

# Immunoglobulin therapies supply and use - patient perspective -



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EDQM stakeholder event

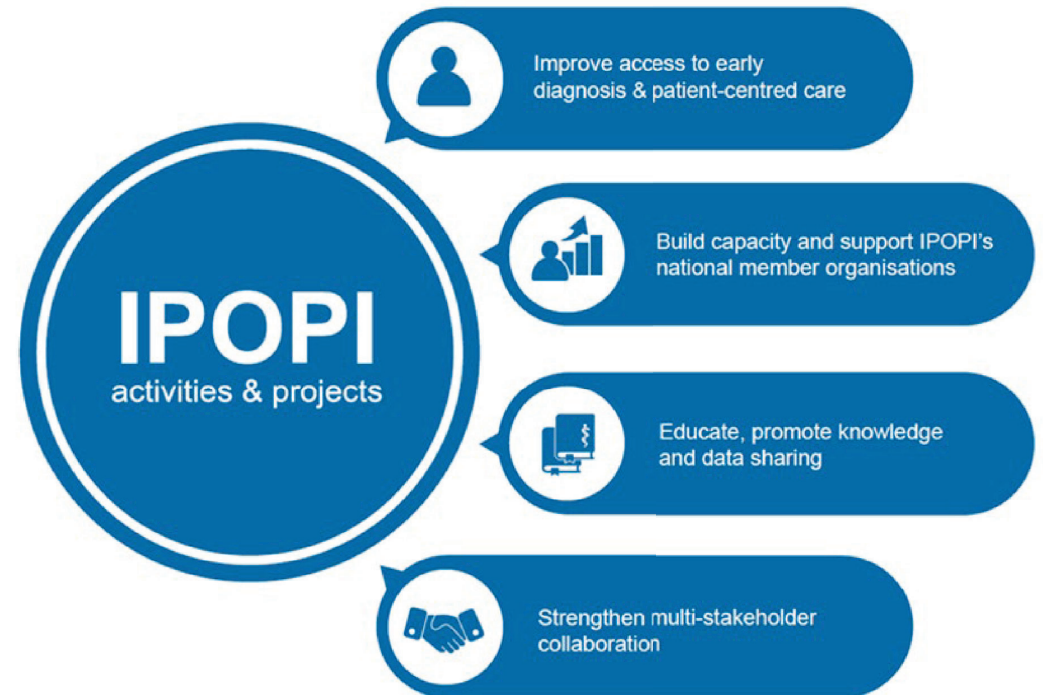
Strasbourg (France), 26-27 March 2025

# Introduction to IPOPI

The association of national patient organisations dedicated to improving:

- Awareness
- Access to early diagnosis
- Access to care

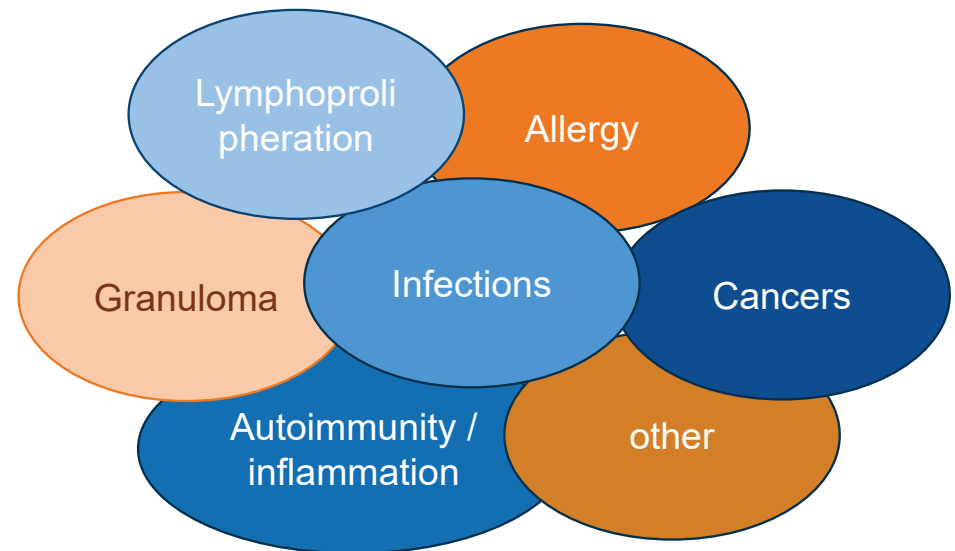
For patients living with primary immunodeficiencies (PIDs) worldwide



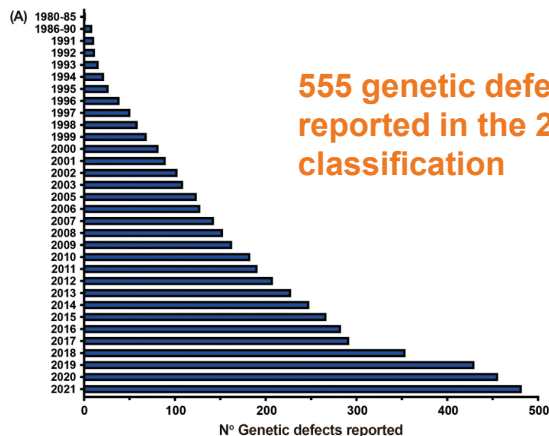
# Introduction to Primary Immunodeficiencies (PIDs)

- 550+ different **genetic rare** and **chronic** diseases
- The **immune system** does not work properly or at all
- Affect **children** and **adults**

- Clinical presentations are variable
- In many cases, **delay in diagnosis**
- **Different treatment options**, depending on the PID
- Patients with PIDs can have:



From: Human Inborn Errors of Immunity: 2022 Update on the Classification from the International Union of Immunological Societies Expert Committee



# Treating patients with PIDs

KEY for a majority of PID patients

- Anti-infectious therapies

- antibiotics,
- antiviral,
- antifungal,
- antiparasitic

- Other supportive therapies

- thrombopoietin receptor agonists,
- C1 inhibitor concentrate,
- growth factors,
- cytokines/interleukins,
- monoclonal antibodies,
- immunosuppressors and immunomodulators,
- enzyme replacement therapy for ADA SCID)

- **Immunoglobulin replacement therapies**

- Intravenous
- Subcutaneous

- Vaccines

- Curative treatments

- Hematopoietic stem cell transplantation
- Gene therapy
- Thymic transplant

# Immunoglobulin (Ig) replacement therapies

- For PID patients, Igs REPLACE what their immune system is missing to fight foreign microorganisms (viruses, bacteria...)
- No alternative treatments to Ig therapy
- No single Ig works for all patients with PIDs → patient-centred care approach & choice of therapies are key
- Igs are needed life-long
- Igs are developed from human plasma. No alternative way to produce them
- Patients in the EU and the rest of the world have been facing challenges in their access to Igs

# Plasma for the development of Igs & other therapies



- One PID patient needs 130 donations of plasma a year to stay alive
- Plasma can be collected from whole blood donations (recovered plasma) or directly through plasmapheresis (source plasma) – **plasmapheresis allows to collect more, more often**
- The demand for Ig therapies has been increasing steadily: new PID described, earlier diagnosis, longer survival of patients, new countries diagnosing PIDs...
- EU patients rely heavily on therapies developed from plasma from:
  - United States (not enough plasma collected in Europe)
  - 4 EU member states: DE + AT + CZ + HU collect significantly more than other EU member states

**The main safety concern for patients with PIDs in the EU is SUPPLY  
Continued and stable access to Igs as prescribed by the treating physician.**

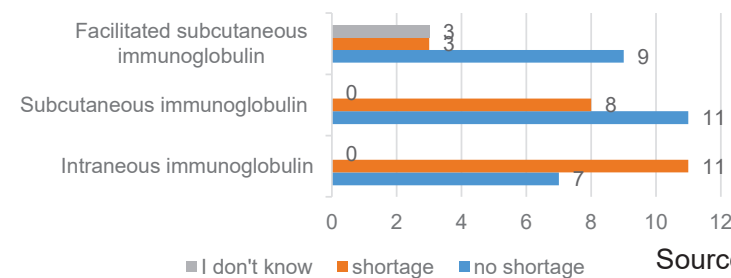
# Challenges in the EU for patients with PIDs

- Patients with PIDs face tensions or discontinuity in their access to Igs as prescribed by the treating physician → shortages of Igs are multifaceted

The screenshot shows the EMA website search interface. The search results for 'human normal immunoglobulins' are displayed, showing one result: 'Human normal immunoglobulins - supply shortage'. The result details include: INN or common name: human normal immunoglobulins; Pharmaceutical form(s): Solution for injection/infusion; Shortage status: Ongoing; Last updated: 3 June 2024. The search filters are set to 'Human' and 'Ongoing'.

- Out of 19 national organisations:
  - IVIg most affected (11 countries)
  - SCIg 2<sup>nd</sup> most (8 countries)
  - fSCIg (3 countries)

Ig shortage by administration route (n=19)

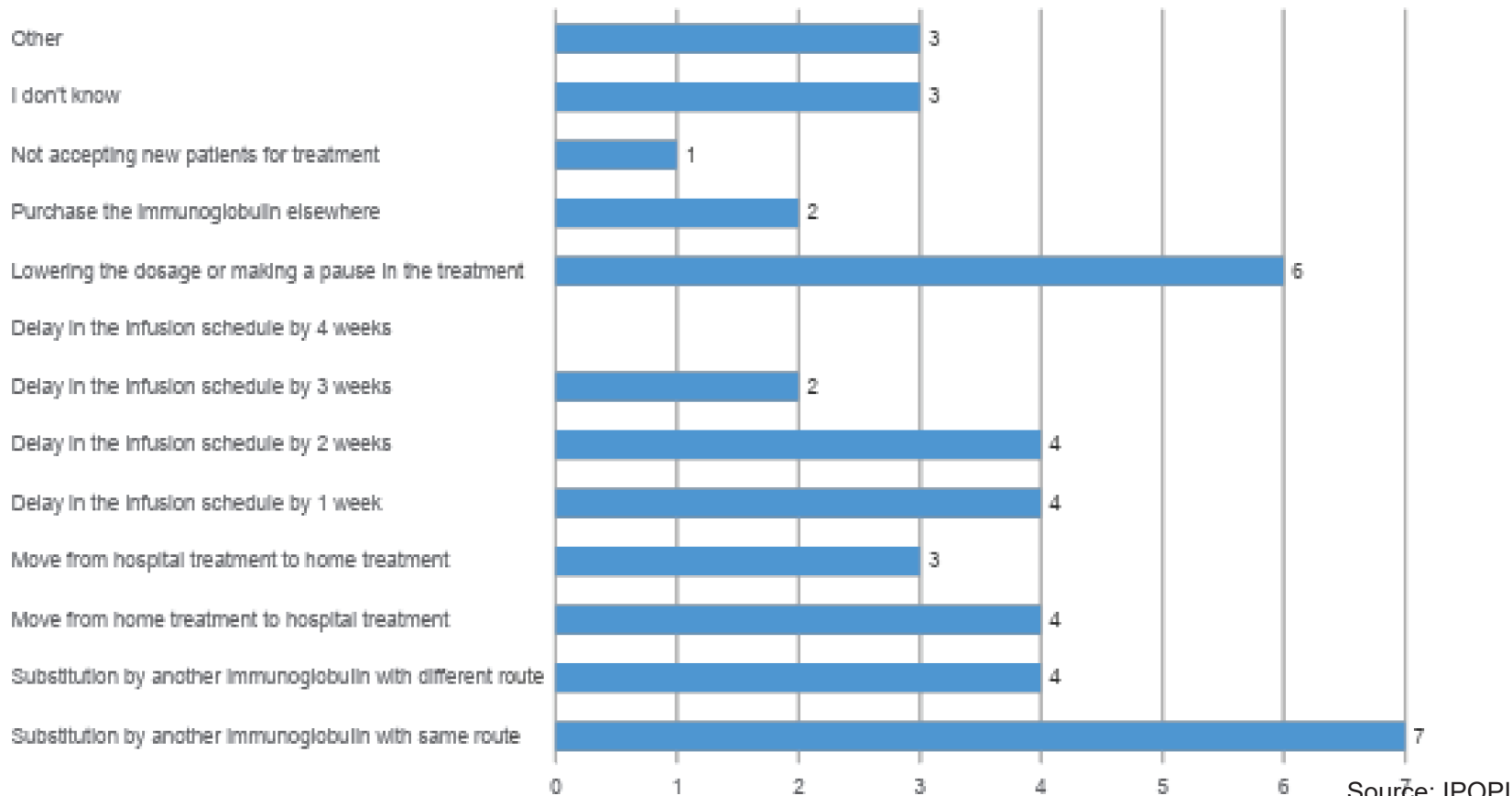


Source: IPOPI survey on Ig shortages (2024)

Source: EMA website (accessed on 4 March 2025). [Link](#)

# Alternative given to patients facing tensions in supply

What alternatives were given to patients? (n=21)

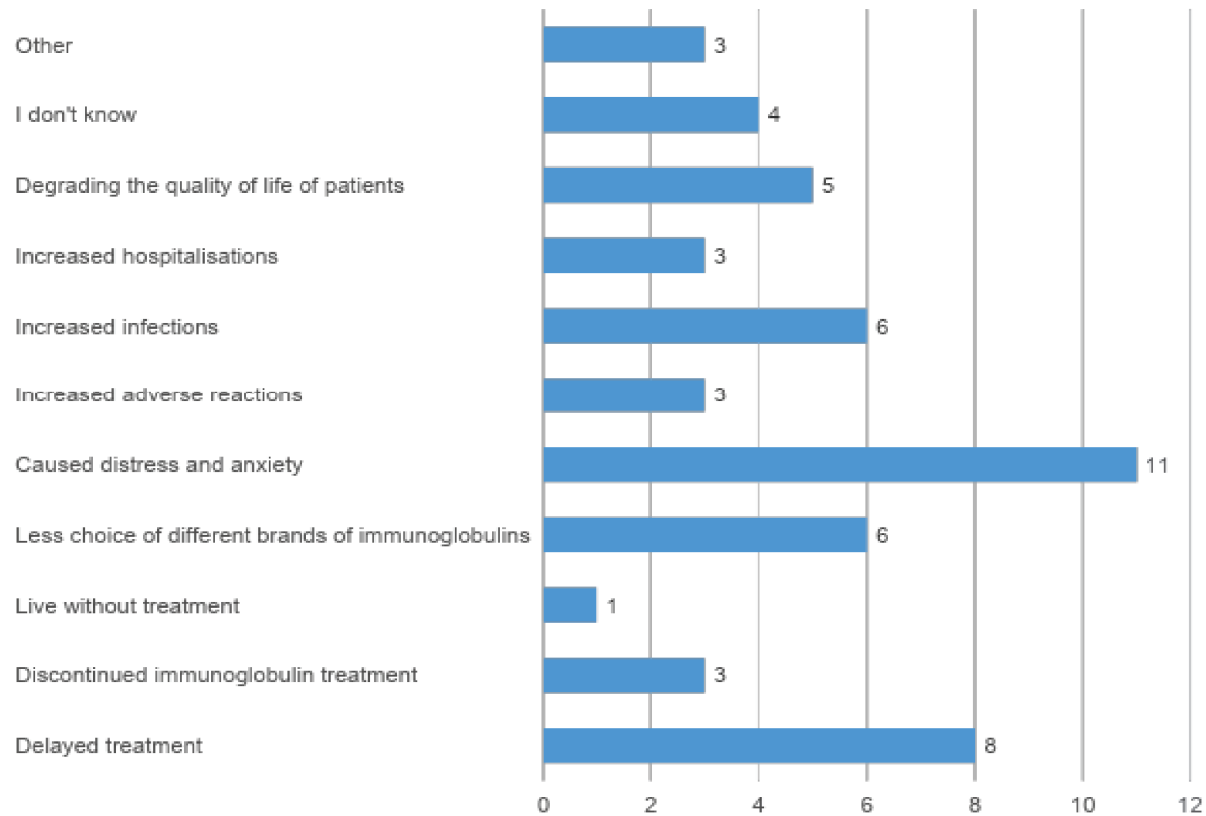


Source: IPOPI Survey on Ig shortages (2024)



# Impact of the shortages in patients with PIDs

Impact of the shortage on the patients (n=21)



Source: IPOPI survey on Ig shortages (2024)

# How to improve the situation?



- Igs are essential medicines that rely on the gift of life of donors
- IPOPI calls for:
  - Respecting and ensuring donors' wellbeing (based on science & data)
  - Using best models to increase collection (plasmapheresis vs whole blood)
  - Establishing national plans on plasma collection with ambitious & concrete goals with realistic deadlines
  - Increasing best practices and learn from countries that are effectively collecting plasma
  - Consider establishment of public-private partnerships when appropriate

# How to improve the situation?



- Igs are essential medicines that rely on the gift of life of donors
- IPOPI calls for:
  - Welcome the adoption of the SoHO Regulation and its approach to financial neutrality, so Member States find the best way possible in their national context, to sustain engaged donors and sustain this engagement through time
  - Any policy or legislation in the field of Igs / PDMPs should be developed in consultation with patients – patient-centred policies
  - Health authorities should provide Ig therapies in a sustained and reliable manner to their nationals, so as to ensure their essential right to health and healthcare

IPOPI is keen to contribute to global sufficiency based on regionally-balanced plasma collection

**Thank you for your attention**



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