Abstract: P824

Title: EPIDEMIOLOGY AND REAL-WORLD TREATMENT MANAGEMENT OF PAROXYSMAL NOCTURNAL HEMOGLOBINURIA IN FRANCE

Abstract Type: Poster Presentation

Topic: Bone marrow failure syndromes incl. PNH - Clinical

Background:

Paroxysmal nocturnal hemoglobinuria (PNH) is a rare, life-threatening, complement mediated hematological disorder, with an estimated prevalence of 1/80,000 in France. Aside from hematopoietic stem cell transplantation, PNH therapy is centered on anti-C5 antibodies (i.e., eculizumab and ravulizumab) and proximal complement inhibition approach (pegcetacoplan, single anti-C3 approved so far), along with iterative red blood cells (RBC) transfusions. French data on PNH epidemiology and management in a real-world setting are scarce in the era of complement inhibition

Aims:

The main objectives of this study were to describe PNH epidemiology and patients' characteristics, and the therapeutic pathways of patients treated with C5 inhibitors in real word settings in France

Methods:

A real-world, national, descriptive study was carried out using secondary data from the French national hospital database (PMSI). Patients with ≥1 diagnosis of PNH (i.e., main or associated diagnosis, with or without C5 inhibitor dispensation) based on the ICD-10 code D59.5 between January 1, 2018, and December 31, 2022, were selected. Patients with other diseases treated with a C5 inhibitor were excluded (hemolytic uremic syndrome, myasthenia gravis and neuromyelitis optica). The first PNH-related hospitalization identified within the study period was considered as the index date. Epidemiology and patients' characteristics were described at the index date according to prevalence status and C5 inhibitor treatment status. Medical history was assessed over a historical period from January 1, 2013. Patients treated with a C5 inhibitor were followed from index date until last hospital stay for treatment management description; a subgroup of patients initiating C5 inhibitor treatment with ≥6 months of follow-up available was also studied. Treatments were identified using the ATC codes (Figure 1).

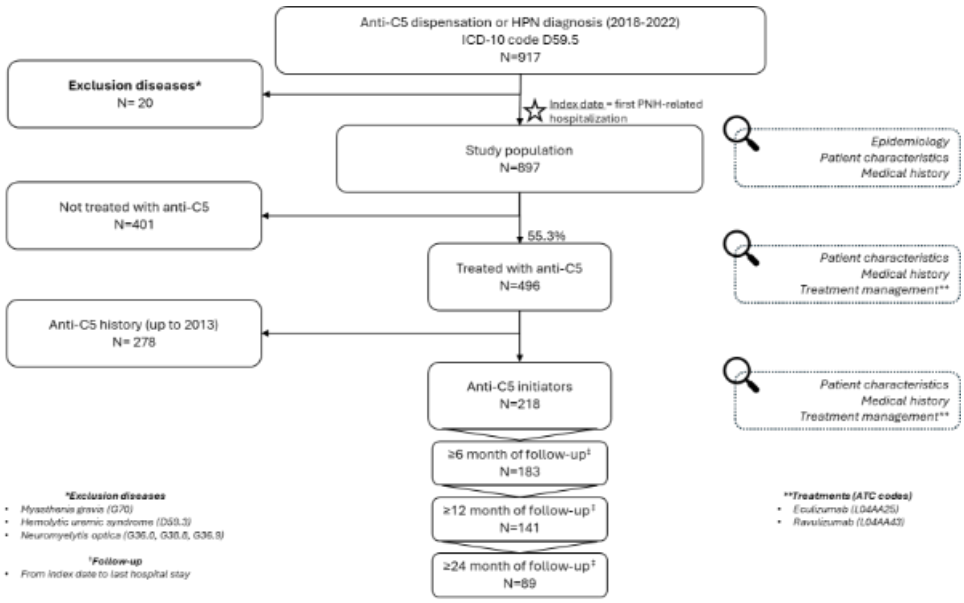
Results:

Among the 917 patients with a PNH diagnosis between 2018 and 2022, 20 (2.2%) patients had exclusion diseases; leaving 897 (97.8%) included in the study population. More than half (n= 496, 55.3%) were treated with a C5 inhibitor, of whom 218 (24.3%) initiated a C5 inhibitor during the selection period, and 183 (20.4%) had ≥6 months of follow-up. Each year, 100 new patients were identified on average, leading to a prevalence increasing from 581 patients in 2018 to 725 in 2022. Mean (SD) age at index date of study population was 52.4 (21.1) years, with 51.4% of women. Hepatic failure (8.7%) and diabetes (8.7%) were the most frequent comorbidities. At C5 inhibitor initiation, mean age was 45.7 (19.4) years, with 6.9% of patients presenting with hepatic failure and 6.9% diabetes. Among C5 inhibitor-initiators with ≥6 months of follow-up (n=183), the mean (SD) annual number of stays for C5 inhibitor infusion was 20.9 (6.9). When considering the type of C5 inhibitor dispensed, 22 (10.1%) patients initiated with ravulizumab, and 196 (89.9%) with eculizumab, of whom 80 (40.8%) switched to ravulizumab in 2022.

Summary/Conclusion:

More than 700 PNH patients were identified in 2022, with 100 newly diagnosed patients, leading to a prevalence of 1 case per 100,000 persons in France. This study brings updated data on the current epidemiology in France and management of PNH which has a high treatment burden due to frequent

infusions.



Keywords: Treatment, Monoclonal antibody, Real world data, Paroxysmal nocturnal hemoglobinuria (PNH)