ADVOCATING THE WHO: IPOPI’S EXPERIENCE

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1. Introduction

2. Brief introduction to Primary Immunodeficiencies (PIDs)

3. Brief introduction to WHO Essential Medicines List

4. IPOPI’s Advocacy campaign

5. Conclusions
IPOPI, the Association of national organisations of patients with PIDs, is dedicated to:

- Improving awareness, access to early diagnosis and optimal treatments for PID patients worldwide
- Working with policy makers to address patients needs
- Collaborating with all relevant stakeholders to design best approach
Primary Immunodeficiencies (PID) is a group of +/- 250 genetic rare disorders that could affect anyone.

PID occur in persons born with failed immune systems.

Prevalence is difficult to establish: PIDs are massively UNDER DIAGNOSED in many countries.

PID patients can go for years being treated for their symptoms & suffer recurring and repeated infections.
BRIEF OVERVIEW OF PRIMARY IMMUNODEFICIENCIES (PI DS)

• Large spectrum of rare and chronic conditions (250) that are mostly treatable and for some, curable

• Need for unique plasma-derived products – Immunoglobulins

• Immunoglobulins:
  • Biological products
  • Each brand product is different
  • Each patient has a different tolerability to the different immunoglobulins
WHO's objective is the attainment by all peoples of the highest possible level of health.

WHO’s vision: people everywhere have access to the essential medicines they need; that the medicines are safe, effective, and of good quality; and that the medicines are prescribed and used rationally.

The WHO Model Lists of Essential Medicines has been updated every two years since 1977.

The list is divided into two sections:

- The core list: minimum medicine needs for a basic healthcare system, listing the most EFFICACIOUS, SAFE and COST EFFECTIVE ones for priority conditions.

- The complementary list: essential medicines for priority diseases for which specialized diagnostic; monitoring facilities; medical care and/or specialized training are needed. (e.g. Immunoglobulins)
### 19. IMMUNOLOGICALS

#### 19.1 Diagnostic agents


| tuberculins, purified protein derivative (PPD) | injection |

#### 19.2 Sera and immunoglobulins


| anti-D immunoglobulin (human) | injection, 250 micrograms in single-dose vial |
| antitetanus immunoglobulin (human) | injection, 500 IU in vial |
| antivenom serum | injection |
| diphtheria antitoxin | injection, 10 000 IU, 20 000 IU in vial |
| immunoglobulin, human normal | injection (intramuscular) |
| immunoglobulin, human normal | (2, 8) injection (intravenous) |
| rabies immunoglobulin | injection, 150 IU/ml in vial |

#### 19.3 Vaccines

The WHO EML had historically included Immunoglobulins – up to the 12th Edition
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In 2003, WHO decides to remove IG from EML

WHO Reasons for removing IG were:
- no need for IG’s in view of the availability of suitable vaccines
- no WHO clinical guidelines recommending its use
IPOPI’s Advocacy Campaign - 2003-2005 Campaign -

- Several organisations requested its reinstatement in the list in 2005: including IPOPI (patients), IUIS (medical soc) and PPTA (industry)
- WHO 2005 decision was to reject Igs reinstatement

<table>
<thead>
<tr>
<th>WHO’s reasons for rejection</th>
<th>Our position</th>
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<tr>
<td>Prevalence very rare</td>
<td>Are meds less essential if rare? Prevalence of approved indications for Igs was above EU threshold</td>
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<tr>
<td>Insufficient evidence of efficacy</td>
<td>Plenty of data of efficacy on various indications</td>
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<td>Cost-effectiveness</td>
<td>Igs allow for treating the cause of symptoms rather than the symptoms alone = cost effectiveness</td>
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IPOPI’s Advocacy Campaign - 2005-2007 Campaign -

• Key learning from 2003-2005 campaign – need a more ambitious, comprehensive and structured campaign
• Joint application submitted by IPOPI and IUIS
• Supportive stakeholders involved:
  – Patient organisations: IPOPI and 26 IPOPI members (incl. South Africa, Iran, India, Morocco, Argentina)
  – Ig manufacturers: including non-for-profit association & industry federation
  – Medical and nurse international organisations: ESID, INGID, European Federation of Immunological Societies (EFIS)
  – Medical and nurse national organisations: i.e. Baltic Society for Paediatric Oncology and Haematology, Australasian Society of Clinical Immunology and Allergy
  – 44 international experts from 21 countries
Carefully prepared application, following the administrative process:

- Application prepared by IPOPI and IUIS + cooperation of leading medical experts
- 7 annexes were included to the application on different aspects:
  - Supportive literature on medical aspects
  - List of supportive IPOPI members;
  - List of supportive national + international medical organisations;
  - List of manufacturers
## IPOPI’s Advocacy Campaign - 2005-2007 Campaign Result -

### 11. Blood Products and Plasma Substitutes

#### 11.1 Plasma Substitutes

- **Injectable solution:** 6%.
  
  * Polygeline, injectable solution, 3.5% is considered as equivalent.

#### 11.2 Plasma Fractions for Specific Use


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* Indicated for primary immune deficiency.

**Indicated for primary immune deficiency and Kawasaki disease.
IPOPI’S ADVOCACY CAMPAIGN
- WHO EML CAMPAIGN: SPILL OVER EFFECT -

• On the basis of the EML contain, IPOPI and IPOPI members have been able to advocate for Ig availability in different countries in the world, including Bosnia and Herzegovina, Bolivia, Poland, etc.

• Thanks to it, a couple of parents from Bosnia and Herzegovinian managed to get access to Ig for their son.

• Given our constructive interaction with the WHO from 2005-2007, IPOPI became engaged in a dialogue with other WHO units and works on different dossiers:
  • Blood donation – key for Igs – in regular contact with the unit in charge of Blood & Transfusion Safety
  • Member of the Global Forum for Blood Safety
  • Achilles project
Thank you for your attention!