Medication Utilization Among Children with Sickle Cell Disease in the United States

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Background

- Sickle cell disease (SCD), a hereditary hemoglobinopathy, is one of the most prevalent genetic disorders affecting children worldwide.[1] SCD is characterized by the mutation at the beta hemoglobin subunit leading to abnormal hemoglobin (HbS), leading to myriads of serious health complications for pediatric patients, significantly impacting their quality of life.[2,3]
- Continuity of care is a challenge encountered by pediatrics with SCD due to limited access to specialized care, and knowledge gaps in SCD clinical management among pediatricians.[4]
- In 2014, a consensus guideline for managing SCD was released by an expert panel convened by the National Heart, Lung, and Blood Institute (NHLBI) in response to an expressed need for the latest clinical guidelines for managing individuals with SCD.[4]
- Previous studies have assessed SCD medication utilization.[5] However, the influence of the 2014 NHLBI SCD treatment guidelines on the medication management of children with SCD is unknown, in addition to the limited knowledge of the overall pharmacological management of children with SCD.

Objective

- This study assessed medication utilization trends overall and by patient age, sex, race/ethnicity, region, and household income.

Methods

- This retrospective study examined children aged 1-17 years diagnosed with SCD from January 1, 2010, to December 31, 2018, in Optum's de-identified Clinformatics® Data Mart database.
- Inclusion: At least one inpatient or two outpatient diagnosis claims for SCD (International Classification of Diseases, Clinical Modification ICD-9-CM, and ICD-10-CM) during the study period. Exclusion: Clinical trials, hematopoietic stem cell transplant, and gene therapy participants before the index date.
- All prescriptions filled by pediatric patients with SCD after the index date, during any enrollment periods over the study period, were captured in the analysis.
- Medication utilization rates were assessed over the entire study period and annually (based on fill date) and were measured as the percent of the study population treated among the enrolled patients with SCD during that timeframe.
- Changes in medication utilization over time were assessed with Joinpoint regression analyses for differing annual percent changes (APCs) post-NHLBI SCD treatment guidelines in 2014 (2010-2014 and 2014-2018) and average annual percent change (AAPC). The AAPC is the weighted average of all APCs over the entire study period. We restricted our analyses to medications with utilization rates of at least 5% over the study period.
- All data management and descriptive statistics were performed using SAS version 9.4 (SAS Institute, Cary, NC).

Results

Figure 1: Prevalence and trends of *medication* utilization for managing pediatrics with sickle cell disease

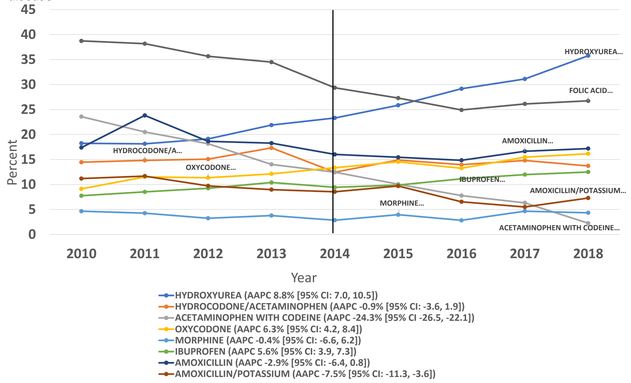


Figure 2: Prevalence and trends of *medication class* utilization for managing sickle cell disease

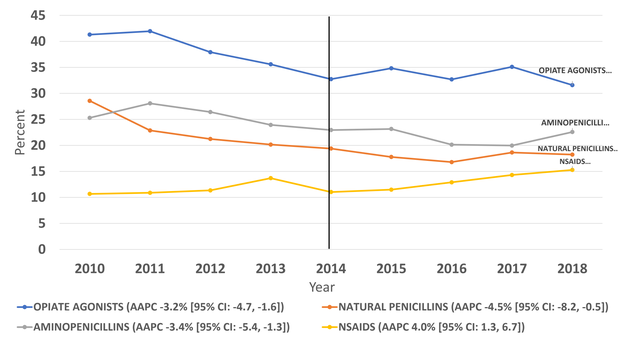


Figure 3: Prevalence and trends of antibiotic utilization among patients with sickle cell disease

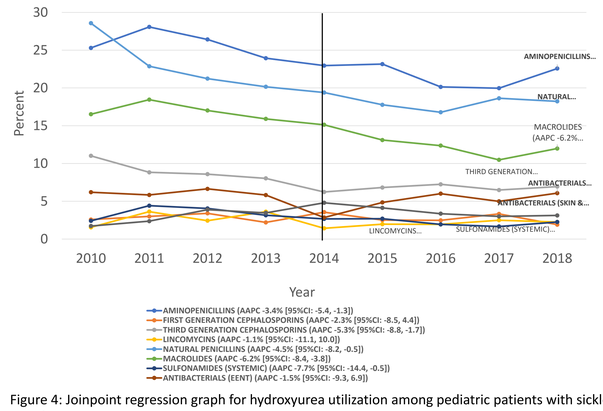


Figure 4: Joinpoint regression graph for hydroxyurea utilization among pediatric patients with sickle cell disease

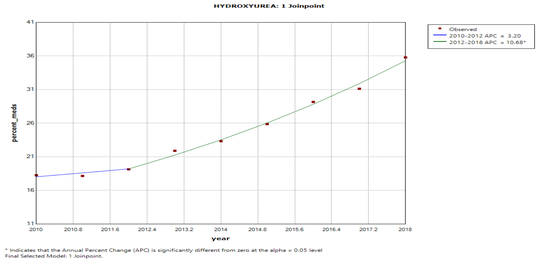


Figure 5A: Joinpoint regression graph for hydroxyurea utilization stratified by age: 1-5 years

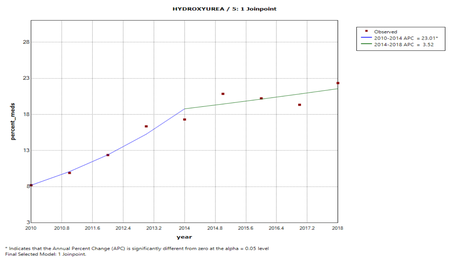


Figure 5B: Joinpoint regression graph for hydroxyurea utilization stratified by age: 6-12 years

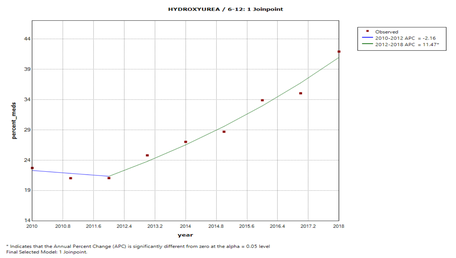


Figure 5C: Joinpoint regression graph for hydroxyurea utilization stratified by age: 13-17 years

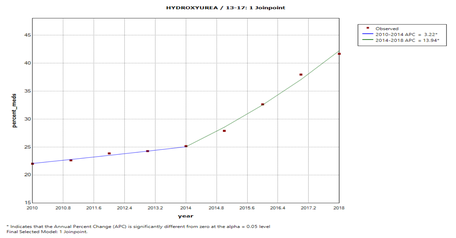


Figure 6A: Joinpoint regression graph for hydroxyurea utilization stratified by sex: Female

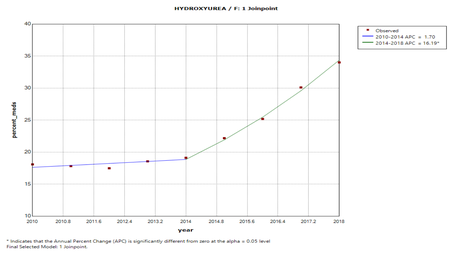
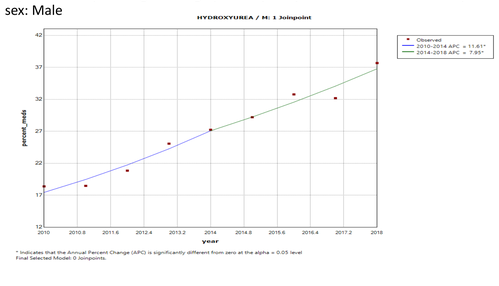


Figure 6B: Joinpoint regression graph for hydroxyurea utilization stratified by sex: Male



Results

- Over the 8-year study period, 1,868 pediatric patients with SCD were eligible for our study. The average age was 8.8 years (standard deviation [SD] 5.0 years), with the majority (40.1%) being 6-12 years old.
- Over the study period, 29.5% of the study population with SCD filled prescriptions for hydroxyurea, with an AAPC demonstrating a significant increase over the study period by 8.8% (p<0.001) each year (Figure 1).
- For pain management, 48.3% of the study population was treated with opiate agonists, with a significant annual decrease of 3.2% (p=0.002), and 19.8% were treated with non-steroidal anti-inflammatory drugs (NSAIDs), with a significant annual increase of 4.0% (p=0.010) (Figure 2).
- In terms of antibiotics, 39.8% and 29.5% of the study population filled prescriptions for aminopenicillins and natural penicillin, respectively. During the study period, the utilization of aminopenicillin and natural penicillin decreased significantly each year by an average of 3.4% (p=0.006) and 4.5% (p=0.027), respectively (Figure 3).
- In age-stratified analyses, hydroxyurea use was highest among 13-17 years old (35.5%), followed by 6-12 (33.5%) and 1-5 years old (19.3%).
- Annual hydroxyurea utilization increased significantly in 2012, prior to the release of the NHLBI SCD treatment guidelines (2012-2018 APC 10.7%, p<0.001; 2010-2012 APC 3.2%, p=0.342; Figure 4).
- Significant changes were observed across most age groups and sexes after the release of the NHLBI SCD treatment guidelines in 2014 (Figures 5A-6B).

Conclusion

- Our study detected notable shifts in prescribing patterns, including higher rates of hydroxyurea use and lower rates of opiate use, which correspond with the 2014 NHLBI SCD treatment guidelines.
- There is still a need for improvement in the prophylactic utilization of hydroxyurea and penicillin.

References

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