TH-PO617

Design and Rationale for PERFORM Patient Registry™: An Observational Cohort of Patients Utilizing TARPEYO® for Immunoglobulin A Nephropathy (IgAN) in the United States

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INTRODUCTION

- Immunoglobulin A Nephropathy (IgAN) is a rare, chronic autoimmune disease characterized by the deposition of galactose-deficient IgA1 in the kidneys[1-4]
- Nefecon is the first treatment approved by the FDA and EMA for patients with IgAN. It is marketed by Calliditas in the US under the brand name TARPEYO®, and by STADA Arzneimittel AG in the European Economic Area (EEA), Switzerland and the UK under the brand name Kinpeygo®[5]
- Given the rarity and progressiveness of IgAN, an understanding of real-world treatment patterns and patient outcomes for patients taking TARPEYO in the US is being assessed in the PERFORM Patient Registry™
- The full scope of this registry went live in June 2023

AIM OF THIS REGISTRY

- To understand the real-world experience of patients with IgAN and receiving TARPEYO as part of routine clinical care

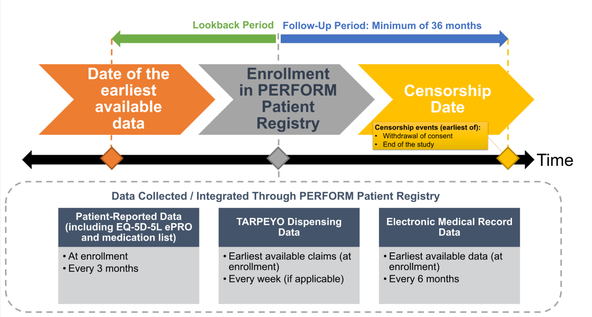
Methods

Study Design Overview:

- The PERFORM Patient Registry™ is a novel, observational, direct-to-patient web-based prospective study to collect longitudinal bi-directional data (retrospective and prospective) for eligible participants **(Figure 1)**
- This registry is enabled by the IQVIA Health Research Space (HRS) study application (formerly Integrated Health Platform [IHP]) which is described in **Figure 2**
- Adult patients who meet the enrollment eligibility criteria will self-enroll in the direct-to-patient registry online via the IQVIA HRS study application accessible on a computer, tablet or smartphone
- This registry aims to enroll 360 participants and participants will be followed for up to 36 months
- The key data variables of interest are summarized in **Table 1**

METHODS (CONT.)

Figure 1. Registry Design Overview



Enrollment Eligibility Criteria:

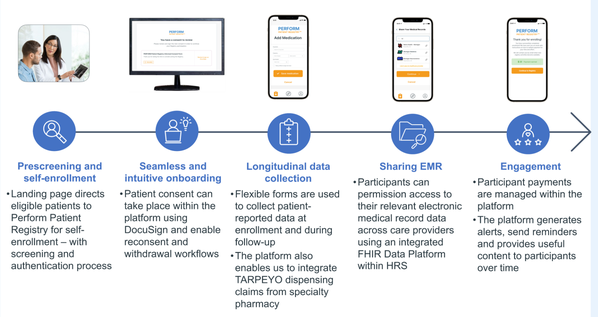
- Age ≥18 years
- Had at least one dispensed prescription of TARPEYO
- Willing and able to provide eConsent to participate in the registry and provision registry access to TARPEYO dispensing claims and electronic medical record data

Table 1. Key Data Variables of Interest (if available)

Demographics
Participation in clinical trial(s) for Nefecon
Age at diagnosis of IgAN
Age at TARPEYO treatment initiation
IgAN Diagnosis Features & Biopsy Findings
Surgical and Medical History
Comorbidities / Diagnoses / Conditions
Medication Use including TARPEYO
Laboratory Results
Health Care Resource Utilization
HRQoL (EQ-5D-5L)
Measurements (e.g., weight, height, vitals)
Date of Censorship

METHODS (CONT.)

Figure 2. Study Application: Health Research Space (HRS)



Research Topics (include but are not limited to):

- The treatment utilization patterns for TARPEYO among patients with IgAN, including dose and dose changes, frequency of administration, duration of use, adherence, discontinuation, and treatment switch or change in treatment regimen
- The clinical management of disease of patients living with IgAN and receiving TARPEYO
- HRQoL among registry patients living with IgAN and receiving TARPEYO
- Additional analyses to gain an understanding of the qualitative and quantitative nature of the data collected

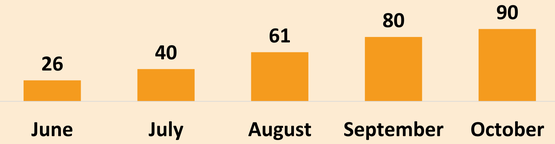
Statistical Analyses:

- Descriptive analyses will be conducted by calculating means (standard deviations) and medians (interquartile ranges) for continuous variables, and numbers with proportions for categorical variables
- Analyses will be performed for patients overall and by subgroups of interest (e.g., stratified by baseline values of the following variables: age, sex, race, IgAN disease duration ≥10 years, CKD stage at first dose of TARPEYO)

ENROLLMENT STATUS

- Participant recruitment is ongoing
- As of October 9th , 2023, a total of 90 patients were enrolled in the registry (**Figure 3**)

Figure 3. Cumulative Number of Enrolled Patients in 2023**



**Data is reported as of the last Monday of each month (June 26th, July 31st, August 28th, and September 25th); October data represents partial data as of October 9th, 2023

CONCLUSIONS

- This is **the first direct-to-patient real-world registry** of patients with IgAN utilizing TARPEYO
- The direct-to-patient approach **enables analysis of the patient journey** including EMR data sharing by connecting journey metadata to provisioning events
- Data collected in this Registry will be **analyzed and disseminated at future nephrology conferences**

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ABBREVIATIONS

CKD, chronic kidney disease; EMA, European Medicines Agency; EMR, electronic medical records; ePRO, electronic patient-reported outcome; EQ-5D-5L, EuroQol 5 Dimension 5 Level; FDA, Food and Drug Administration; FHIR, Fast Healthcare Interoperability Resources; HRQoL, health-related quality of life; HRS, Health Research Space; IgA1, Immunoglobulin A1; IgAN, Immunoglobulin A Nephropathy; UK, United Kingdom; US, United States.

DISCLOSURES

MP and WB are employees of Calliditas, the sponsor of the registry. DZ, NL, IB, and EK are employees of IQVIA, which manages the registry. All authors declare no conflicts of interests.

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