Abstract: P1464

Title: CLINICAL COMPLICATIONS AMONG PATIENTS WITH TRANSFUSION-DEPENDENT BETA-THALASSEMIA IN GERMANY

Abstract Type: Poster Presentation

Session Title: Thalassemias

Background:

β-thalassemia is a rare genetic disorder wherein patients have reduced or absent β-globin production, resulting in chronic anemia, iron overload, and other serious complications that can lead to early mortality. Patients with transfusion-dependent β-thalassemia (TDT) require lifelong supportive care, including regular red blood cell transfusions (RBCTs) and iron chelation therapies for survival.

Aims:

To describe clinical complications in patients with TDT in Germany.

Methods:

This longitudinal, retrospective cohort study utilized statutory health insurance records (Betriebskrankenkasse data source) in Germany to identify patients with a diagnosis for β-thalassemia between January 1, 2010, and December 31, 2018 (eligibility period). Eligible patients with TDT had ≥8 RBCTs in any 1-year period during the eligibility period and ≥1 year of follow-up after the first qualifying RBCT claim (i.e., index date, defined as the first RBCT of the ≥8 RBCTs during the eligibility period). Patients with hereditary persistence of fetal hemoglobin, sickle cell disease, or hematopoietic stem cell transplant in their medical records at any time were excluded. Patients were followed from index until the earliest of death, deregistration due to patient leaving the data source, or end of study period (December 31, 2019). Demographics were assessed at index. Acute and chronic complications (proportion of total population) were summarized descriptively during follow-up.

Results:

In total, 68 patients with TDT were included. Their mean age at index was 50.6 (standard deviation [SD]=26.0) years, and 30 (44.1%) were female. Average length of follow-up was 5.0 years. Patients with TDT averaged 16.4 (SD=11.2) RBCTs per-patient-per-year during follow-up. The most common acute complications were (>20% prevalence) cardiovascular complications (55.9%) and infections (25.0%). The most common cardiovascular complications were arrythmia (47.1%), heart failure (44.1%), and atrial fibrillation (26.5%). The most common chronic complications (>20% prevalence) were endocrine complications/bone disorders (69.1%); mental illness (depression or anxiety [36.8%]); urinary tract complications (36.8%); iron overload/hemochromatosis (33.8%); renal complications (32.4%); malignancies (30.9%); liver complications (25.0%); splenomegaly (23.5%); cardiovascular complications (22.1%); and myelodysplastic syndrome (22.1%). Diabetes (33.8%) and osteoporosis (30.9%) were the most common chronic endocrine complications/bone disorders.

Summary/Conclusion:

Despite the best available care, patients with TDT experience substantial burden due to clinical complications associated with the disease, highlighting the need for innovative therapies in this space.

Keywords: beta thalassemia, Hemoglobinopathy, Complications