

## Patient Voice

# Improving outcomes through patient-generated evidence—the next step in patient advocacy



Patient advocacy—a term that is broad in scope but that essentially describes a range of services for patients, survivors, and caregivers who are living with, or have lived with a disease. The paramount goal of patient advocacy is to truly represent the voice of patients to ensure their needs and preferences are met within an often complex and labyrinthine health-care system, which is rarely built to recognise—let alone accommodate—the needs of those who must attempt its navigation.

Recent decades have seen a transformation of patient advocacy and its role, predominantly through the establishment and development of patient advocacy organisations. Typically not-for-profit, these organisations focus on peer-to-peer support, health-care policy, and research, and are usually established and led by patients, caregivers, or others whose lives have been affected by a disease. While usually neither in the health-care profession nor experts in the traditional sense, these people have the best understanding of living with an often frightening and overwhelming disease. The very nature of the disease in daily life, its influence on ability to work and mental wellbeing, social life, the short-term and long-term impact of the condition and its treatment, are only a few of the many aspects that must be considered from a patient's perspective.

The substantial growth of the patient advocacy community has been only possible through the reiterative processes of learning, sharing, and collaboration. As organisations have grown, the knowledge and skills acquired have spread across the patient community, resulting in the emergence of many established models and programmes that continue this growth trajectory. Through initiatives such as the European Patients' Academy (EUPATI) and Patient-Focused Medicines Development (PFMD), all the essential ingredients for successful patient engagement are made accessible to various stakeholders, addressing the why and how of patient engagement through shared purpose and collaborative spirit, along with education, methods, frameworks, and tools. This maturation of the community has in turn carved a more precise role for patient organisations within the complex health-care landscape and gives patients a seat at the table in multi-stakeholder conversations, which allowed a major shift in multi-stakeholder spaces: a move from the outdated view that doctors, scientists, or policymakers are able to speak on the behalf of patients and can understand their perspectives, priorities, and needs adequately. A 2009 survey conducted by the patient organisation *Myeloma Euronet* among

patients, relatives, doctors, and nurses highlights this gap in perception and preference: for myeloma patients, hair loss, neuropathy, and respiratory problems, for example, were considered to be side effects with a major negative impact on their wellbeing, while doctors considered them to be less problematic, rating thrombotic events or jaw damage as substantially more serious.

This evolution within the health-care spectrum has enabled the patient community to understand new truths from their perspective too: recognition of the patient voice as an equal party in the dialogue around health care and policy is not enough to bring about the changes that patients require. For that patient voice is not one voice alone; organisations have the difficult task and responsibility of representing the needs and views of complex, heterogeneous subpopulations with a wide range of stakeholders with competing agendas and within a complex health-care system. Therefore, to ensure valid and incontestable representation, reliable, robust evidence on the needs and views must be collected and shared; it is the only way to have impact and gauge progress by measuring this evidence against the mission of a patient community. Consequently, this evidence must be generated and reported by the same patient community that it represents, and whose agenda is to protect the needs of that very patient community. There are meaningful datasets that patient organisations can generate, from data on treatment adherence, inequalities in access to diagnostics or therapies, current treatment patterns, quality of life, and burden of disease, to daily lived experience and the impact of illness on society, and disease-related outcomes or patient preferences on benefit and risk.

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For more on the **Myeloma Euronet survey** see [http://myeloma-euronet.lhrm.de/\\_dl/survey/Poster-939-Abstract-Detecting-Myeloma.pdf](http://myeloma-euronet.lhrm.de/_dl/survey/Poster-939-Abstract-Detecting-Myeloma.pdf)

For more on **patient-reported outcomes in cancer clinical trials** see *Health Expect* 2020; 23: 41–51



For more on the **Commission** see *Lancet Haematol* 2018; 5: e563–98

For more on the **WECAN Evidence-Based Advocacy (EBA) Programme** see <https://wecanadvocate.eu/eba/>

One lever for patients to directly influence health-care policy are patient-reported outcomes and patient-reported adverse events. Together with clinical evidence, these reports are used to inform decisions made by industry, clinicians, regulators, health technology assessment bodies, and other decision makers, but they can also support shared decision making and individual patient choice. However, patient-reported outcome and patient-reported adverse event data can only meet their purpose if all stakeholders commit to their use within the clinical and regulatory setting. The 2018 *Lancet Haematology* Commission showed that, despite evidence of substantial improvement in symptoms during first-line oncological therapy due to inclusion of such instruments, clinical trials in patients with haematological malignancies have not typically been incorporating HRQoL or other patient-reported outcome assessments. Indeed, data from National Cancer Institute-sponsored clinical trials between 2004 and 2016 show that less than 10% of clinical trials in patients with leukaemia, lymphoma, and myeloma included patient-reported outcome endpoints. This presents a huge opportunity for impact through evidence generation for the patient community, and since that publication, has remained a priority advocacy area for many patient organisations in this field.

Owing to this recognition of the substantial impact that data-driven advocacy strategies can have in areas such as access to new and improved therapies, clinical trials, and improved quality of life; evidence-based advocacy has quickly become a priority area of skill and knowledge sharing within the patient community. The Workgroup of European Cancer Patient Advocacy Networks (WECAN), has acknowledged this and, being well-placed in terms of reach and mission to provide training for the community, has conceptualised and implemented the Evidence-Based Advocacy (EBA) Programme, first launched in 2020.

WECAN was born out of recognition of the importance of organisation and collaboration within patient advocacy, and brought together 23 European umbrella organisations with the purpose of improving outcomes for all patients with cancer. WECAN's EBA Programme provides robust training to support European cancer patient advocacy networks to develop knowledge and capabilities in generating and publishing patient evidence and data.

The programme equips patients with an understanding of how to use data in advocacy, knowledge of appropriate methods and types of evidence, and how to publish evidence for impact; and provides education and resources to effectively grow capabilities in evidence-based advocacy in a way that meets the current and future needs of the cancer patient community.

Since data must not only be generated and presented, but also published, WECAN, together with Envision Pharma group, offers open-access, virtual publication training called Patients in Publications. First launched in 2021, the training aims to equip patient advocates with the necessary tools to act as authors, co-authors, and peer-reviewers, on an equal standing with academic authors.

2022 sees the introduction of the next chapter of the EBA programme, with the original course converted to an online learning environment on the WECAN e-learning platform, which is accessible to all members, and the launch of an interactive practical workshop series, in which participants develop a real-world plan for evidence generation and use in advocacy and learn practical skills that will help with identifying research goals in line with community needs and priorities. This exciting next step in the programme marks a crucial milestone in evidence-based patient advocacy and its role in shaping health-care systems within and across Europe.

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