A Research Study for Adults with Autoimmune PAP

What is the purpose of this study?

This is a research study designed to learn if **Pioglitazone** is a safe intervention for the lung disease autoimmune pulmonary alveolar proteinosis (aPAP)

Who will be included in this study?

Non-smoking healthy adults 18 to 80 years old with aPAP may be eligible to participate. The study doctor will determine if you qualify for the study by asking you questions about your diagnosis with aPAP, your medical history, doing a full physical examination, performing clinical laboratory tests, and observing your health for two months before you receive the study drug.

What is involved?

Participation in the study involves an initial observation period of about 3 ½ months if you are not already being seen by Dr. Trapnell or his associate for autoimmune PAP or are not enrolled in the National PAP Registry. After the observation period, you will be asked to complete three onsite visits at Cincinnati Children's Hospital Medical and three-five interim telephone calls over a period of up to 12 months.

All eligible participants will receive a quality of life questionnaire, have blood tests, pulmonary function tests, bronchoscopy, a CT scan, and two MRI's. Also included are the six-minute walk test, and an exercise treadmill test. Participants will also take a pill once daily for 9 months.

All of the other tests and procedures that will be performed include, but are not limited to:

- Health and medication history
- Physical exam, including height and weight, temperature, pulse, and blood pressure measurements
- Electrocardiogram to record the electrical activity of the heart
- Pulmonary function tests
- Urine tests
- Pregnancy test (for females of child bearing potential)
- Review of drug accountability and blood sugar record

A detailed list of tests and procedures will be provided to those interested in knowing more about this study.

What are the benefits?

Potential benefits may include benefit from the physical exams, pulmonary function tests, and other study procedures. Each participant will receive the study intervention which is pioglitazone taken orally as a pill. Pioglitazone may not stabilize or improve lung function in participants with autoimmune PAP. The information learned from this research study may benefit other patients with autoimmune PAP in the future.

Will I get all the facts about the study?

Those interested in participating will be given a consent form that explains all of the details of the study. The form covers all of the procedures, the potential risks and benefits, the pay, who to contact with questions or concerns and more. A member of the study staff will review the

consent form with participants to ensure all questions are answered. Study procedures will not begin until the participant has signed this consent form.

What are the risks and discomforts of the study?
There are some potential risks associated with the study drug and with certain study procedures. A complete list of risks and benefits will be provided to those interested in learning more about the study.

What is the pay?
Participants will be paid for the time and travel expenses related to participating in this study. Participants will be compensated for their time to a maximum of $850 ($100/on-site clinic visit; $50 for phone visits, drug diary and blood glucose monitor kit)

Who should I contact for more information?

For participants interested in enrolling at Cincinnati Children's, please contact:

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