A National Registry For Pulmonary Alveolar Proteinosis

Status: RECRUITING

Eligibility Criteria

Sex: ALL
Age:
Healthy Volunteers: This study is NOT accepting healthy volunteers
Inclusion Criteria for Part A and Part B:

- Written informed consent and assent, if applicable

Inclusion Criteria for Part A (Cross Sectional Study of PAP Syndrome)

- History of chest computed tomogram or chest radiograph findings compatible with PAP
- History of diagnosis of PAP made by at least one of the following methods:
    - Positive (Abnormal) serum GMAb test -OR-
    - Lung biopsy clearly documenting the presence of PAP of any type or degree -OR-
    - Bronchoalveolar lavage cytology compatible with PAP -OR-
    - Recessive or compound mutations in genes known to cause PAP, i.e. GM-CSF receptor α or β chain, GM-CSF, surfactant protein B or C or ABCA3, ABCG1, ABCA1, TTF1

Inclusion Criteria For Part B (Longitudinal & PRO Survey Study of autoimmune PAP Patients)

- Diagnosis of autoimmune PAP as indicated by:
    - Positive (Abnormal) Serum GMAb Test -AND-
    - History of chest CT or x-rays findings compatible with PAP -OR-
    - Lung biopsy clearly documenting the presence of PAP of any type or degree -OR-
    - Bronchoalveolar lavage cytology compatible with PAP

Exclusion Criteria or Part A and Part B:

- Individuals who have a serious medical illness that, in the opinion of the investigator, is likely to interfere with completion of the study will be excluded.

For Part A (Cross-sectional Study of PAP Syndrome)

- Individuals that do not have a diagnosis of PAP

For Part B (Longitudinal & PRO Survey Study of autoimmune PAP Patients)

- Individuals that do not have a diagnosis of autoimmune PAP

Conditions & Interventions

Conditions:
Pulmonary Alveolar Proteinosis
Keywords:
Pulmonary Surfactant, Rare Lung Disease, Registry

More Information

Description:
The major goal of Part A of this study is to establish a National PAP Registry to help make reliable new research tests available to doctors to improve the diagnosis of PAP, increase awareness and knowledge of PAP, and give patients a 'seat at the table' in planning and conducting PAP research including the clinical testing of several new potential therapies. The major goal of Part B of this study is to define the natural history of autoimmune PAP (aPAP), develop a disease severity score that reflects how aPAP patients feel and function, and to develop and test novel tools to measure the severity of aPAP lung disease. Funding Source - FDA OOPD

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