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SYSTEMS CHANGE

System change in practice: A report from the EHC Think Tank workstreams on Access Equity and Future Care Pathways

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Introduction: The European Haemophilia Consortium (EHC) Think Tank was established as a platform for system change to ensure the healthcare ecosystem remains effective and relevant for people with bleeding disorders and other rare diseases. Operating alongside traditional advocacy initiatives, it comprised a series of thematic workstreams in which multiple stakeholders explored and co-designed potential solutions for specific aspects of the healthcare system. This final report from the workstreams on Access Equity and Future Care Pathways summarises recommendations for system change and the actions needed to achieve critical goals. **Methodology/Process:** In a three-phase Discovery-Strategy-Innovation process, workstream participants explored vital challenges to the system in which they were working, mapped the system to identify enablers and constraints to progress, and determined leverage points to explore strategies for change before co-creating a set of recommendations for action. **Results:** Participants in the Access Equity workstream identified a need for evidence-based narratives to drive policy change by effectively reaching and engaging target audiences. Fostering trust among stakeholders, supported by 'open, active listening', was seen as essential for progress towards access equity, as was promoting value-based frameworks by ensuring that decision-makers understand the broader impact of progress in access equity for health improvement. Participants in the Future Care Pathways workstream focused on digitalisation, patient preferences and financial incentives as drivers of progress towards

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The EHC Think Tank's recommendations for system change around access equity and future care pathways include using evidence-based narratives to demonstrate value and the integration of digital technologies to enhance patient-centred care

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creating seamless, personalised care pathways that can be measured effectively. Recommendations included fostering a coordination mindset and culture, encouraging patients to take ownership of their digital healthcare records, and enabling personalised care plans through flexibility in care pathways. Conclusions: Among the co-created, innovative strategies and recommendations proposed by workstream participants, key elements to support system change in Access Equity and Future Care Pathways, respectively, include demonstrating value through evidence-based narratives and integrating digital technology into care pathways to enhance patient-centred care. Appropriate capacity-building for all stakeholders, particularly healthcare professionals (HCPs) and patients, will be essential for the success of these initiatives.

Keywords: *Access equity, Care pathway, Behaviour change, Digital health, Health investment*

In 2021, the European Haemophilia Consortium (EHC) Think Tank was established as a platform for system change. In a rapidly evolving healthcare environment, the EHC believed that more than reactive advocacy alone was needed to address the challenges facing patients and decision-makers across health services, regulatory bodies, payers, industries and governments. System change considers the whole ecosystem, particularly the relationships between stakeholders, aiming to enable multiple routes towards agreed goals [1,2].

The Think Tank's purpose was not to invent new systems but to focus on ensuring the current system remains practical and relevant to the changing context in which it exists, and to offer impactful options for advancing the current status quo. A series of thematic workstreams were initiated to explore specific aspects of the healthcare system for rare diseases (Registries, Hub and Spoke Model, Patient Agency, Access Equity and Future Care Pathways). The workstream participants were members of key stakeholder groups including patient advocates, the pharmaceutical industry, policymakers, healthcare consultancies, and organisations involved in health technology assessment (HTA). Workstreams conducted a series of online workshops and (where possible) in-person meetings where workstream participants identified the challenges to progress, established long- and short-term goals, and mapped systems to identify enablers and constraints for change [3-9]. They co-designed potential solutions that can be co-championed, co-owned and co-implemented by stakeholders across health systems. Strategies and actions for system change from the Registries and Patients Agency workstreams have been reported [10]. This final report from the Access Equity and Future Care Pathways workstreams summarises recommendations for system change and the actions needed to achieve key goals.

PROCESS/METHODOLOGY

As previously described, a three-phase Discovery-Strategy-Innovation process was used to establish goals for the Access Equity and Future Care Pathways workstreams, develop strategies for addressing challenges, and make recommendations for putting system change into practice [6-9,10]. The broad objectives and activities for each phase are summarised in Figure 1.

Workstream participants mapped the system in which they participated to refine the challenges, enablers and constraints for achieving their goals, identifying the leverage points through which change might be achieved most effectively [11,12]. During the Strategy phase, workstreams used the Lotus Blossom framework [13] (a 3x3 matrix to develop ideas around a central theme) to build on outputs from previous meetings and generate strategies for change. These strategies for change were further developed into recommendations for actions during the Innovation phase.

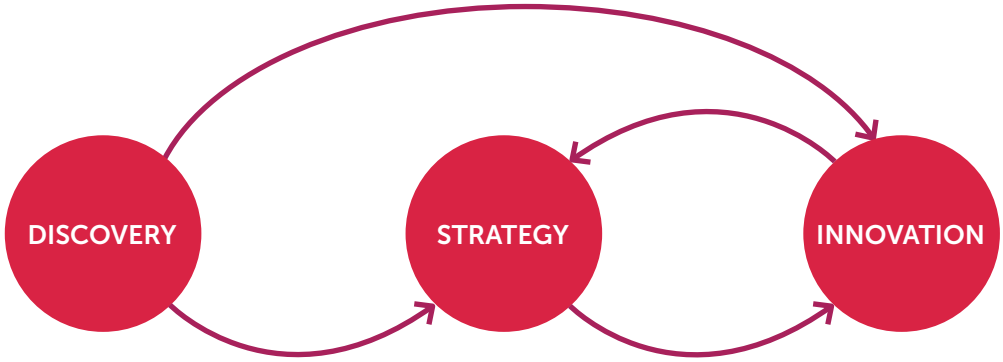
RESULTS/OUTCOMES

Access Equity

During the Discovery phase, the Access Equity workstream participants agreed on a long-term goal ('guiding star') to 'develop a healthcare system that enables patients to benefit from care and treatment fairly and impartially', with short-term ('near star') goals prioritising changes in narrative and behaviour [9]. There was a particular focus on building trust between stakeholders, and giving consideration to value drivers other than cost in rare diseases, e.g. patient-centric value and elements of societal value [14-16].

Participants identified a need for evidence-based narratives to drive policy change by effectively reaching and engaging target audiences. Literature/evidence supporting these narratives should be synthesised and disseminated by partnering with relevant stakeholders to generate reliable, balanced data. The narratives should resonate with and respond to the particular priorities for each stakeholder, but in a way that clearly defines impact on health equity through partnership. A key element will be to describe the need to move

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



OBJECTIVE	**Obtain a broader understanding of the challenges being examined by the workstream**	**Define a strategy to push the system in a desired direction of change**	**Innovate to create impact, incorporating evaluation mechanisms to capture learnings**
ACTIVITIES	• Build connections between workstream members; identify and define the key challenges • Define long- and short-term goals for change ('guiding stars' and 'near stars') and identify enablers and constraints → **System mapping** • Review and fine-tune the system map • Identify leverage points	• Based on analysis of the system map, align leverage points with near star aims • Confirm and unfold leverage points to inform and build a strategy	• Ideate on prototype system interventions based on service design methodology • Assess and refine intervention concepts and strategy • Undertake solution pilots and refine interventions based on review and shared learning

from a 'high cost, low value' system to a more value-based ('high value') system of care that prioritises affordable, preventative, accessible and good quality healthcare, based on identified unmet needs and social determinants of health, and which is supportive of better health equity approaches.

By providing a foundation for presentations and discussions across multiple stakeholder channels including at conferences and policy meetings, evidence-based narratives can be used by motivated champions as a tool to advocate for change. Engaging with patient advocacy groups (PAGs) at national and local levels is crucial in collecting and disseminating narratives and emphasising the need for 'high-value' healthcare.

In developing evidence-based narratives, existing platforms should be explored to make patient data more accessible and available. Gaps in evidence need to be identified, and missing data must be sought with a focus on encouraging better data sharing across stakeholders and in non-competitive spaces. Case studies from a variety of stakeholders demonstrating impact and value, and highlighting learnings will be important to support the narrative. Robust evidence-based advocacy (EBA) initiatives should be encouraged and supported for PAGs using standardised research methodologies and include a diverse and representative demographic [17]. Better connections between private and public health systems are required to optimise data sharing and use. The ethical considerations should be fully addressed, and specific privacy concerns should not be ignored; patients may be willing for their data to be shared in the interests of developing evidence-based narratives that could ultimately help improve their care [18].

Fostering trust is essential for progress towards access equity. The paradigm of viewing others as adversaries ('us vs. them') needs to be replaced with a more inclusive and collaborative approach. Value-based healthcare requires a recognition of individual stakeholder principles in order to be inclusive of these in partnership models that are financially sustainable, resilient and meaningful for all. 'Open, active listening' among stakeholders, will enable a shared

understanding of all groups' constraints. It will also help to differentiate between policymaker and regulator decisions, and to understand the implications of this for change. Establishing HTA processes that are more patient-informed and supplemented by patient-centric and societal value elements and evidence would also support greater inclusivity.

Approaching partnerships with a more strategic mindset, balancing competition with collaboration, and finding common goals and interests will help to build trust. In moving away from a transactional approach towards a more relational one, transparency, honesty, and personal relationships are crucial, as is establishing processes around how to work together. Making a strong business case for collaboration, focusing on the benefits for each organisation involved, is important. For successful collaboration, identifying common ground is key. Learning from each other and establishing standards across sectors can also enhance cross-sector collaboration.

Although workstream participants noted more positive attitudes towards the concept of value rather than cost in the context of access equity, demonstrating value continues to be hampered by a lack of standardised criteria and measures. Economic value and value for individual patients are different [14,19], and perceptions of value may vary across disease areas and geographies. The Transparent Value Framework (TVA), developed by a working group of the Platform on Access to Medicines in Europe [20], was cited as an example of how value could be better communicated and demonstrated, and could provide helpful insights. Designed to help coordinate access pathways for orphan medicinal products in European Union (EU) Member States, the TVA proposes a simple and consistent terminology and methodology based on unmet need, relative effectiveness (e.g., clinical improvement, quality of life, side effects, social impact), response rate according to best available, clinically relevant criteria, and degree of certainty based on documentation [20].

The workstream's recommendations for changing narratives and behaviour to improve access equity are shown in Figure 2, and centre on:

- Developing an evidence-based narrative to highlight the value of access equity
- Engaging with all stakeholders to foster trusting partnerships and collaborations
- Ensuring decision-makers understand the broader impact of progress in access equity for health improvement.

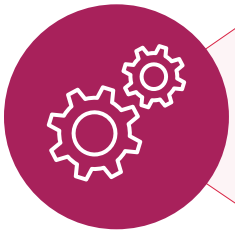
Figure 2. Recommendations for changing narratives and behaviour to improve access equity for rare disease patients

EVIDENCE-BASED NARRATIVE

- Develop an evidence-based narrative to highlight the value of access equity
- Use case studies/storytelling to demonstrate the success of access equity initiatives through financial data and patient lived experience

STAKEHOLDER ENGAGEMENT

- Engage with all stakeholders (e.g., patients, HCPs, providers, payers) to foster trusting partnerships and collaborations
- Take a long-term perspective to achieve secure, sustainable relationships based on trust, respect and visible advantages for all stakeholders



DECISION-MAKER UNDERSTANDING

- Ensure decision-makers (e.g., government, regulators, industry) understand the impact of progress in access equity on the bigger picture for health improvement
- Reinforce the implications of system change for meeting broader, long-term objectives

Future Care Pathways

The long-term goal ('guiding star') previously identified for the Future Care Pathways workstream was 'to develop care pathways that provide the right intervention at the right time by the right healthcare professional (HCP) in the right formats with a variety of delivery methods to suit the person' [9]. Prioritising the development of seamless, personalised care pathways with integrated digital and AI-based technologies to enable real-time measurement of pathway effectiveness was the short-term ('near star') aim. During the Strategy and Innovation phases of the Think Tank process, participants in this workstream focused on digitalisation, patient preferences and financial incentives as themes for driving progress towards this.

In developing strategies, it is important to consider and involve governments, patient communities, insurance companies/payers and technology companies as key players that will influence implementation. Governments play a crucial role in shaping healthcare policy and regulation. Patient communities represent the individuals who will use care pathways and have a stake in the healthcare system. Insurance companies and payers will continue to finance healthcare services and manage reimbursement processes. Technology companies will be instrumental in developing the innovative digital health solutions that underpin future care pathways.

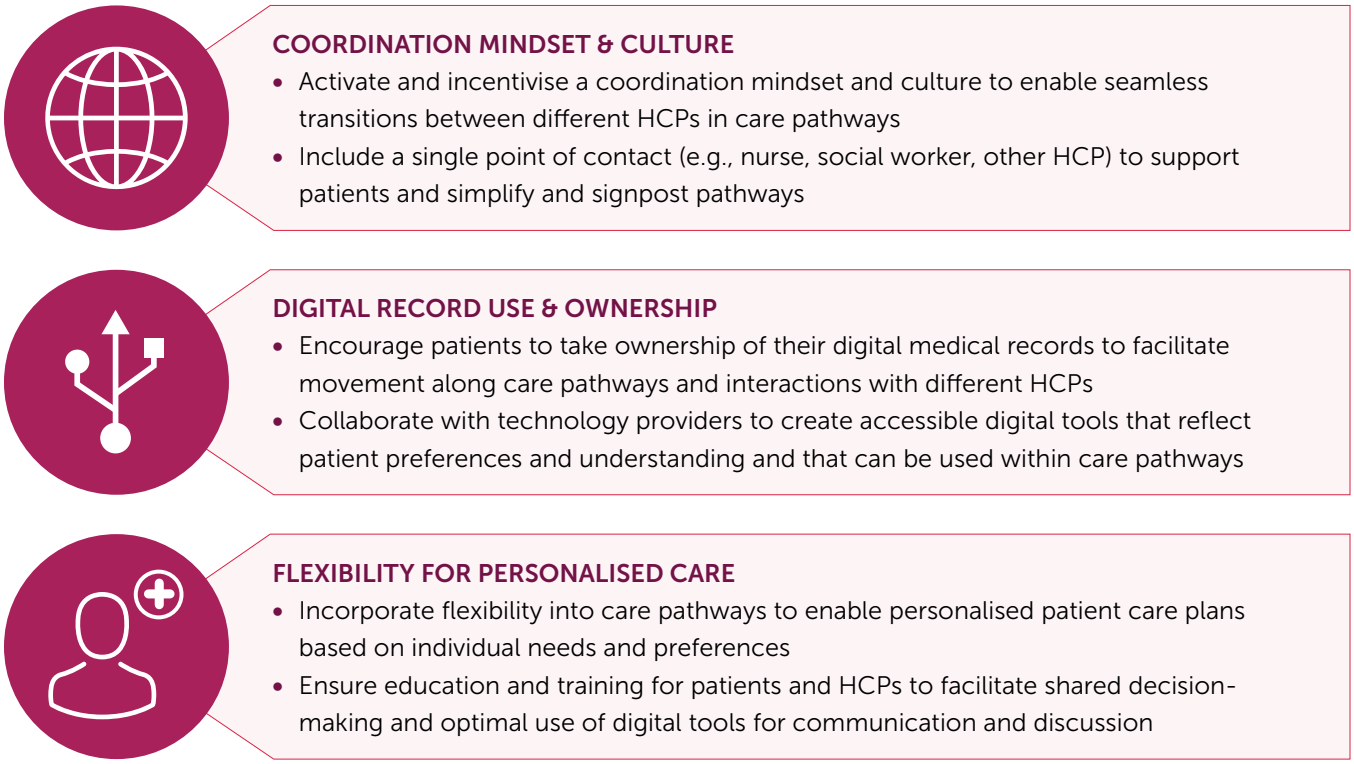
Digitalisation will be key to supporting care coordination and data collection within future pathways for rare diseases. To incorporate digital tools, a framework that addresses ethical questions of accountability and patient consent for data sharing will be needed [21]. Providing these issues can be satisfactorily addressed, digitalisation holds immense potential for app-based and wearable devices allowing patients to monitor and report symptoms and quality of life. It also enables a platform-based exchange of information between members of the multidisciplinary team (MDT) and patients [22,23]. Along with supporting community building and providing valuable insights to patients, collating individual data can provide MDTs with information about patients' real-world experiences between conventional consultations. In addition to personal benefits to individual care, data will help to develop meaningful and evidence-based changes to systems for rare disease communities.

A critical factor towards a success of digitalisation centres on patient preferences. Adaptation will require an understanding of individual patient health, digital literacy, and readiness and capacity to adopt digital tools as part of their routine care [24,25]. Patient communities must be involved in co-creation and decision-making around the development and implementation of digitalisation [26], and in active discussion around the actual and perceived advantages and disadvantages in data collection, primary and secondary use and analysis. In particular when we consider the more vulnerable patient communities, it is apparent that HCPs and patients require education and training to ensure that tools meet their specific needs [25,27], and patient access to services (devices and connectivity speeds) will also need to be addressed. Effective engagement with tech companies will be essential for ensuring the development of patient-friendly tools, together with clear policies and agreements on data usage and sharing. Special considerations for particular concerns or needs of the demographic (i.e., age, disability, social determinants of health) need to be considered in the design and delivery of digital solutions. As with all digital developments there are ethical considerations and the added complexity of artificial intelligence (AI) use in health adds to the need for oversight [28].

Effective care coordination will be essential in creating seamless future care pathways for rare diseases that incorporate digitalisation. Implementing or improving coordinated care will require investment and flexible, multistakeholder commissioning models. Workstream participants identified financial incentives as a powerful driver of change in coordinated care. Encouraging the development of reimbursement models prioritising care coordination over clinician time would further support this. Implementing and enhancing coordinated care approaches for rare diseases could also help overcome work silos within and between healthcare institutions, with more joined-up healthcare bringing less frustration for HCPs and patients as well as likely financial benefits. This would facilitate a system change over time to value-based healthcare, which in turn enables the creation of sustainable and measurable added value for hospitals and patients in their treatments beyond the product [14]. Lessons could be learned from the pathways and processes adopted in comprehensive care centres for haemophilia to deliver multidisciplinary, patient-focused care.

Recommendations to support the development of personalised future care pathways focused on enabling seamless interactions and transitions with HCPs throughout and flexibility within them (Figure 3). This includes:

Figure 3. Recommendations to support the development of seamless, personalised care pathways for rare disease patients



- Active fostering of a coordination, collaboration and co-creation mindset and culture
- Encouraging patients to take ownership of their digital healthcare records and the use of their health data
- Incorporating flexibility to enable personalised care plans.

DISCUSSION

The EHC Think Tank's Access Equity and Future Care Pathways workstreams have made recommendations to support system changes that stakeholders should consider to enhance health services for people with rare disorders such as haemophilia.

Developing an evidence-based narrative to showcase a value-based approach to access equity will enable champions for change to engage with all stakeholders to work towards a fair and impartial healthcare system. Through case studies backed by data and practical examples of how access equity can be successfully initiated and sustained, decision-makers can understand the tangible benefits of system change and relate them to their own experiences and situations. A patient-centred, three-tier, value-based framework for haemophilia has been developed and could be adapted for other rare disorders [29], including:

- **Tier 1** – Health status achieved or retained (e.g., haemophilia-specific bleeding frequency, musculoskeletal complications and life-threatening bleeds, as well as measures of function or activity)
- **Tier 2** – Process of recovery, including time to initial treatment, time to recovery and time missed at education/work
- **Tier 3** – Sustainability of health, maintenance of productive life and good health over time.

Measuring and monitoring the impact of such an approach, and capturing different perspectives, will be important in enabling outcomes to form the basis of case studies that can support further value-based initiatives and be shared and utilised broadly across stakeholders. These need to incorporate quality of life, and social and broad financial outcomes (patients and families/carers as well as healthcare and social services) [30], in order to give a complete picture of the effects of system change.

For advocates of future care pathways with integrated digital technologies, incentivising a mindset and culture of coordination will help lay firm foundations for 'right intervention, right time, right

HCP' services for patients. Implementation requires regulators to mandate the use of digital tools, and for health systems to communicate with each other at local, national and global levels. The involvement of diverse stakeholders and interdisciplinary co-creation underpins the approach of the Think Tank and has been described as a key enabler of digital innovation in healthcare [31]. The patient becomes a crucial central link between different systems, underlining the importance of encouraging patients to take ownership of their digital medical records. There is a critical need to facilitate a deeper understanding of the patient data used to address personal management and the impact of data sharing on the societal health system level, i.e. the secondary use of anonymised data for population health. This approach is aligned with regulatory requirements such as those agreed in the European Health Data Space (EHDS) adopted by the European Parliament in April 2024 [32]. This initiative aims to place individuals at the centre of their healthcare, granting them full control over their data, while allowing the use of health data for research and public health purposes, under strict conditions [22,23]. Importantly, for patients with rare diseases seeking treatment across borders, the EHDS facilitates immediate and simple patient access to their digital health data within the EU, irrespective of location. This will enable HCPs to access key information to support evidence-based decision-making, reduce test repetition, and enhance patient care.

The challenges to achieving significant progress towards access equity and future care pathways must be addressed. Access equity for patients with rare diseases requires a value-based approach. However, issues around cost continue to raise particular challenges due to the low numbers of patients and the resources needed to find and care for them relative to more common conditions [30]. Such an approach must consider epidemiology and evaluation of costs (medical, productivity, financial, etc.), disease progression, associated quality of life, and other health outcomes. Best practices and standards of care need to be identified, and networks created to share research, knowledge and values.

Similarly, while standardising digitally driven future care pathways is desirable from a service delivery viewpoint, this may overlook personal preference, choice, and ethical considerations. Some flexibility will be needed to ensure that care plans can be personalised to meet the needs of individual patients and optimise the way their disease is managed.

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 [33].

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.

CONCLUSIONS

Innovative strategies and recommendations for system change have been developed by a broad range of stakeholders in the Think Tank's Access Equity and Future Care Pathways workstreams. Key elements include developing an evidence-based narrative to showcase the value of access equity to decision-makers and creating future care pathways with integrated digital technologies that put patients at the centre of their care. Appropriate education and training for HCPs, patients and the wider stakeholder group will be essential for the success of these initiatives. It will also be critical to identify and support champions and advocates who can work across stakeholders, using an evidence-based narrative and mutually recognised lexicon, to stimulate and maintain partnerships built on trust and sustained focus on the 'guiding star' for both workstreams.

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REFERENCES

1. Hilton K, Anderson A. IHI Psychology of Change Framework to Advance and Sustain Improvement. IHI White Paper. Boston, Massachusetts: Institute for Healthcare Improvement; 2018. Available from https://www.ihi.org/resources/white-papers (accessed May 2024).
2. Atun R. Health systems, systems thinking and innovation. *Health Policy Plan* 2012; 27 Suppl 4: iv4-iv8. doi: 10.1093/heapol/czs088.
3. Bok A, Noone D, Skouw-Rasmussen N; EHC Think Tank. Key challenges for patient registries – A report from the 1st workshop of the EHC Think Tank Workstream on Registries. *J Haem Pract* 2022; 9 (1): 14-19. doi: 10.2478/jhp-2022-0002.
4. Bok A, Noone D, Skouw-Rasmussen N; EHC Think Tank. Key challenges for hub and spoke models of care – A report from the 1st workshop of the EHC Think Tank on Hub and Spoke Treatment Models. *J Haem Pract* 2022; 9 (1): 20-26. doi: 10.2478/jhp-2022-0003.
5. Bok A, Noone D, Skouw-Rasmussen N; EHC Think Tank. Patient agency: key questions and challenges – A report from the 1st workshop of the EHC Think Tank Workstream on Patient Agency. *J Haem Pract* 2022; 9 (1): 27-35. doi: 10.2478/jhp-2022-0004.
6. Skouw-Rasmussen N, Savini L; EHC Think Tank. Access equity: key questions and challenges – A report from the 1st workshop of the European Haemophilia Consortium (EHC) Think Tank Workstream on Access Equity. *J Haem Pract* 2023; 10(1) 56-61 doi: 10.2478/jhp-2023-0011.
7. Skouw-Rasmussen N, Savini L; EHC Think Tank. Future Care Pathways – A report from the 1st workshop of the EHC Think Tank Workstream on Future Care Pathways. *J Haem Pract* 2023; 10(1) 74-81. doi: 10.2478/jhp-2023-0013.
8. Bok A, Noone D, Skouw-Rasmussen N, on behalf of the EHC Think Tank. 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. *J Haem Pract* 2023; 10(1). doi: 10.2478/jhp-2023-0001.
9. Gacser Z, Skouw-Rasmussen N, Bourke S, ten Ham R, Hosszú D; EHC Think Tank. Short- and longer-term goals for change – A report from the 2nd workshops of the EHC Think Tank Workstreams on Access Equity and Future Care Pathways. J *Haem Pract* 2023; 10(1) 155-163. doi: 10.2478/jhp-2023-0023.
10. Gacser Z, Skouw-Rasmussen N, on behalf of the 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. doi: 10.2478/jhp-2024-0005.
11. EHC Think Tank. Access Equity | System map of the underlying challenges. Available from https://ehcthinktank.eu/workstream/workstream-four-access-equity/ (accessed May 2024).
12. EHC Think Tank. Future Care Pathways | System map of the underlying challenges. Available from https://ehcthinktank.eu/workstream/workstream-five-future-care-pathways/ (accessed May 2024).
13. LUCID. What is the lotus blossom technique? Available from https://www.lucidmeetings.com/glossary/lotus-blossom-technique#:~:text=The%20Lotus%20Blossom%20Technique%20is,the%208%20boxes%20surrounding%20it (accessed May 2024).
14. Teisberg E, Wallace S, O'Hara S. Defining and implementing value-based health care: a strategic framework. *Acad Med* 2020; 95(5): 682-685. doi: 10.1097/ACM.0000000000003122.

15. McQueen RB, Mendola ND, Jakab I, et al. Framework for patient experience value elements in rare disease: a case study demonstrating the applicability of combined qualitative and quantitative methods. *PharmacoEconomics Open* 2023; 7: 217-228. doi: 10.1007/s41669-022-00376-w.
16. De Santis M, Hervás C, Weinman A, Bosi G, Bottarelli V. Patient empowerment of people living with rare diseases: its contribution to sustainable and resilient healthcare systems. *Ann Ist Super Sanita* 2019; 55(3): 283-291. doi: 10.4415/ANN_19_03_15.
17. Chhetri D, Zacarias F. Advocacy for evidence-based policy-making in public health: experiences and the way forward. *J Health Management* 2021; 23(1): 85-94. doi: 10.1177/0972063421994948.
18. Kalkman S, van Delden J, Banerjee A, et al. Patients' and public views and attitudes towards the sharing of health data for research: a narrative review of the empirical evidence. *J Med Ethics* 2022; 48: 3-13. doi: 10.1136/medethics-2019-105651.
19. Koster F, Kok MR, Barreto DL, Weel-Koenders AEAM. Capturing patient value in an economic evaluation. *Arthritis Care Res* 2024; 76(2): 191-199. doi: 10.1002/acr.25229.
20. EURORDIS-Rare Diseases Europe. Process on Corporate Social Responsibility in the Field of Pharmaceuticals Platform on Access to Medicines in Europe Working Group on Mechanism of Coordinated Access to Orphan Medicinal Products (MoCA-OMP). Ref. Ares(2014)3857202 - 19/11/2014. Available from *http://download2.eurordis.org.s3.amazonaws.com/moca/history/WG%20MoCA-OMP%20Transparent%20Value%20Framework.pdf* (accessed May 2024).
21. Andreoletti M, Haller L, Vayena E, Blassime A. Mapping the ethical landscape of digital biomarkers: a scoping review. *PLOS Digit Health* 2024; 3(5): e0000519.
22. Bergier H, Duron L, Sordet C, et al. Digital health, big data and smart technologies for the care of patients with systemic autoimmune diseases: Where do we stand? *Autoimmun Rev* 2021; 20(8): 102864. doi: 10.1016/j.autrev.2021.102864.
23. Mikk KA, Sleeper HA, Topol EJ. The pathway to patient data ownership and better health. *JAMA* 2017; 318(15): 1433-1434. doi: 10.1001/jama.2017.12145.
24. Heijsters F, Santema J, Mullender M, et al. Stakeholders barriers and facilitators for the implementation of a personalised digital care pathway: a qualitative study. *BMJ Open* 2022; 12: e065778. doi: 10.1146/bmjopen-2022-065778.
25. Solebo AL, Hysi P, Horvat-Gitsels LA, Rahi JS. Data saves lives: optimising routinely collected clinical data for rare disease research. *Orphanet J Rare Dis* 2023; 18: 285. doi: 10.1186/s13023-023-02912-1.
26. Jacob C, Bourke S, Heuss S. From testers to cocreators – the value of and approaches to successful patient engagement in the development of eHealth solutions: qualitative expert interview study. *JMIR Hum Factors* 2022; 9(4): e41481. doi: 10.2196/41481.
27. Brasier N, Sempionatto JR, Bourke S, et al. Towards on-skin analysis of sweat for managing disorders of substance abuse. *Nat Biomed Eng* 2024. doi: 10.1038/s41551-024-01187-6.
28. Jobin A, Ienca M, Vayena E. The global landscape of AI ethics guidelines. *Nat Mach Intell* 2019; 1: 389-399. doi: 10.1038/s42256-019-0088-2.
29. O'Mahony B, Dolan G, Nugent D, Goodman C; International Haemophilia Access Strategy Council. Patient-centred value framework for haemophilia. *Haemophilia* 2018; 24(6): 873-879. doi: 10.1111/hae.13456.
30. Fantini B, Vaccaro CM. Value based healthcare for rare diseases: efficiency, efficacy, equity. *Ann Ist Super Sanità* 2019; 55(3): 251-257. doi: 10.4415/ANN_19_03_10.
31. Schlieter H, Marsch LA, Whitehouse D, et al. Scale-up of digital innovations in health care: expert commentary on enablers and barriers. *J Med Internet Res* 2022; 24(3): e24582. doi: 10.2196/24582.
32. European Commission. Commission welcomes European Parliament's adoption of the European Health Data Space and regulation on substances of human origin. 24 April 2024. Available from *https://ec.europa.eu/commission/presscorner/detail/en/IP_24_2250* (accessed May 2024).
33. European Haemophilia Consortium. Think Tank. Available from *https://ehcthinktank.eu* (accessed May 2024).
34. Chatham House. Chatham House rule. Available from *https://www.chathamhouse.org/about-us/chatham-house-rule* (accessed May 2024).

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