Therapeutic Management and Healthcare Resource Use (HCRU) of Patients with Paroxysmal NocturnalHemoglobinuria (PNH) in France between 2018 and 2022

Results of the Hogan Study

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Introduction

Paroxysmal nocturnal hemoglobinuria (PNH) is an acquired clonal disease of hematopoietic stem cells that induce hemolytical anemia and thrombosis.The availability of C5 inhibitors (anti-C5) has considerably improved patients' overall survival and quality of life.

The aim of this study is to describe hospital management and HCRU of patients with PNH in France.

Methods

Data sources

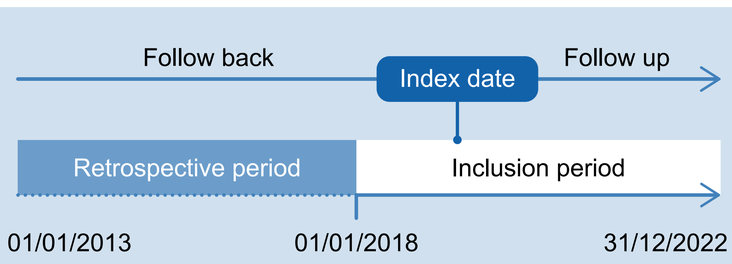
This study is based on hospital reimbursement data available from the Programme de Médicalisation des Systèmes d'Information (PMSI).

Study period

The **index date** is the date of the 1st hospitalization for PNH or the date of the 1st anti-C5 dispensation during the inclusion period.

The **retrospective period** is used for medical history-taking and confirmation of the incident status of PNH patients.

The **end of follow-up** corresponds to the date of death of the patient or the end of the study.



Study population

Total PNH: All patients with at least one hospitalization for PNH (full or partial hospitalization) during the inclusion period Cohort (2018-2022) will be included in this study.

subgroup definition

Patients exposed to anti-C5 drugs: Patients with at least one dispencing of anti-C5 drugs during the extraction period.

Newly exposed: patient not exposed to anti-C5 during the retrospective period.

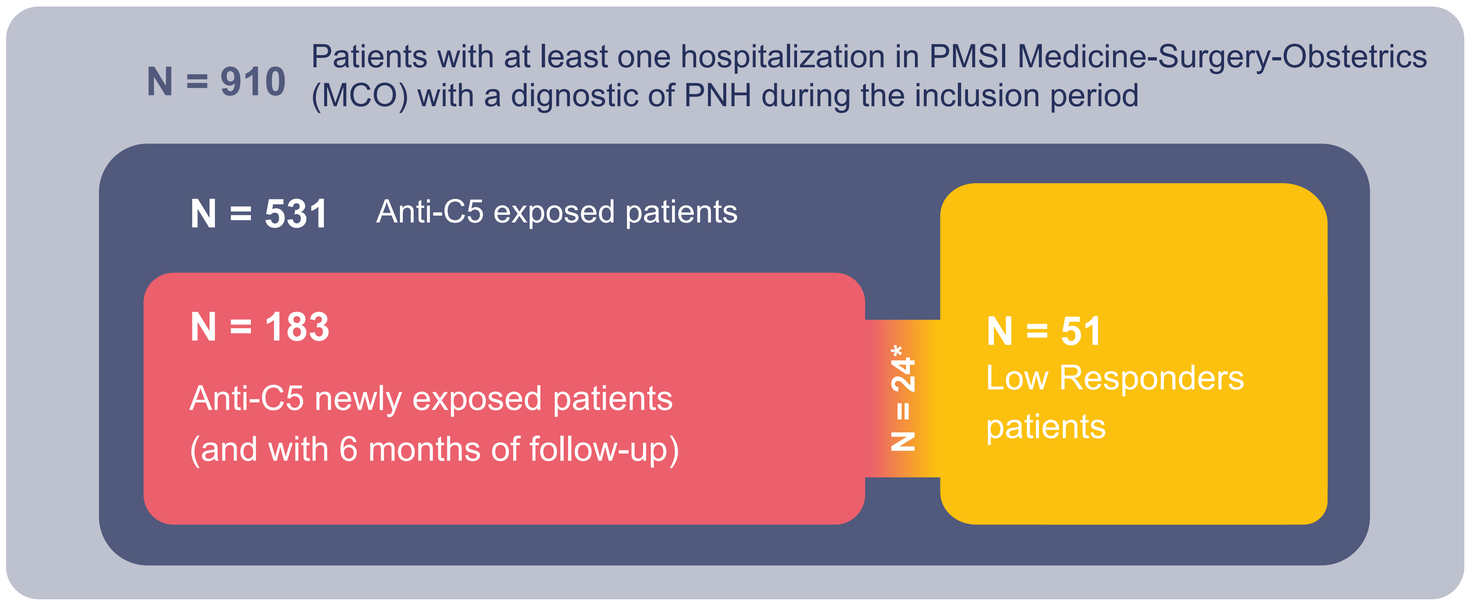
Low Responders: Patients exposed to an anti-C5 who have had either a switch to an anti-C3 (outside clinical trials), or an increase in the number of transfusions: at least 2 consecutive transfusions over a one-year period starting 6 months after initiation of anti-C5.

Conclusion / study limits

This study, based on real-life data from 2018 up to 2022, provides updated knowledge on the therapeutic management and HCRU for PNH patients in France. Selection bias was minimal as there is a specific ICD-10 diagnosis code for PNH which is used in the PMSI. However, the PMSI database does not contain medical data such as laboratory tests results or imaging procedures results. For this reason, algorithms were used, especially to identify individuals with low response to anti-C5.

Results

Study population



*Anti-C5 newly exposed & low responders patients

Healthcare resources use during the follow-up

Results are detailed:

- Since the beginning of exposure to anti-C5 for **newly exposed population**
- Since the date of the « low response » status identified for **low responders population**

N	183	51
Transfusions	**Newly exposed**	**Low responders**
Patients with transfusions	N=85 46.5%	N=50* 98.0%
Mean number of transfusions (all causes)	3.8 (± 9.0)	7.8 (± 9.3)
Patients with transfusions (PNH causes)	N=54 29.5%	N=50* 98.0%
Mean number of transfusions (PNH causes)	1.3 (± 4.1)	5.02 (± 6.8)

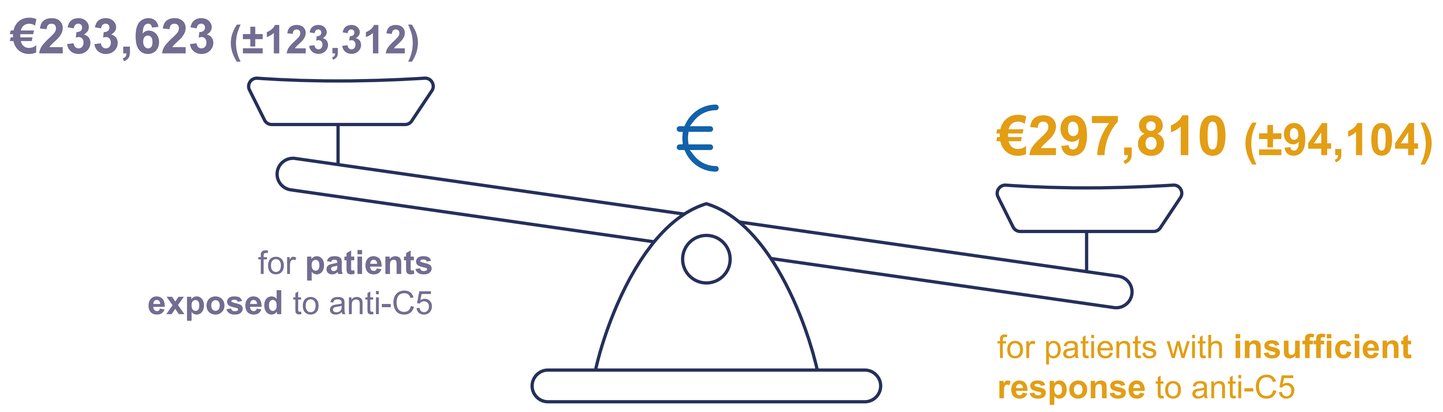
*patients requiring antiC3 were considered as low responder independantly of the numbers of transfusions.

Hospitalizations#	**Newly exposed**	**Low responders**
Hospitalizations (all causes)		
Number*	48.2 (± 32.7)	52.4 (± 38.4)
Length* (days)	1.4 (± 3.5)	1.4 (± 3.1)
Hospitalizations, without anti-C5 administrations		
Number*	8.5 (± 14.3)	17.1 (± 18.7)
Length* (days)	2.3 (± 5.95)	1.8 (± 4.1)
Hospitalizations, with anti-C5 administrations		
Number*	39.6 (± 29.6)	35.3 (± 32.0)
Length* (days)	1.2 (± 2.6)	1.3 (± 2.3)

*mean (+/- sd); #At least 75% of hospital admissions last 1 day or less

Costs

Average annualized cost of all hospitalizations for incident patients with PNH (including treatment costs)



Data sources

https://hevaweb.pages.inadvans.com/qualite-qms/general/Instructions/I-GEN_Creation_Poster/#2-production-du-contenu

PMSI databases supplied by ATIH; Data controller: Novartis; Data processor: Heva; Study registered under MR006 with the Health Data Hub on 08/09/2023 under N° F20230908142017