ELSEVIER

Blood 142 (2023) 3674

The 65th ASH Annual Meeting Abstracts

POSTER ABSTRACTS

901.HEALTH SERVICES AND QUALITY IMPROVEMENT - NON-MALIGNANT CONDITIONS

VOC-Free Status Among Patients with Sickle Cell Disease Following Allogeneic Hematopoietic Stem Cell Transplant: A Cohort Study of Medicaid Enrollees

Mufaddal Mahesri, MDMPH[1], *Su Been Lee, BA*[1], *Raisa Levin, MS*[1], *Suzan Imren, MD*[2], *Lanju Zhang, PhD*[2], *Lina Titievsky, PhD MPH*[2], *Rishi J Desai, PhD MS*[1]

[1] Harvard Medical School/Brigham & Women's Hospital, Boston, MA
[2] Vertex Pharmaceuticals, Boston, MA

Background: Sickle cell disease (SCD) is characterized by recurrent acute vaso-occlusive crises (VOCs), which diminish patient quality-of-life, contribute to end-organ damage and failure, and increase mortality. Currently, approved therapies for SCD are only partially effective and do not fully eliminate VOCs. Allogeneic hematopoietic stem cell transplant (HSCT) is the only known curative option for patients with SCD, however, HSCT is available to <20% of patients who have a matched sibling donor and is associated with significant risks, such as graft-versus-host disease. Autologous gene therapies that have the potential to provide a one-time functional cure to patients with SCD are currently in pivotal trials. The primary endpoint for these pivotal studies is the proportion of patients who become VOC-free for ≥12 months after treatment. Absence of VOCs for ≥12 months is expected to predict long-term efficacy. Given this, we evaluated patients with SCD in the national US Medicaid database who underwent allogeneic-HSCT to determine whether VOC-free status at 12 months was predictive of VOC-free status at 18 and 24 months after HSCT.
Methods: A cohort of patients with SCD who had a record of allogeneic HSCT with at least 12 months of continuous Medicaid enrollment during 2000-2014 was identified. The date of HSCT was defined as the cohort entry date and the covariate assessment window was the 12 months continuous enrollment period preceding the cohort entry date. A VOC event was identified using ICD-9 diagnosis codes in hospitalization and ER visit claims for Hb-SS disease with crisis, other acute pain crisis, acute chest syndrome, priapism, and splenic sequestration. The cohort was further restricted to patients who had ≥24 months of follow-up after cohort entry to accurately assess VOC outcomes. Diagnosis codes associated with the delivery of healthcare and pharmacy claims were used to identify patient characteristics and outcomes of interest. Primary study outcome was the proportion of patients with no VOCs at 6 months, 12 months, 18 months, and 24 months after allogeneic HSCT.
Results: During the study period, 44,685 patients with SCD were identified, out of whom 103 patients had undergone allogeneic HSCT, were enrolled for ≥12 months in the Medicaid program and had ≥24 months of follow-up after HSCT. Mean (SD) age was 10.3 (7.2) years, with 55.3% being male and majority (67%) Black. Patients had an average of 2.7 (SD, 7.9) VOC events during the 12-month period prior to HSCT. The most common (>10%) baseline comorbidities were chronic cardiac disease (42.7%), infections (36.9%), chronic pulmonary disease (31.1%), and acute chest syndrome (12.6%). Among the cohort of 103 patients who had undergone HSCT, 79% (n=81) were VOC-free by 12 months. Furthermore, among patients who were VOC-free at 12 months, 91% (n=74) were VOC-free at 18 months and 90% (n=73) were VOC-free at 24 months.
Conclusions: This population-based study provides valuable information regarding long-term VOC-free status among patients with SCD who have undergone allogeneic HSCT in routine clinical care. Our findings suggest that patients with SCD who are VOC-free by 12 months post-HSCT are highly likely to stay VOC-free for longer durations (i.e. 18 and 24 months) after HSCT.

Disclosures Imren: *Vertex Pharmaceuticals:* Current Employment, Current holder of *stock options* in a privately-held company. **Zhang:** *Vertex Pharmaceuticals:* Current Employment. **Titievsky:** *Vertex Pharmaceuticals:* Ended employment in the past 24 months; *GSK:* Current Employment; *COJECO:* Membership on an entity's Board of Directors or advisory committees. **Desai:** *Bristol Myers Squibb:* Research Funding; *Vertex Pharmaceuticals:* Research Funding.

https://doi.org/10.1182/blood-2023-189427