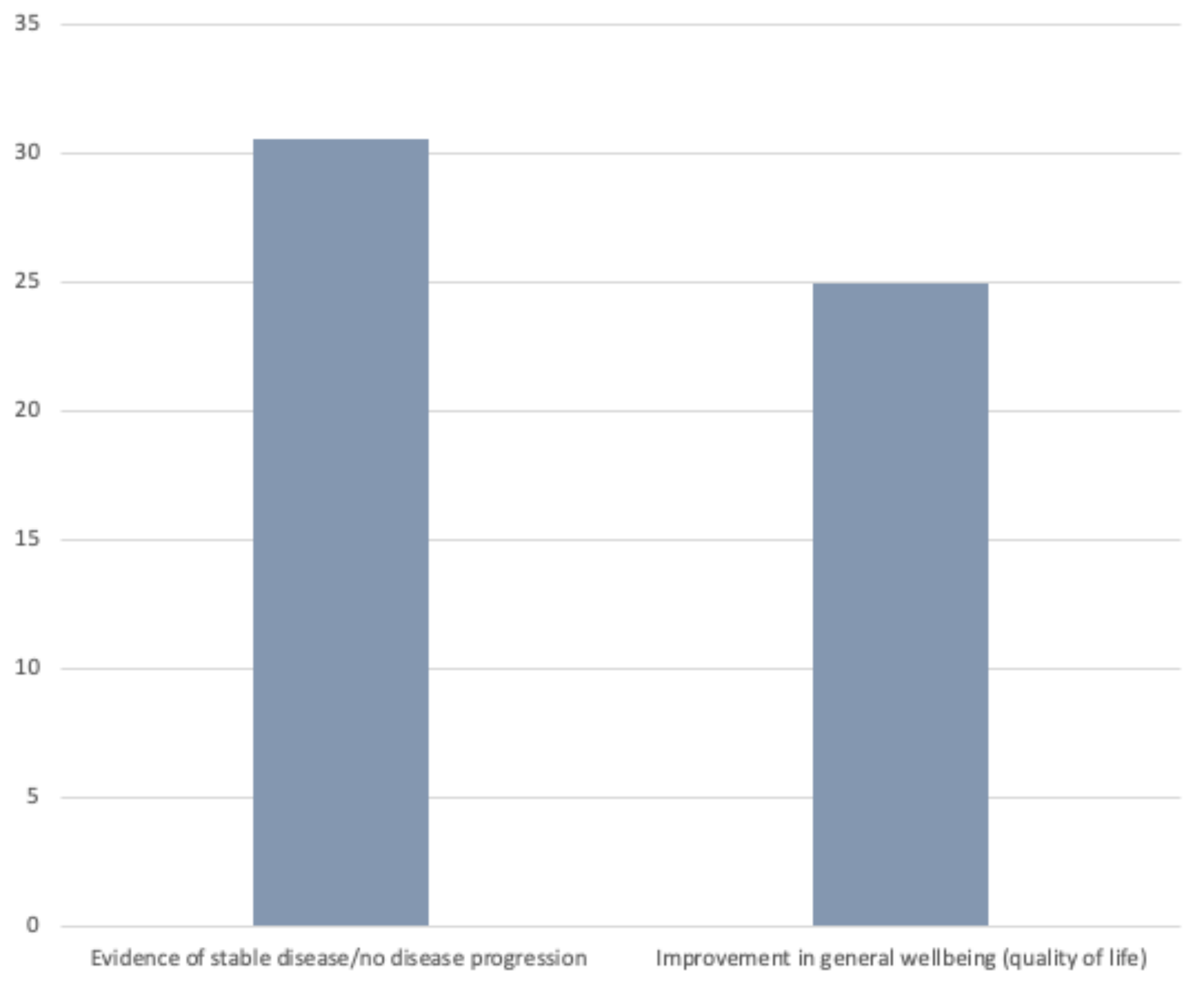
I'm guided by the time advised, but I've always been happy to continue even though you don't feel all that flash, on the basis that it has been advised to at least to go a couple of months to see what it's like. Participant 001AL

I'm again guided by the physician. In my particular case, I'd go in and explain the side effects, we try something different, I go in again. Eventually, he decided this was enough, we can't go any further. We're going to try something else. Participant 001ALX

Well, I would stick with it basically and discuss it with-- I wouldn't make the decision by myself I would discuss it with my practitioners. No, I'm not a self-prescriber or self-treater. I work with the people who have greater knowledge and skills than I do. Participant 001ATR

Adherence to treatment (% all)

Centre for Community-Driven Research



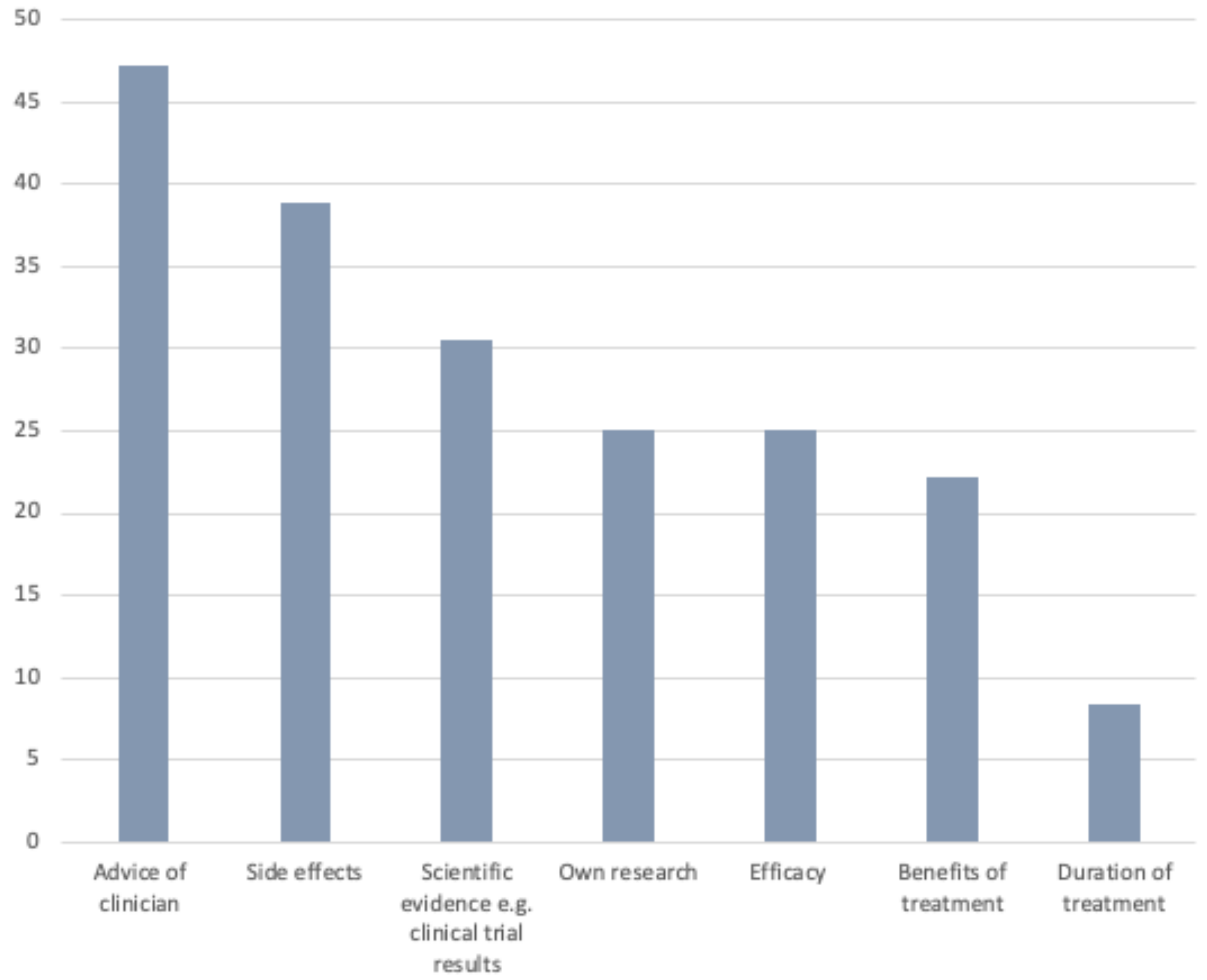
You feel better. Some of these questions I hear them often, but they're very difficult to answer because we're guided by what our doctors tell us and we don't have any knowledge ourselves to be able to decide if yes, yes, oh, this is good or bad. We're guided by what the doctor tells us. Participant 001ALX

It has to actually alter the condition for which it's being prescribed, which in the case of the amyloid and test too, you have to see an improvement in the various blood tests in that and in general health, and things like antibiotics, you think the problem has to go away or it hasn't been successful. Participant 002ALX

If the echocardiogram and the blood test show that this condition stabilised. Participant 011ATR

What needs to change to feel treatment is effective (% all)

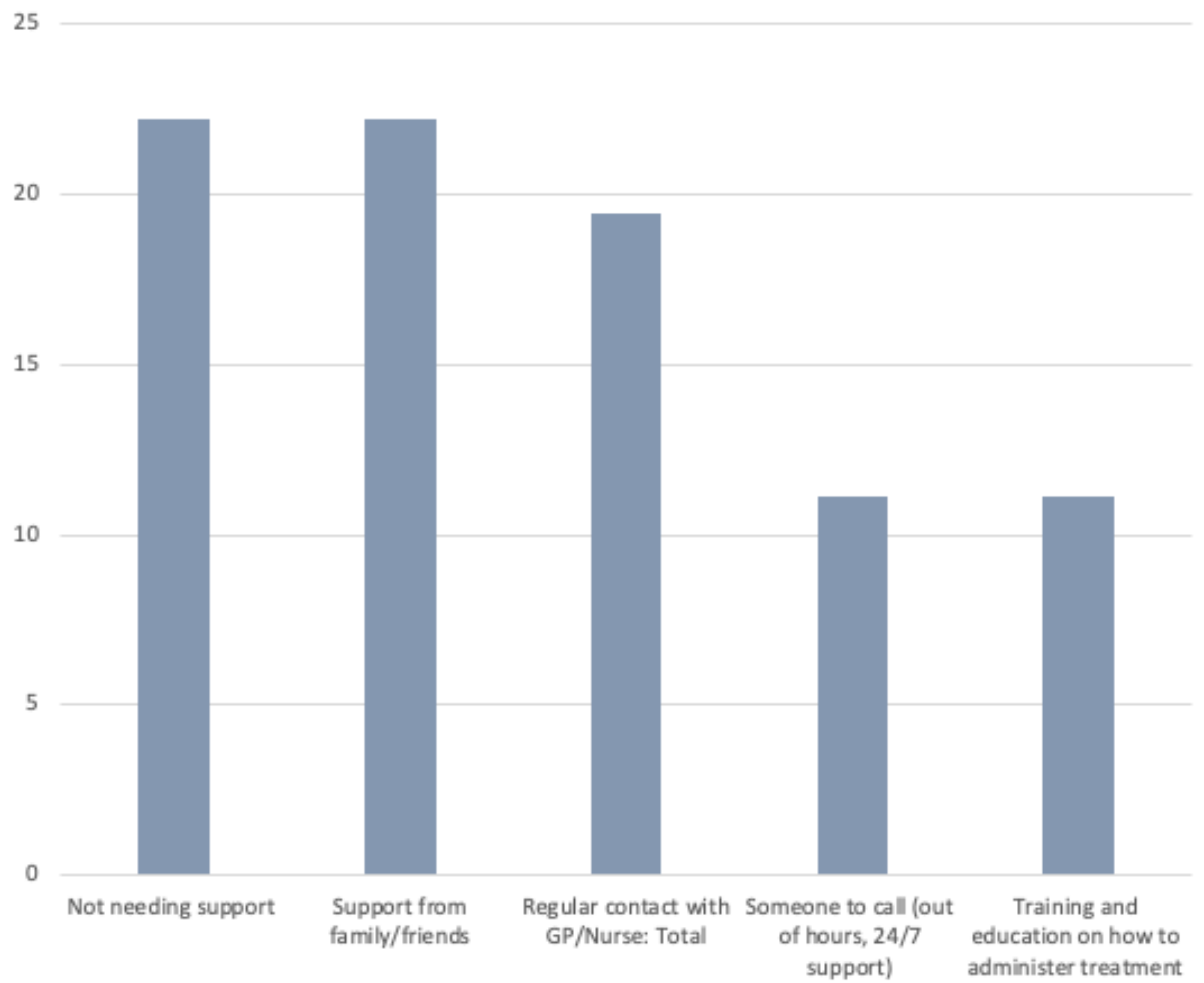
Information to be confident in new treatment (% all)



Just as guided. I have a haematologist now. The haematologist I see, the registrar I see, I have confidence in both of them. What would make me? If somebody told me, "Look, there's a trial or a new drug or something like that. This, and this, and this is the situation, et cetera," I'd be totally guided by what they say because I'm really not in a position to make a decision, but I'm happy to try anything, I suppose. Participant 001AL

Well, I would just go by what the specialists tell me. I would take notice of what they say and their recommendations and I'd go with it. Participant 010ATR

I need to know that my doctors think it'd be good. I've got to have complete faith in the amyloid clinic as a PA. I've been on two trials for them. They both worked. Participant 005AL



Oh, none at all. I'm the one that manages all that so, I don't think-- I keep detailed lists every time the drug regime changes. I make notes every time we see a doctor and I feel quite comfortable doing that. Participant 002CA

It will be the same as the inpatient treatment. I would be having basically in the start weekly blood tests which would include the light chain test. We have a collection point within 2 kilometres of where I live, so that's no problem…There's not even that problem because you don't have to drive on the day that you take them. Really, I don't think I need any additional support at this time. Participant 003AL

I've been taking it home now for around what, three years? I don't need any support. Participant 005AL

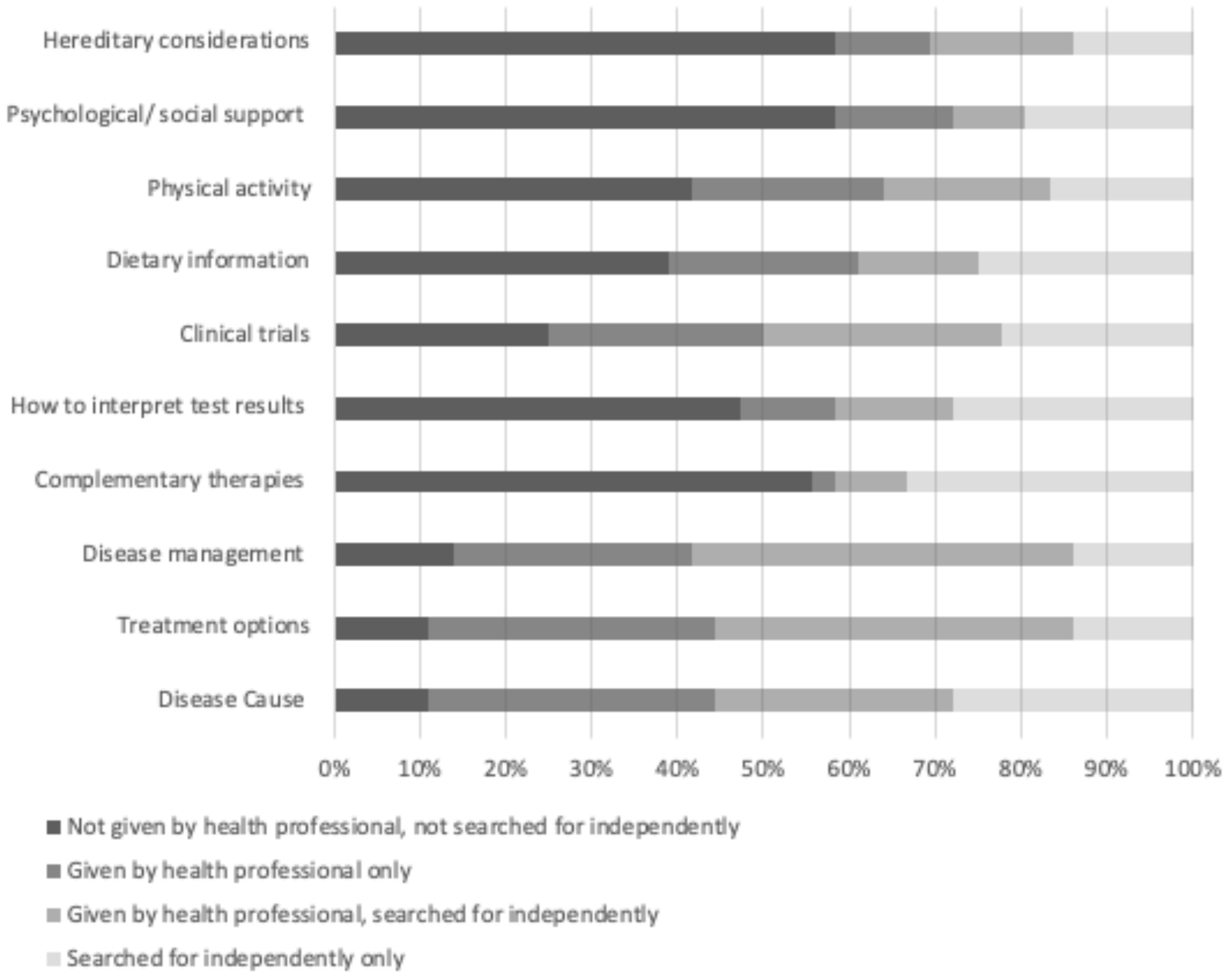
Treatment preference – support needed (% all)

Section 6

Communication
&
Information

This section report access to information, information that was helpful and information that was not helpful, when participants feel they were most receptive to receiving information, information preferences, information topics for which information was received or searched for, as well as when it was received and additional information was required. This is also where there is information about healthcare professional communication and participant’s knowledge and confidence in managing their own health using the Partners in Health tool.

Information accessed and then searched



Information given and searched for

Treatment options, Disease management, Clinical trials and Disease cause

Information given and not searched for

Disease cause, Treatment options, Disease management and Clinical trials

Information not given and searched for

Complementary therapies, Disease cause, How to interpret test results and Dietary information

Information not given and not searched for

Psychological/social support, Hereditary considerations, Complementary therapies and How to interpret test results

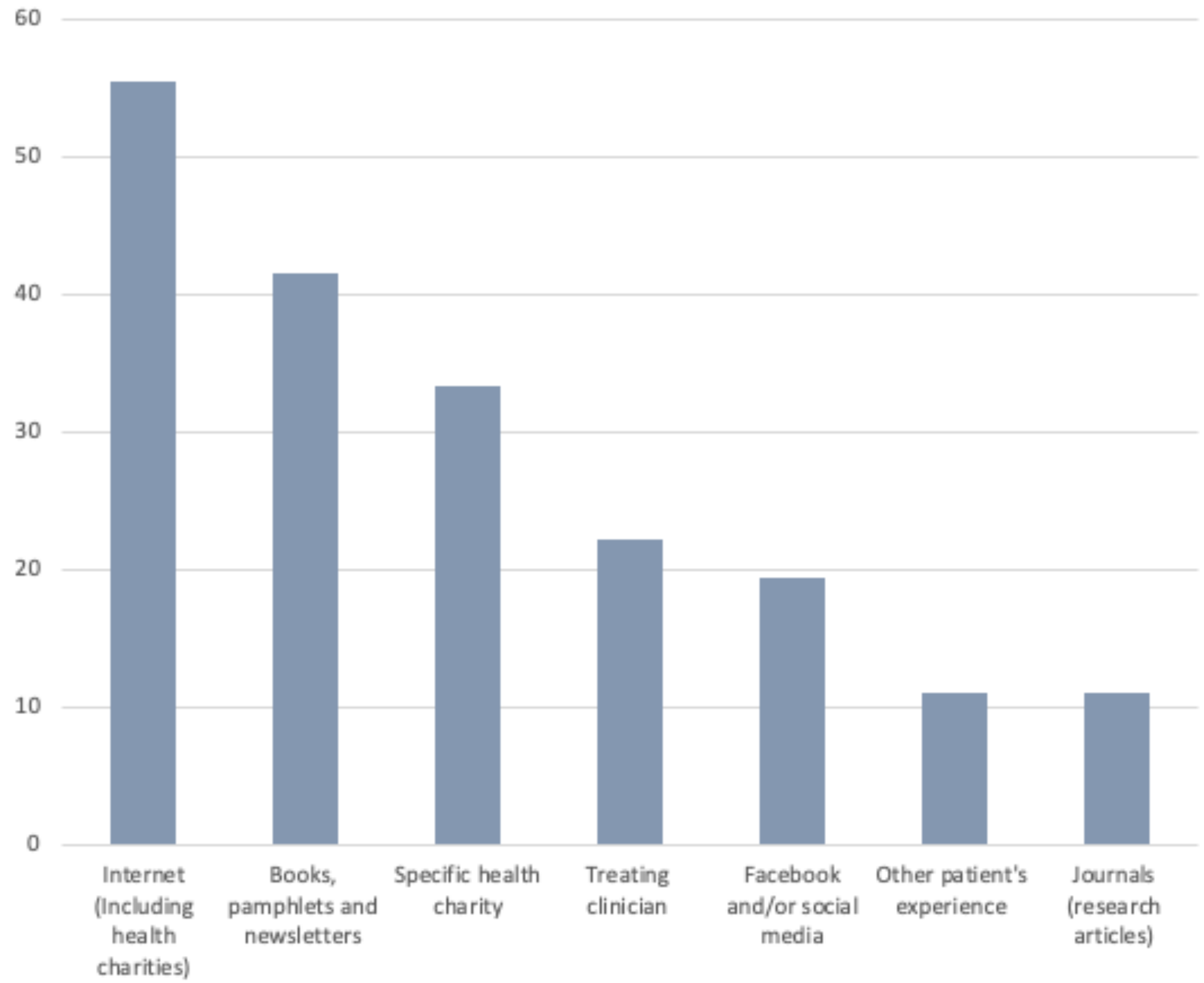
Partners in health scale (n=28)	**Mean**	**SD**	**Median**	**IQR**	**Possible range**
Knowledge	27.36	3.53	28.00	4.25	0 to 32
Coping	17.68	4.46	18.50	7.50	0 to 24
Recognition and management of symptoms*	20.68	2.47	21.00	4.25	0 to 24
Adherence to treatment	15.32	0.98	16.00	1.00	0 to 16
Total score*	81.04	8.66	82.00	12.50	0 to 96

*Normal distribution use mean and SD as measure of central tendency

Clinical trial discussions	Number (n=28)	Percent
I brought up the topic of clinical trials with my doctor for discussion	5	17.86
My doctor brought up the topic of clinical trials for discussion	21	75.00
No one has ever spoken to me about clinical trials	2	7.14

Discussions about clinical trials

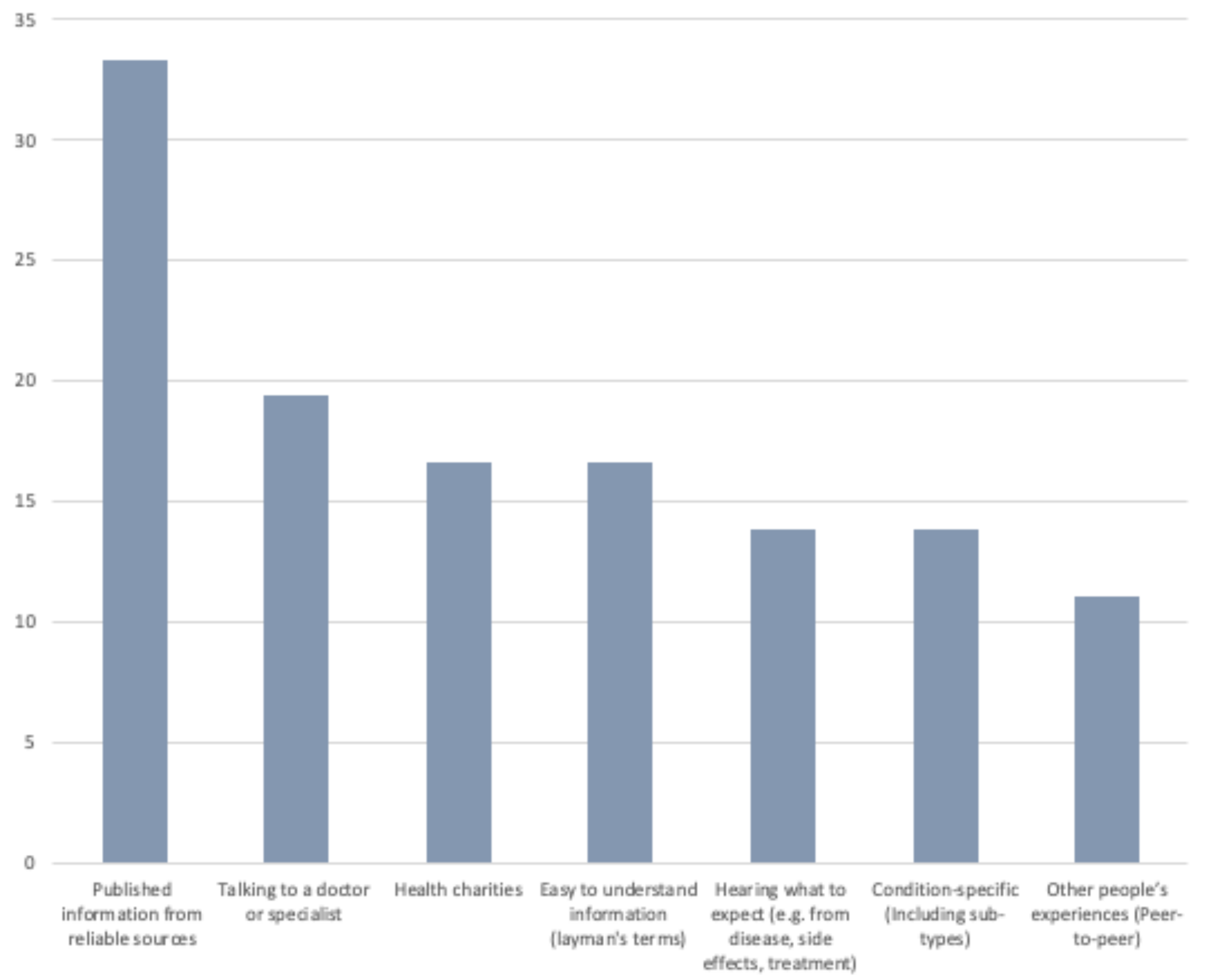
Information accessed (% all)



I've read everything. I've looked up and read everything. I've got literature, internet. I've got stuff from the association that -- I read a lot. I have, I think, read as much as I possibly can without getting too confused. Participant 001AL

Mainly going online. One thing, I find it a bit depressing to go online and read about stuff. Then also some of the case history that are written up by patients, some of the most recent new amyloidosis website in Australia, they are quite confronting some of the-- that I've mentioned before, some of the trials and tribulations that people have been through. Participant 011ATR

Just what's on the websites, and there's quite a bit of it there. I think there's quite a bit of information available there, and talking to the people at the clinics I go to. Participant 017ATR

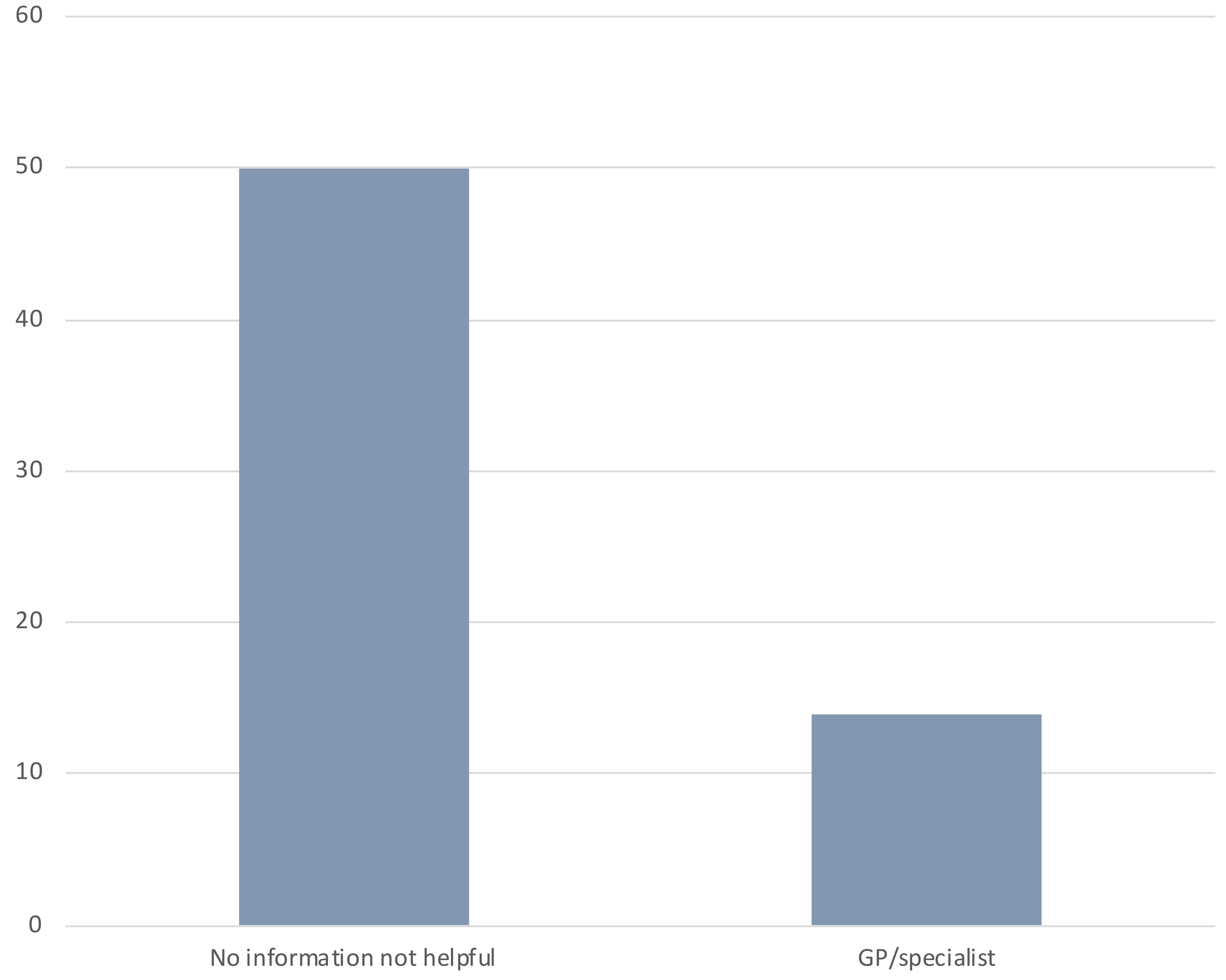


The little booklet, 'Amyloidosis: A guide for patients and families' put out by the Leukemia Foundation, I guess because they're more financially able to do these things, extremely informative, how is it treated...That's very good information, this little booklet, full of information for me which I find very helpful and which I dip into every now and again just to refresh things in my mind. Participant 003ALX

Preferably the booklet about amyloid. Something that was written can be easily understood. I found that very helpful. Participant 003CA

What information's been most helpful. Probably the papers we've researched ourselves. Participant 005CA

Information that has been helpful (% all)

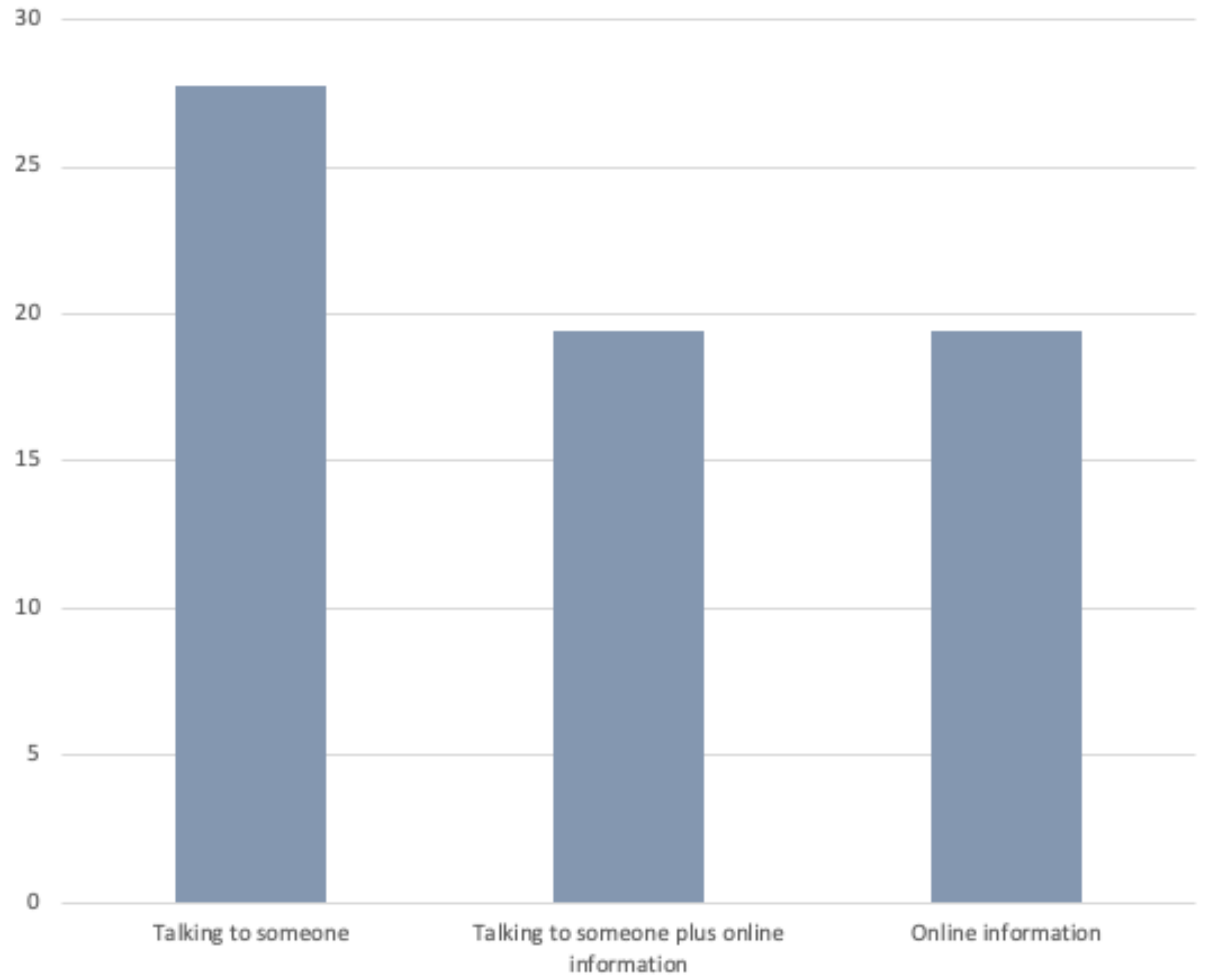


No. I'm trying to think. No, I haven't had any ideas myself. Participant 001ALX

No, not really. I can't say that I have. Participant 001ATR

That has not been helpful. No, I think most of the stuff that I read because, again, I'm only reading stuff and things like the Boston Uni hospital and stuff like that. I don't bother reading-- well, again it's not too much individual stuff because everybody is so different. I don't try to down the track of reading other people's experiences as such. Participant 003ATR

Information that has not been helpful (% all)



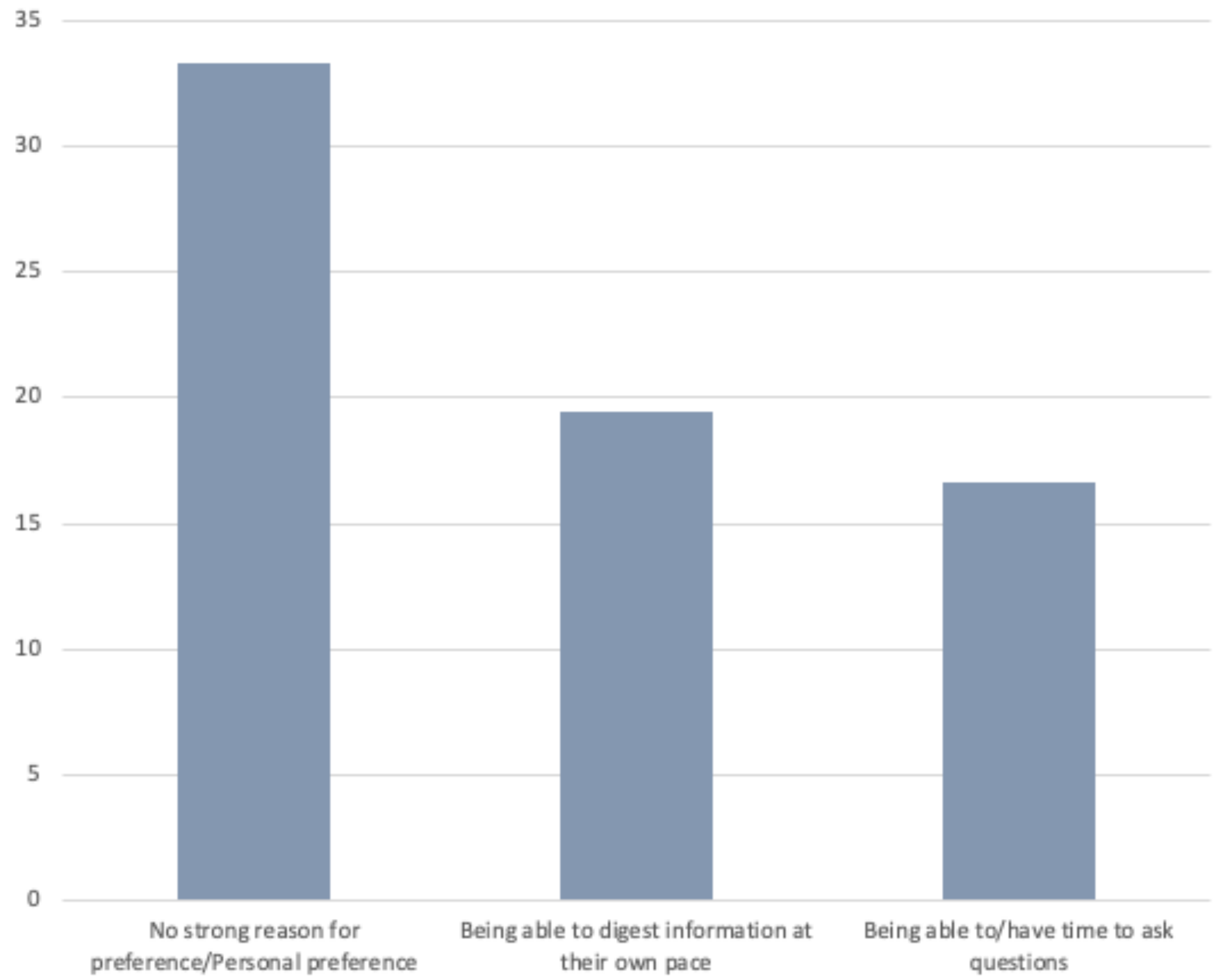
Face-to-face. It's just the way that I've always dealt with that sort of-- In the scientific world, the work I do, over my years, I much prefer face-to-face and I can see from the person whether they-- I guess I get the feel as to whether they're legit or whether I'm beating the wind. Participant 002ALX

If I were to arrange them, I would say talking with someone first and phone app probably second and the journal and the net. Talking with someone, you can ask questions that are more specific to you rather than figuring out if it applies to you, or if it doesn't apply to you. Participant 002ATR

First thing I prefer face-to-face, I think that's just my generation, preferring face-to-face but I'm not-- I'm cynical of website information. Participant 004ATR

Information preferences (% all)

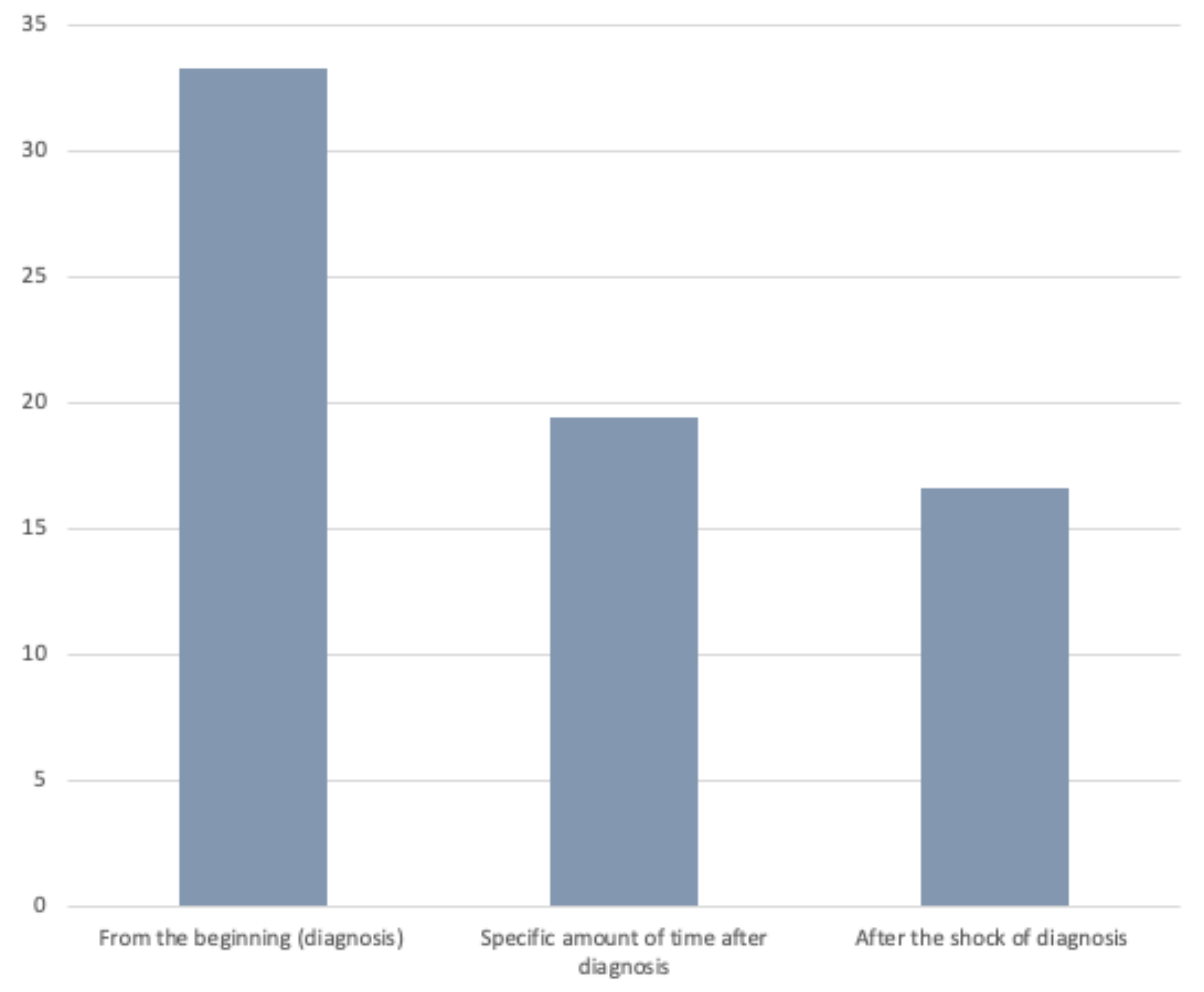
Centre for Community-Driven Research



Well, my generation does go to the computer, et cetera, and I know how to use the computer. I know how to look up the information, et cetera, et cetera, but I'm not what I would call a technical person. My reading of anything, and that means leisure reading or whatever it is, is much preferred in the written form and also in the discussion forum. When I talk to people in a discussion forum, I talk to people and see what happens, whether it's other patients with amyloidosis or at the clinic at NAME HOSPITAL, which I go to, or to the various professions. I find that is the most effective and preferred form of communication. Participant 001AL

All three of them, I have a preference. Talking to doctors, it would be my preferred option. I read about it somewhere, "If it's affecting you, go then ask the doctor, 'I read this, what do you think about that?'" That's the approach I'm taking. Participant 001ALX

Rationale for preference (% all)

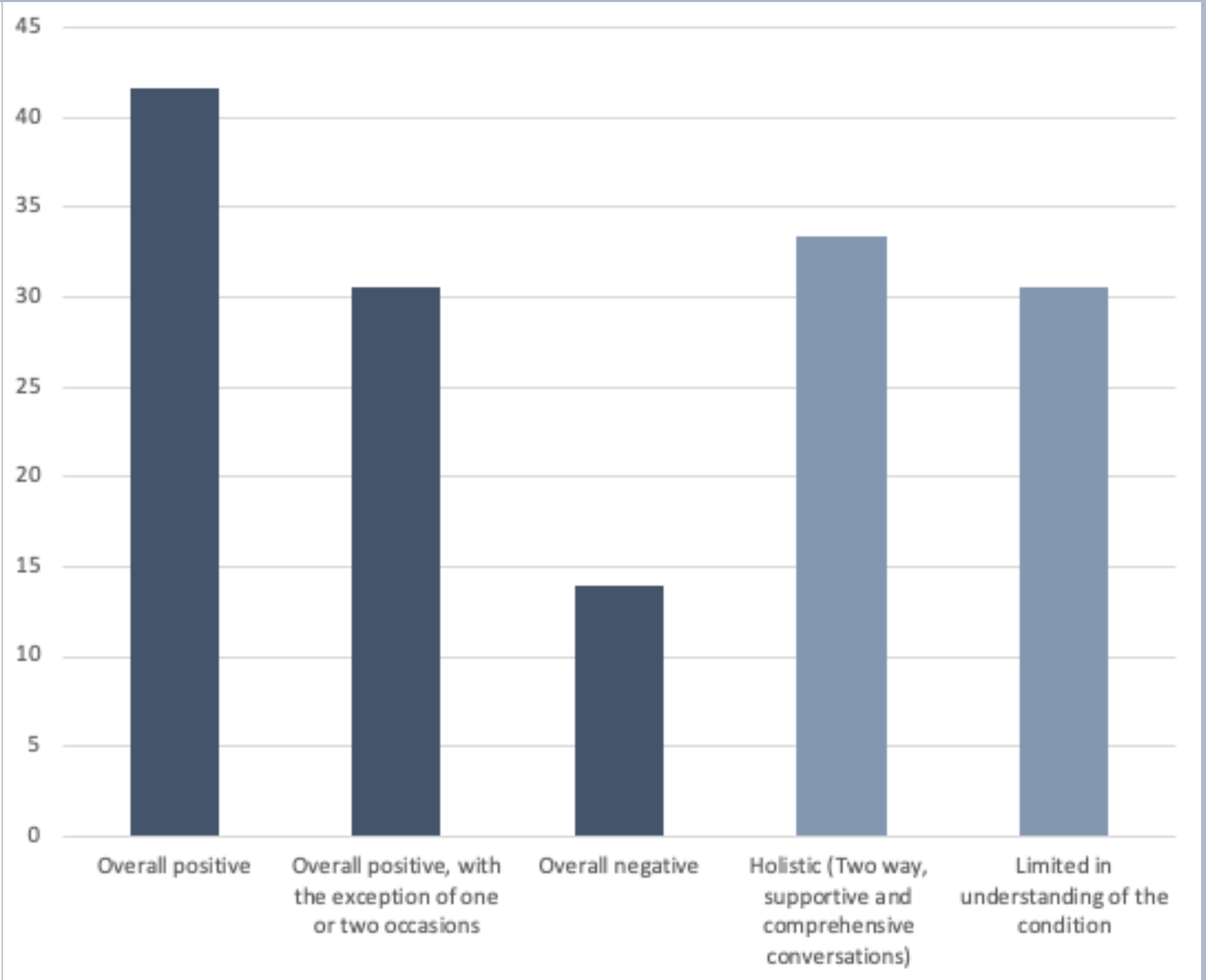


Well, at initial diagnosis, of course, I was more receptive to information, because I had never heard of amyloidosis, and I knew nothing about it. Initially, I was all out getting in every bit of information that I could. Participant 001AL

When was I most receptive? Probably, on initial diagnosis really. Participant 001ATR

I think initially, I was. I was a bit traumatised, obviously, it was a very emotional time because we thought the prognosis was not good. However, at that stage, I just wanted to seek as much information as I possibly could. Participant 001CA

Timing of information (% all)



Good. Everyone is trying to do their best, and the conveyance of that information from various people-- I'm talking about cardiologists. I'm talking about haematologists. I'm talking about other specialists, which I've gone to them. We've talked about exercise. We've talked about all these things. I've found it to be good, helpful, and receptive. I have no complaints, not at all. Participant 001AL

The ones that I'm dealing with? Supportive and informative, but not with information overload. Only enough to maybe make me think a little bit more about the disease and do a little bit of research myself. Participant 001ATR

Well, I think the medical treatments been first class…I've been happy with- extremely happy with my GP, my family and my haematologist. Participant 011ATR

Health professional communication (% all)

Section 7

Care
&
Support

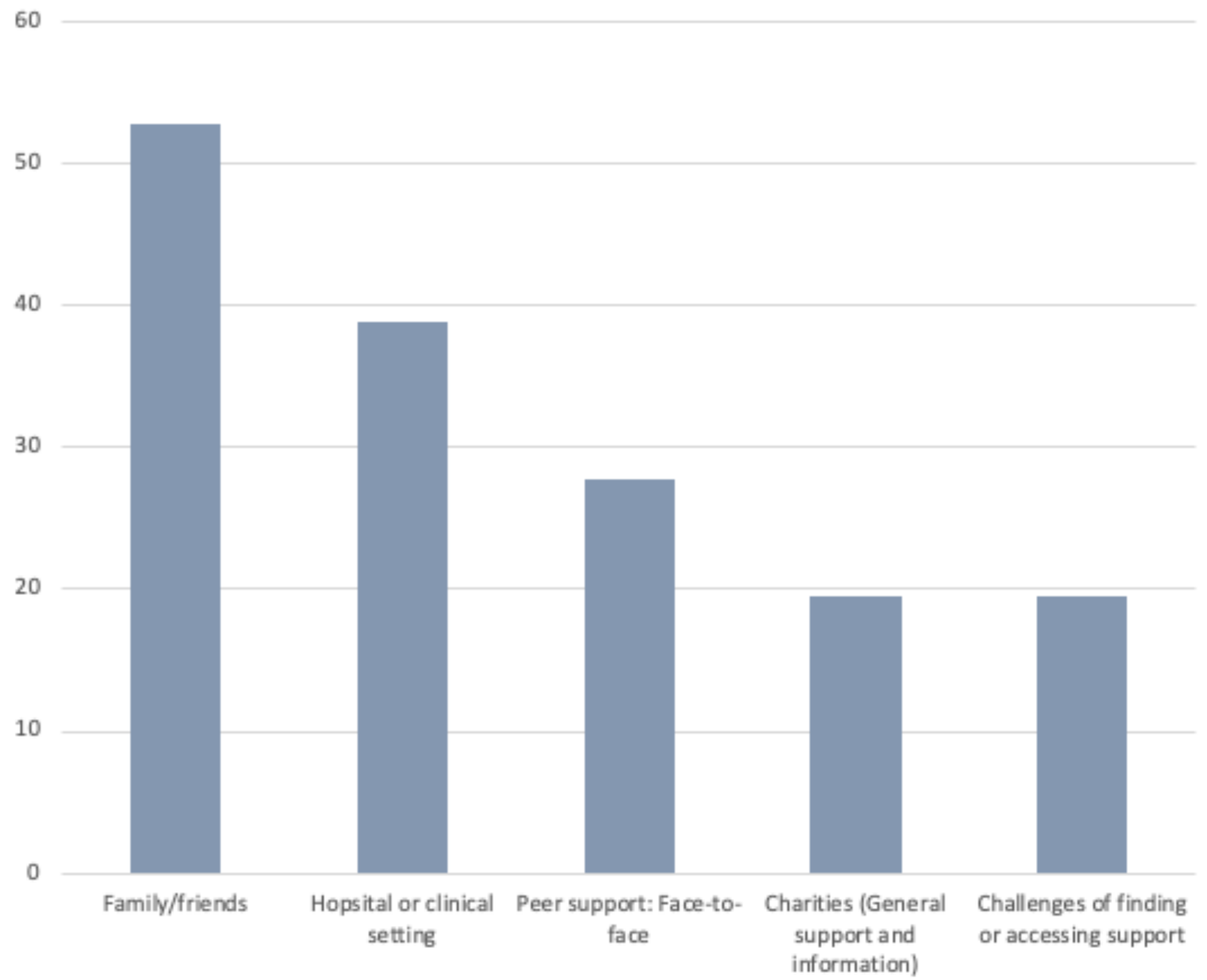
A Care Coordination questionnaire tool is used and reported in this section to demonstrate how people have been able to navigate the health system. There is also information about the care and support people have received, including from the charity sector.

Care coordination scale (n=36)	Mean	SD	Median	IQR	Possible range	Quintile
Communication*	42.17	7.11	42.00	11.00	13 to 65	3
Navigation*	27.56	3.78	27.00	5.00	7 to 35	4
Total score*	69.72	9.15	72.00	12.50	20 to 100	4
Care coordination global measure	7.92	1.54	8.00	2.00	1 to 10	4
Quality of care global measure	8.44	1.34	9.00	1.00	1 to 10	5

*Normal distribution use mean and SD as measure of central tendency

Care coordination sub-scores

Care and support received (% all)



Well, I haven't needed any support or care from the outside. I just generally got my normal family support. My family is concerned, and they're a little bit in the dark about the long-term situation as well. But it's like any illness, terminal or chronic. It's just what life deals out to you sometimes. Participant 001ATR

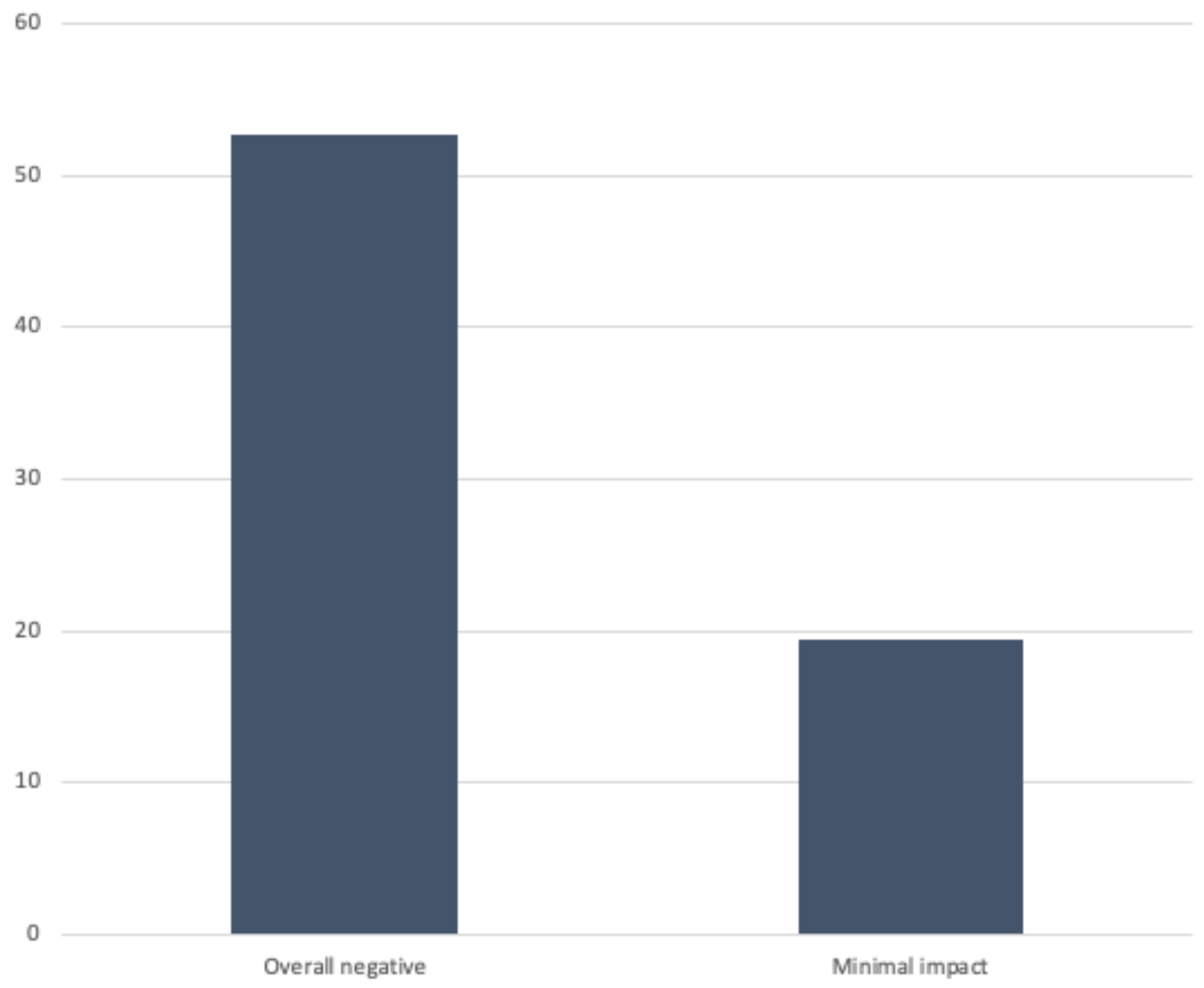
Most of it’s been from my wife. Certainly, the church I attended in LOCATION considered me to be their miracle. I had tremendous support from the people there, I've had great support from friends and family in that period. Participant 002ALX

Since I have been diagnosed, I've gotten support mainly from many friends and family. Friends that we got a long relationship with or some new as well that are like-minded in terms of helping me out. Participant 002ATR

Section 8

Quality of Life

In this section, information is available in relation to the impact that the condition or disease has on quality of life, regular activities to maintain mental health, regular activities to maintain physical health, impact on relationships, impact on family and relationships, cost considerations. The Fear of Progression tool is used and reported here to describe the level of anxiety that participants may have in relation to their condition.

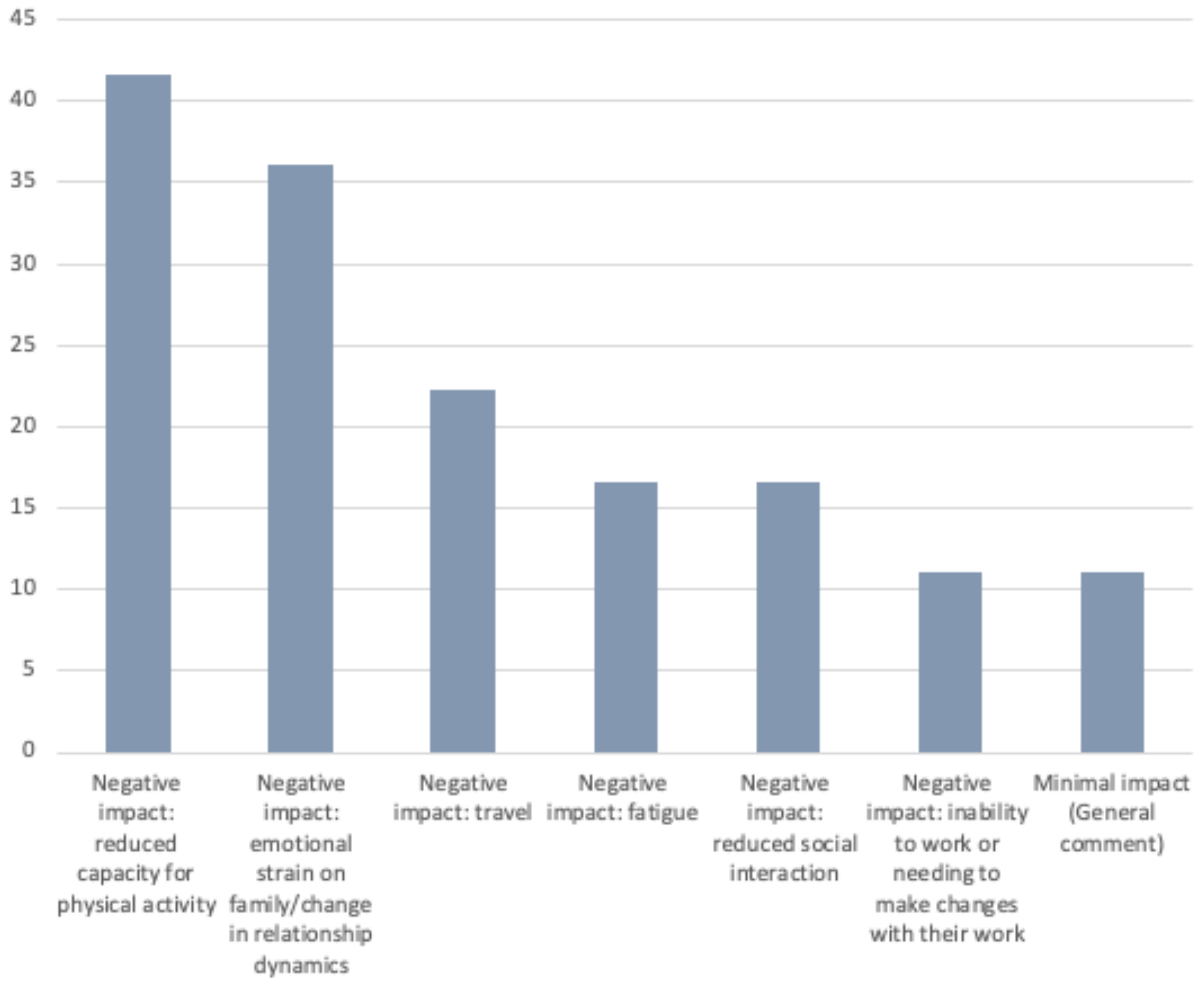


Well, with the amyloidosis, I had to give up work, so literally, yes for me, but for him, it was more or less was suffering from him. Because he was this man that was very physically active in all respects. A man that-- He used to surf, he used to boat, he used to duck dive. He did everything. He was very, very physically fit, and literally, he just became frailer, frailer, and frailer. Participant 004CA

My life has changed. I know that I'm still being the same person, I'm the same person, I know, but all the things around me have changed. The things I wanted to do in my life but have been working hard to achieve, to complete, now I cannot do anymore at the level I wanted to do...I need to go to bed at seven o'clock each evening because I cannot stay up for more than that and the only thing that I want to do is to sleep every day. It's hard, I cannot enjoy a day. Participant 005ATR

Impact on quality of life overall (% all)

Impact on quality of life (% all)



My main issue is I don't have my energy levels. I tire very quickly. I've now designed-- I do things in the morning. In the afternoons, I don't normally plan to do much because I often have a rest, et cetera. Participant 001ALX

I tried taking him on a cruise boat Christmas. We were restricted to the ship because he couldn't get on and off the stages because of his instability. He has to rest a lot; he can't do a lot anymore. He's very tired very quickly. Participant 009ATR

Look, there's so many things, I just keep kicking off things that I can't do anymore, and gardening is starting to become difficult and I'm a very keen gardener. My wife keeps saying we'll get someone in to do that and I say, "Well, how can I do that? Can I stand there, tell him exactly which branches I want cut?" I still want to do all that. Participant 013ATR

Sub-group	Sub-group	Count	Percent	Mean	SD	Median	IQR	Possible range	Quintile
All participants*	All participants*	36	100.00	33.19	9.92	31.50	12.25	12 to 60	3

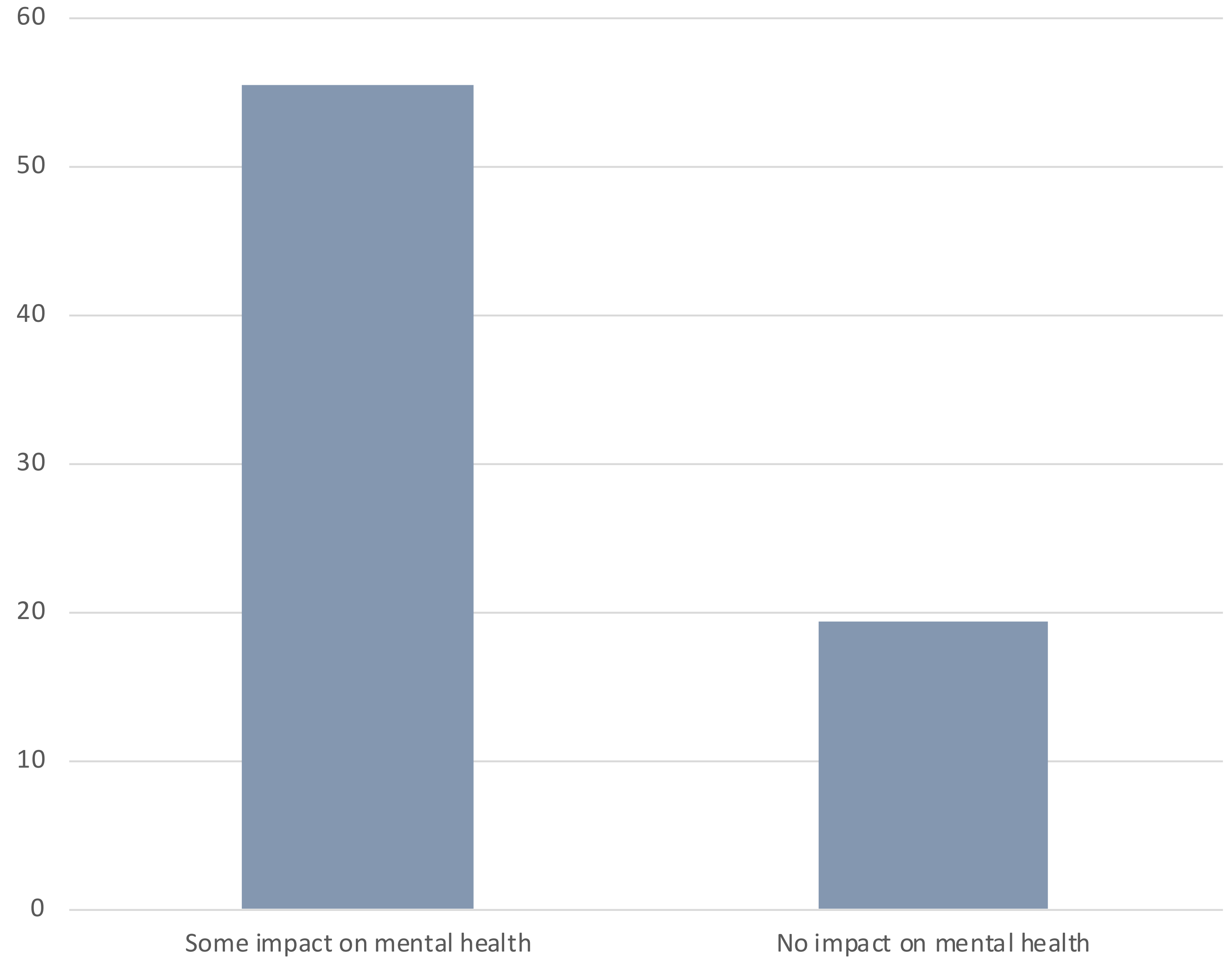
Fear of progression	Sub-group	Number (n=36)	Percent	Mean	SD	Source of difference	Sum of squares	dF	Mean Square	f	p-value
Type	ATTR-cardiac	18	50.00	36.50	9.15	Between groups	615.00	3	204.99	2.39	0.0784
	All-cardiac	25	69.44	33.88	9.26	Within groups	4894.00	57	85.86		
	AL amyloidosis	10	27.78	26.80	5.85	Total	5509.00	60			
	Carer	8	22.22	33.75	12.57						

Fear of progression	Sub-group	Number (n=35)	Percent	Median	IQR	X^2	dF	p-value
Age	Aged 55 to 64	8	22.86	36.00	7.50	3.37	2	0.1859
	Aged 65 to 74	19	54.29	30.00	9.00			
	Aged 75 and older	8	22.86	28.50	9.00			

Fear of progression	Sub-group	Number (n=36)	Percent	Mean	SD	T	dF	p-value
Gender	Female	14	38.89	32.64	10.32	0.26	34	0.7945
	Male	22	61.11	33.55	9.89			
Education	Trade or high school	14	50.00	33.36	10.26	0.18	26	0.8589
	University	14	50.00	32.71	8.62			
Location	Regional or remote	9	25.00	30.56	7.73	-0.92	34	0.3644
	Metropolitan	27	75.00	34.07	10.53			
Economic advantage	Mid to low advantage	11	30.56	36.36	11.41	1.28	34	0.2082
	Higher advantage	25	69.44	31.80	9.09			

*Normal distribution use mean and SD as measure of central tendency

Fear of Progression

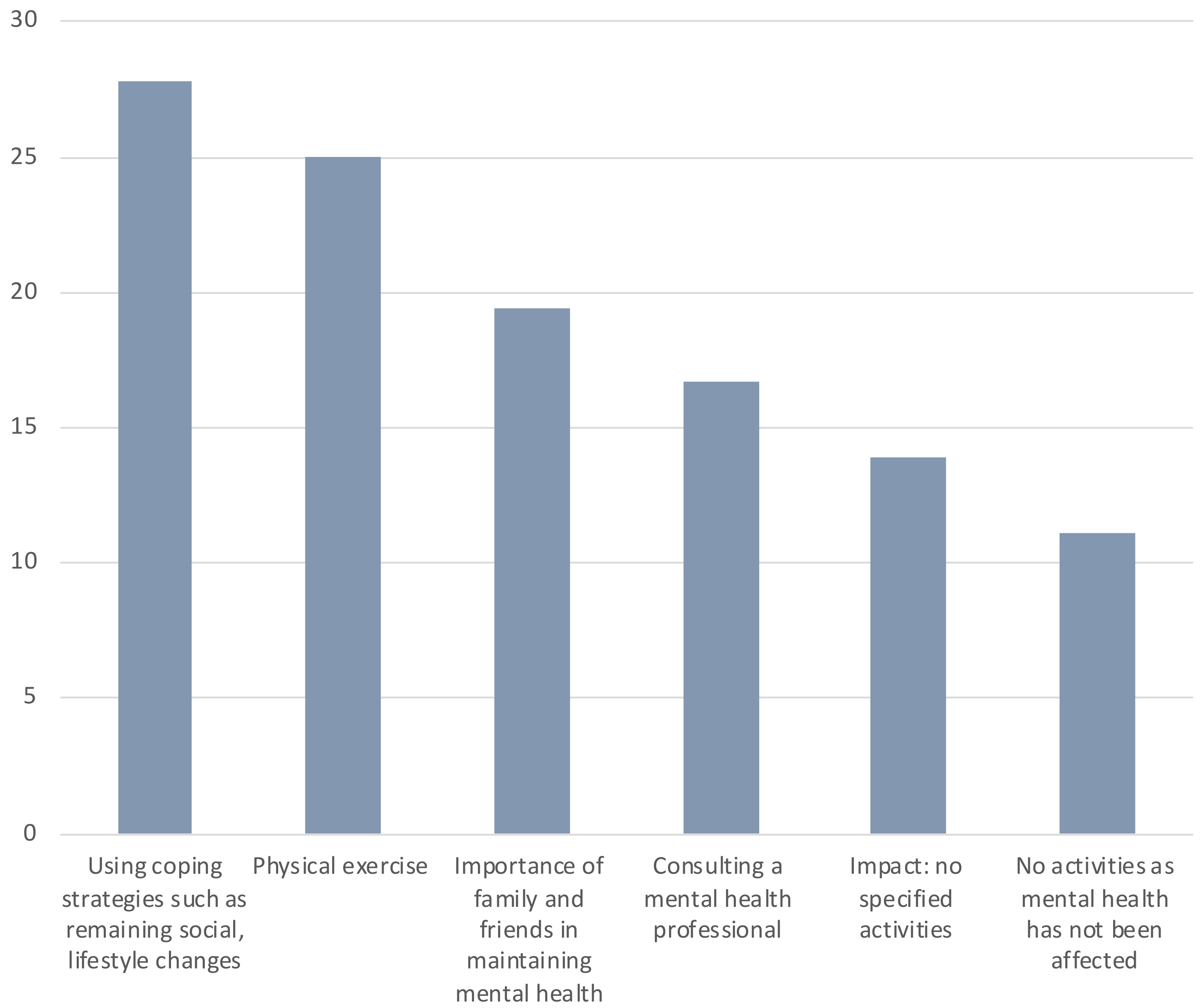


Certainly, in the early times it did before, when I was waiting over a month or whatever it was to have it really explained to me what amyloid basically is. I was very anxious, particularly at that time my husband was in LOCATION actually with our eldest son there. I was dealing with it on my own. Participant 003ALX

With NAME HUSBAND getting more and more frail and that is between age and amyloid…it has had an effect on our relationship, certainly our marital relationship. That was one of the first things that went before he was even diagnosed. Just finding ways around still loving each other without loving each other, not to put too fine a point on it, that has been an interesting journey. Participant 003CA

You can't help it. Anytime you're faced with mortality, you have to contemplate mortality, it becomes an issue that weighs upon your mind. Participant 004AL

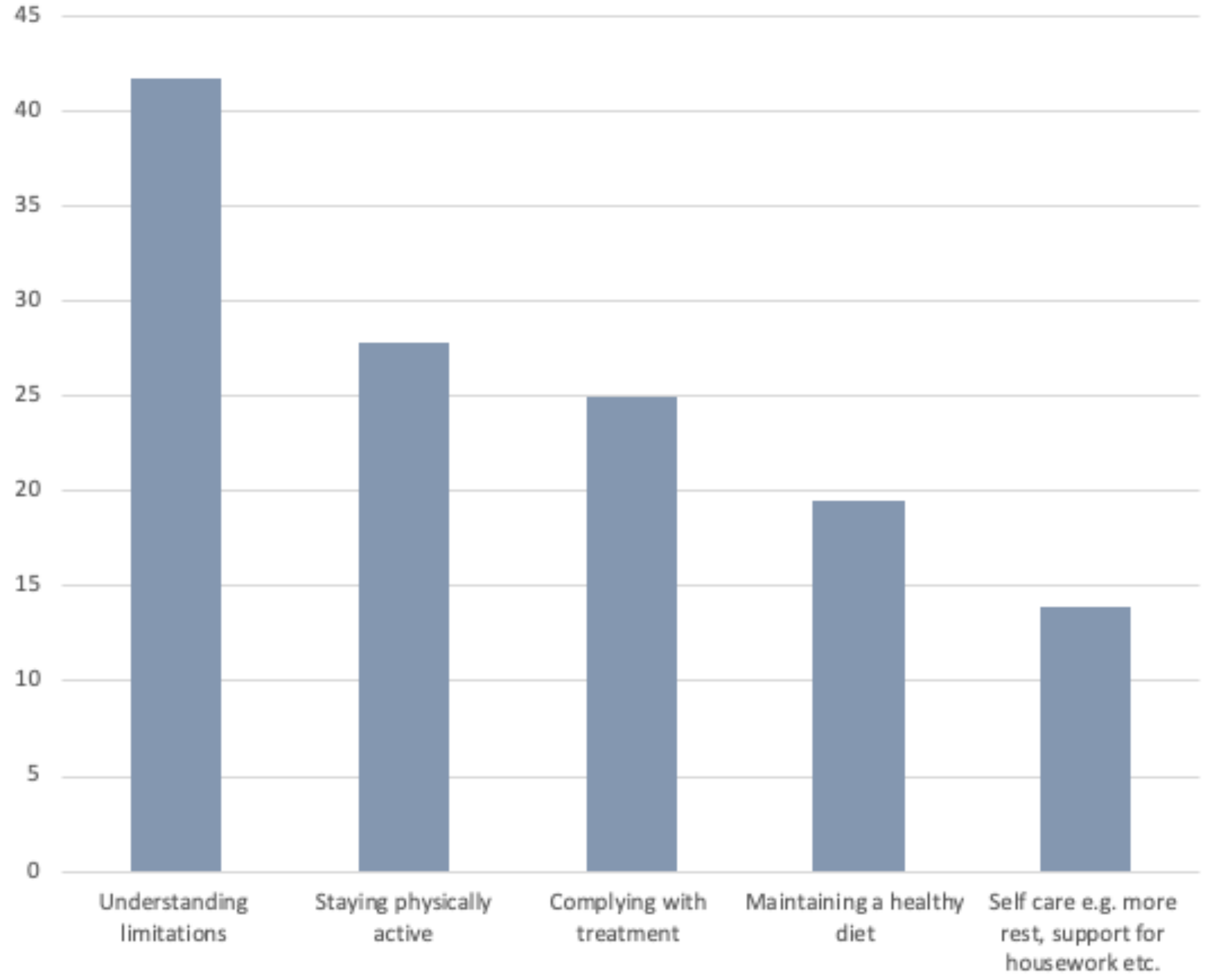
Impact on mental health (% all)



I sing in a choir when we're allowed to sing, which hasn't been for the last three and a half months. I play tennis. I keep myself physically active and outdoors doing useful things…Those things give me a sense of achievement and a sense of worth, because you don't really feel a sense of achievement when someone's not getting better and you can't do anything to make them better. Participant 002CA

I've gone into the business of making family trees as a side effect of that. Also, all through my life, I've written verse, I call it poetry, but I write more verse now as a way of releasing pressure and stress. It's not flat. It's just a way of concentrating the mind and seeing things in a different, non-personal perspective, which helps a lot, I find. Participant 004AL

Regular activities to maintain mental health (% all)

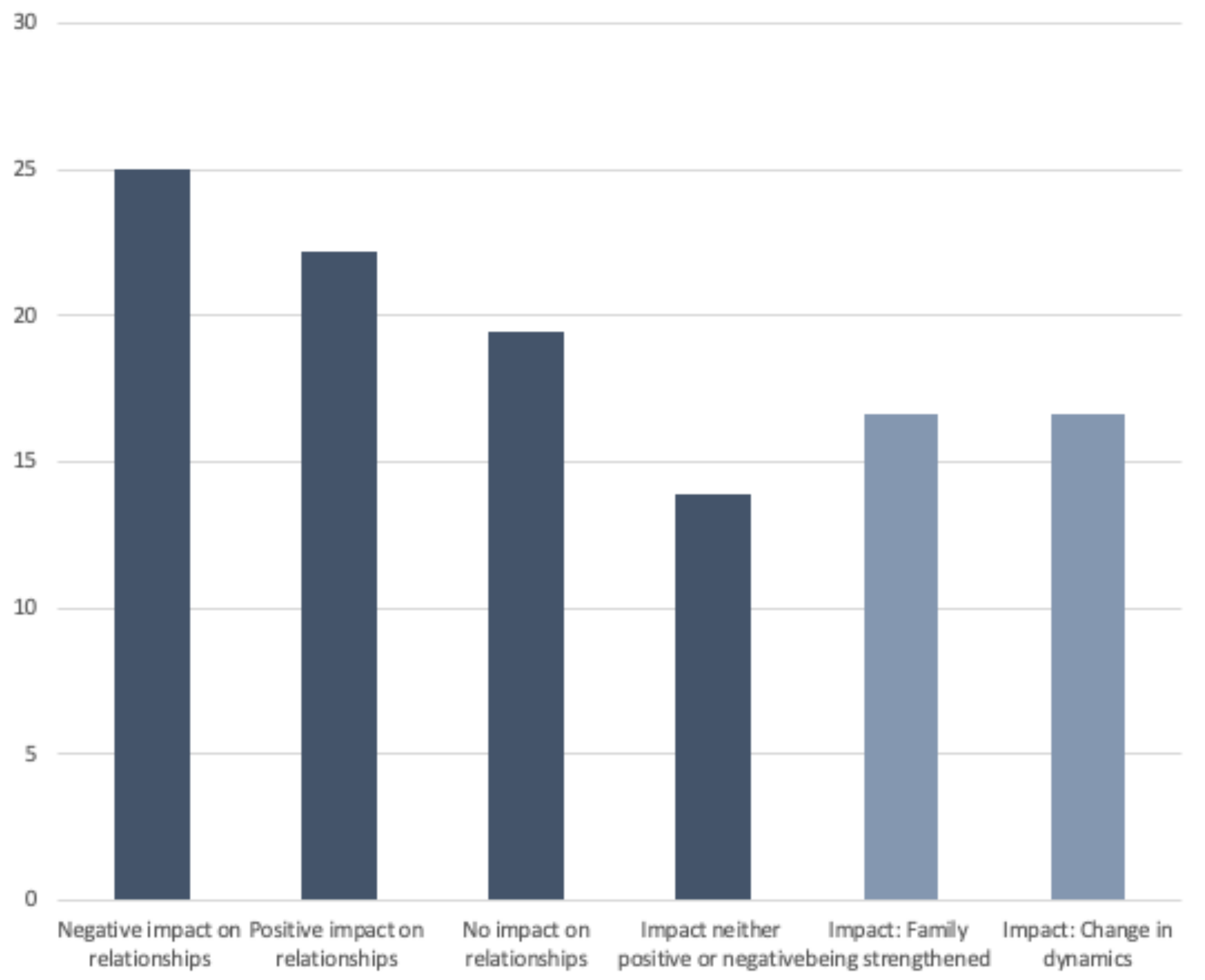


The only thing is watch what activities I do and that I don't overdo things. When I do overdo things, I know I've got to stop. For example, we're part owners of a property down at LOCATION REGIONAL and we have working bees down there, et cetera. Sometimes, well, I know I can only do so much, so I'll sit, and I'll supervise the rest of it. Participant 001ALX

One of the things I have noticed, is not to overdo things, because the next day you might as well have the day off. Participant 003AL

Slow down. I just take things slower. If I take the dog for a walk, I take the dog, it's now a casual walk it's not like a walk of pace. If I go to lift the grand kids, I make sure that that I can, that I'm not over stressed. I don't go bouncing on a trampoline with them chances are the heart will keep bugging me up, but I still do stuff them. Participant 004ATR

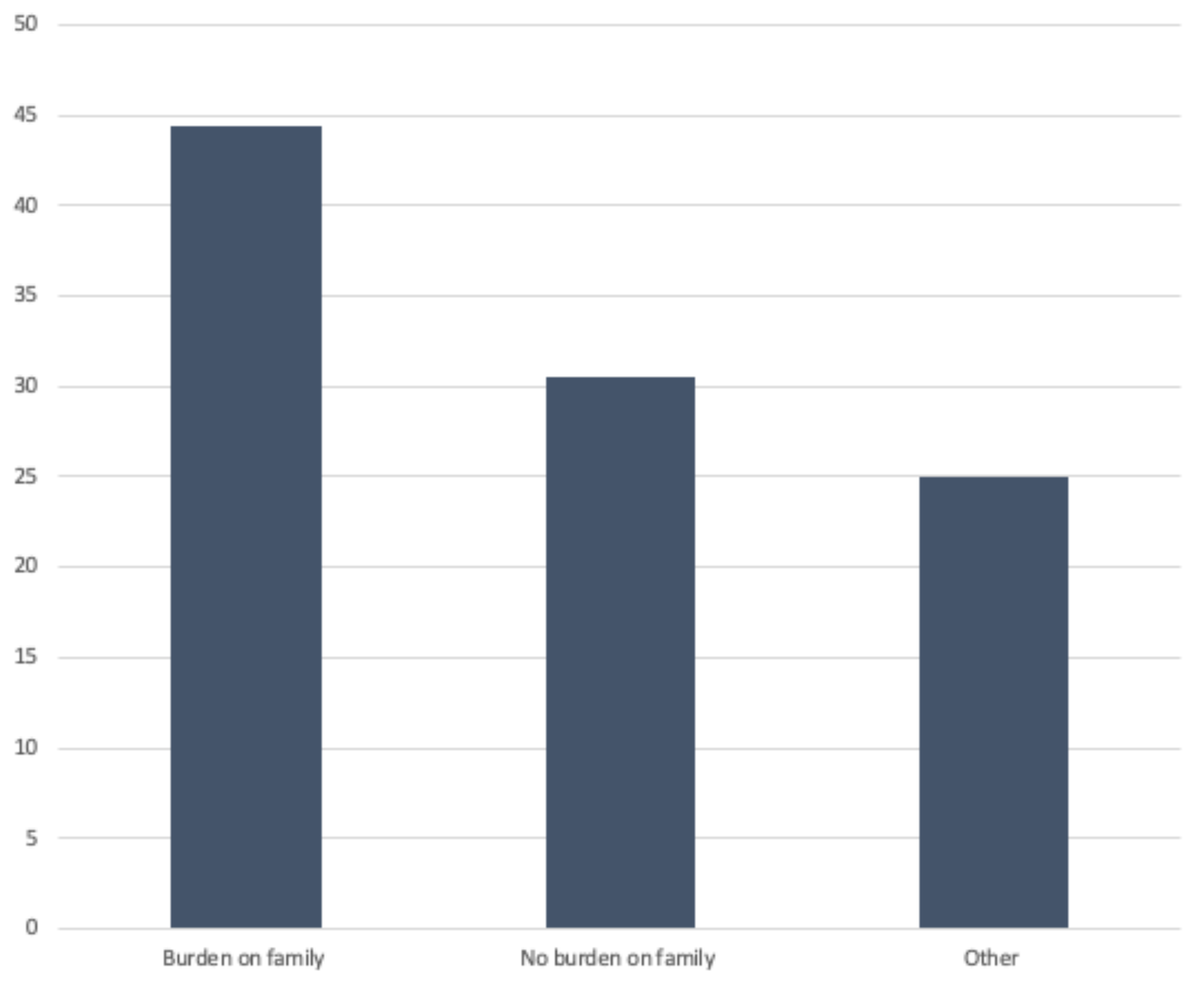
Regular activities to maintain health (% all)



I know it does. It varies depending on the relationship of family and friends, that I have with them. Immediate family, the children and so forth, it does because you don't want it to be-- they don't like to see someone they love going through the pain-- For them to see the effects that they can't do anything about. Participant 002ATR

Our greatest concern is my girls. None of them have been tested yet because literally we thought that if they were tested and they found out they were carrying the gene, then that would be something he'd never forgive himself for. That never happened. At this stage, there's really no cure at this stage for Amyloidosis. They just watch for any symptoms and then they'll go to a doctor. But knowing that they could have it, knowing that my grandchildren could have it, it's like something just like a big black boulder sitting above you. For the girls, it's very scary because they saw how their father died. Participant 004CA

Impact on relationships (% all)

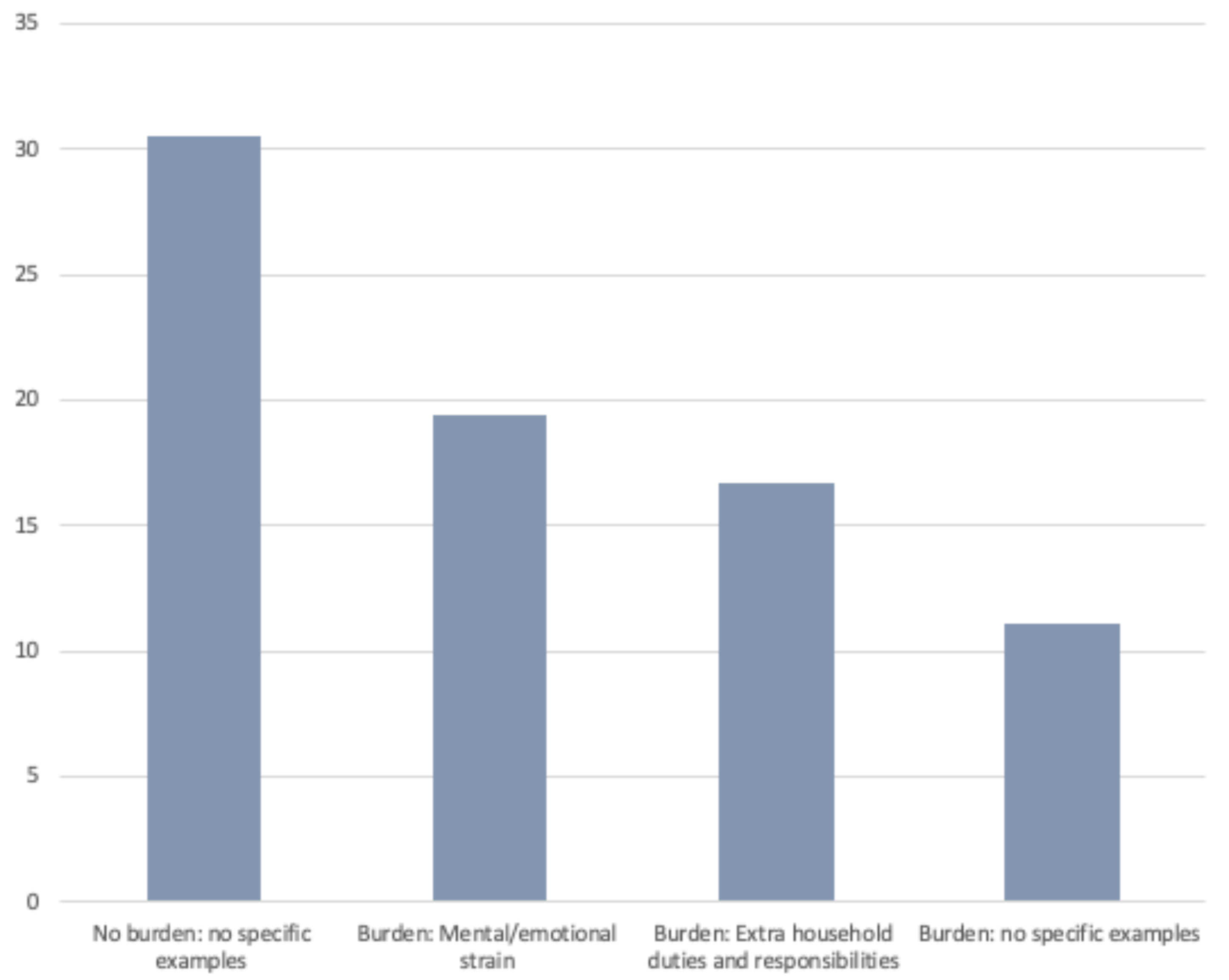


It's more on the emotional side, that there has to be a distinct commitment for managing the issue. My wife sometimes finds it a little bit more difficult. Participant 004AL

Well, that's just natural I think. You feel a burden, as I said, because you've got to limit what you can do and what you can't do. Around the house, you can do all of the simple things, but, again, one of the main reason for me going into a retirement village really was the fact that we're going to have someone come in and clip the hedges or do all of that stuff for me because, A, I couldn't do it and, B, I don't expect my wife to be out there mowing the lawn and clipping hedges. Participant 003ATR

Yes. Probably a bit of extra burden on my wife. We've just accepted that's what it is and get on with life. Make the most of what's left. Participant 008ATR

Burden on family (% all)



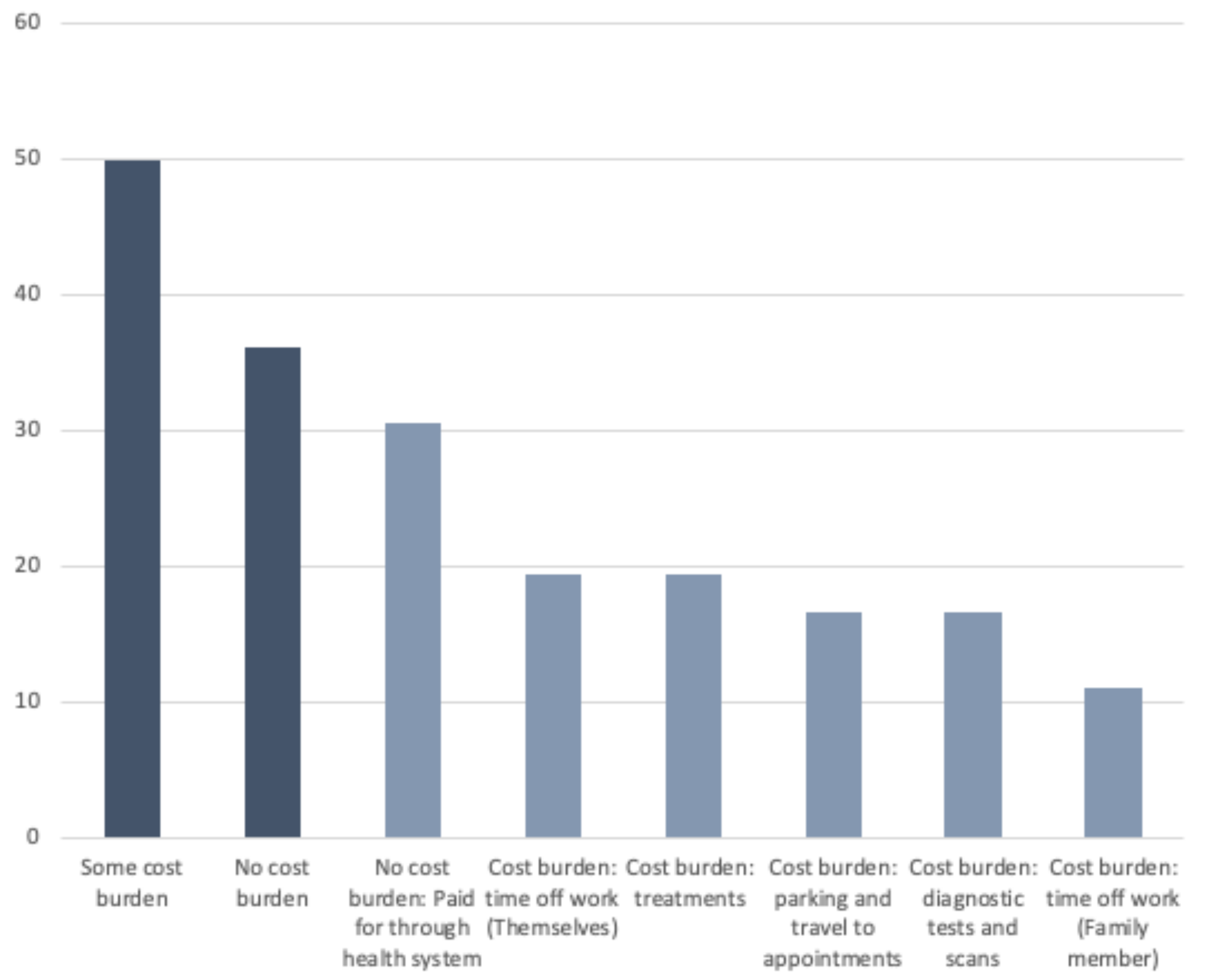
Not at this stage. If it is, well, bad luck, they'll just have to deal with it. And I'll have to deal with it too. But at this stage, no, it's not a burden on my family. Well, because I'm really early in the diagnosis, I'm not really as ill as what some of them are. Participant 001ATR

No, not at the moment. I don't think it was particularly at that time either. My husband did take a bit of time off work but he wasn't burdened financially because he could take some carer's leave. Participant 002AL

No, definitely not. Participant 007ATR

Burden on family (% all)

Cost considerations (% all)



Well, it cost us a hell of a lot in the fact that we had income protection insurance, which we thought would be able to get us through. However, they basically turned around and just said we don't think that you'll have some will ever go back to work, so we decided to pay out a death benefit, which was about $27,000 as compared with 85% of what his normal. Participant 004CA

Yes, it's impacted quite a bit on our bank balance. We're retirees so we're not working, living off our superannuation and pension. It does become a bit of a strain. Participant 009ATR

Unfortunately, because I can only pick it up from the hospital, it costs me half a day to travel to get it and to travel home. The cost of the travelling and the cost of the parking at some hospitals is absolutely disgustingly high. Participant 015ATR

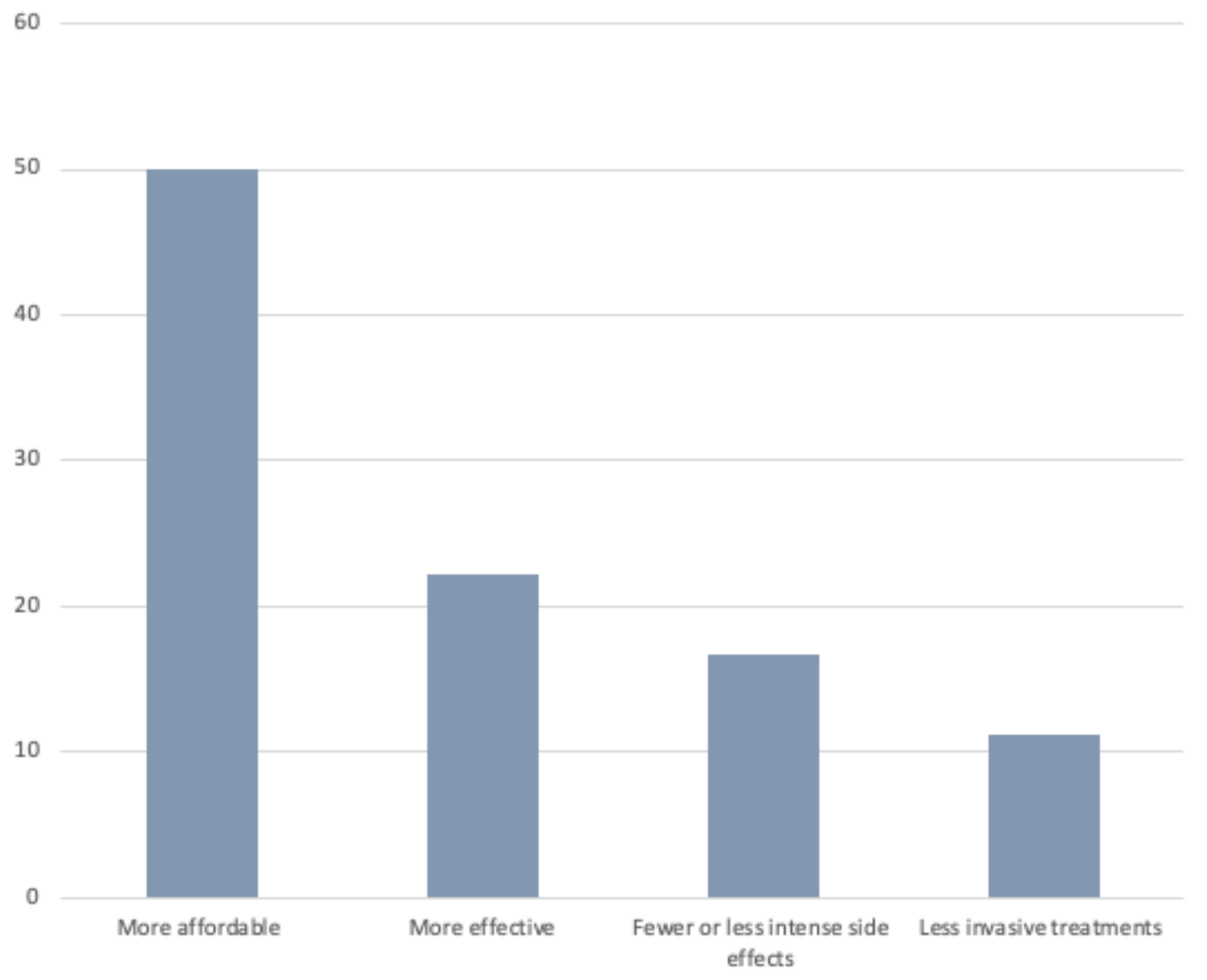
Section 9

Expectations

&

Messages to Decision-Makers

By the time participants are asked questions about their expectations of the future, they have had the chance to talk about everything that they have been through. It means that they have been able to reflect on what worked for them and what was potentially not so great. In this section there is information about what participants would like to see from future treatments, information, care and communication. We present the cohorts message to decision-makers about their condition and the values that are important to them when they make decisions about treatment and care. In this section there is also information about what people have been grateful for.



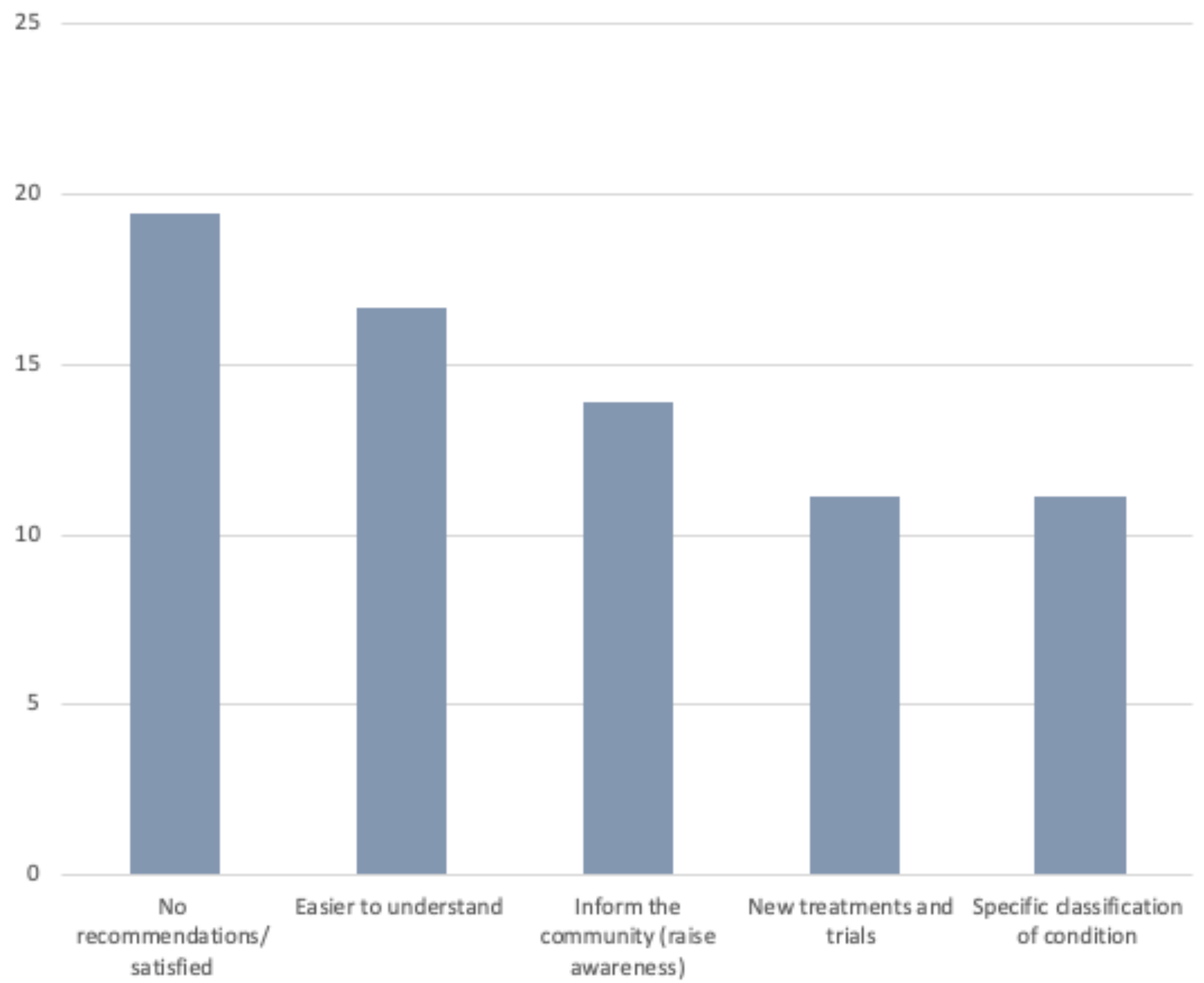
Well, obviously, the main would be costs to be brought down for those ones…I know it costs time to develop with these things, but those prices seem pretty high. Participant 001ALX

I think a lot of the cost, for example, some of the marker ones, not on the PBS though, I'm being charged the full amount for those, $89 a pop. Participant 003AL

Well, obviously the cost. If it ever gets to the point where some of them are paid for, that would be fantastic. Participant 012ATR

Well, of course, I'd love to see things on PBS of course, so they have minimal personal cost. Participant 015ATR

Expectations of future treatments (% all)



No, I think what I have accessed is adequate information for me, personally. Participant 001ATR

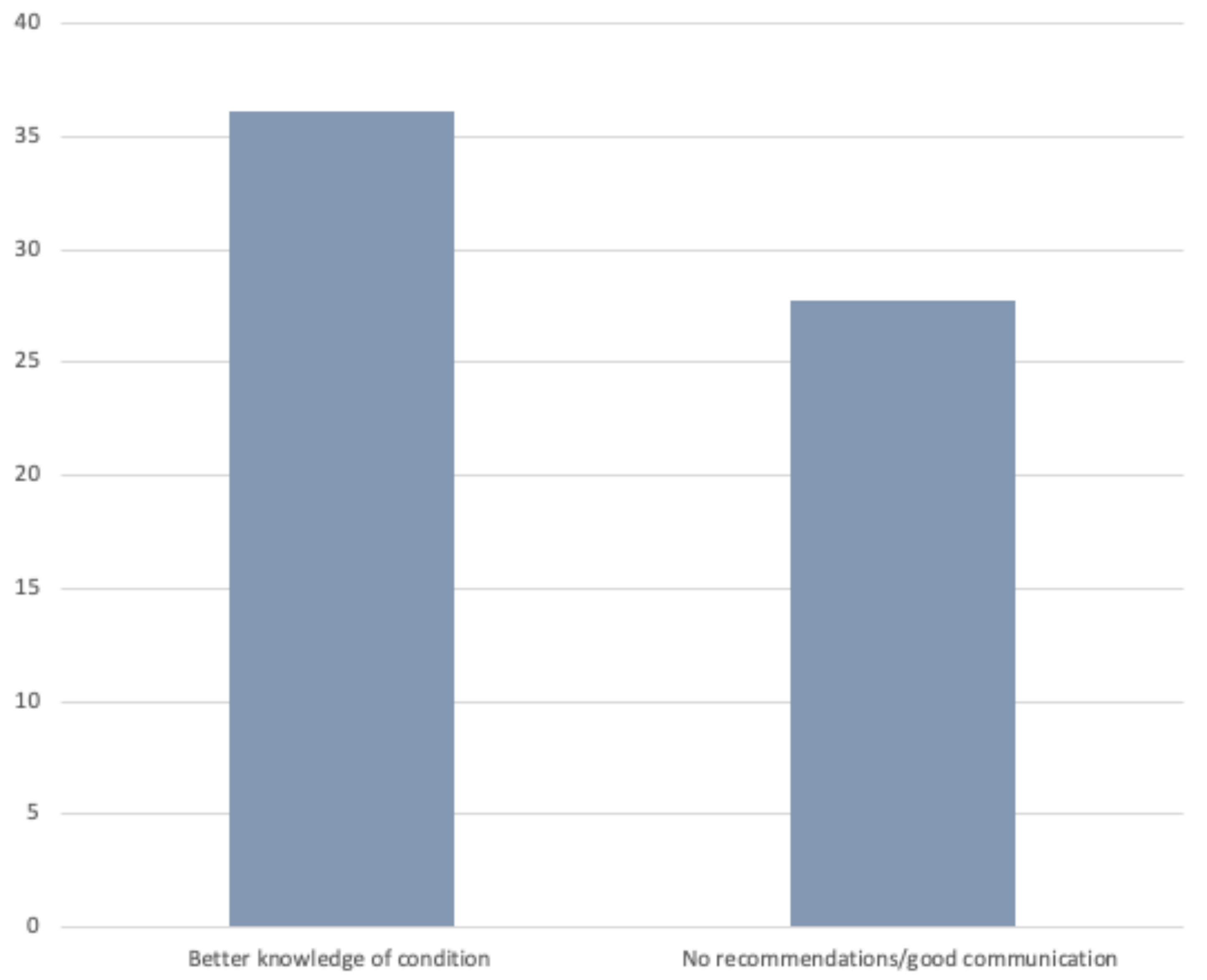
I didn't find researching information difficult and if something didn't answer my question, I was comfortable with finding another source…I was comfortable with the amount of information that was out there definitely. Participant 001CA

I think there is actually quite a lot out there because there were booklets on stem cell transplants and things as well. I'm not really sure that it could be improved on a lot. Participant 002AL

The pamphlets they gave me when I first got diagnosed were comprehensive. They weren't full of jargon, so they were good. The information I'm getting from my doctor it's been pretty accurate…there's not much more they could say or do. Participant 004ATR

Expectations of future information (% all)

Centre for Community-Driven Research

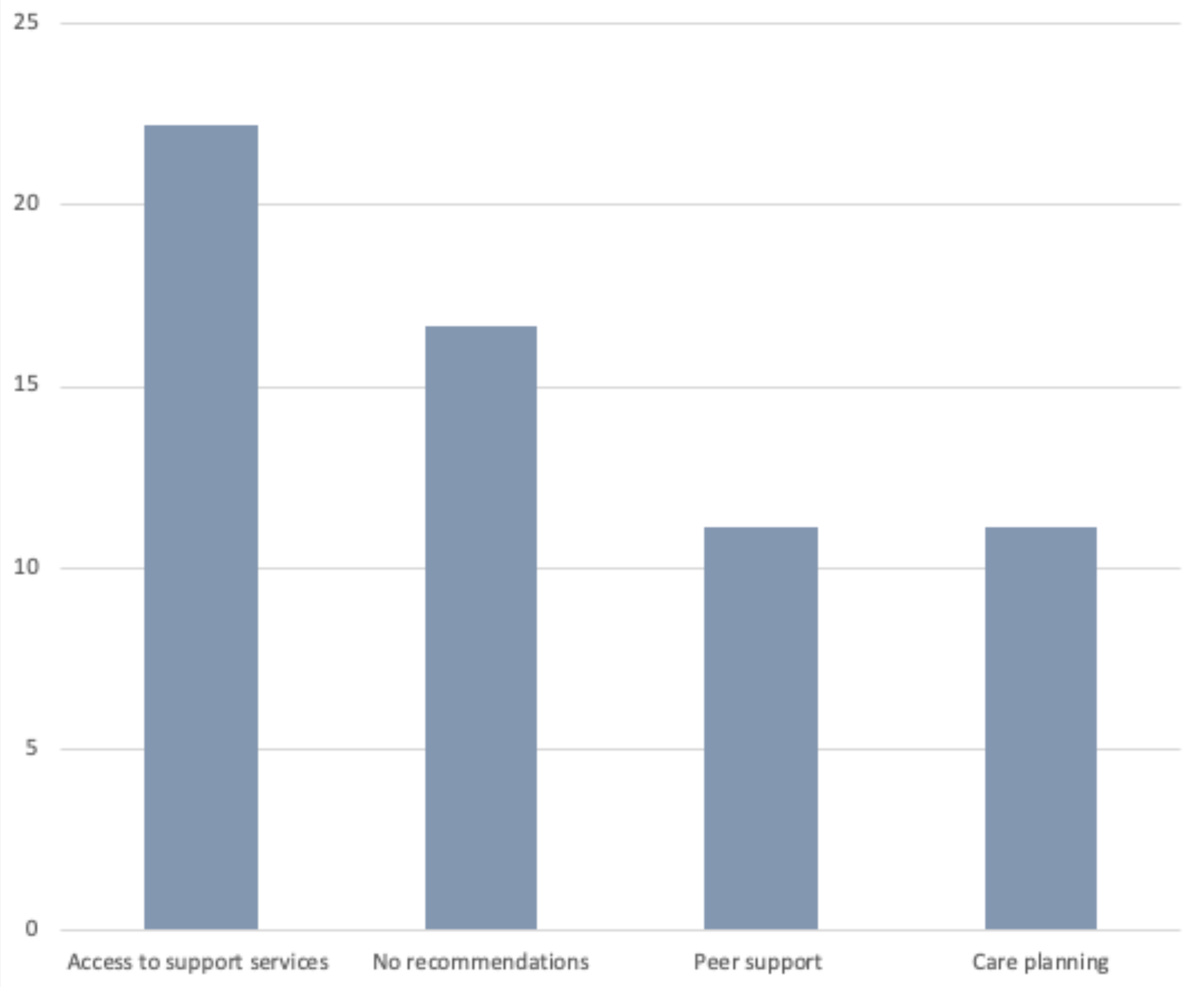


I think health professionals should be honest when they don't know about amyloidosis and that they do reach out to keep the patients informed...I think that there needs to be more education and training for them. Participant 002ATR

The only thing in relation to health professionals is that there needs to be more education at the general practice level to be looking for this as a potential diagnosis, because I haven't met one person who hasn't been on 12 months' worth of looking and trying to find out why they were going downhill. It's more about education. Participant 002CA

I think health professionals need more knowledge about the disease itself. A lot of them don't know about it. They've never heard of it. We've come across a couple of doctors now that- even our own neurosurgeon or neuro guy didn't know. He said, 'I've never heard of it'. Participant 009ATR

Expectations of future communication (% all)

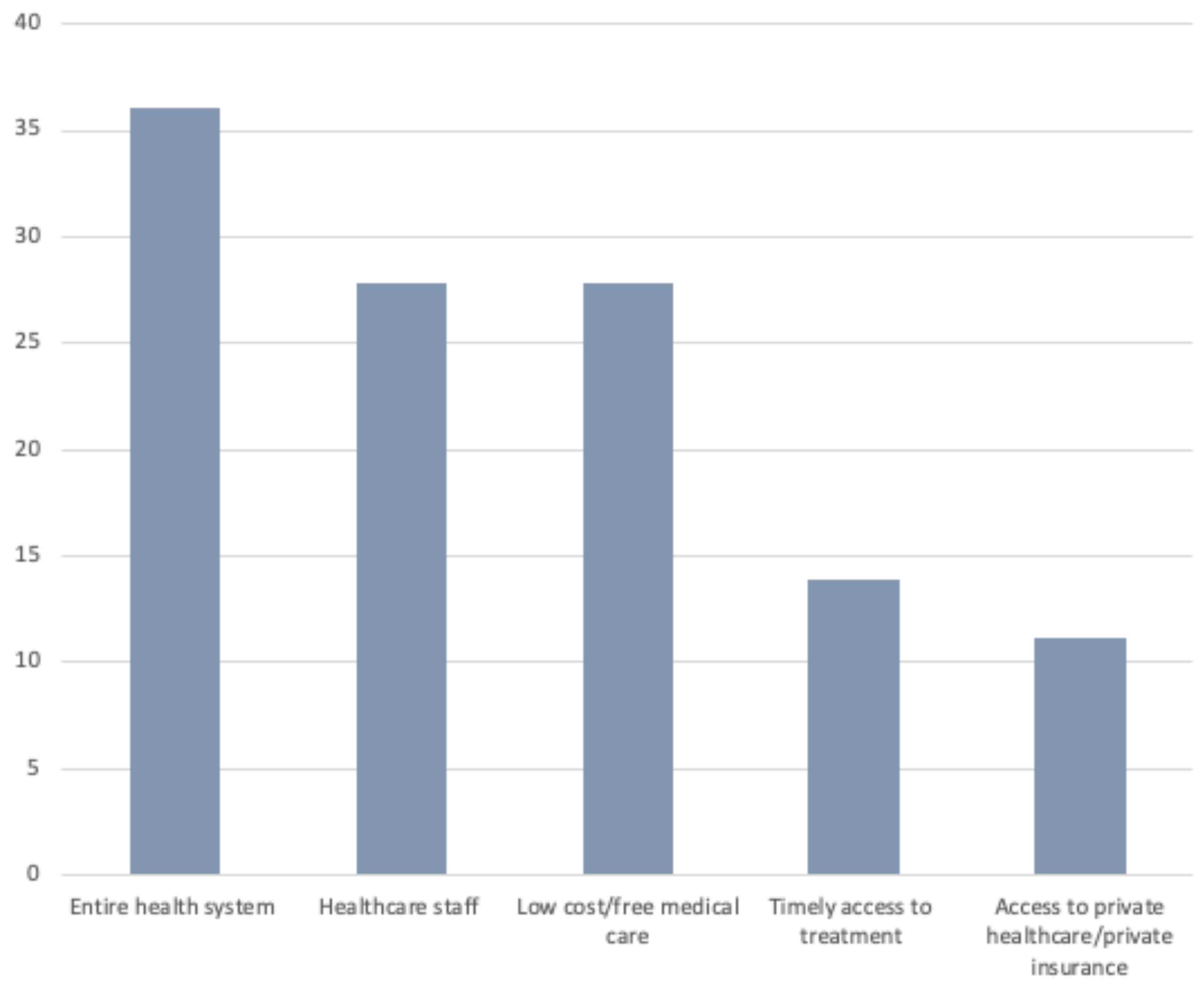


There are people out there miles out in the country that have Amyloidosis and have to come into the hospitals. I believe that there should be accommodation for these people...I feel that there should be people that can pick them up from the airport, take them to places. Participant 004CA

I don't know what other charities there would be, but I would think probably, maybe for a lot of people, it's having someone to talk to, especially in those early days, about it, to find you to get to the right path and know what's available and how to help them would be really helpful. Participant 012ATR

Well, I think I'm pretty all right, but then a person by themselves would probably need a bit of support, someone to talk to or keep an eye on them. As it gets worse, you probably become incapacitated and you'll need probably pretty intensive care. Participant 014ATR

Expectations of future care and support (% all)



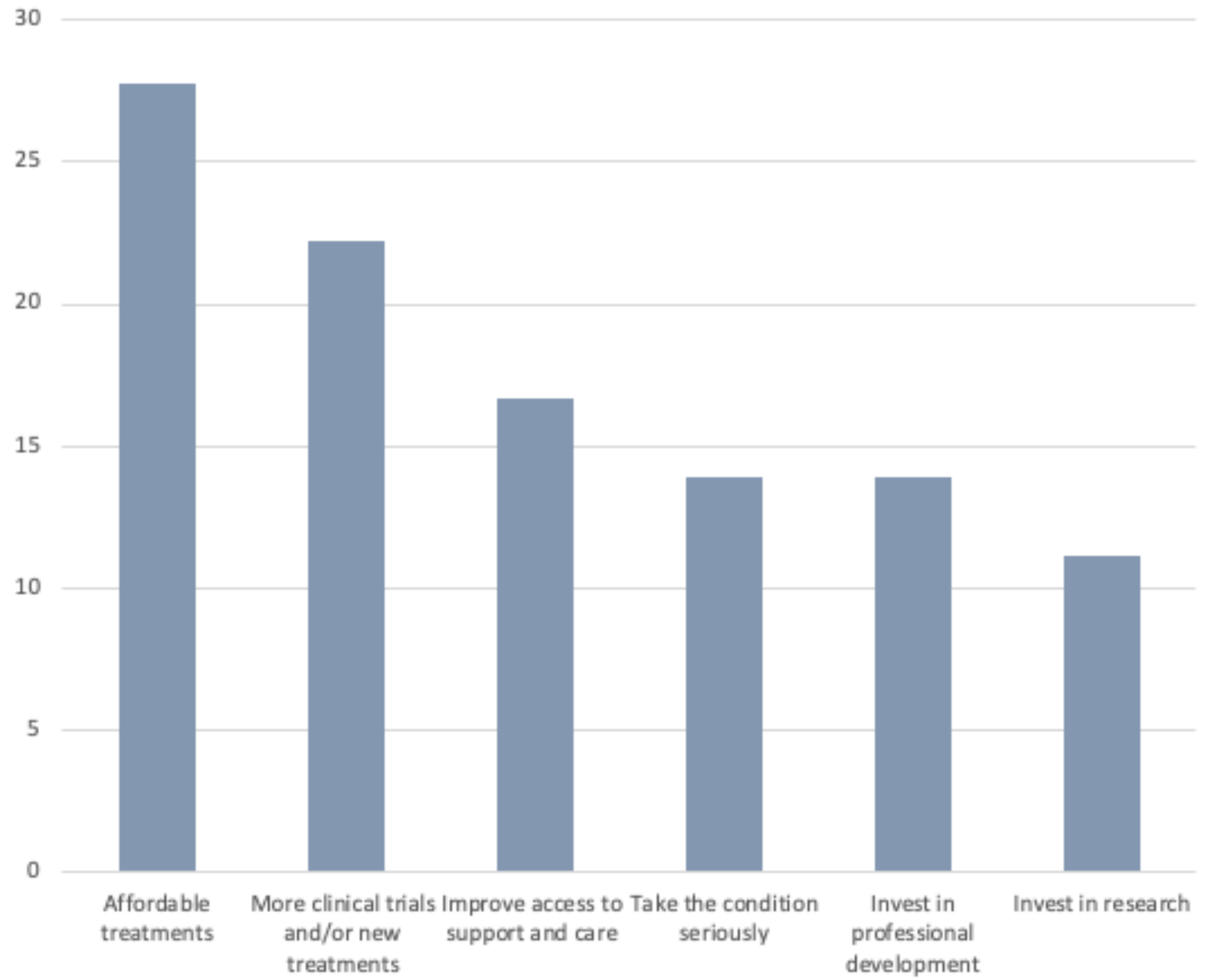
Just for living in the health system that we live in basically. Thank God our taxes go to some good. Participant 001ATR

Yes, I agree with you, we've got a brilliant health system. I think people who complain about the public system in particular need a good boot up the backside because it's probably the best public health system in the world. Participant 002ALX

The Australian health system is second to none. Trust me. I'm just ever so grateful for the assistance that we've had. Participant 003CA

It's been amazing. Australia's health system is amazing…I reckon it's the best health system in the world. Everybody I talk to agrees. LOCATION STATE Health is great. Everybody looks after you. Either prince or pauper, everybody's treated the same, and that's what makes it great. Participant 005AL

Aspects of the health system that people are grateful for (% all)



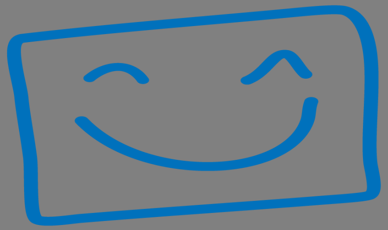
I would say to him that medication that has been shown to be efficacious overseas, and there's plenty of data for that, that medication should be put on the PBS as soon as possible…That should be covered by PBS and that makes it affordable for everybody. Participant 003AL

Probably the big one is the cost of some of the drugs...Supporting those rare diseases is so incredibly important and the sheer cost involved of being sick. We were very fortunate to be close by really good doctors and hospitals and all of that sort of thing, but the people that have got to travel for care that is in then a huge impact on them financially. Participant 003CA

All drug treatments for all cancer patients should be free. Get rid of this, 'It costs too much' bullshit. Just give the people the drugs they need to treat what they've got. Participant 004ATR

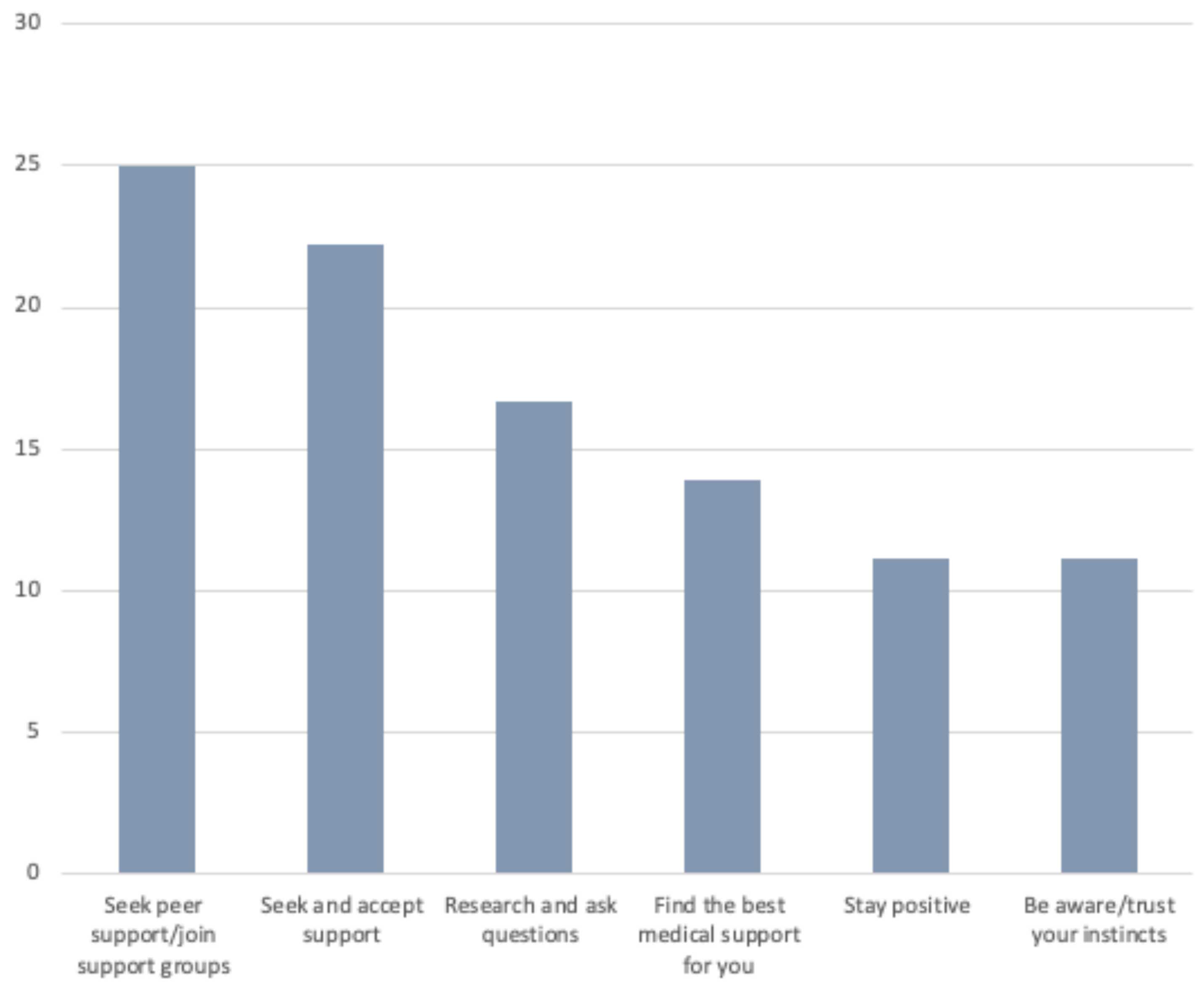
Message to decision-makers (% all)

Section 10



Advice from current patients
to
patients in the future

In this section we present the response to the question 'what is your advice to other people who are diagnosed in the future?' This gives participants the chance to impart knowledge and for patients to feel connected with others in the community.



Talk to others who are in the same boat. Get support from them because those of us who do it find it very helpful realising you're not alone because it is such a rare disease. There aren't that many people around, so get in touch with people attending a group if possible or online or whatever, but try. Participant 001ALX

Other patients or other people with amyloidosis to ring up and chat to each other about that is helpful. Just knowing that you're not alone in these sorts of things. There is someone out there you can reach out to, very important, very balancing. Participant 003ALX

It's good to hear that or someone'd say, 'I've got that too. What do you do? What helps you?' That makes so much of a difference. I think support groups are the most important things in the world. Participant 004CA

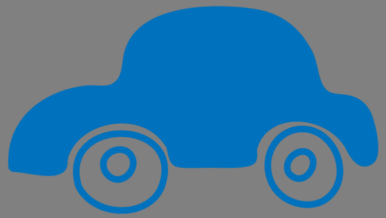
Advice to others in the future (% all)

Section 11

Discussion

The discussion is a review of the current landscape across all of the PEEK domains and commentary on how the PEEK cohort's experience compares to the literature.

Section 12



Where to from here?

The final section summarises three key things that would make life better for people with the condition or disease. There is also a matrix of key findings so that when we repeat the project in the future with a new cohort, we can see whether their experience has improved.

Section 12

1. Information: This is a patient group that is ready for information from the point of diagnosis, however decision-making about treatments is complex and there was a lack of clarity about disease progression and prognosis. This patient population would benefit from more detailed and accessible information about treatment options and discussions about what to expect in the future. This could be aided by the documentation of holistic treatment and care plans with regular revisions.

2. Support: A common theme was the need for specialised support and care, ideally via telephone. This patient population would benefit from a central, dedicated telehealth nurse navigator that can link patients and families to the specific services they need, based on their unique presentation of symptoms. This includes access to mental health support as close to half of the participants noted depression and/or anxiety and the largest gap in information was about psychological/social support.

3. Quality of life: This cohort valued the ability to exercise as a way to maintain their physical and mental health, while the biggest negative impact on quality of life was a reduced capacity for physical activity. This patient population would benefit from targeted physical programs that allow them to exercise within their limitations. This would also have positive social and psychological benefits.

Measure	Mean	Median
Baseline health (SF36)		
Physical functioning	53.47	52.50
Role functioning/physical	37.50	25.00
Role functioning/emotional	62.04	66.67
Energy/fatigue*	43.33	45.00
Emotional well-being	72.44	76.00
Social functioning	60.76	62.50
Pain *	59.58	55.00
General health*	46.81	45.00
Health change	40.28	37.50
Percentage of participants that have accessed My Health Record	39.29	
Percentage of participants that have a discussion about biomarkers/genetic tests	39.29	
Knowledge of condition and treatments (Partners in Health)		
Knowledge	27.36	28.00
Coping	17.68	18.50
Recognition and management of symptoms *	20.68	21.00
Adherence to treatment	15.32	16.00
Total score*	81.04	82.00
Care coordination scale		
Communication*	42.17	42.00
Navigation*	27.56	27.00
Total score*	69.72	72.00
Care coordination global measure	7.92	8.00
Quality of care global measure	8.44	9.00
Fear of progression		
Total Score *	33.19	31.50