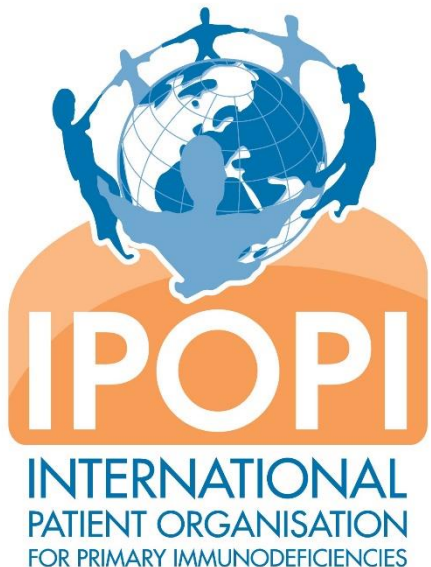


PIDs a public health priority – EU priorities

Johan Prevot
IPOPI Executive Director

World Primary Immunodeficiency Week
European Parliament (Brussels), 26 April 2016



- Thanks to Mr José Inácio Faria MEP and Mr. Carlos Zorrinho MEP for co-hosting this event.
- Thank you to Dr Piecha MEP for accepting taking the floor.
- Thanks to participants and experts who accepted to join and will contribute to the discussions.
- Thanks to Baxalta for supporting this IPOPI initiative.

Baxalta

- **We are stronger if we work together!**

WORLD'S PRIMARY IMMUNODEFICIENCY WEEK

Test

Diagnose

Treat

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Diagnose

Treat






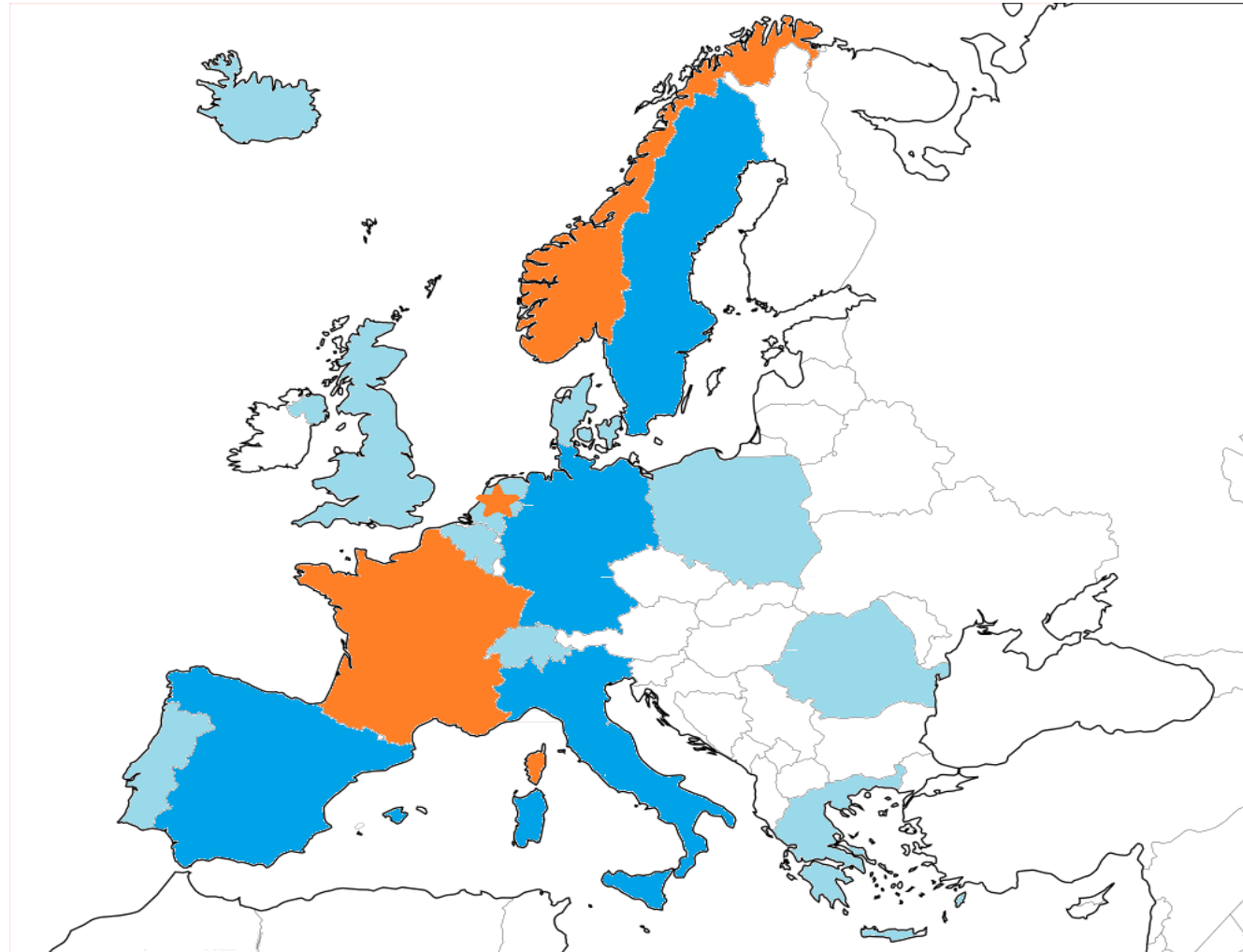
- SCID newborn screening
- Primary Immunodeficiencies Principles of Care

SCID NEWBORN SCREENING

- 2011 - 1st IPOPI European initiative – EU PID Forum
 - Only US was systematically screening for SCID in a few states
 - At EU only some scattered regional pilot projects
- 5 years later:
 - US 35 states, Israel perform SCID NBS
 - Many EU countries have on-going systematic national or regional pilot projects
 - Several on-going patient & physician led initiatives

SCID NEWBORN SCREENING

- SCID NBS nation-wide pilot 
- SCID NBS region-based pilot 
- SCID NBS request to authorities 

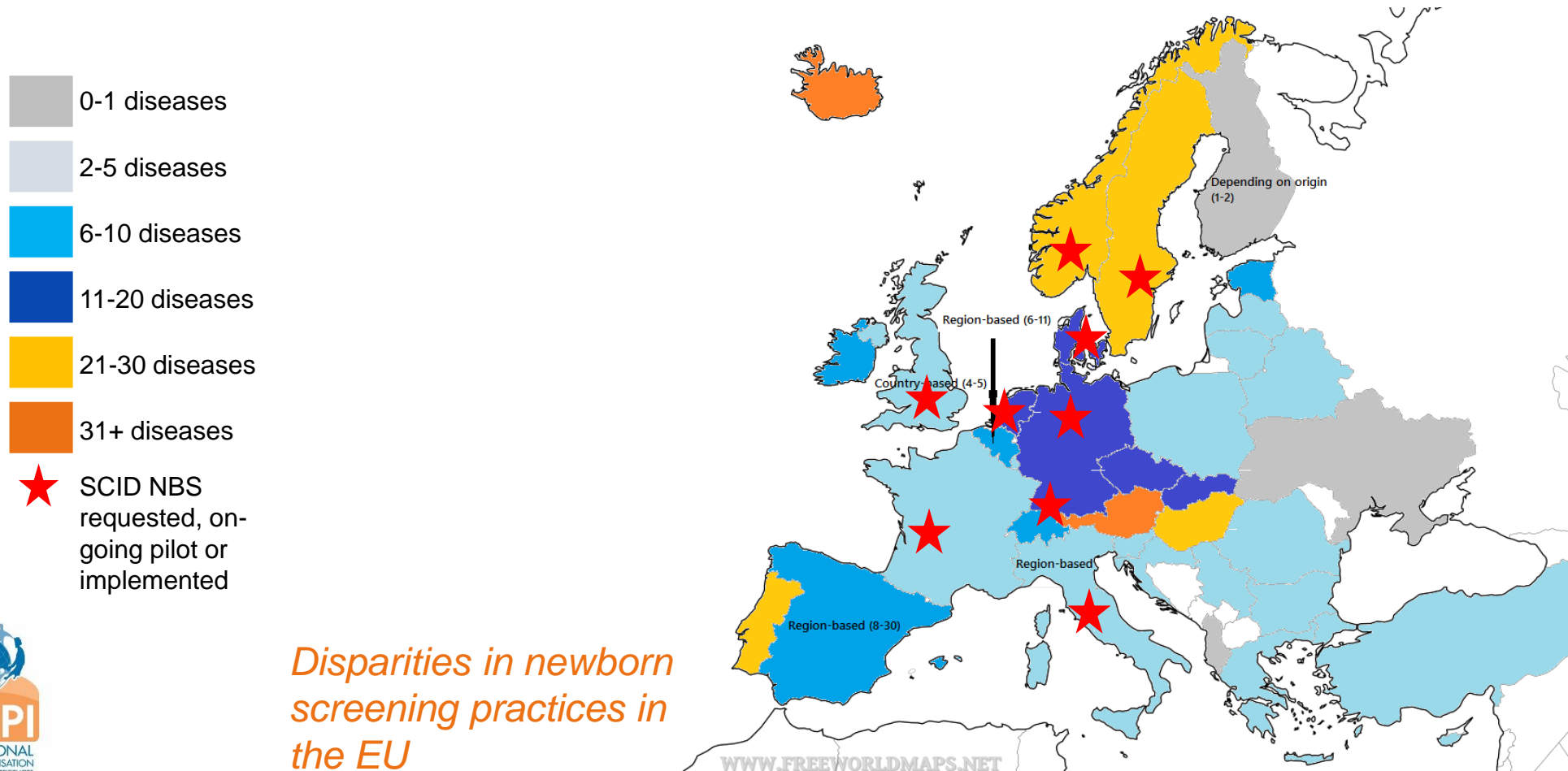


PID PRINCIPLES OF CARE

- Reasons:
 - Scarce knowledge at medical, political and societal level about PIDs.
 - Disparities in screening, diagnosis, treatment and care worldwide & even in the EU!
 - Not all continents benefit from international scientific collaboration; from facilities that would allow patients to get diagnosed, treated and cared for.
- So far:
 - Around 6,000 views of the article from main source.
 - Translated into other languages
 - Development of “Implementation package” & support to NMOs.

PID PRINCIPLES OF CARE

- Disparities in screening in the EU



Disparities in newborn screening practices in the EU

WORLD'S PRIMARY IMMUNODEFICIENCY WEEK



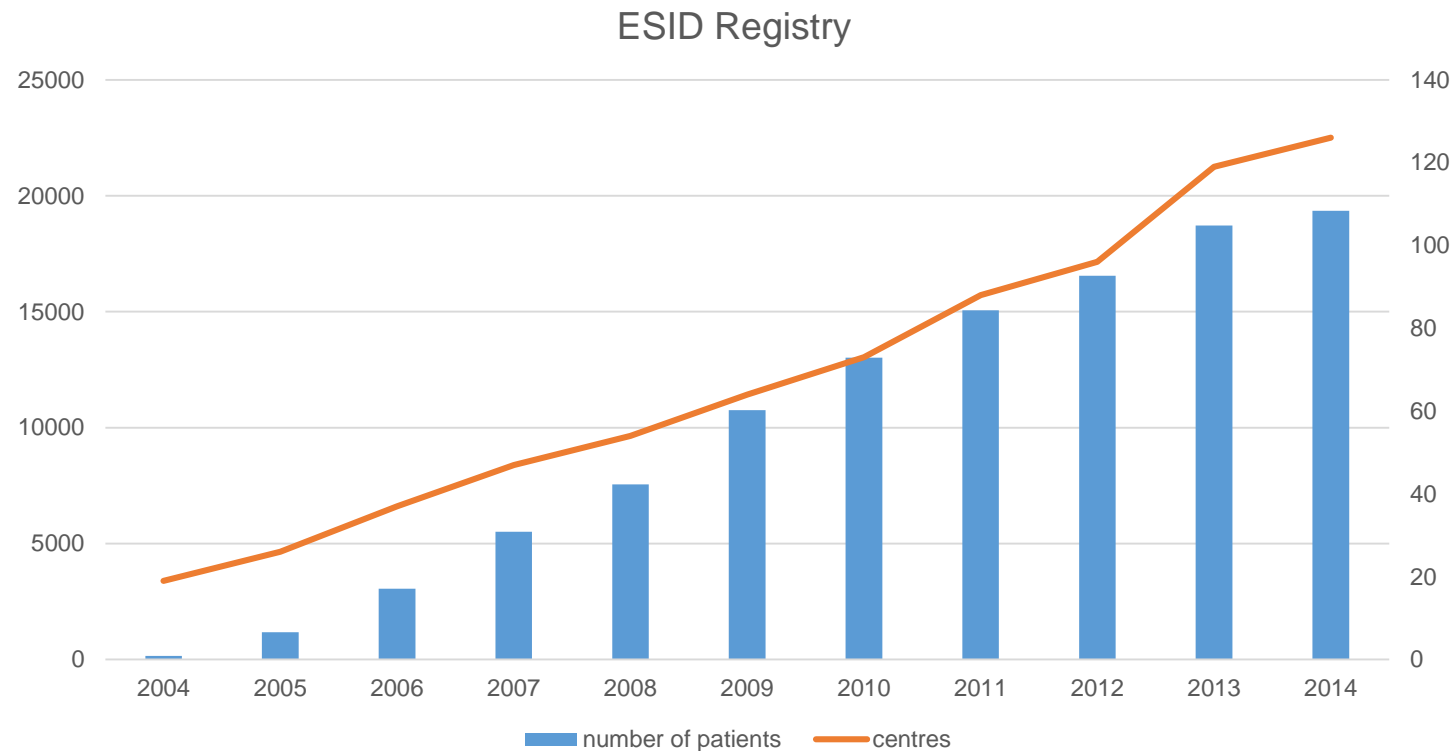
- European Reference Networks
- PID registries

EUROPEAN REFERENCE NETWORKS

- 2012: Only 5 countries had designed centres of reference for PIDs (FR, IT, SE, UK, DK).
 - In many countries: PID patients go through an ordeal for getting diagnosed and treated!
- 2016: IPOPI facilitated initial discussions of top physicians to develop Rare immunological and auto-inflammatory diseases ERN

PID REGISTRIES

ESID Registry (2004) - example in other parts of the world



Source: ESID Registry (www.esid.org)

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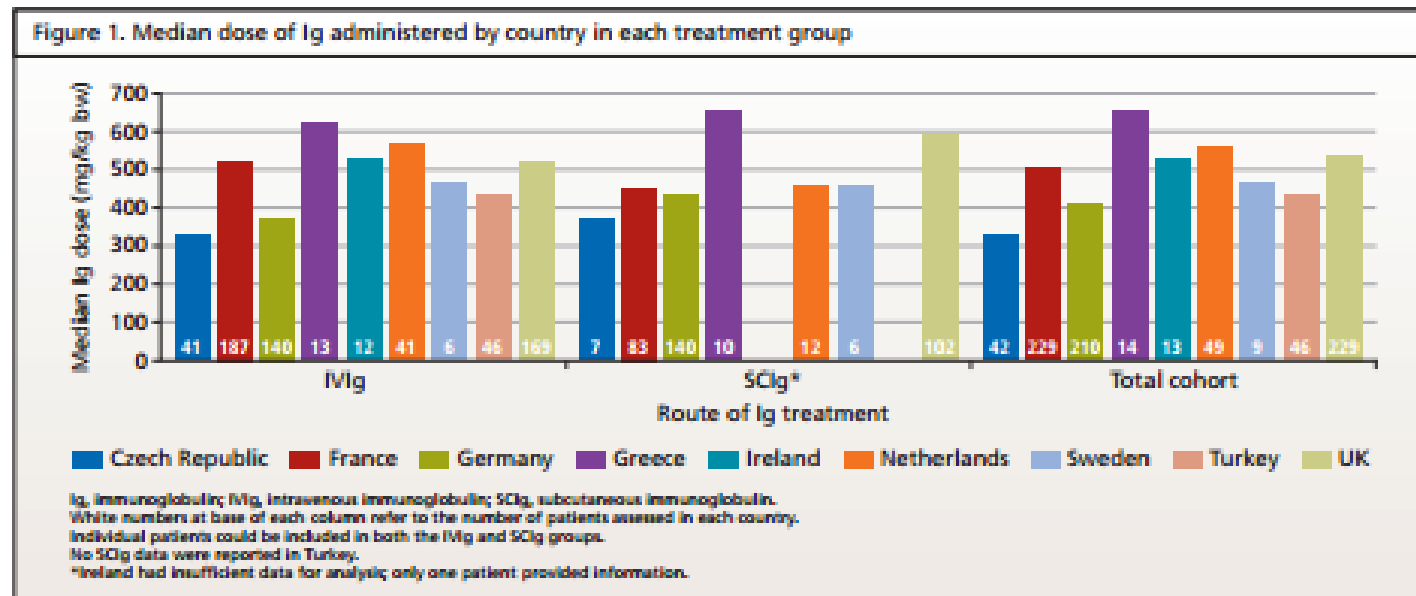
- Crossborder healthcare
- Health Technology Assessment
- Advanced Therapies
- Blood legislation



CROSSBORDER HEALTHCARE TO BRIDGE HEALTH INEQUALITIES

Disparities treatment and care worldwide & even in the EU!

Differences in Ig replacement therapy dosing in patients with CVID in Europe



Source: Results from the ESID Database (2011).

HEALTH ECONOMICS, HTAs & PIDs

- PID patients tolerate certain products more than others.
 - Igs are biological plasma-derived products → different products
 - Need to ensure that patients have access to a wide range of products so as to receive the most suitable for them.
 - Patients should remain with their specific Ig product unless a change is required CLINICALLY.
 - IPOPI BURDEN OF TREATMENT STUDY: 400 patients in 11 countries in 2016
- Igs are treated as similar biological products in many European countries:
 - Hospitals tenders where price is THE important criteria
 - Only one or two Igs are available for doctors to prescribe
 - Patients are switched from Ig to Ig not for their tolerability but for the price

*Importance of patient & doctor collaboration
to secure the right treatment*

ADVANCED THERAPIES & BLOOD LEGISLATION

- PIDs research is evolving rapidly
- Recent EMA positive opinion for licensed gene therapy for ADA-SCID
- SCIDnet

→ Need to ensure that future legislation revisions take into consideration the needs of the patients receiving the therapies:

i.e. Blood legislation:

blood donation \neq plasma donation

labile blood products \neq stable plasma products

no obstacles to free movement of PDMPs

THANK YOU FOR YOUR ATTENTION!