Long QT Syndrome Externally-Led Patient-Focused Drug Development (EL-PFDD) Meeting

Meeting Date: Tuesday, June 11, 2024, 9:30 am to 4:00 pm ET

Report Date: September 23, 2024

SADS Foundation EL-PFDD SPONSORS

Long QT Syndrome *Voice of the Patient* Report

The Sudden Arrhythmia Death Syndromes Foundation or SADS Foundation exists to save the lives and support the families of children and adults who are genetically predisposed to sudden death due to heart rhythm abnormalities. SADS supports those with inherited channelopathies and cardiomyopathies including conditions such as long QT syndrome (LQTS), catecholaminergic polymorphic ventricular tachycardia (CPVT), Brugada syndrome, short QT syndrome, and arrhythmogenic right ventricular cardiomyopathy. This *Voice of the Patient* report was prepared on behalf of the SADS Foundation as a summary of the input shared by families and caregivers living with LQTS during an Externally-Led Patient Focused Drug Development (EL-PFDD) meeting, conducted virtually on June 11, 2024.

Authors and Collaborators: This report was prepared and submitted on behalf of SADS by Alice Lara, RN, BSN, CEO & President; Marcia Baker, MS Ed, Program Director; Anna Goodson, Communication Director; and Genevie Echols, RCIS, Family Support Director, all from the SADS Foundation, and by Chrystal Palaty, medical writer.

Consulting Partners include Larry Bauer, RN, MA, and James Valentine, JD, MHS and from Hyman, Phelps & McNamara, P.C.

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James Valentine, JD, MHS and Larry Bauer, RN, MA are employed by Hyman, Phelps & McNamara, P.C., a law firm that represents patient advocacy organizations and companies that are developing therapeutics and technologies to advance health.

SADS Foundation contracted with Chrystal Palaty, PhD from Metaphase Health Research Consulting Inc. for assistance in writing this report.

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Acknowledgements

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Thank you to all the FDA staff who attended our meeting. Thank you to Dr. Shetarra Walker, MD, MSCR, Lead Physician and Clinical Team Leader in Pediatrics in the Center for Drug Evaluation and Research at the FDA, for providing a welcome from the FDA. Thank you to Ethan Gabbour from the FDA's PFDD staff who guided us through this process over the many months of planning. We are grateful to have had this opportunity to ensure LQTS patient and family perspectives are considered in the drug development and regulatory processes.

Thank you to Dr. Michael J. Ackerman, for providing such an insightful clinical overview of LQTS, and for serving as a scientific advisor to the SADS Foundation for many years.

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The EL-PFDD meeting was the result of many months of planning and hard work. Thank you to everyone who has had a hand in preparing for this meeting, including the SADS Foundation volunteers and staff who have given countless hours including, but not limited to CEO Alice Lara, Program Director Marcia Baker, Communications Director Anna Goodson, Development Director Jan Schiller, Technology and Administrative Director Erin Waite, and Family Support Director Genevie Echols.

Thank you to the many representatives from industry, federal agencies, and physician scientists from across the world who attended our meeting and who, on a daily basis, are striving towards a better understanding of the basic and translational science behind LQTS and are helping to move us closer to future clinical trials. Our hope is that this meeting will encourage future research and successful new product development for people living with LQTS who urgently need better treatment options.

Most importantly, the SADS Foundation would like to thank and acknowledge our LQTS community members who took the time to attend this meeting. Thank you to our speakers, panelists and callers for so honestly sharing how LQTS and its current treatments affect you on a day-to-day basis. This meeting would not have been possible without each one of you.

Key LQTS Insights

- **Long QT syndrome (LQTS) is a genetic heart condition**. Many members of the same family may be affected. LQTS symptoms can differ from one person to another, even within the same family.
- **LQTS can result in premature death from cardiac arrest.**
- **LQTS is a disease characterized by fear and worry, reflecting an uncertain future.** Family members worry about the safety and future of their parents, siblings, children and other family members living with LQTS. They worry whether medications and treatments will be sufficient or enough to prevent an event. They worry about cardiac arrests, shocks, implantable cardioverter defibrillator (ICD) failure, more surgeries, they worry about passing LQTS on to future children.
- **The most burdensome LQTS-related health concerns include anxiety and depression**. Many experience panic attacks and post-traumatic stress disorder (PTSD) because of past cardiac events, and shocks from their ICD. Other symptoms include light-headedness and fainting, cardiac arrest and fatigue. Some living with the disease experience abnormal heart rhythms, near drowning or drowning, seizures, and anoxic effects from the lack of oxygen after a sudden cardiac arrest.
- **LQTS diminishes quality of life.** LQTS and its treatments reduce stamina, impact the ability to fully and freely participate in sport, social and work life. Although exercise is safe for the majority of those with LQTS, some families continue to be restricted from exercise. These restrictions cause or worsen anxiety and depression in people living with LQTS.
- **LQTS can interfere with treatment for other conditions**. Medications that prolong the QTc interval are contraindicated for those living with LQTS as they increase the risk of events. Those living with LQTS may not be able to receive treatment for other medical or psychological conditions that severely impact their daily lives or for conditions that may be life threatening.
- **Some individuals living with LQTS require multiple medications and devices.** Some were even treated from birth. These include beta blockers, ICDs, lifestyle changes including avoiding sports, counseling and psychotherapy, antidepressants and anti-anxiety medications, left cardiac sympathetic denervation (LCSD), as well as many other medications and devices.
- **While some LQTS treatments and lifestyle choices have saved lives, none are curative.**
- **LQTS treatments further compromise quality of life**. Burdensome treatment-related side effects such as fatigue are an experience for many, followed by anxiety and PTSD. Some have many surgical procedures, accompanied by scarring and disfigurements.
- **The LQTS community needs a cure.** Treatments that prevent arrhythmias, increase energy and improve quality of life are urgently needed. Other important treatment considerations and community needs include less painful and less invasive treatments, safe removal of existing implanted devices, more LQTS research, and more information about QTc prolonging medications. As many LQTS treatments are already risky, many would sustain additional risk for themselves, but not their children, for a new treatment.

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Abbreviations

These abbreviations are used throughout the report.

AED - automated external defibrillator

AICD - automatic implantable cardioverter defibrillator

CPR - cardiopulmonary resuscitation

ECG/EKG - electrocardiogram

ICD - implantable cardioverter defibrillator

LSCD - left cardiac sympathetic denervation

LQTS – long QT syndrome

PTSD – post-traumatic stress disorder

SCA – sudden cardiac arrest

SICD - subcutaneous Implantable Cardioverter Defibrillator

SVT - supraventricular tachycardia

VF or **VFib** - ventricular fibrillation

VT or vtach - ventricular tachycardia

Long QT Syndrome (LQTS) Clinical Overview[1]

> *"Long QT Syndrome is a disease that can cause a dangerous rapid heart rate and irregular rhythm involving the bottom pumping chambers of the heart. It usually is a lifelong condition. The risk of having an abnormal heart rhythm that leads to fainting or sudden cardiac arrest may lessen as you age. However, the risk never completely goes away."* - Nicki, living with LQTS

What is Long QT syndrome (LQTS)? LQTS is a genetic heart condition caused by variations in genes encoding ion channels in the heart. LQTS is characterized by a prolonged QTc interval, which can cause torsades de pointes, an uncommon and distinctive form of ventricular tachycardia (VT) and if not managed can progress to ventricular fibrillation (VF), syncope (fainting), seizures, and sudden cardiac death. The majority of individuals (70%) diagnosed with LQTS are asymptomatic. Many are diagnosed after a cardiac event of a family member. Some diagnosed through genetic testing do not demonstrate QTc prolongation.

Who is affected by LQTS? LQTS is the most common channelopathy, affecting 1 in every 2000 individuals across the globe, the number of individuals affected may be much higher. As LQTS is genetically inherited, many members of the same family may be affected. LQTS symptoms can differ from one person to another, even within the same family, some may not have any symptoms.

What are the genetic causes of LQTS? The first causative gene variant for LQTS Type 1 was discovered in the voltage-gated potassium channel in 1995. LQTS Types 1-3 are the three major genotypes, accounting for 80% of all LQTS. Approximately 30-40% have LQTS Type 1 (*KCNQ1/KVLQT1* gene variants), 25-30% have LQTS Type 2 (*KCNH2/HERG* gene variants) and both involve variants in potassium channel genes. Approximately 5-10% have LQTS Type 3 (*SCN5A* gene variants), which involves variants in a sodium channel gene. Currently, 17 different LQTS genotypes have been identified, but types 4 – 17 are very rare. LQTS was previously called Romano–Ward syndrome (RWS) or Jervell and Lange-Nielsen syndrome (JLNS).

Each of the different LQTS types is characterized by unique ECG (electrocardiogram) patterns, specific triggers and displays a different response to beta blockers. LQTS Type 1 is triggered by exercise adrenaline, while LQTS Type 2 is triggered by auditory sounds; the postpartum period is a uniquely challenging time for young women with the LQTS Type 2. Times of non-activity, for

[1] This clinical overview was extracted from the presentation made at the June 11, 2024 LQTS EL-PFDD meeting by Dr. Michael Ackerman, MD, PHD. Dr. Ackerman is an internationally recognized LQTS expert, a genetic cardiologist and the director of Mayo Clinic's Windland Smith Rice Heart Rhythm Clinic, and Windland Smith Rice Sudden Death Genomics Laboratory in Rochester, Minnesota. Dr. Ackerman serves as President of the board of trustees for the SADS Foundation.

example sleep, can be the most common setting for events for those living with LQTS Type 3. Even with these differences, there is considerable overlap in triggering events among the different types.

What are the current LQTS treatments? Because of the potentially life-threatening manifestations of LQTS, most individuals diagnosed with LQTS receive treatment, even when asymptomatic. There are no FDA-approved targeted treatment for this condition, and although existing treatments are very effective, they come with a very steep cost including significant treatment-related side effects.

- **Beta blockers** are the mainstay of treatment, but response varies depending on the LQTS subtype: therapeutic efficacy is greatest in LQTS type 1 but less so in types 2 and 3. Beta blockers can cause fatigue, light-headedness, depression and weight gain.
- **Sodium channel blockers** can be used to treat certain arrhythmias, but can lead to fatigue, light-headedness, GI distress and vomiting.
- I**mplanted cardioverter-defibrillators (ICDs)** shock the heart out of a potentially fatal rhythm. An ICD was generally recommended in those patients who survived a cardiac arrest, however ICDs are over-implanted and complications include life-saving shocks which can lead to anxiety, depression and PTSD (post-traumatic stress disorder). The ICDs can malfunction and be recalled, can result in infections. Treatment recommendations are changing and most individuals living with long QT syndrome no longer need, and should not receive, an ICD.
- **Left cardiac sympathetic denervation (LCSD)** is recommended for high-risk patients when beta blockers are not effective, tolerated or contraindicated, and for whom an ICD is contraindicated or refused. Patients may still experience breakthrough cardiac events as well as post-operative complications such as ptosis (drooping eyelid), neuropathic pain, and the inability to sweat on the upper left side of the body.

What are the newest LQTS treatment approaches?

- **Non-treatment** is a newer approach that balances risk with minimizing unwanted daily side effects. This includes preventative measures such as regular monitoring, electrolyte replenishment, and avoidance of QTc prolonging medications. These patients must only engage in athletic activities under expert guidance. Dr. Ackerman estimates that 5-10% of his patients are currently untreated.
- **Tailored therapy** recommends specific therapies based on the LQTS genotype and aims to help patients live and thrive despite their diagnosis. Currently, more than 20 distinct strategies are being tested. The challenge is to help these patients live and thrive despite their diagnosis. Sudden death rarely happens in the well-diagnosed, well-characterized, well-treated patient.
- **New therapeutic approaches being tested** include a gene therapy (currently being tested in a rabbit LQTS model) and a new drug in clinical trials to address the QT shortening effect.

LQTS EL-PFDD Meeting summary

The Long QT Syndrome (LQTS) Externally-Led Patient Focused Drug Development (EL-PFDD) was held virtually on June 11, 2024. The meeting was an important opportunity for the Sudden Arrhythmia Death Syndromes Foundation (SADS Foundation) to share patient perspectives regarding the symptoms and daily impact of LQTS, as well as current and future approaches to therapies. The meeting was cohosted by **Genevie Echols**, RCIS, Family Support Director, the SADS Foundation, and **James Valentine**, JD, MHS from Hyman, Phelps & McNamara, P.C.

Genevie Echols opened the meeting by welcoming all meeting attendees including the members of the US Food and Drug Administration. She introduced **Shetarra Walker**, Clinical Team Leader in Pediatrics in the Center for Drug Evaluation and Research at the FDA. In her opening remarks from the FDA, Dr. Walker described how this EL-PFDD meeting will help the FDA to obtain insights from LQTS experts, the caregiver and patients living with the disease, and thanked the LQTS community for sharing their personal stories, experiences, and perspectives.

Dr. Michael Ackerman, MD, PhD an internationally recognized LQTS expert, and President of the board of trustees for the SADS Foundation, presented a clinical overview of LQTS which served as a foundation for the first half of the meeting. **James Valentine** provided an overview of the meeting structure and invited all individuals living with LQTS to contribute their voices through online polling, calling in by phone, and to contribute written comments using the online portal. The meeting agenda is in **Appendix 1**.

The LQTS EL-PFDD meeting was held immediately before the Catecholaminergic Polymorphic Ventricular Tachycardia (CPVT) EL-PFDD meeting. Collectively, 240 unique viewers attended the livestream for the two events. This included 59 individuals living with LQTS, 29 family members, 30 parents/caregivers, 16 healthcare providers, six from the government, nine scientists and researchers, 17 from the healthcare industry, 16 healthcare providers, six from non-profit organizations and 18 others including friends, patient advocates and consultants.

EL-PFDD meeting attendees used online polling to indicate meeting demographics. The vast majority of attendees were individuals living with LQTS (78%), and the rest were caregivers of someone living with LQTS (23%). The majority of attendees were from the United States (90%), and the rest were from Europe (10%). Almost half of the individuals living with LQTS represented at the meeting were between 36 - 50 years of age (44%), followed by individuals aged 51-60 years (17%), then equal percentages in the 19-35 age range and over 61 years of age (12%). A small percentage were between 6 – 18 years of age (15%). None of the individuals living with LQTS represented at the meeting were younger than 5 years of age. LQTS can be diagnosed throughout the lifespan; while over a quarter of those attending the meeting were diagnosed between 36 - 50 years of age (26%), other individuals were diagnosed with LQTS between 0-2 years of age, all the way up to 60 years of age. Results of demographic polling are shown in **Appendix 2.**

The LQTS EL-PFDD meeting was structured around two key topics. The first topic focused on *Living with LQTS: Symptoms and Daily Impacts,* and the second topic focused on *Current and Future Approaches for LQTS Treatments.* Discussion topics are listed in **Appendix 3**.

The first session opened with a pre-recorded panel of individuals who shared patient and caregiver perspectives on the symptoms and daily impacts of LQTS. James Valentine moderated a discussion between individuals in a live Zoom panel as well as those who dialed in by phone, and Genevie Echols read out relevant comments entered through an online portal. Meeting panelists and callers are listed in **Appendix 4.**

The second session commenced with a pre-recorded panel of patients and caregivers who described different medications and medical treatments as well as other approaches they use to address LQTS manifestations. Patients, family members and caregivers described all the different treatments, devices and approaches used to manage LQTS-related symptoms, the most significant drawbacks associated with each approach and their hopes for a future LQTS treatments. Again, meeting attendees had an opportunity to participate in online polling, by calling in and submitting written comments.

To conclude, **Larry Bauer, RN, MA** Hyman, Phelps, & McNamara provided a summary of key points. Genevie Echols closed the meeting by thanking all of the meeting attendees.

Additional patient comments were collected through the registration portal and an online comment submission portal was open for four weeks after the meeting to include as many patient voices as possible. All submitted patient comments are included in a separate PDF document, with selected comments included in the body of this report.

The SADS Foundation offered a Zoom support group immediately following the EL-PFDD meeting, as the meeting was anticipated to bring up strong emotions for those whose lives are affected by LQTS.

The LQTS *Voice of the Patient* Report

This *Voice of the Patient* report is provided to all LQTS community supporters including the US FDA, other government agencies, regulatory authorities, medical products developers, academics, clinicians, and any other interested individuals. The input received from the June 11, 2024, EL-PFDD meeting reflects a wide range of LQTS experiences, however not all symptoms and impacts may be captured in this report.

The final report, the accompanying document containing the submitted comments and a video recording of the meeting are available on the SADS Foundation website at https://sads.org.

Note that all abbreviations used in this report are defined the first time they are used.

Living with LQTS: Symptoms and Daily Impacts

LQTS can result in premature death from cardiac arrest

At the June 11, 2024 LQTS EL-PFDD, many shared heartbreaking stories about loved ones passing away prematurely from LQTS-related causes. As LQTS is a genetic condition, many members of the same family can be affected. Some suffered the loss of multiple family members including their children, parents and their siblings.

> *"I lost my dad to Long QT syndrome when I was just 11 years old. My dad was 39. As a child, I struggled with the impact of losing him at such a young age. … My dad's sudden death was a mystery to us for many years until a genetic test revealed that several relatives, including my brother, sister, aunts, uncles, and cousins, all had the marker for LQTS."* - Tom, 40-year-old living with LQTS, a family member of others living with LQTS

> "*My brother's death was a very defining moment in my life, and I feel like a large part of my childhood ended on that day. I was only nine years old, but I became very vigilant and almost fearful of doing anything in my life that could possibly overexcite or lead to syncope and/or sudden death, both things that I was cautioned about as a child with LQTS in the late 1990's."* - Molly, 35-year-old living with LQTS, parent and a family member of others living with LQTS

> *"I just lost my 15-year-old son due to LQTS Type 3. He was diagnosed with the mutated gene and being monitored each year with an EKG* [electrocardiogram], *but as we all know that LQTS deaths can happen with no rhyme or reason or timeline or warning."* - Ruth, parent of a 15-year-old son who died due to LQTS Type 3

LQTS is a disease characterized by fear and worry, reflecting an uncertain future that may include a cardiac arrest, shocks, ICD failure and premature death.

Worry and fear was a key theme throughout the meeting. The top worries shared by individuals living with LQTS are listed below and illustrated with patient quotes.

Fear for the safety and future of family members who are living with LQTS.

Family members worry about their parents, siblings, children and others experiencing a cardiac event or premature death. Parents worried about always needing to know where an automated external defibrillator (AED) is located and worried whether their children will receive appropriately care should an event occur. Some worry that their children will eventually experience the same symptoms and side effects as they do.

> *"And as a family, truly, our biggest concern symptom-wise … is sudden death. That's it. It's just sudden death. And we live 100% of the time in a fight or flight mode. We do. We don't plan a lot long term because we know we have to get through today to get to tomorrow, and that's the life that a long QT patient lives, especially one with a severe case."* - Amanda, parent of a 13-year-old daughter diagnosed at birth with LQTS Type 3

> *"As a mom, I can't let go of that pit in my stomach that at any moment my kids have a risk of a spontaneous cardiac event including spontaneous death which causes me and the rest of the family incredible anxiety and distress."* - Georgia, parent of children living with LQTS and family member of others living with LQTS

Worries that medications and treatments will be sufficient or enough to prevent an event.

> *"She's on a beta blocker, that's great, but is that enough? So I have to always wrestle with whether just the beta blocker is sufficient. I fear that this may not be the case and we will find out that she needs an ICD because she has a sudden cardiac arrest and is revived. It could be that we found out that it wasn't enough."* - Melissa, living with LQTS Type 2 and parent and family member of others living with LQTS Type 2

> *"My grandson was born last August, and he also has long QT and his is symptomatic. …I worry about him. I worry that something will happen with him despite his treatment*." - Julie, living with LQTS Type 2, parent and family member of others living with LQTS Type 2

Fear of shocks, of the ICD failing and the need for more surgeries.

> *"Knowing that other surgeries are going to be coming to replace the defibrillator, creates a lot of anxiety and you learn to work through it and to be able to just live your life regularly."* - Lee, 37-year-old, living with LQTS Type 2

"My ICD was placed behind my pectoral. There are always the fears of the recalls, malfunctions, inappropriate shocks, lead fractures, infections that can go all the way through the rest of your life. … The anxiety that comes with everything. … Dr. Ackerman said there's an over-implantation rate, and I also believe there is an over replacement rate." - Callie, 46-year-old living with LQTS Type 1

Worry about the ability to start their own family and passing this on to future children.

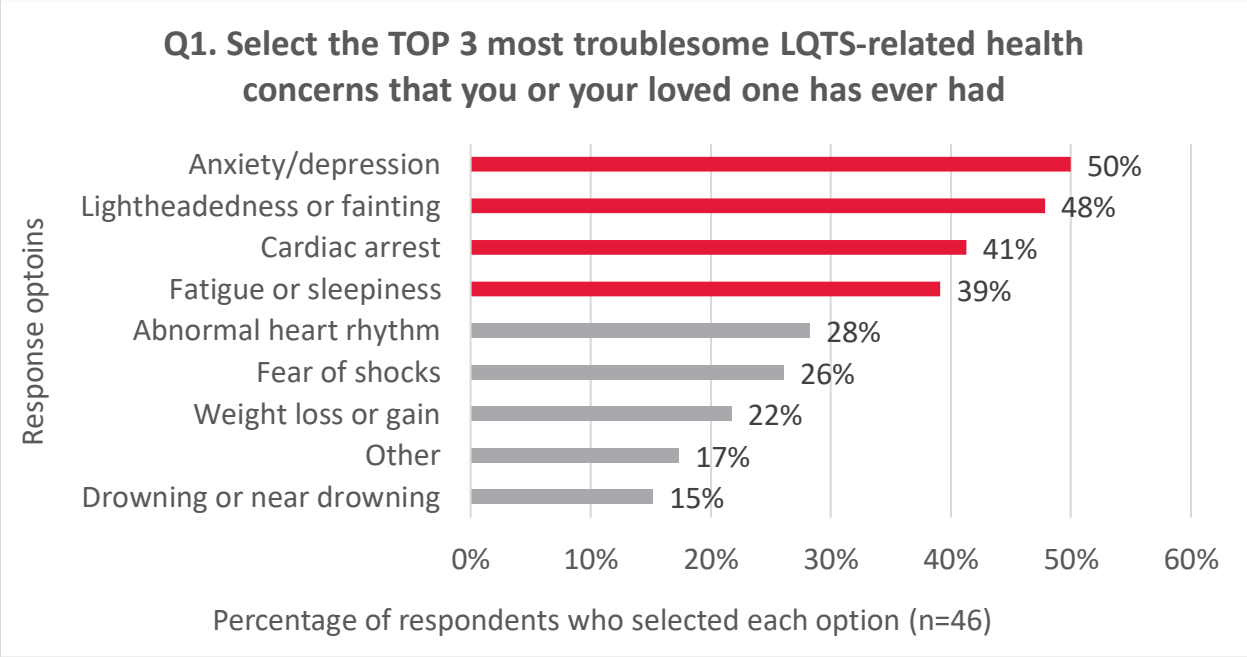
"My biggest fear with having biological children was that I might have to experience the same devastation of losing a child to LQTS as my parents did when my younger brother passed away. I would not wish that on anyone." - Molly, 35-year-old living with LQTS, parent and family member of others living with LQTS

Worries about an unknown future.

"Dealing with a rare disease is life changing for anyone. Dealing with a rare disease when it hits you and your children is a hurt that I would not wish on anyone. From the 'what ifs' and not knowing for a long time and trying to figure out how to navigate life dealing with the fear is beyond belief." - Amy, living with LQTS Type 8 and parent of a child living with LQTS Type 8

Anxiety and depression, light-headedness and fainting, cardiac arrest and fatigue are the most burdensome LQTS-related health concerns.

Meeting attendees used online polling to select the top three most burdensome LQTS-related health concerns that they or their loved one has ever experienced. Poll results are shown below and illustrated with selected patient quotes.



Poll respondents include patients, parents and caregivers who chose to participate in online polling. The number of individuals who responded to each polling question is shown below the X axis (n=x). The responses for these polling questions are not considered scientific data but are intended to complement the patient comments made during and after the meeting. Poll responses selected by more than 33% of poll respondents are highlighted in **red**.

Anxiety, depression and fear of shocks

As previously described, LQTS involves a life of fear and worry, as well as anxiety and depression. Many experience panic attacks and post-traumatic stress disorder (PTSD) because of previous cardiac events and ICD shocks. Exercise and activity restrictions further increase anxiety and depression.

> *"Unfortunately, the first symptom that Rebecca had was sudden death, which is not uncommon for this disease. Our household had changed, everything changed. Uncertainty was my constant shadow. Were we going to lose Ellie too? Although we were on medication, the sudden death of Rebecca always loomed for me, anxiety was my new pesky roommate."* - Lene, 52-year-old living with LQTS and parent of two daughters living with LQTS including Rebecca, who passed away at four and a half years

"I can so relate to the worry and the family impacts because I live that with my own family and seeing that how much the anxiety transcends not only the person who lives with the condition, but to the family as well, maybe more so even with the family. … Back in the day, this was 1989, and we didn't have AEDs in airports and rescue measures that we do now, and my family had to be trained in CPR [cardiopulmonary resuscitation] *before I was able to leave the hospital, and so that just kind of embeds this fear into the process."* - Kristin, 52-year-old living with LQTS Type 2

"As well as the symptoms and side effects of the medication, it should not be underestimated how living with LQTS can impact a person's mental health. At times it can feel like you're a ticking time bomb waiting to go off." - H., living with LQTS Type 1 and parent of two children living with LQTS Type 1

Light-headedness or fainting

Many living with LQTS experienced multiple episodes of light-headedness and fainting. These can be dangerous, especially when driving, swimming or engaged in outdoor activities like hunting or horseback riding. Many experienced concussions or other injuries as the result of fainting. For many, light-headedness and fainting were symptoms that were initially dismissed. Others were investigated for neurological issues or misdiagnosed with seizure disorder or epilepsy.

"When I was 18 years old, I experienced two episodes of sudden syncope. During one episode, I was driving and rolled my car several times before stopping in the median, which was a high hill, enough to keep me from rolling into the other side of the highway. In 2021, I experienced a non-sustained cardiac event after taking a medication I did not know LQTS patients should not take." – Patrei, living with LQTS Type 1

"As a child, I experienced approximately 10 fainting/sudden loss of consciousness episodes during competitive and energetic physical activity ranging from relay races in gym class to volleyball games to trips to our local pool. By age seven, these events led to an incorrect diagnosis of a seizure disorder and corresponding treatment." - Meagan, 37-year-old living with LQTS, parent and a family member of others living with LQTS

"Kayleigh experienced a couple of fainting and lightheaded experiences as a child. Again, though mentioned to doctors, these episodes weren't seen as concerns. ... While at University, Kayleigh had a seizure in her sleep. The doctor said it could have been due to exam stress, but referred her to testing for epilepsy. I worried, but trusted the doctor. Six weeks after her seizure, Kayleigh died in her sleep. She was 19." - Clare, living with LQTS Type 2, parent and a family member of others living with LQTS Type 2

Cardiac arrest

A sudden cardiac arrest is the first LQTS symptom that many experience, often resulting in premature death. Even the very fit and very young can experience cardiac arrests and some experience many throughout their lives.

> Rebecca's cardiac arrest occurred just after Christmas. "*After about one hour of resuscitative procedures, Rebecca's heart is shocked into rhythm, but her brain had been without oxygen for too long and 24 hours later, we made the heart-wrenching decision to take Rebecca off of life support. Our little girl will never get to come home again. …I often get asked if I ever felt symptoms or if my daughter showed any signs or symptoms. I'm afraid the answer to that is no."* - Lene, 52-year-old living with LQTS and parent of two daughters living with LQTS including Rebecca, who passed away at four and a half years

> *"Cardiac arrest events are painful, scary, traumatic, and can be emotionally paralyzing. They affect the patient, the parents and also the siblings. These events occurred during everyday life, on vacation, on holidays, following surgeries, etc. They are so unpredictable."* - Amanda, parent of a 13-year-old daughter diagnosed at birth with LQTS Type 3

Fatigue or sleepiness

Fatigue and sleepiness can result from treatment-related side effects. These side effects severely impact quality of life.

> *"My biggest symptoms, besides the near drowning experience and cardiac arrest at a young age, have been exhaustion, shortness of breath, and the restriction or inability to exercise or do most athletic activities. … I struggle living life as a young adult as most young adults have energy that I can never imagine. I struggle to have the career, social life and ambitions as those without electrical disorders who do not suffer from sever exhaustion."* - Annie, living with LQTS Type 1

> After her brother passed away from LQTS, *"I was also put on whopping doses of beta blockers, which I believe led to a constant feeling of fatigue and always being tired. I was still a kid, but I felt like I couldn't be a kid and the threat of LQTS was always over my shoulder."* - Molly, 35-year-old living with LQTS, parent and family member of others living with LQTS

Abnormal heart rhythm

Meeting attendees described a range of abnormal heart rhythm manifestations including: arrhythmias; atrial fibrillations; supraventricular tachycardia (SVT), an irregularly fast or erratic heartbeat; torsades de pointes; electrical storms; premature ventricular contractions; murmurs; chest fluttering; bradycardia.

After LQTS forced Mona to leave her firefighting career, *"I began a new career as a flight paramedic. And while on duty in 2008, I received a shock, followed by another shock. I was taken to the local hospital, and they found my potassium at 3.4. I was told by my cardiologist that the device recorded torsades de pointes tests followed by SVT."* - Mona, 56-year-old living with LQTS Types 2 and 5

"I've been fortunate in the sense that I've been generally asymptomatic. I have had a lot of heart troubles through the past, whether they be murmurs, arrhythmias, just sentiments from doctors that something was going on with my heart that I needed to keep an eye on, but it was very unclear." - Lorena, 39-year-old living with LQTS and parent of a nine-month-old son living with LQTS

Weight loss or gain

Many described weight changes because of exercise restriction or as a medication side effect.

"I am on exercise restriction I have piled on over 18kgs in weight. My doctor doesn't know what to do for the best and am now talking about LCSD surgery or implanting a pacemaker so I can take beta blockers. … My quality of life has decreased massively. I used to be fit healthy and happy now I am miserable, tired and depressed." - Sharon, living with LQTS Type 1

Drowning or near drowning

Cardiac arrests or fainting in the water result in drowning or near drownings.

"Unfortunately, my own diagnosis at age 11 was preceded by the sudden death of my younger brother; he was 7 years old at the time. He passed out in a pool a few hours into a family vacation and was pronounced dead at the local hospital a short time later. This experience was traumatic and had a massive impact on my parents and two younger sisters and our family dynamics." - Meagan, 37-year-old living with LQTS, parent and a family member of others living with LQTS

Michelle's first cardiac arrest happened during a swim meet. *"I touched the side and slowly sank to the bottom of the pool while my team celebrated. My coach dove in, and she pulled me out. Over 20 minutes of compressions were given until the ambulance arrived and delivered several therapeutic shocks. It wasn't a pretty recovery, with two weeks in a coma, followed by the usual brain recovery therapy. However, I was 16, and youth was on my side. I was diagnosed with having fainted in the pool. Long QT syndrome wasn't on the radar in those days."* - Michele, 56-year-old living with LQTS, and parent of a 12-year-old daughter living with LQTS

Other LQTS-related heath concerns

Other LQTS-related health concerns include **seizures** and **anoxic impacts** as well as sexual dysfunction, and hearing loss (Jervell and Lange Nielson Syndrome).

Seizures. Some living with LQTS are misdiagnosed with epilepsy. Unfortunately, some of the medications to treat epilepsy can prolong the QTc interval.

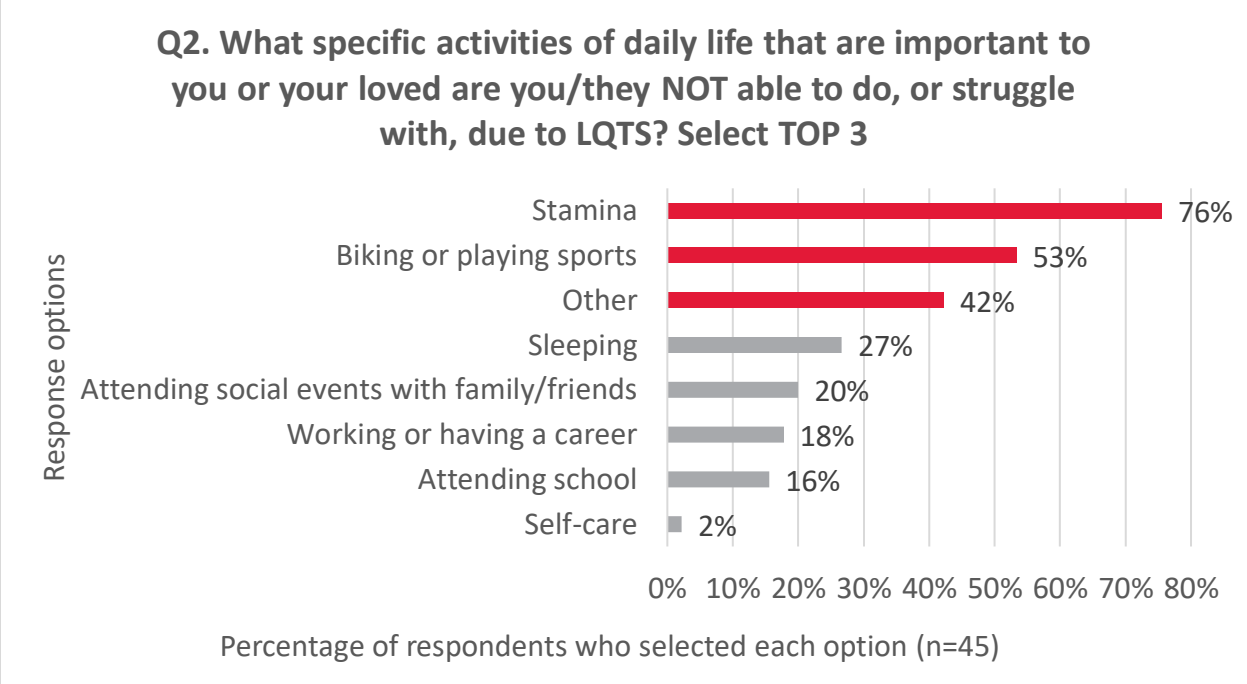
> *"My son had his first seizure on his third birthday whilst playing with his new remote-control car. It came out of nowhere like an explosion and was terrifying to witness. It lasted perhaps a minute before he came around but during the seizure he looked to me like he was dying. … Unfortunately, my little boy was misdiagnosed with epilepsy and given the wrong medication."* - H., living with LQTS Type 1 and parent of two children living with LQTS Type 1

Anoxic impacts. Cardiac arrest can limit the oxygen that goes to the brain, resulting in cognitive impairments, motor challenges and learning disorders.

> *"After suffering traumatic brain injury from the strokes he suffered on both sides of his brain during the sudden cardiac arrest, Tyler received an ICD and physical therapy for months. Around 9 months after his aborted cardiac death, Tyler was honorably discharged from the United States Navy as a 100% disabled veteran."* - Sherri, parent of two sons living with LQTS Type 3

LQTS diminishes quality of life.

Using online polling, meeting attendees selected the top three activities that were most important to them, that they were NOT able to participate in, to the degree they would like, due to LQTS. The results of this poll question are described with patient quotes below.



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Stamina

Fatigue is a treatment-related side effect. As a result, those living with LQTS have a hard time keeping up with their family members and peers in school, at work, or during recreation.

> *"After starting treatment, Slade no longer had the stamina equal to his peers. This led to frustration, a loss in his desire for sports, which led to an out of shape kid, in turn led to a lack of self confidence and overall unhappiness."* - Sherri, parent of two sons living with LQTS Type 3

> *"My children get tired easily and can have exercise intolerance. I myself take a beta blocker and have noticed fatigue and brain fog."* - H., living with LQTS Type 1 and parent of two children living with LQTS Type 1

Biking or playing sports

Most living with LQTS prioritize their health and engage in athletic activities with some precautions such as watching their heartrate or being mindful of electrolytes. Although exercise

is safe for the majority of those with LQTS, some continue to be restricted by their physicians from physical activity and exercise.

> *"Having Long QT syndrome made me feel boxed in. … With it came strict rules, no strenuous activity, no heavy lifting and absolutely no contact sports. … My family changed electrophysiologists to one who is much more forthcoming and also a runner. He supported me in testing my running limits."* Tom is now part of a running club, carries a wallet card for emergencies, and ensures he is adequately hydrated. *"Thankfully, in over 15 years of running, I remain incident free"* - Tom, 40-year-old living with LQTS, family member of others living with LQTS

> *"Running and gym life is my entire life, it's the only thing I want to do, and I cannot do it in the same capacity. The ICD restricts me from many activities at the gym because the impact would damage the device and fracture lead wires and the nadolol restricts my cardio activity. I used to run an easy eight-minute mile and now I can only run a very uncomfortable 13-minute mile. It is beyond comprehension that this is how I will be living the rest of my life."* - Patrei, living with LQTS Type 1

Other LQTS impacts: family planning, pregnancy and birth.

Treatment may begin at birth for diagnosed infants.

> "*I have had a pacemaker implanted at 48 hours old*." - Isabella, 27-year-old living with LQTS and parent of a son living with LQTS

> *"On day two of life, Isla received a pediatric pacemaker to prevent her heart rate dropping too low when she started on beta blockers."* - David, parent of Isla who passed away at six years from LQTS Type 3

Some living with LQTS made the difficult decision not to have an additional child, while others used in vitro fertilization (IVF) to ensure that they would not be transmitting the gene to their children.

> Suzy's daughter's LQTS was detected in utero. "*After the 24-week ultrasound caught her impartial heart block we went in for two to three ultrasounds a week. My Long QT put her at risk of miscarriage, premature delivery, or stillbirth. Possibilities that even today make me sick with worry to contemplate."*- Suzy, 38-year-old living with LQTS, and parent of a three-year-old daughter living with LQTS

> *"Prior of the knowledge that my condition is genetic I had one child with LQTS2. I chose not to have another because I did not want to pass on the gene."* - Grace, living with LQTS Type 2 and parent of a child living with LQTS

Sleeping

Individuals living with LQTS Type 2 need to be mindful of ensuring that they have excellent sleep hygiene and are not startled by alarms or other loud noises when they are sleeping or it may trigger an event.

> *"He has dealt a lot with sleep challenges and major migraines, so it definitely affects many different aspects of your emotional health as well as physical."* - Kristi, wife of man living with LQTS Type 2 and parent of children living with LQTS Type

> "*For me, sleep is an area that I really have to protect because my heart gets really agitated if I don't sleep well, and I really feel the consequence of that. … I have type 2 long QT, so I haven't had an alarm clock in 35 years. … The time of sleep and being startled from sleep as being a trigger, so I have no ringtones on my phone, and I have no alarm clock."* - Kristin, 52-year-old living with LQTS Type 2

Attending social events with family/friends

Many living with LQTS miss out on important social events and activities because of the risk that they, or their child, will have a cardiac event. Some are too tired to attend social events, and others avoid them because they feel stigmatized for carrying around an AED. Individual living with LQTS miss out on many activities including summer camps, sleep overs, pool parties, amusement park rides, haunted houses, saunas and spending time in the pool.

> Lene had to be extra vigilant when her kids were going to sleepovers and birthday parties. *"I didn't just need to know [the host family], I had to ask them so many more questions. Do they know CPR? Are they willing to go to the extra step to ensure my child's safety? Do they know how to operate an AED?"* - Lene, 52-year-old living with LQTS and parent of two daughters living with LQTS including Rebecca, who passed away at four and a half years

> *"LQTS impacted all areas of our lives. No sport to play or view of our teammates, no haunted houses no hide and go seek. No loud sudden noises."* - Susan, living with LQTS and a family member of others living with LQTS

> *"I could not participate in ordinary children and young adult experiences. I was the kid who missed out on amusement park rides, water parks, ski trips, and avoided pool parties. … Since my early 20s, I have been the friend who leaves the party early and cannot "rally" or stay up very late. I am the friend who is ready for bed after a glass of wine, and I am the friend who does not have the energy or who struggles to have the energy to go out after a day of work."* - Annie, living with LQTS Type 1

Working or having a career

Many living with LQTS have been forced to give up the careers that they loved, because of the risk of sudden cardiac arrest, treatment-related side effects, or disability. Others have had to make career choices based on whether they can obtain insurance.

> *"At the age of 35, I was a fit firefighter paramedic running several marathons a year."* After several of her family members were diagnosed with LQTS*, "I called my cardiologist and the only life-saving option for me at that time was an AICD [automatic implantable cardioverter defibrillator] placement. I scheduled the surgery, and as a result, I was now no longer fit to be a firefighter. My 18.5-year career was over."* - Mona, 56-year-old living with LQTS Types 2 and 5

> *"I own an equestrian facility and move thousand-pound horses daily. Nadolol makes it difficult to stay on task without periodic rest. Both the ICD and Nadolol have significantly altered the way in which I live my life. I suffer extreme exhaustion on the meds even on lower doses. … The ICD and beta blocker has finally led to me leaving a job I absolutely loved.*" - Michele, 56-year-old living with LQTS, and parent of a 12-year-old daughter living with LQTS

Attending school

Some living with LQTS have not attended regular school or have been unable to participate as fully as they would like. Many have had to carry their own AED to school or have a phone that is passed from one teacher to another.

> *"Ellie's kindergarten teacher was so worried that Ellie was going to have an episode at school that she made Ellie carry out her AED during recess. Her friends would ask her why she had two lunch pails."* - Lene, 52-year-old living with LQTS and parent of two daughters living with LQTS including Rebecca, who passed away at four and a half years

> *"As I grew older and entered college and higher education, I struggled to stay awake during lectures and to stay up late to finish readings or prepare for exams. Many times, I found myself falling asleep or dosing off when others had the ability and energy to diligently listen and put in the additional study hours. An energy drink or a few cups of coffee throughout the day could fix this issue for most, but not for me, as I had to restrict my caffeine intake to a cup or two of coffee a day."* - Annie, living with LQTS Type 1

Other impacts: LQTS can interfere with treatment for other conditions.

Medications that prolong the QTc interval can be contraindicated for those living with LQTs, as they make symptoms worse and increase the risk of events. As a result, those living with LQTS may not be able to receive treatment for other life-altering conditions.

Increased risk of events from taking medications that prolong the QTc interval

Many living with LQTS have unknowingly taken a medication that prolonged their QTc interval, leading to arrythmia or even cardiac arrest.

> *"I was diagnosed with long QT after an arrhythmia in 1998. I was also taking two cold medications at the time that I should not have been had I known it."* - Callie, 46-year-old living with LQTS Type 1

> *"In 2021, I experienced a non-sustained cardiac event after taking a medication I did not know LQTS patients should not take."* - Patrei, living with LQTS Type 1

> *"In 1984, my sister Leni, while in the shower, had a syncopal episode. She had been ill for some time and been put on erythromycin, common antibiotic. Because she was undiagnosed with long QT, she wasn't aware that this antibiotic could have potentially deadly effects for her."* - Mark, 61-year-old living with LQTS Type 2, parent and a family member of others living with LQTS Type 2

Undertreatment of other conditions and diseases

Due to medical misinformation, some living with LQTS are often denied treatment for attention deficit hyperactivity disorder (ADHD), anxiety, and depression, because the required medications prolong the QTc interval. This can impact quality of life. Some antibiotics are also contraindicated. As well, some patients spoke about being incorrectly denied an MRI or radiotherapy because of their ICD. Those living with LQTS are advised to speak to their physician about a treatment plan for other conditions. CredibleMeds® (https://crediblemeds.org/) is a reliable online resource about QT prolonging medications.

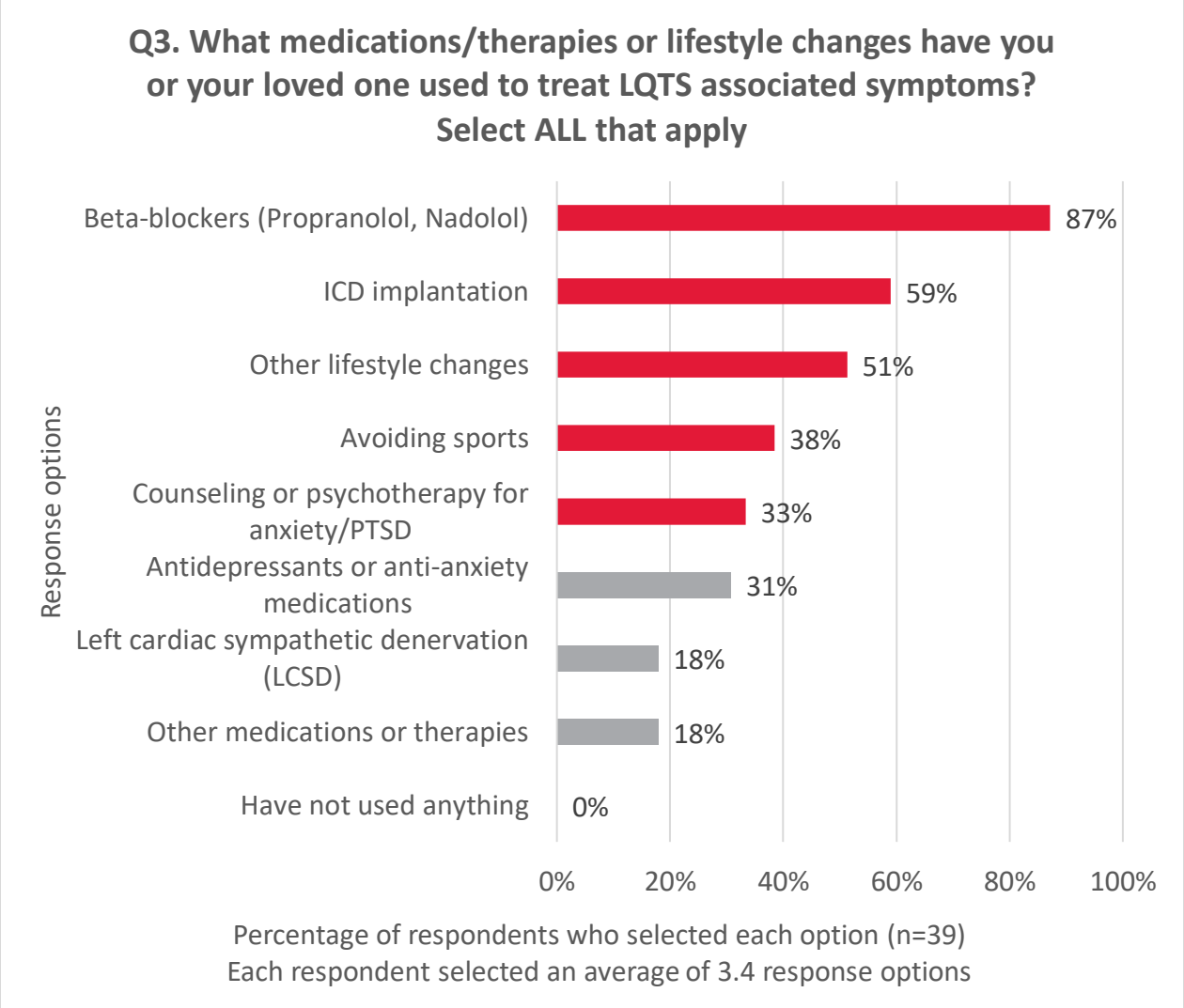
> *"I was diagnosed with breast cancer. Because of long QT syndrome and the ICD, I could not receive an MRI or radiation or lumpectomy. The only safe option was double flat mastectomy with scalpel, and that was quite risky."* - Mona, 56-year-old living with LQTS Types 2 and 5

> *"I have a couple of autoimmune diseases and the recommended medications for them I cannot take because they are known to prolong the QT. I also have migraine disease and there is not one migraine medicine I can tolerate to treat my headaches."* - Katrina, 64-year-old living with LQTS

Current and Future Approaches to LQTS Treatment

Most individuals living with LQTS require multiple medications and devices to manage their disease.

Using online polling, individuals living with LQTS indicated all the medical therapies that they had used to manage symptoms of LQTS. Each respondent selected an average of 3.4 different types of procedures or medications that they have used. Results are shown below and described with patient comments. In addition, the main downsides of each therapy are described.



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Beta blockers (propranolol, nadolol, metoprolol, atenolol)

Experts recommend the non-selective beta blockers nadolol and propranolol but urged avoidance of metoprolol and atenolol. These are the only medications that some living with LQTS require, however others need additional medications and treatments. Beta blockers are accompanied by significant treatment-related side effects such as depression, light-headedness, fatigue, weight gain, delayed reflexes, difficulty breathing, bronchoconstriction, feeling cold, Raynaud's, tremors, GI issues, alopecia, sleep issues, nightmares, and brain fog.

> *"I was on a large dose of beta blockers until the last few years. To this day, if I'm relaxing, I feel okay. But if I want to do something active like play with grandkids or anything else, the tiredness and sluggishness will affect me. To feel crummy from medications or to have no protection is not a great choice to have to make."* - Mark, 61-year-old living with LQTS Type 2, parent and family member of others living with LQTS Type 2

> *"The side effects of nadolol included fatigue, weight gain and low mood. As the mom of young kids, this was intolerable to me... I ended up trying lower and lower doses, which left me undertreated."* - Allison, living with LQTS Type 1 and parent of daughters living with LQTS

> *"I was alarmed by my unexpected LQTS diagnosis, and the beta blockers used to treat it turned me into the equivalent of a tree stump. I had no energy, no ability to get anything done, even on a small dose. I was given different betas, but the side effects only increased. I was exhausted and still had insomnia. When I could sleep, I would have awful nightmares (a side effect of propranolol)."* - Julie, living with LQTS Type 2, parent and family member of others living with LQTS Type 2

ICD implantation

For many years, an ICD was regarded as the standard of care to control arrhythmias. Many living with LQTS depend on their devices, yet ICDs have many downsides. Shocks - both appropriate and inappropriate - can lead to fears, anxiety and PTSD. Smaller sized individuals have challenges accommodating the devices. Many spoke of having permanent cardiac damage due to broken leads and other post-surgical issues. Device failure or replacement requires additional surgeries as described on page 33.

> *"The cardiology team … placed a permanent defibrillator in my chest. I hope to never have to use it, but it is like having insurance on my heart and my life, to help save me, to not ever go into cardiac arrest from this condition again."* - Nicki, living with LQTS

> *"Living with an ICD protects me from dangerous arrhythmias, but it's not without any mental toll. I experience anxiety knowing I live with a device that can shock me even when conscious and even by mistake. There are also aesthetic and sensory discomforts*

with living with an implanted device. There are medical risks with living with an ICD, the leads that connect the ICD to the heart chambers, and with ICD replacement surgeries that occur every decade to replace the battery. There are also lifestyle restrictions to living with an ICD, such as no contact sports." - Kiara, 26-year-old living with LQTS Type 3

"Because I weigh only 90 pounds it has proven impossible for me to keep a defibrillator where it is placed by my doctors. They always migrate, one even going so far as being wedged against the bottom of my sternum. My current device has slipped into my left armpit. I am left-handed and therefore every movement causes discomfort and some level of pain." - Christine, 73-year-old living with LQTS

Other lifestyle changes, including avoiding sports

Many have made significant lifestyle changes to adapt to LQTS, by getting appropriate exercise and enough sleep, avoiding triggers like caffeine and alcohol, taking all their medications as prescribed, and maintaining a good work-life balance. Some have to modulate or avoid athletic activities including sports as described on page 18. Others adapted by taking their AEDs wherever they go and by ensuring that there are AEDs in their workplaces and in their children's schools. Some work to raise awareness so that those around them will know what to do should an event occur.

"I call it work-health balance and being able to take time and be very intentional about how I live my life and have had to adapt in that way in order to minimize the consequence that long QT has in my life. …I try to focus on the things I have control over and the ways that my activity and my behaviors can influence what my heart does, so making sure I exercise and eat well and avoid things that like caffeine or alcohol or things that are known to have an impact on our heart rhythms and really being very mindful, especially when I have other health issues, to not take a medicine that might cause troubles with my heart." - Kristin, 52-year-old living with LQTS Type 2

"I am now very aware of how exercise affects my body and am more careful with my activities. I don't see LQTS as limiting me. It is more like I am able to pay attention to my body and not push myself to do things that do not feel right. I am still very active and love to exercise and do activities outdoors!" - Ana, living with LQTS Type 3 and a parent and family member of others living with LQTS Type 3

"Whether or not it helps, I work harder, try to keep my heart healthy and frankly my mental health in good shape, knowing you have a chronic condition." - Lee, 37-year-old, living with LQTS Type 2

Counseling or psychotherapy for anxiety/PTSD

Anxiety and PTSD can be major challenges for those living with LQTS. Many rely on the support of a counsellor or support groups.

> *"I started participating in the SADS Foundation ICD Support Group, and have been a part of that group for two years. I'm unique in the group in that I've had my device for so long without any shocks. I'm able to share my frustrations with the group while encouraging those whose devices have been lifesaving."* - Callie, 46-year-old living with LQTS

> *"The impact of a life-threatening condition is hard on the whole family. We have pursued counseling for our whole family since this diagnosis and it has been especially hard for our 12-year-old to process this kind of diagnosis."* - Kristi, wife of man living with LQTS Type 2 and parent of children living with LQTS Type 2

> *"Daily beta blockers, seeing various cardiologists, intensive self research, joining Facebook online support groups, psychology sessions, exercise physiology. The online support groups have helped me most. I have felt very alone and people in these groups have listened."*- Jessica, living with LQTS

Antidepressants or anti-anxiety medications

Many antidepressant or anti-anxiety medications are contraindicated for LQTS. As a result, some individuals remain undertreated for their anxiety and depression.

> *"I have been taking paroxetine for years because of panic attacks. After an ablation of the atria, sotalol was prescribed and I suffered a torsade de pointes tachycardia in this combination and had to be resuscitated. Both drugs were stopped immediately, and I was diagnosed with LQTS. I now suffer from a severe anxiety disorder that is becoming more and more pronounced and no doctor dares to give me medication for it (all QTc prolonging). The condition is almost unbearable and my quality of life is extremely limited." - Kertin, living with LQTS*

> *"Once I was prescribed Effexor for depression and it caused my defibrillator to fire repeatedly for hours. Fortunately, I have not had an episode in several years now."* - Andrea, living with LQTS and parent of a child living with LQTS

Left cardiac sympathetic denervation (LCSD)

The decision to have an LCSD is enormous. LCSD is a consideration for those who cannot tolerate beta blockers or for whom an ICD is insufficient. Some individuals living with LQTS even experience bilateral sympathectomies. LCDS treatment-related side effects include flushing and sweating on only the right side, ptosis (drooping upper eyelid) and neuropathic pain, which can sometimes be severe and persistent.

> *"Permanently altering my heart by removing the left sympathetic nerve chain was a big decision to make, one that we've now had to make for our seven-year-old daughter*

whose quality of life has drastically been reduced by beta intolerance. We also are in significant medical debt as a result." - Lea, 49-year-old living with LQTS and parent of seven-year-old daughter living with LQTS

"*I had denervation surgery, which there was nerve pain afterward, which I kind of didn't expect. But one lingering thing is that I have half of my face will flush and sweat when I'm hot and exercising and it's very noticeable, at least it is to me. I worry a little bit about looking a little strange, a little freakish.*" - Julie, living with LQTS Type 2, parent and family member of others living with LQTS Type 2

"My LCSD has had the most life-altering effects, I have suffered with severe 24/7 post-surgical neuropathic pain since four days after my surgery. The pain is immensely difficult to live with and majorly affects what I'm able to do. The pain is so bad that I was hospitalized for a month when I was 19 for attempting suicide. I couldn't imagine having to live with the pain forever, and I still can't, as it has never improved." - Alexis, 23-year-old living with LQTS Type 5 and caregiver for her mother living with LQTS

Other medications

Other medications needed by those living with LQTS include antiarrhythmic medications (flecainide), sodium cannel blockers (mexiletine), antiepileptic medications, and electrolytes (potassium, magnesium). Some described how they needed medication to treat LQTS-related symptoms. Some individuals have participated in clinical trials of new medications.

"I take a beta blocker and mexiletine. I have had several breakthrough episodes and appropriate ICD shocks. This has caused anxiety, PTSD, and depression. I struggle with all of these daily. The treatments have kept me alive but I wouldn't say I have the highest quality of life. But I am grateful to still be here." - Jessica O., 43-year-old living with LQTS

In addition to an ICD and beta blockers, *"I also take Dilantin, which is typically for epilepsy. But since the heart essentially has epileptic seizures, it was discovered that Dilantin was a great treatment for my heart because I was misdiagnosed with epilepsy before my big event at band camp."* - Kori, living with LQTS Type 2

"None of the drugs Isla took were created for long QT syndrome. Mexiletine is not a cardiac-exclusive drug, and although it helped reduce her QT interval by blocking the sodium channels in her heart, they also blocked them in her brain." - David, parent of Isla who passed away at six years from LQTS Type 3

Other devices

Some living with LQTS have pacemakers to control the heartbeat and loop recorders to record the heartbeat.

Suzy was one of the youngest individuals to receive a pacemaker implantation. "*Ultra-sound technology could not explain what was wrong with me before my birth, but they knew that when I arrived as a blue baby, something was very wrong.*"- Suzy, 38-year-old living with LQTS, and parent of a three-year-old daughter living with LQTS

After having the leads for his ICD removed, "*My wife and I chose just to have a pacemaker implanted rather than an ICD because I had gone 30 years without incident before the first arrest, and then another 20 years without incidents. … And three days after the surgery, I had another cardiac arrest in hospital, which I survived, and have thankfully done well since."* - Jay, 64-year-old living with LQTS Type 1 and parent of young adults living with LQTS Type 1

"I had a loop recorder placed. In Dec 2021 I was alone with the kids and was playing when I felt my heartbeat of my chest and I lost control of my body before losing consciousness, my loop recorder showed vtach and a BPM over 335; two days later I received a defibrillator." - Alli, living with LQTS and family member of others living with LQTS

Have not used medications or medical treatments recently

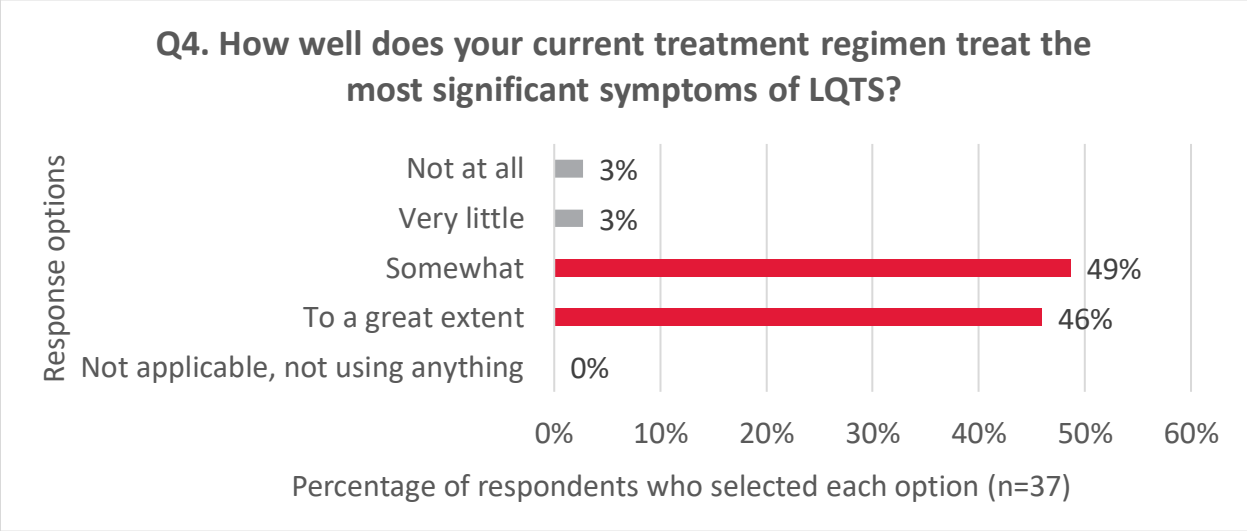
Reasons for not using medications or medical treatments include breast feeding, intolerance of treatment-related side effects, and some have not been offered treatment.

"As of now, I am not taking medication because I am breastfeeding. … Since my daughter is taking medication for LQTS, I cannot take medication in case this will impact my daughter." - Ana, living with LQTS Type 3, parent and family member of others living with LQTS Type 3

"I am currently not being treated for long QT because I could not tolerate the treatment, it made me crazy.. …I tried to take nadolol and I couldn't think straight. I was angry, I was depressed, I was tired. I could not function. … My husband tried taking beta blockers and it gave him arrhythmias that could have killed him, so he couldn't tolerate medication either. Neither one of us are currently being treated, but we don't have symptoms and we're middle-aged now." - Shauna, living with LQTS, parent of two daughters with LQTS including Britnee who passed away at 18 years from LQTS

While some LQTS treatments and lifestyle choices have saved lives, none are curative.

Individuals living with LQTS used online polling to indicate how well their current treatment regimen treats the most significant symptoms of LQTS. Poll responses are described below with patient quotes.



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For many, their current LQTS treatment works "to a great extent".

> *"All three of us are on beta blockers and I also have an ICD. And to be honest, we're all pretty tolerant of our current treatments. It's going okay. I've adjusted dosage with my beta blocker, but for the most part, I feel like it's not negatively affecting other parts of my body like you hear other medications could have. It's a daily pill we take by mouth, it's inexpensive."* - Melissa, living with LQTS Type 2 and parent and family member of others living with LQTS Type 2

> "*Overall, the treatments that I've had have been positive. … The medication is the first line of defense against any symptoms or potential arrhythmias that I may have. And then, the ICD is there as a backup. It has never failed. I've received a few extra ones where I should not have needed that, but it's never failed me, so that's great."* - Kori, living with LQTS Type 2

> *"They did the left cardiac sympathectomy in the hospital as soon as I was admitted. I haven't had a major event since."* - Isabella, 27-year-old living with LQTS and parent of a son living with LQTS

Half of individuals living with LQTS reported that their current treatment works "somewhat". Many acknowledge that the treatment comes with some trade-offs.

> *"We've had a variety of results with our treatments. …The ones that have worked the best, but also had serious side effect, is the ICD implant. For my daughter, Alyssa, it saved her life multiple times. However, my daughter Britnee, her surgery on her ICD took her life."* - Shauna, living with LQTS, parent of two daughters with LQTS including Britnee who passed away at 18 years from LQTS

> *"I don't experience those symptoms anymore. I sometimes experience abnormal heart rhythms and palpitations. I have gained life, stability, and peace from current treatment. … However, I do experience fatigue, headaches, anxiety, and depression, possible side effects of beta blockers."* - Kiara, 26-year-old living with LQTS Type 3

> *"I have been on beta blockers long-term (greater than twenty years) and I believe the medicine has protected me from having any major events due to my LQTS."* Although she experiences fatigue, decreased exercise endurance, icy cold fingers and toes,*"In the long run, those are small sacrifices I am willing to make to treat my LQTS."* - Molly, 35-year-old living with LQTS, parent and family member of others living with LQTS

Only a few indicated that their treatments work "very little" or "not at all".
For these individuals, the consequences can be tragic. For some, their treatment only treats some, not all symptom(s) and is not very effective at treating the target symptoms, consistent with the results of the next poll. Some even felt that they have exhausted all the treatment options.

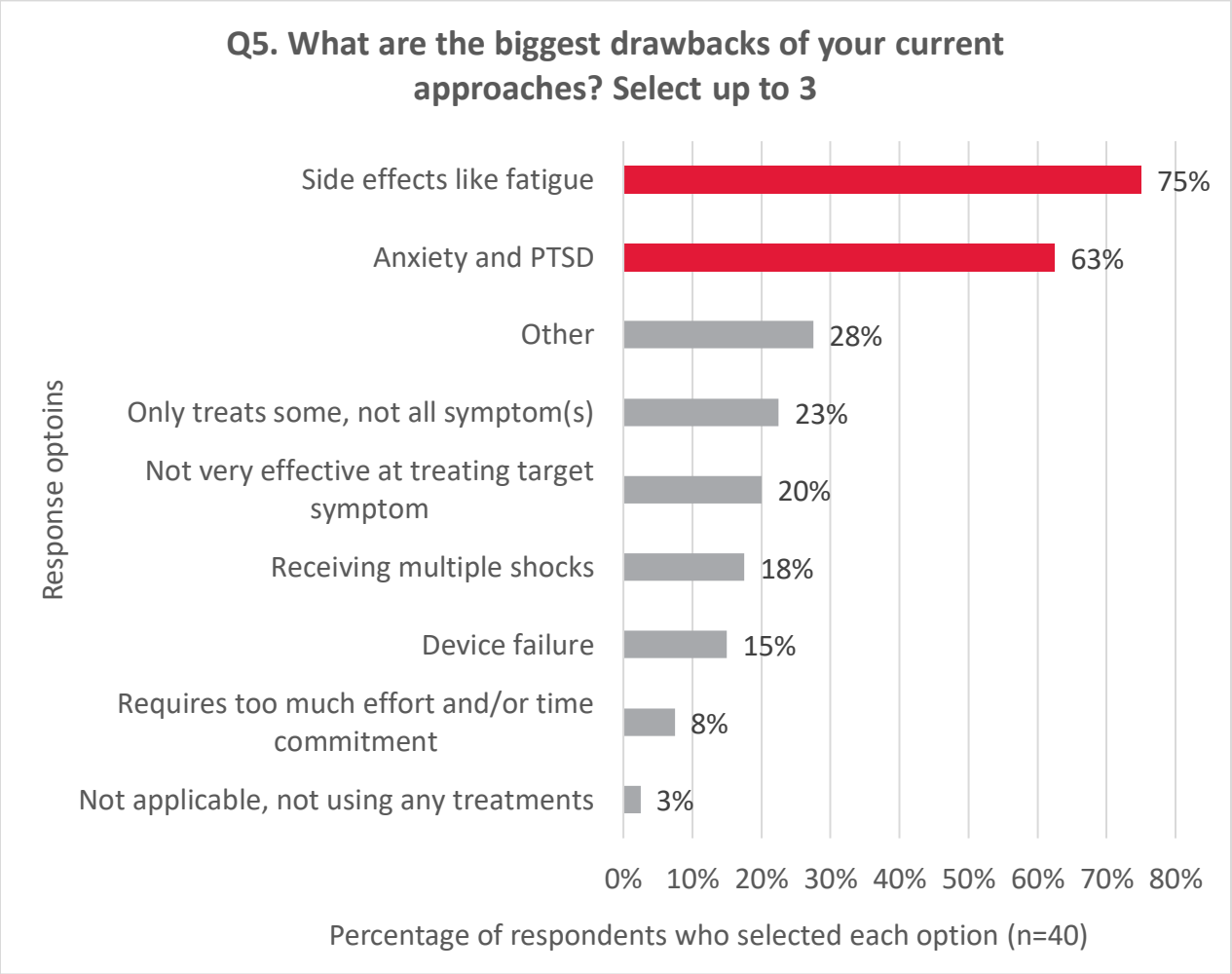
> *"I can't do anything strenuous; the defibrillator has been worse than the LQTS - it has negatively impacted me physically, emotionally, and financially. …Beta blocker has helped; defibrillator has not helped - it has made my life worse."* - Callie, 46-year-old living with LQTS

> *"We've exhausted all of the options. So, if her treatment reverses course and doesn't work for her, we're dependent on what's next, what's in the future for her to be able to continue to live and thrive with long QT."* - Meredith, parent of a daughter living with LQTS Type 2

> *"At age three, following a cardiac arrest at home requiring CPR, Isla's device was upgraded to a dual chamber ICD. …Even though Isla was on a variety of medications, she required 19 life-saving shocks, and CPR three times. … Some of the things that Isla had to go through and we had to endure as a family, I wouldn't wish upon anybody."* - David, parent of Isla who passed away at six years from LQTS Type 3

LQTS treatments further compromise quality of life.

Individuals living with LQTS selected the biggest drawbacks of their current treatment approaches. Not surprisingly, the top two selections were side effects like fatigue, anxiety and PTSD, which were mentioned throughout the meeting. However, participants indicated other important downsides, described with patient quotes below.



Poll respondents include patients, parents and caregivers who chose to participate in online polling. The number of individuals who responded to each polling question is shown below the X axis (n=x). The responses for these polling questions are not considered scientific data. These are intended to complement the patient comments made during and after the meeting. Poll responses selected by more than 33% of poll respondents are shown in **red**.

Side effects like fatigue

Treatment-related side effects like fatigue were described throughout the EL-PFDD meeting and in the comments. Other significant side effects can include headaches, dizziness, light-headedness, tremors, imbalance, blurred vision. Parents of the children who started taking medications early in life are concerned that their children may not be capable of informing their

parents of side effects. Parents were also concerned that medication side-effects may mask the true personalities of their children.

> Joanna wonders how beta blockers affect her five-year-old son. *"Does he have symptoms from the beta blockers he can't tell us about? He started on them so early, we didn't really 'know' him before, so it's hard for us to judge if they have any side effects."* - Joanna, parent of a five-month-old baby living with LQTS
>
> *"I have also witnessed significant side effects of medications in my son. It is awful as a parent to see your child be a whole new kid after days of a medication switch. … His medication change revealed a happy, energetic, funny little boy."* - Hilary, 44-year-old living with LQTS Type 1, parent and family member of others living with LQTS Type 1

Anxiety, PTSD and receiving multiple shocks

Those living with LQTS bear an emotional burden from the treatments, especially from ICD shocks.

> *"In the spring of 2011, I was running a half-marathon when I was defibrillated six times in a row. I was taken to the ER and advised that my heart was perfect, but the AICD was not set correctly for my activity level. I was shaking, crying, silently terrified of this device in my chest and of my heart. I felt helpless and powerless over my situation. I developed PTSD so badly that I was afraid to do more than a slight walk, and I began having phantom shocks so strong that I would awaken in the night and swear I had just been defibrillated. I was a mess. I was now ashamed of this, and I didn't know what to do."* - Mona, 56-year-old living with LQTS Types 2 and 5
>
> *"Probably the hardest thing I've had to deal with as a patient, was the one time my defibrillator has gone off, unclear if it was a proper shock, or an improper shock, but I certainly had PTSD after that and a lot of anxiety, and it took many years really to shed that."* - Lee, 37-year-old, living with LQTS Type 2
>
> *"I pretty much had no symptoms until I was 16 years old and I went into V-fib. I was shocked out of that. I had received probably 14 ICD [shocks], like a storm of shocks. I had six in the ambulance, six in the life helicopter, another one at the hospital. And that was very, very traumatic as a child, I'm still working through that."* - Isabella, 27-year-old living with LQTS and parent of a son living with LQTS

Only treats some not all symptom(s) and is not very effective at treating the target symptoms.

This is consistent with the results of the previous section where people indicated that their treatment works "a little" or "not at all". Throughout the meeting individuals living with LQTS described all of the trade-offs and compromises that they had to make with respect to treatment.

Device failure and multiple surgical procedures

Device failure is a major ICD downside. Leads can break off and devices can malfunction or be recalled. This results in the additional burden of multiple surgeries, scarring and disfigurement as well as time off work and additional medical expenses. Some have even chosen to have their device removed.

> Mona had new ICD wires implanted, *"However, the old wires had to stay because they're attached to my heart muscle. … Now I have the old broken wires in my chest forever connected to my heart. The SICD [subcutaneous implantable cardioverter defibrillator] in my left axillary with a wire that runs across my chest and not my sternum, on top of a barren chest with nerve damage and scar tissue. I cannot exercise the way I would like to due to the SICD popping on my ribs, and I cannot lay on my left side due to the pain. I cannot lay prone due to the pain from the wires. ... I'm now limited with movement and effort due to treatments, not the condition."* - Mona, 56-year-old living with LQTS Types 2 and 5

> *"I've gone through multiple surgeries to replace either the leads or the device. I went through a recall of one of my devices. I had fluid leaking in, I had broken leads. You name it, it probably happened. … In the 31 years that I've had this, I think I've had close to 12 to 15 surgeries, which is double the number of surgeries that someone should have with ICDs."* - Kori, living with LQTS Type 2

> *"On May 31st, I had my ICD and leads removed. It didn't go smoothly. Part of the lead popped off and went into my pulmonary artery. I had that removed by interventional radiology on June 4th. …. Since the surgeries, I had blood clots these past weeks. Blood clots, blood loss, low blood pressure, my color was gray, I have a lot of swelling. But like I said, it's high risk. I had antique parts inside of me that were embedded in my heart."* – Callie , 46-year-old living with LQTS Type 1

Requires too much effort and/or time commitment

Some of the medications are burdensome to administer, especially for very young children.

> *"My daughters are on different medications with different dosing frequencies - managing 6+ medication times across the three of us without missing a dose can be tiring but administration has gotten easier as my oldest has become more independent."* - Meagan, 37-year-old living with LQTS, parent and family member of others living with LQTS

> *"My son takes two medications for LQTS Type 3. One is twice a day and the other is three times a day. It's constant and overwhelming for him. … Mexiletine three times per day, nadalol twice per day. There is never an opportunity for him to sleep in, which he hates. Because he can't swallow a pill yet and the mexiletine has to be kept cold, we have to*

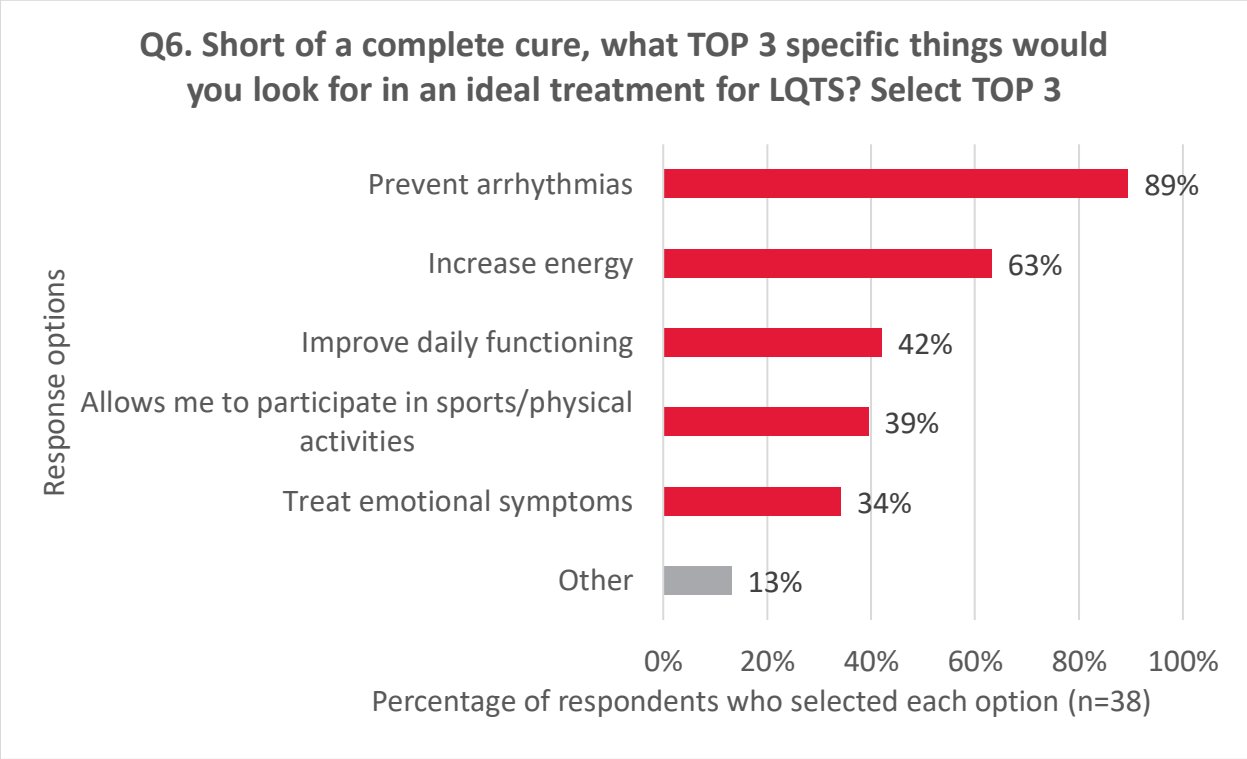
pack a small bag anytime we the house for more than a few hours. Cold packs, apple sauce and a drink, plus the medications. It's embarrassing for him and again just a stressful thing to always have to remember that." - Crystal, parent of a 13-year-old son living with LQTS Type 3

Other treatment downsides: medication unavailability, lack of patient input into treatment decisions.

Mexiletine and nadolol can be hard to obtain in some jurisdictions, and families have tried to import these medications from other countries. A minor theme that deserves recognition, is that many individuals felt that they were not offered a choice when it came to treatments; many were too young to have any influence or choice.

The LQTS community needs a cure. Treatments to prevent arrhythmias, increase energy and improve quality of life are urgently needed.

Individuals living with LQTS used online polling to select the top three specific things that they would look for in an ideal treatment for LQTS, short of a cure. The community selected all the options available, and their choices are listed below and illustrated with patient quotes.



Poll respondents include patients, parents and caregivers who chose to participate in online polling. The number of individuals who responded to each polling question is shown below the X axis (n=x). The responses for these polling questions are not considered scientific data. These are intended to complement the patient comments made during and after the meeting. Poll responses selected by more than 33% of poll respondents are shown in **red**.

The LQTS community needs a cure for this disease

Although a cure for LQTS was not included as a poll option, it was mentioned throughout the meeting and in the submitted comments. Many spoke about the possibilities of a one-time treatment such as gene therapy or CRISPR, to treat the underlying cause of LQTS.

> *"My biggest hope is that one day LQTS can be fully eradicated via gene therapies like CRISPR and that my daughters will not have to worry about passing on LQTS to any potential children of their own."* - Meagan, 37-year-old living with LQTS, parent and family member of others living with LQTS

> *"A new medication that specifically targets the electrical function of the heart for protection from SCA's [sudden cardiac arrests] would be amazing! Gene therapies that*

are deemed safe and effective would be amazing. ICDs that carry low risk of unnecessary shocks would also be good." - Lea, 49-year-old living with LQTS and parent of seven-year-old daughter living with LQTS

Prevent arrhythmias

The LQTS community needs treatments developed specifically for LQTS, which will address the genetic cause of the disease and not just the symptoms. The community asked for specific treatments for some of the less common subtypes of LQTS.

> *"A treatment that targets long QT I think would give me my ultimate goal, which is to feel safe. I want to feel safe in my everyday life for me and for my kids. I don't want the invasive surgeries. … I don't want the emotional burden. … And then the beta blockers maybe just aren't enough if that's what we're on. I want something where I feel like this is targeting what we need in a way that's tolerable and I can feel safe. I don't have to fear surgeries, I don't have to fear ineffective medication. I can feel safe on this treatment."* - Melissa, living with LQTS Type 2 and parent and family member of others living with LQTS Type 2

> *"My family has not endured a sudden cardiac death and I am tremendously grateful for that. What we have experienced are tremendous life alterations due to medication side effects for a medication that we are taking to hopefully prevent an episode. I completely recognize this need, but identifying a medication that actually treats the medical condition would be such an ideal scenario."* - Hilary, 44-year-old living with LQTS Type 1, parent and family member of others living with LQTS Type 1

Improve quality of life:

Individuals living with LQTS need medications with less treatment-related side effects. They need treatments that lead to increased energy, improved daily functioning, and provide a greater ability to participate in sports/physical activities.

> *"To have therapies that are more obviously effective and minimize the risk of a breakthrough event. Right now, all therapies seem more like 'just in case' options, but there's no way to know whether or not they're actually providing value. As a result, it often feels like our family is taking unnecessary risk simply because the potential cost of not doing so is too high."* - Aaron, 40-year-old living with LQTS and parent of an eight-year-old daughter living with LQTS

> *"I would love to come off my beta blocker but still be protected. I don't like the side effects of beta blockers. I want to live freely again without constant fear of death. I hate this. I am hopeful the new medicines being trialled could offer this."* - Jessica, living with LQTS

Treat and prevent emotional symptoms

Many would like a solution that alleviates the emotional burden of LQTS.

> *"I would be very grateful if research could be carried out into a medication for anxiety disorders and panic that does not prolong QT time."* - *Kertin, living with LQTS*

> *"The thing that weighs heavily on me with this question is getting ahead of the emotional impact, the PTSD. … I think having that emotional support right from the beginning. … I would love to have the psychological support and the understanding from the psychological aspect, what this does to a patient so that there can be a quality of life if these are the current treatments today."* - Kori, living with LQTS Type 2

Other LQTS treatment considerations and needs of the community

The LQTS community identified other important treatment considerations as well as other needs from the community in general. These include **less painful and less invasive treatments**, **safe removal of existing implanted devices**, **more LQTS research**, **more information about QTc prolonging medications**, **EKG newborn screening**.

Less painful and less invasive treatments, including smaller and more technically advanced ICDs that are less invasive or do not require battery replacements.

> *"In the maybe more immediate future, less invasive treatments, not having to do those surgeries that are so invasive that my daughter passed away from. The external devices, … would be the next immediate thing."* - Shauna, living with LQTS, parent of two daughters with LQTS including Britnee who passed away at 18 years from LQTS

> *"Possibly a cure/medications that can stop the long QT from getting worse so to avoid having an ICD implanted. Or smaller and more technologically advanced ICD's that are less invasive."* - Janett, living with LQTS and a family member of someone living with LQTS

Safe removal of existing implanted devices.

> *"Ultimately, we need an exit plan for patients who have had ICDs implanted. It never treated me, it only harmed me."* - Callie, 46-year-old living with LQTS Type 1

More LQTS research. Research areas suggested include: LQTS and pregnancy; ways that diet and exercise can help to treat the disease; and which treatments are best for different LQTS subtypes.

> *"As someone living with a rare type of long QT, I also want to make sure we are not left behind. I dream of the day everyone with long QT will have disease-specific treatments. Ever since I was diagnosed, my doctors have had to make highly educated predictions as to whether a treatment would help me based on how it worked for people with the three*

most common types." - Alexis, 23-year-old living with LQTS Type 5 and caregiver for her mother living with LQTS

More information about QTc prolonging medications

"Transparency regarding medications that can alter the QT intervals that are not told to the public, beginning with the most commonly prescribed antibiotic, Zithromax. Changing labels on medications to prevent sudden cardiac arrest/sudden cardiac death." - Genevieve, medical professional

EKG newborn screening

"I wish all babies were tested for SADS conditions at birth so families didn't have to find out the hard way. We are blessed to have Tyler with us today. We are blessed to have the ability to treat Slade, but we long for better treatments for our SADS children." - Sherri, parent of two sons living with LQTS Type 3

The LQTS community is willing to bear some level of risk for a new treatment

Many of the treatments for LQTS already carry a great deal of risk, especially with respect to side effects and neuropathic pain. Many individuals living with LQTS would sustain risk if it would lead to a future cure for LQTS. Most would accept less risk for their children.

"I would personally be willing to accept some level of risk in order to balance quality of life with safety, using the least restrictive methods of doing so. … Having more options available to help treat LQTS and all SADS conditions is very important to me. It is my hope that the drug companies, researchers and the medical community will continue to focus on the needs of people living with SADS and do everything possible to bring new therapies forward." - Kristin, 52-year-old living with LQTS Type 2

"For myself, I would be willing to accept moderate risk in a controlled environment for a new therapy if I felt the potential benefits outweighed the risks. I do not think I would be willing to accept the same level of risk for my daughters." - Meagan, 37-year-old living with LQTS, parent and family member of others living with LQTS

Incorporating Patient Input into a Benefit-Risk Assessment Framework

The LQTS EL-PFDD meeting helped to increase the understanding of how long QT syndrome impact patients and their loved ones. The meeting also reinforced the urgent need for effective therapeutics for this disorder. **Table 1** speaks to the challenge that patients living with LQTS endure. It serves as the proposed introductory framework for the Analysis of Condition and Current Treatment Option to be adapted and incorporated in the FDA's Benefit-Risk Assessment. This may enable a more comprehensive understanding of these disorders for key reviewers in the FDA Centers and Divisions who would be evaluating new treatments for LQTS. The data resulting from this meeting may help inform the development of LQTS -specific, clinically meaningful endpoints for current and future clinical trials, as well as encourage researchers and industry to investigate better treatment.

The information presented captures the perspectives of patients and families living with LQTS presented at the June 11, 2024 EL-PFDD. The collective hope of LQTS patients and families is that this meeting will encourage future research and successful new product development for people living with LQTS who urgently need treatment options. Note that the information in this sample framework is likely to evolve over time.

> *"The lack of appropriate medications and therapies for long QT syndrome, especially for young children, is in my opinion one of the biggest gaps in modern medicine."* - David, parent of Isla who passed away at six years from LQTS Type 3
>
> *"Now is the opportunity to mend our broken hearts."* - Tom, 40-year-old living with LQTS, family member of others living with LQTS

TABLE 1: Benefit-Risk Table for LQTS

	EVIDENCE AND UNCERTAINTIES	CONCLUSIONS AND REASONS
ANALYSIS OF CONDITION/ IMPACTS ON ACTIVITIES OF DAILY LIVING	**Long QT syndrome (LQTS) is a genetic heart condition**. LQTS symptoms can differ from one person to another, even within the same family. **LQTS can result in premature death from cardiac arrest.** **LQTS is a disease characterized by fear and worry, reflecting an uncertain future.** Family members worry about the safety and future of their parents, siblings, children and other family members living with LQTS. They worry whether medications and treatments will be sufficient or enough to prevent an event. They worry about cardiac arrests, shocks, ICD failure, more surgeries, they worry about passing LQTS on to future children. **The most burdensome LQTS-related health concerns include anxiety and depression**. Many experience panic attacks and PTSD because of past cardiac events, and shocks from their ICD. Other symptoms include light-headedness and fainting, cardiac arrest and fatigue. Some living with the disease experience abnormal heart rhythms, near drowning or drowning, seizures, and anoxic effects from the lack of oxygen after a sudden cardiac arrest.	**LQTS diminishes quality of life.** LQTS and its treatments reduce stamina, impact the ability to fully and freely participate in sport, social and work life. Although exercise is safe for the majority of those with LQTS, some families continue to be restricted from exercise. These restrictions cause or worsen anxiety and depression in people living with LQTS. **LQTS can interfere with treatment for other conditions**. Medications that prolong the QTc interval are contraindicated for those living with LQTS as they increase the risk of events. Those living with LQTS may not be able to receive treatment for other medical or psychological conditions that severely impact their daily lives or for conditions that may be life threatening.
CURRENT TREATMENT OPTIONS/ PROSPECTS FOR FUTURE TREATMENTS	**Some individuals living with LQTS require multiple medications and devices.** Some were even treated from birth. These include beta blockers, ICDs, lifestyle changes including avoiding sports, counseling and psychotherapy, antidepressants and anti-anxiety medications, left cardiac sympathetic denervation (LCSD), as well as many other medications and devices. **While some LQTS treatments and lifestyle choices have saved lives, none are curative.**	**LQTS treatments further compromise quality of life**. Burdensome treatment-related side effects such as fatigue are an experience for many, followed by anxiety and PTSD. Some have many surgical procedures, accompanied by scarring and disfigurements. **The LQTS community needs a cure.** Treatments that prevent arrhythmias, increase energy and improve quality of life are urgently needed. Other important treatment considerations and community needs include less painful and less invasive treatments, safe removal of existing implanted devices, more LQTS research, and more information about QTc prolonging medications. As many LQTS treatments are already risky, many would sustain additional risk for themselves, but not their children, for a new treatment.
	See the Voice of the Patient report for a more detailed narrative.	

Appendix 1: LQTS EL-PFDD Meeting Agenda

Date: June 11, 2024

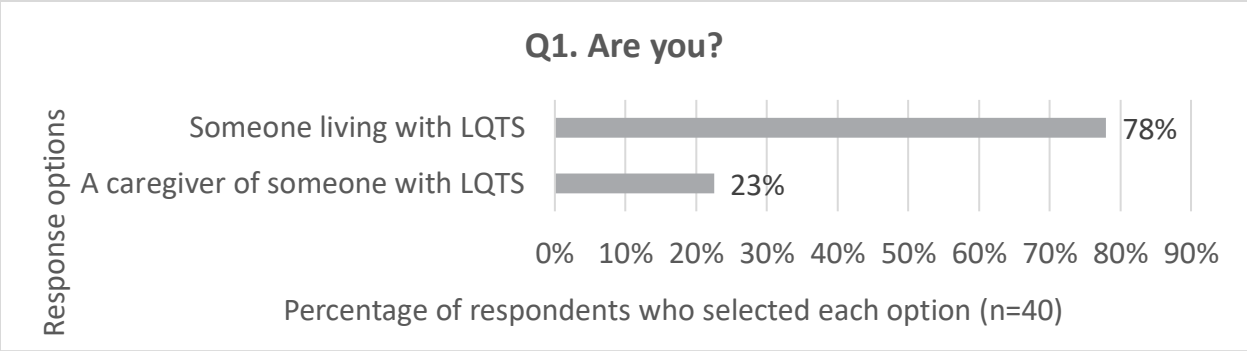
Time: 9:30 am to 4:00 pm ET

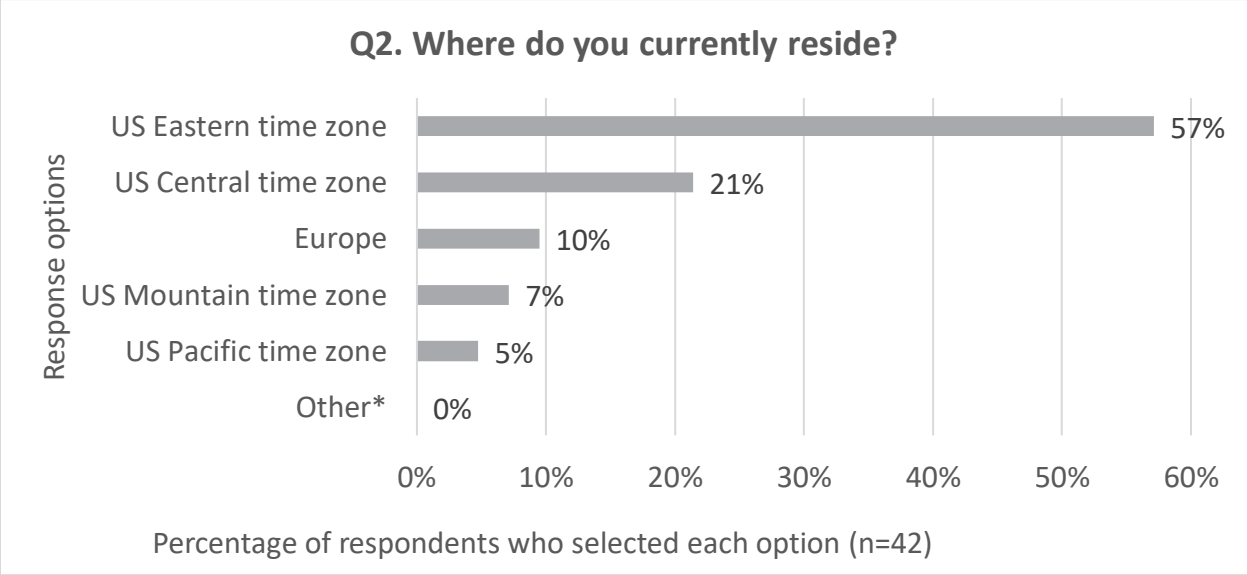
9:30-9:35 am	Welcome: *Genevie Echols, RCIS, Family Support Director, SADS Foundation*
9:35-9:40 am	FDA Opening Remarks and Overview: *Shetarra Walker, MD*
The LQTS Patient Voice	
9:40-9:55 am	Introduction and LQTS session overview & demographic polling: *James Valentine, JD, Hyman, Phelps & McNamara, PC, Genevie Echols*
9:55-10:10 am	LQTS Clinical Overview: *Michael J. Ackerman, MD, PhD, Mayo Clinic*
Topic 1	**Living with LQTS: Symptoms and Daily Impacts**
10:10-10:30 am	Panel discussion (4 panelists)
10:30-11:20 am	Audience and remote polling; Moderated audience discussion (5 discussion starters)
Topic 2	**Current and Future Approaches to LQTS Treatment**
11:20-11:40 am	Panel discussion (4 panelists)
11:40 am -12:30 pm	Audience and remote polling; Moderated audience discussion (5 discussion starters)
12:30-12:35 pm	Summary of LQTS Patient Voice: *Larry Bauer, RN, MA, Sr Regulatory Drug Expert, HPM*

Appendix 2: Demographic polling

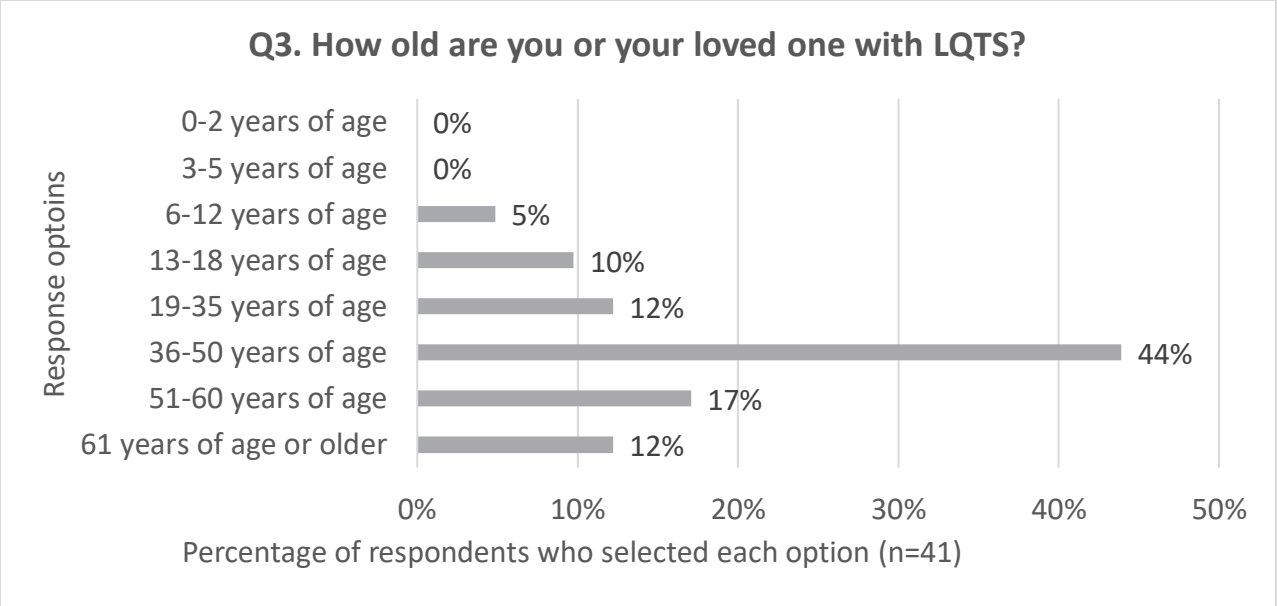
The graphs below include patients, parents and caregivers who chose to participate in online polling at the June 11, 2024 meeting. The number of individuals who responded to each polling question is shown below the X axis (N=x).

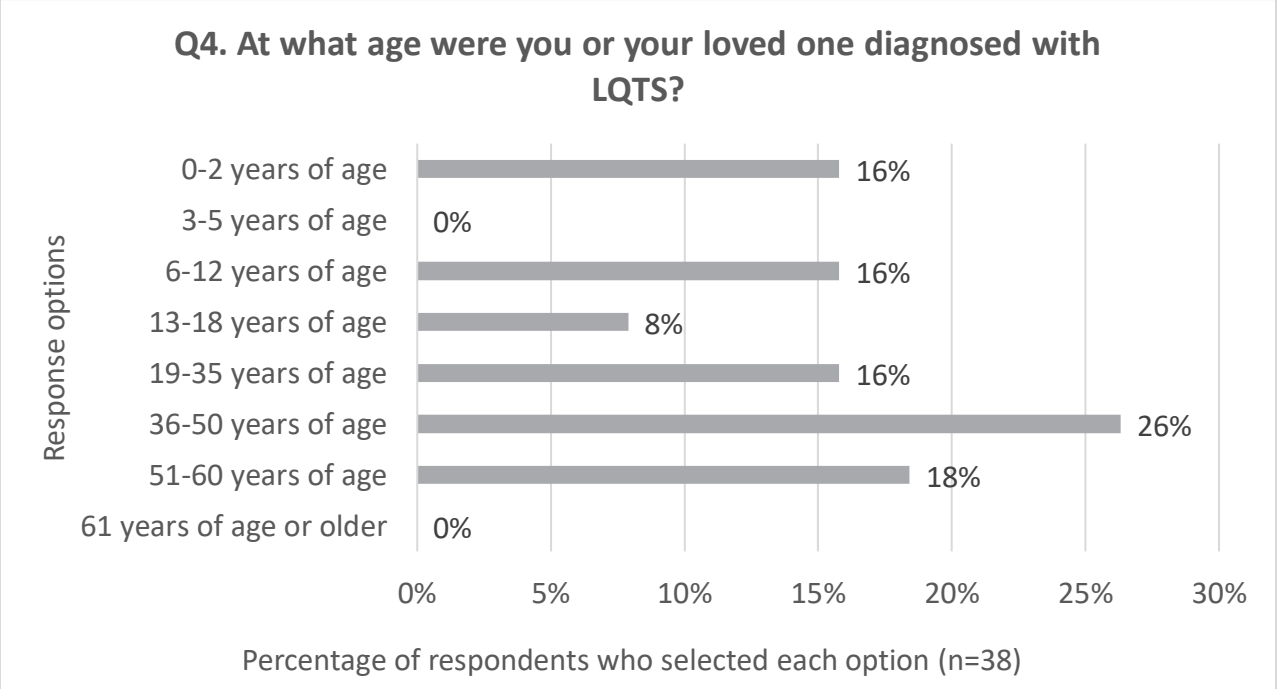
While the response rates for these polling questions is not considered scientific data, it provides a snapshot of those who participated in the LQTS EL-PFDD meeting. Note that meeting demographics are dynamic and may have changed as more individuals joined the meeting.





*The category of "Other" includes the US Alaska and Hawaiian time zones, Canada, Mexico, Asia and the Middle East. Note that individuals from these regions may have joined after this question was asked.


Q3. How old are you or your loved one with LQTS?
Response optoins
0-2 years of age
3-5 years of age
6-12 years of age
13-18 years of age
19-35 years of age
36-50 years of age
51-60 years of age
61 years of age or older
0%
0%
5%
10%
12%
44%
17%
12%
0%
10%
20%
30%
40%
50%
Percentage of respondents who selected each option (n=41)


Q4. At what age were you or your loved one diagnosed with LQTS?
Response options
0-2 years of age
3-5 years of age
6-12 years of age
13-18 years of age
19-35 years of age
36-50 years of age
51-60 years of age
61 years of age or older
16%
0%
16%
8%
16%
26%
18%
0%
0%
5%
10%
15%
20%
25%
30%
Percentage of respondents who selected each option (n=38)

Appendix 3: LQTS Discussion Topics

Topic 1 Living with LQTS: Symptoms and Daily Impact

1. How does LQTS affect you or your loved one on best and on worst days?
2. How have your or your loved one's symptoms changed over time? How has the ability to cope with the symptoms changed over time?
3. Are there specific activities that are important to you or your loved one that you/they cannot do at all or as fully because of LQTS?
4. What do you fear the most as you or your loved one gets older? What worries you most about your or your loved one's condition?

Topic 2 Current and Future Approaches to LQTS Treatment

1. What are you currently doing to manage your or your loved one's LQTS symptoms?
2. How well do these treatments treat the most significant symptoms and health effects of LQTS?
3. What are the most significant downsides to your or your loved one's current treatments and how do they affect daily life?
4. Short of a complete cure, what specific things would you look for in an ideal treatment for LQTS? What factors would be important in deciding whether to participate in a new research trial?

Appendix 4: LQTS EL-PFDD Meeting Panelists and Callers

Topic 1 Pre-recorded Panel

- Lene, 52-year-old living with LQTS and parent of two daughters living with LQTS including Rebecca, who passed away at four and a half years from LQTS
- Mark, 61-year-old living with LQTS Type 2 and family member of others living with LQTS Type 2
- Suzy, 38-year-old living with LQTS and parent of a three-year-old daughter living with LQTS
- Tom, 40-year-old living with LQTS and family member of others living with LQTS

Topic 1 Discussion starters

- Lorena, 39-year-old living with LQTS and parent of a nine-month-old son living with LQTS
- Lee, 37-year-old, living with LQTS Type 2
- Amanda, parent of a 13-year-old daughter diagnosed at birth with LQTS Type 3
- Kristin, 52-year-old living with LQTS Type 2
- Marsha, 71-year-old living with LQTS Type 2 and parent of a daughter living with LQTS Type 2

Topic 1 Callers

- Jay, 64-year-old living with LQTS Type 1 and parent young adults living with LQTS Type 1
- Kristi, wife of man living with LQTS Type 2 and parent of children living with LQTS Type 2
- Julie, living with LQTS Type 2 and parent and family member of others living with LQTS Type 2

Topic 2 Pre-recorded Panel

- Michele, 56-year-old living with LQTS and parent of a 12-year-old daughter living with LQTS
- Mona, 56-year-old living with LQTS Types 2 and 5
- Alexis, 23-year-old living with LQTS Type 5 and caregiver for her mother living with LQTS
- David, parent of Isla who passed away at six years from LQTS Type 3

Topic 2 Discussion starters

- Shauna, living with LQTS, parent of two daughters with LQTS including Britnee who passed away at 18 years from LQTS
- Kori, living with LQTS Type 2
- Meredith, parent of a daughter living with LQTS Type 2
- Melissa, living with LQTS Type 2 and parent and a family member of others living with LQTS Type 2

Topic 2 Callers

- Isabella, 27-year-old living with LQTS and parent of a son living with LQTS
- Callie, 46-year-old living with LQTS Type 1