FAQ for the Global Paroxysmal Nocturnal Hemoglobinuria (PNH) Patient Registry Natural History Study

1. WHAT IS A PATIENT REGISTRY?

A patient registry is a collection of standardized information about a group of patients who share a condition and is used for a variety of purposes such as conducting natural history studies and supporting disease-specific clinical trial recruitment.

2. WHAT IS A NATURAL HISTORY STUDY?

A natural history study is a study designed to track the course of a disease over time and includes people who have a specific medical condition or disease and those who are at risk of developing such. This method of research explores the disease in a comprehensive way and identifies demographic, genetic, environmental and other variables that correlate with the disease and its outcomes. Natural history studies have many potential uses such as developing patient care best practices or may be used for clinical trial recruitment.

3. WHAT IS A RESEARCH STUDY SPONSOR*?

An individual, company, institution or organization that takes responsibility for choosing appropriately trained and experienced researchers as well as the initiation, management and/or financing of a clinical trial. The study sponsor ensures that the study is conducted in an ethical manner and upholds regulations as they apply to the study.

4. WHO IS THE APLASTIC ANEMIA AND MDS INTERNATIONAL FOUNDATION (AAMDSIF)?

AAMDSIF is the world's leading nonprofit health organization dedicated to supporting patients and families living with aplastic anemia, myelodysplastic syndrome (MDS), paroxysmal nocturnal hemoglobinuria (PNH), and related bone marrow failure diseases. The Foundation provides answers, support, and hope to thousands of patients and their families around the world.

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AAMDSIF is a patient-focused, patient-centered organization, serving patients and families throughout the three phases of bone marrow failure diseases; their life-changing phase of diagnosis, their life-threatening phase of treatment, and their life-long phase of living with a chronic disease.

5. WHO IS NORD – THE NATIONAL ORGANIZATION FOR RARE DISORDERS?

NORD is the leading independent advocacy organization representing the approximately 25-30 million Americans affected by a rare disease. NORD is committed to the identification, treatment and cure of the more than 7,000 rare diseases, of which approximately 90 percent are still without an FDA-approved treatment or therapy. Learn more about NORD at https://rarediseases.org/.

6. WHAT IS A PRINCIPAL INVESTIGATOR?

The Principal Investigator is the research group leader or, the person with the primary responsibility for the design and conduct of the research project or study.

7. WHAT IS AN INSTITUTIONAL REVIEW BOARD (IRB)?

Any board, committee or other group formally designated by an institution or investigator to review, approve the initiation of, and to conduct periodic review of research involving human subjects. The primary purpose of such review is to assure the protection of the rights and welfare of the human subjects. Also known as the Ethics Committee (EC).

8. WHAT IS THE PURPOSE OF THE GLOBAL PNH PATIENT REGISTRY?

One of the most important purposes of the Global PNH Patient Registry is to bring the PNH community together and collect data which could be used to create therapeutics and otherwise improve the quality of life for PNH patients. Some other goals of the Global PNH Patient Registry are to:

- Conduct a prospectively-planned natural history study that will result in the most comprehensive understanding of PNH and its progression over time.
- Characterize and describe the PNH population as a whole.
- Assist the PNH community with the development of recommendations for standards of care.
- Assist researchers studying the pathophysiology of PNH.
- Assist researchers studying interventional outcomes.
- Support the design of clinical trials for new treatments.

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9. WHAT TYPES OF DATA WILL BE COLLECTED IN THE GLOBAL PNH PATIENT REGISTRY?

The data collected is uniform and includes but is not limited to:

- Socio-demographics
- Medical and diagnostics
- Treatment and disease progression
- Management of care
- Quality of life

10. HOW IS THE DATA COLLECTED?

Data is collected through a secure web-based system developed by the National Organization for Rare Disorders, Inc. (NORD®), an independent non-profit committed to the identification, treatment, and cure of all 7,000 rare diseases. Study participants respond to questions grouped within a series of surveys developed per study standards and in collaboration with disease specific experts.

11. WHO IS A STUDY PARTICIPANT?

A study participant is the individual who takes part in a research study and whose information is collected for that research. Study participants may consent to enter and share their own personal data.

12. WHO IS A REPORTER/RESPONDENT?

A reporter/respondent is an individual who completes the surveys on behalf of the patient/study participant, when that individual is unable to do so on their own behalf.

13. WHAT IS A LEGALLY AUTHORIZED REPRESENTATIVE (LAR)?

An individual who is authorized under applicable law to consent, on behalf of a prospective subject, to the subject's participation in a clinical trial. The LAR may be a parent, grandparent, caregiver or guardian who has the legal authority to grant consent on behalf of another who is eligible to participate in research. When a LAR acts on behalf of a study participant, they are considered to be the reporter/respondent in the research.

14. WHAT IS AN INFORMED CONSENT?

The Office for Human Research Protections (OHRP) states that, "… the informed consent process is the critical communication link between the prospective human subject and an investigator beginning with the initial approach of an investigator to the potential subject

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(e.g. through a flyer, brochure, or any advertisement regarding the research study) and continuing until the completion of the research study. [...] The informed consent process involves three key features: (1) disclosing to potential research subjects' information needed to make an informed decision; (2) facilitating the understanding of what has been disclosed; and (3) promoting the voluntariness of the decision about whether or not to participate in the research."[1]

15. WHO CAN JOIN THE STUDY?

This study is open to anyone who has been diagnosed with PNH.

16. IS THERE A COST TO PARTICIPATE?

There is no cost for patients to join this study. AAMDSIF absorbs the cost of the registry for its community.

17. HOW LONG WILL THIS STUDY LAST?

This registry will be open for at least five years with the option to renew registration. There is no date of termination or closure at this time.

18. CAN DATA BE COLLECTED WORLDWIDE?

The patient registry uses an online platform which allows participants to contribute data from anywhere in the world. Individuals from other countries who enter data into the registry should be aware that data and privacy laws may be different in the United States from other countries. This US-based registry will protect data and privacy according to US requirements.

19. WHERE IS THE DATA STORED?

The data is stored on NORD's registry platform system which adheres to industry standards regarding security protections.

20. IS THE DATA SAFE?

Yes, the data is safe. The registry follows strict government guidelines to assure patient information is protected. The platform is served over HTTPS, which provides traffic encryptions so as to prevent eavesdropping and man-in-the-middle attacks. Communications between the registry platform application server and the database are also encrypted.

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21. WHO OWNS THE DATA?

The identifiable and de-identifiable data are owned by the study sponsor, AAMDSIF. AAMDSIF decides how and with whom to share the data. A subset of the pseudonymized data collected across the NORD Registry Platform is available to NORD to support cross disease analysis and advocacy activities to members of the rare disease community as a whole. Participants are able to withdraw from the study at any time, however, the researchers may still use the information that they have collected prior to changing your mind in order to complete the research that has already started. Information that has already been shared with the RDCA-DAP or sent to a researcher for a specific study prior to your request for removal cannot be retrieved or removed.

22. WHAT IS A REGISTRY ADVISORY BOARD?

A Registry Advisory Board committee, that may include scientists, doctors, and patient advocates, will be assembled to oversee the conduct of the study. The Advisory Board will review aggregate registry data and the use of this registry, ensure proper evaluation of protocols requesting to use registry data and/or contact registry participants, and will review any protocol or confidentiality deviations on a case-by-case basis and ensure that any such deviations are reported to the IRB.

23. HOW IS THE PATIENT REGISTRY MAINTAINED?

The registry is maintained by NORD who hosts the registry on its cloud-based platform and provides oversight and ongoing support of the system. AAMDSIF and NORD provide the day-to-day management of their patient registry, including the development and adherence to the study procedures.

**=The Global PNH Patient Registry is a collaborative effort between AAMDSIF and NORD, with support from industry partners, Apellis Pharmaceuticals, Inc., Genentech, Inc., and BioCryst Pharmaceuticals, Inc.*

[1]Informed Consent FAQs: https://www.hhs.gov/ohrp/regulations-and-policy/guidance/faq/informed-consent/index.html. Accessed Feb. 9, 2021.

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