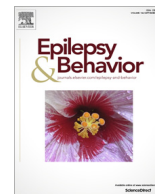




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Patient family engagement and partnership: Pilot survey results in assessing behavior, communication, and quality of life in children with Lennox–Gastaut syndrome and other drug-resistant epilepsy



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ABSTRACT

Objectives: Lennox–Gastaut Syndrome (LGS) and other drug-resistant epilepsy (DRE) can impact behavior, communication, and quality of life (QoL). In collaboration with community engagement efforts with the Lennox–Gastaut Syndrome Foundation (LGSF), we aimed to gain an initial snapshot of patient and family perspectives and experiences with evaluation of behavior, communication, and QoL.

Methods: A cross-sectional survey was conducted to collect self-reported information from caregivers of children with LGS and other DRE regarding their perspectives and experiences with healthcare providers' evaluation of behavior, communication, and QoL. The survey tool was developed by the study investigators in partnership with the LGS Foundation and had diffused to caregivers online by epilepsy advocacy groups including the Pediatric Epilepsy Surgery Alliance (PESA). Responses were analyzed. Descriptive statistics were calculated. The survey asked for caregiver perspectives and assessed which instruments the caregivers had previously been given for measuring these domains.

Results: Responses from 245 caregivers were included, with 132 (54%) caregivers of an individual with LGS and 113 (46%) caregivers of an individual with non-LGS related DRE. Respondents reported that 66% of their loved ones had undergone epilepsy-related surgery. Over 90% agreed that measuring behavior, communication, and QoL was important, but fewer than half felt that their healthcare providers evaluated these domains well. LGS caregivers largely shared non-LGS caregivers' perspectives; however, they reported more frequently that communication was not evaluated enough. Barriers to measuring these domains included a lack of good surveys (developmentally appropriate and specific to the type of epilepsy) or not receiving any survey instruments for these domains during clinic appointments.

Caregivers play a crucial role for individuals with DRE, and their input is essential in identifying challenges and needs. Caregivers believe that measuring behavior, communication, and quality of life is important, and most of them feel that their loved ones are not adequately evaluated during their healthcare encounters. There is a need for appropriately scaled survey instruments to measure areas of importance for patients and caregivers, as well as incorporation of these outcomes in the healthcare discussion.

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Abbreviations: LGS, Lennox–Gastaut Syndrome; LGSF, Lennox–Gastaut Syndrome Foundation; DRE, drug-resistant epilepsy; QoL, quality of life; EEG, electroencephalogram; VNS, vagus nerve stimulation; RNS, responsive neurostimulation; DBS, deep brain stimulation; ABAS, Adaptive Behavior Assessment System; ABC, Aberrant Behavior Checklist; CSBS, Communication and Symbolic Behavior Scales; PROMIS, Patient-Reported Outcomes Measurement Information System questionnaires; PedsQL, Pediatric Quality of Life Inventory; CP-QOL, Cerebral Palsy Quality of Life Questionnaire; PESA, Pediatric Epilepsy Surgery Alliance; DEE, developmental and epileptic encephalopathies.

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1. Introduction

Lennox–Gastaut Syndrome (LGS) is marked by multiple seizure types, developmental delay, cognitive impairments, specific electroencephalogram (EEG) patterns, significant disabilities, and early death [1–3]. There are approximately 48,000 individuals in the United States living with LGS, which constitutes 3%–5% of childhood onset epilepsies; however, it represents over 1/5 cases of drug-resistant epilepsy in children [4,5]. Here, we use the term drug-resistant epilepsy (DRE) as recommended by the International League Against Epilepsy (ILAE), noting that DRE also encompasses the terminology of refractory epilepsy, medically intractable epilepsy, and treatment-resistant epilepsy.

In a survey of caregivers administered through parent-organized foundations, the subgroup of those caring for children with LGS reported that 42% did not walk independently, 25% did not have functional hand grasp, and 42% were entirely dependent on someone else for feeding, including 21% being gastrostomy-tube fed. In addition, 55% of children with LGS did not communicate effectively even with their parents and caregivers, and half were nonverbal [3]. In a caregiver-driven study of developmental and epileptic encephalopathies including LGS, only 48% of individuals >2 years understood more than 100 words, and 52% of individuals >2 years used speech as the primary mode of communication [6]. This severity of impairment has negative effects on quality of life of children with LGS, caregivers, and family members [7].

Community-engaged research is essential to understand and improve healthcare delivery [8]. Community engagement is an important priority for the LGS Foundation (LGSF). The LGSF is an advocacy organization created to assist people with LGS and their families. In partnership with the LGSF, we involved caregivers in this study as the people closest to the problem likely have great perspective, gap understanding, and possible solutions. In identifying outcomes that matter to families of children with LGS, the major domains named *a priori* by the LGSF leadership and community were behavior, communication, and quality of life. Other domains such as sleep were also considered by the LGSF team and may be areas of future and ongoing research. Communication refers to communication by epilepsy patients with their caregivers.

Measurements of behavior, communication, and quality of life (QoL) are not uniformly applied in children with drug-resistant epilepsy. There are validated tools to appropriately assess these domains, but they are not uniformly implemented in clinical practice. Given the clinical heterogeneity and wide-ranging comorbidities of DRE, it is recognized that measuring outcomes is a challenge. Additionally, some of the commonly used assessment tools lack the sensitivity to capture changes in these populations, particularly in those with severe physical impairment and intellectual disability [9–11]. Accurate and meaningful assessment of change in these domains is critical for developing more effective treatments, improving outcomes, understanding gap, and alleviating caregiver burden. Using a cross-sectional survey design, we sought to conduct a pilot survey in collaboration with the community engagement efforts of the LGSF to elicit perspectives of families of children with LGS and other DRE on the importance of evaluating behavior, communication, and QoL, as well as their experience with their healthcare providers' evaluation of these domains.

2. Methods

2.1. Survey Tool

After thoughtful review of the current medical literature, a short unvalidated pilot survey tool was developed by study investigators in partnership with the LGSF leadership and community

engagement team (Fig. 1). Multiple-choice questions and Likert-scale responses were used. Questions evaluated caregivers' perspectives and experiences in the child's epilepsy journey. Questions were developed on interactions with their healthcare teams, and how the teams may be evaluating behavior, communication, and QoL. A review of the medical literature yielded instruments addressing these domains. Based on this review, a non-exhaustive list of instruments was selected. The listed instruments included the Adaptive Behavior Assessment System (ABAS) [12], Aberrant Behavior Checklist (ABC) [13], Communication Matrix [14], and Communication and Symbolic Behavior Scales (CSBS) [15]. Questions also evaluated any assessments by their care team regarding QoL including EuroQol EQ-5D-5L [16], NIH Patient-Reported Outcomes Measurement Information System questionnaires (PROMIS) [17], Pediatric Quality of Life Inventory (PedsQL) [18], and Cerebral Palsy Quality of Life Questionnaire (CP-QoL) [19]. Many of the assessments are intended for young children, those with intellectual disabilities, or those with severe impairments. There are many more questionnaires than those chosen to be listed. Along with the non-exhaustive list of survey instruments, options for "do not know," "other," and write-in of survey information were included. The instruments were listed based on previous reported medical literature and discussion with the study team and the LGSF leadership team. Additional questions assessed perspectives of caregivers regarding the importance and quality of evaluations of their child's behavior, communication, and QoL. The survey tool was not validated in the target population. An electronic version of the survey tool was created in SurveyMonkey™.

2.2. Study population and data collection

The online survey was distributed to caregivers within the LGSF and Pediatric Epilepsy Surgery Alliance (PESA) with online diffusion through social media channels and electronic newsletters in English and Spanish. The study population included caregivers of children with epilepsy who responded to the survey after being reached through those online networks, social media, or other epilepsy-related forums. Responses were recorded over a four-week period through SurveyMonkey™ and were anonymized upon collection. This survey study received exempt status at the Institutional Review Board (IRB) of Lurie Children's Hospital.

2.3. Variables studied

Caregivers were surveyed about the importance of and their satisfaction with their healthcare providers' evaluations of behavior, communication, and QoL. We surveyed the medical literature and selected several existing questionnaires that measure behavior, communication, and QoL to identify what is commonly used in these assessments. We also included a free text field to allow caregivers to name other questionnaires. Caregivers were asked to report if and which of the instruments they had used with their child. Additionally, caregivers were asked to identify perceived barriers to measuring these domains.

2.4. Data analysis

Descriptive statistics are provided for the responses overall. The responses were also stratified by whether the respondent reported being a caregiver of an individual with LGS or non-LGS epilepsy. There was no discrete cap on age range given the intention of the survey to capture caregiver perspectives without limiting the opportunity to respond. Responses were excluded if the respondent was not a caregiver of an individual with LGS or non-LGS epilepsy: these roles and these clinical diagnoses were identified by

LGS and surgery caregiver survey

Survey around Behavior, Communication, and Quality of Life Instruments - Epilepsy Surgery

Dear all,

We are doing a research study funded by the Patient-Centered Outcomes Research Institute (PCORI) looking at treatments and their effect on children and adolescents with Lennox Gastaut syndrome (LGS). One area we would like to look at is related to developmental outcomes such as communication. Also, we would like to look at treatments and their impact on quality of life. We ask your help to give us feedback on your experience and thoughts in epilepsy. We will compare to what we learn in people with LGS. Please note that we plan on publishing this information; however, no private information will be used. This is voluntary and you can complete it if you would like.

Sincerely,

Sandi, Anup, Tracy, and Marc

1. What is your preferred language to speak?

English

Spanish - Puede realizar la encuesta en español. <https://www.surveymonkey.com/r/encuesta-cuidador-epilepsia-LGS>

Arabic

French

>

Other (please specify)

2. Are you the primary caregiver of someone with epilepsy?

Yes, and their seizures are ongoing.

Yes, and their seizures are currently controlled, or they are seizure-free.

No

3. Are you the primary caregiver of someone with epilepsy who has Lennox-Gastaut Syndrome (LGS)?

Yes

No

4. What is the current age (in years) of the person with epilepsy?

0-5

6-11

12-14

15-18

19-21

> 21

5. Are you the primary caregiver of someone with epilepsy who has had any form of epilepsy related surgery?

Yes (then answer all that apply in Question 6)

No (then skip to Question 7)

6. Which type of epilepsy surgery has your child had? (select all that apply)

Vagus Nerve Stimulation (VNS)

Corpus callosotomy

Resective surgery

Hemispherectomy/Hemispherotomy

Other neurostimulation device (RNS or DBS)

Laser ablation (MR guided laser interstitial thermal therapy)

Phase 2 intracranial monitoring (such as subdural grid or strip electrodes, or stereoEEG depth electrodes)

Do not know

None

>

Other (please specify)

7. Have you or someone in your family had to fill out the following? (select all that apply)

Adaptive Behavior Assessment System (ABAS) 0-5 years, parent report

Aberrant Behavior Checklist (ABC)

The Communication Matrix

Communication and Symbolic Behavior Scales (CSBS)

Do not know

>

Other (please specify)

8. Please rate this response: my epilepsy/neurology provider has evaluated communication concerns well in my loved one with epilepsy.

Strongly agree

Agree

Neutral

Disagree

Strongly disagree

Do not know

9. Please rate this response: my epilepsy/neurology provider has evaluated behavior concerns well in my loved one with epilepsy.

Strongly agree

Agree

Neutral

Disagree

Strongly disagree

Do not know

10. Have you or someone in your family had to fill out the following quality of life surveys? (select all that apply)

European Quality of Life - 5 domains (EQ-5D-5L)

Patient-Reported Outcomes Measurement Information System (PROMIS)

Pediatric Quality of Life (PedsQL) Epilepsy

The Cerebral Palsy Quality of Life Questionnaire (CPQ)

Do not know

>

Other (please specify)

11. Please rate this response: my epilepsy/neurology provider has evaluated quality of life well in my loved one with epilepsy.

Strongly agree

Agree

Neutral

Disagree

Strongly disagree

Do not know

12. Please rate the following response: evaluating communication concerns are important to caregivers of people with epilepsy.

Strongly agree

Agree

Neutral

Disagree

Strongly disagree

Not applicable

13. Please rate the following response: evaluating behavior concerns are important to caregivers of people with epilepsy.

Strongly agree

Agree

Neutral

Disagree

Strongly disagree

Not applicable

14. Please rate the following response: evaluating quality of life are important to caregivers of people with epilepsy.

Strongly agree

Agree

Neutral

Disagree

Strongly disagree

Not applicable

15. What are the biggest barriers to measuring behavior, communication, and quality of life in people with epilepsy? (select all that apply)

Lack of good survey instruments specific to the type of epilepsy

Lack of developmentally appropriate survey instruments

Time to complete survey instruments

Not getting survey instruments during clinic appointments

Do not know what surveys I have completed and why

>

Other (please specify)

16. Have you seen or completed other questionnaires that you feel help evaluate communication abilities or issues, behavior, and/or quality of life for people with epilepsy?

No.

>

Yes. If yes, please specify.

Fig. 1. Online survey tool.

respondent self-report. Bivariate analysis was performed using chi-square tests of independence for categorical variables with <20% of expected cell counts less than 5 (questionnaires and barriers to measuring outcomes), Fisher’s exact test for categorical variables with >20% of expected cell counts less than 5 (surgical intervention), and Mann–Whitney U tests for ordinal variables (i.e., age range and Likert-scale responses), with a significance level of 0.05. Statistical analysis was performed on Prism version 9.5.0 (GraphPad Software, LLC) and R 4.3.1.

3. Results

3.1. Study population

Two hundred fifty-one unique survey responses were returned, six of which were excluded for incompleteness or for an individual who did not have epilepsy. Results were analyzed from 245 people (Table 1). 132 (54%) caregivers reported having a loved one with LGS, and 113 (46%) were caregivers of people with drug-resistant epilepsy without the diagnosis of LGS. Twenty percent of individuals were 0–5 years of age, 31% 6–11 years, 14% 12–14 years, 16% 15–18 years, 5% 19–21 years, and 14% >21 years. There was a statistically significant difference in age between those with LGS and those with non-LGS DRE. Those with non-LGS DRE were younger ($p < 0.0001$) with a median age range of 6–11 years, IQR 0–5 – 12–14, as compared with those with LGS with a median age range of 12–14 years, IQR 6–11 – 19–21.

3.2. Questionnaires

Respondents were queried regarding the use of questionnaires examining behavior, communication, and QoL (Table 1). Most caregivers did not know if they had completed a listed communication and behavior assessment (59%) or QoL assessment (78%) in the past. The highest reported communication and behavior instruments were the ABAS (35%), followed by the CSBS (7%), and then the ABC (6%). Few caregivers reported having completed the listed QoL instruments, with 2.3% reporting the PedsQL or its epilepsy module and 2.3% reporting PROMIS. There were no significant differences between LGS caregivers and non-LGS epilepsy caregivers regarding what survey tools were used in a clinical setting.

Of the choices listed for behavior, the most common questionnaire was the Adaptive Behavior Assessment System (ABAS), with just over one-third of respondents recognizing this instrument. Most respondents (59%) did not know of a behavior questionnaire, making follow-up difficult in this context. Other instruments, such as the Aberrant Behavior Checklist (ABC), Vineland-III, the Communication Matrix, and Communication and Symbolic Behavior Scales (CSBS) were recognized by fewer than 10% of respondents as something they had completed during a clinic visit. Along with the non-exhaustive list of survey instruments, options for “do not know,” “other,” and optional free text write-in of survey information were included. No other questionnaires were named in optional free text. In the QoL domains, no *a priori* named survey exceeded 3% of responses.

Table 1
Study population and characteristics.

	All (N, %)	LGS (N, %)	Non-LGS (N, %)	p-value
Age				
0–5	50 (20%)	15 (11%)	35 (31%)	****<0.0001
6–11	75 (31%)	39 (30%)	36 (32%)	
12–14	34 (14%)	18 (14%)	16 (14%)	
15–18	40 (16%)	24 (18%)	16 (14%)	
19–21	11 (4.5%)	7 (5.3%)	4 (3.5%)	
>21	35 (14%)	29 (22%)	6 (5.3%)	
Total	245 (100%)	132 (100%)	113 (100%)	
Epilepsy surgical intervention				
Yes	162 (66%)	71 (54%)	91 (79%)	****<0.0001
VNS	64 (26%)	59 (45%)	5 (4.4%)	
Hemispherectomy	60 (24%)	3 (2.3%)	57 (50%)	
Resective surgery	32 (13%)	4 (3.0%)	28 (24%)	
Corpus callosotomy	26 (11%)	22 (17%)	4 (3.5%)	
RNS/DBS	12 (4.9%)	8 (6.0%)	4 (3.5%)	
Phase 2 intracranial monitoring (subdural grid, strip electrodes, stereoEEG depth electrodes)	22 (8.9%)	1 (0.8%)	21 (18%)	
Laser ablation (MR guided laser interstitial thermal therapy)	6 (2.4%)	0 (0%)	6 (5.2%)	
None	85 (34%)	61 (46%)	24 (21%)	
Questionnaires previously received				
Behavior and Communication				
Adaptive Behavior Assessment System (ABAS)	67 (35%)	39 (39%)	28 (30%)	
Aberrant Behavior Checklist (ABC)	12 (6.2%)	7 (7.0%)	5 (5.3%)	
Vineland-III	3 (1.6%)	2 (2.0%)	1 (1.1%)	
The Communication Matrix	7 (3.6%)	7 (7.0%)	0 (0%)	
Communication and Symbolic Behavior Scales (CSBS)	14 (7.2%)	10 (10%)	4 (4.3%)	
Do not know	114 (59%)	55 (55%)	59 (63%)	
Other	24 (12%)	13 (13%)	11 (12%)	
Quality of Life				
EQ-5D-5L	2 (1.2%)	1 (1.2%)	1 (1.2%)	
PROMIS	4 (2.3%)	3 (3.5%)	1 (1.2%)	
PedsQL	4 (2.3%)	1 (1.2%)	3 (3.5%)	
CP QoL	2 (1.2%)	2 (2.4%)	0 (0%)	
Do not know	133 (78%)	62 (73%)	71 (83%)	
Other	34 (20%)	19 (22%)	15 (17%)	

3.3. Perspectives of caregivers

Caregivers almost unanimously agreed or strongly agreed that it is important for their healthcare providers to measure QoL (99%), behavior (96%), and communication (94%). Fewer than 2% of caregivers disagreed or strongly disagreed (Table 2).

In contrast, in their perspective, fewer than half of caregivers agreed that any of the three domains were evaluated well by their healthcare providers. 42%, 33%, and 29% of caregivers disagreed or strongly disagreed that communication, behavior, and QoL, respectively, had been evaluated well by their healthcare provider. Only 41% agreed or strongly agreed that their healthcare provider had evaluated well in communication, 42% in behavior, and 43% in QoL. There was a significant difference between LGS caregivers and non-LGS caregivers regarding communication. 42% of LGS caregivers disagreed or strongly disagreed that communication had been evaluated well by their healthcare provider, whereas only

28% of non-LGS caregivers disagreed or strongly disagreed ($p = 0.022$). There were no significant differences between LGS caregivers and non-LGS caregivers in their perspectives on the importance of or satisfaction with evaluation of the behavior and quality of life domains.

3.4. Barriers to measuring Behavior, Communication, and quality of life

In their responses, most caregivers reported a lack of developmentally appropriate surveys (51%) and not receiving survey instruments during clinic appointments (51%) as barriers to measurement. Additionally, 43% of caregivers reported a lack of good survey instruments specific to the type of epilepsy, 24% reported not knowing what surveys they had completed and why, and 13% reported that time to complete survey instruments was a barrier.

Table 2
Caregiver Perspectives.

	All (N, %)	LGS (N, %)	Non-LGS (N, %)	p-value
Perspectives of caregivers				
Importance of evaluating communication				0.2436
Strongly agree	163 (66%)	83 (63%)	80 (70%)	
Agree	70 (28 %)	40 (30%)	30 (26%)	
Neutral	5 (2.0%)	3 (2.3%)	2 (1.7%)	
Disagree	6 (2.4%)	4 (3.0%)	2 (1.7%)	
Strongly disagree	2 (0.8%)	2 (1.5%)	0 (0%)	
Not applicable	1 (0.4%)	0 (0%)	1 (0.9%)	
Importance of evaluating behavior				0.0743
Strongly agree	178 (72%)	89 (67%)	89 (77%)	
Agree	59 (24%)	36 (27%)	23 (20%)	
Neutral	4 (1.6%)	3 (2.3%)	1 (0.9%)	
Disagree	1 (0.4%)	0 (0%)	1 (0.9%)	
Strongly disagree	3 (1.2%)	3 (2.3%)	0 (0%)	
Not applicable	2 (0.8%)	1 (0.8%)	1 (0.9%)	
Importance of evaluating QoL				0.7831
Strongly agree	209 (85%)	112 (86%)	97 (84%)	
Agree	34 (14%)	18 (14%)	16 (14%)	
Neutral	2 (0.8%)	0 (0%)	2 (1.7%)	
Disagree	0 (0%)	0 (0%)	0 (0%)	
Strongly disagree	1 (0.4%)	1 (0.8%)	0 (0%)	
Not applicable	0 (0%)	0 (0%)	0 (0%)	
Communication has been evaluated well				*0.0217
Strongly agree	29 (12%)	17 (13%)	12 (10%)	
Agree	71 (29%)	27 (20%)	44 (38%)	
Neutral	54 (22)	30 (23%)	24 (21%)	
Disagree	55 (23%)	31 (23%)	24 (21%)	
Strongly disagree	32 (13%)	24 (18%)	8 (7.0%)	
Do not know	6 (2.4%)	3 (2.3%)	3 (2.6%)	
Behavior has been evaluated well				0.7299
Strongly agree	26 (11%)	17 (13%)	9 (7.8%)	
Agree	78 (32%)	37 (28%)	41 (36%)	
Neutral	55 (22%)	26 (20%)	29 (25%)	
Disagree	49 (20%)	30 (23%)	19 (17%)	
Strongly disagree	31 (13%)	18 (14%)	13 (11%)	
Do not know	7 (2.9%)	3 (2.3%)	4 (3.5%)	
QoL has been evaluated well				0.7733
Strongly agree	31 (13%)	17 (13%)	14 (12%)	
Agree	74 (30%)	38 (29%)	36 (31%)	
Neutral	61 (25%)	30 (23%)	31 (27%)	
Disagree	52 (21%)	32 (24%)	20 (17%)	
Strongly disagree	19 (7.7%)	12 (9.2%)	7 (6.1%)	
Do not know	9 (3.7%)	2 (1.5%)	7 (6.1%)	
Barriers to measuring behavior, communication, and QoL				
Lack of good survey instruments specific to the type of epilepsy	96 (43%)	54 (47%)	42 (39%)	0.1052
Lack of developmentally appropriate survey instruments	115 (51%)	66 (57%)	48 (44%)	
Time to complete survey instruments	28 (13%)	17 (15%)	11 (10%)	
Not getting survey instruments during clinic appointments	114 (51%)	56 (49%)	57 (52%)	
Do not know what surveys I have completed and why	54 (24%)	19 (17%)	34 (31%)	
Other (please specify)	24 (11%)	11 (9.6%)	12 (11%)	

4. Discussion

4.1. Caregiver experience

We present a cross-sectional, pilot study surveying caregivers of children with LGS and non-LGS epilepsy, to examine information on behavior, communication, and QoL. We explore the caregivers' reported experiences with formal assessments, eliciting what questionnaires were used, and areas for improvement. We involved caregivers in this study as we felt the people closest to the problem may be best able to offer important and useful information. Community engagement is an important priority in the LGSF, with an emphasis on return of information to the patient community as well as the medical community at large. This study is focused on honoring that collaboration and information sharing. Fig. 2 reports an infographic created by the LGSF team to share preliminary results back with the participating community and prompt further involvement based on our survey results. Dissemination in the medical literature is an additional important step. Further input from caregivers and patients is essential to understanding and improving the epilepsy journey. Caregiver insight is invaluable in identifying the challenges and needs of individuals with DREs, particularly in offering perspectives on treatment efficacy, health outcomes, healthcare delivery improvement, and the impact of DRE on daily life.

Some research exists on perspectives and experiences of caregivers caring for loved ones with LGS. Several studies have illustrated that patients and caregivers report a poorer QoL with increasing seizure frequency in LGS [20,21]. Beyond seizure-related variables, only a couple of studies exist that explore the impact of LGS on caregiver QoL. Gibson surveyed 96 LGS families and found that caregivers reported significant physical, emotional, financial, and social impact from caring for an individual with LGS [7]. Gallop et al. surveyed 40 parents of individuals with LGS from the United States, United Kingdom, and Italy, using qualitative interviews and quantitative tools, such as the SF-36v2 and Hospital Anxiety and Depression Scale (HADS), to explore health-related QoL [22]. They found parents had worse mental-health scores as compared with the general population, higher levels of anxiety, and lack of social support or respite care. However, there is no existing research that asks caregivers' perspectives on the importance of evaluating behavior, communication, or QoL in their loved one, nor how they feel they have been evaluated in these domains in a clinical setting. It is therefore difficult to compare our descriptive results to existing published literature.

4.2. Involvement of caregivers in improving the epilepsy experience

In this survey, almost all caregivers reported that measuring communication, behavior, and QoL is important. However, fewer than half (communication) and fewer than one-third (behavior and QoL) reported that they felt their healthcare provider had evaluated these domains well. The results in this survey are entirely based on caregiver responses and are not able to be validated by observation or chart review. This reported discrepancy between caregiver perception, needs, and lived experience has not been quantified before. This knowledge may produce more follow-up questions than answers at this stage, as the etiologies of these perceptions can be explored and validated further. Healthcare provider perspectives also need to be elicited in future studies. Given the expressed responses so far, healthcare teams may be more attuned to patient and family needs within the LGS community. The perceived gap in care assessment motivates future study and implementation. By offering assessments in these key areas over time, opportunities to improve outcomes in each domain can

Surgery or Medication for Lennox-Gastaut Syndrome



Powering Research Questions that Matter to Patients & Families.

The Surgery or Meds for LGS (SOM-L) study, sponsored by the Patient-Centered Outcomes Research Institute (PCORI), is a 4-year study that will study people with LGS who had surgery (VNS, Corpus Callosotomy, etc.) and compare them to people with LGS who try another anti-seizure medication.

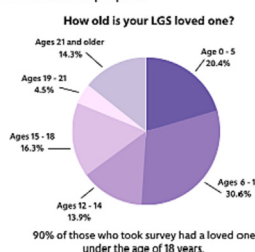
- Researchers will study the impact treatment has on seizures, emergency room visits, and ICU admissions.
- Researchers will also study the impact treatment has on communication, behavior, and quality of life in the person with LGS.

We need your help! Researchers want to know which surveys they should use to ask questions about communication, behavior, and quality of life in LGS. Join our Focus Groups today!

IN 2022, RESEARCHERS DID A SURVEY OF LGS FAMILIES, HERE IS WHAT THEY LEARNED:

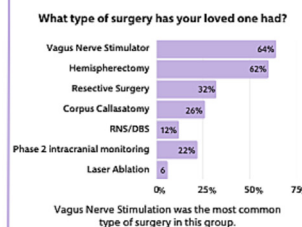
WHO TOOK THE SURVEY:

248 families were surveyed overall. 132 had LGS. 116 had other severe epilepsies.



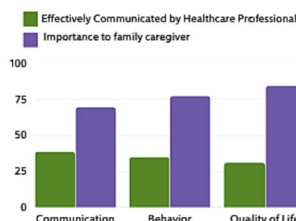
SURGICAL HISTORY:

162 of the 248 respondents previously underwent epilepsy surgery. All had tried anti-seizure meds.



WHAT MATTERS MOST TO FAMILIES:

In addition to seizures, there were 3 top areas that families said mattered to them when deciding if a treatment works or not. These areas are Communication, Behavior, and Quality of Life.



COMMUNICATION:

70% of caregivers reported communication was an important factor in epilepsy treatment for them. 39% said that their epilepsy/neurology provider has effectively evaluated this area.

BEHAVIOR:

77% of caregivers reported behavior was an important factor in epilepsy treatment for them. 35% said that their epilepsy/neurology provider has effectively evaluated this area.

QUALITY OF LIFE:

84% of caregivers reported quality of life was an important factor in epilepsy treatment for them. 31% said that their epilepsy/neurology provider has effectively evaluated this area.



MEASURING WHAT IS MOST IMPORTANT TO FAMILIES:

Most caregivers reported that current evaluation tools and surveys do not effectively address what's important to them.

BARRIERS TO MEASURING WHAT'S IMPORTANT TO FAMILIES:

Caregivers listed the barriers to measuring behavior, communication, and quality of life.



What's Next?

We Need To Do A Better Job Measuring What Matters to Those with LGS!

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Fig. 2. Survey results infographic from the LGSF.

occur, understanding that there are multiple challenges in clinical operations, personnel, and implementation that need to be explored from the healthcare team perspective. Nevertheless, caregivers are critical in the care and management of individuals with DRE. Caregiver perspectives can point to important opportunities to improve care. Only 2% of our current survey respondents dis-

agreed with the importance of evaluating these domains. Caregivers often provide ongoing support and assistance, including (but not limited to) administering medications, monitoring seizure activity, addressing concomitant health issues, and managing a complex healthcare system. It is important to note that this initial survey reflects only caregiver perspectives. Perspectives of healthcare providers are beyond the scope of this current study.

As the survey was accessible online to the LGS and non-LGS communities, we sought to understand if there were shared experiences or differences among caregivers of patients with drug-resistant epilepsy (DRE). Respondents in the non-LGS DRE community had younger children and a higher percentage of people who had undergone epilepsy surgery. With digital dissemination and social media amplification of the survey link, the number of people exposed to the survey is not known. Generalizability is not known. We highlight that 245 respondents are encouraging for engagement, and exceeded the number in other pilot surveys of caregivers for young people with a rare disease: their voices need to be heard. Survey-based and qualitative studies with caregiver feedback in the LGS community are often <100 in sample size [7,20–23].

Perceived barriers to measuring these domains were elicited. In the multiple-choice options selected, over half of caregivers reported a lack of developmentally appropriate instruments and not receiving survey instruments addressing these domains during clinic visits as significant barriers. Notably, approximately one-quarter of survey respondents reported not knowing what surveys they had completed and why. Best practices in community-engaged research should be incorporated to remove these barriers. Important functional domains should be assessed, and these outcomes should be incorporated into patient education, medical management decisions, and care plans.

4.3. Limitations

There are many limitations to our study. First, the survey tool is not validated. It was designed with nonbiased, nonleading, multiple-choice questions to allow categorical classification of variables for analysis. This categorization was based on previous literature reviews and feedback from the research and LGSF leadership teams. Additionally, it was designed to avoid survey burden and specifically to focus on evaluating behavior, communication, and QoL, to identify next steps for engagement and further study. If avoidance of survey fatigue had not been our goal, the survey could have contained many more detailed questions. In identifying outcomes that matter to families of children with LGS, the major domains were behavior, communication, and quality of life, as named *a priori* by the LGS community represented by the LGSF, and with input from previous medical literature. This pilot survey thus focused only on these domains. Other areas, such as sleep are also known to be outcomes that are significant to families, though they are beyond the scope of this current study. It is important to note the possible biases introduced by respondent self-report. This study is subject to selection and response bias. Further, clinical validation of the diagnosis of LGS was not possible. The response rate to the survey is not known as the denominator of possible survey respondents is not quantifiable. We do not know the number of people who may have received and could have answered the survey: the distribution channels included emails to family advocacy groups of the LGSF and then diffused to Pediatric Epilepsy Surgery Alliance membership and social media channels. It is possible that families were not aware that they had completed instruments on our survey's list or that they had answered other instruments which were not enumerated in our survey. The perspectives of those who did not choose to fill out the surveys are not known. Those who responded may bias the results and viewpoints represented, with potential over-reporting or under-reporting of certain

experiences. With response bias, the perspectives represented may reflect a more engaged and better resourced population given the online mechanism used to disseminate the survey. In addition, there is recall bias, with an unknown lag time between experience and report, which is common in similar study designs and publications. Even though there were options of “do not know,” “other,” and optional free text write-in for responses, there are possible ways in which the caregiver experience or understanding was not captured. Another limitation is that there may be an implied assumption that respondents understand when instruments are not “developmentally appropriate.” These limitations will be addressed in future studies. Even though a Spanish version of the survey was available and distributed, all responses were to the English survey. This is a limitation that needs to be explored as a future direction in addressing diversity, equity, inclusion, especially for at-risk groups in healthcare and community engagement. It is important to note the scope of the study: only caregiver perspectives were sought. This study does not contain healthcare provider perspectives. In the near future, a detailed investigation of healthcare provider perspectives is warranted.

4.4. Developing outcome measures that matter to families

Subjective reports from caregivers and family about changes in levels of function are challenging to standardize and evaluate. There have been efforts to develop measurements to achieve these goals, such as the Inchstone project, from the DEE-P Connections group [24], which aims to develop specific outcome measures for individuals affected by developmental and epileptic encephalopathies (DEE). There is not enough information on how these outcomes are being measured and how families feel their children are being evaluated in these domains. This discussion thus highlights opportunities for more holistic care design and opportunities to improve outcomes outside of traditional seizure frequency measurement.

The burden of DRE extends beyond the direct impact of seizures: DRE is associated with effects on cognition, physical abilities, functional and adaptive behaviors, communication, and social and emotional function [7,25]. Seizure-related variables, such as severity or frequency, are not the only determinants of QoL [26,27]. Behavioral challenges, whether resultant or chance comorbidities, are prevalent among those with DRE, often affecting their ability to effectively maintain social relationships and pursue education. Individuals with DRE exhibit higher rates of psychosocial and neuropsychiatric disturbances, which can exacerbate their communication challenges and their caregivers' burden [28]. Also, neurobehavioral changes because of antiseizure medications have been widely reported, including medication-induced cognitive deficit, sedation, and aggression [29,30]. An analysis of clinical trials in LGS found that many outcome measures focused on seizure-related outcomes, such as seizure frequency, and future trials could benefit from measuring outcomes that matter to families, such as QoL, psychosocial, functional, and cognitive outcomes [31]. Despite the importance of a core outcome set for clinical trials, a robust set has not yet been developed for individuals with LGS. We emphasize the importance of having community-engaged research to inform and drive inquiry into outcomes that matter to patients and families and people living with DRE. Appropriately scaled instruments measure meaningful change to empower families and providers for shared decision-making, particularly in the context of clinical trials and determining efficacy of new treatments.

5. Conclusion

We conducted an initial survey to understand caregiver perspectives on the importance of and their satisfaction with evalua-

tion of behavior, communication, and quality of life in their loved one affected by DRE. Caregivers agree that measuring these domains is important; however, a substantial proportion of caregivers believe that their loved ones are not adequately evaluated in these domains by their healthcare providers during clinical care. Furthermore, these results report some perceived barriers to assessing these domains. Future work is needed to explore use of these outcome measures and instruments in the DRE population and specifically in Lennox-Gastaut Syndrome.

Declaration of competing interest

The authors declare that they have no known competing financial interests or personal relationships that could have appeared to influence the work reported in this paper.

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