



# Time to Accelerate: The Use of Patient-Reported Outcome Measures in European Oncology

ACTION REPORT

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# Acknowledgements

This policy action report outlines views and perspectives from across the European cancer community on the current use of patient-reported outcome measures (PROMs) in cancer care. In particular, it reflects and builds upon discussions that took place at a July 2023 Community 365<sup>a</sup> roundtable event of the European Cancer Organisation (ECO) entitled: Patient-Reported Outcome Measures in Oncology: Unrealised Potential?<sup>b</sup> The event was facilitated by the co-chairing of Zorana Maravic, ECO Patient Advisory Committee Member and Chief Executive of Digestive Cancers Europe, and Isabel Rubio, ECO Board Member 2022–2023 and President of the European Society of Surgical Oncology.

We thank all speakers who contributed their expertise and experience, and shared their views on how to more effectively deliver the promise of patient-reported outcome measures towards the achievement of better cancer care. We also thank all attendees to the roundtable who provided views via the chat function during the meeting, and supplied thoughts and material after the meeting as well. We express our gratitude as well to those who provided review of this policy action report as part of ECO's Policy Approval Pathway<sup>c</sup>.

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- a. Community 365 is a group of charity, philanthropy, and industry contributors to the Focused Topic Networks of the European Cancer Organisation. Community 365 provide ideas, guidance, practical support, and resources for our work in convening stakeholders and building consensus in the European cancer community. Community 365 contributors do not have a decision-making role in our policy work. Rather, policies of the European Cancer Organisation, such as those represented in this document, are agreed by our Board after consultation with our Member Societies and Patient Advisory Committee, via our Policy Pathway process. More information here: <https://www.europeancancer.org/community-365>.
- b. More information here: <https://www.europeancancer.org/events/249:community-365-roundtable-meeting-patient-reported-outcome-measures-proms.html>.
- c. European Cancer Organisation's Policy Decision Making Process: <https://www.europeancancer.org/policy-decision-making>

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# Executive Summary

Key recommendations on accelerating the use of patient-reported outcome measures (PROMs) in oncology are set out below.

1. As a principle, **patients should be acknowledged as experts in their experience of their disease and the impact the disease, and its treatment, has upon their daily lives.** This experience should be recorded and made use of in the improvement of their treatment, and for wider improvements to cancer care for all patients.
2. The use of patient-reported outcome measures is an assisting response to meeting the needs of this principle. Advantages of their use include:
  - a. **Providing clinicians and others with the most relevant outcome data** to patients beyond overall survival rates;
  - b. **Facilitating shared decision-making** between patients and clinicians;
  - c. **Assisting judgement and decision-making about the tolerability of a treatment** for the individual in question e.g. the level to which a treatment may be causing fatigue, sleep disturbances or other negative consequences for the patient;
  - d. Enabling health systems to make **better informed decisions about treatment reimbursement.**
3. Although surveys of medical professionals find positive attitudes towards the use of PROMs in clinical practice, results also suggest that **professionals are not experiencing integration of PROMs use into their daily practice to the extent that they might be.** Paper based approaches to the use of PROMs also persist within some healthcare systems.
4. Electronic patient-reported outcome (ePRO) is a preferred means of gathering outcome reports from patients for many reasons, including its support in assisting real time digital monitoring of the patient by healthcare professionals, as well as enabling the patient to conduct reporting outside of the clinical setting. **Digital symptom monitoring with PROMs should be normalised into routine clinical care during systematic cancer treatment across Europe.**
5. To support better uptake of patient-reported outcomes in clinical practice, **clinical personnel should receive professional training on the review and interpretation of PROMs data,** and workflows should be redesigned to ensure PROMs data is reviewed and acted on. Nurses and other allied healthcare professionals should be trained to be first responders to PRO alerts.
6. Studies into the use of PROMs in regulatory processes also find gaps in their exploitation. Tracking all medicines for human use authorised or refused by EMA in 2017-2022 found that **less than half of European Public Assessment Report (EPARs) reported using any PRO/PROM data in the last six years.**
7. If PROMs are to be taken up more widely within oncology care, **ongoing efforts should be made to streamline, harmonise and reduce complexity for the patient in providing the required information.** Use of electronic methods of reporting (i.e. electronic patient-reported outcomes) are recommended to assist with this.
8. The **publication of PRO data should be more standardised** to encourage patient reporting by enabling the use of the data to be better visualised.
9. There is a need to better convert PROMs data into actionable improvements in healthcare. **The policy connection of PROMs data is not yet strong. Policy makers need to be more committed to making this happen.**
10. A **percentage of the budgets of European Health Technology Assessment (HTA)**

**agencies should be ring-fenced each year to support enhanced patient involvement** in their processes, including supporting the time, travel and other costs associated to such participation.

11. When bringing forward PRO data for use in EU level decision-making, such as for medicines approval or HTA, **emphasis should be placed on achieving PRO data from across the geographic spread of Europe and across groups in society**, including those that may be more difficult-to-reach. An example to consider in this respect, are older patients.
12. Once PROMs are integrated into the electronic chart and actions are taken for improvement in the cancer patients care, a quality assurance should be in place to evaluate these outcomes.

## A Word on Definitions

The European Medicines Agency (EMA) **defines a patient-reported outcome (PRO)** as '**any outcome directly evaluated by the patient and based on the patient's perception of a disease and its treatment(s)**'<sup>1</sup>.

A patient-reported outcome can be measured in absolute terms (e.g., the severity of a sign, symptom, or state of a disease) or as a change from a previous measure<sup>2</sup>.

According to the EMA, a PRO can include both single and multi-dimensional domains such as health status and satisfaction with treatment.

Health-related quality of life (HRQoL) is a specific type of the PRO, defined as patient's subjective perception of the effects of the disease and treatment(s) on daily life, well-being, and psychological, physical and social functioning<sup>3</sup>.

Data about PRO concepts are collected using PRO instruments such as questionnaires, leaflets, and documentation that support their use.

# Introduction



**Isabel Rubio, Co-Chair of the Roundtable; Board Member 2022–2023, European Cancer Organisation; President, European Society of Surgical Oncology**  
**Zorana Maravic, Co-Chair of the Roundtable; Member, European Cancer Organisation Patient Advisory Committee; Chief Executive Officer, Digestive Cancers Europe**

and to consider recommendations about their future use.

The European Cancer Organisation invited healthcare professionals, patient advocates, industry representatives, academic researchers, political decision-makers, and other experts to discuss the role of patient-reported outcome measures and the degree to which their greater use could offer opportunities in improving cancer care.

Patient-reported outcome measures are tools used to assess a patient's perception of their own health and well-being, as reported directly by the patients themselves. By incorporating the patient's voice, PROMs contribute to patient-centred care and shared decision making between healthcare providers and patients.

## ISABEL RUBIO AND ZORANA MARAVIC

Co-Chairs of the Roundtable

***"Are patient-reported outcome measures an unrealised potential in oncology care?"***

**Isabel Rubio**, Board Member 2022–2023 of the European Cancer Organisation and President of the European Society of Surgical Oncology, and **Zorana Maravic**, member of the ECO Patient Advisory Committee; Chief Executive Officer of Digestive Cancers Europe, co-chaired the July 2023 ECO Community 365 Roundtable Meeting to collate and better understand the perspectives of many impacted stakeholders about the use of patient-reported outcome measures (PROMs) in oncology,

## WHY THIS ROUNDTABLE?

- To highlight the value of incorporating patients' perspectives into the decision making;
- To examine the role of patient-reported outcome measures in cancer care and the value of their use;
- To evaluate the actual use of PRO/PROMs in daily practice;
- To discuss how to better integrate PROMs into the healthcare professionals' daily practice;
- To generate policy recommendations based on the insights and perspectives of the speakers and roundtable participants.

# The Value of Patient-Reported Outcome Measurements (PROMs) in Oncology

## KEY POINTS

- Emphasis should be placed on the individuality of each person's experience with cancer, as well as the ongoing need to promote steady improvement and innovation in cancer care.
- Use of electronic patient-reported outcome measures can facilitate real-time monitoring. Key recommendations are available from the [ESMO Clinical Practice Guideline](#).
- The EMA Regulatory Science strategy to 2025 aims to incorporate PROs and patient preferences into the risk-benefit evaluation of medicines.
- Patient-reported outcomes can be quite complex and time consuming to complete for the patient. The questionnaire must therefore be appropriate for the patient and sensitive to the complexity of language.



**Jammbe Musoro**, Senior Statistician and Quality of Life Specialist, European Organisation for Research and Treatment of Cancer (EORTC)

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**Sarah Jayne Liptrott**, Member of the European Cancer Organisation's Patient Advisory Committee representing MDS Foundation

**Marko Skelin**, Board Member of the European Society of Oncology Pharmacy (ESOP); Assistant Professor, Faculty of Medicine, University of Rijeka

outcome that patients are concerned with after a cancer diagnosis. The PROMs agenda speaks well to all of these points.

The first session of the roundtable was focused on understanding the different ways PROMs are being made use of for cancer care, and areas for improvement therein. The session was co-chaired by Isabel Rubio, and Ilana Widera, Global Senior Director, Breast Cancer Opinion Leader and Stakeholder Liaison, Pfizer.

**Jammbe Musoro**, Senior Statistician and Quality of Life Specialist at the European Organisation for Research and Treatment of Cancer (EORTC) gave an overview of what PROMs are and how they are used in different ways, including cancer research. In doing so, he emphasised as a starting principle, the main goals of cancer treatment, as expressed by the World Health Organization. That is, to:

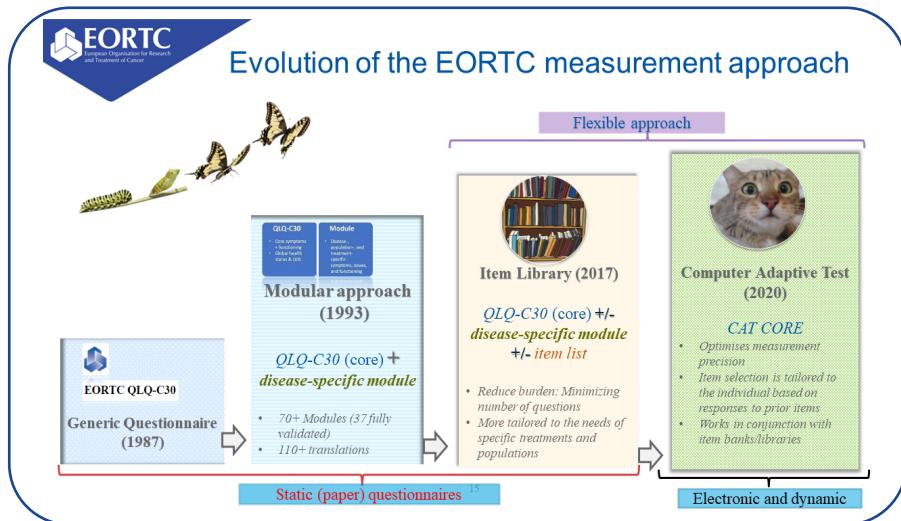
- Cure;
- Considerably prolong the life of patients; and,
- Ensure the best quality of life for cancer survivors.

Patient-reported outcomes (PROs) play an important part of delivering these goals, including the goal of ensuring the best quality of life for cancer survivors.

Jammbe clarified that PROs are reported directly by patients without modification or interpretation by healthcare professionals. These may include, for instance, some disease and treatment related

In opening the roundtable event on patient-reported outcome measures (PROMs), event co-chairs Isabel Rubio and Zorana Maravic placed emphasis on the individuality of each person's experience with cancer, as well as the ongoing need to promote steady improvement and innovation in cancer care. Furthermore, it is not the case that survival is the only

**Figure 1. Evolution of the EORTC Measurement Approach**

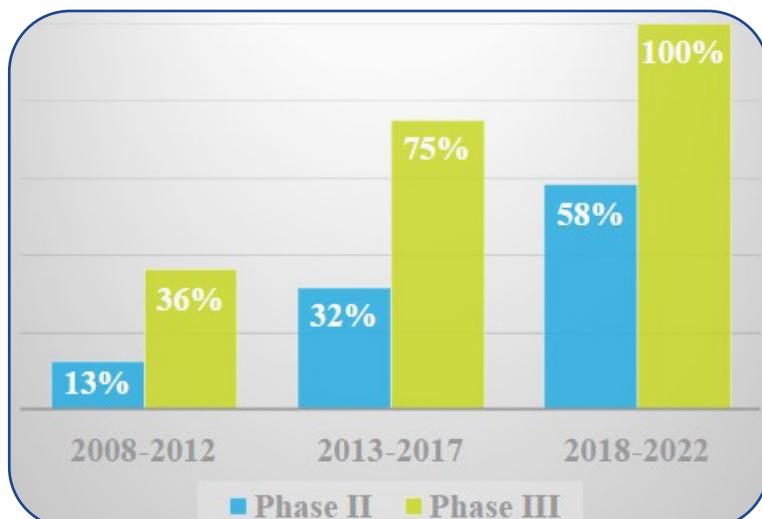


symptoms that are very particular to the individual patient. Health-Related Quality of Life (HRQOL) is an important type of PRO in this respect.

The benefits that Jammbe stated for using PROs included:

- Providing clinicians and others with the most relevant outcome data to patients beyond overall survival rates;
- Facilitating shared decision-making between patients and clinicians;
- Assisting judgement and decision-making about the tolerability of a treatment for the individual in question e.g. the level to which a treatment may be causing fatigue, sleep disturbances or other negative consequences for the patient; and,

**Figure 2. Upward trend in inclusion of HRQOL endpoints in EORTC-led trials**



- Enabling health systems to make better informed decisions about treatment reimbursement.

As evidence of the growing use of PROs in cancer research, Jammbe displayed figures showing the rising use of Health-Related Quality of Life (HRQOL) endpoints in EORTC led trials. Areas identified by Jammbe for further enhancing PRO use included: improving the means by which PRO data is collected, and further standardising the methods by which PRO data is analysed.

**Massimo Di Maio**, Professor at Department of Oncology, Department of Oncology, University of Turi, stated his clear view that patient-reported outcomes should be considered crucial in clinical practice and the routine management of cancer patients. His personal practice experience, as well as studies he has been involved with, convinces him that better use of PROs in clinical practice can lead

to tangible improvements in a patient's quality of life, and higher satisfaction with their treatment.

However, one of the challenges holding back the use of PROs into clinical practice is persistent use of paper-based model for reporting, when digital-based approaches can be much more readily made use of. With that said, it is also recognised that there are certain values from limited use of paper-based models where it can help to avoid underreporting that could otherwise exist. In any case, evidence suggests that in too many cancer centres, neither paper-based or digital PROs are in use in daily clinical practice.

Greater use of electronic patient-reported outcomes (ePROs) can assist, for example, with digital monitoring by healthcare professionals of a patient's current status, in much more real time. This can be facilitated, for instance, by the patient's use of a tablet, smartphone or a computer to produce a real-time report of symptoms. This is different from the traditional paper-based approach, which only allows the report at the moment of the clinical visit. Importantly, it should be understood that digital communication can be bidirectional. Not only do we have more complete information from the patient side, but the nurse and the oncologist can react to these signals or alerts more promptly and can proactively manage symptoms.

In this respect, clinical trials have been able to show the improvement such digital use of PROs for symptom management and telephone counselling can achieve for the patients' quality of life, and their adherence to treatment. This in turn can reduce emergency room visits and make the care for

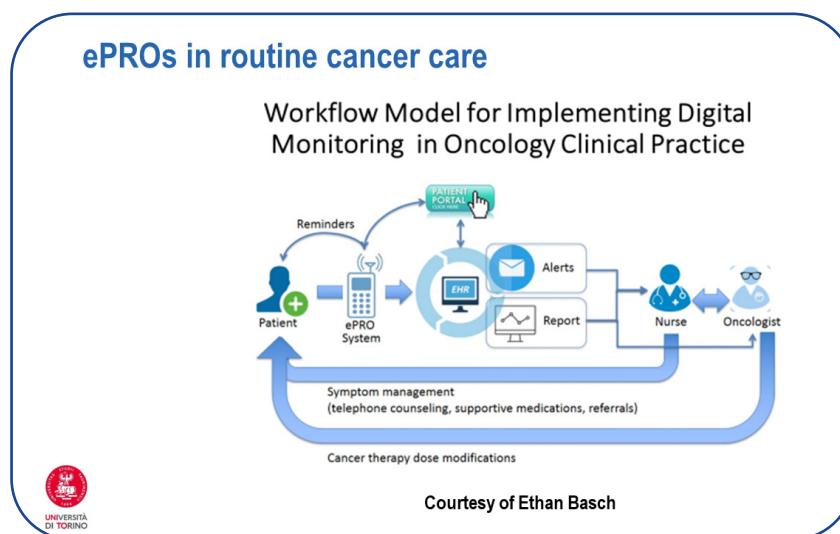
the patient more cost-effective as well as higher quality. Some trials have even indicated potential improvement in overall survival from use of ePROs.

Responding to this agenda, and seeking to support uptake, the [European Society of Medical Oncology \(ESMO\)](#) produced the first international guideline on the role of patient-reported outcome measures in the continuum of cancer care. International experts, including medical oncologists, nurses, psychologists, and patient representatives were involved.

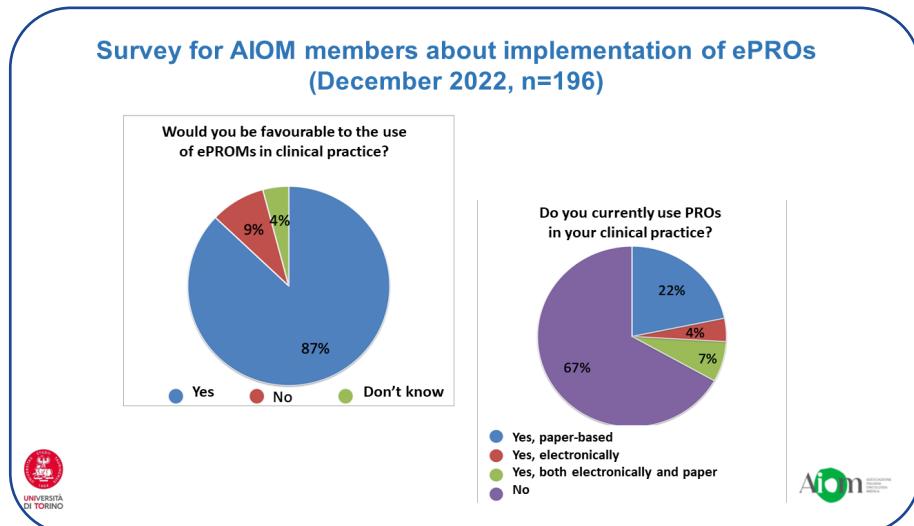
Key recommendations of the [ESMO Clinical Practice Guideline](#) include:

- **The use of PROMs in patients undergoing active treatment.** In particular, it is recommended that digital symptom monitoring with PROMs in routine clinical care during systemic cancer treatment is recommended, based on evidence of improved communication, satisfaction, treatment adherence, symptom control, QoL, emergency room and hospital admissions and survival.
- **Responding to PROMs data and remote monitoring alerts.** Clinical personnel at sites routinely collecting PROMs should receive training on the review and interpretation of PROMs data. Provider organisations and clinical teams should clarify personnel roles and responsibilities and redesign workflow to ensure PROMs data are reviewed and acted upon. Oncology nurses or other allied health support (e.g., social workers) with appropriate training should serve as first responders to PRO alerts.

**Figure 3. ePROs in Routine Cancer Care**



**Figure 4. AIOM Members Survey on ePROs**



- **Applicability and limitations.** The allocation of funds for validated software reimbursement, dedicated resources (nurses, physicians, etc.) and systematic evaluation of PRO implementation programmes in oncology clinics is recommended.

In closing, Professor Di Maio shared results from a survey conducted in Italy in December 2022 among the members of the Society of Medical Oncology (AIOM) in that country. 87% of the nearly 200 respondents indicated they are favourable to the use of ePROs in clinical practice. Yet the results of the survey also show only a small number make use of the PROMs during clinical practice and many of them use paper-based PROMs.

**Michela Meregaglia**, Researcher of Health Economics & HTA at the Government, Health and Not for Profit Knowledge Group, Bocconi University, Milan, presented the main result of a study investigating PROs and their measures (PROMs) in the authorisation of medicines in Europe between 2017 and 2022.

Regulators, HTA bodies and payers worldwide are increasingly considering the patient's perspective at all stages of drug development and regulatory decision-making.

In 2020, the [European Medicines Agency \(EMA\)](#) launched the [Regulatory Science Strategy to 2025](#), to promote patient-centred drug development and evaluation.

Patient-reported outcomes (PROs) refer to a "health or treatment outcome reported directly by the patient without the interpretation of a healthcare professional or anyone else" (FDA, 2009)

In this respect, the EMA Regulatory Science strategy to 2025 aims to incorporate PROs and patient preferences into the risk-benefit evaluation of medicines.

Methods of the study included:

- Identifying all medicines for human use authorised or refused by EMA in 2017-2022.
- Reviewing related European Public Assessment Report (EPARs).
- Identifying presence of PROs and PROMs by using EPARs using a list of keywords.
- Considering the presence of PROs and PROMs and relevant data on medicines.
- Considering PRO-PROM dyads.
- Performing multivariate logic regression to identify variables associated with the use of patient-reported evidence in EPARs.

The results showed that, despite EMA's discussions and recommendations for the use of PROs/PROMs for medicine evaluation since 2005, the consideration of patient-reported evidence is still limited, with **less than half of EPARs reporting any PRO/PROM data in the last six years**.

PROs were mostly used as secondary or exploratory endpoints in clinical trials, in line with EMA recommendations about the classification of PRO data in the clinical trial outcome hierarchy.

The strategic vision to strengthen patient relevance in evidence generation, launched by EMA in 2020 for 2025, requires a higher promotion of PROs in the evaluation of medicines for the purposes of marketing authorization at European level.

**Sarah Jayne Liptrott**, Member of the ECO Patient Advisory Committee representing MDS Foundation cautioned that PROs can be quite complex and time consuming to complete for the patient. The questionnaire must therefore be appropriate for the patient and sensitive to the complexity of language. If patients are not completing questionnaires, that requires response, to see, for example, if there are further opportunities for simplification. The importance of explaining to patients how their reported outcomes are used should not be underestimated either. If patients receive no feedback on the use of their data either, this can have a dispiriting impact on their future complete of outcome reports.

**Marko Skelin**, Board Member of the European Society of Oncology Pharmacy (ESOP) and

Assistant Professor, Faculty of Medicine, University of Rijeka, gave perspective on PROMs use from a pharmacy perspective. He reflected on the strong use of surrogate endpoints in market approval decisions, such as progression free survival. There are limitations on being over-reliant on such and similar endpoints alone, and we need to put more emphasis on the outcomes that are of utmost importance for our patients such as duration and quality of life in order to balance the information available to decision-makers. With cancer being an age-related disease, understanding how a treatment impacts the quality of life of older persons is very important, for example, with often less treatment tolerance being experienced in this specific population. The pharmacist has a role in these aspects in advising and informing patients about potential side effects of certain treatments, and how these could be reduced.

Reflecting on the session content, Roundtable Co-chair Zorana Maravic concurred strongly with the remarks from Sarah Jayne Liptrott about getting PRO questionnaires right. Her own experience with PROs very much reflected the principle that patients will respond better to questionnaires the more relevant they are to their circumstances.

# Overcoming Challenges to the Application of PROMs in Daily Life

## KEY POINTS

- OECD launched the Patient-Reported Indicator Surveys (PaRIS) initiative in 2018 to measure health systems performance through patient-reported outcomes and experiences. Results of the data collection were published in the 'Health at a Glance [2019](#) and [2021](#)', and the [technical report](#) on PROMs for breast cancer care was released detailing further analysis and interpretation of the findings.
- Results from the [European Atlas of Clinical Trials in Cancer and Hematology \(EuroACT\)](#) are guiding the understanding of inequalities and differences in the availability of clinical trial sites across European countries and are providing evidence on the use of relevant and meaningful QoL instruments in clinical trials.



**Candan Kendir, Health Policy Analyst, Organisation for Economic Co-operation and Development (OECD)**

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**Mercè Cases Escuté, Senior Research Manager, Patvocates**

**André Deschamps, Past Chairman, Europa Uomo**

**Candan Kendir**, Health Policy Analyst, Organisation for Economic Co-operation and Development (OECD). The Organisation for Economic Co-operation and Development (OECD), in its role in providing policy advice to its member and partner countries, has long experience in measuring and reporting performance of health systems. With the goal of making health systems more people centred, OECD launched the Patient-Reported Indicator Surveys (PaRIS) initiative in 2018 to measure health

systems performance through patient-reported outcomes (PROs) and experiences. This initiative has brought countries together in working towards developing, standardising and implementing a new generation of indicators to measure the outcomes and experiences of healthcare that matter most to people. Areas of focus include both generic measures for people living with chronic conditions, including cancer and specific measures for conditions or procedures such as Hip and Knee Replacement Surgery, Mental Health and Breast Cancer.

PaRIS has two main objectives:

1. Upscaling existing PROMs data collections for hip and knee replacement, breast cancer and mental health;
2. Developing a new international survey of people living with chronic conditions- the PaRIS survey.

Building on the lessons learnt from the pilot data collection in 2019 and 2021, the PaRIS Breast Cancer Working Group collected PROMs data by using the BREAST-Q Breast Satisfaction tool after breast conserving therapy and reconstruction. The results of the data collection were published in the 'Health at a Glance [2019](#) and [2021](#)', and the [technical report](#) on PROMs for breast cancer care was released detailing further analysis and interpretation of the findings.

Participating programmes collect their data in different ways, through electronic medical records or other online tools, such as a patient portal, so that patients can fill in their questionnaires at anytime,

although some use paper and questionnaires that are applied in clinical visits.

In 2023, the Working Group expanded its measurement tools by including pre-operative and post-operative scales from the BREAST-Q, EORTC QLQ-C30 and EORTC QLQ-BR23 questionnaires. Participating programmes submitted data on other dimensions such as physical well-being, emotional and social functioning and satisfaction with information.

Sample sizes have increased over time, with more countries participating in international benchmarking exercises in 2023. Some countries such as Sweden have even upscaled their performance data collection on breast cancer to regional or national levels.

OECD has been collecting data on breast cancer PROMs for the purpose of international benchmarking. However, small sample sizes and variations in data collection tools limit international comparability and interpretability of data. To guide policy decisions, further efforts are needed.

Experiences from PaRIS Breast Cancer PROMs stress five key learnings:

- Increasing countries and programmes show interest in the measurement and use of disease-specific PROMs, with PRO sample sizes and participating programmes rising year on year.
- We can capture the perspectives of patients, who use healthcare services at hospital. If we want to transform health systems, we need a broader participation, including those who are vulnerable/hard to reach.
- Collecting PROMs data is only the beginning. There is a need to work towards making PROMs actionable to improve healthcare. They can be part of clinical improvement cycles, quality improvement initiatives or they can guide policy decisions.
- Disease-specific measures provide an in-depth understanding in relation to a specific condition or diseases. While disease-specific measures are helpful, further progress on the collection and use of generic measures is needed. Besides physical functioning, people's global health-related quality of life, mental and social

functioning are also important.

- Advancing the use of PROMs in transforming health systems requires policymakers' commitment. Systematic collection and use of PROMs data require adapted data infrastructures, adequate training of patients, healthcare professionals, and integration into broader agenda of quality improvement strategies.

**Zoltán Kaló**, Professor of Health Economics, Center for Health Technology Assessment, Semmelweis University; and Lead Partner, Syreon Research Institute, emphasised that patient centricity and value-based healthcare are areas of significant focus for healthcare systems all over the world. Assessing health technologies for their value is important in order to ensure that investment in the most difference-making innovations can be sustained. Here PROs have an important role to play.

Ways to strengthen the patient voice include patient-centric evaluation criteria for health technologies and services, and patient engagement in the HTA process and policy decisions. However, a certain degree of misrepresentation is recognised in Europe as the patient's voice is usually highlighted more in Western European countries. Professor Kaló underlined that patient-reported outcomes and experience may not necessarily be the same in different countries, so it is key to pay attention to the heterogeneity of the patient voice. In the joint European Health Technology Assessment process, it is critical to develop a proper representation of the patient voice both from higher and lower income EU Member States.

The EU funded project [HTx H2020](#) (under Horizon Europe 2020) provides several recommendations for patient involvement in HTA in lower income EU countries, including:

1. Educate HTA/payer organizations on the value and good practices of patient involvement.
2. Acknowledge patients as experts on their condition similar to health care professionals.
3. Revise local HTA guidelines and procedures to facilitate patient involvement.
4. Nominate a dedicated person to be responsible for patient involvement activities with sufficient

capacities at HTA bodies.

5. Set a certain percentage of the public HTA annual budget to be spent on patient involvement as a goal.
6. Fair compensation for time and transportation should be provided for the patients involved in the HTA process.
7. EU-funded calls for the implementation of patient-centric evaluation of health technologies in countries with limited experience in patient involvement
8. Set up an open call for individual patients or patient organisations to register for involvement into HTA with a clear policy on managing conflict of interests.
9. Provide tailored training(s) and training materials for patients on HTA and local health policy decision-making procedures. Set up a working group of experienced organisations to act as a training centre for patient experts.
10. Educate patient organisations on collecting data and interpreting scientific evidence based on international materials.
11. Patient organisations should aim for a diversified portfolio of funders and publicly declare funding sources.
12. Normative state funding for NGOs with close auditing and detailed expectations from and responsibilities of patient organisations. Neither public, nor private funding should be banned by legislation.

**Katharina Beyer**, Postdoctoral researcher at Erasmus MC, BSc in European Public Health; MSc in Public Policy and Human Development, mentioned that one of the key challenges in measuring patient-reported outcomes is linked to standardisation. In this respect, she referenced the IMI project Pioneer which has investigated the use of big data in respect to the screening, diagnosis and treatment of prostate cancer patients in Europe. This has found that different PRO formats for the same disease condition can have different uptake levels depending on their perceived usefulness and relevance.

There can be a gap between a PRO that has high validity from the research perspective, and PROs with high validity for patients. In designing PROs therefore its vital that the patient perspective is fully taken on board.

**Mercè Cases Escuté**, Senior Research Manager, Patvocates, presented the [European Atlas of Clinical Trials in Cancer and Hematology \(EuroACT\)](#) which is a research project initiated by WECAN and the European hematology community. The project aims to understand the clinical trial landscape in the WHO European region, based on data extracted from all relevant European and Global clinical trial registers. Data from the past five years reveals differences where clinical trials have been run in European countries and gives insights into how and where PROs and quality-of-life (QoL) instruments have been used in clinical trials.

Results from EuroACT are guiding the understanding of inequalities and differences in the availability of clinical trial sites across European countries and are providing evidence on the use of relevant and meaningful QoL instruments in clinical trials.

What is being evidenced so far, and of relevance for the roundtable discussion, includes:

- The great variability in approach across Europe to publishing PRO data; and,
- The ongoing scope to make PRO questionnaires more relevant for particular disease areas.

**André Deschamps**, Past Chairman, Europa Uomo, confirmed that, from the prostate cancer perspective, important QOL data to collect and reflect upon is the impact from a patient's cancer, and their treatment, on their sexual function. Such data can be important for patients, clinicians and health systems to evaluate the best forms of treatment. Asking questions to patients on sensitive matters such as this emphasises the importance of providing patients the opportunity to answer questionnaires outside of the clinical setting, and rather at a time and place of the patient's choosing and convenience.

Mr Deschamps also raised the importance to use appropriate language for patients, including their mother tongue language.

# Patient-Reported Outcome Measures and the Current Political Context

## KEY POINTS

- The EU's '[Pharmaceutical Strategy for Europe](#)' supports the idea that patients are systematically considered in the authorisation process. It is hoped that such European Commission initiatives can support the patient centricity objectives inherent to the PROMs agenda.
- Patient-reported outcomes within Health Technology Assessment (HTA) procedures need to go further. Studies show ongoing reliance of HTA decision-making on survival indicators, with much data not considered or not even reviewed.
- The European Oncology Quality of Life toolkit (EUonQoL-Kit), to use in future periodic surveys for health policy intervention evaluation, will be a new unified system for the self-assessment of quality of life in cancer patients undergoing treatment, palliative care, and in cancer survivors.
- There is a continued variance in PROMs use across countries, in Europe and beyond. The environments in which patients live and work can be completely different, so country specification and cultural differences should be taken into account



**Kostas Papadakis, Head of Oncology Market Access, Bayer**

**Philippe Roux, Deputy Director General for Health, DG Health and Food Safety, European Commission**

**Jörg Ruof, Professor of Health Outcomes and Management, Hanover Medical School; Founder, European Access Academy**

**Cinzia Brunelli, Senior Researcher Palliative Care, Pain Therapy and Rehabilitation Unit, Fondazione IRCCS Istituto Nazionale Tumori; Chairperson, Organisation of European Cancer Institutes (OECI) Cancer Outcome Research Working Group**

**Matti Aapro, Past-President, European Cancer Organisation; President, All.Can**

This session was co-chaired by **Zorana Maravic** and **Kostas Papadakis** Head of Oncology Market Access Bayer. Kostas set the scene for the session by emphasising aspects of political context such as the ongoing implementation of EU HTA regulation, and the presentation of a new legal proposal by the

**Philippe Roux**, Deputy Director General for Health, DG Health and Food Safety, European Commission, applauded the themes of the roundtable, noting that the EU's '[Pharmaceutical Strategy for Europe](#)' supports the idea that patients are systematically considered in the authorisation process. It is hoped that such European Commission initiatives can support the patient centricity objectives inherent to the patient-reported outcome measures (PROMs) agenda.

In the past few years, the European Commission has worked on projects and initiatives around patient-centrality in cancer care. A few examples include:

- Since 2017, the European Commission has supported the OECD in conducting the [Patient-Reported Indicator Surveys \(PaRIS\)](#) initiative which aims to make health systems more patient-centred by supporting greater use of PROMs to understand health service quality.
- The EU Research Mission on Cancer has created a new project, the [EUonQoL Quality of Life in Oncology](#) project (funded by Horizon Europe). This project aims to develop, pilot and validate the EUonQoL-Kit, a patient-driven, unified system for the assessment of quality of life (QoL), a pilot programme that will validate identified systems for the assessment of quality of life across the EU-27.

- Via the [2023 EU4Health programme](#), the European Commission is supporting a call for tender for quality of life of cancer patients and survivors which complements the above-mentioned Horizon Europe project.
- The EU is also supporting the [European quality assurance scheme for breast cancer services](#), which includes requirements to have a policy to measure patient wellbeing throughout the cancer care pathway. It defines a common set of quality and safety requirements for breast cancer services to improve the care offered to women.

**Jörg Ruof**, Professor of Health Outcomes and Management, Hanover Medical School; Founder, European Access Academy, delivered the following key message: is that patient-reported outcomes within Health Technology Assessment (HTA) procedures need to go further. Too often it comes across that PRO data is only given lip service in importance.

In making this case, Professor Ruof reminded participants of the three main pillars of Evidence Based Medicine (EBM), namely:

1. Individual clinical expertise
2. Best external evidence
3. Patient values and expectations

It is to this third pillar that the use of PROMs can give special support. Yet, studies presented by Professor Ruof show ongoing reliance of HTA decision-making on survival indicators, with much PRO data not considered or not even reviewed. Professor Ruof also expressed concern that the present

EUnehta 21 Guidance document D4.4 – Endpoints downplays PRO data. This is further supported by questionnaires of patients on the degree to which they perceive they are involved in HTA decisions.

**Cinzia Brunelli**, Senior Researcher Palliative Care, Pain Therapy and Rehabilitation Unit, Fondazione IRCCS Istituto Nazionale Tumori; Chairperson, Organisation of European Cancer Institutes (OECI) Cancer Outcome Research Working Group presented the [EUonQoL Quality of Life in Oncology project](#). As part of the EU Research Mission on Cancer, the project aims at developing, validating, and disseminating the European Oncology Quality of Life toolkit (EUonQoL-Kit) among European cancer patients and survivors, to use in future periodic surveys for health policy intervention evaluation. It will be a **new unified system for the self-assessment of quality of life** in cancer patients undergoing treatment, palliative care, and in cancer survivors. It will be:

- developed from the patient perspective.
- digitally completed.
- available in several European languages, in static and dynamic versions.
- psychometrically sound.
- applicable to cancer survivors, patients still undergoing treatment and patients in need of palliative care.

The project will be based on participatory, co-designed research principles through the involvement of a representative panel of stakeholders, including patients and their caregivers.

**Figure 5. The EUonQoL Main Visual**



It includes the support of six patient co-researchers; a 12-member, multidisciplinary stakeholder board; and consensus conference and stakeholder fora.

One work package is dedicated to defining the methodology and providing organizational support through active engagement of citizens and patient representatives. It also includes healthcare providers and administrators, researchers with expertise in QoL and Data Science, industry, regulatory authorities, and policymakers.

The project started on 1 Jan 2023 and will end on 31 Dec 2026. It includes 25 EU Member States, and 5 (+ UK) associated countries.

- Key takeaways from this project include:
- Actionability of the areas investigated.
- Applicability across borders.
- Attention to future implementation.
- Integration with other EU initiatives and programmes.
- Need to integrate the approaches to promote QoL evaluation in different settings:
  - a. Clinical practice
  - b. Research and HTA
  - c. Quality assessment and benchmarking

Cinzia expressed her aspirations that the EUonQoL project can address the gap in PRO data and its use in improving health policy. This is especially the case as, unlike other forms of PRO discussed at the roundtable, EUonQoL focuses on PROs for the purposes of quality assessment and benchmarking.

**Matti Aapro**, Past-President, European Cancer Organisation, reflected on key messages he had heard during the roundtable. Among these was the continued variance in PROMs us across countries, in Europe and beyond. The environments in which patients live and work can be completely different, so country specification and cultural differences should be taken into account. Translation of PRO questionnaires can also lead to a change in focus in questionnaires between languages.

In concluding the roundtable, the Co-Chairs Isabel Rubio and Zorana Maravic highlighted the importance of so many international projects in PRO development share and connect with each other, sharing lessons and insights, and reducing duplication of effort where possible. It was hoped that the roundtable meeting made a contribution to that effort. Zorana especially mentioned the need to guard against creating new divides and inequalities in PRO use both between countries, but also between cancers. A multi-speed Europe in this regard should be prevented.

## FURTHER COMMENTS FROM ROUNDTABLE ATTENDEES

We'd like to thank the many attendees to the Community 365 roundtable for their active contributions to the discussion, including to the online chat, and to all those who provided written comments after the event. Further to the roundtable discussions, some of the additional recommendations that have been shared with the European Cancer Organisation are summarised below:

1. **Structured Training Programmes:** Develop and implement structured training programmes for clinical personnel on the interpretation and effective use of PROMs data. This training should include practical exercises, case studies, and guidelines on responding to various types of patient-reported data.
2. **Role Clarification and Workflow Redesign:** Clearly define the roles and responsibilities of different healthcare professionals in responding to PROMs. Redesign clinical workflows to integrate PROMs data review as a regular part of patient management. This can be facilitated by setting up dedicated sessions or incorporating PROMs data review into routine patient care discussions.
3. **First Responder Protocol:** Establish a 'first responder' protocol, where trained oncology nurses or allied health professionals such as social workers are designated to initially respond to PRO alerts. This ensures prompt attention to patient-reported issues and more efficient use of physician time.
4. **Investment in Technology:** Allocate funds specifically for the acquisition and maintenance of validated ePRO software. Ensure that the technology is user-friendly and accessible to both patients and healthcare providers.
5. **Systematic Evaluation:** Regularly evaluate the effectiveness of PROMs implementation programs in oncology clinics. This could include measuring patient satisfaction, treatment adherence, and the impact on clinical outcomes. Use these evaluations to refine and improve the PROMs process continually.
6. **Patient Feedback Loop:** Create a feedback loop where patients are informed about how their reported data is being used. This transparency can increase patient engagement and trust in the process.
7. **Integration with Electronic Health Records (EHRs):** Integrate PROMs data into the EHR system to allow seamless access and analysis by healthcare providers. This integration can also facilitate the tracking of longitudinal patient data and trends over time.
8. **Address the Digital Divide in the Context of Transitioning to ePRO Systems:** Implement hybrid systems that allow both digital and paper-based reporting. This should take into consideration additional factors and elements such as: accessibility features, digital literacy training, technology access programs, flexible reporting options, community-based support, constant monitoring and evaluation.
9. **Expand on the Aspect of Cost-effectiveness Analysis for Implementing ePRO Systems** via the following factors: conduct a comprehensive cost-benefit analysis for the adoption of ePRO systems; undertake comparative studies to evaluate the cost-effectiveness of ePROs versus traditional paper-based systems; start with pilot programs in select oncology centers to assess the cost-effectiveness of ePRO systems before wider implementation; use real-world data from existing ePRO implementations to inform results; engage with key stakeholders, including medical oncologists, nurses, psychologists, patient representatives, and health economists, to gain a comprehensive view; consider the recommendations and guidelines from international bodies; evaluate the long-term financial impact of ePRO systems on the healthcare system, including potential improvements in overall survival and long-term care costs.

## References

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2. US food and drug administration guidance for industry: patient-reported outcome measures: use in medical product development to support labeling claims. Rockville, MD: Department of Health and Human Services, Food and Drug Administration, Center for Drug Evaluation and Research; 2009
3. Patrick DL, Burke LB, Gwaltney CJ, et al. Content validity—establishing and reporting the evidence in newly developed patient-reported outcomes (PRO) instruments for medical product evaluation: ISPOR PRO good research practices task force report: part 1—eliciting concepts for a new PRO instrument. *Value Health*. 2011;14:1–9.

## FIND OUT MORE

- Roundtable recording  
<https://vimeo.com/842873473?share=copy>
- ESMO Clinical Practice Guidelines  
<https://www.esmo.org/guidelines/guidelines-by-topic>
- EMA Regulatory Science Strategy to 2025  
<https://www.ema.europa.eu/en/about-us/how-we-work/regulatory-science-strategy#regulatory-science-strategy-to-2025-section>
- European Atlas of Clinical Trials in Cancer and Hematology (EuroACT)  
<https://wecanadvocate.eu/projects-and-initiatives/euroact/>
- Patient-Reported Indicator Surveys (PaRIS) initiative  
<https://www.oecd.org/health/paris/>
- EUonQoL QUality of Life in Oncology project  
<https://euonqol.eu/>
- 2023 EU4Health programme  
[https://health.ec.europa.eu/publications/2023-eu4health-work-programme\\_en](https://health.ec.europa.eu/publications/2023-eu4health-work-programme_en)
- European quality assurance scheme for breast cancer services  
<https://healthcare-quality.jrc.ec.europa.eu/ecibc/breast-quality-assurance-scheme>

As the not-for-profit federation of member organisations working in cancer at a European level, the European Cancer Organisation convenes oncology professionals and patients to agree policy, advocate for positive change and speak up for the European cancer community.



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