



Clinical Trials Survey

About the Study

These are findings from an Ipsos survey of 551 ALS patients on behalf of I AM ALS, carried out online between February 6-12, 2019. The survey was designed in consultation with a wide range of ALS stakeholders, including ALS patients; caregivers to ALS patients; top ALS researchers and physicians; pharmaceutical companies working on ALS treatments; primary investigators and those running trials on ALS treatments; patients of other neuro-degenerative conditions; leadership at I AM ALS, ALS-TDI, and ALSA; and others. The survey was distributed via the I AM ALS and ALS-TDI mailing lists of ALS patients, and amplified to additional ALS patients via email and social media, yielding a “snowball” sampling approach that is necessary given the extremely low population incidence of this patient group. The data are not weighted but have been cleaned for quality purposes.

1. Are you yourself living with any of the following diseases? If you have more than one of the diseases listed below, please choose the disease with which you were first diagnosed.

	Total
	N = 551
ALS	100%

2. To the best of your knowledge, have you participated in this same survey before?

	Total
	N = 551
No	100%

3. What is your age?

	Total
	N = 551
18-34	5%
35-44	11%
45-54	26%
55-64	31%
65+	26%
Mean	56.1



4. What was your age when the first symptoms of ALS appeared?

	Total
	N = 551
<18	1%
18-34	9%
35-44	18%
45-54	30%
55-64	28%
Mean	51.3

5. What was your age when you were officially diagnosed?

	Total
	N = 551
18-34	8%
35-44	15%
45-54	30%
55-64	30%
65+	17%
Mean	53

6. In your own words, what do you think is the main purpose of clinical trials?

SEE EXCEL FILE OF OPEN-ENDED RESPONSES

7. How much, if anything, do you know about clinical trials?

	Total
	N = 551
Know a great deal	20%
Know a fair amount	44%
Know a little	32%
Know nothing at all	4%
Top 2 (Net)	64%
Bottom 2 (Net)	36%



8. And how well, if at all, would you say you understand the differences between Phases of clinical trials (e.g. Phase 1, Phase 2, and Phase 3 trials)?

	Total
	N = 551
Understand very well	24%
Understand fairly well	36%
Understand a little	30%
Do not understand at all	9%
Top 2 (Net)	60%
Bottom 2 (Net)	40%

9. Have you participated in any clinical trials related to your condition?

	Total
	N = 551
Yes (Any)	41%
Yes - one involving a drug or treatment	28%
Yes - one involving imaging or biomarker identification to learn more	21%
No	59%

10. What type(s) of clinical trials have you participated in? Choose all that apply

	Total
Base: participated in clinical trials	N = 227
Phase 1	18%
Phase 2	37%
Phase 3	24%
Other	23%
Don't know	18%



11. Why haven't you participated in any clinical trials?

	Total
Base: has not participated in clinical trials	N = 324
I don't meet the criteria for participating in trials	43%
I don't want to risk receiving a placebo drug	25%
I have been excluded from participating in trials	19%
I can't afford to travel to them	19%
I am not aware of any trials	17%
I can't travel to them because of my disease progression	14%
I don't know how to find information about trials	13%
It is too risky - worried it will worsen my condition	9%
I just do not want to	2%
I can get the drugs I need without participating in trials	2%
Other	28%
Don't know	3%

12. Why don't you qualify / think you don't qualify?

	Total
Base: don't qualify or don't think they qualify for trials	N = 154
Has been 24 months since symptoms started	61%
Respiratory function too low	25%
Taking another medication that I won't stop using	12%
Other	40%
Don't know	1%



13. Where do you find information about participating in clinical trials?

	Total
	N = 551
From organizations / associations for my disease	54%
My doctor or medical team(s)	53%
www.ClinicalTrials.gov or other government websites	50%
Web searches	42%
Social media (Facebook, Twitter, Instagram, etc)	26%
Other patients I know	20%
From friends or family	15%
I don't look for or find this information at all	3%
Other	5%
Don't know	3%

ASK ALS PATIENTS WHO GET CLINICAL TRIAL PARTICIPATION INFO FROM ORGS/ASSNS FOR ALS (if respondent selected code 5 in Q13 "From organizations / associations for my disease " and if Q1 for ALS = yes)

14. Which, if any, ALS organizations do you use to find information on clinical trials?

SEE EXCEL FILE OF OPEN-ENDED RESPONSES

15. How far, if at all, are you willing to travel by car / van / from your home in order to participate in a trial, assuming you qualify?

	Total
	N = 551
I am not willing to travel by car / van / SUV	3%
Less than 30 minutes	4%
Half hour to an hour	13%
1-2 hours	26%
3-5 hours	20%
6 hours or more	28%
Don't know	6%



16. How far, if at all, are you willing to travel by air from your home in order to participate in a trial, assuming you qualify?

	Total
	N = 551
I am not willing to travel by air	23%
Short flight only in the United states (1-2 hours)	12%
Anywhere at all in the United States	24%
Anywhere in the world	28%
Don't know	13%

17. Which, if any, of the following have you discussed with your doctor?

	Total
Base: have discussed with doctor	N = 290
Clinical trials taking place at my doctor's hospital or practice	80%
Clinical trials taking place at other hospitals in the United States	54%
Clinical trials taking place at other hospitals outside the United States	15%
Don't know / Don't remember	6%

18. Did the discussion(s) about trials with your doctor make you more or less likely to consider participating in a clinical trial or trials?

	Total
Base: have discussed with doctor	N = 290
Made me more likely to consider trial participation	57%
Did not impact my likelihood of participating in trials	33%
Made me less likely to consider trial participation	8%
Don't know	2%



19. For each idea below, please indicate whether it would make you MORE or LESS likely to participate in each trial phase.

- a. If there was a lower likelihood that I would be assigned to a placebo, which would increase the odds that I would get the trial drug

	Total		
	N = 551		
	Phase 1	Phase 2	Phase 3
More likely	59%	70%	74%
Not impact	24%	18%	15%
Less likely	10%	6%	5%
Don't know	7%	6%	5%

- b. Phase 1: If placebo groups were removed so that all participants received the trial drug

	Total		
	N = 551		
	Phase 1	Phase 2	Phase 3
More likely	71%	79%	83%
Not impact	17%	12%	9%
Less likely	6%	5%	4%
Don't know	6%	5%	4%

- c. If I knew that I was more likely to receive a placebo than the drug, but that the trial design made the trial more likely to provide definitive, meaningful data

	Total		
	N = 551		
	Phase 1	Phase 2	Phase 3
More likely	13%	14%	17%
Not impact	29%	28%	26%
Less likely	47%	47%	47%
Don't know	11%	11%	10%

- d. If I would have guaranteed access to the drug (if I wanted it) after the trial was over (expanded or post-trial access)

	Total		
	N = 551		
	Phase 1	Phase 2	Phase 3
More likely	76%	85%	91%
Not impact	15%	9%	5%
Less likely	4%	1%	1%
Don't know	5%	5%	3%



- e. If I knew that I would not receive any drug during or after the trial, but that the trial would help confirm or disprove a potential biomarker for treatment of all ALS patients

	Total		
	N = 551		
	Phase 1	Phase 2	Phase 3
More likely	26%	28%	31%
Not impact	38%	39%	38%
Less likely	25%	21%	21%
Don't know	12%	12%	11%

- f. If the trial was testing multiple drugs at once with different patient groups, producing more data more quickly

	Total		
	N = 551		
	Phase 1	Phase 2	Phase 3
More likely	49%	55%	60%
Not impact	33%	31%	28%
Less likely	9%	5%	4%
Don't know	9%	9%	8%

- g. If the trial did not require me to stop taking other medications or supplements that I am on

	Total		
	N = 551		
	Phase 1	Phase 2	Phase 3
More likely	56%	62%	67%
Not impact	34%	30%	27%
Less likely	5%	3%	2%
Don't know	5%	5%	3%

- h. If the trial required me to stop taking supplements not approved by the FDA to treat ALS

	Total		
	N = 551		
	Phase 1	Phase 2	Phase 3
More likely	5%	7%	10%
Not impact	53%	54%	54%
Less likely	34%	32%	28%
Don't know	8%	8%	7%



i. If your travel expenses, including caregiver travel, were reimbursed by the drug manufacturer

	Total		
	N = 551		
	Phase 1	Phase 2	Phase 3
More likely	74%	79%	83%
Not impact	19%	16%	14%
Less likely	5%	2%	1%
Don't know	3%	3%	2%

j. If a company or organization helped to provide loaner equipment during travel for the trial (e.g. hooyer lifts and wheelchairs)

	Total		
	N = 551		
	Phase 1	Phase 2	Phase 3
More likely	53%	58%	61%
Not impact	36%	34%	33%
Less likely	5%	2%	1%
Don't know	7%	6%	5%

k. If you knew much sooner after the trial whether you had received an active drug or a placebo (faster “unblinding”)

	Total		
	N = 551		
	Phase 1	Phase 2	Phase 3
More likely	45%	51%	56%
Not impact	38%	36%	32%
Less likely	7%	4%	3%
Don't know	10%	10%	9%

l. If the length of trials was six months or less?

	Total		
	N = 551		
	Phase 1	Phase 2	Phase 3
More likely	56%	59%	63%
Not impact	36%	35%	33%
Less likely	4%	2%	1%
Don't know	4%	4%	3%



m. If the length of trials was one year or less?

	Total		
	N = 551		
	Phase 1	Phase 2	Phase 3
More likely	28%	31%	33%
Not impact	45%	44%	44%
Less likely	18%	15%	14%
Don't know	9%	10%	9%

n. If I had a patient navigator or advocate at my primary hospital who could help me with the logistics of participating in trials

	Total		
	N = 551		
	Phase 1	Phase 2	Phase 3
More likely	68%	72%	75%
Not impact	24%	22%	21%
Less likely	2%	1%	1%
Don't know	5%	5%	4%

o. If ALSFRS and follow up questionnaires were completed via phone or online (rather than in person)

	Total		
	N = 551		
	Phase 1	Phase 2	Phase 3
More likely	59%	64%	66%
Not impact	31%	29%	28%
Less likely	4%	2%	1%
Don't know	6%	5%	4%

p. If you could have a blood test or imaging scan to assess progression rather than using ALSFRS

	Total		
	N = 551		
	Phase 1	Phase 2	Phase 3
More likely	49%	53%	55%
Not impact	37%	34%	33%
Less likely	3%	2%	2%
Don't know	11%	11%	10%



q. If you could have a wearable device to assess progression rather than using ALSFRS

	Total		
	N = 551		
	Phase 1	Phase 2	Phase 3
More likely	54%	57%	60%
Not impact	30%	28%	26%
Less likely	5%	3%	3%
Don't know	12%	12%	12%

20. Please indicate which, if any, of the following you would be willing to do as part of a clinical trial?

“Definitely Willing” Summary Table	Total
	N = 551
Have a study team visit you at your home to make assessments	83%
Televisits'(via Skype or FaceTime) to supplement in-person visits	82%
Go to a local hospital or clinic for blood draws	82%
Submit voice recordings to be analyzed for speech progression tracking	80%
Record your own data at home to report back to the trial	79%
Wear a 'wearable' device during a trial that remotely monitors your movement and other clinically relevant data	75%
Visit a trial site for a period of time before the trial started to provide advance data on your progression	55%

a. Wear a “wearable” device during a trial that remotely monitors your movement and other clinically relevant data

	Total
	N = 551
Definitely willing	75%
Maybe willing	20%
Definitely Not willing	2%
Don't know	3%

b. Submit voice recordings to be analyzed for speech progression tracking

	Total
	N = 551
Definitely willing	80%
Maybe willing	13%
Definitely Not willing	3%
Don't know	4%



- c. "Televisits" (via Skype or FaceTime) to supplement in-person visits

	Total
	N = 551
Definitely willing	82%
Maybe willing	12%
Definitely Not willing	3%
Don't know	3%

- d. Visit a trial site for a period of time before the trial started to provide advance data on your progression

	Total
	N = 551
Definitely willing	55%
Maybe willing	33%
Definitely Not willing	5%
Don't know	6%

- e. Record your own data at home to report back to the trial

	Total
	N = 551
Definitely willing	79%
Maybe willing	17%
Definitely Not willing	1%
Don't know	3%

- f. Have a study team visit you at your home to make assessments

	Total
	N = 551
Definitely willing	83%
Maybe willing	13%
Definitely Not willing	1%
Don't know	3%

- g. Go to a local hospital or clinic for blood draws

	Total
	N = 551
Definitely willing	82%
Maybe willing	15%
Definitely Not willing	1%
Don't know	2%



21. Please indicate the extent to which you agree or disagree with each statement below.

Top 2 (Agree) Summary Table	Total
	N = 551
I would be willing to submit blood, saliva, skin, or other samples for genetic testing	94%
I wish there were more trials I could participate in	86%
Because this disease is fatal, I am more willing to take risks during a clinical trial than a non-terminal patient	78%
Because this disease is fatal, I am more willing to participate in trials to help find a cure for ALS, even though I myself will not benefit from them	78%
I would be more likely to consider clinical trial participation if had 'patient navigator'/advocate at primary hospital to help with participating in trials	72%
I wish I had someone to help me understand trials and how to participate	55%
I find making the decision about participating in a clinical trial to be easy	54%
I don't think that the ALSFRS scoring is a good way to assess ALS progression.	45%
I know people with this disease who can't afford to participate in clinical trials, even though they wish they could	39%
Clinical trial participation scares me	25%

a. I find making the decision about participating in a clinical trial to be easy

	Total
	N = 551
Strongly Agree	26%
Somewhat Agree	28%
Neither Agree Nor Disagree	17%
Somewhat Disagree	18%
Strongly Disagree	10%
Don't know	2%
Top 2 (Net)	54%
Bottom 2 (Net)	28%

b. I wish there were more trials I could participate in

	Total
	N = 551
Strongly Agree	67%
Somewhat Agree	19%
Neither Agree Nor Disagree	8%
Somewhat Disagree	2%
Strongly Disagree	1%
Don't know	3%
Top 2 (Net)	86%
Bottom 2 (Net)	3%



- c. I would be willing to submit blood, saliva, skin, or other samples for genetic testing (with information on the results provided to you on request)

	Total
	N = 551
Strongly Agree	79%
Somewhat Agree	15%
Neither Agree Nor Disagree	3%
Somewhat Disagree	0%
Strongly Disagree	1%
Don't know	2%
Top 2 (Net)	94%
Bottom 2 (Net)	1%

- d. Clinical trial participation scares me

	Total
	N = 551
Strongly Agree	4%
Somewhat Agree	21%
Neither Agree Nor Disagree	19%
Somewhat Disagree	15%
Strongly Disagree	38%
Don't know	3%
Top 2 (Net)	25%
Bottom 2 (Net)	53%

- e. I wish I had someone to help me understand trials and how to participate

	Total
	N = 551
Strongly Agree	26%
Somewhat Agree	29%
Neither Agree Nor Disagree	27%
Somewhat Disagree	8%
Strongly Disagree	7%
Don't know	2%
Top 2 (Net)	55%
Bottom 2 (Net)	16%



f. I know people with this disease who can't afford to participate in clinical trials, even though they wish they could

	Total
	N = 551
Strongly Agree	28%
Somewhat Agree	11%
Neither Agree Nor Disagree	21%
Somewhat Disagree	4%
Strongly Disagree	8%
Don't know	28%
Top 2 (Net)	39%
Bottom 2 (Net)	12%

g. ASK FOR ALS ONLY: Because this disease is fatal, I am more willing to take risks during a clinical trial than a non-terminal patient

	Total
	N = 551
Strongly Agree	51%
Somewhat Agree	27%
Neither Agree Nor Disagree	10%
Somewhat Disagree	6%
Strongly Disagree	4%
Don't know	2%
Top 2 (Net)	78%
Bottom 2 (Net)	10%

h. ASK FOR ALS ONLY: Because this disease is fatal, I am more willing to participate in trials to help find a cure for ALS, even though I myself will not benefit from them

	Total
	N = 551
Strongly Agree	48%
Somewhat Agree	31%
Neither Agree Nor Disagree	10%
Somewhat Disagree	6%
Strongly Disagree	3%
Don't know	3%
Top 2 (Net)	78%
Bottom 2 (Net)	9%



i. ASK FOR ALS ONLY: I don't think that the ALSFRS scoring is a good way to assess ALS progression.

	Total
	N = 551
Strongly Agree	15%
Somewhat Agree	31%
Neither Agree Nor Disagree	26%
Somewhat Disagree	7%
Strongly Disagree	4%
Don't know	18%
Top 2 (Net)	45%
Bottom 2 (Net)	11%

j. I would be more likely to consider clinical trial participation if I had a "patient navigator" or advocate at my primary hospital who could help me with the logistics of participating in trials

	Total
	N = 551
Strongly Agree	38%
Somewhat Agree	34%
Neither Agree Nor Disagree	20%
Somewhat Disagree	3%
Strongly Disagree	3%
Don't know	3%
Top 2 (Net)	72%
Bottom 2 (Net)	6%

22. Approximately how far away from your home is ...?

a. The nearest hospital, clinic, or facility for your regular, ongoing care and management

	Total
	N = 551
A car ride	96%
A train or bus ride	1%
A plane ride	1%
Don't know	2%



b. Your main doctor for care and management

	Total
	N = 551
A car ride	95%
A train or bus ride	2%
A plane ride	2%
Don't know	2%

c. The nearest hospital or facility that does clinical research trials?

	Total
	N = 551
A car ride	70%
A train or bus ride	2%
A plane ride	7%
Don't know	20%

23. If you know it, please enter your ALSFRS or ALSFRS-R below: [NUMERIC RESPONSE CODED]

a. ALSFRS

	Total
	N = 551
Blank	87%
1-20	1%
21-30	4%
31-40	8%

b. ALSFRS-R

	Total
	N = 551
Blank	81%
1-20	3%
21-30	4%
31-47	13%



24. If you know it, please enter your FVC below:

	Total
	N = 551
0	66%
1-50	11%
51-100	21%
101-136	3%

25. And are you mostly walking or mostly using a wheelchair to get around?

	Total
	N = 551
Mostly walking	50%
Mostly wheelchair	48%
Prefer not to answer	1%