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

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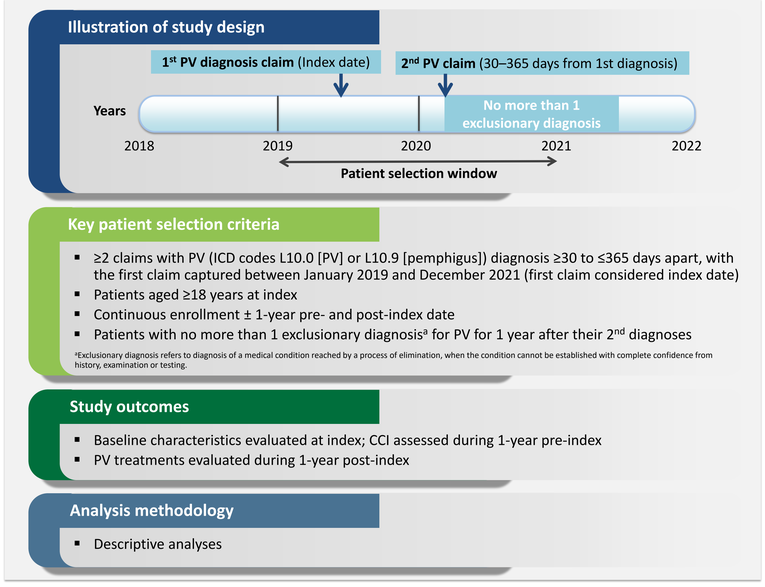
Introduction and Purpose

- Pemphigus vulgaris (PV) is a rare, chronic autoimmune disease affecting epithelial tissues of oral mucosa, skin, conjunctiva larynges, esophagus, genital and nasal cavity.[1,2]
- The overall crude prevalence of pemphigus in the United States (US) was 0.006% in 2019, with slightly increased prevalence in females (0.007%).[3] Apart from the disease itself, substantial impact of PV on psychological, social, and financial aspects of patients' lives has been reported.[4]
- Current clinical guidelines (2020) recommend initial treatment with rituximab (anti-CD20 antibody) in combination with corticosteroids (CS) for patients with moderate-to-severe PV.[5] However, evidence is limited on real-world treatment patterns, including steroid dosing, among patients with PV based on real-world data.
- Based on current evidence, the objective of this research was focused on (1) descriptive analysis of characteristics of patients living with PV, and (2) evaluating PV 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



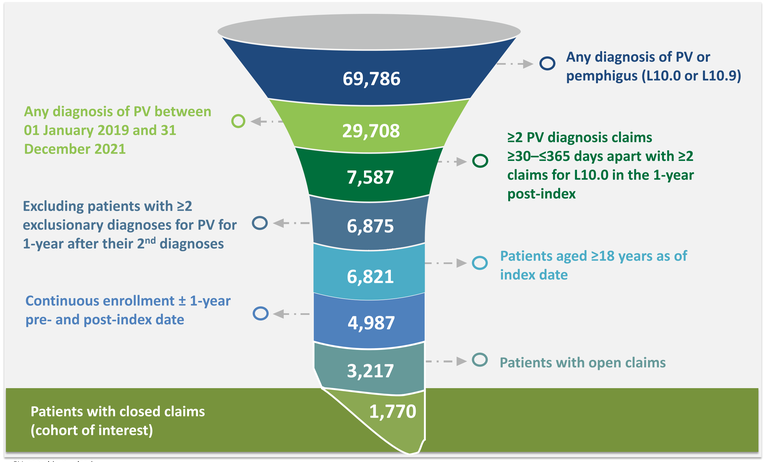
CCI, Charlson Comorbidity Index; PV, pemphigus vulgaris.

Results

Study population

- Overall, 1,770 patients met the inclusion criteria and were included in the analysis (**Figure 2**).

Figure 2. Patient selection



PV, pemphigus vulgaris.

Results

Baseline patient demographics and characteristics

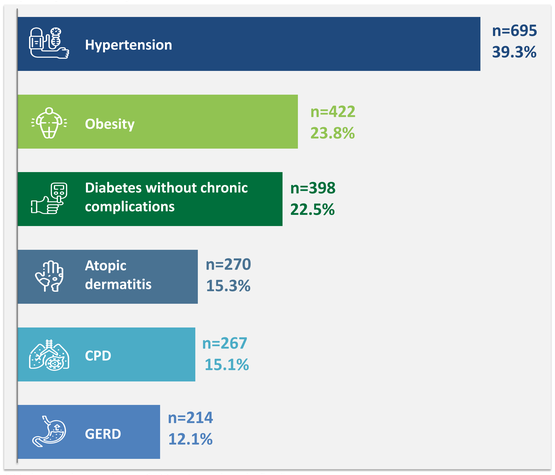
- Mean (standard deviation [SD]) age of patients was 57.0 (15.0) years (**Table 1**).
- PV was predominant in women (60.8%) in general, and was more common in the 41 to 65 years age group across males and females.
- The mean (SD) Charlson Comorbidity Index (CCI) at baseline was 1.3 (2.1).
- The most common comorbidities observed were hypertension (39.3%), obesity (23.8%), and diabetes without chronic complications (22.5%; **Figure 3, Table 2**).

Table 1. Baseline patient demographics and characteristics

	N=1,770
Age, years, mean (SD)	57.0 (15.0)
Male, n (%)	693 (39.2)
Aged 18–40 years	105 (5.9)
Aged 41–65 years	410 (23.2)
Aged 65+ years	178 (10.1)
Female, n (%)	1,077 (60.8)
Aged 18–40 years	154 (8.7)
Aged 41–65 years	618 (34.9)
Aged 65+ years	305 (17.2)
Race, n (%)	
Caucasian	781 (44.1)
African American	161 (9.1)
Asian	133 (7.5)
Other/Unknown	695 (39.3)
Health insurance, n (%)	
Commercial	1,157 (65.4)
Medicare/Medicare advantage	564 (31.9)
Self-insured	506 (28.6)
Medicaid	484 (27.3)
Dual eligible/other/unknown	279 (15.8)
Charlson Comorbidity Index, mean (SD)	1.3 (2.1)
0, n (%)	897 (50.7)
1–2, n (%)	570 (32.2)
3–4, n (%)	168 (9.5)
≥5, n (%)	135 (7.6)

SD, standard deviation.

Figure 3. Most common comorbidities (observed in ≥10% of patients in 1-year pre-index)



CPD, chronic pulmonary disease; GERD, gastroesophageal reflux disease.

Table 2. Other comorbidities (observed in <10% of patients in 1-year pre-index)

	N=1,770
Urinary tract infection, n (%)	175 (9.9)
Diabetes with chronic complication, n (%)	147 (8.3)
Depression, n (%)	145 (8.2)
Anxiety, n (%)	137 (7.7)
Osteoporosis, n (%)	128 (7.2)
Peripheral vascular disease, n (%)	124 (7.0)
Any malignancy, including lymphoma and leukemia, except malignant neoplasm of skin, n (%)	118 (6.7)
Mild liver disease, n (%)	104 (5.9)
Cerebrovascular disease, n (%)	98 (5.5)
Renal disease, n (%)	94 (5.3)
Congestive heart failure, n (%)	93 (5.3)

Treatment patterns during 1-year post-index

- Majority of the patients (n=1,521, 85.9%) were treated for PV during the 1-year post-index period, while 14.1% were untreated for PV (**Figure 4**).
- Overall, more than two-thirds (67.8%) of patients required combination therapies during the 1-year post-index, with almost half (44.8%) exposed to ≥3 treatment classes (**Figure 4**).
- Corticosteroids (CS) were the most common PV treatment utilized at least once during the 1-year post-index (n=1,397/1,770, 78.9%; **Table 3**).
- Among 1,088 patients who utilized oral CS, the majority had regular oral CS usage (>20 mg/day for 14 consecutive days) (n=660/1,088, 60.7%; **Table 4**).
- Over half of patients treated with rituximab in the 1-year post-index had their 1st instance of rituximab treatment in combination with oral CS (n=274/503, 54.5%), with the majority using >20 mg/day (n=152/274, 55.5%; **Table 5**).

Figure 4. Proportion of patients with PV by number of treatment classes exposed to in 1-year post-index (N=1,770)

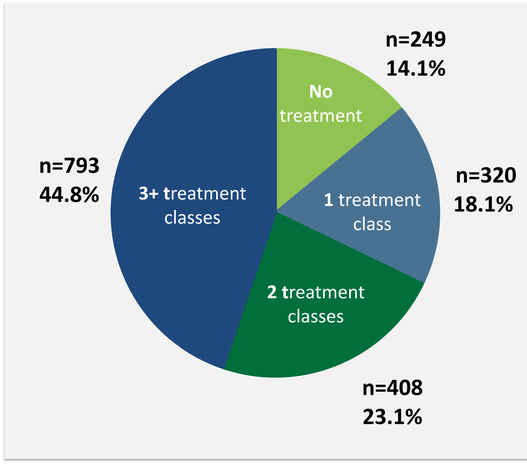


Table 3. Number and proportion of patients who received each PV treatment at least once in 1-year post-index

	N=1,770
Total treated patients[a], n (%)	1,521 (85.9)
Treatment class[a], n (%)	
Corticosteroids	1,397 (78.9)
Oral	1,088 (61.5)
Topical	804 (45.4)
IV	582 (32.9)
IS	691 (39.0)
Rituximab	503 (28.4)
BS antibiotics	414 (23.4)
IVIg/SCIg	90 (5.1)

Analysis was performed using Komodo data available from January 2015-December 2022; Oral and IV CS: Bethamethasone, Budesonide, Cortisone, Hydrocortisone, Dexamethasone, Methylprednisolone, Prednisolone, Prednisone; Topical CS: Triamcinolone, Flucocinonide, Clobestal, Dexamethasone; Immunosuppressants: Tacrolimus, Cyclosporine, Cyclophosphamide, Methotrexate, Azathioprine, Mycophenolate, Mycophenolic acids; BS antibiotics: Doxycycline, Dapsone Treatment distribution has been sorted based on newly diagnosed patients.
[a]% have been calculated against total patients in cohort.
BS, broad spectrum; CS, corticosteroids; IS, immunosuppressant; IV, intravenous; IVIg, intravenous immunoglobulin, SCIg, subcutaneous immunoglobulin.

Table 4. Oral CS usage in the 1-year post-index

	N=1,770
Patients prescribed Oral CS, n (%)	1,088 (61.5)
Average time on Oral CS treatment, days	141.2
Patients with regular Oral CS usage[a,b,c], n (%)	660 (60.7)
Dosage breakdown over Oral CS usage period[a,c], n (%)	
<5 mg	64 (5.9)
≥5–10 mg	134 (12.3)
≥10–15 mg	137 (12.6)
≥15–40 mg	517 (47.5)
≥40 mg	229 (21)
N/A[d]	7 (0.6)

[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=1,088).
[d]Seven patients did not have any claims for oral CS in the pharmacy table. These patients were not considered in the OS dosing analysis.
CS, corticosteroids; N/A, not available; mg, milligram.

Table 5. First instance of rituximab usage in the 1-year post-index

	N=1,770
Patients prescribed rituximab[a], n (%)	503 (28.4)
Monotherapy[b]	59 (11.7)
Combination[b]	
Rituximab+Oral CS[b]	274 (54.5)
0–10 mg/day[c]	51 (18.6)
10–20 mg/day[c]	51 (18.6)
>20 mg/day[c]	152 (55.5)
Unknown[c]	20 (7.3)
Rituximab+IV CS[b]	126 (25.0)
Rituximab+other[b]	41 (8.2)

[a]Patients on rituximab may be underrepresented due the dosing schedule for PV and the limited follow-up (1-year post-index) in this study. Studies with longer follow-up are needed to confirm results.
[b]Percentages shown were calculated against total patients prescribed rituximab (n=503). 0.6% of patients on rituximab (n=3) 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=274).
CS, corticosteroids; IV, intravenous.

Conclusions

- **Among 1,770 patients with PV identified from a US claims database, corticosteroids were the most common treatment utilized in the 1-year post-index.**
- **As a substantial proportion of patients used regular oral CS as well as combination therapies, future studies should be conducted to better understand whether certain treatment patterns are associated with additional burden and unmet need in PV.**

Limitations

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

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References:

1. Han K, et al. *Indian J Dermatol*. 2023;68(6):724.
2. Porro AM, et al. *An Bras Dermatol*. 2019;94(3):264-278.
3. Werteteil S, et al. JAMA Dermatol. 2019;155(5):627-629.
4. Kianfar N, et al. *Int J Womens Dermatol*. 2022;8(3):e056.
5. Joly P, et al. *J Eur Acad Dermatol Venereol*. 2020;34(9):1900-1913.

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