

Paroxysmal Nocturnal Hemoglobinuria:

at the crossroads of somatic mutations, clonal expansion and immunity



Florence, October 3-4, 2024

Grand Hotel Baglioni

PNH Global Alliance

Maria Piggin

Chair, PNH Global Alliance

contact@pnhga.org, www.pnhga.org



Disclosures



- **PNH Global Alliance** – grants received from:
 - Alexion AstraZeneca Rare Diseases
 - Amgen
 - Novartis
 - Regeneron
 - Roche
- **PNH Support (UK)** (of which I am Chair) received consultancy fees from:
 - Alexion
 - Novartis
 - Roche
 - Alexion AstraZeneca Rare Diseases, Roche and Swedish Orphan Biovitrum contributed to funding for a National Community Survey project which surveyed 7 UK rare disease communities including PNH Support.





Who we are



PNH
GLOBAL
ALLIANCE



UK

Maria Piggin
(Chair)



GERMANY

Ulrike Göbel
(Treasurer)



GERMANY

Pascale Burmester
(Secretary)



Patvocates -
Secretariat



THE
NETHERLANDS



SPAIN



UKRAINE



POLAND



RUSSIA



USA

Paroxysmal Nocturnal Hemoglobinuria:
at the crossroads of somatic mutations, clonal expansion and immunity

Florence, October 3-4, 2024
Grand Hotel Baglioni





Why we formed an Alliance



PNH
GLOBAL
ALLIANCE

- To co-operate globally
- To use our privilege to assist other patients
- To have a louder united voice in science, drug development & improving access to PNH treatments
- To be taken seriously by stakeholders e.g. EHA, EuroBloodNet, IPIG
- To provide leadership to patients globally



Paroxysmal Nocturnal Hemoglobinuria:
at the crossroads of somatic mutations, clonal expansion and immunity

Florence, October 3-4, 2024
Grand Hotel Baglioni





What we do



PNH
GLOBAL
ALLIANCE

Our Vision: all patient globally have access to optimal care and treatment

Our Mission: collectively advocating on behalf of all PNH patients

Our Objectives:

1. Maximising **access** to medicines for all patients
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
3. **Representing patients, advocating and raising awareness** with stakeholders relevant to the PNH community e. g. regulators, IPIG, EuroBloodNet, congresses, policy makers
4. Sharing **relevant information** and **educating** ourselves





Our Objectives



1. Maximising **access** to medicines for all patients
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
3. **Representing patients, advocating and raising awareness** with stakeholders relevant to the PNH community e. g. regulators, IPIG, EuroBloodNet, congresses, policy makers
4. Sharing **relevant information** and **educating** ourselves





The reality of access



- Based on list prices stated by NICE (UK) per patient, per year:
 - £245,700 for eculizumab (289,926€)
 - £324,109.50 for ravulizumab (382,449.21€)
 - £322,400 for pegcetacoplan (380,432€)
 - \$550,000 for iptacopan (512,012.60€) (EndpointsNews 06.12.23)
 - Biosimilars only 20-25% less expensive

- Approximately 80% of patients globally have no access to any licenced PNH therapies due to cost



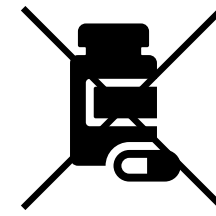


The disparity



Patients with access to PNH treatments

- Survival revolutionised
- Partially improved QoL (remaining unmet need)
- Can contribute to society (employment, caregiving, family life)
- Can establish national patient organisations/advocate on behalf of other patients (including for access)



Patients without access to PNH treatments

- Significant survival risk (thrombosis)
- Limited quality of life (impacts family, ability to work, study, start a family)
- Absence of patient organization
- Absence of support





What we are doing about maximising access



EuroBloodNet



Hematological Diseases
(ERN EuroBloodNet)

- Current project on PNH drug access in Europe
 - Survey: 20/27 Member States responded (results published as poster at EHA 2024)
 - Access to PNH treatments is not just a global issue - more drugs available than accessible due to reimbursement pathways, pricing and financial sustainability
 - Survey be repeated following licencing of further treatments & results used for lobbying
- Utilise network to lobby health ministers/governments re patient access to treatment in countries e.g. Poland, Lithuania & Malta

- **EUROACT (EUROpean Atlas on Clinical Trials in Cancer and Haematology)** - mapping clinical trials & collecting evidence on PRO/HRQoL use over last 5 years - represented on steering committee
- **ASCERTAIN (Affordability and Sustainability improvements through new pricing Cost-Effectiveness and Reimbursement models to Appraise INnovative health technologies)** – Advisory Board member (EU funded)
- Members inform **Health Technology Assessment** processes (e.g. Holland, UK, Poland)





Our Objectives



1. Maximising **access** to medicines to all patients
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
3. **Representing patients, advocating and raising awareness** with stakeholders relevant to the PNH community e. g. regulators, IPIG, EuroBloodNet, congresses, policy makers
4. Sharing **relevant information** and **educating** ourselves





What we are doing to support R&D



PNH
GLOBAL
ALLIANCE

International PNH Interest Group (IPIG) PNH Registry (in development):



- 2 seats on the IPIG PNH Registry Committee
- 4 seats on the IPIG PNH Registry Stakeholder Committee
- part of the validation study for the QLQ-AA/PNH-54
- Supported **biosimilar trials**
- Non-voting member on **International PNH Registry**
- Represented on **COMMODORE trial steering committee**
- German members informed national **PNH clinical guidelines** and are to be co-authors
- UK patient advocates **review PIS/ICF** (now mandated for ethics approval)
- Members registered stakeholders with **EMA**
- Review study **protocols**
- **Co - author** papers
- Participate in Novartis Global Oncology Patient Involvement Panel (**GOPIP**)

Paroxysmal Nocturnal Hemoglobinuria:
at the crossroads of somatic mutations, clonal expansion and immunity

Florence, October 3-4, 2024
Grand Hotel Baglioni





Our Objectives



1. Maximising **access** to medicines for all patients
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
- 3. Representing patients, advocating and raising awareness with stakeholders relevant to the PNH community e. g. regulators, IPIG, EuroBloodNet, congresses, policy makers**
4. Sharing **relevant information** and **educating** ourselves





Representing patients, advocating, raising awareness



PNH
GLOBAL
ALLIANCE

- Represented on the **EHA Patient Advocacy Committee**
 - raised **awareness of access issues** - EHA Congress 2024 – Co-chair and panel member on EHA/Patient Joint Symposium panel on "*Fair Pricing: does the value of new medicines in hematology justify the high prices?*"
- Patient representative in **EuroBloodNet**
- Participation in the Haematology **Community Advisory Board**

Paroxysmal Nocturnal Hemoglobinuria:
at the crossroads of somatic mutations, clonal expansion and immunity

Florence, October 3-4, 2024
Grand Hotel Baglioni





Global PNH Awareness Campaign



PNH
GLOBAL
ALLIANCE

- 6 September to 12 October: Global PNH Awareness Day
- #DidYouKnow, #TreatingPNH
- Our website will include global maps of:
 - PNH specialists
 - PNH patient organisations
- Please follow us on Facebook, Instagram, LinkedIn, Twitter (X) and share!



Paroxysmal Nocturnal Hemoglobinuria:
at the crossroads of somatic mutations, clonal expansion and immunity

Florence, Grand Hôtel Baglioni, 4, 2024





Our Objectives



1. Maximising **access** to medicines for all patients
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
3. **Representing patients, advocating and raising awareness** with stakeholders relevant to the PNH community e. g. regulators, IPIG, EuroBloodNet, congresses, policy makers
4. Sharing **relevant information** and **educating** ourselves





PNH Advocate Development Programme



- November 2023 to November 2024
- **10 patients** from China, India, Turkey, Ukraine, UK, Denmark, USA, Poland
- Included training programme at **EHA Congress 2024** in Madrid
- Examples of topics covered:
 - Biology, clinical mechanisms of PNH
 - R&D & how patients can input into the process
 - Quality of Life and PROs
 - Key elements of clinical trial protocols and study endpoints
 - Health Technology Assessment and pricing



Our requests to the wider PNH community

- **listen** to patients
- utilise patient groups as **trusted allies and collaborators**
- seek to further understand the **pathophysiology and causes** of PNH including remission
- keep in mind the aim of a **non-BMT cure** for patients and that patients need **affordable drugs** with few side-effects
- support **access** to diagnosis, medicines and vaccines for all patients
- be open minded about **symptoms patients identify** as being linked to PNH even though research evidence is currently limited
- explore **wider symptoms & non-clinical aspects** e.g. psychological impact
- use the **QLQ AA/PNH 54** to understand patients' quality of life (even before it is validated).





And remember...

...Patients have a different perspective than physicians, which is also valuable for shaping and informing research and furthering knowledge





Thank you



P N H
GLOBAL
ALLIANCE

FOR LISTENING TO PATIENTS!

Please feel free to contact us or pass this information to patients.

contact@pnhga.org; www.pnhga.org



Paroxysmal Nocturnal Hemoglobinuria:
at the crossroads of somatic mutations, clonal expansion and immunity

Florence, October 3-4, 2024
Grand Hotel Baglioni

