Section 5

Treatment

Section 5 Summary: Treatment

Main provider of treatment

- The haematologist was the main provider of amyloidosis treatment for the majority of participants (n=19, 67.86%).

Access to healthcare professionals

- All participants had access to a general practitioner (n=28, 100.00%) and the majority had access to a cardiologist (n=26, 92.86%), and haematologist (n=24, 85.71%) for the treatment of their amyloidosis.

Respect shown

- The majority of participants indicated that they had been treated with respect throughout their experience (n=31, 86.11%), five participants (13.89%) participants felt they had been treated with respect with the exception of one or two occasions, there were no participants who felt they weren't treated with respect.

Healthcare system

- The majority of participants had private healthcare insurance (n=23, 82.14%), five participants (17.86%) asked if they want to be treated as a public or private patient. The majority of participants had not been asked if they had private health insurance (n=15, 53.57%). Throughout their treatment, equal numbers of participants were treated as a public patient (n=11, 39.29%), or private patient (n=11, 39.29%), and most commonly in the public hospital system (n=13, 46.43%) (Table 5.4).

Affordability of healthcare

- The majority of participants never cancelled their appointments due to cost (n=23, 82.14.00%), while four (14.29%) participants rarely had to cancel appointments. Almost all participants (n=27, 96.43%) never had any trouble paying for prescriptions.

Cost of amyloidosis

- Almost all participants never or rarely found it difficult to pay for basic necessities such as housing food and electricity (n=25, 89.29%). There were two participants (7.14%) had to pay for additional carers for themselves or their family. Participants spent between $0 and $1400 per month on amyloidosis. The amount spent was extremely significant or moderately significant burden for 4 participants (14.29%), five found it somewhat significant (17.86%), and 19 participants found costs slightly or not at all significant (67.86%).

Changes to employment status

- Half of the participants (n=18, 50.00%) of this PEEK study were retired at the time of the amyloidosis diagnosis. There were six participant (16.67%) that quit their job, and four (11.11%) reduced their work hours.

- There were 25 (89.29%) participants with a main partner or carer, 13 partners or main carers (46.43%) did not have a job or were retired at the time of diagnosis, seven (25.00%) had no change in employment status, and three (10.71%) quit their job.

Reduced income due to amyloidosis

- A third of participants (32.14%) had a reduced family income due to amyloidosis. Participants noted a drop in monthly income of between $100 to over $5,000 per month. For 18 of these participants (54.54%), the burden of this reduced income was extremely or moderately significant.

Treatment

- The most common drugs taken for *ATTR-cardiac* subgroup were loop-acting diuretics (n=8, 44.44%), followed by doxycycline (n=7, 38.89%), and Diffusional (n=5, 27.78%).

- The most common treatment for *AL-amyloidosis* subgroup was Melphalan and Dexamethasone (50.00%).

Surgery

- There were five participants that had surgery, four participants had a single surgery for amyloidosis, and one patient had four or more surgeries. The types of surgeries that participants had include pacemaker related surgeries, liver transplant, defibrillator fitting, and carpal tunnel surgery.

Lifestyle changes

- Nearly half of the participants made no lifestyle changes (n=13, 46.43%). The most common lifestyle changes were exercise (n=12, 42.86%), and diet (n=9, 32.14%).

Complementary therapies

- There were 24 participants (85.71%) that used some form of complementary therapies to manage their amyloidosis. The most common complementary therapies used were exercise (n=18, 64.29%) and dietary supplements (n=13, 46.43%), and for ATTR-cardiac participant, half weighed themselves daily (n=9, 50.00%).

Clinical trials

- There was a total of 26 participants (92.86%) that had discussions about clinical trials, either by bringing up the topic themselves (n=5, 17.86%) or their doctor bringing up the topic (n=21, 75.00%).

- There was a single participant (3.57%) who had taken part in a clinical trial, and 22 (78.57%) who would like to take part in a clinical trial if there was a suitable one.

Description of mild side effects

- In the structured interview, participants were asked how they would describe the term ‘mild side effects’. The most common description of ‘mild side effects’ was in relation to a specific symptom as an example (n=19, 52.78%). The most common specific side effects given as an example was fatigue and/or tiredness (n=7, 19.44%) followed by diarrhoea (n=4, 11.11%). Another description of ‘mild side effects’ was those that can be self-managed and do not interfere with daily life (n=15, 41.67%).

Description of severe side effects

- In the structured interview, participants were asked how they would describe the term ‘severe side effects’. The most common description of ‘severe side effects’ given was a specific side effect given as an example (n=17, 47.22%). The most common specific side effect given was pain (n=6, 16.67%), followed by neuropathy/sensory disturbance (n=4, 11.11%) and nausea or vomiting (n=4, 11.11%). Other descriptions

of ‘severe side effects’ included those that impact everyday life/ability to conduct activities of daily living (n=12, 33.33%). Four participants described coping with all side effects (11.11%).

Adherence to treatment

- Participants were asked in the structured interview what influences their decision to continue with a treatment regime. The most common theme described was adhering as per the advice of their specialist or as long as its prescribed (n=16, 44.44%). Participants also reported not giving up on any treatment (n=6, 16.67%) and adhering to treatment for a specific amount of time (n=5, 13.89%).

What needs to change to feel like treatment is effective

- Participants were asked to describe what needs to change to feel like treatment is effective. The most common response from 11 participants (30.56%) was needing to experience evidence of stable disease or no disease progression. This was followed by needing to experience an improvement in general wellbeing (n=9, 25.00%).

Information needed to be confident in new treatments

- Participants were asked to describe what information would be needed to be confident in a new treatment. The most common response from17 participants (47.22%) was needing the advice of their clinician followed by 14 participants (38.89%) was needing to know about side effects to feel confident about trying a new treatment. There were 11 participants (30.56%) that reported needing scientific evidence and this was followed by needing to conduct their own research (n=9, 25.00%); needing to know about efficacy (n=9, 25.00%) and needing to know the overall benefits (n=8, 22.22%).

Support needed for treatment at home

- Participants were asked to describe what support they would need if they were having treatment at home. The two most common responses were participants not needing support (n=8, 22.22%) and needing support from their friends or family (n=8, 22.22%). There were seven participants that reported needing regular check-ups with a GP or nurse (19.44%) and this was followed by needing someone to call if they have a question or issue (n=4, 11.11%). Four participants described needing training and education on how to administer treatment.

Main provider of treatment

Participants were asked in the online questionnaire who was the main healthcare professional that provided treatment and management of amyloidosis.

The haematologist was the main provider of amyloidosis treatment for the majority of participants (n=19, 67.86%) (Table 5.1, Figure 5.1).

Table 5.1: Main provider of treatment

Main provider of treatment	Number (n=28)	Percent
Cardiologist (Heart doctor)	3	10.71
Haematologist (blood doctor)	19	67.86
Amyloidosis clinic/specialist team in clinic	4	14.29
Immunologist	1	3.57
Ophthalmologist	1	3.57

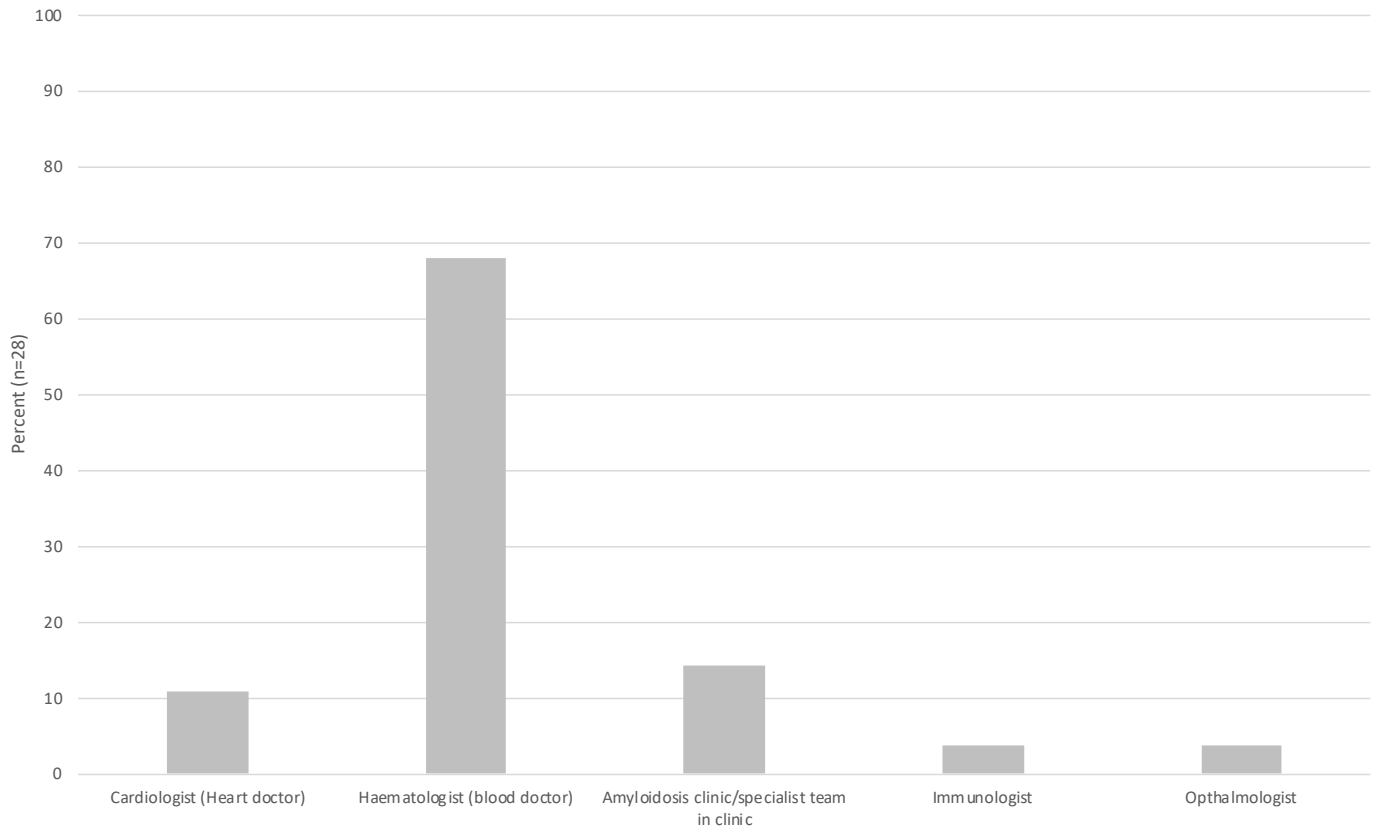

Figure 5.1: Main provider of treatment

Access to healthcare professionals

In the online questionnaire, participants shared the healthcare professionals they had access to for the treatment and management of amyloidosis.

All participants had access to a general practitioner (n=28, 100.00%) and the majority had access to a cardiologist (n=26, 92.86%), and haematologist (n=24, 85.71%) (Table 5.2, Figure 5.2).

Table 5.2: Access to healthcare professionals

Healthcare professional	Number (n=28)	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	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 Counselor	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

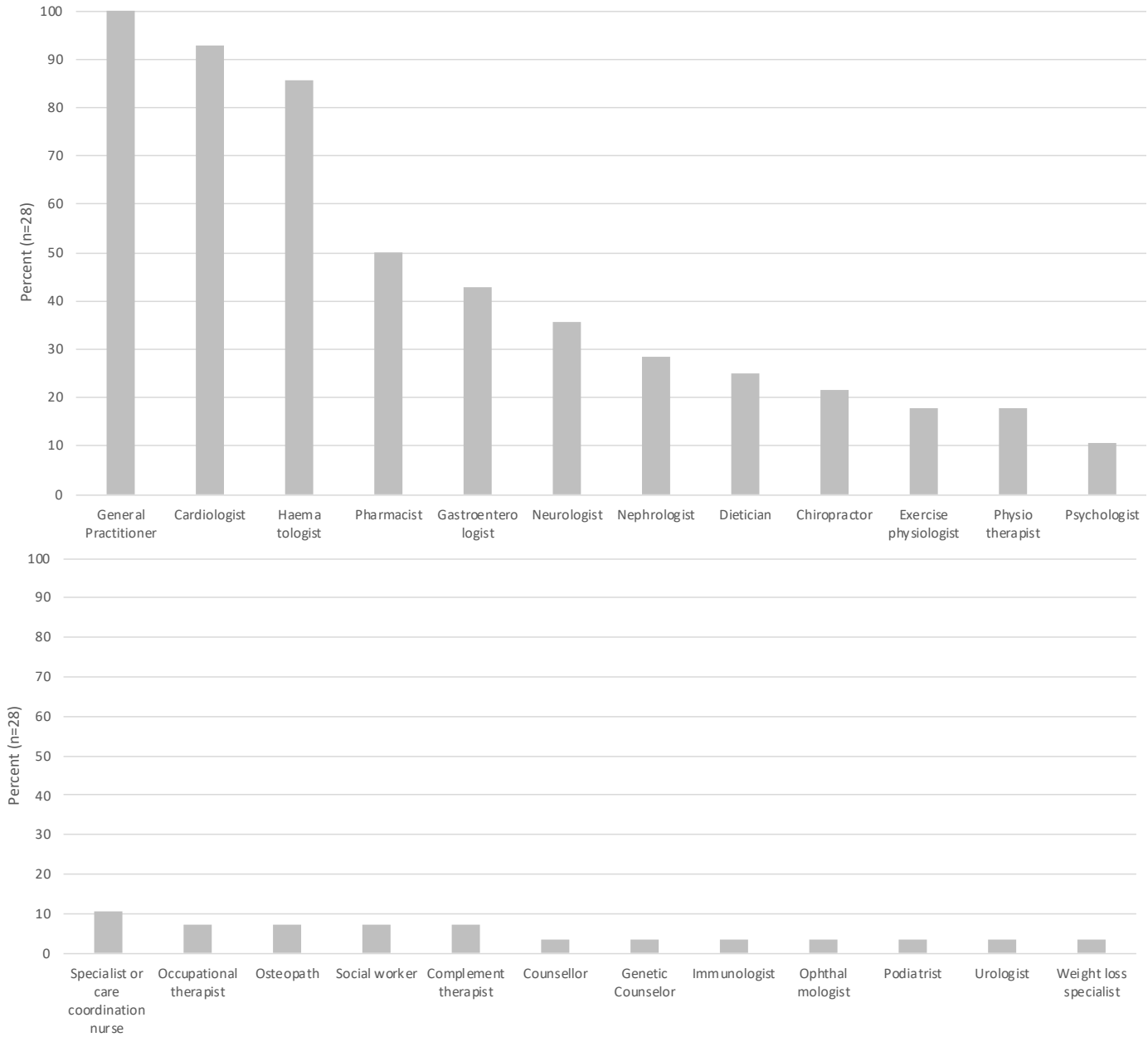

Figure 5.2: Access to healthcare professionals

Respect shown

Participants were asked to think about how respectfully they were treated throughout their experience, this question was asked in the online questionnaire.

The majority of participants indicated that they had been treated with respect throughout their experience (n=31, 86.11%), five participants (13.89%) felt they had been treated with respect with the exception of one or two occasions, there were no participants who felt they weren't treated with respect (Table 5.3, Figure 5.3).

Table 5.3: Respect shown

Respect shown	Number (n=28)	Percent
Yes	31	86.11
Yes, with the exception of one or two occasions	5	13.89
No	0	0.00

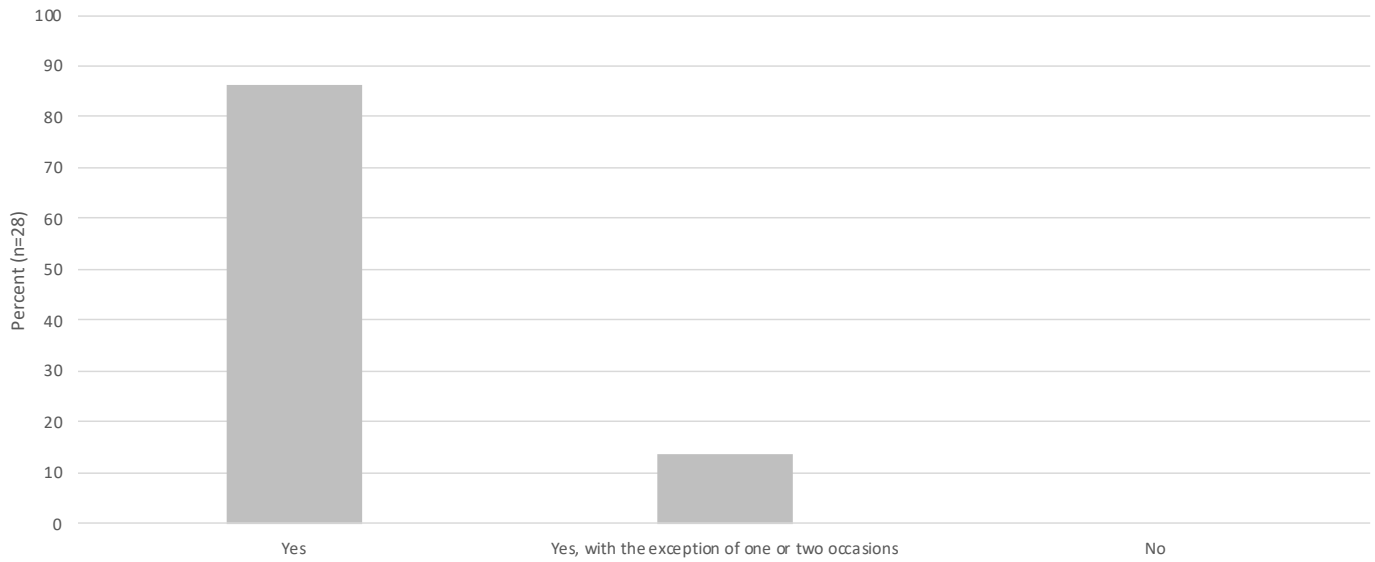

Figure 5.3: Respect shown

Healthcare system

In the online questionnaire, participants were asked questions about the healthcare system they used, about private insurance and about whether they were treated as a public or private patient.

The majority of participants had private healthcare insurance (n=23, 82.14%), five participants (17.86%) asked if they want to be treated as a public or private patient. The majority of participants had not been asked if they had private health insurance (n=15, 53.57%).

Throughout their treatment, equal numbers of participants were treated as a public patient (n=11, 39.29%), or private patient (n=11, 39.29%), and most commonly in the public hospital system (n=13, 46.43%) (Table 5.4).

Table 5.4: Healthcare system

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

Affordability of healthcare

Participants were asked a series of questions about affordability of healthcare in the online questionnaire. The first question was about having to delay or cancer healthcare appointments because they were unable to afford them.

The majority of participants never canceled their appointments due to cost (n=23, 82.14.00%), while four (14.29%) participants rarely had to cancel appointments (Table 5.5, Figure 5.4).

Table 5.5: Healthcare appointments

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

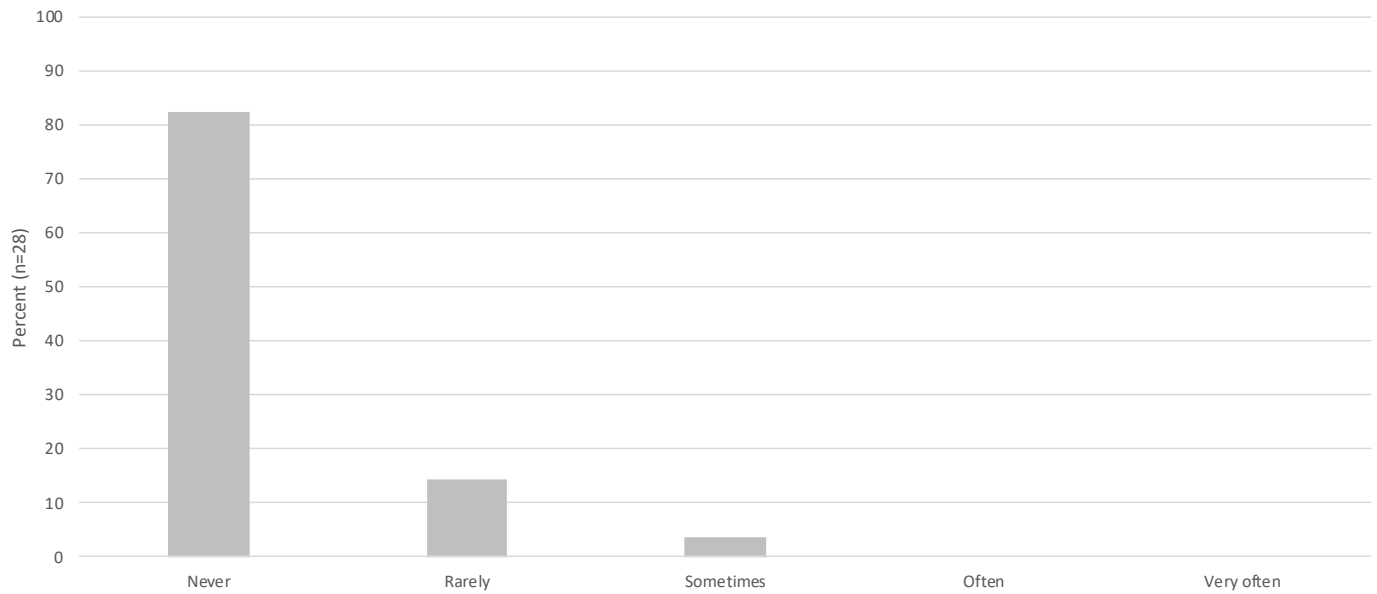

Figure 5.4: Healthcare appointments

Filling prescriptions

Participants were then asked if they were unable to fill prescriptions for essential medicines due to cost.

Almost all participants (n=27, 96.43%) never had any trouble paying for prescriptions (Table 5.6, Figure 5.5).

Table 5.6: Filling prescriptions

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

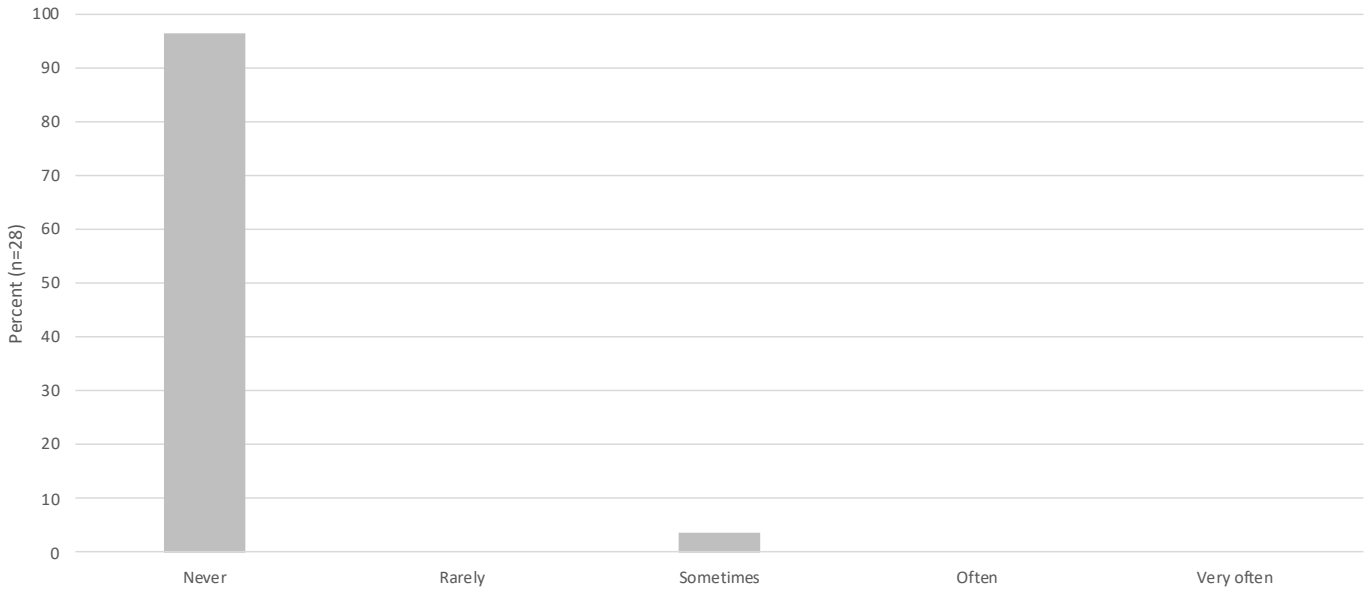

Figure 5.5: Filling prescriptions

Paying for basic essentials

Participants were asked as a result of their diagnosis with amyloidosis, if it made it difficult to pay for basic necessities such as housing, food and electricity.

Almost all participants never or rarely found it difficult to pay for basic necessities such as housing food and electricity (n=25, 89.29%) (Table 5.7, Figure 5.6).

Table 5.7: Paying for basic essentials

Difficult to pay for basic essentials	Number (n=28)	Percent
Never	23	82.14
Rarely	2	7.14
Sometimes	2	7.14
Often	0	0.00
Very often	1	0.00

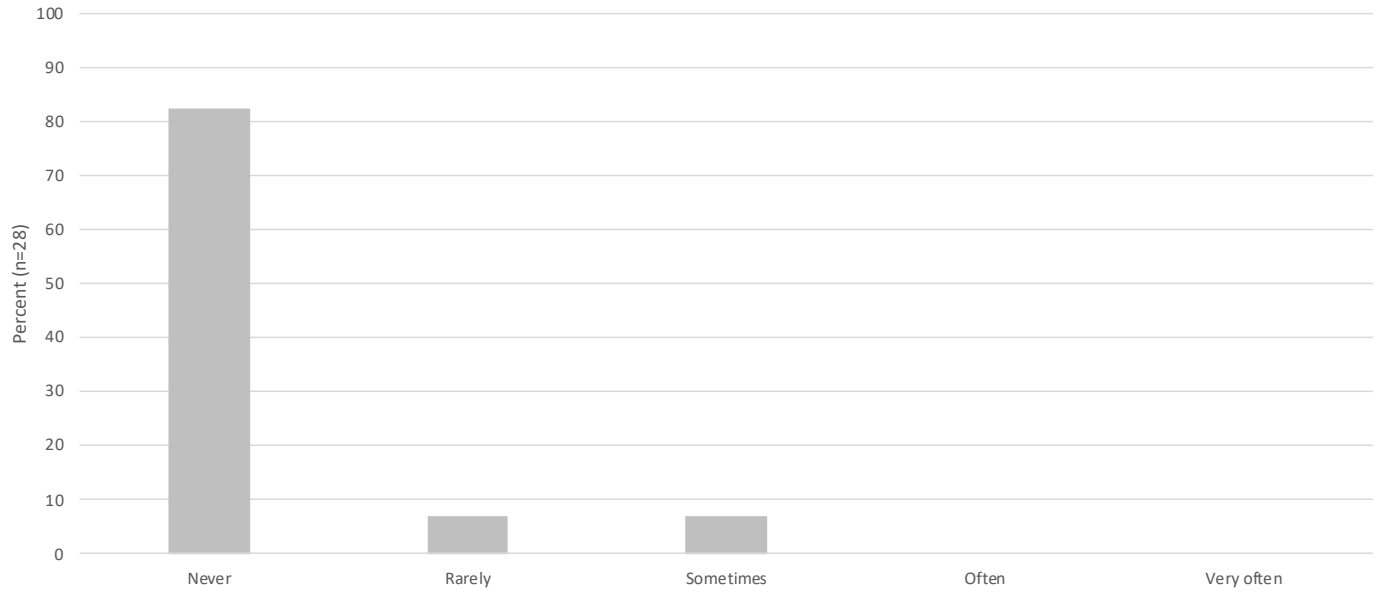

Figure 5.6: Paying for basic essentials

Pay for additional carers

Participants were then asked if as a result of their diagnosis with amyloidosis, if they had to pay for additional carers for themselves or their family.

There were two participants (7.14%) who had to pay for additional carers for themselves or their family (Table 5.8, Figure 5.7).

Table 5.8: Pay for additional carers

Pay for additional carers for self or family	Number (n=28)	Percent
No	26	92.86
Yes	2	7.14

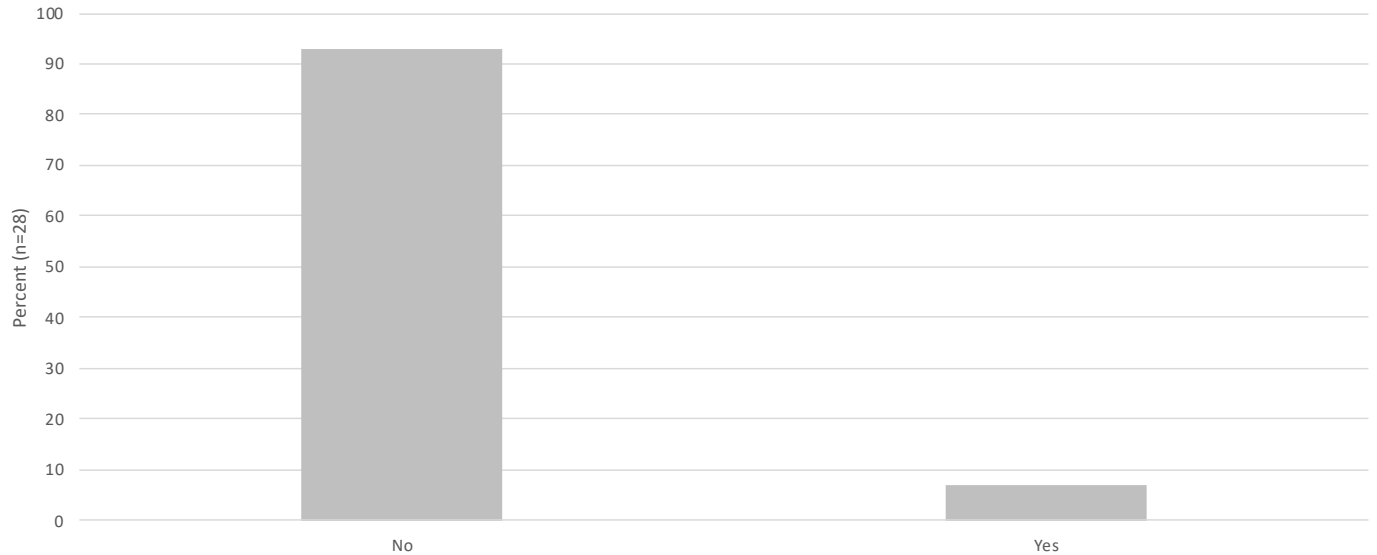

Figure 5.7: Pay for additional carers

Cost of amyloidosis

In the online questionnaire, participants estimated the amount they spend per month due to amyloidosis, including doctors' fees, transport, carers, health insurance gaps and complementary therapies.

Where the response was given in a dollar amount, it is listed in the table below. Overall, participants described spending between $0 and $1400 per month on amyloidosis (Table 5.9, Figure 5.8).

Table 5.9: Cost of amyloidosis

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

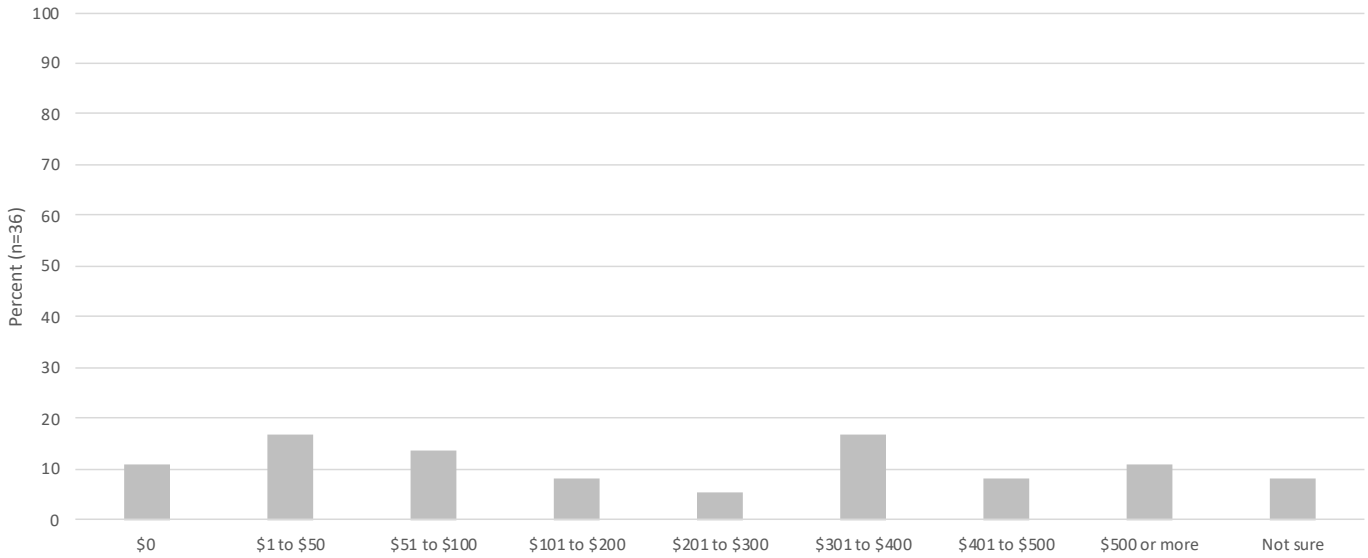

Figure 5.8: Cost of amyloidosis

Burden of cost

As a follow up question, for participants who had monthly expenses due to amyloidosis were asked if the amount spent was a burden.

The amount spent was extremely significant or moderately significant burden for 4 participants (14.29%), five found it somewhat significant (17.86%), and 19 participants found costs slightly or not at all significant (67.86%) (Table 5.10, Figure 9).

Table 5.10: Burden of cost

Burden of 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

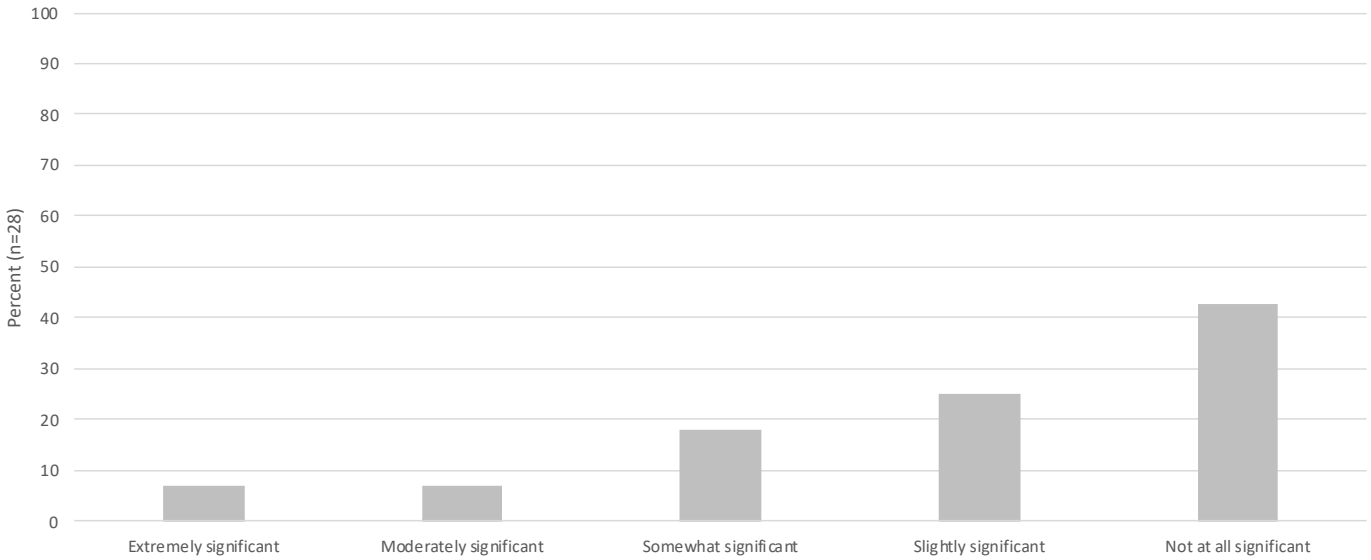

Figure 5.9: Burden of cost

Changes to employment status

Participants were asked, in the online questionnaire, if they had any changes to their employment status due to their condition. Participants were able to choose multiple changes to employment.

Half of the participants (n=18, 50.00%) of this PEEK study were retired at the time of the amyloidosis diagnosis. There were six participant (16.67%) that quit their job, and four (11.11%) reduced their work hours (Table 5.11, Figure 5.10).

Table 5.11: Changes to employment status

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

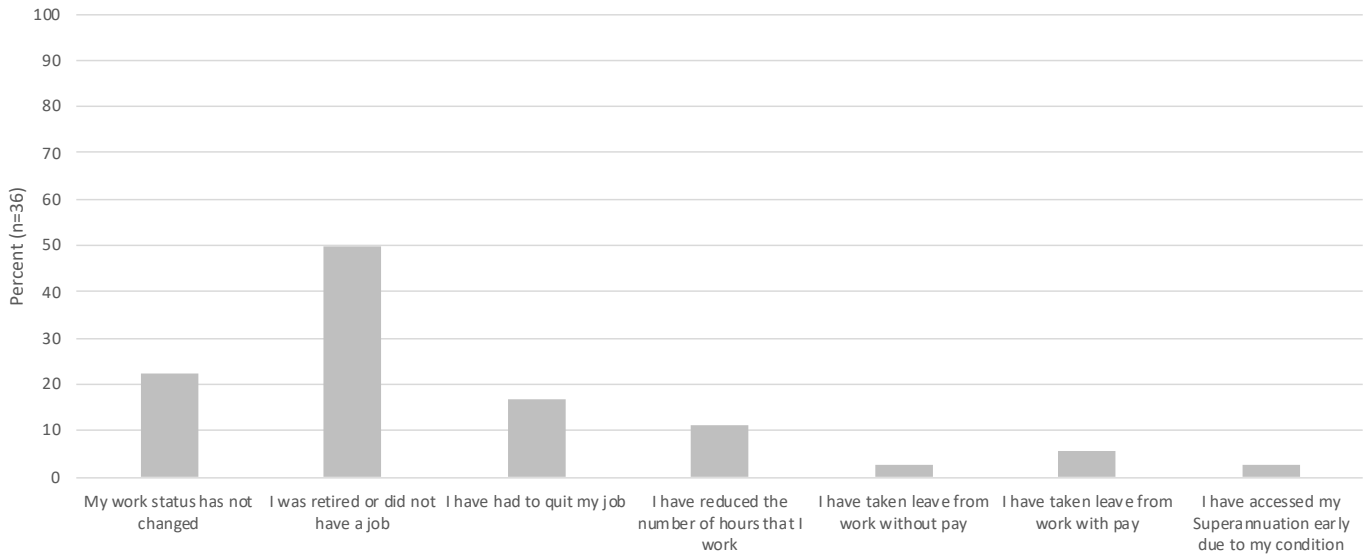

Figure 5.10: Changes to employment status

Changes to partner/main carer employment status

Participants were asked, in the online questionnaire, if they had any changes to the employment status of their carer or partner due to amyloidosis. Participants were able to choose multiple changes to employment.

There were 25 (89.29%) participants with a main partner or carer, 13 partners or main carers (46.43%) did not have a job or were retired at the time of diagnosis, seven (25.00%) had no change in employment status, and three (10.71%) quit their job (Table 5.12, Figure 5.11).

Table 5.12: Changes to partner/main carer employment status

Changes to partner/main carer work	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

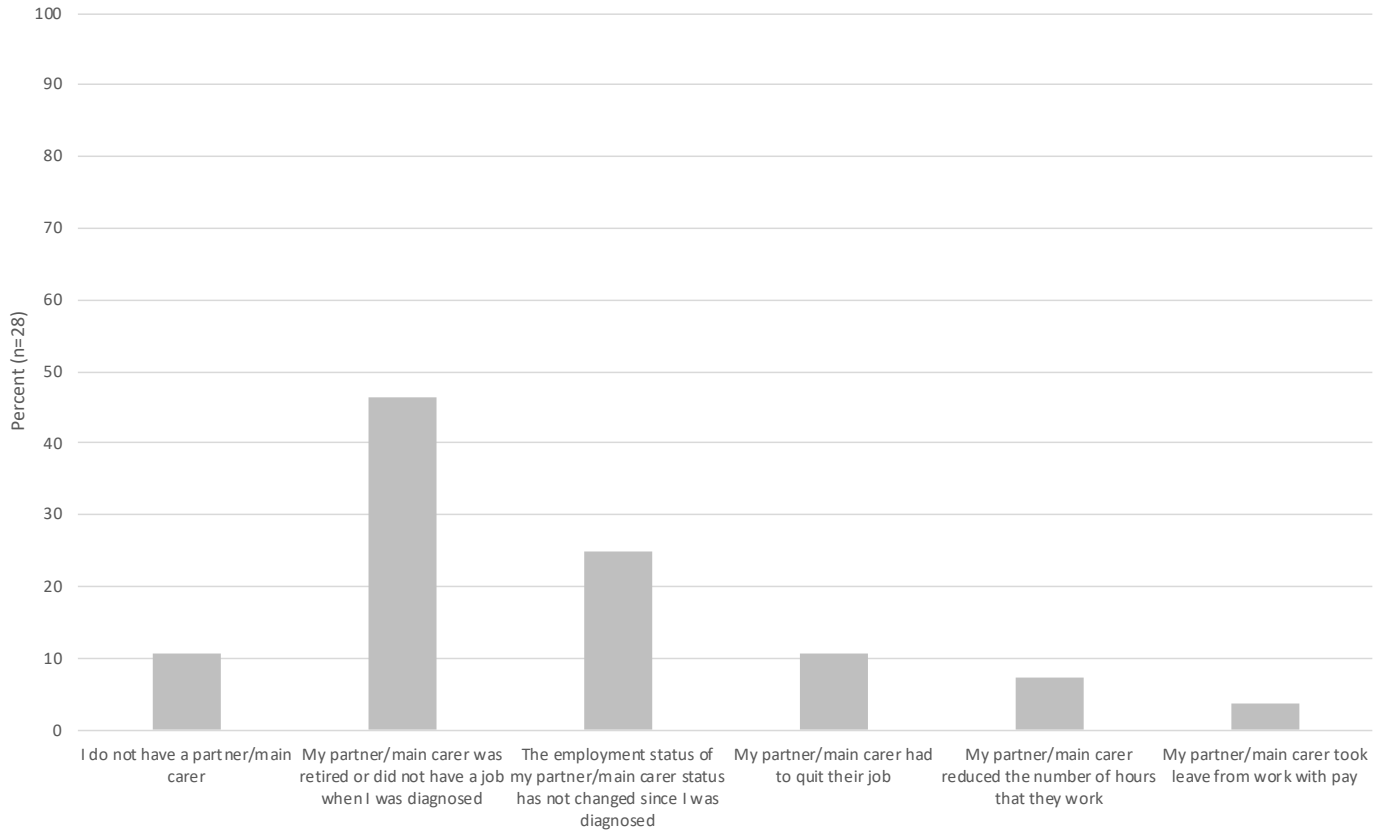

Figure 5.11: Changes to partner/main carer employment status

Reduced income due to amyloidosis

Participants were then asked if they had a reduced family or household income due to amyloidosis.

A third of participants (32.14%) had a reduced family income due to amyloidosis.

Estimated reduction monthly income

As a follow up question, participants were asked if their family or household income had reduced due to amyloidosis. Where a dollar amount was given, it is listed in the table below.

Participants noted a drop in monthly income of between $100 to over $5,000 per month (Table 5.13, Figure 5.12).

Table 5.13: Estimated monthly loss of income

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

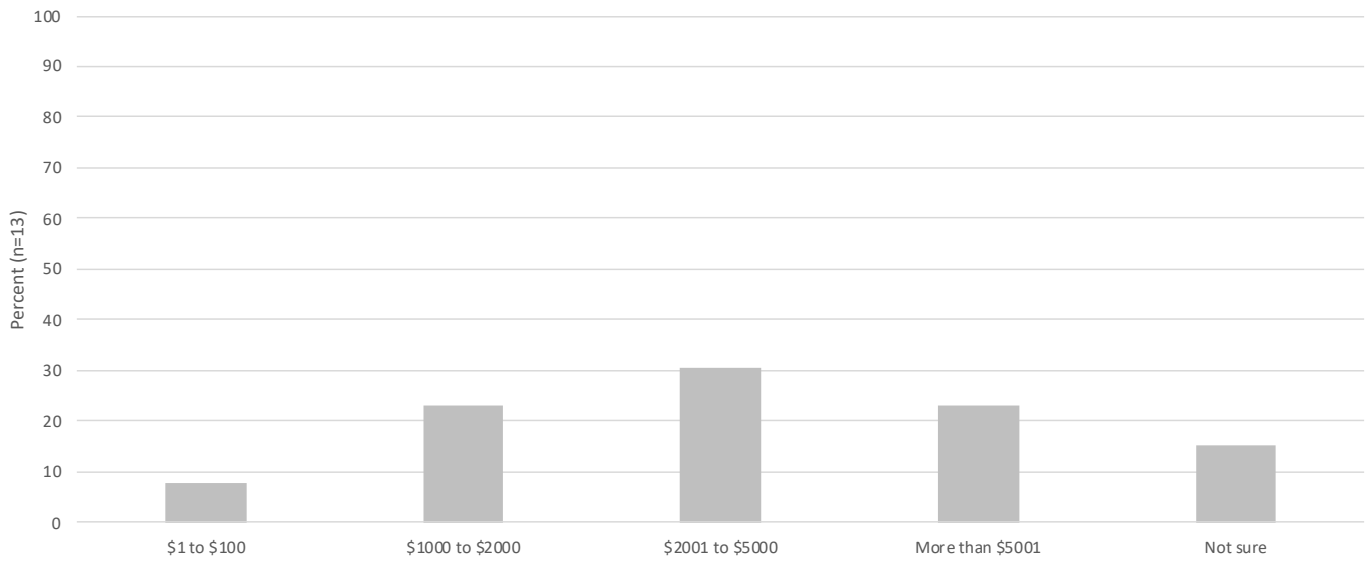

Figure 5.12: Estimated monthly loss of income

Burden of reduced income

Participants were then asked if this reduced family or household income was a burden.

For five of these participants (55.55%), the burden of this reduced income was extremely or moderately significant (Table 5.14, Figure 5.13).

Table 5.14: Burden of reduced income

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

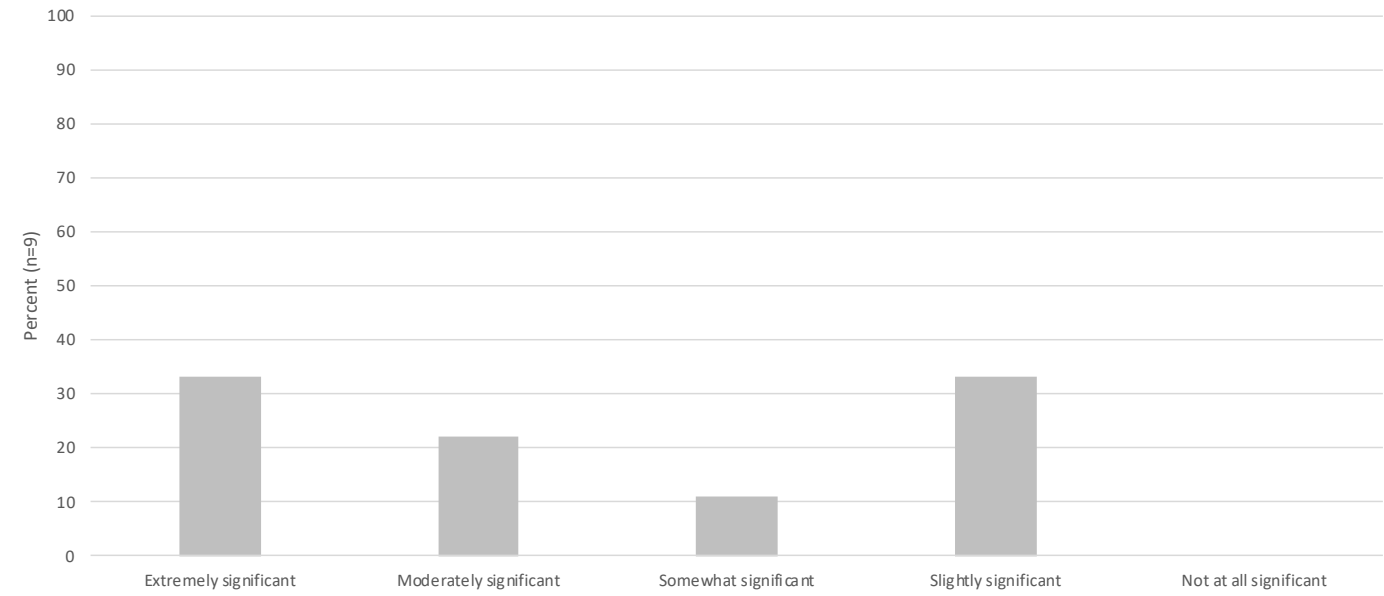

Figure 5.13: Burden of reduced income

Treatment – ATTR

In the online questionnaire, participants answered a series of questions about their treatment, including treatment given, quality of life from treatment, side effects from treatment and how effective they thought the treatment was. A summary of the treatments is given in Table 5.15.

As a follow-up question (within the questionnaire), participants were asked to rate their quality of life on a scale of 1 to 7, while using each specific treatment (with 1 being 'Life was very distressing and 7 being 'Life was great').

Another follow-up question was asked in relation to how effective the participant felt the treatment was on a scale of 1 to 5 (with 1 being ineffective and 5 being very effective).

The most common treatment for ATTR-cardiac was Loop-acting diuretics (n=8, 44.44%), with most participants still taking this treatment. The median quality of life was 3.5, in the life was a little distressing to average range, and the median effectiveness was 4, in the effective range. The next most common treatment was doxycycline (n=7, 38.89%). Most participants were still taking this treatment, the median quality of life was 3, in the life was a little distressing range, and the median effectiveness was 4, in the effective range.

Table 5.15: Summary of treatments for ATTR-cardiac

Treatment summary	Tafamidis	Patisiran	Diflunisal	Doxycycline	Loop-acting diuretics	Potassium-sparing diuretics
Number (n=18)	n=2	n=1	n=5	n=7	n=8	n=5
Percent	11.11	5.56	27.78	38.89	44.44	27.78
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

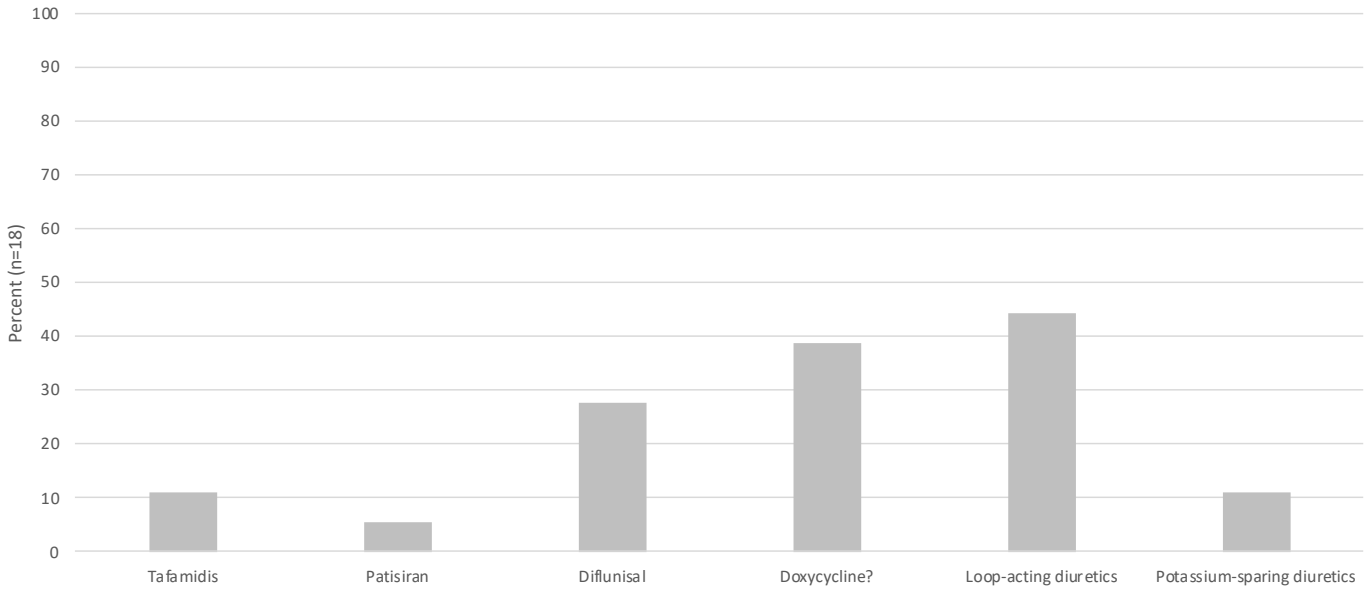

Figure 5.14: Summary of treatments for ATTR-cardiac

Treatment – AL amyloidosis

In the online questionnaire, participants answered a series of questions about their treatment, including treatment given, quality of life from treatment, side effects from treatment and how effective they thought the treatment was. A summary of the treatments is given in Table 5.16.

As a follow-up question (within the questionnaire), participants were asked to rate their quality of life on a scale of 1 to 7, while using each specific treatment (with 1 being 'Life was very distressing and 7 being 'Life was great').

Another follow-up question was asked in relation to how effective the participant felt the treatment was on a scale of 1 to 5 (with 1 being ineffective and 5 being very effective).

The most common treatment for AL amyloidosis was Melphalan and Dexamethasone (n=5, 50.00%), the median quality of life was 2 in the life was distressing range, and the median effectiveness was 4, in the effective range. Bortezomib, Cyclophosphamide, Dexamethasone was taken by 5 participants with AL amyloidosis (50.00%). The median quality of life was 3 in the life was a little distressing range, and the median effectiveness was 3, in the moderately effective range.

Table 5.16: Summary of treatments AL amyloidosis

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

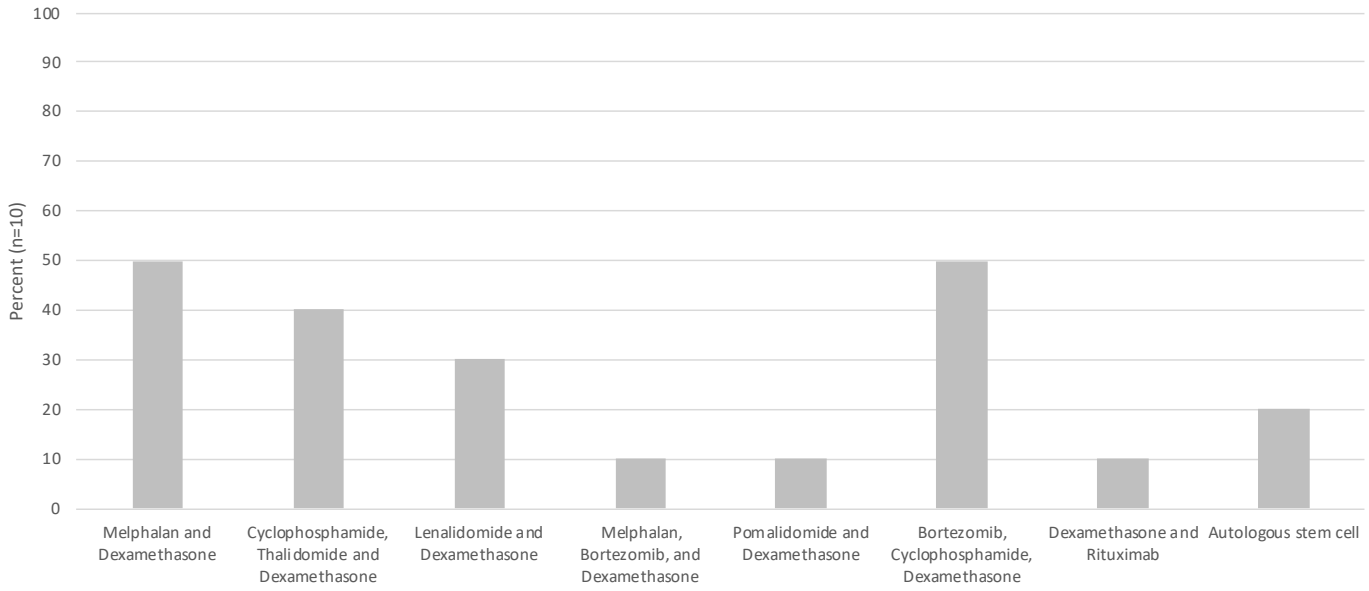

Figure 5.15: Summary of treatments AL amyloidosis

Surgery

In the online questionnaire, participants noted which surgeries they had for the treatment of amyloidosis, excluding biopsies.

There were five participants that had surgery, four participants had a single surgery for amyloidosis, and one patient had four or more surgeries. The types of surgeries that participants had include pacemaker related surgeries, liver transplant, defibrillator fitting, and carpal tunnel surgery (Table 5.17).

Table 5.17: Summary of surgeries

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 since diagnosis

Participants selected from a list the lifestyle changes they had made since being diagnosed with amyloidosis.

As a follow-up question (within the questionnaire), participants were asked to rate their quality of life on a scale of 1 to 7, while using each specific treatment (with 1 being 'Life was very distressing and 7 being 'Life was great').

Another follow-up question was asked in relation to how effective the participant felt the treatment was on a scale of 1 to 5 (with 1 being ineffective and 5 being very effective).

Nearly half of the participants made no lifestyle changes (n=13, 46.43%). The most common lifestyle changes were exercise (n=12, 42.86%), and diet (n=9, 32.14%) (Table 5.18, Figure 5.16).

Table 5.18: Lifestyle changes

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

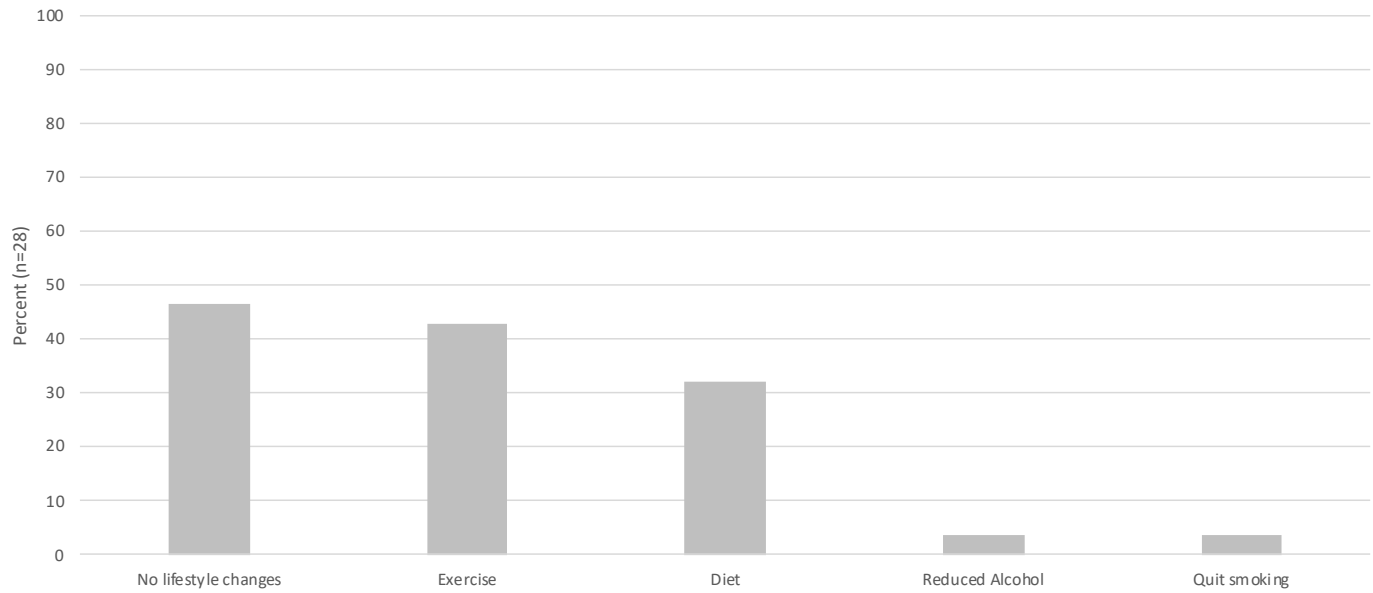

Figure 5.16: Lifestyle changes

Complementary therapies

In the online questionnaire, participants noted the complementary therapies that they used. In particular, they noted their experience of relaxation techniques, massage therapy, acupuncture, dietary supplements, homeopathy, and naturopathy.

As a follow-up question (within the questionnaire), participants were asked to rate their quality of life on a scale of 1 to 7, while using each specific treatment (with 1 being 'Life was very distressing and 7 being 'Life was great').

Another follow-up question was asked in relation to how effective the participant felt the treatment was on a scale of 1 to 5 (with 1 being ineffective and 5 being very effective).

There were 24 participants (85.71%) that used some form of complementary therapies to manage their amyloidosis.

The most common complementary therapies used were exercise (n=18, 64.29%) and dietary supplements (n=13, 46.43%), and for ATTR-cardiac participant, half weighed themselves daily (n=9, 50.00%) (Table 5.19).

The median quality of life for the most common complementary therapies are as follows: the median for exercise was 4.5, in the life was average to good range, the median quality of life for supplements was 3, in the life was a little distressing range, and the median quality of life for daily weighing was 4, in the life was average range.

The median effectiveness for exercise, and daily weighing was 3, in the moderately effective range. The median effectiveness for supplements was 2, in the somewhat effective range.

Table 5.19: Complementary therapies summary

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 effecti

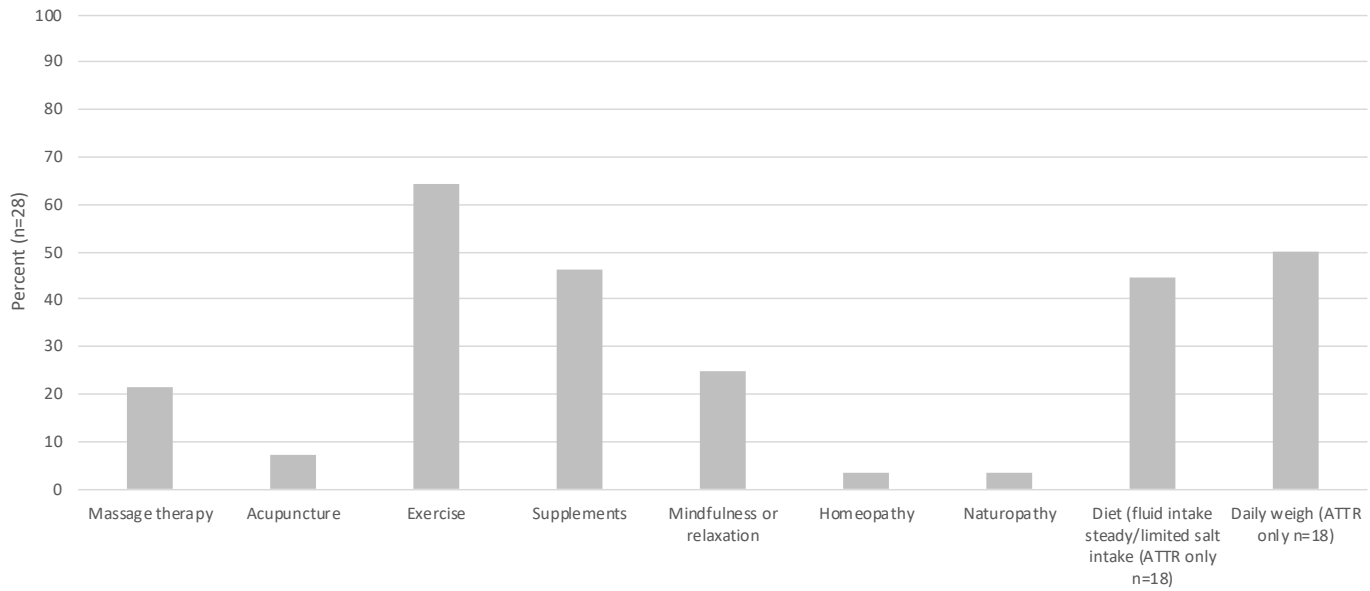

Figure 5.17: Complementary therapies

Clinical trials discussions

In the online questionnaire, participants were asked if they had discussions with their doctor about clinical trials, and if they did, who initiated the discussion.

There was a total of 26 participants (92.86%) that had discussions about clinical trials, either by bringing up the topic themselves (n=5, 17.86%) or their doctor bringing up the topic (n=21, 75.00%) (Table 5.20, Figure 5.18).

Table 5.20: Discussions about clinical trials

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

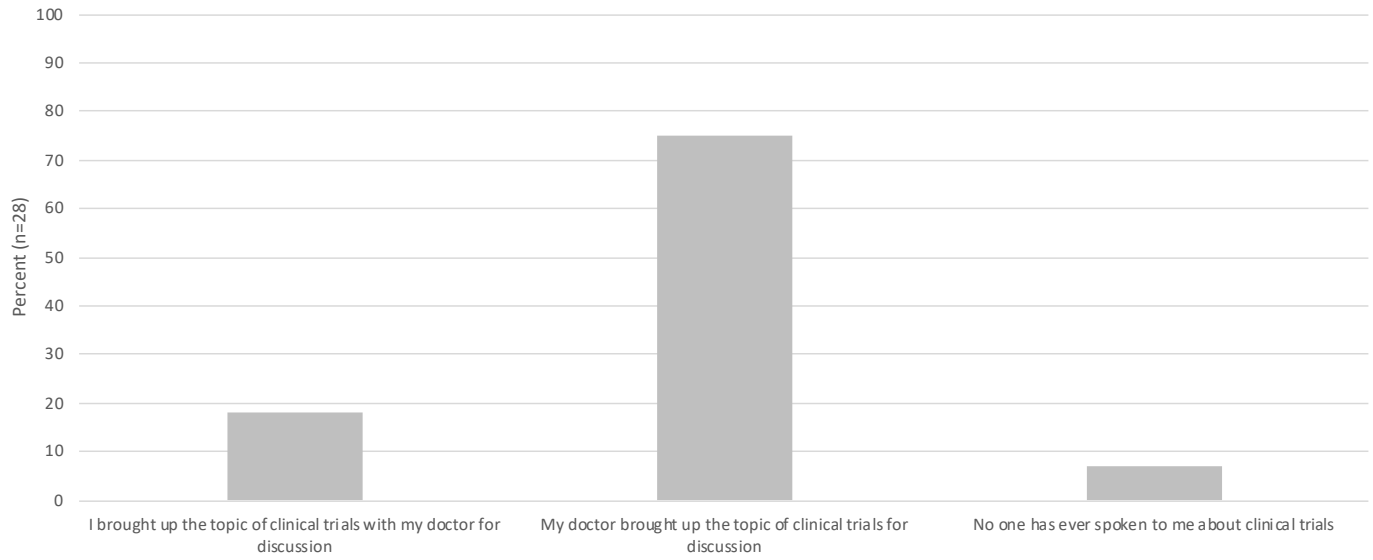

Figure 5.18: Discussions about clinical trials

Clinical trial participation

As a follow up question, participants were asked if they had taken part in a clinical trial, and if they had not taken part if they were interested in taking part.

There was a single participant (3.57%) who had taken part in a clinical trial, and 22 (78.57%) who would like to take part in a clinical trial if there was a suitable one (Table 5.21, Figure 5.19).

Table 5.21: Clinical trial participation

Clinical trial participation	Number (n=28)	Percent
I have not participated in a clinical trial and do not want to	5	17.86
I have not participated in a clinical trial but would like to if there is one for me	22	78.57
I have participated in a clinical trial	1	3.57

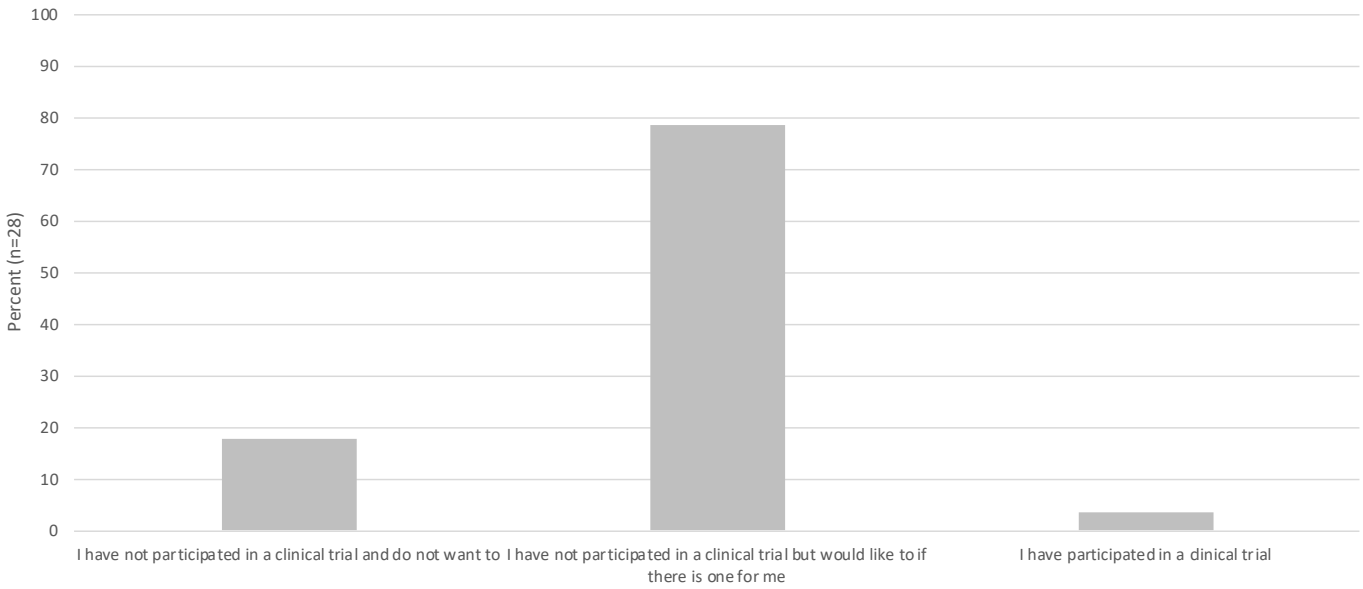

Figure 5.19: Clinical trial participation

Description of mild side effects

In the structured interview, participants were asked how they would describe the term 'mild side effects'. The most common description of mild side effects was in relation to a specific symptom as an example (n=19, 52.78%). The most common specific side effects given as an example was fatigue and/or tiredness (n=7, 19.44%) followed by diarrhoea (n=4, 11.11%). Another description of mild side effects was those that can be self-managed and do not interfere with daily life (n=15, 41.67%).

The general population (19.44%) described mild side effects as fatigue and/or tiredness, while participants in *Regional or remote* subgroup did not describe this at all (0.00%).

Participants in the subgroups *Aged 75 or older* (25.00%) and *Female* (21.43%) described mild side effects as diarrhoea more frequently than the general population (11.11%), while those in subgroups *Aged 55 to 64* (0.00%), *Regional or remote* (0.00%), and *Mid to low SEIFA* (0.00%) did not describe this at all.

Overall participants in the subgroups *Aged 75 or older* (87.50%) and *Trade or high school* (64.29%) described a specific side as an example more frequently than the general population (52.78%), while participants in the *Carer* (37.50%), and *Aged 55 to 64* (25.00%) subgroups described this less frequently.

Participants in the subgroups *AL amyloidosis* (60.00%), *Aged 55 to 64* (62.50%), *University* (64.29%) and *Higher SEIFA* (52.00%) described mild side effects as those that can be self-managed and do not interfere with daily life more frequently than the general population (41.67%) while those in the subgroups *Trade or high school* (28.57%), *Mid to low SEIFA* (18.18%), *Aged 65 to 74* (26.32%), and *Carer* (25.00%) described this less frequently.

Example provided to describe mild side effects

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

Self-managed/Do not interfere with life

Mild, is just so that I can continue on with your life, but yes, it's an inconvenience, so I guess it's mild. Participant 001ALX

I think everything that I had I would probably term mild because I could cope with them. I didn't feel I needed any additional medication to prevent sickness or diarrhoea or things like that. They were all manageable. I think mild side effects to me are manageable and hopefully, they're relatively short term. Participant 002AL

It's inconvenient, but I'm able and understand how to control it. Participant 001AL

Fatigue/tiredness

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

Diarrhoea

Can I also probably put in there, in the mild, I had constant diarrhoea and constant gas problems. That's been virtually constant forever since the diagnosis. Again, sometimes the bowel movement becomes urgent, which can be very inconvenient, of course, if you're doing things. I control this with Imodium. Participant 001AL

I don't know the answer really to that because for NAME HUSBAND, one of the side effects of just about every drug he is on is diarrhoea, and the diarrhoea is part of his disease right from the very beginning. Participant 002CA

My bowels as I said, over the last few months, have been quite loose, and a doctor has been giving me- a specialist has been giving me some tablets for that. Participant 010ATR

Table 5.22: Description of mild side effects

Description of mild side effects	All participants		ATTR-cardiac		All cardiac		AL amyloidosis		Carer		Male		Female		Regional or remote		Metropolitan	
	n=36	%	n=18	%	n=25	%	n=10	%	n=8	%	n=22	%	n=14	%	n=9	%	n=27	%
Participant describes mild side effects giving the specific example of fatigue/tiredness	7	19.44	3	16.67	5	20.00	2	20.00	2	25.00	4	18.18	3	21.43	0	0.00	7	25.93
Participant describes mild side effects giving the specific example of diarrhoea	4	11.11	1	5.56	3	12.00	2	20.00	1	12.50	1	4.55	3	21.43	0	0.00	4	14.81
Participant provides a specific side effect as an example	19	52.78	10	55.56	15	60.00	6	60.00	3	37.50	13	59.09	6	42.86	4	44.44	15	55.56
Participant describes mild side effects as those that can be self-managed and do not interfere with daily life	15	41.67	7	38.89	11	44.00	6	60.00	2	25.00	9	40.91	6	42.86	4	44.44	11	40.74

Description of mild side effects	All participants		Aged 55 to 64		Aged 65 to 74		Aged 75 or older		Trade or high school		University		Mid to low SEIFA		Higher SEIFA	
	n=36	%	n=8	%	n=19	%	n=8	%	n=14	%	n=14	%	n=11	%	n=25	%
Participant describes mild side effects giving the specific example of fatigue/tiredness	7	19.44	2	25.00	4	21.05	1	12.50	3	21.43	2	14.29	2	18.18	5	20.00
Participant describes mild side effects giving the specific example of diarrhoea	4	11.11	0	0.00	2	10.53	2	25.00	1	7.14	2	14.29	0	0.00	4	16.00
Participant provides a specific side effect as an example	19	52.78	2	25.00	10	52.63	7	87.50	9	64.29	7	50.00	5	45.45	14	56.00
Participant describes mild side effects as those that can be self-managed and do not interfere with daily life	15	41.67	5	62.50	5	26.32	4	50.00	4	28.57	9	64.29	2	18.18	13	52.00

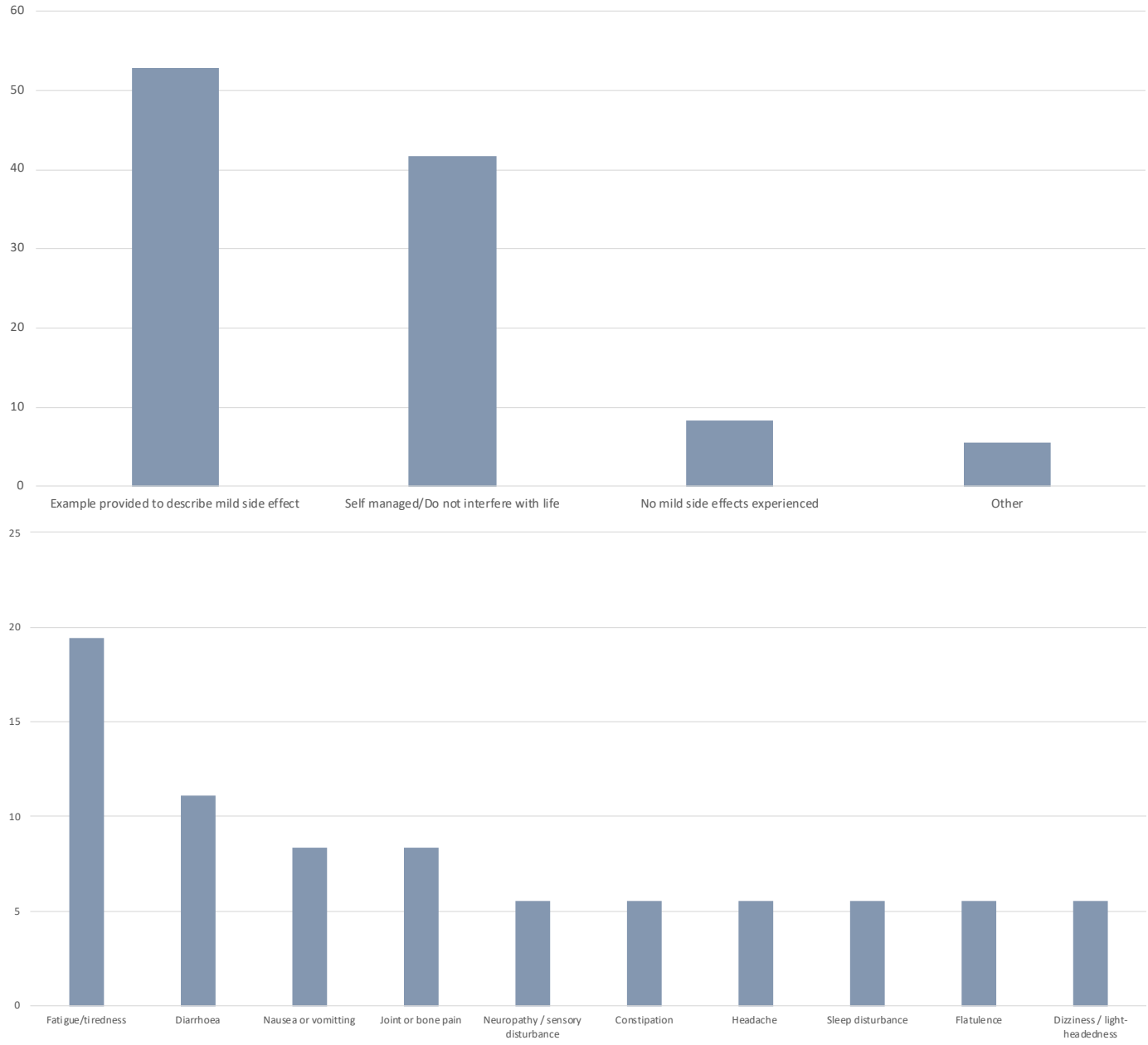

Figure 5.20: Description of mild side effects

Description of severe side effects

In the structured interview, participants were asked how they would describe the term 'severe side effects'. The most common description of severe side effects given was a specific side effect given as an example (n=17, 47.22%). The most common specific side effect given was pain (n=6, 16.67%), followed by neuropathy/sensory disturbance (n=4, 11.11%) and nausea or vomiting (n=4, 11.11%). Other descriptions of severe side effects included those that impact everyday life/ability to conduct activities of daily living (n=12, 33.33%). Four participants described coping with all side effects (11.11%).

In relation to subgroup variations, participants in the ATTR-cardiac (5.56%) subgroup described severe side effects as pain less frequently than the general population (16.67%), while those in the subgroups *AL amyloidosis* (50.00%) and *University* (28.57%) described this more frequently. Participants in the *Carer* (0.00%) subgroup did not describe this at all.

Participants in the *Regional or remote* (22.22%) and *University* (21.43%) subgroups described severe side effects as neuropathy more frequently than the general population (11.11%), while those in the *Carer* (0.00%), and *Female* (0.00%) subgroups did not describe this at all.

Participants in the *Regional or remote* subgroup described severe side effects as nausea or vomiting more frequently (22.22%) than the general population (11.11%).

Overall, participants in the subgroups *AL amyloidosis* (60.00%), *Aged 55 to 64* (62.50%), and *Regional or remote* (66.67%) described severe side effects as a specific side effect more frequently than the general population (47.22%) while those in the subgroup *Aged 75 or older* (25.00%) describe this less often.

Participants in the subgroups *Aged 55 to 64* (50.00%), *Regional or remote* (44.44%), and *Mid to low SEIFA* (45.45%) described severe side effects as those that impact everyday life/ability to conduct activities of daily living more frequently than the general population (33.33%) while those in the *Aged 75 or older* subgroup (12.50%) described this less frequently.

Participants in the *Aged 75 or older* (37.50%), and *Trade or high school* (28.57%) subgroups described coping with all side effects more frequently than the general population (11.11%), while those in the *Carer* (0.00%), *Aged 55 to 64* (0.00%), *University* (0.00%), and *Regional or remote* (0.00%) subgroups did not describe this at all.

Example provided to described severe side effect

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

For instance, we have Dex. 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, hospitalized, couldn't move, couldn't stand up, couldn't do anything. That was severe pain. Participant 005AL

Impact everyday life/conduct daily living

Well, as you mentioned there, I couldn't continue with my normal life. It put me out of work and possibly admitted to hospital. Participant 001ALX

Severe where it gets a problem or something that becomes much bigger, and it becomes a roadblock. If he's too sick to be doing something. If it interferes with the day to day running of your life a lot, then to me that's more severe. Participant 003CA

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. Haemorrhoids definitely ended up in that category, difficult sitting, difficult making bowel movement, et cetera. The discomfort was definitely in the severe or significant. The neuropathy, at times, got like that, not often but from time to time. Participant 004AL

Coped with all side effects/Had to

Well, I could cope with them, it was just that anybody around me couldn't cope with me, that's the dexamethasone. Participant 002ALX

Like if I had it in my heart or something like that, that would be very disturbing, but I guess I would cope with it because I'm the sort of person who thinks, if you've got that you just cope with it. If they told me there's no treatment, well, I guess I'd accept it and just think that's the way it is.
. Participant 017ATR

Well, I coped with them all. I thought one of the severe side effects was weight loss. I guess I lost about 20 kilos. Yes, I don't think I had any real severe side effects. Participant 017

Pain

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

Neuropathy/Sensory disturbance

The skin-- Paresthesia and that's on the chest. Again, that ranges from mild to sometimes quite severe in the sense that it's like jabs. Participant 001AL

The neuropathy, at times, got like that, not often but from time to time. Participant 004AL

That's like the stage where I am now with different things, with the neuropathy, my shortness of breath. My eyes, I've got problems with my eyes. My feet, my hands, I've got the carpal tunnel in my hands really bad. In my feet, I've got no feeling in my hands or my feet. In my mouth, the left side of my face. I've got no taste, no smell. It's all gone. Participant 009ATR

Nausea or vomiting

I think that when once he started chemotherapy, severe [clears throat] side effects were nausea and not being able to eat. In fact, not totally not able to eat but I had to choose pretty carefully about what I prepared for food. Participant 001CA

You have an ability to cope with things and if I felt that I was not able to cope with the side effects, then I would probably label it as a severe side effect. 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

Again, nausea, aches, pains, not being able to see straight, that sort of stuff, yes. Stuff that would-- for instance, under the mild side effects I start working-- I'm back at work three days a week part time so I can deal with that. Severe side effects I wouldn't be doing all that, so yes. Participant 004ATR

Table 5.23: Description of severe side effects

Description of severe side effects	All participants		ATTR-cardiac		All cardiac		AL amyloidosis		Carer		Male		Female		Regional or remote		Metropolitan	
	n=36	%	n=18	%	n=25	%	n=10	%	n=8	%	n=22	%	n=14	%	n=9	%	n=27	%
Participant describes severe side effects giving the specific example of pain	6	16.67	1	5.56	6	24.00	5	50.00	0	0.00	5	22.73	1	7.14	2	22.22	4	14.81
Participant describes severe side effects giving the specific example of neuropathy/sensory disturbance e.g. tingling or numbness	4	11.11	2	11.11	4	16.00	2	20.00	0	0.00	4	18.18	0	0.00	2	22.22	2	7.41
Participant describes severe side effects giving the specific example of nausea or vomiting	4	11.11	2	11.11	3	12.00	1	10.00	1	12.50	2	9.09	2	14.29	2	22.22	2	7.41
Participant provides a specific side effect as an example	17	47.22	8	44.44	14	56.00	6	60.00	3	37.50	11	50.00	6	42.86	6	66.67	11	40.74
Participant describes severe side effects as those that impact everyday life/ability to conduct activities of daily living	12	33.33	7	38.89	9	36.00	3	30.00	2	25.00	8	36.36	4	28.57	4	44.44	8	29.63
Participant describes coping with all side effects (because you have to or it's all that they've known)	4	11.11	2	11.11	3	12.00	2	20.00	0	0.00	3	13.64	1	7.14	0	0.00	4	14.81

Description of severe side effects	All participants		Aged 55 to 64		Aged 65 to 74		Aged 75 or older		Trade or high school		University		Mid to low SEIFA		Higher SEIFA	
	n=36	%	n=8	%	n=19	%	n=8	%	n=14	%	n=14	%	n=11	%	n=25	%
Participant describes severe side effects giving the specific example of pain	6	16.67	1	12.50	4	21.05	1	12.50	2	14.29	4	28.57	1	9.09	5	20.00
Participant describes severe side effects giving the specific example of neuropathy/sensory disturbance e.g. tingling or numbness	4	11.11	1	12.50	2	10.53	1	12.50	1	7.14	3	21.43	1	9.09	3	12.00
Participant describes severe side effects giving the specific example of nausea or vomiting	4	11.11	1	12.50	2	10.53	1	12.50	1	7.14	2	14.29	1	9.09	3	12.00
Participant provides a specific side effect as an example	17	47.22	5	62.50	10	52.63	2	25.00	6	42.86	8	57.14	5	45.45	12	48.00
Participant describes severe side effects as those that impact everyday life/ability to conduct activities of daily living	12	33.33	4	50.00	7	36.84	1	12.50	6	42.86	4	28.57	5	45.45	7	28.00
Participant describes coping with all side effects (because you have to or it's all that they've known)	4	11.11	0	0.00	1	5.26	3	37.50	4	28.57	0	0.00	1	9.09	3	12.00

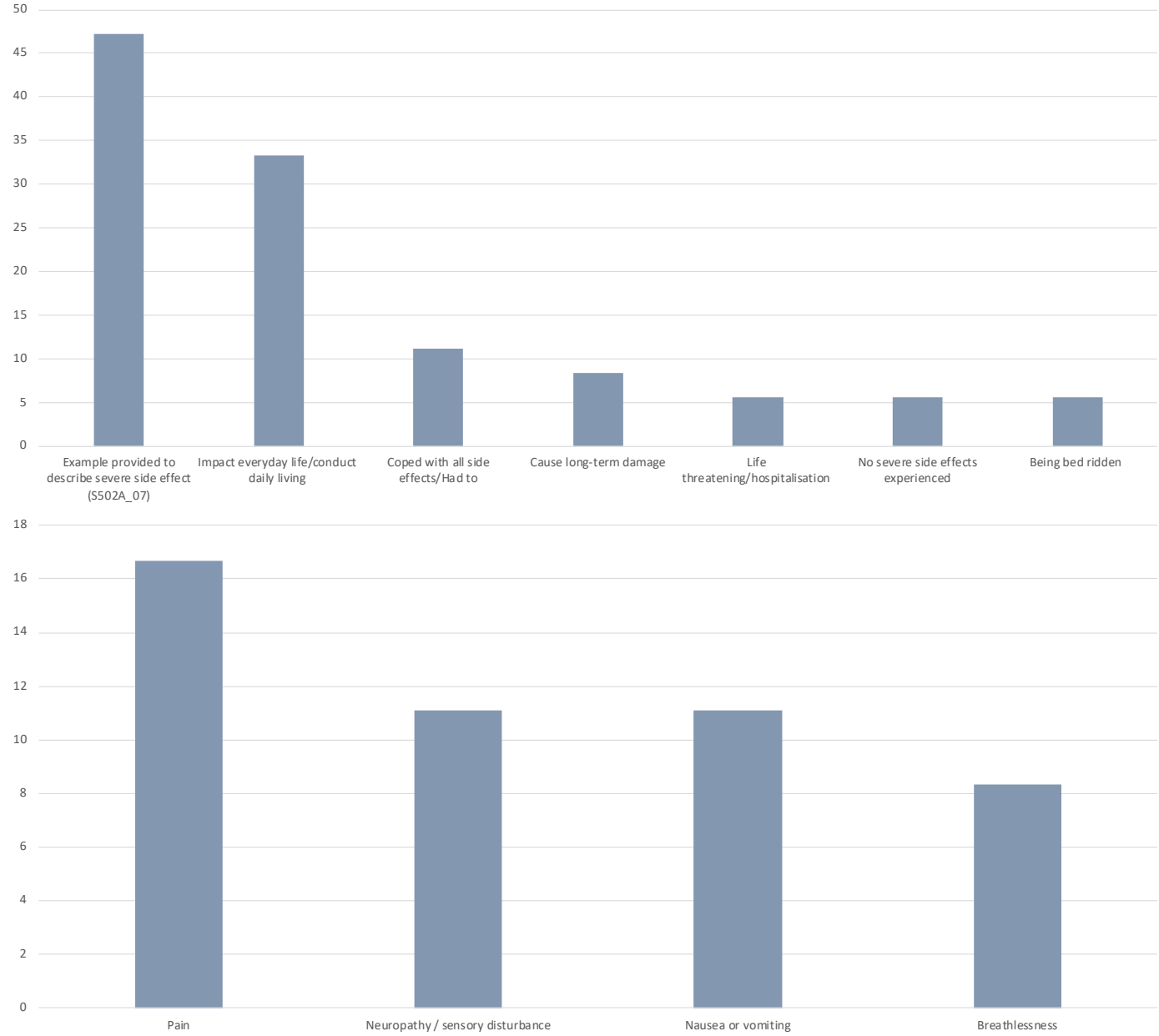

Figure 5.21: Description of severe side effects

Adherence to treatment

Participants were asked in the structured interview what influences their decision to continue with a treatment regime. The most common theme described was adhering as per the advice of their specialist or as long as its prescribed (n=16, 44.44%). Participants also reported not giving up on any treatment (n=6, 16.67%) and adhering to treatment for a specific amount of time (n=5, 13.89%).

In relation to subgroup variations, participants in the subgroups *ATTR-cardiac* (61.11%), *All cardiac* (56.00%), *Aged 75 or older* (75.00%) and *Trade or high school* (64.29%) described adhering to treatment as per the advice of their specialist or for as long as prescribed more frequently than the general population (44.44%), while those in the *Regional or remote* (22.22%) subgroup described this less frequently. No participants in the *Carer* (0.00%) subgroup described this at all.

Participants in the *AL amyloidosis* (30.00%), and *Trade or high school* (35.71%) subgroups described not giving up on any treatment more frequently than the general population (16.67%), while those in the *Carer* (0.00%), and *Aged 55 to 64* (0.00%) subgroups did not describe this at all.

There were no participants in the *Carer* subgroup that described adhering to treatment for a specific amount of time (0.00%), compared to the general population (13.89%).

Advice of specialist/as prescribed

Again, it's on the advice of a haematologist who said, 'It will take a little while. We need to see this for at least four weeks, six weeks, and then have a look and whatever it is.' 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

Has not given up on any treatment

I've never been in that situation, so I really don't know. I can't answer that one. I've never stopped a medication other than on doctor's advice. Up until 20 years ago, I'd probably never in my life had very much medication. Participant 002ALX

I don't give up. I keep going until I'm told and take it. Participant 005AL

I've only had experience with the Velcade and I stayed with the whole course, 16 weeks. Even after six weeks, the blood markers on the light chains indicated that it was working and so that made perfect sense to continue using it right to the end. I dare say that I would do exactly the same with a new treatment that we're going to start whenever the medication arrives. Participant 003AL

Specific amount of time

Two to four weeks is what I would do. If it's severe, then it's less than two weeks. If it's tolerable between mild and severe, I'll only give it two weeks and see how it takes, because some medications do take longer. Some medications would take more than a month until it will take effect. If I read the material that says what happened, and the case studies out there are patients or people who are taking their medications and their input into consideration of how long I should stay on it. Participant 002ATR

With the green tea, I only stuck with it for one week regardless of whether it would've been beneficial in my life. I guess that's the only one I can go on at present because, like the Difluzole when they put me on that, we did a blood test within the first month to make sure that it didn't have some side effects against my kidney or it was giving me some side effect that was more noticeable like more drowsiness or more dizziness or something like that. I guess you're probably going to rely a lot on the person who's prescribing it. That you have a bit of a follow-up. Let's say one month would be about the maximum you want to stay on it before you did some checks to see if it was affecting you. Participant 003ATR

It was a couple of months for both Velcade and Revlimid. I'm no physician nor a doctor or MD specialist, but if you've been taking it for 8 to 10 weeks and nothing's happening, you've got to say, 'That's long enough.' That was the benchmark that NAME CLINICIAN and I agreed with, 8 to 10 weeks.

You may say as a specialist you may know more quickly than that, but as a non-specialist then I wouldn't know, but I being-- if you aren't seeing any reaction after 10 weeks, you have to say, 'Something's not right.' Participant 004AL

Table 5.24: Adherence to treatment

Adherence to treatment	All participants		ATTR-cardiac		All cardiac		AL amyloidosis		Carer		Male		Female		Regional or remote		Metropolitan	
	n=36	%	n=18	%	n=25	%	n=10	%	n=8	%	n=22	%	n=14	%	n=9	%	n=27	%
Participant describes adhering to treatment as per the advice of their specialist/as long as prescribed	16	44.44	11	61.11	14	56.00	5	50.00	0	0.00	10	45.45	6	42.86	2	22.22	14	51.85
Participant describes not giving up on any treatment	6	16.67	3	16.67	5	20.00	3	30.00	0	0.00	5	22.73	1	7.14	2	22.22	4	14.81
Participant describes adhering to treatment for a specific amount of time: Total	5	13.89	4	22.22	5	20.00	1	10.00	0	0.00	3	13.64	2	14.29	1	11.11	4	14.81

Adherence to treatment	All participants		Aged 55 to 64		Aged 65 to 74		Aged 75 or older		Trade or high school		University		Mid to low SEIFA		Higher SEIFA	
	n=36	%	n=8	%	n=19	%	n=8	%	n=14	%	n=14	%	n=11	%	n=25	%
Participant describes adhering to treatment as per the advice of their specialist/as long as prescribed	16	44.44	3	37.50	7	36.84	6	75.00	9	64.29	7	50.00	5	45.45	11	44.00
Participant describes not giving up on any treatment	6	16.67	0	0.00	4	21.05	2	25.00	5	35.71	1	7.14	2	18.18	4	16.00
Participant describes adhering to treatment for a specific amount of time: Total	5	13.89	1	12.50	2	10.53	1	12.50	2	14.29	3	21.43	1	9.09	4	16.00

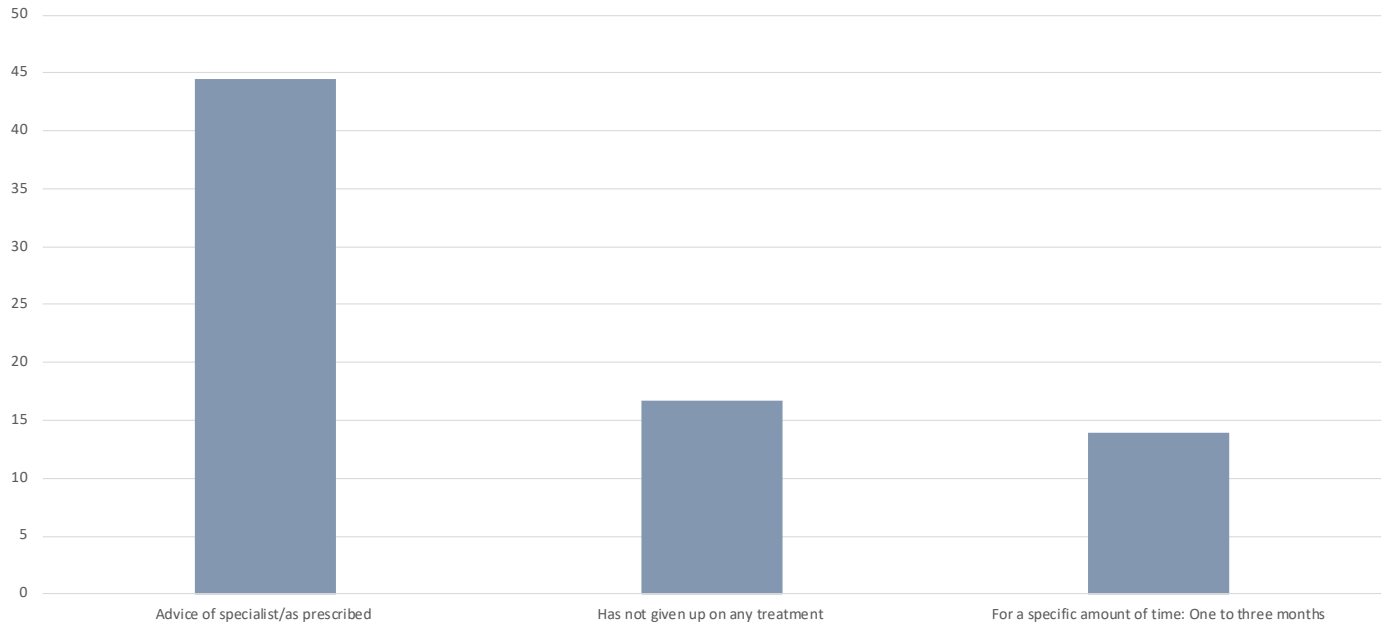

Figure 5.22: Adherence to treatment

What needs to change to feel like treatment is effective

Participants were asked to describe what needs to change to feel like treatment is effective. The most common response from 11 participants (30.56%) was needing to experience evidence of stable disease or no disease progression. This was followed by needing to experience an improvement in general wellbeing (n=9, 25.00%).

In relation to subgroup variations, participants in the *ATTR-cardiac* (16.67%), and *Mid to low SEIFA* (9.09%) subgroups described needing to experience evidence of stable disease or no disease progression less frequently than the general population (30.56%), while those in the subgroups *Regional or remote* (44.44%), and *AL amyloidosis* (60.00%) described this more frequently.

Participants in the subgroups *AL amyloidosis* (50.00%), *Aged 75 or older* (37.50%), and *Mid to low SEIFA* (36.36%) described needing to experience an improvement in general wellbeing more frequently than the general population (25.00%), while those in the subgroups *Aged 55 to 64* (12.50%), and *Carer* (12.50%) described this less frequently.

Evidence of stable/no disease progression

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. To me, so long as the anticipated improvement is achieved or close to, then I consider the treatment has been successful. Participant 002ALX

Well, I'll keep going with it because I think the-- If the echocardiogram and the blood test show that this condition stabilized. The other alternative I would consider is if they established a new drug which would not just keep it stabilized but remove the traces of amyloids that are on my heart. Participant 011ATR

Improvement in general wellbeing (quality of life)

Gauging that is how much can you walk? Walking up. We have three stories at home, so two sets of stairs. How much can I do outside? Things like that. It's general activity level and lethargy, so it's physical, the way you feel and just general well-being. That's how I gauge it. That's what I look for always. Can I keep doing or do at least what I'm doing or more? It does also affect moods. It affects mood. If you're feeling unwell all the time and you're a bit frustrated and things like that, yes, it affects the mood too. Participant 001AL

Certainly, the physical feeling of betterment, which would lead to an overall mental feeling of wellness whatever the treatment would be that I might be on. Participant 003ALX

Just being able to do a bit more. With the Pomalidomide, when I'm on it, I'm not as anxious to get out. You know what I mean? I go to the shops and when someone says, 'Do you want to go for a walk,' and I'll say no, but I just procrastinate a lot. I'll say, 'We'll go here, and we'll do that', but when it really comes to the crunch, I'm more likely to say, 'Unless it's important to both of us or our grandchildren or something--', I'll just say, 'Oh no. I'll just let it go.' Participant 005AL

Table 5.25: What needs to change to feel like treatment is effective

What needs to change to feel treatment is effective	All participants		ATTR-cardiac		All cardiac		AL amyloidosis		Carer		Male		Female		Regional or remote		Metropolitan	
	n=36	%	n=18	%	n=25	%	n=10	%	n=8	%	n=22	%	n=14	%	n=9	%	n=27	%
Participants reported needing to experience evidence of stable disease/no disease progression	11	30.56	3	16.67	6	24.00	6	60.00	2	25.00	7	31.82	4	28.57	4	44.44	7	25.93
Participants reported needing to experience an improvement in general wellbeing (quality of life)	9	25.00	3	16.67	6	24.00	5	50.00	1	12.50	6	27.27	3	21.43	2	22.22	7	25.93

What needs to change to feel treatment is effective	All participants		Aged 55 to 64		Aged 65 to 74		Aged 75 or older		Trade or high school		University		Mid to low SEIFA		Higher SEIFA	
	n=36	%	n=8	%	n=19	%	n=8	%	n=14	%	n=14	%	n=11	%	n=25	%
Participants reported needing to experience evidence of stable disease/no disease progression	11	30.56	2	25.00	6	31.58	3	37.50	4	28.57	5	35.71	1	9.09	10	40.00
Participants reported needing to experience an improvement in general wellbeing (quality of life)	9	25.00	1	12.50	5	26.32	3	37.50	4	28.57	4	28.57	4	36.36	5	20.00

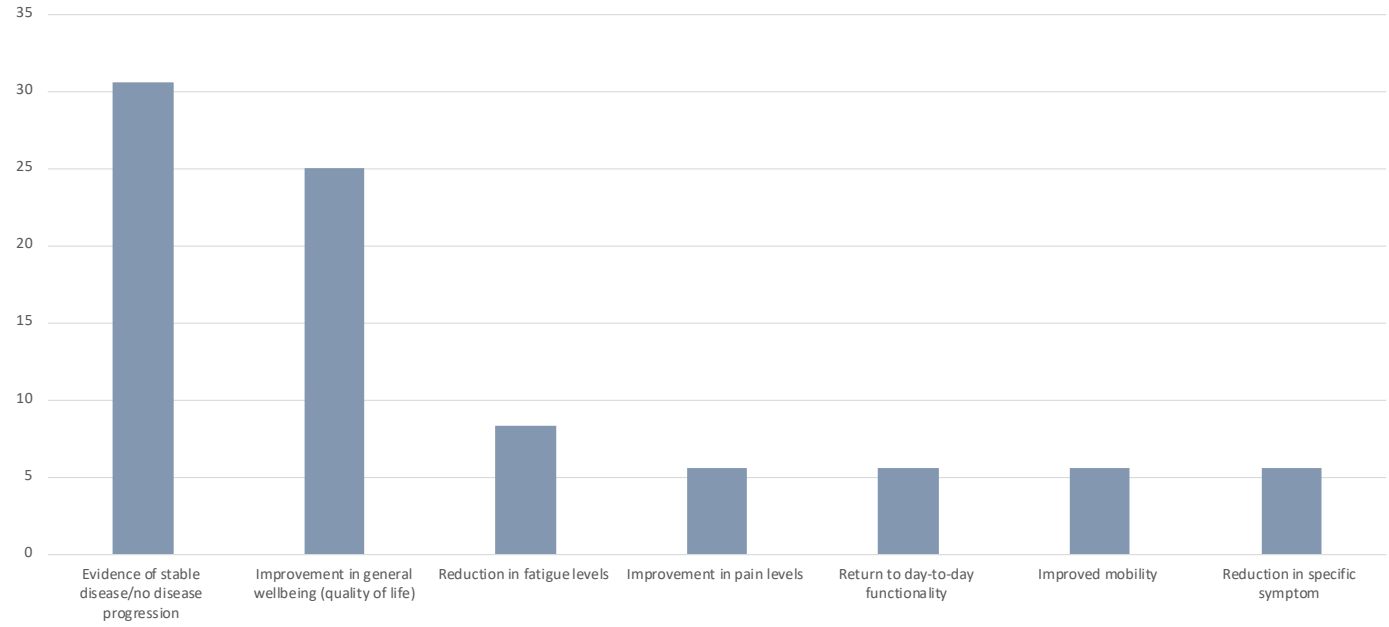

Figure 5.23: What needs to change to feel like treatment is effective

Information needed to be confident in a new treatment

Participants were asked to describe what information would be needed to be confident in a new treatment. The most common response, from 17 participants (47.22%), was needing the advice of their clinician followed by 14 participants (38.89%) needing to know about side effects to feel confident about trying a new treatment. There were 11 participants (30.56%) that reported needing scientific evidence, followed by those who described needing to conduct their own research (n=9, 25.00%); needing to know about efficacy (n=9, 25.00%) and needing to know the overall benefits (n=8, 22.22%).

In relation to subgroup variations, participants in the *Aged 55 to 64* (37.50%) subgroup described needing the advice of their clinician more frequently than the general population (47.22%), while those in *Mid to low SEIFA* (36.36%), *Regional or remote* (22.22%), and *Carer* (37.50%) subgroups described this less frequently.

Participants in the subgroups *University* (50.00%), *Aged 55 to 64* (62.50%) and *75 or older* (50.00%) described needing scientific evidence more frequently than the general population (30.56%), while those in the subgroups *Aged 65 to 74* (26.32%), *Trade or high school* (28.57%) and *Mid to low SEIFA* (27.27%) described this less frequently.

Participants in the *Carer* (12.50%) subgroup described needing to know about efficacy less frequently than the general population (25.00%), while those in the *Aged 75 and older* (9.09%) subgroup described this less frequently.

Participants in the *Carer* (37.50%), *Male* (36.36%), *Trade or high school* (50.00%), and *Mid to low SEIFA* (36.36%) subgroups needing to know about side effects to feel confident about trying a new treatment more frequently than the general population (38.89%), while those in the *University* (14.29%), *Female* (7.14%) subgroups described this less frequently. No participants described this in the *Aged 55 to 64* (0.00%) subgroup.

Participants in the *Regional or remote* (50.00%) subgroup described needing to conduct their own research more frequently than the general population (25.00%), while those in the *Trade or high school* (14.29%), and *Mid to low SEIFA* (9.90%) described this less often.

Participants in the subgroups *Aged 55 to 64* (37.50%), *Female* (35.71%) described needing to know the overall benefits more frequently than the general population (22.22%) while those in the *Regional or remote* (11.11%), *Mid to low SEIFA* (9.09%) subgroups described this less frequently. No participants *Aged 75 or older* (0.00%) described this at all.

Advice of clinician

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 would 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. If they said to me, 'PARTICIPANT, we're going to take you off the Pomalidomide and we're going to try this for you.' No hesitation. Participant 005AL

Side effects

Well, I'd certainly need to know the side effects. I'd certainly need to know because if it came up it was a drug that was going to be trialled and they were trialling it. They started off doing the trial and then one person died, so they took that straight off the thing, so they're the sort of things you need to know. You need to know the possible side effects, the possible number of people that maybe because this is all quite legal stuff anyway, there's only so many they can have in the trials, et cetera. Participant 003ATR

The second set of tests, as I mentioned before, would be the side effects. Some medications have some very benign side effects or very mild. If they were harrowing, then I could put up with them for some time but not forever. Participant 004AL

For instance, if a treatment came available that would dissolve the stuff that's sitting around my heart at the moment, the thing I'd want to know is what is the toxicity of the treatment. Is the treatment likely be do me more damage in another way? Is it going to kill my liver as well as my kidneys stuff like that. We need to get the side effects of the treatment or how they got to trying it out. Participant 004ATR

Scientific evidence e.g. clinical trial results

Personally, I would probably like to have a look at the science and look at some evidence from clinical trials that might have been done, whether they're phase one or phase two trials just to see what the potential benefits of the treatment might be. Me being me, I would probably want to understand a bit about the science. Participant 002AL

I would read a hell of a lot about it. I would sit in NAME CLINICIAN's office and pick his brains until we came to an accord. I would want to know the scientific detail of the treatment. I'd also in my condition want to know why I was being put back on treatment. Participant 002ALX

I guess I'd want to know what the results of the clinical trials were and then normal information that you get about drugs, like what are the chances that it could work for me, what possible side effects could occur, those sorts of things. Participant 011ATR

Own research

Personally, I would look it up. I'm very good with computers so I would seek out as much information as I could. I'd rely on my doctor because clinical trials often have a baseline about what other people have experienced. I would consider my options, and if there was real hope for a better outcome, then I'd most likely go ahead with a clinical trial. I'd most likely go ahead. Participant 006AL

I would do some research myself. I'd also need to know that it wasn't going to adversely impact your quality of life. Again, whether or not I would go back to it would depend on how much good versus bad it was going to do. I'd also have to know that I can afford it, which is an interesting one with these kind of things. Participant 006ATR

I'd have to do some research on it. Talk to different people about the- doctors and my family just to make sure it was something that we- that it's the right direction for us. Participant 009ATR

Efficacy

I think you'd have to be certain of if the treatment that you're on is actually doing what it's designed to do, lowering the light chain levels in your blood and the plasma levels, then you would have to choose a new treatment if it had the same effectiveness but with lesser side effects. Participant 002CA

The likelihood of efficacy. It has a high probability, it's not a low probability for a person with my particular series of issues. That would be the first thing. It's got to have a reasonable chance or that's better than 50:50 chance of being effective and preferably having a long-term effectiveness. Participant 004AL

I'd like to know that there has been something done, or something tested that said whatever that or in RACS or whatever that we've determined that yes it does dissolve the amyloid from around the heart. Participant 004ATR

Benefits of treatment

For me, what would be the advantages of changing the treatment. If wasn't going to be better than what I'm already on and I would discuss it with as I said with a professional that I am dealing with, I wouldn't make the decision. If they advise me that this is a better option than what I was on and explain to me that all and why then, yes, I'd give it a try. Participant 001ATR

I think you'd have to be certain of if the treatment that you're on is actually doing what it's designed to do, lowering the light chain levels in your blood and the plasma levels, then you would have to choose a new treatment if it had the same effectiveness but with lesser side effects. Participant 002CA

I think the benefits and then the side effects. If the side effects outweigh the benefits so that he's not got a good quality of life that would be quite high up there. I think, and where it is on the trial, if it's the first trial and we don't know then what damage is possible. Participant 005CA

Table 5.26: Information needed to be confident in a new treatment

Information needed to be confident in new treatment	All participants		ATTR-cardiac		All cardiac		AL amyloidosis		Carer		Male		Female		Regional or remote		Metropolitan	
	n=36	%	n=18	%	n=25	%	n=10	%	n=8	%	n=22	%	n=14	%	n=9	%	n=27	%
Participant describes needing the advice of their clinician	17	47.22	9	50.00	12	48.00	5	50.00	3	37.50	10	45.45	7	50.00	2	22.22	15	55.56
Participant describes needing to know about side effects to feel confident about trying a new treatment	14	38.89	7	38.89	9	36.00	4	40.00	2	25.00	9	40.91	5	35.71	4	44.44	10	37.04
Participant describes needing scientific evidence to feel confident about trying a new treatment	11	30.56	6	33.33	8	32.00	3	30.00	1	12.50	7	31.82	4	28.57	3	33.33	8	29.63
Participant describes needing to conduct their own research to feel confident about trying a new treatment	9	25.00	6	33.33	8	32.00	3	30.00	3	37.50	8	36.36	1	7.14	3	33.33	6	22.22
Participant describes needing to know about efficacy to feel confident about trying a new treatment	9	25.00	4	22.22	6	24.00	2	20.00	2	25.00	6	27.27	3	21.43	4	44.44	5	18.52
Participant describes needing to know the overall benefits to feel confident about trying a new treatment (e.g. versus their current treatment)	8	22.22	3	16.67	5	20.00	3	30.00	2	25.00	3	13.64	5	35.71	1	11.11	7	25.93

Information needed to be confident in new treatment	All participants		Aged 55 to 64		Aged 65 to 74		Aged 75 or older		Trade or high school		University		Mid to low SEIFA		Higher SEIFA	
	n=36	%	n=8	%	n=19	%	n=8	%	n=14	%	n=14	%	n=11	%	n=25	%
Participant describes needing the advice of their clinician	17	47.22	3	37.50	9	47.37	4	50.00	7	50.00	7	50.00	4	36.36	13	52.00
Participant describes needing to know about side effects to feel confident about trying a new treatment	14	38.89	5	62.50	5	26.32	4	50.00	4	28.57	7	50.00	3	27.27	11	44.00
Participant describes needing scientific evidence to feel confident about trying a new treatment	11	30.56	3	37.50	4	21.05	4	50.00	4	28.57	5	35.71	3	27.27	8	32.00
Participant describes needing to conduct their own research to feel confident about trying a new treatment	9	25.00	0	0.00	6	31.58	2	25.00	7	50.00	2	14.29	4	36.36	5	20.00
Participant describes needing to know about efficacy to feel confident about trying a new treatment	9	25.00	2	25.00	5	26.32	2	25.00	2	14.29	4	28.57	1	9.09	8	32.00
Participant describes needing to know the overall benefits to feel confident about trying a new treatment (e.g. versus their current treatment)	8	22.22	3	37.50	5	26.32	0	0.00	2	14.29	4	28.57	1	9.09	7	28.00

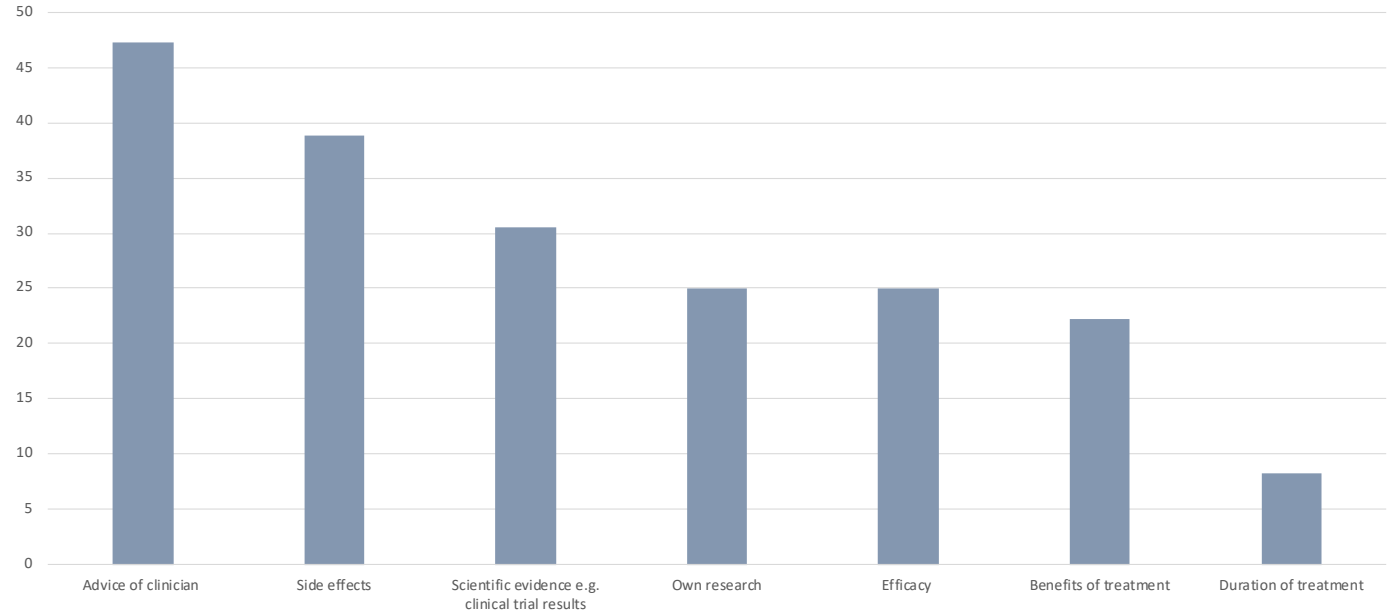

Figure 5.24: Information needed to be confident in a new treatment

Support needed for treatment at home

Participants were asked to describe what support they would need if they were having treatment at home. The two most common responses were participants not needing support (n=8, 22.22%) and needing support from their friends or family (n=8, 22.22%). There were seven participants that reported needing regular check-ups with a GP or nurse (19.44%). This was followed by needing someone to call if they have a question or issue (n=4, 11.11%). Four participants (11.11%) described needing training and education on how to administer treatment.

In relation to subgroup variations, no participants in the *Aged 75 or older* subgroup described not needing support (0.00%), compared to the general population (22.22%).

Participants in the *Mid to low SEIFA* (9.09%) subgroup described needing support from their friends or family less frequently than the general population (22.22%), while those in the subgroups *Trade or high school* (35.71%), *Aged 75 or older* (37.50%), and *AL amyloidosis* (40.00%) described this more frequently. Participants in the *Carer* (0.00%), and *Aged 55 to 64* (0.00%) did not describe this at all.

Participants in the *Mid to low SEIFA* subgroup described needing regular check-ups with a GP or nurse more frequently (36.36%) than the general population (19.44%).

Participants in *Female* subgroup described needing to have someone to call if they have a question or issue more frequently (21.43%) than the general population (11.11%) while no one in the *Regional or remote* subgroup described this (0.00%).

No participants in the *AL amyloidosis* subgroup (0.00%) described needing training and education whereas those in the subgroups *Aged 75 or older* (25.00%) and *Regional or remote* (22.22%) described this more frequently than the general population (11.11%).

Not needing support

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. I've never been told that I couldn't drive when I went on that treatment and I did ask, and I said, 'No, it's not a problem,' although when I was having it in the first lot my wife used to drop me off at the hospital and sometimes stay there, and then she'd drive me home. If this is at home, 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

Support from family/friends

I don't think I'd need a lot of support. I've got my husband here in terms of someone else in the house, so I'm not alone. My doctors always said, if I have any worries, I can just contact him, call him, email him. I think with that support, and there's obviously support through the NAME CLINIC as well, of course, there is a little support group. I wouldn't feel uncomfortable having a treatment at home that involved a pill or a tablet kind of thing. I don't think I would need much additional support at all. Participant 002AL

I've got the all the support here, I think, I could need with my wife and family here. They're very good with me. Participant 009ATR

I would probably- one of my daughters. I've got three daughters. One of them would be here at the time when I need them, I'm sure. That's about all I need, I think, just for the reassurance, but as I said, I'm not a panicky person. Participant 010ATR

Checked regularly by GP/Nurse

Regular contact by the prescribing doctor or nurse. Participant 001ALX

I went to a new heart guy and he gave me access to a heart nurse. I could ring her anytime if I was worried about my blood pressure or something like that to say just, 'What do you think I should do,' because a couple of times I did need to go to hospital and things. That was terrific because you can't get onto the doctors usually, but you had access to her. She would come to the home and she did some blood tests here and she'd give me different things here. That was great and maybe it was just getting confident with them coming in that they can do the same job. Whereas with the chemo, I didn't feel comfortable with that, so I never had that at home. I always went to the hospital. Participant 012ATR

Yes, and somebody to check-in regularly and that sort of thing, which we have now in amyloidosis. Participant 017ATR

Someone to call (out of hours, 24/7 support)

Someone I can ring up the phone and say, 'NAME, I love NAME HUSBAND but I'm ready to knock his block off.' She'll say, 'Well, what's going on?' and we'd sort it all out. That sort of support is absolutely critical. Participant 003CA

I don't think I'd need a lot of support. I've got my husband here in terms of someone else in the house, so I'm not alone. My doctors always said, if I have any worries, I can just contact him, call him, email him. I think with that support, and there's obviously support through the NAME CLINIC as well, of course, there is a little support group. I wouldn't feel uncomfortable having a treatment at home that involved a pill or a tablet kind of thing. I don't think I would need much additional support at all. Participant 002AL

Well, if you just got to take, I'd want to have a 24-hour contact with somebody, if things weren't going well. Participant 017ATR

Training and education on how to administer treatment

Well, only the fact that you've got to be careful on how many you take I suppose. Some of the treatments I had to deal with at home after like with injecting stuff, you can either get a nurse to come and help you do that, or you'd have to just learn to do it yourself. Participant 003ATR

Well, it's the background from the doctor which I'm going to take with me at home will be there as an information to assist me in that respect, and secondly, and it is for certain people around the home the children, my wife, a friend, people just like that. I need to have their support. Even if is only one person, one person is enough. Participant 005ATR

The only thing I would need is some reference material that I could read up. I would like some data that I could read. Participant 007ATR

Table 5.27: Support needed for treatment at home

Treatment preference – support needed	All participants		ATTR-cardiac		All cardiac		AL amyloidosis		Carer		Male		Female		Regional or remote		Metropolitan	
	n=36	%	n=18	%	n=25	%	n=10	%	n=8	%	n=22	%	n=14	%	n=9	%	n=27	%
Participant describes not needing support	8	22.22	4	22.22	7	28.00	3	30.00	1	12.50	6	27.27	2	14.29	2	22.22	6	22.22
Participant describes needing support from their friends or family	8	22.22	4	22.22	6	24.00	4	40.00	0	0.00	5	22.73	3	21.43	2	22.22	6	22.22
Participant describes needing regular check ups with a GP or nurse to feel comfortable: Total (e.g. Various locations)	7	19.44	3	16.67	4	16.00	2	20.00	2	25.00	4	18.18	3	21.43	2	22.22	5	18.52
Participant describes that they would need to have someone to call if they have a question or issue (out of hours, 24/7 support)	4	11.11	2	11.11	3	12.00	1	10.00	1	12.50	1	4.55	3	21.43	0	0.00	4	14.81
Participant describes needing training and education on how to administer treatment	4	11.11	3	16.67	3	12.00	0	0.00	1	12.50	3	13.64	1	7.14	2	22.22	2	7.41

Treatment preference – support needed	All participants		Aged 55 to 64		Aged 65 to 74		Aged 75 or older		Trade or high school		University		Mid to low SEIFA		Higher SEIFA	
	n=36	%	n=8	%	n=19	%	n=8	%	n=14	%	n=14	%	n=11	%	n=25	%
Participant describes not needing support	8	22.22	2	25.00	6	31.58	0	0.00	4	28.57	3	21.43	2	18.18	6	24.00
Participant describes needing support from their friends or family	8	22.22	0	0.00	5	26.32	3	37.50	5	35.71	3	21.43	1	9.09	7	28.00
Participant describes needing regular check ups with a GP or nurse to feel comfortable: Total (e.g. Various locations)	7	19.44	2	25.00	4	21.05	1	12.50	2	14.29	3	21.43	4	36.36	3	12.00
Participant describes that they would need to have someone to call if they have a question or issue (out of hours, 24/7 support)	4	11.11	1	12.50	2	10.53	1	12.50	2	14.29	1	7.14	2	18.18	2	8.00
Participant describes needing training and education on how to administer treatment	4	11.11	1	12.50	1	5.26	2	25.00	1	7.14	2	14.29	2	18.18	2	8.00

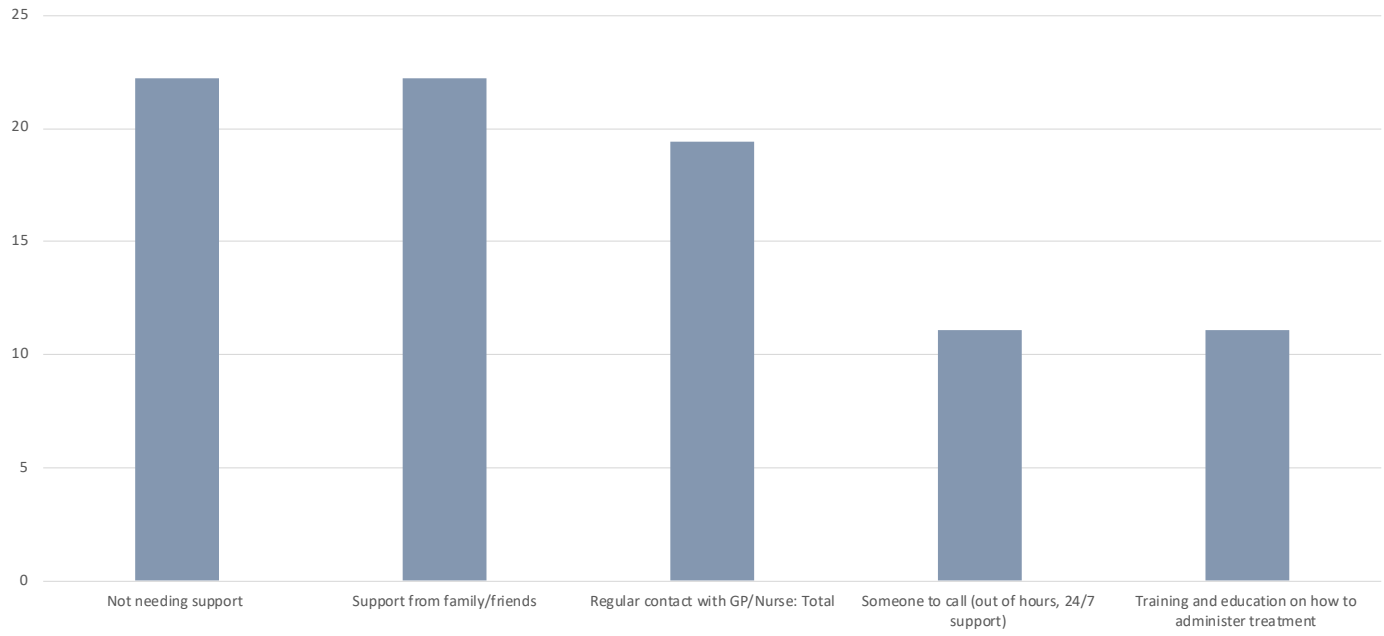

Figure 5.25: Support needed for treatment at home