PULMONARY HYPERTENSION ASSOCIATION OF CANADA

L'ASSOCIATION D'HYPERTENSION PULMONAIRE DU CANADA

Canadian Pulmonary Hypertension Registry: Background for Patient Partners

The Canadian Pulmonary Hypertension Registry (CPHR) is a database that stores medical information about pulmonary hypertension (PH) patients. Because pulmonary hypertension is a relatively rare disease, finding enough participants for clinical trials and other research studies can be difficult.

The registry includes clinical data- information that is either generated by your doctor during your clinic visits or by researchers conducting clinical trials and studies. The patients themselves generate other data. These data are called Patient Reported Outcomes (PROs). They give doctors and researchers a better understanding of how pulmonary hypertension patients perceive their quality of life: people's daily lived experience, the health outcomes they think are important, and the side effects of current treatments.

"Real-world data from disease registries can help improve understanding about the natural history of a disease and assess patient outcomes beyond just those patients who would be eligible for a clinical trial" (Canada's Drug Agency).

What the registry is for (goals and objectives)

It has two overall goals:

- To improve care for Canadians with pulmonary hypertension.
- To improve the ability to monitor treatment, diagnosis, and therapy outcomes.

The CPHR tracks many people with pulmonary hypertension over time, making it a powerful tool for understanding the needs of this population, its changing characteristics, and the impact of interventions on health outcomes. Clinicians and researchers can use it to better understand disease patterns and answer research and quality improvement questions.

Other objectives for the registry:

- To track clinical outcomes such as hospitalizations, lung transplantation and death.
- To investigate clinical, imaging and prognostic parameters associated with outcomes in pulmonary hypertension.
- To investigate the effect of pulmonary hypertension therapies on hospital admissions, patient quality of life, and clinical and demographic factors associated with good responses.
- To serve as a repository of data for future retrospective research on pulmonary hypertension in Canada.

- To permit contact for future research participation (optional).

An additional goal now is to include more patient-reported outcomes – things not included in clinical trial data but useful for regulators (Health Canada) and other kinds of assessments (Canada's Drug Agency). This kind of data is needed both before and after drugs are approved.[1]

How the registry works

Each participating pulmonary hypertension clinic enters patient data into the CPHR, which is stored on a secure server at the University of British Columbia and accessed using a tool called REDCap. Currently, 11 adult and two pediatric centers are participating, and more sites may join soon.

How people join the registry

Patients with diagnosed or suspected pulmonary hypertension can participate in the registry if their PH clinic is a CPHR participant. If patients consent, their data are included in the registry. Clinics may ask patients if they want to be included, but patients can also proactively ask.

How data get into the registry

Participants have specific data collected for the registry whenever they visit their pulmonary hypertension clinic. These data are then entered into the registry by trained and authorized people at each PH center. Since this is in addition to regular medical charting, entering data into the registry has a significant administrative cost (covering this cost is an ongoing challenge).

Participants can stop participating in the registry at any time without giving reasons. People leaving the registry can choose to have their data destroyed (to the extent that it is possible) or kept for use in future research projects.

Data security

The only possibly identifying healthcare data stored in the registry is the participant's date of birth (sometimes just the month and year) and postal code (sometimes just the first three digits), but these data are not shared with anyone. Each participating patient is given a unique identifier (an alias, essentially) used in the registry. People's actual names are not in the registry. Each center stores its list of study identifiers in an encrypted, password-protected file separate from the registry. Only authorized people can access this list.

[1] If you would like to read more about how rare disease patient voices and data are having an impact at the regulatory level, the executive summary of Building a National Strategy for Drugs for Rare Diseases: What We Heard from Canadians is worth a read.

When the data from the registry is studied, it is “de-identified” (any data that might identify someone is removed) and aggregated, meaning a patient's name is not associated with any data and individual data is only examined as part of a group.

Each participating clinic must obtain Research Ethics Board (REB) approval from their institution and signed consent forms from each patient who has agreed to participate in the CPHR. Clinics must ask every participating patient for consent[2], even if they previously gave consent at another clinic. Clinics are responsible for submitting only consented data[2].

Medical information in the registry is confidential and cannot be shared with third parties except the patient’s medical team.

Individual centers control their data and can use it at their discretion, pending local Research Ethics Board approval. They may also grant other investigators access to their data for proposals approved by an oversight Steering Committee (which includes patient representatives) and local ethics boards.

Projects other than retrospective data review must seek informed consent from registry participants before the data can be extracted from the database.

How the registry can be used

De-identified aggregate data from the registry can be used to:

- Study the effectiveness of medications, products, and treatments.
- Improve the efficiency of future clinical trials. The registry makes it easier to find appropriate patients for a particular trial, and historical data can be reinterpreted more easily.
- Study the long-term effects of medications.
- Seek funding for and conduct research.
- Inform decision-makers such as the Canadian Drug Agency and Health Canada.
- Conduct quality improvement initiatives.
- Advocate for resources from provincial and national governments.
- Educate the public about pulmonary hypertension

Aggregated and anonymized patient data provide researchers with a significant cache of data, with patient-reported quality-of-life information included alongside clinical data. Clinical trial sponsors are working with drug approval agencies to adapt clinical trials to better fit the needs of small rare disease populations. Drug approval agencies are also looking at how to get new therapies to more rare disease patients quickly by trying to determine what and how patient-reported information can improve and speed up their decision-making. Registries such as the CPHR will be an important source of patient-generated information throughout the drug development and approval process.

[2] The Vancouver site has received a waiver of consent for some data from their ethics board.

What data are collected

A steering committee of Canadian pulmonary hypertension specialists agreed on a core dataset. When available, data is collected uniformly for every patient.

In projects like the CPHR, there is always a balance between what is practical and what would be ideal. The more data are collected per patient; the less likely data entry is sustainable because the cost of conducting the necessary tests and entering the data in the registry is higher. But, of course, everyone involved wants the registry to be as useful and future-proof as possible. There is always a need to make carefully considered compromises.

Table 1 shows all the data points collected for each patient in the registry. Some data, such as sex and ethnicity, are collected only at the initial visit as they are unlikely to change. Data points that may change are collected at all visits.

Table 1. Canadian Dataset

Core Data Points	Initial Visit	Follow Up Visits
Age	X	X
Sex (born)	X	
Self-reported Gender	X	
Self-reported Ethnicity	X	
Postal Code	X	X
WHO Group	X	X
Incident/Prevalent/Screening	X	
Comorbidities	X	X
Height Weight	X	X [weight only]
Syncope (fainting)	X	X
Clinical signs of Right Heart Failure (RHF)	X	X
Heart Rate	X	X
emPHasis-10 Quality of Life Questionnaire	X	X
NYHA/ WHO Functional Class	X	X
6 Minute Walk Distance O_2 use O_2 saturation	X	X
Blood Pressure	X	X
Right Heart Catheterization (measures pressures in the pulmonary artery to definitively diagnose PAH): • Mean right atrial pressure (mRAP) • Pulmonary artery pressure systolic/diastolic/mean (PA S/D/M) • Pulmonary artery wedge pressure (PAWP) and/or left-ventricular end-diastolic pressure (LVEDP)	X	X

Core Data Points	Initial Visit	Follow Up Visits
• Cardiac output (CO) • Venous oxygen saturation ($SvO_{2)}$ • Vasoreactivity		
Echocardiogram (ultrasound of the heart, estimates pressures in the right heart and assesses how well the heart is functioning): • Pericardial effusion • Right ventricular size • Right ventricular function • Tricuspid annular plane systolic excursion (TAPSE) • Right atrial area • Tricuspid regurgitation (TR) • Tricuspid regurgitant velocity (TR velocity) • Right ventricular systolic wave prime (RV S')	X	X
Pulmonary Function Tests (PFTs) (to potentially identify the cause of PH, these tests measure how much air your lungs can hold, how much air moves in and out of them, and the lungs' ability to exchange oxygen): • Forced expiratory volume ($FEV_{1)}$ • Forced vital capacity (FVC) • Diffusing capacity of the lungs for carbon monoxide (DLCO)	X	X
Ventilation–perfusion (V/Q) (uses radioactive material to produce a picture of air and blood flow to the lungs) and computed tomography (CT) (uses radiation to produce precise pictures of structures in the chest) scan	X	X
B-type natriuretic peptide (BNP) or N-terminal pro b-type natriuretic peptide (NT-pro-BNP) (helps assess heart function and response to treatment)	X	X
Creatinine (measures how well the kidneys are working)	X	X
Pulmonary Hypertension Medications	X	X
Anticoagulation Medications	X	X
Home Oxygen Use/CPAP/BiPAP	X	X
Progression of Symptoms		X
Hospitalization	X	X
Outcomes The primary outcome measurement is all-cause mortality. Secondary outcomes: 1. Quality of Life (emPHasis-10 questionnaire) 2. Hospitalization (all-cause) 3. Hospitalization for PAH 4. Emergency department visits 5. Central line infections 6. Lung transplantation		X

Core Data Points	Initial Visit	Follow Up Visits
7. Listing on a lung transplantation wait-list 8. Achievement of low risk status		
Optional Data Points	**Initial Visit**	**Follow Up Visits**
Clinical Frailty Scale	X	X
36-Item Short Form Health Survey (SF-36)	X	X
Virtual vs In-person visit	X	X
COVID-19 Vaccination Status	X	X
Participation in Pulmonary Rehabilitation	X	X
Diuretic, Calcium Channel Blocker medications	X	X

Document last updated: April 2025