ADVOCATING THE WHO: IPOPI'S EXPERIENCE

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INTRODUCTION

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

Brief Overview of Primary Immunodeficiencies (PIDs)

- *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 (PIDs)

- Large spectrum of rare and chornic 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

Some Basics About the WHO & the Essential Medicines List

- 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)

IPOPI's Advocacy Campaign
- Why was it initiated? -

- The WHO EML had historically included Immunoglobulins – up to the 12th Edition

19. IMMUNOLOGICALS		
19.1 Diagnostic agents		
All tuberculins should comply with the WHO Requirements for Tuberculins (Revised 1985). WHO Expert Committee on Biological Standardization Thirty-sixth report, (WHO Technical Report Series, No. 745, 1987, Annex 1).		
tuberculin, purified protein derivative (PPD)		injection
19.2 Sera and immunoglobulins		
All plasma fractions should comply with the WHO Requirements for the Collection, Processing and Quality Control of Blood, Blood Components and Plasma Derivatives (Revised 1992). WHO Expert Committee on Biological Standardization Forty-third report, (WHO Technical Report Series, No. 840, 1994, Annex 2).		
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	(2)	injection (intramuscular)
★ immunoglobulin, human normal	(2, 8)	injection (intravenous)
□ rabies immunoglobulin		injection, 150 IU/ml in vial
19.3 Vaccines		

IPOPI's Advocacy Campaign
- Why was it initiated? -

- The WHO EML had historically included Immunoglobulins – up to the 12th Edition
- 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)

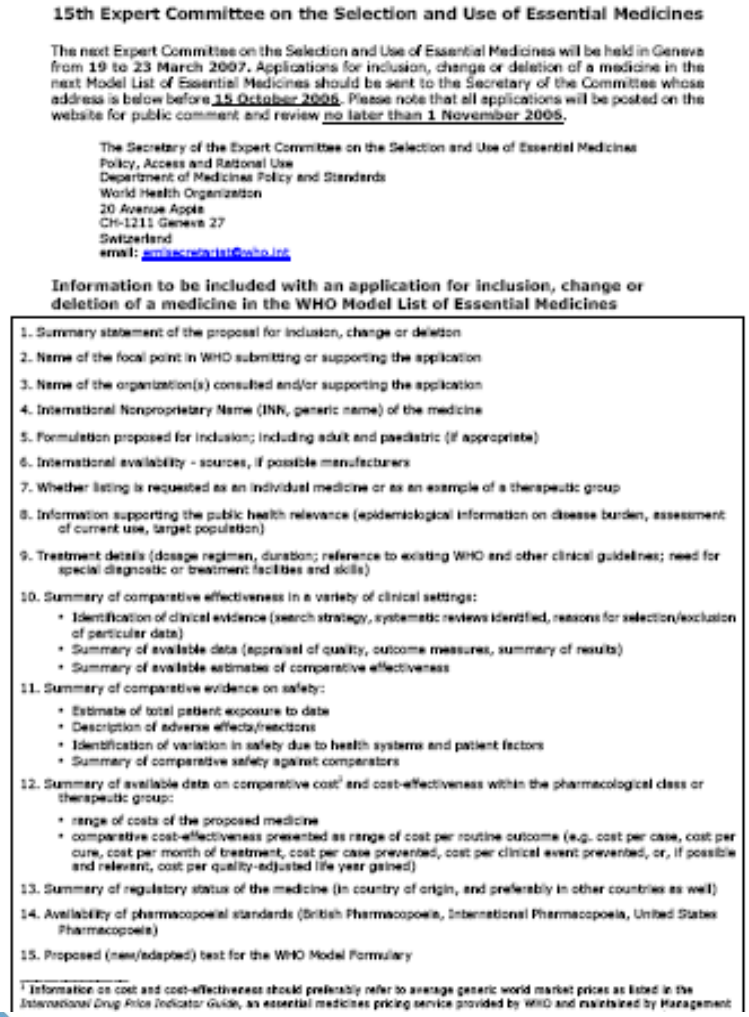
- WHO 2005 decision was to reject Igs reinstatement

WHO's reasons for rejection	Our position
Prevalence very rare	Are meds less essential if rare? Prevalence of approved indications for Igs was above EU threshold
Insufficient evidence of efficacy	Plenty of data of efficacy on various indications
Cost-effectiveness	Igs allow for treating the cause of symptoms rather than the symptoms alone = cost effectiveness

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

IPOPI's Advocacy Campaign
- 2005-2007 Campaign-



15th Expert Committee on the Selection and Use of Essential Medicines

The next Expert Committee on the Selection and Use of Essential Medicines will be held in Geneva from 19 to 23 March 2007. Applications for inclusion, change or deletion of a medicine in the next Model List of Essential Medicines should be sent to the Secretary of the Committee whose address is below before 15 October 2006. Please note that all applications will be posted on the website for public comment and review no later than 1 November 2006.

The Secretary of the Expert Committee on the Selection and Use of Essential Medicines
Policy, Access and Rational Use
Department of Medicines Policy and Standards
World Health Organization
20 Avenue Appia
CH-1211 Geneva 27
Switzerland
email: emlsecretariat@who.int

Information to be included with an application for inclusion, change or deletion of a medicine in the WHO Model List of Essential Medicines

1. Summary statement of the proposal for inclusion, change or deletion
2. Name of the focal point in WHO submitting or supporting the application
3. Name of the organization(s) consulted and/or supporting the application
4. International Nonproprietary Name (INN, generic name) of the medicine
5. Formulation proposed for inclusion; including adult and paediatric (if appropriate)
6. International availability - sources, if possible manufacturers
7. Whether listing is requested as an individual medicine or as an example of a therapeutic group
8. Information supporting the public health relevance (epidemiological information on disease burden, assessment of current use, target population)
9. Treatment details (dosage regimen, duration; reference to existing WHO and other clinical guidelines; need for special diagnostic or treatment facilities and skills)
10. Summary of comparative effectiveness in a variety of clinical settings:
    - Identification of clinical evidence (search strategy, systematic reviews identified, reasons for selection/exclusion of particular data)
    - Summary of available data (appraisal of quality, outcome measures, summary of results)
    - Summary of available estimates of comparative effectiveness
11. Summary of comparative evidence on safety:
    - Estimate of total patient exposure to date
    - Description of adverse effects/reactions
    - Identification of variation in safety due to health systems and patient factors
    - Summary of comparative safety against comparators
12. Summary of available data on comparative cost[1] and cost-effectiveness within the pharmacological class or therapeutic group:
    - range of costs of the proposed medicine
    - comparative cost-effectiveness presented as range of cost per routine outcome (e.g. cost per case, cost per cure, cost per month of treatment, cost per case prevented, cost per clinical event prevented, or, if possible and relevant, cost per quality-adjusted life year gained)
13. Summary of regulatory status of the medicine (in country of origin, and preferably in other countries as well)
14. Availability of pharmacopoeial standards (British Pharmacopoeia, International Pharmacopoeia, United States Pharmacopoeia)
15. Proposed (new/adapted) text for the WHO Model Formulary

[1] Information on cost and cost-effectiveness should preferably refer to average generic world market prices as listed in the *International Drug Price Indicator Guide*, an essential medicines pricing service provided by WHO and maintained by Management

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 -

WHO 2007 EML for adults

11. BLOOD PRODUCTS AND PLASMA SUBSTITUTES	
11.1 Plasma substitutes	
☐ dextran 70*	**Injectable solution:** 6%. * Polygeline, injectable solution, 3.5% is considered as equivalent.
11.2 Plasma fractions for specific use	
All plasma fractions should comply with the WHO Requirements for the Collection, Processing and Quality Control of Blood, Blood Components and Plasma Derivatives (Revised 1992). (WHO Technical Report Series, No. 840, 1994, Annex 2).	
Complementary List	
human normal immunoglobulin	***Intramuscular administration:*** *16% protein solution.* ***Intravenous administration:*** *5%; 10% protein solution.*

WHO 2007 EML for children

11.2 Plasma fractions for specific use	
All plasma fractions should comply with the WHO Requirements for the Collection, Processing and Quality Control of Blood, Blood Components and Plasma Derivatives (Revised 1992). (WHO Technical Report Series, No. 840, 1994, Annex 2).	
Complementary List	
human normal immunoglobulin	***Intramuscular administration:*** *16% protein solution.** ***Intravenous administration:*** *5%; 10% protein solution.*** ***Subcutaneous administration:*** *15%; 16% protein solution.** * *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!