Community Voices in Research (CVR): A lived experience expert-centred approach to advance the future of inheritable bleeding disorders

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Introduction: Web-based registries are essential tools for gathering patient-reported information for research purposes. The National Bleeding Disorders Foundation recognised and responded to the imperative to actively include persons with inheritable bleeding disorders and their immediate family members, as Lived Experience Experts (LEEs), in all aspects of the research process. Consequently, *Community Voices in Research* (*CVR*) was developed and launched with the aims of creating a LEE-centred approach to complement existing research efforts, highlighting mechanisms to actively incorporate them, and using the data collected to identify relevant gaps in knowledge. **Methods:** The evolution of *CVR* reflects active LEE involvement in its development. *CVR*, a community-powered registry, collects information through electronic surveys to understand the unique LEE experience and incorporate it into research. Information collected includes demographics, social determinants of health, access to care, diagnosis, symptoms, pain, and mental health. A Personalised Dashboard engages LEEs by providing data collected and linking them to educational resources and other research opportunities. **Results:** *CVR* provides several mechanisms that allow LEEs to actively engage in different facets of the research process. LEEs share their experiences through timely surveys, and access publications using *CVR* data, opportunities to participate in other projects, and advisory boards. **Conclusions:** *CVR* is a vehicle to collect both historical and contemporary data directly from LEEs to address research gaps. *CVR* provides researchers with a better understanding of the needs of LEEs and incorporates their unique and important perspectives in research, education, and advocacy.

Community Voices in Research
National **Bleeding Disorders** Foundation

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There are multiple methods for gathering electronic and web-based data for research purposes including crowdsourcing [1,2], registries [3,4], web-based questionnaires, and patient-reported information [1-13]. These methods can recruit large cohorts of diverse samples in a

cost-efficient and convenient manner [1,8]. Patient-reported data and medical records agreement for clinical information on comorbidities can be up to 90% [9], although agreement between patient-reported data versus what is reported in the medical record can depend upon variables such as race [10], sociodemographics [11], complexity of disease [11], comorbidities [13], and age [11]. For example, in one study when subjects were older and reported depression and anxiety, only fair agreement was noted between participants and their health records when reporting on their hypertension and diabetic management [13].

Inheritable bleeding disorders (BDs) are a group of rare diseases that increase the tendency to bleed, and include coagulation factor deficiencies like haemophilia, von Willebrand disease, and platelet dysfunctions. Historically, efforts to collect data in the United States (US) from persons with inheritable bleeding disorders (PIBD) have centred on clinical information entered by the healthcare team. Since 1998, the Centers for Disease Control and Prevention (CDC) has collected surveillance data from federally supported specialised haemophilia treatment centres (HTCs), initially, via the Universal Data Collection [14] initiative, progressing to Community Counts in partnership with the American Thrombosis & Hemostasis Network (ATHN) [15]. Additional clinical and diagnostic data collected by HTCs through ATHN provides a national research infrastructure [16-17].

The National Bleeding Disorders Foundation (NBDF; formerly known as the National Hemophilia Foundation) recognised the need to amplify the patient voice in research. PIBD are appropriately deemed 'lived experience experts' (LEEs) because they know better than anyone else how BDs affects their lives, what they go through to get a diagnosis and treatment, how treatments affect them, how symptoms or side effects impact daily life, and the challenges of interacting with the health care system. The term LEE also includes immediate family members (parents, partners, siblings, and grandparents) because they develop similar knowledge through their own lived experience, gained from another important viewpoint. Including LEEs' perspectives in research ensures that research questions are relatable, recruitment and retention strategies are optimised, and study designs are less burdensome. Dissemination of results in lay language provides valuable and clear evidence to incorporate in shared decision making, promoting LEEs as active members of their health care team [18].

NBDF, founded in 1948, is the largest patient advocacy organisation in the US serving LEEs. Its mission is *"finding cures for inheritable blood and bleeding disorders and addressing and preventing the complications of these disorders through research, education, and advocacy enabling people and families to thrive"* [19]. NBDF has supported research for over five decades via grants and fellowships available to multidisciplinary HTC team members [19-21]. The NBDF Research Department's focus is three-fold:
a. address gaps in knowledge to improve the lives of LEEs;
b. ensure that the lived experience drives and influences the research process;
c. promote equity, diversity, and inclusion so that research findings include all.

As a person-centred organisation committed to working with rather than for LEEs, NBDF understands the need to incorporate LEE perspectives in all matters that involve them. To support this vision, NBDF created *My Bleeding Disorders Community* (MyBDC) in 2018, later rebranded to *Community Voices in Research* (*CVR*). *CVR* is a community-powered registry focused on comprehensively understanding the lived experience of inheritable BDs directly from individuals most affected by them.

In this paper, we:
- Document the evolution of *CVR* as a LEE-centric means to complement other community research efforts
- Highlight mechanisms to incorporate LEEs in different stages of the research process
- Identify LEE-centred gaps in research based on *CVR* data.

EVOLUTION OF CVR
Established in 1975, the Nurse's Health Study stands among the largest prospective investigations into the risk factors for major chronic diseases in women, with over 275,000 participants [22]. Motivated by this extensive longitudinal study, NBDF recognised a lost opportunity within the inheritable BD community to better understand LEE experiences, including those during the HIV and hepatitis crisis of the 1970s and 1980s, when at least 50% of LEEs with haemophilia contracted HIV [23] and 80% contracted Hepatitis C [24]. Consequently, the idea to establish a patient-reported, web-based registry to gather the experiences of PIBD and their families as LEEs was born, enabling timely data collection and reporting of their experiences and concerns.
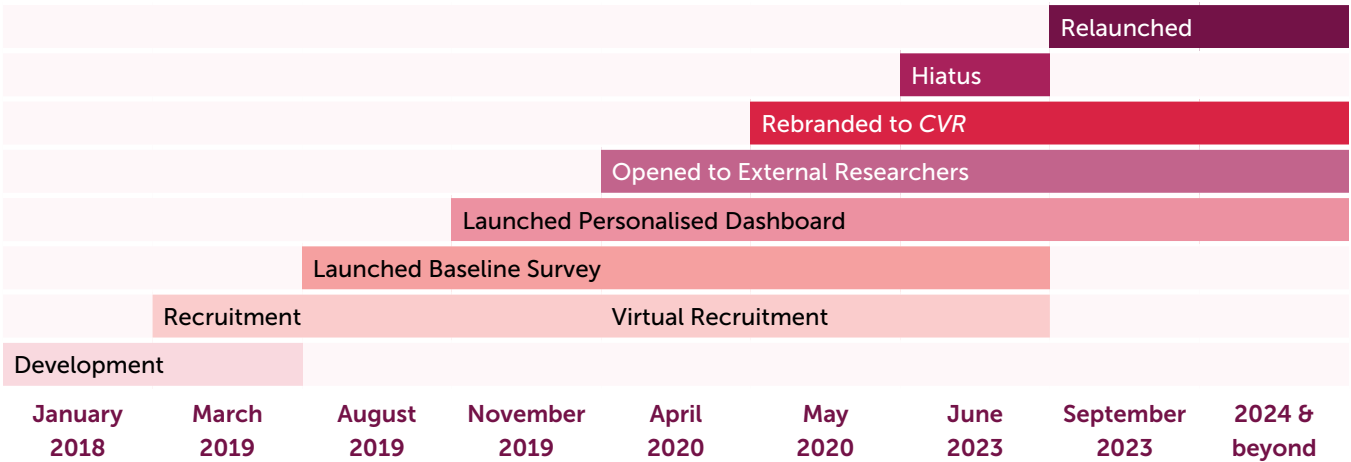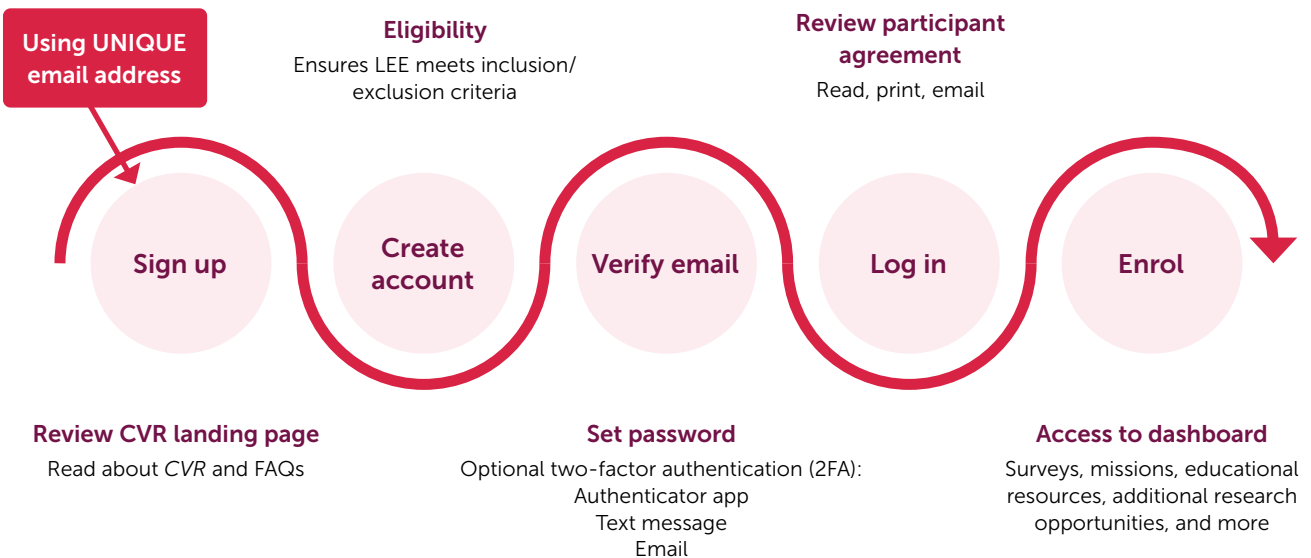
Figure 1. *CVR* development timeline

								Relaunched
							Hiatus	
						Rebranded to *CVR*		
					Opened to External Researchers			
				Launched Personalised Dashboard				
			Launched Baseline Survey					
	Recruitment		Virtual Recruitment					
Development								
January 2018	March 2019	August 2019	November 2019	April 2020	May 2020	June 2023	September 2023	2024 & beyond

Figure 2. *CVR* enrolment process

Using UNIQUE email address

Eligibility
Ensures LEE meets inclusion/ exclusion criteria

Review participant agreement
Read, print, email

Sign up → Create account → Verify email → Log in → Enrol

Review CVR landing page
Read about *CVR* and FAQs

Set password
Optional two-factor authentication (2FA):
Authenticator app
Text message
Email

Access to dashboard
Surveys, missions, educational resources, additional research opportunities, and more

MyBDC was NBDF's first effort to comprehensively describe the LEE perspective longitudinally through electronic surveys and place it at the center of research. Initial goals were to transform the lived experience into evidence, identify trends and patterns, and develop LEE-inspired research priorities. This would include input from LEE subgroups who had not previously participated in data collection efforts, e.g. those receiving care outside of HTCs.

The development of MyBDC, which evolved into *CVR*, included input from key stakeholders and diverse LEEs. Initial stakeholders were identified through NBDF chapters and other advocacy organisations, and then invited to focus groups held during large national meetings. Later, stakeholder groups were built around identifying and meeting certain diversity metrics in order to ensure a wide range of perspectives. These metrics

include race, ethnicity, gender, sexual orientation, age, and roles within the community (e.g., LEEs with various diagnoses, HTC clinicians, researchers, NBDF chapter and Hemophilia Federation of America representatives, government and industry partners, payors, and other research entities, such as ATHN).

METHODS

Aims
CVR aims to:
- comprehensively describe the experience of living with an inheritable BD;
- recognise relationships between health outcomes and variables such as bleeding, treatment, access to care, pain, anxiety, depression and social determinants of health;

- develop research questions and prioritise protocols based upon survey responses;
- provide participants with other research opportunities, research findings from data collected, and tailored educational resources;
- identify opportunities to improve health behaviours and quality of life through research, education, and advocacy.

Design
Recruitment
Recruitment began in March of 2019 (Figure 1) and focused on LEEs with self-reported inheritable BDs and their immediate family members over the age of majority living in the US. In-person educational and advocacy events, such as NBDF's Washington Days and Bleeding Disorders Conferences, and social media campaigns were targeted for recruitment.

Achieving a diverse and representative sample is more likely to happen with registries when compared to more traditional methods [1]. To ensure ongoing equity, diversity, and representation in *CVR*, recruitment efforts focus on participants seeking care outside the HTC network and on historically marginalised and understudied communities. The availability of a Spanish version of the surveys and dashboard, and Spanish speaking Hispanic recruiters support efforts focused on this traditionally underrepresented ethnic group. In 2020, the COVID-19 pandemic forced the research team to pivot to virtual settings where recruitment continued solely through webinars and social media.

In 2020, MyBDC was rebranded to *"Community Voices in Research"* (*CVR*), reflecting NBDF's increased emphasis on research and reaffirming its mission. Both MyBDC and *CVR* protocols have been reviewed and classified as 'exempt' with each major revision by a centralised Institutional Review Board. A brief enrolment hiatus occurred in 2023 while the *CVR* registry was transferred to a new platform with expanded capabilities focused on enhancing an intuitive and impactful participant experience (Figure 1).

Surveys
CVR collects data through web-based electronic self-reported surveys containing validated psychometric patient reported outcome measures (PROMs), and expert developed questions to assess demographic information and other relevant topics, see Table 1. The inclusion of LEEs since inception ensures that the data collected are relevant to them and encourages

participation and continued engagement. Enrolment begins by creating an account and taking a short survey that collects basic demographic and diagnosis information (Figure 2).

The *Baseline Survey*, which is repeated annually, gathers self-reported perspectives including diagnostic journey, bleeding history and treatment, pain, physical and mental health, comorbidities, functional status, access to specialised care and treatment, and social determinants of health - topics not being systematically collected elsewhere (Figure 3). Additional questions regarding reproductive health and menstrual bleeding are asked to women and persons who have or have had the potential to menstruate.

The experiences of immediate family members (mothers, fathers, siblings, grandparents, and other relatives) are explored via a separate *Family Baseline Survey*. *CVR* serves as an instrument for both internal NBDF staff and external researchers interested in LEE-reported information (Figure 4).

Personalised Dashboard
In addition to information via electronic surveys, *CVR* provides direct benefits to participants through a *Personalised Dashboard* (PD), which was launched in November 2019 once there was sufficient data for display. The PD allows participants to:
- create a health summary to share with health care providers and others;
- take available surveys;
- access aggregated, de-identified data, allowing them to follow their responses over time and compare them with others;
- connect with additional research opportunities (both internal and external);
- access research publications using *CVR* data in easy-to-understand language;
- explore vetted educational information.

Access to the *PD* is password protected and can be further secured with optional two-factor authentication.

Virtual and In-person Advisory Panels
CVR is used to recruit to NBDF-supported virtual and in-person advisory panels (VAPs and PAPs), providing the opportunity for LEEs to participate in research study design, and share their knowledge in the development of educational and market research materials (Figure 4). Invitations are based on predetermined inclusion and exclusion criteria. One goal of VAPs/PAPs is to diversify and expand the range of geographic and sociodemographic characteristics of LEE consultants.
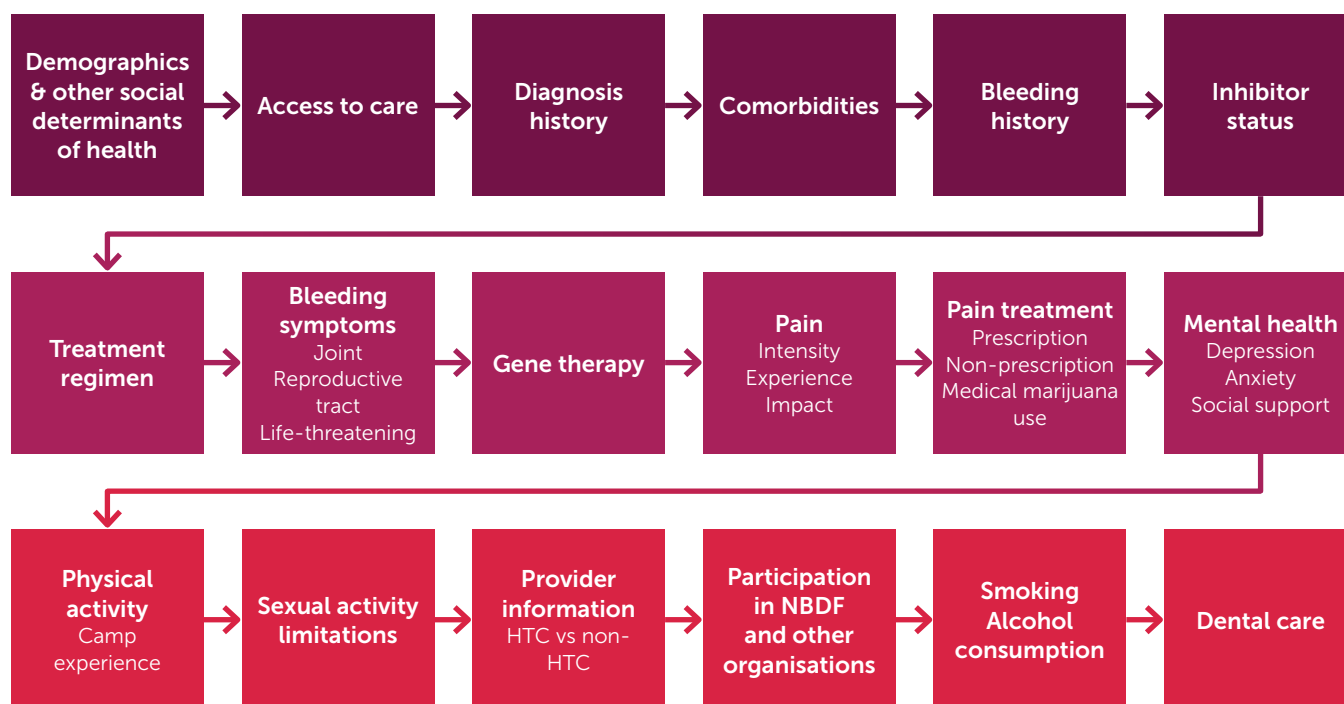
Figure 3. *CVR* data points

Table 1. Validated tools included in *CVR* surveys

TOOL	ABBREVIATION	*CVR* VERSION	FAMILY OR AFFECTED SURVEYS
Adult Sickle Cell Quality of Life Measurement Information System (Pain Impact)	ASCQ-Me Pain Impact	Both	Both
Alcohol Use Disorders Identification Test-Concise	AUDIT-C	New	Both
Area Deprivation Index	ADI	New	Both
Brief Resilient Coping Scale	BRCS	New	Both
Duke-UNC Functional Social Support Questionnaire	FSSQ	Both	Both
EuroQual 5 dimensions of health-5 levels	EQ-5D-5L	New	Both
Family Experience with Health Care Provider Questionnaire	FHCPQ	New	Family only
International Physical Activity Questionnaire-Short form	IPAQ-SF	New	Both
Pain questions from the 2016 National Health Interview Survey	NHIS pain	Both	Both
Pain Self-Efficacy Questionnaire	PSEQ	New	Both
PROMIS anxiety short form 7a	PROMIS-Anxiety 7a	Both	Both
PROMIS depression short form 8b	PROMIS-Depression 8b	Both	Both
PROMIS neuropathic pain quality 5a	PROMIS-Neuro Pain Quality 5a	Both	Both
PROMIS pain intensity 3a	PROMIS-Pain Intensity 3a	Both	Both
PROMIS pain interference 4a	PROMIS-Pain Interference 4a	Both	Both
Protocol for Responding to and Assessing Patients' Assets, Risks, and Experiences	PRAPARE	New	Both
Ruta Menorrhagia Questionnaire	RMQ	New	Both
Zarit Scale of Caregiver Burden (Zarit Burden Interview)	ZBI	New	Family only

Figure 4. *CVR* as a community tool

Participants (via Personalised Dashboard)	NBDF	External Collaborators
• My health summary • My surveys • My data • Comparison to others • My impact (publications) • Educational resources • Additional research opportunities	• Virtual and in-person advisory panels • Data queries and analysis • Rapid research cycles	• Data queries • Focused surveys • Input towards study design • Link LEEs to research opportunities

NBDF holds approximately 8-10 advisory panels per year facilitated by experts in the field. VAPs are usually two to three hours long and PAPs are around six to eight hours. Panels are composed of 6-10 LEEs to ensure that everyone can share their perspective. When outside researchers or industry sponsors are involved, participants' personal or identifying information is kept private. Spanish and additional language translators are available during the eligibility process and panel discussions as needed. NBDF pre-screens questions and scripts with researchers to ensure session goals. Participants are reimbursed for their time. A final comprehensive written report is available to the sponsor/partner.

Examples of past advisory panel topics include:
• improving the relationship between researchers and the BD community
• understanding emergency room experiences
• exploring health equity, diversity, and inclusion issues
• defining challenges with diagnosis and treatment
• clinical trial design
• development of educational programs and materials.

Data Collection, Storage, and Analyses of CVR
Data are safely gathered and stored on a secure server using the strictest of industry standards. NBDF staff do not have access to participants' identifiable information. The information collected remains confidential and is only de-identified and aggregated data is reported.

Data are de-identified by a professional statistician with extensive research experience, in collaboration with the platform vendor via a secure file transfer protocol (SFTP). The data are then analysed,

summarised, and aggregated by the statistician before results are shared. Raw data and individual responses are not accessible to NBDF, the community, or external researchers to protect data security and participant privacy – this is a priority to NBDF.

CVR has a certificate of confidentiality from the National Institute of Health (NIH) protecting the privacy of enrolled participants' identifiable and sensitive information [25]. With limited exceptions, this certificate also protects NBDF from having to disclose information gathered to legal authorities.

RESULTS
Since the launch of *CVR*, enrolment has continued to increase (Figure 5). Data on topics collected (Figure 3) provide information to stimulate ideas for research and answer descriptive or correlational research questions.

Starting in early 2020 and in partnership with NBDF, external researchers have been able to develop and launch surveys via *CVR* with specific research questions based on the literature and pre-existing data. Examples include the validation of a Pain PROM and a discrete choice experiment. Table 2 shows publications to date. Surveys can be tailored and sent to participants conforming to specific inclusion and exclusion criteria. Results are provided to external researchers in a de-identified and aggregated manner.

Through the PD, participants continue to have access to pending and completed surveys. They may also view aggregated survey responses in chart format, with the option to custom filter (e.g., by age and diagnosis) to further understand how their answers compare to others like them. NBDF staff regularly develop and curate new content aimed at continuing engagement.

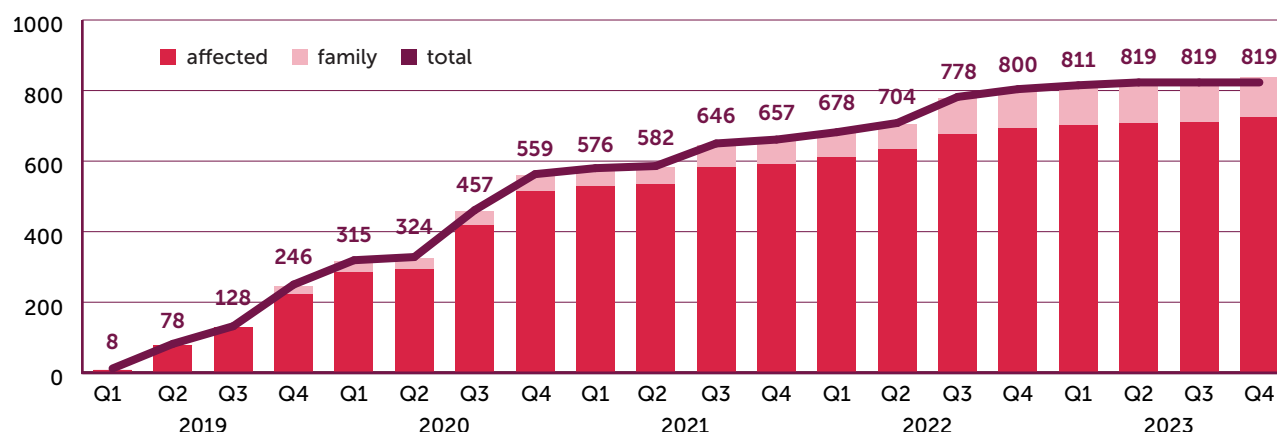Figure 5. Cumulative enrolment in *CVR* since 2019

Table 2. Publications and abstract presentations using *CVR* data to date

YEAR	TITLE	PUBLICATION	MEETING
2024	The relationship between different pain measures, depression, and social support and race and ethnicity in persons with hemophilia	AJH	THSNA
	Glanzmann Thrombasthenia beyond bleeding: Insights from lived experience experts	Haemophilia	WFH
	Pain attitudes and pain outcomes among people with bleeding disorders: Results from community voices in research [27]	Haemophilia	
2023	Gene Therapy for Hemophilia A: A Mixed methods study of patient preferences & decision-making [28]	PPA	
	Development of a haemophilia A gene therapy shared decision making tool for clinicians [29]	Haemophilia	
	Gene therapy preferences & informed decision-making: Results from NHF *CVR* survey [30]	Haemophilia	
2022	Relationship between perceived social support, mental health, activity, & chronic pain in PIBD [31]	Blood	ASH
2021	Community Voices in Research (*CVR*): A patient-centric approach moving the future of IBDs forward [32]	RPTH	HTRS
	Community Voices in Research (*CVR*): A patient-centric approach to study design phase through Community Research Network		DIA
	Poor outcomes in people with hemophilia: Physician & subject matter expert perspectives		ATHN
	Bleeding disorder data registry reveals racial/ethnic disparities that could significantly impact patient journey [33]	Blood	ASH
	Utilizing a community registry to analyze pain limitations in PIBDs		ASPMN
	The relationship between self-reported physical activity, treatment, regimen, mental health, & pain in persons with hemophilia [34]	Haemophilia	WFH-MSK
	Patient preferences & priorities for haemophilia gene therapy in the US: A discrete choice experiment [35]	Haemophilia	
	Evidence of a disability paradox in patient-reported outcomes in haemophilia [36]	Haemophilia	
	Preferences of people with hemophilia A and B for treatments including gene therapy in the US: A discrete choice experiment [37]	RPTH	THSNA
2020	Developing My Bleeding Disorders Community (MyBDC): A community-powered registry to provide a 360-view of living with a bleeding disorder [38]	Haemophilia	

AJH: American Journal of Hematology
ASH: American Society of Hematology
ASPMN: American Society for Pain Management Nursing
DIA: Drug Information Association
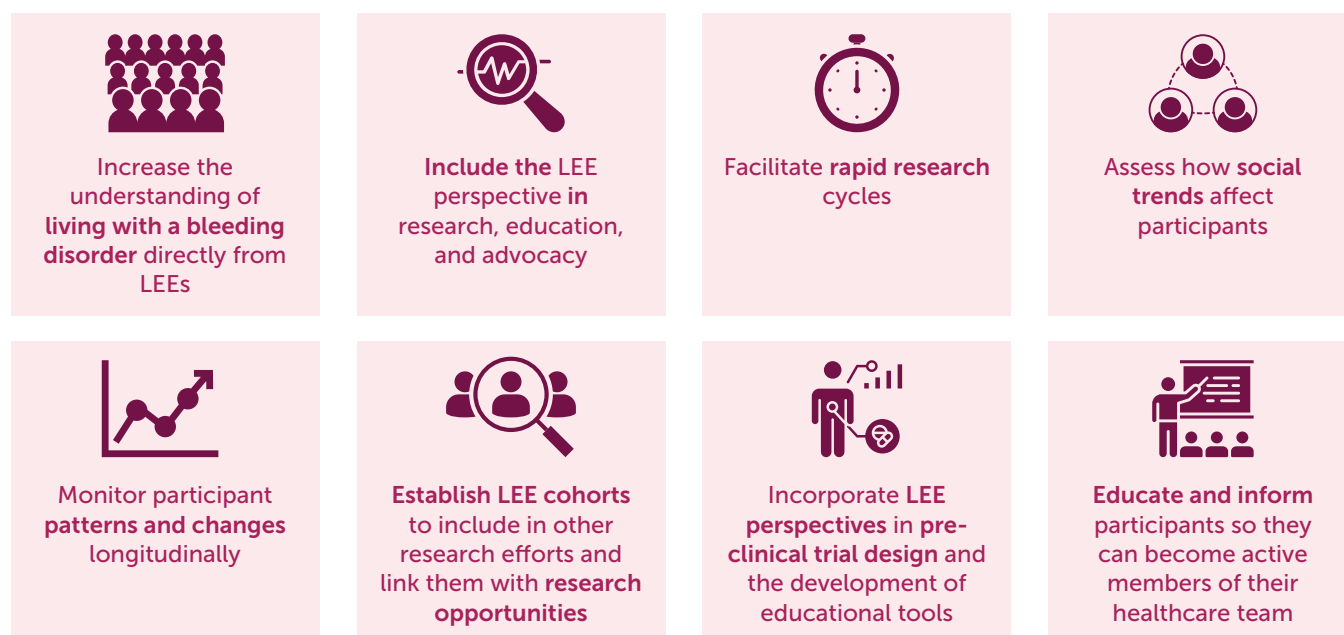PPA: Patient Preference and Adherence

RPTH: Research and Practice in Thrombosis and Haemostasis
THSNA: Thrombosis and Hemostasis Summit of North America
WFH: World Federation of Hemophilia Annual Congress
WFH-MSK: World Federation of Hemophilia Musculoskeletal Congress

Figure 6. The power of *CVR*

Increase the understanding of **living with a bleeding disorder** directly from LEEs	**Include the** LEE perspective **in** research, education, and advocacy	Facilitate **rapid research** cycles	Assess how **social trends** affect participants
Monitor participant **patterns and changes** longitudinally	**Establish LEE cohorts** to include in other research efforts and link them with **research opportunities**	Incorporate **LEE perspectives** in **pre-clinical trial design** and the development of educational tools	**Educate and inform** participants so they can become active members of their healthcare team

CVR's ability to swiftly query participants on relevant topics and gauge the community's stance in real time enables a "rapid research cycle". A five-question Vaping Survey, aiming to understand the impact of lung injury associated with the use of e-cigarettes and launched in August 2019, was the first test of this capability. Upon survey completion, respondents were directed to the CDC website and offered additional vetted education [37]. Another example is the 'COVID-19 Questionnaire', launched in April 2020, aimed at understanding how the pandemic was initially affecting participants. Based on the results, NBDF developed and launched COVID-19-specific resources to support LEE needs.

As *CVR* evolved and expanded, it became necessary to change platform vendors to improve its capabilities for both LEEs and researchers. A transition and re-development period in 2023 resulted in a more attractive and intuitive tool, offering enhanced functionality and safety measures.

CVR surveys are voluntary and, generally, uncompensated. Periodically, however, incentives are offered to encourage enrolment and participation in additional research surveys. During the COVID-19 pandemic, recruitment through social media resulted in rapid enrolment, raising concerns about potential bot activity and the creation of false accounts. A complex algorithm based on multiple criteria (e.g., mismatches between enrolment and baseline demographic information, survey completion time impossibly short, patterns of consecutive surveys with systematic numerical sequences, etc.) was developed to identify potential invalid entries. If more than two criteria were met, the statistician reviewed the individual responses, and a clinical expert validated the final determination for inclusion or exclusion from analyses. Entries that did not meet these criteria were permanently deleted. To mitigate the chances of this reoccurring, the *CVR* team implemented a stricter enrolment process, including the Completely Automated Public Turing Test to Tell Computers and Humans Apart (CAPTCHA), email addresses verification and use of pre-established clinical and demographic criteria to flag or deny enrolment. The team also monitors enrolment trends, addressing suspicious activity quickly, and financial incentives are used judiciously.

DISCUSSION

CVR is a nimble, versatile, and powerful community tool with the primary aim of incorporating the LEE firsthand experience and perspective into research (Figure 6).

During the inception of *CVR*, NBDF identified an unexplored opportunity within the inheritable BD community to gain deeper insights into the LEE experience. With nearly five years of implementation, *CVR* is making substantial strides in comprehensively gathering the experiences directly from those most impacted by inheritable BDs and their family members, devoid of filters from clinicians or researchers. The current interaction of *CVR* facilitates swift responses to societal events that may impact the lives of LEEs by furnishing timely and pertinent data for research purposes.

CVR has enriched researchers' understanding of the nuanced needs of LEEs and their families directly

from them, enabling their unique viewpoints to be incorporated into research, education, and advocacy. It ensures that the information collected is relevant and accurately represents participants' views and experiences. As *CVR's* enrolment and vision expansion outpaced the initial platform's capabilities, a new vendor was engaged in 2023 to accommodate growth and analytical needs, paving the way for ongoing adaptation to the evolving research landscape. Collaboration with state NBDF chapters, other non-profits, government entities, and ATHN remains integral to the research process, fostering regular interactions both virtually and in person.

The *CVR* PD serves as a bridge between clinicians, researchers, and LEEs, facilitating effective information exchange. It provides education and research findings and enables LEEs to link with additional research opportunities. *CVR* also furnishes a robust cohort for research purposes and integrates participants' input into pre-clinical trial designs, as well as the development of educational and promotional materials. Its infrastructure facilitates rapid research cycles, monitors longitudinal changes, and identifies forthcoming research inquiries pertinent to all stakeholders.

CVR represents a pivotal starting point for researchers to actively engage with LEEs through the VAPs/PAPs programmes. Advisory boards now include LEEs in the discussion of future research proposals, enabling a unique, timely, and personal perspective to research questions and relevant findings. Including LEEs early in the research process as members of the team and utilising *CVR* data helps identify and pursue community-prioritised research gaps.

CVR has accelerated the movement to engage LEEs at regional and national levels, in rural communities, and outside the HTC network. Strategies to augment LEE engagement with *CVR* include the development of the personal 'health summary' (summarising participants' health history and current health concerns), and an updated family baseline survey (including self-reported caregiver burden items), and a direct linkage to additional research opportunities beyond *CVR* surveys and clinicaltrials.gov.

CVR data has enabled assessment of several LEE-centred gaps in research, including pain issues [27,31,34], gene therapy preferences [28-30,37], perceived social support and mental health [31,34], racial and ethnic disparities among PIBD [33], and the disability paradox when comparing persons with haemophilia to the general population [36]. It has also increased awareness of how timely issues affect LEEs participating in *CVR* and has resulted in educational initiatives focused on vaping and COVID-19. As more participants enrol and the data matures, the opportunities to identify additional gaps in research will increase further.

Challenges, Barriers, and Limitations

The data collected through *CVR* represents the perspectives of self-selected enrolled participants and may not fully represent that of all LEEs. LEEs without access to a computer, smartphone, or Wi-Fi may be excluded from the registry.

Reliance on web-based recruitment during the COVID-19 pandemic highlighted issues associated with this method and the importance of close monitoring to ensure data validity.

Protection of data remains a top priority. During the 2023 hiatus, the *CVR* research team established a strict enrolment protocol including CAPTCHA and two-factor authentication and ensured that only entries meeting predefined criteria were enrolled.

While some clinicians have expressed concerns that self-reported data may not be as accurate as data collected directly from the clinician, many studies have shown that self-reported data [40-42] and patient-reported outcomes are accurate and valid [43,44]. Their use is recommended to better understand LEE views and measure outcomes relevant to them [45].

CONCLUSION

The inclusion of LEEs to enrich research has been effectively highlighted through the implementation of *CVR*. This approach not only offers a mechanism for engagement, but also allows for a deeper insight into their perspectives. It is imperative that LEEs become active partners in every phase of research. Such involvement presents LEEs and researchers with the opportunity to develop more relevant studies and educational initiatives in a timely manner.

Author contributions

MES, CN, and MLW equally participated in the conceptualisation and preparation of the review. All authors read and approved final manuscript.

Ethics

CVR has a certificate of confidentiality from the National Institute of Health (NIH) that protects the privacy of enrolled participants' identifiable and sensitive information. Both MyBDC and *CVR* protocols have been reviewed and classified as Exempt with each major revision by a centralised institutional review board.

Data availability

Available on reasonable request.

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