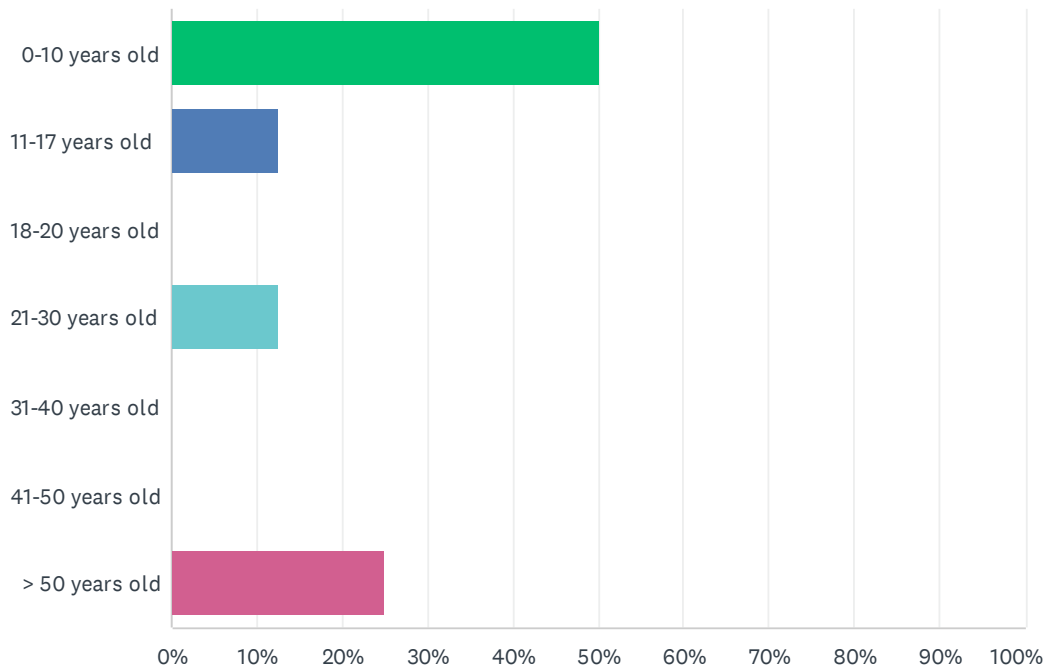


Q6 How old is the patient?

Answered: 8 Skipped: 3

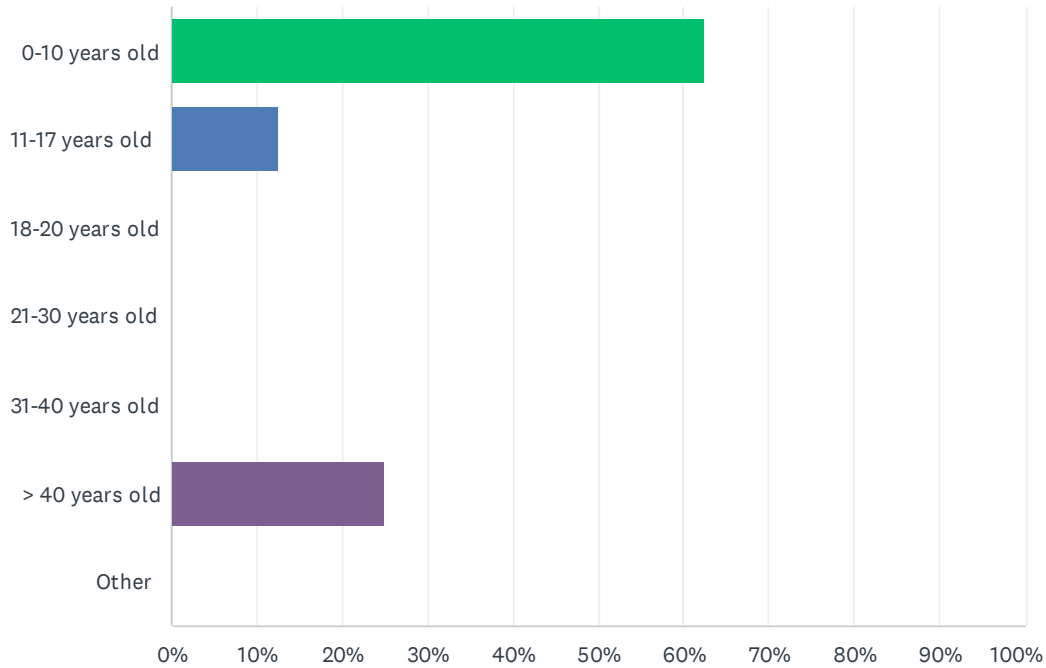


ANSWER CHOICES	RESPONSES	
0-10 years old	50.00%	4
11-17 years old	12.50%	1
18-20 years old	0.00%	0
21-30 years old	12.50%	1
31-40 years old	0.00%	0
41-50 years old	0.00%	0
> 50 years old	25.00%	2
TOTAL		8

#	OTHER	DATE
1	3 years old	1/20/2022 2:20 PM

Q7 At what age were you, or the patient, diagnosed with TK2D?

Answered: 8 Skipped: 3

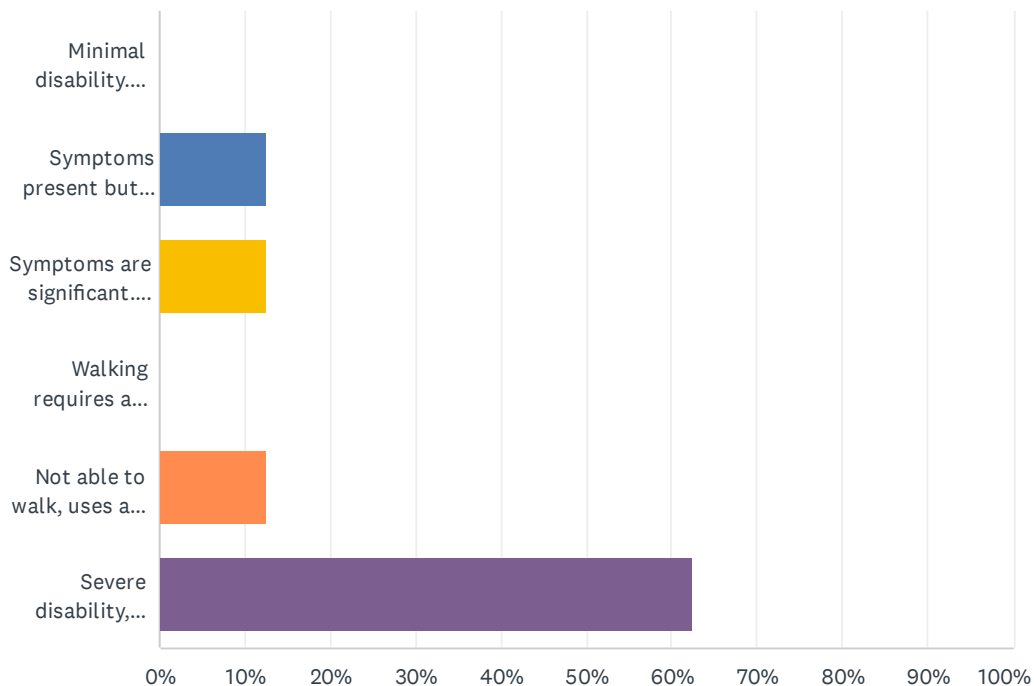


ANSWER CHOICES	RESPONSES
0-10 years old	62.50% 5
11-17 years old	12.50% 1
18-20 years old	0.00% 0
21-30 years old	0.00% 0
31-40 years old	0.00% 0
> 40 years old	25.00% 2
Other	0.00% 0
TOTAL	8

#	OTHER	DATE
	There are no responses.	

Q8 Please select the answer that best describes the stage of physical disability for you or the person for whom you care

Answered: 8 Skipped: 3



ANSWER CHOICES	RESPONSES
Minimal disability. Able to run or jump.	0.00% 0
Symptoms present but mild, able to walk and capable of leading independent life.	12.50% 1
Symptoms are significant. Require regular or periodic holding on to wall or another person for stability and walking.	12.50% 1
Walking requires a walker or other aid such as a service dog. May use a wheelchair or scooter for some activities or to conserve energy. Can perform several activities of daily living. Ability may vary from day to day.	0.00% 0
Not able to walk, uses a wheelchair exclusively. Can perform some activities of daily living that do not require standing or walking.	12.50% 1
Severe disability, dependency on others for assistance with all activities of daily living.	62.50% 5
TOTAL	8

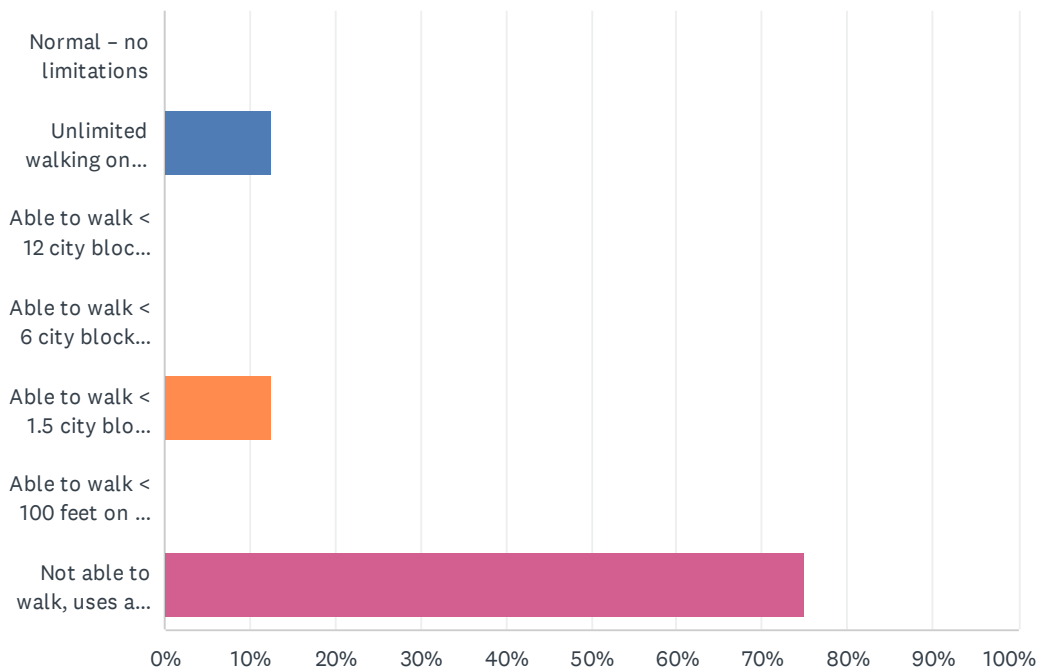
#	IF ANY ADDITIONAL COMMENTS, PLEASE FEEL FREE TO SHARE:	DATE
1	Trach, gtube, ventilator dependent toddler	1/17/2022 8:02 AM
2	I have enough movement in my thumbs to operate the touchpad on a laptop. It is my only means of communicating and interacting with others because I can't speak. I can't even lift my arms from my table or turn my head. If I need my head turned, someone has to do that for me. If I need my arms adjusted at my laptop, someone has to do that for me. Recently even my eye movement has become more limited, but it's not bad yet. The movement in my thumbs is enough to operate a wheelchair, but it's getting increasingly difficult. Anytime my hand slips	1/11/2022 1:29 AM

TK2D - Patient/Caregiver "Voice of the Patient" Survey

from the right position, even by a fraction of an inch, I can't drive anymore until someone repositions my hand. in all, I am wholly dependent on other people.

Q9 Please select the answer that best describes your ability to carry out daily activities

Answered: 8 Skipped: 3

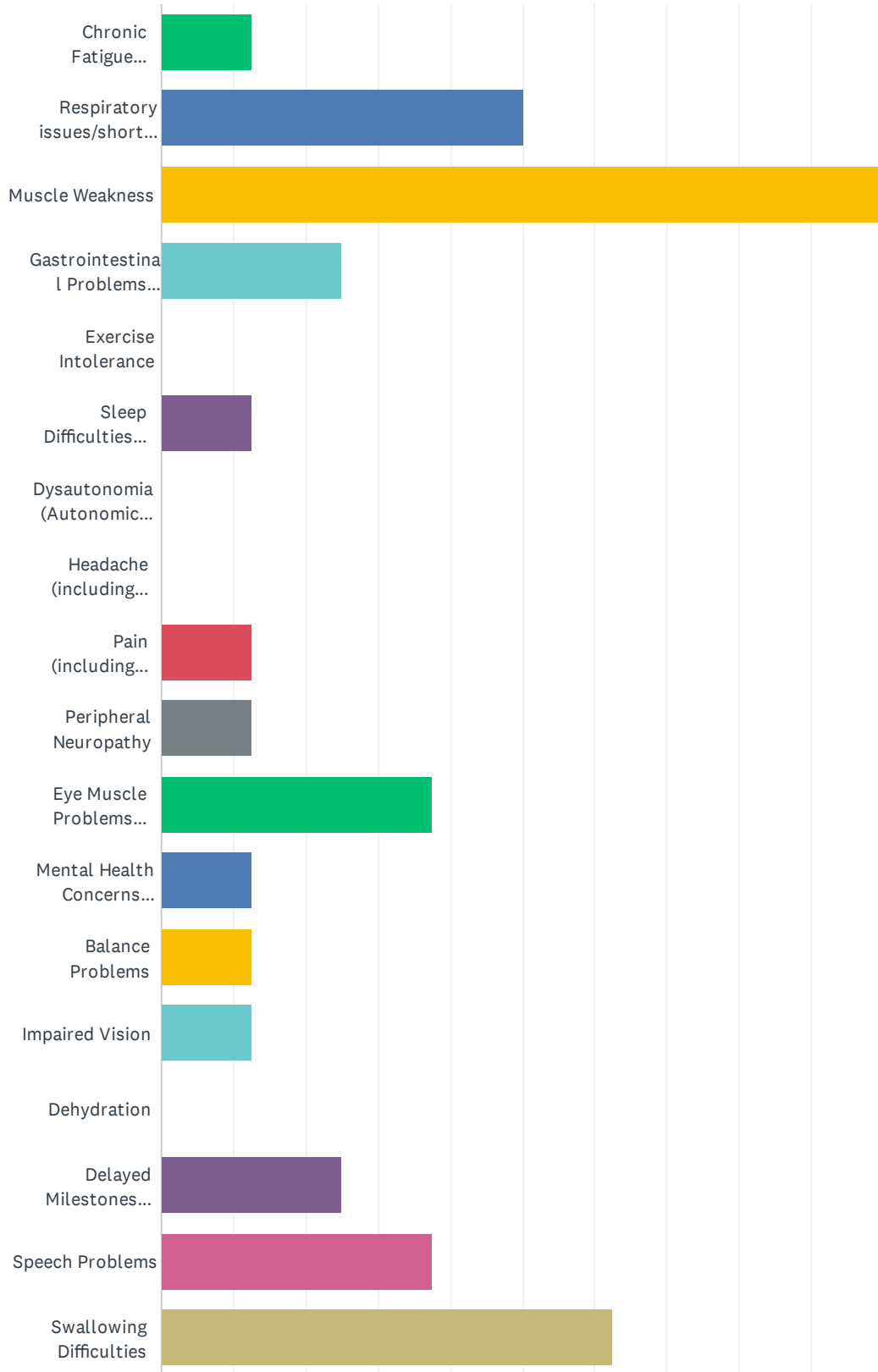


ANSWER CHOICES	RESPONSES
Normal – no limitations	0.00% 0
Unlimited walking on flat, but symptomatic on inclines or stairs.	12.50% 1
Able to walk < 12 city blocks on the flat, (or 1 mile) but restricted on inclines or stairs (rest needed after 12 steps on stairs)	0.00% 0
Able to walk < 6 city blocks on the flat, (or 1/2 mile) Rest needed after 8 steps on stairs.	0.00% 0
Able to walk < 1.5 city blocks on the flat (or 1/8 mile) Rest needed after 4 steps on stairs.	12.50% 1
Able to walk < 100 feet on the flat. Unable to do stairs alone.	0.00% 0
Not able to walk, uses a wheelchair exclusively. Can perform some activities of daily living that do not require standing or walking.	75.00% 6
TOTAL	8

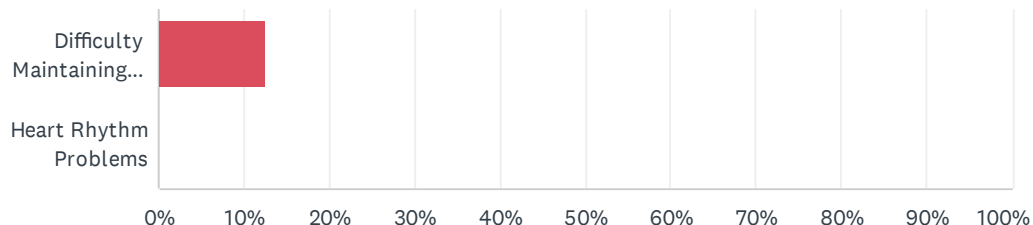
#	ADDITIONAL COMMENTS:	DATE
1	He can't do any activities of daily living on his own.	1/17/2022 9:26 PM
2	Not in a wheelchair as yet	1/17/2022 8:02 AM

Q10 Select the symptoms that most impact your daily quality of life. Select up to 5

Answered: 8 Skipped: 3



TK2D - Patient/Caregiver "Voice of the Patient" Survey



ANSWER CHOICES	RESPONSES	
Chronic Fatigue (including tiredness, excessive sleeping, brain fog or mental fatigue)	12.50%	1
Respiratory issues/shortness of breath	50.00%	4
Muscle Weakness	100.00%	8
Gastrointestinal Problems (Gastroparesis, acid reflux, constipation, diarrhea, nausea, GI pain)	25.00%	2
Exercise Intolerance	0.00%	0
Sleep Difficulties (sleep apnea, insomnia, restless leg syndrome, narcolepsy)	12.50%	1
Dysautonomia (Autonomic nervous systems problems, dizziness, difficulty with temperature modulation, low blood sugar, blood pressure issues)	0.00%	0
Headache (including migraine headaches)	0.00%	0
Pain (including Nerve Pain, Numbness, neuropathy, muscle pain, joint pain)	12.50%	1
Peripheral Neuropathy	12.50%	1
Eye Muscle Problems (including droopy eyelids, limited eye movement)	37.50%	3
Mental Health Concerns (depression, anxiety, bipolar disorders, mood disorders)	12.50%	1
Balance Problems	12.50%	1
Impaired Vision	12.50%	1
Dehydration	0.00%	0
Delayed Milestones (including developmental delays)	25.00%	2
Speech Problems	37.50%	3
Swallowing Difficulties	62.50%	5
Difficulty Maintaining Ideal Weight	12.50%	1
Heart Rhythm Problems	0.00%	0
Total Respondents: 8		

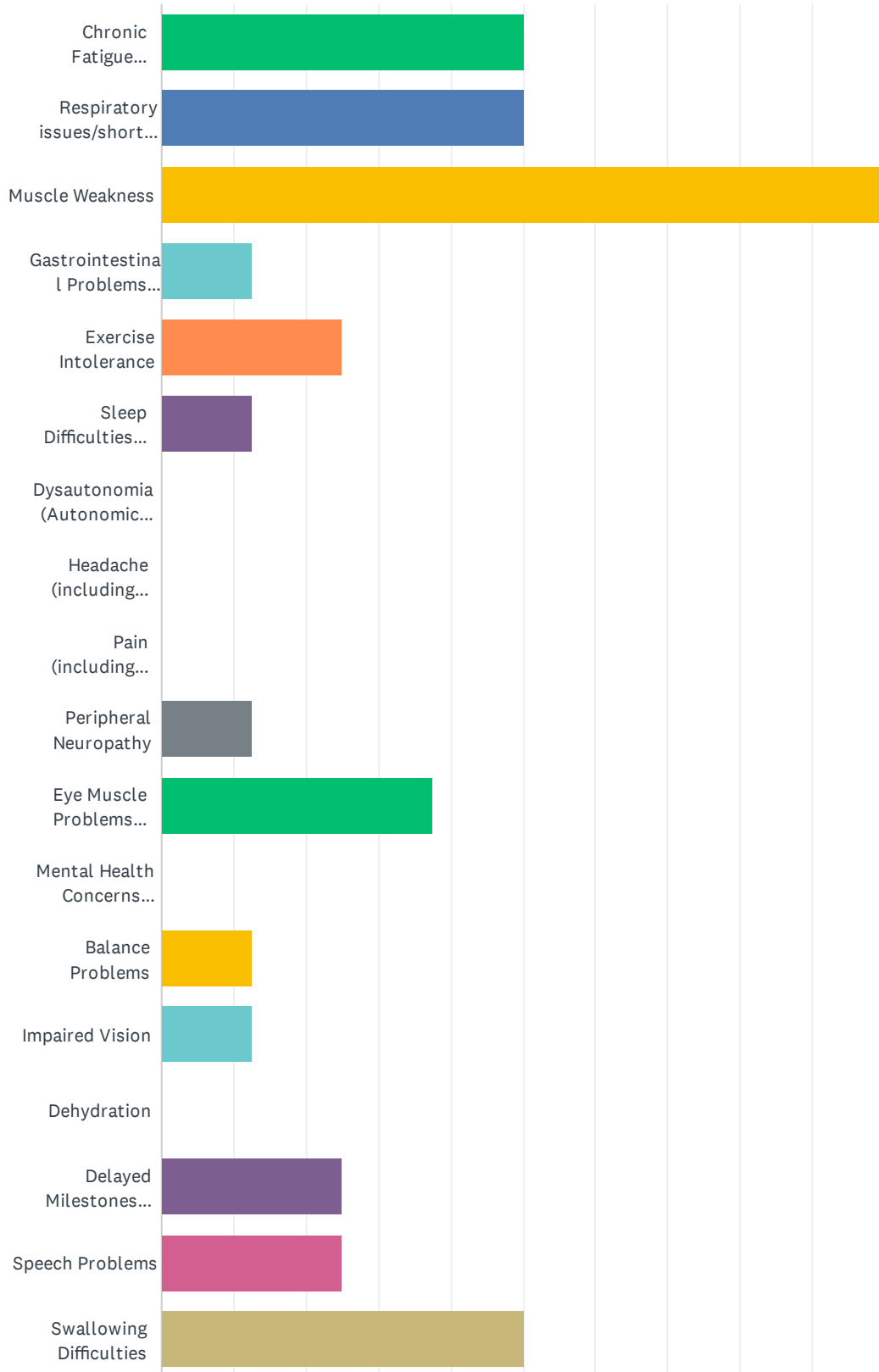
#	FROM THE PATIENT'S PERSPECTIVE, PLEASE SHARE HOW THESE FIVE SYMPTOMS IMPACT DAILY LIFE. IF YOUR SYMPTOMS ARE NOT LISTED, PLEASE SHARE HERE.	DATE
1	At all times my toddler needs supervision and care. With his respiratory issues he is ventilator dependent which is hands on care in itself. Also he is fed via a gtube due to his low muscle tone affecting his ability to swallow. The impact to daily life is our huge adaptation to what normalcy looks like. It doesn't look like him in a high chair or on my side eating food, or him swimming in the pool outside. It's him always connected to something with support keeping him alive.	1/17/2022 8:02 AM

TK2D - Patient/Caregiver "Voice of the Patient" Survey

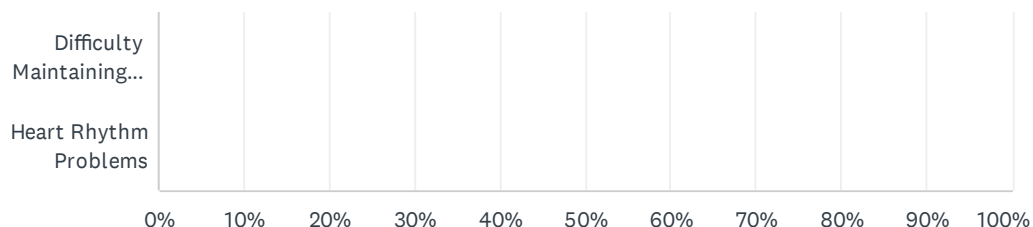
2	<p>I can't breathe on my own, which has contributed to a lot of anxiety issues over the years. If my circuit becomes disconnected, I will die in just a couple minutes unless someone is there to fix it. So someone has to be with me at all times day and night, or at least close enough to hear my vent alarm. I'm fortunate enough to have family that cares to do that, but I've still had multiple close calls over the years. All it takes is for someone to step outside at the wrong moment without thinking, or a nurse who panics and doesn't know what to do. Besides that immediate threat of disconnection, getting enough air in general is difficult. Air leaks around my trach when I get a breath, and when the leak becomes too big, I get headaches and brain fog. I also can't swallow my spit, so that will often drain into my lungs and cause problems. I get pneumonia from it about once a year on average. My eyelids are extremely droopy. A couple years ago I had to start taping them open so I can see, and that causes dryness. I have scoliosis and bilateral hip dysplasia which both cause chronic pain. My skeleton didn't develop properly because I had so much muscle atrophy as a kid. Going back to the mental health part, it's hard to describe the full toll of this disease. It's hard being so dependent on others. It's hard being unable speak. But the worst part is knowing that every day I'll wake up weaker than the last. I cling desperately to every last bit of strength, and it still slips away. One day I won't be able to drive my chair or operate my laptop, and that terrifies me.</p>	1/11/2022 1:29 AM
3	<p>Not able to walk or drive. Very limited independence. Have to rely upon wife and daughters to complete daily activities. Anxious for greatly improved health to get back to living a great quality of life.</p>	1/11/2022 12:44 AM

Q11 As TK2D disease progresses, the development or progression of which of the following symptoms worries you the most? Select up to 5

Answered: 8 Skipped: 3



TK2D - Patient/Caregiver "Voice of the Patient" Survey



ANSWER CHOICES	RESPONSES	
Chronic Fatigue (including tiredness, excessive sleeping, brain fog or mental fatigue)	50.00%	4
Respiratory issues/shortness of breath	50.00%	4
Muscle Weakness	100.00%	8
Gastrointestinal Problems (Gastroparesis, acid reflux, constipation, diarrhea, nausea, GI pain)	12.50%	1
Exercise Intolerance	25.00%	2
Sleep Difficulties (sleep apnea, insomnia, restless leg syndrome, narcolepsy)	12.50%	1
Dysautonomia (Autonomic nervous systems problems, dizziness, difficulty with temperature modulation, low blood sugar, blood pressure issues)	0.00%	0
Headache (including migraine headaches)	0.00%	0
Pain (including Nerve Pain, Numbness, neuropathy, muscle pain, joint pain)	0.00%	0
Peripheral Neuropathy	12.50%	1
Eye Muscle Problems (including droopy eyelids, limited eye movement)	37.50%	3
Mental Health Concerns (depression, anxiety, bipolar disorders, mood disorders)	0.00%	0
Balance Problems	12.50%	1
Impaired Vision	12.50%	1
Dehydration	0.00%	0
Delayed Milestones (including developmental delays)	25.00%	2
Speech Problems	25.00%	2
Swallowing Difficulties	50.00%	4
Difficulty Maintaining Ideal Weight	0.00%	0
Heart Rhythm Problems	0.00%	0
Total Respondents: 8		

#	FROM THE PATIENT'S PERSPECTIVE, PLEASE EXPLAIN WHY THESE FIVE SYMPTOMS ARE MOST WORRISOME. IF YOUR TOP SYMPTOMS ARE NOT LISTED, PLEASE SHARE HERE.	DATE
1	Stop him from having a normal life	1/17/2022 9:26 PM
2	To limit to 5 is a wish, but the disease affecting muscles which we have abs need so many in our bodies to function. The most worrisome is the progression and not having a timeline.	1/17/2022 8:02 AM
3	I am afraid these things will eventually lead to me losing my window into the world. I already	1/11/2022 1:29 AM

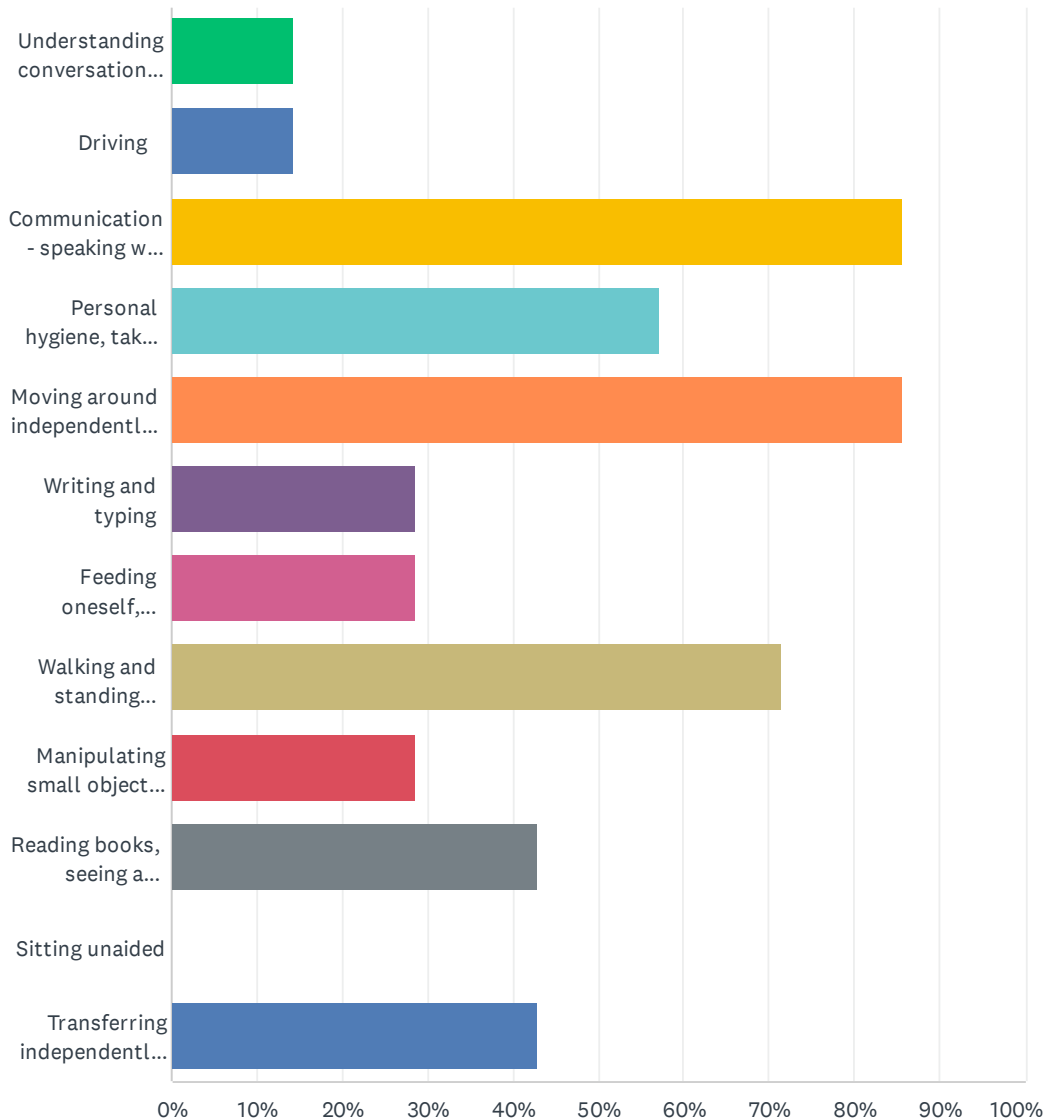
TK2D - Patient/Caregiver "Voice of the Patient" Survey

can't speak. What if I could no longer communicate via my laptop?

4	Not able to walk or drive. Very limited independence. Have to rely upon wife and daughters to complete daily activities. Anxious for greatly improved health to get back to living a great quality of life.	1/11/2022 12:44 AM
---	---	--------------------

Q12 What specific activities of daily life are most important to you that you (or the person for whom you care) are NOT able to do because of TK2D? Select TOP 3

Answered: 7 Skipped: 4



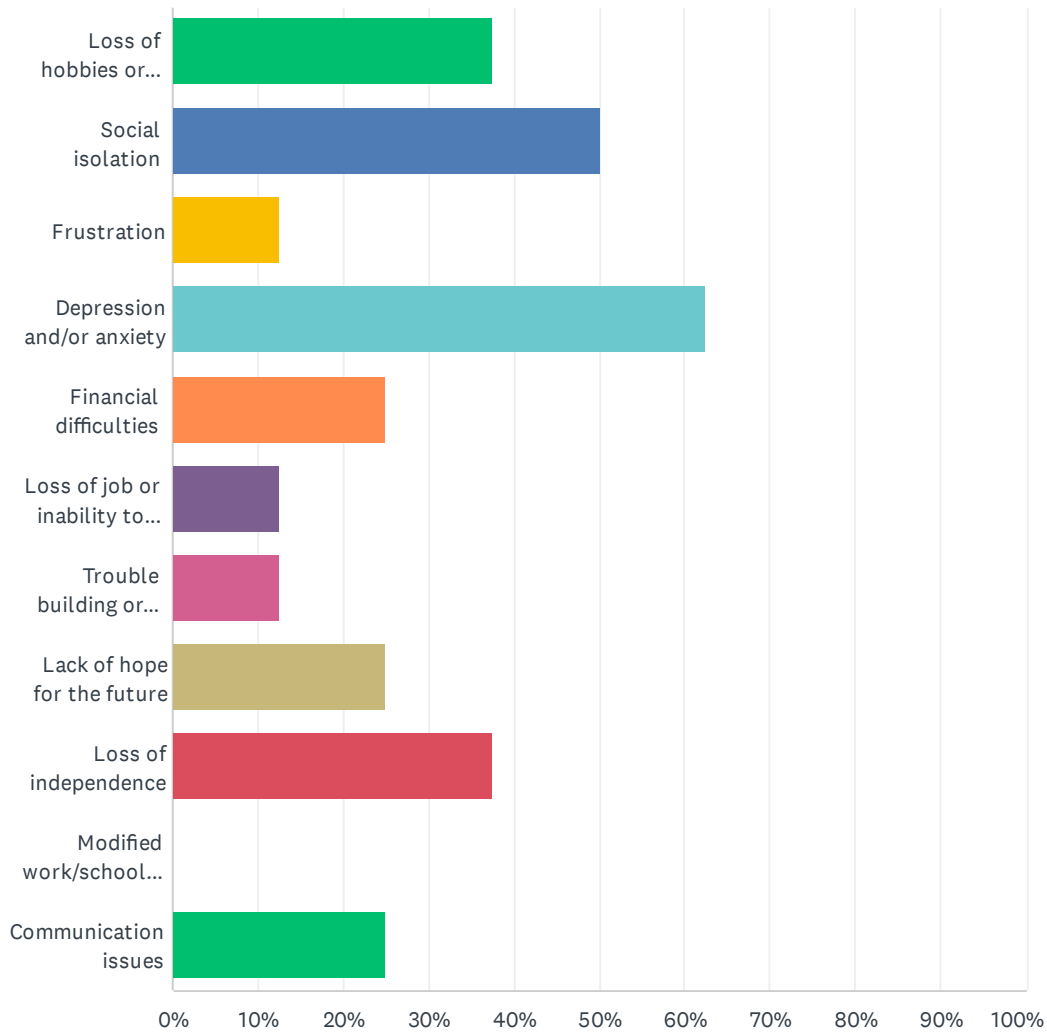
TK2D - Patient/Caregiver "Voice of the Patient" Survey

ANSWER CHOICES	RESPONSES	
Understanding conversation in noisy settings	14.29%	1
Driving	14.29%	1
Communication - speaking with others and being understood	85.71%	6
Personal hygiene, taking a shower, bathing or dressing independently	57.14%	4
Moving around independently and safely	85.71%	6
Writing and typing	28.57%	2
Feeding oneself, cutting food and handling utensils	28.57%	2
Walking and standing independently	71.43%	5
Manipulating small objects (e.g., a key, picking up items)	28.57%	2
Reading books, seeing a computer screen or phone	42.86%	3
Sitting unaided	0.00%	0
Transferring independently (e.g. from wheelchair/scooter to bed, toilet, etc.)	42.86%	3
Total Respondents: 7		

#	EXPLAIN WHY THESE ARE IMPORTANT TO THE PATIENT. SHARE ANY ADDITIONAL ACTIVITIES THAT ARE IMPORTANT TO THE PATIENT AND NOT LISTED.	DATE
1	For us I wish I knew what makes him upset, if my son is in pain or even what he is thinking. Who wouldn't want to see their toddler take their first steps. I can write an essay on this but everything above is important.	1/17/2022 8:02 AM
2	I communicate entirely through text and email. That makes it very hard to have any real conversation with anyone. My family just kind of talks to me without expecting a response, but that still gets lonely. My parents do most of my care, and they are getting older. Moving me around is becoming difficult for them.	1/11/2022 1:29 AM
3	Quality of life and independence	1/11/2022 12:44 AM

Q13 As a result of living with TK2D, which of the following social, emotional or economic consequences are most significant to you? Select up to 4

Answered: 8 Skipped: 3



TK2D - Patient/Caregiver "Voice of the Patient" Survey

ANSWER CHOICES	RESPONSES	
Loss of hobbies or activities	37.50%	3
Social isolation	50.00%	4
Frustration	12.50%	1
Depression and/or anxiety	62.50%	5
Financial difficulties	25.00%	2
Loss of job or inability to get a job	12.50%	1
Trouble building or maintaining relationships	12.50%	1
Lack of hope for the future	25.00%	2
Loss of independence	37.50%	3
Modified work/school hours	0.00%	0
Communication issues	25.00%	2
Total Respondents: 8		

#	EXPLAIN WHY THESE ARE SIGNIFICANT TO THE PATIENT. SHARE ANY ADDITIONAL INFORMATION THAT IS NOT LISTED.	DATE
1	For my son to have the best level of care and insurance, it limits my free time for anything else. His care is 24/7 and there is a nursing shortage so I'm often exhausted and moody. Also there are income limits to all these social programs etc in the state of SC. Even for me to have insurance with Medicaid I can't make over \$800 monthly which just so happens to be what we get from his SSI. The financial benchmarks are all so outdated and do not truly reflect life. I am isolated to protect him from germs and also my mind is always on my kids wellbeing so social things don't interest me.	1/17/2022 8:02 AM
2	See my previous responses. I don't have a normal life. It is often lonely, and I don't see a way for it to get better as long as my symptoms are so severe.	1/11/2022 1:29 AM
3	Not able to walk or drive. Very limited independence. Have to rely upon wife and daughters to complete daily activities. Anxious for greatly improved health to get back to living a great quality of life.	1/11/2022 12:44 AM

Q14 How do your symptoms and their negative impacts affect your daily life on the best days? On the worst days? (if you are the caregiver, please provide information from patient's perspective).

Answered: 3 Skipped: 8

#	RESPONSES	DATE
1	In every way, lose independence .	1/17/2022 9:26 PM
2	Most days I wake up, have someone set me up with my laptop, and then I stay on my laptop all day until I go to sleep. Sometimes people will have time to turn pages in a book for me, but generally I have to read on my laptop. The laptop is the only thing I can use independently. On my best days, my parents can get me in my chair so we can do something. See a movie. Visit my brothers. Go to an NBA game. On my worst days I'm sick because of the spit that drains into my lungs. Then I can't do much of anything and neither can my parents.	1/11/2022 1:29 AM
3	Not able to walk or drive. Very limited independence. Have to rely upon wife and daughters to complete daily activities. Anxious for greatly improved health to get back to living a great quality of life.	1/11/2022 12:44 AM

Q15 How has your condition and its symptoms changed over time? (if you are the caregiver, please provide information from patient's perspective).

Answered: 3 Skipped: 8

#	RESPONSES	DATE
1	He was "normal" walking , eating and talking. He lost all those abilities .	1/17/2022 9:26 PM
2	I've been dependent on a vent and wheelchair since I was 3 years old. I lost the ability to speak when I was around 9. Since then it's been a slow decline with my hands and eyes. 10 years ago I could write pretty well. Not so much anymore. 3 years ago I didn't need to tape open my eyelids to see. Now I do. I've also had some hearing problems start this past year, and after some testing it seems like it's from eustachian tube dysfunction. I suspect that's a progression of my disease.	1/11/2022 1:29 AM
3	Gotten worse	1/11/2022 12:44 AM

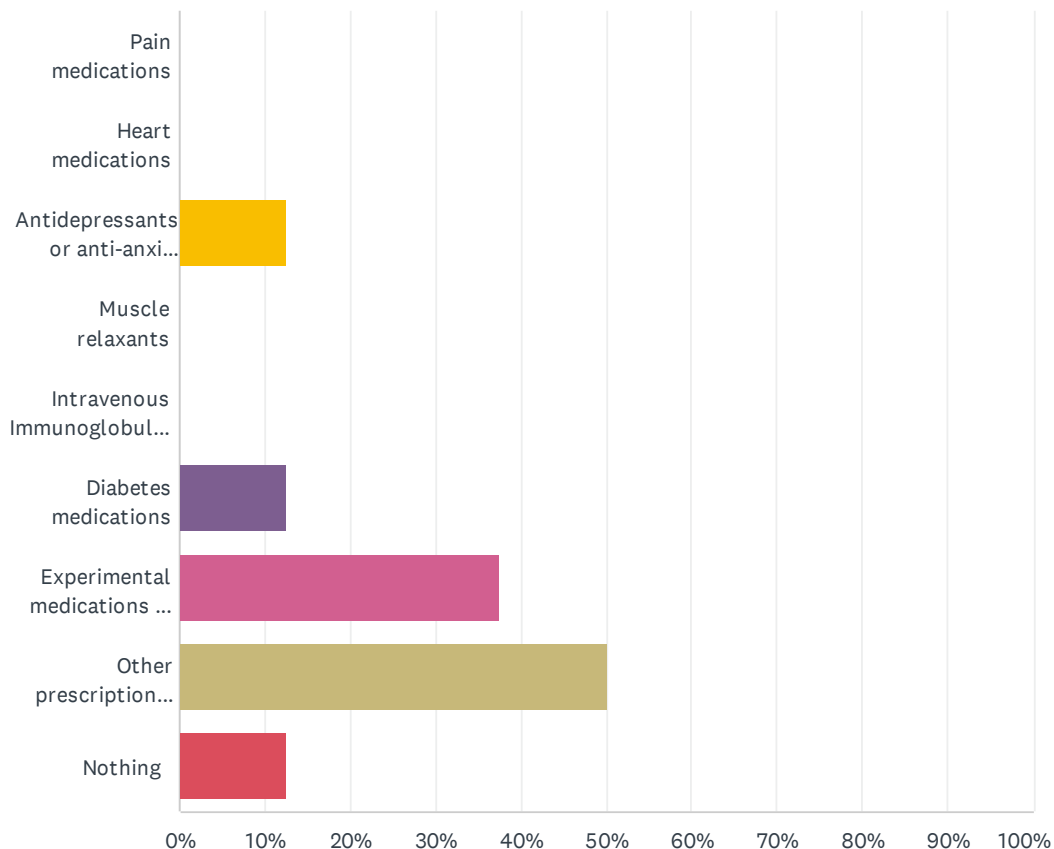
Q16 Do your symptoms come and go? If so, do you know of anything that makes your symptoms better? Worse? (if you are the caregiver, please provide information from patient's perspective).

Answered: 3 Skipped: 8

#	RESPONSES	DATE
1	Anything can make him tired , even stress, but the symptoms don't go away, just get worse if he overwork	1/17/2022 9:26 PM
2	The symptoms don't vary. Nothing makes them better. I am weaker every day. I know other diseases have ups and downs, peaks and valleys. But here there is only down, and it never reaches bottom.	1/11/2022 1:29 AM
3	Steady - not walking, difficulty swallowing	1/11/2022 12:44 AM

Q17 What prescription medications do you take now to treat symptoms of TK2D? Select ALL that apply

Answered: 8 Skipped: 3



ANSWER CHOICES	RESPONSES
Pain medications	0.00% 0
Heart medications	0.00% 0
Antidepressants or anti-anxiety medications	12.50% 1
Muscle relaxants	0.00% 0
Intravenous Immunoglobulin therapy (IVIg)	0.00% 0
Diabetes medications	12.50% 1
Experimental medications as a part of a clinical trial	37.50% 3
Other prescription medications not listed	50.00% 4
Nothing	12.50% 1
Total Respondents: 8	

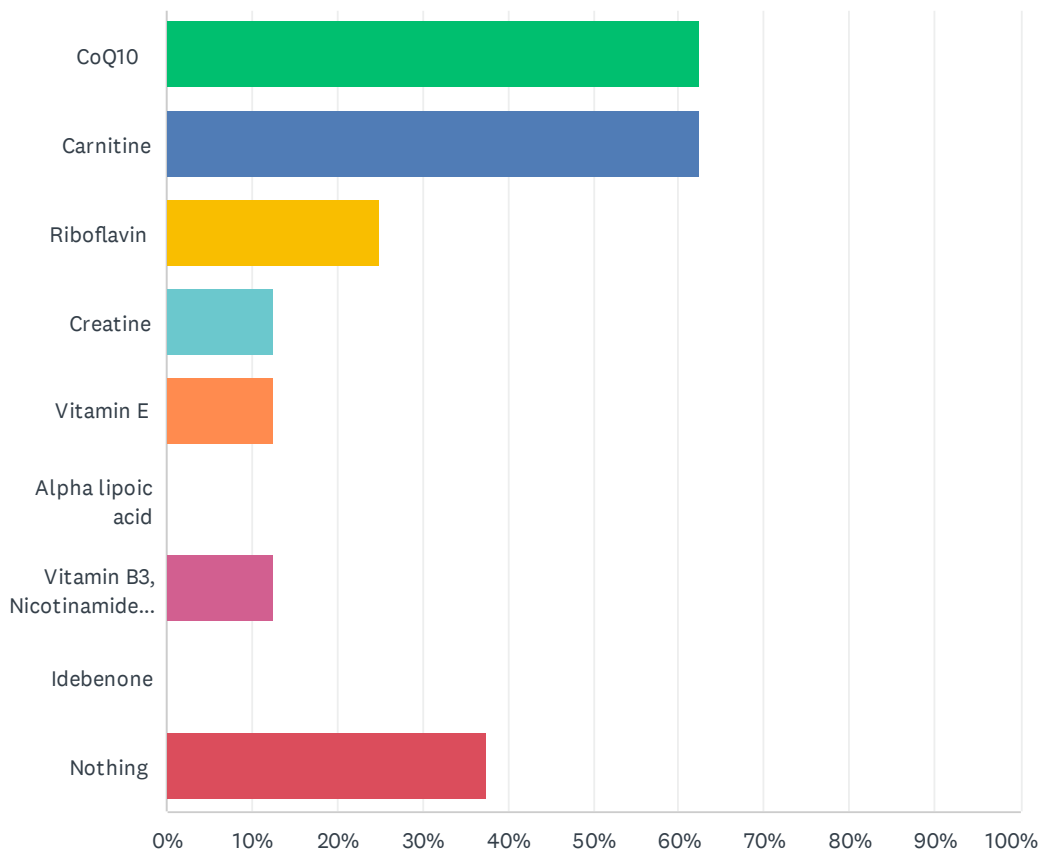
#	PLEASE LIST OTHER PRESCRIPTION MEDICATIONS NOT LISTED	DATE
---	---	------

TK2D - Patient/Caregiver "Voice of the Patient" Survey

1	Vitamin D, CoQ10, multivitamin, Levocarnitive	1/17/2022 9:26 PM
2	Omeprazole, melatonin, pepcid, nystatin, Flovent, atropine, glycopporate, ceririzine	1/17/2022 8:02 AM
3	I've had steroid injections for my hip pain.	1/11/2022 1:29 AM

Q18 What vitamins or supplements do you take now to treat symptoms of TK2D? Select ALL that apply

Answered: 8 Skipped: 3



ANSWER CHOICES	RESPONSES
CoQ10	62.50% 5
Carnitine	62.50% 5
Riboflavin	25.00% 2
Creatine	12.50% 1
Vitamin E	12.50% 1
Alpha lipoic acid	0.00% 0
Vitamin B3, Nicotinamide or Niacin	12.50% 1
Idebenone	0.00% 0
Nothing	37.50% 3
Total Respondents: 8	

#	PLEASE LIST OTHER SUPPLEMENTS OR VITAMINS NOT LISTED	DATE
---	--	------

TK2D - Patient/Caregiver "Voice of the Patient" Survey

1	MultiVitamins	1/21/2022 12:04 AM
2	Biotin, Selenium, Thiamine, Vitamin C, Antioxidant, Vitamin D3, Flaxseed Oil	1/11/2022 1:29 AM

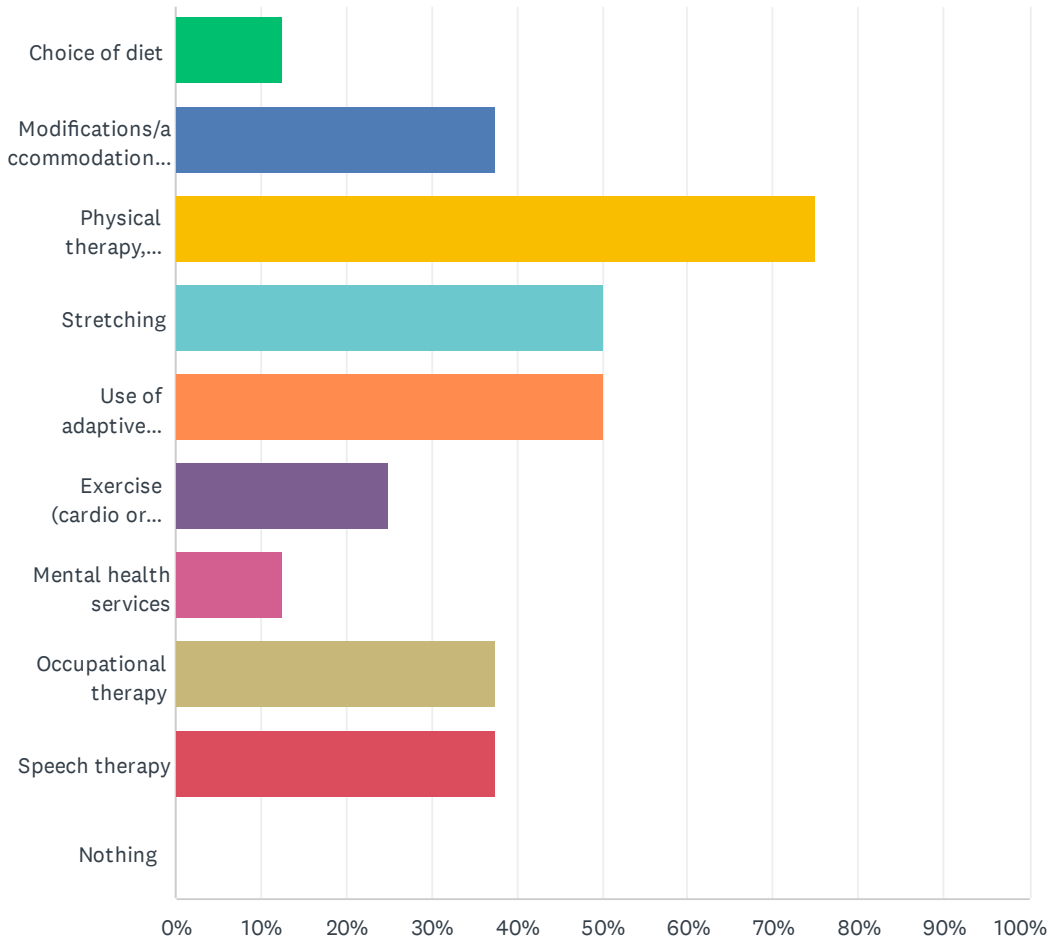
Q19 Please further explain what specific symptoms your treatments address.

Answered: 2 Skipped: 9

#	RESPONSES	DATE
1	NO treatment till now! we only rely on supplements and daily exercises.	1/20/2022 2:20 PM
2	The supplements are the "mitochondrial cocktail" prescribed by my neurologist. I suppose in theory it's meant to reduce muscle fatigue or something. I'm not convinced it does much, but there's no alternative.	1/11/2022 1:29 AM

Q20 What are you currently doing to help manage TK2D symptoms? Select ALL that apply

Answered: 8 Skipped: 3



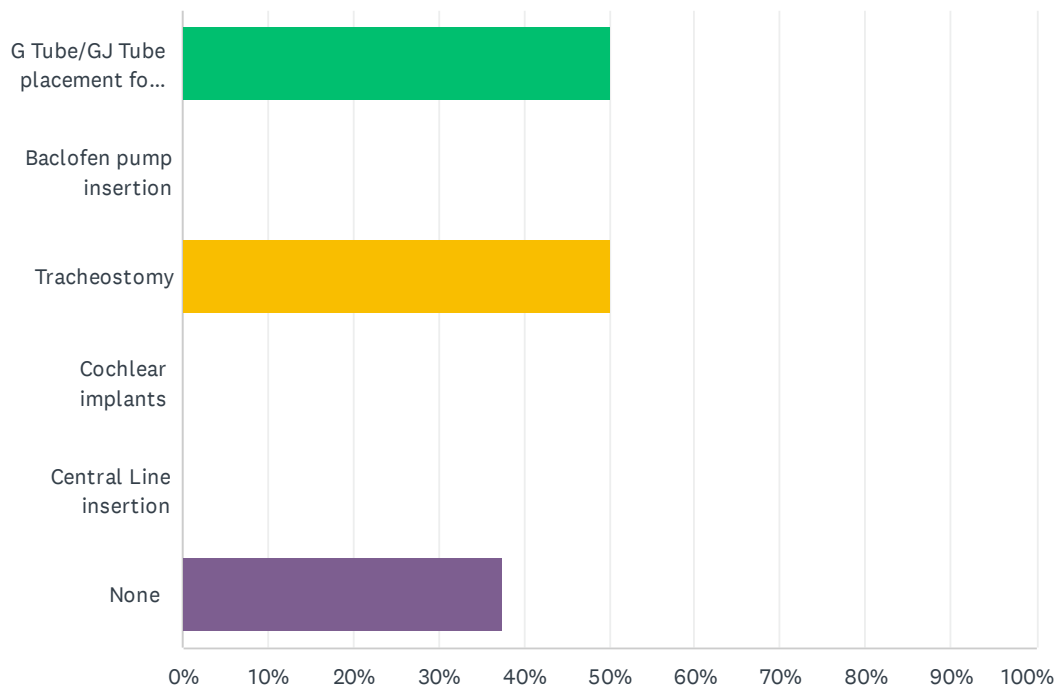
TK2D - Patient/Caregiver "Voice of the Patient" Survey

ANSWER CHOICES	RESPONSES	
Choice of diet	12.50%	1
Modifications/accommodations at work/in school/at home	37.50%	3
Physical therapy, including aqua or hippo therapy	75.00%	6
Stretching	50.00%	4
Use of adaptive devices	50.00%	4
Exercise (cardio or strength training)	25.00%	2
Mental health services	12.50%	1
Occupational therapy	37.50%	3
Speech therapy	37.50%	3
Nothing	0.00%	0
Total Respondents: 8		

#	PLEASE LIST OTHER WAYS YOU MANAGE YOUR CONDITION.	DATE
1	Yoga	1/17/2022 8:02 AM
2	I manage by trying to focus my mind on other things. The physical part is hopeless, and no therapy ever helped.	1/11/2022 1:29 AM

Q21 Which surgical procedures have you undergone to treat or manage symptoms of TK2D? Select ALL that apply

Answered: 8 Skipped: 3

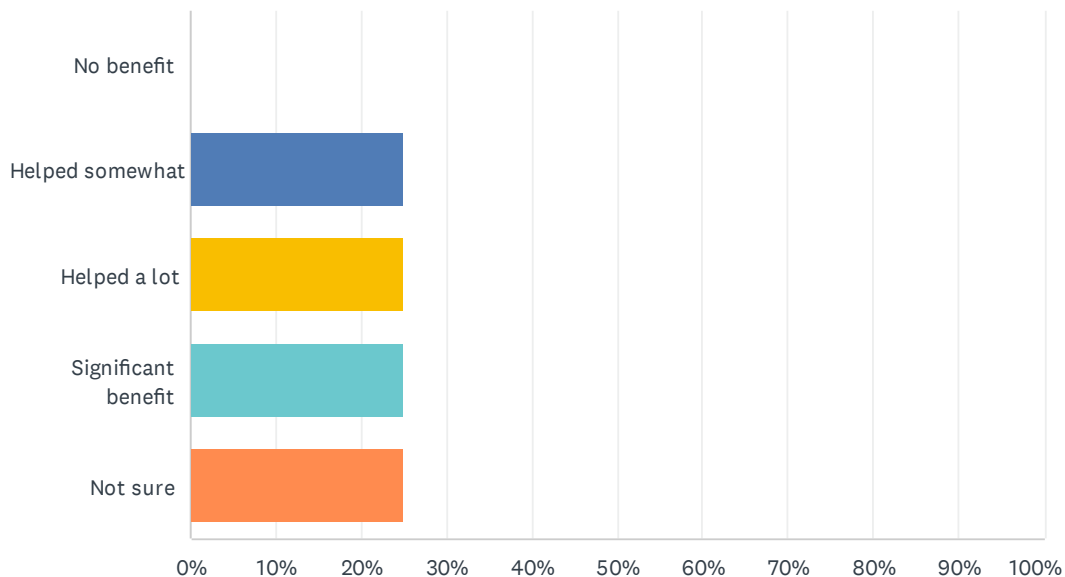


ANSWER CHOICES	RESPONSES
G Tube/GJ Tube placement for nutrition	50.00% 4
Baclofen pump insertion	0.00% 0
Tracheostomy	50.00% 4
Cochlear implants	0.00% 0
Central Line insertion	0.00% 0
None	37.50% 3
Total Respondents: 8	

#	OTHER (PLEASE SPECIFY)	DATE
1	Nissen fundoplication	1/17/2022 8:02 AM

Q22 In general, how much do the medications, supplements, therapies or lifestyle changes used improve your quality of life:

Answered: 8 Skipped: 3



ANSWER CHOICES	RESPONSES
No benefit	0.00% 0
Helped somewhat	25.00% 2
Helped a lot	25.00% 2
Significant benefit	25.00% 2
Not sure	25.00% 2
Total Respondents: 8	

#	PLEASE DESCRIBE HOW YOUR MEDICATIONS AND/OR TREATMENTS HAVE HELPED OR NOT HELPED.	DATE
1	He is getting stronger, his communication is better, and social life too.	1/17/2022 9:26 PM
2	The trach and g-tube keep me alive. I really don't think the supplements do much, but I can't say for sure. The steroid injections have helped my hip pain though. The adaptive software I use to uhelp operate my laptop at least gives me something to do during the day.	1/11/2022 1:29 AM

Q23 How has your treatment regimen changed over time, and why?

Answered: 3 Skipped: 8

#	RESPONSES	DATE
1	NO treatment till now! we only rely on supplements and daily exercises.	1/20/2022 2:20 PM
2	I stopped all physical and occupational therapy because it didn't help. I can't exercise in any meaningful capacity because fatigue comes on almost immediately. My doctor has steadily increased my CoQ10 dosage in the hope that it will help something, but it doesn't seem to.	1/11/2022 1:29 AM
3	yes.	1/11/2022 12:44 AM

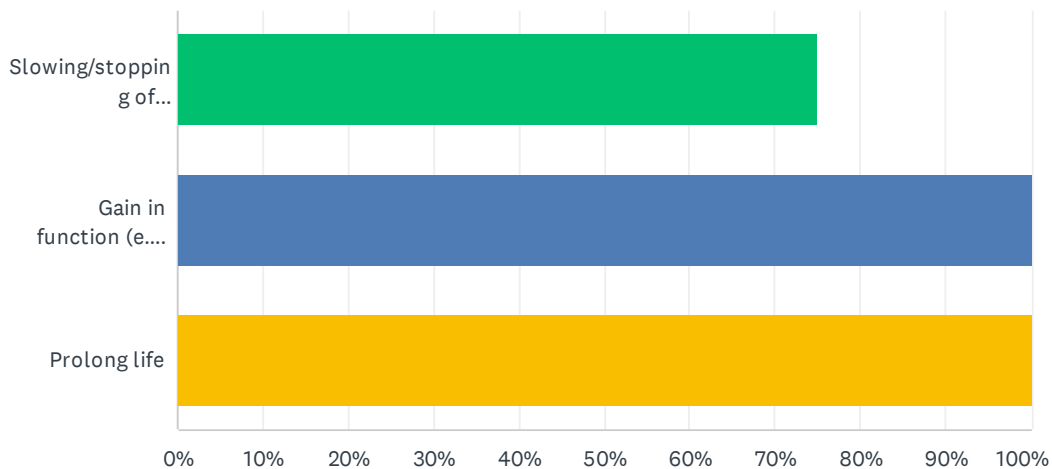
Q24 How well have these treatments worked for you as your condition changed over time?

Answered: 3 Skipped: 8

#	RESPONSES	DATE
1	NO treatment till now! we only rely on supplements and daily exercises.	1/20/2022 2:20 PM
2	They haven't. Except the steroid injections that help my hip pain.	1/11/2022 1:29 AM
3	moderate	1/11/2022 12:44 AM

Q25 Which outcomes would be meaningful to you for a possible drug treatment? Select ALL that apply

Answered: 8 Skipped: 3

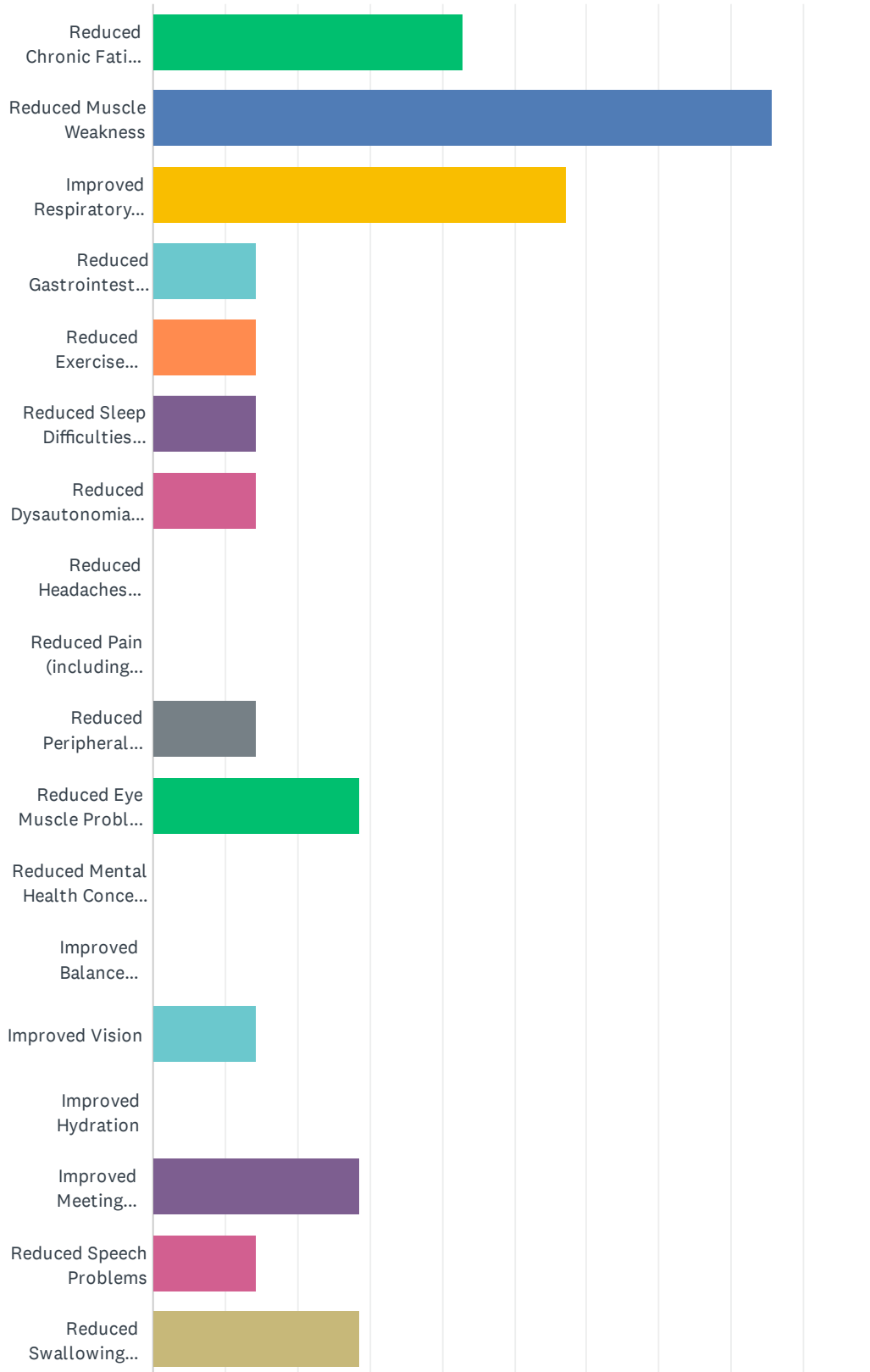


ANSWER CHOICES	RESPONSES	
Slowing/stopping of progression (even if no gain in function, symptoms won't get worse)	75.00%	6
Gain in function (e.g. energy, strength, mobility, dexterity, cardiac function, speech)	100.00%	8
Prolong life	100.00%	8
Total Respondents: 8		

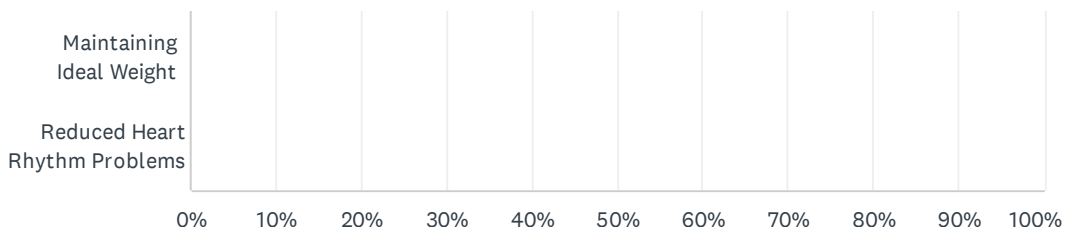
#	PLEASE EXPLAIN WHY THESE ARE MOST MEANINGFUL AND INCLUDE ADDITIONAL OUTCOMES NOT LISTED.	DATE
1	I can live as I am. I can't afford to lose anything more. If my symptoms continue to progress, I don't know what I will do.	1/11/2022 1:29 AM

Q26 Which ability or symptom would you rank as most important for a possible drug treatment today? Select up to THREE options

Answered: 7 Skipped: 4



TK2D - Patient/Caregiver "Voice of the Patient" Survey

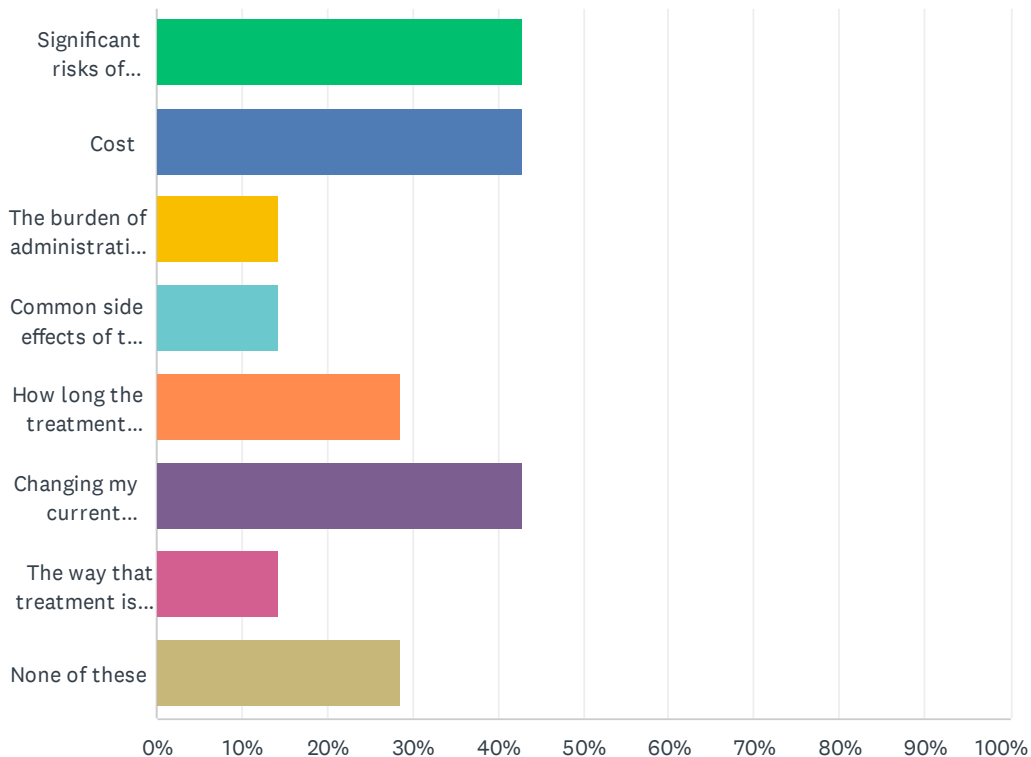


ANSWER CHOICES	RESPONSES	
Reduced Chronic Fatigue (including tiredness, excessive sleeping, brain fog or mental fatigue)	42.86%	3
Reduced Muscle Weakness	85.71%	6
Improved Respiratory Function	57.14%	4
Reduced Gastrointestinal Problems (Gastroparesis, acid reflux, constipation, diarrhea, nausea, GI pain)	14.29%	1
Reduced Exercise Intolerance	14.29%	1
Reduced Sleep Difficulties (sleep apnea, insomnia, restless leg syndrome, narcolepsy)	14.29%	1
Reduced Dysautonomia (Autonomic nervous systems problems, dizziness, difficulty with temperature modulation, low blood sugar, blood pressure issues)	14.29%	1
Reduced Headaches (including migraine headaches)	0.00%	0
Reduced Pain (including Nerve Pain, Numbness, neuropathy, muscle pain, joint pain)	0.00%	0
Reduced Peripheral Neuropathy	14.29%	1
Reduced Eye Muscle Problems (including droopy eyelids, limited eye movement)	28.57%	2
Reduced Mental Health Concerns (depression, anxiety, bipolar disorders, mood disorders)	0.00%	0
Improved Balance Problems	0.00%	0
Improved Vision	14.29%	1
Improved Hydration	0.00%	0
Improved Meeting Milestones (including reduced developmental delays)	28.57%	2
Reduced Speech Problems	14.29%	1
Reduced Swallowing Difficulties	28.57%	2
Maintaining Ideal Weight	0.00%	0
Reduced Heart Rhythm Problems	0.00%	0
Total Respondents: 7		

#	PLEASE EXPLAIN WHY THESE ARE MOST IMPORTANT TO YOU.	DATE
1	All of my problems boil down to the muscle weakness and fatigue.	1/11/2022 1:29 AM

Q27 Which of the following factors would influence your decision to take a new medication? Select ALL that apply

Answered: 7 Skipped: 4



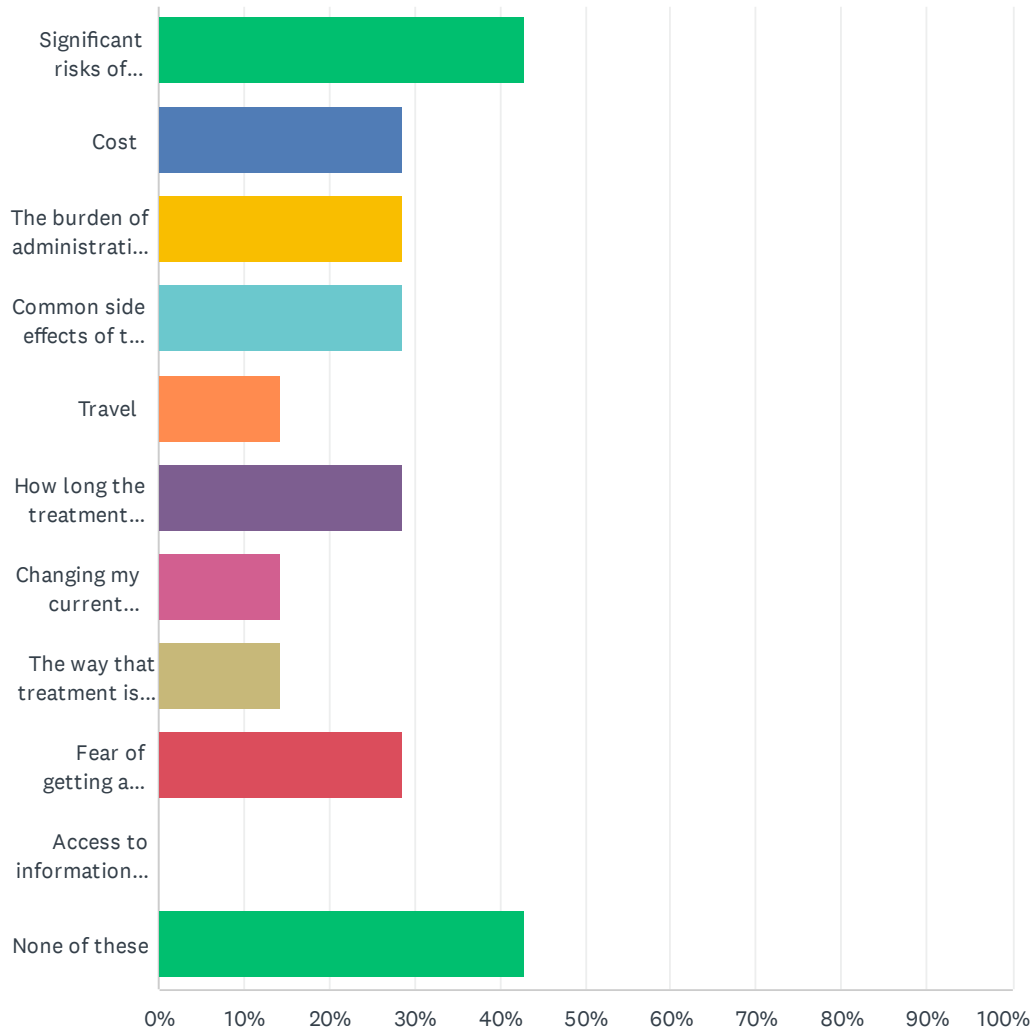
ANSWER CHOICES	RESPONSES	
Significant risks of serious side effects such as cardiac or kidney issues	42.86%	3
Cost	42.86%	3
The burden of administration, such as the need for anesthesia, radiation exposure, surgical procedure, etc.	14.29%	1
Common side effects of the treatment, such as nausea, loss of appetite, headache etc.	14.29%	1
How long the treatment takes, whether it requires hospitalization, required doctor's visits, etc.	28.57%	2
Changing my current treatment or management plan (stopping a medication or supplement, stopping exercise)	42.86%	3
The way that treatment is administered (for example, orally, intravenously, subcutaneous)	14.29%	1
None of these	28.57%	2
Total Respondents: 7		

#	PLEASE EXPLAIN WHY THESE INFLUENCE YOUR DECISION AND ANY ADDITIONAL COMMENTS.	DATE
1	our only decision is to provide the treatment to our kid no matter what!	1/20/2022 2:20 PM
2	If a new medication promised any kind of hope, I would take it almost no matter what. Serious cardiac side effects would potentially deter me, but even then I would consider taking it. This	1/11/2022 1:29 AM

disease is hopeless as is.

Q28 Which of the following factors would influence your decision to participate in a clinical trial or research study? Select ALL that apply

Answered: 7 Skipped: 4



TK2D - Patient/Caregiver "Voice of the Patient" Survey

ANSWER CHOICES	RESPONSES	
Significant risks of serious side effects such as cardiac or kidney issues	42.86%	3
Cost	28.57%	2
The burden of administration, such as the need for anesthesia, radiation exposure, surgical procedure, etc.	28.57%	2
Common side effects of the treatment, such as nausea, loss of appetite, headache etc.	28.57%	2
Travel	14.29%	1
How long the treatment takes, whether it requires hospitalization, required doctor's visits, etc.	28.57%	2
Changing my current treatment or management plan (stopping a medication or supplement, stopping exercise)	14.29%	1
The way that treatment is administered (for example, orally, intravenously, subcutaneous)	14.29%	1
Fear of getting a placebo instead trial drug/therapy	28.57%	2
Access to information about trials	0.00%	0
None of these	42.86%	3
Total Respondents: 7		

#	PLEASE EXPLAIN WHY THESE INFLUENCE YOUR DECISION AND ANY ADDITIONAL COMMENTS.	DATE
1	NO factors would influence our decision to let our kid participate in a clinical trial.	1/20/2022 2:20 PM
2	Again, the disease is hopeless as is. I would be discouraged if I might be getting a placebo because I would be left in the same hopeless situation I'm in now. But a 50/50 chance of getting the real medication is better than nothing. I already have nothing.	1/11/2022 1:29 AM

Q29 Short of a cure for TK2D, what specific things would you look for in an ideal treatment for your condition?

Answered: 6 Skipped: 5

#	RESPONSES	DATE
1	Medication that would reduce muscle weakness and fatigue.	1/21/2022 12:04 AM
2	in case of short cure in TK2D we can only rely on supplements and daily exercises.	1/20/2022 2:20 PM
3	Walk again, breathe on my own again, eating on my own again.	1/17/2022 9:26 PM
4	One that would work quickly to improve all symptoms.	1/17/2022 8:02 AM
5	I would like to be able to open my eyes again without tape. That's a dream scenario for me. But even just maintaining my ability to use a laptop would be amazing at this point.	1/11/2022 1:29 AM
6	Anxious for greatly improved health to get back to living a great quality of life. Ability to walk. Gain independence.	1/11/2022 12:44 AM