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SHORT COMMUNICATION

Patient-reported outcomes in epilepsy: a case study exploring their usage and impact

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ABSTRACT

Background: This study aimed to obtain insights from epilepsy specialists on the use of Patient-Reported Outcome (PRO) measures and how they can affect the management of people with epilepsy and healthcare resource utilization.

Methods: The heads of two referral units for people with epilepsy at one tertiary care hospital were invited to respond to a structured survey.

Results: Paper-based questionnaires and face-to-face interviews were the main modalities used to measure the quality of life of people with epilepsy. The Quality of Life in Epilepsy Inventory-31 (QOLIE-31), the Adverse Event Profile (adult centre), the Generalized Anxiety Disorder-7, Short-Form Health Survey 36, PSY-Flex, SAFA and Child Behavior Checklist (paediatric centre) were the most used scales. There was consensus about the favourable impact of PRO upon patient management, disease management and measurement of the success of a treatment. Both respondents considered the PRO as important as other main indicators like efficacy and tolerability of the treatment. Lack of time, personnel and economic resources was identified as a barrier on the use of PRO. The PRO could reduce the number of visits, exams and treatments, and increase the time spent on each patient and the number of neuropsychological, psychological and rehabilitation services. The standardized use of PRO was considered useful and the increase in human resources was considered a priority to achieve this goal.

Conclusions: Despite the heterogeneity in the actual collection of PRO, there was a uniform perception about their role to optimize the care of people with epilepsy.

Keywords: Economics, Epilepsy, Patient-Reported Outcomes, Seizure

Introduction

In recent years there has been an increasing attention to place people with epilepsy at the centre of clinical care and health research with the aim to improve their experience and ensure that the research is robust and of maximum value for the use of medicines, therapy and health services.

Patient-Reported Outcomes (PROs) are any report of the status of a patient's health condition that comes directly from the patient, without interpretation of the patient's response by anyone else (1).

The use of PRO measures in clinical practice is gaining interest from different perspectives. Potential benefits include the opportunity to improve and facilitate the communication between patients and clinicians, to provide a comprehensive evaluation of the functional and mental health domains of patients, to monitor more accurately the effects of the interventions and to support the clinical decision-making and patient self-management (2). The adoption of PRO measures has been also suggested to improve the allocation of healthcare resources according to individualized needs and increase the efficiency of patient management and practices (3).

This study aimed to obtain preliminary insights from epilepsy specialists on the use of PRO measures and how they can affect the management of people with epilepsy and improve healthcare resource utilization.

Materials and methods

Participants and procedures

Between January and February 2023, the heads of two referral units for adult and paediatric people with epilepsy at the academic, tertiary care hospital of Bari (Italy) were invited to respond to a structured survey about the use of PRO measures in epilepsy. The epilepsy centre for adults has around 2,200 outpatients in charge and 11 professionals are

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involved in the activity (3 neurologists, 1 neuropsychologist, 3 residents, 2 nurses, and 2 people as admission staff). The paediatric epilepsy centre has around 1,600 outpatients in charge and 9 professionals involved (2 neurologists, 1 child neuropsychiatrist, 1 neuropsychologist, 3 nurses, and 2 neurophysiopathology technicians).

The survey was developed by three Italian neurologists with expertise in the diagnosis and treatment of epilepsy. The survey was formulated in English and included 20 questions related to patient empowerment and PRO (Supplementary appendix). It was administered via the web through a dedicated platform and required approximately 15-20 minutes to be completed.

Statistical analysis

A descriptive summary of the responses given by the participants to each question of the survey was provided.

Results

A summary of the responses to the interview is provided in Table 1. Both respondents stated that people with epilepsy and, when appropriate, family members/caregivers are always involved in the decision-making process on the management of their condition and they are informed about the possibility and the importance of their involvement in the decision-making process.

Both centres assessed patient satisfaction with their care path and treatment process dedicating around 10 minutes to each patient during visits; the epileptologist or both the epileptologist and neuropsychiatrist are the persons in charge for this activity at the adult and paediatric centres, respectively.

Different domains are evaluated at the centres, including the quality of life, emotional state, anxiety, mood, irritability, and limitations at work, school or in other activities; vitality, emotional-behavioural control, subjective assessment of their well-being were reported to be also assessed at the adult epilepsy unit. Paper-based questionnaires and face-to-face interviews were the main modalities used to measure the quality of life of the people with epilepsy at the interviewed centres. Among the scales to identify the PRO for psychopathological and emotional aspects of the disease, the Quality of Life in Epilepsy Inventory-31 (QOLIE-31) and the Adverse Event Profile were reported as the most used at the adult epilepsy centre, and the Generalized Anxiety Disorder-7, Short-Form Health Survey 36, PSY-Flex, SAFA, Child Behavior Checklist at the paediatric one.

The participants to the survey answered that recognized assessment scales to identify the PRO are mainly used within profit and no-profit studies like clinical trials sponsored by pharmaceutical companies, academic/hospital observational studies and research projects at the adulthood site and are administered by clinicians, while they are part of the routine clinical practice and are administered by psychologists at the paediatric centre. The time dedicated to the collection of

TABLE 1 - Answers of participants to the structured interview

	Adulthood	Childhood
1. Does your centre involve people with epilepsy and, when appropriate, family members/caregivers, in the decision-making process on the management of their condition?		
YES, always	☑	☑
YES, often	–	–
YES, sometimes	–	–
NO, never	–	–
2. Does your centre inform people with epilepsy and, when appropriate, family members/caregivers, about the possibility and the importance of their involvement in the decision-making process?		
YES	☑	☑
NO [Why? ____________________]	–	–
3. Does your centre involve people with epilepsy and, when appropriate family members, in the following clinical decision:		
Diagnostic exams (e.g., neuroimaging, genetic exams, electroencephalographic monitoring)	YES	YES
Choice of the first antiseizure medication	YES	YES
Understanding of the risk of non-treatment/poor adherence to treatment	YES	YES
Addition of new drug/s	YES	YES
Changing doses of drug/s	YES	YES
Withdrawal of drug/s	YES	YES

	Adulthood	Childhood
Epilepsy surgery	YES	YES
Non-pharmacological treatment (e.g., vagus nerve stimulator [VNS], ketogenic diet)	YES	YES
Social aspects of epilepsy	YES	YES
4. From a managerial/organizational standpoint does your centre assess patient satisfaction with their care path and treatment process?		
YES, always	☑	–
YES, often	–	☑
YES, sometimes	–	–
NO, never [Why____________________]	–	–
5. How does your centre assess patient satisfaction with their care path and treatment process? *[Please select all that apply]*		
We do with specific questionnaires	☑	–
We do by questioning during visits	☑	☑
Another professional (e.g., a nurse) is responsible for it with specific questionnaires	–	–
Another professional (e.g., a nurse) is responsible for it by questioning it	–	–
We don’t assess it [Why?________________]	–	–
If your centre assesses patient satisfaction, please specify the amount of time (minutes or hours) dedicated to each patient	10 min	10 min
Please specify the person in charge for the above-mentioned activity	Epileptologist	Epileptologist/neuropsychiatrist
6. Does your centre evaluate outpatients with epilepsy for the following aspects?		
Impairment of vitality	YES	NO
Emotional-behavioural control	YES	NO
Subjective assessment of their well-being	YES	NO
Assessment of emotional state in terms of limitations at work or school or for other activities	YES	YES
Dysphoria together with nervous tension, anxiety, and depressed mood	YES	YES
Denial of emotional problems with increased irritability in the interpersonal sphere	YES	YES
Other [specify:____________________]	–	–
7. What tools does your centre use to measure the Quality of Life of the people with epilepsy? *[Please select all that apply]*		
Paper-based questionnaires	☑	–
Electronic questionnaires	–	–
Face-to-face interviews	☑	☑
Other [specify:____________________]	–	–
8. Which recognized assessment scales does your centre use to identify the Patient-Reported Outcomes (PROs) for psychopathological and emotional aspects of the disease?		
Quality of Life in Epilepsy Inventory-31 (QOLIE-31)	☑	–
AEP (Adverse Event Profile)	☑	–
GAD-7 (Generalized Anxiety Disorder)	–	☑
Other specify: Short-Form Health Survey 36, PSY-Flex, SAFA and Child Behavior Checklist	–	☑

(*Continued*)

TABLE 1 - (*Continued*)

	Adulthood	Childhood
9. How frequently does your centre use recognized assessment scales to identify the PRO for psychopathological and emotional aspects of the disease?		
Always	–	–
Often	–	–
Sometimes	☑	☑
Never [Why? ________________]	–	–
10. When does your centre use recognized assessment scales to identify the PRO for psychopathological and emotional aspects of the disease? *[Please select all that apply]*		
In-profit studies (e.g., trials sponsored by pharmaceutical companies)	☑	–
In no-profit studies (e.g., academic/hospital OS, research projects at my centre)	☑	–
In routine clinical practice	–	☑
Other [specify:____________________________]	–	–
11. For what purposes does your centre use the PRO collected by the recognized assessment scales? *[Please select all that apply]*		
To monitor therapy tolerability/side effects	☑	☑
To evaluate the impact of the treatment on the quality of life of the patient	☑	–
To evaluate the impact of the treatment on the quality of life of the caregiver	☑	–
To evaluate the impact of the treatment on the social domains of the patient (e.g., social functioning, productivity loss, work, school, etc.)	☑	–
To guide/inform decisions on the treatment (e.g., to add/discontinue a treatment)	☑	☑
To justify access/use of a new drug/technology	☑	–
To assess the quality of life of the patient	☑	–
To assess the quality of life of the caregiver	☑	–
To assess the social impact of the epilepsy or the treatment (e.g., social functioning, work productivity, school, etc.)	☑	☑
To assess the impact of the epilepsy or the treatment on the mental health (e.g., cognition, anxiety, depression, etc.)	–	☑
Other [specify ____________________________]		
12. In your centre, who takes care of administering the questionnaires to the people with epilepsy?		
Clinicians	☑	–
Nurses	–	–
Other [specify: Psychologist]	–	☑
13. How much time does your centre spend to collect PRO during the visit with the people with epilepsy/caregivers?		
Less than 5 minutes	–	–
Between 5 and 10 minutes	–	–
Between 10 and 20 minutes	–	–
Between 20 and 30 minutes	☑	–
More than 30 minutes	–	☑
We do not collect PROs	–	–
Please specify the person in charge for the collection and analysis of PRO	Epileptologist	Psychologist Epileptologist/Neuropsychiatrist

	Adulthood	Childhood
14. Does your centre think that PRO can be beneficial in terms of: *[Please select all that apply]*		
Patient management	☑	☑
Disease management	☑	☑
Access to care (in terms of timing)	–	–
Access to care (in terms of facilitation)	–	–
Healthcare resource utilization improvement	☑	–
Measurement of the success of a treatment	☑	☑
Assessment of the value of a treatment	☑	–
15. Does your centre consider the PRO as important as other main indicators like the efficacy and tolerability of the treatment?		
YES	☑	☑
NO [Why? ____________________]	–	–
16. Which are the barriers on the use of PRO in clinical practice at your centre?		
Lack of time	☑	–
Lack of personnel	☑	–
Lack of economic resources	–	☑
Lack of knowledge/expertise in PROs	–	–
Do not believe PROs are useful in clinical practice	–	–
Other [specify:_______________]	–	–
17. Does your centre think PRO could impact reducing or increasing the following activities: *[Please indicate for each one the estimated amount as per percentage]*		
The number of visits	10%	–
The number of exams	30% ↓	–
The time spent on each patient	20%	30%
The number of neuropsychological services	15% ↑	50% ↑
The number of psychological services	15%	50%
The number of rehabilitation services	10% ↑	–
The number of other services [specify: ________]	–	–
The number of treatments (ASM)	40% ↓	–
The number of other treatments [specify: Psychiatric drugs]	20%	20%
18. Would your centre consider it useful to implement the standardized use of PRO in your centre?		
YES	☑	☑
NO [Why? ________________________________]	–	–
19. What method would you adopt to collect PRO in your centre:		
Capturing outcome data in-person (paper form questionnaire)	☑	–
Remotely (online survey platform)	–	–
A combination of both	–	☑
20. In your opinion, what kind of resources should be deployed in your centre to:		
Implement PRO process	Availability of a dedicated case manager	More questionnaires
Collect and manage PRO	Increasing dedicate personnel	More professionals
Perform analysis and assessment of PRO	Availability of trained dedicated personnel	More professionals

PRO has been estimated between 20 and 30 minutes at the adult centre and more than 30 minutes at the childhood centre; epileptologists were mainly involved in the collection and analysis of PRO in the adult setting, whereas epileptologists, psychologists, or neuropsychiatrists were in charge of these activities at the childhood centre.

There was a complete consensus among the respondents about the favourable impact that the PRO can have in terms of patient management, disease management and measurement of the success of a treatment, and both considered the PRO as important as other main indicators like the efficacy and tolerability of the treatment. The respondent from the adult epilepsy centre believed that PRO can also contribute to assess the value of a treatment and improve the utilization of healthcare resource. Lack of time, lack of personnel and lack of economic resources were identified as barriers on the use of PRO in clinical practice.

Regarding the future perspectives, the respondents agreed that PRO could reduce the number of visits, exams and treatments, and increase the time spent on each patient and the number of neuropsychological, psychological and rehabilitation services. The implementation of the standardized use of PRO was considered useful at both sites and the increase in human resources was considered a priority to achieve this goal. A digitalization of data collection besides traditional papers from questionnaire could also have an increasing role in the future.

Discussion

This study provided a picture of the attitudes towards the use of PRO measure at two adult and paediatric referral units for epilepsy care in Italy, identified similarities and differences between the centres, and delineated perspectives for the possible advancement of the field in the future.

Both adult and paediatric epilepsy centres measured different domains that may affect the quality of life of people with epilepsy, including emotional state and limitations at work or school. Differences emerged in the tools adopted to assess these domains, with face-to-face interviews being the way preferred in children and adolescents and both face-to-face interviews and paper-based questionnaires adopted in adult outpatients. Despite the increasing digitalization of healthcare services, electronic questionnaires still do not have a significant role in everyday clinical practice and so far, they are utilized almost exclusively within the frame of clinical trials.

In adults, psychopathological and emotional aspect of the disease are mainly explored by means of a short form, rather than comprehensive questionnaire of quality of life, namely the QOLIE-31; in paediatric age, a wider variety of instruments are used to assess the quality of life and evaluate more specifically anxiety disorders, psychological flexibility, psychiatric symptoms, and emotional and behavioural domains.

The PROs are collected during the different phases of patient care, including the patient access to mainly investigate the overall impact of the illness and quality of life, the treatment to mainly monitor the tolerability and define the impact of the therapy on comorbidities, mental health, social functioning and the follow-up to guide and inform decisions on the treatment.

At the adult epilepsy centre, the collection, analysis and use of the PRO take place mostly within the context of clinical trials or specific projects, whereas at the paediatric epilepsy centre PRO are part of everyday clinical activity. This difference may be explained not only by a different attitude between the epilepsy specialists and the differences in the populations seen but also by differences in the organization of the centres interviewed. The adult centre, indeed, is more commonly involved in clinical studies and the PRO assessment is entrusted to the clinicians without the availability of other healthcare professionals (e.g., nurses and psychologists), while the paediatric centre has a minor involvement in research and clinical investigations and can count on the resource of psychologists and neuropsychologists. Of note, the lack of time and lack of human resources were acknowledged as the main barriers for the implementation of the PRO in routine practice at the adult centre; the lack of economic resources was recognized by the paediatric unit as a limit for the purchase of the copyright for specific online tests and questionnaires and, hence, the further improvement of PRO assessment in clinical practice. This is consistent with the available evidence suggesting that several barriers still exist for the widespread use of PROs in everyday practice (4).

Despite the heterogeneity in the actual use of the PRO in clinical practice, there was a uniform perception throughout the two centres involved that PRO are important as other main indicators like the efficacy and tolerability of a treatment and their use can be beneficial to optimize the care of people with epilepsy. The belief that the PRO can be included among the indicators of the value of a treatment and the information obtained from their use can also affect the utilization of resources emerged. In this regard, the respondents to the questionnaire considered the reduction of clinical visits as a potential consequence of a more widespread utilization of PRO, together with a decrease in the burden of antiseizure medication and an increase in psychological/neuropsychological services and treatments. These changes may be interpreted as a more rational utilization of healthcare resource, an increase in the value of the services provided and a better management of both the epilepsy, with the avoidance of unnecessary and potentially harmful medications, and the associated comorbidities.

Finally, both the interviewees considered useful to implement the collection of the PRO in clinical practice in a standardized manner and allocate more resources for this purpose. Digitalization might also play an increasing role in the collection of PRO in the future, and the possibility of identifying populations more inclined to use digital tools could be considered.

This study was an explorative attempt to provide a brief overview of the current and future usage and impact of PRO in epilepsy. The contrast between adult and paediatric units represented a plus in the study. Several shortcomings need to be acknowledged. Only two Italian centres were involved in the initiative; in this regard, however, it is worth noticing that respondents to the survey worked at a tertiary, academic hospital, and both centres were accredited by the Italian League

Against Epilepsy and represent crucial referral points for a vast territory in the country. In addition, the questionnaire was not piloted before administration to the two respondents, although framing the question in one way or another can have significant influence on the answer gathered.

While the respondents gave their perceptions on how PROs could reduce the number of visits, exams and treatments, and increase the time spent on each patient and the number of neuropsychological, psychological and rehabilitation services, no actual data were available to triangulate these self-reported perspective. Although the perspectives of the respondents did not necessarily represent the national scenario and could not inform about the situation in other countries, this pilot study can pave the way for similar experiences to get insights and identify geographical differences about the role and contribution of PRO measures in epilepsy across care centres representative of the Italian context and worldwide.

In summary, the perspective revealed by the survey indicated that clinicians are well aware that epilepsy does not represent just a question of reducing seizures, and the disease burden extends into many psychological and social domains that ultimately impact the quality of life. The time dedicated to “listen to” the person with epilepsy is crucial and a more robust inclusion of his/her view in the management of his/her condition is of paramount importance. The assessment of the seizure frequency is not sufficient, and the PRO are thought to provide a more reliable assessment of treatment effectiveness and tolerability, to contribute to inform and guide clinical decisions, to have a favourable impact on clinical outcomes and satisfaction of people with epilepsy, and to improve the utilization of healthcare resources. Initiatives to identify measurement instrument sets for people with epilepsy are currently on the road (5,6). An active plan involving the key stakeholders would be needed to harmonize the adoption of PRO and ensure they can actually have favourable impacts for people with epilepsy and health services (7).

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Disclosures

Conflict of interest: Simona Lattanzi has received speaker’s or consultancy fees from Angelini Pharma, Eisai, GW Pharmaceuticals, Jazz Pharmaceuticals, Medscape and UCB Pharma and has served on advisory boards for Angelini Pharma, Arvelle Therapeutics, BIAL, Eisai, GW Pharmaceuticals and Rapport Therapeutics outside the submitted work; she has received research grant support from the Italian Ministry of Health and Ministry of University and Research. Angela La Neve has received speaker’s or consultancy fees from Eisai, Mylan, Sanofi, Bial, GW, Arvelle Therapeutics, Angelini Pharma and UCB Pharma outside the submitted work. Both authors reported personal fees from Ethos S.r.l. in compensation of time for the development of the survey.

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Data Availability: Anonymized data will be shared upon reasonable request of any qualified investigator.

Supplementary appendix. Structured survey about Patient-Reported Outcomes.

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