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SYSTEMS CHANGE

Short- and longer-term goals for change – A report from the 2nd workshops of the EHC Think Tank Workstreams on Registries, the Hub and Spoke Model and Patient Agency

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At the second series of workshops for the EHC Think Tank Workstreams on Registries, Hub and Spoke Model and Patient Agency, stakeholder participants worked towards consensus on addressing challenges to progress in areas identified in the first series of workshops. Each workshop identified a 'guiding star' determining the direction of ongoing focus, defined achievable 'near star' milestones, and explored the enablers and 'constraints' to achieving these. **Guiding Stars:** The Registries Workstream recommended establishing rights- and responsibility-based international guidance to ensure accountability from all stakeholders contributing, collecting, handling and registry data. The Hub and Spoke Model Workstream proposed the development of a dynamic and agile health provision system to meet individual treatment, care and quality of life goals for people with rare disorders as they evolve. The Patient Agency Workstream recommended achieving a new cultural norm for patient agency embedded at all systemic levels, whereby health care is collaborative and based on patients' ability to make choices and take ownership of decisions

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The concept of a 'guiding star' and associated 'near star' milestones has been used to identify steps towards achieving long-term solutions to challenges identified in the EHC Think Tank Workstreams on Registries, the Hub and Spoke Model, and Patient Agency

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relating to their care and quality of life. **Near Stars:** Four common themes emerged in near star milestones across all workstreams: 1. Mapping the system; 2. Collaborating and sharing; 3. Education and training; 4. Organisational change and good governance. Enablers include stakeholder experience in other specialties undergoing similar change; relevant examples of best practice; tapping into existing policy-making networks; adherence to government, regulatory, and inter-/intra-institutional quality standards; leveraging frustration in current systems to challenge mindsets and demonstrate the benefit of patient-centred insights to improve outcomes; and application of technologies (e.g. distributed analytics, algorithms, telemedicine, remote monitoring). Constraints include limited understanding of national and cross-border legal and regulatory requirements; a lack of awareness of and reluctance to accept the need for change or to take responsibility for making it happen, or a misunderstanding of whose responsibility it is; time limitations; a lack of meaningful outcome measures; a lack of understanding of key factors for success; and financial issues.

Keywords: *Bleeding disorders, Registries, Hub and spoke model, Patient agency, Mapping, Collaboration, Education, Organisational change, Governance*

At the first workshops of the EHC Think Tank Workstreams on Registries, Hub and Spoke Models and Patient Agency, stakeholders representing health care providers, patient groups, regulators, policy makers, research and industry, participated in virtual meetings to identify challenges to progress in these three important areas related to patient care, and to propose potential solutions [1,2,3]. At the second workshop in each workstream, participants worked towards consensus on:

1. Identifying a 'guiding star' to determine the direction/course for ongoing focus
2. Defining achievable 'near star' milestones
3. Exploring the enablers and constraints to achieving these milestones.

GUIDING STARS

The symbol of a 'guiding star' was used to align each workstream around a long-term, ambitious, but realistically achievable solution to the challenges identified in previous workshops.

The **Workstream on Registries** recommended establishing rights- and responsibility-based international cross-border guidance. This could help ensure accountability from all stakeholders contributing, collecting and handling as well as using registry data.

The **Workstream on the Hub and Spoke Model** proposed the development of a dynamic and agile health provision system that is able to meet individual treatment, care and quality of life goals for people with rare disorders as these evolve. This should be based on an organisational system that embraces innovation, focuses on equitable and individualised treatment, care and support for all patients, and recognises the importance of multidisciplinary health care teams.

The **Workstream on Patient Agency** recommended achieving a new cultural norm for patient agency embedded at all systemic levels whereby health care is collaborative and based on patients' ability to make choices and take ownership of decisions relating to their care and quality of life [4].

NEAR STARS

The symbol of 'near stars' was used to chart a number of shorter-term, more readily achievable milestones along the path towards the long-term 'guiding star' goal for each workstream (Figures 1–3). All near star goals are subject to review, renewal, or adjustment based on work progressing, efforts acting on a complex living system, and the system reacting in response. This dynamic process may lead to new learnings that reorient towards other near star goals that ultimately progress towards the guiding star.

Across the workstreams, four common and sometimes linked themes emerged in relation to near stars and to the enablers and constraints likely to affect achieving them:

1. Mapping the system
2. Collaborating and sharing
3. Education and training
4. Organisational change and good governance.

1. MAPPING THE SYSTEM

An important near star for participants across all three workstreams is mapping the system. Through pinpointing existing relevant knowledge and services, the mapping process can help to identify best practice and capitalise on these so as not to reinvent existing practices. The mapping process in each of the three workstreams can also support progress towards near stars across the other three thematic areas.

Figure 1. Guiding star and near star aims for the EHC Think Tank Workstream on Registries

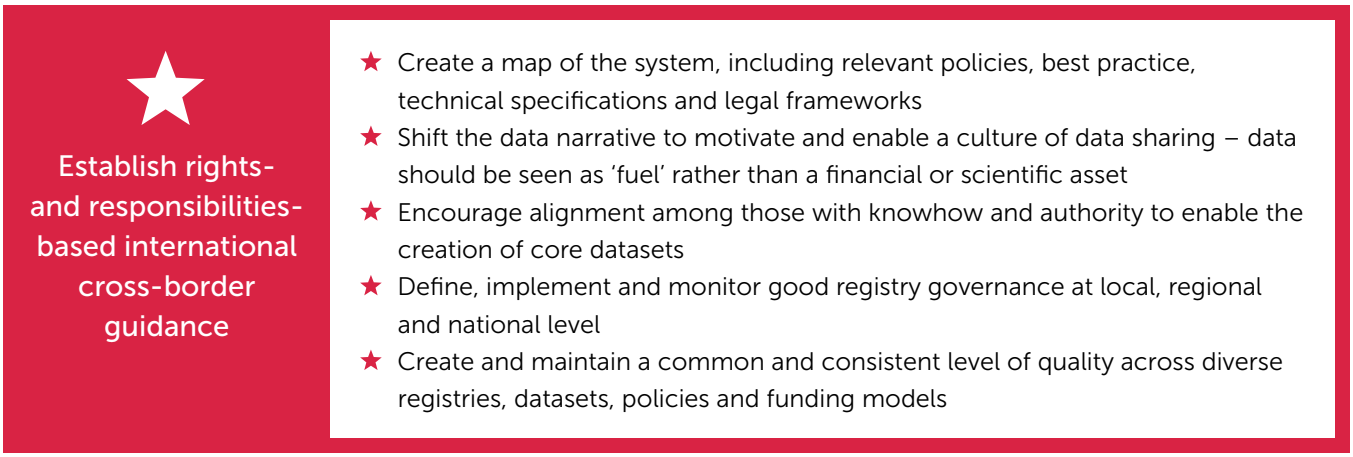

Figure 2. Guiding star and near star aims for the EHC Think Tank Workstream on the Hub and Spoke Model

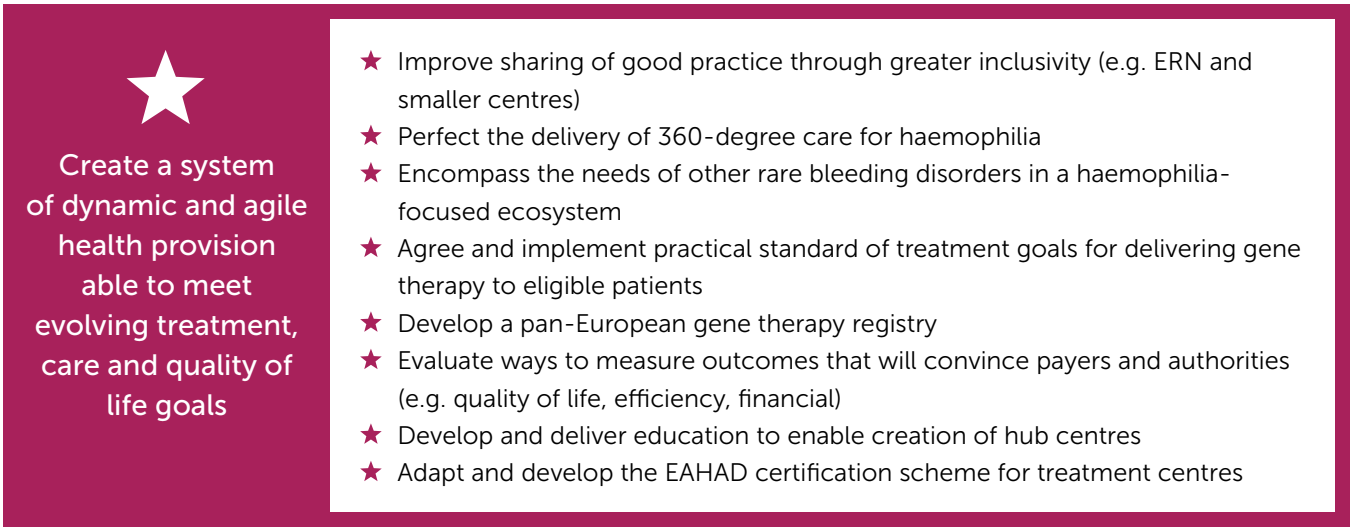

Figure 3. Guiding star and near star aims for the EHC Think Tank Workstream on Patient Agency

The **Workstream on Registries** identified a need for mapping existing registries. This should include information on type (e.g. disease, patient, treatment), administrative information (e.g. registry owner/custodian, legal aspects) and technical information (e.g. data collection, flow and management). Mapping is also needed of existing requirements for data across relevant institutions and countries (e.g. by health technology assessment (HTA) bodies, regulatory approval, clinical development, etc.) and of legislation and policies governing data sharing, governance, and cross-border data exchange. Establishing best practice for data sharing between health systems is essential, and this must consider existing relevant legal requirements and agreements on the sharing of data [5].

In the **Workstream on the Hub and Spoke Model,** the need for mapping is centred on establishing a clear overview of different national approaches to the hub and spoke model of care across Europe. This should include detail on policies, financial systems, clinical expertise, care pathways and patient rights. This would help support a related exercise evaluating what is and is not working in the current set-up of European haemophilia treatment centres (HTCs) and comprehensive care centres (CCCs), as well as learning from the European Reference Network and other systems.

The **Workstream on Patient Agency** identified the need for a mapping exercise that establishes what best practice looks like at all levels (policy, operational, individuals) in Europe and beyond. Much could be learned from mapping initiatives targeting patient agency for systems change across different disease areas. Mapping is also key to ascertaining how different stakeholders define patient agency, how they perceive their own role for driving patient agency within the system, what patient-focused educational opportunities are available for health care professionals (HCPs), and to what extent this is patient-led. The mapping process will be enabled by a literature review that helps to consolidate the definition of patient agency across different disease areas. This could potentially support the development of a charter specific to patient agency in rare diseases, while a map of the patient journey will aid understanding of where and when patient agency is incremental. Any definition of patient agency must be relevant to all stakeholders to ensure shared ownership and responsibility.

2. COLLABORATION AND SHARING

Collaboration and the sharing of skills, expertise and data is considered another important near star across the three workstreams.

In relation to **registries**, collaboration and sharing is key to establishing standardised systems and a seamless flow of information that addresses stakeholder needs. This would be based, ideally, on linking and aligning existing registries rather than starting over [6,7,8]. Breaking down barriers to data sharing at intra- and international levels and a move away from data 'ownership' (mine vs. yours) are key, alongside a new narrative whereby all stakeholders – including patients and registry holders – recognise that 'our data' is the fuel that drives better care and benefits everyone. As part of this shift, there must be a willingness among those with the relevant knowhow and authority to align on the development of core datasets [9,10]. To achieve this, good communication will be needed so that all stakeholders, including patients, understand how data are used and why sharing is so important [6]. This will require trust, reassurance and transparency.

Constraints that may limit collaboration and sharing between registries include limited understanding of laws and regulations governing data sharing, a lack of harmonised datasets, pathways and data transfer agreements. These constraints may be at least partially addressed by other near star activities. For example, mapping will enable greater understanding around national and international laws and regulations that affect registries. Similarly, mapping, organisational change and good governance will help support the harmonisation of datasets and improve the interoperability of registry data (e.g. by identifying best practice in other disease areas, supporting quality management and establishing agreed data standards).

At institutional level, constraints may relate to refusal to respond to reasonable requests to share data, EU General Data Protection Regulation (GDPR) and cybersecurity risks. In addition, data quality may impact on potential for data sharing, i.e. there will be reluctance to accept data from an institution where quality is known to be poor. Even within institutions, concerns about whether departments are using best practice, ego and trust issues may result in an unwillingness to share data.

Collaboration is essential for the development of **hub and spoke** models of care [11,12]. Greater inclusivity and better alignment of European Reference Network (ERN) centres, smaller centres and practising clinicians and patient organisations can support better sharing of good practice. Ensuring effective delivery of 360-degree care for haemophilia, from diagnostics and treatment to social and holistic elements, is key and organisational change must encompass the needs of all

patients with bleeding disorders, including rare bleeding disorders. In the short term, achieving optimal delivery of haemophilia gene therapy for eligible patients is an important near star milestone [12]. As gene therapy becomes more widely available, establishing a pan-European gene therapy registry is a further near star milestone which could play an important part in a hub and spoke care model, with smaller centres benefitting from the expertise of clinical colleagues who have gained experience of gene therapy in clinical trials.

Variation in practice and funding (e.g. insurance and reimbursement) may restrict what can be achieved in developing the hub and spoke care model. Time is another important constraint on achieving change, especially in smaller centres. Even just seeing patients puts pressure on clinical time, so finding time to plan and implement new practices may be considered impractical. Time may also be an issue for patients, especially in relation to cost of travel to see different specialists and loss of earnings while attending appointments. At micro level, lack of flexibility is a constraint. Both clinicians and patients may feel comfortable within current traditional care systems and reluctant to consider change. Both will therefore need education to understand that, as needs change, other approaches may be beneficial; change cannot occur unless all stakeholders are on board.

For **patient agency**, collaboration and better communication between all relevant stakeholders and organisations – including the EHC and other umbrella patient organisations – are seen as an important near star. Achieving this will require use of a common language, robust transparency in the decision-making process with regard to policy, and a platform for sharing experiences and exchanging new ideas. Partnering with HCP bodies and organisations (and satellite groups) to influence cultural shifts in patient-centred practices, including the role of shared decision-making, will be key to this. Lack of awareness of the benefits and value of collaboration and sharing to facilitate patient agency may impede progress, as may a reluctance to accept the need for change or to take responsibility for making it happen. A lack of understanding of the key success factors needed for efficient collaboration and sharing may also limit progress, and the perceived difficulties of introducing a more collaborative and sharing environment could be a further barrier.

At all levels, technology is an important enabler of collaboration and sharing. For example, it can improve interoperability between **registries**, break down language barriers and facilitate generation of core data [7,13]. Developments in distributed analytics may also play a role in enabling improved communication and sharing between databases, with algorithms collecting and aggregating agreed data from individual registries to answer specific questions. For example, in the Netherlands, the Personal Health Train is designed to enable health care innovators and researchers to work with health data from various sources, providing controlled access to data, while ensuring privacy protection and optimal engagement of individual patients [14,15]. Technology also aids collaborative working in **hub and spoke** care models and helps facilitate **patient agency** through enabling remote communication and collaboration, telemedicine and remote care, and through digital tools for a range of applications including remote monitoring, patient reported outcome measures (PROMS) and online training.

3. EDUCATION AND TRAINING

All workstreams recognised the importance of education and training tailored to the needs of relevant stakeholders.

The **Workstream on Registries** highlighted a need for education at multiple levels to help standardise and optimise data quality. This should include improving understanding of disease areas by registry owners to ensure appropriate data collection, setting common terminology for data entry, and designing and implementing dashboards to evaluate data quality [6,10]. Enablers for education aimed at improving data quality include the development of a reporting format that is easy to understand, ensuring balanced datasets (not too many, not too few), establishing data and statistical analysis plans, and facilitating real-time data entry.

There are various constraints that could impact data quality, despite efforts at standardisation. While registries typically use a common language (generally English), some registry holders may lack sufficient fluency, leaving data quality impacted by language proficiency. Similarly, variation in drug names for some medicines used in different countries (some of them out of date) may make cross-border data analysis and comparisons difficult and time-consuming if additional checks have to be made. Delay in data submission by both patients and clinicians has been identified as a marker of data quality as delays can increase the risk of errors. Good governance can help to mitigate and overcome these constraints, with effective management and monitoring of data entry at local level being key.

Multiple near star milestones identified for the development of **hub and spoke** models of care focus on education and certification. In haemophilia care, the introduction of a hub and spoke model provides an opportunity to adapt the current European Association of Haemophilia and Allied Disorders (EAHAD) certification scheme [16] to allow for broader criteria and multiple levels of certification based on the operational level of CCCs. The availability of increasingly complex and innovative treatments for haemophilia brings with it the need for a second near star milestone for stakeholder education (clinicians and patients), particularly in relation to the creation of hub centres. Clinicians working at smaller centres may not have the clinical expertise of those at large centres taking part in clinical trials of new treatments [11,12]. However, they know their patients well and want to be involved and advocate on their behalf, so it is important that all stakeholders work together. A lack of experience and follow-up may inhibit developments and there is uncertainty around whether there is a new generation of young clinicians who are interested in making a career in haemostasis.

Understanding the experiences of other specialties undergoing similar change could help inform how best to develop a strategy to ensure that all stakeholders involved in the hub and spoke model of care have the knowledge they need. This will be particularly important in supporting decision-making around new treatments including gene therapy. Existing networks for policymaking (such as the EHC), existing clinical networks and the expertise in multidisciplinary teams are also important enablers. From the patient perspective, a lack of up-to-date information about the innovative new therapies likely to be delivered through the hub and spoke model may limit their interest and commitment to learning and engaging. Patient education would therefore benefit from the creation of an up-to-date knowledge base, including answers to frequently asked questions about innovative therapies.

Related to this, another early goal is educating stakeholders at all levels (e.g. patients, HCPs, regulators) about the benefits and value of **patient agency** in driving personalised care and improved quality of life for people with rare diseases. Consistent information tailored to the different stakeholder groups should be made available so that there is shared understanding on this for all parties when it comes to making decisions around care [17,18]. This requires a good understanding of the educational needs, capabilities and capacities of all those involved – individually and collectively. Developments in education and training around patient agency may be constrained by a lack of awareness of best practice. Without a good understanding of what has been achieved in other disease areas and health systems, it will be difficult to generate education and training programmes that can achieve the required outcomes. Patients themselves will be important enablers in the education process and should be consulted from the start to help shape their own care and that of their peers. The use of appropriate language and vocabulary in educational materials is also key – it is important that the language used around patient agency is consistent. Role-modelling case studies could help to establish what patient agency should look like at all levels (i.e. patient to HCP, patient-HTA to payer, payer to HCP, patient organisation to HTA/payer, etc.) [19].

4. ORGANISATIONAL CHANGE AND GOOD GOVERNANCE

The importance of organisational change and good governance were themes running through many of the discussions about achieving key near star aims across the workstreams.

The **Workstream on Registries** recommended that good registry governance should be defined, implemented and monitored/evaluated at local, regional and international levels over the next few years. This will enable a standard level of quality to be maintained across diverse registries, datasets, policies and funding models. To facilitate this goal, relevant competencies will need to be present among registry owners [10], with data quality standards consistently maintained to meet the requirements of government and regulatory bodies, such as the European Medicines Agency (EMA) and HTA bodies. Adherence to government, regulatory, and inter-/intra- institutional quality standards, and regular registry audits of all stages, from data input to analysis (ideally by external auditors) will be key to enabling this near star milestone. Audits may help to maintain quality and should include validation of primary source data to identify inconsistencies. Benchmarking against established quality standards (e.g. compatibility with requirements for EMA registries) could also be a useful tool to maintain good data quality.

Lack of interoperability between datasets/registries and the absence of an external body or supra-natural guidance agreement able to guide developments towards greater collaboration have the potential to constrain organisational change and good governance

of registries. Data may be collected more than once, resulting in 'dead ends'. Collaboration on establishing core datasets and standards may help to overcome this. The reluctance to share registry data noted under 'Collaboration and sharing' could also be an issue, although it should be acknowledged that this is may not solely be the result of 'mine vs. yours' outlook. Financial issues may play a role where registries receive income from data entry or access, as they will not want to lose this under any new arrangements. In terms of funding for registries, it is inappropriate for pharmaceutical companies to be sole funders – if their priorities change, a registry may be unable to continue. At the individual level, patients may not want to submit data to multiple registries and clinicians may be reluctant to share their patients' data. Education and discussion around the benefit of sharing data may help to overcome this.

For the **Workstream on the Hub and Spoke Model,** agreement and implementation of practical, standard of care treatment goals for delivering gene therapy for eligible patients will be a major near-term goal. This will require evaluation of key outcome measures (e.g. quality of life, efficiency, financial impact) that can be monitored in the longer term as part of governance procedures. There are, however, significant macro-level constraints on achieving organisational milestones for the hub and spoke model of care, particularly where haemophilia gene therapy is concerned. The large diversity of national organisations involved in bleeding disorders care, current challenges in gaining market access to gene therapy in all rare bleeding disorders (although this is now beginning to change), and the geo-localisation of centres may make insurance coverage challenging. Payers may express reservations about seeming to favour patients with haemophilia for treatment and may look more favourably at establishing a broader-based gene therapy initiative for a wider range of rare bleeding and other disorders. Existing policies at all levels – local, national and transnational – may also be significant constraints, and it is essential that policy developments at European level look to improving national policies for hub and spoke models of care.

The EAHAD-EHC joint statement calling for all first-generation gene therapies to be managed using a hub and spoke model will be an important enabler for optimising gene therapy services [2,12], and it is helpful that a network of high achieving HTCs is already in place and can lead on wider implementation of highest quality standards. The World Federation of Hemophilia Gene Therapy Registry will also be a valuable resource for monitoring efficacy and safety and informing optimisation of future care [20,21,22], as will US Food and Drug Administration (FDA) and EMA reporting. At micro level, there are many misconceptions around gene therapy which may slow down organisational change and need to be addressed by education.

In relation to **patient agency**, increased awareness of the need for change will be important and the concept of patients as 'end users' must be challenged. Truly listening to patients and their families and focusing on their highest priority needs will be essential. Removing generational trends of paternalism and moving towards a new model of patient-centred care will mean HCPs challenging the system of which they are part.

Frustration in the current system can be leveraged to challenge the existing mindset and status quo within institutions and demonstrate the benefit of a patient-centred lens to improve outcomes. However, failure to clearly define and disseminate the meaning and value of patient agency to health systems, patients and clinicians will constrain its implementation and limit its impact on organisational change and good governance. Without meaningful outcome measures and demonstrable benefits across haemophilia care services, it will be difficult to convince stakeholders and decision-makers to prioritise patient agency within the context of all the competing demands for funding. Patient-led and self-reported outcomes via registries and real-world data sources can act as enablers for change. Systemic feedback mechanisms will be beneficial, with input routes available at all levels including regulatory, institutional, patient group and individual stakeholders. Global and locally meaningful improvements in patient agency will need to be achieved and good governance implemented and maintained.

MOVING FORWARD

Moving forward, mapping best practice and capturing different data sharing agreements across multiple health care systems will inform stakeholder strategies for data sharing and **registry** harmonisation, as well as helping to change attitudes to data ownership that are currently impeding progress. Improvements in data quality can be achieved through regular audit and compatibility with and adherence to EMA, HTA and other regulatory standards. It will be important to identify a broad range of supporters for change, from governing bodies such as the EMA, FDA and HTA, through to HCPs, key opinion leaders and patients.

The value of registries and the data being generated to each of these stakeholders will need to be defined. With this in mind, a protocol (roadmap) will be needed to achieve buy-in, including aims, targets and desired outcomes.

For the **hub and spoke** model, there is a need for a clearly mapped view of different national situations and the identification of pertinent potential partners with expertise in achieving similar goals. These will not always be in the haemophilia field (e.g. the more broadly focused Fondazione Telethon in Italy funds research into rare diseases [23]). More information is needed about the HTCs that want/need to be hubs, their education and training needs, and the centres that already have experience of gene therapy. Developing stories/case studies demonstrating the heterogeneity of patient experiences is likely to play a useful role in describing the likely impact of a hub and spoke model of care and how people will engage. Care pathways will need to be developed as structures change and implementation of the hub and spoke model begins [12], and as timelines are established for the introduction of gene therapy and other novel treatments. More information will be needed about the motivations and decision-making of people with milder haemophilia phenotypes and, in the future, those who have successful gene therapy who may be reluctant to maintain regular contact with treatment centres. Reporting and follow-up will also be essential over the long term (e.g. 15+ years), as will support for clinicians working with evolving evidence generation.

For **patient agency**, the mapping of initiatives targeting patient agency and an understanding of best practice across disease areas and health systems will inform future strategies. By mapping the global patient journey, it will be possible to identify significant patient agency touch points for intervention. Key success factors for efficient collaboration need to be identified and optimal approaches to education and training agreed for all stakeholders, including patients, clinicians, service providers and regulators. Role modelling and case studies of what patient agency should look like will aid development of initiatives, and patient-led measures will need to be devised to assess impact.

SUMMARY AND NEXT STEPS

The EHC Think Tank's workstreams on Registries, the Hub and Spoke Model and Patient Agency have set a direction of focus (or guiding star) with a view to achieving a long-term solution to challenges

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 [24]. The EHC Think Tanks 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: 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 [25]. Each 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. 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.

Since the Think Tank's inaugural workstream meetings in 2021, the following key topic areas have been the subject of ongoing discussion:

- Registries
- The Hub and Spoke Model
- Patient Agency.

2023 sees the introduction of two new workstreams:

- Access Equity
- Future Care Pathways.

identified in previous workshops. In doing so, they have identified a number of 'near star' challenges, with associated constraints and enablers that are likely to be encountered on the way.

The next step for each workstream will be to decide which particular near star challenge to address, based on what actions will leverage the most change at this point in time. Influencing factors will include the background, knowledge base and skillset of the workstream participants, together with developments and trends in the surrounding environment.

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