Abstract: P2255

Title: EVALUATION OF HEREDITARY FRUCTOSE TOLERANCE IN PREVALENT PATIENTS WITH PAROXYSMAL NOCTURNAL HEMOGLOBINURIA, HEMOLYTIC UREMIC SYNDROME, OR MYASTHENIA GRAVIS

Abstract Type: e-Poster Presentation

Topic: Bleeding disorders (congenital and acquired)

Background:

Eculizumab therapy is the gold standard for complement-driven diseases: paroxysmal nocturnal hemoglobinuria (PNH), hemolytic uremic syndrome (HUS) and myasthenia gravis (MG). Biosimilar manufacturers may develop alternative formulations without impacting the biosimilarity to the originator in terms of clinical efficacy, safety, and immunogenicity. Inclusion of sorbitol in ABP 959, an eculizumab biosimilar, enhances the stability of ABP 959 when frozen, but may raise concern for patients with hereditary fructose intolerance (HFI) who cannot metabolize sorbitol. Data on the likelihood of a patient to have both HFI and an eculizumab-indicated condition will facilitate appropriate treatment decision making for patients and healthcare providers.

Aims:

To evaluate the prevalence of hereditary fructose intolerance (HFI) in eculizumab-indicated patient populations.

Methods:

Using multiple data sources including US administrative claims databases (MarketScan Commercial and Medicare, Optum Clinformatics Data Mart), German Disease Analyzer (GDA) electronic medical record (EMR) data, and the UK Clinical Practice Research Datalink (CPRD) Gold EMR dataset, we retrospectively evaluated PNH, HUS and MG cohorts and screened for patients with HFI. Diagnosis of PNH, HUS, MG, and HFI was determined using the International Classification of Diseases (ICD)-9/10 diagnosis codes for US datasets, ICD-10 for GDA data, and Read codes in the UK CPRD data.

Results:

Over 250 million patients across databases (MarketScan, coverage of 1996-2023: n=134,279,963; Optum, coverage of 2000-2023: n=97,295,000; GDA, coverage of 2012-2022, n=16,966,659; CPRD, coverage of 1987-2023: n=21,210,719) were analyzed. Prevalent patients included 2,412 (18.0 per million), 1,908 (19.6 per million), 58 (3.4 per million), and 126 (5.9 per million) for PNH; 3,949 (29.4 per million), 3,320 (34.1 per million), 117 (6.9 per million), and 1,129 (53.2 per million) for HUS; and 37,395 (278.5 per million), 38,644 (397.2 per million), 3,065 (181 per million), and 5,608 (264 per million) for MG in the MarketScan, Optum, GDA, and CPRD database, respectively. Among those patients with PNH, HUS, or MG across databases, 1 in 4,504 (0.022%) patients with PNH, none in 8,515 patients with HUS, and 9 in 84,712 (0.011%) with MG were also diagnosed with HFI.

Summary/Conclusion:

By analyzing decades of patient medical records and claims data from the US, Germany and the UK, the likelihood for a patient of having both HFI and eculizumab indicated conditions is very low. If HFI is suspected, a detailed symptom history should be collected prior to initiating treatment with biosimilar ABP 959.

Keywords: Metabolic syndrome, Incidence, Complement, Paroxysmal nocturnal hemoglobinuria (PNH)