Real-world treatment patterns in adults with pemphigus foliaceus in the United States

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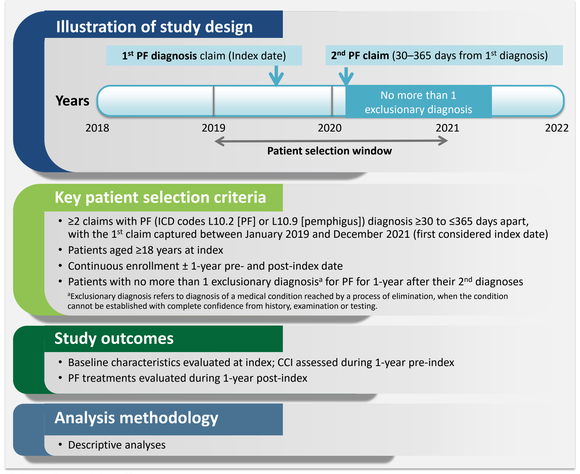
Introduction and Purpose

- Pemphigus foliaceus (PF) is an autoimmune blistering disease of the skin characterized by multiple, pruritic, scaly, cutaneous erosions and flaky patches primarily appearing on the face, scalp, trunk and extremities.[1,2]
- Although rare worldwide, with <50,000 individuals in the United States (US)[2] diagnosed with PF, it was lethal in almost 60% of patients prior to the implementation of corticosteroid (CS) therapy.[3]
- Current first-line treatment options for PF include CS, immunosuppressants (IS), and rituximab. Other treatment options, used alone or in combination, include broad-spectrum antibiotics and immunoglobulins.[4,5]
- Better understanding of real-world treatment patterns in PF is critical to identify any subpopulations with pronounced burden or unmet need. Therefore, this study aimed to (1) descriptively analyze the characteristics of patients with PF, and (2) evaluate their PF treatment utilization, using a US claims database.

Methods

- The Komodo Health closed claims database (January 2015-December 2022), containing complete medical and prescription claims information from >150 payers across all geographic regions of the US, was utilized for the analysis. The details of the study design are provided in **Figure 1**.

Figure 1. Study design



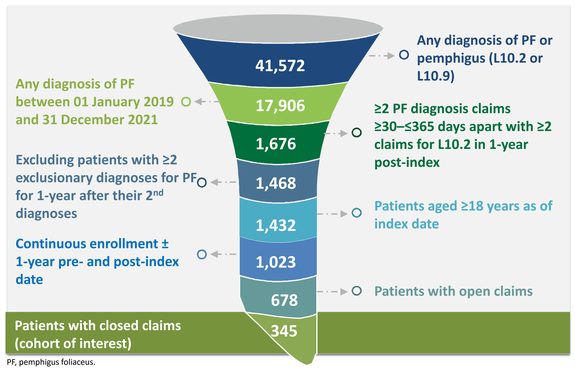
CCI, Charlson Comorbidity Index; PF, pemphigus foliaceus.

Results

Study population

- Overall, 345 patients met the inclusion criteria and were included in the analysis (**Figure 2**).

Figure 2. Patient selection



PF, pemphigus foliaceus.

Results

Baseline patient demographics and characteristics

- Mean (SD) age of patients was 57.0 (16.0) years (**Table 1**).
- PF was predominant in women (56.5%) in general, and was more common in the 41 to 65 years age group across males and females.
- The mean (SD) Charlson Comorbidity Index (CCI) was 1.2 (2.0).
- The most commonly observed comorbidities were hypertension (36.5%), atopic dermatitis (33.6%), and obesity (22.0%).

Table 1. Baseline patient demographics and characteristics

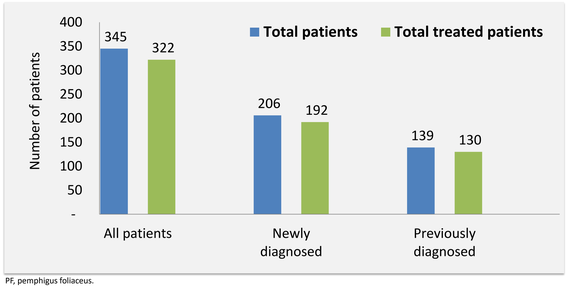
	N=345
Age, years, mean (SD)	57 (16.0)
Male, n (%)	150 (43.5)
Aged 18–40 years	21 (6.1)
Aged 41–65 years	81 (23.5)
Aged 65+ years	48 (13.9)
Female, n (%)	195 (56.5)
Aged 18–40 years	34 (9.9)
Aged 41–65 years	111 (32.2)
Aged 65+ years	50 (14.5)
Race, n (%)	
Caucasian	141 (40.9)
African American	47 (13.6)
Asian	16 (4.6)
Other/unknown	141 (40.9)
Insurance, n (%)	
Commercial	219 (63.5)
Self insured	107 (31.0)
Medicare/Medicare advantage	99 (28.7)
Medicaid	90 (26.1)
Dual eligible/other/unknown	34 (9.8)
Charlson Comorbidity Index, mean (SD)	1.2 (2.0)
0, n (%)	186 (53.9)
1–2, n (%)	105 (30.4)
3–4, n (%)	37 (10.7)
≥5, n (%)	17 (4.9)
Comorbidities, n (%)	
Hypertension	126 (36.5)
Atopic dermatitis	116 (33.6)
Obesity	76 (22.0)
Diabetes without chronic complication	61 (17.7)
CPD	50 (14.5)
Anxiety	32 (9.3)
Depression	32 (9.3)
Diabetes with chronic complication	26 (7.5)

CPD, chronic pulmonary disease; SD, standard deviation.

Overall PF treatment utilization in 1-year post-index

- Almost all patients identified with PF (n=322; 93.3%) were treated in the 1-year post-index.
- Of 345 patients identified, 206 were newly diagnosed with PF and 139 had previous diagnosis of PF.
- The proportion of patients treated for PF during the 1-year post-index were similar across newly and previously diagnosed patients (**Figure 3**).

Figure 3. Number of treated patients with PF

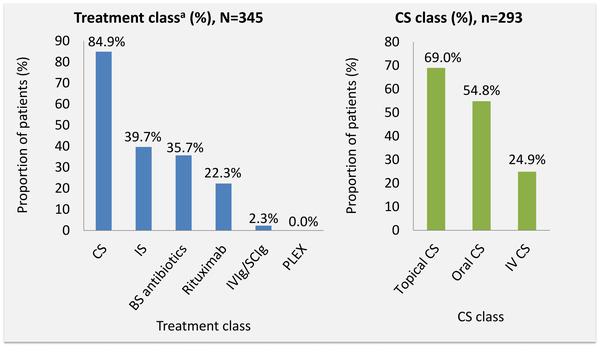


PF, pemphigus foliaceus.

Overall treatment patterns during 1-year post-index

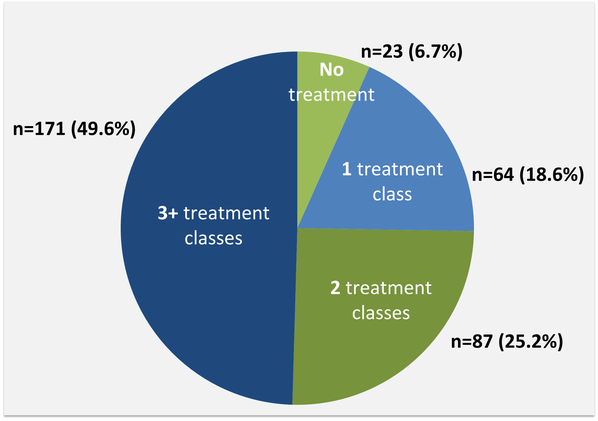
- The most common PF treatment utilized 1-year post-index was corticosteroids (CS; n=293; 84.9%), followed by IS, broad-spectrum antibiotics, and rituximab (**Figure 4**).
- Among 293 patients who used CS at least once, the majority were treated with topical CS (69.0%), followed by oral CS (54.8%; **Figure 4**).
- Almost 75.0% of patients required combination therapies for PF during the 1-year post-index period, with 49.6% using 3 or more treatment classes (**Figure 5**).

Figure 4. Proportion of patients using PF treatment at least once during 1-year post-index



Oral CS: Prednisolone, Prednisone. Topical CS: Triamcinolone, Flucocinonide, Clobestal, Betamethasone, Hydrocortisone, Dexamethasone. IS: Methotrexate, Azathioprine, Mycophenolate. BS antibiotics: Doxycycline, Dapsone; IV CS: Prednisone, Prednisolone, Dexamethasone, Methylprednisolone.
[a]Percentages have been calculated against total patients in cohort.
BS, broad-spectrum; CS, corticosteroids; IS, immunosuppressants; IV, intravenous; IVIg, intravenous immunoglobulin; PLEX, plasma exchange; SCIg, subcutaneous immunoglobulin.

Figure 5. Proportion of patients with PF by number of treatment classes exposed to in 1-year post-index (N=345)



Oral CS utilization during 1-year post-index

- Among the 189 patients who utilized oral CS at least once during 1-year post-index, more than half had regular usage of oral CS (n=128/189; 67.7%, >20 mg/day on average for 14 consecutive days, **Table 2**).

Table 2. Oral CS usage during 1-year post-index

	N=345
Patients prescribed oral CS, n (%)	189 (54.8)
Average time on oral CS treatment, days	130.2
Patients with regular oral CS usage[a,b,c], n (%)	128 (67.7)
Dosage breakdown over oral CS usage period[a,c], n (%)	
<5 mg	7 (3.7)
≥5–10 mg	14 (7.4)
≥10–15 mg	29 (15.3)
≥15–40 mg	101 (53.4)
≥40 mg	36 (19.0)
N/A[d]	2 (1.1)

[a]All data points are reported among patients who have been prescribed at least one dose of oral CS.
[b]Regular oral CS usage was defined as ≥20 mg daily Prednisone equivalent for 14 consecutive days at any point.
[c]Percentages were calculated against all patients who were prescribed oral CS (n=189).
[d]Two patients did not have any claims for oral CS in the pharmacy table. These patients were not considered in the Oral CS dosing analysis.
CS, corticosteroids; mg, milligram; N/A, not available.

Rituximab utilization during 1-year post-index

- Nearly half of patients treated with rituximab in the 1-year post-index had their 1st instance of rituximab treatment post-index in combination with oral CS (n=34/77; 44.2%; **Table 3**).
- Patients using rituximab concomitantly with oral CS commonly used >20 mg/day of oral CS, with approximately two-thirds using >20 mg/day as their initial dose (64.7%).

Table 3. First instance of rituximab usage during 1-year post-index

	N=345
Patients prescribed rituximab[a], n (%)	77 (22.3)
Monotherapy[b]	6 (7.8)
Combination[b]	
Rituximab+oral CS[b]	34 (44.2)
0–10 mg/day[c]	4 (11.8)
10–20 mg/day[c]	6 (17.6)
>20 mg/day[c]	22 (64.7)
Unknown[c]	2 (5.9)
Rituximab+IV CS[b]	28 (36.4)
Rituximab+other[b]	8 (10.4)

[a]Patients on rituximab may be underrepresented due the dosing schedule for PF and the limited follow-up (1-year post-index) in this study. Studies with longer follow-up are needed to confirm these results.
[b]Percentages shown were calculated against total patients prescribed rituximab (n=77). 1.3% of patients on rituximab (n=1) had too short of exposure to be considered a complete line of therapy and were excluded from subsequent subgroup analyses.
[c]Percentages shown were calculated against total patients using rituximab + oral CS (n=34).
CS, corticosteroids; IV, intravenous.

Conclusions

- **Among 345 patients with PF, CS were the most frequently utilized treatments during 1-year post-index.**
- **Most patients used combination treatments as well as regular oral CS, suggesting potential high treatment burden.**
- **Further research should assess the impact of these treatment patterns on clinical, humanistic and economic burden in PF to identify any areas of potential unmet need.**

Limitations

- As the study was based on one US claims database, the results may not be representative of the full US or global landscape of patients with PF.
- Being a retrospective study, parameters such as disease activity and effectiveness could not be analyzed.

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