

Disease-Modifying Therapies Remain Underused in Sickle Cell Disease Despite Safety and Efficacy

Only one quarter of patients living with sickle cell disease are prescribed disease modifying therapies, particularly newer ones.

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Disease-modifying therapies (DMTs), particularly newer ones, for sickle cell disease (SCD) are substantially under prescribed, according to [new research](#) presented today [December 7] at the [66th American Society of Hematology \(ASH\) Annual Meeting](#) in San Diego.

“Underuse of these therapies is a real problem,” said lead study author Omar Niss, MD, director of [classical hematology](#) at Cincinnati Children’s Hospital Medical Center and a faculty member of the University of Cincinnati’s department of pediatrics. “These medications help individuals with SCD on a daily basis by reducing pain and increasing hemoglobin levels, and in the long term by protecting their organs and improving their overall well-being.”

SCD is the most common inherited blood disorder, affecting around 100,000 individuals in the United States and one out of every 365 Black or African American births. It is characterized by abnormal blood cells that can block blood vessels, leading to complications such as episodes of severe pain, as well as damage to tissues and organs.

“The mainstay treatment for SCD is hydroxyurea, which is a safe and effective drug that has been available for over 35 years, though newer medications have also become available in the last five years,” said Dr. Niss. He and his colleagues aimed to fill a data gap on DMT utilization among patients with SCD by using the [ASH Research Collaborative SCD Data Hub](#), a resource that collects and aggregates real-world data to accelerate research and enhance patient outcomes.

The researchers used electronic health records from 14 U.S. sites participating in the Data Hub to analyze data on 22,793 patients with any genotype of SCD. The data spanned 2015 to 2023; just over 55% of the study cohort was female and the average age was 24 years. DMT use was defined as treatment with hydroxyurea, voxelotor, or L-glutamine for at least 90 days within a year, or as six or more transfusions within a year.

Of the entire study cohort, 5,547 patients (24%) were prescribed one or more DMT, with 3% having been prescribed two or more DMTs to be used together. Among patients with verified hemoglobin SS genotype, the most severe variant of SCD, 35% were prescribed DMT. Generally, patients prescribed DMTs were younger (22.6 vs 24.6 years) had increased acute care use (2.7 vs 1.4 emergency department visits or hospitalizations per year), were more likely to be in-patient (17.3 vs 10.8 days per year), and had a lower level of hemoglobin, the part of the blood responsible for transporting oxygen (8.8 vs 9.9 g/dL). This shows DMT prescription was more common among patients with more severe disease. The researchers also noted geographic variation in rates of DMT utilization, with the highest rates occurring in the Northeast U.S. (33%) and the lowest in the Southwest (15.4%).

Dr. Niss and his colleagues found that between 2015 to 2023, DMT use increased slightly from 7.2% to 19.8%, though the bulk of the increase could be attributed to hydroxyurea, which increased from 5.5% to 16.5%. Following their respective approvals, voxelotor use increased from 0.7% in 2020 to 1.6% in 2023, while L-glutamine use increased from 1.3% in 2018 to 1.2% in 2023. This study included patients up to 2023 before voxelotor was withdrawn in September 2024 due to safety concerns. The researchers plan to further analyze voxelotor data in the Data Hub.

These data are “disappointing but not surprising,” said Dr. Niss. Ideally, all patients with a severe form of SCD, about 80% of the total population living with the disorder, should be receiving at least one DMT, he added. “We know these medications work, and our data confirmed that patients on hydroxyurea with fetal hemoglobin levels of more than 20%, a marker of hydroxyurea efficacy, had significant improvement in health care utilization and laboratory markers,” said Dr. Niss. “We need to identify what is preventing providers from using these medications more often – whether it’s a specific patient characteristic or geosystemic barriers – so that we can address these issues. There is no justification for underusing these therapies.”

The study did have some limitations, including the use of registry data and exclusion of crizanlizumab, a more recently approved DMT, due to insufficient data. Dr. Niss and his colleagues plan to conduct further studies to validate their findings.

This [news release](#) was published by the American Society of Hematology on December 7, 2024.

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