IPOPI
REVIEW OF ACTIVITIES AND ACHIEVEMENTS
2023

IPOPI
INTERNATIONAL
PATIENT ORGANISATION
FOR PRIMARY IMMUNODEFICIENCIES

XVIII
IPOPI GLOBAL PATIENTS' MEETING

an IPOPI event

16-19 OCTOBER 2024
MARSEILLE, FRANCE

#GPM2024

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A MESSAGE FROM IPOPI'S CHAIRPERSON AND EXECUTIVE DIRECTOR

Martine Pergent
IPOPI President

Johan Prevot
IPOPI Executive Director

In 2023, IPOPI's collective efforts were focused on various crucial areas, encompassing education, advocacy, support for patient organisations, and collaborative engagements among stakeholders. Together, we have taken significant strides towards improving the lives of individuals living with PIDs and their families.

Of course, the International Primary Immunodeficiencies Congress (IPIC), held in Rotterdam, emerged as a milestone event of unparalleled success. It broke records in terms of attendance, content, and the fostering of a smooth cooperative atmosphere. IPIC served as a catalyst for knowledge exchange, laying the groundwork for cutting-edge advancements in the clinical field. However, despite our strides, concerns persisted throughout the year regarding namely plasma collection and the supply of immunoglobulins. The steady growth in global needs, particularly due to improved diagnoses of PIDs and the increasing population affected by Secondary Antibody Deficiencies, calls for growing attention. IPOPI tackled these issues highlighting the need for patient-centred policies in various geographies notably in Europe by addressing legislative revisions at the European level (SoHO legislation), engage with UNITAR in Argentina and Malaysia, pushing for increasing the quality and safety of plasma in line with international regulatory standards and with a view to allow African countries to access plasma contract fractionation within its discussions with the ICSPP, and tailoring specific advocacy advice in diverse countries like Brazil or Indonesia to ensure equitable access to essential treatments.

A significant impulse of our work was dedicated to enabling patient access to transformative and regenerative medicines. Initiatives like AGORA aim to facilitate access to gene therapies for patients with ultra-rare diseases in Europe, while the Transform Alliance aims at advocating for improving the regulatory and legislative systems that apply to transformative therapies and allow patients to access a timely treatment. In parallel, we strongly advocated for newborn screening, championing the case of SCID as a condition with existing curative treatment, emphasising the need for a stepwise approach that prioritises the development of robust and shared practices when it comes to rare diseases.

Throughout the year, IPOPI led many prospective and future oriented discussions to envision the future of our field, enabling us to proactively prepare for the challenges and opportunities that lie ahead. Not least with our highly successful Global Stakeholder's Summit which brought together high-level experts to prepare for the years to come. The insights gathered during the event will no doubt help guide our community forward, with one common goal in mind: improving the quality of life for those living with immunodeficiencies!

We warmly invite you to delve into our annual review where you can explore the key highlights and remarkable moments that made 2023 a very special year!

Martine Pergent
IPOPI President

Johan Prevot
IPOPI Executive Director

EXECUTIVE COMMITTEE, OFFICERS, AND STAFF 2023

EXECUTIVE COMMITTEE

Martine Pergent, Chair
Bruce Lim, Vice - Chair
Whitney Ayoub Goulstone
Jose Drabwell
Cynthia Olotch
Roberta Anido de Pena
John Seymour
Otilia Stanga (Treasurer)

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Dr Nizar Mahlaoui, France

Vice Chair
Prof Martin van Hagen

PANEL MEMBERS

Dr Adli Ali - Malaysia
Prof Tadej Avcin - Slovenia
Prof Aziz Bousfiha - Morocco
Prof Antonio Condino-Neto - Brazil
Prof Charlotte Cunningham-Rundles - United States of America
Dr Virgil Dalm - The Netherlands
Prof Tandakha Dieye - Senegal
Dr Nahla Hashim Hassan Erwa - Sudan
Prof Alain Fischer - France
Dr Elie Haddad - Canada
Prof Steven Holland - United States of America
Dr Pamela Lee - Hong Kong
Prof Isabelle Meyts - Belgium
Dr Olaf Neth - Spain
Dr Cecilia Poli - Chile
Dr Elizabeth Rivers - United Kingdom
Dr Silvia Sánchez-Ramón - Spain
Prof Anna Shcherbina - Russia
Prof Surjit Singh - India
Prof Stuart Tangye - Australia
Prof Klaus Warnatz - Germany

HONORARY CHAIRPERSON

Prof Helen Chapel - UK

Honorary Vice-Chair
Dr Teresa Español – Spain

STAFF

Johan Prevot: Executive Director
Patricia Boldescu: Communications and Events Assistant
Samya Van Coillie: Medical Affairs Project Manager
Miriam Ferreira: NMO Programmes Officer
Clare Glynn: Accounting and Administration Manager
Carla Morgado: Office Manager
Mercedes Muraca: Events and Logistics Coordinator
Julia Nordin: Communications Manager
Leire Solis: Health Policy and Advocacy Senior Manager

NETWORK OF EXPERTS

The IPOPI Network of Experts (IPOPI NEX) serves as a valuable consultative network within IPOPI's framework. While not formally integrated into IPOPI's governance structure, IPOPI NEX was established to offer voluntary expert advice and information to the IPOPI Board of Directors. Distinguished by their diverse fields of expertise, IPOPI NEX consultants provide a crucial supplement to the clinical and scientific proficiency of the IPOPI Medical Advisory Panel (MAP).

IPOPI NEX MEMBERS (2023)

- Nurse Mary Louise Daly (Nursing)
- Dr Lotte Denning (General Practitioner)
- Prof Albert Farrugia (Plasma industry/access specialist)
- Prof Bobby Gaspar (Advanced therapies industry specialist)
- Prof Jacqueline Kerr (Regulator)
- Prof 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.

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*

IPOPI CORE ACTIVITIES & PROJECTS

- DIAGNOSIS AND CARE
- NATIONAL MEMBER ORGANISATIONS
- AWARENESS
- STAKEHOLDER COLLABORATION

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. It also helps in preventing inflammation, autoimmunity, severe allergies and malignancies. Because their immune systems do not work properly, people with PIDs are more prone than others to infections.

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 defects improves, more and more genetic causes of PID are being 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, who, 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.

60%

OF PIDS

can be easily diagnosed with simple and inexpensive blood tests

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. They are considered essential medicines for PIDs by the World Health Organisation and can be administered intravenously or subcutaneously. 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 administered 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.

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 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 as well as advice on how to stay healthy please refer to the IPOPI leaflets.

IPOPI'S KEY ACHIEVEMENTS AND ACTIVITIES 2023

01

STRATEGIC OBJECTIVE

Improve access to early diagnosis
and patient-centred care

IPOPI remains dedicated to fostering long-term improvements in PID diagnosis and ensuring that all PID patients have access to appropriate, patient-centred care. Through various advocacy, awareness-raising, and educational campaigns and programmes, IPOPI works diligently to make a difference. By continuing these efforts, IPOPI aims to create a better future where the diagnosis of PID is improved, and all patients receive the quality care they deserve.

EU PID FORUM – THE EU'S SOHO REGULATION PROPOSAL AND ITS IMPACT ON PID PATIENTS

On January 26, 2023, IPOPI hosted the EU PID Forum on the legislation of substances of human origin at the European Parliament in Brussels, Belgium. This Forum was co-chaired by the Members of the European Parliament (MEPs) Kateřina Konečná (the Left, Czechia) and Nicolás González Casares (S&D, Spain). The event was moderated by Martine Pergent and had the interventions of the following speakers: Johan Prevot, IPOPI executive director, Matthew Hotchko, president of the Marketing Research Bureau (MRB), Nancy Di Salvo, volunteer at the GBS/CIDP Foundation International, Milan Malý, founder and CEO of UNICAplasma. A video was also displayed in which persons from different countries of the EU shared the difficulties they faced in their daily lives as patients or relatives of patients with PIDs relying on immunoglobulin therapies. These first-hand experiences were supported by interventions from different patient representatives in the room during the Forum.

The objective of the meeting was to ensure that policy makers in the room could hear the perspectives of persons with PIDs and their representatives, so as to get a better understanding of the importance of plasma derived medicinal products such as immunoglobulins for patients dependent on these medicines in the EU and reflect the key asks of patients in the revision of the SoHO legislation.

22nd EU PID Forum

The EU's SoHO Regulation Proposal and its impact on PID patients

European Parliament (room number: SPINELLI1G1), Brussels
26th January 2023, 15:00 — 16:30 CET
Co-hosted by MEP Kateřina Konečná (The Left, Czech Republic)
and MEP Nicolás González Casares (S&D, Spain)

EU PID FORUM – NAVIGATING THE COMPLEXITIES OF THE PHARMACEUTICAL LEGISLATION

On June 7, IPOPI held the PID Forum entitled "Navigating the complexities of the pharmaceutical legislation" at the European Parliament in Brussels. The event was co-chaired by three Members of the European Parliament: MEP Billy Kelleher (Renew, Ireland); MEP Cyrus Engerer (S&D, Malta) and MEP Tomislav Sokol (EPP, Croatia).

The PID Forum was used to discuss and analyse the proposal for a pharmaceutical legislation from the perspective of patients with primary immunodeficiencies (PIDs) and to be the basis for a Call to Action. The proposal for the pharmaceutical legislation, published by the European

Commission, includes measures for the development of medicines for rare diseases, aiming to foster more innovation in the development of medicines and avoid shortages of therapies.

The event was moderated by Martine Pergent, President of IPOPI and had the interventions of Leire Solis, IPOPI Health Policy and Advocacy Senior Manager, Julia Schmitz, from the European Commission, Luisa Antunes, from the European Parliament Directorate-General for Parliamentary Research Services, Juan García-Burgos, from the European Medicines Agency and Otilia Stanga, ARPID President and IPOPI Treasurer.

The PID Forum also had the participation of patient representatives: David Jimenez (IPOPI's Spanish member), José Verstegen and Janine Smith (IPOPI's Dutch member) and Kersti Urbala (IPOPI's Estonian member).

EU PID FORUM – UNLOCKING THE POTENTIAL OF ATMPS & GENE THERAPY

On September 27, 2023, IPOPI organised a PID Forum titled "Unlocking the Potential of Advanced Therapeutic Medicinal Products (ATMPs) & Gene Therapy". The event took place in the European Parliament in Brussels (Belgium) and was hosted by MEP Kateřina Konečná (The Left, Czechia).

The Forum, moderated by Johan Prevot, aimed at exchanging opinions on the challenges and opportunities that the field is facing and how the main issues that the development, approval, and access of these therapies could be jointly tackled by experts, patients and policymakers. The experts that spoke at the Forum were: Prof Claire Booth, AGORA co-founder; Prof Fabio Candotti, ESID President; Prof Arjan Lankester, RECOMB project coordinator and principal investigator and AGORA co-founder; Brian O'Mahony, Irish Haemophilia Society Chief Executive; Martine Pergent, IPOPI President.

EU PID FORUM — IPOPI HOLDS A PID FORUM ON MENTAL HEALTH AND PIDS

IPOPI held a PID Forum on the topic of "Breaking the silence on mental health and PIDs" on December 5, 2023, at the European Parliament in Brussels. The Forum was co-hosted by the Members of the European Parliament Ms Estrella Durá Ferrandis (S&D, Spain), Tomász Zdechovský (EPP, Czechia) and Radka Maxová (EPP, Czechia).

The objective of the Forum was to really focus on the challenges faced by individuals with a PID and their carers, and how sometimes, the PID itself can bring along symptoms associated with mental health problems. The speakers of the event included Jose Drabwell, IPOPI board member, Dr Virgil Dalm, from Erasmus MC, Vida Ramsak, patient representative from the Slovenian Society for Immune Disorders, Ellen Desmet, board member of the Belgian Patient organisation for Primary Immunodeficiencies (BePOPI), Rosalind Fisher, President of the International Nursing Group for Immunodeficiencies (INGID), Janne Houben, clinical psychologist, and behavioural therapist from University Hospital Leuven.

IPIC2023 IN ROTTERDAM, NETHERLANDS

The International Primary Immunodeficiencies Congress (IPIC) 2023 took place in Rotterdam, the Netherlands, in November 2023. IPIC2023 surpassed expectations, achieving remarkable success with increased attendance and enhanced content quality whilst fostering a more collaborative atmosphere than ever before.

With nearly 900 participants from 70 countries, IPIC solidified its reputation as the global conference for those dedicated to advancing their understanding of PID diagnosis and clinical care. Moreover, the congress showcased 223 approved posters, with a significant contribution from passionate young physicians propelling PID research and clinical practices forward.

The IPIC2023 congress report is available online here.

With nearly

900

PARTICIPANTS

IPIC solidified its reputation as the global conference for those dedicated to advancing their understanding of PID diagnosis and clinical care.

PIDETECT PROGRAMME: INTENSIVE PID DIAGNOSTICS TRAINING

The IPOPI PIDetect Programme provides intensive training to selected doctors from LMIC to improve their skills in recognising and diagnosing PIDs. The programme is hosted in a PID reference centre working in partnership with IPOPI and is aimed both at clinicians and laboratory scientists with an interest in PIDs. It comprises intensive clinical and/or laboratory training tailored to the individual needs and background of each attendee, followed by a local implementation activity whereby the acquired expertise is shared more widely with doctors in the target country.

The IPOPI PIDetect programme aims to enhance PID diagnosis in low- and middle-income countries by providing intensive training to selected doctors. In 2023, one doctor from Bangladesh received training at the Post Graduate Institute of Medical Education & Research (PGIMER), Chandigarh, India, under the guidance of Prof. Surjit Singh. Building from this, the three doctors who trained in 2022 and 2023 utilised the World Primary Immunodeficiency Week (WPIW) 2024 opportunity to organise an educational meeting to share their acquired knowledge with the medical community. Additionally, in 2023, two Moldovan clinicians received training at the Romanian PID reference centre in Timisoara, led by Prof Mihaela Bataneant. Following this, a hybrid conference in Chisinau helped to disseminate the acquired knowledge on PID to a broader medical community.

IPOPI PID HARD TALKS WEBINARS

IPOPI started a new series of digital events, called “IPOPI Hard Talks”. This engaging series is aimed at all relevant stakeholders with an interest in primary immunodeficiency (PID) diagnosis and clinical management, including clinicians, nurses and patient group leaders. Featuring interactive discussions and a lively debate between expert speakers, “IPOPI Hard Talks” aims to address the more challenging topics in a stimulating fashion. The series also seeks to maximise outreach to key opinion leaders across different regions and increase expertise around key clinical care issues.

PRIMARY IMMUNODEFICIENCIES AND ALLERGY CROSSOVER

The first Hard Talk webinar took place on July 11 and explored the crossover between PID and allergy, with 114 participants from 34 countries registered for the event. Dr Leif Hanitsch from the Institute for Medical Immunology, Charité, in Berlin, Germany, started off by providing a well-structured and overarching introduction lecture. He explained the pathophysiological mechanisms underlying allergy and how they relate to PID, after which clinical management and various treatment options were addressed.

During this presentation, several treatment dilemmas and points for discussion were highlighted, which sparked an engaging discussion led by Dr Joshua Milner, director of the Allergy/Immunology and Rheumatology department at Columbia University Irving Medical Center in New York, USA. The importance of defining, whenever possible, the genetic variant underlying the disease for proper treatment selection was raised, as was the role of serum cytokines and other biomarkers, among other topics.

APDS DIAGNOSIS

The second Hard Talks webinar held on October 3, gathered 92 participants from 39 countries to explore the crucial topic of Activated PI3K-Delta Syndrome (APDS) diagnosis. Dr Jacques Rivière from Vall d'Hebron Hospital, Spain, introduced the topic, outlining diagnostic challenges based on patient characteristics and available tools. Prof Stephen Jolles from University Hospital of Wales led a debate on identifying clinical symptoms in the absence of genetic or flow cytometry testing. The discussion also covered laboratory testing tips for resource-limited settings, the challenge of accessing standardised functional tests, and the importance of regularly re-evaluating genetic information.

All IPOPI Hard Talks webinars can be viewed on the IPOPI YouTube channel.

NEWBORN SCREENING

EU PID FORUM – THE EU'S SOHO REGULATION PROPOSAL AND ITS IMPACT ON PID PATIENTS

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. To achieve this objective, Screen4Rare organised, in collaboration with the MEP Alliance on Newborn Screening, two events at the European Parliament in 2023.

On June 28, on the occasion of International Neonatal Screening Day, Screen4Rare organised a meeting hosted by MEP Katerina Konečná (The Left, Czechia) on "What role for neonatal screening in EU rare diseases policies?". The meeting had the participation of the offices of MEP Stelios Kympouropoulos (EPP, Greece) and Brando Benifei (S&D, Italy), as well as Screen4Rare's partners and supporters. Speakers to this event were: Leire Solis, IPOPI health policy and advocacy senior manager; Marie-Chritistine Ouillade, board member of SMA Europe and Chair of the Steering Committee of the European Alliance for Newborn Screening in Spinal Muscular Atrophy, Dr Peter Schielen, and Prof Maurizio Scarpa, chairman of MetabERN, through a written statement, and Prof Jim Bonham, president of ISNS.

screen4rare

The speakers agreed on the need to continue the dialogue with EU policy makers to find the best solution to approach the NBS element in the current and upcoming legislative files. Another point of agreement was the need to keep an eye on the public trust element. Currently there is almost full compliance of the public. This trust needs to be maintained; the genetic screening aspect of the future of newborn screening makes this point particularly sensitive.

On December 6, 2023, Screen4Rare, (ESID) and (ISNS), in collaboration with the Alliance on Newborn screening for rare diseases held a meeting at the European Parliament (Brussels). The meeting focused on "The European Reference Networks and newborn screening – What has been achieved and what still needs to be done?" and was co-hosted by MEP Billy Kelleher (Renew, Ireland) and MEP Stelios Kympouropoulos (EPP, Greece) and counted with the participation of MEP Maria da Graça Carvalho (S&D, Portugal).

The meeting featured a video message from Commissioner for Health and Food Safety, Stella Kyriakides, presenting how the European Commission has been and continues to work towards supporting the development of newborn screening for rare diseases from different angles. Amongst the speakers in this meeting, IPOPI was represented by Leire Solis, Health Policy and Advocacy Senior Manager, and ISNS was represented by Prof Jim Bonham, President of ISNS, and Dr Peter Schielen, office manager of ISNS. Physicians representing 3 European Reference Networks working on newborn screening explained the work on newborn screening that their ERN had been doing: Prof Fabian Hauck (ERN Rita), Prof Teresinha Evangelista (EuroNMD), and Prof Maurizio Scarpa (MetabERN).

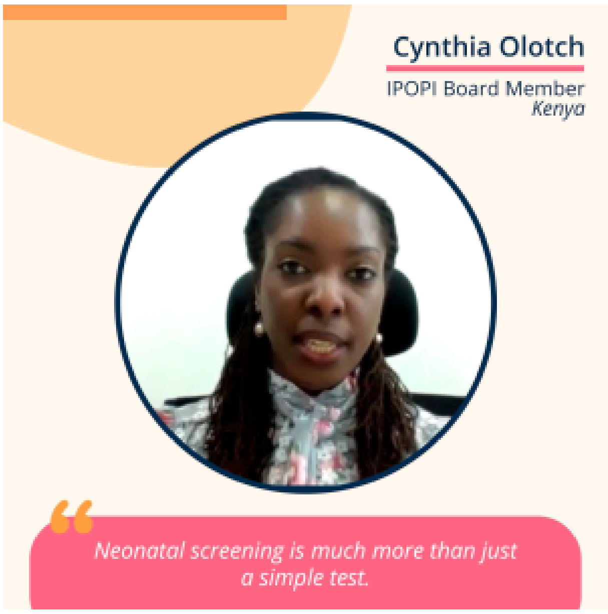
Other patient organisations participated in this meeting, such as SMA Europe, Eurordis and the SMA Screening Alliance.

INTERNATIONAL NEONATAL SCREENING DAY

Every June 28, for the past three years, the world commemorates the International Neonatal Screening Day (INSD). INSD is a Screen4Rare initiative, and during this day, we highlight the immense impact of neonatal screening for treatable rare diseases, in facilitating early detection and diagnosis of diseases and timely access to treatment and care.

INTERNATIONAL
NEONATAL
SCREENING DAY
JUNE 28

Screen4Rare's political event on June 28 at the European Parliament was coupled with a global social media campaign joined by many stakeholders including numerous IPOPI National Member Organisations, highlighting the importance of screening for severe combined immunodeficiency. IPOPI contributed to this campaign by sharing SCID newborn screening videos from different regions of the world. It was great to hear IPOPI Board members discuss newborn screening in Latin America, Malaysia, Africa, Canada & the US. Many countries around the world joined and celebrated the global call to ensure that every newborn with SCID is provided with the best possible start in life, no matter where they are born.



Neonatal screening is much more than just a simple test.

Today, on International Neonatal Screening Day,

Roberta Anido de Pena

IPOPI Board Member
Argentina

“Latin America and the Caribbean face many challenges,

Whitney Ayoub Goulstone

IPOPI Board Member
Canada

“Newborn screening identifies conditions in infants

EFFORTS TO SECURE A STABLE IMMUNOGLOBULIN SUPPLY

IPOPI firmly believes that it is crucial to continue working towards increasing plasma collection and, in turn, boost the supply of immunoglobulin, so that patients have a reliable and secure supply of their therapies. In all policy discussions on these topics, the primary focus should be on the patients, ensuring that those in need have access to life-saving treatments. Efforts must be made to strengthen plasma collection in all regions of the world and establish appropriate fractionation programmes. Throughout 2023, IPOPI took the lead in several collaborative patient-centred initiatives to advocate for global sufficiency in plasma-derived medicinal products (PDMPs) by promoting a more balanced distribution of plasma collection across different regions.

UNITAR JOINS HANDS WITH IPOPI

On July 6, 2023, UNITAR, the United Nations Institute for Training and Research, and IPOPI signed a Memorandum of Understanding (MoU). The MoU enhances our existing collaboration, elevating it to a true partnership. It promotes cooperation through various means, such as knowledge sharing, innovative education, training, capacity-building solutions, and raising awareness to improve access to healthcare in the field of plasma-derived therapies.

Through this new collaboration, we aim to make significant progress in healthcare and capacity-building initiatives. Together, we will strive to ensure that our efforts will result in real and lasting benefits for communities worldwide. Two pilot programmes have been started in Malaysia and Argentina.

WORLD BLOOD DONOR DAY

Under the slogan “Give blood, give plasma, share life, share often”, IPOPI commemorated World Blood Donor Day on June 14. The global celebration was used to raise awareness of the need for safe blood and blood products, including immunoglobulin replacement therapies, and to thank donors for their life-saving gifts. For the first time, the 2023 campaign included plasma in its slogan, showing its increasing importance as well as increasing awareness among decision-makers and stakeholders.

IPOPI communications campaign had a double objective: engage with the global audience on the importance of plasma donation and, at EU level, to reinforce the advocacy campaign and inform EU policy makers and EU public opinion about why the revision of the SoHO legislation was important for patients with primary immunodeficiencies relying on immunoglobulin replacement therapies. The campaign was structured on the basis of a combination of key messages with educational facts and figures, including visuals to facilitate sharing.

INTERNATIONAL PLASMA AWARENESS WEEK

Every year, at the beginning of October, the primary immunodeficiency (PID) community comes together to shed light on the need for plasma collection during International Plasma Awareness Week (IPAW), organised by the Plasma Protein Therapeutics Association on October 2-6, 2023.

IPOPI and its members joined IPAW under the slogan #YouAreMyMedicine. Our campaign spoke directly to donors, expressed profound gratitude for their generosity, and inspired new individuals to step forward and start donating. The message was clear: plasma donors are true lifelines for patients who depend on these life-saving medicines.

By joining this campaign, IPOPI highlights the urgency for increased and regionally balanced plasma collection to ensure continuous patient access to therapies.

#YOU
ARE MY
MEDICINE

NEW LEGISLATION UNDERWAY IN THE EUROPEAN UNION

The European Union launched in 2022 a process to review the legislation on blood and plasma collection. The European legislative process is lengthy and IPOPI was very active during 2023 to educate policy makers about the uniqueness of plasma and how it is critical for patients with primary immunodeficiencies. IPOPI efforts translated into a series of activities, including the EU PID Forum on the topic held at the European Parliament in January 2023, IPOPI's intervention at the HMA/EMA multi-stakeholder workshop on shortages in March 2023, the communication campaigns organised for World Blood Donor Day with dedicated EU messages and the International Plasma Awareness Week. Many of IPOPI's NMOs in the European Union have joined these efforts and helped spread the word about plasma collection's importance in their countries and in the EU in general.

IPOPI PARTICIPATES AT THE EUROPEAN MEDICINES AGENCY HMA/EMA MULTI-STAKEHOLDER WORKSHOP ON SHORTAGES

IPOPI took part in a European Medicines Agency HMA/EMA multi-stakeholder workshop on shortages in March 2023 and was invited to speak during the session dedicated to immunoglobulin replacement therapies. This workshop was a great opportunity to discuss the link between plasma supply and supply of immunoglobulins, provide stakeholders' views on the availability and supply of immunoglobulins and understand the recent developments, at the EMA, that are looking into shortages of medicines, including immunoglobulins.

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

02

STRATEGIC OBJECTIVE

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

IPOPI AFRICAN PID PATIENTS MEETING IN MOROCCO

IPOPI's African PID Patients' Meeting was held in Casablanca, Morocco on June 14-15 alongside the African Society for Immunodeficiency Congress. This gathering brought together more than 50 participants, including adults and children, from various countries across the African region (Algeria, Kenya, Morocco, Namibia, Rwanda, Senegal, South Africa, Sudan, Tunisia, Uganda).

The two-day patients' meeting provided a valuable platform for attendees to share insights on the current state of primary immunodeficiency disorders (PIDs) in the region. The discussions focused on the latest advancements in the treatment and care of PIDs in Africa, the role of technology in healthcare, and various other relevant topics. In addition, the participants actively engaged in a workshop focused on raising awareness about the importance of national financial coverage for PID treatments.

One highlight of the event was the impactful lecture delivered by Prof Aziz Bousfiha, who shed light on the importance of immunisation in safeguarding individuals with immunodeficiency disorders, to increase their protection.

IPOPI LATIN AMERICAN PID PATIENTS MEETING IN MEXICO CITY

The vibrant city of Mexico City served as the host to the IPOPI Latin American PID Patients Meeting on October 19-20, 2023, held in parallel with the Latin American Society for Primary Immunodeficiencies (LASID) meeting. A total of 35 participants from 12 Latin American countries, including Argentina, Bolivia, Brazil, Chile, Costa Rica, Ecuador, El Salvador, Mexico, Paraguay, Peru, Puerto Rico, and Uruguay, gathered to share experiences and insights.

A highlight of the meeting was the opportunity to hear from various national member organisations (NMOs) about their latest impactful projects in their respective countries. This exchange allowed participants to learn from each other's successes and innovations, fostering inspiration and collaboration.

The meeting has fostered a joint commitment to ongoing collaboration in addressing regional challenges, marking a step forward for those affected by primary immunodeficiencies in Latin America.

IPOPI NATIONAL PID EVENTS

IPOPI NATIONAL WORKSHOP IN INDONESIA

The IPOPI National PID Advocacy and Awareness Workshop was held in Jakarta, Indonesia on November 25-26, 2023, in collaboration with IPOPI´s Indonesian member, IPIPS, and its medical advisory panel.

Bringing together key patient leaders and medical advisors, the workshop focused on analysing challenges and identifying opportunities for the improvement of care for individuals with Primary Immunodeficiencies (PIDs) in Indonesia.

The IPOPI workshop in Indonesia emphasised crucial advocacy skills. Participants learned the importance of fostering connections, tailoring messages, and seizing opportunities in the dynamic advocacy landscape. The workshop highlighted the need to be strategic, prioritise effectively, and collaborate with fellow PID stakeholders for a more impactful and focused advocacy approach.

WORLD PI WEEK 2023 – TURNING REAL-WORLD DATA INTO KNOWLEDGE FOR BETTER PID CARE

IPOPI supports the yearly World Primary Immunodeficiencies Week (WPIW). This year’s campaign, held on April 22-29, focused on turning real-world data into knowledge for better care for patients with Primary Immunodeficiencies (PIDs).

An impressive milestone was achieved as IPOPI supported a record number of 38 National Member Organisations (NMOs), underscoring the strong engagement of patient organisations worldwide.

Throughout that week, diverse activities were organised across the globe to shed light on PIDs. These activities included PID congresses, impactful social media campaigns, walks, kite-flying events, press conferences, and patient surveys, among so many others. IPOPI thanks CSL Behring, Grifols, and Takeda for their support, which enabled the support of numerous outstanding initiatives during World PI Week.

Additionally, IPOPI launched a fantastic campaign during World PI Week, featuring video interviews with individuals from four continents. These videos emphasised the crucial need for improved data collection to enhance the quality of care for PID patients and showcased examples of the hurdles that patient organisations face when engaging in this activity.

During World PI Week 2023, there was an impressive level of engagement from the global PID community, patient organisations, healthcare professionals, and other stakeholders. The campaign demonstrated the collective power of collaboration and highlighted the crucial role of data collection in improving the quality of life for individuals with PIDs.

IPOPI NMO SUPPORT PROGRAMME 2023

Throughout 2023, IPOPI was pleased to be able to fund several NMO projects and campaigns through its NMO Support Programme among which:

- Bolivia - VI PID International Meeting
- China - Translation of IPOPI´s mini documentary into Chinese
- Iran - Translation of 28 pamphlets in a form of a book for PID
- United Kingdom - Printing and distribution of IPOPI leaflets
- Ukraine - Organise a series of seminars with a psychologist to relieve emotional stress

IPOPI NMO WEBCHATS

The IPOPI NMO Webchats are organised exclusively for IPOPI national patient organisations (NMOs), on a variety of topics identified as beneficial for our PID community. These online meetings provide both an educational lecture as well as an open forum for NMOs from around the world to discuss and ask questions. In 2023, we organised 5 webchats on subjects such as access to plasma, newborn screening and strategic planning workshop.

2023

5 WEBCHATS

IPOPI NMO NEWS BITES

The IPOPI NMO News Bites is a member-focused newsletter released 4 times a year. It features key sections like “Global Events”, “Medical Affairs News”, “Health Policy and Advocacy News”, proving essential information on important events, international campaigns, and internal deadlines for our members.

03

STRATEGIC OBJECTIVE

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.

4ID, IPOPI'S HEALTH APPLICATION HAS NEW FEATURES

The 4ID app is a digital health agenda designed specifically for patients with immunodeficiency, offers a range of features to help patients track their health.

On Rare Disease Day, February 28, we released new features for 4ID, our health management app designed specifically for patients with primary and secondary immunodeficiency. These features will help patients track their general health as well as their immunodeficiency-related parameters, schedule appointments, log absences from work and school, and much more.

https://4id.ipopi.org/

THREE NEW IPOPI PID INFORMATION LEAFLETS IN 2023

For a number of years, IPOPI has been producing information leaflets on a range of topics related to primary immunodeficiencies (PID). In 2023 three new IPOPI information leaflets were published, covering the following topics:

- The Management of SCID
- Management of Hyper IgM Syndromes
- PID Warning Signs Across Medical Specialties

The Management of SCID explains what SCID is, the manifestations of SCID, how it is diagnosed, treated and managed and the role of newborn screening in achieving an early diagnosis.

Management of Hyper IgM Syndromes explains what hyper IgM syndromes are, the signs and symptoms of the diseases, and how they are diagnosed, treated and managed.

PID Warning Signs Across Medical Specialties outlines the warning signs of primary immunodeficiency (PID) from the perspectives of the different medical specialties that may encounter these patients.

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.

NEW VIDEO SERIES ON PRIMARY AND SECONDARY IMMUNODEFICIENCIES

NEW MINI DOCUMENTARY

IPOPI has expanded its educational resources by releasing an insightful mini documentary: A Closer Look at Primary and Secondary Immunodeficiencies.

This informative 15-minute film delves into the similarities and differences between these conditions, the complexity of diagnosing primary immunodeficiencies post-malignancy treatment, the importance of pre-treatment screening, and the challenges faced in diagnosing immunodeficiencies in low and middle-income countries.

The documentary offers a comprehensive exploration of primary and secondary immunodeficiencies, unravelling their similarities and differences. Through in-depth interviews with leading experts from Spain, Wales, and India, viewers gain valuable knowledge about these conditions, their underlying causes, and their impact on individuals' immune systems. We thank Professor Silvia Sánchez-Ramón, Professor Stephen Jolles and Professor Surjit Singh for these contributions.

We also featured interviews with Juan Manuel Martínez Cuenca and Vivian Kohlberg, two remarkable patients, offering insights into the unique challenges they face and their perspective on managing their conditions. Vivian particularly highlights her experience from living with immunodeficiency in Bolivia, demonstrating the need for improved access to care worldwide.

THE COMPLEXITY OF DIAGNOSING PRIMARY IMMUNODEFICIENCIES IN PATIENTS WITH MALIGNANCY

One of the documentary's focal points is the complexity surrounding the diagnosis of primary immunodeficiencies in individuals who have undergone treatment for cancer. We shed light on the challenges faced by healthcare professionals in differentiating between treatment-related secondary immunodeficiencies and pre-existing primary immunodeficiencies. This critical topic underscores the significance of early detection and emphasises the need for PID screening before initiating malignancy treatment, and multidisciplinary collaboration across disciplines.

ADDRESSING GLOBAL CHALLENGES IN IMMUNODEFICIENCY DIAGNOSIS

Our mini documentary also takes a global perspective, highlighting the challenges faced in diagnosing immunodeficiencies in low and middle-income countries and accessing treatment. By shedding light on resource limitations, lack of awareness, and inadequate access to diagnostic tools, we aim to raise awareness and promote discussions around addressing these disparities to ensure timely and accurate diagnoses for patients worldwide.

This documentary was developed to serve as an educational tool for healthcare professionals and patient advocates. By exploring the complexities of diagnosis, global challenges, and real-life patient experiences, we aim to foster understanding, advocacy, and advancements in the field of primary and secondary immunodeficiencies.

Q-ID QUIZ: ALL ABOUT PRIMARY AND SECONDARY IMMUNODEFICIENCIES

In 2023, IPOPI through its Q-ID quiz app introduced a quiz designed to test its users' knowledge about primary and secondary immunodeficiencies. The quiz served as a valuable tool to engage with the subject and further educate individuals in this specialised field.

These resources, while complementary, offer unique insights into the world of immunodeficiencies.

IPOPI strives to broaden understanding and knowledge of primary and secondary immunodeficiencies. We hope that the above-mentioned resources will prove useful for the community and improve the diagnosis and care for these conditions.

IPOPI E-NEWS

One of our flagship publications, IPOPI e-News, is sent directly to thousands of readers and is also shared on social media. Published every quarter, e-News highlights the most significant achievements of IPOPI and its members in the previous months. 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

04

STRATEGIC OBJECTIVE

Strengthen multi-stakeholder cooperation

IPOPI values the benefits of working together with partners and stakeholders and 2023 was a shining example of new partnerships being built.

TRANSFORM ALLIANCE

Martine Pergent, President of IPOPI, was elected as patient representative in the Board of the European Alliance for Transformative Therapies (TRANSFORM). This Alliance is a multi-stakeholder initiative representing patient groups, medical experts and associations, scientist and researchers and industry interested in the area of cell and gene therapies. TRANSFORM aims to foster dialogue between the initiative and European decision-makers so that evidence-based policy recommendations can be issued with the objective of enabling safe and timely patient access to cell and gene therapies, while ensuring the sustainability of healthcare systems.

European Alliance for Transformative Therapies

RECOMB'S ANNUAL MEETING

Recomb is a European research consortium dedicated to developing gene therapy for recombination-deficient severe combined immunodeficiency (RAG1-SCID), of which IPOPI is a partner. In 2023, several new clinical sites received ethical approval to treat patients including in Barcelona (Spain), Wroclaw (Poland), Kayseri (Turkey) and Melbourne (Australia).

The trial recruited its second patient which diagnosed with RAG1-SCID through newborn screening in the Netherlands, a few months post-treatment, the patient is in good condition and awaiting immune recovery.

Recomb gene therapy

The Recomb research programme was also granted an additional 24 months, setting a new official end date for December 31, 2024. Thanks to the generous decision by the EU Horizon 2020 programme, this extension allows for continued advancements for this groundbreaking project.

Building on the positive experience in 2023, and with additional international clinical sites opening, Recomb hopes to recruit additional patients.

As the leader of the work package for dissemination, IPOPI is very pleased to participate in this project to make a difference for patients with RAG1-SCID.

www.recomb.eu

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

ACCESS TO GENE THERAPIES FOR RARE DISEASES (AGORA)

Johan Prevot, Executive Director of IPOPI, became a board member of the recently established AGORA initiative (Access to Gene therapies fOr RAre diseases). AGORA's mission is to facilitate access to effective gene therapies for patients with ultra-rare diseases in Europe. Specifically, AGORA aims to establish a sustainable framework that increases access to pre-marketing authorisation phase treatments for ultra-rare diseases, with a focus on curative, one-shot treatments for single-gene disorders. As gene therapy holds immense potential for PID treatment, IPOPI is delighted that its patient community can be represented in this consortium.

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.

rita
Primary Immunodeficiencies / Autoinflammatory Disorders
Autoimmune Diseases / Paediatric Rheumatic Diseases

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. As a member of the EU Health Coalition, IPOPI has actively participated in the Coalition's Partners' exchange of views on the revision of the pharmaceutical legislation held in April as well as in several other relevant committee and plenary meetings.

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 represents 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.

On January 24-25, in Estoril, PLUS organised its Stakeholder Consensus Conference. The meeting was very well attended by representatives from the following organisations: Alpha-1 Belgium, American Plasma Users Coalition (APLUS), European Blood Alliance (EBA), European Haemophilia Consortium (EHC), European Plasma Alliance, GBS-CIDP Foundation International, International Federation of Blood Donor Organisations, International Plasma and Fractionation Association (IPFA), International Patient Organisation for Primary

Immunodeficiencies (IPOPI), Plasma Protein Therapeutics Association (PPTA).

The two-day conference was very productive and resulted in a joint PLUS Stakeholders' statement on the Commission proposal for a regulation on substances of human origin (SoHO) that gathered the endorsement of all participating organisations.

110,000
EUROPEANS
living with treatable rare plasma-related disorders.

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, medicine shortages, involvement in clinical trials, data transparency and more. IPOPI also takes part in topic-specific meetings and at the Enpr-EMA (the European Network of Paediatric Research at the European Medicines Agency). Leire Solis, senior health policy and advocacy manager supports Jose Drabwell in this role.

IPOPI NATIONAL PID EVENTS

IPOPI attended and participated in the following events among others:

- EU Health Coalition Health Systems Integration policy group, throughout the year
- ERN-RITA Board Meetings, Patients Committee, Communications WP, throughout 2023
- ERN RITA transition working group meetings, throughout 2023
- EMA PCWP/HCPWP meetings, throughout 2023
- EMA SPOC meetings, throughout 2023
- European Alliance for Transformative Therapies (TRANSFORM) meetings, throughout the year
- ICSPP steering committee meeting, throughout 2023
- RDI advocacy committee webinars, throughout the year
- RIPAG meetings, throughout the year
- UNITAR liaison meetings, throughout 2023
- World PI Week steering committee meetings, throughout 2023
- PLUS stakeholders meeting, January 2023
- Skills building programme, Clinical Immunology Department of Erasmus Medical Center in Rotterdam, January 2023
- Consensus Workshop on Structured Treatment Plan for ADA-SCID-GT, January 2023
- Portuguese MoH meeting "Reunião Imunodeficiências combinadas graves", January 2023

- Webinar “Regulatory Systems and regulations to support clinical trial conduct in Africa”, January 2023
- Webinar “Therapeutic adherence during the COVID-19 pandemic across Europe”, January 2023
- EU Health Horizon webinar “Patient-centred approaches to Long COVID”, February 2023

Africa CDC PGI Webinar, February 2023

- “Improving health literacy & protecting the value of access to care for better public and patient involvement in mitigating COVID: EU training session”, February 2023
- ESID Grand Rounds: Contemporary Management of DN-STAT3 / Job’s syndrome, February 2023
- DIA Europe Transparency webinar, February 2023
- European Commission workshop on “Best practices in the public procurement of medicines”, February 2023
- TRANSFORM alliance meeting, February 2023
- EU Parliament ENVI Committee debate on SoHo legislation draft report, February 2023
- IPOPI NMO Webchat “It’s all about data!”, February 2023
- CORD Global Workshop on Access to Rare Disease Diagnosis and Treatment, February 2023

“Laboratory Medicine and its challenges in low- and middle-income countries: redefining its leadership, impact of laboratory tests and advantages of a clinical laboratory 4.0” webinar, March 2023

- European Medicines Agency HMA/EMA multi-stakeholder workshop on shortages, March 2023
- EMA PCWP meeting, March 2023
- IFPMA workshop on African Medicines Agency, March 2023
- EMA patient experts training session, March 2023
- Cerus Round Table - Blood, Tissues and Cells equity of care in Europe, March 2023
- “Time to act: Building towards a pharmaceutical framework fit for rare diseases” event at the European Parliament, March 2023
- GATA2 research consortium meeting, March 2023
- AGORA meeting, March 2023
- Advanced therapies congress, March 2023
- DIA Europe - Learnings from the COVID-19 for future transparency principles, March 2023
- EU Health Coalition - Partners’ exchange of views on the revision of the pharmaceutical legislation, April 2023
- UNITAR webinar “Leaving no one behind: Meeting patients’ need for plasma-derived therapies”, April 2023
- TRANSFORM Alliance meeting: Pharmaceutical legislation proposal, April 2023
- Politico round table on revision of the pharma package, April 2023
- WHO Expert Committee on Selection and use of EML, April 2023
- EU Parliament ENVI session on pharma legislation, April 2023
- Workshop on “The impact of COVID-19 on immunocompromised patients and the lessons learned”, May 2023
- Health First Europe steering committee meeting, May 2023
- First European Update Congress in Rheumatology, May 2023
- U-IMD 2nd Workshop on ERNs registries, May 2023

- APPLN 2023 Meeting: Forging Ahead with Access to Plasma Therapies, May 2023
- Politico event on preventing shortages, May 2023
- EU Health Coalition plenary meeting, May 2023
- PCWP/HCPWP webinar on ICH draft principles, May 2023
- ESPID Lisbon 2023 conference, June 2023
- ERN-RITA General Assembly Meeting, Utrecht, June 2023
- EURORDIS Open Academy School on Medicines R&D, June 2023
- Workshop “EU Pharmaceutical Package: A once-in-a-generation opportunity to deliver the promise of transformative therapies to all European patients”, June 2023
- AI-ID workshop, June 2023
- PHFE Annual General Meeting, June 2023
- AGORA Board meeting, June 2023
- International Plasma Protein Congress, June 2023
- Indian World Immunology Day conference, June 2023
- RECOMB consortium meeting, June 2023
- EU-PAD Consortium meeting, July 2023
- IPOPI NMO Webchat “Plasma in the spotlight”, July 2023
- EBA SUPPLY multi-stakeholder workshop, September 2023
- AGORA Board meeting, September 2023
- Meeting with EU Health Commissioner on newborn screening, September 2023
- EU Health Coalition plenary meeting, October 2023
- EU Health Summit (Friends of Europe think tank), October 2023
- TRANSFORM MEP Alliance event, October 2023
- ESGCT conference, October 2023
- AMR One Health EU Parliament meeting, October 2023
- PLUS General Assembly, October 2023
- Health First Europe General Assembly, November 2023
- EMA Workshop on the Generation of Clinical Evidence for the Treatment and Prevention of Long-COVID and PASC, November 2023
- EuropaBio Forum Unmet Medical Needs, November 2023
- ERICA & EJP RD Joint Conference, November 2023
- World Allergy Congress, December 2023
- Active Citizenship Network webinar vaccines and AMR, November 2023
- TRANSFORM workplan 2024 meeting, December 2023
- The Economist “Leaving no one behind” Webinar, December 2023
- EMA Clinical Trials Information System Forum, December 2023
- Access to care / patient empowerment meetings and/or calls with doctors and patients in Algeria, Argentina, Australia, Brazil, Bangladesh, Belgium, Bolivia, Cambodia, Canada, Chile, China, Ecuador, Egypt, Ethiopia, France, Greece, Hungary, India, Indonesia, Iran, Ireland, Israel, Italy, Kenya, Malaysia, Moldova, Morocco, Mexico, Netherlands, Nepal, Philippines, Poland, Romania, Russia, Senegal, South Africa, Sudan, Thailand, Uganda, Ukraine, United Kingdom, Venezuela, Zimbabwe among others

IPOPI IN 2023 AND BEYOND

IPOPI remains dedicated to assisting the PID community through international awareness, advocacy, and medical education.

Exciting initiatives are underway, including two mini-documentaries on plasma and immunoglobulins, our XVIII Global Patients Meeting (GPM), an Asian regional meeting in Japan, forums at the European Parliament, national advocacy campaigns, and much more.

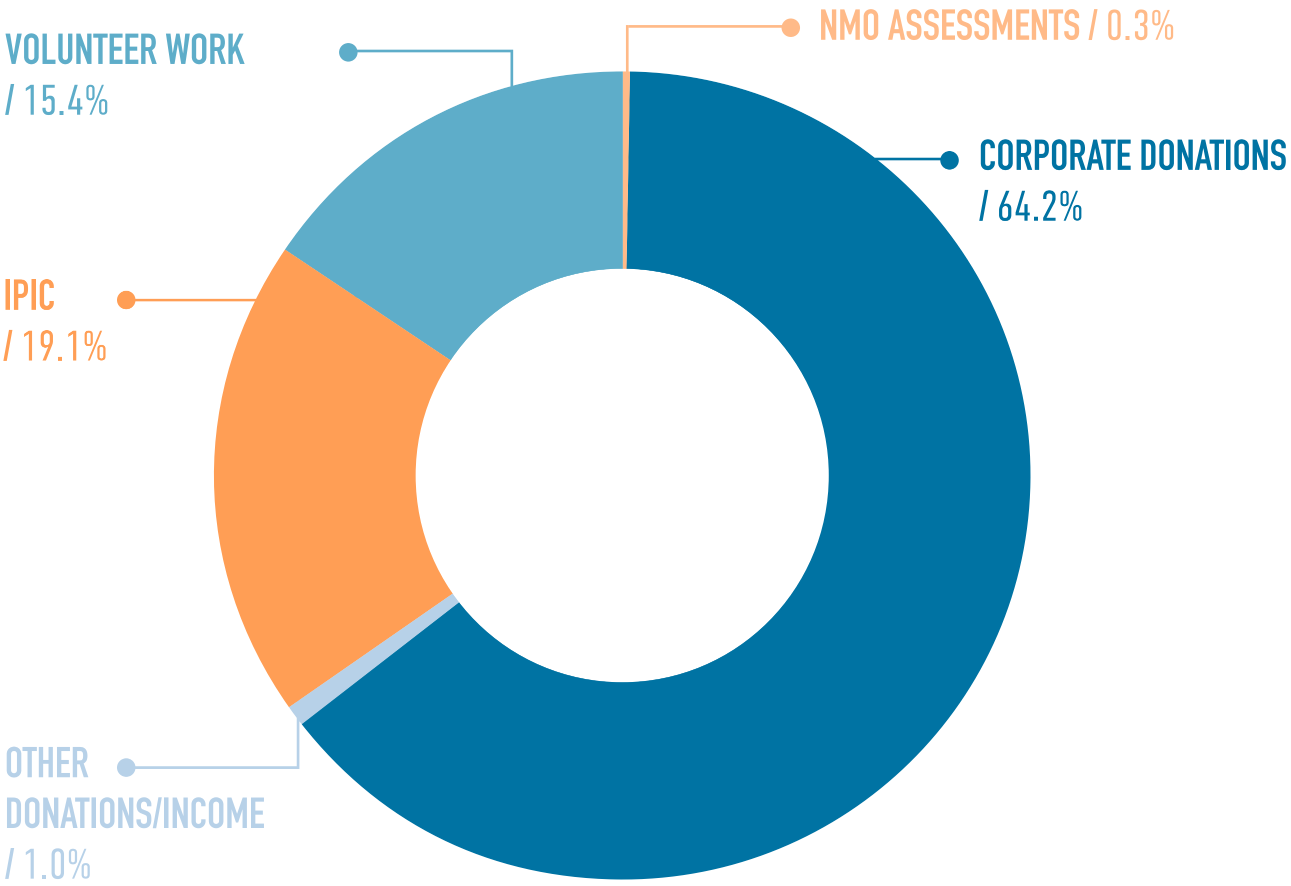
The launch of new projects and NMO support programmes, as well as national and regional meetings and workshops, will help to strengthen PID patient organisations worldwide.

We look forward to bringing together IPOPI members at the GPM, to be held in Marseille, France, on October 16-19, 2024.

FINANCIAL REPORT

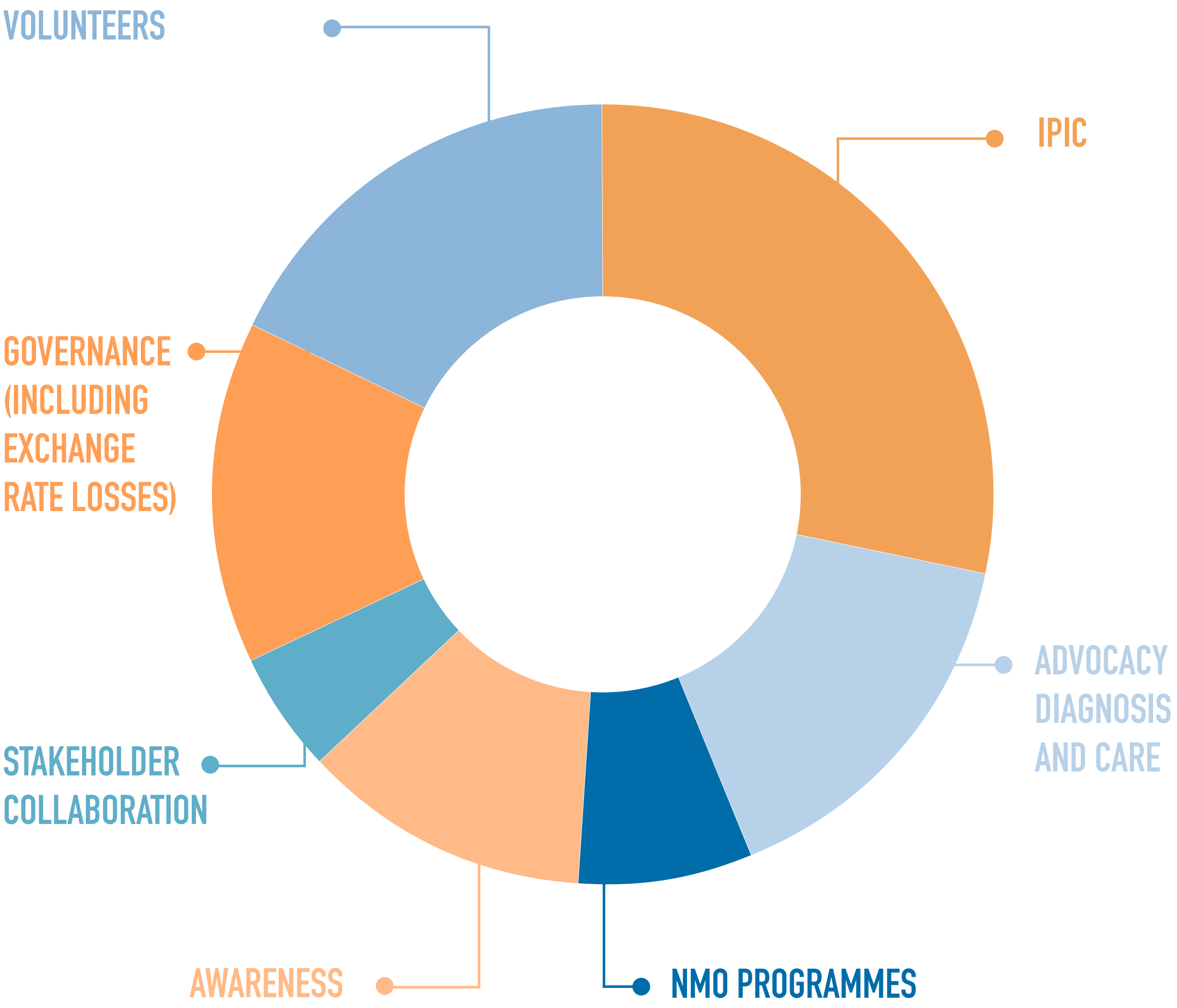
2023

INCOME 2023 (EURO)



SUMMARY OF INCOME (2023)	€ EURO	% PERCENTAGE
NMO Assessments	6 450	0.3
Corporate donations	1 571 424	64.2
Other donations/income	24 735	1.0
IPIC	468 498	19.1
Volunteer work	375 606	15.4
TOTAL INCOME	2,446,713	100.0

EXPENDITURE 2023 (EURO)



EXPENDITURE 2023	€ EURO
IPIC	604 790
Advocacy Diagnosis and Care	331 275
NMO programmes	158 055
Awareness	252 048
Stakeholder Collaboration	105 771
Governance (including exchange rate losses)	305 064
Volunteers	375 606
Towards reserve and GPM2024	314,104
TOTAL EXPENDITURE	2,446,713

No contribution received from a single company reached the majority of the total income. The highest single contribution by a company represented 12.4% of the total income including volunteer time contributions and 14.6% 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 73 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.

73
NATIONAL MEMBER ORGANISATIONS

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

Takeda

GRIFOLS

GOLD

CSL Behring

Chiesi
global rare diseases

SILVER

Pfizer

Pharming

SUPPORTERS

ENZYVANT

Biotest
From Nature for Life

LFB
ETHICAL COMMITMENT

PROJECT SPONSORS

In 2023, the following corporate sponsors provided unrestricted grants towards various IPOPI ad-hoc projects that enhance our awareness, advocacy, communications, events and representational activities.

- Astra Zeneca
- BD
- BioMerieux
- Biotest
- BSD Robotics
- Cane Spa
- Chiesi
- CSL Behring
- Enzyvant
- FDE – So CONNECT
- Grifols
- ImmunoIVD
- Innovative Health Sciences
- Kedrion
- Koru
- Novartis gene therapies
- Octapharma
- Orchard Therapeutics
- Perkin Elmer
- Pfizer
- Pharming
- Takeda
- The Binding Site
- 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 2023 including but not limited to:
Active Citizenship Network, ACT4EU research consortium on ATMPs, AGORA gene therapies consortium, African Society for Immunodeficiencies (ASID), Asia Pacific Plasma Leaders Network (APPLN), Asia Pacific Economic Cooperation (APEC), Asia Pacific Society for Immunodeficiency (APSID), Rare Diseases committee, Council of Europe, Care-for-Rare Foundation, EU-PAD microbiome research consortium, European Centre for Disease Prevention and Control (ECDC), European Medicines Agency's Patient and Consumer Working Party(PCWP), European Medicines Agency's COVID-19 Task Force, European Medicines Agency's Medicines Shortages Single Point of Contact (SPOC) Working Party, EuropaBio Patients Bio-Forum, European Commission SoHo Unit, European Foundation for the Care of Newborn Infants (EFCNI), European Health Parliament Think Tank, European Network For Paediatric Research (Enpr-EMA), European Patients Forum (EPF), European Rare Disease Organisation (EURORDIS), European Reference Network on Immunological Diseases (ERN-RITA), European Reference Network Expert Platform on NBS, ESID Registry steering committee, ESID Clinical Working Party, the European Society for Immunodeficiencies (ESID), EU Health Coalition Steering Committee, Friends of Europe think tank, 3D GATA2 research consortium, Health First Europe (HFE), Integrate ATMP Research Consortium, International Coalition for Safe Plasma Proteins (ICSPP), International Nursing Group for Immunodeficiencies (INGID), International Immunocompromised Host Society (ICHS), International Society Blood Transfusion (ISBT), International Society for Neonatal Screening (ISNS),International Union of Immunological Societies (IUIS), J-Project, Latin American Society for Immunodeficiencies (LASID), NAUTILUS research consortium, NGO Committee for Rare Diseases, Platform of Plasma Protein Users consulting to the European Commission (PLUS), PPTA Stakeholders Group, Rare Diseases International (RDI) advocacy committee, RECOMB consortium, South East Asia Primary Immunodeficiencies Network (SEAPID), SPRING research consortium on newborn screening, TRANSFORM, TREAT JAK-STAT research consortium, United Nations Institute for Training and Research (UNITAR), UK Plasma Action Group, World Allergy Organization, World Health Organisation expert committees, World PI Week Steering Committee, other national PID doctors societies.

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

IPOPI.org

FOLLOW IPOPI

IPOPI
INTERNATIONAL
PATIENT ORGANISATION
FOR PRIMARY IMMUNODEFICIENCIES