Section 5

Treatment

Section 5: Experience of treatment

Respect shown

Participants were asked to think about how respectfully they were treated throughout their experience, this question was asked in the online questionnaire. Just under half of the participants indicated that they had been treated with respect throughout their experience (n=133, 41.43%), and 134 participants (41.74%) were treated with respect with the exception of one or two occasions. There were 54 participants (16.82%) felt they had not been treated respectfully.

Health care 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 health insurance (n=201, 64.63%). The majority of participants were not asked if they wanted to be treated as a public or private patient (n=157, 60.15%), however, they were asked if they had private health insurance (n=153, 58.62%). Throughout their treatment, there were 71 participants (23.05%) that were treated as a private patient, 156 participants (50.65%) were mostly treated as a public patient, and there were 68 participants (22.08%) that were equally treated as a private and public patient. Throughout their treatment, there were 42 participants (11.73%) that were treated mostly in the private hospital system, 228 participants (63.69%) were mostly treated in the public system, and there were 88 participants (24.58%) that were equally treated in the private and public systems.

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. Almost all the participants never or rarely had to delay or cancel appointments due to affordability (n = 259, 71.75%).

The next question was about the ability to fill prescriptions. Almost all of the participants never or rarely were unable to fill prescriptions (n=66, 18.28%).

The third question was about the affordability of basic essentials such as such as food, housing and power. There were 36 participants (9.97%) that never or rarely had trouble paying for essentials, and 13 participants (3.60%) that sometimes found it difficult, and 48 participants (13.30%) often or very often found it difficult to pay for basic essentials.

The final question was about paying for additional carers for themselves or for their family, there were 74 participants (23.79%) that paid for additional carers due to their condition.

Cost of condition

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

The most common amount was between $1001 or more (n=32, 8.74%), followed by between $101 to $250 (n=61, 16.67%). There were 41 participants (11.20%), that spent $501 to $1000 a month.

Burden of cost

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

The amount spent was an extremely significant or moderately significant burden for 102 participants (33.44%), somewhat significant for 77 participants (25.25%), and slightly or not at all significant for 126 participants (41.31%).

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.

Work status for 63 participants (23.95%) had not changed since diagnosis, and 33 participants (12.55%) were retired or did not have a job. There were 79 participants (30.04%) had to quit their job, 78 participants (29.66%) reduced the number of hours they worked, and 28 participants (10.65%) that accessed their superannuation early. There were 49 participants (18.63%) that took leave from work without pay, and 48 participants (18.25%) that took leave from work with pay.

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

There were 71 participants (24.40%), without a main partner or carer. Most commonly, participants had partners or carers that did not change their work status due to their condition (n=100, 34.36%). There were 43 participants (14.78%) whose partners reduced the numbers of hours they worked, and 19 partners, (6.53%) that quit their job. The partners of 26 participants (8.93%) took leave without pay, and there were 34 partners (11.68%) that took leave with pay.

Reduced income due to condition

More than half of the participants (n=217, 57.05%) indicated in the online questionnaire that they had a reduced family income due to their condition.

Estimated reduction monthly income

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

Most commonly, participants were not sure about the amount their monthly income was reduced by $2501 to 5000 (n=32, 10.74%), or reduced by between $1501 to 2500 per month (n=38, 12.75%).

Burden of reduced income

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

For 22 of these participants (16.30%), the burden of this reduced income was extremely or moderately significant, for 28 participants (20.74%) the burden was somewhat significant, and for 85 participants (62.96%) the burden was slightly or not all significant.

Lifestyle changes

Participants were asked about any lifestyle changes they had made since diagnosis, the quality of life from these changes, and how effective they found them.

Most participants used at made at least one lifestyle change (n=204, 67.77%), and on average made 1 changes (median=1.00, IQR=1.00).

The most common lifestyle change used was diet changes (n=150, 51.02%), followed by exercise (n=146, 59.84%), and reduce alcohol (n=56, 22.95%)

Complementary therapies

Participants were asked about any complementary therapies they used to manage their condition, the quality of life from these changes, and how effective they found them.

Most participants used at made at least one complementary therapy (n=216, 68.35%), and on average used 1 therapies (median=1.00, IQR=2.00).

The most common complementary therapy used was supplements (n=136, 46.10%), followed by mindfulness or relaxation (n=121, 45.83%), and massage therapy (n=80, 30.30%).

Clinical trials

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 111 participants (35.81%) that had discussions about clinical trials, 32 participants (10.32%) had brought up the topic with their doctor, and the doctor of 79 participants (25.48%) brought up the topic. The majority of participants had not spoken to anyone about clinical trials (n=199, 64.19%).

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 were 37 participants (11.86%) that had taken part in a clinical trial, 155 participants (49.68%) that would like to take part in a clinical trial if there was a suitable one, and 120 participants, that have not participated in a clinical trial and do not want to (38.46%).

Description of mild side effects

In the structured interview, participants were asked how they would describe the term ‘mild side effects’. The most common descriptions of mild side effects were described using a specific example (53.69%), and those that do not interfere with life (33.24%). Other themes included those that are resolved in a short amount of time (9.66%) and those that can be managed with self-medication or self-management (3.98 %).

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 were described using a specific example (47.73%), and those that impact everyday life or ability to conduct activities of daily living (28.13%). Other themes included those that are life threatening or result in hospitalisation (8.52%), those that cause long-term damage to their body (7.67%).

When a specific side effect was described, the most common examples were aches and pain (17.33%), emotional and mental impact (7.39%), and nausea with vomiting (6.53%). Other themes included fatigues (5.11%), gastrointestinal distress (4.83%), impact on sleep (4.26%), vision problems (3.98%), and impact on sleep (4.55%).

Adherence to treatment

Participants were asked in the structured interview what influences their decision to continue with a treatment regime. The most common responses were adhering to treatment for a specific amount of time (38.35%), adhering to treatment according to the advice of their specialist or as long as prescribed (36.08%), and adhering to treatment as long as side effects are tolerable (24.43 %). Other themes included never giving up on any treatment (11.36%), adhering to treatment as long as treatment is working (7.10%).

What needs to change to feel like treatment is working

Participants were asked to describe what needs to change to feel like treatment is effective. The most common responses were needing to see a specific symptom reduction (26.70%), needing to see needing to see physical signs and symptoms disappear or reduce side effects (25.85%), a needing to see improvements in general wellbeing (quality of life) (14.49%), needing to see evidence of stable disease (14.20%), needing to see a return to day-to-day functionality (14.20%), and needing to see improvement in pain levels (12.50%).

What it would mean if treatment worked

As a follow up question, participants were asked what it would mean to them if the treatment worked in the way they described. The most common responses were that it would allow them to do everyday activities/return to normal life (29.44%) and allow them to engage more with social activities and family life (11.67%). Other themes included allow them to return to work (9.44%), allow them to do more exercise (11.28%), will have a positive impact on their mental health (7.89%), allow the

Respect shown

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

Just under half of the participants indicated that they had been treated with respect throughout their experience (n=133, 41.43%), and 134 participants (41.74%) were treated with respect with the exception of one or two occasions. There were 54 participants (16.82%) felt they had not been treated respectfully.

Table 5.1: Respect shown

Respect shown	Number (n=321)	Percent
Respect shown	133	41.43
Respect shown, with the exception of one or two occasions	134	41.74
Respect not shown	54	16.82

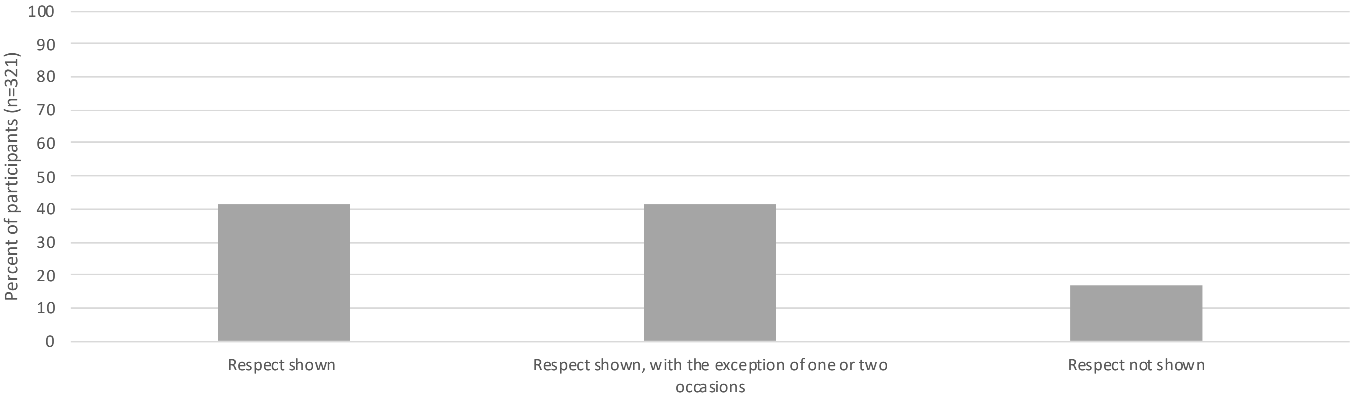

Figure 5.1: Respect shown

Health care 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 health insurance (n=201, 64.63%). The majority of participants were not asked if they wanted to be treated as a public or private patient (n=157, 60.15%), however, they were asked if they had private health insurance (n=153, 58.62%).

Throughout their treatment, there were 71 participants (23.05%) that were treated as a private patient, 156 participants (50.65%) were mostly treated as a public patient, and there were 68 participants (22.08%) that were equally treated as a private and public patient.

Throughout their treatment, there were 42 participants (11.73%) that were treated mostly in the private hospital system, 228 participants (63.69%) were mostly treated in the public system, and there were 88 participants (24.58%) that were equally treated in the private and public systems.

Table 5.2: Health care system

Health care services	Response	Number	Percent
Private health insurance	No	110	35.37
	Yes	201	64.63
Asked whether you want to be treated as a public or private patient	No	157	60.15
	Yes	104	39.85
Asked whether you had private health insurance	No	108	41.38
	Yes	153	58.62
Throughout your treatment in hospital, have you most been treated as a public or a private patient	Equally as a public and private patient	68	22.08
	Private patient	71	23.05
	Public patient	156	50.65
	Not sure	13	4.22
Which hospital system have you primarily been treated in	Both public and private	88	24.58
	Private	42	11.73
	Public	228	63.69
	Not sure	0	0.00

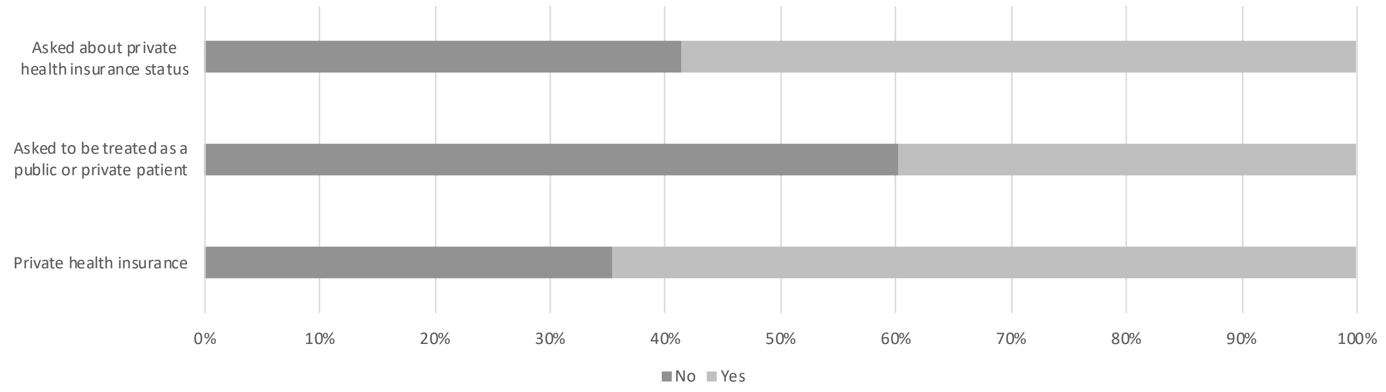

Figure 5.2: Health insurance

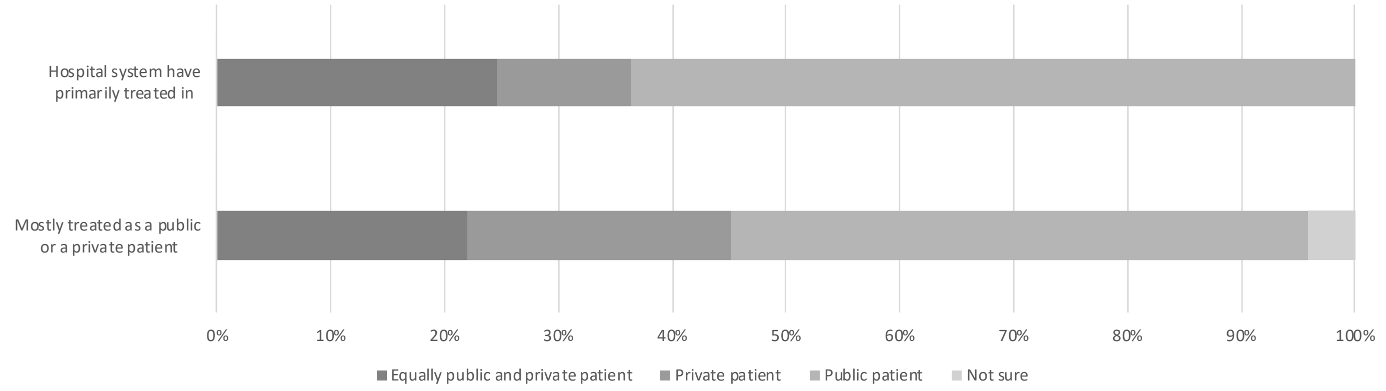

Figure 5.3: Hospital system

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. Almost all the participants never or rarely had to delay or cancel appointments due to affordability (n = 259, 71.75%).

The next question was about the ability to fill prescriptions. Almost all of the participants never or rarely were unable to fill prescriptions (n=66, 18.28%).

The third question was about the affordability of basic essentials such as such as food, housing and power. There were 36 participants (9.97%) that never or rarely had trouble paying for essentials, and 13 participants (3.60%) that sometimes found it difficult, and 48 participants (13.30%) often or very often found it difficult to pay for basic essentials.

The final question was about paying for additional carers for themselves or for their family, there were 74 participants (23.79%) that paid for additional carers due to their condition.

Table 5.3: Affordability of healthcare

Affordability of healthcare	Response	Number	Percent
Delay or cancel healthcare appointments due to affordability	Never	215	59.56
	Rarely	44	12.19
	Sometimes	66	18.28
	Often	25	6.93
	Very often	11	3.05
Did not fill prescriptions due to cost	Never	260	72.02
	Rarely	45	12.47
	Sometimes	43	11.91
	Often	10	2.77
	Very often	3	0.83
Difficult to pay for basic essentials	Never	172	47.65
	Rarely	52	14.40
	Sometimes	89	24.65
	Often	31	8.59
	Very often	17	4.71
Pay for additional carers for self or family	Yes	74	23.79
	No	237	76.21

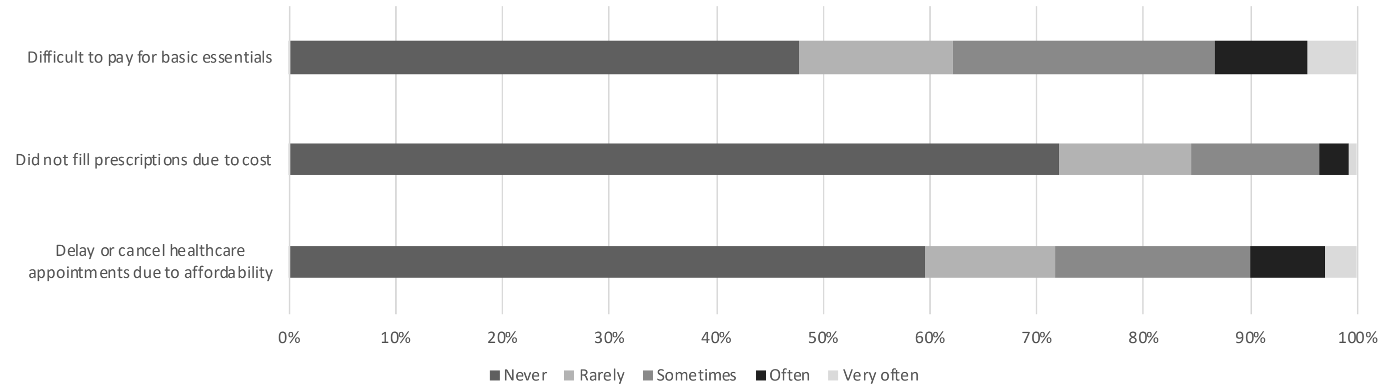

Figure 5.4: Affordability of healthcare

Cost of condition

In the online questionnaire, participants estimated the amount they spend per month due to their condition, including doctors' fees, transport, carers, health insurance gaps and complementary therapies. Where the response was given in a dollar amount, it is listed below.

The most common amount was between $1001 or more (n=32, 8.74%), followed by between $101 to $250 (n=61, 16.67%). There were 41 participants (11.20%), that spent $501 to $1000 a month.

Burden of cost

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

The amount spent was an extremely significant or moderately significant burden for 102 participants (33.44%), somewhat significant for 77 participants (25.25%), and slightly or not at all significant for 126 participants (41.31%).

Table 5.4: Estimated monthly out of pocket expenses due to condition

Estimated monthly out of pocket expenses	Number (n=366)	Percent
$0	19	5.19
$100 or less	72	19.67
$1001 or more	32	8.74
$101 to $250	61	16.67
$251 to $500	79	21.58
$501 to $1000	41	11.20
Not sure of amount	62	16.94

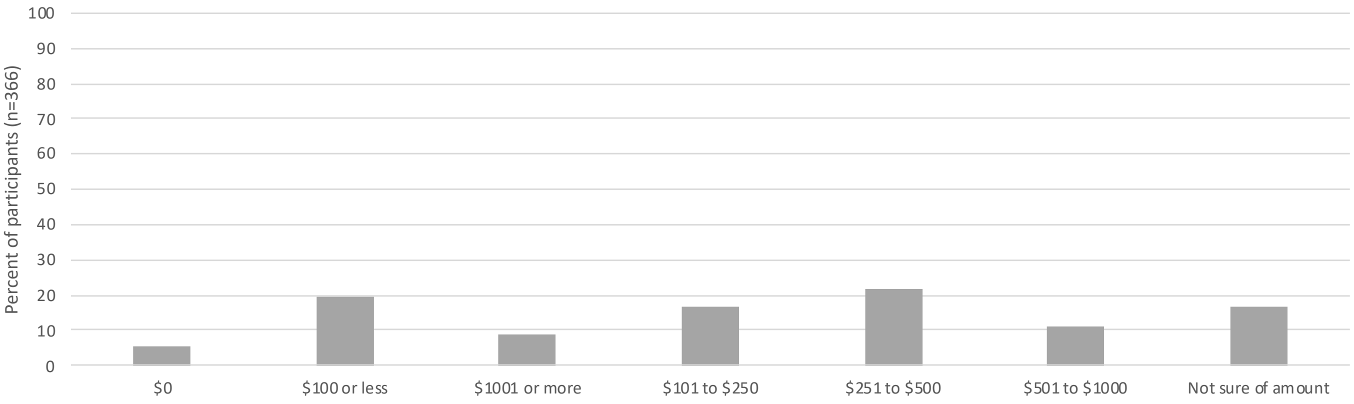

Figure 5.5: Estimated monthly out of pocket expenses due to condition

Table 5.5: Burden of out-of-pocket expenses due to condition

Burden of out of pocket expenses	Number (n=305)	Percent
Extremely significant	64	20.98
Moderately significant	38	12.46
Somewhat significant	77	25.25
Slightly significant	67	21.97
Not at all significant	59	19.34

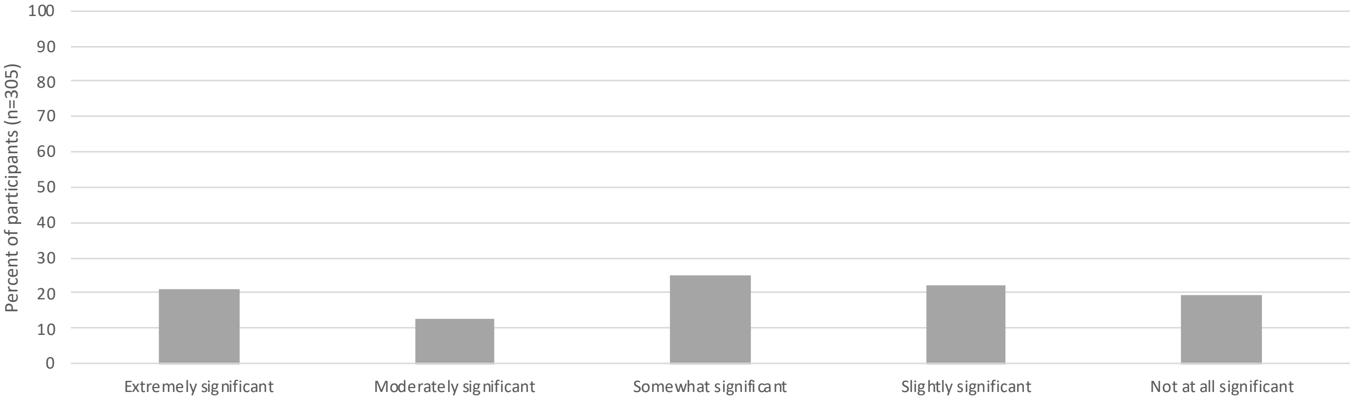

Figure 5.6: Burden of out-of-pocket expenses due to condition

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.

Work status for 63 participants (23.95%) had not changed since diagnosis, and 33 participants (12.55%) were retired or did not have a job. There were 79 participants (30.04%) had to quit their job, 78 participants (29.66%) reduced the number of hours they worked, and 28 participants (10.65%) that accessed their superannuation early. There were 49 participants (18.63%) that took leave from work without pay, and 48 participants (18.25%) that took leave from work with pay.

Changes to carer/partner employment status

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

There were 71 participants (24.40%), without a main partner or carer. Most commonly, participants had partners or carers that did not change their work status due to their condition (n=100, 34.36%). There were 43 participants (14.78%) whose partners reduced the numbers of hours they worked, and 19 partners, (6.53%) that quit their job. The partners of 26 participants (8.93%) took leave without pay, and there were 34 partners (11.68%) that took leave with pay.

Table 5.6: Changes to employment status

Changes in work status due to condition	Number (n=263)	Percent
Work status has not changed	63	23.95
Retired or did not have a job	33	12.55
Had to quit job	79	30.04
Reduced number of hours worked	78	29.66
Leave from work without pay	49	18.63
Leave from work with pay	48	18.25
Accessed Superannuation early due to condition	28	10.65

Figure 5.7: Changes to employment status

Table 5.7: Changes to care/partner employment status

Changes in partner or main carer work status due to condition	Number (n=291)	Percent
Does not have a partner/main carer	71	24.40
Work status has not changed	100	34.36
Retired or did not have a job	32	11.00
Had to quit job	19	6.53
Reduced number of hours worked	43	14.78
Leave from work without pay	26	8.93
Leave from work with pay	34	11.68

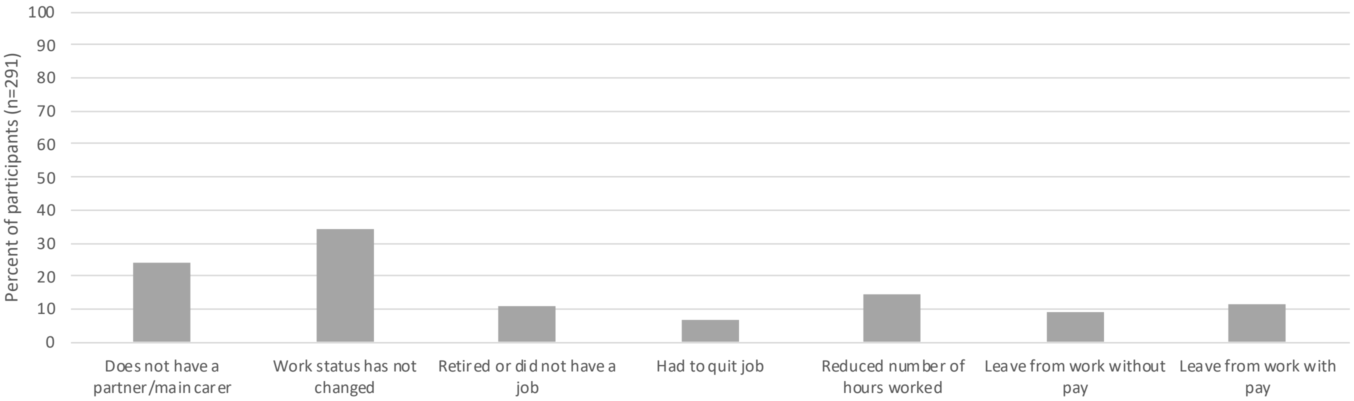

Figure 5.8: Changes to care/partner employment status

Reduced income due to condition

More than half of the participants (n=217, 57.05%) indicated in the online questionnaire that they had a reduced family income due to their condition.

Estimated reduction monthly income

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

Most commonly, participants were not sure about the amount their monthly income was reduced by $2501 to 5000 (n=32, 10.74%), or reduced by between $1501 to 2500 per month (n=38, 12.75%).

Burden of reduced income

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

For 22 of these participants (16.30%), the burden of this reduced income was extremely or moderately significant, for 28 participants (20.74%) the burden was somewhat significant, and for 85 participants (62.96%) the burden was slightly or not all significant.

Table 5.8: Estimated monthly loss of income

Estimated monthly loss of income	Number (n=298)	Percent
$0	128	42.95
$1501 to 2500	38	12.75
$2501 to 5000	32	10.74
$500 to 1500	29	9.73
More than $5000	15	5.03
Not sure/not specified	56	18.79

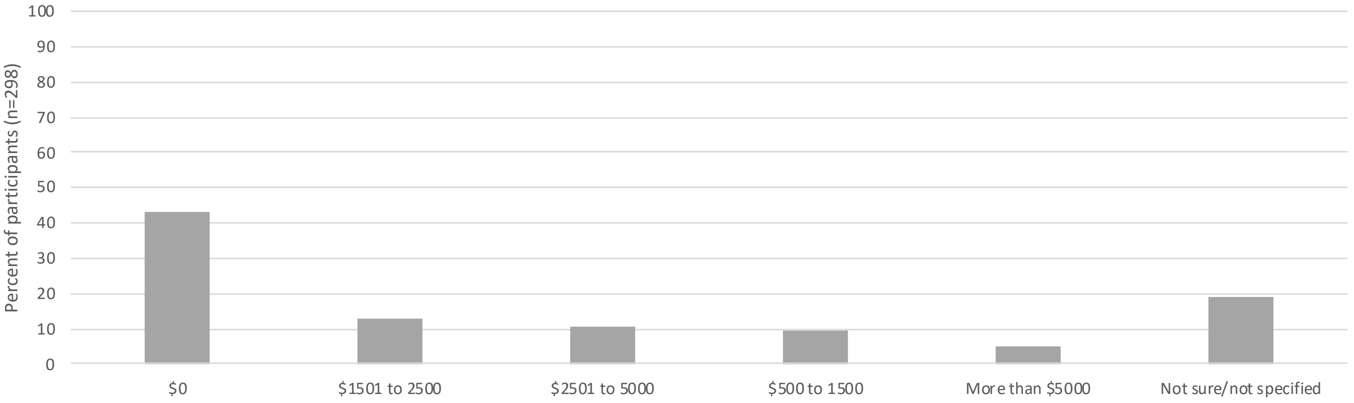

Figure 5.9: Estimated monthly loss of income

Table 5.9: Burden of reduced income

Burden of reduced income	Number (n=135)	Percent
Extremely significant	52	38.52
Moderately significant	33	24.44
Somewhat significant	28	20.74
Slightly significant	20	14.81
Not at all significant	2	1.48

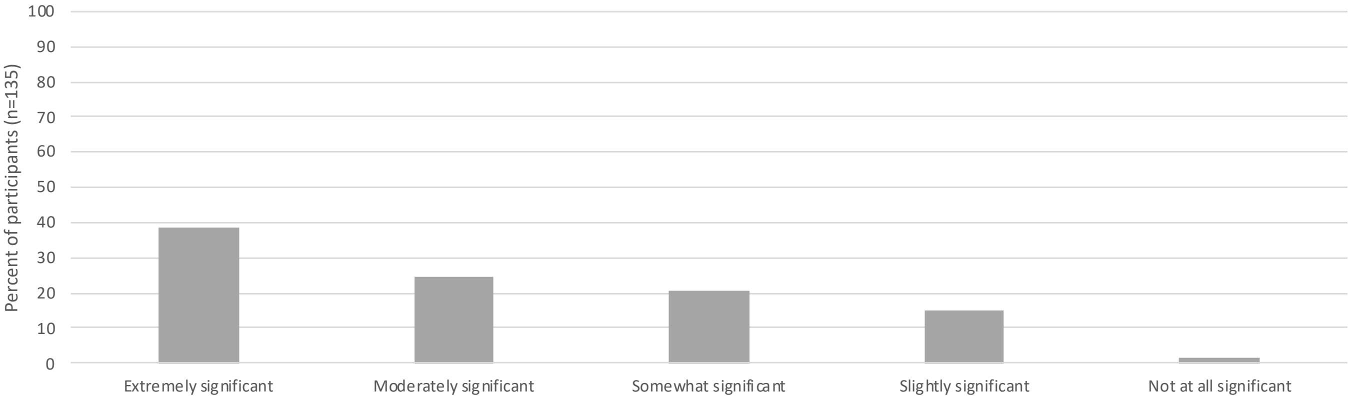

Figure 5.10: Burden of reduced income

Table 5.10: Allied health

Allied health	Number	Percent		Median quality of life	IQR	Median effectiveness	IQR
Physiotherapy (n=286)	135	135	47.20	4.00	4.00	2.00	1.50
Psychology (n=236)	92	92	38.98	2.50	3.00	2.00	2.00
Occupational therapy (n=236)	82	82	34.75	4.00	3.00	3.00	1.00
Dietary (n=217)	72	72	33.18	3.00	2.00	2.00	2.25
Speech therapy (n=286)	70	70	24.48	4.00	4.00	2.00	2.00
Podiatry (n=216)	67	67	31.02	4.00	4.00	1.50	1.50
Social work (n=236)	22	22	9.32	1.00	2.50	3.00	2.00

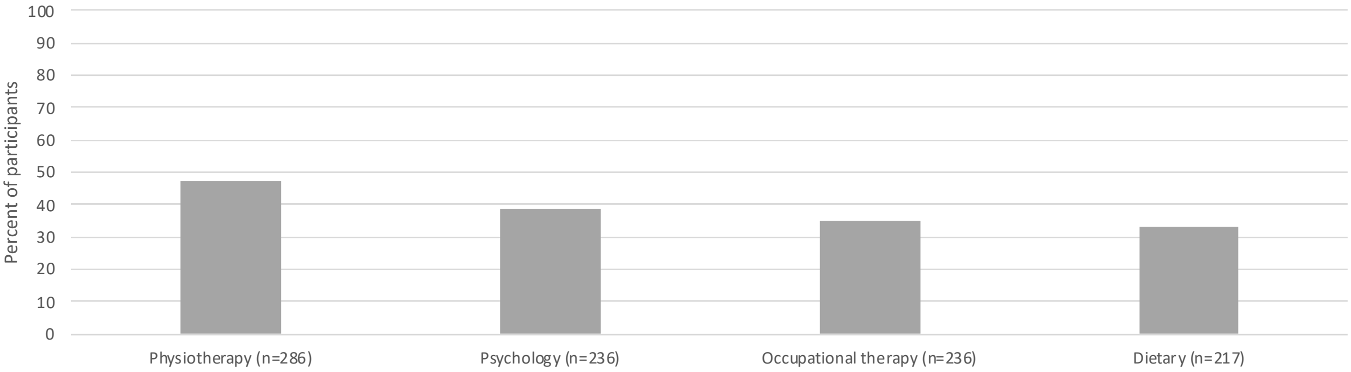

Figure 5.11: Allied health

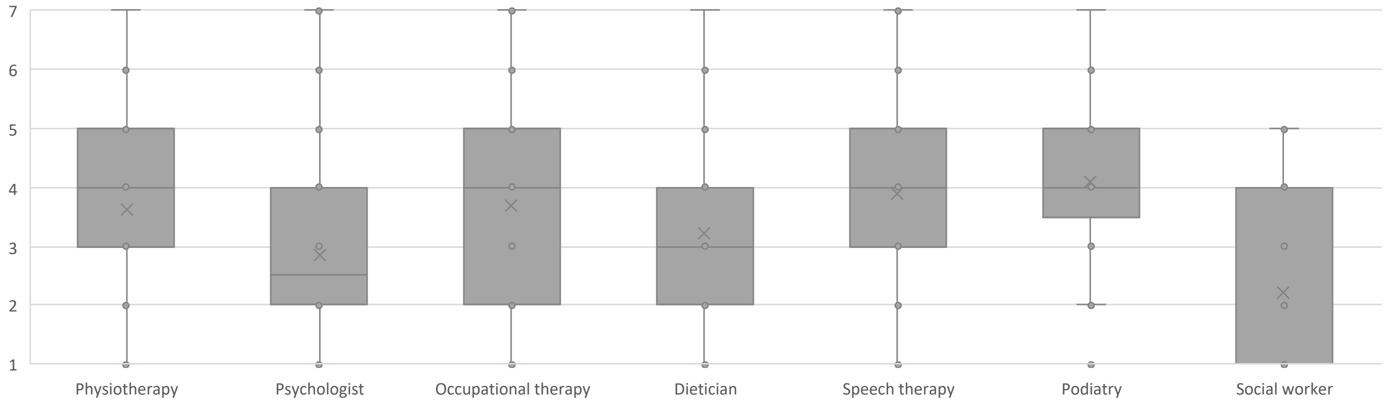

Figure 5.12: Quality of life from allied health

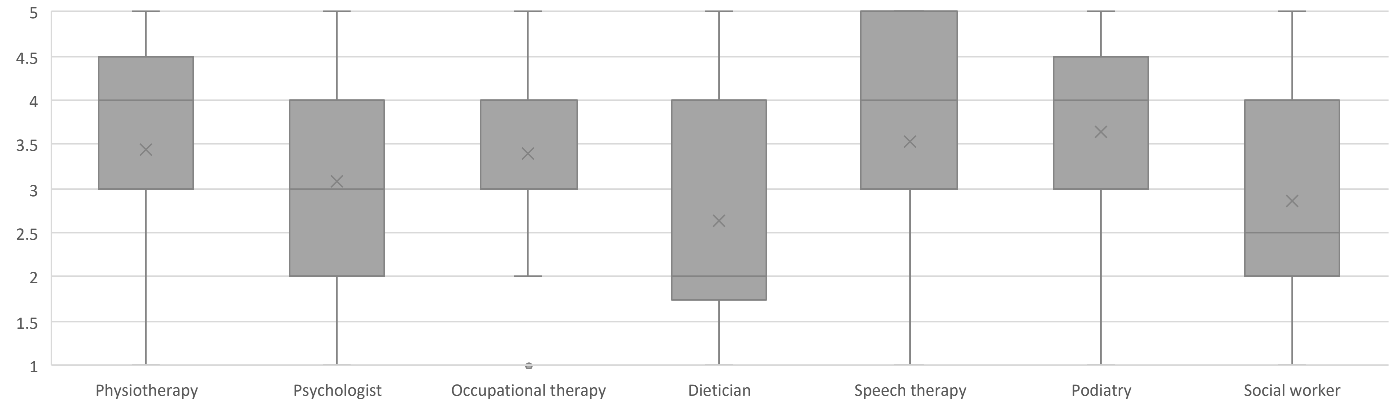

Figure 5.13: Effectiveness of allied health

Lifestyle changes

Participants were asked about any lifestyle changes they had made since diagnosis, the quality of life from these changes, and how effective they found them.

Most participants used at made at least one lifestyle change (n=204, 67.77%), and on average made 1 changes (median=1.00, IQR=1.00).

The most common lifestyle change used was diet changes (n=150, 51.02%), followed by exercise (n=146, 59.84%), and reduce alcohol (n=56, 22.95%).

On average, quality of life from diet changes was in the 'life was average' range (median=4.00, IQR=3.00), and was found to be ineffective (median=2.00, IQR=2.00).

On average, quality of life from exercise was in the 'life was average' range (median=4.00, IQR=3.00), and was found to be ineffective (median=2.00, IQR=2.00).

On average, quality of life from reducing alcohol was in the 'life was average' range (median=4.00, IQR=2.50), and was found to be ineffective (median=2.00, IQR=2.00).

On average, quality of life from quitting smoking was in the 'life was a little distressing' range (median=3.00, IQR=1.00), and was found to be ineffective (median=2.00, IQR=3.00).

Table 5.11: Lifestyle changes

Lifestyle changes	Number	Percent	Median quality of life	IQR	Median effectiveness	IQR
Diet changes (n=294)	150	51.02	4.00	3.00	2.00	2.00
Exercise (n=244)	146	59.84	4.00	3.00	2.00	2.00
Reduce alcohol (n=244)	56	22.95	4.00	2.50	2.00	2.00
Quit smoking (n=244)	19	7.79	3.00	1.00	2.00	3.00

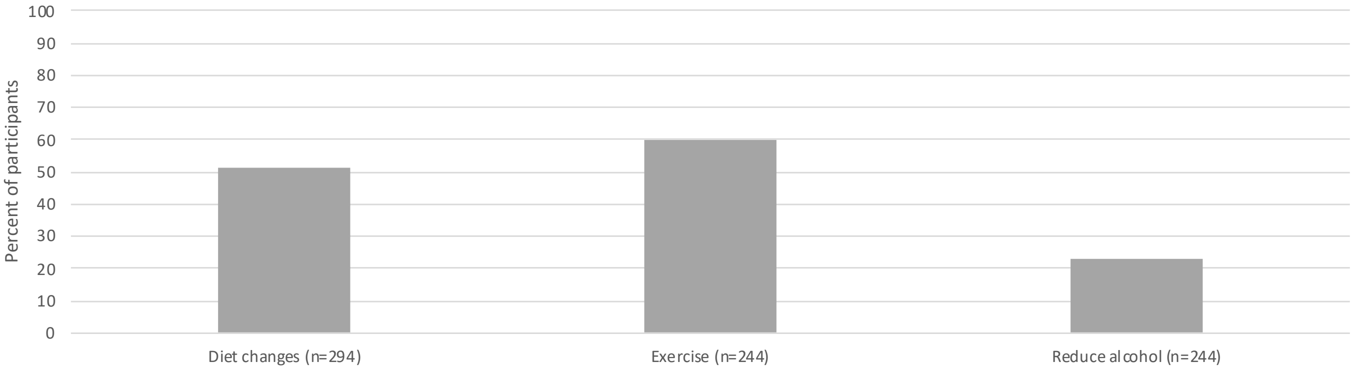

Figure 5.14: Lifestyle changes

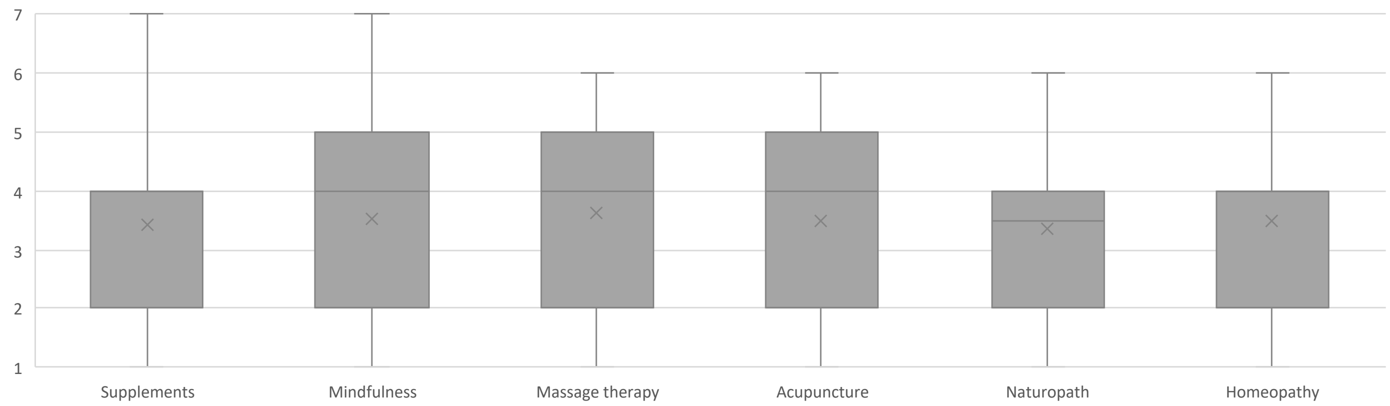

Figure 5.15: Quality of life from lifestyle changes

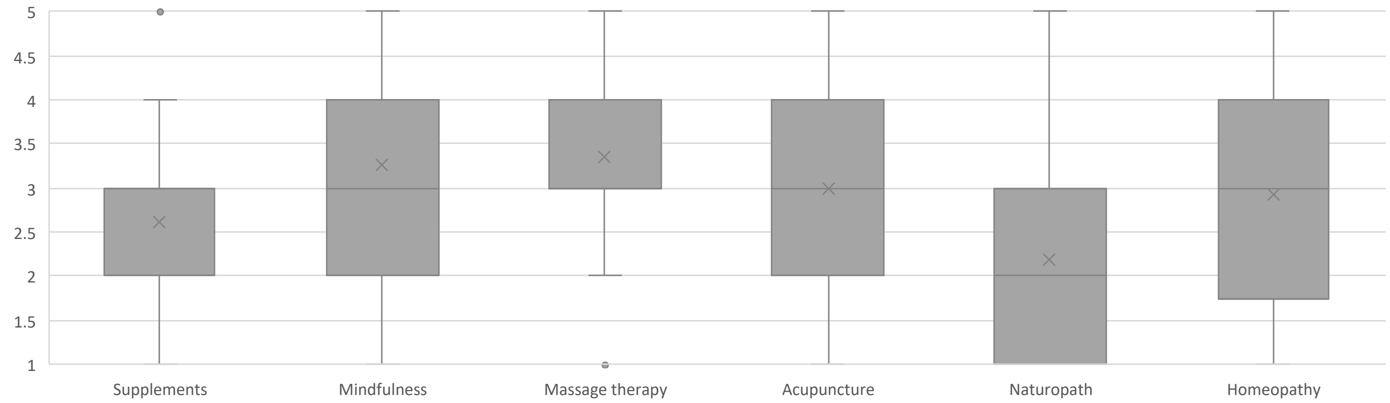

Figure 5.16: Effectiveness from lifestyle changes

Complementary therapies

Participants were asked about any complementary therapies they used to manage their condition, the quality of life from these changes, and how effective they found them.

Most participants used at made at least one complementary therapy (n=216, 68.35%), and on average used 1 therapies (median=1.00, IQR=2.00).

The most common complementary therapy used was supplements (n=136, 46.10%), followed by mindfulness or relaxation (n=121, 45.83%), and massage therapy (n=80, 30.30%).

On average, quality of life from supplements was in the 'life was average' range (median=4.00, IQR=3.00), and was found to be somewhat effective (median=2.00, IQR=1.00).

On average, quality of life from mindfulness or relaxation was in the 'life was average' range (median=4.00, IQR=3.00), and was found to be moderately effective (median=3.00, IQR=2.00).

On average, quality of life from massage therapy was in the 'life was average' range (median=4.00, IQR=3.00), and was found to be moderately effective (median=3.00, IQR=1.00).

On average, quality of life from acupuncture was in the 'life was average' range (median=4.00, IQR=3.00), and was found to be moderately effective (median=3.00, IQR=2.00).

On average, quality of life from naturopathy was in the 'life was a little distressing to average' range (median=3.50, IQR=2.00), and was found to be somewhat effective (median=2.00, IQR=2.00).

On average, quality of life from homeopathy was in the 'life was average' range (median=4.00, IQR=3.00), and was found to be somewhat effective (median=2.00, IQR=2.25).

Table 5.12: Complementary therapies

Complementary therapies	Number	Percent	Median quality of life	IQR	Median effectiveness	IQR
Supplements (n=295)	136	46.10	4.00	3.00	2.00	1.00
Mindfulness or relaxation (n=264)	121	45.83	4.00	3.00	3.00	2.00
Massage therapy (n=264)	80	30.30	4.00	3.00	3.00	1.00
Acupuncture (n=264)	34	12.88	4.00	3.00	3.00	2.00
Naturopathy (n=245)	20	8.16	3.50	2.00	2.00	2.00
Homeopathy (n=245)	16	6.53	4.00	3.00	2.00	2.25

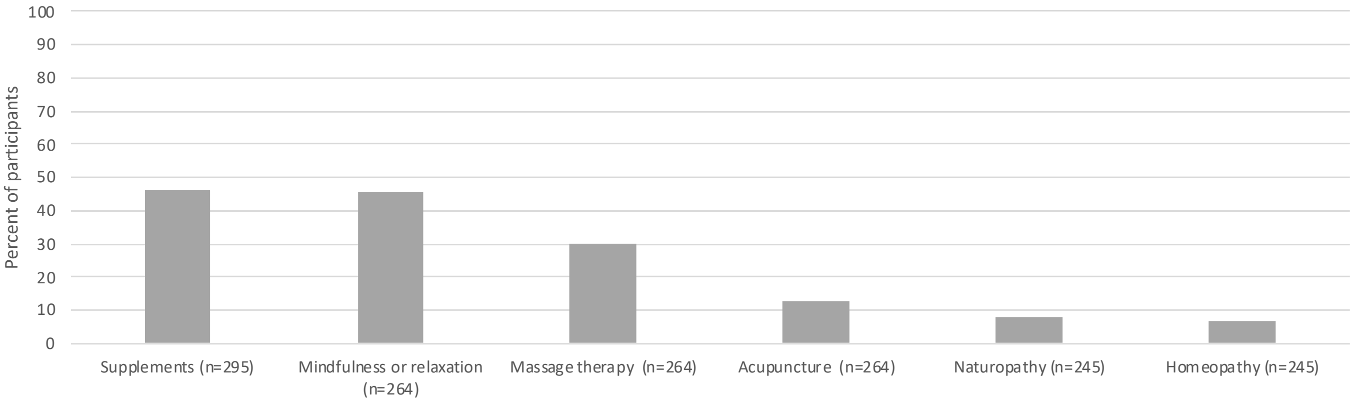

Figure 5.17: Complementary therapies

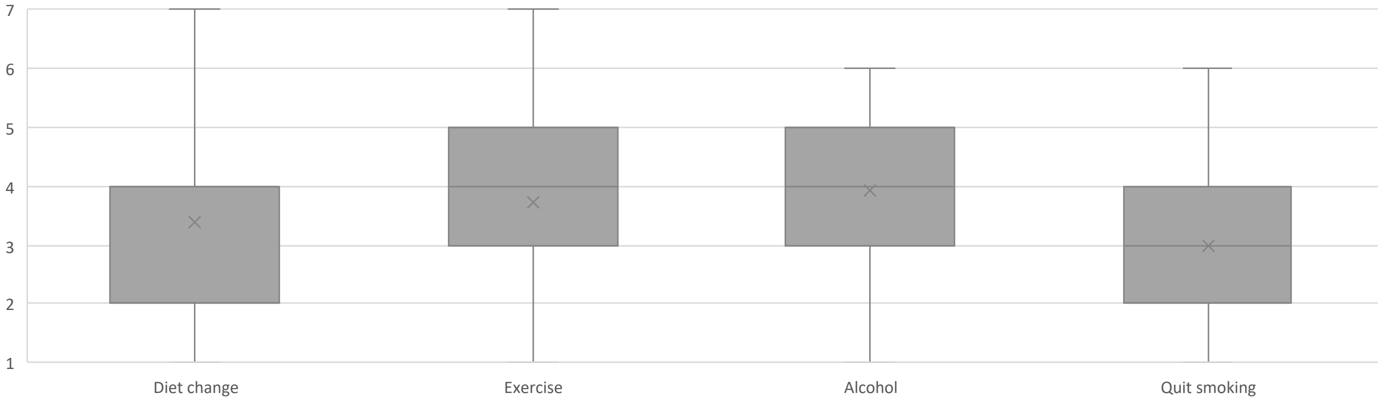

Figure 5.18: Quality of life from complementary therapies

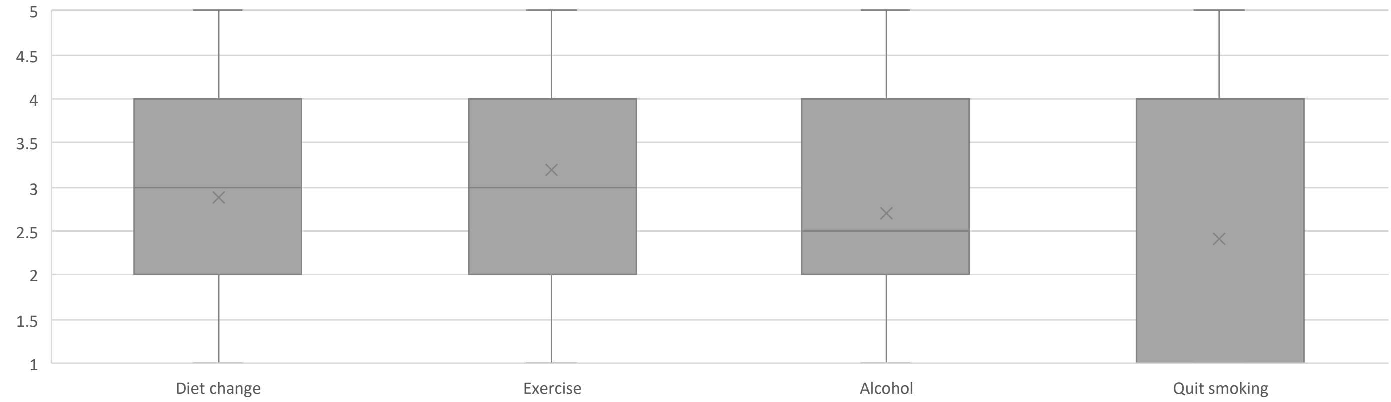

Figure 5.19: Effectiveness of complementary therapies

Clinical trials

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 111 participants (35.81%) that had discussions about clinical trials, 32 participants (10.32%) had brought up the topic with their doctor, and the doctor of 79 participants (25.48%) brought up the topic. The majority of participants had not spoken to anyone about clinical trials (n=199, 64.19%).

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 were 37 participants (11.86%) that had taken part in a clinical trial, 155 participants (49.68%) that would like to take part in a clinical trial if there was a suitable one, and 120 participants, that have not participated in a clinical trial and do not want to (38.46%).

Table 5.13: Clinical trial discussions

Clinical trial discussions	Number (n=310)	Percent
Participant brought up the topic of clinical trials doctor for discussion	32	10.32
Doctor brought up the topic of clinical trials for discussion	79	25.48
Participant has never spoken about clinical trials	199	64.19

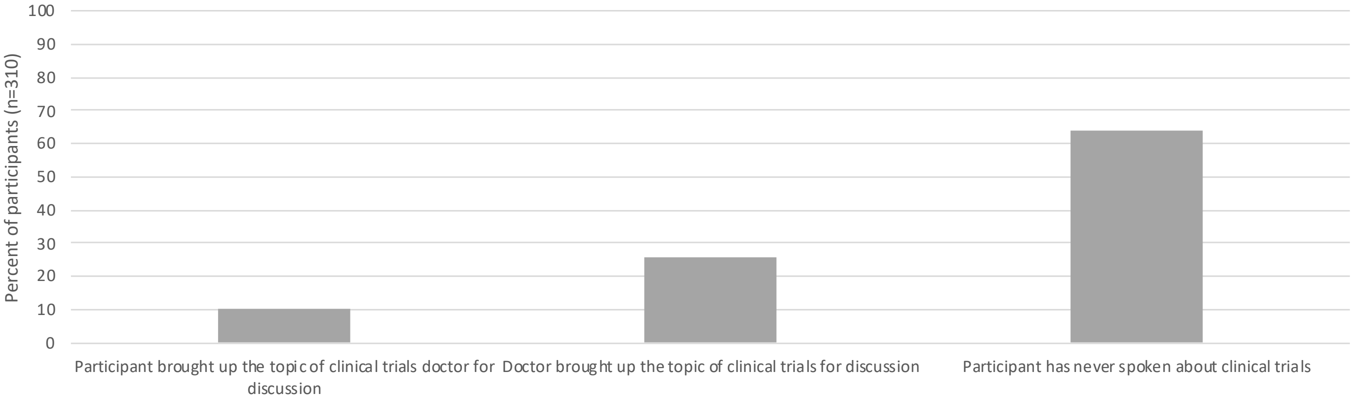

Figure 5.20: Clinical trial discussions

Table 5.14: Clinical trial participation

Clinical trial participation	Number (n=312)	Percent
Has not participated in a clinical trial	120	38.46
Has not participated in a clinical trial but would like to if there is one	155	49.68
Has participated in a clinical trial	37	11.86

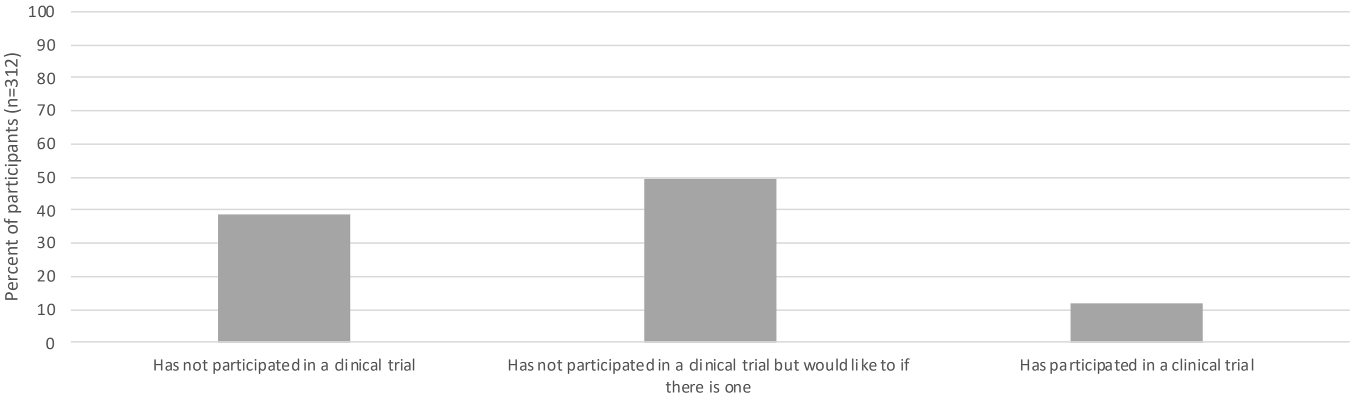

Figure 5.21: Clinical trial participation

Description of mild side effects

In the structured interview, participants were asked how they would describe the term ‘mild side effects’. The most common descriptions of mild side effects were described using a specific example (53.69%), and those that do not interfere with life (33.24%).
Other themes included those that are resolved in a short amount of time (9.66%) and those that can be managed with self-medication or self-management (3.98 %).

When a specific side effect was described, the most common responses were fatigue and lethargy (12.50%), headaches (11.36%), aches/pain (10.51%) and gastrointestinal distress (9.38%). Other themes included lost of appetite (7.95%), skin rash, dry or itchy skin (7.67%) and nausea (3.98%).

Participant provides a specific side effect as an example

So mild would be like, I guess, you know, bruising from the the needle or yeah, the tummy discomfort from the antibiotics, that sort of thing. And just like if I was using creams, just the uncomfortableness of having it in the areas that they were in and having to sort of deal with that. Yeah.
Participant 022_2023AUDSK

Feeling a bit nauseous sometimes, yeah, but nothing that goes away or well, headaches. So just to get headaches from different medication, that was something that makes you sleep. I forget what it was called. It's melatonin.
Participant 088_2023AUENM

Fingers bending, the fibrosis, I suppose. I have lost muscle tone. I drop a lot of things, so that's a mild side effect.
Participant 008_2023AUDIS

A mild effect, I would say it's more like a headache. A severe thing is like constantly going back and forth to the toilet, because you can't take your medication and know you'd be out in public because you only think, oh, where's the toilet going to be?
Participant 023_2023AUDIS

Participant describes mild side effects as those that do not interfere with daily life

Don't really impact your ability to function normally, you know? So maybe feeling a bit squirmy in the stomach or yeah, having a bit of a headache or some itchy skin.
Participant 005_2023AUDSK

Basically he can like still go and do his go to school and do his things. It doesn't impact too much on his daily life.
Participant 009_2023AUDSK

Side effects, to me, I think that would be something that's, whilst you have side effects, doesn't necessarily impact your daily life too much. Yeah. Do you know, doesn't sort of decrease the quality of life I guess.
Participant 032_2023AUDSK

That you can continue to function. There's a little bit of with, a little bit of impact, but it's a manageable one and nobody external would be able to notice that you got.
Participant 092_2023AUENM

Participant describes mild side effects as those that can be self-managed

Well, you know, like I said, I had a, I had a sort of dull headache and I didn't feel the need to take anything. And then one night I had a more severe headache. But I just took two Panadol and I was fine and increased my water. So I drank a lot of water and I didn't have any severe.
Participant 010_2023AUORC

Well, I've only had the dry lips. Mine was just mild. Just Vaseline on the lips and just live with it.
Participant 013_2023AUDSK

So mild side effects I would kind of refer to as probably something like thrush and you can just use Canestan and you know sort it out you can, you can sort out the side effect.
Participant 017_2023AUDSK

That's an interesting question. Mild side effects I guess would be something that it would like mild side effect, something that you can kind of maybe manage daily on your own like with the correct mild side. Well, that's interesting. Something easily treatable at home, I guess.
Participant 029_2023AUDPA

Participant describes mild side effects as those that are resolved in a short amount of time

So I guess with mild side effects like things like you know, just I guess short term changes in like bowel habit, bowel habits, I guess it's hard to tell because of his age whether he gets it's just he, I don't even think he would know if he had a headache or anything like that. But short term I guess would be my description I guess of mild side effects or…Especially with like the liver enzyme changing and things like, I wouldn't call that a mild side effect, but if it was only short term then I would probably describe it more as mild. But I guess anything that's not going to cause long term damage or is only for a short period of time I would describe as mild side effects.
Participant 020_2023AUORC

A mild side effect might be a slight rash. That's not irritating. A mild side effect maybe something that dissipates really quickly and that doesn't affect the mental, mental and mental health wise. So not headaches or anxiety or depression. Yeah, a mild side effect of yeah not, not…doesn't make the lifestyle different.
Participant 023_2023AUORC

The mild side effect to me would be something that rarely impacts at all, maybe like slight nausea for like 10 minutes after taking it or whatever, but there's no actual impact on it.
Participant 025_2023AUORC

Maybe makes me drowsy, a bit drowsy. Something that's not added-- reacting in a minimal way but short-term as well.
Participant 058_2023AUDPA

Participants reports not experiencing any mild side effects

Mild side effects is level. He's very, very he's got a high tolerance, you know. I know it's really, really bad before he speaks up.
Participant 026_2023AUORC

She's very, like, resilient and guess she's been in the hospital so much. She's just used to everything, like nothing really bothers her.
Participant 013_2023AUDPA

In relation to her, well, she didn't really have any side effects from the medical procedures like that if her heart was repaired and she's been fine ever since.
Participant 027_2023AUDPA

Table 5.15: Description of mild side effects

Description of mild side effects	All participants		Aged under 18		Aged 18 to 44		Aged 45 to 64		Aged 65 plus		Trade or high school		University		Regional or remote		Metropolitan		Mid to low status		Higher status	
	n=352	%	n=352	%	n=81	%	n=45	%	n=32	%	n=95	%	n=32	%	n=247	%	n=105	%	n=252	%	n=98	%
Specific example	189	53.69	34	50.75	51	62.96	**32**	**71.11**	15	48.39	**36**	**37.89**	**21**	**67.74**	129	52.23	60	57.14	134	53.17	53	54.08
Do not interfere with life	117	33.24	19	28.36	29	35.80	15	33.33	**15**	**48.39**	24	25.26	**15**	**48.39**	88	35.63	29	27.62	86	34.13	30	30.61
Resolves in short time/Temporary}	34	9.66	7	10.45	2	2.47	0	0.00	4	12.90	17	17.89	4	12.90	21	8.50	13	12.38	25	9.92	9	9.18
No mild side effects experienced (can not describe)	16	4.55	6	8.96	4	4.94	0	0.00	0	0.00	3	3.16	3	9.68	9	3.64	7	6.67	10	3.97	6	6.12
Can be managed with self-medication or self-management (Over-the-counter)	14	3.98	6	8.96	0	0.00	1	2.22	3	9.68	2	2.11	2	6.45	6	2.43	8	7.62	11	4.37	3	3.06

Description of mild side effects	All participants		Developmental anomalies		Diseases of the immune system		Diseases of the nervous system		Diseases of the skin		Endocrine, nutritional or metabolic diseases		Other rare condition		Person with condition		Family or carer		Female		Male	
	n=352	%	n=69	%	n=116	%	n=108	%	n=59	%	n=172	%	n=172	%	n=100	%	n=252	%	n=176	%	n=176	%
Specific example	189	53.69	40	57.97	63	54.31	52	48.15	34	57.63	97	56.40	89	51.74	47	58.75	142	56.35	94	53.41	95	53.98
Do not interfere with life	117	33.24	22	31.88	40	34.48	34	31.48	21	35.59	50	29.07	65	37.79	30	37.50	87	34.52	51	28.98	66	37.50
Resolves in short time/Temporary}	34	9.66	7	10.14	14	12.07	8	7.41	5	8.47	18	10.47	16	9.30	13	16.25	21	8.33	15	8.52	19	10.80
No mild side effects experienced (can not describe)	16	4.55	5	7.25	5	4.31	3	2.78	3	5.08	10	5.81	6	3.49	7	8.75	9	3.57	8	4.55	8	4.55
Can be managed with self-medication or self-management (Over-the-counter)	14	3.98	6	8.70	3	2.59	3	2.78	2	3.39	5	2.91	9	5.23	5	6.25	9	3.57	9	5.11	5	2.84

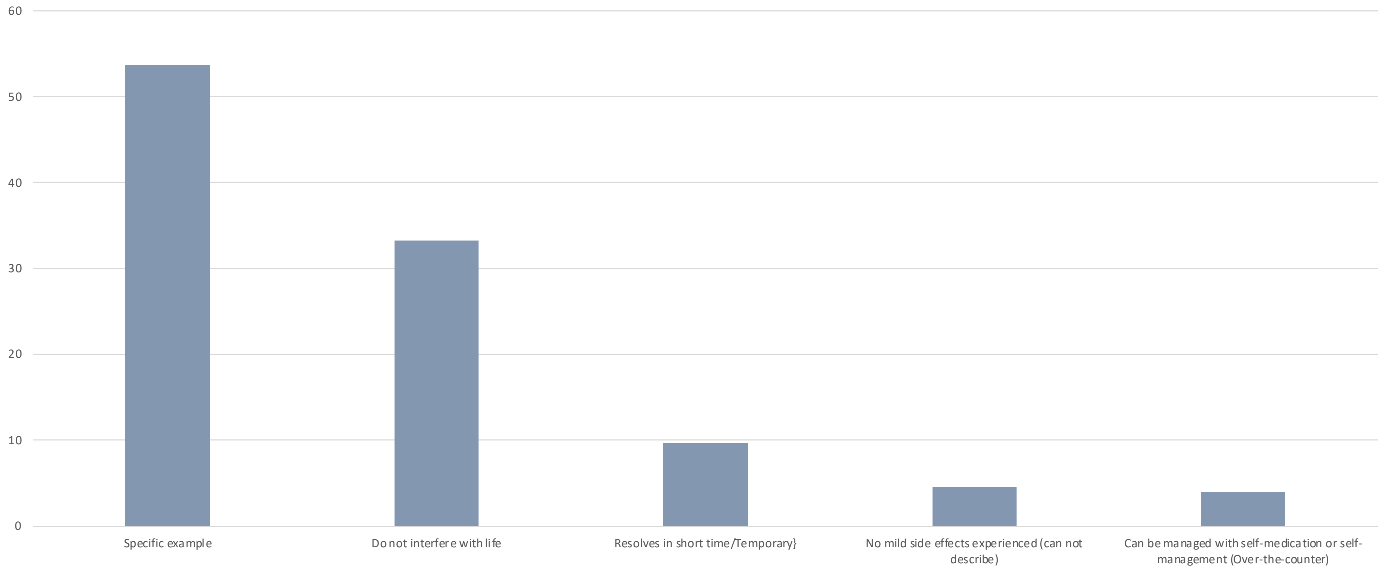

Figure 5.22: Description of mild side effects

Table 5.16: Description of mild side effects – subgroup variations

Description of mild side effects	Reported less frequently	Reported more frequently
Participant provides a specific side effect as an example	Endocrine, nutritional or metabolic diseases	Diseases of the nervous system Other rare condition
Participant describes mild side effects as those that do not interfere with daily life		Diseases of the skin Other rare condition
Participant describes mild side effects as those that can be self-managed		-

Table 5.17: Description of mild side effects (Specific side effects)

Description of mild side effects (Specific side effects)	All participants		Developmental anomalies		Diseases of the immune system		Diseases of the nervous system		Diseases of the skin		Endocrine, nutritional or metabolic diseases		Other rare condition		Person with condition		Family or carer		Female		Male	
	n=352	%	n=352	%	n=81	%	n=45	%	n=32	%	n=95	%	n=32	%	n=247	%	n=105	%	n=252	%	n=98	%
Fatigue/lethargy	44	12.50	12	17.91	8	9.88	8	17.78	0	0.00	13	13.68	3	9.68	22	8.91	22	20.95	27	10.71	17	17.35
Headaches	40	11.36	4	5.97	15	18.52	5	11.11	2	6.45	8	8.42	6	19.35	29	11.74	11	10.48	32	12.70	7	7.14
Aches/pain	37	10.51	7	10.45	9	11.11	6	13.33	6	19.35	6	6.32	3	9.68	27	10.93	9	8.57	31	12.30	5	5.10
Gastrointestinal distress	33	9.38	3	4.48	9	11.11	7	15.56	1	3.23	10	10.53	3	9.68	29	11.74	4	3.81	28	11.11	5	5.10
Loss of appetite	28	7.95	1	1.49	13	16.05	4	8.89	2	6.45	5	5.26	3	9.68	25	10.12	3	2.86	21	8.33	7	7.14
Rash, dry or itchy skin/sensitive skin	27	7.67	2	2.99	8	9.88	2	4.44	10	32.26	1	1.05	4	12.90	22	8.91	5	4.76	21	8.33	6	6.12
Nausea	14	3.98	3	4.48	6	7.41	2	4.44	1	3.23	1	1.05	1	3.23	9	3.64	5	4.76	12	4.76	2	2.04

Description of mild side effects (Specific side effects)	All participants		Aged under 18		Aged 18 to 44		Aged 45 to 64		Aged 65 plus		Trade or high school		University		Regional or remote		Metropolitan		Mid to low status		Higher status	
	n=352	%	n=69	%	n=116	%	n=108	%	n=59	%	n=172	%	n=172	%	n=100	%	n=252	%	n=176	%	n=176	%
Fatigue/lethargy	44	12.50	13	18.84	13	11.21	12	11.11	6	10.17	21	12.21	21	12.21	9	11.25	35	13.89	18	10.23	26	14.77
Headaches	40	11.36	6	8.70	16	13.79	12	11.11	6	10.17	19	11.05	21	12.21	14	17.50	26	10.32	25	14.20	15	8.52
Aches/pain	37	10.51	6	8.70	13	11.21	11	10.19	7	11.86	17	9.88	20	11.63	13	16.25	24	9.52	21	11.93	16	9.09
Gastrointestinal distress	33	9.38	3	4.35	7	6.03	10	9.26	13	22.03	16	9.30	16	9.30	4	5.00	29	11.51	13	7.39	20	11.36
Loss of appetite	28	7.95	0	0.00	11	9.48	13	12.04	4	6.78	16	9.30	12	6.98	5	6.25	23	9.13	13	7.39	15	8.52
Rash, dry or itchy skin/sensitive skin	27	7.67	5	7.25	15	12.93	5	4.63	2	3.39	13	7.56	14	8.14	10	12.50	17	6.75	18	10.23	9	5.11
Nausea	14	3.98	3	4.35	7	6.03	4	3.70	0	0.00	9	5.23	5	2.91	6	7.50	8	3.17	8	4.55	6	3.41

Figure 5.23: Description of mild side effects (Specific side effects)

Table 5.18: Description of mild side effects (Specific side effects) – subgroup variations

Description of mild side effects (Specific side effects)	Reported less frequently	Reported more frequently
Fatigue/lethargy	Diseases of the skin	
Gastrointestinal distress		Aged 65 plus
Rash, dry or itchy skin/sensitive skin		Diseases of the skin

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 were described using a specific example (47.73%), and those that impact everyday life or ability to conduct activities of daily living (28.13%). Other themes included those that are life threatening or result in hospitalisation (8.52%), those that cause long-term damage to their body (7.67%).

When a specific side effect was described, the most common examples were aches and pain (17.33%), emotional and mental impact (7.39%), and nausea with vomiting (6.53%). Other themes included fatigues (5.11%), gastrointestinal distress (4.83%), impact on sleep (4.26%), vision problems (3.98%), and impact on sleep (4.55%).

Participant provides a specific side effect as an example

And I guess the severe one is the depression. But obviously I know what it is, I know what it feels like. I can pretty much handle it until it goes away.
Participant 014_2023AUDSK

Severe would just be mostly the pain, then the pain after I guess, the treatment…like the pain after the surgery or the excision and that sort of thing. That's probably been the worst.
Participant 022_2023AUDSK

Apart from like vomiting and nausea and gastrointestinal upset would be, we'll see…You would consider it mild, but in my child with their issues, it's actually quite a severe side effect. I can't really think of anything else.
Participant 021_2023AUORC

Massive, massive migraine, nauseous, vomiting. Can't cope with the vomiting and the really well, the migraines were like well I used to get migraines years ago. So every now and then I don't know seem to get it. But yeah some medications can give me headache, really bad headaches and I can't cope with that and I can't cope with the vomiting. But actually anything else is just yeah, you know.
Participant 088_2023AUENM

I would say it's that horrendous scream. He's been a really happy, really calm, really relaxed baby the whole way through. I think it's because of the steroids. He got more breakouts pretty badly. It seems that it never left that instant scream. He can scream for hours. I can be holding him or feeding him or patting him like it doesn't matter. He will now scream for hours at a time. If he's distracted in any way, he'll just scream so that's probably the most severe side effects for us.
Participant 064_2023AUDPA

Participant describes severe side effects as those that impact everyday life/ability to conduct activities of daily living

That would be medication when I tried the medication too fast and I just felt super spacey and couldn't walk properly and couldn't remember anything. I couldn't function and had to come off it. That was pretty severe. Also, with physical treatments, if I've say seen someone I'm not familiar with or it's just been a really difficult session because something was quite painful. Then there are times when the pain's too high, and I've had to stop treatment, or I've just had to sleep after. I've had to take stronger pain medication and sleep it off and not been able to function well for a couple of days, then I'd say that was probably a more severe side effect.
Participant 004_2023AUDPA

Well, like not being able to eat, or, yeah, not being able to get out of bed, or not being able to perform the normal functions like mild side effects. I'd say you should still be able to do things that you were already going to do, just maybe not at full 100% capacity. Yeah, with, you know, severe side effects. I feel like that's, you know, when you having something prevent you from being able to do something in order to try and fix another problem. You're then creating a myriad, you know, like a circle, a cycle where you're going to need to take something else for this. You know…it was like, well, here's some pills for your stomach, and then here's some pills for making those pills, for making you nauseous, for making your stomach feel better from the doxy. Participant 006_2023AUDSK

Daily life severe is when he can't go to school or he can't do his daily activities.
Participant 009_2023AUDSK

So severe side effects would mean that you couldn't go about your daily living, or your daily living would be severely impacted.
Participant 031_2023AUORC

Participant describes severe side effects as those that are life threatening or result in hospitalisation

Worst case scenario, allergy and an ambulance.
Participant 007_2023AUORC

Well, severe side effects like I said would be more along the life-threatening things which would require fairly urgent treatment or, or you know, to be looked at by someone. And PATIENT never really had anything like that. I suppose the worst side effects she would have had, it would be some pain when it comes to the operations on her legs. But apart from that and I wouldn't say that was severe, you know, more moderate. Yeah. And you know, Panadol and she's fixed like there was nothing really, you know, that would impact her greatly, really.
Participant 024_2023AUDPA

The severe side effects would be, in terms of the Afinitor which we keep an eye out for would be the effects of immuno-suppression. If he was to get sick, a simple cold could get a lot worse and he couldn't eat, he'd be hospitalised, so we just keep an eye. Not that we've ever had to do that, but I would think that that would be a severe symptom. The mild symptoms are skin reactions to the point that the dental debits that he got from the Vigabatrin. They're all things that we could deal with and fix. They're more severe stuff, anaphylactic reactions and severe immuno-suppression where we have, yes. I have to do a lot of things to help him out.
Participant 044_2023AUDPA

Participant describes severe side effects as those that cause long-term damage to their body

Yeah, kind of hinge it off what I said what we said about mild, it's like where it does interfere with your day-to-day functions. So yeah, where it's, where it's, it's having a negative and again fairly like noticeable, measurable and immediate or long-term impact on your quality of life. So it's, you know, it's having a negative impact.
Participant 011_2023AUORC

So when we were in hospital, one of the IV antibiotics he had a reaction to and it was like fever and labuored breathing and he came up in a rash and I would have…I describe that as a as a severe side effect… and it happened and they ended up taking him off it, but they weren't sure whether it was the reaction to the anaesthetic. But then the next time when he was on a different medication, it didn't happen at all. So I would describe that like the laboured breathing, the fevers, and the rash altogether. And just that really awful discomfort. I'm assuming he had a headache as well but like he was three at the time so he wouldn't have been able to communicate that but just overall was very miserable and also we deal can be with the risk of cataracts. I would describe the cataracts developing as a, as a quite a you know not a good side effect and I would also and I think like I guess very high level raised liver function over a long period of time that would lead to eventually to raise the liver. I would call that any…Yeah, in that category.
Participant 020_2023AUORC

Prednisone. Anything over 20 milligrams or even 15 milligrams. I don't sleep agitated. My hair grows and my face grows a nice downy peach fur. My bone density has dropped a couple of times, and I've had long stints on Prednisone, so yeah, I think Prednisone I would class as a severe side effect.
Participant 019_2023AUDPA

Yeah, well, liver failure, it would be severe and, and when the doctor tells you it will be fatal if you ever have this again, I think that's severe.
Participant 025_2023AUDPA

Participants reported not experiencing any severe side effects

Yeah, I haven't considered the…any treatment at the moment was I've been getting good results for the past 18 years, so.
Participant 001_2023AUORC

Well, I don't think I've actually experienced any from the medication anyway. Yeah, so that mean that's good so far.
Participant 003_2023AUDSK

Yeah. So I haven't had that experience, so.
Participant 010_2023AUDIS

Participant identifies severe side effects as impacting their everyday life by being bed ridden

Severe would probably be something where I'm in a bit more pain where I maybe need to lay down and get a heat pack out and put it on the area or I have an intense migraine. To the point where I need to turn off the lights and put myself to sleep and I can't really do much.
Participant 010_2023AUDSK

Yeah, so not something I've experienced with this particular medication, but I would class it as

something that makes me bedridden, home ridden. Something incredibly painful that doesn't allow me to get through my day and do my normal tasks. Yeah.
Participant 027_2023AUDSK

Mild is, with treatment I've had before, a bit of diarrhea or a bit of nausea, things like that. Severe side effects is where you just can't even move, [laughs] your whole body aches and you just can't move, you've got fatigue and you can't even get out of bed. That wasn't mild because I don't like staying in bed. [laughs]
Participant 001_2023AUDIS

Participant identifies severe side effects as requiring medical intervention

Something that you would have to seek regular treatment for, like someone else, like something that you..a side effect or something. Something that affects you that you need constant, constant and considerable assistance with managing all.
Participant 029_2023AUDPA

Well, something that requires, you know, a doctor's visit or an emergency presentation or more than just a day off school, like a day off school where I'd have to monitor her or something would be a severe side effect. I could...a day off school where she missed school and I couldn't.. I need to monitor her.
Participant 035_2023AUDPA

I think that's something that would interfere with his daily living, so something that required some intervention and he hasn't had anything like that.
Participant 040_2023AUDPA

Table 5.19: Description of severe side effects

Description of severe side effects	All participants		Developmental anomalies		Diseases of the immune system		Diseases of the nervous system		Diseases of the skin		Endocrine, nutritional or metabolic diseases		Other rare condition		Person with condition		Family or carer		Female		Male	
	n=352	%	n=352	%	n=81	%	n=45	%	n=32	%	n=95	%	n=32	%	n=247	%	n=105	%	n=252	%	n=98	%
Specific example	168	47.73	34	50.75	45	55.56	32	71.11	11	35.48	32	33.68	14	45.16	112	45.34	56	53.33	117	46.43	49	50.00
Impact everyday life/ability to conduct activities of daily living	99	28.13	13	19.40	23	28.40	8	17.78	14	45.16	34	35.79	7	22.58	77	31.17	22	20.95	71	28.17	28	28.57
Life threatening or result in hospitalisation	30	8.52	6	8.96	10	12.35	3	6.67	3	9.68	5	5.26	3	9.68	21	8.50	9	8.57	23	9.13	7	7.14
Cause long-term damage to their body/last long term	27	7.67	9	13.43	10	12.35	1	2.22	1	3.23	3	3.16	3	9.68	17	6.88	10	9.52	20	7.94	7	7.14

Description of severe side effects	All participants		Aged under 18		Aged 18 to 44		Aged 45 to 64		Aged 65 plus		Trade or high school		University		Regional or remote		Metropolitan		Mid to low status		Higher status	
	n=352	%	n=69	%	n=116	%	n=108	%	n=59	%	n=172	%	n=172	%	n=100	%	n=252	%	n=176	%	n=176	%
Specific example	168	47.73	36	52.17	54	46.55	51	47.22	27	45.76	84	48.84	81	47.09	47	58.75	121	48.02	88	50.00	80	45.45
Impact everyday life/ability to conduct activities of daily living	99	28.13	15	21.74	37	31.90	30	27.78	17	28.81	49	28.49	48	27.91	26	32.50	73	28.97	47	26.70	52	29.55
Life threatening or result in hospitalisation	30	8.52	7	10.14	7	6.03	11	10.19	5	8.47	16	9.30	14	8.14	7	8.75	23	9.13	16	9.09	14	7.95
Cause long-term damage to their body/last long term	27	7.67	8	11.59	9	7.76	7	6.48	3	5.08	13	7.56	14	8.14	12	15.00	15	5.95	15	8.52	12	6.82

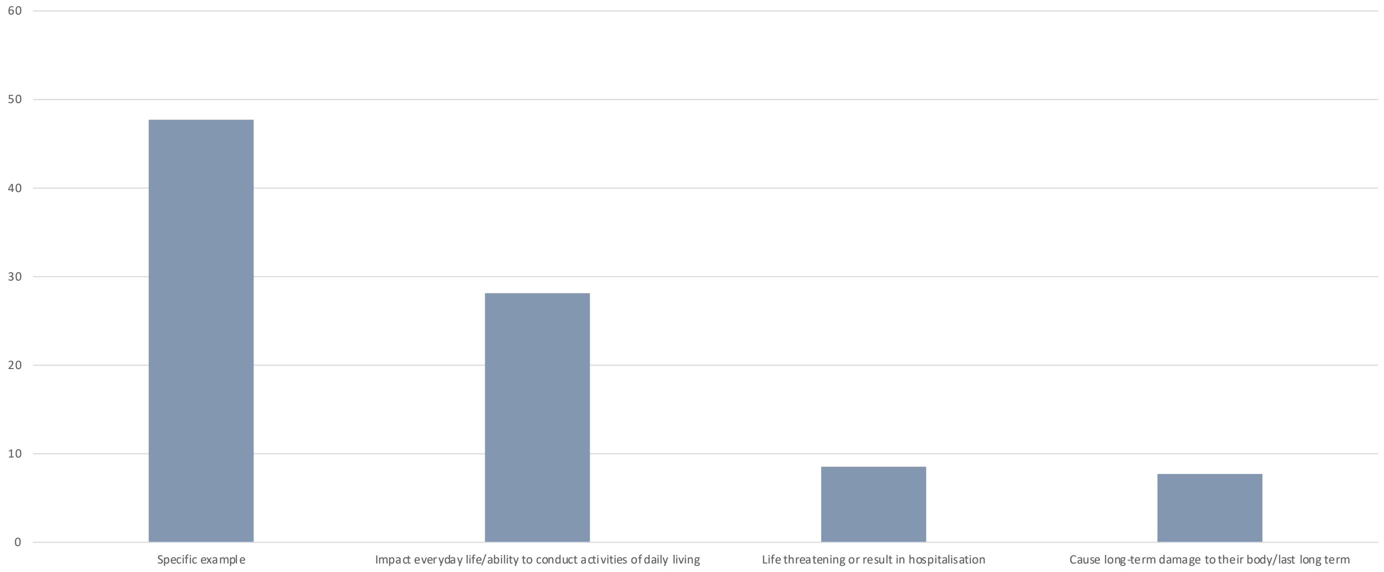

Figure 5.24: Description of severe side effects

Table 5.20: Description of severe side effects – subgroup variations

Description of severe side effects	Reported less frequently	Reported more frequently
Specific example	Diseases of the skin Endocrine, nutritional or metabolic diseases	Diseases of the nervous system Regional or remote
Impact everyday life/ability to conduct activities of daily living	Diseases of the nervous system	Diseases of the skin
Life threatening or result in hospitalisation		
Cause long-term damage to their body/last long term		

Table 5.21: Description of severe side effects (Specific example)

Description of severe side effects (Specific side effects)	All participants		Developmental anomalies		Diseases of the immune system		Diseases of the nervous system		Diseases of the skin		Endocrine, nutritional or metabolic diseases		Other rare condition		Person with condition		Family or carer		Female		Male	
	n=352	%	n=352	%	n=81	%	n=45	%	n=32	%	n=95	%	n=32	%	n=247	%	n=105	%	n=252	%	n=98	%
Aches/pain	61	17.33	5	7.46	20	24.69	**13**	**28.89**	**9**	**29.03**	9	9.47	5	16.13	49	19.84	12	11.43	45	17.86	15	15.31
Emotion/mental impact	26	7.39	5	7.46	4	4.94	6	13.33	2	6.45	5	5.26	4	12.90	16	6.48	10	9.52	16	6.35	10	10.20
Nausea with vomiting	24	6.82	2	2.99	8	9.88	4	8.89	1	3.23	7	7.37	2	6.45	17	6.88	7	6.67	15	5.95	9	9.18
Fatigue/lethargy	18	5.11	0	0.00	8	9.88	6	13.33	1	3.23	1	1.05	2	6.45	16	6.48	2	1.90	15	5.95	3	3.06
Gastrointestinal distress	17	4.83	3	4.48	3	3.70	1	2.22	**5**	**16.13**	3	3.16	2	6.45	12	4.86	5	4.76	9	3.57	8	8.16
Impact on sleep	16	4.55	3	4.48	6	7.41	1	2.22	1	3.23	3	3.16	2	6.45	11	4.45	5	4.76	14	5.56	2	2.04

Description of severe side effects (Specific side effects)	All participants		Aged under 18		Aged 18 to 44		Aged 45 to 64		Aged 65 plus		Trade or high school		University		Regional or remote		Metropolitan		Mid to low status		Higher status	
	n=352	%	n=69	%	n=116	%	n=108	%	n=59	%	n=172	%	n=172	%	n=100	%	n=252	%	n=176	%	n=176	%
Aches/pain	61	17.33	9	13.04	20	17.24	23	21.30	9	15.25	29	16.86	32	18.60	19	23.75	42	16.67	36	20.45	25	14.20
Emotion/mental impact	26	7.39	7	10.14	10	8.62	5	4.63	4	6.78	14	8.14	12	6.98	4	5.00	22	8.73	12	6.82	14	7.95
Nausea with vomiting	24	6.82	2	2.90	8	6.90	8	7.41	6	10.17	14	8.14	8	4.65	11	13.75	13	5.16	15	8.52	9	5.11
Fatigue/lethargy	18	5.11	1	1.45	8	6.90	8	7.41	1	1.69	9	5.23	9	5.23	5	6.25	13	5.16	10	5.68	8	4.55
Gastrointestinal distress	17	4.83	5	7.25	4	3.45	7	6.48	1	1.69	9	5.23	8	4.65	3	3.75	14	5.56	11	6.25	6	3.41
Impact on sleep	16	4.55	3	4.35	8	6.90	2	1.85	3	5.08	6	3.49	9	5.23	7	8.75	9	3.57	9	5.11	7	3.98

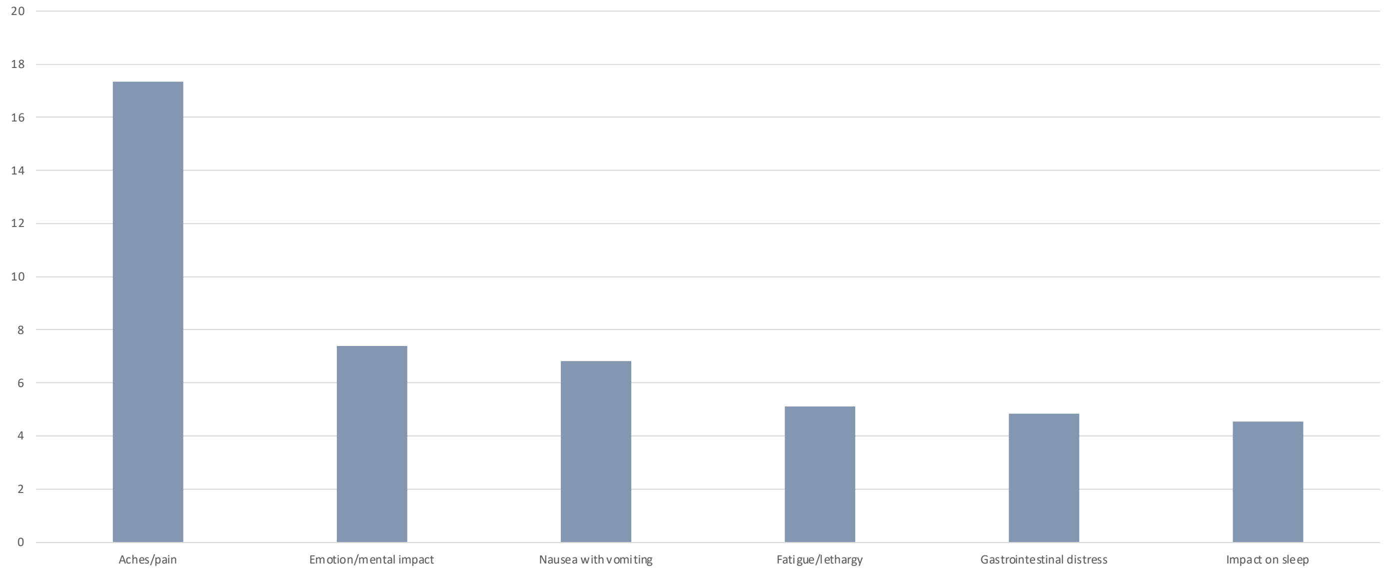

Figure 5.25: Description of severe side effects (Specific example)

Table 5.22: Description of severe side effects (Specific side effects)– subgroup variations

Description of severe side effects (Specific side effects)	Reported less frequently	Reported more frequently
Aches/pain		Diseases of the nervous system Diseases of the skin
Emotion/mental impact		
Nausea with vomiting		
Fatigue/lethargy		
Gastrointestinal distress		Diseases of the skin
Impact on sleep		

Adherence to treatment

Participants were asked in the structured interview what influences their decision to continue with a treatment regime. The most common responses were adhering to treatment for a specific amount of time (38.35%), adhering to treatment according to the advice of their specialist or as long as prescribed (36.08%), and adhering to treatment as long as side effects are tolerable (24.43 %). Other themes included never giving up on any treatment (11.36%), adhering to treatment as long as treatment is working (7.10%).

When participants stated a specific amount of time to adhere to a treatment, the most common amount of time was two to three months (14.20%), followed by one month (10.23%).

Participant describes adhering to treatment as per the advice of their specialist/as long as prescribed

Sorry, with the medications, we wouldn't stop anything without input from the doctors, from the medical team. So we persevered with those. We did raise our concerns quite frequently about various aspects of her treatment, but either the medical team thought it was worth continuing or we just hadn't got to the point yet where we had enough evidence. One of the issues we had as well with some of her treatments were that we were dependent on receiving…having ECGs to see whether they were having an impact and unfortunately the original neurologist was not supportive of doing ECGs. So we weren't really able to track her the efficacy of her treatments as closely as we would have liked.
Participant 090_2023AUENM

Okay. I usually stick to what the doctor-- as in if the doctor says, "Okay, you are on this medication." They usually give you two weeks to a month to -- if that's going to take a month to get into the system, I usually work on that particular length of time. If the doctor says this is X, you're going to take this for X and you're going to take it at this time, this time, and this time, that's what happened until the next time I see the doctor. The way I work where trying to take medication is concerned is, it might be a side effect or it might be that it's doing what it's supposed to in cleaning up my system so that it can start to work. It's based on what the doctor says as far as the medication is concerned. Basically, I do what the doctor tells me.
Participant 005_2023AUDPA

I'll keep using it until the next checkup because anytime I'm on a new medication, I have a checkup in, what, three to six months? So I won't stop until I'm actually given the clear, just in case there's some side effects.
Participant 026_2023AUDSK

Well, if there's any new treatment, so I'll stick to it till my doctor says I have to stop it. So it's just based on my doctor.
Participant 006_2023AUORC

Participant describes adhering to treatment for a specific amount of time

Usually at least a month for medications. When we try like new specialists or allied health, we obviously give them sort of like three to six months.
Participant 032_2023AUDPA

I don't know. I guess at least a few months to see. Well, it depends how long it takes the treatment to... So if she's taking her medicine, if it takes a good four weeks for it to start working or you've got to give it at least, you know, a couple of months.
Participant 010_2023AUDPA

I guess my rule for any medication is 3 months and if it's not working then it's probably not going to work. But yeah, this is my first lot of actual meds that I've tried.
Participant 014_2023AUDSK

Probably a month and then if it doesn't work, I kind of give up on it. And then when I go to the doctor's next day, I, you know, why do you stop that? And then if it's something that, if it's in regards to physio, they'll try and teach you a new technique of how to do that, or try and see if there's a different medication that does the same thing. Or just to clear the mucus out of the lungs. Yeah, they'll see if there is something else that they can do.
Participant 013_2023AUORC

Participant describes adhering to treatment as long as side effects are tolerable

If it wasn't showing any bad side effects, I'd probably persist, probably for about three months or so and then I'd be questioning. If we weren't seeing any positive improvement, I'd be questioning it with our paediatrician. I'd give it a little while, but if it had severe side effects, I'd be stopping pretty quick if I couldn't see a big change.
Participant 048_2023AUDPA

I will usually I follow the, the prescribed course unless I think I've had a big reaction to it, in which case I'll reach out to the prescriber and say this is what's going on. Can I stop this or should I be stopping this?
Participant 007_2023AUDSK

I would probably be like in constant consultation with the hospital, like the hospital care team, if I thought that it wasn't working. And I guess it depends on the severity of the side effects, whether I would cease it or continue it and try and push through. Yeah, I think I'd,

I'd throw the ball straight back to the kids hospital if I felt that it wasn't. I mean, he's a child. I don't want him to have any undue, you know side effects if he doesn't need them, so I don't really know how to answer that question other than say probably within a day or less depending on what the side effect was.
Participant 021_2023AUORC

Well, I'm not that kind of person. If I get a treatment and I'm told to take it from the beginning to the end, I take it until it's finished. I never, I never stop unless, unless it's giving me a severe side effect which really makes me ill, then I take it till it's finished.
Participant 010_2023AUORC

Participant describes not giving up on any treatment

I don't know. Because he's always wanted to persevere. He never gives up. Now he always sticks through with things. Yeah.
Participant 011_2023AUDPA

I've been on this injection for three years or something. I don't, I can't tell you. Like I said, I, I don't give up. I mean, you know, I, I was never a pill taker, but I started, you know, seeking assistance for mental health conditions when I was 30. And I understand I'm not medicated now, but I understand relapse and those sorts of things occur from people just saying and and I can tell you, there were times where I thought, 'what's this doing anyway?' But I never did. I mean, never missed a dose. Same with my, you know, contraceptive, those sorts of things. So how long before I gave up? Probably never until circumstances changed where it was, you know, time. So there's no real give up, but it's a more of a measured decision that I would, I would make.
Participant 015_2023AUDSK

I don't think I've ever given up on one. I'm compliant.
Participant 004_2023AUDIS

I probably haven't had any treatment as in, you know, any treatment that I've canned on. So, you know, my doctor has obviously put me through this first round of radiation. So I don't know what my next sort of outcome is going to be if it does start growing again or I find it somewhere else. So all we've really done so far is just had the radiation and that's about it. So I really don't know what my next sort of scenario will be whether the doctor's gonna try and cut it out or whether he's gonna, I don't know, put a probe in it and try and kill it from the inside or I'm not 100% sure at this stage, so yeah.
Participant 024_2023AUORC

Now, I wouldn't say we're actually ever really given up. We've always found persistence pays, and sometimes it might be frustrating for her, or she's a bit psych or lazy and doesn't want to do it, but so we find repetition working with us and therapists. It might take a few weeks to click on, but persistence pays, and as far as I can recall, we've never given up on any particular strategy or intervention.
Participant 022_2023AUORC

Participant describes adhering to treatment as long as treatment is working

That I guess would depend on what we are treating. We haven't had any issues with medication where we felt it was ineffective because he hasn't, he doesn't need to take a lot of medication. He, he was on medication for his reflux but that was very effective so you know that was a there was no discussion to be had it worked, kept taking it. I listen to the doctor's advice a lot. I would say if he's been taking a medication for something for three months and we're not seeing any improvement, I, I would, I'd take him off it because I think if you haven't seen any improvement in three months, then clearly either the medication's ineffective or you're treating the wrong thing. But we haven't had to do a lot medication wise it's, it's more been surgeries and because those surgeries have all been so successful you know we haven't really had to wonder if they were worth it.
Participant 091_2023AUENM

Well, the dose that they gave me in hospital kind of worked within that few hours, but then after that it come it came back and then after that the doses weren't really doing much of anything at all. So I don't know, I was on them for a few weeks before they changed to another one. But I guess I don't know because it wasn't working. My tolerance level of wanting to keep going with it wasn't good. OK, I think if it worked. I probably would have prolonged the medication, if you know what I mean.
Participant 032_2023AUORC

Participant describes needing to see test results/no evidence or reduction of disease in order to adhere to treatment

I just have, I haven't really challenged it. I've been told that I'm on this medication pretty much for life until they tell me otherwise. I think what helps is the regular blood tests of monitoring the levels of what is happening with my body.
Participant 004_2023AUORC

It's really hard to tell whether they're working or not because you need to have cultures after the medication is completed so it's not you…we don't usually question whether it's working or not, although we can tell. We are usually hopeful with symptoms decreasing, but it's more medications within sort of two or three days. We would know if he has like a really bad rash come up actually. Thinking about it, we've had a lot of drugs where the side effect might be really cranky, bad behaviour, and we probably put that one down as a mild because we have to just live with it. But, it's very obvious that it's for drugs and we, we usually stick it out with those, but yeah, it'd be the rash within to the 28, you know, 48 hours, yeah. Participant 023_2023AUORC

Yeah. So normally at the beginning we did a few things for like a month at a time and then we gave up because it wasn't working, which was probably the wrong decision when you look back. Yeah, normally we try things for at least two months. Yeah, but it's tricky. It's a tricky one because you can't really, the outward symptoms don't always match what's happening on the inside. So we you typically have to do a scope to really know the answer. OK, yeah.
Participant 079_2023AUDIS

Participant is unable to answer because they have not had treatment and/or cannot answer hypothetical question

Actually there's nothing I can think of that I know has worked in the sense that it's really just being careful reducing these amounts, being slower or that sort of thing. That has always helped but I don't feel that anyone is ever given me, certainly no magic bullets. Not even something I could try and say, oh, well, it didn't work. The approach from everybody is the peripherals, the things that show and the things that I can say like the dryness and the cold of my mouth and the cold disease and the oesophageal problems and things. They're all peripheral in a way. They're the result of the combination of things…no one can treat that because there isn't any treatment as far as I know.
Participant 012_2023AUDIS

Yeah, I don't think I can really answer this question. It's been yeah, I've just never had to experience it. I can answer for my brother, but that's not me. And if we he tried a couple of different ones, but it was like quite extensive. You give it a bit of time and in his circumstance like he had the time to give it where it's not sort of effect. He wasn't working, he was still at school, but he was like taking time off. So in that sense it was like not as detrimental. Whereas like if I was working, I think you'd have to consider a much different approach. But I can't really give you much of an answer for that question.
Participant 030_2023AUORC

We haven't really had to do any of that, I don't think, to be honest. We haven't had to sort of go in that this isn't, you know, working. It's useless. Yeah, we haven't, we haven't experienced that. So yeah, I guess I can't, I can't really answer that one.
Participant 034_2023AUDPA

Table 5.23: Adherence to treatment

Adherence to treatment	All participants		Aged under 18		Aged 18 to 44		Aged 45 to 64		Aged 65 plus		Trade or high school		University		Regional or remote		Metropolitan		Mid to low status		Higher status	
	n=352	%	n=352	%	n=81	%	n=45	%	n=32	%	n=95	%	n=32	%	n=247	%	n=105	%	n=252	%	n=98	%
Adhering to treatment for a specific amount of time	135	38.35	23	34.33	42	51.85	17	37.78	11	35.48	36	37.89	6	19.35	100	40.49	35	33.33	107	42.46	28	28.57
Advice of their specialist/as long as prescribed	127	36.08	26	38.81	27	33.33	13	28.89	18	58.06	30	31.58	13	41.94	93	37.65	34	32.38	89	35.32	37	37.76
As long as side effects are tolerable	86	24.43	20	29.85	22	27.16	17	37.78	9	29.03	10	10.53	8	25.81	57	23.08	29	27.62	62	24.60	24	24.49
Participant describes not giving up on any treatment	40	11.36	8	11.94	11	13.58	9	20.00	1	3.23	7	7.37	4	12.90	29	11.74	11	10.48	28	11.11	12	12.24
As long as treatment is working	25	7.10	6	8.96	4	4.94	5	11.11	4	12.90	4	4.21	2	6.45	15	6.07	10	9.52	16	6.35	9	9.18

Adherence to treatment	All participants		Aged under 18		Aged 18 to 44		Aged 45 to 64		Aged 65 plus		Trade or high school		University		Regional or remote		Metropolitan		Mid to low status		Higher status	
	n=352	%	n=352	%	n=81	%	n=45	%	n=32	%	n=95	%	n=32	%	n=247	%	n=105	%	n=252	%	n=98	%
Adhering to treatment for a specific amount of time	135	38.35	23	34.33	42	51.85	17	37.78	11	35.48	36	37.89	6	19.35	100	40.49	35	33.33	107	42.46	28	28.57
Advice of their specialist/as long as prescribed	127	36.08	26	38.81	27	33.33	13	28.89	18	58.06	30	31.58	13	41.94	93	37.65	34	32.38	89	35.32	37	37.76
As long as side effects are tolerable	86	24.43	20	29.85	22	27.16	17	37.78	9	29.03	10	10.53	8	25.81	57	23.08	29	27.62	62	24.60	24	24.49
Participant describes not giving up on any treatment	40	11.36	8	11.94	11	13.58	9	20.00	1	3.23	7	7.37	4	12.90	29	11.74	11	10.48	28	11.11	12	12.24
As long as treatment is working	25	7.10	6	8.96	4	4.94	5	11.11	4	12.90	4	4.21	2	6.45	15	6.07	10	9.52	16	6.35	9	9.18

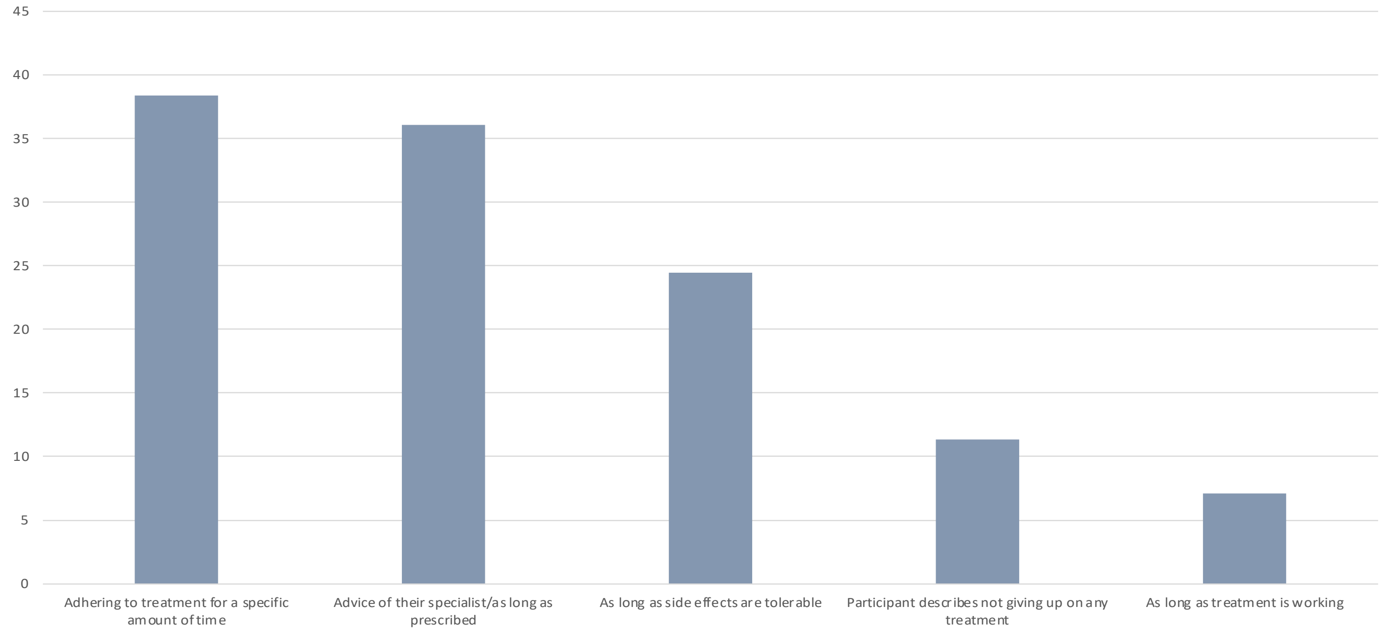

Figure 5.26: Adherence to treatment

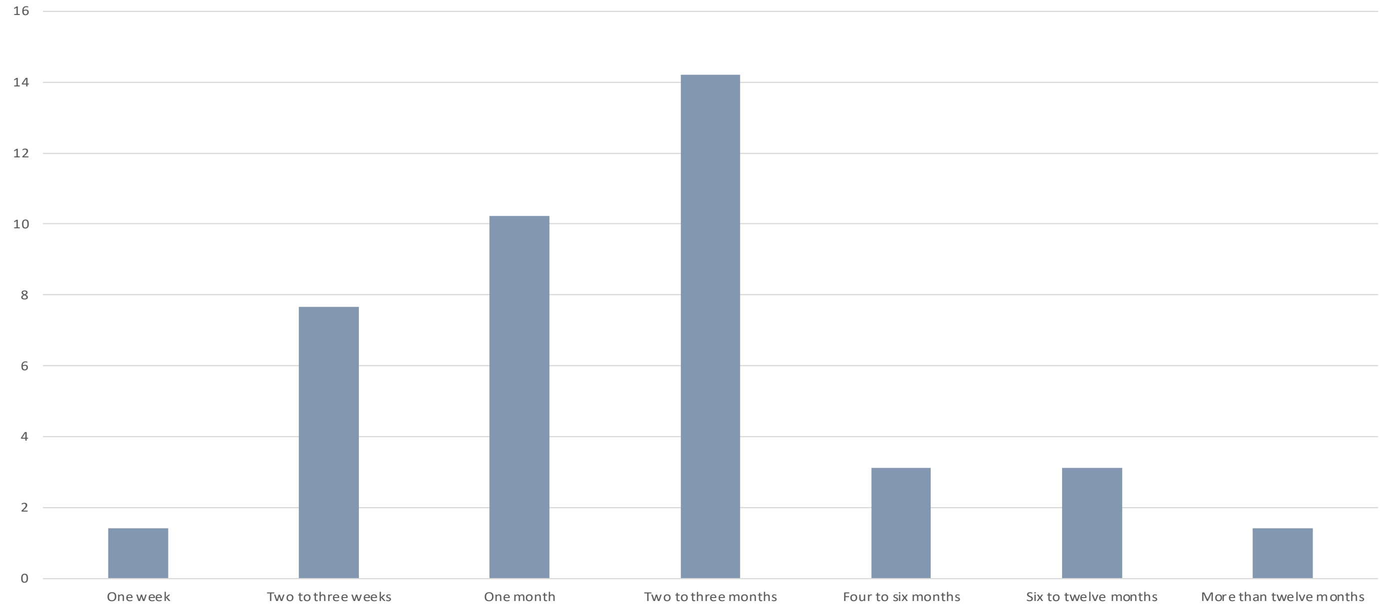

Figure 5.27: Adherence to treatment (Time to adhere to treatment)

Table 5.24: Adherence to treatment – subgroup variations

Adherence to treatment	Reported less frequently	Reported more frequently
Adhering to treatment for a specific amount of time	Other rare condition	Diseases of the immune system Regional or remote
Advice of their specialist/as long as prescribed		Diseases of the skin Regional or remote
As long as side effects are tolerable	Endocrine, nutritional or metabolic diseases Aged 65 plus	Diseases of the nervous system
Participant describes not giving up on any treatment		
As long as treatment is working		

What needs to change to feel like treatment is working

Participants were asked to describe what needs to change to feel like treatment is effective. The most common responses were needing to see a specific symptom reduction (26.70%), needing to see needing to see physical signs and symptoms disappear or reduce side effects (25.85%), a needing to see improvements in general wellbeing (quality of life) (14.49%), needing to see evidence of stable disease (14.20%), needing to see a return to day-to-day functionality (14.20%), and needing to see improvement in pain levels (12.50%).

When a specific side effect or symptom was described, the most common examples were emotional and mental impact (3.41%), and impact on sleep (2.84%).

Participants reported needing to see all physical signs and symptoms disappear

So I'm in the most recent medication. Obviously the Humira is probably the easiest one for me to talk about, and it has…there are a lot of side effects for Humira. I'm very fortunate that I am not one of the people that experiences any nasty side effects, but for me the improvement was that I wasn't getting 6 to 8 new lesions a day. Yeah, so now I might have three come up in a month. 4 come up in a month and generally speaking they're very mild. They come up and they go away as quickly as they come up. Occasionally they flare up, but generally that's, you know, it's it's once in a blue moon that it flares up the leg, the leg and the breast abscess that, as I said, there's been no change with those whatsoever. They still flare every week.
Participant 005_2023AUDSK

With it, yeah, one probably reduction in side effects or if they are quite severe, they've at least come down to a mild state. Like for example, we had hair loss when we first started that was pretty, pretty bad, but we just sort of waited it out and see if it got worse then we could always stop it, if not then but it went down to mild and also just obviously whatever you're treating. So like we're treating his seizures, obviously a reduction in seizures is always a good statistic and good see.
Participant 081_2023AUDIS

I guess it would just be that I can see some sort of visible positive difference, whether that's a reduction in negative symptoms, so whether something is actually, it was safe. It's his sleep apnoea, whether I can actually hear that it's getting better or hearing breathing better or whether it's something positive being added, like maybe he's starting to add a bit more communication in as a result of the speech pathology. So yeah, I think the main things would be that either a reduction in the negative symptoms or an addition of something that positive.
Participant 089_2023AUENM

I guess that you need to see that those symptoms are resolving, yeah, within the time frame mm.
Participant 038_2023AUDPA

Participant describes needing to see a reduction in a specific symptom

For me, for this condition, I would say pain relief is #1. And because it is kind of a visible condition, seeing like those boils on the skin, I'd say just a visible reduction in inflammation and redness, really. Yeah.
Participant 027_2023AUDSK

Yeah, definitely reduction in side effects. And for me it's things like it's easier to swallow, less pain, less rigidity, things sliding down a lot easier not getting that breathlessness feeling and that kind of heavy feeling that need to stretch. The specific symptoms. Yeah, just that those things ease and I can kind of eat or drink. Sometimes it's even drinking. Just like a normal person, I guess.
Participant 078_2023AUDIS

Going with this, yeah, the reduction will basically the reduction of the seizures. I think that's probably the in terms of the treatment that she has for that then, yeah.
Participant 016_2023AUORC

I understand about little people. You'd want to see progress against the goal that you had for the treatment. If you're taking an anti-constipation medication, you want to see that it's having the desired effect of relieving constipation. If you're doing speech therapy, you want to, over time, see some improvement in articulation or being able to apply the social and pragmatic communication skills in practice or whatever it might be.
Participant 067_2023AUDPA

Participants reported needing to experience evidence of stable disease/no disease progression

I think you just need to see some level of results and sometimes I think the blood test results are just a stronger indication of how I'm feeling day-to-day to attribute that. So I think the monitoring of the actual condition and knowing that, you know, when you do do my blood test, you are looking for viral load, you're looking for all the other impacts.
Participant 004_2023AUORC

Probably better monitoring instead of like here, take this medication and come back in 6 to 12 months. I should be seeing you to see how you're going and reassure you that yes, while you're not seeing signs, it is actually working. So better monitoring.
Participant 013_2023AUDSK

Yeah, you know. I can't really say that because a lot of these aren't standardized treatments, so you don't really have a particular measure. But if we were to say that, then we would say obviously blood test results. For example, if they're in hospital or they're having medication, we would be comparing blood work, which isn't my job, it's the doctor's job. If it's something like Allied health, then it would be about the improved level of functioning, which would be perceived by me. Or reported by the Allied health professional, because, again, these aren't standardized treatments. Really, I think that's it.
Participant 021_2023AUORC

Participants reported needing to experience an improvement in pain levels

Okay. As far as that's concerned, it's if I can feel there is a difference in whether the actual treatment is working. As in, if I have pain, if the pain is diminishing and I can feel that there's less pain because of the new treatment is working or something like that. As far as other medication is concerned, if I can feel that there's a marked improvement. Just to expand, because of some of the issues that my daughter's been having…When she was living at home, we changed from basically a normal diet to a gluten-free diet because she was put onto a strict diet and we worked too. We were cutting out gluten and et cetera. There was an improvement, the way she was feeling was improving in the way I was feeling. It's a good side effect in that I can feel something is changing to the better.
Participant 005_2023AUDPA

Probably in some situations less pain, and in other situations it's test results showing that things haven't progressed or things are getting better, so it's a big combination of things really, isn't it.
Participant 007_2023AUDIS

For me, it would be, I would need to have equal or better than relief from the symptoms. The fact that this particular Botox is less harmful is probably not useful for me if it's not providing me pain relief and relief from the spasming. It would have to do that. It would have to stop the pain and the spasming for me to consider it.
Participant 006_2023AUDNS

Participants reported needing to experience an improvement in general wellbeing (quality of life)

Positive outcomes and reaching milestones, I guess. Yeah. So positive improvement looks like, you know, our son's being happy, enjoying life, reengaging with community, doing hobbies and activities that he likes and finds meaningful and fulfilling. Yeah, and I guess maturing in a way that he's developing his self identity of who he is and developing as his own person.
Participant 031_2023AUDPA

Well, the aim is to have less spasms and better quality of life.
Participant 001_2023AUDNS

I guess just a sense of normality. Obviously, that's quite broad, but just a sense of normality, I guess, in the sense where it's helping whatever I'm trying to target to a point where I can get out in the community and actually exist rather than being stuck at home.
Participant 004_2023AUDNS

If it was a treatment for seizures, they would need to stop entirely. If it was a treatment for the spots on his face, they would have to go because he has these sensory problems and it would be more of a battle to get it on than it would be worth. Because his is only a mild case, it is hard to say. If it wasn't going to improve his life or if it wasn't going to make our lives easier to reduce stress or knowing that we weren't going to have to put up with many things and all that sort of stuff or because there's nothing that you could do about his sensory issues or autistic tendencies. It would have to make our lives easier for us to continue to take it. Not necessarily ours, but his life. It would have to mean that it was going to make his life better in the long run.
Participant 046_2023AUDPA

Participants reported needing to experience a return to day-to-day functionality

They don't feel like side effects, but anyway, they feel like just the effects. It's a hard thing to quantify because at the moment, everything that's involved in just having a day, like getting up, putting on clothes, washing myself is as much as I can handle in a day. I don't wash myself fully every day because if I do that, I can't do anything else. It's really hard. That's a really, really hard question because I don't know anything from being able to have a shower every day or have a shower whenever I felt like it without needing to ask someone for help. I would be able to clean my teeth every day without that having an impact on my fatigue and my arm. At the same time, if I had medication that was managing all of that, maybe I could do more than all of that. Maybe I could go out sometimes. It feels like an almost impossible question to answer.
Participant 001_2023AUDPA

Functions might be good. I just want to be able to be as normal as I can. For example, the pain medication, it's like, "Oh, I can get through a whole workday now," or I'd say muscle-wise like I'm able to garden once a week without pain or something like that. I have quite specific markers for my daily life. That's how I do it. I'm able to play with my children on the floor comfortably. My back must be good. My knees aren't popping out, great, that sort of thing. I tend to use the day-to-day functional things as my markers or able to walk to drop off rather than drive, those types of things. I'm doing well with it.
Participant 004_2023AUDPA

So one of the medications she's on is for anxiety, and that made a major difference. It allowed her to function. The other one she's on is for her thyroid. And again, you could see. I mean, you could see a quantitative difference there in her thyroid levels.
Participant 021_2023AUDPA

I'd like to be able to drink out of a cup in public and eat with a knife and fork. I can't. I cut all my food up with the pair of scissors, right? I eat it out of, I eat out of a bowl. That's alright. A lot of people lay out of a bowl, but I couldn't, I can't eat a steak, for instance. I mean, I probably couldn't cut it up with the..anyway. I don't.
Participant 003_2023AUDNS

Participants reported needing to experience a reduction in fatigue levels

If I'm taking a treatment that is making me feeling more fit, less tired. If I have more energy and if I feel less tense and stiff that's happening.
Participant 020_2023AUDIS

I guess I think like I know it's working if I feel well enough to do everyday life things. Like, I think I didn't realize quite how fatigued and sick I felt until I stopped feeling like that because, you know, it's been so such a long time.
Participant 024_2023AUDIS

For example, with the epilepsy meds, the drowsiness, I need to see that he can actually manage and get through the day without falling to sleep in class, and that's not going to impact on his learning.
Participant 063_2023AUDPA

Participants reported needing to experience improved mobility

To feel it has worked. Reduction of, reduction of symptoms, so reduction in in reports of pain, reduction in cramps. He's walking better.
Participant 026_2023AUORC

So a reduction in side effects go up with her medication, so the side effects went away. OK, well, that's working. Reports back from people she interacts with. So like schools saying yes, it's been a much improvement in her ability to engage in the classroom. Yeah. So positive, positive feedback or if she's being able to do things independently. So yeah, so seeing improvements in her in her life, like with the OT being able to all like physio with her now, being able to jump up and down or go up and down stairs properly or seeing that or people saying, oh, now she can do that or she's reading things, she's able to decode words or we see her working through her issues. So we're going, Oh yeah, that. So those things are working because we're seeing results.
Participant 017_2023AUDPA

My tolerance of exercise and my shortness of breath. So when I'm not well, it's a struggle to walk up one side of stairs. I'm acquiring anything, so at the moment, post COVID, I'm still struggling to bring a bag of groceries up my stairs. But when I'm well, I know I can walk up with two bags in each hand.
Participant 019_2023AUDPA

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

What needs to change to feel like treatment is working	All participants		Developmental anomalies		Diseases of the immune system		Diseases of the nervous system		Diseases of the skin		Endocrine, nutritional or metabolic diseases		Other rare condition		Person with condition		Family or carer		Female		Male	
	n=352	%	n=352	%	n=81	%	n=45	%	n=32	%	n=95	%	n=32	%	n=247	%	n=105	%	n=252	%	n=98	%
Specific symptom reduction	94	26.70	31	46.27	18	22.22	15	33.33	4	12.90	19	20.00	7	22.58	51	20.65	43	40.95	66	26.19	27	27.55
Physical signs and symptoms disappear/reduce side effects	91	25.85	13	19.40	13	16.05	18	40.00	25	80.65	6	6.32	16	51.61	63	25.51	28	26.67	67	26.59	23	23.47
Improvement in general wellbeing (quality of life)	51	14.49	12	17.91	6	7.41	5	11.11	2	6.45	25	26.32	1	3.23	35	14.17	16	15.24	34	13.49	17	17.35
Evidence of stable disease/no disease progression	50	14.20	0	0.00	19	23.46	14	31.11	1	3.23	12	12.63	4	12.90	42	17.00	8	7.62	37	14.68	13	13.27
Return to day-to-day functionality	50	14.20	11	16.42	12	14.81	2	4.44	2	6.45	22	23.16	1	3.23	38	15.38	12	11.43	39	15.48	11	11.22
Improvement in pain levels	44	12.50	4	5.97	22	27.16	10	22.22	0	0.00	8	8.42	0	0.00	39	15.79	5	4.76	39	15.48	5	5.10

What needs to change to feel like treatment is working	All participants		Aged under 18		Aged 18 to 44		Aged 45 to 64		Aged 65 plus		Trade or high school		University		Regional or remote		Metropolitan		Mid to low status		Higher status	
	n=352	%	n=69	%	n=116	%	n=108	%	n=59	%	n=172	%	n=172	%	n=100	%	n=252	%	n=176	%	n=176	%
Specific symptom reduction	94	26.70	30	43.48	23	19.83	26	24.07	15	25.42	45	26.16	48	27.91	28	35.00	66	26.19	48	27.27	46	26.14
Physical signs and symptoms disappear/reduce side effects	91	25.85	20	28.99	34	29.31	25	23.15	12	20.34	41	23.84	50	29.07	22	27.50	69	27.38	47	26.70	44	25.00
Improvement in general wellbeing (quality of life)	51	14.49	10	14.49	19	16.38	9	8.33	13	22.03	23	13.37	27	15.70	15	18.75	36	14.29	26	14.77	25	14.20
Evidence of stable disease/no disease progression	50	14.20	3	4.35	17	14.66	20	18.52	10	16.95	22	12.79	26	15.12	14	17.50	36	14.29	21	11.93	29	16.48
Return to day-to-day functionality	50	14.20	11	15.94	13	11.21	18	16.67	8	13.56	25	14.53	25	14.53	19	23.75	31	12.30	28	15.91	22	12.50
Improvement in pain levels	44	12.50	2	2.90	18	15.52	16	14.81	8	13.56	26	15.12	18	10.47	9	11.25	35	13.89	20	11.36	24	13.64

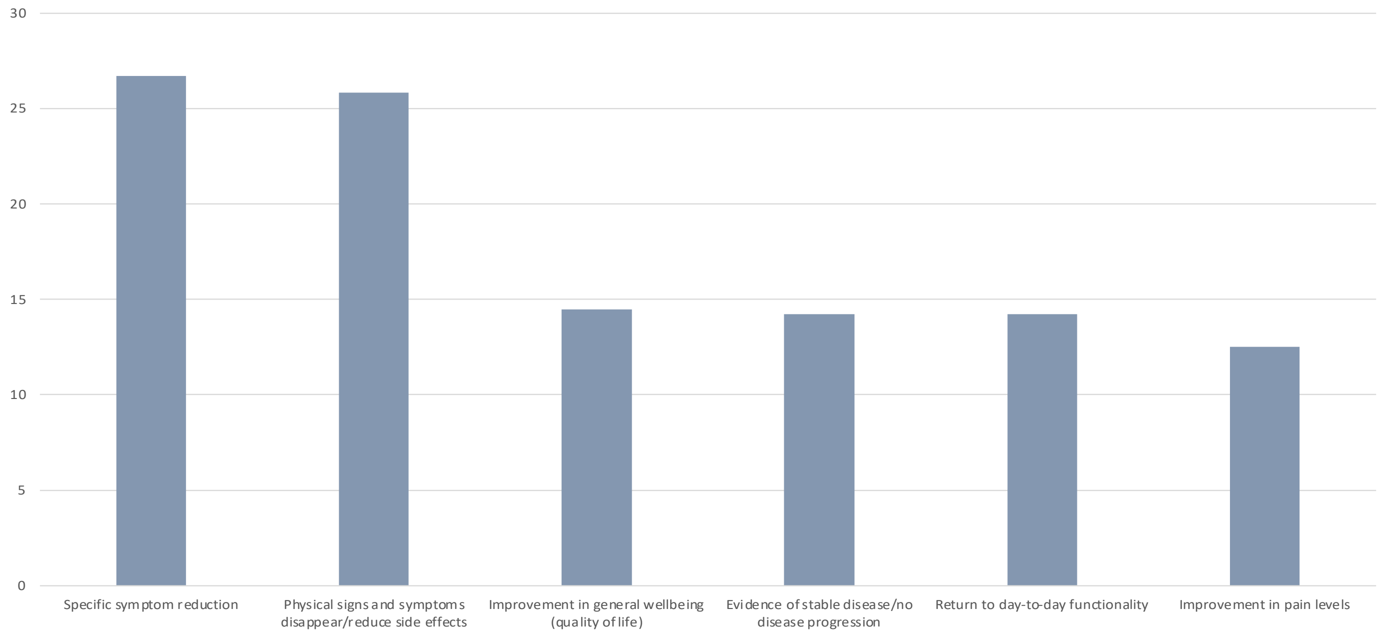

Figure 5.28: What needs to change to feel like treatment is working

Table 5.26: What needs to change to feel like treatment is working – subgroup variations

What needs to change to feel like treatment is working	Reported less frequently	Reported more frequently
Specific symptom reduction	Diseases of the skin	Developmental anomalies Family or carer Aged under 18
Physical signs and symptoms disappear/reduce side effects	Endocrine, nutritional or metabolic diseases	Diseases of the nervous system Diseases of the skin Other rare condition
Improvement in general wellbeing (quality of life)	Other rare condition	Endocrine, nutritional or metabolic diseases
Evidence of stable disease/no disease progression	Developmental anomalies Diseases of the skin	Diseases of the nervous system
Return to day-to-day functionality	Other rare condition	
Improvement in pain levels	Diseases of the skin Other rare condition	Diseases of the immune system

What it would mean if treatment worked

As a follow up question, participants were asked what it would mean to them if the treatment worked in the way they described. The most common responses were that it would allow them to do everyday activities/return to normal life (29.44%) and allow them to engage more with social activities and family life (11.67%). Other themes included allow them to return to work (9.44%), allow them to do more exercise (11.28%), will have a positive impact on their mental health (7.89%), allow them to do domestic tasks (6.77%), and lead to a reduction in symptoms/side effects (5.64%).

Allowing them to do everyday activities/return to normal life

Life. It would be life changing. I have to, I often say to my husband, I would just love to have one day of my life. Just one day. Like between now and death where I don't feel pain 24/7 so that would mean everything.
To be able to live without the disease, it would probably one of the biggest breakthroughs I think for me. Like if I have to live with this disease for the rest of my life and I'm assuming I do one of the biggest things that could change my life is GP's understanding what the disease is.
Participant 005_2023AUDSK

If there was something I could take easily that wasn't going to have any other effects on my health that were detrimental in any way, and it just helped with my HS, it would help my life a lot…like I would have the security to be able to go to the beach or go swimming or wear a dress because you have to wear underwear. Just, just normal things like that that I can't do and, you know, feel more confident myself if you were being intimate with somebody. I know mine isn't very bad because nobody in my life has ever noticed. So I guess that's, you know, even in a relationship for three years, no one, no one noticed. So I guess that's something. Other people have it worse than me, but I am aware of it and it's just a conversation you don't want to have to have with somebody. Because it's not like it's an STD, but you just can't help but you lean that way, can you really, if you were uninformed. So yeah, it's always in the back of my head. So yeah, something like that would just make my life a lot easier.
Participant 006_2023AUDSK

Well, I guess because like the, the dizziness and the soreness and things like that, that kind of went away on its own after a few weeks. I think it only stuck around for maybe three weeks, four weeks maximum, and then it kind of settled down a bit. That was not too bad. I feel like I'm kind of OK now. And yeah, the depression, I guess that would be good. I guess it would just help with everyday life, trying to get through work and everything like that. Yeah.
Participant 014_2023AUDSK

Allowing them to engage more with social activities and family life

Look, I cope all right with what I've got at the moment, except when it starts to get quite inflamed and sore and if it becomes embarrassing or I can't go out or something like that, that's difficult from a bit of the social bit as well. And if I can't wear certain clothes it's just…and if I leak on the bed at night, well that's a pain because you've got to go change the sheets and all that stuff. If I go to friends places to stay, I've got to make sure I don't destroy the sheets. If something happens and you just don't know when it's gonna go pop, so. Yeah, you gotta make sure you take enough things away with you and underpants and pads and all that and actually wear your underpants to bed in case you do. But also you gotta make sure you are also get enough air in your bottom area so it doesn't keep doing that. It's one of those hard things so.
Participant 024_2023AUDSK

Well, firstly, it reduces the anxiety around food. You can actually have a life you can like…At the moment, I can't go out for dinner, I can't eat outside of the house. I can, but it's very difficult. Like it takes away the social side of eating and doing those kinds of things. So I guess you can just live a more full, normal life.
Participant 078_2023AUDIS

I'm able to actually have a few hours a day where I can be involved in things. For the last two 2 1/2 years I haven't been able to do pretty much anything. You know, I've been pretty much housebound. You know, where now I can actually get out and go shopping with my wife. And, you know, I'm not worried about having to run to the toilet every 10 minutes and, you know, stuff like that. I just want to be able…I don't want to exist. I want to live.
Participant 014_2023AUORC

I could do a lot. I could go out. I could feel. I could feel happy. So I could, yeah, as I said, there's just days where you just go, no, that's OK. I'll just, yeah, stay

home. I've become a bit of a, I'll just go out to do the grocery shop. I'll go out to do the market. That's it. We might go back to the kids, but yeah, whereas I used to be out all the time. You know, go out some things. But, but yeah, it it changes. You just go now. Not going to risk that because, you know, the first thing I look for is where, where's the closest loo? As soon as I get to a shopping center, use the loo. Yeah. And you know, I can I can empty my gut up to 10 times a day. Yeah. And to me that's not good. That's not healthy.
Participant 019_2023AUDIS

Allowing them to return to work

I could my enjoy my passion and what I've worked at for most of my working life. I'm a carpenter, enjoy my life, but I do like doing joining work. I have a workshop down the church. I can spend more time doing work that I enjoy and making things that are fun. At the moment because I had an accident and fell off of a ladder and broke my heel and aggravated some previous injuries. At the moment, I'm not enjoying any of it. Because as in the pain medications not doing what it's supposed to either. I'm trying to enjoy my enjoy life anyway
Participant 005_2023AUDPA

It would mean so much better. Like I I could go out and get an actual job which I can lift up my arms to reach something.
Participant 004_2023AUDSK

Probably a lot and I'd probably go back to work. [chuckles] I probably wouldn't go back to work because of lung disease now, it's a bit hard to cope. I suppose it's just, just everyday things like I've made adjustments in my own life to cope with it but I suppose just being able to put my hair up by myself, being able to wash my hair by myself. It's just those sort of things, I suppose. I still manage to do my makeup all by myself. I don't wear it often but when I do have to wear it, I can still manage to do it. [chuckles] My sister is always surprised, she's like, 'Who did your makeup? It looks fantastic." I'm like, "I did it myself."
Participant 018_2023AUDIS

It would be amazing. I'd be able to drive all the time, not take time off work. That's a big one. I only started a new job in Feb and I'm already negative like 80 hours sick leave because I've just been in hospital so much. I guess they're the main two for me not having to miss work and being able to drive all the time.
Participant 096_2023AUDNS

Allowing them to do more exercise

Walk further. Being able to breathe while I'm walking and up and down the stairs, in hills is another thing. When I started taking it, I was able to walk further.
Participant 004_2023AUDIS

Walk. I still love walking, you know, I can still walk, but I can't walk much, you know, sort of just getting up, gets out of breath and I have to stop, have a rest and then carry on. Yes, yes. Where I used to be so energetic, really, you know, and I can find my. I strengthen my hands. I've really decreased.
Participant 005_2023AUDIS

Oh, wow. That would be awesome. I'd be able to cook again, I'd be able to just clean and keep the house tidy. Sounds ridiculous, but you see things, and it's frustrating, you can't do anything about it. I would be able to do more exercise. I used to walk the dog every morning, probably for 45 minutes in a brisk walk. Now, I'd say it's more of a stroll than a walk, and I'm out for about 20 minutes. I'd like to be able to do more exercise and swim. I've lost…I can't move my shoulders or arms. I used to enjoy swimming, I go in the water, although [crosstalk] about the chlorine on my skin, so I can't go into those indoor pools. I can't actually swim breaststroke or freestyle or anything, but if [crosstalk] the mobility back, that would definitely be something I'd do.
Participant 017_2023AUDIS

That would mean a lot. If I feel less stiff I can do more stretching, more exercises and if I do more exercises, it'll help me to feel again less stiff than before and I can do more activity, more house chores and be more dependent.
Participant 020_2023AUDIS

A positive impact on their mental health

I would…I'd be working. I would be able to afford dental care. I could improve my mental health, my mental outlook. I could reconnect with my family. I could basically, in inverted commas, get a life. Yeah, I'm just hiding away from the world at the moment. That's what my life is.
Participant 008_2023AUDSK

I think it's, it's high impact because on a really bad day, I can't get off the couch, which means I can't feed my animals. I can't, you know, clean the house. I can't. Even focus on studying or working at that given time. So the less flare ups, the less energy my body has to fight the infections. I would feel much better, think

much clearer and, you know, be able to actually enjoy life because currently it's 5050, yeah.
Participant 026_2023AUDSK

Well, if this, if it was something like that, it would mean that he would be able to better express his needs and have his have his needs met better, probably reduce overall stress for him and everybody, and reduce his frustration. Yeah, it it would just mean that he would be able to function better and achieve more of his desires and more of what he wants, yeah.
Participant 089_2023AUENM

I guess it would just be, I mean it's psychologically for us it's just difficult seeing your child in pain and struggling because of medication that they're on to try and treat the condition that they there's no cure for. So if we're able to. If there was some other way around it or something that we could have to get rid of those unwanted side effects, then for us it would be just a major relief. You know, no parents would see their kid in pain or anything. So yeah, it's just be. It wouldn't change, I guess it wouldn't change what he can and can't do because he can't do much at the moment anyway. But it would just be a relief for us to see him more settled and happy.
Participant 029_2023AUORC

Allowing them to do domestic tasks

Just everything. If my pain's flared up, or my backs out or something like that, then I can't even unload the dishwasher, or I can't comfortably get my kids ready for bed, can't bath them. There's so many slow on effects from that type of thing. Often we push through pain so often that you do get used to it. I do notice that it will affect my mood, I'll be more irritable or more tired or I won't want to go out. I'm not going to book-- seeing my friends in my spare time. I'll just be resting. Again, that functional impact is the big one. It's day-to-day things.
Participant 004_2023AUDPA

I'd be able to go for walks again and be able to actually play with my kids properly clean the house. Stack the dishwasher. I can't even stack the bloody dishwasher at the moment. Be able to go horse riding again and and do the activities and and go back to competition. So like, I haven't been able to do that since I had the really bad flair in 2019. That's just gone from spot to spot to spot.
Participant 012_2023AUDSK

I can, you know, go back to doing, you know, cooking and baking. I can, you know, better help my parents with, you know, various gardening activities and things like that. I stopped. I stopped some of the voluntary work that I was doing before, and, you know, being able to pick that up again would be lovely. Having said that, I've found other ways to volunteer, obviously, so.
Participant 019_2023AUORC

About 100 things like work, have my 16 year old son live with me because I could care for him. I could do all his washing, you know, domestic chores and cooking. It would mean I could go back to doing all the sport I would normally do. It would mean I could see all my friends, so I could be social because I would have enough energy to do that. It would mean I could drive longer distances, which also would mean I could be more social because I could, you know, drive to see friends. That would also mean I have greater independence. Therefore I would have greater I would have income because at the moment I'm on a pension because I can't work. So it would change my life enormously. To increase the quality of life enormously.
Participant 010_2023AUDIS

Leading to a reduction in symptoms/side effects

More energy and you know, a knowledge that you know the medication has worked and that, what would you…it's going to give me more energy. I won't have brain fog, I won't have aching limbs. I won't have night sweats. I'm, you know, I'm going to be a much more productive member of society and to my family.
Participant 010_2023AUORC

It would mean a lot. It's, it's something that I deal with daily and kind of dealing with the pain. So it would allow me to just sort of not have to think about it. It would be great, yeah.
Participant 027_2023AUDSK

Look. If the treatment wasn't doing its job, you know all three in my life, I went to work pretty well, but in pain. So I did. I went to work, I went shopping…I, you know, I did a normal life, but I had a pain level that upset me. But it was also the social thing as well, so I sometimes, if I was looking bad, I wouldn't go out. So that that that wasn't because I couldn't go out. It was just because I made the choice that I didn't want people to see me. Yeah, and there was sometimes I didn't accept social events because I wasn't looking good, but I could have gone. It's just that I made the the choice but it was more more about pain for me like.
Participant 029_2023AUDSK

I'll be able to shower easy. The shortness of breath is the most annoying thing ever because I have to constantly stop. I got to remind myself to stop, to breathe. People look at you and think, "oh God, she's unfit." Well, I'm not. It's just I can't breathe, like my lungs are destroyed.

Participant 023_2023AUDIS

Table 5.27: What it would mean if treatment worked

What it would mean if treatment worked	All participants		Developmental anomalies		Diseases of the immune system		Diseases of the nervous system		Diseases of the skin		Endocrine, nutritional or metabolic diseases		Other rare condition		Person with condition		Family or carer		Female		Male	
	n=180	%	n=67	%	n=31	%	n=9	%	n=32	%	n=9	%	n=32	%	n=100	%	n=80	%	n=121	%	n=57	%
Allowing them to do everyday activities/return to normal life	53	29.44	3	4.48	17	54.84	1	11.11	15	46.88	4	44.44	13	40.63	42	42.00	11	13.75	42	34.71	10	17.54
Allowing them to engage more with social activities and family life	21	11.67	2	2.99	7	22.58	0	0.00	8	25.00	0	0.00	4	12.50	19	19.00	2	2.50	18	14.88	2	3.51
Allowing them to return to work	17	9.44	1	1.49	4	12.90	0	0.00	6	18.75	1	11.11	5	15.63	15	15.00	2	2.50	10	8.26	7	12.28
Allowing them to do more exercise	16	8.89	0	0.00	8	25.81	0	0.00	2	6.25	1	11.11	5	15.63	10	10.00	6	7.50	13	10.74	3	5.26
A positive impact on their mental health	12	6.67	1	1.49	4	12.90	1	11.11	5	15.63	0	0.00	1	3.13	12	12.00	0	0.00	9	7.44	3	5.26

What it would mean if treatment worked	All participants		Aged under 18		Aged 18 to 44		Aged 45 to 64		Aged 65 plus		Trade or high school		University		Regional or remote		Metropolitan		Mid to low status		Higher status	
	n=180	%	n=61	%	n=63	%	n=37	%	n=19	%	n=88	%	n=92	%	n=48	%	n=132	%	n=94	%	n=86	%
Allowing them to do everyday activities/return to normal life	53	29.44	10	16.39	18	28.57	15	40.54	10	52.63	24	27.27	29	31.52	14	29.17	39	29.55	28	29.79	25	29.07
Allowing them to engage more with social activities and family life	21	11.67	2	3.28	6	9.52	9	24.32	4	21.05	8	9.09	13	14.13	7	14.58	14	10.61	14	14.89	7	8.14
Allowing them to return to work	17	9.44	2	3.28	7	11.11	6	16.22	2	10.53	7	7.95	10	10.87	1	2.08	16	12.12	6	6.38	11	12.79
Allowing them to do more exercise	16	8.89	5	8.20	4	6.35	3	8.11	4	21.05	6	6.82	10	10.87	2	4.17	14	10.61	6	6.38	10	11.63
A positive impact on their mental health	12	6.67	0	0.00	3	4.76	8	21.62	1	5.26	6	6.82	6	6.52	0	0.00	12	9.09	7	7.45	5	5.81

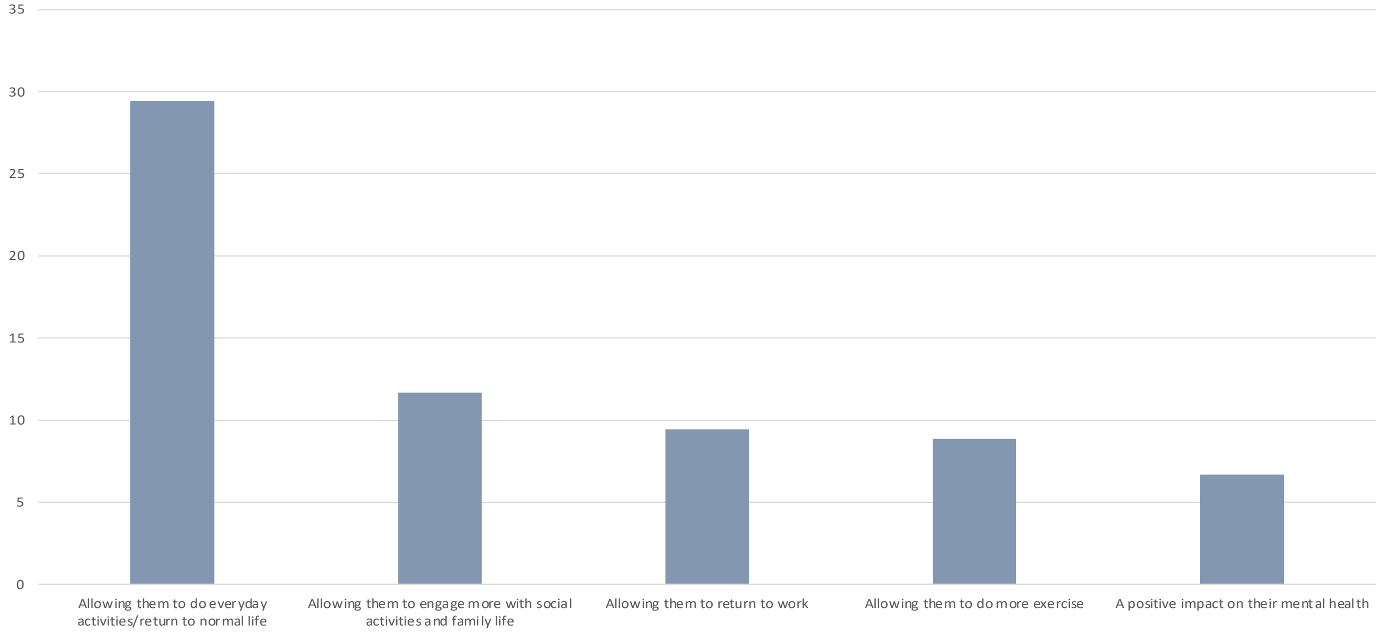

Figure 5.29: What it would mean if treatment worked

Table 5.28: What it would mean if treatment worked – subgroup variations

What it would mean if treatment worked	Reported less frequently	Reported more frequently
Allowing them to do everyday activities/return to normal life	Developmental anomalies Diseases of the nervous system Family or carer Male Aged under 18	Diseases of the immune system Diseases of the skin Endocrine, nutritional or metabolic diseases Other rare condition Person with condition Aged 45 to 64 Aged 65 plus
Allowing them to engage more with social activities and family life	Diseases of the nervous system Endocrine, nutritional or metabolic diseases	Diseases of the immune system Diseases of the skin Aged 45 to 64
Allowing them to return to work		Aged 45 to 64
Allowing them to do more exercise		Diseases of the immune system Aged 65 plus
A positive impact on their mental health		