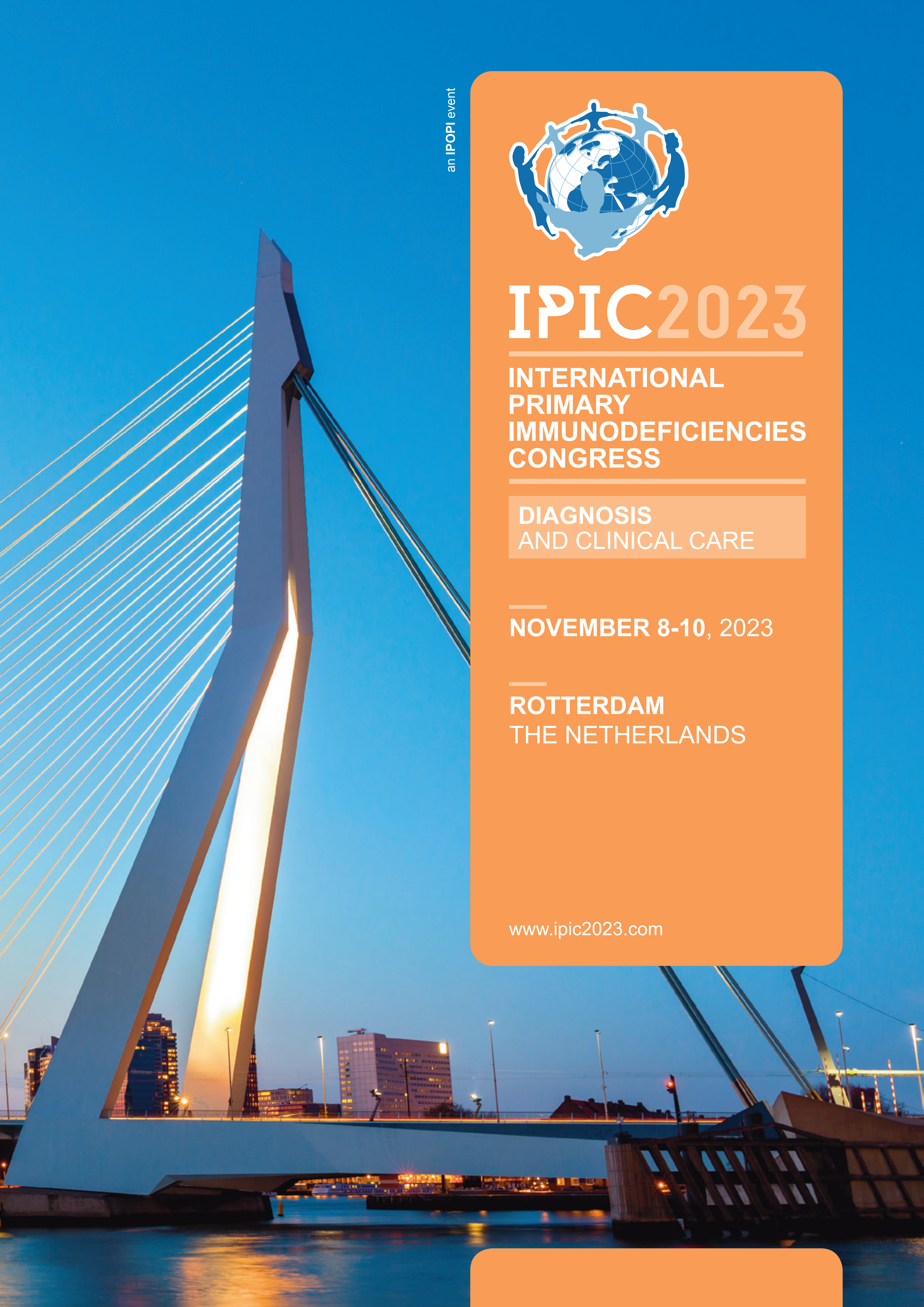
REVIEW OF ACTIVITIES AND ACHIEVEMENTS

IPOPI.ORG

2022

IPOPI
INTERNATIONAL
PATIENT ORGANISATION
FOR PRIMARY IMMUNODEFICIENCIES



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

In 2022, we celebrated IPOPI's 30th anniversary! Three decades ago, a group of determined parents and doctors came together, recognising the potential for organised primary immunodeficiency (PID) patients to collaborate with medical professionals in addressing the challenges posed by rare and unknown diseases like PIDs.

The anniversary was commemorated during our Global Patients Meeting in Gothenburg, Sweden, with 100 delegates from 34 countries. During this 30th-anniversary celebration, we seized the opportunity to appreciate current and successive Board and Staff members, who all have played an integral role in shaping IPOPI's remarkable journey. Today, IPOPI stands strong with 70 member countries, fostering a warm family of patient representatives and a resilient community of devoted patient advocates working tirelessly for the health and improved quality of life of individuals with PID.

As we mark this anniversary, we can't help but feel a sense of pride and joy. It's a milestone that holds special significance for us, as it represents the countless moments of hard work, shared laughter, and unwavering determination that have shaped our organisation. Over the years, we have grown and evolved, facing both triumphs and challenges along the way. But it is the people - our dedicated team, supportive partners, and cherished community - who have truly made this journey worthwhile.

As we conclude our eventful 30th year, we warmly invite you to enjoy our annual review and explore the highlights from 2022. It's a chance to catch a glimpse of the moments that made this year one to remember.

Martine Pergent
IPOPI President

Johan Prévot
IPOPI Executive Director

EXECUTIVE COMMITTEE, OFFICERS AND STAFF 2022

EXECUTIVE COMMITTEE

Martine Pergent, Chair

Christine Jeffery, Vice-Chair (Until October)

Bruce Lim, Treasurer

Whitney Ayoub Goulstone (From October)

Jose Drabwell

Andrea Gressani

Cynthia Olotch

Roberta Anido de Pena

John Seymour

Otilia Stanga

MEDICAL ADVISORY PANEL

Dr Nizar Mahlaoui, Chair – France

Prof Martin van Hagen, Vice Chair – Netherlands

PANEL MEMBERS

Dr Adli Ali – Malaysia

Prof Tadej Avcin – Slovenia

Prof Aziz Bousfiha – Morocco

Prof Antonio Condino-Neto – Brazil

Prof Charlotte Cunningham-Rundles – US

Dr Virgil Dalm – The Netherlands

Prof Tandakha Dieye – Senegal

Prof Alain Fischer – France

Dr Elie Haddad – Canada

Dr Nahla Hashim Hassan Erwa – Sudan

Prof Steven Holland – US

Dr Pamela Lee – Hong Kong

Prof Isabelle Meyts – Belgium

Dr Olaf Neth – Spain

Dr Cecilia Poli – Chile

Dr Elizabeth Rivers – UK

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

Miriam Ferreira: Programmes Assistant

Clare Glynn: Accounting and Administration Manager

Natalie Helena: Events Manager

Renée King: Communications Officer

Carla Morgado: Office Manager

Julia Nordin: NMO Programmes Manager

Leire Solis: Health Policy and Advocacy Senior Manager

Carol Tavener: Accounting Assistant

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

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.

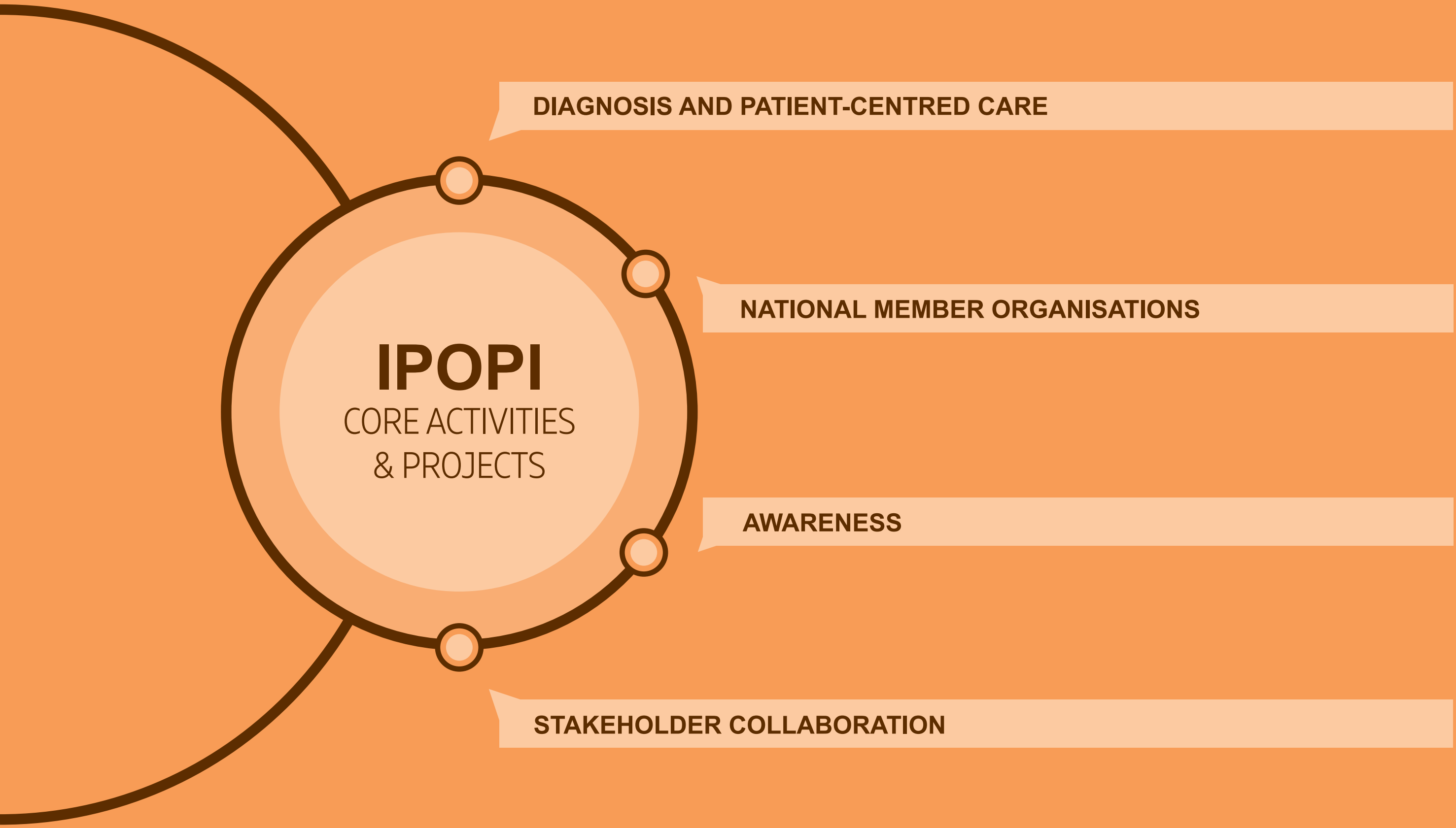
IPOPI STRATEGIC OBJECTIVES 2021-2025

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. 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.

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 Organization, 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 a subcutaneous infusion of recombinant human hyaluronidase (known as ‘facilitated subcutaneous immunglobulin, fSCIG), allows patients to receive 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, please refer to the IPOPI leaflets available on the website.

IPOPI'S KEY ACHIEVEMENTS AND ACTIVITIES, 2022

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

IPOPI remains steadfast in its commitment to bringing about long-term sustainable improvements in the diagnosis of PID and ensuring that all PID patients have access to appropriate, patient-centered care. Through various advocacy, awareness-raising, and educational campaigns and programmes, IPOPI works tirelessly to make a difference. By continuing these efforts, IPOPI strives to create a better future where the diagnosis of PID is improved, and all patients receive the quality care they deserve.

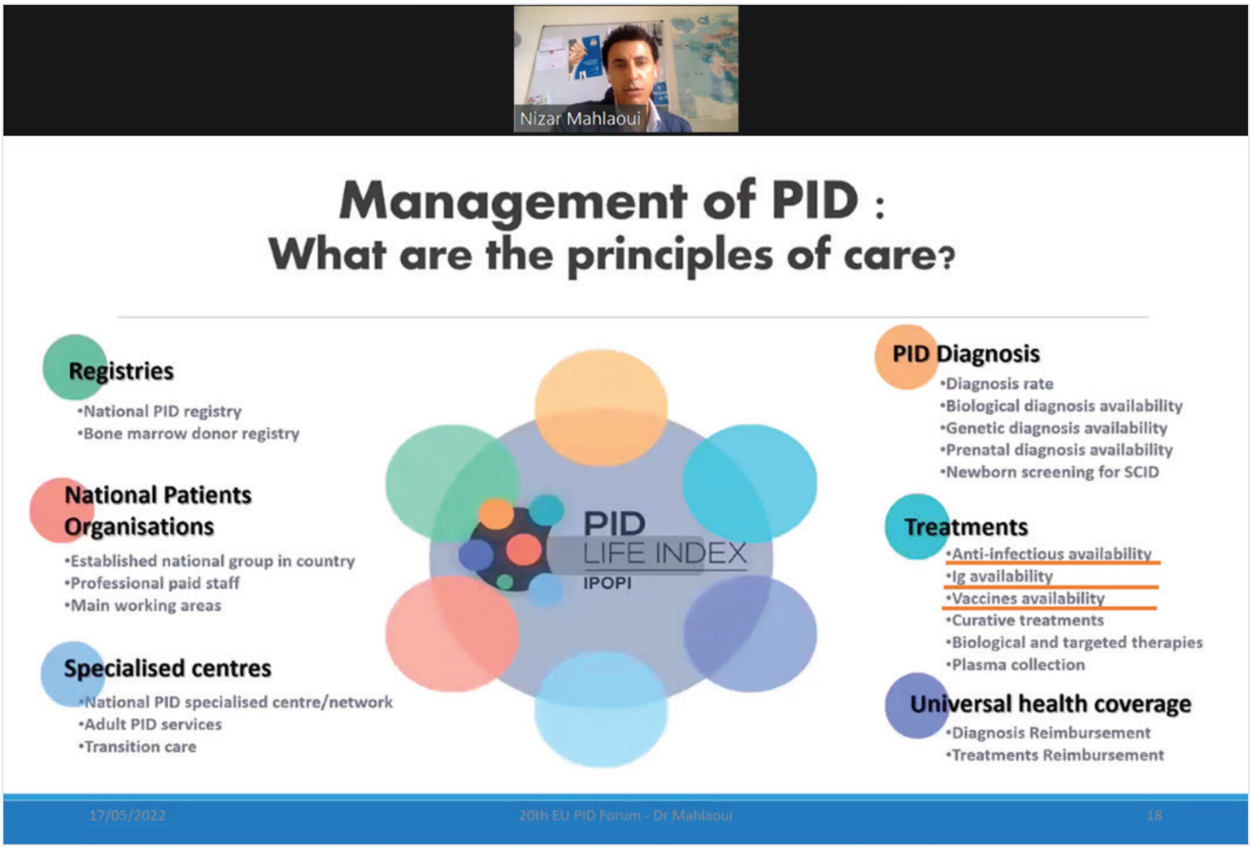
20TH IPOPI EU PID FORUM – ANTI-MICROBIAL RESISTANCE AND THE PID PERSPECTIVE

In May 2022, IPOPI organised the 20th IPOPI PID Forum on the topic of Anti-microbial resistance (AMR) from the PID perspective. The event was chaired by Member of European Parliament (MEP) Juocas Olekas (S&D, Lithuania) and MEP Sarah Wiener (Greens/EFA, Austria), with Johan Prevot (IPOPI Executive Director) and Martine Pergent (IPOPI President) serving as moderators. The forum featured a diverse range of speakers, including Davide Catena, a PID patient, Dr Nizar Mahlaoui from Necker-Enfants Malades Hospital, Kristine Peers representing EFPIA, and Dr Dominique Monnet from ECDC. Discussions centred around AMR challenges for PID patients, recent EU developments, and the importance of considering vulnerable populations. The event aimed to raise awareness, advocate for antibiotic development support and research, and express gratitude to participants and policymakers for their contributions to PID patients.

20th PID Forum

Anti-Microbial Resistance and the PID Perspective

Virtual Event
17th May 2022 15:30-17:00 CET
Co-hosted by MEPs Juozas Olekas (S&D, Lithuania) & Sarah Wiener (Greens/EFA, Austria)



Management of PID :
What are the principles of care?

Registries
- National PID registry
- Bone marrow donor registry

National Patients Organisations
- Established national group in country
- Professional paid staff
- Main working areas

Specialised centres
- National PID specialised centre/network
- Adult PID services
- Transition care

PID Diagnosis
- Diagnosis rate
- Biological diagnosis availability
- Genetic diagnosis availability
- Prenatal diagnosis availability
- Newborn screening for SCID

Treatments
- Anti-infectious availability
- Ig availability
- Vaccines availability
- Curative treatments
- Biological and targeted therapies
- Plasma collection

Universal health coverage
- Diagnosis Reimbursement
- Treatments Reimbursement

17/05/2022 20th EU PID Forum - Dr Mahlaoui 18

21ST IPOPI EU PID FORUM – IMMUNOGLOBULIN SHORTAGES FOR PATIENTS WITH PIDS

IPOPI's 21st PID Forum took place virtually in September 2022, and centred around the crucial topic of "Immunoglobulin shortages for patients with PIDs." The event proved impactful, thanks to the collaborative efforts of Members of the European Parliament Istvan Ujhelyi (S&D, Hungary) and Kateřina Konečná (GUE/NGL, Czech Republic), who served as co-hosts. Guiding the discussions as the moderator was Johan Prevot, IPOPI Executive Director. The Forum focused on Ig replacement therapies, plasma collection, and the impact of supply tensions on PID patients. It aimed to explore collaborative approaches to address these challenges and ensure patients' access to treatment. Presentations were given by Dominika Misztela (PPTA) on Ig therapy development, Deirdre Fehily (European Commission) on proposed regulation's impact on plasma shortages, and Leire Solis (IPOPI Health Policy and Advocacy Senior Manager) on Ig shortages from a patient perspective. An interactive panel discussion included Otilia Stanga (ARPID), Prof. Siobhan Burns (ESID), and Leni von Bonsdorff (IPFA). Open floor discussions benefited from diverse participant expertise.

21st PID Forum

Medicine shortages in the EU:
the case of immunoglobulin and PIDs

Virtual Event
8th September 13:30-15:00 CEST
Hosted by MEP István Ujhelyi (S&D, Hungary)

22ND IPOPI EU PID FORUM – THE EU'S SOHO REGULATION PROPOSAL AND ITS IMPACT ON PID PATIENTS

Following a long virtual period due to the pandemic, the 22nd PID Forum took place in-person in the European Parliament in Brussels. The forum was co-chaired by the Members of the European Parliament Katerina Konecna (the Left, Czechia) and Nicolás González Casares (S&D, Spain), and focused on the upcoming revision of the EU legislation on Substances of Human Origin (SoHO). The SoHO legislation rules on, amongst other areas, how blood and plasma for the manufacturing of plasma-derived medicines such as immunoglobulins is collected and managed. The revision of this European legislation can have a big impact on patient access to immunoglobulins and other plasma-derived medicinal products. This is why IPOPI organised the Forum on this topic and is working with several MEPs to ensure that the legislation is patient-centred and ensures a high protection to donors.

The Forum was moderated by Ms Martine Pergent, IPOPI President, and had the interventions of Mr Johan Prevot, IPOPI Executive Director, Mr Matthew Hotchko, President of the Marketing Research Bureau, Nancy di Salvo, CIDP-GBS Foundation International, and Mr Milan Maly, UNICAplasma.

IPIC 5TH EDITION IN VILAMOURA, PORTUGAL

The 5th Edition of the International Primary Immunodeficiencies Congress (IPIC) took place in Vilamoura, Portugal, in April 2022. The congress had been postponed from November 2021 because of the COVID-19 pandemic and despite remaining challenges posed by the latter and the unrest in Ukraine, the organising committee successfully held an in-person congress.

IPIC5th Edition brought together 600 delegates interested in the diagnosis and clinical care of primary immunodeficiencies (PIDs). Young scientists and physicians presented over 130 approved abstracts, showcasing their research in the field.

The programme celebrated the achievements of the global PID community while highlighting the remaining challenges. IPIC's success lies in its well-balanced scientific program, incorporating input from doctors, scientists, patients, and nurses, to complement other international congresses and scientific meetings. IPOPI was very pleased to have been able to organise such a successful congress despite the complicated circumstances at the time.


NEW MEMBERS OF IPOPI'S MEDICAL ADVISORY PANEL

DR NAHLA HASHIM HASSAN ERWA (SUDAN)

PROF ELIE HADDAD (CANADA)

PROF STEVEN HOLLAND (USA)

DR PAMELA LEE (HONGKONG)

DR CECILIA POLI (CHILE)

DR ELIZABETH RIVERS (UK)

MEDICAL ADVISORY PANEL

The MAP provides scientific and clinical advice to the Board of Directors and Staff Members. They contribute invaluable medical expertise to IPOPI PID information leaflets and the organisation of the International Primary Immunodeficiencies Congress (IPIC). In addition, they provide guidance to patients from all over the world and support IPOPI in addressing public health priorities and new developments in the PID field such as plasma and immunoglobulin supply, SCID Newborn Screening and gene therapy, among others.

In 2022, IPOPI had the pleasure of welcoming six new members, namely: Dr Nahla Hashim Hassan Erwa (Sudan), Professor Elie Haddad (Canada), Professor Steven Holland (USA), Dr Pamela Lee (Hongkong), Dr Cecilia Poli (Chile) and Dr Elizabeth Rivers (UK).
IPOPI appreciates the commitment of all Panel members, but especially Dr Nizar Mahlaoui (Chairman) and Prof Martin van Hagen (Vice-Chairman) for their kind contribution and dedication.

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.

Dr. Laila Kamrul and Dr. Ismet Nigar, healthcare professionals from Dhaka, Bangladesh, took part in the IPOPI PIDetect programme in 2022. During their participation, they received four weeks of clinical and laboratory diagnostic training provided by Prof. Surjit Singh at the Chandigarh Post Graduate Institute of Medical Education and Research, (PGIMER) India.

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 2022, IPOPI organised a webinar dedicated to Wiskott-Aldrich Syndrome (WAS), presented by Prof. Siobhan Burns (UK) and Dr. Adli Ali (Malaysia). The successful webinar provided a two-part global approach to WAS and had 163 registered viewers from 41 countries.

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

IPOPI CLINICAL CARE WEBINAR

WISKOTT-ALDRICH SYNDROME (WAS)

OCTOBER 4TH 2022 — 14:00WEST - 15:00 CET

IPOPI.ORG

Prof Siobhan Burns

Dr Adli Ali

NEWBORN SCREENING

SCREEN4RARE

Aside from implementing SCID NBS national programmes in collaboration with its members, 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 start off 2022, the Screen4Rare partners unveiled a brand-new project website . This was just the beginning of a range of activities that took place throughout the year.

In July 2022, Martine Pergent (IPOPI President) spoke at an event organised by the Czech Republic Presidency of the EU on the topic of “**Early diagnosis of patients with rare disorders in the EU: Crucial role of newborn screenings**”, in Brno, Czech Republic. Martine Pergent, as IPOPI and Screen4Rare representative, took part in a panel discussion on “current experience and future developments in newborn screening”.

screen4rare

2nd Event Of The MEP Alliance For Rare Diseases NBS

THE PIVOTAL ROLE OF NEWBORN SCREENING IN FUTURE EU RARE DISEASES POLICY

The event is co-hosted by:
MEP Stelios Kympouropoulos & MEP Deirdre Clune

30th of November | 16.00 – 18.00
EU Parliament Brussels, Room ASF385

The **second meeting of Screen4Rare and the MEP Alliance on Newborn Screening** took place at the European Parliament in November 2022. The meeting was chaired by MEPs Stelios Kympouropoulos and Deirdre Clune and focused on the importance of including newborn screening in future rare disease legislation. The meeting highlighted the benefits of early diagnosis and treatment for conditions like severe combined immunodeficiencies (SCID). Representatives from EU countries and European Reference Networks shared their experiences and efforts in this area.

INTERNATIONAL NEONATAL SCREENING DAY
JUNE 28

THE SECOND INTERNATIONAL NEONATAL SCREENING DAY

The global IPOPI community celebrated the 2nd International Neonatal Screening Day (INSD) on June 28. Led by Screen4Rare and co-founded by IPOPI, INSD raises awareness about the life-changing benefits of neonatal screening, enabling early detection of rare diseases and ensuring timely treatment and care. The campaign featured various activities, including an official event both at the European Parliament and online, centred around: ‘*Establishing Best Practices – how can newborn screening be supported in the European Union?*’. This event was hosted in collaboration with the Member of the European Parliament (MEP) Stelios Kympouropoulos and the MEP Alliance for Rare Disease Newborn Screening (NBS).

INSD 2022 facilitated valuable exchanges and collaborations with policymakers, delivering transformative benefits for children with rare disorders.

International Neonatal Screening Day 2022

Establishing Best Practices - How can newborn screening be supported in the European Union?

Hosted by MEP Stelios Kympouropoulos (EPP, Greece)

INTERNATIONAL NEONATAL SCREENING DAY
JUNE 28

29th June
15:00-17:00 CEST

Hybrid Event – Brussels

register here!

NEWBORN SCREENING PUBLICATION IN THE LANCET

In addition to the above, IPOPI, along with its Screen4Rare partners, continued its collaboration with the ERN Expert Platform on Newborn Screening and particularly with ERN-RITA, and MetabERN. The collaborative effort resulted in the publication of a significant paper titled “**Newborn screening as a fully integrated system to stimulate equity in neonatal screening in Europe**” in The Lancet journal. The paper outlines ten key elements for effective NBS programs in Europe.

EFFORTS TO SECURE IMMUNOGLOBULIN SUPPLY

IPOPI firmly believes that it is crucial to continue working towards increasing plasma collection and, in turn, boost the supply of immunoglobulin. 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 2022, IPOPI took the lead in several collaborative initiatives to advocate for global sufficiency in plasma-derived medicinal products (PDMPs) by promoting a more balanced distribution of plasma collection across different regions.

ICSPP

LAUNCH OF THE INTERNATIONAL COALITION FOR SAFE PLASMA PROTEINS (ICSPP)

The International Coalition for Safe Plasma Proteins (ICSPP) was launched in October 2022, to advance global access to safe plasma proteins. The founding members of ICSPP include IPOPI, the International Society for Blood Transfusion, World Federation of Hemophilia, International Federation of Blood Donor Organisations, the International Plasma and Fractionation Association and the Plasma Protein Therapeutics Association.

This coalition aims to support the World Health Organization's blood programme to facilitate in-country projects on the stepwise enhancement of the quality, safety and volume of domestic plasma; local small-scale purification, concentration and virus-inactivation of plasma protein fractions (e.g., clotting factors and immunoglobulins); and progress toward industrial plasma fractionation.

IPOPI COLLABORATES WITH THE UNITED NATIONS INSTITUTE FOR TRAINING & RESEARCH

In 2022, IPOPI started a first collaboration with the United Nations Institute for Training and Research (UNITAR) around a project entitled "Strengthening Healthcare Systems to meet patients' need for plasma and plasma-derived therapies" focusing on Argentina and Malaysia in the first instance. As such, Mr Bruce Lim, IPOPI Vice-Chair, and Ms Roberta Anido de Pena, IPOPI Board Member, are representing the patient's voice in their respective national pilot programmes aimed at establishing a multistakeholder platform that will work towards strengthening the resilience of health systems to meet the global patient need for plasma and plasma-derived therapies.

REVISED LEGISLATION UNDERWAY IN EUROPE

The revision of the EU legislation on Substances of Human Origin (which includes the collection of blood and plasma) started in July 2022 when the European Commission shared their proposal. The Commission's text provides a good basis to improve the availability of blood and plasma for the development of therapies: with the obligation to ensure crisis preparedness and monitor supply to address shortages and dependencies on other Member States or third countries; the inclusion of definitions that will clarify important concepts and the recognition of the importance of guaranteeing the availability of substances of human origin to ensure optimal patient access and the sufficiency of supply.

The proposal is now being discussed by the European Parliament and IPOPI has been working with Members of the European Parliament to ensure that the future legislation is patient centred. In addition, IPOPI strives to ensure that plasma collection is more effectively organised throughout the EU for the benefit of patients who rely on plasma-derived medicinal products such as immunoglobulins.

PROMOTING COLLABORATION AND AWARENESS: VISITS TO KUALA LUMPUR AND BANGKOK BLOOD CENTRES

IPOPI was delighted to visit the Kuala Lumpur National Blood Centre on November 17, 2022, and the National Blood Centre of the Thai Red Cross Society on November 23, 2022. During these visits, IPOPI got the opportunity to discuss ways of collaborating to increase awareness of the need for plasma and implement necessary policy changes, notably to promote increased plasma apheresis collection. The visit was also an opportunity for patients, and blood and plasma donors to meet.

WORLD BLOOD DONOR DAY

IPOPI continued to demonstrate a strong commitment to the World Health Organization's World Blood Donor Day campaign, held every year on June 14. This global campaign offered an opportunity for the PID community to stress the need to collect more plasma, through both blood and plasma donations, and raise awareness about the need for safe and effective blood and plasma products.

The majority of patients with PIDs rely on plasma-derived medicinal products: immunoglobulins. Engaging various stakeholders to raise awareness of the importance of both blood and plasma collection on World Blood Donor Day was a way for IPOPI and its members to create meaningful change at a global level.

IPOPI and the PID patients community are thankful to both blood and plasma donors for their gift of life.

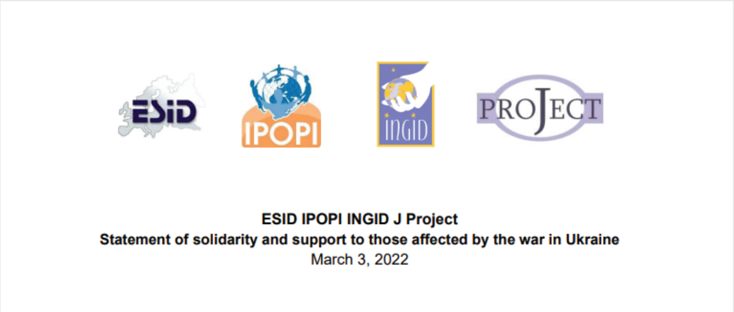
INTERNATIONAL PLASMA AWARENESS WEEK

Every October, the primary immunodeficiency (PID) community joins International Plasma Awareness Week (IPAW) to highlight the importance of plasma collection. IPAW aims to raise global awareness, recognise plasma donors' contributions, and promote understanding of plasma protein therapies and rare diseases. IPOPI actively participated in the campaign in 2022, advocating for increased and regionally balanced plasma collection. Each year, IPOPI NMOs are encouraged to join IPAW, empowering them to raise awareness and advocate for plasma collection in their countries.

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 AND THE PID COMMUNITY UNITE IN SUPPORT OF ALL PEOPLE AFFECTED BY THE CONFLICT IN UKRAINE



ESID IPOPI INGID J Project
Statement of solidarity and support to those affected by the war in Ukraine
March 3, 2022

IPOPI has closely collaborated with its National Member Organisations and stakeholders to support individuals with PIDs affected by the conflict in Ukraine. The crisis management plan focused on securing treatment access, coordinating support, and providing solidarity. IPOPI commends the dedication of its members and urges NMOs to reach out for assistance if needs increase.

IPOPI REGIONAL PID EVENTS

XVII GLOBAL PATIENTS' MEETING (GPM)

In October 2022, IPOPI welcomed 100 delegates from 34 countries to the XVII Global Patients' Meeting (GPM) in Gothenburg, Sweden. This is a key biennial event organised in conjunction with the ESID and INGID congresses. The GPM provided participants with critical updates on primary immunodeficiency (PID), including topics like targeted therapies, clinical research, trials, and newborn screening. Exciting additions this year were sessions on the future of PIDs and a speed dating event showcasing 18 projects from IPOPI members. Moreover, a session held by IPOPI's Medical Advisory Panel (MAP) fostered insightful discussions and addressed complex questions from participants.

Attendees were also invited to attend the **PID Awards Dinner** where people whose actions have contributed positively to the PID community were acknowledged. The Luciano Vassalli award is given to a young person who has made a difference for the PID community. The 2022 recipient was Zheng Jian Lei (Jerry Zheng), for his outstanding work in the PID Care China organisation. The LeBien award, on the other hand, honours an adult who has improved the conditions for PID patients internationally. This year's award was given to Professor Surjit Singh from India, for his remarkable work and dedication to PID patients.

For the very first time, IPOPI also announced the winner of the BIG Award, a new community award recognising Bold, Innovative, and Game-changing (BIG) projects by NMOs. The winner of the first BIG Award was the Sudanese Organisation for Patients with Primary Immunodeficiencies (SOPPI).

IPOPI'S 4TH ASIAN REGIONAL PID PATIENTS MEETING IN KUALA LUMPUR

IPOPI was thrilled to return in person to Kuala Lumpur and host the 4th Regional Asian Doctors & Patients PID Clinical Care Meeting on November 19-20, 2022. The meeting brought together patient representatives and healthcare professionals from 11 countries. It featured an inspiring programme that provided attendees with the chance to get updated on the latest advancements in primary immunodeficiencies in Asia. Renowned regional and international PID experts delivered presentations. This was a successful meeting, focusing on the overall journey of a PID patient from diagnosis to treatment, highlighting the importance of multistakeholder partnerships to further improve the life of PID patients in the Asian region.

IPOPI NATIONAL PID EVENTS

IPOPI NATIONAL THAI PRIMARY IMMUNODEFICIENCY MEETING

On November 22, IPOPI held a doctors' meeting in Thailand with 200 participants at Chulalongkorn Memorial Hospital, in Bangkok and online. Co-organised with Care-for-Rare and the Faculty of Medicine at Chulalongkorn University, the meeting covered topics such as the JAK-STAT pathway, gene sequencing in PIDs, COVID-19's impact on PID patients, and a newly identified immunodeficiency syndrome. On November 23, IPOPI also hosted a patients' meeting with our member organisation Thai Patient Organisation for Primary Immunodeficiencies (ThaiPOPI), to address infusion services and vaccination access for PID patients. IPOPI is grateful to our collaborating organisations including Care-for-Rare Foundation, Chulalongkorn University, and ThaiPOPI.

WORLD PI WEEK 2022 – LIVES CAN BE SAVED

IPOPI supports the yearly World Primary Immunodeficiencies Week (WPIW). The main theme of this year's campaign was Lives Can Be Saved, with four sub-themes guiding the activities:1) Screening and diagnosis; 2) Treatment and quality care; 3) Health awareness. 4) Research. Through the social media campaign #MyPhysicianAndI, IPOPI highlighted the important relationship between physicians and patients for rare chronic diseases like PIDs. In addition, and for the first time in the history of the campaign, the global community got an exciting opportunity to meet in person during IPOPI's IPIC congress which coincided with the campaign week.

Moreover, we were proud to be able to support campaigns from 28 different National Member Organisations (NMOs) through IPOPI's World PI Week grant programme. During WPIW 2022, IPOPI's grant programme provided invaluable support to national member organisations (NMOs) in organising social media campaigns, informative webinars, educational videos and lectures, virtual runs, books and immune deficiency bracelets distribution, petition writing campaigns directed at policymakers, and so much more. The collective efforts of IPOPI and its NMOs resulted in a comprehensive and multifaceted approach to raising awareness and advocating for PIDs worldwide.

IPOPI NMO SUPPORT PROGRAMME 2022

Throughout 2022, IPOPI was pleased to be able to fund several NMO projects and campaigns through its NMO Support Programme among which:

- Bolivia - V International meeting of primary immunodeficiency
- Malaysia – Translation to Bahasa Malaysia and revival of IPOPI´s publication "Living with Primary Immunodeficiency"
- Spain - National campaign for newborn screening for SCID

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.

Q-ID, THE QUIZ APP FOR THE PID COMMUNITY

In February 2022, IPOPI launched Q-ID, a new quiz app that offers a fun and easy way to test and and boost people's knowledge of primary immunodefiencies. Q-ID aims to raise awareness and educate players on key aspects of PIDs, ranging from diagnosis, treatment, and management to research advances and much more.

Q-ID.IPOPI.ORG

SIX NEW IPOPI PID INFORMATION LEAFLETS IN 2022

Since 2011, IPOPI has been producing primary immunodeficiencies (PID) information leaflets. In 2022 two leaflet series were published, covering the following topics:

- Essentials of PID Diagnosis
- Rheumatological, Autoimmune & Autoinflammatory Crossovers
- Targeted Therapies for PIDs
- X-linked Agammaglobulinaemia
- Chronic Granulomatous Disease (CGD)
- Wiskott-Aldrich Syndrome (WAS)

Essentials of PID Diagnosis explains how clinical signs and symptoms, family history, tests and genetic analysis can be used in the diagnosis of primary immunodeficiencies.

Rheumatological, Autoimmune & Autoinflammatory Crossovers explores the crossovers between PIDs and rheumatological, autoimmune and autoinflammatory diseases and how these diseases are managed in patients with PIDs.

Targeted therapies for PIDs covers how targeted therapy, with biological agents or small molecule drugs, might be used to treat PIDs.

The **XLA, CGD and WAS leaflets** cover the characteristics of each disease, inheritance patterns, steps to diagnosis, treatment, and management. This is the 2nd edition of these leaflets, which have been reworked to ensure they are up to date with the current understanding of these diseases and the medical advances over the past 10 years.

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.

PRIMARY IMMUNODEFICIENCIES
ESSENTIALS
OF PID DIAGNOSIS

PRIMARY IMMUNODEFICIENCIES
RHEUMATOLOGICAL, AUTOIMMUNE
& AUTOINFLAMMATORY
CROSSOVERS

PRIMARY IMMUNODEFICIENCIES
TARGETED
THERAPIES FOR PIDs

PRIMARY IMMUNODEFICIENCIES
CHRONIC
GRANULOMATOUS DISEASE

PRIMARY IMMUNODEFICIENCIES
WISKOTT-ALDRICH
SYNDROME

PRIMARY IMMUNODEFICIENCIES
X-LINKED
AGAMMAGLOBULINAEMIA

PRIMARY IMMUNODEFICIENCIES

WHAT IS THE RISK OF PASSING THE DISEASE ON TO THE CHILDREN OF PEOPLE WITH WAS?

Female carriers of an X-linked disorder, such as WAS, have a 25% chance with each pregnancy of having a carrier daughter like themselves, a 25% chance of having a non-carrier daughter, a 25% chance of having a son affected with the disease and a 25% chance of having an unaffected son (**Figure 1**).

If a male with an X-linked disorder has children, he will pass the defective gene to all of his daughters, who will be carriers. His sons will not be affected as an affected male cannot pass an X-linked gene to his sons because males always pass their Y chromosome to male offspring (**Figure 2**). As mutations are germline, they can be transmitted to the patient's offspring even after the patient has been cured. Hence, the need for a genetic counselling for all affected individuals and their families.

FIGURE 2. Inheritance pattern of X-linked WAS with affected father

NEW VIDEO SERIES ON PRIMARY IMMUNODEFICIENCIES

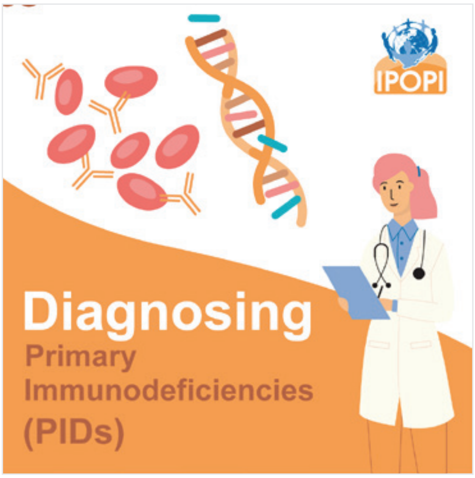
IPOPI released a new educational video series on primary immunodeficiencies in October 2022. By creating these videos, IPOPI aims to increase understanding of these conditions and stimulate conversations about the importance of early detection and treatment accessibility.

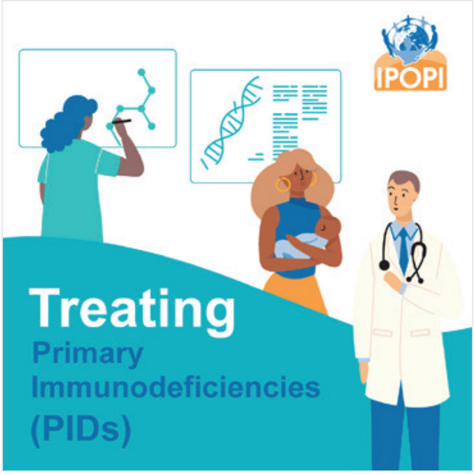
The videos cover three fundamental aspects of PIDs:

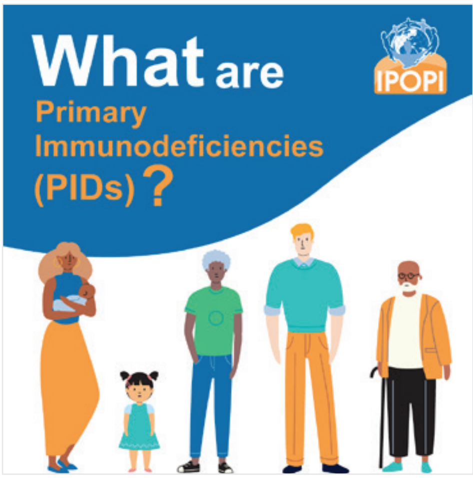
- What are Primary Immunodeficiencies?
- Diagnosing Primary Immunodeficiencies
- Treating Primary Immunodeficiencies

The educational video series is now accessible to a wider global audience. Subtitles in 10 languages: English, Arabic, Chinese, Danish, Dutch, French, Italian, Portuguese, Romanian and Spanish, have been made available through the collaboration with our national member organisations.

The videos are available on **TV.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. 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

WORLD ANTIMICROBIAL AWARENESS WEEK AND EUROPEAN ANTIBIOTIC AWARENESS DAY 2022

IPOPI participated in World Antimicrobial Awareness Week (WAAW) and European Antibiotic Awareness Day (EAAD) to address the impact of antimicrobial resistance (AMR) on patients with primary immunodeficiency (PID). As an EAAD campaign partner, IPOPI contributed to raising awareness and offered insights from the PID perspective. Antibiotic prophylaxis plays a crucial role in managing PID, and IPOPI supports efforts to combat antimicrobial resistance and preserve the effectiveness of antibiotics for PID patients.

STRATEGIC OBJECTIVE 4 – STRENGTHEN MULTI-STAKEHOLDER COOPERATION

IPOPI values the benefits of working together with partners and stakeholders and 2022 was a shining example of new partnerships being built.

IPOPI JOINS TRANSFORM

IPOPI is happy to announce that we are now part of TRANSFORM, the European Alliance for Transformative Therapies, a multi-stakeholder Alliance that connects Members of the European Parliament (MEPs) and other 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'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 June 2022, IPOPI attended the annual Recomb meeting in Leiden, The Netherlands. This meeting provided an opportunity for all Recomb partners, including IPOPI, to share progress and discuss future steps.

A significant highlight of this meeting was the announcement by Professor Frank Staal and Professor Arjan Lankester, the Recomb coordinators from Leiden University Medical Center (LUMC), about the successful treatment of the first patient using this new therapy. The patient, diagnosed with RAG1-SCID shortly after birth, underwent treatment in August 2021 at LUMC and has shown positive signs of T and B cell reconstitution. Other centres involved in the trial celebrated this news, as they await regulatory approval to enroll their patients.

IPOPI, leading the dissemination and exploitation work package, presented their recent efforts during the meeting. This included highlighting their work in publications like Health Europa Quarterly and promoting Recomb at the IPIC congress in April 2022.

www.recomb.eu

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

IPOPI JOINS THE AGORA INITIATIVE

IPOPI participated in the launch meeting of the AGORA consortium in September 2022, in London (UK). Moreover, IPOPI has been invited to be a partner to the founding members group of AGORA (Access to Gene therapies fOr Rare diseases). AGORA aims to facilitate access to effective gene therapies for the treatment of patients with ultra-rare diseases, in Europe. In the short-term, AGORA will seek to explore the hurdles that currently exist and identify areas of potential change to enable sustainable access to treatments, better highlighting the scientific value, competence and high expertise of the consortium which will provide new ideas and solutions.

IPOPI is committed to continuing its collaboration with the AGORA consortium and all relevant stakeholders in the field of gene therapies.

rita
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 map the future of healthcare in Europe. As a member of the EU Health Coalition, IPOPI participated and co-funded the 3rd EU Health Summit: Towards an EU Health Union in Brussels. The summit focused on building a successful EU Health Union by addressing unmet health needs and advancing prevention, diagnosis, and care. Recommendations were launched, highlighting digital transformation, health system integration, research and innovation, and access to innovation for improved patient outcomes. IPOPI actively contributed to these discussions, advocating for the goals of the EU Health Union.

THE INTERNATIONAL PLASMA PROTEIN CONGRESS

The International Plasma Protein Congress was held in Berlin, Germany, focusing on the need to increase plasma collection in Europe for better patient access to plasma-derived medicinal products (PDMPs). Martine Pergent, IPOPI President, spoke about EU approaches to boost plasma collection. The event also discussed the value of PDMPs and how the EU Pharmaceutical Strategy can improve access. Leire Solis, IPOPI Health Policy and Advocacy Senior Manager, provided the patient perspective on EU legislation revision. Professor Volker Wahn received the Hilhenhaus Award for his contributions to raising awareness about primary immunodeficiencies.

IPFA/PEI INTERNATIONAL WORKSHOP ON SURVEILLANCE AND SCREENING OF BLOOD-BORNE PATHOGENS

In September, IPOPI had the opportunity to attend the 28th International Workshop on Surveillance and Screening of Blood Borne Pathogens, organised by the International Plasma and Fractionation Association (IPFA) and Paul Ehrlich Institute (PEI) in Porto, Portugal. The workshop brought together international experts with an interest in and responsibility for maintaining a safe and secure supply of transfusion products for patient care in all regions of the world. The meeting was attended by 158 delegates from 30 countries.

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.

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. Leire Solis, Senior Health Policy and Advocacy Manager supports Jose Drabwell in this role. In 2022, Jose Drabwell was appointed one of the two patient representatives to participate at the Emergency Task Force (ETF). This was a newly created task force that will convene in preparation for and during public health emergencies.

RELATED MEETINGS

IPOPI attended and participated in the following events among others:

- EU Health Coalition steering committee meetings, throughout the year
- LFB Site visit Lille (France)
- ERN-RITA Board Meetings, Patients Committee, Communications WP, Education WP, throughout the year
- ICSPP meetings, throughout the year
- HFE Thematic Network Webinars, throughout the year
- World PI Week steering committee meetings, throughout the year
- TRANSFORM Alliance Meetings, throughout the year
- 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
- Granulomatous Lymphocytic Interstitial Lung Disease (e-GLILD) Steering Committee Meetings, throughout the year
- Introducing the Innovative Health Initiative: Europe's new partnership for health, Virtual launch meeting, January 2022
- IPOPI 2nd WHIM workshop, 27 January 2022
- ESID-IPOPI-INGID site visit and prep meetings, Gothenburg (Sweden), 21-22 February, 2022
- European Patients' Forum webinar on the evaluation of the EU's Blood, Tissues and Cells Directives – what is at stake for the patient community?", 9 February 2022
- 2022 Global Rare Disease Day Event "Rare Diseases: A Global Priority for Equity", organised by NGO Committee for Rare Diseases, Ågrenska, Rare Diseases International and EURORDIS, 28 February 2022
- The symposium "Breaking the Bubble", 2 March 2022
- Virtual European Medicines Agency PCWP/HCPWP joint meeting, 2-3 March 2022
- EU PAD Consortium Meeting, 10 March 2022
- IPFA/EBA Symposium on Plasma Collection and Supply, Amsterdam, 15-16 March 2022
- Politico Live's event "The shake-up of orphan and paediatric rules", 17 March 2022
- PLUS Stakeholders Meeting, Estoril, 22-23 March 2022
- Chan-Zuckerberg Research Consortium application proposal coordination call "Profiling paediatric immunodeficiencies one cell at a time", 30 March 2022
- Multistakeholder workshop on EMA's extended mandate, 1 April 2022
- EU PAD Consortium meeting, 8 April 2022
- International primary immunodeficiencies congress (IPIC) 5th edition, 27-29 April 2022
- EBA webinar: Blood is Priceless: the future of EU Blood Directives, perspectives from the European Establishments, May 2022
- ERN-RITA Strategic Meeting, Cascais, Portugal, 6-7 May 2022
- Maximise EHDS impact on better health data sharing - European Health
- Coalition Hybrid Workshop on the new European Health Data Space Proposal, 12 May 2022, virtual & in-person
- World Health Assembly (WHA) side event, "Saving the lives of Mothers and Babies – what more should we do?", 18 May 2022, virtual
- VITA Training seminar: "ATMPs revolution: empowering leaders of EU civic and patients' advocacy groups", 19 May 2022, virtual
- Advanced Therapies Congress, London, 24-25 May 2022
- Blood and plasma campaign for World Blood Donor Day, 14 June 2022
- IPPC, 14-15 June 2022, Berlin, Germany
- RECOMB annual consortium meeting 2022, 14-15 June 2022

- PLUS Annual General Meeting, 20 June 2022
- TREAT-JAK STAT research consortium, 20 June 2022
- European Commission Conference on “The Regulation on Health Technology Assessment – What’s next”, 22 June 2022, virtual
- European Patients’ Forum Congress 2022, 23-24 June 2022, virtual
- TREAT JAK-STAT consortium meeting 2022, 27 June 2022
- European Medicines Agency Information session on ATMPs with PCWP and HCPWP, 28 June 2022, Virtual
- Transform MEP Interest Group: The Regulatory Framework for ATMPs, 16 June, 2022
- European Patient Forum Congress: Digitalisation in Health, 22-24 June, 2022
- Rare Disease International: Formal Side-Event to the High-Level Political Forum, 6 July, 2022
- IPOPI - Shwachman-Diamond Syndrome Alliance online meeting, 27 July 2022
- ImmUnity Canada - Primary Immunodeficiency Society Conference, 10 September, 2022
- IPOPI’s 3rd WHIM Workshop, 12 September 2022
- Health First Europe, “Strengthening the resilience and skills of the future health workforce”, 22 September 2022
- AGORA initiative meeting, London, 22 September 2022
- RECOMB research project, EU project review meeting, 27 September, 2022
- 2nd webinar of the AMATA webinar series – AMATA in conversation with key opinion leaders on the shaping of AMA, 28 September 2022, virtual
- IPFA/PEI 28th International Workshop on Surveillance and Screening of Blood-borne Pathogens, 21-22 september, 2022
- Event Championing the Paediatric Regulation in the future revision of the EU pharmaceutical legislation, 29 September 2022, virtual
- ERN-RITA Webinar: Two years of SCID newborn screening in Germany – What have we learned?, October 2022
- NBS Meeting Brno, 2022
- Interview for RARE Magazine, 2022
- Online expert briefing session on ‘How to make Europe more attractive for gene and cell therapy development?’, 24 October 2022, virtual
- Czech EU Presidency High Level Meeting ‘Towards a New European Policy Framework: Building the future together for rare diseases: Session on early diagnosis and newborn screening’, 25 October 2022
- Health First Europe Engaging with patients and closing knowledge gaps to fight AMR, 27 October 2022, virtual
- TREAT JAK-STAT consortium meeting, 27 October 2022
- Roundtable EHA, EBA & patients on plasma donations & Ig use, 8 November 2022, virtual
- Together4RareDiseases EU Round Table Meeting, Brussels, 10 November 2022
- European Antibiotic Awareness Day launch event, 17 November 2022, virtual
- Event of the Spanish Society for Primary Immunodeficiencies (AEDIP) on the Consensus document on plasma, 17 November 2022, virtual
- IPOPI-Malaysian National Blood Services Meeting, Kuala Lumpur, 18 November 2022
- IPOPI-Thai Red Cross Meeting, Bangkok, 22 November 2022
- “Newborn screening in Inherited Metabolic Disorders: multi-stakeholder meeting moving towards equity and a common approach Workshop “, 28 November 2022, virtual
- European Medicines Agency, Single Point Of Contact (SPOC) meeting, 18 October 2022, Amsterdam
- ERN-RITA Webinar: Tattoos and Immune mediated diseases, November 2022
- INTEGRATE-ATMP workshop, 24 November 2022
- ITINERARE Symposium, 1 December 2022, University of Zurich
- JAKi treatment consensus first meeting, 5 December 2022
- AGORA Initiative webinar, 6 December 2022

- EU4HEALTH 2023 Work Programme - Information session Operating Grants Webinar ,13 December 2022
- ISNS Annual General Assembly, 15 December 2022
- ERN-RITA Webinar: Cytokine Storms, December 2022

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 a mini documentary on primary and secondary immunodeficiencies, new features in our 4ID health agenda app, Regional meetings in Africa and Latin America, 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 gathering the community at IPIC2023, to be held in Rotterdam, the Netherlands, on November 8-10, 2023.

IPIC2023

INTERNATIONAL
PRIMARY
IMMUNODEFICIENCIES
CONGRESS

DIAGNOSIS
AND CLINICAL CARE

NOVEMBER 8-10, 2023

ROTTERDAM
THE NETHERLANDS

www.ipic2023.com

an **IPOPI** event

REVIEW
OF ACTIVITIES
AND ACHIEVEMENTS

FINANCIAL REPORT

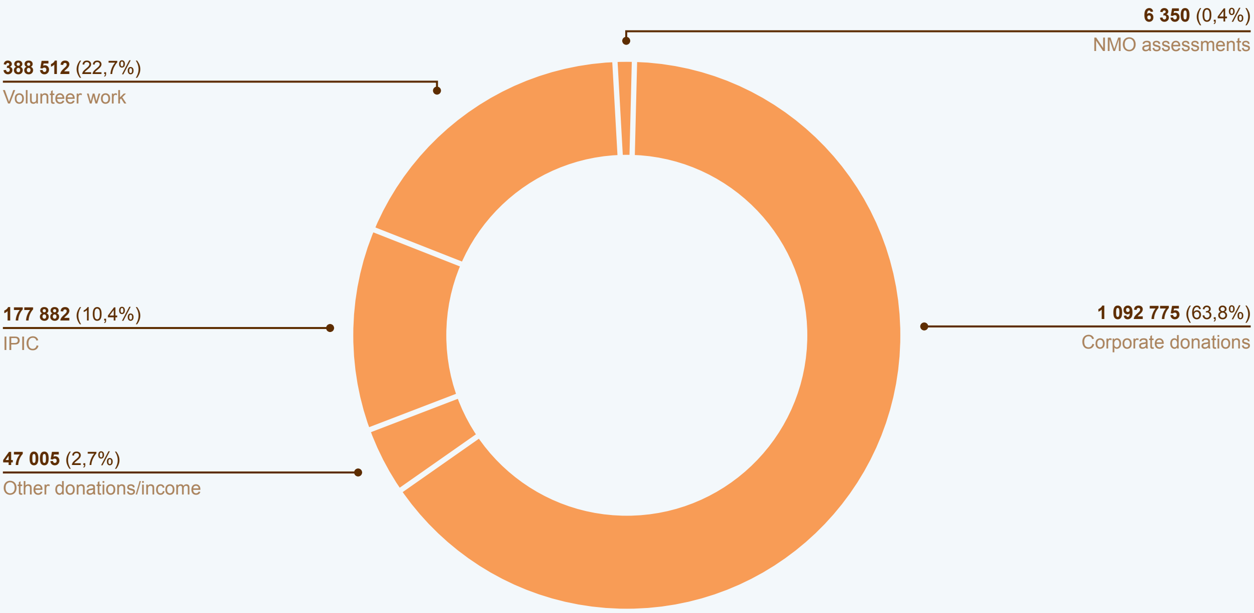
2022

FINANCIAL REPORT 2022

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.

Most transactions during 2022 were carried out by IPOPI aisbl and the intention is to wind up the UK Charity in 2023. Following two years greatly impacted by the COVID-19 pandemic, 2022 was a shift year both in terms of activities and financial aspects due to the gradual return to in-person activities for IPOPI as described above. The organisation of the 5th edition of the International Primary Immunodeficiencies Congress also translated into a good income year for IPOPI. Following two years of surpluses due mainly to the reduced expenditure from the transition of in-person to virtual programmes, we are reporting a small deficit of £46,000 which is mostly due to catching up with postponed projects due to the pandemic.

INCOME 2022 (EURO)



INCOME 2022 (EURO)

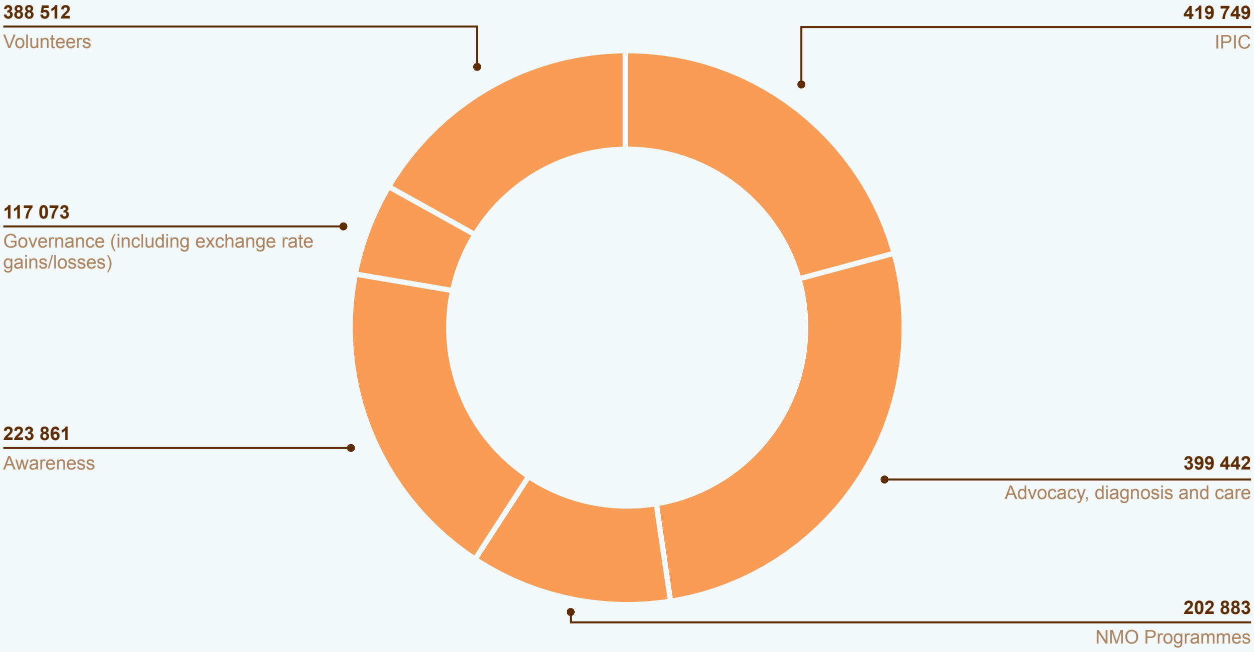
NMO assessments	6 350
Corporate donations	1 092 775
Other donations/income	47 005
IPIC	177 882
Volunteer work	388 512
TOTAL	**1 712 524**

INCOME 2022 (%)

NMO assessments	0,4%
Corporate donations	63,8%
Other donations/income	2,7%
IPIC	10,4%
Volunteer work	22,7%
TOTAL	**100,00%**

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 provided charts provide a summary of key aspects of our financial accounts in 2022. 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 displayed charts. This value has been independently reviewed by our UK chartered accountants.

RESOURCES EXPENDED 2022 (EURO)



EXPENDITURE 2022* (EURO)

IPIC	419 749
Advocacy Diagnosis and Care	399 442
NMO programmes	202 883
Awareness	223 861
Governance (including exchange rate gains/losses)	117 073
Volunteers	388 512
Towards reserve	-
TOTAL EXPENDITURE	**1 751 520**

SOURCES OF FUNDING 2022 (%)

Membership fees	0,4%
Corporate donations	63,8%
Other	2,7%
IPIC	10,4%
Volunteers	22,7%
TOTAL	**100,00%**

* GBP-EURO conversion rate = 0.859845

No contribution received from a single company reached the majority of the total income. The highest single contribution by a company represented 17.38% of the total income including volunteer time contributions and 22,48% 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 70 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
GRIFOLS	CSL Behring Biotherapies for Life™	Pfizer
Takeda	Chiesi global rare diseases	Pharming

PROJECT SPONSORS

In 2022, the following corporate sponsors provided unrestricted grants towards various IPOPI ad-hoc projects that enhance our awareness, advocacy, communications, and representational activities.

- Biotest
- Cane Spa
- Chiesi
- CSL Behring
- Cytognos
- Enzyvant
- Epimune
- FDE – So CONNECT
- Grifols
- ImmunoIVD
- Innovative Health Sciences
- Invitae
- Kedrion
- Koru
- NBI
- 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.

SUPPORTERS

- LFB
- Orchard Therapeutics
- Enzyvant

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 2022 including but not limited to:

Access to Gene therapies for Rare diseases (AGORA), African Society for Immunodeficiencies (ASID), Alpha-1 Global, Arab Society for Primary Immunodeficiencies (ARApid), Asia Pacific Economic Cooperation (APEC), Asia Pacific Plasma Leaders Network (APPLN), Asociación Espanola de Pediatría (AEP), 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 Coalition for Safe Plasma Proteins (ICSPP), 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 Inmulogia (SEI), SEAPID (South East Asia Primary Immunodeficiencies Network), Sociedade Portuguesa Alergologia Imunologia Clínica, Sociedade Portuguesa de Pediatria-infecciologia pediatica, Thai Immunology Society (AAIAT), United Nations Institute for Training and Research (UNITAR), Vietnamese Paediatrics Association, World Federation of Haemophilia, World Health Organisation (WHO), World PI Week and its member organisations.

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)

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