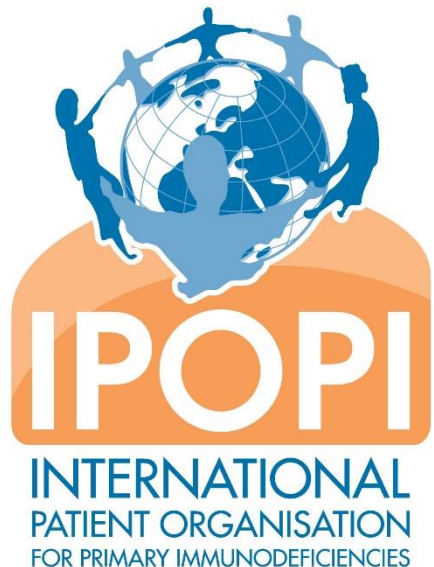


Case study of a patient group that obtained the inclusion of an essential treatment and diagnostic tests in the WHO Priority Lists



Johan Prevot

Executive Director, IPOPI

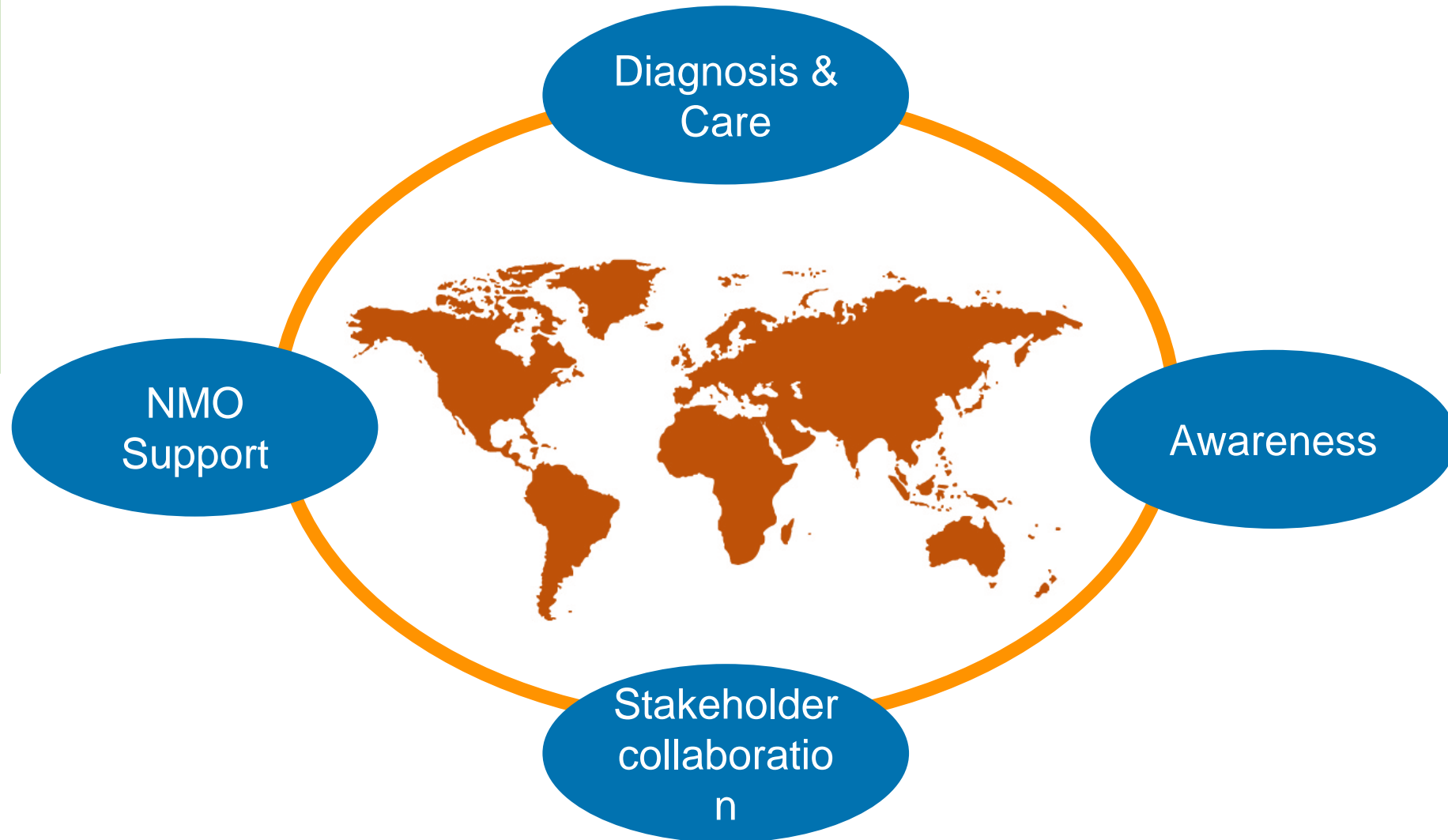
RDI Global Meeting, 19th May 2020

IPOPI in a nutshell

- The association of national patient organisations dedicated to improving:
 - Awareness
 - Access to early diagnosis
 - Access to care
- For patients living with primary immunodeficiencies (PIDs), worldwide



IPOPI Strategic Objectives 2016-2020





IPOPI has 68
National Member
Organisations

**Latest to join:
Nicaragua -
Tunisia – China –
Kenya**

THE GLOBAL ORGANISATION WORKING TO IMPROVE
THE QUALITY OF LIFE FOR PEOPLE WITH PRIMARY IMMUNODEFICIENCIES

www.ipopi.org



IPOPI's collaboration with the WHO

Longstanding collaboration based on IPOPI's involvement in:

- Global Collaboration for Blood Safety
- Model Lists of Essential Medicines for adult & paediatric patients
- Model List of Essential In Vitro Diagnostics

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Model Lists of Essential Medicines (EML)

- The WHO Essential Medicines List (EML) and the WHO Essential Medicines List for Children include two sections:
 - Core list: minimum medicine needs for a basis healthcare system
 - Complementary list: essential medicines for priority diseases for which specialised diagnostic, monitoring facilities, medical care and/or specialised training are needed (e.g. **Immunoglobulins**)
- The WHO EML had historically included Immunoglobulins up to the 12th Edition (2003). Reasons for withdrawal:
 - No need for Ig in view of the availability of suitable vaccines
 - No WHO clinical guidelines recommending its use

Model Lists of Essential Medicines (EML)

- Initial individual efforts to reinstate Igs on the 2005 edition of the list were unsuccessful
- WHO Reason for rejecting reinstatement at the time were:
 - Prevalence of target diseases is very rare
 - Insufficient evidence of its efficacy
 - Cost effectiveness
- Questions we asked ourselves:
 - Are medicines less essential if they are rare?
 - Plenty of data on efficacy of IG's on various indications
 - Treating the cause of the symptoms with IG's rather than the symptoms alone = cost effectiveness

So what did we do wrong?

- These reflexions formed the basis for a more ambitious, comprehensive, structured and joint campaign

Model Lists of Essential Medicines (EML)

- Joint application submitted by IPOPI and IUIS
- Numerous annexes – in depth review of evidence, joint call for action by supporting organisations, survey results...
- Supportive stakeholders involved:
 - Patient organisations: IPOPI and 26 IPOPI members at the time
 - Ig Manufacturers: non-for-profit association & industry federation
 - Medical and nurse international organisations: ESID, INGIG, EFIS
 - Medical and nurse national organisations: i.e. Australasian Society of Clinical Immunology and Allergy
 - 44 international experts from 21 countries
- WHO 2007 EML for adults and for children included Igs

Model List of Essential In Vitro Diagnostics (EDL)

- Joint application submitted by IPOPI and IUIS, led by IPOPI in 2018 & 2019
- “Internal” effort:
 - 1 staff member dedicated to coordination, submission and liaison with WHO EDL staff and reviewers
 - 3 IPOPI medical advisors involved
 - 1 lead laboratory expert involved + 3 additional laboratory staff members from labs in developing world consulted
- External effort:
 - 7 international scientific societies co-signing letter of support
 - 2 researchers networks
 - Industry associations

Model List of Essential In Vitro Diagnostics (EDL)

- Results for the 2019 edition:
 - PIDs included as a stand-alone disease category
 - Inclusion of
 - Automated CBC
 - HIV ½ antibody for differential diagnosis
 - Serum plasma levels (IgA, IgG, IgM) (*)
 - Lymphocyte subtype enumeration (*)
- Results for the 2020 edition: WHO EDL Secretariat currently focussing on COVID-19 pandemic → postponement of other activities

Model Lists why do they matter?

- EML & EDL need to be adapted to national situations → patient advocacy groups matter!
- EML:
 - Has proved to be a very good advocacy tool for some of our NMOs
 - A great reference for other legislative & policy texts (national & international level)
- EDL:
 - It is a very **good awareness raising tool** that can be used by HCPs and patient organisations to increase access to diagnosis
 - It has the **potential of facilitating the diagnosis of PIDs** even by non-specialists – at least in the first steps of the diagnosis
- Link between **IVDs and Essential Medicines will be strengthened** in future editions by WHO & in country assessments

Lessons learnt

Time consuming exercise (dedicated staff, involved experts...)

WHO guidelines and requirements need to be followed scrupulously

EML & EDL are just the beginning of the advocacy – need adequate implementation at national level!

BUT... Help raising awareness about specific rare diseases esp in LMIC

Are a great first recognition of RDs from a therapeutic & diagnostic perspective at international level

Thank you!



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