PNH GLOBAL ALLIANCE

Annual Report 2025

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A financial report 2025 has been made separately and is summarised at section 7.

This Annual Report 2025 has been adopted in the general assembly of XXX, 2026

1. Background and Introduction

a) The PNH Global Alliance (Alliance) was founded on 8 December 2018, in Amsterdam. The Alliance and its predecessor, the PNH European Alliance, were initiated by Maria Piggin of PNH Support (England, Wales, and Northern Ireland).

b) During 2025, five PNH patient groups joined the Alliance: PNH Nordic (Denmark, Nordic Countries), AFAG (Brazil), PNH Serbia (Serbia), PNH New Zealand (New Zealand), and PNH Patient Support India (Informal patient group, India). AAMDSIF (USA) left the Alliance in 2025.

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 licensed 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 needs and are that they are acceptable to patients.

d) During 2025, Maria Piggin continued as Chair, Ulrike Göbel continued as Treasurer, and Pascale and Burmester continued as Secretary. Olena Wagner withdrew as Board Member in April 2025.

e) Michal Wozniak and Daniel Webby were appointed as the Audit Committee Members in 2025.

2. Aims, Objectives and Progress

Specific objectives and progress:

1. ***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.

b) The Alliance is represented on the advisory board of the Affordability and Sustainability improvements through new pricing, Cost- Effectiveness and ReimbursemenT models to Appraise INnovative health technologies (ASCERTAIN) project to promote access to affordable technologies, the need to stimulate innovation and entrepreneurship, and the need to consider the environmental impact of innovations.

2. ***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 on the International PNH Interest Group (IPIG) PNH Registry Committee. Three Alliance members are members of the IPIG PNH Registry Stakeholder Committee.

b) The Alliance is represented on the IPIG working group established to validate the Quality of Life Tool: QLQ AA/PNH-54. 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 on priorities identified by patients. The following article has been published: Taylor, K.J., Singer, S., Langemeijer, S. Kelly, R. J., Arnold, L., Panse, J., Patriquin, C. J., Nishimura, J., Piggin, M., Burmester, P.: What are the most important quality of life domains for patients with aplastic anemia and paroxysmal nocturnal hemoglobinuria?.*Ann Hematol* 104, 3073–3075 (2025). https://doi.org/10.1007/s00277-025-06377-z

c) The Alliance is represented on the steering group of the European Atlas on Clinical Trials in Cancer and Hematology (EuroACT) project, which aims to understand the clinical trial landscape in the European region, based on data extracted from all relevant European clinical trial registers. The following papers have been published by this project:

   a. Cases M, Giles R, Ágh T, Piggin M, Geissler J, Racovita M, Hagymásy J, Ruth Wogu L, Józwiak Á, Hyseni-Bocolli A, Hosszú D, Plate A and EuroACT Working Group (2025) Correction: Methodological study protocol for the European Atlas of clinical trials in cancer and haematology. *Front. Pharmacol.* 16:1743351. doi: 10.3389/fphar.2025.1743351
   b. Cases, Mercè and Imre, Attila and Giles, Rachel H. and Puga, Lis and Piggin, Maria and Geissler, Jan and Racovita, Monica and Leto di Priolo, Susanna and Wogu, Lora Ruth and Hyseni-Bocolli, Albina and Morgan, Kate and Hosszú, Dalma and Pitter, János György and Ágh, Tamás and Plate, Ananda, Variation in Use of Patient-Reported Outcome Measures and in Geographical Distribution in Clinical Trials of Selected Malignant and Non-Malignant Haematological Diseases in Europe: Findings from EuroACT. Available at SSRN: https://ssrn.com/abstract=5646744 or http://dx.doi.org/10.2139/ssrn.5646744
   c. Poster_ https://wecanadvocate.eu/wp-content/uploads/2025/10/2025-13-10-EUROACT_poster_ESMO_2025_1013-2.pdf
   d. Cases, Mercè and Imre, Attila and Giles, Rachel H. and Puga, Lis and Piggin,Maria and Geissler, Jan and Racovita, Monica and Leto di Priolo, Susanna and Wogu, Lora Ruth and Hyseni-Bocolli, Albina and Morgan, Kate and Hosszú, Dalma and Józwiák, Ákos and Ágh, Tamás and Plate, Ananda and Group, EuroACT Working, Geographic Inequities in Patient-Reported Outcome Measures in Clinical Trials of Selected Solid Tumours in Europe: Findings from EuroACT. Available at SSRN: https://ssrn.com/abstract=5390471 or http://dx.doi.org/10.2139/ssrn.5390471

3. 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 where possible for PNH patients to be actively involved in the research and development process of medicines to progress this objective.

b) The Alliance continues to be represented on the European Hematology Association (EHA) Patient Advocacy Committee (PAC).

c) The Alliance delivered a PNH Awareness Campaign in October 2025, which included several social media channels and videos recorded during the Global PNH Advocacy Forum 2025 (Forum). The campaign concluded with the second global PNH Awareness Day on 12 October 2025.

d) The Alliance was presented as a faculty member at the Complement Masters 2025: PNH, which is conducted by Alexion Pharma Inc. To educate 91 physicians (with PNH experience) from 27 countries on the latest on PNH

4. Sharing relevant information and educating ourselves

a) The Alliance organized a Forum in September, inviting both members and non-members of the Alliance, representing 16 countries. This was a 3-day event held in Barcelona, Spain.

3. Member organisations

The following organisations and patient groups were members of the PNH Global Alliance (the "Alliance") in 2025:

a) AFAG (Brazil)
b) Another Life (Russia)
c) Aplastische Anämie & PNH e.V. (Germany)
d) Asociación de Hemoglobinuria Paroxística Nocturna (Spain)
e) One in a Million - PNH Patients Association (Poland)
f) PNH New Zealand (New Zealand)
g) PNH Nordic (Denmark, Nordic Countries)
h) PNH Patient Support India (patient group, India)
i) PNH Serbia (Serbia)
j) PNH Support (UK)
k) PNH Ukraine (Ukraine)
l) Stichting AA & PNH Contactgroep (The Netherlands)
m) Stiftung Lichterzellen (Germany)

4. Further Alliance activities

a) During 2025, the Alliance met virtually twice, each time by video conference, and met in person once, in September 2025. 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 continued to update its Instagram account, Facebook page, LinkedIn, and Bluesky account.

b) The Alliance also updated the website in 2025, adding new members and other information/pages.

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.

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), and Pascale Burmester (Secretary). An audit committee consisted of Michal Wozniak and Daniel Webby.

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) In November 2024, membership criteria were widened to include informal PNH patient groups, and the annual membership fee was waived until further notice to allow for more informal PNH groups to join. These criteria continued in 2025.

h) The Alliance secured grants from six pharmaceutical companies to undertake the projects detailed at 2.3.c and 2.4.a) and a) above.

i) The Alliance contracted Patvocates to undertake secretariat services for the Alliance in 2025.

j) 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:

k) The European Federation of Pharmaceutical Industries and Association (EPFIA) Code of Conduct

l) EURORDIS Code of Practice

7. Financial Review

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

Receipts	€ 180,000
Expenditure	€ 132,286