



The global organisation working to improve the quality of life for people
with primary immunodeficiencies.

IPOPI EU POLICY FORUM

HEALTH TECHNOLOGY ASSESSMENTS & PRIMARY IMMUNODEFICIENCIES: THE VIEW FROM THE PATIENTS

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Introduction

- IPOPI, the Association of national patient organisations dedicated to improving awareness, access to early diagnosis and optimal treatments for PID patients worldwide
- Work with policy makers to address patients needs
- Dialogue with other stakeholders to design best approach





Introduction

- Thanks to Mrs. Willmott, MEP.
- Thanks to participants and experts who accepted to join this important meeting
- Thanks to sponsors supporting this IPOPI initiative

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Introduction

- *Primary Immunodeficiencies* (PID) is a group of +/- 200 rare disorders that can affect anyone regardless of age or gender
- PID occur in persons born with failed immune systems
- PID are UNDER DIAGNOSED in most countries due to misdiagnosis
- PID patients can go for years being treated for their symptoms & suffer recurring and repeated infections



Health Technology Assessments

- Government & national agencies increasingly implement cost containment mechanisms on healthcare budgets to respond to rising cost of healthcare
- HTAs = *The comparative evaluation of the effectiveness, the cost and other implications of a health technology, be it a medicinal product, medical device, diagnostic tool or other health systems*
- HTA models and the way they are used will vary from country to country
- Common trait: From clinical efficacy to cost effectiveness



Health Technology Assessments

- Assessment of therapies – some factors to take into account:
 - Advocacy efforts by stakeholders
 - National political environment
 - Willingness by national agencies to involve key stakeholders
 - These factors are further complicated when it comes to rare diseases such as primary immunodeficiencies (PIDs)
 - Lack of large randomized clinical trials
 - Lack of pharmaco-economic data
 - HTA models applied to common conditions often are not applicable to seemingly ‘costly’ rare diseases therapies
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Health Technology Assessments & PIDs

- In the case of PIDs it is extremely important to consider the impact:
 - ✓ Of a therapy on the life expectancy and the quality of life of the patients
 - ✓ In terms of societal and professional life (ie the cost to society related to inability to work and lead a productive life without treatment) - MACRO-ECONOMIC IMPACT!
 - ✓ Of a restricted access to the appropriate therapy and the subsequent medical costs that would be incurred in the treatment of the symptoms rather than the cause of the symptoms.
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Health Technology Assessments & PIDs

- Studies conducted in the United States have shown that:
 - ✓ “once [PID] patients begin receiving IVIG on a regular basis their health status improves markedly, their activity limitations drop significantly and their quality of life improves dramatically” (IDF)
 - ✓ Each undiagnosed PID patient costs the healthcare system \$102,736 vs \$ 22,696 for a diagnosed patient annually and so “The economic impact of undiagnosed PID patients to the healthcare system in the United States totals over \$40 billion annually”.
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EU actions on HTAs

- The EU is leading the way in terms of promoting the involvement of patients into the HTA process
 - ✓ **EunetHTA (2009 EU health programme work plan):** “a multidisciplinary process that summarises information about the medical, social, economic and ethical issues related to the use of a health technology in a systematic, transparent, un-biased, robust manner. Its aim is to inform the formulation of safe, effective, health policies that are patient focused and seek to achieve best value”



EU actions on HTAs

- **Directive 2011/24/EU on the application of patients' rights in cross-border healthcare & Council roadmap following the adoption of the Directive on the application of Patients' Rights in Cross-Border Healthcare:**
 - ✓ Creation of an HTA network that “shall be based on the principle of good governance including transparency, objectivity, independence of expertise, fairness of procedure **and appropriate stakeholder consultations**”
- **Health for Growth programme proposal 2014-2020**



Conclusions & recommendations

- Immunoglobulin therapies are **THE ONLY** efficacious & **LIFE SAVING** treatment for patients with a PID and they are cost effective
- It is important to take into account the macro-economic and societal benefits of diagnosing patients early and treating them with the appropriate therapy.
- **HEALTH is WEALTH!**



Recommendations of the PID Expert group chaired by
Jorgo Chatzimarkakis MEP



Conclusions & recommendations

- Member States and the European Commission should make sure that the spirit of the Crossborder Healthcare Directive is respected by ensuring appropriate consultation of stakeholders and therefore patients in HTA processes
- No choice concerning the life-saving treatment PID patients need should be made without consulting them or in a non-transparent fashion. HTA can and should take patients' views into account.



Conclusions & recommendations

- In addition of its policy goals, HTA techniques must have a macro-economic approach and provide clear information on the impact of decisions on patients quality of life.
 - Economic arguments should not be used to limit access to well-established life-saving medicinal products that ultimately will prevent unnecessary expenses such as hospitalisation or days-off work/school due to the disease.
 - Physicians should be protected from any sort of pressure, including economic considerations aiming at limiting access to life-saving treatments.
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Conclusions & recommendations

- The impact of HTAs can be enhanced if:
 - key stakeholders (e.g. patients, academic experts, treating physicians, industry) are appropriately involved at all stages;
 - decision-makers give advance commitments to use assessment reports (and assessments meet their needs);
 - necessary resources are available for implementing decisions;
 - there is transparency in the assessment and decision-making processes;
 - and collaboration, knowledge and skills are transferred across jurisdictions..
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THANK YOU!