



IPOPI 4TH REGIONAL ASIAN PID MEETING

19-20 NOVEMBER 2022
KUALA LUMPUR, MALAYSIA

an **IPOPI** event

CARE FOR RARE
GLOBAL ALLIANCE EDUCATIONAL SEMINAR



SUPPORTING ORGANISATIONS



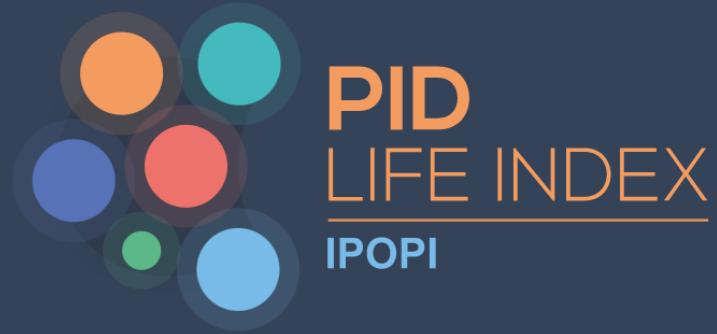
SUPPORTED BY



Access to treatment – An overview based on the PID Life Index

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The PID Life Index aggregates information submitted voluntarily by IPOPI national member organisations and by medical contacts. While not exhaustive, the PID Life Index sheds light on the worldwide PID environment. IPOPI welcomes any feedback to help optimizing this index.

Management of PID :

What are the principles of care?

Registries

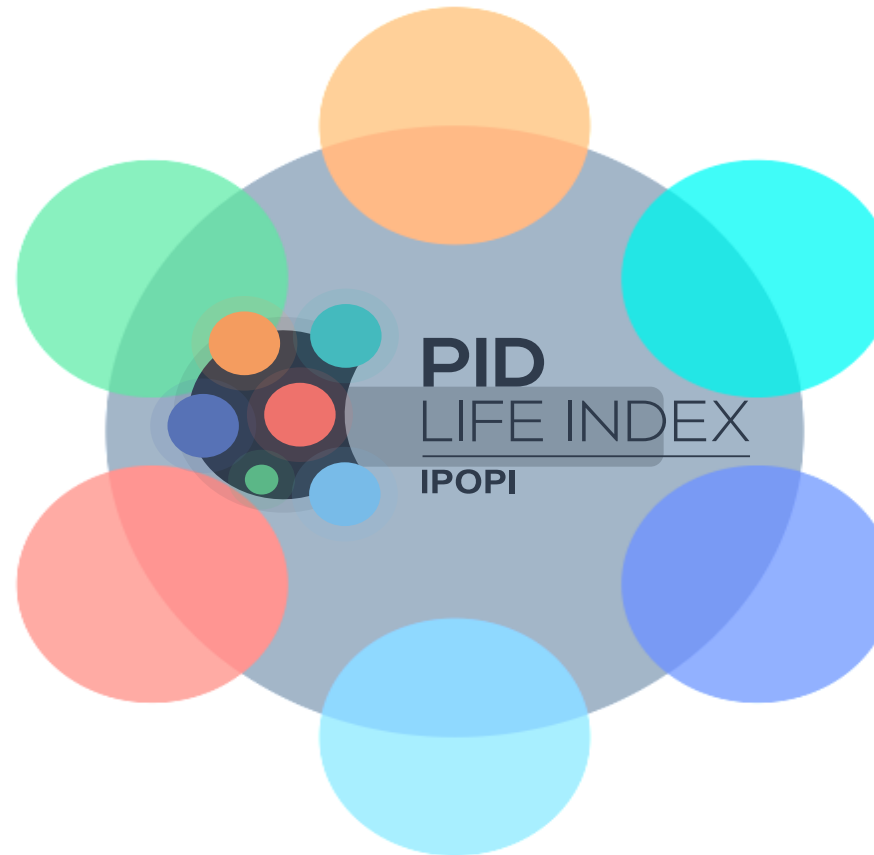
- National PID registry
- Bone marrow donor registry

National Patients Organisations

- Established national group in country
- Professional paid staff
- Main working areas

Specialised centres

- National PID specialised centre/network
- Adult PID services
- Transition care



PID Diagnosis

- Diagnosis rate
- Biological diagnosis availability
- Genetic diagnosis availability
- Prenatal diagnosis availability
- Newborn screening for SCID

Treatments

- Anti-infectious availability
- Ig availability
- Vaccines availability
- Curative treatments
- Biological and targeted therapies
- Plasma collection

Universal health coverage

- Diagnosis Reimbursement
- Treatments Reimbursement

RESEARCH

Open Access

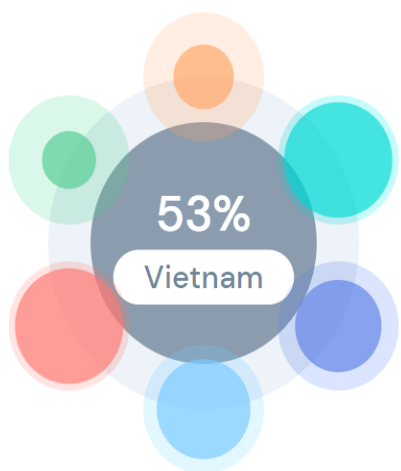
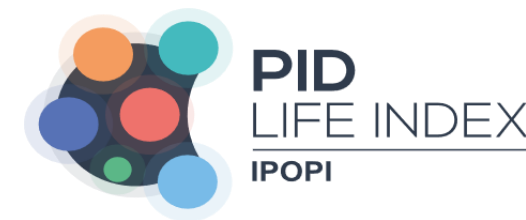


The PID Life Index: an interactive tool to measure the status of the PID healthcare environment in any given country

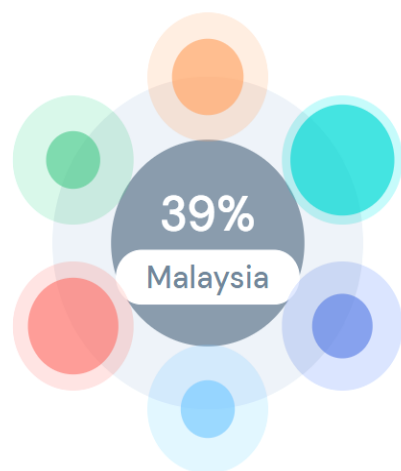
Leire Solís¹, Julia Nordin¹, Johan Prevot¹, Nizar Mahlaoui^{2,3}, Silvia Sánchez-Ramón^{4,5}, Adli Ali^{6,7}, Elodie Cassignol⁸, John W. Seymour^{1,9} and Martine Pergent^{1*} 

Where are SEAs in PID management?

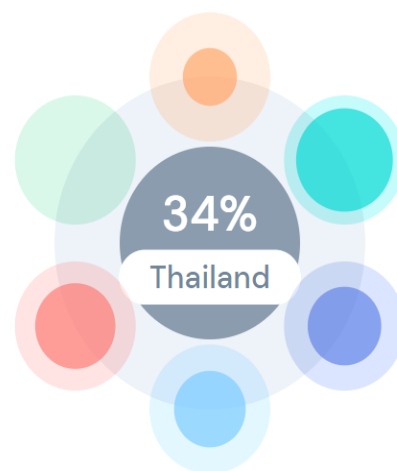
Data from IPOPI PID Life Index



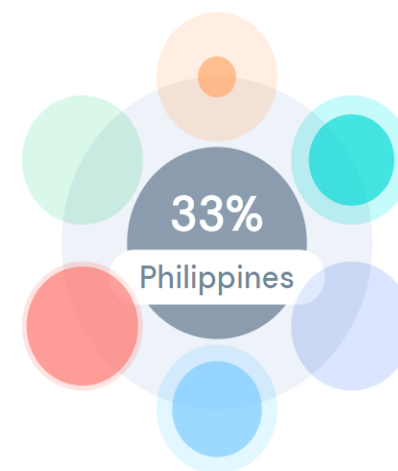
26th



41th



42nd



44th



47th

out of 62 contributing countries*
Data extracted on May 2021

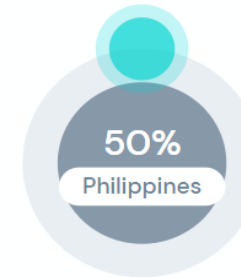
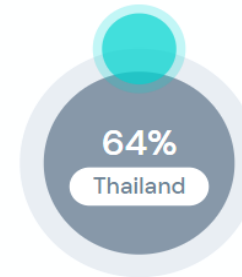
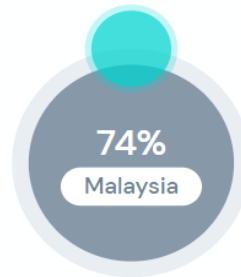
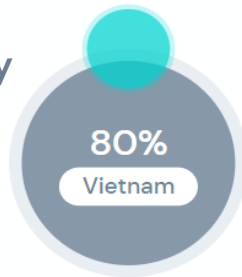
Where are SEAs in PID management?

Data from IPOPI PID Life Index



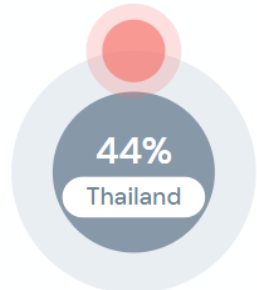
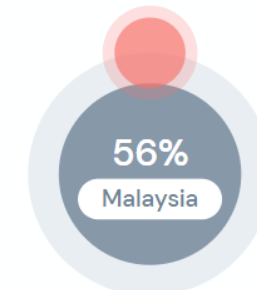
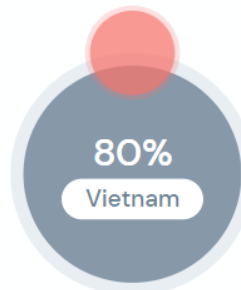
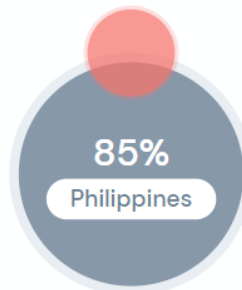
Treatments

- Anti-infectious availability
- Ig availability
- Vaccines availability
- Curative treatments
- Biological and targeted therapies
- Plasma collection



National Patients Organisations

- Established national group
- Professional paid staff
- Main working areas

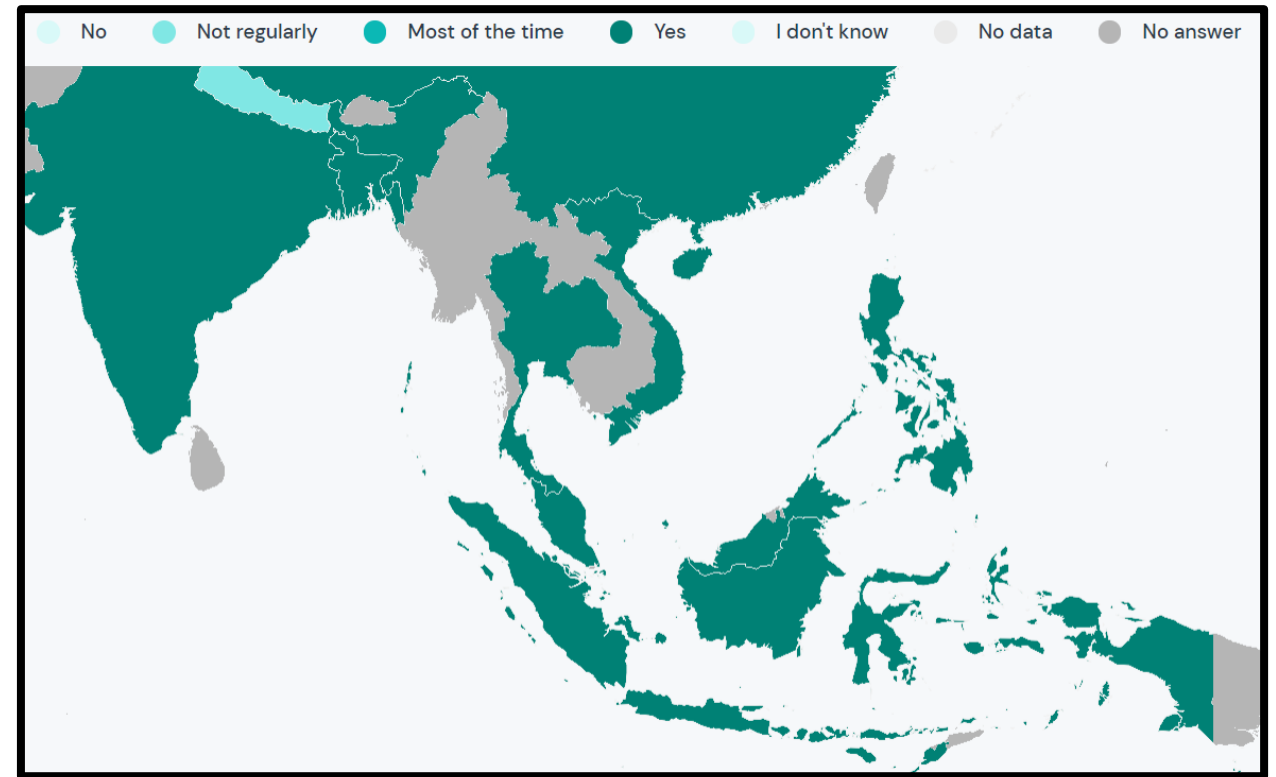


PID Treatment: IVIg availability



Intravenous immunoglobulin therapy

Immunoglobulin therapy administered intravenously is given straight into the bloodstream through a vein. The immunoglobulin is administered by a slow infusion directly in the vein and it can be delivered intravenously by gravitation or through a pump.

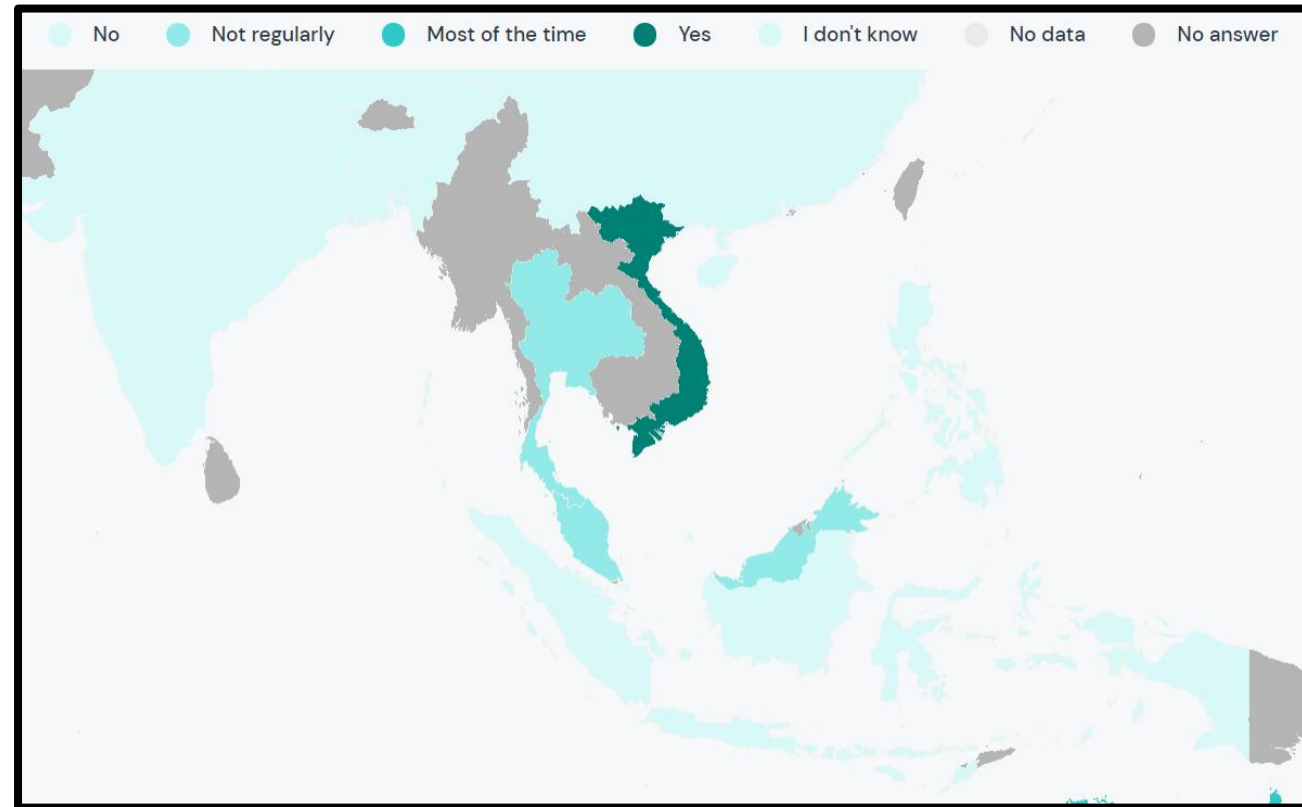


PID Treatment: SCIg availability



Subcutaneous immunoglobulin therapy

Immunoglobulin therapy administered subcutaneously is given under the skin by a slow injection through a device that controls the speed of infusion. Immunoglobulin can be delivered using a mechanical infusion pump or a rapid manual push (the infusion is pushed by hand through a syringe, not requiring a pump).



PID Treatment:

Curative treatment (BMT/ HSCT)



Bone marrow transplantation or haematopoietic progenitor cell transplantation

Specialised treatments in which HSC of an HLA compatible donor are provided to patients with usually severe PIDs (SCID, severe CGD) when the immune cells are missing or malfunctioning.

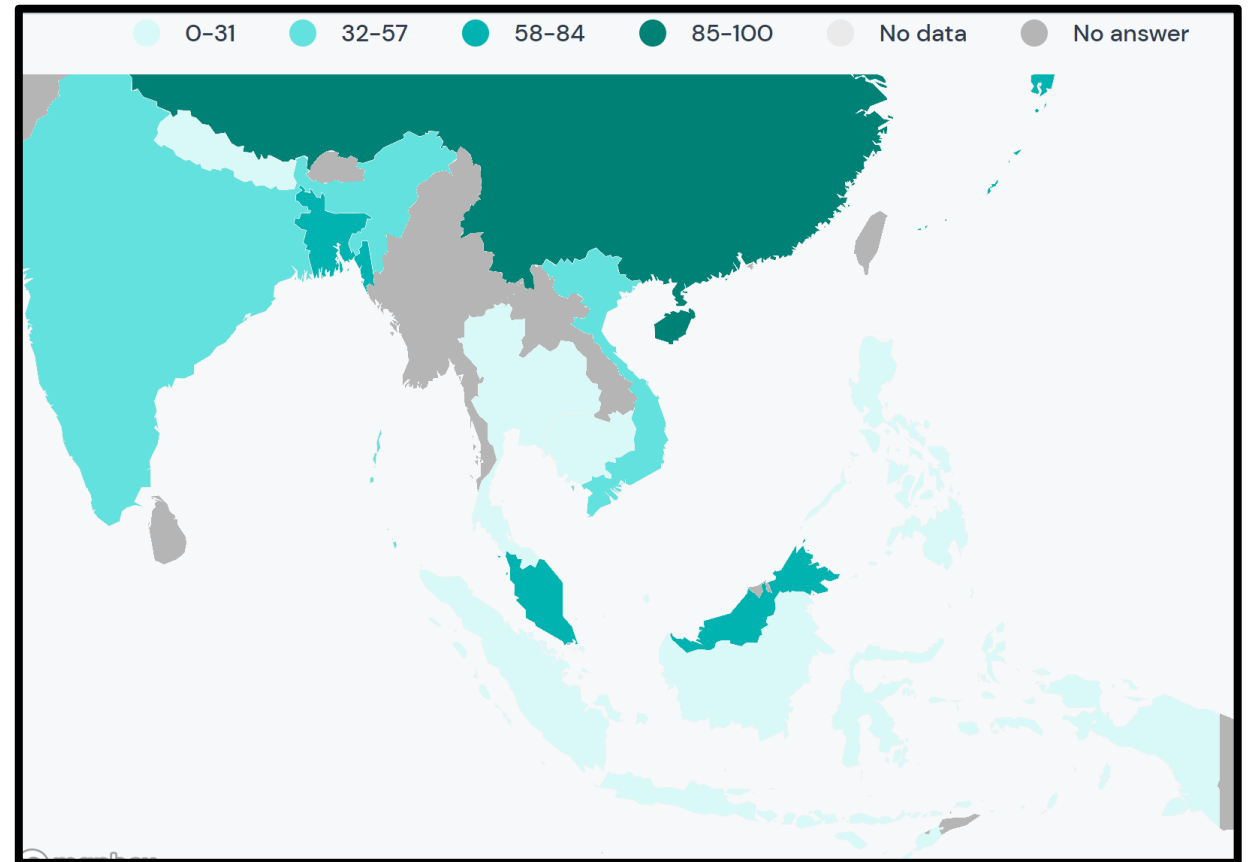
- **Gene Therapy**
- **Thymic Transplant**



PID Treatment: Biological & Targeted Therapies



- Enzyme Replacement Therapy for ADA SCID
- Immunosuppressors/ Immunomodulators
- Monoclonal antibodies
- Cytokines/ Interleukins
- Growth Factors (G-CSF/ EPO)
- C1 inhibitor concentrate
- Thrombopoietin receptor antagonist

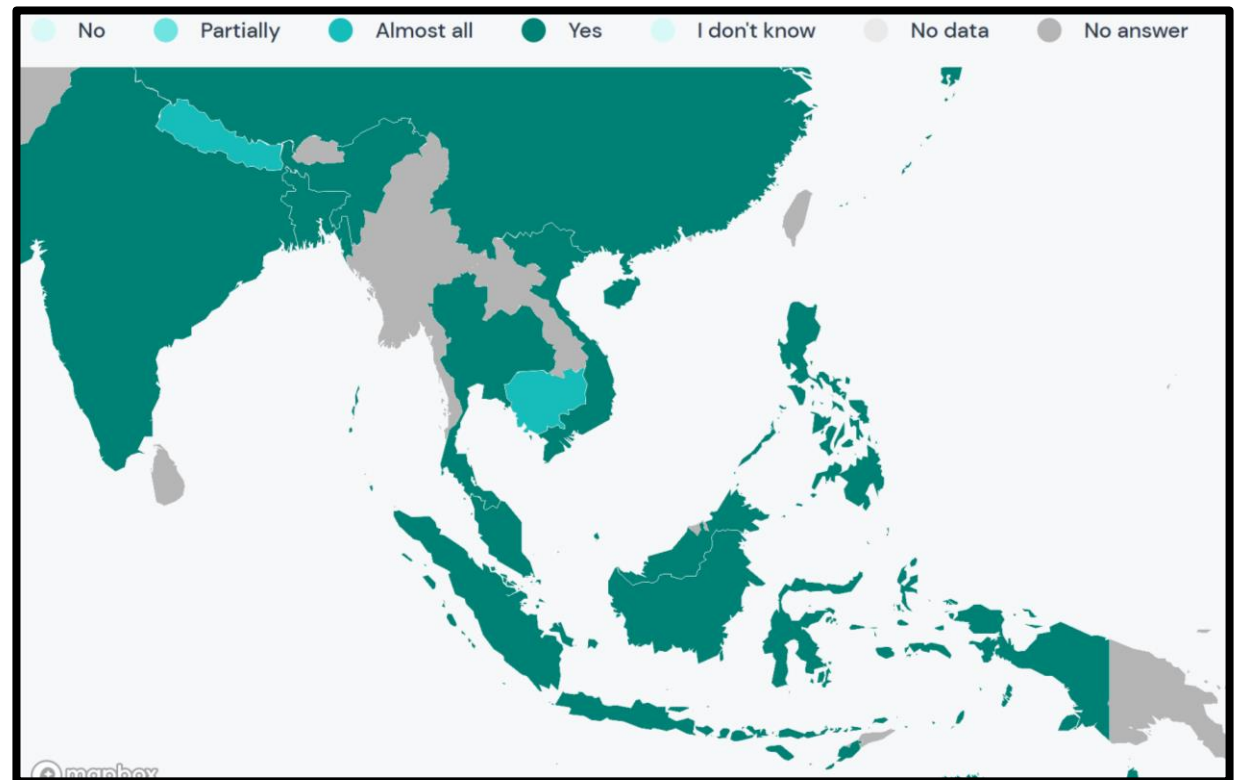


PID Treatment: Anti-Infectious Availability

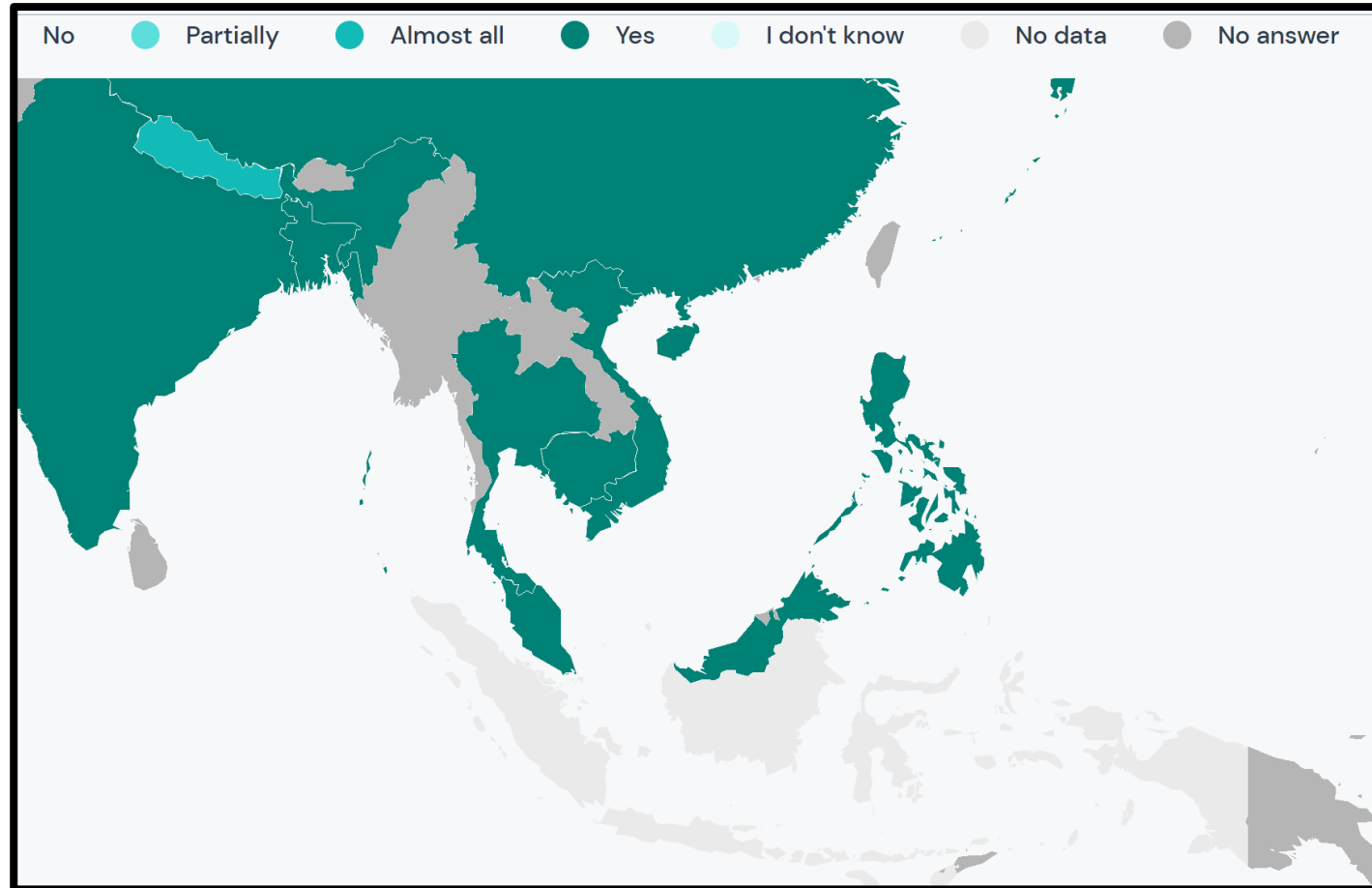


Anti-Infectious Availability

- Antibiotics
- Antifungals
- Antivirals
- Antiparasitics



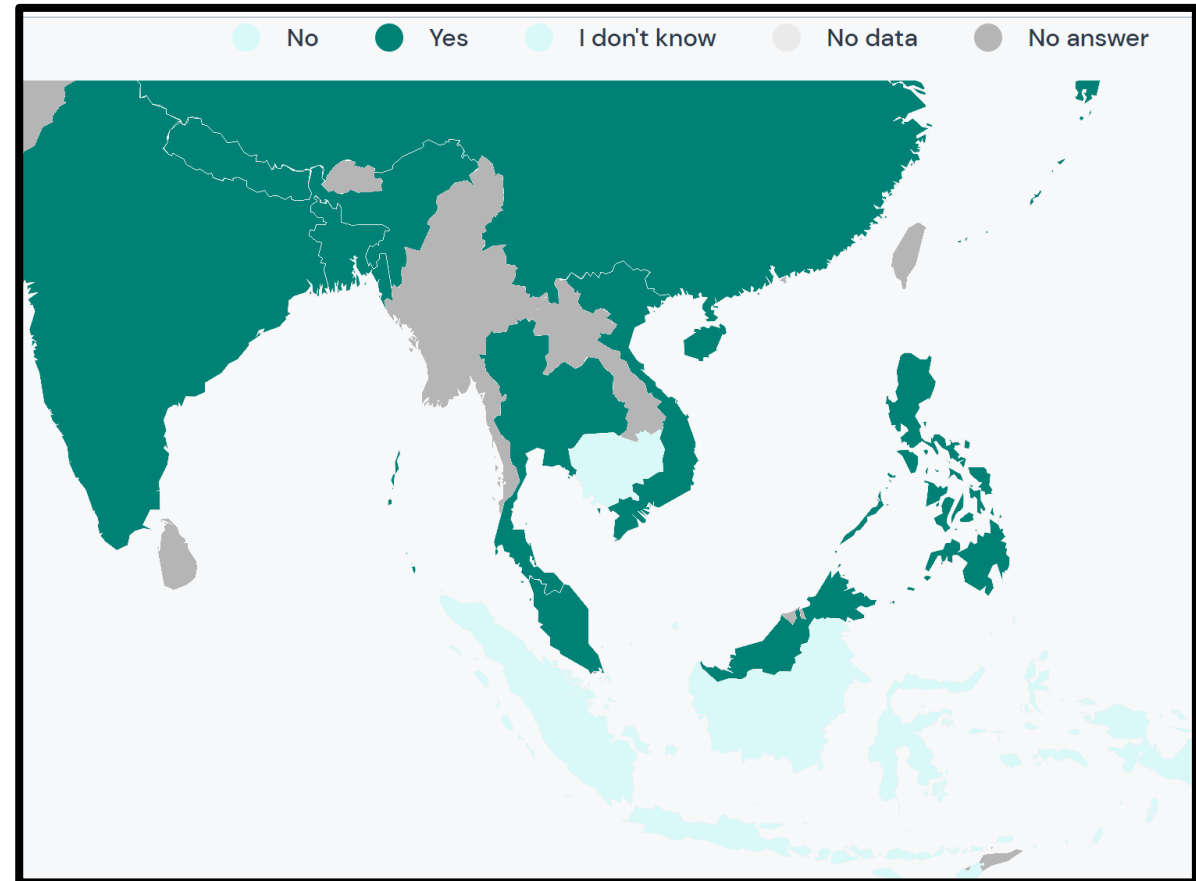
PID Treatment: Vaccines Availability



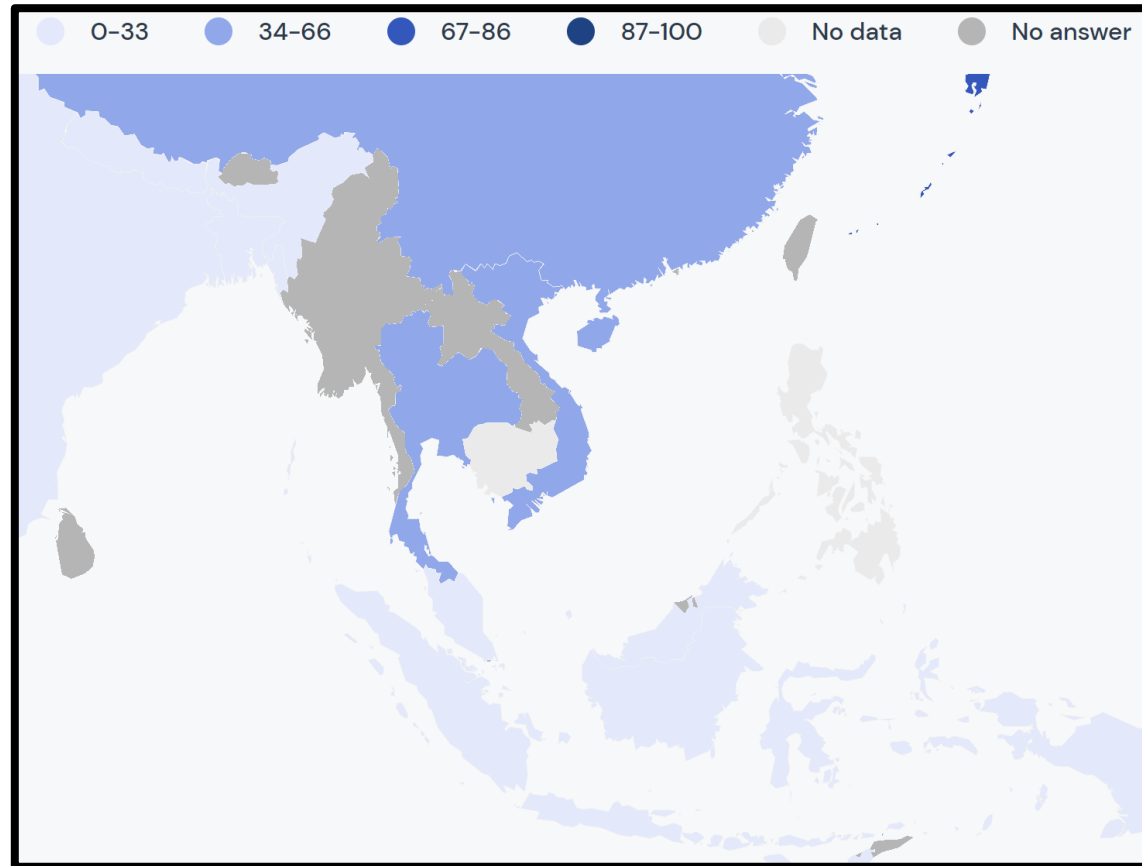
PID Treatment: Plasma Collection



- Availability of reliable collection system for plasma for fractionation
- **Availability of reliable collection system for plasma for fractionation**



PID Treatment: Universal Health Coverage





The PID Principles of Care: Where Are We Now? A Global Status Report Based on the PID Life Index

*Julia Nordin^{1†}, Leire Solís^{1†}, Johan Prévot¹, Nizar Mahlaoui^{2,3}, Helen Chapel⁴,
Silvia Sánchez-Ramón^{5,6}, Adli Ali^{7,8}, John W. Seymour^{1,9} and Martine Pergent^{1*}*

Conclusions



- ❖ **PID Management in SEA had progressed significantly**
 - ❖ Accelerated progress and interests on PID over the last 6 years

- ❖ **IPOPI PID Life Index: informative tool for assessing PID care and management**
 - ❖ Amazing platform for information, awareness and support advocacy initiatives on PID

- ❖ **NMO and SEAPID doctors need to strategize to ensure broad accessibility to treatment for PID patients**
 - ❖ Use PID Life Index platform for advocacy and engagement