Lay Summary Template

Title 5711: National PAP Registry

Status: Recruiting

Study Summary (includes: disclaimer, background, about this study)

Please Note: The Rare Diseases Clinical Research Network will make every effort to enroll all the patients we can, but we cannot make any guarantees that we will be able to enroll everyone in a particular study who wants to participate.

Background (disease/disorder and general purpose of study)

The **PURPOSE** of this study is assemble a group of people with pulmonary alveolar proteinosis (PAP) large enough to accelerate research to improve our understanding, diagnosis, and treatment of PAP.

The **REASON** for doing the study is that medical progress for PAP is hampered by how infrequently it occurs. About 2000 – 3000 people in the United States have PAP and most doctors have either never seen anyone with PAP or only a few cases in their lifetime. Collecting medical information from many people with PAP in a registry will provide enough information to overcome this barrier to advancement.

Some **USEFUL FACTS** about PAP include:

- PAP is a *condition* that can occur in many different diseases – *PAP is not one specific disease*.
- No tests are available for routine use by doctors to diagnose diseases that cause PAP.
- New tests have been developed but, currently, can only be used in research studies.
- To make these new tests available for clinical use, they must first be evaluated in a research study.
- New treatments for some diseases causing PAP are being developed. However, it will be important to know what disease is causing PAP before any such treatment could be used.

Some **QUESTIONS** this study will address are:

1. How reliable is the new 'blood droplet' test for diagnosis of PAP?
2. How common are the various diseases that cause PAP?
3. What other diseases cause PAP?
4. Are there better biomarkers (potential diagnostic tests) for diseases that cause PAP?

About this Study (includes: Type of study (longitudinal, pilot, etc) length of study, number of participants, methods of examination and/or treatment)

This is a cross-sectional study of individuals with Pulmonary Alveolar Proteinosis (PAP) to create the National PAP Registry. A cross-sectional study involves collecting data from PAP patients at one specific time point. We plan to enroll 500 individuals over a 5 year period. Participants will be asked to answer questions about their health and provide a blood sample. We will test your blood and give you the results. With your permission, we will also give the results to your doctor. You will not receive any drug or treatment. The testing is free of charge because the National Institutes of Health (NIH) is providing money for this study. We will look at your medical records from your local doctor's office so that we can collect information about your medical history. Educational information may be included on the Rare Diseases Clinical Research Network Rare Lung Diseases Consortium website and the PAP Foundation website. Information may include, but is not limited to, updates on PAP research, updates on upcoming studies, progress of current trials, and PAP related events, such as education days and patient/doctor meetings. Further, PAP patients may choose to have their contact information shared with the PAP Foundation. The PAP Foundation may provide patients and their families with newsletters and updates about PAP. These newsletters and updates may include, but are not limited to, updates on PAP research, updates on upcoming

trials, progress of current trials, and PAP related events, such as education days and patient/doctor meetings.

Targeted Enrollment (inclusion/exclusion)

You are eligible to participate, if:

- You are interested in participating in the study and able to provide written informed consent.
- You have been diagnosed with PAP by a doctor. The diagnosis could have be made by several methods including EITHER a chest x-ray, chest CT scan, lung biopsy, bronchoscopic lung lavage (washing), or genetic testing.

You are not eligible to participate if:

- You do not have a diagnosis of PAP

How to participate: (participating institutions with confirmed IRB approval)

In order to participate in a study, you must personally contact the study coordinator of any of the participating institutions by phone or by e-mail. Please use the information below to inquire about participation.

Institution Name: Cincinnati Children's Hospital Medical Center

Contact Name:	Brenna Carey
Phone:	(513) 636-8916
Toll Free Phone Number:	(844) 843-8772 (Administrative assistant, Stacy Shirley)
E-mail:	Brenna.Carey@cchmc.org

**Participating clinical sites will be added as each site is activated for a specific protocol*