Healthcare Burden of Pulmonary Alveolar Proteinosis (PAP)

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OBJECTIVE

To compare the clinical and economic burden between a sample of diagnosed PAP patients and non-PAP matched controls

CONCLUSIONS

PAP patients have a large incremental burden of disease vs. non-PAP patients

Patients with PAP experience higher healthcare resource utilization, pharmaceutical and non-pharmaceutical costs, comorbidities, procedures, and therapy use, highlighting a significant unmet need in this rare disease patient population

This study includes the largest sample of diagnosed PAP patients and matched controls to be analyzed to date

DISCLOSURES

Savara Inc. participated in the study design and interpretation of data, and reviewed and approved this poster for submission. All authors participated in the development, review, and approval of this poster. Savara Inc. funded this study.

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Background

- PAP is a rare lung syndrome characterized by the accumulation of surfactant in the alveoli leading to respiratory distress, hypoxemia, and increased infection risk[1,2]
- The path to a definitive diagnosis and management of PAP can be challenging due to nonspecific clinical symptoms and findings, limited access to testing, and the lack of approved therapies[2-4]
- The only available treatment is whole-lung lavage, an invasive procedure that has several known complications[2]
- Previous research demonstrated higher comorbidities, increased healthcare utilization, and elevated costs in PAP patients compared with controls[3]
- This claims database study was conducted to validate these findings within a larger U.S. cohort of PAP patients and matched non-PAP control patients

Methods

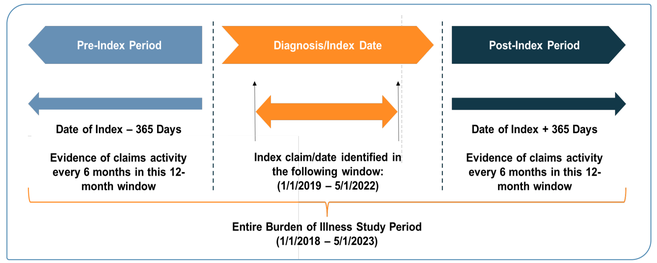
Data Source

- Longitudinal claims database (IPM.ai) that includes ~300 million patients in the U.S.

Study Design

- A retrospective cohort analysis using claims data from January 1, 2018 through May 1, 2023
- Patients with PAP were included if they had:
  1. >1 claim with a diagnosis code for PAP (ICD-9-CM code: 516.0 or ICD-10-CM code: J84.01) between January 1, 2019, and May 1, 2022, and
  2. No claims for other rare respiratory diseases after the last PAP diagnosis code during the study period
- A non-PAP control cohort was created (1:4 case:controls) matched for age, gender, and geographic location
- Diagnosed PAP patients must have had evidence of continual claims activity (≥1 claim in two 6-month windows) in both the 12 months prior to (baseline period) and after (follow-up period) the index date **(Figure 1**)

Figure 1. Patient Inclusion Criteria



Study Measures

- At baseline, demographic and clinical characteristics, payers, and Charlson Comorbidity Index (CCI) were measured
- Comorbidities, procedures, medication use, healthcare resource utilization, costs, and charges were measured during the post-index period

Results

Baseline Demographic and Clinical Characteristics (Table 1)

- A sample of 2,312 PAP patients and 9,247 control patients was identified. The cohorts were well balanced in age and gender
- Mean CCI was significantly higher for PAP patients compared with the control cohort
- Significantly higher rates of COPD, diabetes, renal disease, congestive heart failure, non-metastatic malignancies, and peripheral vascular disease were observed in PAP patients compared with controls

Table 1. Baseline Characteristics

	PAP (N = 2,312)		Matched Controls (N = 9,247)		P-value
	n	%	n	%	
Age					0.997
Mean (SD)	61	17.75	61	17.75	
Gender					0.997
Male	740	32	2,960	32	
Female	1,572	68	6,287	68	
Race & Ethnicity					
White (Non-Hispanic)	1,461	63	5,839	63	
White (Hispanic)	211	9	743	8	
Black or African American	139	6	435	5	
Asian	42	2	195	2	
American Indian or Alaska Native	0	0	0	0	
Native Hawaiian or Other Pacific Islander	5	0	22	0	
Unknown Race	454	20	2,013	22	
Patient Payer Coverage					
Commercial	759	33	2,636	29	<0.001
FFS Medicaid	110	5	280	3	<0.001
Managed Medicaid	348	15	587	6	<0.001
Medicare	831	36	2,114	23	<0.001
Other Payer	46	2	295	3	<0.005
Unknown Payer	218	9	3,335	36	<0.001

Comorbidities, Procedures, and Medication Use (Table 2)

- During the post-index period, diagnosis rates for other respiratory conditions, hypertension, hyperlipidemia, psychiatric conditions, obesity, and weakness or fatigue were all significantly higher for PAP patients compared with controls
- Procedure rates and medications use were significantly higher among PAP patients during the post-index period

Healthcare Resource Utilization and Costs (Table 3)

- Outpatient visits, emergency room visits, inpatient visits, and inpatient hospital length of stay were significantly higher among PAP vs. control patients
- Mean plan-paid and out-of-pocket pharmacy costs during the 1-year follow-up period were significantly higher for PAP patients compared with control patients
- Average charges during the post-index period were significantly higher for the PAP cohort compared with the control cohort

Table 2. Comorbidities, Procedures, and Medication Use

	PAP (N = 2,312)		Matched Controls (N = 9,247)		P-value
	n	%	n	%	
Comorbidities					
Other Respiratory Conditions	1,415	61	1,724	19	<0.001
Hypertension	1,132	49	2,075	22	<0.001
Hyperlipidemia	828	36	1,561	17	<0.001
Psychiatric Conditions	659	29	1,039	11	<0.001
Obesity	430	19	614	7	<0.001
Weakness or Fatigue	383	17	603	7	<0.001
Procedures					
Imaging of Chest	932	40	932	10	<0.001
Oxygen Treatment	339	15	114	1	<0.001
Pulmonary Function Tests	267	12	69	1	<0.001
COVID-19 Testing	279	12	401	4	<0.001
Spirometry	196	8	91	1	<0.001
Bronchoscopy	178	8	11	0	<0.001
Mobility Assistance	144	6	177	2	<0.001
Bronchoalveolar Lavage	110	5	5	0	<0.001
Total Lung Lavage	53	2	0	0	<0.001
Thoracoscopy	18	1	3	0	<0.001
Medications					
Antibiotics	457	20	767	8	<0.001
Inhaled Beta Agonists	341	15	399	4	<0.001
Inhaled Anticholinergics	155	7	118	1	<0.001
Inhaled Bronchial Combination Therapies	139	6	130	1	<0.001
Inhaled Steroids	62	3	52	1	<0.001
Sargramostim	26	1	0	0	<0.001
Other Respiratory Therapies	9	0	3	0	<0.001
Respiratory Biologics	4	0	1	0	<0.001
Rituximab	6	0	6	0	<0.001

Table 3. Healthcare Resource Utilization and Costs

	PAP Cohort (N = 2,312)		Matched Controls (N = 9,247)		P-value
	Mean	SD	Mean	SD	
Outpatient Visits					
Patients, n (%)	2,002 (87%)		5,142 (56%)		
Number of visits	10.7	10.9	4.0	7	<0.001
ER Visits					
Patients, n (%)	802 (35%)		1,260 (14%)		
Number of visits	1.0	2.3	0.29	1	<0.001
Inpatient Visits					
Patients, n (%)	467 (20%)		424 (5%)		
Number of visits	2.3	7.8	0.38	2.5	<0.001
Length of Stay* (Days)	2.8	7.6	0.56	2.9	<0.001
Pharmacy Costs					
Plan-Paid	$3,685	$17,532	$839	$6,457	<0.001
Patient Out-of-Pocket	$346	$1,276	$153	$836	<0.001
Non-Pharmacy Charges	$71,673	$226,118	$14,656	$74,791	<0.001

ER, emergency room; *Among patients with inpatient hospitalizations