Abstract: P1918

Title: PATIENT EXPERIENCE OF IPTACOPAN IN THREE CLINICAL TRIALS FOR PAROXYSMAL NOCTURNAL HEMOGLOBINURIA

Abstract Type: e-Poster Presentation

Topic: Bone marrow failure syndromes incl. PNH - Clinical

Background:

Paroxysmal nocturnal hemoglobinuria (PNH) is an acquired, clonal hematological disorder characterized by intravascular hemolysis, thrombosis, and bone marrow failure. Along with smooth muscle dystonia and hemoglobinuria, symptoms of PNH also include chronic and severe fatigue which significantly impacts patients' health-related quality of life. Data suggests a substantial proportion of patients on intravenous anti-C5 antibody therapies do not achieve normal or near normal hemoglobin levels. Iptacopan is a novel orally-administered proximal complement inhibitor treatment for PNH, that has demonstrated efficacy and safety in two Phase 3 clinical trials, APPLY-PNH (NCT0482053019) and APPOINT-PNH (NCT0455891820), and is being further explored in a roll-over extension (NCT04747613).

Aims:

Qualitative in-trial interviews were conducted with a subset of patients involving diverse populations with varying cultural backgrounds and languages in the three clinical trials to better understand changes in PNH symptoms they experienced since beginning treatment with iptacopan, and their perceptions of and preference for iptacopan.

Methods:

Clinical sites in eight countries, the United States, United Kingdom, China, France, Germany, Italy, Japan, and Spain, obtained informed consent from a total of 61 PNH patients enrolled in the three clinical trials to take part in the qualitative in-trial interviews. Interviewers trained in qualitative research techniques conducted audio-recorded telephone interviews with patients in their local language using a semi-structured interview guide designed to capture spontaneous and probed reports from patients about their treatment experience. Audio recordings were transcribed verbatim, translated into English (if applicable), and anonymized. Transcripts were coded and analyzed using qualitative data analytic methods.

Results:

In describing changes to their PNH symptoms, ≥90.0% of interviewed patients across the three trials who experienced PNH symptoms prior to their treatment with iptacopan reported that their symptoms improved with treatment (see Table 1). In describing their perceptions of iptacopan, all 22 patients in the roll-over extension trial who completed interviews indicated that they were either very satisfied (n=14, 63.6%) or satisfied (n=8, 36.4%) with iptacopan; and all 18 patients who were asked reported that they preferred iptacopan to their prior anti-C5 PNH treatments, with its oral administration and reduction in symptoms being key preference points. These findings were consistent with interviews conducted with patients in APPLY-PNH and APPOINT-PNH, who most frequently identified the convenience of iptacopan's oral administration and its efficacy as aspects they liked about the treatment.

Summary/Conclusion:

These qualitative in-trial interviews provide valuable patient reported insights on PNH symptoms and treatment experience. Moreover, the findings from this research demonstrate that patients experienced meaningful improvement in PNH symptoms, particularly those related to fatigue, and that patients reported high levels of satisfaction with, and a preference for iptacopan due to its oral administration, convenience, and efficacy.

Table 1. Patient-reported improvement in PNH symptoms

Symptom	Patients reporting symptom before iptacopan	Patients reporting symptom improved after iptacopan
Fatigue	n=41	n=40 (97.6%)
Tiredness	n=38	n=37 (97.4%)
Weakness	n=31	n=30 (96.8%)
Low energy	n=27	n=27 (100.0%)
Listlessness	n=23	n=23 (100.0%)
Shortness of breath	n=10	n=9 (90.0%)
Yellow/pale skin/eyes	n=6	n=6 (100.0%)

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