

REVIEW OF ACTIVITIES AND ACHIEVEMENTS 2020



IPIC 5TH EDITION

INTERNATIONAL PRIMARY
IMMUNODEFICIENCIES
CONGRESS

APRIL 27-29, 2022
VILAMOURA - PORTUGAL



A MESSAGE FROM IPOPI'S PRESIDENT AND EXECUTIVE DIRECTOR

2020: The footprint of many challenges



MARTINE PERGENT
President



JOHAN PRÉVOT
Executive Director

There is no doubt about it, SARS-CoV-2 has put its footprint on 2020! Once the perplexity passed IPOPI reacted and carried out our projects, turning challenges into opportunities.

One of our great challenges was to accompany our national members and their patients in this "science in the making" period, bringing answers to the many questions that were brought up by our community that was in an immense fear of being contaminated while immunodeficient. This could not be without an international move of all PID leading medical societies. Globally they cooperated with our Medical Advisory Panel to provide a regular updated joint statement on the epidemics of COVID-19. In addition to this, we dedicated many of our educational,

and even our advocacy tools, to address the evolving topics that the circumstances provided. We really did adapt our working methods and innovated to offer our audiences the most professional experiences when taking part into our activities. This applies to our Global Patient meeting which was held successfully online back-to-back with our partners ESID¹ and INGID², allowing the best attendance ever. This applies as well to our Patients and Doctors Asian PID meeting, held virtually in Japan, along with our regular webchats held with our national members where we addressed their many questions with the support of experts.

The immunoglobulin's global supply is a constant key issue. It was aggravated by the pandemic due to the drop in plasma collection as well as by an unbalanced collection of this resource across the world. IPOPI had several actions in place to call on regional or national health authorities, directly or through the frame of PLUS³, to ensure appropriate supply, especially for patients who have no therapeutic alternative.

Another topic on which IPOPI has a steady commitment is newborn screening (NBS) for rare diseases. It can be a life-saving tool for conditions such as SCID⁴: a condition where treatment does exist and can offer the possibility of a cure. IPOPI together with ISNS⁶ and ESID¹ have created Screen4Rare a multi-stakeholder initiative aimed at exchanging knowledge and best practices on NBS for rare diseases. The ultimate objective is to ensure all babies born in the EU and beyond, can have equal access to NBS.

Coming back to our members, for whom WPIW⁵ is a momentous event each year, we have shifted our way of supporting our members, giving priority to digital campaigns. We can be proud of our members' creativity and efficiency in raising awareness and making their points, aligned with their national priorities.

These few words are meant to give you a flavor of this very special year during which we had many more other activities, new ones, as well as the usual and much needed ones. Building year after year a stronger community and constantly making these conditions better known, taking into account (and despite the fact) that they are rare and not always visible. The ensuing pages will recount more about this all, happy reading!

¹ **ESID** – European Society for Immunodeficiencies

² **INGID** – International Nursing Group for Immunodeficiencies

³ **PLUS** – Platform of Plasma Protein Users

⁴ **SCID** – Severe Combined Immunodeficiency

⁵ **WPIW** – World Primary Immunodeficiencies Week

⁶ **ISNS** – International Neonatal Screening Society

Martine Pergent
IPOPI President

Johan Prévot
IPOPI Executive Director

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IPOPI STRATEGIC PLAN

MISSION STATEMENT

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

IPOPI is the leading advocate for primary immunodeficiency patients worldwide. We work in collaboration with patients, doctors, politicians, regulators, pharmaceutical industry and other relevant stakeholders.

STRATEGIC OBJECTIVES 2016 - 2020

IPOPI's various projects and programmes are geared towards the 4 following strategic objectives:

- 1 -To promote early diagnosis & ensure optimal access to care
- 2 -To develop, strengthen and support National Member Organisations
- 3 -To raise PID awareness globally
- 4 -To stimulate stakeholder collaboration



WHAT ARE PIDS?

Primary immunodeficiencies (PIDs) are a large and growing group of over 430 different disorders caused when some components of the immune system (mainly cells and proteins) 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.

PIDs are now considered to be human inborn errors of immunity caused by genetic defects of the immune system which are hereditary. The immune system normally helps the body fight off infections by germs (or 'micro-organisms') such as bacteria, viruses, fungi and protozoa. Because their immune systems do not work properly, people with PIDs are more prone than others to infections. 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, 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 will be identified. Several PID diagnostic tests have been included in the World Health Organization (WHO) as Essential Diagnostics List (EDL) in a dedicated PID section recognizing them as essential health coverage means.

Medicine is advancing very rapidly and novel therapies that target the specific cause of the disease are becoming available. These may have significant advantages for PID patients' and it is important that patients throughout the world 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 and improve treatment outcomes.

TREATMENT

With appropriate access to the different and very effective therapies which can only happen if 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 are specialized in diseases of the immune system. How a patient is treated depends on which PID they have and on many other factors. Treatments for PIDs include: immunoglobulin (IG) therapies, Hematopoietic Stem Cell Transplantation (BMT), gene therapy, Granulocyte-colony stimulating factor (G-CSF), Gamma interferon, PEG adenosine deaminase, prophylactic antibiotics, among others.

In the case of IG therapies it is important to know that different IG products are not interchangeable or mixable. IG can be administered intravenously or subcutaneously. They are considered as Essential Medicines for PIDs by the WHO. 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) only takes 1-2 hours and can often be given at home by patients themselves, parents, or carers. It may therefore be more convenient for some patients. However only small doses can be given by SC infusion, meaning they are given more frequently than IV infusions, usually once a week. Facilitated subcutaneous immunoglobulin (FSCIg), a type of SC infusion containing recombinant human hyaluronidase, allows patients to have SC infusions every 3-4 weeks. IG therapy should be chosen on an individualized basis according to patient needs and preferences.

Tremendous advances have also been made with the development of new gene therapy treatments, a field pioneered by the PID experts community, where further advances are currently being made.

During difficult health access periods, such as **the COVID-19 pandemic** we are living in, it is crucial that national and regional authorities prioritise PID patients' access to IGs in case of any supply tensions or shortages.

CLASSIFICATION

PIDs or Inborn Error of Immunity are currently classified into ten groups: immunodeficiencies affecting cellular and humoral immunity; CID with associated or syndromic features; predominantly antibody deficiencies; diseases of immune dysregulation; congenital defects of phagocyte or function; defects in Intrinsic and Innate immunity; auto-inflammatory disorders; complement deficiencies; bone marrow failure; phenocopies of inborn errors of immunity.

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

For more information about PIDs and how they are treated and diagnosed, and to get advice on how to stay healthy please refer to the IPOPI leaflets page on our website www.ipopi.org/publications/leaflets



IPOPI'S KEY ACHIEVEMENTS AND ACTIVITIES, 2020

STRATEGIC OBJECTIVE 1 – TO PROMOTE EARLY DIAGNOSIS AND ENSURE OPTIMAL ACCESS TO CARE

IPOPI strives to ensure that patients' interest is part of policy decision-making through advocacy at national, regional and international level. Our actions promote the importance of access, choice and supply of treatment for patients.

14th IPOPI EU PID FORUM – COVID-19 AND PANDEMICS MANAGEMENT

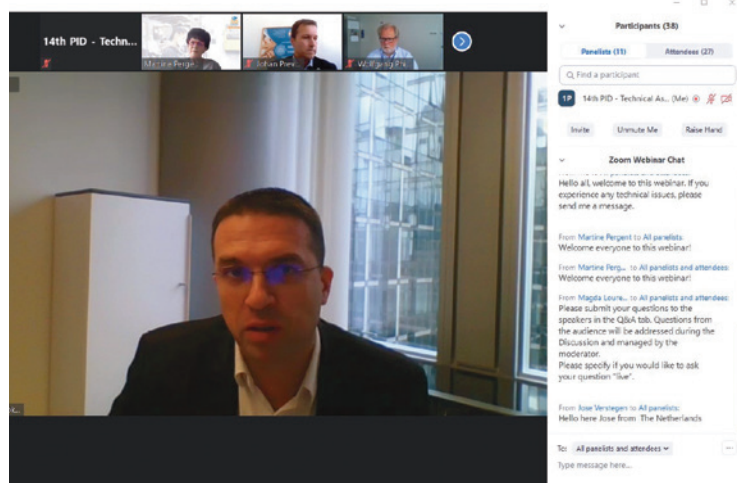
IPOPI organised its 14th EU PID Forum on "COVID-19 and Pandemics Management: protecting the most vulnerable" on September 30, 2020. The Forum was hosted virtually under the patronage of the Member of the European Parliament, Tomislav Sokol (EPP, Croatia) and moderated by Johan Prevot, IPOPI's Executive Director.

Four IPOPI National Member Organisations' (NMOs) representatives took part in the event. Birgit Schlennert (from DSAI, Germany), Isabella Moraswka (from Immunoprotect, Poland), Savvas Savva (Cyprus Primary Association and Friends) and Anneli Larsson (from PIO, Sweden) presented about the challenges their organisations faced in the first months of the COVID-19 pandemic.

IPOPI President, Martine Pergent, provided a short overview of the COVID-19 LEX initiative, a Learning Expedition launched by IPOPI to learn from its NMOs' specific needs. Pressing issues identified by the LEX were information, communication, cooperation, innovation and, importantly, access to care for all.

Dr Nizar Mahlaoui (Necker Enfants Malades University Hospital and Chair of IPOPI Medical Advisory Panel), Ms Nathalie Bere (European Medicines Agency) and Mr Wolfgang Philipp (European Commission) complemented the medical and the regulatory perspectives and lessons learnt from the pandemic.

The very interesting presentations and speeches, coupled with the responses provided to some of the questions raised during the event, would be the ground for the development of a set of recommendations where the different challenges posed by a pandemic will be described and solutions will be suggested to facilitate the approach and management of future pandemics.



15th IPOPI EU PID FORUM - NEWBORN SCREENING TO STRENGTHEN EARLY DIAGNOSIS OF PRIMARY IMMUNODEFICIENCIES: GERMANY AS GOOD-PRACTICE EXAMPLE FOR EUROPE?

The event took place on November 18, 2020, was held online and under the patronage of the German Member of Parliament (MP) Erich Irlstorfer and co-organized by IPOPI and the German patient organisation for congenital immunodeficiencies (dsai e.V.).

This Forum helped make clear that the early detection of immunodeficiencies is crucial for the well-being of patients and relatives. Patient organisations such as IPOPI members as dsai e.V. make an important contribution to the support of those affected. The speakers advocated for the introduction of further screenings based on scientific evidence and the exchange of experiences in the European context.

Germany is one of the first countries in the European Union that has implemented SCID at a nation-wide level. These practices and the lessons learnt from the journey of including SCIDs in the list of conditions that can be screened can certainly help other countries when assessing the inclusion of SCIDs within the panel of diseases newborns are screened for and contribute to construct an EU-wide database.

16th IPOPI EU PID FORUM - VACCINES AND PID

On December 3, 2020, IPOPI held its 16th PID Forum on “PIDs and vaccines: protecting the most vulnerable” hosted by the Member of the European Parliament (MEP) Maria da Graça Carvalho (S&D, Portugal). The Forum, moderated by Martine Pergent, provided a comprehensive overview of vaccines, from the perspective of a medical expert such as Prof Alain Fischer, from Necker-Enfants Malades and member of IPOPI’s Medical Advisory Panel, and from the perspective of a nurse, with Prof Ann Gardulf, member of the board of INGID.

The Forum then looked into the approval of the vaccines with the very interesting presentation of Manuela Mura, scientific officer at the European Medicines Agency (EMA), and the presentation of Maria Syrochkina, representing Vaccines Europe.

Vaccines’ accessibility is a huge issue across Europe. We looked into whether the vaccines authorised by regulatory agencies such as the EMA, were actually available in European Member States and accessible to patients on the basis of data pooled from the IPOPI PID Life Index, with a presentation by Leire Solis, health policy and advocacy senior manager at IPOPI.



IPOPI PID CLINICAL CARE WEBINARS

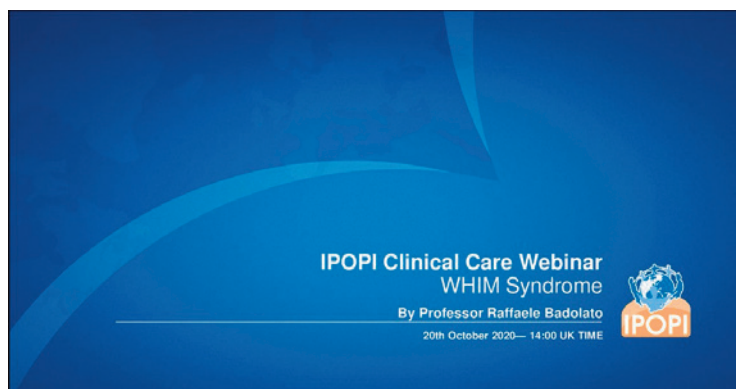
IPOPI Clinical Care Webinars are aimed at clinicians with an interest in PID clinical care management, nurses, patient group leaders and other PID stakeholders from all parts of the world. Increasing the understanding around key clinical care issues in this easy and accessible way has proved to be a popular concept in 2020 and we are excited to continue providing other online educational programmes on relevant issues to the PID community.

In 2020, IPOPI organised the following 7 webinars with the help of several dedicated PID specialists:

- Crossover of PID and Cancer with Professor Isabelle Meyts
- Hyperinflammation in PID and how it differs from COVID-19 with Professor Frédéric Rieux-Laucat
- Calculated Globulins with Professor Stephen Jolles,
- PID and Vaccines with Dr Charlotte Cunningham-Rundles
- WHIM Syndrome with Prof Rafaele Badolato
- PID and SID Crossover with Dr Virgil Dalm
- Genetic Testing with Dr Pamela Lee

IPOPI’s Medical Advisory Panel President, Dr Nizar Mahlaoui took part in all Webinars, making the Q&A part more dynamic.

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



NEWBORN SCREENING & SCREEN4RARE

In addition to its work on the implementation of SCID Newborn Screening (NBS) national programmes in collaboration with its NMOs, IPOPI continued its work on newborn screening at EU level through Screen4Rare. This multi-stakeholder initiative launched by IPOPI, the International Society for Neonatal Screening (ISNS) and the European Society for Immunodeficiencies (ESID) is aimed at exchanging knowledge and best practices on NBS for rare diseases. Its ultimate objective is to ensure all babies born in the EU can have equal access to Neonatal Screening which can be a life-saving tool for conditions such as Severe Combined Immunodeficiency (SCID). A Screen4Rare Stakeholders Network was launched in 2020 on the EU Health Policy Platform which is developed by the European Commission and gives stakeholders the opportunity to share knowledge and information on their domain, in this case, newborn screening for rare diseases. In addition, Screen4Rare started its outreach towards the European Reference Networks with a view to engage them on the topic of newborn screening.



MEDICAL ADVISORY PANEL

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

IPOPI is grateful for all the Panel members' commitment but wishes to specifically express its thanks to Dr Nizar Mahlaoui (Chairman) and to Prof Martin van Hagen (Vice-Chairman) for their kind contribution and dedication.

IMMUNOGLOBULIN SUPPLY ADVOCACY AND AWARENESS EFFORTS

IPOPI strongly believes that efforts to increase plasma collection and therefore Immunoglobulin supply are vitally needed. Policy discussions on these topics should be patient-centred. IPOPI led several efforts made in partnership with stakeholders, advocating for global sufficiency in plasma-derived medicinal products (PDMPs) based on more regionally balanced plasma collection.

SAFE TASK FORCE

With the COVID-19 pandemic entering into scene, IPOPI launched in September 2020 the S.A.F.E. (Supply & Access For Everyone) Task Force. The Task Force aims at monitoring the situation of tensions and/or shortages of plasma and immunoglobulin replacement therapies, as well as suggest recommendations for IPOPI to follow.

The Task Force is composed of patient representatives as well as PID expert physicians from different geographical areas. The idea is to gather as many different perspectives as possible, so as to have a comprehensive view of the challenges in accessing the PID patients therapies in the different continents.

Amongst its activities, the Task Force has been engaging with relevant stakeholders and issued two statements to aid IPOPI NMOs in their advocacy work and opposed calls to reduce IG for PID patients.

Read the statements: ipopi.org/safe-task-force/



PUBLICATION

IPOPI's Executive Director Johan Prevot and Prof Stephen Jolles (University Hospital of Wales) published an article entitled **Global immunoglobulin supply: steaming towards the iceberg?** describing how plasma is sourced for fractionation into plasma-derived medicinal products (PDMPs), such as immunoglobulin (Ig) and provide an overview of the global plasma supply environment.



PLUS STATEMENTS

PLUS - Open letter to European Member States on the need for a continued access to plasma-derived medicinal products and for strengthening the collection of blood and plasma



PLUS - Statement on Access to Plasma Derived Medicinal Products & home care during the COVID-19 pandemic



STRATEGIC OBJECTIVE 2 – TO DEVELOP, STRENGTHEN AND SUPPORT NATIONAL MEMBER ORGANISATIONS

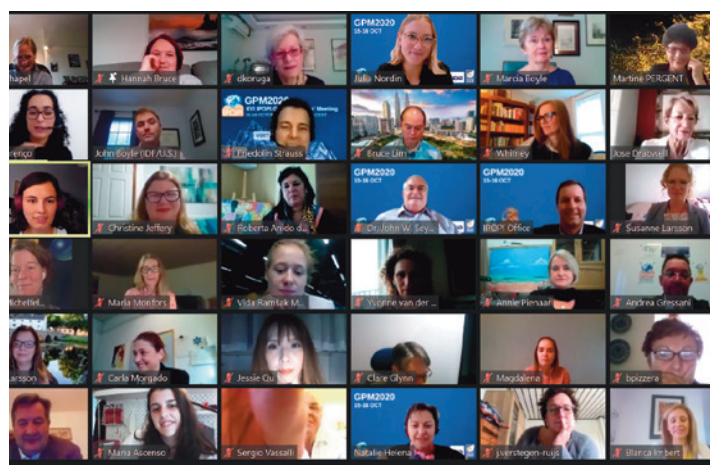
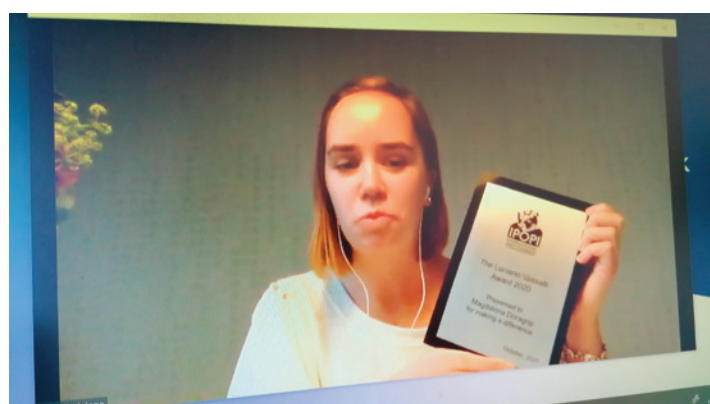
IPOPI assists the establishment of new national member organisations and promotes the development of existing National Member Organisations.

IPOPI GLOBAL PID PATIENTS MEETING

IPOPI had the pleasure of welcoming 200 delegates from 40 countries from all over the world to the XVI Global Patients Meeting (GPM). **The GPM is one of IPOPI's key biennial events** and is organised back to back with the European Society for Immunodeficiencies (ESID) and International Nursing Group for Immunodeficiencies' (INGID) biennial congresses. The meeting was originally taking place in Birmingham, UK, but was moved online due to the COVID-19 pandemic. We were grateful for the opportunity to organise this virtually for the very first time and ensure that the PID patient community could continue to unite and grow stronger together.

During this well attended meeting the participants were updated on the latest development for primary immunodeficiency (PID) by a range of PID experts. The interesting programme offered a wide selection of topics, with sessions such as **Bad Bugs – Managing infections, Living life with PID: from childhood to ageing and PID crossovers**. These medical lectures provided a good platform for discussions, with participants submitting a number of tricky questions for the speakers. The programme also offered 5 parallel interactive skill-building workshops, all connected to the umbrella topic of **How to grow your NMO**.

IPOPI's members were also invited to participate in **the IPOPI PID Champions Awards ceremonies** where two people whose actions have contributed positively to the PID community were acknowledged. The 2020 **Luciano Vassalli award**, given to a young person who has made a difference for the PID community, was presented to **Magdalena Döragrip** (PIO Sweden), for her outstanding work for PID patients in Sweden. **The LeBien award** on the other hand, honors an adult who has improved the conditions for PID patients internationally. This year this award was given to **Professor Helen Chapel** for her many years of tireless work and support for PID patients.



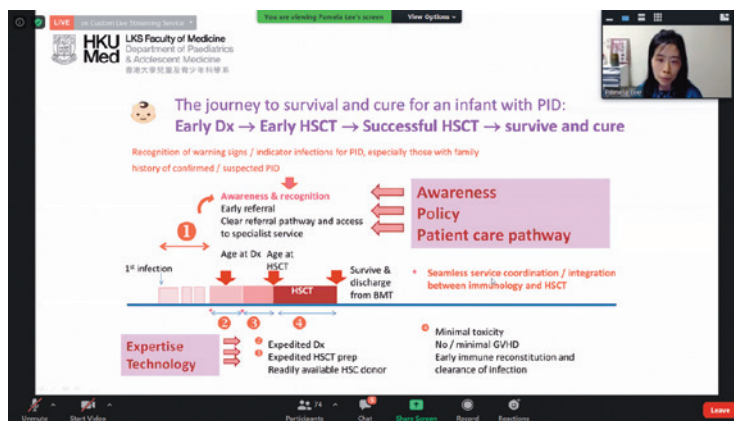
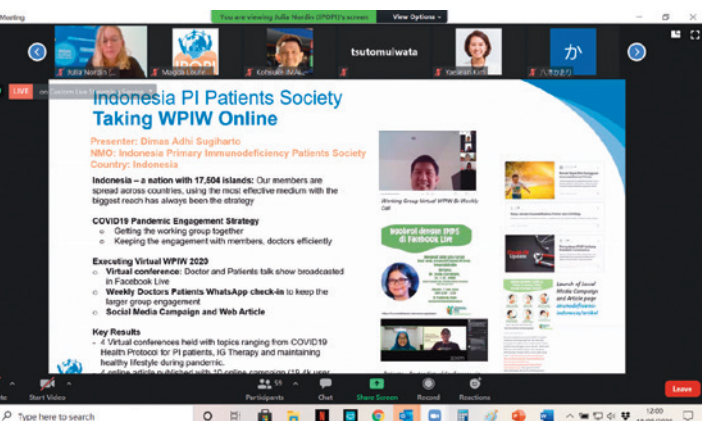
IPOPI PID REGIONAL EVENTS

IPOPI REGIONAL ASIAN PID MEETING

IPOPI welcomed delegates from 19 countries from the Asia-Pacific region to the **IPOPI 3rd Regional Asian PID Meeting**, held virtually due to the COVID19 pandemic on September 18-19, 2020. Participants learned from both international and regional primary immunodeficiencies (PID) experts on the latest developments in Asia.

The meeting featured many key speakers, explored topics such as **PID diagnosis and care priorities in Japan, SEAPID and APSID updates, transition care** and much more.

This meeting was initially scheduled to take place in Kyoto, Japan, in September 2020, back-to-back with the JSA/WAO/APAPARI 2020 Congress.



WORLD PI WEEK 2020 - BRINGING ABOUT CHANGE FOR PID PATIENTS WORLDWIDE

As a response to the COVID-19 pandemic and to ensure safe participation the **World PI Week 2020 campaign was extended from 1 to 10 weeks**. This opened for a great opportunity to really commemorate the 10-year anniversary while at the same time adjusting to the new COVID-19 reality. The campaign had a different shape than in previous years, but core remained the same, shedding important light over the need for early diagnosis and access to appropriate care for primary immunodeficiency patients. This year's campaign naturally increased its focus towards facilitating online activities and encouraging participation from the safety of home. IPOPI was able to facilitate funds to 25 national WPIW campaigns, among which: webinars, videos, online awareness raising activities, medical education conferences, from all around the world.



IPOPI NMO SUPPORT PROGRAMME 2020

2020 was a very different year for all, including IPOPI's NMOs whose projects had to be adapted to a new environment impacted by the COVID-19 pandemic with many awareness-raising efforts moving to an online basis. Still, IPOPI was pleased to be in a position to fund NMO programmes through its NMO Support Programme.

- The Netherlands, membership activities
- China – awareness campaign activities
- Kenya- awareness campaign activities
- Italy – awareness campaign activities
- IPOPI information leaflet translations into:
 - Icelandic
 - French
 - Russian
 - Portuguese

IPOPI also engaged in several awareness campaigns with its NMOs aimed at highlighting the importance of both blood and plasma donors and of access to life saving plasma-derived medicinal products, such as IG therapies.



STRATEGIC OBJECTIVE 3 – TO RAISE PID AWARENESS GLOBALLY

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

IPOPI PID LIFE INDEX

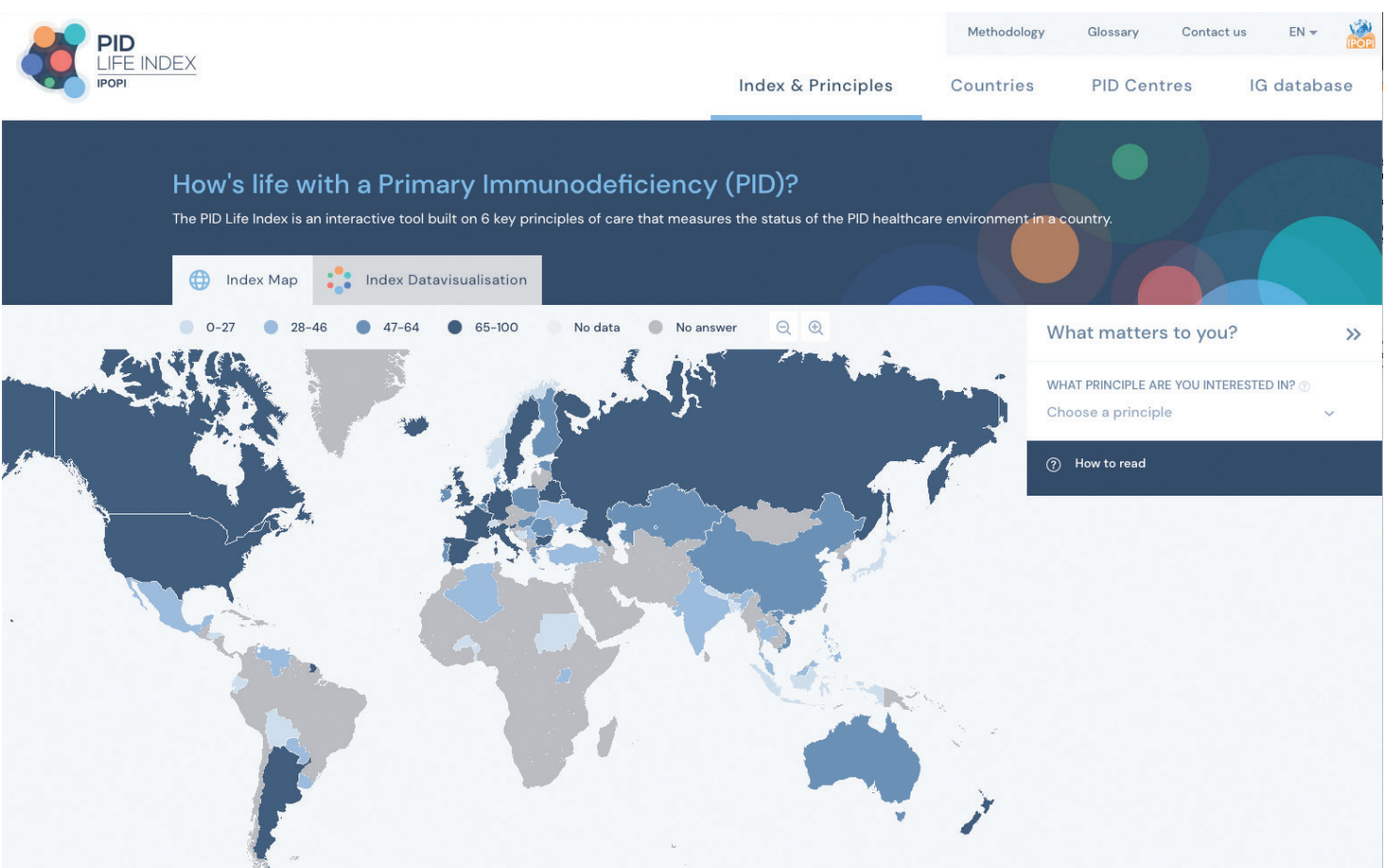
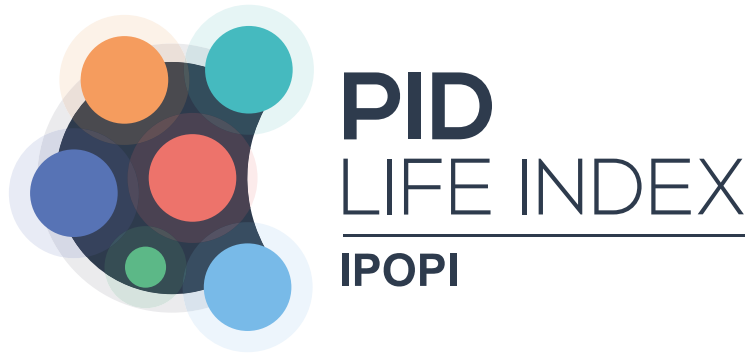
In 2020, IPOPI launched a novel interactive web-based tool to capture the state of the PID environment around the world. The PID Life Index is harboured in a web platform and built on 6 key principles of care that measure the status of the primary immunodeficiency (PID) healthcare environment across the globe, displayed in an interactive tool.

The PID Life Index has been established with the co-operation of PID medical specialists and patient leaders from all world regions. The large set of data, provided by IPOPI member organisations and physicians, is displayed through map and data visualisation interfaces that allow users to navigate the index according to their interest. This is a first-of-its-kind care status measurement index in the rare diseases field.

With it IPOPI aims at showing the status of PIDs at national, regional or global level as well as supporting advocacy initiatives.

The PID Life Index results from a long process of translating the “Principles of Care for Primary Immunodeficiencies”, published in 2014 and establishing the gold standard of care for PID, into the current political and health-care environment.

pidlifeindex.ipopi.org

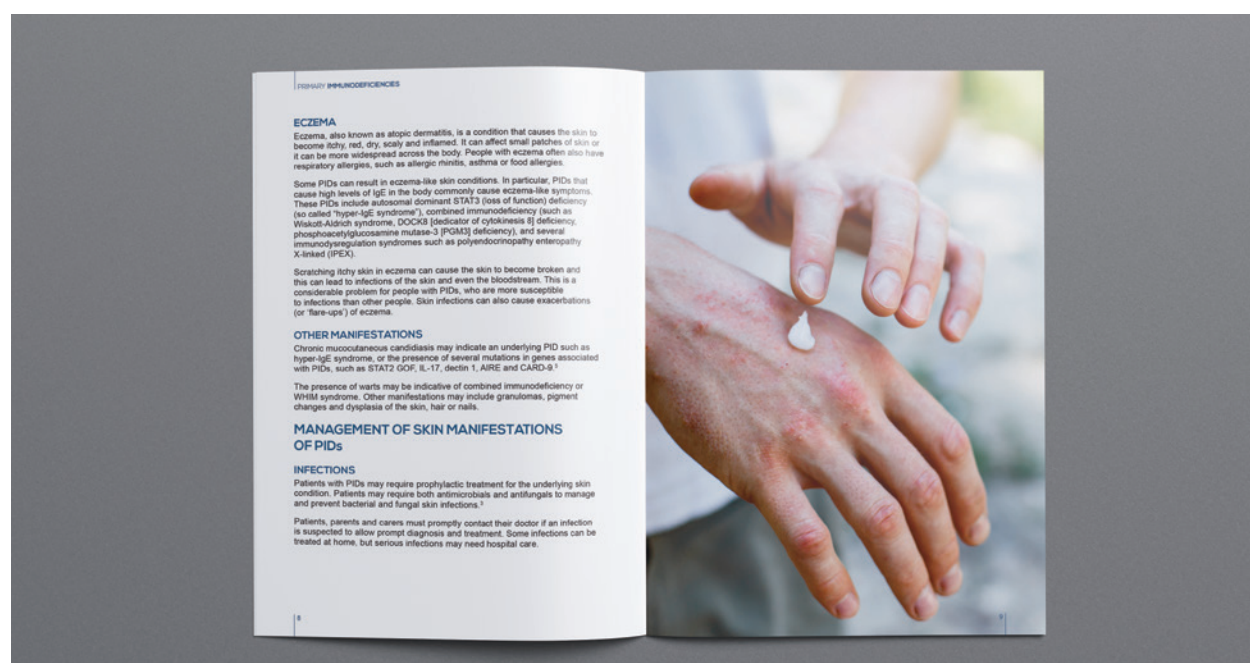


NEW IPOPI PID INFORMATION LEAFLETS

IPOPI has since 2011 been producing primary immunodeficiencies (PID) information and a new series was published last year. In 2020 IPOPI published six leaflets dedicated to **PIDs and Skin**, **PIDs and Cancer**, **Hyper IgE Syndrome**, **Ataxia-Telangiectasia**, **A Guide for Hepatologists** and **Genetic Diagnosis of PIDs**.

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

The leaflets were initially made available in English in PDF and in eBook format, soon will be available in many other languages.



COVID19 – FIGHTING MISINFORMATION AND PROVIDING EVIDENCE-BASED INFORMATION

IPOPI is committed to fighting misinformation and to closely monitor the COVID-19 pandemic, as well as other emerging situations, by sharing evidence-based and expert validated information to the global primary immunodeficiency (PID) patients' community and to its stakeholders.

Our priority is the safety of all PID patients and the safety of supply of their life-saving treatments both during and after the COVID-19 pandemic.

IPOPI has taken the lead on engaging its community stakeholders to fast track the publication of joint statements, answer frequently asked questions (FAQs) and participate in international efforts to collect data on COVID-19 in PIDs.

You can follow the updates at:

<https://ipopi.org/latest-news-on-covid-19-and-pid-1/>

<https://ipopi.org/pids/covid-19-and-pids-faqs/>

IPOPI E-NEWS

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

e-news.ipopi.org

IPOPI News



Latest news on COVID-19 and PID

IPOPI is committed to monitor closely the COVID-19 pandemic and to share evidence-based and expert validated information to the global primary immunodeficiency (PID) patients' community and to our stakeholders

[Read more](#)



World PI week celebrates 10 years for 10 weeks

In light of the COVID 19 pandemic, the World PI Week Steering Committee decided to extend in time the WPIW campaign.

[Read more](#)



STRATEGIC OBJECTIVE 4 – TO STIMULATE STAKEHOLDER COLLABORATION

IPOPI values the benefits of working together with partners and stakeholders and 2019 was a shining example of new partnerships being built.

MORE PID DIAGNOSTIC TESTS INCLUDED IN THE WHO EDL

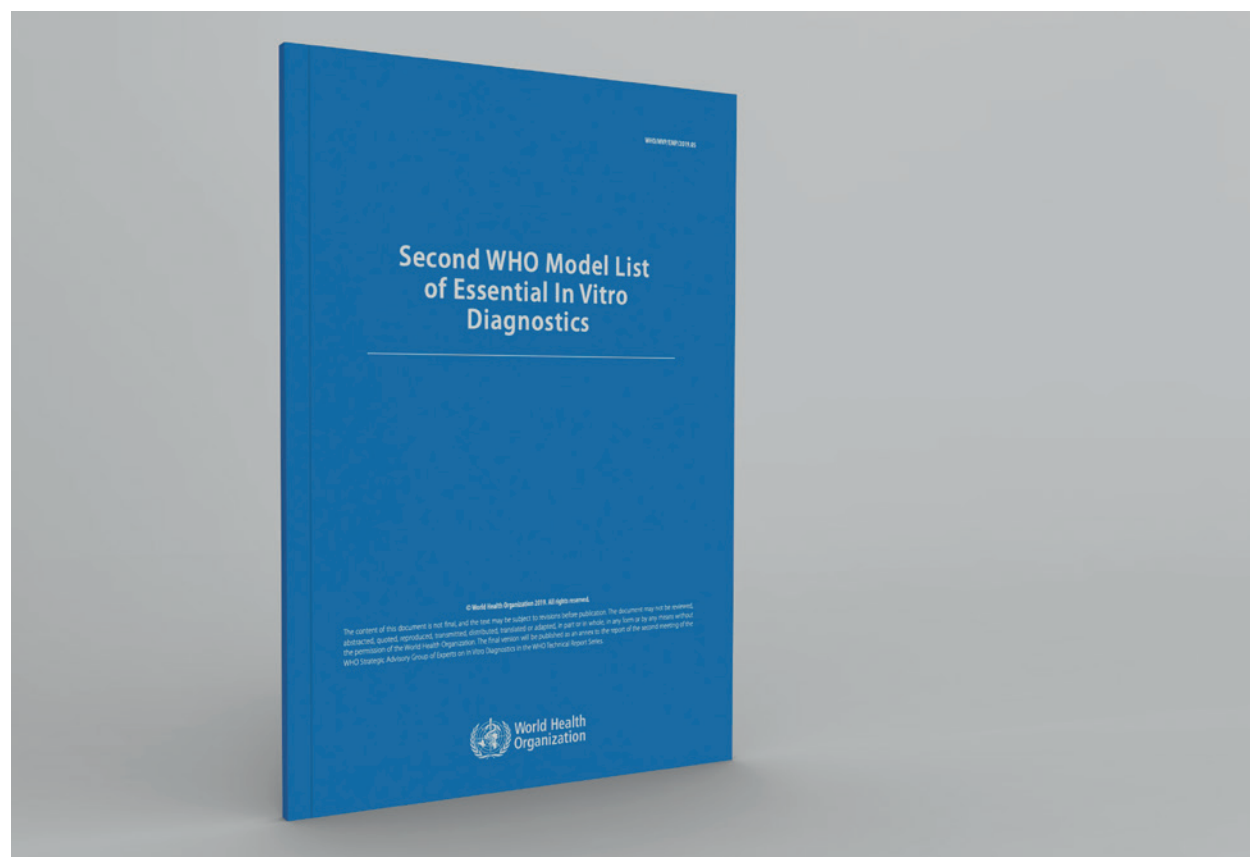
Throughout 2020, IPOPI led efforts in collaboration with its stakeholders towards the inclusion of additional new PID diagnostic tests in the World Health Organization (WHO) third edition of the Model List of Essential In vitro Diagnostics. These efforts were once again successful in demonstrating the importance of stakeholder collaboration. The World Health Organization (WHO) published on January 29, 2021, the third edition of the Model List of Essential In vitro Diagnostics (EDL).

The WHO accepted IPOPI's 2020 proposal to expand the list to include lymphocyte subtype enumeration and also fully accepted the tests provisionally accepted in 2019 for which additional evidence was provided in 2020.

Altogether, the tests now considered as essential for PID diagnosis are:

- Complete blood count;
- HIV ½ antibody (anti-HIV Ab);
- Immunoglobulin plasma levels (IgG, IgA, IgM)
- Lymphocyte subtype enumeration (CD3, CD4, CD8, B cells CD19 and/or CD20, CD16/56 T cells and NK cells)

IPOPI expresses its gratitude to Prof Helen Chapel, Dr Nizar Mahlaoui, and the many medical experts that with their time and guidance made the applications a success. IPOPI also thanks all of the regional and international immunological societies that supported and contributed to the application.



EUROPEAN REFERENCE NETWORK FOR PIDS

IPOPI continued its collaboration with the European Reference Network RITA ensuring that the voice of PID patients is taken into account in the network's activities and decisions.

RITA stands for Rare Immunodeficiency, Autoinflammatory and Autoimmune and unites specialised centres in Europe dedicated to primary immunodeficiencies and other autoinflammatory and autoimmune disorders.

In 2020 ERN RITA started a series of webinars with different topics of medical education of the conditions represented. They are available for replay: ern-rita.org



European Reference Network

RECOMB & SCIDNET PROJECTS

IPOPI has been an active contributor in these two ground-breaking research programmes to bring in the voice of the patients with PID into the research and development process of advanced therapies for severe combined immunodeficiency (SCID).

The SCIDNET research project was concluded in 2020 after 5 active years. SCIDNET, led by Prof Bobby Gaspar (former IPOPI Medical Advisory Panel President), was focused on developing gene therapy for SCID-ADA. It received its funding from the European Commission through the EU Horizon 2020 research budget. IPOPI actively contributed to the programme to advance the development of new treatments for the PID patients' community.

Launched more recently, RECOMB, is another EU Horizon 2020 funded programme, aimed at developing and testing a gene therapy for children affected by a specific type of SCID: Recombinase Activating Genes (RAG) deficient SCID. The project is led by Prof Frank Staal, from Leiden University Medical Center and IPOPI plays a key role as work package leader for dissemination and exploitation.

Despite some delays due to the pandemic the consortium has been able to continue making progress. IPOPI participated actively in the RECOMB annual 2020 meeting. For more information: www.recomb.eu

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

PLUS - PLATFORM OF PLASMA PROTEIN USERS

PLUS CONSENSUS MEETING

The Platform of Plasma Protein Users – PLUS – organised its annual meeting with relevant stakeholders in Estoril (Portugal) on January 23-24. The meeting was **chaired by Dr Jacqueline Kerr**, from the Paul Ehrlich Institute, and counted with the participation of PLUS member organisations, associations representing the pharmaceutical industry and the Marketing Research Bureau.

Participants discussed the revision of the European Blood legislation and took part in an interactive session on potential scenarios of plasma supply issues, including case study examples that foresaw a hit on plasma supply due to new viral threats, an eerie prediction of what was yet to come with the COVID-19 pandemic.



PLUS - EU PARLIAMENT EVENT - FUTURE ACCESS TO PDMPs: PATIENT CENTRED DECISION MAKING

On September 29, 2020, PLUS organised, in collaboration with MEP Sirpa Pietikäinen (EPP, Finland), an event entitled “Future access to PDMPs: Patient centred decision making”. The event, moderated by Dr Jacqueline Kerr, from the Paul Ehrlich-Institute, and Brian O’Mahony, from the Irish Haemophilia Society, called for a patient centred approach in future decision-making around the need for plasma and access to Plasma Derived Medicinal Products (PDMPs).

Johan Prevot, IPOPI’s Executive Director, called upon health authorities and policy makers to include patients, meaningfully, in decision-making, so that, nothing about patients is decided without patients. He added that plasma users want to be listened to when legislation that will have an impact on their daily lives is being discussed, such as, the EU blood and tissues and cells legislation, the implementation reports, specific campaigns on blood and plasma donation or the considerations of the impact of COVID19 on the supply of substances of human origin.

Patients call for:

1. Increased supply and free movement of safe and efficacious PDMPs developed on robust GMPs with the goal to meet patients' growing needs
2. Development of guidelines, policy & legislation should be based on **FACTS & SCIENCE** & experience (not ideology)
3. Safety of patients means global sufficiency based on regionally balanced plasma collection (each region has to do more, incl. the EU)
4. Avoid wastage of plasma
5. Develop or strengthen plasmapheresis programmes when possible, only way to increase plasma collection
6. Encourage the co-existence of public & private plasma collection to face the needed investments and benefit from existent knowledge and experience
7. Future EU legislation on PDMPs should be **patient-centred**.

The EU already relies on compensated US plasma donors in the EU the ones collecting significantly more plasma are Germany, Austria, Hungary and Czech Republic.

Are we ready to equally encourage compensated plasma donations as VU blood and plasma donations?



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 Drabwell in this role.

RELATED MEETINGS

IPOPI attended and participated in the following events among others:

- EFPIA State of Health in the EU Event, Brussels, January 2020
- APSID congress, Chandigarh, January 2020
- EU Health Coalition steering committee meeting, January 2020
- ERN-RITA Board Meetings, Patients Committee, Communications WP, throughout 2020
- ERN RITA transition working group meeting, January, 2020
- IPFA conference, January 2020
- ERN RITA General Assembly, January 2020
- HFE Thematic Network Webinars, throughout 2020
- World PI Week Steering Committee meetings, all year
- World PI Week April 2020
- EDQM conference for Blood Establishments, April 2020
- EFPIA Webinar COVID19, April 2020
- Granulomatous Lymphocytic Interstitial Lung Disease (e-GLILD) Steering Committee Meetings, throughout the year
- RDI Webinar RD global network, May 2020
- European Parliament Meeting "The next Generation's recommendations on EU Health Policy", Brussels, April 2019
- Rare Diseases International Conference, May 2020
- Eurordis AGM, May 2020
- European Rare Disease Conference (online), May 2020
- Blood and plasma campaign for World Blood Donor Day, 14 June 2020
- EC Eurordis COVID 19 RDs webinar, June 2020
- IUIS COVID-19 webinar, August 2020
- European Society for Immunodeficiencies conference (online), October 2020
- PPTA roundtable on "Ensuring Appropriate Patient Access to Plasma Derived Medicinal Products", October 2020
- EuropaBio Patient BioForum, October 2020
- PPTA Global Plasma Summit, October 2020
- EDQM webinar "Keeping up with Reality and Quality: A Challenge for European Blood Establishments", October 2020
- EU Health Summit, October 2020
- ERN-RITA Webinar: Newborn screening for SCID, November 2020
- Politico Health Summit (online), November 2020
- OECD webinar on "Health at a Glance: Europe 2020", November 2020
- RDI - WHO Collaborative Global Network for Rare Diseases (CGN4RD) Meeting, November 2020
- Recomb clinical centres meeting, December 2020
- APEC Virtual Meeting in Chile, December 2020

LOOKING AHEAD – 2021 AND BEYOND

IPOPI looks forward to continuous support of the PIDs community through international awareness, advocacy work and medical education.

Due to the COVID-19 pandemic several of our upcoming projects and meetings had to be readjusted and moved online. IPOPI is actively supporting the PID community during these difficult times including in the form of web chats, FAQs on COVID-19 and PID, several policy statements.

IPOPI and its Board Members keep monitoring this difficult worldwide situation in all regions of the world and adjusting our activities where needed.

IPOPI and its member organisations will continue their awareness and advocacy campaigns across the globe to ensure the PID patients' community is included in important policy-making decisions.

The launch of new projects and NMO programmes including national and regional meetings and workshops will continue to strengthen PID patient organisations around the world.

Don't miss IPIC 5th Edition, to be held in person in Vilamoura, Portugal, on April 27-29, following the postponement of IPIC2021 to 2022 and the move from Berlin, Germany, to Portugal.

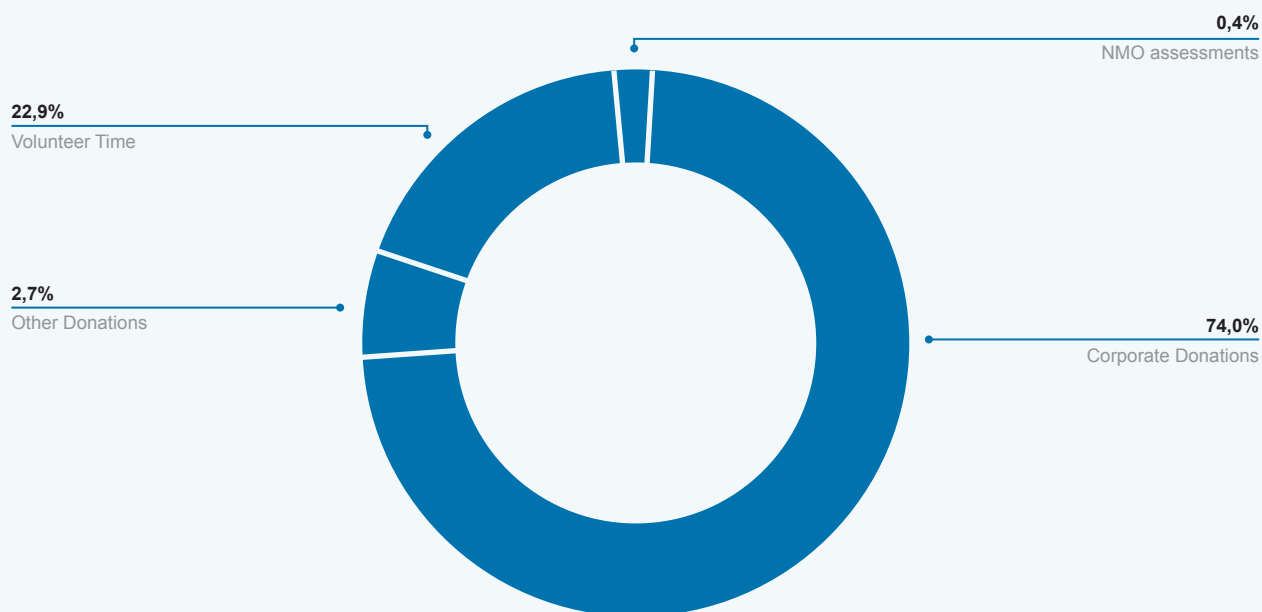


FINANCIAL REPORT 2020

FINANCIAL REPORT 2020

2020 was a good financial year for IPOPI with the highest annual income for a non-congress year. Despite the COVID-19 pandemic, IPOPI was able to successfully carry out its policy and advocacy efforts, patient awareness campaigns, skills-building and educational programmes, all geared towards improving PID care and access to treatments across the globe.

INCOME 2020 (GBP)



INCOME 2020 (GBP)

| | |
|---------------------|---------------------|
| NMO assessments | 5,871.00 |
| Corporate donations | 992,339.00 |
| Other donations | 35,693.00 |
| Volunteer Work | 306,360.00 |
| TOTAL | 1,340,263.00 |

INCOME 2020 (%)

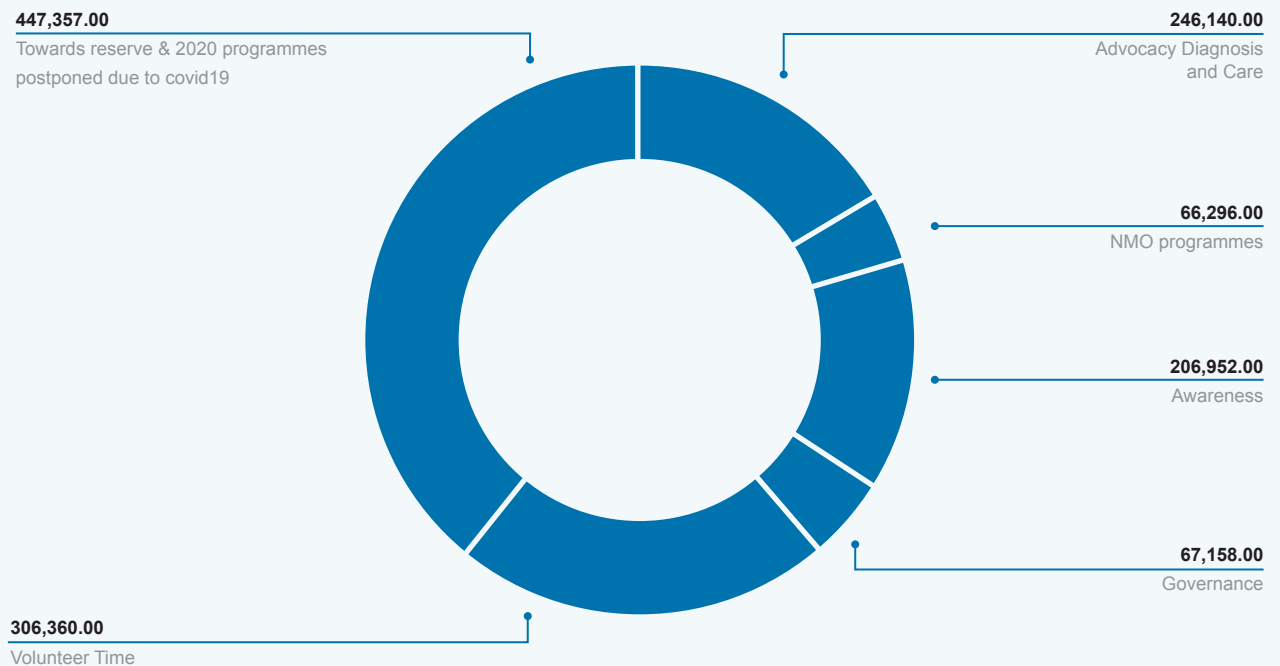
| | |
|---------------------|---------------|
| NMO assessments | 0,4% |
| Corporate donations | 74,0% |
| Other donations | 2,7% |
| Volunteer Time | 22,9% |
| TOTAL | 100.0% |

IPOPI is a non-profit charity registered in the United Kingdom and under UK Charity Law. We are required to have our accounts audited every year by qualified accountants and submitted to the Charity Commission for approval. As per UK accounting rules, IPOPI reports all income received in 2020 which includes 2021 income received in advance during 2020.

The following charts provide a summary of key aspects of our financial accounts in 2020. Our full audited accounts are available on the Charity Commission website and on request.

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

RESOURCES EXPENDED 2020 (GBP)



EXPENDITURE 2020 (GBP)

| | |
|------------------------------------------------------------|---------------------|
| Advocacy Diagnosis and Care | 246,140.00 |
| NMO programmes | 66,296.00 |
| Awareness | 206,952.00 |
| Governance | 67,158.00 |
| Volunteers | 306,360.00 |
| Towards reserve & postponed 2020 programmes due to COVID19 | 447,357.00 |
| TOTAL EXPENDITURE | 1,340,263.00 |

SOURCES OF FUNDING 2020 (%)

| | |
|---------------------|----------------|
| Membership fees | 0,4% |
| Corporate donations | 74% |
| Other | 2,7% |
| Volunteers | 22,9% |
| TOTAL | 100,00% |

No contribution received from a single company reached the majority of total income. The highest single contribution by a company represented 26% of the total income.

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 a primary immunodeficiency worldwide.

We also express our gratitude to our 67 National Member Organisations whose important activities and efforts in their respective countries are vital to improve 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.

PLATINUM



GOLD



SILVER



SUPPORTERS



PROJECT SPONSORS

In 2020, the following corporate sponsors provided unrestricted grants towards various IPOPI ad-hoc projects that enhance our awareness, advocacy, communications and representational activities.

- BioMarin
- Biotest
- Bluebird Bio
- CSL Behring
- Epimmune
- Grifols
- Horizon Pharma
- ImmunoIVD
- Kedrion
- LFB
- Novartis NIBR/Pharming
- Octapharma
- Orchard
- Perkin Elmer
- Pfizer
- SOBI
- Takeda
- X4 Pharma
- PPTA

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 2020 including but not limited to:

Alpha-1 Global, African Society for Immunodeficiencies (ASID), Arab Society for Primary Immunodeficiencies (ARApid), Asociación Española de Pediatría (AEP), Asia Pacific Economic Cooperation (APEC) Blood Policy Stakeholders Forum, Asia Pacific Society for Immunodeficiency (APSID), Australasian Society of Clinical Immunology and Allergy (ASCIA), Belgian Primary Immune Deficiency Group (BPIDG), British Society for Immunology (BSI), Care-for-Rare Foundation, Chulalongkorn University Hospital Bangkok, Clinical Immunology Society (CIS), Cipto Mangunkusumo Hospital Jakarta, Council of Europe, European 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 ERN-RITA, 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), GBS/CIPD Foundation International, Grupo Português de Imunodeficiências Primárias (GPIP), HAE International, Hanoi National Hospital of Paediatrics, Health First Europe (HFE), Hungarian Paediatrics Society, Hungarian Society for Allergology and Immunology, Indian Academy of Pediatrics, Indian Society for Primary Immunodeficiency, International Alliance for Patient Organizations (IAPO), International Nursing Group for Immunodeficiencies (INGID), International Immunocompromised Host Society (ICHS), International Plasma Fractionation Association (IPFA), International Society for Blood Transfusion (ISBT), International Society for Neonatal Screening (ISNS), International Union of Immunological Societies (IUIS), Latin American Society for Immunodeficiencies (LASID), Le Centre de Référence Déficits Immunitaires Héritaires (CEREDIH), METABERN, MSAI (Malaysian Society for Allergy and Immunology), Plasma Protein Therapeutics Association (PPTA), Platform of Plasma Protein Users (PLUS) and its member organisations, RECOMB H2020 consortium, SCIDNET H2020 consortium, Sociedad Española de Inmología (SEI), SEAPID (South East Asia Primary Immunodeficiencies Network), Sociedade Portuguesa Alergologia Imunologia Clínica, Sociedade Portuguesa de Pediatria-infecciologia pediática, Thai Immunology Society (AAIAT), Vietnamese Paediatrics Association, World Federation of Haemophilia, World Health Organisation (WHO), World PI Week and its member organisations.

IPOPI is an international charity registered in the UK (No. 1058005)

IPOPI.ORG



INTERNATIONAL PATIENT ORGANISATION FOR PRIMARY IMMUNODEFICIENCIES
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