Poster# **MDS-568**

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Development of APPRISE-PNH: A Patient Platform for Real-World Data on Iptacopan in the United States for Patients With Paroxysmal Nocturnal Hemoglobinuria

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KEY FINDINGS & CONCLUSIONS

- This poster describes the creation of APPRISE-PNH, a comprehensive real-world data platform on patients with paroxysmal nocturnal hemoglobinuria (PNH) treated with iptacopan in the United States (US)
- APPRISE-PNH constitutes a longitudinal set of deidentified patient-level data collected from multiple sources across the US
- Enrollment into APPRISE-PNH is currently ongoing. To date, 17 patients with PNH have enrolled
- This platform aims to serve as a data source to address multiple studies on unmet need, treatment patterns, and outcomes for patients treated with iptacopan in the US real-world setting

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This study is sponsored by Novartis Pharmaceuticals Corporation.
Poster presented at the SOHO 2024 Annual Meeting | September 4–7, 2024 | Houston, Texas, USA

INTRODUCTION

- Paroxysmal nocturnal hemoglobinuria (PNH) is a rare, acquired, life-threatening hematological disorder characterized by the premature destruction of red blood cells[1]
  - Between 2018-2022, the 5-year prevalence of PNH was 24 people per million in the United States (US)[2]
- The standard of care for PNH has traditionally been centered around blood transfusions and anticoagulant therapy to manage symptoms and prevent complications[3]
  - The introduction of targeted therapies has transformed the management of PNH, significantly improving both quality of life and survival[3]
- Iptacopan, the first orally administered monotherapy for PNH, received US Food and Drug Administration (FDA) approval in December 2023[4]
- Several real-world data sources on US patients with PNH currently exist; however, they have various limitations including limited granularity on laboratory and treatment information[5]

OBJECTIVE

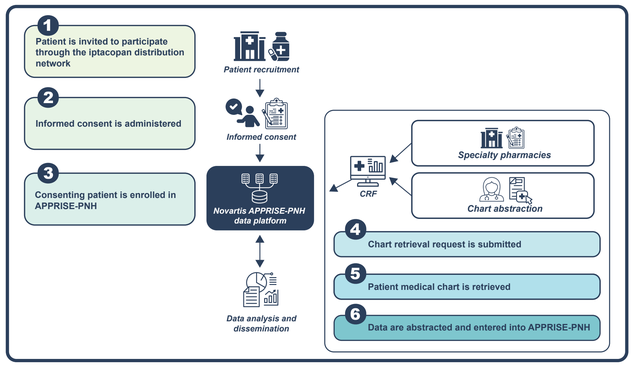
- The evolving PNH treatment landscape necessitates the generation of up-to-date real-world evidence on the effectiveness of emerging therapies, such as iptacopan, to inform clinical decision-making
- This poster describes the creation of APPRISE-PNH *(*A Patient Platform for Real-world data on Iptacopan in the United StatEs for patients with PNH)
  - APPRISE-PNH is a comprehensive real-world data platform on patients with PNH in the US treated with iptacopan

STUDY DESIGN

APPRISE-PNH Development

- APPRISE-PNH is a longitudinal set of deidentified patient-level data collected from multiple sources, including pharmacy and medical records, across the US
- Patients are recruited through the iptacopan distribution network (**Figure 1**)
  - Study participants will be required to provide written consent prior to enrollment
  - Participants are free to withdraw from the study at any time

Figure 1. Study design for APPRISE-PNH



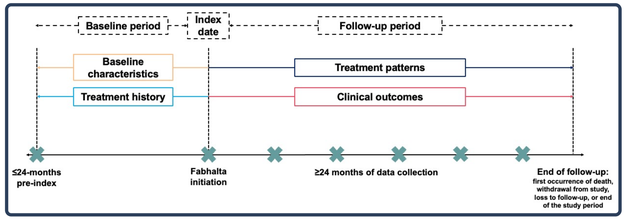
Study Population

- The population of interest is adult (aged ≥18 years) patients with PNH treated with iptacopan in routine clinical care in the US real-world setting
  - Study participation will not impact medical care or treatment in any way; all patients will have initiated treatment with iptacopan prior to recruitment
- APPRISE-PNH will include both patients who have previously received treatment for PNH and patients who are treatment-naïve prior to iptacopan initiation

Study Period

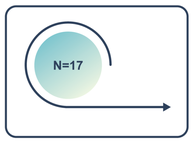
- The study period will begin at the time of iptacopan initiation for the first patient and will continue through to the end of follow-up for the last patient (**Figure 2**)
  - The index date will be the date of iptacopan initiation for each patient
  - Baseline information, including medical and treatment history, will be extracted during the pre-index period
  - Patients will be followed from the date of enrollment until death, withdrawal of consent, loss to follow-up, or the end of the study period, whichever comes first

Figure 2. APPRISE-PNH data capture



PARTICIPANT RECRUITMENT

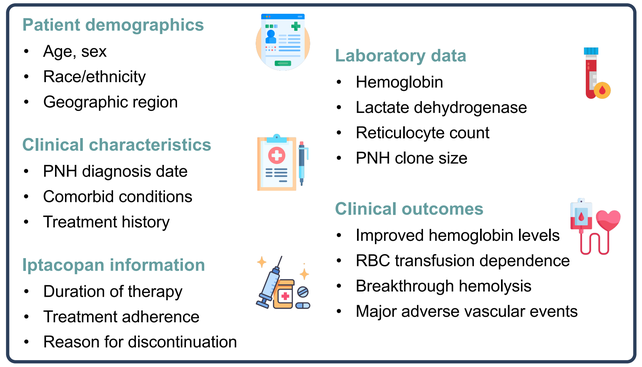
- Enrollment into APPRISE-PNH is currently ongoing
- In the first month since the platform launched, 17 patients have been enrolled in APPRISE-PNH



DATA ELEMENTS

- Data will be sourced directly from patient medical records (**Figure 3**)
  - Iptacopan dosing information will be sourced from specialty pharmacy records
  - Information on demographics, medical history, disease characteristics, laboratory results, treatment patterns, and clinical outcomes will be sourced from medical charts
- Only de-identified data will be included in APPRISE-PNH

Figure 3. Example of data elements included in APPRISE-PNH



DISCUSSION

- APPRISE-PNH will allow for rapid and direct access to a range of real-world patient data from the time of iptacopan FDA approval (December 2023)
  - Sourcing treatment data directly from specialty pharmacies will provide the most accurate and comprehensive understanding of real-world iptacopan use
- Given the rarity of PNH and the clinical potential of iptacopan[6], there is a need for timely real-world evidence gathering to inform treatment decision-making
- This new data source will help to provide timely insights into the characteristics, treatment patterns, and outcomes of patients with PNH receiving this novel therapy in the real-world setting

CONCLUSIONS

- APPRISE-PNH aims to be the largest and most comprehensive real-world data source on patients with PNH treated with iptacopan in the US
- This platform aims to serve as a data source to address multiple studies on unmet need, treatment patterns, and outcomes for patients treated with iptacopan in the US real-world setting

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Disclosures

Drs. DD and AW have received funding from Novartis Pharmaceuticals Corporation. GY, NK, JP, MC, and SL are employees of Novartis Pharmaceuticals Corporation. TB, CB, and JT are consultants engaged by Novartis Pharmaceuticals Corporation to conduct this study. JB is an employee of Onco360 Oncology Pharmacy who is engaged by Novartis Pharmaceuticals Corporation to conduct this study.