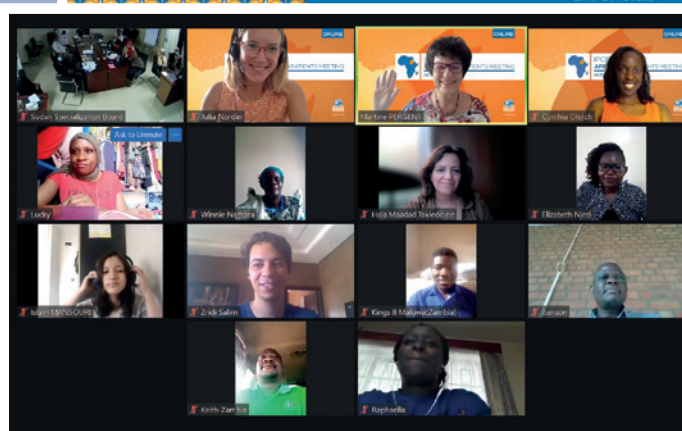
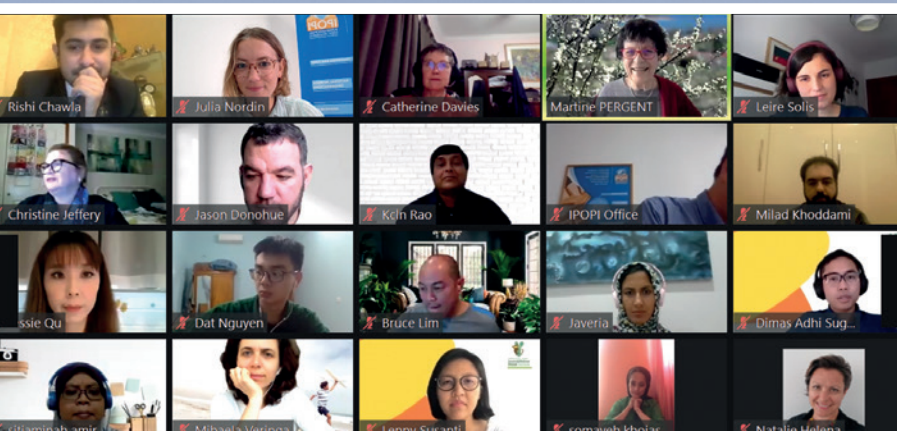


REVIEW OF ACTIVITIES AND ACHIEVEMENTS 2021



JOIN IN TO DISCUSS HOW TO BEST TACKLE PID PATIENTS CHALLENGES IN INDONESIA



an IPOPI event



IPIC2023

INTERNATIONAL
PRIMARY
IMMUNODEFICIENCIES
CONGRESS

DIAGNOSIS
AND CLINICAL CARE

NOVEMBER 8-10, 2023

ROTTERDAM
THE NETHERLANDS

www.ipic2023.com

A MESSAGE FROM IPOPI'S PRESIDENT AND EXECUTIVE DIRECTOR



MARTINE PERGENT
President



JOHAN PRÉVOT
Executive Director

During the beginning of 2021, we were still battling the COVID-19 pandemic, participating in the race for the SARS-COV-2 vaccine in every country. Improvements were observed as the year went by, with better management of the disease and vaccines proving their efficacy.

IPOPI swiftly adapted to the circumstances during this time. Because of the COVID-19 pandemic, we made the wise decision to move the 5th International Primary Immunodeficiencies Congress (IPIC) from November 2021 in Berlin, Germany, to April 2022 in Vilamoura, Portugal. The bold decision to relocate and postpone our dates was motivated by our desire to provide an optimal solu-

tion for our delegates with the hope to be able to meet again in-person. As a result, clinicians, patient group leaders and representatives, nurses, industry stakeholders, and other healthcare professionals were able to gather in Vilamoura in April 2022 and contribute to a very successful congress. We could not be more pleased with the outcomes of this meeting and are already preparing for IPIC2023 in Rotterdam, 8-10 November 2023.

At the end of 2020, IPOPI launched its new strategic plan aimed at shaping its activities between 2021-2025. The plan was designed considering IPOPI's significant growth over the last few years whilst being clearly geared to the future. In the coming years IPOPI will shape its activities to improve access to early diagnosis and patient-centred care, build capacity and support IPOPI's national member organisations, educate, promote knowledge and data sharing to increase understanding of PID and strengthen multi-stakeholder cooperation. Guided by the strategic plan, IPOPI has continued to strengthen its educational programme in 2021 with six new educational PID leaflets, a mini documentary and several informative clinical webinars on a range of topics. We have also been working hard to organise three successful regional PID patient meetings in Africa (Hybrid), Latin American (Online) and in Asia (Online advocacy workshop). In addition, we have continued to contribute to stakeholder collaboration in initiatives such as Screen4Rare and ERN-Rita, among many others.

When it comes to advocacy, we also did not rest. As a result of IPOPI's collaborative efforts with stakeholders, several new PID diagnostic tests were included in the World Health Organization's (WHO) second Model List of Essential In Vitro Diagnostic in 2021. This is a significant accomplishment and benefit for the PID community, and we remain committed to working with the WHO as the EDL evolves in the future. IPOPI also started to engage in strategic discussions with the WHO and the International Society for Blood Transfusion around a plan to improve access to plasma medicinal products in low- and middle-income countries. More news on this in our next report!

Newborn screening has also been an important topic for IPOPI for many years. In 2021, IPOPI played a pivotal role in the launch of the International Neonatal Screening Day (INSD), held on June 28, as a joint initiative with the International Society for Neonatal Screening, and the European Society for Immunodeficiencies. INSD is an excellent opportunity to raise awareness about the importance of neonatal screening while also considering the best way to implement, improve, or expand its practises.

Information is a powerful tool, and we aim to continue empowering our community through increased awareness of key PID patient priorities. We were therefore pleased to publish data from 60 countries in the PID Life Index, IPOPI's tool for aggregating national PID data, in November 2021. The data show that, despite global progress, access to and affordability of PID diagnostic tests and treatment remains uneven around the world. Collaborative efforts are thus required to continue advancing PID care globally. IPOPI is fully committed to taking on this challenge, and we look forward to collaborating with our NMOs and other stakeholders to achieve this goal in the coming years.

Martine Pergent
IPOPI President

Johan Prévot
IPOPI Executive Director

EXECUTIVE COMMITTEE, OFFICERS AND STAFF 2021

EXECUTIVE COMMITTEE		PANEL MEMBERS	
<div>Martine Pergent – Chair</div> <div>Christine Jeffery – Vice-Chair</div> <div>Bruce Lim – Treasurer</div> <div>Jose Drabwell</div> <div>Andrea Gressani</div> <div>Cynthia Olotch</div> <div>Roberta Anido de Pena</div> <div>John Seymour</div> <div>Otilia Stanga</div>		<div>Dr Adli Ali – Malaysia</div> <div>Prof Tadej Avcin – Slovenia</div> <div>Prof Aziz Bousfiha – Morocco</div> <div>Prof Antonio Condino-Neto – Brazil</div> <div>Prof Charlotte Cunningham-Rundles – US</div> <div>Dr Virgil Dalm – The Netherlands</div> <div>Prof Tandakha Dieye – Senegal</div> <div>Prof Alain Fischer – France</div> <div>Prof Isabelle Meyts – Belgium</div> <div>Dr Olaf Neth – Spain</div> <div>Dr Silvia Sánchez-Ramón – Spain</div> <div>Prof Anna Shcherbina – Russia</div> <div>Prof Surjit Singh – India</div> <div>Prof Stuart Tangye – Australia</div> <div>Prof Klaus Warnatz – Germany</div>	<div>HONORARY CHAIRPERSON</div> <div>Prof Helen Chapel – UK</div> <div>HONORARY VICE-CHAIR</div> <div>Dr Teresa Español – Spain</div>
MEDICAL ADVISORY PANEL			
<div>Dr Nizar Mahlaoui, Chair – France</div> <div>Prof Martin van Hagen, Vice Chair – Netherlands</div>			
STAFF			
<div>Johan Prevot: Executive Director</div> <div>Maria Ascenso: Administrative Assistant</div> <div>Clare Glynn: Accounting and Administration Manager</div> <div>Natalie Helena: Events Manager</div> <div>Magda Lourenço: Communications Manager</div>		<div>Carla Morgado: Office Manager</div> <div>Julia Nordin: NMO Programmes Manager</div> <div>Leire Solis: Health Policy and Advocacy Senior Manager</div> <div>Carol Tavener: Accounting Assistant</div>	

NETWORK OF EXPERTS

The **IPOPI Network of Experts** (IPOPI NEX) was launched in 2021 as a consultative network providing expert advice and information to the IPOPI Board of directors. NEX's consultants do so on a voluntary and needs-driven basis. Their fields of expertise are diverse but differ from the PID clinical and scientific expertise of IPOPI Medical Advisory Panel (MAP) members. Unlike the MAP, the IPOPI NEX is not a formal body in the governance structure of IPOPI. It is a consultative network of experts that helps to ensure IPOPI can implement a multidisciplinary approach to its work.

CURRENT MEMBERS OF IPOPI NEX

Nurse Mary Louise Daly (Nursing)
Dr Lotte Denning (General Practitioner)
Professor Albert Farrugia
 (Plasma industry/access specialist)
Professor Bobby Gaspar
 (Advanced therapies industry specialist)
Professor Jacqueline Kerr (Regulator)
Professor James Taylor (Ethicist)

IPOPI STRATEGIC PLAN

MISSION AND OBJECTIVES

IPOPI is a non-profit international organisation and the leading advocate for primary immunodeficiency (PID) patients worldwide, working in collaboration with patients, doctors, politicians, regulators, the pharmaceutical industry and other relevant stakeholders.

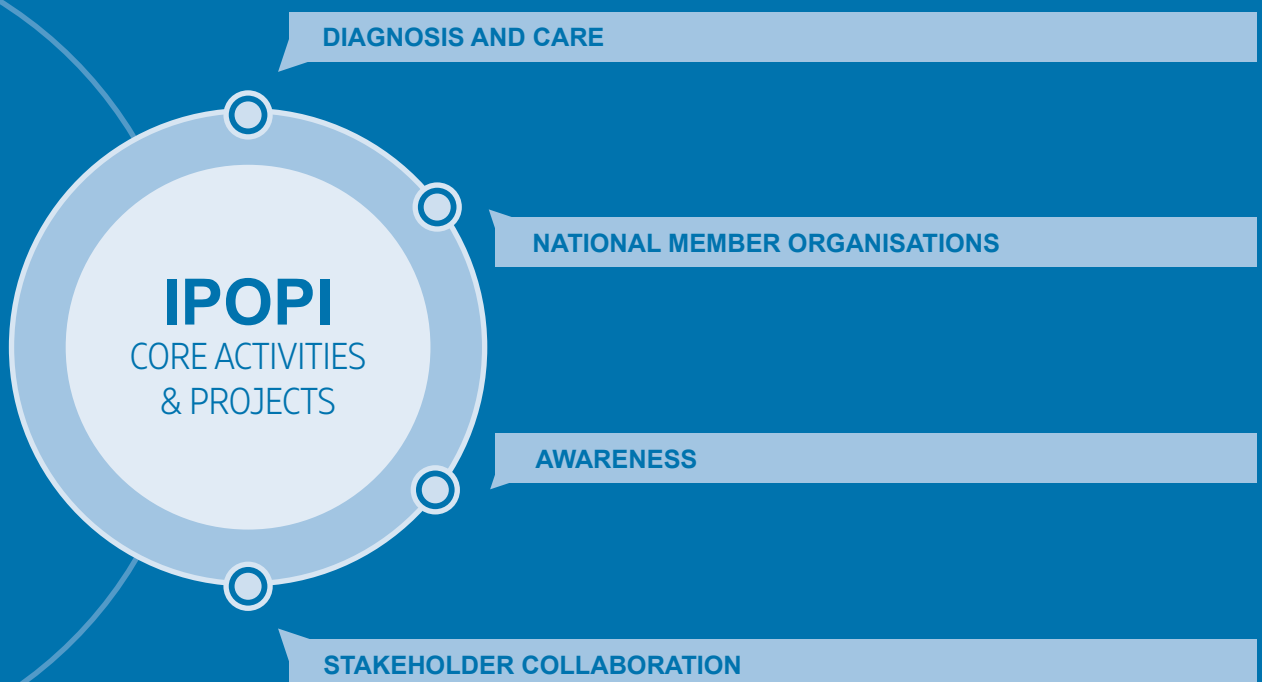
IPOPI is the Association of national PID patient organisations dedicated to improving awareness, access to early diagnosis and optimal treatments for primary immunodeficiency patients worldwide through global collaboration.

NEW STRATEGIC PLAN FOR 2021-2025

IPOPI undertook a comprehensive process to develop its new 5-year strategic plan for the 2021-2025 period. The plan was designed to take into account IPOPI's significant growth over the last few years whilst being clearly geared to the future. An in-depth analysis of the fast-evolving primary immunodeficiency (PID) environment and patients' priority needs and outlooks, enriched by the consultative input of key stakeholders, guided IPOPI's Board and Staff in the plan's development process. IPOPI's 2021-2025 strategic plan will ensure the continued success of IPOPI in its mission to increase awareness of PIDs, improve access to care and diagnosis for all PID patients in the years to come and ultimately improve their quality of life.

IPOPI STRATEGIC OBJECTIVES 2021-2025

- 1 - Improve access to early diagnosis and patient-centred care *through advocacy and awareness*
- 2 - Build capacity and support IPOPI's national member organisations *to improve living conditions for people living with PID*
- 3 - Educate, promote knowledge and data sharing *to increase understanding of PID, improve clinical care and advance research*
- 4 - Strengthen multi-stakeholder cooperation *to optimise all programmes and activities*



WHAT ARE PIDS?

Primary immunodeficiencies (PIDs, also referred to as 'Inborn errors of Immunity' — IEI) are a large and growing group of over 485 different genetic defects caused when some components of the immune system (mainly cells and proteins) are lacking or do not work properly.

Whilst PIDs are generally recognised as rare disorders, some are more common than others and, taken as a whole, they represent an important group of people whose lives are profoundly impacted by their condition.

The majority of PIDs are caused by genetic defects of the immune system which are hereditary for most of them. The immune system normally helps the body fight off infections caused by germs (or 'micro-organisms') such as bacteria, viruses, fungi and protozoa. Because their immune systems do not work properly, people with PIDs are more prone than others to infections. It also helps in preventing inflammation, autoimmunity, severe allergies and malignancies.

When PIDs are left underdiagnosed or are misdiagnosed, the immune system remains defective, often leading to illness, disability, permanent organ damage or even death.

DIAGNOSIS

Whilst it is estimated that around 60% of PIDs can be easily diagnosed with simple and inexpensive blood tests, many PIDs remain underdiagnosed on a global scale. Hopefully, as the technical ability to identify gene improves, more and more genetic causes of PID will be identified.

Medicine and Science are advancing very rapidly and novel therapies that target the specific cause of the disease are becoming available. These may have significant advantages for PID patients, and patients throughout the world must have access to these treatment possibilities.

Screening for some of the most severe forms of immunodeficiency, such as Severe Combined Immunodeficiencies (SCID), will also diagnose more individuals at an earlier stage, thus improving treatment outcomes.

TREATMENT

With appropriate access to different and very effective therapies, which can only happen if an early and correct diagnosis is made, many sequelae will be avoided and lives of patients will be saved.

People with PIDs are normally treated by doctors who specialise in diseases of the immune system. How a patient is treated depends on which PID they have and on many other factors. Mainstay or curative treatments for PIDs include immunoglobulin (IG) replacement therapies, Hematopoietic Stem Cell Transplantation (HSCT, also known as 'bone marrow transplantation'), gene therapy, cytokines such as Granulocyte-colony stimulating factor (G-CSF), recombinant gamma interferon, enzyme replacement therapy such as recombinant adenosine deaminase for patients with ADA1 deficiency, anti-infectious prophylaxis (with antibiotics, antifungals and/or antivirals), monoclonal antibodies, among others.

In the case of IG replacement therapies, it is important to know that different IG products are not interchangeable or mixable. IG can be administered intravenously or subcutaneously. They are considered essential medicines for PIDs by the World Health Organisation. Intravenous immunoglobulin (IVIG) infusion usually takes 2-4 hours allowing high doses to be given and only needs to be given every 3-4 weeks which may suit some patients better. Subcutaneous immunoglobulin (SCIG) can only be given in small doses. It only takes 1-2 hours and can often be given at home by patients themselves, parents or carers. It may therefore be more convenient for some patients. SCIG infusion preceded by an SC infusion of recombinant human hyaluronidase, which is known as 'facilitated' subcutaneous immunoglobulin (fSCIG) allows patients to have SC infusions every 3-4 weeks. IG therapy should be discussed on a shared-decision basis for each individualised case, according to patient needs and preferences.

CLASSIFICATION

PIDs are currently classified by the IUIS PID/IEI expert committee into 10 groups: predominantly antibody deficiencies; combined immunodeficiencies; combined immunodeficiencies with associated or syndromic features; diseases of immune regulation; congenital defects of phagocyte number or function, or both; defects of innate immunity; auto-inflammatory disorders; complement deficiencies, based on the main immunological defects; phenocopies of Primary Immunodeficiencies and inherited bone marrow failures.

For more details on the classification of PID read the IPOPI leaflet 'How are primary immunodeficiencies classified?'

IPOPI is committed to continuing to disseminate information to patients, their relatives and doctors and work towards better access to early diagnosis and treatment for all patients living with a PID.

For more information about PIDs and how they are treated and diagnosed and advice on how to stay healthy please refer to the IPOPI leaflets.



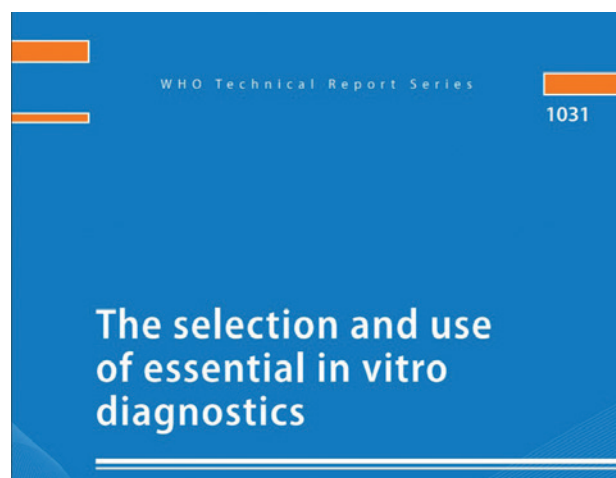
IPOPI'S KEY ACHIEVEMENTS AND ACTIVITIES, 2021

STRATEGIC OBJECTIVE 1 – IMPROVE ACCESS TO EARLY DIAGNOSIS AND PATIENT-CENTRED CARE

Through a range of advocacy, awareness raising and educational campaigns and programmes (including some aimed at improving clinical care standards) IPOPI remains steadfast in its commitment to bring about long-term sustainable improvements in the diagnosis of PID and equitable access to appropriate patient-centred care for all PID patients.

NEW PRIMARY IMMUNODEFICIENCY DIAGNOSTIC TESTS INCLUDED IN THE WHO EDL

The World Health Organization (WHO) published, on January 29, 2021, the third edition of the Model List of Essential In-vitro Diagnostics (EDL). For the second year in a row, tests for diagnosing primary immunodeficiencies (PIDs) had been included in this Model List.



In this edition, the WHO accepted IPOPI's proposal to expand the list for the lymphocyte subtype enumeration and the additional evidence required for tests provisionally accepted in 2019. The tests considered essential for PID diagnosis are:

- Complete blood count;
- HIV 1/2 antibody (anti-HIV Ab);
- Immunoglobulin plasma levels (IgG, IgA, IgM)
- Lymphocyte subtype enumeration (CD3, CD4, CD8, B cells CD19 and/or CD20, CD16/56 T cells and NK cells)

This is a key achievement for the PID community and IPOPI has been very keen in ensuring that PIDs were included in the WHO Model List of Essential In vitro

Diagnostics, as they were several years ago, recognised as a priority indication for immunoglobulin replacement therapies for children and adults.

IPOPI would like to express its gratitude to Prof Helen Chapel, Dr Nizar Mahlaoui, and the many medical experts that, with their time and guidance, made the applications a success. We would also like to thank all the experts from the international immunological societies that contributed to the application.

17TH IPOPI EU PID FORUM – PRIMARY IMMUNODEFICIENCIES. FIGHTING CANCER

The 17th IPOPI PID Forum on the topic of PIDs and Cancers was organised on April 22, 2021. The event was co-chaired by 2 Members of the European Parliament, Ms Irena Joveva (Renew Europe, Slovenia) and Ms Deirdre Clune (EPP, Ireland).

The event had the participation of Ms Martine Pergent, IPOPI President, Prof Isabelle Meyts, ESID President, Dr Fabian Hauck, from the Ludwig-Maximilians University of Munich (Germany), Ms Adriana Radovan, nurse at the Children hospital "Louis Turcanu" (Romania), Mr Raf Leijskens, parent of a patient with a PID (Belgium) and Ms Antonella Cardone, Director of the European Cancer Patient Coalition (ECPC).

The banner features a woman wearing a brown headscarf and holding a bouquet of yellow tulips. On the left, there is the IPOPI logo (a globe with a blue and orange design) and the text '17th PID Forum'. Below this, it says 'Primary Immunodeficiencies. Fighting cancer'. At the bottom, there is a blue bar with white text providing the date and time of the event, and the names of the co-hosts.

IPOPI 17th PID Forum
INTERNATIONAL PATIENT ORGANISATION FOR PRIMARY IMMUNODEFICIENCIES

**Primary Immunodeficiencies.
Fighting cancer**

22th April 2021
14:00-15:30 CEST

Event is co-hosted by: MEPs Irena Joveva (RE, Slovenia) and Deirdre Clune (EPP, Ireland)

Participants highlighted the complex relationship between primary immunodeficiencies (PIDs) and cancer. IPOPI made key recommendations for addressing the needs of cancer high-risk patient populations such as PIDs.

18TH IPOPI EU PID FORUM – CROSS-BORDER HEALTHCARE DIRECTIVE: SPOTLIGHT ON CURATIVE TREATMENTS FOR PID PATIENTS

IPOPI organised its 18th PID Forum titled “Cross-Border Healthcare Directive: Spotlight on curative treatments for PID patients”, on July 13, 2021. This online event was under the patronage of Members of the European Parliament (MEPs) Tomislav Sokol (EPP, Croatia), Alex Agius Saliba (S&D, Malta) and Radka Maxová (S&D, Czech Republic).

During the event, participants analysed the status of curative treatments for primary immunodeficiency (PID) patients in the EU and whether the Directive 2011/24/EU of the European Parliament and of the Council of 9 March 2011 on the application of patients’ rights in cross-border healthcare (also known as the Cross-Border Healthcare Directive - CHBD), is an opportunity to make these treatments more accessible to patients. The outcomes of these discussions were presented to the European Commission via their public consultation on “Cross-border healthcare – evaluation of patients’ rights”.

19TH IPOPI EU PID FORUM – DIGITALISATION OF HEALTHCARE: IMPROVING MEDICAL CARE FOR PID PATIENTS

On December 2, 2021, IPOPI organised its 19th PID Forum titled “Digitalisation of healthcare: Improving medical care for PID patients”. The online event was co-hosted by Members of the European Parliament (MEPs) Carlos Zorrinho (S&D, Portugal) and Marina Kaljurand (S&D, Estonia), and moderated by Johan Prevot, Executive Director, and Martine Pergent, IPOPI President.

During the event, participants analysed the state of digitalisation of healthcare for primary immunodeficiency patients in the EU, as well as how the European Commission’s digital initiatives provide an opportunity to make treatments more accessible to patients, improve their daily lives with digital tools, and how digitalisation could help medical providers and research and innovation in the field of rare diseases. The expert speakers shared their perspectives on policy developments and made recommendations on what needs to be done at the EU level to address current policy challenges. The results of these discussions were used to develop policy recommendations.



 **19th PID Forum**

Digitalisation of healthcare:
Improving medical care for PID patients

2nd December 2021
14:00-15:30 CET

Co-hosted by MEPs Carlos Zorrinho (S&D, Portugal)
and Marina Kaljurand (S&D, Estonia)

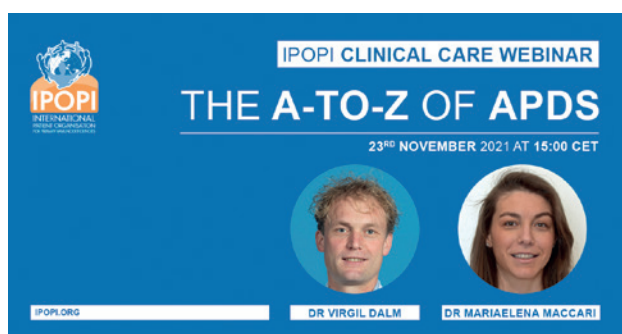
IPOPI PID CLINICAL CARE WEBINARS

The IPOPI Clinical Care Webinars are designed for clinicians interested in PID clinical care management, nurses, patient group leaders, and other PID stakeholders from around the world. Increasing understanding of key clinical care issues in this easy and straightforward manner has proven to be a popular concept and we are excited to continue providing other online educational programmes on relevant topics to the PID community.

In 2021, IPOPI organised the following webinars with the help of several dedicated PID specialists:

- Lung complications in PID with Prof John Hurst
- Transition care with Dr Nizar Mahlaoui
- Calculated globulins: clinical and lab perspectives with Prof Antonio Condino-Neto and Prof Stephen Holding
- The A to Z of APDS with Dr Virgil Dalm and Dr Maria Elena Maccari

All IPOPI Clinical Care webinars can be viewed on **TV.IPOPI.org**.



RELEASE OF COVID-19 LEARNING EXPEDITION REPORT

At the beginning of 2021, IPOPI released a report from the COVID-19 Learning Expedition (LEX). The LEX was an IPOPI project led by Martine Pergent (President) and Julia Nordin (NMO Programmes Manager) to identify, analyse, and provide recommendations on COVID-19 pandemic issues for patients with primary immunodeficiency (PID). Because people with PID are susceptible to infections, the COVID-19 pandemic created uncertainty and a desire to learn how to best manage this condition in the current environment. The goal of this project was to help the global PID community navigate the current health crisis and to strengthen patient expertise.

All of the common experiences were channelled into recommendations in the report, which covered a wide range of areas where PID patients have been affected since the pandemic began. The report is a content base created to assist IPOPI's National Member Organisations (NMOs) in their work during the COVID-19 pandemic or in future similar scenarios.

NEWBORN SCREENING & SCREEN4RARE



Aside from implementing SCID NBS national programmes in collaboration with its NMOs, IPOPI continued its newborn screening work at EU level through Screen4Rare. Screen4Rare is a multi-stakeholder initiative launched by IPOPI, the International Society for Neonatal Screening (ISNS), and the European Society for Immunodeficiencies (ESID), with the aim to exchange knowledge and best practises on NBS for rare diseases.

In February 2021, a **high-level stakeholder meeting** was hosted by the Members of the European Parliament Manuel Pizarro (S&D, Portugal) and Sirpa Pietikäinen (EPP; Finland). It gathered national and European health stakeholders to discuss the implementation of a European approach to newborn screening for rare diseases.

In September 2021, Screen4Rare and the European Reference Networks (ERNs) for metabolic disorders (MetabERN) and for primary immunodeficiencies (ERN RITA) promoted the **ERN Newborn Screening Initiative** in collaboration with the European Commission. At the meeting, three streams of work were presented to focus efforts on fields where meaningful progress could be made in neonatal screening.

Screen4Rare launched in November the **MEP Alliance for Rare Disease Newborn Screening**, which has a starting group of eight Members of the European Parliament.

MEDICAL ADVISORY PANEL

The MAP provides scientific and clinical advice to the Board of Directors. They contribute invaluable medical expertise to IPOPI PID information leaflets and the organisation of the IPIC (International Primary Immunodeficiencies Congress), as well as assistance with contacts from patients all over the world and emerging situations such as the COVID-19 pandemic, plasma and immunoglobulin supply, SCID Newborn Screening and gene therapy, among others.

IPOPI appreciates the commitment of all Panel members, but are especially grateful to Dr Nizar Mahlaoui (Chairman) and Prof Martin van Hagen (Vice-Chairman) for their kind contribution and dedication.



EFFORTS TO SECURE IMMUNOGLOBULIN SUPPLY

IPOPI strongly believes that it is critical to continue efforts to increase plasma collection and, thus, immunoglobulin supply. All policy discussions on these topics should be patient-centred, to allow people in need to access life-saving treatments, strengthen plasma collection in all world regions, and develop fractionation programmes where appropriate. During 2021, IPOPI led several collaborative efforts to advocate for global sufficiency in plasma-derived medicinal products (PDMPs) based on more regionally balanced plasma collection.

SAFE TASK FORCE RELEASES TWO POSITION STATEMENTS

The SAFE Task Force was launched by IPOPI in 2020 to monitor plasma collection and the availability of immunoglobulin replacement therapies for patients with PIDs worldwide, as well as provide guidance and support to patients with PIDs in their access to life-saving immunoglobulin therapies. During 2021, the SAFE task force released two position statements on key topics of relevance to patients with PID who need sustained & continued access to their Ig therapies.

Patients with PID need sustained & continued access to their Ig therapies! (QR Code 1)

Managing demand for immunoglobulins: PIDs are a priority indication at all times (QR Code 2)

The task force is composed of experts from different parts of the world and IPOPI staff (in alphabetical order): Ms Roberta Anido de Pena, Ms Jose Drabwell, Dr Nahla Erwa, Prof Stephen Jolles, Dr Nizar Mahlaoui, Ms Martine Pergent, Mr Johan Prevot, Prof John Seymour, Prof Surjit Singh and Ms Leire Solis.



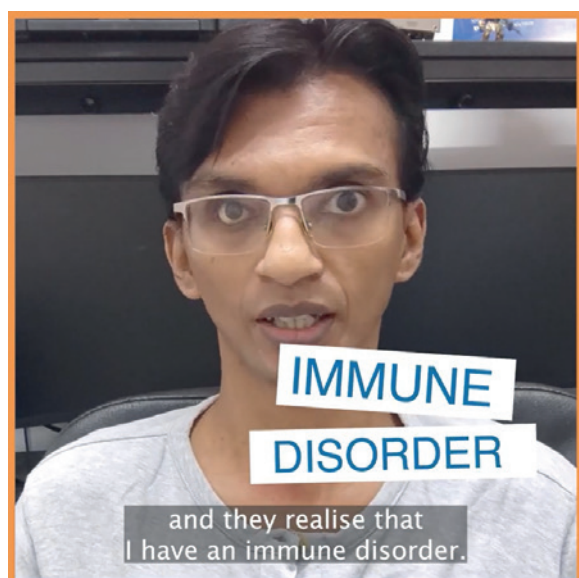
QR Code 1



QR Code 2

PID PATIENT TRUE STORIES: AWARENESS CAMPAIGN ON THE NEED FOR PLASMA

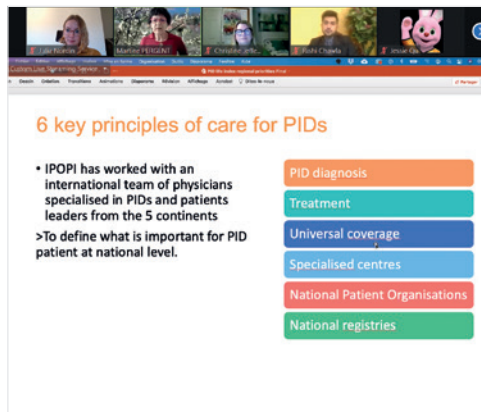
In October 2021, IPOPI launched the PID patients' true stories' campaign, dedicated to addressing the worldwide need for a more regionally balanced plasma collection. This campaign took place during International Plasma Awareness Week (October 4–10, 2021), organised by the PPTA (Plasma Protein Therapeutics Association). Five short videos depicting the lives of five PID patients from various continents demonstrated the patients' need for plasma-derived medicinal products and how important this therapy is to them.



STRATEGIC OBJECTIVE 2 - BUILD CAPACITY AND SUPPORT IPOPI'S NATIONAL MEMBER ORGANISATIONS

IPOPI assists the establishment of new national member organisations and promotes the development of existing National Member Organisations.

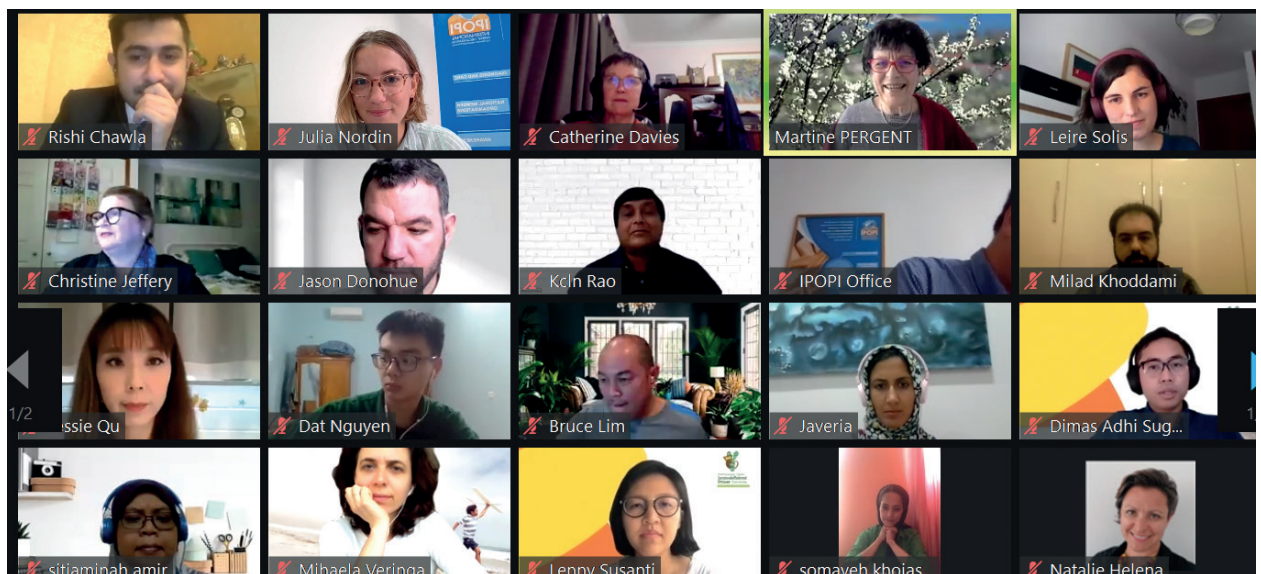
IPOPI REGIONAL PID EVENTS



ASIAN ADVOCACY WORKSHOP

On April 17-18, 2021, IPOPI's National Member Organisations (NMOs), from Asia and neighbouring regions, took part in IPOPI's Regional Advocacy Workshop, aimed at increasing the participants' knowledge about strategic planning on advocacy and communication. The workshop welcomed participation from 9 countries (Australia, Bangladesh, China, India, Indonesia, Iran, Malaysia, Thailand, Vietnam) and allowed for an interesting exchange of experiences from countries with both similar and different national challenges and opportunities.

Using the metaphor of marathon training, the patient representatives were encouraged to think of advocacy campaigning as a long-term effort with both short and long-term goals guiding the work. Long-term and short-term planning allows for more successful advocacy efforts, with milestones reached along the way to keep people motivated. Not only did the participants identify national and regional priorities, but they also contributed great action points to counter the challenges they face, as well as communication strategies to successfully disseminate their work to their target audiences.

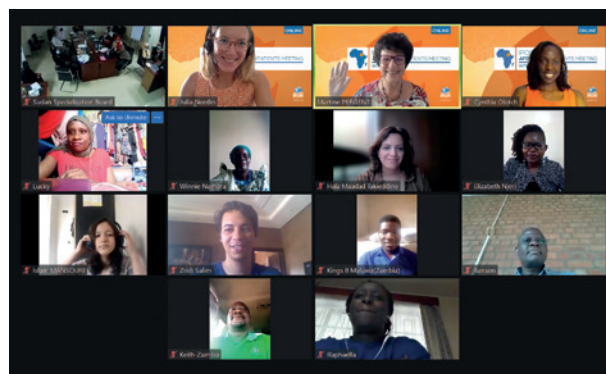
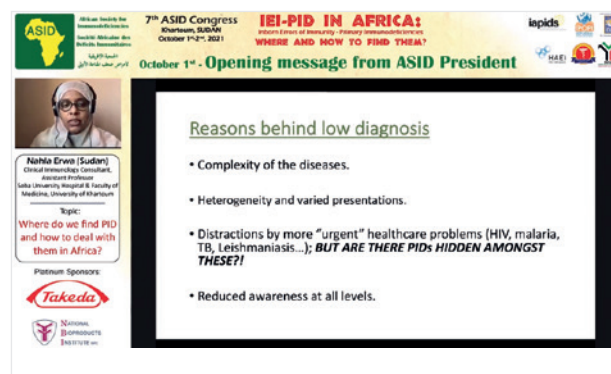
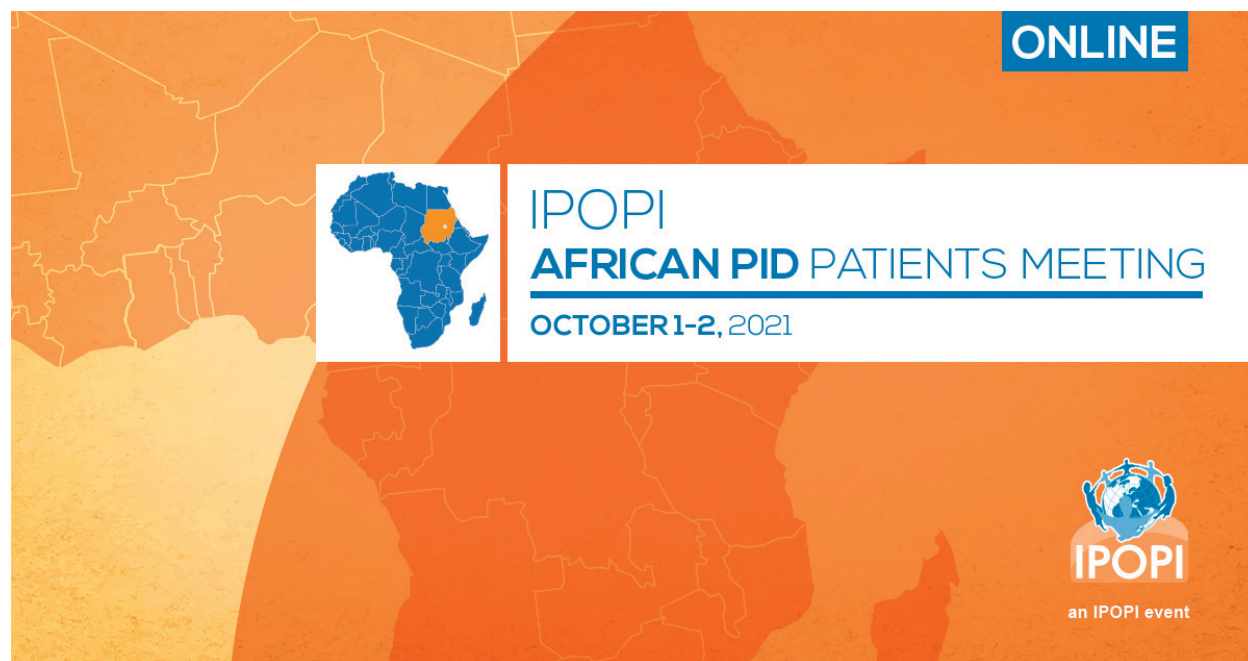


IPOPI AFRICAN PID PATIENTS MEETING – (HYBRID)

Our African PID Patient's Meeting, held October 1-2, 2021, alongside the 7th Congress of the African Society for Immunodeficiencies (ASID), was one of the highlights in October. The meeting welcomed 57 participants from 11 countries and was held in a hybrid format for the first time, with Sudanese patient representatives attending in person in Khartoum and the other participants joining the session online.

This meeting provided an excellent opportunity for patient representatives from across the region to share their experiences to strengthen their national campaigns for better PID care in their respective countries and increase regional cooperation. A joint opening ceremony with ASID was followed by a two-day IPOPI meeting that included educational lectures and patient workshops.

The objectives of the meeting were met, resulting in increased awareness of PID diagnosis and treatment and a stronger African PID community.

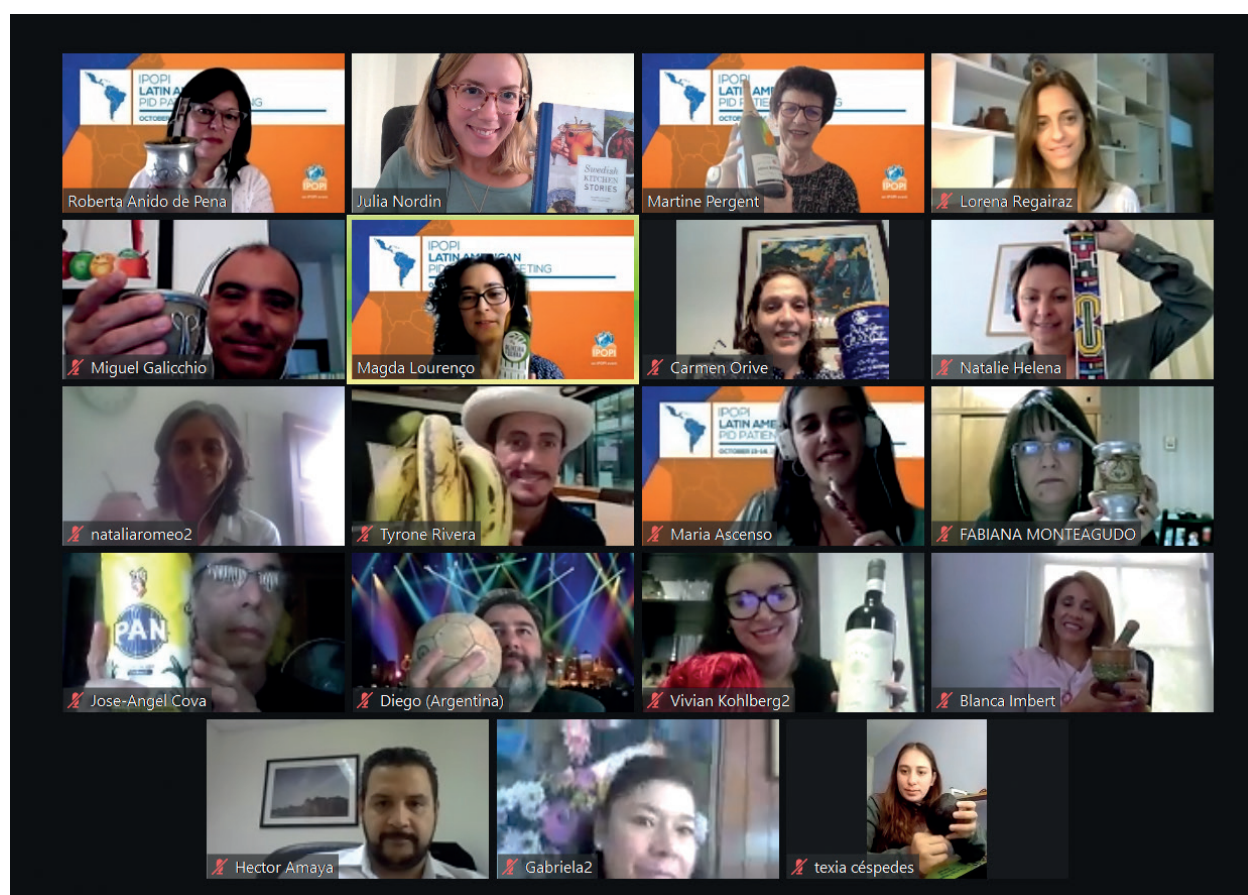


IPOPI LATIN AMERICAN PID PATIENTS MEETING – (ONLINE)

Halfway through October, we had the pleasure of welcoming our Latin American members to the biennial regional meeting, organised in conjunction with the Latin American Society for Immunodeficiencies (LASID) congress. This well-attended virtual meeting, held on October 13-14, gathered 35 patient representatives from ten countries in the region (Argentina, Bolivia, Chile, Ecuador, El Salvador, Mexico, Nicaragua, Paraguay, Puerto Rico, Venezuela).

The programme focused on topics such as the COVID-19 status in Latin America, keys to diagnosis, opening the door to treatments, and short-term thinking and acting. The participants also discussed how to lead an NMO during the COVID-19 pandemic, demonstrating the vital work they are doing in their respective countries.

It was a well-attended two-day meeting with attendees from all over Latin America. Attendees had the opportunity to learn from PID experts on a wide range of important topics, strengthen peer relationships and exchange experience and skills for the future, in order to continue the work in their national patient organisations.



IPOPI NATIONAL PID EVENTS

IPOPI INDONESIAN PID CONFERENCE – (ONLINE)

On June 19-20 and 26, 2021, IPOPI hosted a virtual Indonesian PID Conference. The meeting was divided into two parts, with each hosting a Clinical Care Meeting and a PID Patients' Meeting, both of which were well attended and well received by the audiences. The meeting attendance exceeded expectations, demonstrating the ongoing demand for educational sessions on primary immunodeficiencies in Indonesia. This was IPOPI's third Indonesian meeting, four years after the previous two, in 2017 and 2015. Since the last meeting, the PID community has grown, and IPOPI was delighted to reconnect with patients and doctors in this country once more.



ONLINE

IPOPI INDONESIAN CLINICAL CARE MEETING

JUNE 19-20

REGISTER TO LEARN MORE ABOUT DIAGNOSIS AND MANAGEMENT OF PID
FROM INDONESIAN AND INTERNACIONAL EXPERTS

PROF STUART TANGYE	PROF SURGOT SINGH	DR PAMELA LEE	PROF MARTIN VAN HAGEN	DR NIZAR MAHLAOU	DR MARTINE PERGENT
DR ADILALE	DR NARISSARA SURATANNON	DR DINA BUKTIARTI			

an IPOPI event



ONLINE

IPOPI INDONESIAN PID PATIENTS MEETING

JUNE 26

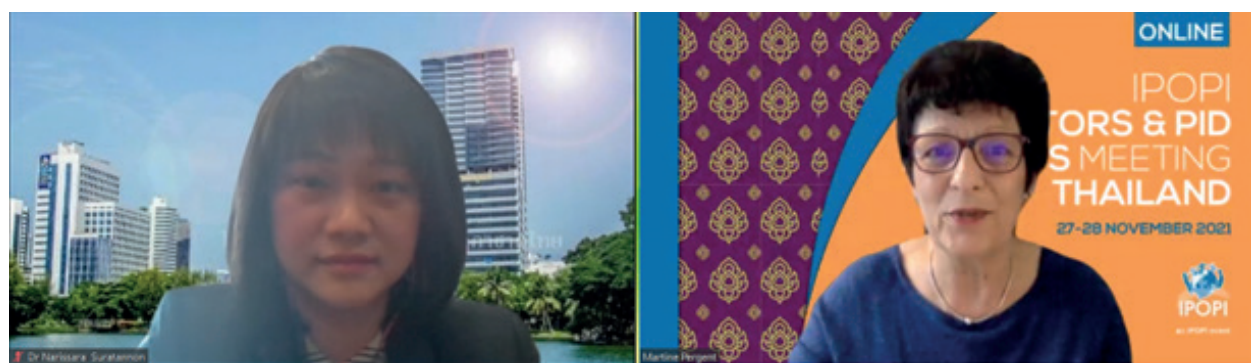
JOIN IN TO DISCUSS HOW TO BEST TACKLE PID PATIENTS
CHALLENGES IN INDONESIA

MARTINE PERGENT, IPOPI PRESIDENT FRANCE	BRUCE LIM, IPOPI TREASURER, MALAYSIA	DR NIZAR MAHLAOU, IPOPI MEDICAL ADVISORY PANEL PRESIDENT, FRANCE	DIMAS SUGIHARTO, IPIPS PRESIDENT, INDONESIA
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an IPOPI event

IPOPI DOCTORS AND PID PATIENTS MEETING THAILAND – (ONLINE)

IPOPI returned to Thailand in November 2021 to host a doctor and patient meeting for participants interested in learning more about primary immunodeficiencies. This virtual meeting took place on November 27-28 and was divided into two parts: the first day focused on medical education for physicians, and the second day offered sessions for patient representatives, patients, and their relatives. Since the first IPOPI National PID meeting in Bangkok in 2013, IPOPI has directed its efforts toward strengthening the PID community in Thailand on numerous occasions and it was a pleasure to reconnect with patients and doctors in the country again.



ONLINE

IPOPI DOCTORS & PID PATIENTS MEETING THAILAND

27-28 NOVEMBER 2021

Dr Nantassara Suratannan

Martine Pargent

IPOPI

WORLD PI WEEK 2021 – BRINGING ABOUT CHANGE FOR PID PATIENTS WORLDWIDE



IPOPI supports the yearly World Primary Immunodeficiencies Week (WPIW). Impacted by the COVID-19 pandemic, WPIW 2021 was focused on three themes: 1) Plasma availability; 2) Patient safety and protection – focus on vaccination; 3) Quality of life and health outcomes. These themes represent areas of particular concern during the pandemic, but they are recurring issues in the lives of PID patients and, thus, deserve special attention.

During WPIW 2021, IPOPI's grant programme assisted national member organisations (NMOs) in organising social media campaigns, webinars, live streams, drawing contests, educational videos, photo contests, virtual runs, letter-writing campaigns to policymakers and much more.



IPOPI NMO SUPPORT PROGRAMME 2021

Throughout 2021, IPOPI was pleased to be able to fund several NMO projects and campaigns through its NMO Support Programme among which:

- Indonesia – awareness campaign
- Greece – organisational support
- Malaysia – research paper on quality of life for patients with PID in Malaysia
- Netherlands – rewriting and launching new brochures
- Belgium – promotional materials and printing of PID leaflets

STRATEGIC OBJECTIVE 3 – EDUCATE, PROMOTE KNOWLEDGE AND DATA SHARING

IPOPI uses communication tools and strategies to spread awareness of PID, share information, for medical education and promote the collection of key PID data.

SIX NEW IPOPI PID INFORMATION LEAFLETS

Since 2011, IPOPI has been producing primary immunodeficiencies (PID) information and a new series was published in 2021, covering topics such as PIDs and Skin, PIDs and Cancer, Hyper IgE Syndrome, Ataxia-Telangiectasia, A Guide for Hepatologists and Genetic Diagnosis of PIDs.

PIDs and Skin and PIDs and Cancer cover what kind of skin issues and what cancers most affect PID patients; Hyper IgE Syndrome and Ataxia-Telangiectasia explain how these conditions are diagnosed and treated; A Guide for Hepatologists details the clinical indicator that may raise suspicion of PIDs; Genetic Diagnosis of PIDs explains how genetic analysis can be used to diagnose PIDs.

IPOPI is pleased to make these leaflets available in English and expects newly translated versions to be made available with the invaluable help of its national member organisations.



MINI DOCUMENTARY: PIDS AND AUTOINFLAMMATORY AND RHEUMATOLOGICAL MANIFESTATIONS

To continue increasing awareness about primary immunodeficiency (PID) crossovers, IPOPI produced a mini documentary dedicated to PIDs with autoinflammatory and rheumatological manifestations. The documentary is split into two parts, one dedicated to management in children and one to management in adults.



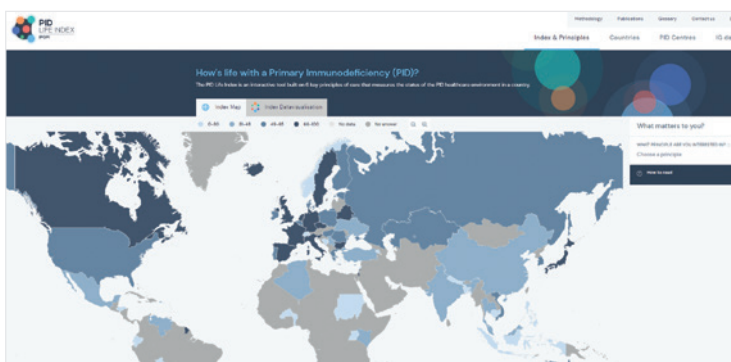
Autoinflammatory and rheumatological manifestations are frequently seen in PID patients. Its causes are increasingly better understood, and treatment is available. For some manifestations, it is possible to fully reserve the symptoms, for others, careful weighing of risk-benefit is necessary as treating autoinflammation can increase the risk for infection in PID patients.

The recording started in France in February 2020 but was postponed due to the COVID-19 pandemic and ultimately finished with online recordings in January 2021.

The documentary can be viewed on Youtube and on **TV.ipopi.org**

A SCIENTIFIC PUBLICATION PRESENTING THE PID ENVIRONMENT IN 60 COUNTRIES

IPOPI's efforts to collect key PID data for information sharing continues and the publication of "The PID Principles of Care: Where Are We Now?" was one step in this effort. The goal of this scientific article, published in November 2021, was to present data from the PID Life Index covering the PID environment in 60 countries. It was designed around the six PID Principles of Care which were published in 2014 and have since been revised in preparation for the development of the PID Life Index in 2020, to take into account recent advances in the field.



The PID Principles of Care: Where Are We Now? A Global Status Report Based on the PID Life Index

Julia Nordin^{1†}, Leire Solís^{1†}, Johan Prévot¹, Nizar Mahlaoui^{2,3}, Helen Chapel⁴,
Silvia Sánchez-Ramón^{5,6}, Adli Ali^{7,8}, John W. Seymour^{1,9} and Martine Pergent^{1*}

¹ The International Patient Organisation for Primary Immunodeficiencies, Downderry, United Kingdom

² Pediatric Immunology-Hematology and Rheumatology Unit, Necker Children's University Hospital, Assistance Publique-Hôpitaux de Paris (AP-HP), Paris, France

³ French National Reference Center for Primary Immune Deficiencies (CEREDIH), Necker Children's University Hospital, Assistance Publique-Hôpitaux de Paris (AP-HP), Paris, France

⁴ Nuffield Department of Medicine, University of Oxford, Oxford, United Kingdom

⁵ Department of Clinical Immunology, Instituto de Medicina del Laboratorio (IML) and Instituto de Investigación Clínico San Carlos (IdiSSC), Hospital Clínico San Carlos, Madrid, Spain

⁶ Department of Immunology, ENT and Ophthalmology, Complutense University School of Medicine, Madrid, Spain

⁷ Clinical Immunology Unit, Department of Paediatrics, Faculty of Medicine, Universiti Kebangsaan Malaysia Medical Centre, Kuala Lumpur, Malaysia

⁸ Institute of IR4.0, Universiti Kebangsaan Malaysia, Bangi, Malaysia

⁹ Department of Counseling and Student Personnel, Minnesota State University, Mankato, MN, United States

A global gold standard framework for primary immunodeficiency (PID) care, structured around six principles, was published in 2014. To measure the implementation status of these principles IPOPI developed the PID Life

The revision resulted in the following six principles: PID diagnosis, treatments, universal health coverage, specialised centres, national patient organisations and registries for PIDs. A questionnaire corresponding to the principles was distributed to IPOPI's national member organisations as well as countries where IPOPI had medical contacts and data from 60 countries were collected.

The data show that, despite global scientific progress on PIDs, with a growing number of diagnostic tools and better treatment options becoming available, access and affordability remain uneven around the world. Although some countries perform better than others, no country has fully implemented the PID Principles of Care to date, indicating that there is still a long way to go to ensure an optimal environment for patients with PID in every country.



4ID HEALTH AGENDA – THE NEW PID APPLICATION

The 4ID Health Agenda is an application for patients with immunodeficiencies, launched by IPOPI in 2021. 4ID offers patients a simple way to keep track of their conditions. Log symptoms, update prescriptions, follow treatments and view daily health summaries; these key features are all easily accessible in 4ID. Many people with immunodeficiency need lifelong treatments and support from a variety of specialists and IPOPI has developed 4ID to support them in following their conditions and allow them to access their health record at any time.

4ID is available on [4ID.ipopi.org](https://4id.ipopi.org)

IPOPI E-NEWS

Our most important publication, IPOPI e-News, is sent directly to thousands of readers and is also shared on social media. Every March, June, September, and December, e-News is published, highlighting the most significant achievements of IPOPI and its members. The editorial in our newsletter, signed by IPOPI's President, is always one of the most read articles, as are the interviews with prominent PID community members.

e-news.ipopi.org



STRATEGIC OBJECTIVE 4 - STRENGTHEN MULTI-STAKEHOLDER COOPERATION

IPOPI values the benefits of working together with partners and stakeholders and 2021 was a shining example of new partnerships being built.

WHO AND ISBT DIALOGUE ON IMPROVED ACCESS TO PLASMA DERIVED MEDICINAL PRODUCTS

IPOPI actively took part in a Workshop on “Stepwise Access to Safe Plasma Proteins in Resource-Constrained Countries: Local Production & Pathways to Fractionation” organised by the International Society for Blood Transfusion (ISBT) in September 2021. The workshop was aimed at identifying pragmatic technical options for stepwise access to safe plasma protein therapies in resource-constrained countries to support the implementation of the WHO guidance on “Increasing Supplies of Plasma-derived Medicinal Products in Low- and Middle-income Countries through Fractionation of Domestic Plasma” and “Centralisation of Blood Donation Testing and Processing.” As an outcome of the workshop, IPOPI, WHO, ISBT, FIODS and WFH engaged in an effort to launch an international coalition that would aim to facilitate in-country projects on stepwise enhancement of the quality, safety and volume of domestic plasma; local small-scale preparation of virus-safe clotting factors and immunoglobulins; and progress toward industrial plasma fractionation.

LAUNCH OF INTERNATIONAL NEONATAL SCREENING DAY



In 2021, IPOPI, in partnership with the International Society for Neonatal Screening (ISNS) and the European Society for Immunodeficiencies (ESID), announced the launch of the first International Neonatal Screening Day (INSD) on June 28, 2021. For years, IPOPI, ISNS, and ESID have worked together under the multi-stakeholder Screen4Rare initiative to promote the importance of neonatal (newborn) screening. The INSD initiative arose from discussions between the three organisations in response to a proposal by IPOPI's Executive Director Johan Prevot,

which was later formally endorsed by all three partner organisations. The date of June 28 was proposed by ISNS as it coincides with the birthday of Dr Robert Guthrie and his landmark contribution to Neonatal Screening, not just a test but also a means of sample collection to identify children with rare disease shortly after birth. The first celebration took place on June 28, 2021, and had participation from the scientific community, patient organisations, health agencies and industry.



European Alliance for Transformative Therapies

IPOPI JOINS TRANSFORM

IPOPI is happy to announce that it is now part of TRANSFORM, the European Alliance for Transformative Therapies, a multi-stakeholder Alliance that connects Members of the European Parliament (MEPs) and policy-makers with patient groups, medical experts and associations, scientists, researchers, industry actors, networks and other relevant stakeholders.

Together with TRANSFORM, IPOPI intends to expand its collaboration in the development and dissemination of evidence-based

recommendations to enable patient access to cell and gene therapies, to educate MEPs on the specificities of these transformative therapies in order to shape policy and to prepare healthcare systems for the adoption of these transformative therapies.

RECOMB RESEARCH PROGRAMME

As the leader of the work package on dissemination and exploitation, IPOPI has been an active contributor to the Recomb research programme to develop gene therapy for RAG1 severe combined immunodeficiency (SCID).

Recomb is an EU Horizon 2020-funded programme launched in 2018, aiming at developing gene therapy for children affected by a specific type of SCID: Recombinase Activating Genes (RAG1) deficient SCID. The project is coordinated by Prof Frank Staal, from Leiden University Medical Center.

In January 2021, IPOPI organised a successful webinar to highlight the Recomb project's progress. In addition, Julia Nordin (NMO Programmes Manager) and Johan Prevot (Executive Director) attended a scientific review meeting to evaluate the programme. The reviewers praised Recomb and the progress made by the various working groups.

www.recomb.eu

RECOMB has received funding from the European Union's Horizon 2020 research and innovation programme under grant agreement no. 755170 (RECOMB).


www.recomb.eu

GENE THERAPY for RAG1-SCID

Jan 29 2021, 15h CET


Prof Arjan Lankester


Prof Karin Pike-Overzet


Prof Frank Staal


Moderated by
Mr Johan Prevot



This project has received funding from the European Union's Horizon 2020 research and innovation programme under grant agreement no. 755170 (RECOMB)



Primary Immunodeficiencies / Autoinflammatory Disorders
Autoimmune Diseases / Paediatric Rheumatic Diseases

EUROPEAN REFERENCE NETWORK FOR PIDS

IPOPI's collaboration with the European Reference Network RITA has continued, ensuring that the voices of PID patients are heard in the network's activities and decisions.

RITA stands for Rare Immunodeficiency, Autoinflammatory, and Autoimmune. It brings together specialised centres in Europe that focus on primary immunodeficiencies as well as other autoinflammatory and autoimmune disorders.



EU HEALTH COALITION

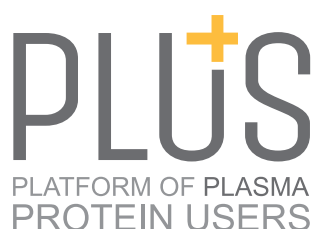
IPOPI continued its collaboration with the multistakeholder EU Health Coalition in a joint effort to look at mapping the future of healthcare in Europe in order to make the most of the innovation at our fingertips, at the role of the European Union in addressing the challenges we face and critically at how different sectors can converge to deliver the best outcomes for patients in Europe. Among its activities in 2021, the Coalition called for the establishment of a permanent multi-stakeholder Forum for Better Access to Health Innovation.



RARE
DISEASES
INTERNATIONAL

RARE DISEASE INTERNATIONAL (RDI)

IPOPI continued participating in the activities organised by Rare Disease International, the global alliance of persons living with a rare disease. As such, IPOPI was involved in meetings aimed at developing the Collaborative Global Network for Rare Diseases or discussing the UN Resolution on Persons Living with a Rare Disease with the objective of making rare diseases a topic of relevance for the World Health Organization and the United Nations.



PLUS – PLATFORM OF PLASMA PROTEIN USERS

The Platform of Plasma Protein Users (PLUS) is a consortium of seven patient organisations that represent people with treatable rare plasma-related disorders like haemophilia, primary immunodeficiencies, and alpha1 antitrypsin deficiency, among others. IPOPI is actively taking part in this consortium which collectively represent the views of over 110,000 Europeans living with treatable rare plasma-related disorders. PLUS represents organisations of patients with treatable rare diseases who are linked by common therapies based on human plasma products.



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMA

The European Medicines Agency consider patients, as the end-users of medicines, as stakeholders. IPOPI's Board Member Jose Drabwell (UK) is a member of the Patient's and Consumers' Working Party and continues to develop an important role voicing patient's needs and perspectives in pharmacovigilance, involvement in clinical trials, data transparency and more. Ms Leire Solis, Senior Health Policy and Advocacy Manager supports Mrs Drabwell in this role.

RELATED MEETINGS

IPOPI attended and participated in the following events among others:

- EU Health Coalition steering committee meetings throughout the year
- ERN-RITA Board Meetings, Patients Committee, Communications WP, throughout 2021
- ERN RITA transition working group meeting, January, 2021
- IPFA-PEI Virtual Workshop on Surveillance and Screening of Blood-borne Pathogens, May 2021
- RECOMB - Webinar: Gene Therapy for RAG1-SCID, January 2021
- HFE Thematic Network Webinars, throughout 2021
- World PI Week steering committee meetings, throughout 2021
- ERN-RITA Webinar: Primary HLH from birth to adulthood – a disease with many faces, February 2021
- Rare 2030 Final Policy Conference, February 2021
- PPTA webinar on “The Global Journey of Plasma”, February 2021
- IPOPI’s Russian NMO - 1st International Congress “Immunity 2021: Doctor-Patient-Society”, February 2021
- RDI Rare Disease Day Policy Event, March 2021
- ERN-RITA Webinar: Introduction to the Human Phenotype Ontology project, March 2021
- EMA stakeholder meeting on COVID-19 vaccines, March 2021
- ERN-RITA Webinar: COVID-19 vaccination for PIDs & rare Autoimmune rheumatic diseases, April 2021
- PLUS AGM, April 2021
- ERN-RITA Webinar: COVID-19 vaccination for PIDs & rare Autoimmune rheumatic diseases, April 2021
- PPTA webinar on the Revision of the EU Blood Directive: “How to enhance plasma collection by getting more donors via increased regulatory efficiency?”, April 2021
- IPOPI’s Spanish NMO – 1st Meeting on PID patients during the COVID-19 pandemic, Madrid (Spain), April 2021
- Granulomatous Lymphocytic Interstitial Lung Disease (e-GLILD) Steering Committee Meetings, throughout the year
- World PI Week social media campaign, April 2021
- RDI – WHO Collaborative Global Network for Rare Diseases (WHO CGN4RD), April 2021
- XV European Patients’ Rights Day, May 2021
- European Commission Stakeholder Workshop with Blood Competent Authorities, workshop “Regulating for sufficiency – blood and plasma”, May 2021
- IPFA/PEI 27th International Workshop on Surveillance and Screening of Blood-borne Pathogens (online), May 2021
- RDI Webinar RD global network, May 2021
- EuropaBio Taking health up a gear: value of advanced therapies, May 2021
- WHO Webinar on Introduction to WHO publication on blood related topics, May 2021
- EURORDIS Membership Meeting 2021, May 2021
- Patient BioForum: Advancing patient access to innovative treatments, May 2021
- RECOMB research consortium mid-term review meeting by the European Commission, May 2021
- ERN-RITA Webinar: Introduction of the HyperPED COVID Registry, May 2021
- ERN-RITA Webinar: Clinical guidance for vaccination of children with rheumatic diseases, May 2021
- Autoimmune Association 12th International congress on autoimmunity online, May 2021
- EBA webinar: Blood is Priceless: the future of EU Blood Directives, perspectives from the European Establishments, May 2021
- Blood and plasma campaign for World Blood Donor Day, 14 June 2021
- IPOPI NMO Webchat on IG shortages, June 2021
- The African Medicines Agency Vision and Strategy for the African Continent, June 2021
- RDI High Level Side-Event at the UN Political Forum on Sustainable Development , July 2021

- EUPATI – Bluebird Bio Education Webinar On Cross-border Healthcare in EU, July 2021
- Immune Deficiencies Foundation Australia’s Webinar on Nutrition and Healthy Lifestyle, July 2021
- Meeting on COVID19MaBs for patients with PID, September 2021
- Immune Deficiencies Foundation Australia’s Webinar on COVID-19 Vaccination & Immunodeficiency, September 2021
- Perkin Elmer Webinar, “What actions should be taken to improve maternal and newborn health outcomes in Europe?”, September 2021
- EHMA 2021 Annual Conference, September 2021
- European Society for Immunodeficiencies conference (online), September 2021
- ERN RITA Transition working group online meeting, September 2021
- TRANSFORM Alliance Meeting, September 2021
- EU PAD Consortium meeting, September 2021
- NMO Webchat on COVID-19, September 2021
- Immune Deficiencies Foundation Australia’s Webinar on Severe combined immunodeficiency, September 2021
- ESID 2021 - The Clinical Working Party of the European Society of Immunodeficiencies. Multidisciplinary Symposium on the Immunological Consequences of Targeted Immune Therapies, September 2021
- EuropaBio webinar - Treatment without borders: the EU case for equitable patient access to advanced therapies, September 2021
- ISBT online workshop on Stepwise access to safe plasma proteins in resource-constrained countries: local production and pathways to fractionation, September 2021
- Slovenian Presidency of the Council of the European Union 2021 event on “Achieving Equity and Innovation in Newborn Screening and in Familial Hypercholesterolemia Paediatric Screening across Europe”, October 2021
- HFE Annual General Meeting online, October 2021
- 42nd Annual SPAIC Meeting online, October 2021
- PKU EP Online event, October 2021
- Rare summit, October 2021
- Patient BioForum, October 2021
- International Plasma Awareness Week, PID Patient true stories: awareness campaign on the need of plasma, October 2021
- EMA - EFT weekly meetings, from October to December
- Politico Health Summit (online), October 2021
- HFE ExCom meeting, October 2021
- PPTA Global Plasma Summit, November 2021
- Recomb annual meeting, 2021, November 2021
- ISNS and MetabERN workshop on Newborn Screening, November 2021
- TRANSFORM MEP Interest Group discussion on ‘How to Optimise Cross-border Cooperation to Support Equitable Access to Advanced Therapies’, November 2021
- Orchard workshop: Introducing a NOVEL ALGORITHM for newborn screening horizon scanning, November 2021
- MEET-UNMET research consortium meeting, November 2021
- EU Big Data stakeholders Forum - HMA/EMA, December 2021
- TRANSFORM Alliance call, December 2021
- Recomb online meeting, December 2021
- EMA PCWP/HCPWP meetings throughout the year
- EMA COVID-19 Emergency Task Force meetings, throughout the year

- RDI advocacy committee webinars, throughout the year
- RIPAG meetings, throughout the year
- European Alliance for Transformative Therapies (TRANSFORM) meetings, throughout the year
- World PI Week steering committee meetings, throughout the year
- Access to care / patient empowerment meetings and/or calls with doctors and patients in France, Sweden, Germany, Estonia, Italy, Romania, Cyprus, Greece, the Netherlands, UK, Slovenia, Turkey, Thailand, China, Indonesia, India, Malaysia, Australia, USA, Ecuador, Venezuela, Chile, Argentina, Mexico, El Salvador, Puerto Rico, Nicaragua, Bolivia, Kenya, Morocco, Uganda, Egypt, Zambia, Senegal, South Africa, Sudan, Lebanon, Israel, among others

LOOKING AHEAD – 2022 AND BEYOND

IPOPI plans to continue assisting the PIDs' community through international awareness, advocacy, and medical education.

New initiatives are in the works, including a new educational video series on PIDs, a mini documentary on primary and secondary immunodeficiencies, an updated 4ID health agenda app, European Parliament events, EU PID Forums and much more. National advocacy campaigns around the world will ensure that the PID patient community is included in key policy decisions.

The launch of new projects and NMO programmes, as well as national and regional meetings and workshops, will help to strengthen PID patient organisations worldwide.

Don't miss IPIC2023, to be held in Rotterdam, the Netherlands, on November 8-10, 2023.



REVIEW
OF ACTIVITIES
AND ACHIEVEMENTS

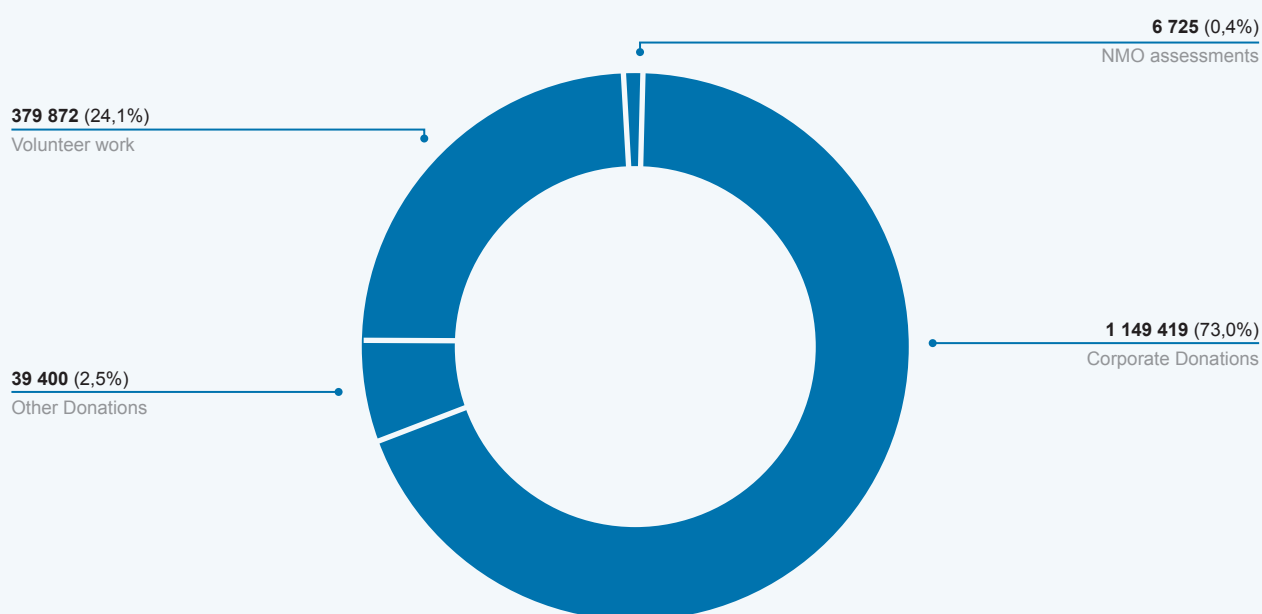
FINANCIAL REPORT

2021

FINANCIAL REPORT 2021

Despite the postponement of the 5th edition of its International Primary Immunodeficiency congress, 2021 was a good financial year for IPOPI. IPOPI was able to successfully adapt many of its events and programmes to the unique circumstances brought about by the COVID19 pandemic and continue to carry out its policy and advocacy efforts, patient awareness campaigns, skills-building and educational programmes, all geared towards improving PID care and access to treatments across the globe.

INCOME 2021 (EURO)



INCOME 2021 (EURO)

NMO assessments	6 725
Corporate donations	1 149 419
Other donations	39 400
Volunteer work	379 872
TOTAL	1 575 416

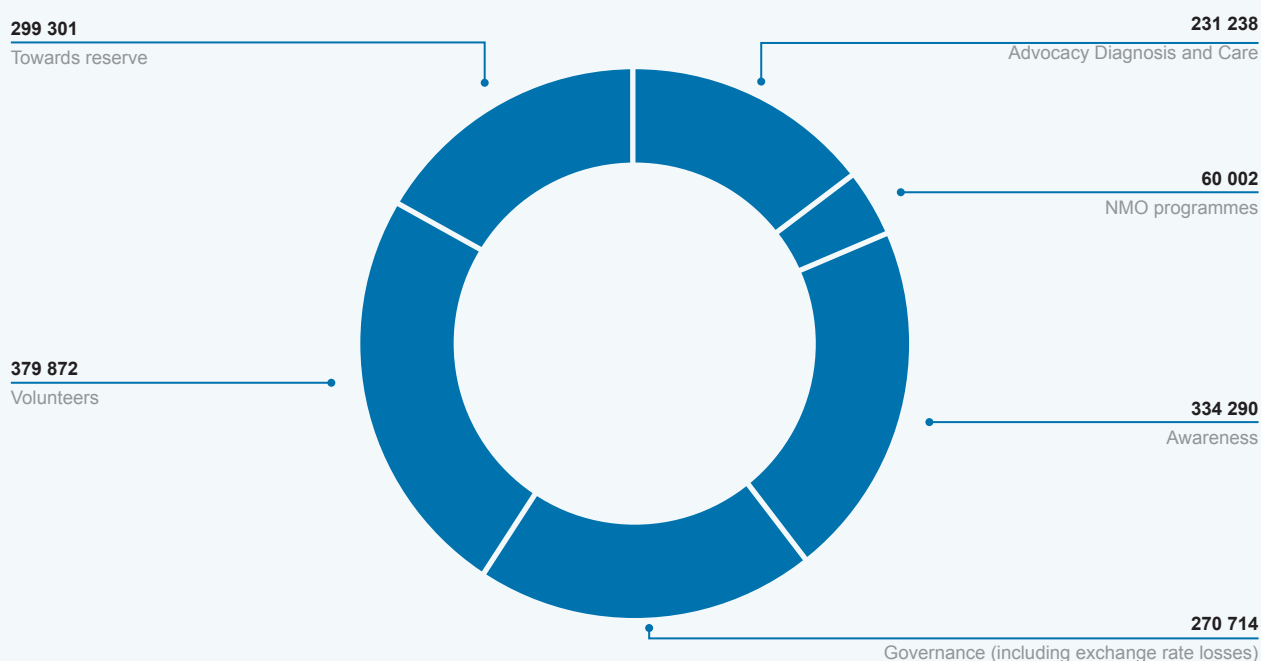
INCOME 2021 (%)

NMO assessments	0,4%
Corporate donations	73,0%
Other donations	2,5%
Volunteer work	24,1%
TOTAL	100,00%

In 2021, and as a direct consequence of Brexit which directly impacted IPOPI's ability to carry out EU-level actions and benefit from EU funding, IPOPI registered a new legal entity in Belgium as an international non-profit association (aisbl). IPOPI maintained its non-profit charity registered in the United Kingdom aiming to gradually transition its operation to the new legal entity. Every year, IPOPI is required to have its accounts audited both in Belgium and in the UK by qualified accountants/auditors and submitted for approval. The following charts provide a summary of key aspects of our financial accounts in 2021. Our audited accounts are available on the Charity Commission website and Belgian National Bank register, and on request.

IPOPI has included an economic valorisation of the work of its volunteers in the following charts. This value has been independently reviewed by our UK chartered accountants.

RESOURCES EXPENDED 2021 (EURO)



EXPENDITURE 2021* (EURO)

Advocacy Diagnosis and Care	231 238
NMO programmes	60 002
Awareness	334 290
Governance	270 714
Volunteers	379 872
Towards reserve and postponed 2021 programmes due to COVID-19	299 301
TOTAL EXPENDITURE	1 575 416

SOURCES OF FUNDING 2021 (%)

Membership fees	0,4%
Corporate donations	73,0%
Other	2,5%
Volunteers	24,1%
TOTAL	100,00%

* GBP-EURO conversion rate = 0,8765

No contribution received from a single company reached the majority of the total income.

The highest single contribution by a company represented 14,95% of the total income including volunteer time contributions and 19,7 % excluding volunteer time contributions.

IPOPI would like to recognise the large number of people and stakeholder organisations who support our work through personal effort, shared objectives, alliances or financial support. These collaborations help IPOPI achieve more year after year for people living with primary immunodeficiency worldwide.

We also express our gratitude to our 69 National Member Organisations whose important activities and efforts in their respective countries are vital to improving the conditions for people living with a PID. Our NMOs are key stakeholders who contribute towards the global mission of IPOPI.

CORPORATE PARTNERS

IPOPI would like to extend its sincere thanks to the following corporate partners whose contributions helped us implement our mission through raising awareness, advocacy/ policy efforts, representation in key events, communications activities, organisation of international and regional meetings as well as support to national patient programmes and campaigns.

DIAMOND



GOLD



SILVER



SUPPORTERS



PROJECT SPONSORS

In 2021, the following corporate sponsors provided unrestricted grants towards various IPOPI ad-hoc projects that enhance our awareness, advocacy, communications and representational activities.

- Bluebird Bio
- Chiesi
- CSL Behring
- Enzyvant
- Grifols
- Horizon Pharma
- ImmunoIVD
- Kedrion
- NBI
- Novartis gene therapies
- Octapharma
- Orchard
- Perkin Elmer
- Pfizer
- Pharming
- SOBI
- Takeda
- X4 Pharmaceuticals

IPOPI's corporate relations with the above-mentioned sponsors are subject to IPOPI's corporate relations guiding principles available on IPOPI's website.

KEY STAKEHOLDERS

IPOPI has strategically been growing its stakeholder cooperation and we would like to express our most sincere thanks to a number of stakeholder organisations for their partnership and collaboration in 2021 including but not limited to:

Alpha-1 Global, African Society for Immunodeficiencies (ASID), Arab Society for Primary Immunodeficiencies (ARAPid), Asociación Española de Pediatría (AEP), Asia Pacific Economic Cooperation (APEC) Blood Policy Stakeholders Forum, Asia Pacific Society for Immunodeficiency (APSID), Australasian Society of Clinical Immunology and Allergy (ASCIA), Belgian Primary Immune Deficiency Group (BPIDG), British Society for Immunology (BSI), Care-for-Rare Foundation, Chulalongkorn University Hospital Bangkok, Clinical Immunology Society (CIS), Cipto Mangunkusumo Hospital Jakarta, Council of Europe, European Alliance for Transformative Therapies (TRANSFORM), EU Health Coalition, European Commission, European Federation of Immunology Societies (EFIS), European Foundation for the Care of Newborn Infants (EFCNI), European Health Coalition, European Medicines Agency (EMA), the European Haemophilia Consortium (EHC), European Network For Paediatric Research (Enpr-EMA), European Parliament, European Patients Forum (EPF), European Platform for Patient Organisations, Science and Industry (EPPOSI), Eurordis, European Reference Network RITA-ERN, European Society for Blood and Marrow Transplantation (EBMT), European Society for Immunodeficiencies (ESID), Faculty of Medicine Universitas Indonesia (FMUI), Federation of African Immunology Societies (FAIS), Federation of Clinical Immunology Societies (FOCIS), Federation of Immunological Societies of Asia-Oceania (FIMSA), Food and Drug Administration (FDA-US), Grupo Português de Imunodeficiências Primárias (GPIP), Hanoi National Hospital of Paediatrics, Health First Europe (HFE), Hungarian Paediatrics Society, Hungarian Society for Allergology and Immunology, Indian Academy of Pediatrics, Indian Society for Primary Immunodeficiency, International Alliance for Patient Organizations (IAPO), International Nursing Group for Immunodeficiencies (INGID), International Immunocompromised Host Society (ICHS), International Plasma Fractionation Association (IPFA), International Society for Blood Transfusion (ISBT), International Society for Neonatal Screening (ISNS), International Union of Immunological Societies (IUIS), Latin American Society for Immunodeficiencies (LASID), Le Centre de Référence Déficits Immunitaires Héréditaires (CEREDIH), MSAI (Malaysian Society for Allergy and Immunology), Plasma Protein Therapeutics Association (PPTA), Platform of Plasma Protein Users (PLUS) and its member organisations, RECOMB H2020 consortium, SCIDNET H2020 consortium, Sociedad Española de Inmología (SEI), SEAPID (South East Asia Primary Immunodeficiencies Network), Sociedade Portuguesa Alergologia Imunologia Clínica, Sociedade Portuguesa de Pediatria-infecciologia pediática, Thai Immunology Society (AAIAT), Vietnamese Paediatrics Association, World Federation of Haemophilia, World Health Organisation (WHO), World PI Week and its member organisations.

IPOPI is an international charity registered in the UK (No. 1058005) and IPOPI aisbl is an international non-profit association registered in Belgium (BE 0761.784.055)

IPOPI.ORG



INTERNATIONAL PATIENT ORGANISATION FOR PRIMARY IMMUNODEFICIENCIES

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email: info@ipopi.org - website: **IPOPI.ORG**