

GLOBAL PORPHYRIA ADVOCACY COALITION

England & Wales · Charity number 1189694

Details

Other names GLOBAL PORPHYRIA ADVOCACY COALITON

Status Registered

Legal form CIO

Registered 2020-05-28

Register [View on the Charity Commission register](#)

Contact

Address 10 Ruskin Road
New Costessey
Norwich
NR5 0LL

Phone 03003020030

Email PRESIDENT@GPAC-PORPHYRIA.ORG

Website WWW.GPAC-PORPHYRIA.ORG

Activities

Objects: FOR THE PUBLIC BENEFIT, TO RELIEVE THE NEEDS OF PATIENTS SUFFERING FROM PORPHYRIA WORLDWIDE, IN PARTICULAR, BUT NOT EXCLUSIVELY, BY PROVIDING SUPPORT AND INFORMATION TO PATIENTS AND PATIENT GROUPS, AND BY RAISING AWARENESS OF THE DISORDER AMONGST HEALTH PROFESSIONALS, POLICY MAKERS AND THE GENERAL PUBLIC, INCLUDING BY PROVIDING INFORMATION ABOUT THE PORPHYRIA GROUP OF DISEASES, THEIR DIAGNOSES AND TREATMENTS, PROMOTING RESEARCH AND SHARING THE USEFUL RESULTS OF LATEST RESEARCH.

Activities: The Global Porphyria Advocacy Coalition (GPAC) operates as an umbrella organization for national porphyria patient advocacy organizations, representing all types of porphyria. We work to support all individuals impacted by porphyria and to safeguard their interests. GPAC connects, supports and engages porphyria patients around the world through an integrated international network.

Classification

- **How:** Provides Services, Provides Advocacy/advice/information, Sponsors Or Undertakes Research, Acts As An Umbrella Or Resource Body
- **What:** The Advancement Of Health Or Saving Of Lives, Disability
- **Who:** Children/young People, Elderly/old People, People With Disabilities, Other Charities Or Voluntary Bodies, Other Defined Groups

Geography

- Australia
- Austria
- Belgium
- Brazil
- Canada
- Denmark
- France
- Germany
- Ireland
- Italy
- Mexico
- Netherlands
- Northern Ireland
- Poland
- Scotland
- South Africa
- Spain
- Sweden
- Switzerland
- United States
- Throughout England And Wales

Finances

Period end	Income	Expenditure	Assets	Employees
2024-11-10	£24,853	£4,313	-	-
2023-11-10	£8,884	£6,917	-	-
2022-11-10	£6,867	£714	-	-
2021-11-10	£0	£0	-	-

Trustees

Name	Role	Appointed
SUZANNE BURRELL	Chair	2020-05-28
Derry Johna Wilcox		2024-05-11
Enrica Cecilia Pupko Katz Kalb		2024-05-11

GLOBAL PORPHYRIA ADVOCACY COALITION

England & Wales - Charity number 1189694

Accounts

President's Report

It is now a full 5 years since GPAC's first official meeting at ICPP in Milan, in 2019. Sue Burrell is still in position as the current President. We would like to thank Sean Hegarty (Past Vice-President) and Joaquin Montoto (Past-Secretary) for their time and commitment to being part of the inaugural GPAC Executive Team. Both Sean and Joaquin are remaining in Stewardship roles until 2025 as we officially welcomed Kika Shabot into the role of Acting Vice-President and Acting Derry Wilcox as GPAC's new Treasurer at our delayed AGM in February 2024. Once officially in role, the Charity Commission will be updated to reflect the new Trustee Board (the Executive Board), and Derry will be transitioned in to be a Bank Signatory too.

Our last AGM (February 2024 – Delayed from Sept 2023) took place via Zoom, and was well represented with engagement from 13 different countries/PAGs. We also a new face join our meeting, Nadežda Pejović from the new porphyria PAG in Serbia – we welcome Nadezda who is engaged and keen to get involved.

Since February, GPAC Executives and Members have been meeting regularly to accomplish shared goals and initiatives for and with the global porphyria community. GPAC also spent significant time, considering a matter within our community. After consultation with the Charities Commission, and with external guidance on how to proceed, we spoke with all parties and have identified a position that ensures GPAC activities are unhindered – whilst remaining engaged and focused to work with all groups/organisations working to serve the porphyria community.

Looking forward: We are excited to be holding our next AGM at the International Congress of Porphyrins and Porphyria (F2F) in Pamplona on Saturday 21st September 2024, and via Zoom for those unable to travel to the Congress. The GPAC meeting will be structured to hold a 5 hour meeting which will include our AGM, an interactive workshop focusing on GPAC priorities and goal setting for 2025 and beyond. The meeting will then conclude with presentations from various pharmaceutical companies, including: Alnylam Pharmaceuticals, Disc Medicine, Portal Therapeutics, Mitsubishi Tanabe as well as an update from Dr Oscar Millet on current developments and research into CEP. The hope is that this style of programming will foster great opportunities for engagement with other PAGs, as well as other stakeholders, researchers and clinicians that we may be able to partner with for the benefit of the porphyria families across the world.

Progress to date

Meetings, connections and fostering global relationships: The GPAC Executive Board have met on many occasions since February, 2024, to work on GPAC priorities and working tasks (including the domestic matter aforementioned), ICPP planning (for the Spanish Patient Day, the Patient and Doctor Workshop [formerly the Patient Day], Poster displays and GPAC's Meeting), as well as the general management of GPAC. The President and Vice-President have been meeting weekly or biweekly to engage and support initiatives on a global level. The Executive has also been working with pharma/industry representatives and clinical leaders, on numerous occasions, about sponsorship, disease awareness events/communications and to foster partnering relationships to help share information about trials, trial data, new medicines, research and awareness.

We are excited to build further upon this work with our community at ICPP in Pamplona, in September, with many meetings and social engagements planned where we hope to build relations further to align on plans to benefit the porphyria patient community.

Sue Burrell was involved in a paid social media collaboration with Katri Parri (from the Spanish Patient Association, Katri is a social media influencer/content creator and now works for the United Porphyria Association). This project was funded by Alnylam Pharmaceuticals, it aimed to raise awareness and reach and was released on Global Porphyria Day (18-May-2023). Sue Burrell received honorariums for this engagement via her paid role with the BPA.

PAGs becoming Members of GPAC: Following the AGM in February 2024, GPAC made plans for PAGs to complete their Joining Form, over the months leading up to the next AGM in Pamplona, where it was planned that all those ready to be voted on as Members would be voted on at the AGM in Pamplona.

GPAC's Working Groups (WG): Across 2023 and to date, most of GPAC's WGs have not been meeting regularly and it as decided at the Member meeting in July that the Working Groups would be reviewed to reflect more of the work that GPAC are actually doing. Work in all areas will continue, on an ad-hoc basis as needed/proposed by the community of PAGs that GPAC represent. As reported at our last AGM, much work is still ongoing via our Members (namely, between groups in America [APF and UPA], Brazil, Canada, Chile, Italy, Mexico, Serbia, Spain – to name a few)! There have been specific collaborations between a number of organisations to deliver education events/conferences (face-to-face, hybrid and virtually) to educate, connect and empower patients and physicians. The success of which demonstrates the benefits that can be achieved from working together.

The *Awareness WG* has remained active and successfully deployed the remaining sponsorship/funding of the £10,000 GBP sponsorship from Alnylam, Disc Medicine and a donation via the UPA from Mitsubishi Tanabe for 2023's *Global Porphyria Day project* for GPD on 18 May 2024. Anna Mann again took a significant and active role in the deployment of this project which achieved the successful delivery of disease specific awareness videos on EPP, AIP, VP and HCP and a final video on CEP which will be shared at ICPP in Pamplona. The #MyPorphyria awareness campaign was deployed across social media and encouraged the sharing of further #MyPorphyria content. We are very grateful to the patients that bravely and openly shared their stories of the lived realities of living with porphyria, in their own words and in their own language, #MyPorphyria! We are very grateful that our sponsors agreed that GPAC could retain the 2023 sponsorship funds to fulfil the project aims in 2024. Thank you to the Awareness Team (Anna, Kristen, Nicole, Kelly) for your work on this successful GPD/#MyPorphyria videos and 'Light up for porphyria' – steered predominantly by Kelly Burns (part of the Awareness WG, from the Canadian association).

Website and Office 365: For the last few years GPAC have been planning on setting up a new website and transferring our emailing/file structures to Office 365. GPAC would like to specifically thank Anna Mann for her initial work on the new website –

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which has a look and feel that represents our organisation better and which was timed to be ready to launch the new GPD videos. This is just the first stage of the website and we look forward to developing this further. We'd also like to thank Antony Fearn for his voluntary work in transferring GPAC's email platform from Mijndomein to Office 365, a free platform to GPAC, as we are a registered charity in the UK.

Research and GPAC: Over the last year or so, various global patient representative from the GPAC community have been involved in publications relevant to porphyria, the rare disease community, trials and regulatory processes/bodies responsible for providing access of medicines to patients. Thank you to all for your commitment to these areas.

Challenges and resolutions: GPAC has been working find ways to work in a collaborative and inclusive manner and all New Members will be encourage to abide by this approach to working together, in a respectful and committed professional manner. *GPAC's Bylaws and information pack, GPAC's Membership guidelines and joining form* as well as *GPAC's Executive and trustee declaration form* were all agreed at our last AGM and are being implemented to support all of GPAC's work.

Internal GPAC resource became a significant problem in 2023, when the President was off sick injured for a number of months! Since February 2024, this workload is being spread more between the Executives and we have also noted a shift as a number of key individuals are coming forward to help steer and share the workload. This model appears to be working better and at the meeting in July, it was decided to 'refocus' our Working Groups to be more focused on tangible outputs.

As Kika Shabot and Derry Wilcox move into their Executive roles, there has been a positive shift and new fuel within the Trustee Board to prioritise and accomplish clear and tangible action points. With this shift, Sue Burrell is currently happy to remain in the post of President and suggests looking to step back in around 12 or 24 months.

Strategic aims identified for 2024-2025

In addition to healing divisions within GPAC, which will be imperative for success. GPAC has many aims for the next 12 months.

- **Executives will develop GPAC's infrastructure** to enable the succeeding Executive Board to continue to move GPAC forward. Transition of Derry into the Treasurer role and as a bank signatory, as well as development of the necessary *policies and procedures* to protect GPAC and our Members.
- **GPAC resilience: Identify candidates** interested in Working Groups and succession planning for GPAC to ensure the Executive Team is sustainable and resilient.
- **Members: Elections of Members:** the Joining Forms will be finalised and all PAGs wishing to become Members will be voted as such at the AGM in Pamplona, September 2024.
- **Finances: Evolve workload plans and resources to fund projects:** funding will be secured to enable GPAC to work professionally and recruit appropriate resources to manage projects identified by the Working Groups. In Pamplona, they set tangible and clear aims, objectives and action/endpoints that can be reviewed at each meeting, we have received nearly £18000 as of November 2024
- **Awareness WG** will identify end points around RDD/GPD/PAW.
- **Mental health WG** will focus on how specific points on protecting and supporting the minds of the porphyria community.
- **Information sharing WG** will identify specific aims to include finalising the website, management of social media, newsletter which will be fully translatable, via our website, in order to increase accessibility for the whole porphyria community.
- **New groups WG** will be welcoming and supportive of individuals and rare disease groups who are committed to working together, professionally and in-line with our Charter for Working Together.
- **Humanitarian WG** will work alongside physicians and patient groups to guidepost patients and potential patients to diagnostics, care and treatments.

GPAC also hopes to work in 2025 to establish more formalised collaboration routes with physicians, scientists, rare disease networks and other stakeholders, including pharmaceutical networks in order to establish meaningful alignment with shared goals/purpose for priorities and goals. We'd also like to acknowledge thanks to the patient advocacy researchers who keep contributing/pushing research to improve things for porphyria patients.

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Global Porphyria Advocacy Coalition Income Statement

November 11 2023 to November 10, 2024

Income	
Grants	£0.00
Donation	£24,290.00
Sponsorship	£0.00
Membership Donations	£562.60
Other Income	£0.00
Total Income	£24,852.60
Expenses	
Awareness	£4,000.00
Communications	£143.88
Website	£168.78
Meetings/ Gatherings	£0.00
Administration	£0.00
Travel	£0.00
Conferences	£0.00
Dues/ Membership fee	£0.00
Tax	£0.00
Total Expenses	£4,312.66
Net Income (Loss)	£20,539.94
Current Balance	£28,660.73

Income Statement	
September 11 2023 to November 10,	
Income	
Grants	£0.00
Donation	£24,290.00
Sponsorship	£0.00
Membership Donations	£562.60
Other Income	£0.00
Total Income	£24,852.60
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England & Wales - Charity number 1189694

Accounts

President's Report

It is now 5 years since the Global Porphyria Advocacy Coalition's (GPAC's) first official meeting in 2019 in Milan. The Executives of Sue Burrell (President), Sean Hegarty (Vice President) and Joaquin Montoto (Secretary) are still in position, but are looking to take on Stewardship roles in 2024, in readiness for a new set of Executives taking up positions, by our next AGM in September 2024.

Our last AGM (Sept 2023) took place at the International Congress on Porphyrins and Porphyrias (ICPP) in Sofia, Bulgaria. It was wonderful to see so many PAG leaders from around the world back together in a face-to-face setting after the Covid pandemic! A further 5 country Members joined our meeting via Zoom. In advance of the AGM, Marco Mo resigned from the role of GPAC Treasurer, a post which still remains unfilled. Over the last 12 months, GPAC have not managed to meet regularly, due to various life-changing personal/family matters and a period of complete inactivity due to illness/injury effecting the voluntary contributions of the President and Vice President. This has had a direct and significant impact on GPAC's activities and achievements. GPAC has also been drawn into a problem within the community which has taken time away from pushing GPAC initiatives at the pace seen in previous years. Collectively, this has resulted in the halting of many GPAC initiatives and progress.

We are excited to be holding our Delayed-2023 AGM, via Zoom, on Saturday 3rd February 2024. On completion of the AGM, the Executives will encourage collaboration, professionalism and working together, as this is in the best interests of GPAC and will help to foster an engaged and stronger global porphyria patient community - a voice for patients that needs to be heard!

Progress to date

Meetings, connections and fostering global relationships: The GPAC Executive Board have only met on a couple of occasions to move actions forward for GPAC, since September 2022. The President has individually spent many additional hours working to resolve conflict in a way that all parties can share their voice. The President and Vice-President have met to discuss aims, strategic direction and other tasks. The Executive also worked with pharma/industry representatives and clinical leaders, on numerous occasions, about sponsorship, disease awareness events/communications and to foster partnering relationships to help share information about trials, trial data, new medicines, research and awareness.

GPAC had a very successful AGM and Global Gathering in Sofia, Bulgaria, 2022 with excellent presentations/talks/updates from Modern Inc., Disc Medicine and Alnylam Pharmaceuticals. After the meeting, there was an opportunity to network for all attendees, this was enabled with (gratefully received) sponsorship from Alnylam Pharmaceuticals and Disc Medicine. These networking opportunities are invaluable, they foster relationships which help enable deeper connections and understanding around the patient and their needs for trials and end-points, as well as research and change needed within the porphyria sphere, that may not otherwise have occurred.

Sue Burrell (GPAC's President) attended and presented (as a PAG Leader for GPAC and the British Porphyria Association, BPA) at two leadership meetings organised by Alnylam Pharmaceuticals. The first in Berlin, in February, alongside Ipnnet physicians on 'The importance of leveraging the power of patient voices' to a group of European clinicians interested in the acute porphyrias. The second, was alongside two other patient leaders from other rare disease areas at a series of 'Patient advocate consulting sessions' in Boston, USA, to discuss the challenges affecting patients in rare disease areas. Sue Burrell received honorariums for these engagements via her paid role with the BPA.

GPAC's Executives: Following the AGM in 2022, GPAC finalised a number of pivotal documents which are now able to support the running and day-to-day management of GPAC, including: *GPAC Bylaws and information pack; Charter for working together; GPAC Membership guidelines and joining form; GPAC Executive and trustee declaration form.*

GPAC's Working Groups (WG) despite GPAC having a number of working groups, including: *Cross Border Support WG; Media WG; Treatments and Research WG and GPAC and Research.* Most have not met 'officially' over the course of 2023. Nonetheless, work has still been ongoing via our Members (namely, between groups in America [APF and UPA], Brazil, Canada, Chile, Italy, Mexico, Spain – to name a few)! There have been specific collaborations between a number of organisations to deliver education events/conferences (face-to-face, hybrid and virtually) to educate, connect and empower patients and physicians. The success of which demonstrates the benefits that can be achieved from working together.

The *Awareness WG* secured just over £10,000 GBP sponsorship from Alnylam, Disc Medicine and a donation via the UPA from Mitsubishi Tanabe for 2023's *Global Porphyria Day project* (April 19th 2023) which fit with the APF's Porphyria Awareness Week. In 2024, the decision is to move this to have GPD on 18 May, to fit with the Spanish International Porphyria Day, it was suggested that the 11-18 May 2024 could be Porphyria Awareness Week which will be confirmed in due course. The Global Porphyria Day project aimed to record and release a number of disease specific awareness videos as part of the #MyPorphyria awareness campaign. The goals were to raise awareness of porphyria to help with diagnosis, testing and understanding of the lived realities of porphyria by engaging the global porphyria community to share their real-life stories, in their own words and in their own language, #MyPorphyria! The project paused when the President had an injury and was off work for several months. All sponsors have agreed that the sponsorship funds received in 2023 can be retained by GPAC in order to fulfil the project aims in 2024. We are very grateful for the funding and look forward to continuing the project. The first video was greatly received and much of the work on the pre-recorded content can be continued in time for 2024 awareness efforts. The EPP video will be the next footage circulated.

Research and GPAC: Over the last year or so, various global patient representative from the GPAC community have been involved in publications relevant to porphyria, the rare disease community, trials and regulatory processes/bodies responsible for providing access of medicines to patients.

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Delayed to
03-Feb-2024



Challenges faced: GPAC is still finding ways to work in a collaborative and inclusive manner and our *Charter for Working Together* asks all GPAC Members to respect and commit to working together professionally. *GPAC's Bylaws and information pack, GPAC's Membership guidelines and joining form* as well as *GPAC's Executive and trustee declaration form* were all agreed at our last AGM and will be implemented to support all of GPAC's work.

Internal GPAC resource became a significant problem in 2023, when the President was off sick injured for a number of months! Upon returning to fitness and work, the President needed to prioritise paid work, rather than voluntary endeavours which has stunted GPAC's progress across 2023. This highlighted very clearly that the current President has been doing the bulk of the work and this is an unsustainable format – which needs to be addressed.

The existing Executives would like to step down from their roles (ASAP). The current Trustees are looking for a new and enthusiastic leadership team to steer GPAC forward in an engaged, professional and inclusive manner. The process for the Election of Officers was agreed at the last AGM in Sofia and was planned for early 2023, this will now occur in 2024 ready for a formal transition as soon as possible.

The conflict noted within the GPAC community has taken time away from other porphyria work and has had an emotional impact too. This will be addressed at the Delayed- 2023 AGM to find a way of drawing a line under it and moving GPAC forward.

Strategic aims for 2024

In addition to healing divisions within GPAC, which will be imperative for success. GPAC has many aims for the next 8 months.

- **Develop its infrastructure** to enable the succeeding Executive Board to continue to move GPAC forward, including tasks around the *website, Office 365* and a series of necessary *policies and procedures* to protect GPAC and our Members.
- **Identify candidates** interested in Executive positions
- **Elections:** hold an EGM (Extraordinary General Meeting), if possible. Or work towards election of new officials (with a supported transfer via Stewardship) – by the latest date of 2024 AGM in Pamplona, September 2024.
- **Evolve workload plans** to ensure that the Executives are not overstretched and that the entity remains sustainable now and in the future. A plan will be agreed at the AGM to determine how this will work.
- The **Awareness** group will fulfil the GPD/PAW plans measured by the delivery of a successful awareness campaign.
- The Working Groups will continue to evolve by setting tangible and clear aims, objectives and action/endpoints that can be reviewed at each meeting.
- Explore the suggested **Buddy System** to support New Groups to assess whether it is achievable
- Assess whether it is achievable to source full **translation services** - which are costly, but would significantly increase accessibility for the whole porphyria community.
- Be involved in **future research** with other industry representatives so the burden of porphyria is more widely understood and to further validate to the existing patients that they are not alone in the impact their porphyria has on them and their families. Patient Leadership Council's may form some of this work. We'd also like to acknowledge thanks to the patient advocacy researchers who keep contributing/pushing research to improve things for porphyria patients.
- GPAC still has desires for a **Research and Information** WG which will develop an online resource that will store papers such as these, to increase access for the whole GPAC community.

Huge THANKS: GPAC would like to thank Desiree Lyon for her dedication and service to the global porphyria community over 40+ years, she has been a force and inspiration to patients around the world for many years... we wish her the very best in her retirement!

Treasurer's Report

All funds were very gratefully received by GPAC.

GPAC's accounting period runs from 11 November to 10 November – and is reported to the Charity Commission Annually.

Detailed income/expenditure for 11-Nov-2021 to 10-Nov-2022 and 11-Nov-2022 to 10-Nov-2023 will be circulated with this Annual Report for 2023.

Summary: Since GPAC began, we have received an overall income and expenditure as follows and currently have £7,976.91 in GPAC's Bank Account (around £6,000 is already allocated to GPD/PAW 2024).

Summary of GPAC Accounts to date: as of 3-February 2024		All figures noted in GBP	
GPAC Total Income received to 3-February 2024		GBP	
		Income	GBP
Total Income			£15,752.11
GPAC Total Expenditure to 3-February 2024			
		Expenditure	
Total expenditure			-7,775.20
Total funds available for GPAC (as of 3-February-2024)		£7,976.91	

Income and Expenditure Account for the period 11 November 2022 - 10 November 2023
Global Porphyria Advocacy Coalition
CIO Reg No: 1189694

	GBP £	GBP £
Income	8884.8	8884.8
Less Expenditure		
Software subscriptions (Zoom)	143.88	
Website and telephone	153.73	
GPAC events - room hire/catering	1819.72	
Financial fees	50.04	
Video editing	4750	
	<u>6917.37</u>	6917.37
Income/expenditure		<u>1967.43</u>

GLOBAL PORPHYRIA ADVOCACY COALITION

England & Wales - Charity number 1189694

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President's Report

The Global Porphyria Advocacy Coalition's (GPAC's) first official meeting was in Milan 3 years ago, at which point the first Executive Board was elected. At our last AGM (November 2021), Peder Sorenson (from Denmark) resigned as Treasurer and Marco Mo (from Canada) was voted as GPAC's new Treasurer. Over the last 12 months, GPAC have met regularly via Zoom to continue to establish GPAC's infrastructure. The rare disease community and GPAC have been impacted over the last three years in terms of networking opportunities and time to progress with GPAC aims, as a result of Covid/pandemic. We are very excited to be holding our 2022 AGM in Sofia, Bulgaria on Saturday, 4th September, where we will meet with many of our porphyria friends and colleagues as part of the biennial International Congress on Porphyrins and Porphyrin.

Progress to date

Meetings, connections and fostering global relationships: The GPAC Executive Board met virtually on 6 separate occasions to move actions forward for GPAC (20+ hours). The President and Vice-President met to discuss aims, strategic direction and tasks on at least 12 occasions (18+ hours). The President met on more than 15 individual occasions with various patient organisation leaders (12+ hours). The Executive also met with pharmaceutical/industry representatives on more than 6 occasions, resulting in a patient talk at one of our Member Meetings in May from Disc Medicine and planned talks/updates at our Global Gathering in Sofia from Modern Inc., Disc Medicine and Alnylam Pharmaceuticals. These connections for our rare community are invaluable in fostering change around trials and end-points as well as research conducted into the porphyrias, including quality of life studies. At our AGM Meeting last November, Professor Jean-Charles Deybach kindly delivered a very well-received pre-recorded presentation on new developments within the field of porphyria. GPAC Members meet virtually on 2 further occasions (7-8 hours), with representation from between 9 and 12 organisations. We are delighted to see that many organisations are including others from their group on the calls which is further engaging the community and allowing us to have a greater impact within the porphyria field. GPAC also represented the global porphyria community at an AHP Global Advisory Board with Alnylam, alongside other global porphyria leaders – an honorarium for which was donated to GPAC.

GPAC's Executives: In the last 12 months, GPAC has continued developing its infrastructure to enable they succeeding Executive Team to continue to move GPAC forward. GPAC managed to open a Global Community Bank Account with Barclays earlier this year, which resulted in various pledged donations being received from the APF, the BPA and Alnylam.

GPAC's Working Groups have focused on a number of projects which have evolved based on need/importance. In April, the **Awareness WG** delivered a wonderful Porphyria Awareness Week campaign – the theme 'We are porphyria together' was received well and observed as a great coming together of the porphyria patient community. The focus on the Friday of 'Purple for Porphyria' was visually impactful across social media and will be considered for inclusion moving forward.

Two new Working Groups emerged in response to needs in 2022. The **Cross-Border Connections WG** emerged from the desire to offer more support directly to patients, especially in countries where the communities are still small. The group held their first Global EPP Connect event, for people of all languages and ages. The event was a great success with around 25 people joining – a number of which had never met anyone with EPP before. This WG plans to hold another Global Connect event later this year, aimed at the Acute Porphyrias. GPAC will spend longer advertising and also focus more on native languages to generate the best discussions and accessibility for all.

More recently, in response to some very negative portrayals of porphyria in TV/streaming channels, a new **Media Working Group** has formed. The group is already active, responding to a specific TV programme on Netflix, as well as some other shows that are looking for storylines. The WG aims to develop strong responses and a general statement that can be endorsed by GPAC, patient groups and by the clinician networks. Thus giving a balanced voice for patients witnessing these negative portrayals. Further, the aim is to foster good relations that may lead to excellent portrayals of the porphyrias rather than sensationalised/inaccurate shows that are very upsetting to the porphyria patient community.

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. The German EPP group were delighted to report, earlier this year that Scenesse will be automatically renewed every year without the need for renewing/review. Scenesse is also available in Israel – congratulations to all involved in both of these achievements! Givlaari is available to patients in Poland too – again creating a step-change in the treatments available to patients. The APF Global Program continues to support numerous fledgling porphyria patient groups that GPAC hopes will become Members and support in due course.

Research and GPAC: Over the last year or so, various global patient representative from the GPAC community have been involved in the *Porphyria Worldwide Patient Experience Research (POWER) study*. Contributing to the design of the study, promoting participation amongst the global community and, over the last 12 months, being included within authorship for journal and conference submissions. The full manuscript was published in the Journal of Inherited Metabolic Diseases, it has since been presented with a different focus at 5 other conferences, and endorsed at further 5 conferences. This piece of work is a wonderful demonstration of the collaboration between industry, patient representatives and the patients themselves.

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2 New Working Groups

60+ Hours of collaborative working

3 New actively involved groups

Challenges faced: GPAC is still a very new organisation and we're still finding ways to work in a collaborative and inclusive manner. At our February 2022 Meeting the Vice-President led an interactive session aimed at developing a Charter for Working Together. The information gathered will be built into a Charter that we will ask all GPAC Members to respect and commit to. Alongside this, the Executives have been working on a Membership and Joining Procedure as well as a general Information Pack to support the work GPAC does and will do in the future.

Resource continues to be a huge problem and has resulted in a delay to finalising Membership processes and the Election of a new set of Executives to GPAC. A number of the Executives wish to step down/change roles and new enthusiasm and tenacity is needed within GPAC. The process for the Election of Officers will be presented, alongside a Membership Process (whilst in Sofia), once agreed a planned Extraordinary General Meeting (EGM) will be scheduled for early 2023.

Strategic aims for 2022

GPAC has many aims for the next 12 months. The Executive Committee will continue to **formalise GPAC's official infrastructure** over the coming weeks (including establishing bylaws, policies and procedures, roles and responsibilities, Member joining protocols as well as a membership fee collection processes), by the end of January 2023. To ensure all is ready for the EGM and to allow for **Elections** and a strong transfer of skilled individuals to a **New Executive Team**.

GPAC has plans to update the **website** – offer of assistance has come from the UPA and it may be that a WG could take this on as a project, to share the work. And, there has been an offer of help to register GPAC for **Office365** by a member of the BPA. The Working Groups will continue to evolve by setting tangible and clear aims and objectives (and roles and responsibilities) that can be reflected upon at our next AGM in 2023. As part of this, GPAC needs to evolve to ensure that the Executives are not overstretched and that the entity remains sustainable now and in the future.

It is hoped that GPAC will start working more with a **Buddy System** to ensure that New Groups are identified and better supported by the Members Organisations. **Translation services** will also be investigated as a real option to increase accessibility to our meetings and the community – it may be necessary to apply for a specific budget for this.

GPAC hopes to be involved in **future research** with other industry representatives so the burden of porphyria is more widely understood and to further validate to the existing patients that they are not alone in the impact their porphyria has on them and their families.

GPAC would also like to acknowledge and thank a few key people who have been involved in a number of other research publications. A new **Research and Information WG** will be starting work shortly on an online resource that will store papers such as these, to make it easy for the GPAC community to access them.

Treasurer's Report

In March 2022, we had confirmation that GPAC's Community Account with Barclays Bank was officially open. Soon after which GPAC contacted the APF, the BPA and Alnylam regarding the staff donation from 2021 to GPAC. All funds were very gratefully received by GPAC, as follows.

GPAC Income 2022		GBP	GBP
Date received		Income	
29/03/2022	BPA – initial donation 500 GBP	£500.00	
17/05/2022	APF – initial donation 1,000 USD	£780.47	
10/06/2022	Alnylam staff donation 5,000 USD*	£3,981.16	
09/08/2022	Honorarium payment for ad-board Alnylam	675.00	
01/09/2022	Alnylam Sponsorship for AGM & Global Gathering	£500	
Total Income		£6436.63	£6436.63
Expenditure in 2022			
31/08/2022	Expenses to S.Burrell of £713.95 (All paid and reimbursed to S.Burrell - Webdomain 2020-2022, Zoom, Epnet Associate Membership of 400 Euros 2019-2022)	£713.95	
Total expenditure		- £713.95	- £713.95
Gross profit			£5,722.68

Please Note: On GPAC's projected income on the last financial report, there was a note to reimburse 30 Euros to Danny De Roode. However, Danny kindly decided to gift/donate this cost to GPAC, so has been removed from the accounts. *Also note, the Alnylam staff donation of 5,000 USD, not the 3,000 USD initially documented.

Income and Expenditure Account for the period 11 November 2021 - 10 November 2022

Global Porphyria Advocacy Coalition

CIO Reg No: 1189694

	GBP £	GBP £
Income	6867.31	6867.31
Less Expenditure		
Software subscriptions (Zoom)	143.88	
Website and telephone	213.07	
Memberships (professional)	357	
	<u>713.95</u>	713.95
Income/expenditure		<u>6153.36</u>

GLOBAL PORPHYRIA ADVOCACY COALITION

England & Wales - Charity number 1189694

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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 – ICPP) – 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 (Epn¹, APEX², BIPNET³ and Porphyria South Africa⁴). GPAC also formulated a Covid-19 Statement, based on guidance from APEX and Epn¹, 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 IPPN⁵ 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

¹ Epn¹: European Porphyria Network

² APEX: American Porphyrias Expert Collaborative

³ BIPNET: British and Irish Porphyria Network

⁴ Porphyria South Africa associated with University of Kwazulu-Natal

⁵ IPPN: International Porphyria Patient Network

Annual Report 2021



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	
TOTAL forecasted income	£3,430.00	£3,430.00
Expenditure (ACTUAL – to be reimbursed to relevant parties once bank account open)		Detail
Mijndomein website/domain 2019-2020 (15-Sep-2019 – 35 Euros)	£30.00	Paid by Danny De Roode – still due to Danny De Roode
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 – still due to S Burrell
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 – still due to S Burrell
Zoom annual meeting account 2021-2022 (10-Nov-2021)	£143.88	Paid by S Burrell – still due to S Burrell
Expenditure (DUE)		
Epnet Associate Membership – 3yrs – 2019-2021 (04-Nov-2021) – 300 Euros (due)	£260.00	
Total projected expenditure*	£646.95	£646.95
		Gross projected profit
		£2,783.05

*Including reimbursement to all parties and owing invoices