

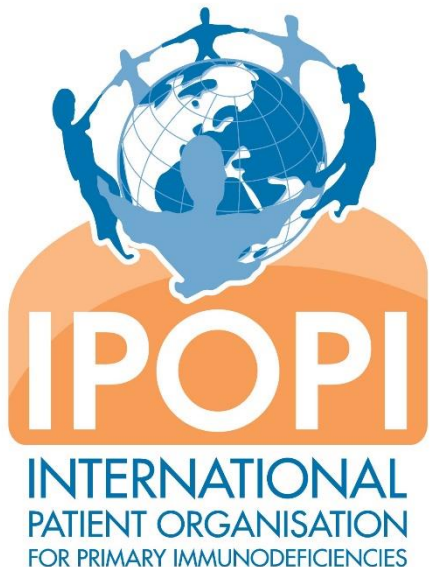
Principles of Care – lessons learnt

PID Principles of Care Session

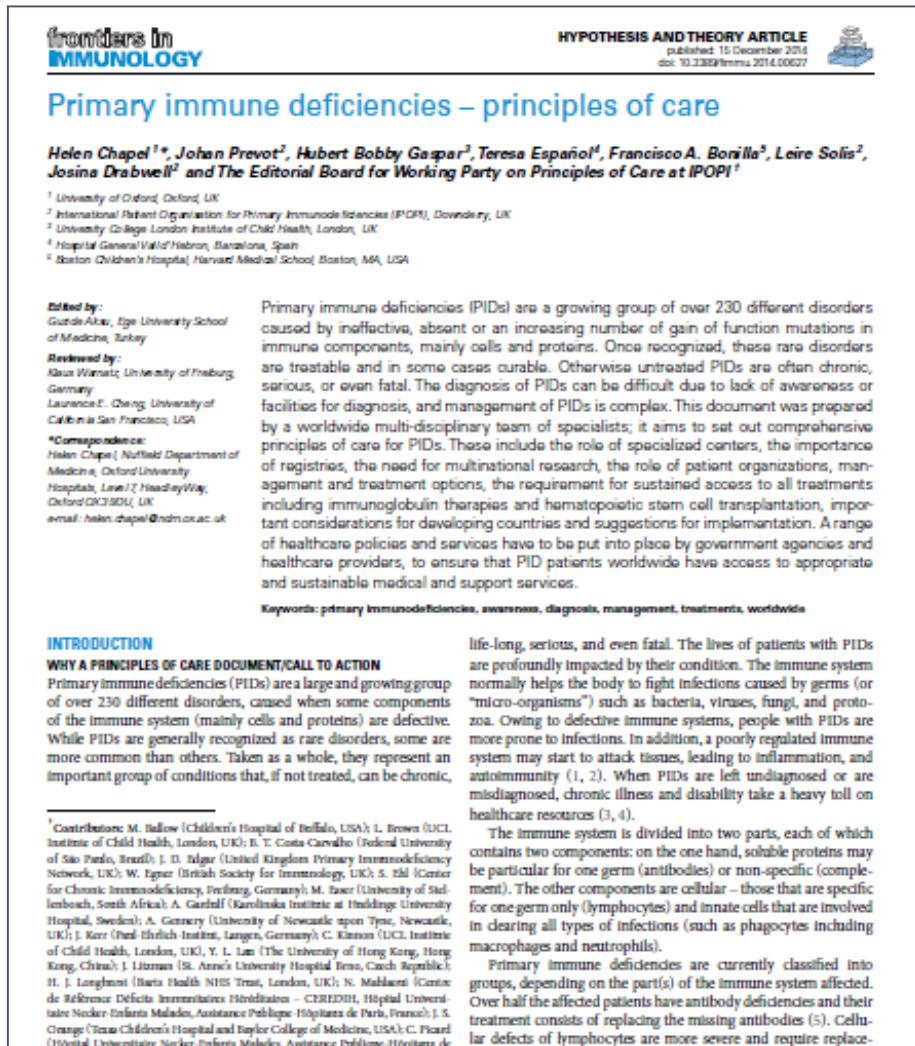
Leire Solís

IPOPI

October 25, 2018 – Lisbon (Portugal)
VXth IPOPI Global PID Patients meeting



When did it all start?



- IPOPI-led multi-stakeholder project
- Lays out 6 fundamental principles of care that should be met to reach gold standard care for PIDs
- Published in December 2014

Primary immune deficiencies – principles of care

Helen Chapel^{1*}, Johan Prevot², Hubert Bobby Gaspar³, Teresa Español⁴, Francisco A. Bonilla⁵, Leire Solis², Josina Drabwell² and The Editorial Board for Working Party on Principles of Care at IPOPI[†]

17,706

TOTAL VIEWS

Am score 38

View Article Impact

Overview of the 6 PID Principles of Care

Principle 1
The role of specialised
centres

Principle 2
The importance of
registries

Principle 3
The need for
international
collaborations

Principle 4
The role of patient
groups

Principle 5
Management and
treatment options for
PIDs

Principle 6
Managing PID
diagnosis and care in
all countries

Professional networks, recognised by
HC providers
Answer queries
Establishment/use registries
Awareness promotion medical com

Data catches attention policy makers
Enables:
- costs to be calculated
- Publications (awareness)
- Comparisons w. other diseases

Awareness on dis., new
therapies, etc.
Gain training
Comparisons with other
countries

Principle 1
The role of specialised
centres

Principle 2
The importance of
registries

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collaborations

Principle 4
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Principle 5
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treatment options for
PIDs

Principle 6
Managing PID
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all countries

Show the patient's perspective of
the PID
Diagnosis real impact on patients'
QoL, prognosis
Educational, support, advisory role
of groups

Criteria for fast & reliable
diagnosis
Challenges to Ig therapy, HSCT
WHO EML

Need to establish networks to spread
knowledge, diagnosis, etc.
Support from well established societies
& patient groups.

That's nice, but how do we use it?

- PID Principles of Care – comprehensive document
- Needs of adaptation to national reality
 - ↳ Implementation toolkit

Implementation toolkit

- What do YOU want for your country? What are YOUR priorities?
- Making complex issues understandable to your **specific audience**:
 - Facts & figures
 - Infographics

Now that we know how to use it, where do we stand?

- How's the care provided to PID patients in the world? Different regions? In your country?

↳ PID implementation survey

- Reasoning behind:
 - Provision of data to **you**
 - Basis for comparison
 - Advocacy tool at international, regional and national level

PID Implementation survey

- Key learnings:
 - Ambitious first step
 - Great exercise and lots of feedback received – still not all NMOs!
 - Complex, perhaps more explanations /rationale behind questions should be given
 - Looking forward to improving for the next edition!



GLOBAL STATUS OF PID PRINCIPLES OF CARE

Authors: Martine Pargent¹, John Probst², Ghazal Mahdavi³, Seann Kierns⁴, Leila Solis⁵

1- BACKGROUND

IPOPI (the International Patient Organisation for Primary/Immune-deficiency) provided in 2014, in collaboration with experts in the field, a gold standard framework for the PID care worldwide, identifying 6 key Principles of Care¹.

- 1) PID diagnosis and care,
- 2) Management and treatment options,
- 3) Role of specialised centres,
- 4) Importance of registries,
- 5) International collaboration and research,
- 6) Role of patient groups.

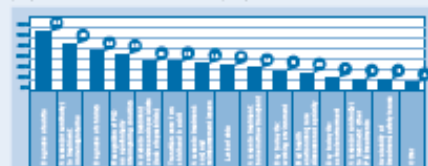
As a follow-up, IPOPI has launched a PID Principles of Care implementation survey, to give an overview of the current reality in PID care, in 60 countries in all continents.

2- METHOD

IPOPI conducted its PID Principles of Care implementation survey through a self-administered questionnaire, that was sent to the head of the 60 HMOs/charities/Non-profit Organizations and to health care professionals in 28 countries. The questionnaire, prepared by IPOPI in collaboration with the Medical Advisory Panel, addressed the 6 Principles of Care.

3- RESULTS

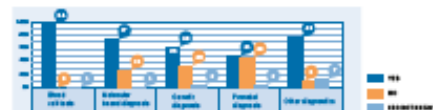
Diagnosis of PID, especially for adults and adolescent access to immunoglobulins and specialised therapies are expected to be the most common concerns in PID care today (see developments below). Other key areas of concern include lack of specialised centres and transition care, poor recognition of PID, cost and reimbursement of issues, day-to-day life (working, schooling), as well as a poor health policy environment where care conditions are not a priority.



Q1A17b: What are the most common concerns for PID patients in your country?

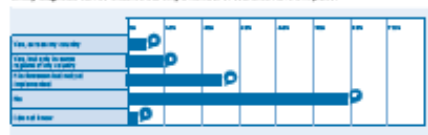
PRINCIPLE #1: PID DIAGNOSIS AND CARE

Many PID patients, such as those affected with common variable immunodeficiency (CVID) face significantly delayed diagnosis, being treated for their symptoms rather than the cause. Access to a range of diagnostic tools is key to properly diagnose PID. Despite good access to blood cell tests many countries still lack access to more specialised diagnostics.



Q1A17b: How often PID diagnosis is available in your country?

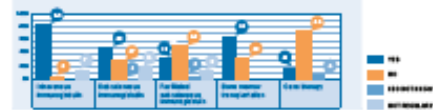
PIDs occurring in childhood, such as severe Combined Immunodeficiency (SCID), are more severe than those occurring in adults, and can be cured if diagnosis is done on time. With newborn screening, timely diagnosis can be ensured but only a handful of countries have it in place.



Q1A17b: Is newborn screening for SCID in your country?

PRINCIPLE #2: MANAGEMENT AND TREATMENT OPTIONS - AVAILABILITY

Immunoglobulins (γG) are available in a majority of surveyed countries, whilst access to subcutaneous Ig (SCIG) is marginal. Bone marrow transplant (BMT) is available in about 70% of countries, however not all of them are able to perform it locally and patients must travel abroad to get treated. Only in a minority of countries PID patients have access to gene therapy.



Q1A17b: How often management options are available in your country for PID patients?

Immunoglobulins are plasma-derived medicines that are limited in quantity. Their use is characteristically growing worldwide. Patients face many challenges related to Ig therapies, amongst which: limited choice, rapid depletion, limited monitoring, limited supply, shortages. Patients also face difficulties with cost coverage of their treatment, with close to 40% stating this as a challenge.



Q1A17b: Do patients in your country face the following challenges with regards to availability of immunoglobulin?

PRINCIPLE #2: MANAGEMENT AND TREATMENT OPTIONS - ACCESS

PID treatments are often costly and even if available, they are not always fully covered by national health systems. First covered, patients travel abroad, unless they are paid out of pocket, which causes economical issues on both personal and societal levels.



Q1A17b: Do patients in your country face the following challenges with regards to access to immunoglobulin?

Transition care is mainly accessible only in some hospitals or not addressed at all. Patients living in rural areas have no access or must travel long distances to treatment centres, complicating transition care.



Q1A17b: Do patients in your country face the following challenges with regards to transition care?

PRINCIPLE #3: ROLE OF SPECIALISED CENTRES

Out of 60 countries, 16 have no specialised PID centres or experts in their country. Adult services are mostly available but only in large health centres. 10 countries do not have adult PID services available at all.



Q1A17b: Are there specialised PID centres or experts available in your country?

CONCLUSIONS

There are great discrepancies globally and many countries are still far from a gold standard framework for the care of PID patients. Access and availability vary from absolute lack of care to lack of choice and access to travel therapies. Health policies should address PIDs as rare conditions not only affecting paediatric populations as well as long-term with advances in research and treatment possibilities.

Simultaneously, PID patients are healthier, reaching adulthood and growing older. Patient education is increasingly making patients more empowered, yet leaving them in a frustrating situation: still having to face delays in diagnosis and effective treatment (diagnosis and care). IPOPI is collaborating with its HMOs and the global PID community on global and national policy, regulatory and legislative levels to ensure that the PID Principles of care are addressed worldwide.

Pass by the Poster!
N° 145

PID Implementation survey

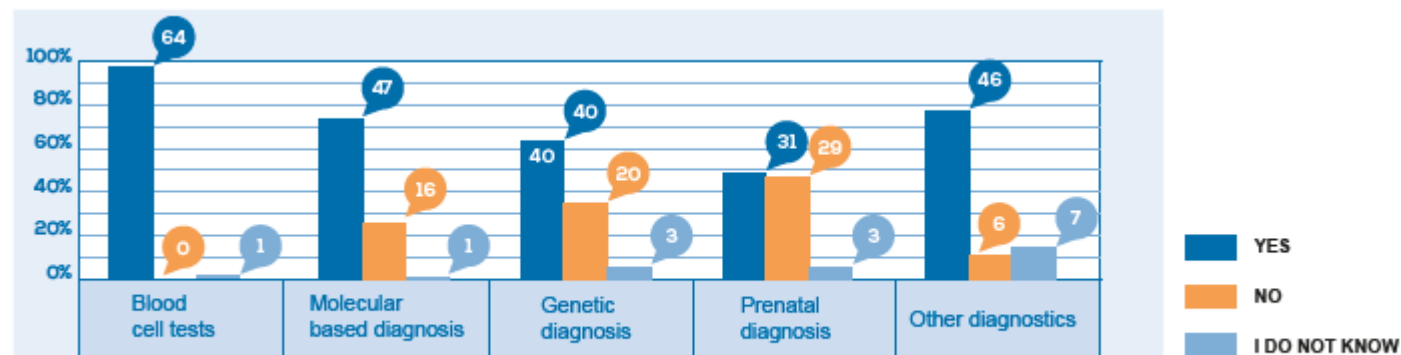


CHART2: Are these PID diagnostics available in your country?

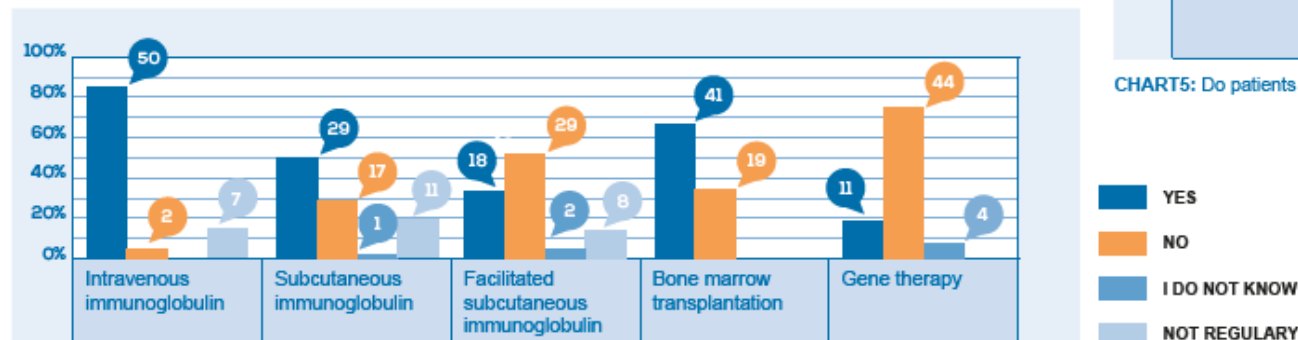


CHART4: Are these therapies available in your country for PID patients?

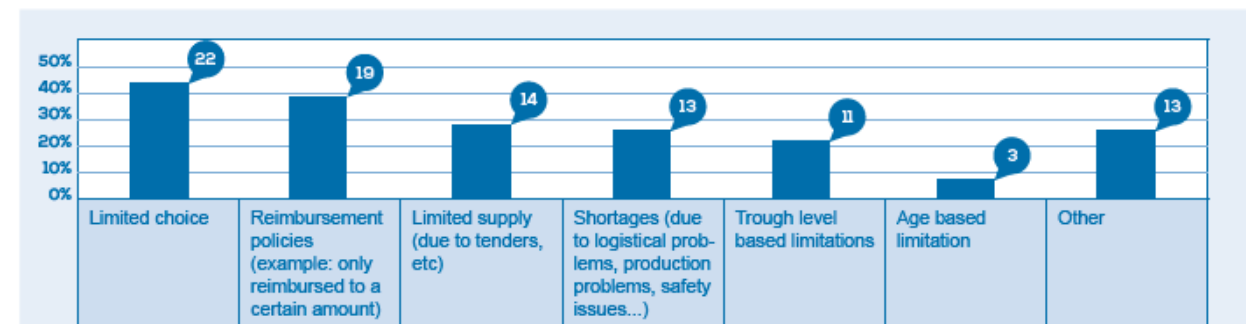


CHART5: Do patients in your country face the following challenges, with regards to availability of immunoglobulins?

PID Principles of Care – next steps

- Analysis of data extracted from the Survey
- Publications
- PID Life Index

Thank you!



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