



# PNH GLOBAL ALLIANCE

## Annual Report 2021

PNH Global Alliance

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A financial report 2021 has been made separately and is summarised at section 7.

This Annual Report 2021 has been adopted in the general assembly of 2 April 2022.

## 1 Introduction

- a) The PNH Global Alliance (Alliance) was founded on 8 December 2018, in Amsterdam, The Netherlands, by representatives of PNH patient groups from England, The Netherlands, Canada, Germany, France and Spain. The Alliance and its predecessor the PNH European Alliance were initiated by Maria Piggin of PNH Support in England and Wales. Maria is also a patient advocate (ePAG) on the European Reference Network EuroBloodNet. Since our last report, patient groups from the following countries have joined as members of the Alliance: The following organisation became members of the Alliance in 2021: Aplastic Anaemia & Myelodysplasia Association of Canada (AAMAC) (Canada), Aplastic Anaemia & MDS International Foundation (AAMDSIF) (USA), PNH Ukraine and One in a Million - PNH Patients Association (Poland). Other PNH patient organisations of which the Alliance is aware have also been invited to join the Alliance.
- b) Despite the continuing coronavirus pandemic the Alliance continued its work.
- c) The members of the Alliance continue to exchange information on current treatments, trials and new medicines. The Alliance continues to be concerned about the ability of patients globally to access licenced medicines due to their extremely high cost. The Alliance advocates that pharmaceutical companies should involve patients in the research and development process from its inception and throughout in order that treatments address patients' unmet need and are that they are acceptable to patients.
- d) During 2021, the following Alliance board members continued their roles: Maria Piggin as Chair, Barry Katsof as Secretary and Ulrike Goebel took up the role of Treasurer when Angeline Jansen retired from the Board in March 2021. Pascale Burmester and Cor Koekkoek also continued as Board members (see also section 6).

## 2 Aims and results

- a) The overarching aim of the Alliance is to collaborate in order to advocate in the best interests of PNH patients worldwide.
- b) The Alliance is pleased to be able to report the following in relation to progressing our aim and objectives:

### **Specific objectives and progress:**

- c) **maximising access and availability of medicines for all PNH patients**

From time-to-time members of the Alliance or their individual patient organisations are contacted by PNH patients needing access to treatment. Each situation is different and requires our understanding of the particular request. Whilst we can make no guarantees of success in assisting a patient to gain access to treatment, we are committed to doing all that we can to assist to facilitate a positive outcome. The European Reference Network, EuroBloodNet network has continued to provide invaluable assistance in this regard. We provided advice and assistance to patients in Azerbaijan, Lithuania, Bosnia, South-Africa, Poland, Malta and India.

- d) **promoting research and development for medicines and procedures to treat and cure PNH**

- i. The Alliance continues to be represented at the Executive Committee of the PNH Registry.
- ii. The Alliance is represented on the IPIG PNH Registry Committee and in the Stakeholder Committee.
- iii. Two Alliance members representatives continue to provide advice on matters including recruitment as members of the Steering Committee to the NORD PNH Registry sponsored by AAMDSIF

- e) **advocating for PNH patients to be actively involved in the research and development process of medicines from as early as possible in order that these medicines better reflect patients' unmet needs**

- i. The Alliance advocates for PNH patients to be actively involved in the research and development process of medicines to effect this objective.
- ii. Two Alliance member representatives continued to inform two different burden of disease studies undertaken by pharmaceutical companies.
- iii. Two Alliance member representatives are co-authors on two papers resulting from one burden of illness studies.

- f) **advocating for PNH patients to be actively involved in the research and development process of medicines from as early as possible in order that these medicines reflect clinical outcome measures relevant to patients**

- i. The Alliance advocates for PNH patients to be actively involved in the research and development process of medicines to effect this objective.
- ii. Two Alliance members representatives joined the IPIG PNH Registry Committee to inform the development of this Registry's protocol.

- iii. Lobbying for the removal of “confusion” as a variable collected in PNH research to be replaced by more specific relevant symptoms such as cognitive issues including memory loss, brain fog etc.
- g) advocating for PNH patients to be actively involved in the research and development process of medicines from as early as possible in order that these medicines measure patient experience of specific medicines (preferably with a PNH relevant Quality of Life measure)**
- i. Since the availability of C5-Inhibitors, the main burden of PNH (haemolysis and thromboembolic events) have been better controlled. In countries where a C5-inhibitor is available, other unmet needs still exist: one of which is extravascular haemolysis and one is the general quality of life of patients. The quality of life of PNH patients is still fairly limited for a majority of patients. This has not been addressed as physicians, science and pharmaceutical companies have not yet sufficiently addressed this burden and there has been no validated Quality of Life tool to assess quality of life specifically for PNH. The PNH/AA QLQ-54 published in 2019 and funded by one of the Alliance’s members is the tool that comes closest to what the Alliance considers is needed, but it has not yet been fully validated to be able to be used in scientific trials or in registries. The validation and use of this tool is an important step for assessing quality of life specifically for PNH patients and strongly supported by the Alliance in different ways.
  - ii. The Alliance continued to lobby for the validation of the PNH and Aplastic Anaemia (AA) specific Quality of Life Questionnaire (QLQ PNH/AA) which is a continuing priority for PNH patients in order that relevant data are collected before, during and after treatment to enable meaningful data to be collected and research to be undertaken from the patient perspective.
- h) being involved in assessments by regulatory authorities including the European Medicines Agency (EMA), Medicines Healthcare Regulatory Authority (MHRA), Health Canada and the US Food and Drug Administration (FDA) in order that the appropriate submissions from the patient perspective are made**
- a) Alliance members continue to undertake these activities at their national level.
- i) being represented at groups and events relevant to the PNH community including the International PNH Interest Group (IPIG) in order that the appropriate submissions from the patient perspective are made**
- i. The Alliance was accepted as a member of the European Hematology Association Patient Organisation Working Group.
  - ii. In 2021, the IPIG Annual Scientific meeting was held virtually for the second year in a row as a result of the pandemic and was attended by five Alliance member representatives.
  - iii. An Alliance member representative is a patient representative in EuroBloodNet and delivered a presentation to a EuroBloodNet meeting on collaboration with EuroBloodNet to lobby for access to treatment for patients.
- j) educating ourselves on the latest developments in research for dissemination to our communities**
- i. Two Alliance members representatives informed the curation of EuroBloodNet’s educational sessions for clinicians on bone marrow failure.

- ii. Alliance members attended the 26th Virtual Congress of the European Hematology Association (EHA), IPIG's 2021 Annual Scientific Meeting on 1 December 2021 and the American Society of Hematology (ASH) 2021 in December 2021 and shared relevant content with the Alliance and member patient groups.
- k) **raising awareness among healthcare professionals of the symptoms of PNH in order to promote timely diagnosis and appropriate treatment**
  - i. Two Alliance members representatives informed the curation of EuroBloodNet's educational sessions for clinicians on bone marrow failure.

### 3 Member organisations

- a. The following organisations are current members of the PNH Global Alliance (the "Alliance") in 2021:
  - i. PNH Support (England, Wales and Northern Ireland)
  - ii. Stichting AA & PNH Contactgroep (The Netherlands)
  - iii. Canadian Association of PNH Patients (Canada)
  - iv. Stiftung Lichterzellen (Germany)
  - v. Aplastische Anämie & PNH e.V. (Germany)
  - vi. Asociación de Hemoglobinuria Paroxística Nocturna (Spain)
  - vii. Another Life (Russia)
  - viii. AAMAC (Canada)
  - ix. AAMDSIF (USA)
  - x. PNH Ukraine (Ukraine)
  - xi. One in a Million - PNH Patients Association (Poland)

### 4 Further Alliance activities

- a) The Alliance met virtually four times in 2021, each time by video conference as meeting in person was not possible because of the pandemic. In between meetings the Alliance member representatives are in close contact by email and video calling in smaller groups.
- b) Alliance members are in regular contact with healthcare professionals, universities and regulatory bodies in their different countries.
- c) Alliance members share country level information about the availability and access to medicines, experience with medical trials for new medicines, relevant scientific articles and the creation of registries.

The Alliance maintains a register of PNH trials globally which is uploaded to its website three times per year.

## 5 Communication

### Website

- a) The Alliance's website was updated in early 2021: [www.pnhglobalalliance.org](http://www.pnhglobalalliance.org).
- b) The Alliance continues to update its Instagram account, Facebook page and Twitter handle.

## 6. Governance and organisation

- a) On 25 February 2020 the PNH Global Alliance became an Association (vereniging) in The Netherlands by a notarial deed. The Alliance is a Dutch Association with international members under Dutch law.
- b) The Alliance is registered at the Dutch Chamber of Commerce under number 75270854. Data can be requested in Dutch and English on [www.kvk.nl](http://www.kvk.nl).
- c) The Alliance is governed by the Articles of Association. The more detailed Memorandum of Understanding (MoU) (house rules) signed by member representatives enlarges the Articles of Association.
- d) On 4 June 2020, a bank account was opened at ABNAMRO with number NL77ABNA0877597847 in the name of PNH Global Alliance.
- e) On 19 June 2020, the general assembly elected Maria Piggini as Chair, Barry Katsof as Secretary, Angeline Jansen as Treasurer (now replaced by Ulrike Goebel). There are two other board members, namely Pascale Burmester and Cor Koekkoek. An audit committee consists of Anastasiya Tatarinkova.
- f) Each member organisation has one vote. A member organisation can delegate one or two people to be represented on the Alliance. All participants at Alliance meetings, including interpreters, must sign the MoU and their organisation must complete the current Membership Application Form, on which the member representatives are named.
- g) As of 1 January 2020 Alliance member organisations pay an annual membership fee of 250 euro. This generates funding to allow the outsourcing of the financial and regulatory administration, and some secretarial work to APN Backoffice BV at Tiel in The Netherlands. This company's address is the registered address of the Alliance.
- h) In addition to country specific codes of conduct and patient organisation policies regarding relationships between patient organisations and pharmaceutical companies, the Alliance adheres to the following codes of conduct when engaging with pharmaceutical companies as an Alliance:

- i. [EURORDIS Code of Conduct between Patient Organisations’ and the Healthcare Industry](#)
- ii. The European Federation of Pharmaceutical Industries and Association (EPFIA) [Code of Practice](#) refers to [“Working Together with Patients: Principles for Remunerating Patients, Patient Organisation Representatives and Carers for Work Undertaken with the Pharmaceutical Industry”](#)
- iii. [IMI PARADIGM Patient Engagement toolbox](#)

## 7. Financial Review

The Alliance received the following receipts and undertook the following expenditure in the year:

Receipts	€5560
Expenditure	€4183