Abstract: PB2660

Title: TREATMENT BURDEN OF PNH PATIENTS IN JAPAN: RESULTS OF THE QUALITATIVE INTERVIEW SURVEY STUDY

Abstract Type: Publication Only

Topic: Bone marrow failure syndromes incl. PNH - Clinical

Background:

Paroxysmal nocturnal hemoglobinuria (PNH) is a life-threatening, clonal hematologic disease with a global prevalence of 15.9 individuals per million. In Japan, more than 1000 individuals are diagnosed with PNH. The prognosis of PNH has been improved since C5 inhibitors became available for the treatment of PNH, and currently, C5 inhibitors are established as a standard of care. Considering that PNH is a life-long disease and treatment options are currently expanding, the future treatment goal is focused on not only PNH management but also on meeting the needs of patients' daily lives and life stages. Here, we report a qualitative analysis of interviews with 28 patients with PNH in Japan to understand their treatment burden and preferences. To our best knowledge, this is the first study in Japan to grasp the treatment burden in patients with PNH using interviews.

Aims:

The objective of this study is to understand the burden of PNH symptoms and treatment and their impact on daily life as well as potential factors influencing treatment preferences.

Methods:

A qualitative interview survey was conducted among PNH patients in Japan using a semi-structured interview guide developed based on a literature review and expert opinion. The responses were classified into spontaneous responses or responses to follow-up probing with hypothetical examples provided. Treatment preferences and the rationale behind them were surveyed using hypothetical treatment options. Conversational contents were recorded and verbatim records were created. Qualitative analysis (item coding, categorization, and classification of the causal relationships) of the verbatim records was performed.

Results:

Interview data of 28 patients were analyzed. The median age of the patients was 48.5 years (17-81) and 14 patients (50.0 %) were female. The median time from PNH diagnosis to enrollment was 8.6 years (n=24, 0.9 – 31). Treatments utilized at enrollment included eculizumab for 3 patients (10.7 %) and ravulizumab for 16 patients (57.1 %). 19 patients (67.9 %) had either full or part-time jobs. The median time to visit the hospital was 1 hour (0.3 – 3.5) and 14 patients (50.0 %) were required to take a day off from work or school for hospital visits. The frequency of visits to the hospital was every 2 weeks for 2 patients (7.1 %), every 4 weeks for 13 patients (46.4 %), every 8 weeks for 12 patients (42.9 %), less than every 8 weeks for 1 patient (3.6 %), respectively. Frequently reported burdens of PNH treatment were financial concerns about treatment (21 patients, 75.0 %), waiting time at a hospital (13 patients, 46.4 %), and time to spend on medical care (consultation, diagnosis, and treatment) (10 patients, 35.7 %) (Figure 1). Furthermore, analysis of treatment preference suggested that the preferences were related to the impact on daily life, personal values, and social attributes.

Summary/Conclusion:

This qualitative interview survey study identified PNH treatment burdens beyond the efficacy and safety of PNH treatments. Understanding the treatment burden and factors influencing treatment preference are vital for shared decision-making with patients during PNH management and will become especially important as treatments advance. Further studies with large populations are needed to confirm these findings.

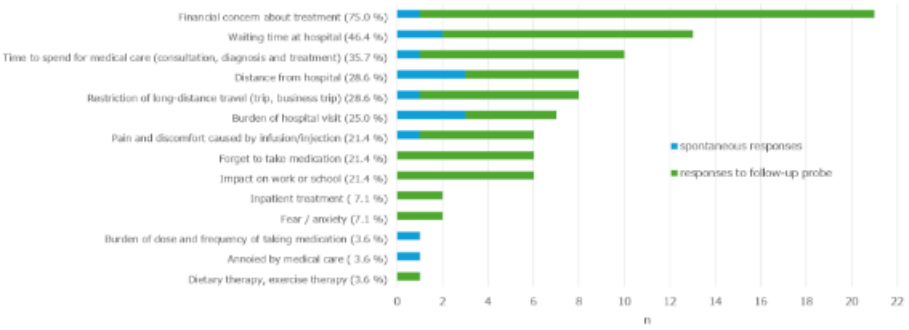

Figure1. PNH treatment burden patients experienced (multiple answers were allowed)

Keywords: Patient, Paroxysmal nocturnal hemoglobinuria (PNH), Treatment