

# Annual Report 2021



## President's Report

Since the Global Porphyria Advocacy Coalition's (GPAC's) inaugural face-to-face meeting in Milan (September 2019, at the International Congress on Porphyrins and Porphyria – ICCPP) – when the Executive Board were elected – the world has changed somewhat! The global pandemic has impacted the rare disease community and GPAC significantly in terms of capacity and delays in the ability to build certain infrastructure.

### Progress to date

The GPAC Executive Board met virtually on 9 separate occasions to make strategic plans for GPAC (totalling 36 hours). Furthermore, GPAC Members met virtually on 8 occasions (totalling 16 hours), with regular representation from over 13 countries.

We are very pleased to report that we have still made great strides as a global umbrella organization. Most significantly, we became officially registered with the Charities Commission in the UK as a Charitable Incorporated Organization (CIO) in May 2020. GPAC has 21 Members (representing 25 countries), including the newly formed South African Porphyria Foundation.

GPAC coordinated the development of a much needed Accurate Information Statement that was endorsed by 15 porphyria member countries and four porphyria physician networks (Epnet<sup>1</sup>, APEX<sup>2</sup>, BIPNET<sup>3</sup> and Porphyria South Africa<sup>4</sup>). GPAC also formulated a Covid-19 Statement, based on guidance from APEX and Epnet, that was shared widely.

GPAC represented the global porphyria community at a number of international virtual meetings, including the Global Rare Disease Patient Advocacy Collaborative Summit (hosted and funded by Alnylam Pharmaceuticals) speaking alongside the Mexican Society for Porphyria, and other rare disease organisations. Additionally, as part of Porphyria Awareness Week, GPAC spoke alongside the Global Director of the APF about global awareness initiatives at a Townhall with Alnylam.

GPAC acted as a stakeholder, alongside the British Porphyria Association and the IPPN<sup>5</sup> in the NICE/HST application for Givosiran as a treatment for AHP patients with severe recurrent attacks. GPAC's involvement strengthened the national approach and a positive recommendation to approve was received in October 2021. NICE decisions are often followed in other countries around the world, so we are delighted to have been part of this pivotal work in providing the patient voice to the process.

The Treatments and Research Working Group has provided regular updates at meetings which has generated much discussion and awareness of the state of trials/drug development and reimbursement across many countries. In addition, the APF Global Program has helped to support numerous fledgling porphyria patient groups that GPAC hopes to support too.

The Awareness Working Group coordinated the global community's efforts for Porphyria Awareness Week in 2020 *#Ask Me About Porphyria*. In 2021 we had a successful global project *#Let's Talk Porphyria* (represented by 9 different countries) which resulted in the development of multilingual video resources that will likely be used for years to come. Plans for 2022 Porphyria Awareness Week are already underway, with a team reviewing a rare disease video focusing on AHP due to be launched in 2022.

GPAC has also been involved in the *Porphyria worldwide patient experience research study: Impact of acute hepatic porphyria assessed through an international patient survey (POWER Study)*. Contributing to the design of the study, promoting participation amongst the global community and also being included within authorship for journal and conference submissions. This work will continue over the next 12 months. Additionally, GPAC would like to acknowledge and thank a few key people from GPAC Member countries who have been involved in a larger number of research publications over the last 3 years – your hard work is contributing significantly to the international library of scientific literature on porphyria (see website for details).

**21** GPAC  
Members

**9** Executive Board meetings  
**36** Hours of collaboration

**8** GPAC Member meetings  
**16** Hours of meetings

<sup>1</sup> Epnet: European Porphyria Network

<sup>2</sup> APEX: American Porphyrias Expert Collaborative

<sup>3</sup> BIPNET: British and Irish Porphyria Network

<sup>4</sup> Porphyria South Africa associated with University of Kwazulu-Natal

<sup>5</sup> IPPN: International Porphyria Patient Network

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## Challenges faced

The significant constraints of the Covid-19 pandemic have affected/delayed a number of aspects for GPAC. Most notable have been difficulties in the opening of a suitable international bank account. Covid-restrictions on face-to-face meetings in the UK, resulted in a significant delay/backlog within the banking sector. GPAC have an appointment in late November 2021 where they hope to open a Barclays Community Account.

## Strategic aims for 2022

GPAC has many aims for the next 12 months. The Executive Committee will continue to formalise GPAC's official infrastructure, with the opening of a UK bank account, updating of the website and formalising all of the elements required for GPAC to become the fully functioning global umbrella entity envisioned in 2019 (including establishing bylaws, policies and procedures, roles and responsibilities, Member joining protocols as well as a membership fee collection processes). Additionally, the Working Groups will be strengthened further with clear aims and objectives (and roles and responsibilities) that can be reflected upon in 2022 at our next AGM.

GPAC aims to develop a survey to identify what GPAC members want in order to better support the Members and new organizations joining GPAC. As part of this, GPAC will also consider ways in which it can ensure that the entity remains sustainable now and in the future.

## Treasurer's Report

### Projected income and expenditure

Forecasted income		Current exchange rate 10-11-21 GBP (rounded)	
APF – initial donation 1,000 USD	£730.00		
BPA – initial donation 500 GBP	£500.00		
Alnylam staff donation 3,000 USD	£2,200.00		
<b>TOTAL forecasted income</b>	<b>£3,430.00</b>		<b>£3,430.00</b>
<b>Expenditure (ACTUAL – to be reimbursed to relevant parties once bank account open)</b>		<b>Detail</b>	
Mijndomein website/domain 2019-2020 (15-Sep-2019 – 35 Euros)	£30.00	Paid by Danny De Roode – <b>still due to Danny De Roode</b>	
Mijndomein website/domain 2020-2021 (16-Sep-2020 – 115.00 Euros)	£108.45	Paid by Danny De Roode, reimbursed by S Burrell 3-Oct-2020 – <b>still due to S Burrell</b>	
Mijndomein website/domain 2021-2022 (15-Sep-2021 – 117.07 Euros)	£104.62	Paid by Danny De Roode, reimbursed by S Burrell on 28-Sep-2021 – <b>still due to S Burrell</b>	
Zoom annual meeting account 2021-2022 (10-Nov-2021)	£143.88	Paid by S Burrell – <b>still due to S Burrell</b>	
<b>Expenditure (DUE)</b>			
Epnet Associate Membership – 3yrs – 2019-2021 (04-Nov-2021) – 300 Euros (due)	£260.00		
<b>Total projected expenditure*</b>	<b>£646.95</b>		<b>£646.95</b>
		<b>Gross projected profit</b>	<b>£2,783.05</b>

\*Including reimbursement to all parties and owing invoices