

Adherence Outcomes of a Liquid Hydroxyurea Delivery Program in a Pediatric Population

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Abstract

Background: Hydroxyurea remains underutilized in the pediatric sickle cell population despite its well-known efficacy in decreasing sickle cell complications and hospitalizations. Access to refills and liquid formulation remains a critical barrier to adherence to hydroxyurea regimens. This study was undertaken to determine the clinical impact of home-delivering compounded liquid hydroxyurea (LHU) to pediatric patients with sickle cell disease. **Procedure/Methods:** A retrospective cohort study was conducted using electronic health records and pharmacy databases. Pediatric patients younger than 21 years of hydroxyurea initiation from March 2016 to July 2020 who received compounded LHU from Boston Medical Center Pharmacy were included. The primary outcomes of the study were drug adherence (assessed by evaluating the proportion of days covered), rates of acute care utilization, laboratory values, and growth metrics before and after enrolling in the LHU delivery program. **Results:** The final cohort included 41 patients. Significant increases in hemoglobin 0.34 g/dl (95% CI: 0.04-0.63, p=0.02) and mean corpuscular volume 3.2 fL (95% CI: 0.92-5.4, p=0.007) occurred. Hospitalizations decreased by 51.3% (p=0.01), and acute chest syndrome episodes decreased by 86.4% (p=0.02) post-initiation of the LHU delivery program. Drug adherence had a median value of 0.95 one-year post-initiation of LHU. **Conclusions:** Home delivery of compounded LHU improved drug adherence, decreased hospitalizations, and improved laboratory outcomes in pediatric patients with sickle cell disease by overcoming barriers to access. Nationwide implementation of similar home delivery programs can significantly improve outcomes among pediatric patients with sickle cell disease.

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