



PRIMARY IMMUNODEFICIENCIES

# European Primary Immunodeficiencies Consensus Conference

# Consensus Statement



## EU PID Consensus Conference 2006 Executive Summary

On 19-20 June 2006, the International Patient Organisation for Primary Immunodeficiencies (IPOPI), the European Society for Immunodeficiencies (ESID), the International Nurses Group for Immunodeficiencies (INGID) and the European Federation for Immunological Societies (EFIS) in partnership with the European Commission, held a two-day Consensus Conference on Primary Immunodeficiencies (PIDs) at the Paul-Ehrlich-Institut in Langen, Germany.

More than 100 experts in clinical immunology, PID care, public health, genetics, EU/national ministries of health and agencies, academic centres, public health laboratories, industry, professional organisations and patient groups were brought together to identify and develop public health strategies for PID.

PIDs are a diverse group of more than 100 immune disorders, many of which result from single-gene defects. The defects lead to increased susceptibility to recurrent and persistent infections. If PIDs are left un/misdiagnosed, the immune system remains compromised leading to chronic illness, disability, reduced working capacity, decreased quality of life for patients and families, permanent organ damage or even death.

At the conference, the multi-discipline experts concluded that;

PIDs are widely undiagnosed and there is a lack of awareness of PID among the general public, healthcare professionals, healthcare policy makers and implementers.

Effective therapies for PIDs exist and early treatment saves lives, prevents morbidity and improves quality of life. There is also evidence that early treatment is cost effective.

There is a significant disparity of care within and across EU member states.

The multi-discipline experts developed a Consensus Statement containing these conclusions and also approved a series of recommendations that focus on three key areas where priority action is needed to be taken by Member State governments of the EU;

### **Awareness and education**

Clinical protocols to reliably identify PIDs

Epidemiological studies into the prevalence and incidence of PIDs and their impact on public health and costs.

International patient registries expanded to assess the clinical presentation, natural history and genetic patterns of PIDs.  
Health campaigns developed to raise awareness of PIDs among the general public.  
Education programmes targeting the general public, healthcare professionals and healthcare policy makers and implementers.

### **Screening and diagnosis.**

Practical tools for efficient diagnosis of PID made available in every country.  
Evaluation of diagnostic tools for PID and research into the feasibility of screening programmes to prevent damage.

### **Treatment and management.**

EU guidelines developed to provide equal access to treatment and assure an optimum standard and quality of patient care in the appropriate treatment setting.  
Cross country initiatives set up to allow exchange of expert experience and education.  
EU treatment centre networks established in order to determine disease outcomes.  
Safe immunoglobulin treatments available to all patients who require antibody replacement.

The following report includes the full Consensus Statement and summaries of the presentations given at the EU PID Consensus Conference. Full copies of the presentations and further details can be found at the conference web site: [www.eupidconference.com](http://www.eupidconference.com).



**PID**

PRIMARY IMMUNODEFICIENCIES

# European Primary Immunodeficiencies Consensus Conference

19 – 20 June 2006

Paul-Ehrlich-Institut, Langen, Germany

## European Primary Immunodeficiencies Consensus Statement

In partnership with the European Commission, the International Patient Organisation for Primary Immunodeficiencies (IPOPI), the European Society for Immunodeficiencies (ESID), the International Nurses Group for Immunodeficiencies (INGID) and the European Federation for Immunological Societies (EFIS) held a two-day EU Consensus Conference on Primary Immunodeficiencies (PIDs) on 19-20 June 2006 at the Paul-Ehrlich-Institut in Langen, Germany.

Attending this conference were representatives drawn from physician, patient, nurse, industry and health policy maker networks from across the EU.

Together, the expert attendees of this conference agreed this Consensus Statement on PIDs which focuses on:

The extent of the negative impact PIDs currently have on healthcare systems and undiagnosed patients.

The disparities of care and treatment that exist for people with PIDs across the EU.

Examples of immediate actions and initiatives that EU Member State governments can take to reduce the burden of PIDs in three key areas:

1. Awareness and Education
2. Screening and Diagnosis
3. Treatment and Management

# 1. Awareness & Education

## Consensus Statement

### *I. General public*

There is a lack of awareness of PIDs amongst the general public,  
There is misunderstanding of the impact of PIDs on schooling, work and social life of individual patients,  
The huge differences between PIDs and HIV/AIDS are not understood.

### *II. Healthcare professionals*

Due to a failure to include applied Immunology within professional training programmes, there is a lack of awareness of PIDs by:

- First line healthcare professionals (family GPs, doctors, nurses, midwives) i.e. lack of awareness of symptoms,
- Secondary healthcare professionals (doctors in community and teaching hospitals) i.e. lack of understanding of availability and efficacy of treatments,
- Allied professionals (physiotherapists, dieticians, genetics nurse specialists, pharmacists, psychologists, dentists).

### *III. Healthcare policy makers and implementers*

There is a lack of awareness among healthcare policy makers, at national and EU level of the negative impact on healthcare resources caused by the chronic under diagnosis of PIDs,  
There is a lack of understanding of the level of disease prevention that could be obtained if PIDs were adequately diagnosed.

## Recommendations for Action:

### *I. General public*

To increase awareness of PIDs, public health campaigns and educational programmes are needed; this is enabled by development, implementation and evaluation of:

Updated, translated (for non-native speakers) and adjusted (for special groups) material used for the recognition of potential patients,  
Material suitable for primary school curricula, including books, leaflets, letters for parents and information for school nurses to distribute,  
Material suitable for public health campaigns worldwide; this might include awareness days, as well as standard TV, print and internet advertisements to be used (with translations) in all EU member states,

Inclusion of a PIDs story line in national TV soap-operas.

## *II. Healthcare professionals*

To increase awareness of PIDs, better education is needed; this is enabled by:

- Provision of standards for basic and applied immunology training in the core content for medical & nursing schools, with particular emphasis on PIDs,
- Coupling nurse education with protocols for vaccine failures and recognition of excessive numbers of infections,
- Integrating basic and applied immunology teaching, particularly alongside immunisation, into programmes for training fellows in general paediatric internal medicine, rheumatology, respiratory medicine, and infectious disease,
- Distribute information used for education of all groups on accessible websites,
- Enabling accrual of educational credits from shared material,
- Reciprocation of information on PIDs, including guidelines and education, at professional meetings of related medical specialties,
- Including PIDs as a topic in continued professional development for related medical specialists in career posts, physiotherapists, nurses and midwives.

*III. Healthcare policy makers and implementers at EU and national levels i.e. EU level: Institutions, Parliament, Member states, EMEA. National level: regulators, legislators, national advisory bodies, Insurers. Worldwide level: WHO, pharmaceutical companies, vaccine manufacturers*

To increase awareness of PIDs by:

- Studies on impact of diseases and therapy, coupled with epidemiology, public health impact and cost effectiveness studies to demonstrate savings and improvement in quality of life,
- Strong patient organisations in all EU countries, with identification of prominent patient advocates,
- Easily accessible information for health managers/insurers,
- Regular publications from national registries.

## 2. Screening & Diagnosis

### Consensus Statement

PIDs are widely under diagnosed.

Early identification of PIDs will:

- Save lives,
- Improve health, quality of life, and lifespan in identified patients through adequate treatment,
- Enable genetic counseling and prenatal diagnosis within the family.

Tools for identification of PIDs are:

- Diagnostic guidelines for recognition of symptomatic patients,
- Appropriate immunologic and genetic laboratory tests,
- Screening tests for suitable diseases.

### Recommendations for Action:

#### *I. Gathering Information*

Clinical protocols are needed to reliably identify PIDs; these can be created by development, implementation and evaluation of:

- Diagnostic guidelines on a scientific basis,
- Standardised diagnostic criteria for PIDs.

Assessment of the impact of PIDs on the community is needed; this is enabled by epidemiologic studies to assess:

- The prevalence and incidence of PIDs in the population,
- The impact of PIDs on public health,
- The impact of PIDs on health care costs.

International PID registries enable future diagnostic processes by identifying:

- The pattern of clinical presentation of these diseases,
- The natural history of the various PIDs (morbidity, mortality, complications),
- Relationships between clinical disease patterns and genetic backgrounds.

#### *II. Appropriate Diagnostic Tools*

Practical tools for efficient diagnosis of PIDs are needed in every country; this is enabled by availability of:

- Simple diagnostic tests at the local level,
- Immunologic tests in specialist diagnostic centres at the national level,

Elaborate tests through networks of excellence across Europe.

Appropriate evaluation of diagnostic tools is needed; this is enabled by:

- Development of age-matched reference values for all diagnostic immunologic tests,
- Regular quality control of immunologic laboratories.

Research on the feasibility of screening programmes for PIDs is needed to prevent damage, including:

- Development of suitable tests,
- Assessment of costs and benefits,
- Evaluation of ethical aspects,
- Development of management guidelines for identified patients.

### 3. Treatment & Management

#### **Consensus Statement**

Effective therapies for PIDs exist.

Early treatment saves lives, prevents morbidity and improves quality of life.

Experts have reported that early treatment of PIDs is cost effective.

Safety of immunoglobulin treatments are a priority.

There is a significant disparity of care within and across EU Member States:

- There is a lack of specialised care in many countries,
- There are wide variations in the availability and funding of existing therapies,
- The availability of self treatment at home is inconsistent throughout the EU.

There are not enough trials for new therapeutic strategies.

Variation in methods in post marketing surveillance trials of products makes effective comparative analysis difficult.

#### **Recommendations for Action:**

##### *1. Guidelines*

Develop and implement European guidelines to ensure equal access to treatment within the EU for those with PIDs, assuring an optimum standard and quality of patient care in the appropriate treatment setting.



## *II. Education & Expertise Exchange*

Cross country initiatives should be developed to allow the exchange of expert experience and education in order to:

- Organise specialist nurse/midwife training courses in the EU,
- Fund medical & nurse specialists to visit other immunology centres,
- Educate related healthcare professionals,
- Support the on-going development of the ESID registry.

## *III. Centre Networks*

EU diagnosis and treatment centre networks should be established to develop methods in order to determine disease outcomes through:

- Standardising clinical trials and post marketing surveillance,
- Using the online professional registry facility from ESID.

## *IV. Treatment*

Adequate funding should be made available to provide:

- Optimum levels of treatment in each EU Member State,
- Safe immunoglobulin treatments,
- The appropriate supply of treatment, specifically immunoglobulins, for PID patients requiring this life saving therapy.

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