

S2A Table. Symptoms Experienced by All patients, Adults and Children.

All (N=290)¹		Adults (N=169)¹		Children (N=121)¹	
Symptom	% (n)	Symptom	% (n)	Symptom	% (n)
Muscle weakness	95.4 (270/283)	Chronic fatigue	94.6 (156/165)	Muscle weakness	98.3 (116/118)
Chronic fatigue	95.1 (269/283)	Muscle weakness	93.3 (154/165)	Exercise intolerance	97.5 (115/118)
Exercise intolerance	94.7 (268/283)	Exercise intolerance	92.7 (153/165)	Chronic fatigue	95.8 (113/118)
GI Problems	79.6 (223/280)	Balance problems	79.6 (129/162)	Gastrointestinal problems	87.2 (102/117)
Balance Problems	78.8 (219/278)	Gastrointestinal problems	74.2 (121/163)	Delayed milestones	86.3 (101/117)
Sleep Problems	68.7 (191/278)	Sleep problems	72.0 (116/161)	Speech problems	77.8 (91/117)
Decreased Vision	64.7 (180/278)	Decreased vision	72.0 (116/161)	Balance problems	77.6 (90/116)
Headache	63.3 (176/278)	Headache	65.4 (106/162)	Learning disability	73.9 (85/115)
Dehydration	56.7 (157/277)	Peripheral neuropathy	58.9 (93/158)	Dehydration	68.7 (79/115)
Peripheral Neuropathy	55.9 (151/270)	Eye muscle problems	57.9 (92/159)	Intellectual disability	65.5 (76/115)
Eye Muscle Problems	55.3 (151/273)	Ptosis	56.6 (90/159)	Sleep problems	64.1 (75/117)
Delayed milestones	55.2 (149/270)	Mood disorder	50.9 (83/163)	Headache	60.3 (70/116)
Speech Problems	55.0 (155/282)	Tinnitus	50.0 (80/160)	Behavioral problem	57.9 (66/114)
Mood Disorder	51.1 (142/278)	Difficulty losing weight	49.4 (77/156)	Dysautonomia	57.5 (65/113)
Ptosis	50.9 (139/273)	Dehydration	48.2 (78/162)	Decreased vision	54.7 (64/117)
Dysautonomia	48.1 (124/258)	Hyperlipidemia	44.8 (73/163)	Difficulty gaining weight	54.4 (62/114)
Learning Disability	43.5 (120/276)	Heart rhythm problems	44.2 (72/163)	Peripheral neuropathy	51.8 (58/112)
Difficulty Gaining weight	40.6 (112/276)	Hearing loss	41.7 (68/163)	Eye muscle problems	51.8 (59/114)
Hearth Rhythm Problems	39.2 (109/278)	Dysautonomia	40.7 (59/145)	Mood disorder	51.3 (59/115)
Difficulty losing weight	38.9 (102/262)	Speech problems	38.8 (64/165)	Epilepsy	45.8 (54/118)
Intellectual Disability	38.7 (108/279)	Delayed milestones	31.4 (48/153)	Ptosis	43.0 (49/114)
Sleep Apnea	35.4 (97/274)	Difficulty gaining weight	30.9 (50/162)	Sleep apnea	42.1 (48/114)
Tinnitus	34.3 (93/271)	Sleep apnea	30.6 (49/160)	Autism spectrum behavior	39.1 (45/115)

Behavioral Problems	34.3 (95/277)	Optic nerve problems	28.5 (45/158)	Heart rhythm problems	32.2 (37/115)
Hearing Loss	33.5 (93/278)	Retinal problems	26.0 (41/158)	Optic nerve problems	23.7 (27/114)
Hyperlipidemia	33.3 (92/276)	Heart muscle problems	22.0 (35/159)	Difficulty losing weight	23.6 (25/106)
Epilepsy	31.2 (88/282)	Learning disability	21.7 (35/161)	Heart muscle problems	22.8 (26/114)
Optic Nerve Problems	26.5 (72/272)	Diabetes	21.6 (35/162)	Hearing loss	21.7 (25/115)
Heart Muscle Problems	22.3 (61/273)	Epilepsy	20.7 (34/164)	Hyperlipidemia	16.8 (19/113)
Autism Spectrum Disorder	22.0 (60/273)	Intellectual disability	19.6 (32/163)	Retinal problems	15.8 (18/114)
Retinal Problems	21.7 (59/272)	Behavioral problem	17.8 (29/163)	Stroke	13.0 (15)
Diabetes	14.7 (41/278)	Stroke	12.4 (20/161)	Liver disease	12.8 (15/117)
Stroke	12.7 (35/276)	Liver disease	11.7 (19/162)	Tinnitus	11.7 (13/111)
Liver Disease	12.2 (34/279)	Kidney disease	10.6 (17/161)	Kidney disease	6.0 (7/116)
Kidney Disease	8.7 (24/277)	Autism spectrum behavior	9.5 (15/158)	Diabetes	5.2 (6/116)

¹Nonrespondents on individual symptoms (maximum for 16 [5.5%] Adults and 15 [5.2%] for Children) are excluded. Lower denominator (N) indicates the total number of respondents.

S2B Table. All patients, Adults and Children likely to participate¹ in a clinical trial, by symptom targeted.

All (N=290)²		Adults (N=169)²		Children (N=121)²	
Symptom targeted by the trial	% (n)	Symptom targeted by the trial	% (n)	Symptom targeted by the trial	% (n)
Muscle weakness	91.0 (244/268)	Chronic fatigue	94.2 (146/155)	Muscle weakness	88.6 (101/114)
Chronic fatigue	87.7 (236/269)	Exercise intolerance	94.2 (146/155)	Chronic fatigue	79.0 (90/114)
Exercise intolerance	87.3 (234/268)	Muscle weakness	92.9 (143/154)	Exercise intolerance	77.9 (88/113)
Gastrointestinal problems	73.0 (192/263)	Balance problems	74.0 (111/150)	Gastrointestinal problems	75.9 (85/112)
Balance problems	72.9 (188/258)	Eye muscle problems	71.4 (105/147)	Delayed milestones	73.5 (83/113)
Peripheral neuropathy	65.4 (166/254)	Peripheral neuropathy	71.0 (103/145)	Balance problems	71.3 (77/108)
Sleep problems	64.8 (169/261)	Gastrointestinal problems	70.9 (107/151)	Learning disability	71.2 (79/111)
Eye muscle problems	62.6 (161/257)	Decreased vision	67.8 (101/149)	Intellectual disability	68.8 (75/109)
Decreased vision	60.7 (159/262)	Sleep problems	66.9 (101/151)	Speech problems	68.5 (76/111)
Headache	57.1 (149/261)	Ptosis	63.8 (95/149)	Sleep problems	61.8 (68/110)
Ptosis	56.2 (146/260)	Headache	58.0 (87/150)	Dehydration	60.7 (68/112)
Dehydration	56.1 (148/264)	Difficulty losing weight	56.1 (83/148)	Dysautonomia	58.9 (63/107)
Speech problems	53.7 (139/259)	Heart rhythm problems	54.1 (79/146)	Peripheral neuropathy	57.8 (63/109)
Dysautonomia	52.0 (131/252)	Hyperlipidemia	53.0 (79/149)	Headache	55.9 (62/111)
Delayed milestones	50.8 (131/258)	Dehydration	52.6 (80/152)	Behavioral problem	52.3 (57/109)
Learning disability	50.4 (130/258)	Hearing loss	52.4 (76/145)	Mood disorder	51.8 (57/110)
Heart rhythm problems	50.0 (128/256)	Optic nerve problems	51.4 (76/148)	Decreased vision	51.3 (58/113)
Sleep apnea	49.6 (129/260)	Retinal problems	51.0 (73/143)	Eye muscle problems	50.9 (56/110)
Intellectual disability	49.2 (126/256)	Heart muscle problems	50.7 (75/148)	Sleep apnea	50.9 (57/112)
Mood disorder	49.0 (127/259)	Sleep apnea	48.7 (72/148)	Epilepsy	48.7 (54/111)
Optic nerve problems	48.5 (126/260)	Tinnitus	48.6 (71/146)	Difficulty gaining weight	48.7 (54/111)
Heart muscle problems	46.9 (121/258)	Mood disorder	47.0 (70/149)	Autism spectrum behavior	46.4 (51/110)
Difficulty losing weight	46.5 (120/258)	Dysautonomia	46.9 (68/145)	Ptosis	46.0 (51/111)
Hearing loss	46.1 (118/256)	Diabetes	45.1 (64/142)	Optic nerve problems	44.6 (50/112)

Retinal problems	45.5 (116/255)	Speech problems	42.6 (63/148)	Heart rhythm problems	44.6 (49/110)
Hyperlipidemia	45.4 (118/260)	Kidney disease	40.5 (60/148)	Heart muscle problems	41.8 (46/110)
Difficulty gaining weight	41.0 (105/256)	Stroke	38.1 (56/147)	Retinal problems	38.4 (43/112)
Behavioral problem	40.9 (105/257)	Liver disease	37.2 (55/148)	Hearing loss	37.8 (42/111)
Tinnitus	40.5 (104/257)	Difficulty gaining weight	35.2 (51/145)	Hyperlipidemia	35.1 (39/111)
Diabetes	40.2 (101/251)	Intellectual disability	34.7 (51/147)	Stroke	34.8 (39/112)
Epilepsy	40.2 (104/259)	Learning disability	34.7 (51/147)	Diabetes	33.9 (37/109)
Stroke	36.7 (95/259)	Epilepsy	33.8 (50/148)	Difficulty losing weight	33.6 (37/110)
Kidney disease	36.2 (93/257)	Delayed milestones	33.1 (48/145)	Liver disease	31.8 (35/110)
Autism spectrum behavior	34.9 (89/255)	Behavioral problem	32.4 (48/148)	Kidney disease	30.3 (33/109)
Liver disease	34.9 (90/258)	Autism spectrum behavior	26.2 (38/145)	Tinnitus	29.7 (33/111)

¹ Respondents coded “likely to participate” responded “Would Participate” or “Likely to participate” in a trial aiming to treat the listed symptom.

² **Nonrespondents on individual symptoms (maximum 27 [16.0%] for Adults and 14 [4.8%] for Children) are excluded. Lower denominator (N) indicates the total number of respondents**

S2C Table. All patients, Adults and Children likely to participate¹ in a clinical trial if experienced symptom is targeted.

All (N=290)²		Adults (N=169)²		Children (N=121)²	
Experienced symptom targeted by the trial	% (n)	Experienced symptom targeted by the trial	% (n)	Experienced symptom targeted by the trial	% (n)
Muscle Weakness	93.0 (239/257)	Kidney Disease	100.0 (16/16)	Diabetes	100.0 (4/4)
Chronic Fatigue	90.3 (232/257)	Exercise Intolerance	97.2 (140/144)	Muscle Weakness	89.3 (100/112)
Exercise Intolerance	89.4 (227/254)	Chronic Fatigue	96.6 (143/148)	Intellectual Disability	87.5 (63/72)
Diabetes	86.5 (32/37)	Muscle Weakness	95.9 (139/145)	Learning Disability	86.6 (71/82)
Kidney Disease	86.4 (19/22)	Ptosis	89.4 (76/85)	Stroke	85.7 (12/14)
Intellectual Disability	84.3 (86/102)	Eye Muscle Problems	88.6 (78/88)	Optic Nerve Problems	84.6 (22/26)
Optic Nerve Problems	84.3 (57/68)	Peripheral Neuropathy	88.4 (76/86)	Dysautonomia	83.6 (51/61)
Dysautonomia	83.8 (97/116)	Gastrointestinal Problems	85.8 (97/113)	Gastrointestinal Problems	82.5 (80/97)
Peripheral Neuropathy	83.6 (116/139)	Sleep Apnea	85.4 (41/48)	Chronic Fatigue	81.7 (89/109)
Balance Problems	83.5 (168/201)	Diabetes	84.8 (28/33)	Delayed Milestones	81.6 (80/98)
Ptosis	82.0 (107/131)	Difficulty Losing Weight	84.7 (61/72)	Balance Problems	81.2 (69/85)
Eye Muscle Problems	81.7 (118/144)	Dysautonomia	83.6 (46/55)	Autism Spectrum Behaviors	81.0 (34/42)
Learning Disability	81.4 (94/116)	Optic Nerve Problems	83.3 (35/42)	Speech Problem	80.5 (70/87)
Hearing Loss	81.0 (68/84)	Hearing Loss	82.5 (52/63)	Exercise Intolerance	79.1 (87/110)
Sleep Apnea	81.0 (74/91)	Balance Problems	82.5 (99/120)	Sleep Difficulties	78.6 (55/70)
Stroke	79.6 (27/34)	Decreased Vision	82.1 (87/106)	Hearing Loss	76.2 (16/21)
Difficulty Losing Weight	79.4 (76/96)	Heart Rhythm Problems	80.6 (54/65)	Heart Rhythm Problems	75.8 (25/33)
Sleep Difficulties	79.2 (140/177)	Sleep Difficulties	79.4 (85/107)	Peripheral Neuropathy	75.5 (40/53)
Heart Rhythm Problems	79.1 (79/100)	Retinal Problems	78.4 (29/37)	Epilepsy	74.0 (37/50)
Gastrointestinal Problems	79.0 (117/148)	Intellectual Disability	76.7 (23/30)	Sleep Apnea	73.3 (33/45)
Decreased Vision	76.5 (127/166)	Heart Muscle Problems	75.8 (25/33)	Headache	72.7 (48/66)

Autism Spectrum Behaviors	76.4 (42/55)	Dehydration	75.7 (56/74)	Retinal Problems	72.2 (13/18)
Retinal Problems	76.4 (42/55)	Stroke	75.0 (15/20)	Difficulty Gaining Weight	72.1 (44/61)
Speech Problem	76.4 (113/148)	Headache	74.7 (74/99)	Dehydration	71.4 (55/77)
Delayed Milestones	76.1 (108/142)	Hyperlipidemia	74.3 (52/70)	Heart Muscle Problems	70.8 (17/24)
Headache	73.9 (122/165)	Tinnitus	71.6 (53/74)	Eye Muscle Problems	70.2 (40/57)
Heart Muscle Problems	73.7 (42/57)	Speech Problem	70.5 (43/61)	Liver Disease	69.2 (9/13)
Dehydration	73.5 (111/151)	Behavioral Problems	70.4 (19/27)	Behavioral Problems	68.8 (44/64)
Hyperlipidemia	72.4 (63/87)	Epilepsy	69.7 (23/33)	Ptosis	67.4 (31/46)
Epilepsy	72.3 (60/83)	Learning Disability	67.6 (23/34)	Decreased Vision	66.7 (40/60)
Tinnitus	69.8 (60/86)	Mood Disorder	67.5 (54/80)	Mood Disorder	64.9 (37/57)
Behavioral Problems	69.2 (63/91)	Delayed Milestones	63.6 (28/44)	Hyperlipidemia	64.7 (11/17)
Difficulty Gaining Weight	66.7 (72/108)	Liver Disease	63.2 (12/19)	Difficulty Losing Weight	62.5 (15/24)
Mood Disorder	66.4 (91/137)	Autism Spectrum Behaviors	61.5 (8/13)	Tinnitus	58.3 (7/12)
Liver Disease	65.6 (21/32)	Difficulty Gaining Weight	59.6 (28/47)	Kidney Disease	50.0 (3/6)

Respondents coded "likely to participate" responded "Would Participate" or "Likely to participate" in a trial aiming to treat the listed symptom.

² Nonrespondents on individual symptoms (maximum 258 [88.9%] for Adults and Children) are excluded. Lower denominator (N) indicates the total number of respondents.

S2D Table. Symptoms most frequently selected by individuals in the top 3¹ that would prompt their participation in a clinical trial, among All patients, Adults and Children.

All (N=290)²		Adults (N=169)²		Children (N=121)²	
Symptom	% (n)	Symptom	% (n)	Symptom	% (n)
Muscle weakness	43.1 (125/290)	Chronic fatigue	52.7 (89/169)	Muscle weakness	40.5 (49/121)
Chronic fatigue	41.4 (120/290)	Muscle weakness	45.0 (76/169)	Chronic fatigue	25.6 (31/121)
Exercise intolerance	23.8 (69/290)	Exercise intolerance	32.5 (55/169)	Gastrointestinal problems	24.0 (29/121)
Dysautonomia	17.9 (62/290)	Eye muscle problems	17.8 (30/169)	Dysautonomia	23.1 (28/121)
Gastrointestinal problems	17.9 (52/290)	Dysautonomia	14.2 (24/169)	Epilepsy	19.0 (23/121)
Epilepsy	10.7 (31/290)	Ptosis	14.2 (24/169)	Speech problems	12.4 (15/121)
Eye muscle problems	10.7 (31/290)	Gastrointestinal problems	13.6 (23/169)	Exercise intolerance	11.6 (14/121)
Ptosis	8.6 (25/290)	Peripheral neuropathy	10.7 (18/169)	Intellectual disability	10.7 (13/121)
Peripheral neuropathy	7.2 (21/290)	Decreased vision	8.3 (14/169)	Autism spectrum behavior	9.9 (12/121)
Imbalance	6.9 (20/290)	Headache	8.3 (14/169)	Learning disability	9.9 (12/121)
Decreased vision	6.2 (18/290)	Imbalance	7.7 (13/169)	Behavioral problem	9.1 (11/121)
Speech problem	6.2 (18/290)	Difficulty losing weight	4.7 (8/169)	Delayed milestones	8.3 (10/121)
Headache	5.5 (16/290)	Epilepsy	4.7 (8/169)	Difficulty gaining weight	8.3 (10/121)
Sleep problems	5.2 (15/290)	Sleep problems	4.1 (7/169)	Sleep problems	6.6 (8/121)
Intellectual disability	5.2 (15/290)	Retinal problems	4.1 (7/169)	Balance problems	5.8 (7/121)
Difficulty gaining weight	4.5 (13/290)	Heart rhythm problems	3.6 (6/169)	Heart rhythm problems	4.1 (5/121)
Autism spectrum behavior	4.1 (12/290)	Optic nerve problems	3.6 (6/169)	Liver disease	4.1 (5/121)
Learning disability	4.1 (12/290)	Dehydration	3.0 (5/169)	Mood disorder	4.1 (5/121)
Retinal problems	4.1 (12/290)	Diabetes	3.0 (5/169)	Retinal problems	4.1 (5/121)
Behavioral problem	3.8 (11/290)	Heart muscle problems	3.0 (5/169)	Decreased vision	3.3 (4/121)
Heart rhythm problems	3.4 (11/290)	Hearing loss	2.4 (4/169)	Hearing loss	3.3 (4/121)
Delayed milestones	3.4 (10/290)	Stroke	2.4 (4/169)	Heart muscle problems	3.3 (4/121)
Difficulty losing weight	3.4 (10/290)	Difficulty gaining weight	1.8 (3/169)	Optic nerve problems	3.3 (4/121)
Optic nerve problems	3.4 (10/290)	Speech problems	1.8 (3/169)	Sleep apnea	3.3 (4/121)

Heart muscle problems	3.1 (9/290)	Tinnitus	1.8 (3/169)	Peripheral neuropathy	2.5 (3/121)
Hearing loss	2.8 (8/290)	Intellectual disability	1.2 (2/169)	Dehydration	1.7 (2/121)
Dehydration	2.4 (7/290)	Mood disorder	1.2 (2/169)	Difficulty losing weight	1.7 (2/121)
Mood disorder	2.4 (7/290)	Hyperlipidemia	0.6 (1/169)	Headache	1.7 (2/121)
Diabetes	2.1 (6/290)	Liver disease	0.6 (1/169)	Stroke	1.7 (2/121)
Liver disease	2.1 (6/290)	Sleep apnea	0.6 (1/169)	Diabetes	0.8 (1/121)
Stroke	2.1 (6/290)	Autism spectrum behavior	0.0 (0/169)	Eye muscle problems	0.8 (1/121)
Sleep apnea	1.7 (5/290)	Behavioral problem	0.0 (0/169)	Hyperlipidemia	0.8 (1/121)
Tinnitus	1.0 (3/290)	Delayed milestones	0.0 (0/169)	Kidney disease	0.8 (1/121)
Hyperlipidemia	0.7 (2/290)	Kidney disease	0.0 (0/169)	Ptosis	0.8 (1/121)
Kidney disease	0.3 (1/290)	Learning disability	0.0 (0/169)	Tinnitus	0. (0.0/121)

¹Participants were asked to select, from a list of 35 symptoms, the top 3 that would prompt their participation in a clinical trial.

² **Nonrespondents on individual symptoms (maximum 0 [0.0%] for Adults and 0 [0.0%] for Children) are excluded. Lower denominator (N) indicates the total number of respondents.**