SYSTEMS CHANGE

Patient agency: key questions and challenges – A report from the 1st workshop of the EHC Think Tank Workstream on Patient Agency

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Introduction: Patient agency refers to the abilities and capabilities of patients to act, contribute, influence and make decisions about their healthcare. It depends on both the willingness of patients to participate and the constraints imposed by healthcare providers, services and systems. To determine the factors affecting patient agency, especially for patients with chronic, rare diseases such as haemophilia requiring lifelong care, it is important to consider the patterns, structures, and mental models that define the ecosystem that patients are a part of, irrespective of their level of engagement. **Identifying key challenges:** At the first workshop of the EHC Think Tank Workstream on Patient Agency in December 2021, participants identified five key themes for in-depth discussion relevant to patient agency: the concept of shared decision-making (SDM), patient empowerment, the spectrum of engagement, cultural change and health literacy. The Iceberg Model was used to unpack challenges by identifying composite factors on four levels: events, patterns, structures and mental models. **Summary:** Across the five themes, four common perceived challenges stand out: uneven relationships between patients and healthcare professionals, services and systems; paternalism and hierarchical cultures; failure to recognise problems; conservatism and resistance to change. Despite some progress towards patient empowerment, a 'glass

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Uneven patient-clinician relationships, paternalism, hierarchical cultures and resistance to change are among the key challenges identified in ensuring that patients with lifelong rare diseases, such as haemophilia, are empowered to engage with and share in decision-making around their care

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ceiling' prevents patients from driving transformation and taking leadership roles in strategy, policymaking and governance. Patient engagement is fluid and those who could benefit most are least likely to engage. Health literacy is perceived as the problem of the patient, not the system, and patients rather than healthcare providers are typically expected to adapt. Preliminary suggestions for addressing these challenges include behavioural communication training for patients and healthcare professionals, a learning system for empowered patient and family care, and a level playing field for stakeholders to interact equally, leading to mutual acceptance and respect.

Keywords: *Patient agency, Empowerment, Engagement, Cultural change, Health literacy*

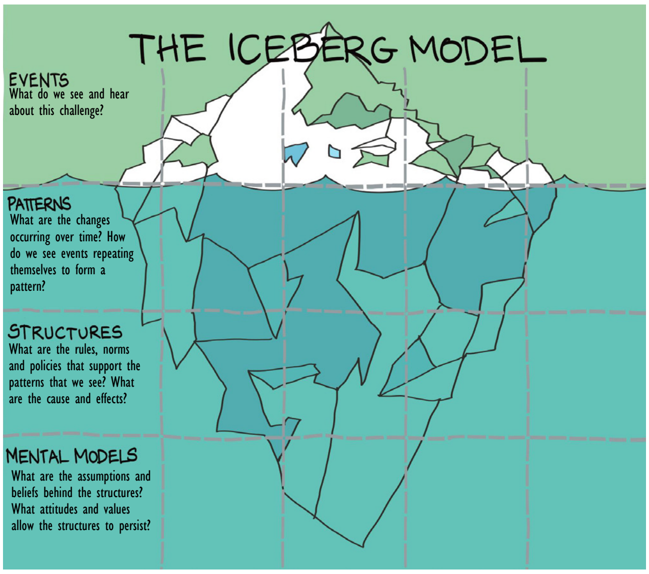

Figure 1. Iceberg Model template used to identify events, patterns, structures and mental models in challenges for patient agency. Image: Ia Brix Ohmann / Overlap (*https://www.overlap.dk/english*)

Patient agency refers to the abilities and capabilities of patients to act, contribute, influence and make decisions within the healthcare system in which they find themselves. Such agency is dependent not only on patients' willingness to participate but on the constraints they encounter [1,2]. To determine the factors affecting patient agency, it is therefore necessary to consider the patterns, structures, and mental models that define the ecosystem that patients are a part of, irrespective of their level of engagement. This is particularly important for patients with chronic, rare diseases such as haemophilia who are likely to have lifelong interactions with healthcare providers, services and systems.

The first workshop of the European Haemophilia Consortium (EHC) Think Tank Workstream on Patient Agency marked the start of a sensemaking process for all involved stakeholders to identify and examine relevant variables and challenges within the healthcare system as a basis for creating change.

At the virtual workshop on 16 December 2021, the 20 members of the Patient Agency Workstream identified five key inter-related themes for in-depth discussion relevant to patient agency: the concept of shared decision-making, patient empowerment, the spectrum of engagement, cultural change, and health literacy.

Group discussions took place using the Iceberg Model, a method of unpacking challenges by identifying composite factors on four levels: events, patterns, structures and mental models (Figure 1). The Iceberg Model helps to expand the perception of a situation within the context of the whole system instead of being limited to a single activity or event [3]. It is designed to help people step back and identify the different patterns that the event is part of, the possible structures that might be causing it to occur, and finally, the mental models or perceptions and thought processes that underpin and therefore create those structures.

1. CONCEPT OF SHARED DECISION-MAKING

Shared decision-making (SDM) is a collaborative process that involves a person and their healthcare team working together to reach joint decisions about their care, based on clinical evidence and the patient's informed preferences [4,5]. Multiple tools are available and more are being developed to aid SDM. However, there is not a standardised methodology and data on effectiveness is limited, resulting in a lack of systematic learning on what is most helpful. The healthcare professional (HCP) is often seen as the giver of information and the patient as the recipient – patients may be heard in this scenario, but it does not mean they are listened to [2,6]. For true SDM to occur, a balanced dialogue is necessary with contributions from both patients and HCPs, often with input from multiple specialists cooperating together [7]. This can only occur if patients and HCPs are aligned on the meaning of SDM. Simply giving patients a choice of treatments and allowing them to decide is not SDM.

SDM is affected by culture and hierarchy and may evolve with time. Patients with rare and chronic conditions, such as haemophilia, become more knowledgeable about their illness and treatment through their lived experience [8], and this expertise should be valued and encouraged by their care team [9]. Such patients may be more knowledgeable than non-specialist HCPs involved in their care, which may be difficult for them to accept. Patients will have different insights on the impact of their disease and treatment on their daily life, and SDM needs to take account of this [6]. By taking a holistic approach, HCPs will be able to frame the clinical discussion in a way that takes better account of the patient viewpoint.

Some patients may be reluctant to get involved in SDM and prefer their HCP to make decisions for them, especially if they have already experienced a loss of control over their body and health. However, providing developmentally appropriate, culturally relevant opportunities for such patients to become involved in care decisions remains important and may enable SDM at some level [10,11].

Building trust between patients and HCPs is essential for a balanced and equal dialogue for SDM [9,12], yet this can be challenging if there is a lack of continuity of care [13]. Clinicians need to be honest with patients and with themselves about what is known about a disease and its treatment and what is not. Both patients and HCPs also need to accept that they can make mistakes in their decision-making and they need to allow for uncertainty [7]. A request for a second opinion may appear to question trust between patient and HCP but needs to be accepted as part of SDM.

Good communication is an essential part of SDM [9,10,14]. Research in Denmark has shown that making audio recordings of consultations and keeping them in patient notes is a valuable reminder to both patients and clinicians of what has been discussed and can help to create a more even dialogue [15].

In conclusion, understanding underlying character traits, social influences and hospital structures can help to ‘even out’ the conversation between patient and clinician for SDM. Creating collective intelligence focused on the real-world experiences of patients, for example through audio recordings of consultations, is likely to be more effective for SDM than use of decision-making tools with uncertain value. Behavioural communications training is needed for patients and HCPs so that all are able to speak the same language and facilitate knowledge transfer [8].

2. PATIENT EMPOWERMENT

Patient empowerment has been defined as a process that helps people gain control over their own lives and increases their capacity to act on issues they define as important [16]. Patient groups including the European Patients’ Forum and EURORDIS–Rare Diseases Europe advocate for patient empowerment as it underpins patient engagement and involvement, and is seen as a pre-requisite for patient-centred care – without empowerment, a patient cannot be engaged [15,17,18].

In recent years, considerable progress has been made in advancing patient empowerment, through growth in patient knowledge and understanding, and more permanent and systematic engagement through institutions such as the World Health Organization (WHO) [19]. In Europe, the Innovative Medicines Initiative (IMI) is making a substantial contribution to patient empowerment with projects such as EUPATI [20], PARADIGM [21] and PREFER [22], which bring stakeholders together to develop tools to support education and engagement in the research and development of new medicines, and provide widespread opportunities for patient empowerment. In the United States, the Patient-Centered Outcomes Research Institute (PCORI) supports patient empowerment through patient-focused clinical effectiveness research [23], while in Canada patient empowerment initiatives include the development of a competency framework for collaborative practice and patient partnership in healthcare and social services [24].

Patient empowerment is growing in multiple areas of healthcare, including services and policy, research, education and regulation, driven by greater capacity and better methodology, science, processes and tools. However, while a change in mindset has given greater recognition to the value of patient experience and engagement, there is a need to continue to demonstrate the benefits of patient involvement in outcomes. All stakeholders, including patients themselves, need to know that patient input makes a difference. This means collecting and sharing concrete evidence of the value of patient experience in improving outcomes. Ongoing research is investigating how this can be achieved most effectively [25]. Sharing best practice will encourage others to implement approaches that have been shown to work.

Patients are increasingly involved in leadership and governance roles, and patient organisations are well placed to contribute to debates around the re-shaping of healthcare systems [17]. However, there is a glass ceiling which can act as a barrier for patient involvement in decision-making in this context. Structures are still needed to ensure that progress in patient empowerment is not lost and beneficial changes are permanent. Patients need to be involved at all levels – from new patients with fresh experiences to professional patient advocates with many years of experience.

Despite advances in patient empowerment, not all patients are benefitting. While the digital revolution brings a multitude of sources of information to patients' mobile phones, tablets and computers, it can be difficult to know which are accurate and reliable. If patients are to be fully empowered, they need guidance about the sources of information they can trust [26,27]. In the case of haemophilia care, for example, access to clear, unbiased information is essential for patients, particularly in a treatment landscape that includes novel therapeutic options [28]. Where internet access is limited, unavailable or beyond individual means, some patients may be left behind by the digital revolution and unable to access online information or other digital resources relevant to their health and care [29,30]. These patients also need support and it is important that learning systems take account of the needs of patients, families and carers wherever they live and whatever their level of education.

Enabling patient knowledge and cognitive skills forms a key part of empowerment but is not enough on its own to realise patients' active participation in healthcare delivery. A positive relationship with the healthcare provider is also needed, and education to promote patient empowerment should not be limited to patients [17,31]. HCPs need to be familiar with the evidence supporting the value of patient involvement, and they need training to understand how to facilitate and encourage patient empowerment.

In conclusion, there is growing capacity for education, competency and knowledge aimed at advancing patient empowerment but it needs to be reinforced, integrated and embedded into healthcare services and structures. Patients need to be the drivers of transformation through leadership roles in strategy, policymaking and governance, with the aim of building and sharing practices and evidence so that patient empowerment becomes systemic at all levels (care, education, research, regulators, etc.).

3. SPECTRUM OF ENGAGEMENT

The concept of the spectrum of engagement is inherently linked with patient empowerment (which is necessary for engagement) and SDM. There is a broad spectrum of patient engagement, ranging from patients who do not wish to engage or are unable to engage through to those who are very highly engaged.

At the non-engaged end of the spectrum, patients may be passive and expect to be told their diagnosis and how they will be treated, with no input from themselves. At the other end of the spectrum, patients may feel they know more about their illness and treatment than their doctor and should therefore be directing decision-making (i.e. beyond SDM) [14,32].

The question is whether non-engagers choose not to engage or i) face barriers of health literacy and do not want to admit their lack of understanding, ii) struggle to communicate, or iii) do not want to challenge their doctor. Cultural factors are likely to affect a patient's level of engagement, especially their willingness to challenge their doctor. This may similarly affect a clinician's ability to engage with patients; some welcome the opportunity to engage with a well-informed patient while others may feel uncomfortable being challenged [13,14]. Issues connected with cultural change are discussed further in section 4, below.

Patients who are more engaged in their healthcare often experience better health outcomes than those who are less engaged [33]. Related to SDM, they may also benefit from a focus on outcome measures in their care that are particular to their individual needs and goals [34,35]. In haemophilia care, for example, there is evidence that patient engagement and involvement in care decisions, combined with this kind of focus, can lead to better outcomes and better quality of life [36]. Patients who do not – or who are not enabled to – engage are likely to be the most disadvantaged.

Engagement may be affected by a patient's mental health. If their mood is low they may not want to engage or, if they have a chronic disease, they may see little point in engaging if they do not believe anything will work better than their current treatment. When the clinician sees a patient, they only witness a 'snapshot' of their level of engagement in relation to their healthcare. This may belie their (dis-)engagement in other and multiple aspects of their lives, such as in their work or personal relationships – but this also needs to be considered alongside any (dis-)engagement with their condition and treatment.

Patients with chronic diseases may appear disengaged but simply know what works for them without discussing it with their doctor. System structures such as payer recommendations may also affect engagement: if a patient knows that cost of treatment is a barrier to access this may affect their willingness to engage.

A patient's level of engagement may well change over time, and opportunities for engagement may also change. For example, patients may be more likely to engage at the time of diagnosis and initial decisions around treatment and, subsequently, as and when there are changes in their treatment. Age may play a part: older patients may still be accepting of paternalism in healthcare and assume 'doctor knows best' [13], while younger patients may be more questioning and engaged with HCPs and with support networks, and have better access to digital information. Time may be a factor, with individuals who are busy with career and family not having time to engage as regards their health. There is also a risk that if the 'patient voice' is based on the experiences of highly educated and knowledgeable patient advocates, the needs of those who are at very different places on the spectrum of patient engagement will not be met.

In conclusion, a patient's level of engagement is fluid and dependent on where they are in life. The question is how to enable engagement at all stages of the patient journey. It is important to enable all patients to engage, regardless of their background and circumstances. Different methods are needed to ensure this, alongside a greater understanding of the barriers to engagement (e.g. health literacy, language, wider life issues) and the opportunities for engagement (e.g. when new treatments become available). People who stand to benefit most from better engagement are often those who engage the least.

4. CULTURAL CHANGE

The discussion of cultural change – or the need for cultural change – concerns both individual behaviour and the impact of cultural differences within and between health services and systems, communities and society. As such, it considers how these elements relate to each other, and how the nature of these relationships impact on patient agency. The challenge is to identify how best to 'reset' the processes underlying cultural differences and, with so many options for cultural change, to prioritise areas of greatest need.

With the need to address inequities so that patients are empowered to access the care they need and are involved in treatment decisions, the immediate focus of cultural change may be on how it affects the patient. However, it also needs to consider the broader context in which the patient lives and functions (societal), and the service and systems level environments that impact patient agency [2].

At service level, there is a need to address cultural barriers that prevent patients accessing and being involved in their care due to a lack of appropriate policies and patient education. At system level, there is a need for long-term awareness building around rare diseases such as haemophilia, particularly in low- and middle-income countries (LMICs) [37], with data generation to support policy development and budgetary requirements for treatment. Cultural barriers may also prevent patients from being equal partners at societal level. For example, in some countries and communities, women face cultural barriers in accessing care for themselves or, as primary caregivers, in accessing care for family members or others. At an individual level, patients may not realise that they can live better with their illness (e.g. living with treatment side effects instead of seeking change).

Patient organisations, such as the EHC, have an important role to play in moving the agenda forward and ensuring consistent, standardised treatment across the region. Medical training needs to discourage paternalistic approaches and the sense that the doctor is 'in charge' of care. Instead, training should encourage doctors to think about how to empower patient involvement in their care.

An organisation-wide approach is key to achieving the cultural change that can promote patient empowerment [38]. However, difficulties may arise when there is lack of understanding between stakeholders about how to bring about that change. Different stakeholders may have fixed ideas about their cultural position and efforts to align viewpoints may turn into struggles for position and confrontation, especially in hierarchical, 'top-down' organisations. Respect for different roles and social positions and a willingness to see problems from different perspectives are essential for progress, and resistance to change and fear of moving out of comfort zones need to be addressed.

In conclusion, systems have a tendency towards self-preservation and, in the case of healthcare, this is rooted in a legacy of paternalism and hierarchical structures. Meaningful change, such as empowerment of patients in their own care, requires a paradigm shift in approach that can only be achieved by identifying, connecting and nurturing true innovators and giving them space to redesign services, showcase new practices, and demonstrate they can be successful [37]. A balance needs to be struck between standardisation of care and a tailored approach. Systems are built according to the needs of patients with more common conditions, so it can be difficult to introduce cultural change that recognises the needs of those with rare diseases such as haemophilia. There is a need for a level playing field where stakeholders are able to interact equally with each other – recognising the contributions that all can make – as this will facilitate mutual acceptance and respect.

5. HEALTH LITERACY

Although there is increased recognition of the importance of health literacy at a policy and global level, for example, through WHO and EU initiatives [19,39], many clinicians believe that, as they already work in a patient-centred way, there is no need to do more. There is also an attitude that low health literacy is the problem of the patient not the system, which implies that the onus is on patients to improve their literacy rather than on healthcare providers to adapt to the different levels of health literacy of their patients. Reframing limited health literacy as a challenge for healthcare providers rather than being a patient problem is key [40]. Some level of paternalism remains which can impede efforts around health literacy, SDM and patient empowerment [11,41]. Arguably, the Covid pandemic has inhibited health literacy: the higher the level of pressure on the healthcare system, the greater the risk of reverting back to a paternalistic approach [2].

There is confusion about health literacy and digital literacy – while they are linked, they are inherently different. With the advent of digital transformation, there is an assumption that people who are digitally literate are also health literate, but that is not always the case. Digital technologies may have enabled increased access to health information and healthcare applications, but this does not mean that everyone has the knowledge or capacity to access them effectively [11]. With the growing range of treatments available for haemophilia, for example, how are patients going to make the right decisions without good health literacy? To achieve health literacy, patients need high quality information but it can be hard to identify reliable, accurate information within the vast range of resources now available to them online. Clinicians and healthcare systems have a role to play in ensuring this [26].

It may be argued that it is impossible for people to be truly health literate unless they have a condition from birth and gradually acquire complete knowledge, or they study medicine. However, there are aspects of health literacy, such as an understanding of risk and benefit, that are useful for everyone for discussions of treatment options and decision-making. There may need to be a compromise between the optimal clinical course of action and what the patient feels able to do, and that may shift depending on the level of risk and the seriousness and severity of the disease. Patients need to be able to define their needs and goals and the outcomes that matter to them – bringing person-centred care together with value-based care [31,34].

There is also a need for clinicians to be cognisant of health literacy issues as they have a key role in helping to overcome them [42]. In the clinic setting, it is important that health-related concepts are communicated, both verbally and in printed information, in a way that patients with different levels of health literacy can understand [43]. For example, in explaining the differences between absolute, relative and individual risk to a patient, a doubling of risk sounds worrying; presenting it in the context of whether the risk is 1 in 5 or 1 in 1000 helps to clarify this. Greater awareness of patient health literacy among clinicians should aid more effective communication and more productive engagement with patients in the clinic setting.

Health literacy needs to work at a systems level too. For example, before a consultation, patients should be sent relevant test results and supportive information so that they are prepared for seeing the HCP, with sufficient consultation time to optimise their understanding. However, even when patients have good levels of health literacy and understanding of their diseases and the types of treatment, this becomes worthless if they are not able to access therapies from which they could benefit. The strong focus on processes within systems is problematic in this respect – for example, reimbursement structures may impact on or prevent individual choice and SDM [34].

In conclusion, health literacy affects all stakeholders (individual patients, physicians from junior and family doctors to hospital specialists, and service providers) so all their needs for education and training must be addressed and met, with resources allocated to facilitate this [41]. At a systems level, relevant structures also need to be in place to support this: the system needs to be health literate too.

SUMMARY AND NEXT STEPS

Across the five themes discussed in relation to patient agency, four common perceived challenges stand out: uneven relationships between patients and healthcare professionals, services and systems; paternalism and hierarchical cultures; failure to recognise problems; conservatism and resistance to change. Underlying character traits, social influences and structures within healthcare settings contribute to uneven conversations between clinicians and patients during SDM, often with a failure to recognise patient expertise and experience and to build trust. Despite some progress towards patient empowerment, a 'glass ceiling' continues to prevent patients from driving transformation, taking leadership roles in strategy, policymaking and governance, and ensuring empowerment at all levels (care, education, research, regulators, etc). A patient's level of engagement is fluid depending on their stage of life, and greater understanding is needed of the main barriers (e.g. health literacy, language, wider life issues) so that tailored interventions can be developed. People who stand to benefit most from engagement are often those who engage least. In relation to cultural change, healthcare systems remain rooted in paternalistic and hierarchical approaches, and individual, community and societal cultural differences can also be challenging. A level playing field is needed so that stakeholders can interact equally, leading to mutual acceptance and respect. Health literacy is perceived as the problem of the patient, not the system, and patients rather than healthcare providers are typically expected to adapt. However, health literacy affects all stakeholders (patients, service providers and systems), and cultural and educational changes are needed to overcome barriers to health literacy.

In order to expand understanding of these themes and the challenges associated with them, the next steps are to look more closely at the events, patterns and structures identified, and to validate the findings with a broader group of external stakeholders before bringing their perspectives back to the Workstream on Patient Agency.

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THE EHC THINK TANK

The European Haemophilia Consortium (EHC) Think Tank was launched in June 2021. Building on existing advocacy activities, the initiative brings together a broad group of stakeholders who will engage with key thematic areas or workstreams identified as priority areas for "systems change" within European healthcare systems [17]. The EHC Think Tanks seeks to mobilise the agency and purpose of all stakeholders in the healthcare system to collectively design and champion potential solutions to existing problems.

The EHC steering committee was presented with more than 20 topic areas identified from patient, medical and scientific volunteers within the broad community. Following a prioritisation process in early 2021, three key topic areas were identified for Think Tank workstreams to tackle:

- Registries
- Hub-and-spoke treatment models
- Patient agency.

Workstream members are invited based on their expertise and potential for constructive engagement, including patient and industry perspectives alongside a balance of healthcare professional, academic, regulatory, governmental and geographical representation. All workstream activities are held under the Chatham House rule to enable inclusive and open discussion: participants are free to use the information received, but neither the identity nor the affiliation of the speakers, nor that of any other participant, may be revealed [18].

Each is project-managed from within its individual membership. Members will set their own agendas, timelines, and targeted outputs, with operational, logistical, methodological and facilitation support from EHC staff and Think Tank practitioners.

While concrete outcomes and results will vary across workstreams, they are likely to include (but not be limited to) manuscripts, consensus-based guidelines, monographs, white papers, and so on.

https://www.ehcthinktank.eu

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