

Patients as Partners: Investing in a New Era of Patient-Oriented Research

Introducing the Research Capacity Building Project. In recent years, PHA Canada has deepened our commitment to supporting PH research that advances the care of patients and brings us closer to a cure. We have been proud to invest in the next generation of PH researchers through our PH research scholarship program, partner on innovative research projects like the PH Priority Setting Partnership, and support collaboration among researchers and health care providers. 2022 saw us scale up our efforts with the launch of a new multiyear initiative that aims to enhance Canada's PH research environment and build capacity for patient-oriented research.

Research Capacity Building Project

The Research Capacity Building Project will focus on three main priorities:

1. Establishment of a Canadian PH Clinical Trials Network
2. Expansion of the Canadian PH Registry to include a biobank
3. Development of patient education and training initiatives

These three project areas reflect a broad-based approach that seeks to improve coordination and collaboration among PH researchers, increase access to critical new sources of patient data, and empower patients to be meaningfully engaged in all aspects of the research process.

For 15 years, PHA Canada has thrived as a collaboration between patients, caregivers, and health care providers. Each pillar of the community has drawn strength and inspiration from the other. The Research Capacity Building Project will expand the partnership between patients/caregivers and the PH medical community to ensure that the needs, values, and priorities of patients/caregivers are at the heart of the PH research agenda in Canada.

PHA Canada has always worked closely with the medical community to advance our mission. In addition to the leadership of medical experts on PHA Canada's Board of Directors, the organization is supported by volunteer advisory committees of physicians (the Canadian PH Medical Committee) and nurses (the Canadian PH Professionals Network) who work with staff and the Board to ensure outstanding care and support, through research, advocacy, awareness, and education. The Research Capacity Building Project reflects the strength and solidarity of Canada's PH community and the enduring hope we share that patients and caregivers can live well with PH.

Canadian PH Clinical Trials Network

One example of PHA Canada's leadership in advancing quality care and research is the annual *National PH Medical Think Tank*, which brings together experts for an interactive program aimed at optimizing the diagnosis and management of PH in Canada. At the 2021 Medical Think Tank, participants identified the need for a "Canadian PH Clinical Trials Network."

A clinical trial is a research study that evaluates the safety and effects of one or more treatments on actual patients. Unlike clinical trials for more common diseases such as cancer or arthritis, there are unique challenges associated with rare disease clinical trials. For instance, about 30% of clinical trials fail because they can't recruit enough participants¹. This is especially true in rare disease clinical trials where the patients are fewer and scattered around the globe. Additionally, because patient populations for rare diseases are smaller and often diverse in terms of age, sex, and/or severity of illness, common methods for clinical trial design often fail to demonstrate significant outcomes with certainty. As a result, studies for rare disease research must find ways to adapt their designs without compromising the quality of the research.

Planning rare disease clinical trials can get complicated. To help overcome challenges like these, the emerging Canadian PH Clinical Trials Network will focus on improving information sharing, coordination, and collaboration across research centres and on sharing expertise and resources more efficiently.

Patient Registries and Biobanks

Clinical trials are not the only way for patients to contribute to innovative research. When assisted by new information technologies, the collection of patient data—such as found in patient registries and biobanks—provides researchers with access to information that can reveal previously unknown similarities between patients (i.e., genetic mutations) and potential new treatment pathways.

A disease registry is a unique database that contains information about people diagnosed with a specific type of disease. Human biobanks collect and supply human tissue, blood specimens, and associated data for research. These approaches have the potential to increase scientific efficiency by maximizing the use of valuable and rare data and samples.

The Canadian PH Registry (CPHR) was created in 2017. Since then, approximately half of Canada's established PH expert centres, including two pediatric centres, have joined the registry or taken steps to include their patient population. Adding a biobank to the CPHR—beginning with blood samples that can easily be collected during routine right heart catheterization—has the potential to advance Canada's research capacity significantly. Furthermore, the *Canadian PH Community Survey* firmly established the willingness of patients to provide blood or tissue samples for storage in the Canadian PH Registry, with 78% of patients responding yes to the option.

Patient Education and Training

The third pillar of the Research Capacity Building Project includes a broad range of learning opportunities for patients and caregivers. This includes increasing the availability of research-related blogs, articles, and updates through PHA Canada's regular communication channels, as well as offering research-related workshops via webinars and at the National Community Conference.

This pillar also includes training for community leaders seeking to level up their advocacy skills on behalf of the PH community. Research advocates are patients and caregivers trained to provide patient perspectives for the purpose of advancing science. Most research advocates are not scientists. Training for community leaders—such as PHA Canada's Ambassadors—will help advocates gain skills to collaborate with scientists and clinical researchers.

We also need to increase the PH community's knowledge and understanding of the value of real-world evidence (data generated in settings outside clinical trials) and the role of patient-reported outcomes (an outcome reported directly by patient who experienced it). Both will become increasingly important to the design of future clinical trials and the drug approval and reimbursement process.

Summary

For 15 years, PHA Canada has brought together pulmonary hypertension patients, caregivers, and healthcare professionals to better the lives of all Canadians affected by PH. An underlying principle of the Research Capacity Building Project is that we invest our attention and resources on activities that will be impactful on a national level. We want this initiative to create a foundation of collaboration and cooperation that ushers in a new era in patient-oriented PH research in Canada. Because the only way to achieve better outcomes for patients, is to include patients every step of the way.

We will also be including you every step of the way. Stay tuned for regular updates from the Research Capacity Building Project in future editions of Connections!

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¹ Khaleel, S. L. (n.d.). Rare Disease Patient Recruitment And Retention. Retrieved from <https://www.clinicalleader.com/doc/rare-disease-patient-recruitment-and-retention-0001>.