Poster # EPH212

Incident complications and treatment use in sickle cell disease in US commercial and Medicare-insured patients

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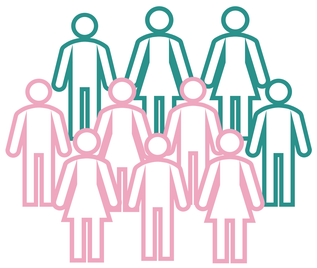
Despite availability of a variety of maintenance therapies for SCD, utilization remains limited



Most patients use only as-needed therapies despite development of complications and progression of disease

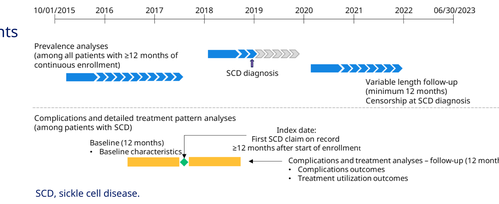
BACKGROUND & AIMS

- Sickle cell disease (SCD) is a group of hereditary conditions that influence erythrocyte morphology in response to oxygen levels[1]
- SCD is associated with acute pain due to vaso-occlusion and a wide variety of acute and chronic complications[1]
- This study assessed the prevalence of SCD, rates of SCD complications, and SCD management strategies within the Merative™ MarketScan® Commercial/Medicare database to provide a current assessment of the clinical burden of SCD in the United States **(Figure 1)**
- The primary objectives of this study were as follows:
  - Estimate the prevalence of SCD among those with commercial/Medicare insurance in the United States
  - Describe the prevalence and incidence of SCD-related complications among patients with SCD, overall and stratified by age
  - Characterize SCD treatment utilization, including use of both preventive maintenance and as-needed therapies for management of SCD complications

MATERIALS & METHODS

- This noninterventional, retrospective study used Merative™ MarketScan® Commercial/Medicare database to evaluate the prevalence of SCD, rates of SCD-related complications, and SCD treatment strategies in commercial and Medicare populations using data from October 1, 2015, through June 30, 2023 **(Figure 1)**
- For assessment of SCD complications and treatment utilization among patients with SCD, the index date was defined as the first observed SCD diagnosis occurring ≥12 months after the start of patient eligibility; patients were followed over 12-month baseline and follow-up periods
  - Outcomes included prevalence and incidence of SCD-related complications and high-level treatment utilization characteristics reported in the baseline and follow-up periods
  - Incidence proportions were defined as the presence of a diagnosis for the condition of interest in the 12-month follow-up period among the subset of patients with no diagnosis in the baseline period
  - Results were reported for the overall eligible patient populations and for four age-based subgroups (<18, 18-30, 31-64, and ≥65 years)
- For assessment of SCD treatment patterns, SCD regimens were evaluated: description of regimens, adherence, switching/discontinuation patterns, and use of as-needed therapies (antibiotics, pain medications [gabapentinoids, opioids, prescription nonsteroidal anti-inflammatory drugs, serotonin-norepinephrine reuptake inhibitors, and tricyclic antidepressants], and blood transfusions)

Figure 1 Study schematic



SCD, sickle cell disease.

RESULTS

- Of the 50,311,522 patients included in the prevalence analysis, 12,422 had SCD, corresponding to a prevalence rate of 0.02% in the commercial/Medicare population
- The complications and treatment analyses included 6,449 patients **(Table 1)**
- Analysis of treatment patterns showed that 37.5% of patients had maintenance therapy. The most common regimen among patients with a maintenance therapy was hydroxyurea monotherapy (80.5% of patients), and complete discontinuation was the primary reason for ending treatment (59.4%) **(Figure 2)**

Table 1 Baseline demographic and clinical characteristics

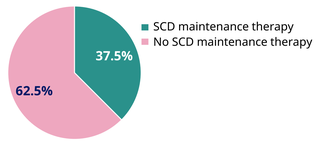
	All patients N=6,449 (100.0%)	<18 years n=1,937 (30.0%)	18-30 years n=1,399 (21.7%)	31-64 years n=2,830 (43.9%)	≥65 years n=283 (4.4%)
Age, years					
Mean (SD)	31.0 (19.5)	9.1 (5.2)	23.5 (3.7)	45.4 (9.3)	74.4 (7.1)
Sex, n (%)					
Female	3,843 (59.6)	971 (50.1)	833 (59.5)	1,860 (65.7)	179 (63.3)
Male	2,606 (40.4)	966 (49.9)	566 (40.5)	970 (34.3)	104 (36.7)
Region of residence, n (%)					
Northeast	1,110 (17.2)	303 (15.6)	228 (16.3)	518 (18.3)	61 (21.6)
North central	1,025 (15.9)	309 (16.0)	235 (16.8)	365 (12.9)	116 (41.0)
South	3,971 (61.6)	1,228 (63.4)	860 (61.5)	1,793 (63.4)	90 (31.8)
West	319 (4.9)	83 (4.3)	71 (5.1)	149 (5.3)	16 (5.7)
Unknown	24 (0.4)	14 (0.7)	5 (0.4)	5 (0.2)	0 (0.0)
Duration of continuous enrollment, years					
Mean (SD)	4.1 (1.8)	4.4 (1.9)	3.9 (1.6)	4.1 (1.7)	4.0 (1.6)
Insurance plan type, n (%)					
Comprehensive/ indemnity	310 (4.8)	68 (3.5)	54 (3.9)	91 (3.2)	97 (34.3)
EPO/PPO	3,185 (49.4)	956 (49.4)	702 (50.2)	1,404 (49.6)	123 (43.5)
POS/POS with capitation	646 (10.0)	193 (10.0)	117 (8.4)	326 (11.5)	10 (3.5)
HMO	914 (14.2)	266 (13.7)	200 (14.3)	412 (14.6)	36 (12.7)
CDHP/HDHP	1,247 (19.3)	421 (21.7)	293 (20.9)	532 (18.8)	1 (0.4)
Other/ unknown	147 (2.3)	33 (1.7)	33 (2.4)	65 (2.3)	16 (5.7)
SCD genotype,[a] n (%)					
Hb-SS	1,763 (65.0)	649 (63.2)	446 (79.2)	652 (61.3)	16 (27.6)
Hb-SC	909 (33.5)	357 (34.8)	110 (19.5)	401 (37.7)	41 (70.7)
HbS β^0 thalassemia	11 (0.4)	4 (0.4)	1 (0.2)	5 (0.5)	1 (1.7)
HbS β^+ thalassemia	29 (1.1)	17 (1.7)	6 (1.1)	6 (0.6)	0 (0.0)
CCI, mean (SD)	0.7 (1.4)	0.3 (0.7)	0.4 (1.0)	0.8 (1.5)	2.9 (2.5)

[a]Claims with reference to SCD genotype were available for two-thirds of the sample; claims above reflect those among patients with only 1 coded genotype.

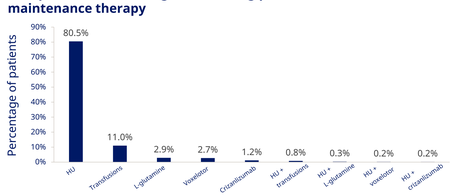
CCI, Charlson Comorbidity Index; CDHP, consumer-driven health plan; EPO, exclusive provider organization; Hb, hemoglobin; HDHP, high-deductible health plan; HMO, health maintenance organization; POS, point of service; PPO, preferred provider organization; SCD, sickle cell disease. Hb gene

Figure 2 SCD maintenance treatment[a] patterns

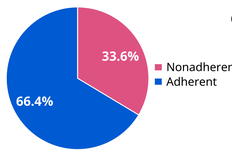
A. SCD treatment use



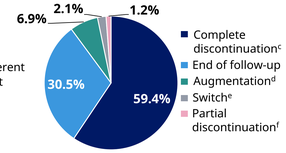
B. Top SCD treatment regimens among patients with a maintenance therapy



C. Treatment adherence



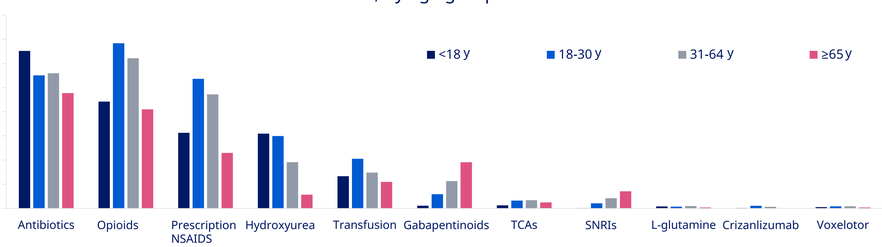
D. Reasons for ending treatment[b]



[a]SCD medications included HU, L-glutamine, crizanlizumab, voxelotor, and regular blood transfusions (defined as ≥4 transfusions between 20 and 50 days apart). [b]Due to rounding, values do not sum to 100. [c]Defined as stopping all components of a regimen for ≥90 days. [d]Defined as addition of a new regimen component. [e]Defined as stopping ≥1 regimen component and initiating a new therapy within 90 days. [f]Defined as stopping ≥1 but not all components of a regimen for ≥90 days.
HU, hydroxyurea; SCD, sickle cell disease.

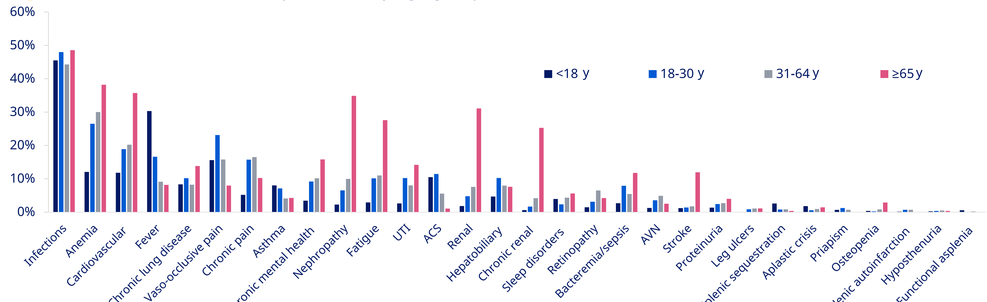
- Treatment utilization trends were similar in the baseline and follow-up periods. As-needed therapies—including antibiotics, opioids, and prescription nonsteroidal anti-inflammatory drugs (baseline: 35.9%; follow-up: 42.7%)—were the most used
  - Regular blood transfusions (≥4 transfusions 20-50 days apart) were used in approximately 4% of patients
  - Medication use varied across age groups with younger patients having increased use of antibiotics and late adolescents/early adults with the greatest proportions of use for most pain medications **(Figure 3)**
- During the 12-month baseline and follow-up periods, the most common incident complications were infections, anemia, and cardiovascular **(Figure 4)**. These complications were also the most prevalent in the baseline and follow-up periods (data not shown)
- Prevalence of conditions differed across age groups; pain-related conditions tended to emerge in late adolescence or young adulthood and chronic long-term complications primarily occurred in older adults (data not shown)

Figure 3 12-month incident treatment utilization, by age group



NSAIDs, nonsteroidal anti-inflammatory drugs; SNRIs, serotonin-norepinephrine reuptake inhibitors; TCAs, tricyclic antidepressants.

Figure 4 12-month incident complications, by age group



ACS, acute chest syndrome; AVN, avascular necrosis; UTI, urinary tract infection.

CONCLUSIONS

- Overall, utilization of SCD maintenance therapies was limited—less than 40% of patients used a maintenance SCD therapy
- The majority of patients used only as-needed therapies (eg, antibiotics, pain medications) rather than maintenance therapies
- There were very few changes in the incidence of SCD complications or utilization of SCD treatments from the baseline to follow-up period
- There is a need for improvement in the management of SCD, including increasing the use of maintenance therapies to help limit the development of complications and the progression of disease

PLAIN LANGUAGE SUMMARY

- **Why does it matter?** Sickle cell disease (SCD) is associated with acute pain due to blocking of blood vessels and a wide variety of acute and chronic complications
- **How does it work?** This study assessed the prevalence of SCD, rates of SCD complications, and SCD management strategies within the MarketScan® Commercial and Medicare databases to provide a current assessment of the clinical burden of SCD in the United States
- **What did we find?** We found that overall utilization of SCD maintenance therapies was limited among patients with commercial or Medicare insurance over the study period. Although a variety of maintenance treatments are available for SCD, including new medications introduced in 2019, utilization of these maintenance therapies remains low

ACKNOWLEDGMENTS AND DISCLOSURES

EFE and AW are employees of Novo Nordisk Inc.

BB and CL are employees of Merative.

This study was sponsored by Novo Nordisk Inc. The authors acknowledge the medical writing assistance of Alana Vagnozzi, PhD, and Amy Leach, PhD, of Precision AQ.

REFERENCE

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