

System change in practice: A report from the EHC Think Tank workstreams on Registries and Patient Agency

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Introduction: System change addresses the fundamental causes of persistent, complex problems to achieve large-scale, sustainable solutions at multiple levels. Recognising the need for system change to ensure equitable access to healthcare for people with bleeding disorders and other rare diseases, the European Haemophilia Consortium (EHC) established a Think Tank to work alongside its traditional advocacy initiatives. The Think Tank has mobilised a broad range of healthcare stakeholders to identify challenges and co-create potential solutions through a series of thematic workstreams exploring specific aspects of the healthcare system. This paper reports on outcomes and learnings from the Registries and Patient Agency workstreams. **Methodology/Process:** During a series of online meetings and face-to-face discussions, workstream stakeholders contributed to a three-phase process: 1. Discovery; 2. Strategy; 3. Innovation. Having identified key challenges to system change for Registries and Patient Agency,



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Through multistakeholder collaboration, EHC Think Tank workstream members have developed strategies for system change to address challenges around lack of common purpose and data quality in patient registries, and relating to the role of the patient and patient influence in the healthcare system

stakeholders mapped the system in which they were working to refine the challenges, recognise enablers and constraints to progress, and use leverage points to co-create strategies for change. **Results:** The Registries workstream prioritised actions to address challenges around lack of common purpose and data quality, and agreed to move forward with developing a consensus statement to facilitate buy-in from key stakeholders, working on good governance for registries, hosting a network for registry owners, and creating a model for patient data input and feedback. A Registries

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Roadmap was completed for 2025 and 2030, and a project initiated to align registries in Europe. The Patient Agency workstream agreed actions should focus on challenges related to the role of the patient, recognising the need to elevate patient influence in all aspects of the healthcare system. Actions aimed to address the current stakeholder hierarchy and gaps in patient health literacy, and to optimise the potential of digital tools to enable patient contributions to patient-reported outcome and experience measures (PROMs and PREMs). Projects include developing a patient agency guidebook and a patient experience data (PED) dossier on von Willebrand disease, to provide a one-stop repository for regulators, researchers, clinicians and patients. **Conclusions:** There is a clear need for system change to ensure equitable access to healthcare for people with rare diseases such as bleeding disorders. Bringing together multiple stakeholders with different and complementary knowledge and approaches has facilitated the development of innovative strategies for system change in relation to Registries and Patient Agency. Work has started on pilot projects to move these strategies forward.

Keywords: *Registries, Patient agency, Mapping, Co-creation*

System change addresses the fundamental causes of persistent, complex problems in order to achieve large-scale, sustainable solutions at multiple levels ^[1]. Rather than giving rise to new systems, system change focuses on changing the current system so that it remains effective and relevant to the changing context in which it exists. As such, it considers the whole ecosystem, with a particular focus on relationships between stakeholders and enabling multiple routes towards agreed goals ^[2,3].

In healthcare provision, there is an almost continual cycle of change in response to ageing populations, evolving patterns of disease, and technological advances ^[4]. However, this frequently fails to take account of patient needs and expectations, or to involve patients in decisions about care provision or the quality of life-related outcomes that matter to them ^[5]. The European Haemophilia Consortium (EHC) recognised the need for system change to ensure equitable access to healthcare for people with rare diseases such as bleeding disorders. Historically, the EHC has used traditional, campaign-driven advocacy to draw attention to the unmet needs of people with

bleeding disorders. Traditional advocacy approaches focus on implementing pre-determined solutions to identified problems and processes; however, the rapid pace of change in treatment and care for bleeding disorders means reactive advocacy alone is not enough, and there are too many unknowns for proactive advocacy to address. Therefore, in 2021, the EHC Think Tank was established to work in parallel with longstanding traditional advocacy initiatives.

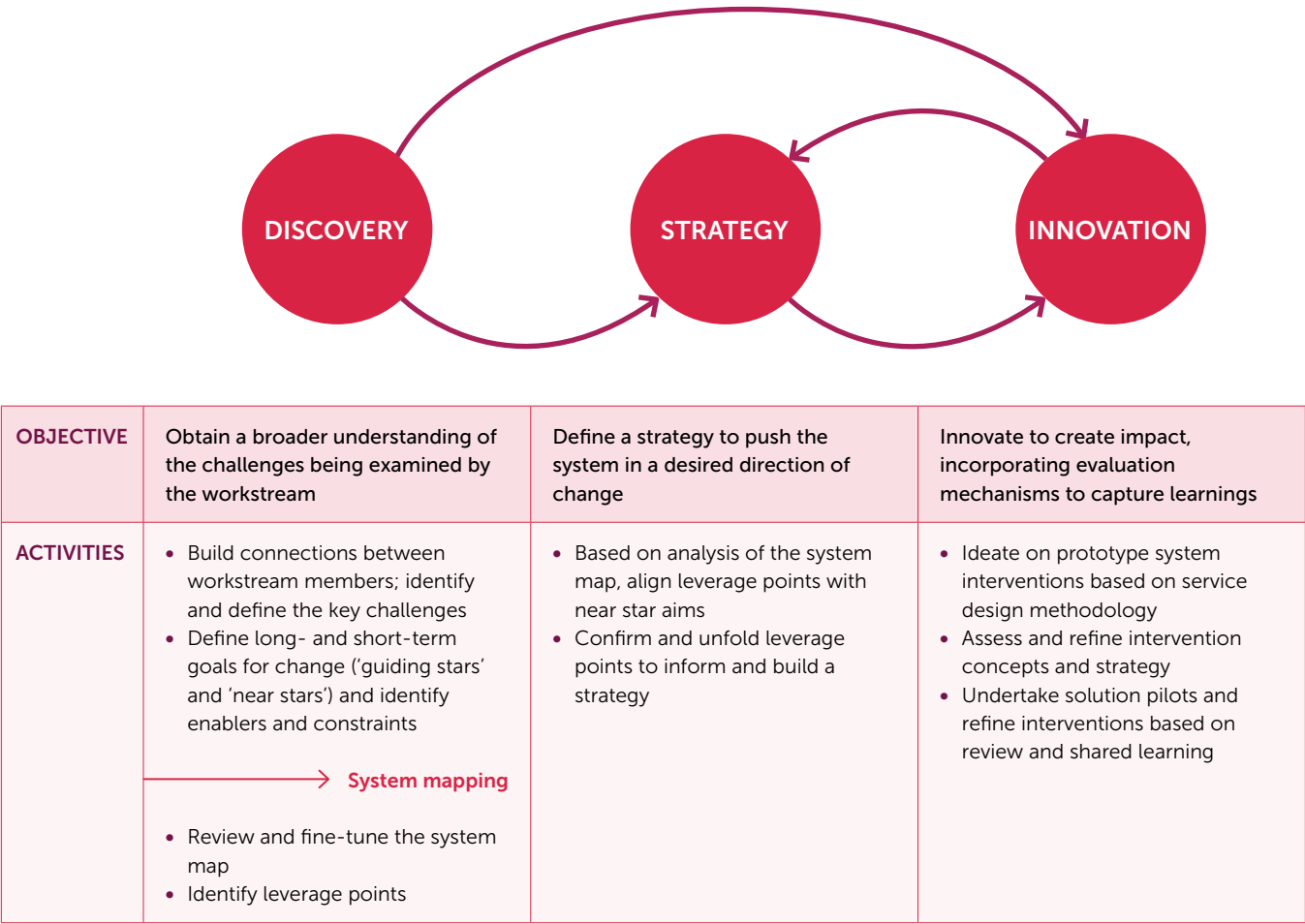
By targeting system change, the EHC Think Tank seeks to mobilise the agency and purpose of all healthcare stakeholders, including patients, to identify challenges from their collective perspectives and to co-design potential solutions and trajectories of positive future change, that can be co-owned, co-championed and co-implemented. The Think Tank initiated a series of thematic workstreams to explore specific aspects of the healthcare system, including Registries, the Hub and Spoke Model, Patient Agency, Access Equity and Future Care Pathways. Stakeholders representing health care providers, patient groups, regulators, policymakers, research and industry participated in workshops to identify key challenges ^[6-10], and short- and longer-term goals for change ^[11-12], together with the enablers and constraints likely to affect progress. This report summarises the subsequent progress of two workstreams, Registries and Patient Agency, towards strategy development and initiation of activities and projects designed to lay the foundations for system change in relation to the challenges that were identified by workstream participants.

PROCESS/METHODOLOGY

The EHC Think Tank developed a process based on system change and design thinking methodologies, and co-creation principles, to embark on creating change collaboratively with workstream members. The process comprises three phases: 1. Discovery; 2. Strategy; 3. Innovation. The broad objectives and activities for each phase are summarised in Figure 1.

Interactions with workstream members took place predominately in online workshops, but also at face-to-face events. Connecting stakeholders to stimulate development of trust and relationships was an important element of the workshops, and central to enabling creativity and long-term visionary work ^[13]. During the 'Discovery' phase, the Iceberg Model ^[14] was used to unpack the challenges that affect the management and utilisation of patient registries by identifying composite factors on four levels – events, patterns, structures and mental models – that

Figure 1. Workstream methodology
Summary of broad objectives and activities for EHC Think Tank workstreams



contribute to or are related to the challenges. The same model was used to explore the challenges involved in integrating patient agency (e.g. shared decision-making, patient empowerment and engagement) within health systems. Having carried out this detailed analysis of the challenges they faced, the workstreams set a direction for future work by identifying long- and short-term goals (guiding and near stars) and the enabling and constraining factors likely to affect progress towards these goals.

For each workstream, stakeholders then mapped the system in which they participated to refine the challenges, enablers and constraints. By visualising the interactions between each challenge and its related constraints and enablers and seeing how these, in turn, interact with other challenges and their related enablers and constraints, it was possible to identify and prioritise leverage points through which change might be achieved most effectively. At further online meetings, strategies were developed and elaborated to define the desired direction of change.

An Innovation Summit was held at which participants in the Registries and Patient Agency workstreams explored potential system interventions based on service design methodology. During this innovation phase, pilot projects were proposed and work initiated, together with dissemination of shared learnings and agreed next steps. At each stage of the innovation process, stakeholders reviewed progress and considered how best to optimise the impact of their interventions, discussed feedback, and further refined strategies and projects as appropriate.

RESULTS/OUTCOMES

Registries

Registries are instrumental for knowledge about bleeding disorders for patients, healthcare professionals, payers, insurers, researchers and policymakers. Registries can be used to generate epidemiological data about a disorder and demonstrate the efficacy of therapeutic and other management options, as well as being a significant enabler of clinical

research. As previously described, the Registries workstream identified key challenges for bleeding disorder registries to fulfil their potential identified as: 1. The multiplicity of registries and datasets; 2. Data quality; 3. Data sharing; 4. Expanding the scope of registries; 5. The role of the patient in registries ^[3]. The long-term goal for system change, agreed upon by the Registries workstream, was to establish rights- and responsibility-based international guidance to ensure accountability from all stakeholders contributing, collecting and handling registry data ^[8], with shorter-term goals focused on:

- Mapping the system
- Changing the narrative (to enable a data-sharing culture)
- Encouraging alignment
- Good governance.

System mapping by the Registries workstream added to and expanded on the challenges previously described, and identified the associated enablers and constraints ^[15]. For example, it was agreed that a lack of common purpose, with different stakeholders being interested in different questions and collecting different data could limit use of individual registries. This could result in even more registries being established, adding to the challenge of the multiplicity of registries and datasets. In addition, stakeholders recognised that finding sustainable sources of funding to develop and maintain registries was a considerable challenge.

Alongside system mapping, an exercise mapping registries for rare bleeding disorders in 18 European countries was undertaken as a first step towards defining a pan-European governance structure which would enable more effective use of real-world data for evaluating new therapies ^[16]. Considerable variation was reported in the type of data and level of detail being collected by the registries included in the mapping exercise. Although data on treatment and bleeding events were good, data on socioeconomic status and quality of life were limited. The most significant barrier to collaboration between registries resulted from differences in legal regulations for data sharing and patient consent procedures. Setting up a well-defined governance structure, agreeing on common goals, standardising clinical report forms, and synchronising data collection will therefore be important for future collaboration between registries.

Following the completion of system mapping, it was agreed to prioritise two key challenges for intervention:

Lack of common purpose: This hampers registry effectiveness due to sub-optimal governance, variability of data definitions, and conflicting stakeholder interests. Participants proposed strategies around organisational governance, harmonised language, defined outcomes and collaborations. It was agreed that European Union (EU) initiatives, such as the European Health Data Space ^[17], are useful enablers for encouraging shared purpose. Involving patients and stakeholders and providing educational opportunities may strengthen positive dynamics and collaboration, and European Medicines Agency (EMA) workshops may facilitate progress. Interoperability and stakeholder synergy are indicators of success.

Data quality: This is essential for a registry to work effectively. Lack of data quality in registries stems from factors such as lack of agreements, governance, common purpose, standardisation and prioritisation. Workshop participants emphasised the importance of defining data quality and using Findability, Accessibility, Interoperability and Reuse (FAIR) principles of data management and stewardship ^[18]. They suggested verifying data sources, setting objectives, establishing data acceptance thresholds, addressing missing data, and creating feedback loops for patients. Involving the International Consortium for Health Outcomes Measurement (ICHOM), the European Medicines Agency (EMA), health technology assessment (HTA) organisations and regulators can facilitate progress, and active patient involvement and transparent data use will indicate advances.

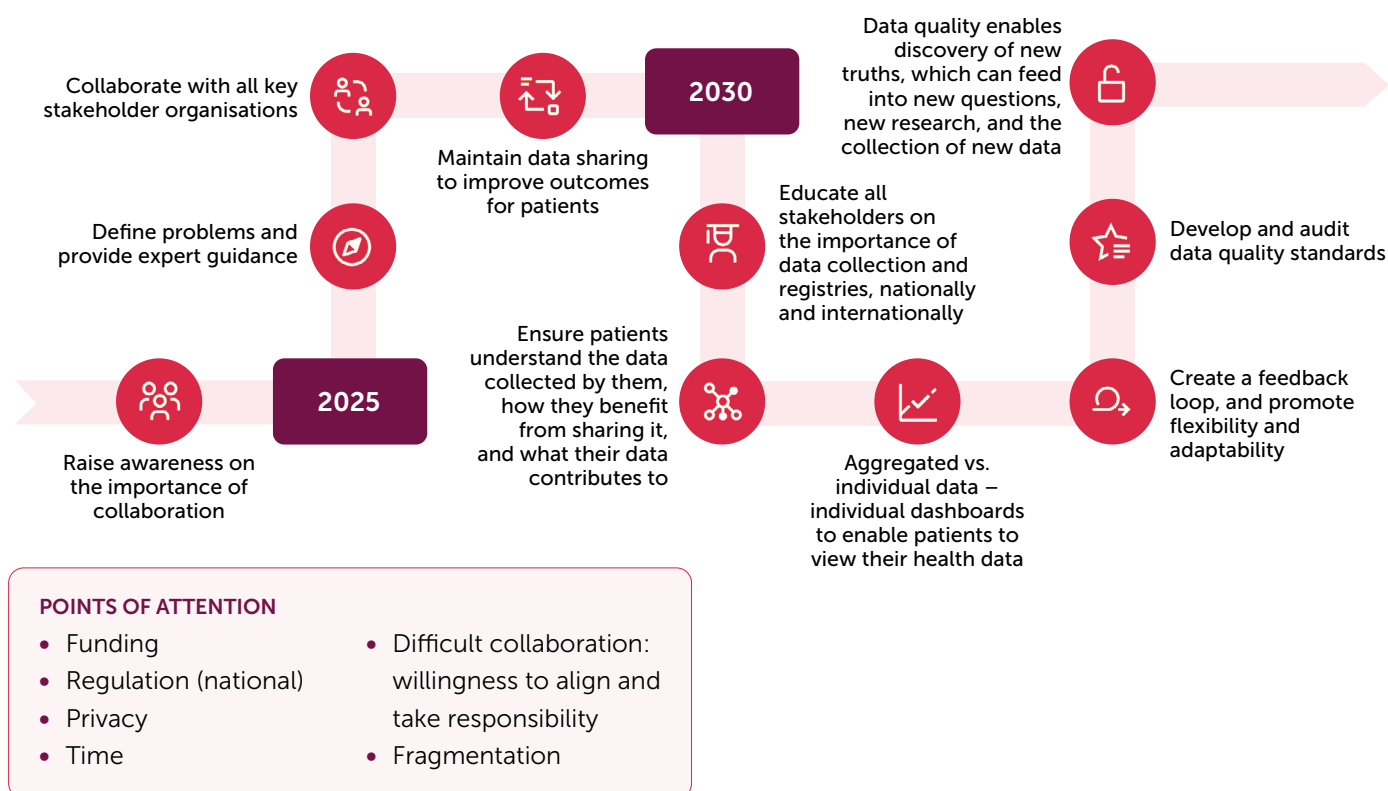
It was agreed that the workstream would move forward with multiple actions, including developing a consensus statement facilitating buy-in from key stakeholders, working on good governance for registries, hosting a network for registry owners, and creating a model for patient data input and feedback.

At the Innovation Summit, a Registries roadmap was completed for 2025 and 2030, and concrete actions identified, e.g. submitting abstracts and writing an academic paper combining all the learnings and lines of thought from the workshops. The roadmap suggests areas of focus to support the modernisation of bleeding disorder registries and prepare them for capturing real-world evidence (Figure 2). However, the first priority concerns alignment and data sharing between registries, which are essential to enabling this.

To pursue the guiding star aim of establishing rights- and responsibility-based international guidance for registries – and keeping in mind that the guidance should be motivational – the workstream plans to

Figure 2. Roadmap to Align Registries for 2025 and 2030

Summary of key points of action to enable the alignment of bleeding disorder registries in Europe, with a view to better supporting patient quality of life



produce a paper emphasising the need for alignment and data sharing to advance treatment and care, and to answer scientific questions as they arise.

Patient Agency

Patient agency refers to the abilities and capabilities of patients to act, contribute, influence and make decisions about their healthcare. The Patient Agency workstream has addressed how to meaningfully embed and integrate patient expertise upfront and throughout the design, organisation, implementation, and evaluation of treatment and care, as well as capturing and utilising patient preferences, for example, for the unmet needs of women with bleeding disorders.

The workstream identified the key challenges that need to be addressed as: 1. The concept of shared decision-making; 2. Patient empowerment; 3. Spectrum of engagement; 4. Cultural change; 5. Health literacy ^[5]. The long-term goal for system change, initially agreed upon by workstream participants, was to achieve a new cultural norm, embedded at all systemic levels, whereby healthcare is collaborative and based on patients' ability to make choices and take ownership of decisions relating to their care and quality of life ^[8]. Multiple shorter-term goals focused on:

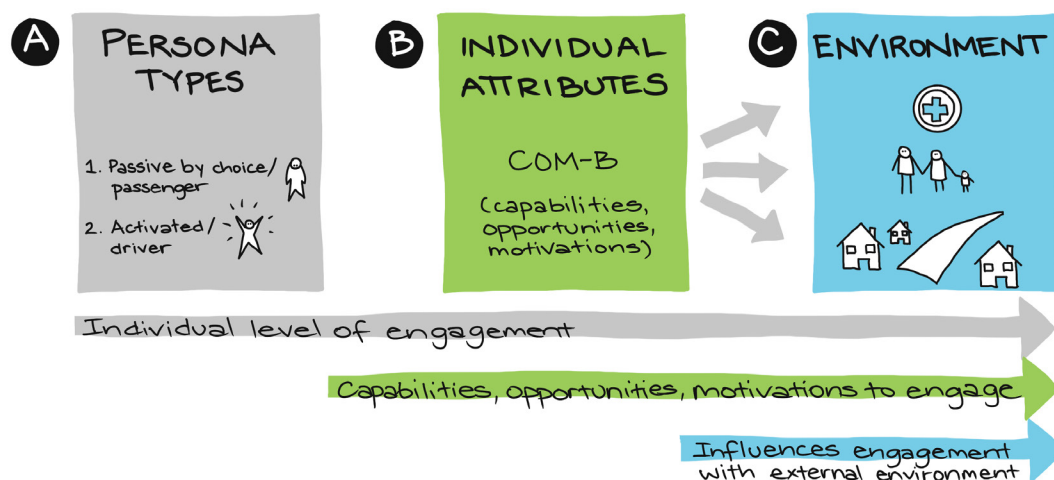
- Defining and enabling patient agency through multi-stakeholder collaboration and communication
- Education and knowledge building
- Establishing patient-led and self-reported outcomes
- Establishing systemic feedback mechanisms and input routes
- Challenging the concept of the patient as an 'end user'
- Improving understanding of the role of patient experience in different parts of the system to inform best practice.

At a subsequent workshop, participants reconsidered the definition of patient agency used for the workstream, based on findings from a targeted literature review on how patient agency is generally defined and applied ^[19]. Greater emphasis was placed on behaviours and relationships, and it was agreed to integrate the COM-B model, which proposes there are three components to any behaviour (B): capability (C), opportunity (O), and motivation (M) ^[20,21]. Thus, the proposed patient agency framework (Figure 3) includes:

- Level of engagement (taking/deciding on the level of ownership of one's health)
- Individual attributes (capabilities, motivation)
- Environmental interactions (opportunities).

Figure 3. Elements of the proposed patient agency framework

Participants in the Patient Agency workstream identified individual level of engagement in their healthcare (based on personas), behavioural attribute (based on the COM-B model) and engagement with their external environment as core facets in the definition of patient agency. Image: la Brix Ohmann / Overlap (<https://www.overlap.dk/english>)



System mapping was undertaken, taking account of the root causes of the challenges identified ^[22]. This enabled a more in-depth understanding of the key challenges for patient agency, together with their enablers and constraints, focused on:

- The role of the patient
- Collaboration
- Narrative in healthcare and about the patient
- Education and training
- Patient-centric healthcare system services
- Increased diversity.

Recognising the role of the patient as both a challenge and a leverage point, workshop participants agreed that actions should focus on this area. Challenges around health systems and services were perceived as being more difficult to address, and as collaboration already occurs among bleeding disorder stakeholders within the system, the level of additional investment required to 'move the needle' might not be cost effective.

Shifting the narrative around the patient role and changing the stakeholder hierarchy were coupled together as a leverage point for system change. Participants recognised a need to elevate patient influence in all aspects of the healthcare system, and to place the patient at the centre of system change. Increasing awareness of patient involvement challenges the traditional healthcare hierarchy, and empowering patient communities and engaging macro-level system shapers is crucial for progress. Patient outcome measures can serve as indicators, while structural changes and storytelling can drive change.

Health literacy gaps caused by complex medical terminology were identified as a second leverage point. Workshop participants emphasised three tiers of leverage: trained healthcare professionals (HCPs), prepared patients, and health system set-up. Training, policy changes, and breaking down silos can strengthen positive dynamics, while involving patients, caregivers, providers, policymakers, and media can bridge gaps in communication and understanding. The introduction of patient-reported outcome measures (PROMs) and patient-reported experience measures (PREMs), use of case studies, and reduction in disease burden can all demonstrate progress towards better health literacy. Effective change requires coordination, communication, and identification of best practices.

Though potentially limited by data protection regulation, PROM limitations, health literacy and engagement barriers, the use of digital health and tools for patient monitoring was also seen as a leverage point. Stakeholder interest, relevant questions and feedback loops provide positive dynamics, however it will be crucial to ensure there is clarity on data ownership and trust in tools at all stages. While there is a need to address and acknowledge the risk of bias in artificial intelligence (AI) ^[23], combining AI tools with human input will enable the best outcomes.

PROMs were identified as the final leverage point, linking patient roles and digital health, and with stakeholders including patients, HCPs, the EMA and the International Consortium for Health Outcomes Measures (ICHOM). Although providers are embracing PROMs and increasingly recognising the importance of real-world evidence, there is a need to reinforce patient

involvement, user-friendly tech, professional training, and inclusion of patient organisations. Challenges to greater use of PROMS include their diversity, variability, and need for validation, as well as patient participation.

Workstream participants agreed to action two initiatives aimed at addressing challenges relating to the role of the patient: a patient agency guidebook and the creation of disease-specific patient experience data (PED) dossiers.

Patient agency guidebook: This project is designed to inspire and support change agents in understanding where and why they should focus their efforts on system change in relation to patient agency. Each chapter will focus on a leverage point derived from the system map (e.g. patient role, stakeholder hierarchy, health literacy, lived experience data and digital health), providing an introduction to the topic and a list of ideas for transitioning a healthcare system to be better equipped for patient agency.

PED dossiers: PED is broadly defined as data collected by individuals (including patients, family members, caregivers and advocacy organisations) that are “intended to provide information about patients’ experiences with a disease or condition”, including its impact and their treatment preferences, and encompassing their perspectives, needs, and priorities”^[24,25]. Based on a solution gaining traction in the United States^[26], the purpose of disease-specific PED dossiers is to collect together lived experience data that is otherwise dispersed across peer-reviewed literature, white papers, reports, and patient group websites. Creating comprehensive, disease-specific records, alongside a corresponding PED dossier directory, will enable better communication of the true implications of living with a rare disease to clinicians, payers, policymakers, and medical developers, and by doing so has the potential to help diminish health inequality. This initiative will include the development and distribution of a PED dossier for von Willebrand disease, with the aim of promoting care and treatment innovation.

Workstream participants also identified potential future activities to address challenges concerning the role of the patient, including raising awareness among medical students about patient agency to help establish a new patient-focused culture through education.

DISCUSSION

The EHC Think Tank’s Registries and Patient Agency workstreams have identified key elements of system change that can radically affect the care of people with

bleeding disorders and other rare diseases in the near and more distant future. The Registries workstream emphasised the need for all stakeholders to share a common purpose in aligning registries across Europe to produce accessible, high-quality data through transparent governance, effective collaboration, and encouraging the use of harmonised language and defined outcomes. The Patient Agency workstream agreed on the importance of putting the patient front and centre of the healthcare ecosystem by addressing the current stakeholder hierarchy and gaps in patient health literacy, as well as optimising the potential of digital tools to enable patients to contribute with PROMS and PREMS.

The rights and responsibilities-based guidance paper, proposed by the Registries workstream, is being developed collaboratively with all stakeholders, including patients, as an important step towards ensuring that patient needs, priorities and concerns are addressed. Recommendations for greater transparency can also foster increased collaboration. Similarly, the Patient Agency workstream’s planned guidebook can help ensure that patient agency is embedded in healthcare systems at all levels by showing how change can be achieved in stakeholder hierarchy, health literacy, recognition of lived experience, and digital health. Developing a PED dossier for von Willebrand disease, in the first instance, can demonstrate the importance of compiling lived experience data and support advances in this field. Moving forward, this has the potential to be modified for use in other patient populations.

The shift towards patient-focused care highlighted by these workstreams is already evidenced in the literature^[27-30]. Ensuring this movement becomes a reality across the care of patients with bleeding disorders and other rare diseases means there is a need for support, advocacy and innovation within the system. As elements of a complex healthcare ecosystem, registries and patient agency are impacted by multifaceted and multicausal challenges, and to bring meaningful change it is necessary to consider their function within and interactions with it^[2]. Traditional advocacy alone, through which individual stakeholders endeavour to convince others to make changes, is not sufficient. Instead, there is a need to move towards system change advocacy where, by necessity, stakeholders with varied experience collaborate to innovate and bring change across the system.

Multistakeholder collaboration has been key to the success of the Registries and Patient Agency workstreams, not least in giving a voice to all

stakeholders in the system who will be affected by system change. Joint thinking and group learning across disciplines, sectors and communities is a core element of systems thinking ^[2], and collaboratively exploring challenges to system change and how different types of intervention may affect these enables a greater understanding of the system's complexities and the development of shared goals ^[31]. Holding a series of online workshops and some face-to-face discussions ensured full exploration of the challenges identified, with opportunities to revisit initial findings in the light of new evidence, reflect on concepts, learn from each other's experiences, and co-create, review and refine strategies for change. System mapping was also an important component of the activities of both workstreams. In addition to facilitating stakeholder understanding, mapping the components of complex systems and how they are 'causally interlinked' fosters inclusion and participation, and therefore supports information sharing, trust, and coordinated change towards integration across organisational and professional boundaries ^[32]. Drawing on insights from systems mapping research, workshop participants were able to identify and link strategic goals, together with the

enablers and constraints – both fixed and fluid – that were likely to influence the achievement of these goals as part of the overarching theme of systems change.

CONCLUSION

There is a clear need for system change to ensure equitable access to healthcare for people with rare diseases such as bleeding disorders. By bringing together multiple stakeholders with different knowledge and approaches, the EHC Think Tank has facilitated the development of innovative strategies for system change in relation to Registries and Patient Agency. Work has started on pilot projects to move these strategies forward. In the longer term, it will be important to develop frameworks to measure their effectiveness and assess the ways in which they have contributed to system change.

ACKNOWLEDGEMENTS

This paper became feasible through the valuable contribution of the members of the EHC Think Tank Workstreams on Registries and Patient Agency. The authors would like to thank all participants in these workstreams.

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 to engage with key thematic areas or workstreams identified as priority areas for 'systems change' within European health care systems ^[33].

The EHC Think Tank seeks to mobilise the agency and purpose of all stakeholders in the health care system to collectively design and champion potential solutions to existing problems. Workstream members are invited based on their expertise and potential for constructive engagement, including patient and industry perspectives alongside a balance of HCP, academic, regulatory, governmental and geographical representation. All workstream activities are held under the Chatham House rule to enable inclusive and open discussion ^[34]. Each workstream is project-managed from within its individual membership. Members set their own agendas, timelines, and targeted outputs, with operational, logistical, methodological and facilitation support from EHC staff and Think Tank practitioners.

The following key topic areas have been the subject of workstream discussion and activity:

- Registries
- The Hub and Spoke Model
- Patient Agency
- Access Equity
- Future Care Pathways

Details of each workstream and published outputs, including interactive system maps, are available via the EHC Think Tank website ^[31].

Note: The Hub and Spoke Model Workstream was discontinued in February 2023. The workstream was convened to address the need to rethink traditional approaches to bleeding disorders care, with a focus on adapting to novel therapeutic options and evolving technologies. Workstream members identified two key related issues which continue to be addressed within and beyond the EHC Think Tank. The first, addressing immediate concerns on the delivery of gene therapy, will now involve collaborations between European medical and patient organisations to support national implementation and cross-border treatment. The second, regarding long-term considerations for future care, was transferred to the Future Care Pathways workstream.

Copenhagen Economics undertook the mapping of registries for rare bleeding disorders across 18 European countries for the Registries workstream.


OPEN Health prepared the targeted literature review on how patient agency is defined and applied for the Patient Agency workstream.

The authors have advised no interests that might be perceived as posing a conflict or bias.

This paper does not contain any studies involving human participants or animals performed by any of the authors.

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HOW TO CITE THIS ARTICLE:

Gacser Z, Skouw-Rasmussen N; EHC Think Tank. System change in practice: A report from the EHC Think Tank workstreams on Registries and Patient Agency. *J Haem Pract* 2024; 11(1): 1-10. <https://doi.org/10.2478/jhp-2024-0005>

