



**P N H**  
**G L O B A L**  
**A L L I A N C E**

# Annual Report 2022

**PNH Global Alliance**

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A financial report 2022 has been made separately and is summarised at section 7.

This Annual Report 2022 has been adopted in the general assembly of 13 May 2023

## **1 Background and 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 (England and Wales). Since our last report, no additional patient groups joined as members of the Alliance however the Aplastic Anaemia & Myelodysplasia Association of Canada (AAMAC) (Canada) resigned its membership. Other PNH patient organisations of which the Alliance is aware have also been invited to join the Alliance.
- b) 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.
- c) During 2022, Maria Piggin continued as Chair, Barry Katsof resigned as Secretary and Ulrike Göbel continued as Treasurer. Pascale Burmester and Cor Koekkoek also continued as Board members (see also section 6). The Secretary role remained vacant after Barry's resignation.
- d) On 11 October 2022, the Alliance members revised the Mission, Vision and Objectives of the Alliance.

## **2 Aims, Objectives and Progress**

- a) The revised Mission of the Alliance is "All PNH patients globally have access to optimal care and treatment."
- b) The revised Vision of the Alliance is "We collectively advocate on behalf of all PNH patients"
- c) The revised objectives of the Alliance are as follows:
  - i. maximising access to medicines for all patients
  - ii. supporting R&D to understand, test, treat and cure PNH including by advocating for PNH patients to be actively involved in R&D from start to end
  - iii. representing patients, advocating and raising awareness with stakeholders relevant to the PNH community e.g. regulators, IPIG, EuroBloodNet, congresses, policy makers
  - iv. sharing relevant information and educating ourselves

## **Specific objectives and progress:**

### ***Maximising access to medicines for all patients***

- a) The European Reference Network, EuroBloodNet network has continued to provide invaluable assistance to continue to assist with PNH patients needing access to treatment including assistance provided to patients in Malta and Morocco.
- b) The Alliance has been supporting the Dutch organisation Stichting AA & PNH Contactgroep to lobby for access to ravulizumab.

### ***Supporting R&D to understand, test, treat and cure PNH including by advocating for PNH patients to be actively involved in R&D from start to end***

- a) The Alliance continues to be represented at the Executive Committee of the PNH Registry.
- b) The Alliance continues to be represented on the International PNH Interest Group (IPIG) PNH Registry Committee informing the Registry's protocol. Three Alliance members are on the IPIG Registry Stakeholder Committee. The IPIG PNH Registry is planning to validate the QLQ AA/PNH-54 once the registry is established. This 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.
- c) One Alliance member is a member of the COMMODORE trial steering committee.

### ***Representing patients, advocating and raising awareness with stakeholders relevant to the PNH community e.g. regulators, IPIG, EuroBloodNet, congresses, policy makers***

- a) The Alliance advocates for PNH patients to be actively involved in the research and development process of medicines to effect this objective.
- b) Two Alliance member representatives are co-authors on two papers resulting from one burden of illness study.
- c) The Alliance wrote to European Society of Hematology (ESH) to allow access by patient advocates to their European Bone Marrow Transplant (EBMT) conference where a poster was presented on an ethnographic study highlighting the breadth of symptoms experienced by PNH patients which are not usually associated with PNH by clinicians. The paper and poster was co-authored by three Alliance representatives.
- d) The Alliance is represented on the European Hematology Association (EHA) Patient Advocacy Committee.
- e) The Alliance is a member of the EUROACT project led by Patvocates seeking to explore the inequalities in clinical trial access across Europe.
- f) The Alliance is collaborating with EuroBloodnet on a project concerning access to PNH medicines.
- g) Three member representatives of the Alliance took part in a policy initiative called Partnering for PNH for which a paper was written.

### ***Sharing relevant information and educating ourselves***

- a) Two member representatives of the Alliance attended the EHA Congress 2022 in Vienna and shared relevant content with the Alliance and member patient groups.

- b) The Chair of the Alliance wrote a commentary piece published in The Lancet which included reference to the work of the Alliance.
- c) Two Alliance member representatives co-created and three Alliance members co-presented a series of webinars with EuroBloodNet on topics of interest to PNH and Aplastic Anaemia patients.
- d) An Alliance member representative is a patient representative in EuroBloodNet and co-delivered a EuroBloodNet webinar on the value of registries for research.
- e) The Alliance maintains a register of PNH trials globally which is uploaded to its website three times per year.
- f) An Alliance member attended the American Society for Hematology (ASH) conference virtually and informed other members about relevant content.

### **3 Member organisations**

- a. The following organisations are current members of the PNH Global Alliance (the “Alliance”) in 2022:
  - 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. AAMDSIF (USA)
  - ix. PNH Ukraine (Ukraine)
  - x. One in a Million - PNH Patients Association (Poland)
  - xi. Aplastic Anemia & Myelodysplasia Association of Canada (AAMAC)

### **4 Further Alliance activities**

- a) The Alliance met virtually four times in 2022, each time by video conference as meeting in person was not feasible. In between meetings, the Alliance member representatives are in close contact by email.
- 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.

## **5 Communication**

- a) The Alliance continues to update its Instagram account, Facebook page and Twitter handle.
- b) The Alliance updated its websites with contact details of PNH patient organisations globally in order that patients could have access to these details.

## **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 Articles of Association. The more detailed Memorandum of Understanding (MoU) (house rules) signed by member representatives enlarges the Articles of Association.
- d) Our bank account is held at ABNAMRO with number NL77ABNA0877597847 in the name of PNH Global Alliance.
- e) The current board members are Maria Piggin (Chair), Ulrike Göbel (Treasurer), Pascale Burmester (Secretary) and Cor Koekkoek. An audit committee consisted of Anastasiya Tatarinkova and Tamir Orbach.
- f) Each member organisation has one vote. A member organisation can delegate one or two people to be represented on the Alliance (member representatives). 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) Alliance member organisations pay an annual membership fee of 250 euro. This generates funding to allow the outsourcing of the financial and regulatory administration. 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 Conduct refers to “Working Together with Patients: Principles for Remunerating Patients, Patient Organisation Representatives and Carers for Work Undertaken with the Pharmaceutical Industry”

## **7 Financial Review**

The Alliance received the following receipts and undertook the following expenditure in the year. We received a grant of €500 from SOBI to pay for leaflets to take to the EHA Congress to advertise the Alliance.

Receipts	€4478
Expenditure	€1476