FOR IMMEDIATE RELEASE

Aplastic Anemia and MDS International Foundation and NORD Launch New Natural History Study of Paroxysmal Nocturnal Hemoglobinuria (PNH)

The worldwide registry will advance understanding of and spur innovation in treating PNH.

Bethesda, MD, May 6, 2021—The Aplastic Anemia and MDS International Foundation (AAMDSIF) and the National Organization for Rare Disorders, Inc. (NORD)® today launched the largest-ever study to research Paroxysmal Nocturnal Hemoglobinuria (PNH), a rare bone marrow failure disease. PNH is characterized by the destruction of red blood cells, blood clots, and impaired bone marrow function. While there are treatments available to improve the patients' quality of life, PNH currently has no cure.

The new study, the Global PNH Patient Registry*, creates a platform for patients around the world to share information about their experience with PNH. In gathering data from as many affected individuals as possible, the study will serve as an international resource for researchers looking to improve the lives of PNH patients.

"The Aplastic Anemia and MDS International Foundation is a 37-year-old patient-focused organization providing answers, support, and hope to bone marrow failure patients and their families. The Global PNH Patient Registry is an opportunity to give hope to PNH patients throughout the world by sharing their information with each other and with researchers to continue the quest for

better treatment options and a cure."
-Janice Frey-Angel (CEO of AAMDSIF)

To help drive awareness of PNH and encourage participation in the study, AAMDSIF and its community partners are working in collaboration with NORD to reach the patient community, which in the United States alone consists of around 400 to 500 individuals diagnosed with PNH each year. "Our goal is to enroll as many patients, or their parents or legal guardians, as possible," said Frey-Angel. "The success of the registry is dependent upon broad community participation."

The Global PNH Patient Registry is a natural history study that consists of electronic surveys to collect information about the patient experience and disease progression. Patients, or their caregivers or guardians, can enter information from anywhere in the world, making it easy to contribute. The data is made anonymous and stored securely in the registry. AAMDSIF may share the data with individuals or institutions conducting research or clinical trials, as approved by the study's governing board that includes scientists, doctors, and patient advocates.

"Patient-powered registries are changing the landscape of rare disease research," said Stephanie Christopher, NORD's Associate Director of Research Programs. "By building strong partnerships within the community and with leading scientific experts, NORD's Registry Program is well-positioned to address knowledge gaps and accelerate the development of discoveries that save lives. We are so pleased to welcome the Aplastic Anemia and MDS International Foundation as a partner in our IAMRARE® Registry Community!"

PNH is a rare blood disease that affects at least one person out of every million people. PNH occurs because the surface of a person's blood cells is missing a protein that protects them from the body's immune system. This lack of protection causes red blood cells to break and release hemoglobin. PNH can appear at any age and in any race or gender but is diagnosed most often in people in their 30s and 40s.

For more information, visit PNH.iamrare.org.

About 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.

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.

About National Organization for Rare Disorders, Inc. (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.

NORD began as a small group of patient advocates that formed a coalition to unify and mobilize support to pass the Orphan Drug Act of 1983. For almost 40 years, NORD has led the way in voicing the needs of the rare disease community, driving supportive policies and education, advancing medical research, and providing patient and family services for those who need them most. NORD is made strong together with over 325 disease-specific member organizations and their communities and collaborates with many other organizations on specific causes of importance to the rare disease community.

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

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