

Diagnosis and Treatment Experiences of aPAP Patients: Patient Advisory Board Meeting Findings

Authors: Michele Rhee, Savara Inc.; Michelle Slattery, Slattery Health Communications

Purpose:

Understanding patient experiences, needs, perspectives, and priorities is the cornerstone of patient-focused drug development.

Background:

Autoimmune pulmonary alveolar proteinosis (aPAP) is a rare lung disease in which surfactant builds up in the alveoli in the lungs, making it hard to breathe. Some diagnostic tests for aPAP may be invasive and may require hospitalization (e.g. lung biopsy), but

aPAP can be diagnosed with a simple blood test. There are currently no drugs approved in the U.S. to treat aPAP. Whole lung lavage (WLL) is an invasive procedure used to manage symptoms and clear surfactant. WLL is performed under general anesthesia and involves filling one lung at a time with saline and

then draining it to remove surfactant. Savara, a clinical-stage biopharmaceutical company focused on developing therapies for people with rare respiratory diseases, sought to learn more about the diagnosis and treatment experiences of people living with aPAP.

Methods:

Savara engaged Slattery Health Communications to convene an advisory board meeting with seven adults in the U.S. living with aPAP. Participants ranged in age from 24-62 years old, and most had been living with an aPAP diagnosis for six years or longer. Two virtual group meetings were held in December 2023 with five participants, and

one-on-one interviews were conducted with two additional participants who could not join the group. Topics included diagnosis and treatment experiences. Responses were gathered from group discussions, polling, and post-meeting follow-up.

EARLY SYMPTOMS

Type & Severity VARY

A Cycle of MISDIAGNOSIS

Despite Availability of Blood Tests

Newly Diagnosed

PATIENTS LACK ADEQUATE RESOURCES

LIVING WITH aPAP

Severely Limits Work, Activity, & Quality of Life

PATIENTS DESERVE BETTER TREATMENT

NOVEL, NON-INVASIVE, FDA-APPROVED OPTIONS TO IMPROVE LUNG FUNCTION & DAILY LIVING



WENT TO DOCTOR FOR...

- SEVERE / RESPIRATORY:
 - ▶ COUGH / SHORTNESS OF BREATH, PAIN IN SIDE
- MODERATE / NON-RESPIRATORY:
 - ▶ GASTRITIS, HEADACHE, FEVER, FATIGUE

"MY COUGH WAS SO SEVERE I COULD BARELY WALK"

COMMON MISDIAGNOSES:

- PNEUMONIA • AIDS • ALLERGIES • ANXIETY/PANIC ATTACKS •
- LEGIONNAIRE'S DISEASE • PULMONARY EMBOLISM • TUBERCULOSIS •

5 OF 7 DIAGNOSED BY LUNG BIOPSY

BUT ALL COULD HAVE BEEN DIAGNOSED WITH A BLOOD TEST

HCP AWARENESS IS LACKING

- ▶ 85% PARTICIPANTS SAW ≥ 2 PULMONOLOGISTS BEFORE DIAGNOSIS
- ▶ PATIENTS OFTEN MUST EDUCATE THEIR HCPs ABOUT TREATMENT OPTIONS

85%

COULD NOT QUICKLY FIND THE RESOURCES THAT THEY NEEDED

71%

RECEIVED NO aPAP RESOURCES FROM THEIR DOCTOR

71%

DID NOT CONNECT WITH OTHERS LIVING WITH aPAP

KEY NEEDS:

- ▶ DISEASE EDUCATION
- ▶ TREATMENT DECISION GUIDANCE

WHOLE LUNG LAVAGE:

TEMPORARY RELIEF AT BRUTAL COST

FINANCIAL BURDEN

INVASIVE & PAINFUL

PATIENTS DELAY TREATMENT

RISK OF SERIOUS COMPLICATION:

- INFECTION
- PANICK ATTACKS
- SEVERE THROAT SWELLING
- TORN/PUNCTURED LUNG
- SEVERE STEROID REACTION
- NEAR-DEATH EXPERIENCES

OFF-LABEL MEDICATION

IS USED TO AVOID WHOLE LUNG LAVAGE, BUT...

LEADS TO CHALLENGES:

- ▶ ACCESS ISSUES AT SPECIALTY PHARMACIES
- ▶ INSURANCE REQUIRING QUARTERLY REAUTHORIZATION, OR 28 DOSES/MONTH MAXIMUM
- ▶ LACK OF DOSING & SAFETY INFORMATION
- ▶ LACK OF DRUG ADMINISTRATION INFORMATION

Questions For Discussion

How can we all work together to raise awareness of aPAP and the aPAP ClearPath™ blood test?

As more rare diseases are identified how can we reach the right HCPs and patients at the right time with the right information?

How can we better support the mental health needs of people living with aPAP?

What other questions should we be asking patients, HCPs, and the broader aPAP community in future advisory board meetings?

Savara deeply appreciates and would like to thank all the patients who participated in the meetings and shared their personal experiences.