



Primary Immunodeficiencies (PI) – Global Call to Action - Driving Diagnosis for Optimal Care -

We invite you to **endorse and sign** the following **Primary Immunodeficiencies (PI) Call to Action**, which offers guidance for governments on the steps they can take to understand, appropriately diagnose and manage **Primary Immunodeficiency Diseases in their respective countries**.

The **PI Community** calls upon countries across the world to take heed of these devastating and under-diagnosed diseases by developing national strategies that address PI. We encourage governments to consider the following components when developing future disease strategies and rare disease plans:

Raising Awareness on Primary Immunodeficiencies

- Encourage and support governments to implement targeted campaigns to increase recognition of PI amongst the medical profession, parents, schools, day care centre employees, researchers and nurses, with the objective to increase understanding of the disease, knowledge of the '10 Warning Signs' and available testing methods and treatments.

Education of Health Professionals and Expertise Exchange

- Work together with medical specialists organisations (e.g. ESID, ASID, LASID, CIS, EFIS, INGRID, UMES, etc) and academia to encourage and support individual countries to:
 - provide standards for basic and applied immunology training, with emphasis on PI, in the education programmes for general practitioners and nurses;
 - integrate basic and applied immunology teaching, particularly alongside immunisation, into specialised programmes for training fellows in general paediatric internal medicine, rheumatology, respiratory medicine, and infectious diseases;
 - develop cross country initiatives to allow the exchange of expert experience and education, including capacity to network and funding of visits of immunology centres representatives in other countries.

Early Diagnosis and Screening

- Support the development of clinical protocols to reliably identify all forms of PI;
- Support the development of simple diagnostic tests for PI and promote such tests for use widely;
- Support the development a neonatal screening test for severe combined immunodeficiency
- Promote the creation of International guidance for the recognition of symptomatic patients and ensure appropriate immunologic and genetic laboratory tests are available at national level via centres of excellence;
- Promote the creation and support of patient registries that can provide support for patients and also scientific insights into the make-up of PID.
- Promote transnational research into the feasibility of screening programmes to ensure early detection.
- Promote funding research into better understanding of PI to improve diagnosis and the management of the conditions and general understanding of the immune system

Gathering Knowledge and Data

- Provide funding to conduct epidemiological studies to assess the prevalence and incidence of PI
- Encourage the creation of and input into international registries which will enable future diagnostic processes
- Create national PI patient registry;
- Encourage transnational, research and remove the administrative obstacles to multinational clinical trials;
- Establish Centres of Reference to promote best practice in terms of disease classification, treatment outcome measures, assessment of cost of treatments for an integrated approach to PI recognition and treatment.

Comprehensive and Adequate Treatment

- Ensure access to treatment for people with PI as it has been shown to prevent and improve chronic disease, improve social well-being and reduce the burden of the disease;



- Ensure that safe immunoglobulin treatments are available to all patients who require antibody replacement; and ensure the adequate and high quality supply of human plasma;
- Recognise the social needs of PI patients and their families and facilitate access to adequate support services;
- Ensure the reimbursement of orphan drugs according to their medical efficacy for the patient and not according to the price of the product.

SIGNATORIES – GLOBAL STAKEHOLDERS

Name	Organisation
Vicki and Fred MODELL	Jeffrey Modell Foundation (JMF)
Jose DRABWELL	International Patient Organisation for Primary Immunodeficiencies (IPOPI)
David WATTERS	International Patient Organisation for Primary Immunodeficiencies (IPOPI)
Prof. Jean-Laurent CASANOVA	European Society for Immunodeficiencies (ESID)
Catherine SAUTES-FRIDMAN	European Federation of Immunological Societies (EFIS)
Gaby STROTMANN	International Nurses Group for Immunodeficiencies (INGID)
Frédéric DESTREBECQ	European Union of Medical Specialists (UEMS)

SIGNATORIES - MEMBERS OF THE EUROPEAN PARLIAMENT

Member of the European Parliament	Country
Milan CABRNOCH	Czech Republic
Jorgo CHATZIMARKAKIS	Germany
Nessa CHILDERS	Ireland
Chris DAVIES	United Kingdom
Mairead McGUINNESS	Ireland
Miroslav MIKOLASIK	Slovakia
Alojz PETERLE	Slovenia
Inese VAIDERE	Latvia
Glenis WILLMOTT	United Kingdom
Tatjana ZDANOKA	Latvia

SIGNATORIES

Name	Organisation
Jo AULD	Kings College London, United Kingdom
Ségolène AYME	Orphanet France
Prof. Isil B BARLAN	Division of Pediatric Allergy/Immunology Marmara University Tophanelioglu, Istanbul, Turkey
Simona BELLAGAMBI	UNIAMO FIMR, Federazione Italiana Malattie Rare, Italy
Svetlana BENIS	"Tej"
Vinciane BERCKMANS	H.A.E. Belgium
Elżbieta BERDEJ – SZCZOT	GCZD Katowice, Poland
Prof. Ewa BERNATOWSKA	Children's Memorial Health Institute, Warsaw, Poland
Robert BIERNAT	Children's Memorial Health Institute, Warsaw, Poland
Prof. Dr. Michael BORTE	Municipal Hospital St. Georg Leipzig, Academic Teaching Hospital of the University of Leipzig, Germany



Dragica BOSNJAK	DELO Scientia
Sonja BRACKE	MSS (Marshall-Smith Syndrome) Research Foundation
Anette BRUNNER	Swiss Ichthyosis Association
Kate BUSHBY	Newcastle University, United Kingdom
Name	Organisation
Dr. Helen CHAPEL	Oxford Radcliffe Hospital, John Radcliffe Site Oxford, United Kingdom
Katarzyna CHROBAK – GORNA	SZOZ Poznań, Poland
Wojciech CICHY	Matio Foundation
Szczepan COFTA	Poznan University of Medical Sciences, Poland
Iwona CZERWINSSKA-KARTOWICZ	Institute of Immunology and Experimental Therapy, Wrocław, Poland
Ewa ĆWIEK	Children's Memorial Health Institute, Warsaw, Poland
Bożena DEBOWSKA-BOGINSKA	Children's Memorial Health Institute, Warsaw, Poland
Sotiri DESPINA	Hellenic Center for Disease Control & Prevention (HCDCP), Greece
Izabela DŁUGOSZ	EP Office of MEP Elżbieta Katarzyna ŁUKACIJEWSKA
Natalia DOBRZYBSKA	Children's Memorial Health Institute, Warsaw, Poland
Katarzyna DRABKA	Medical University, Lublin, Poland
Wojciech FELESZKO	Medical University of Warsaw, Poland
Marta FIKUS-KRYNSKA	Copernicus Science Centre, Poland
Prof. Alain FISCHER	Hopital Necker - Enfants Malades, France
Selena FREISENS	Genzyme, Germany
Iwona GAJ	Children's Memorial Health Institute, Warsaw, Poland
Pascal GAREL	European Hospital and Healthcare Federation (HOPE)
Anna GAWORCZYK	Children's University Hospital, Poland
Tomasz GRODZICKI	Medical Faculty, Jagiellonian University, Poland
Elżbieta GRZESLI	Collegium Medicum, Bydgoszcz, Poland
Prof. Lennart HAMMARSTRÖM	Karolinska Institut, Stockholm, Sweden
Edyta HEROPOLITANSKA-PLISKA	Children's Memorial Health Institute, Warsaw, Poland
Prof. Dr. Ilja M. HOPELMAN	University Medical Center, Utrecht, Netherlands
Ulrike HOLZER	Ektodermale Dysplasie
Ninela IRGA	Children's Haematology Clinic, Gdańsk, Poland
Grzegorz JASTRZEBSKI	EP Office of MEP Elżbieta Katarzyna ŁUKACIJEWSKA
Dominika KAMINIARCZYK	Klinika Endokrynologii i Diabetologii Wieku Rozwojowego Uniwersytetu Medycznego, Poland
Dr. Mary KEOGAN	Beaumont Hospital, Dublin, Ireland
Maja KLAUDEL- DRESZLER	Children's Memorial Health Institute, Warsaw, Poland
Jakub KMIOTEK	Children's Memorial Health Institute, Warsaw, Poland
Sylvia KOLTAN	SMUMK Bydgoszcz, Poland
Joanna KOWALSKA	Warsaw University, Poland
Michal KUBINA	Paediatric Clinic Bratislava, Slovakia
Bożena KUSMIREK	Children's Memorial Health Institute, Warsaw, Poland
Joanna KUSZYK	Children's Memorial Health Institute, Warsaw, Poland
Barbara KWIATKOWSKA	Polish Health Ministry, Mother and Child Department
Herk Willen LAAN	MSS (Marshall-Smith Syndrome) Research Foundation
Aleksandra LEWANDOWICZ – USZYNSKA	3th Department and Clinic of Pediatrics, Immunology and Rheumatology of Developmental Age, Poland
Marko LIMBEK	EP Office of MEP Alojz Peterle
Dr. Jacobien MAARSCHALK - ELLERBROEK	University Medical Center, Utrecht, the Netherlands
Prof. László MARÓDI	Medical and Health Science Center, University of Debrecen Debrecen, Hungary



Dorota MARYNOWICZ	Association "IMMUNOPROTECT", Poland
Kinga MIKOLAJCZYK	International Federation of Medical Students' Associations (IFMSA), Poland
Joanna MITKOWSKA	SZOZ Poznań, Poland
Radosław MOTKOWSKI	Medical University Białystok, Poland
Name	Organisation
Prof. Tim NIEHUES	Academic Hospital, Heinrich Heine University of Düsseldorf, Immunodeficiency and Pediatric Rheumatology Centre, Krefeld, Germany
Jerzy NOWAK	Foundation "Together in Time"
Yvonne OHX	SAS Stichting voor Afweerstoornissen
Małgorzata PAC	Children's Memorial Health Institute, Warsaw, Poland
Martine PERGENT	IPOPI/IRIS, France
Prof. Hans-Hartmuut PETER	University Hospital of Chronic Immunodeficiency Freiburg, Germany
Barbara PIATOSA	Children's Memorial Health Institute, Warsaw, Poland
Justyna PIETRASIK	Children's Memorial Health Institute, Warsaw, Poland
Barbara PIETRUCHA	Children's Memorial Health Institute, Warsaw, Poland
Bianca PIZZERA	IPOPI/AIP, Italy
Michaela PLONQUET-STRAKOVÁ	EP Assistant to MEP Milan Cabrnoc
Johan PREVOT	Plasma Protein Therapeutics Association (PPTA)
Tomasz PYZALKA	Matio Foundation
Dr. Isabella QUINTI	Sapienza University of Rome, Italy
PD Dr.med. Janine REICHENBACH	University Children's Hospital, Zürich, Switzerland
Alexey SANDOV	Association Genetig
B. SCHAUP	ADCIA Netherlands
Prof. Dr. med. Reinhold E. SCHMIDT	Clinic for Immunology and Rheumatology at Hanover Medical School, Germany
Dr. Ines SCHÖNDORF	Find -Id Network , Germany
Prof. Dr. med. Reinhard SEGER	University Children's Hospital, Zürich, Switzerland
Malgorzata SIKORA	Children's Heart Foundation
Urszula SKARZYNSKA	Children's Memorial Health Institute, Warsaw, Poland
Michał SKOCZYŁAS	Physician, Poland
Magdalena SKORNIIEWSKA	Nutricia Polska
Marta SOBCZYNSKA	Pediatric Clinic, Bielański Hospital CMKP Warsaw, Poland
Krzysztof SUCHNICKI	Dolnośląskie Centrum Transplantacji Komórkowych z Krajowym Bankiem Dawców Szpiku (DCTK), Wrocław, Poland
Jolanta SYKUT-CEGIELSKA	Polish Metabolic Group of Polish Paediatric Society, Poland
Michał SZCZEPAŃSKI	Children's Memorial Health Institute, Warsaw, Poland
Domenica TARUSCIO	National Center Rare Diseases ISS – Rome, Italy
Dr. Adrian THRASHER	Great Ormond Street Children's Hospital, Institute of Child Health, London, United Kingdom
Vladimir TOMOV	NAPRD Bulgaria
Dr. Martin van HAGEN	Erasmus University, Rotterdam, the Netherlands
Dr. Joris van MONTFRANS	University Medical Centre Utrecht, The Netherlands
Valérie van MUYLEN	BOPPI, Belgian Organisation for Patients with Immuno Deficiencies



Christian van MUYLEN	BOPPI, Belgian Organisation for Patients with Immuno Deficiencies
Gerissimos VANTSIMAS	Tuberous Sclerosis Association of Greece
José VERSTEGEN	SAS Stichting voor Afweerstoornissen
Charles WALLER	Plasma Protein Therapeutics Association (PPTA)
R. WEST	B Syndrome Society
Ewa WITALIS	Nutricia Polska
Name	Organisation
Marzena WOJTKOWSKA	Children's Memorial Health Institute, Warsaw, Poland
Beata WOLSKA– KUSNIERZ	Children's Memorial Health Institute, Warsaw, Poland
Jose WUEMUSE	Netherlands Genetic Alliance
Angieszka ZAKRZEWSKA	International Federation of Medical Students' Associations (IFMSA), Poland
Vlasta ZAMZEK	Debra Croatia
Daniel ZIELONKA	European Huntington's Disease Network
Isabela ZIEMINSKA-MARYNOWICZ	Association "IMMUNOPROTECT", Poland
Cornelia ZNIDAR	Severe Chronic Neutropenia International Registry (SCNIR)
Jana ZUŠČINOVA	EP Assistant to MEP Miroslav Mikolášik