



IPOPI 5TH REGIONAL ASIAN PID MEETING

24-25 MARCH 2024

TOKYO, JAPAN

an **IPOPI** event

COLLABORATION



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WELCOME TO IPOPI 5TH REGIONAL ASIAN PID PATIENTS' MEETING



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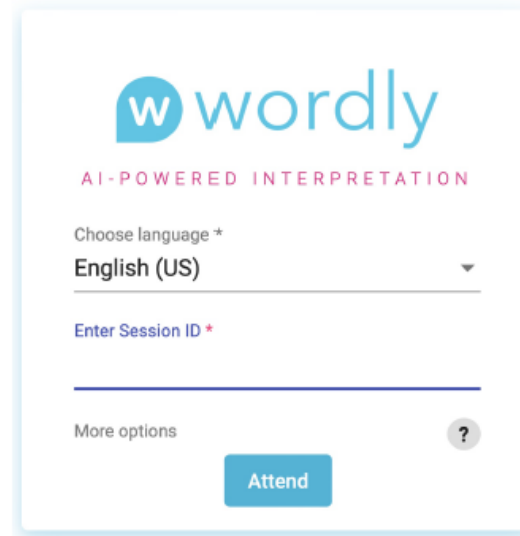
Live Translation

Step 1



Scan QR Code or Go To:
<https://attend.wordly.ai/join/KPYA-6720>

Step 2

A screenshot of the Wordly web interface. At the top is the 'wordly' logo in blue. Below it, in pink, is the text 'AI-POWERED INTERPRETATION'. There are two input fields: 'Choose language *' with a dropdown menu showing 'English (US)', and 'Enter Session ID *' with a text input field. Below these is a 'More options' link and a question mark icon. At the bottom is a blue 'Attend' button.

Choose Language
Click Attend

Step 3



Read Captions on Device
Use Headset for Audio

SESSION 1 | WELCOME SESSION

Moderator:

Ms Martine Pergent | IPOPI President



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WELCOME REMARKS

Ms Martine Pergent | IPOPI President



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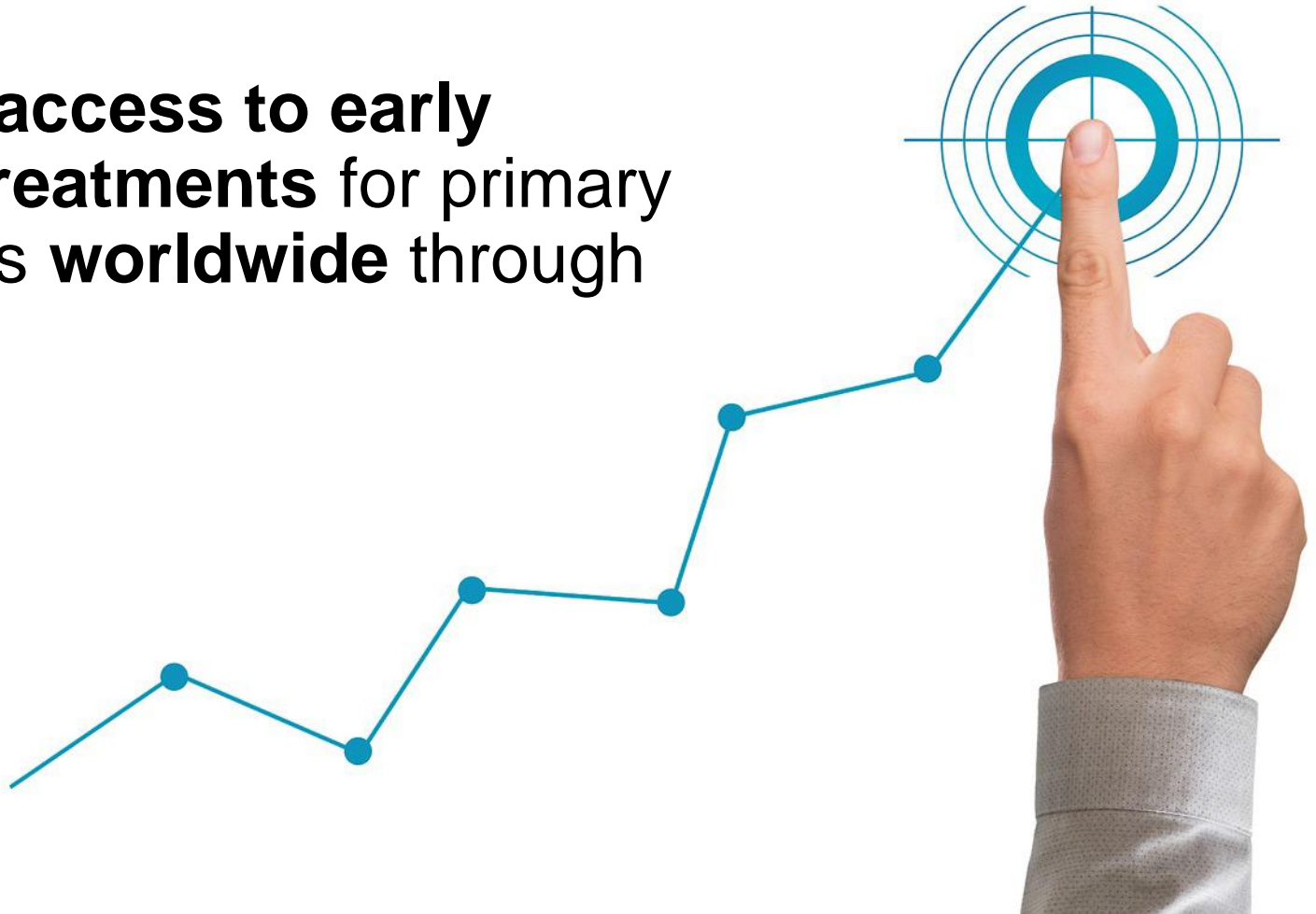
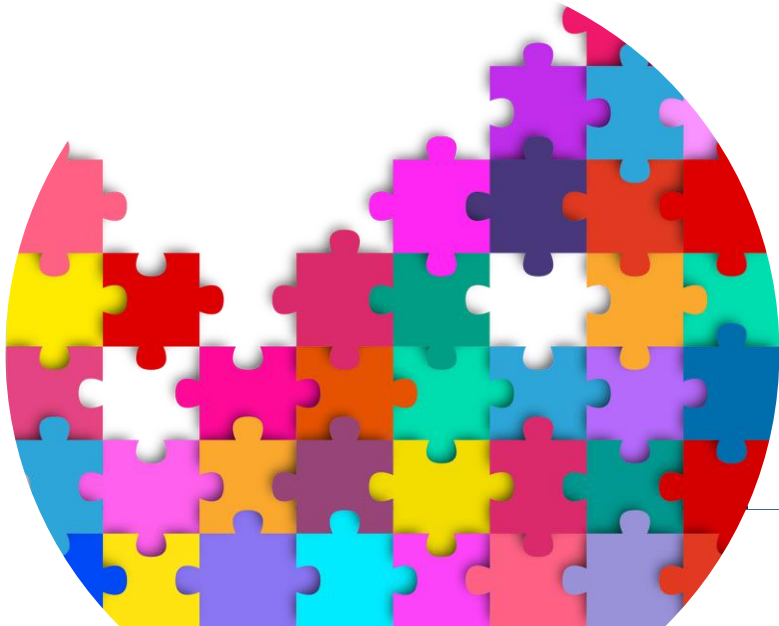


What is IPOPI?

- a **non-profit international** organization
- the **leading advocate** for primary immunodeficiency (PID) patients worldwide
- working in **collaboration** with patients, doctors, politicians, regulators, pharmaceutical industry and other relevant stakeholders.

Our mission

To improve awareness, access to early diagnosis and optimal treatments for primary immunodeficiency patients worldwide through global collaboration.



IPOPI works as an umbrella organisation

A vibrant global
community



Zoom on the Asian region

Bangladesh
Cambodia
China
Hong Kong
India
Indonesia
Japan
South Korea
Malaysia
Nepal
Philippines
Thailand
Vietnam





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IPOPI dynamic Staff



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Chairman (FR)



Prof Martin van Hagen
Vice-Chair (NL)



IPOPI LeBien AWARD

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- Professor Steven Holland – (USA)
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- **Prof Stuart Tangye (Australia)**
- Prof Klaus Warnatz (Germany)



Improve access to early
diagnosis & patient-centred care

an IPOPI event

IPOPI

activities & projects



Build capacity and support IPOPI's
national member organisations



Educate, promote knowledge
and data sharing



Strengthen multi-stakeholder
collaboration



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A patient centred approach



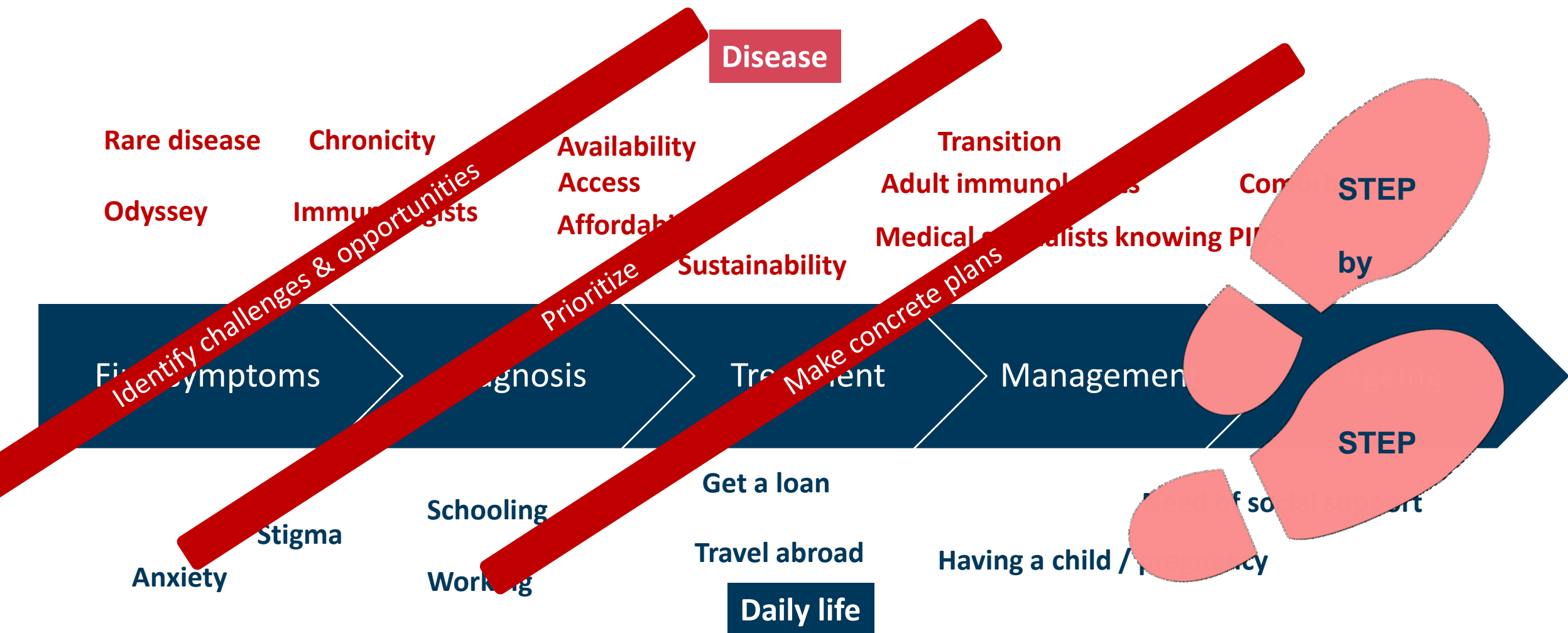
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What does a PID patient journey look like?



Patients' voice
can be very powerful!

Welcome to Tokyo

Ms Emi Homareda | PID Tsubasa-no-Kai



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Welcome remarks from sponsors

Takeda, Pharming, Grifols and Chiesi



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Inspire & Connect: Regional updates

Ms Miriam Ferreira | IPOPI NMO Programmes Officer



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Inspire & Connect: Regional updates

Mr Mohammad Iqbal Hossain,
Primary Immunodeficiency Patients Society of
Bangladesh (PIDPSB), Bangladesh



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Bangladesh – PIDPSB

Challenges:

We have two main challenges in Bangladesh: 1) Increasing awareness and 2) Ensuring quality healthcare of PIDs

Summary of the action:

- V2K30 & V2K40- In and around 2040 PIDPSB would like to work with Hospitals, Doctors, Nurses, University Teachers & Students, Schools, TV & Print Media as long Digital Media Campaign.
- PIDPSB would like to assist PIDs to ensuring affordable and appropriate medical care for them.
- Taking steps for Fundraising
- Asking help from Ministry of Health for PIDs
- Trying to get other support from the government of Bangladesh

Financial support:

Till now we don't have any sponsor. PIDPSB need yearly financial support to grow-up and work for PID patients

Outcomes:

- V2K30: 10 Million People will know about Primary Immunodeficiency; Ensuring Treatment for PIDs in Divisional/District Hospitals
- V2K40 :100 Million People will know about Primary Immunodeficiency; PID Patients in Leading healthy, Beautiful and Normal Life

Inspire & Connect: Regional updates

Ms Jessie Qu,
PID Care China



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PID CARE CHINA



Challenge:

Currently, the immunoglobulin shortage is a pressing issue for our patients, with about 80% unable to access IVIG treatment for various reasons. So, we decided to make a video to raise awareness and educate the public on why immunoglobulin is crucial for our patients.

Summary of the action:

In January 2024, PID Care China released an advocacy video titled "Calling for Immunoglobulin." The video focuses on the challenges faced by PID patients due to a shortage of immunoglobulin. We had 12 participants in the video recording, including patients, caregivers, doctors, and volunteers. Each participant recorded their segment, and we had a volunteer edit everything together.

Financial support:

ZERO Cost

Outcomes:

After its release, the video received over 3000 views on the same day. This caught the attention of a local plasma product company, which offered to help our patients access immunoglobulin. Additionally, a foreign plasma company expressed interest in exploring collaboration opportunities. I believe this project is a great example of how organizations with limited budgets can still make a significant impact.

Inspire & Connect: Regional updates

Mr Adhi Sugiharto,
Indonesian Primary Immunodeficiencies Patients Society
(IPIPS), Indonesia



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Indonesia PID Patients Society (IPIPS)

Challenge

Awareness (only 70 patients are diagnosed properly), **Limited Diagnostic** (2 out 2.5k hospital can diagnose and treat PID) and **therapies options is very limited** (Govt. hospital recently implement upper limit cap for IVIG usage).

Summary of the action

1. **WPIW 2024:** continue awareness campaign with National Medical University Resident Doctors and live broadcast of patients talks in our IG and FB (22 April 2024).
2. **Target National Media for Awareness:** to release press kit and information about PID, its challenges and how government can support to National Media (May 2024).
3. **Advocacy Letter to Minister of Health:** to press the needs of IVIG to be treated as essential medicine for PID patients and requesting time to meet (April 2024).

Financial support

IPOPI (remaining fund from WPIW 2023) and private donor (IPIPS members).

Target Outcomes

1. Media Coverage of PID / IPIPS in national media as an indicator of increased in awareness.
2. Respond and acceptance from MOH for audience / meeting.



Inspire & Connect: Regional updates

Ms Emi Homareda,
PID Tsubasa no Kai, Japan



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Japan - PID Tsubasa no Kai

Challenge:

- Advocating for newborn mass screenings for PID (since 2014)

Summary of the action:

- Flyer distribution program (since 2014)
- Hosting lectures by medical advisors (2019 & 2021)
- Attending the academic meeting of the Japanese Society for Neonatal Screening (2021)
- Speaking at a civic lecture to promote newborn mass screening (2023)
- Lobbying & Submitting a request to the Minister (2023)

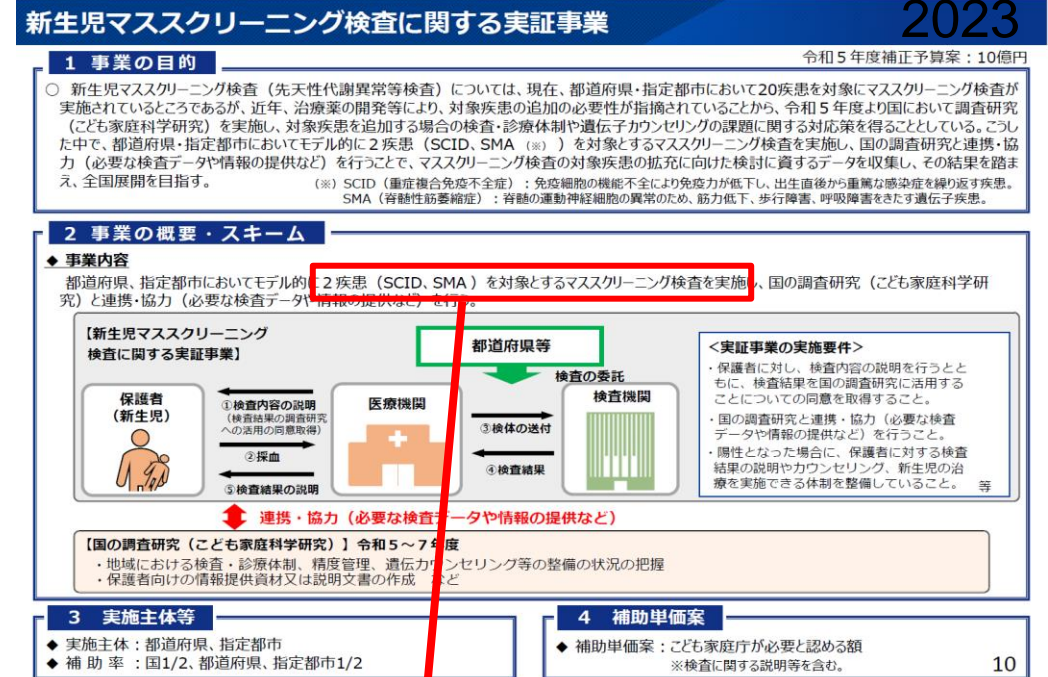
Financial support:

- Paid by our fund composed of membership fees, donation from supporting members and “vending machines”
- Supporting member system began (2008)
- Introduction of donation vending machine (2020): to date, 4 such machines deployed

Outcomes:

- Newborn mass screenings available in 40 prefectures, and some children could start treatment before getting infected (e.g. SCID in Aichi, XLA in Tokyo)
- Children and Families Agency approved the subsidies for half of the expense of newborn mass screenings for SCID and SMA. Regional disparities are expected to be eliminated.

The Children and Families Agency Supplementary budget for fiscal year



Mass screening will be available for SCID and SMA.

Inspire & Connect: Regional updates

Mr Bruce Lim,
Malaysia Patient Organization of Primary
Immunodeficiencies (MyPOPI), Malaysia



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Malaysia —

Challenge:

Access to national newborn screening for rare diseases.

Summary of the action:

- Reached out & Collaborate with other relevant stakeholders.
- Appoint a healthcare policy agency to develop & facilitate programme with aim to expand newborn screening for treatable rare diseases focusing on SCID and SMA.
- On November 18, 2023, organised a workshop with multiple stakeholders to map the challenges and action plan.
- On March 9, 2024, organised a roundtable discussion with key stakeholders in the Ministry of Health, physicians, and patient organisations for way forward.

Financial support:

- IPOPI – NBS Pilot Programme

Outcomes:

- MOH Key Stakeholders agreed to include PID & SMA into their work policy of extended National NBS policy to be tabled for MOH / Parliament approval.



Inspire & Connect: Regional updates

Mr Sandip Paudel,
NePOPII, Nepal



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Challenges

- Lack of awareness among professionals, doctors, health activists, patients, parents & public
- Gross lack of funds
- lack of support and innitiation
- No plan and policy, no initiatives for such diseases from the government
- Lack of diagnostic facilities for PID (e.g. Folwcytometry, Genetic tests)
- Lack of treatment modalities
- Lack of research due to funds, support, and ethical clearance from authority
- Limited Immunologists from Nepal in international conferences and forums
- Lack of health care facilities for all and lack of treatment due to poor affordability
- Misconceptions and stigma to PID, and immune dysregulation



Summary of the action

- Awareness/informative activities by the doctors
- Television and audio-visual media interviews of Dr Dharmagat Bhattarai
- Fundless diagnostic efforts
- Establishment of NePOPII
- Spreading words, and lobbying with provincial and central government
- Pleas made from immunologists, parents (PIDs) to international societies and forums

Financial Support

No financial Support yet
No sponsors

Outcomes

- Awareness increased from 2 to 6%, and the diagnosis of around 200 patients with PID/IEI (immunodeficiency and immune dysregulation diseases) with the reference to Dr. Dharmagat.
- We are continuing to knock doors of the public and the government for diagnosis, treatment, medicine.
- Establishment of NePOPII

Executive committee: Nepal Patient of Primary Immunodeficiencies and Inborn Errors Initiatives

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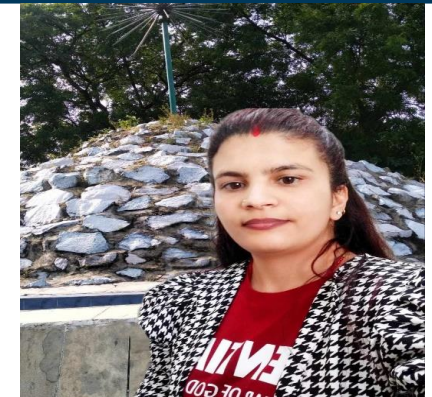
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Immunologist, Scientific Lead, and Patron
Dr Dharmagat Bhattarai, MBBS, MD, DM, FESID
(CHOP, USA)



- President- Sandip Paudel



- Vice-president- Shanta Subedi



Treasurer- Smriti Aryal



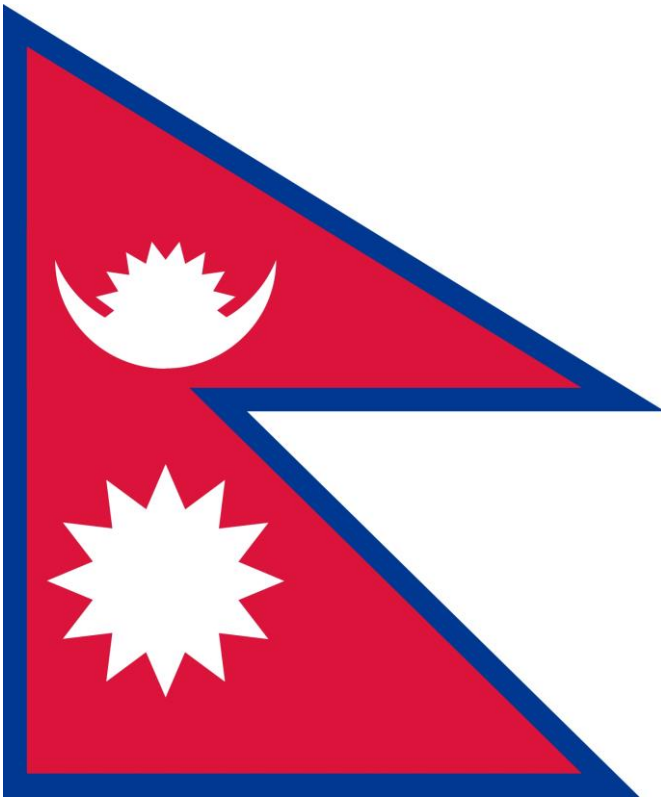
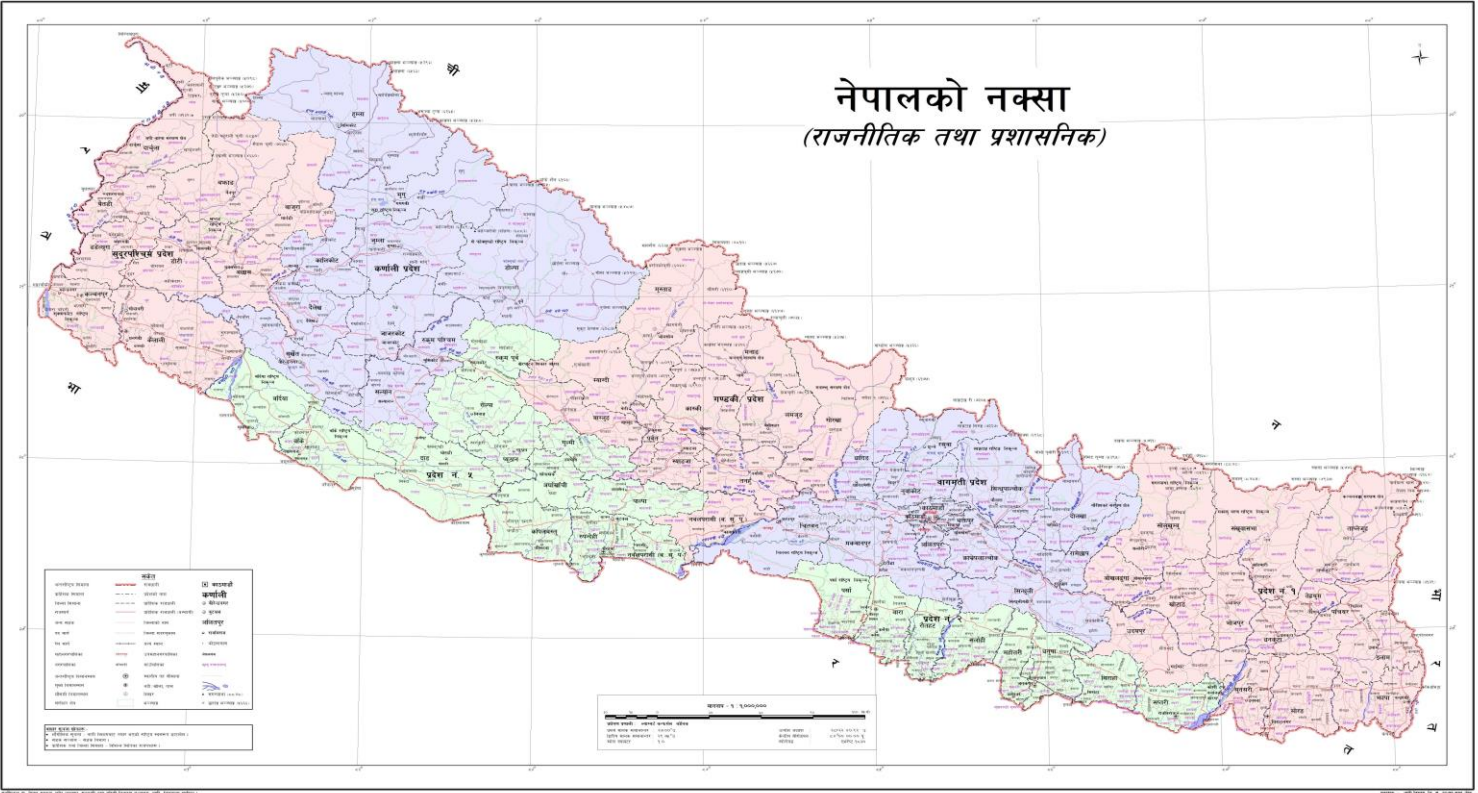
- Member- Narayan Lal Shrestha



- Member- Jhalak Aryal

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THANK YOU



Inspire & Connect: Regional updates

Mr Kevin Basaños,
Philippine Patient Organization for Primary
Immunodeficiencies (PhilPOPI), Philippines



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Philippines - Philippine Patient Organization for Primary Immunodeficiencies (PhilPOPI)

Challenge:

Late Diagnosis or Incomplete Diagnosis

- delayed or unclear test results
- expensive cost for diagnostic procedures

IvIG is an expensive life-saving treatment

- increase in cost of medication
- no increase in family income
- high out-of-pocket

Summary of action: Effects of PID on a patient having life-time treatment as quality of life suffers

- mental and psychological
- emotional impact on family
- financial impact
- education and work
- social

Financial support:

- Government through PhilHealth is slowly carrying out the universal Health care law.
- The outpatient drug benefit package and pooled procurement at state hospitals could be improved by increasing government funding for medicines and hospitalization.
- Families with no income have no line-up, follow a process, and hope and access to these Health benefits are granted.

Outcomes: Fund raising activities

- Patients and volunteer members already burneded with financial and health conditions cannot participate in the fundraising activities.
- No source of funds for PhilPOPI in 2023, except for IPOPI World PI Week awareness campaign

Inspire & Connect: Regional updates

Ms Jitchanwichai Narumon,
Thai Patient Organization for Primary Immunodeficiency
(ThaiPOPI), Thailand



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Thailand – Thai Patient Organization for Primary Immunodeficiency (ThaiPOPI)

Challenge:

Raise awareness among patients and the Thai government.

Summary of the action:

- *We provided souvenirs to the Thai Red Cross Society and distributed them to donors during WPIW to establish a connection with the Thai community.*
- *We've crafted a standardized document outlining 10 warning signs aimed at raising awareness across the Thai population..*
- *Create a podcast featuring a selected case study of a PID member, and launch it via Facebook.*
- *Collaborate with The Thai Red Cross Society to raise public awareness about rare diseases through impactful initiatives.*

Financial support:

No sponsor

Outcomes:

Gain attention and increase member participation.



10 Warning sign in Thai

Inspire & Connect: Regional updates

Mr Nguyen Tien Dat,
Vietnam Primary Immunodeficiency Patient Support
Group (VietPIPS)



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Inspire & Connect: Regional updates

Conclusions



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PID development in the last 2 years in Asia

Associate Professor Dr Adli Ali | Malaysia
Consultant Pediatric Immunologist
Universiti Kebangsaan Malaysia



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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*} 

IPOPI PID Life Index :

What are the principles of care?

Registries

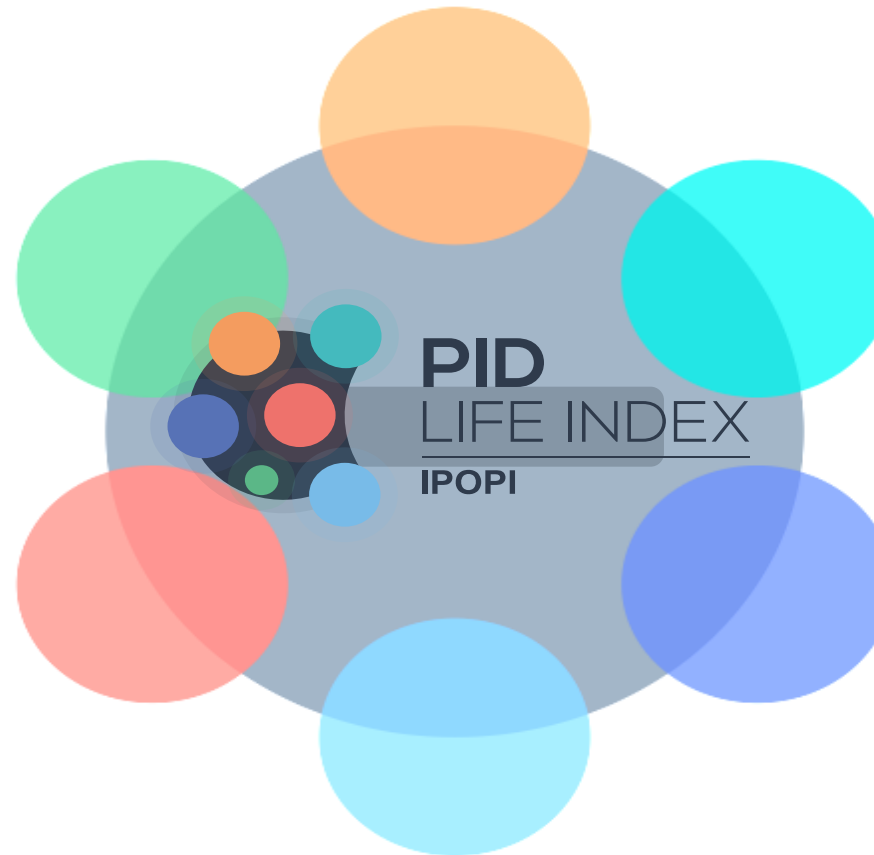
- National PID registry
- Bone marrow donor registry

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



WHAT ARE THE PROGRESS OVER THE LAST 2 YEARS?

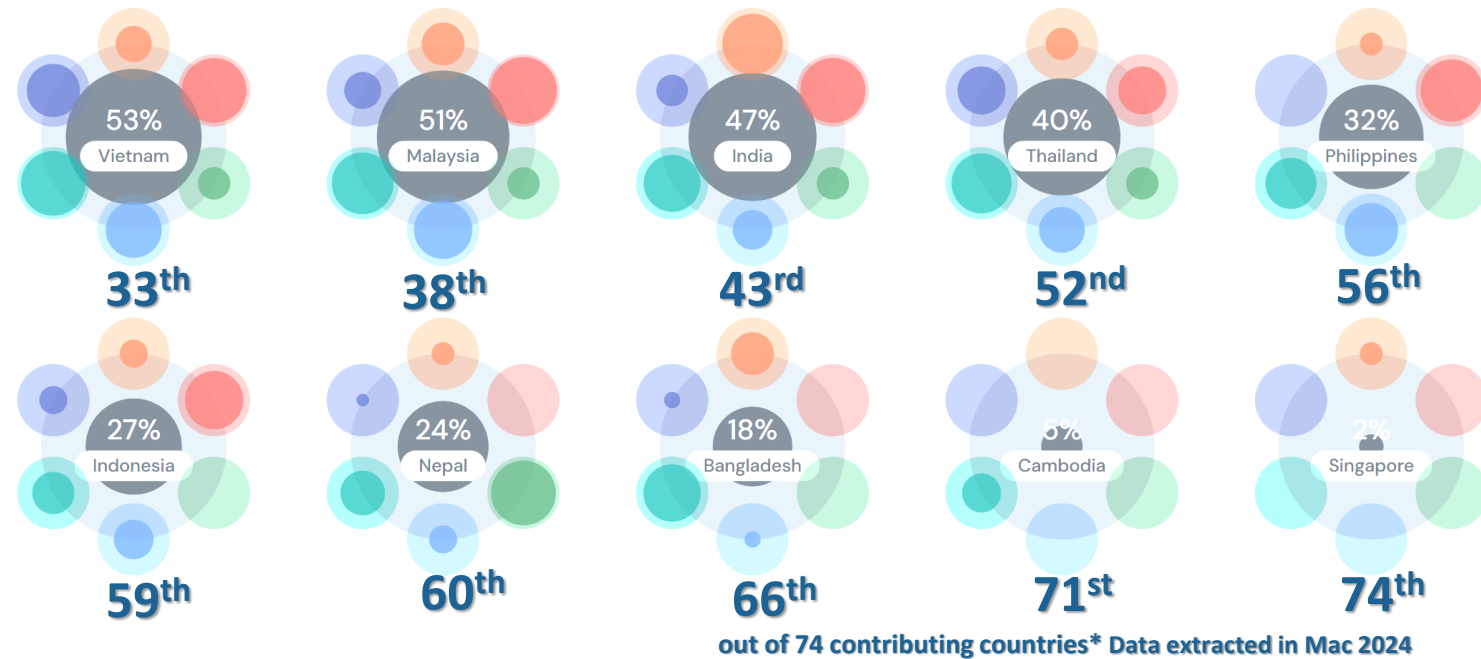
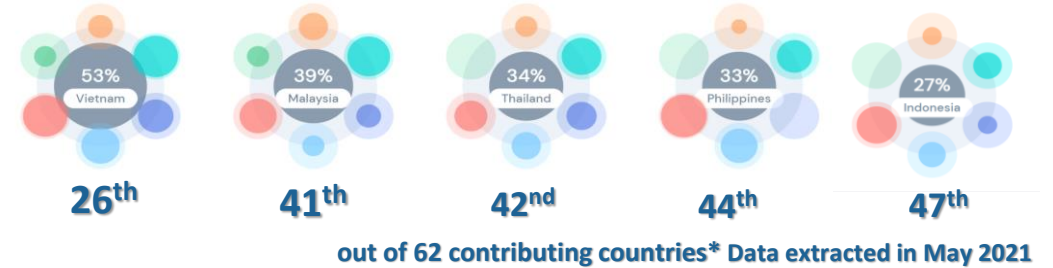
Where are Asia Pacific in PID management?

Data from IPOPI PID Life Index



- More Asia Pacific countries contributed

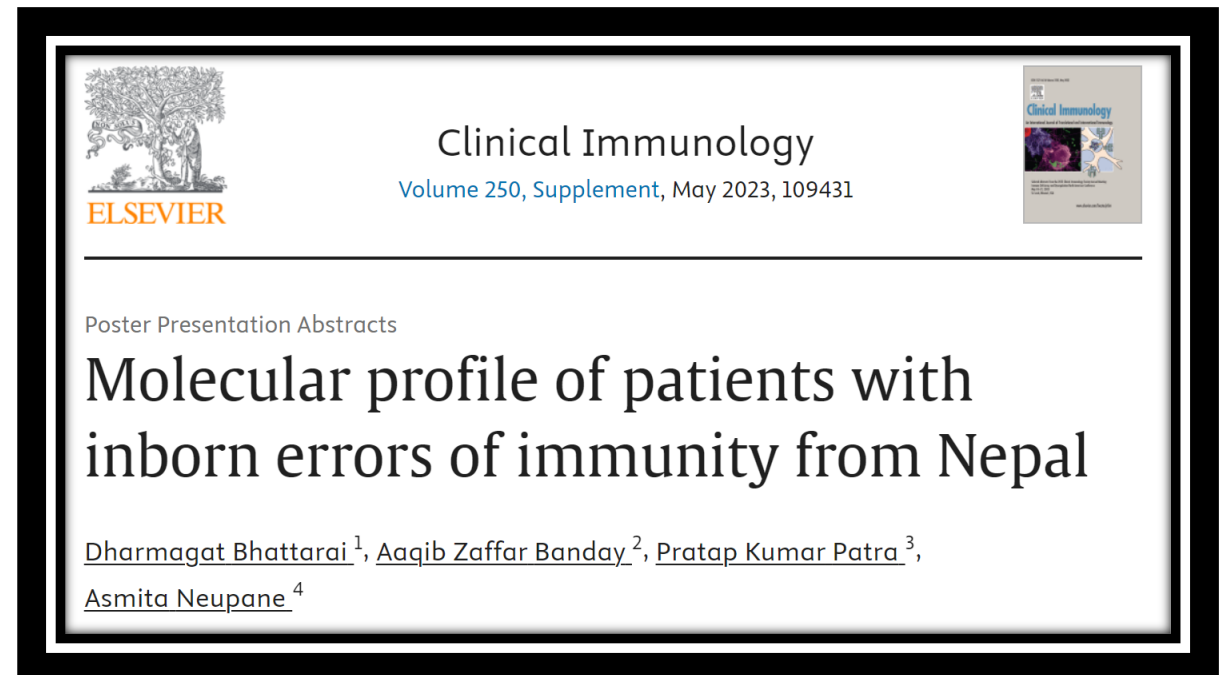
- *Nepal, Bangladesh, Cambodia*
- Improved in PID Life Index
- *Score: Malaysia, India, Thailand*
- *Ranking: Malaysia*
- Precise/ Regular Reporting
- *Singapore, Phillipnes, Indonesia*



PID STATUS IN ASIA: REGISTRIES

UPDATE OVER THE LAST 2 YEARS

- No proper registries still
 - Current status: National-based-database
 - Few recent publications
 - country based
 - Disease based
- Movement towards national-based-registries
 - Proper documentation-albeit simpler
 - Strategies: start with national interest
- Regional based registries
 - APSID→ dedicated working committee



PID STATUS IN ASIA: PATIENT ORGANIZATION

UPDATE OVER THE LAST 2 YEARS

- New associate member
 - Bangladesh (PIDPSB) and Nepal (NePOPI)
- More established working NMO
 - More activities/ advocacy
 - MyPOPI: Industry internship / partnership
 - Collaborative NGO



PID STATUS IN ASIA: SPECIALISED CENTRE UPDATE OVER THE LAST 2 YEARS

- More trained immunologist – More satellite unit
 - Training : IPOPI PIDetect (IPOPI-PGIMER)
 - National-based-fellowship
- Recognized center of excellence for PID?
- Adult immunologist – increased in number
 - More interest within other subspeciality
- Transition care: no proper pathway
 - developing consensus/guidelines



PID STATUS IN ASIA: PID DIAGNOSIS

UPDATE OVER THE LAST 2 YEARS

- Diagnosis rate: dramatically improved
 - better access to diagnosis (partnership)
 - cheaper / logistically-accessible test
 - Awareness and dedicated doctors
 - Repurposing/ Expanding test indication
 - Calculated globulin/ Isohemagglutinin
- Newborn screening for SCID
 - National advocacy initiative
 - Public-private-partnership
- Introduction of LDT/ RUO tests for wider screening?
 - Functional / Protein expression panel



PID STATUS IN ASIA: PID DIAGNOSIS

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 - Functional / Protein expression panel



PID STATUS IN ASIA: TREATMENT UPDATE OVER THE LAST 2 YEARS

- Immunoglobulin treatment
 - SCIg usage: wider / trans-countries?
 - More options
- Plasma collection
 - Better quality (UNITAR-Malaysia)
- Curative treatments
 - HSCT: more provider/ better international policy
 - Gene therapy? (Regional Viral Vector CDMO)
- Biological and targeted therapies
 - Extension indication for PID
 - Immuno-cytokine profiling for PID patients?



PID STATUS IN ASIA: UNIVERSAL HEALTH COVERAGE

UPDATE OVER THE LAST 2 YEARS

- Small movement in government-based-coverage
 - In Malaysia: National Rare Disease Committee
 - Guideline for Orphan Drug
 - Assessment using Health Technology
- Insurance company
 - Minimal movement in commercial based
 - Inclusion of NBS as part of maternal package?
 - Social based insurance?



FUTURE OUTLOOK: PID STATUS IN ASIA

IN THE NEXT 2 YEARS

- ❖ **Registries:** Standardized / Wider-national-based / Comparable data
- ❖ **Patients Organisation :** More structured and proactive organisations
- ❖ **Specialized Centre :** Integrated adult care with transition pathway
- ❖ **PID Diagnosis :** accessible cost-effective option / trans-countries COE (Reviving SEA6)
- ❖ **Treatment :** Improving equity / earlier access to novel treatment (Clinical Trial in Asia?)
- ❖ **Health Coverage :** ASEAN-WHO AP engagement / Commercial Insurance partnership

Q&A



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COFFEE BREAK

30 MIN

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