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SEEKING A CURE
WIND-PSC
PROSPECTIVE SYNTHETIC COHORT

What is WIND-PSC?
An Introduction for PSC Patients and Caregivers

A PSC Partners-Sponsored Study

A Global Multi-Center Prospective Observational Cohort to Support Drug Development in Adult Patients with Primary Sclerosing Cholangitis (WIND-PSC)

What is WIND-PSC?

WIND-PSC is a PSC Partners-led and -sponsored initiative launched in 2024 that will make valuable patient data available to researchers and regulators. This will give them a better understanding of the lived PSC experience.

The WIND-PSC database will serve as a central location where PSC clinical data is securely collected with the intention that it be accessible in a secure way for research, drug development, and regulatory needs. Medical centers from around the world have been invited to contribute clinician-reported data, and study participants will contribute patient-reported data on symptom experiences.

Data will be securely stored in a central WIND-PSC database managed by a data coordinating center, Arbor Research. This will ultimately support and accelerate drug development for PSC by facilitating consistent, comprehensive, and monitored data collection.

Why is the natural history of a disease important?

Natural history refers to the usual course of a disease over time. Natural history studies are crucial to PSC research. When natural history is captured, patients are followed over time as their PSC progresses. With a patient’s consent, important data, such as treatments, complications, and more, can be stored securely in a *natural history registry*.

How is WIND-PSC different from a patient registry or a natural history registry?

The main differences between a *natural history registry (or database)* and a *patient registry* are the types of data, and how the data is captured. The PSC Partners Patient Registry is a *patient-reported registry*. A n*atural history registr*y includes systematic and regular collection of past or current data from medical records, imaging data, hospitalization records, and lab test results. The systematic capture of these health data over time is key to enabling the completion of *natural history studies*.

WIND-PSC has features of both a patient-reported registry and a natural history study. However, the aims of the WIND-PSC exceed what most patient-reported registries and natural history studies can achieve because the data is being collected with the following features:

- Study group is prospectively determined by the study protocol.
- Data is collected with the standards of a clinical trial for an experimental drug, without an experimental drug. What this means for a participant is that the study will collect any change in health, not only that believed to be related to PSC.
- Data is monitored and stored in documented and audited regulatory-compliant databases similar to interventional clinical trials so the data can be included in future regulatory submissions to support new drug filings.

What are some of the main objectives of WIND-PSC?

In rare disease drug development, typically data is collected from an enrolled, blinded control group receiving a placebo along the same timeline. However, the regulatory agencies have affirmed that under certain conditions, it is allowable to submit data on an “external control group”.

The first objective of WIND-PSC is to collect this data on participants that are not receiving an experimental drug, with the intent that it can be used for the purpose of an external control group (Sometimes this is referred to as a *prospective synthetic cohort*). WIND-PSC has been designed in such a way that the data will meet stringent regulatory collection and monitoring requirements, as well as patient confidentiality and data security.

A biomarker is a characteristic of the body that can be measured. For PSC, this might include blood tests or liver imaging. One reason clinical trials for PSC are challenging is because there is a lack of *noninvasive* biomarkers proven to correlate with PSC disease progression. WIND-PSC aims to address this by contributing to development of biomarkers which could be used to demonstrate effectiveness of an investigational drug for PSC.

The WIND-PSC study also collects various patient-reported measures of quality-of-life and symptom experiences, simultaneously with physician-reported clinical data. This will support the study over time of PSC symptoms and quality-of-life during the progression of the disease and will support the development of survey tools that could potentially be used to measure if a drug being studied in a clinical trial affects how a patient feels and functions.

Who is involved in WIND-PSC? How can I join?

WIND-PSC is the inaugural project of the PSC Partners International Collaborative Research Network (ICRNetwork). The success of WIND-PSC relies on support from the entire PSC community including patients, caregivers, clinicians, and researchers.
It is important to know that the goals of WIND-PSC can be accomplished by enrolling a few thousand dedicated PSC patients who are willing and able to follow up regularly with a dedicated research center.

WIND-PSC is being activated at up to 20 study sites in the United States, Canada, Germany, and Italy, with additional locations under consideration. You can follow study sites at the study site listings on clinicaltrials.gov at study NCT06297993. As of September 2024, enrollment is active at the following sites:

United States Sites:

- Florida: Schiff Center for Liver Diseases / University of Miami, Miami
- Massachusetts: Massachusetts General Hospital, Boston
- Virginia: Virginia Commonwealth University, Richmond

Canada Sites:

- Ontario: University Health Network,Toronto

Not all interested PSC patients will be able to join this initial cohort, due to factors like overall program budget and whether patients live near participating research centers.

However, we value every PSC patient's voice. Regardless of whether you are able to join the initial WIND-PSC cohort, we encourage you to join the PSC Partners Patient Registry, through which you can give priceless input to support the WIND-PSC initiative and other PSC research studies. Through the Registry, PSC Partners may connect you to ongoing natural history registries, research studies, and clinical trials for PSC.

Where can I learn more?

For more details, visit the WIND-PSC page of the PSC Partners website at go.pscpartners.org/WIND-PSC.