Patient and physician satisfaction of treatment with eculizumab or ravulizumab in paroxysmal nocturnal hemoglobinuria: results from a real-world Disease-Specific Programme

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INTRODUCTION

- Complement component 5 (C5) inhibitors eculizumab and ravulizumab are the standard of care in patients with paroxysmal nocturnal hemoglobinuria (PNH).
- Despite the wealth of clinical trial data highlighting complete and sustained inhibition of C5, symptom relief and improved quality of life in patients with PNH treated with eculizumab and ravulizumab, there is a lack of supporting real-world data describing patient and physician satisfaction with these treatments.
- Understanding patient satisfaction with C5 inhibitor treatment is important to ensure a patient's PNH health-related needs are fulfilled.

OBJECTIVE

• To describe physician-reported and self-reported patient satisfaction with eculizumab or ravulizumab for the treatment of PNH using real-world patient-level data generated using the PNH Adelphi Disease-Specific Programme (DSP)™.

CONCLUSIONS

- In the real-world setting, most physicians and patients were satisfied with eculizumab and ravulizumab for the treatment of PNH.
- Most patients and physicians were 'more satisfied' with ravulizumab treatment following switching from previous treatment with eculizumab (81.8% and 71.4%, respectively).
- These results demonstrate real-world perceptions and experiences of physicians who treat PNH and of patients with PNH treated with eculizumab or ravulizumab, and may improve the treatment decision process.
- Aside from the need for less infusions, common reasons for preference to ravulizumab over eculizumab included improved disease and overall symptom control, reduced patient fatigue and improved patient quality of life.

METHODS

- The PNH Adelphi DSP™ is a point-in-time survey of hematologists and oncologists, and their consulting patients with PNH, conducted in eleven countries between December 2021 and July 2022 (**Figure 1**).
- Physicians participated in a comprehensive clinical survey and provided data on their patients' demographics, symptoms, laboratory values and disease control, and satisfaction with eculizumab or ravulizumab.
- At the time of the clinical consultation, consenting patients provided self-reported data on their satisfaction with eculizumab or ravulizumab.

RESULTS

- Data were reported for 441 patients receiving eculizumab (n = 278) or ravulizumab (n = 163) (Table 1).
- Matched physician-reported and patient self-reported data were available for 164 patients.
- Overall, 92.3% of physicians and 86.8% of patients were either completely satisfied or satisfied with eculizumab and ravulizumab treatment.
- Physician-reported and patient self-reported overall satisfaction with ravulizumab treatment are described in Figure 2.
- For 93.2% and 97.9% of the matched patients, their physician was either completely satisfied or satisfied with current eculizumab and ravulizumab treatment, respectively; the patient-reported satisfaction was 84.6% and 91.5%, respectively.
- For 90.0% and 96.6% of patients treated for over 12 months, their physician was satisfied with eculizumab and ravulizumab, respectively; patient-reported satisfaction was 86.0% and 93.1%, respectively (**Figure 3**).
- Of the 163 patients who received ravulizumab,
 56 had switched from previous eculizumab treatment.
- Physicians reported greater satisfaction with ravulizumab compared with eculizumab for 71.4% of patients (Figure 4).
 - The remaining 28.6% were equally satisfied.
- Neither physicians nor patients were less satisfied with ravulizumab treatment after switching from eculizumab (**Figure 4**).
- Reasons for greater physician-reported satisfaction with ravulizumab compared with eculizumab are described in **Figure 5**.
- In a subgroup of patients with aplastic anemia or another bone marrow disorder, for 85.1% and 90.4% of patients, their physicians were satisfied with eculizumab and ravulizumab, respectively (**Figure 6**).

Figure 1. Study design of the PNH Adelphi DSP™ Physician survey PHYSICIAN PRESENTING PATIENTS Completed by all enrolled physicians Physician perceptions Completed online by each physician **MATCHED** participating in the study (approximately 20 minutes) Patient record forms Patient/caregiver self-completion Physician inclusion criteria: Prospectively completed by physicians Completed by same patients or caregiver of patien Specialist in hematology or oncology Treats at least one patient with PNH Clinical patient data Patient/caregiver perspective Patient inclusion criteria: Diagnosis of PNH

Completed for their next eligible patients

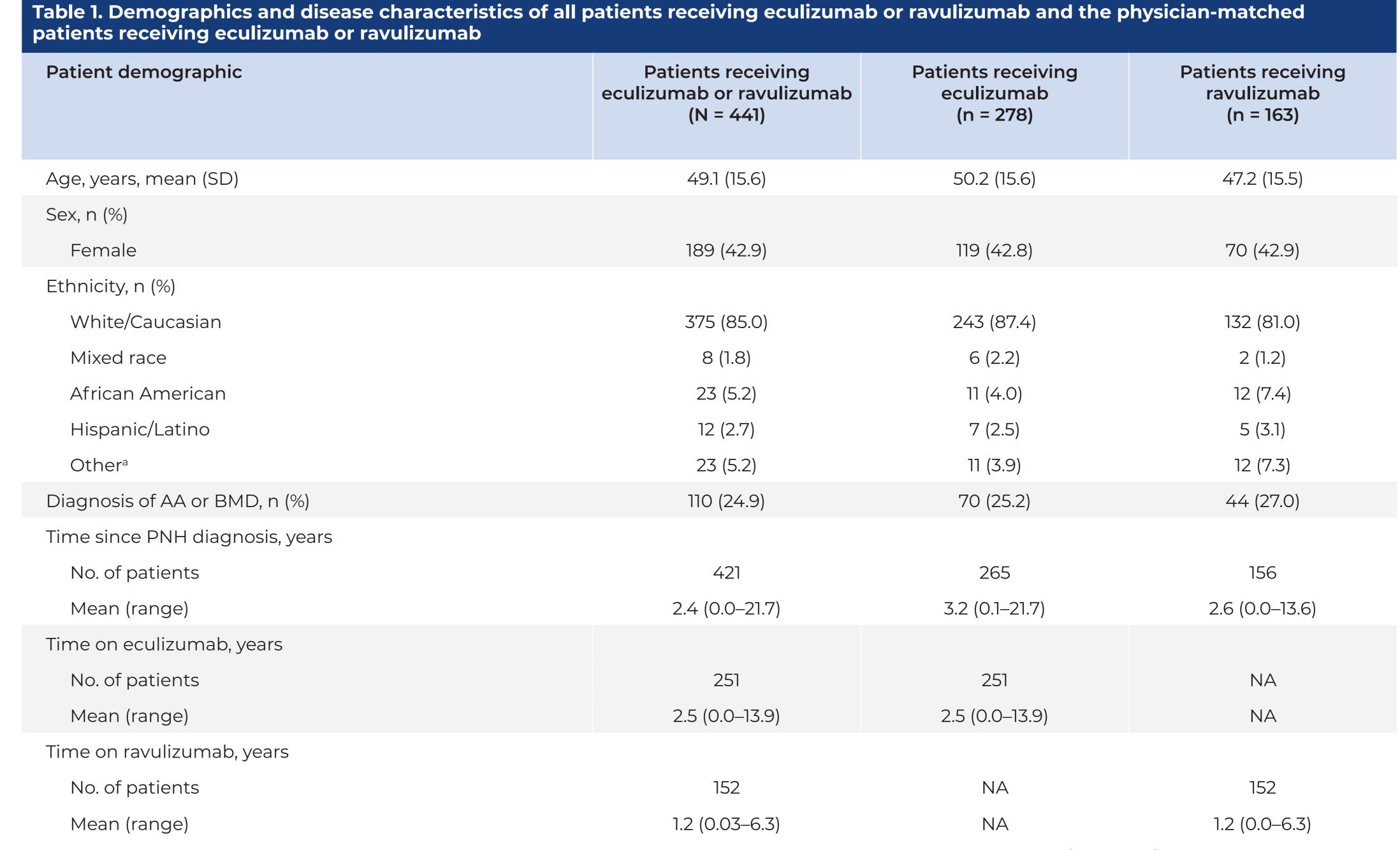
Location
Australia

Australia, Austria, Canada, France, Germany, Italy, Spain, UK and USA

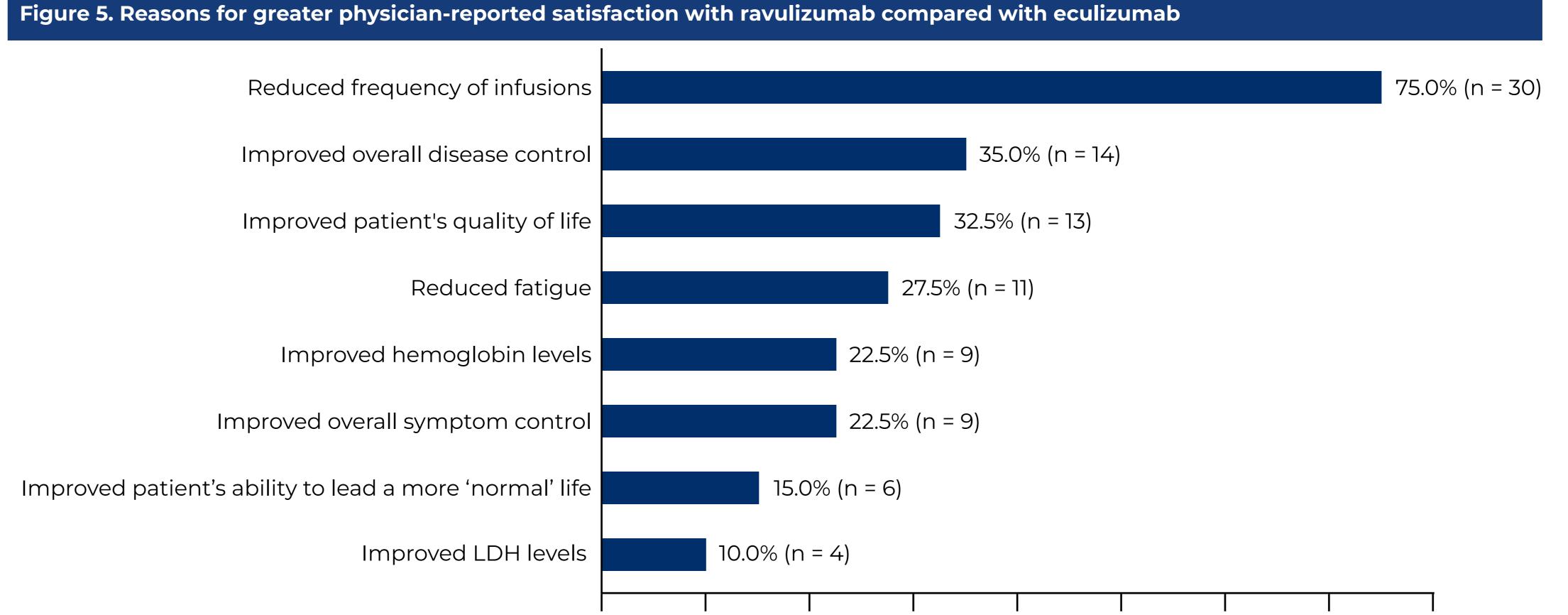
DSP, Disease-Specific Programme; PNH, paroxysmal nocturnal hemoglobinuria Adelphi DSP methodology: Anderson P et al. Curr Med Res Opin 2008;24:3063–72.

Data collection:

December 2021 – July 2022



^aOther ethnicities included: Native American (USA), Asian-Indian subcontinent, Asian – other, Middle Eastern, Afro-Caribbean (EU), South-East Asian, Melanesian/Micronesian/Polynesian and other.
AA, aplastic anemia; BMD, bone marrow disorder; NA, not applicable; PNH, paroxysmal nocturnal hemoglobinuria; SD, standard deviation.



Proportion of physicians (%)

Reported in ≥ 10% of physicians. LDH, lactate dehydrogenase.

Poster PCR187



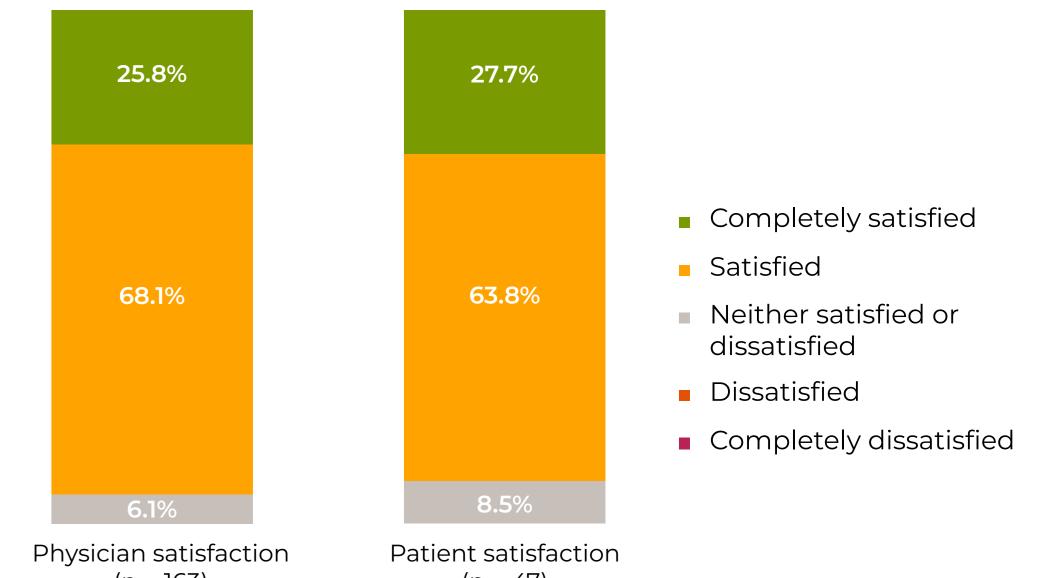


Figure 3. Matched physician- and patient-reported satisfaction with ravulizumab for those receiving treatment for more than 12 months

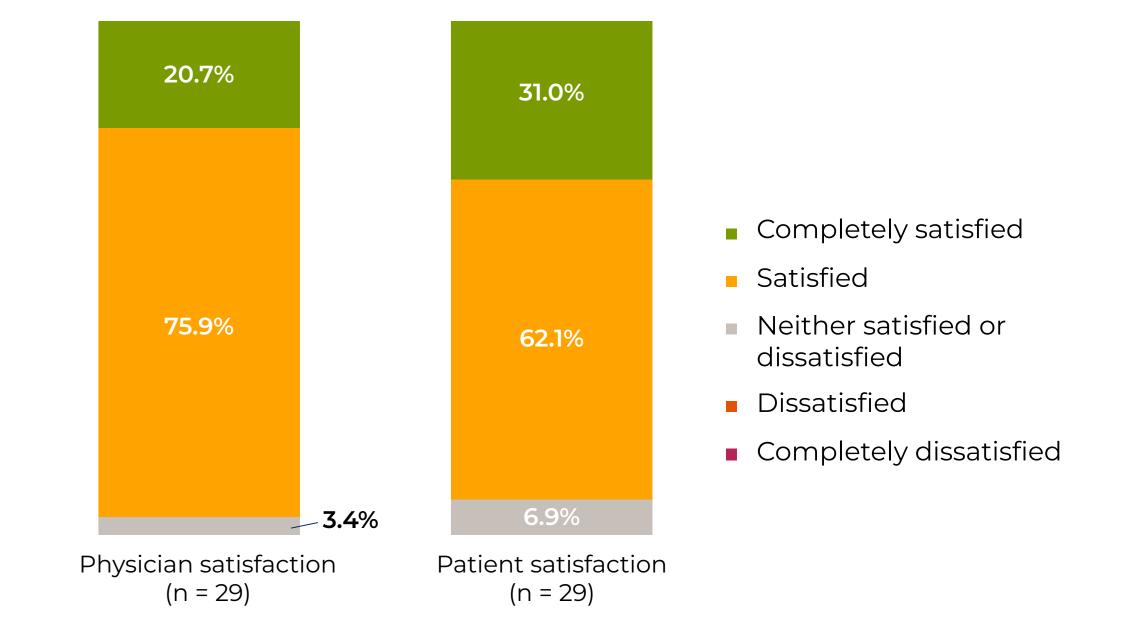


Figure 4. Physician-reported and patient self-reported satisfaction with ravulizumab compared with eculizumab after switching

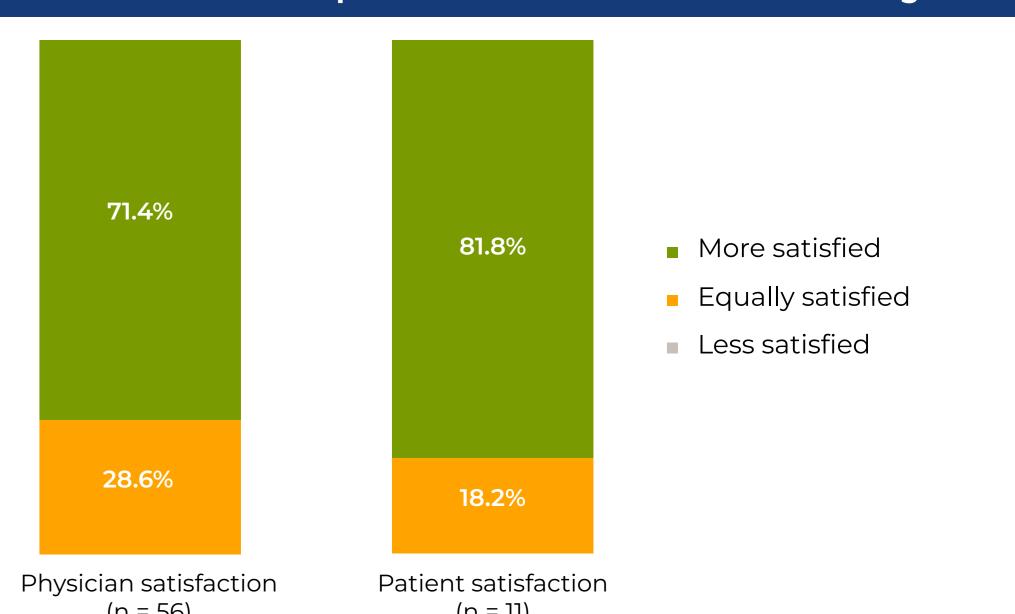
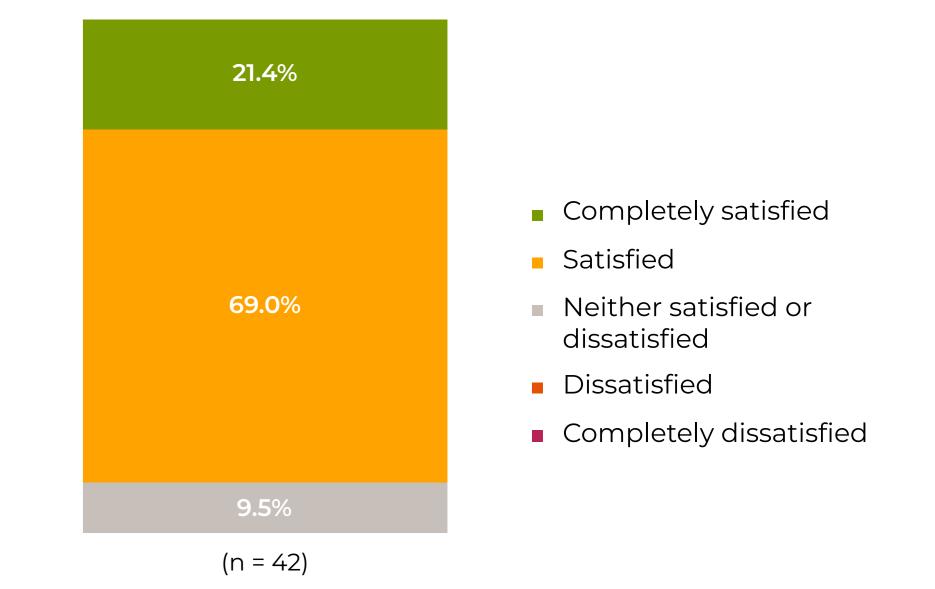


Figure 6. Physician-reported patient satisfaction with ravulizumal treatment in patients with PNH and concomitant aplastic anemia or another bone marrow disorder



PNH, paroxysmal nocturnal hemoglobinuria

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Disclosures

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