

## Background

- Sickle cell disease (SCD) is a genetic disorder affecting millions globally. According to the Centers for Disease Control and Prevention (CDC), SCD has about 100,000 prevalent cases and about 3.5 million heterozygous carriers in the United States.<sup>1</sup>
- Sickle cell management includes disease-modifying therapies, antibiotics, opioid and non-opioid analgesics, and curative treatments (hematopoietic stem cell transplant or gene therapy).<sup>2</sup>
- In 2014, the National Heart, Lung, and Blood Institute (NHLBI) released a consensus guideline for managing SCD in response to an expressed need for the latest clinical guidelines for managing individuals with SCD.<sup>3</sup>
- Previous studies have assessed utilization rates of hydroxyurea and opioids in individuals with SCD in state Medicaid populations. However, there is limited knowledge of other prescription medications utilized by individuals with SCD, either for SCD or for comorbidities. In addition, change in medication management of adults with SCD over time and since the release of the 2014 NHLBI SCD treatment guidelines.<sup>4,5</sup>

## Objective

- This study aimed to assess the real-world medication utilization in adult patients with SCD in the United States, including trends in medication utilization over time and by patient age, sex, race/ethnicity, and region.

## Methods

- A retrospective study among adults aged at least 18 years at index date, with at least one inpatient or two outpatient International Classification of Diseases, Ninth or Tenth Revision, Clinical Modification diagnosis claims for SCD from January 1, 2010, to December 31, 2018, in the Optum Clinformatics® Data Mart database.
- The first date of SCD diagnosis during the study period served as the index date. Our study included all prescriptions filled by patients with SCD after the index date, during any enrollment periods over the study period.
- Medication utilization rates were assessed over the entire study period and each year, based on fill date. This was 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 (Joinpoint software version 4.9.1). Average annual percent changes (AAPCs) were calculated for the entire study period. Changes in trends after the 2014 NHLBI SCD treatment guidelines were also assessed as differing annual percent change (APCs) from 2010-2014 and 2014-2018.
- 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 adults with sickle cell disease

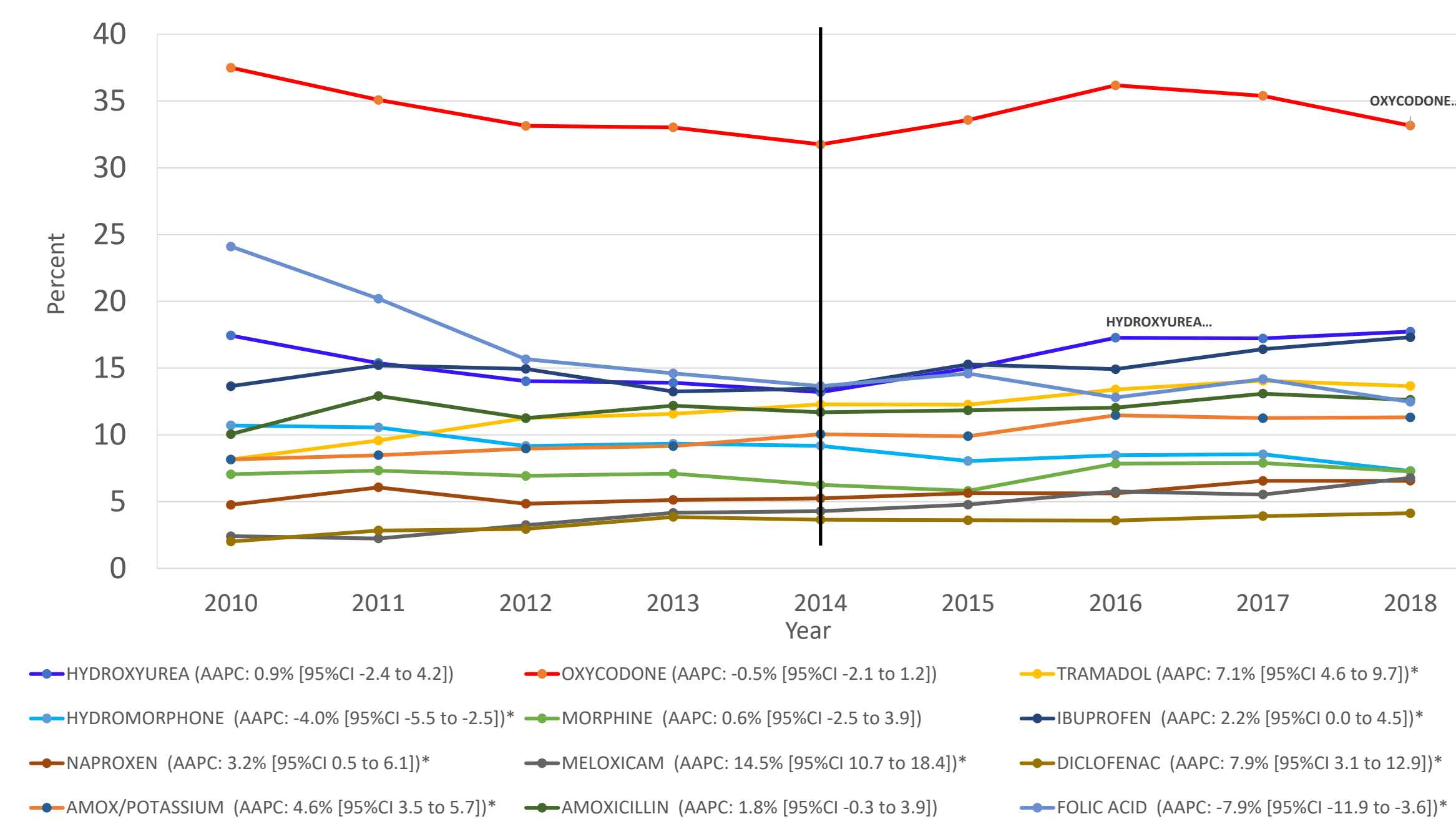


Figure 2: Prevalence and trends medication class utilization for managing adults with sickle cell disease

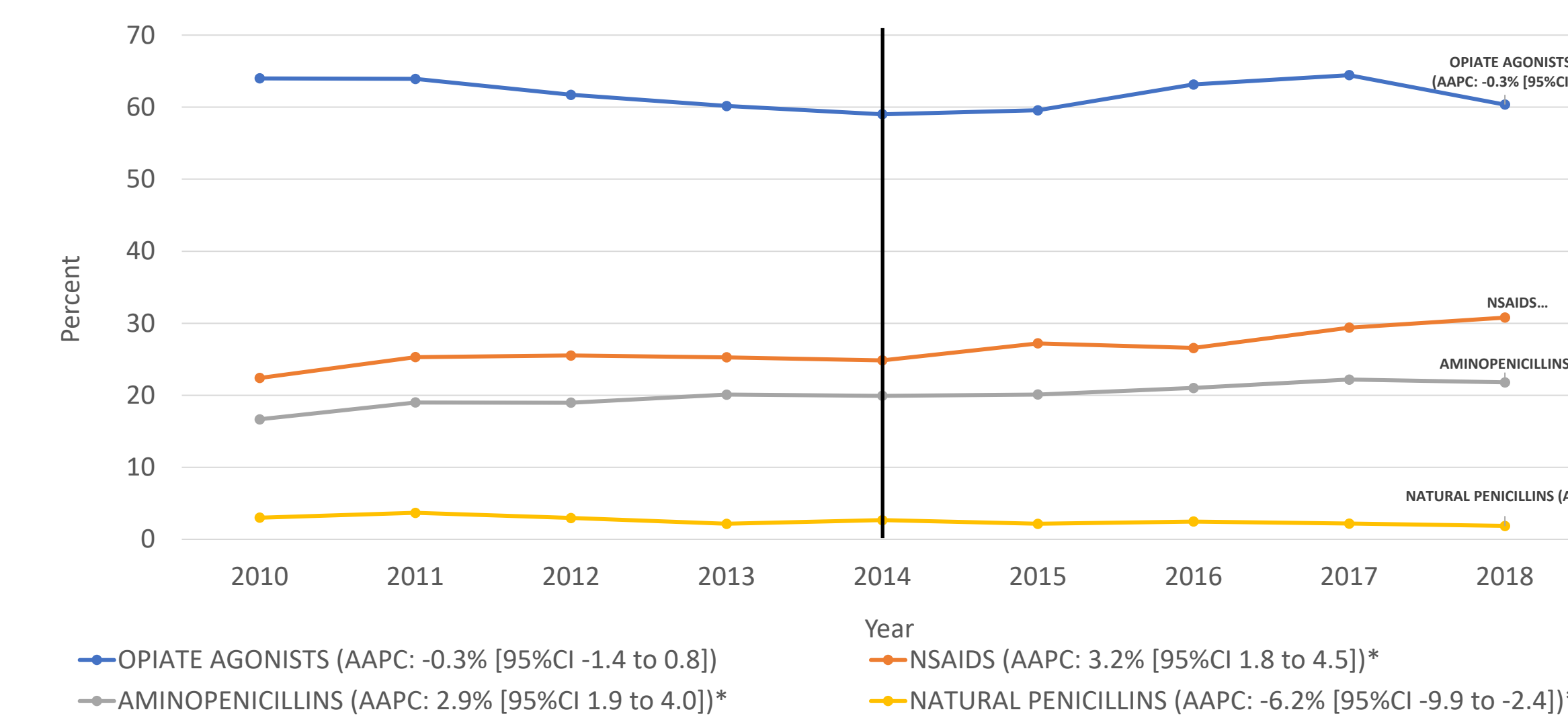


Figure 3: Prevalence and significant trends of hydroxyurea utilization overall and stratified by race, age, sex, and region group

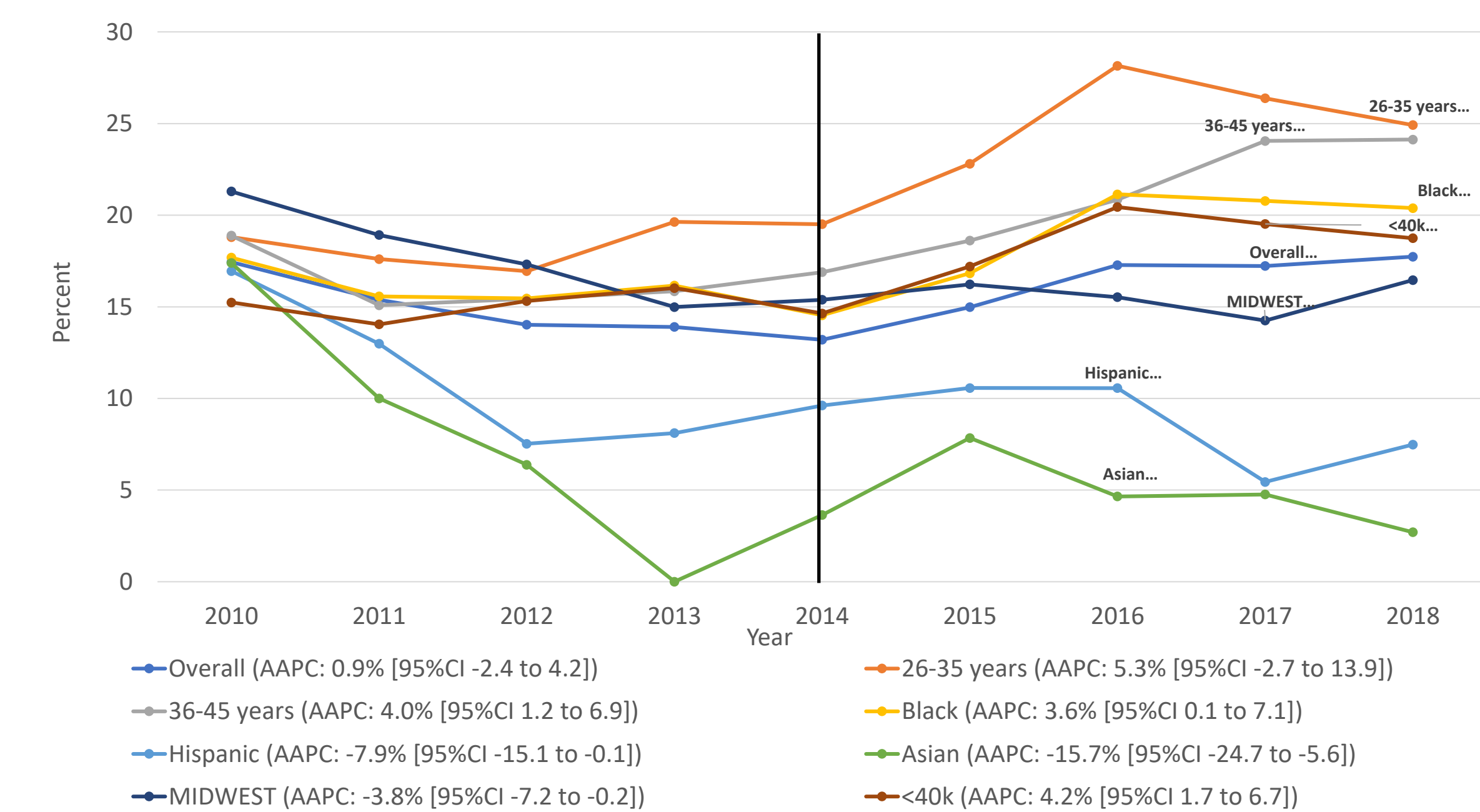


Figure 4: Joinpoint regression graph for hydroxyurea utilization among patients with sickle cell disease

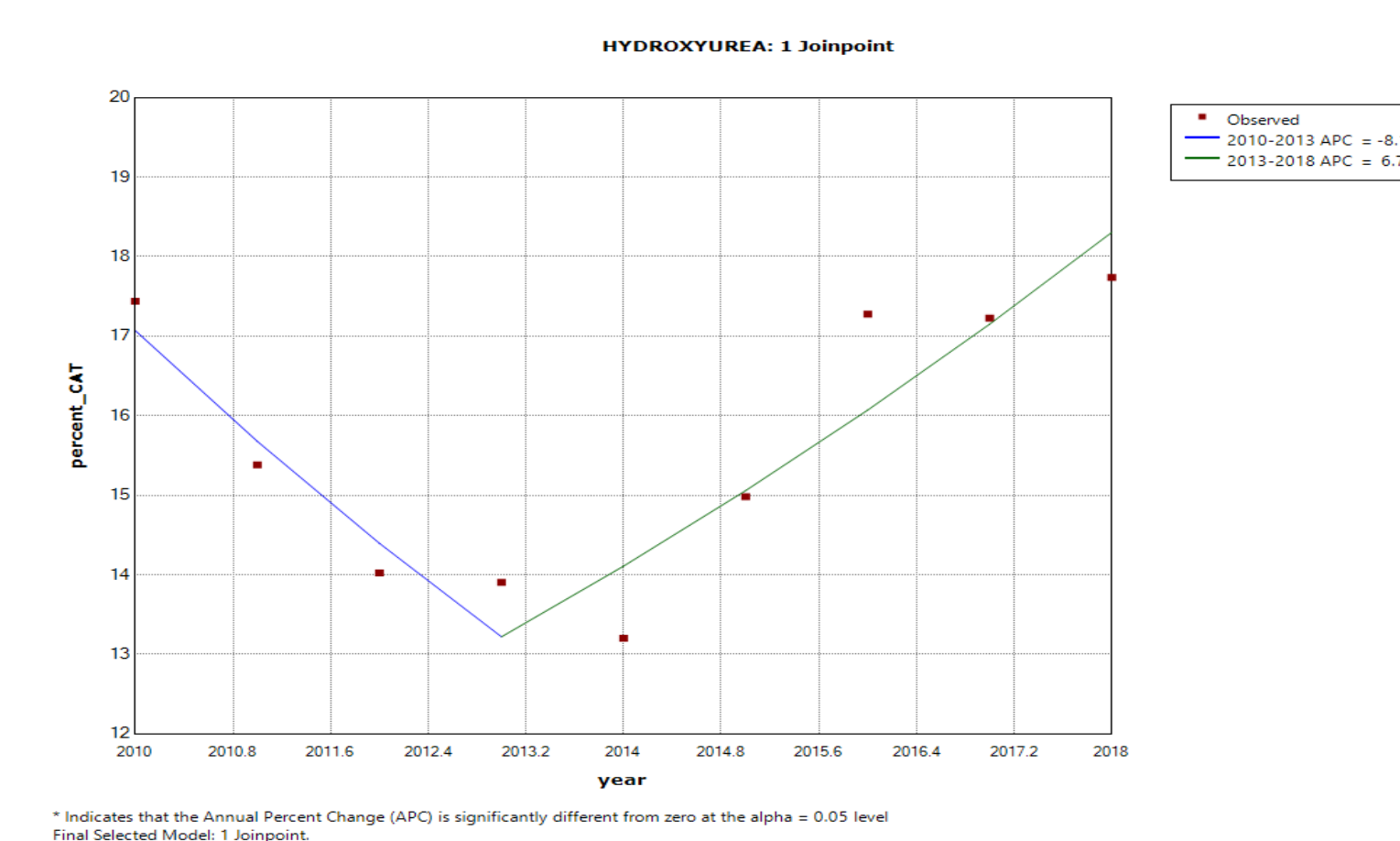


Figure 5A: Joinpoint regression graph for hydroxyurea utilization stratified by age: 18-25 years

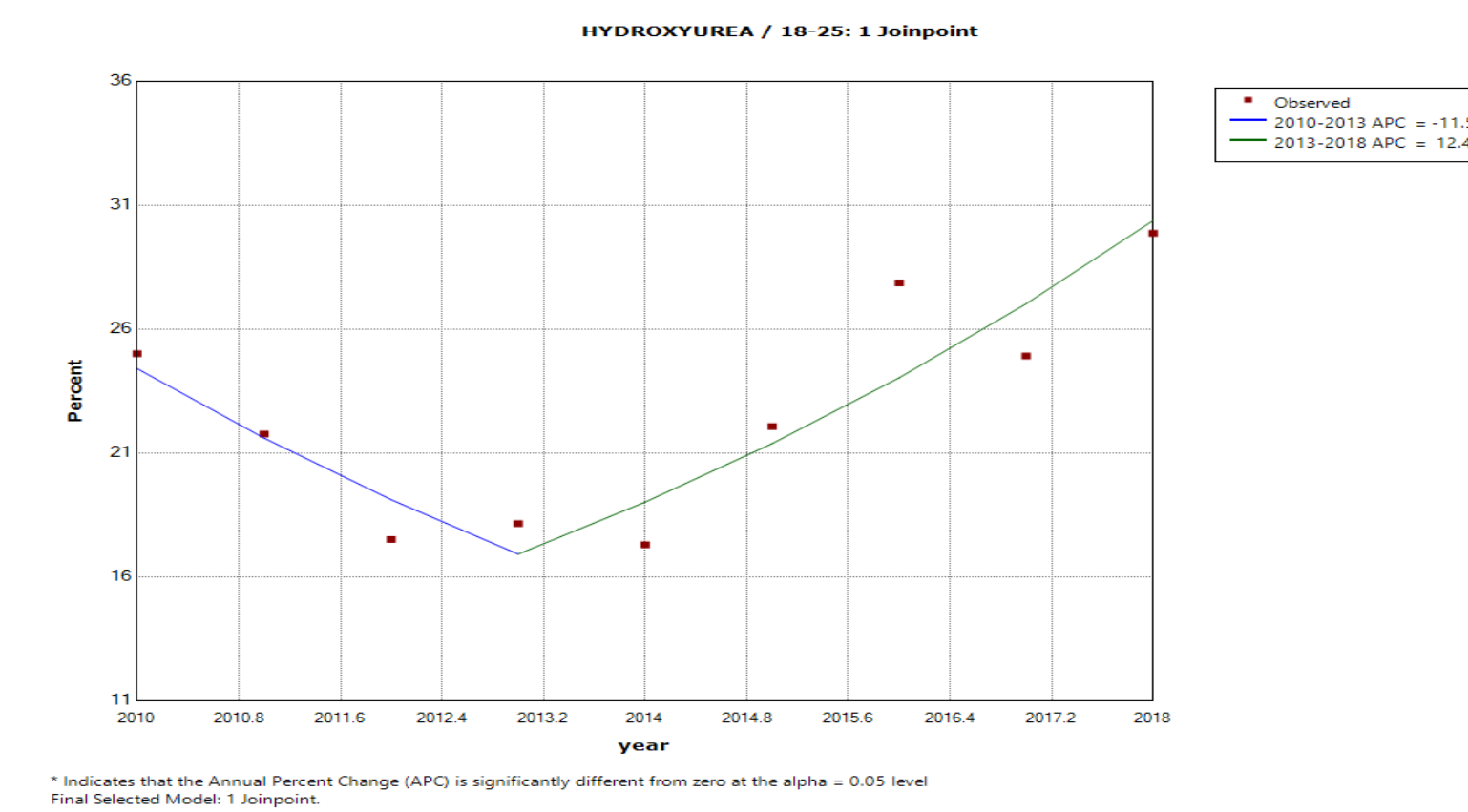


Figure 5B: Joinpoint regression graph for hydroxyurea utilization stratified by age: ≥65 years

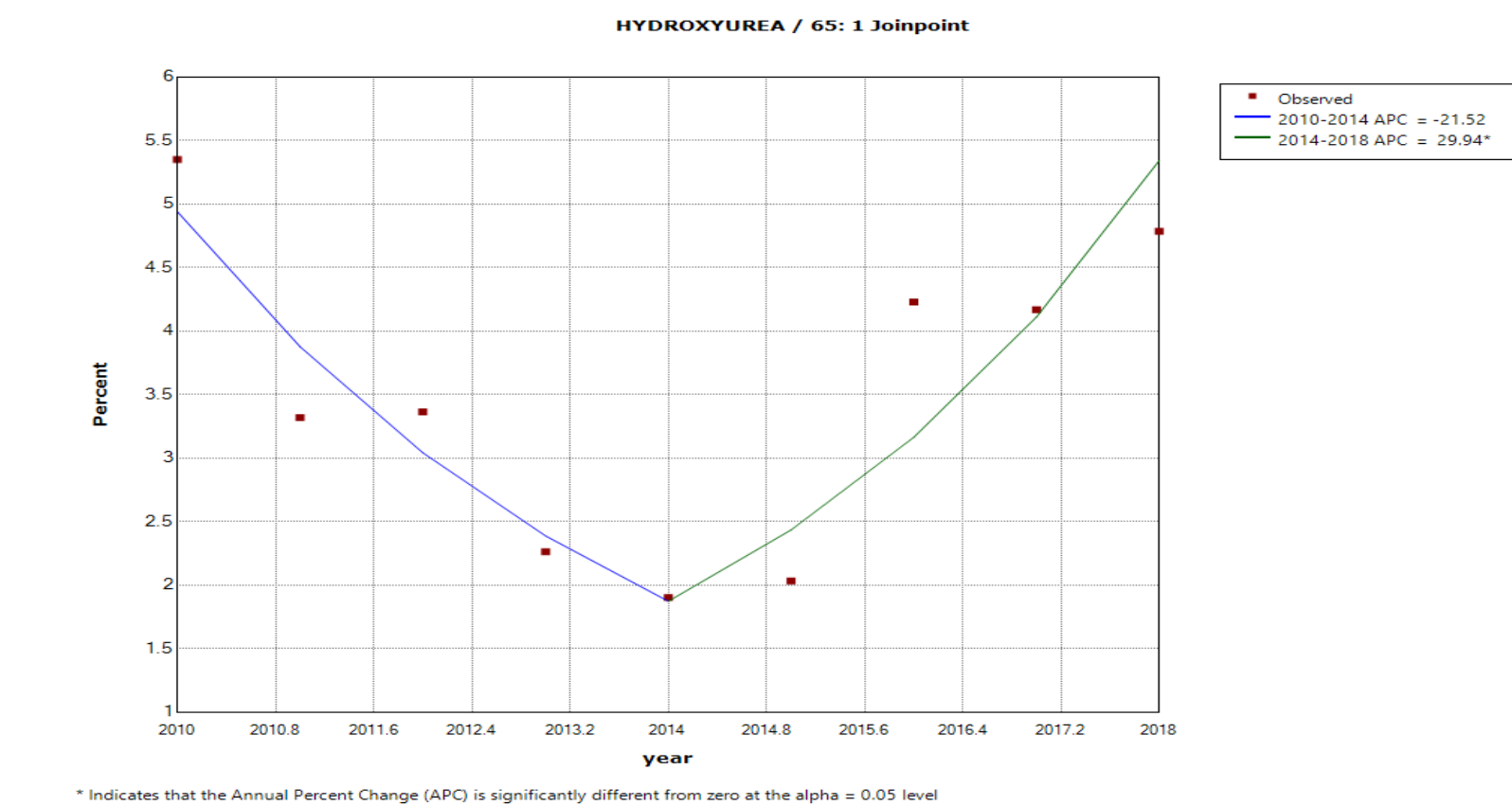


Figure 6: Joinpoint regression graph for hydroxyurea utilization by household income level: \$40,000-\$49,000

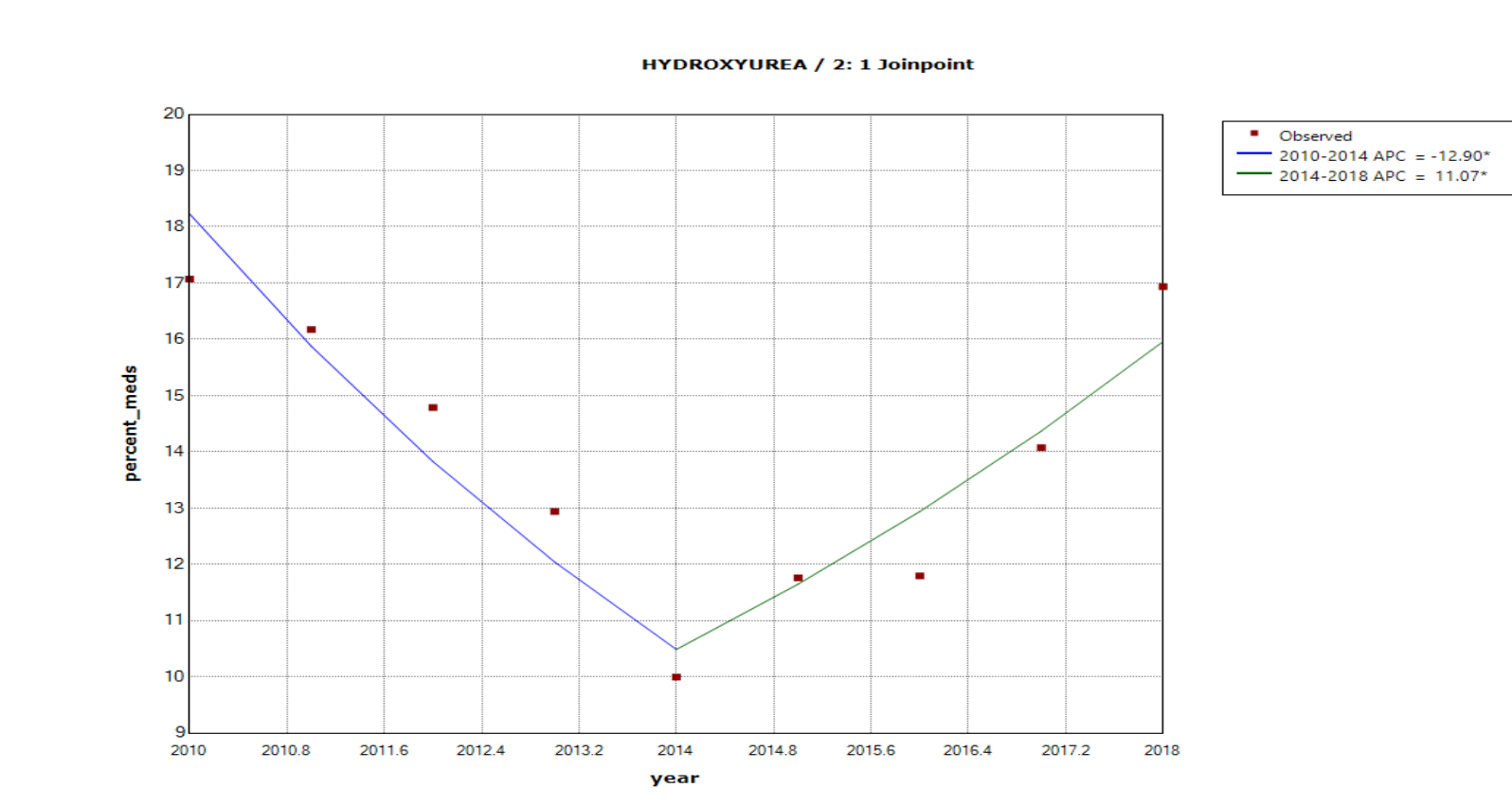


Figure 7A: Joinpoint regression graph for hydroxyurea utilization stratified by region: Northeast

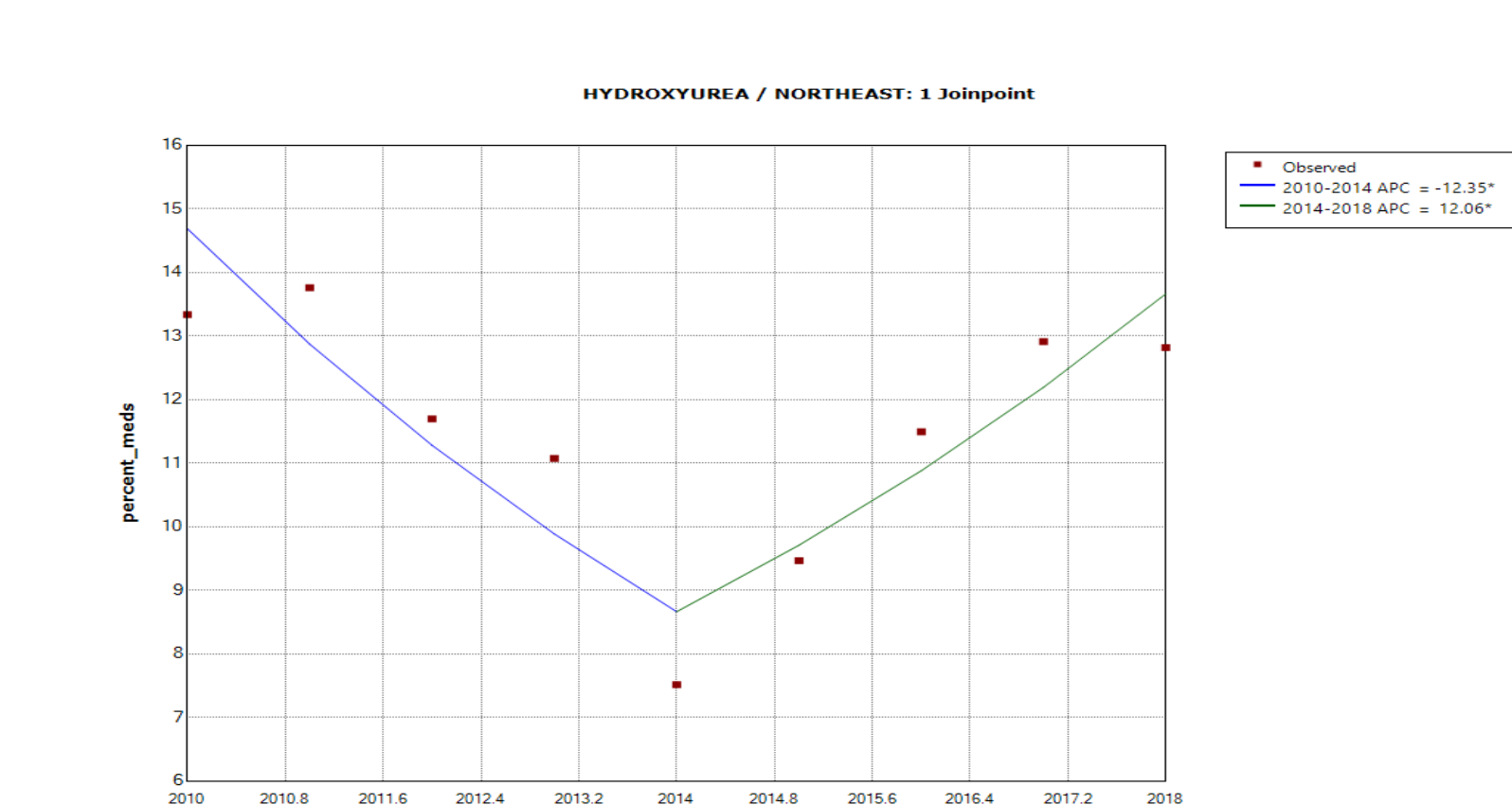
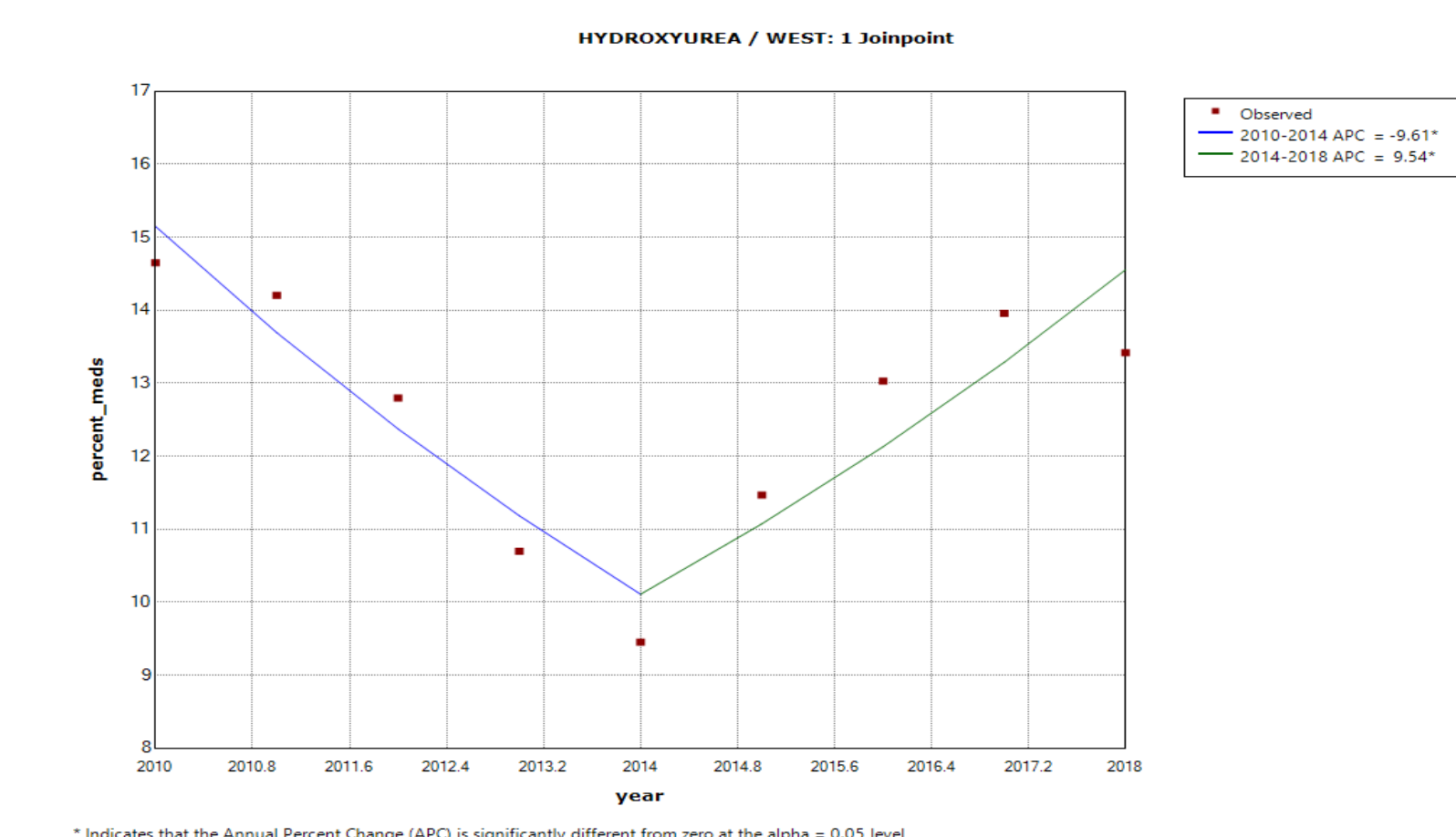


Figure 7B: Joinpoint regression graph for hydroxyurea utilization stratified by region: West



## Results

- Over the 8-year study period, 8,459 individuals with SCD met the study eligibility criteria. The mean age of study participants was 46.7 years (standard deviation [SD] 18.1 years), with 20.7% being 26-35 years old and 20.6% being ≥65 years old.
- Over the study period, 18.3% of the study population with SCD filled prescriptions for hydroxyurea, with an AAPC demonstrating an increase of 0.9% (p=non-significant) over the study period.
- For pain management, about 69.4% and 39.4% of the study population were treated with opiate agonists and non-steroidal anti-inflammatory drugs (NSAIDs), with significant average annual increases of 3.5% (p=0.013) and 8.2% (p<0.001), respectively.
- In age-stratified analysis, hydroxyurea utilization had the highest prevalence among 18-25 years old (29.2%), then 26-35 years old (24.1%), and 36-45 years old (22.3%). There was a significant increase in annual hydroxyurea utilization among some age groups: 26-35 years AAPC 6.0% (p=0.002) and 36-45 years 4.0% (p=0.005) each year.
- Hydroxyurea was mostly filled by individuals residing in the South (19.9%), followed by the Midwest (17.8%), with annual significant decreases in the Midwest (AAPC -3.8%, p=0.041).
- Opiate agonists were common among those residing in the South (71.9%) and less common in the Northeast (57.1%), with significant annual decreases in utilization in the Northeast (AAPC -3.7%, p=0.005). Tramadol utilization increased significantly in the South (AAPC 7.3%, p=0.001), West (AAPC 8.9%, p=0.005), Northeast (AAPC 4.3%, p=0.021), and Midwest (AAPC 6.7%, p=0.040) each year.

## Conclusion

- Prevalence of hydroxyurea is very low without any significant change in hydroxyurea use since the release of the NHLBI SCD treatment guidelines in 2014. However, a moderate increase was observed in opiate agonist prescriptions over the study period.
- There is still a need for improvement in the prophylactic utilization of hydroxyurea.

## References

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