

# PID Forum: Exploring The Impact of Medicine Shortages on PID Patients

5 November 2024

PID FORUM

## Exploring the impact of medicine shortages on PID patients

AN IPOPI EVENT



 IPOPI

**5 Nov 2024**  
**13.00 - 15.00**  
**ASP3H1**

Hosted by  
MEP Vytenis Andriukaitis (S&D, Lithuania)  
MEP Tomislav Sokol (EPP, Croatia)

# EVENT PROGRAMME

- 13:30**      *Welcome address*
- 13:40**      *Setting the scene*
- 13:50**      *How to overcome medicine shortages in the EU*
- 14:00**      *Panel: the impact of medicine shortages on rare disease patients*
- 14:40**      *Open floor discussion*
- 14:55**      *Closing statements*



**WiFi:** *(to be inserted)*

**Social media:** *@ipoppi\_info*

**Hashtag:**

- *#PIDForum*



**MEP Vytenis Andriukaitis**

(S&D, Lithuania)

***Welcome Address***



**MEP Tomislav Sokol**

(EPP, Croatia)

***Welcome Address***



## *Setting the Scene*

**Leire Solis**, Health Policy and Advocacy Senior Manager

IPOPI

# Setting the scene

## Impact of medicine shortages on PID patients

Leire Solis

Health policy and advocacy senior manager, IPOPI

IPOPI PID Forum

Exploring the impact of medicine shortages on PID patients

5 November 2024, European Parliament, Brussels





# Introduction to IPOPI

---

The association of national patient organisations dedicated to improving:

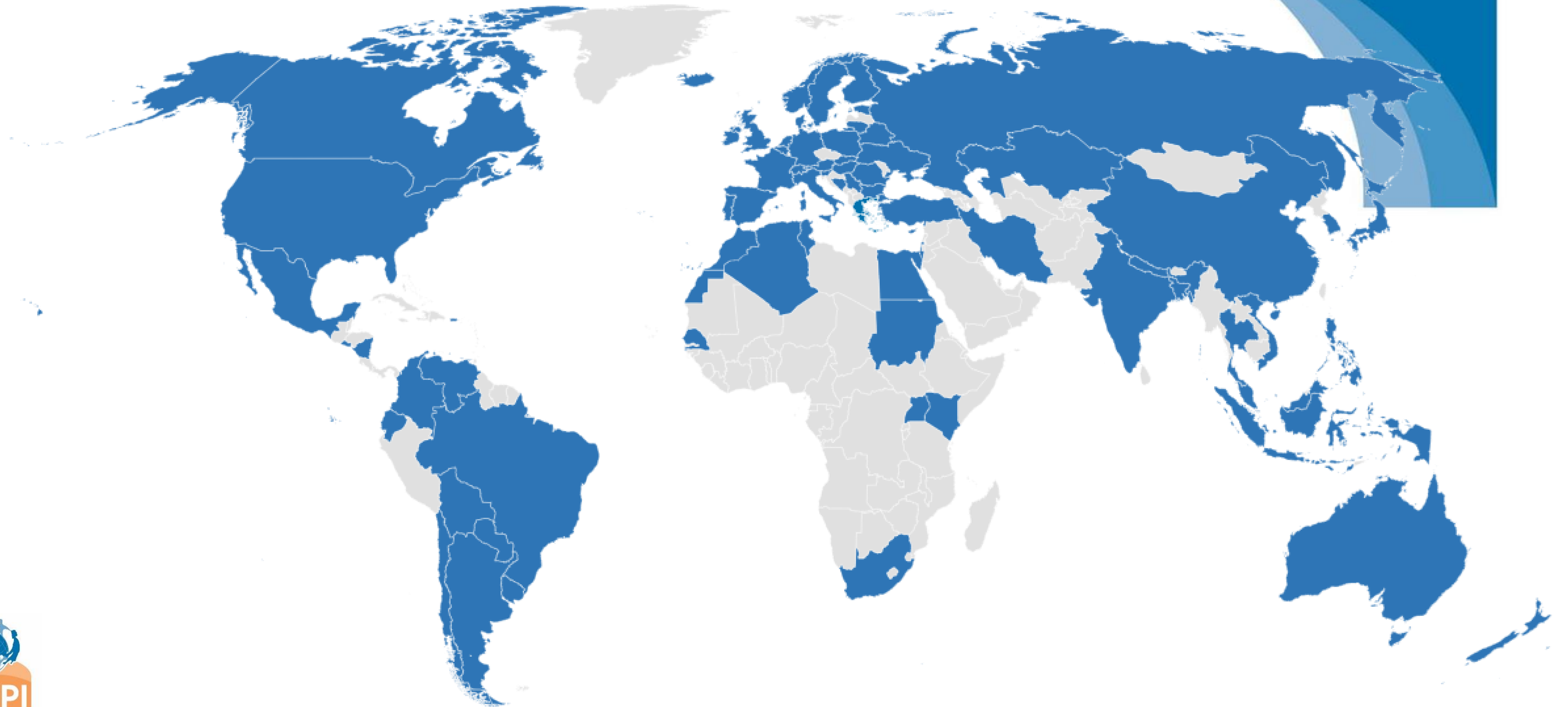
- Awareness
- Access to early diagnosis
- Access to care

For patients living with primary immunodeficiencies (PIDs) worldwide





# IPOPI members



# What are Primary Immunodeficiencies (PIDs)?

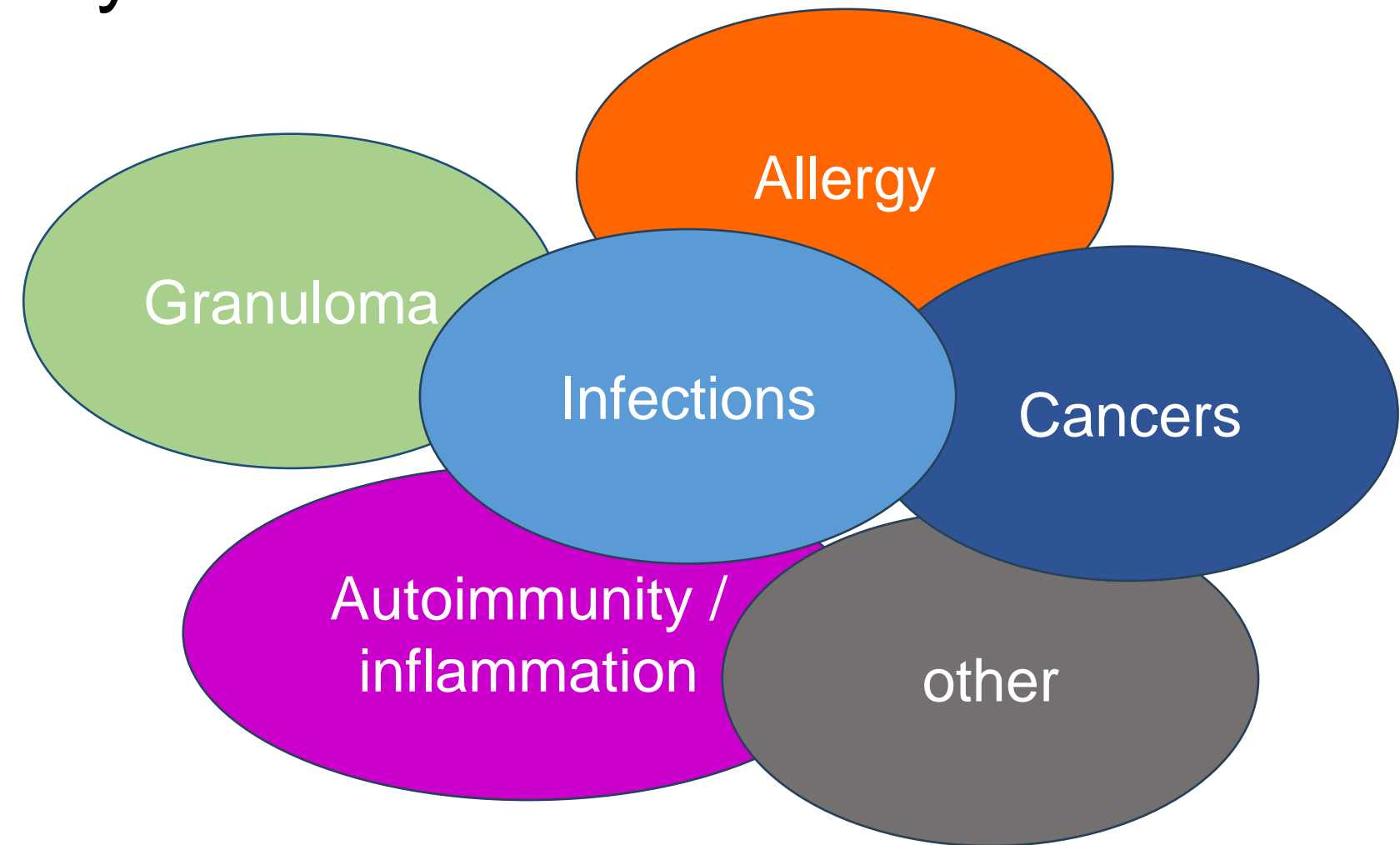
---

485+ different genetic rare and chronic diseases  
The immune system does not work properly or at all

Affect children and adults  
Clinical presentations are variable  
Patients with PIDs can have:

- Increased susceptibility to infections
- Autoimmunity
- Inflammation
- Lymphoproliferation
- Allergy
- Malignancy

...



# What are the treatment options for patients with PIDs?

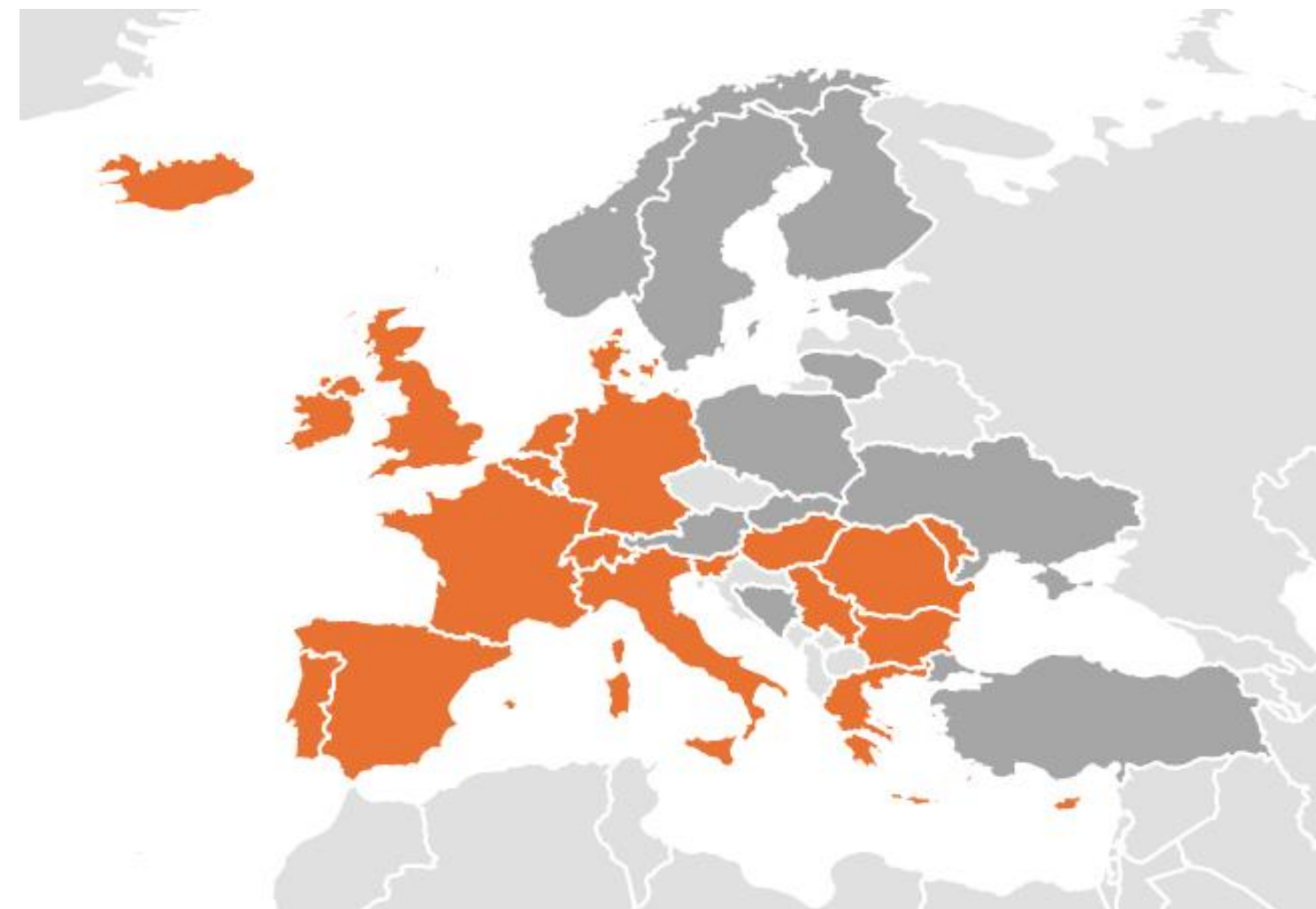
---

- Anti-infectious therapies
- Vaccines
- Immunoglobulin replacement therapies
- Biological and targeted therapies
- Curative therapies
  - Hematopoietic stem cell transplantation / bone marrow transplantation
  - Gene therapy
  - thymic transplant / cultured thymic tissues

# IPOPI survey on Ig shortages in the EU

---

- From 15 July to 6 September 2024
- 31 European national patient organisations invited
- 21 European national patient organisations replied



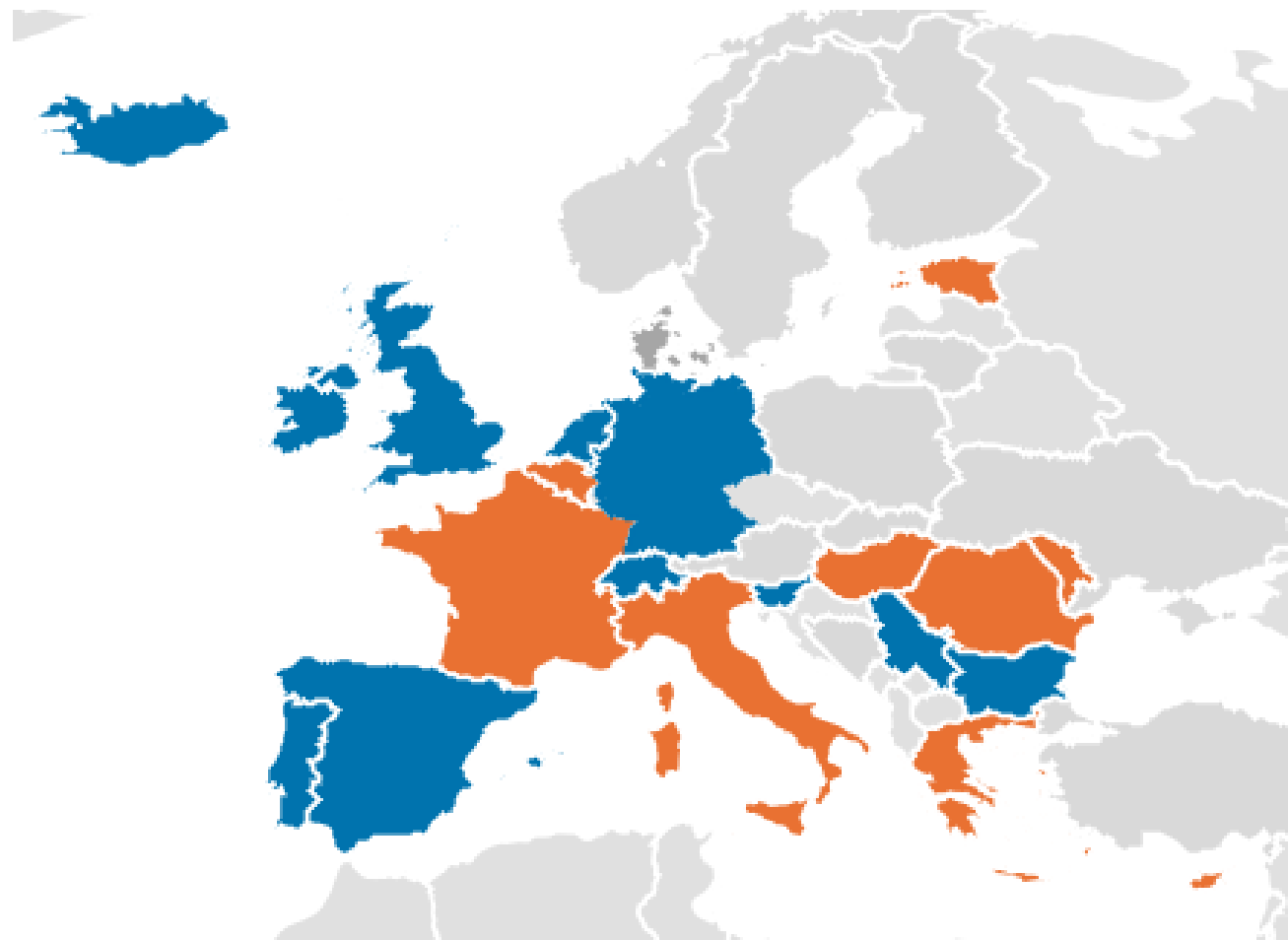
# National organisations reporting an Ig shortage & route affected

- Out of 21 national organisations:

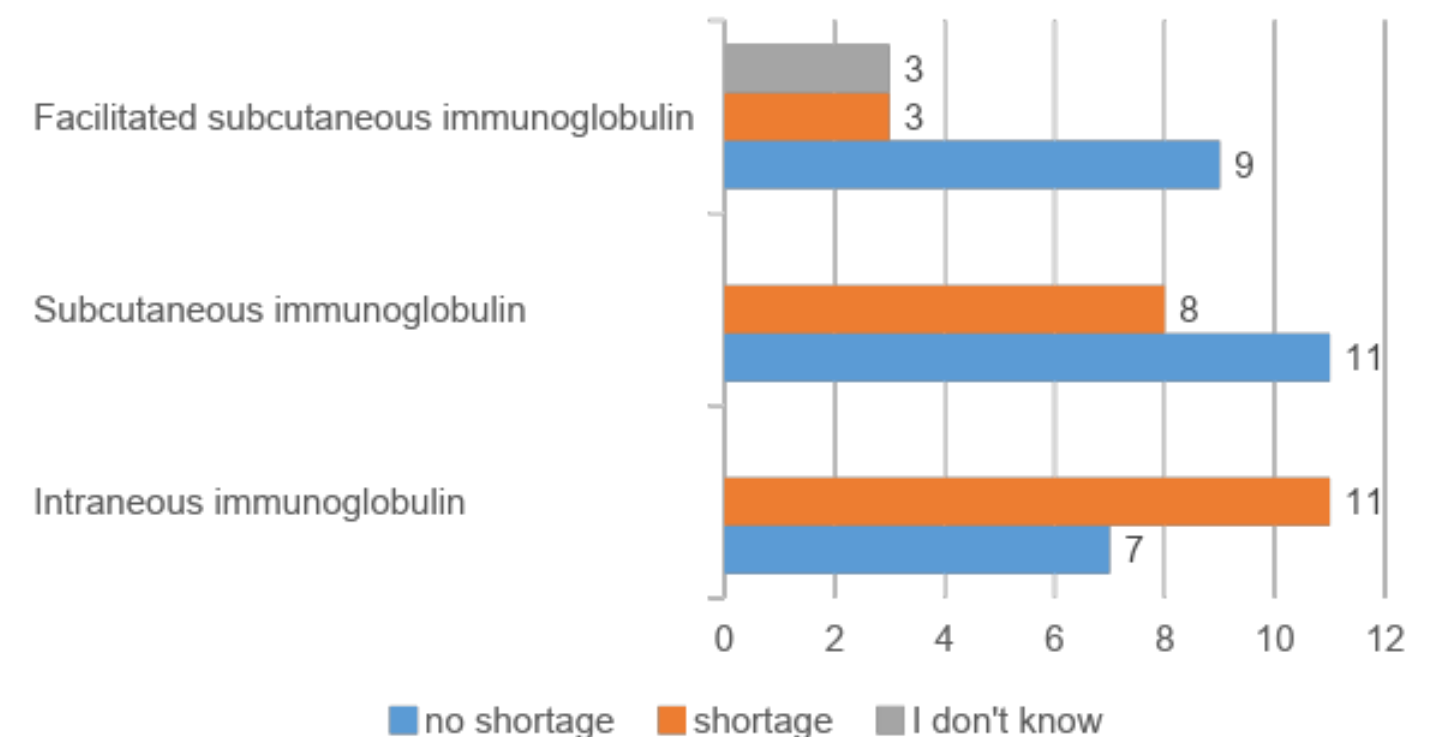
- 12 organisations reported not experiencing an Ig shortage (blue)
- 9 reported experiencing an Ig shortage (orange)

- Out of 19 national organisations:

- IVIg most affected (11 countries)
- SCIg 2<sup>nd</sup> most (8 countries)
- fSCIg (3 countries)

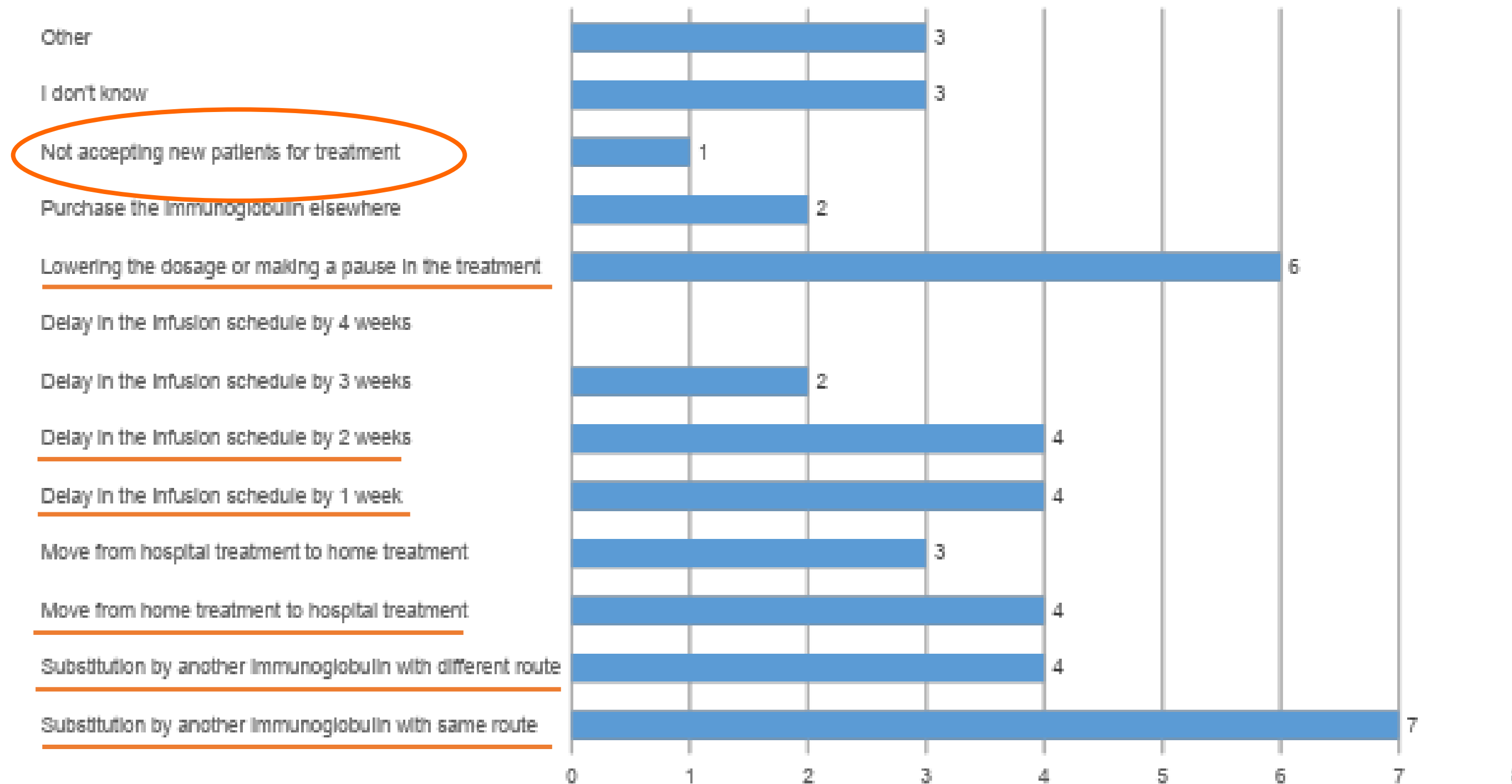


Ig shortage by administration route (n=19)



# Alternatives offered to patients during the Ig shortage

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



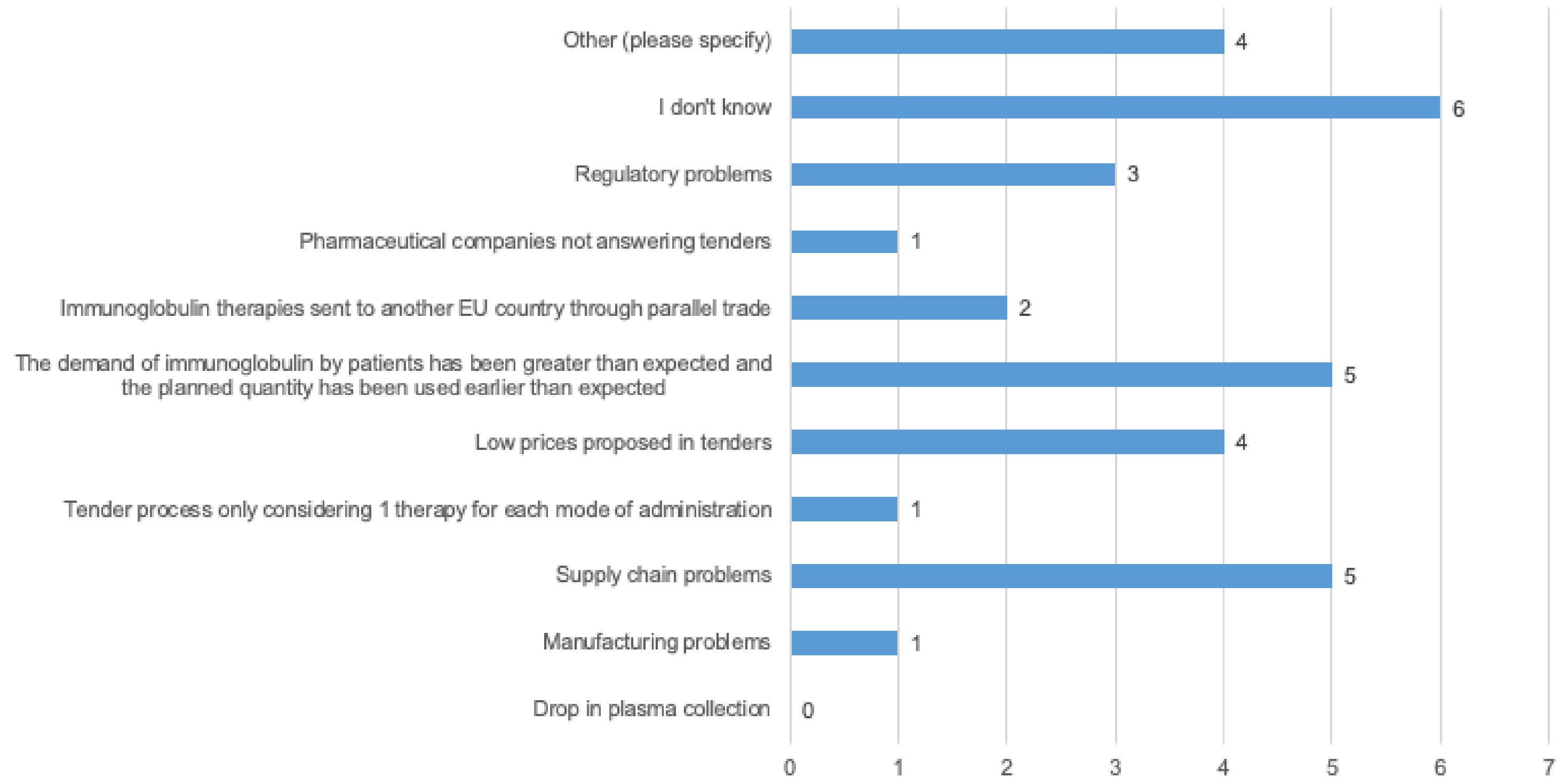
# Impact of the shortage on patients with PIDs

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



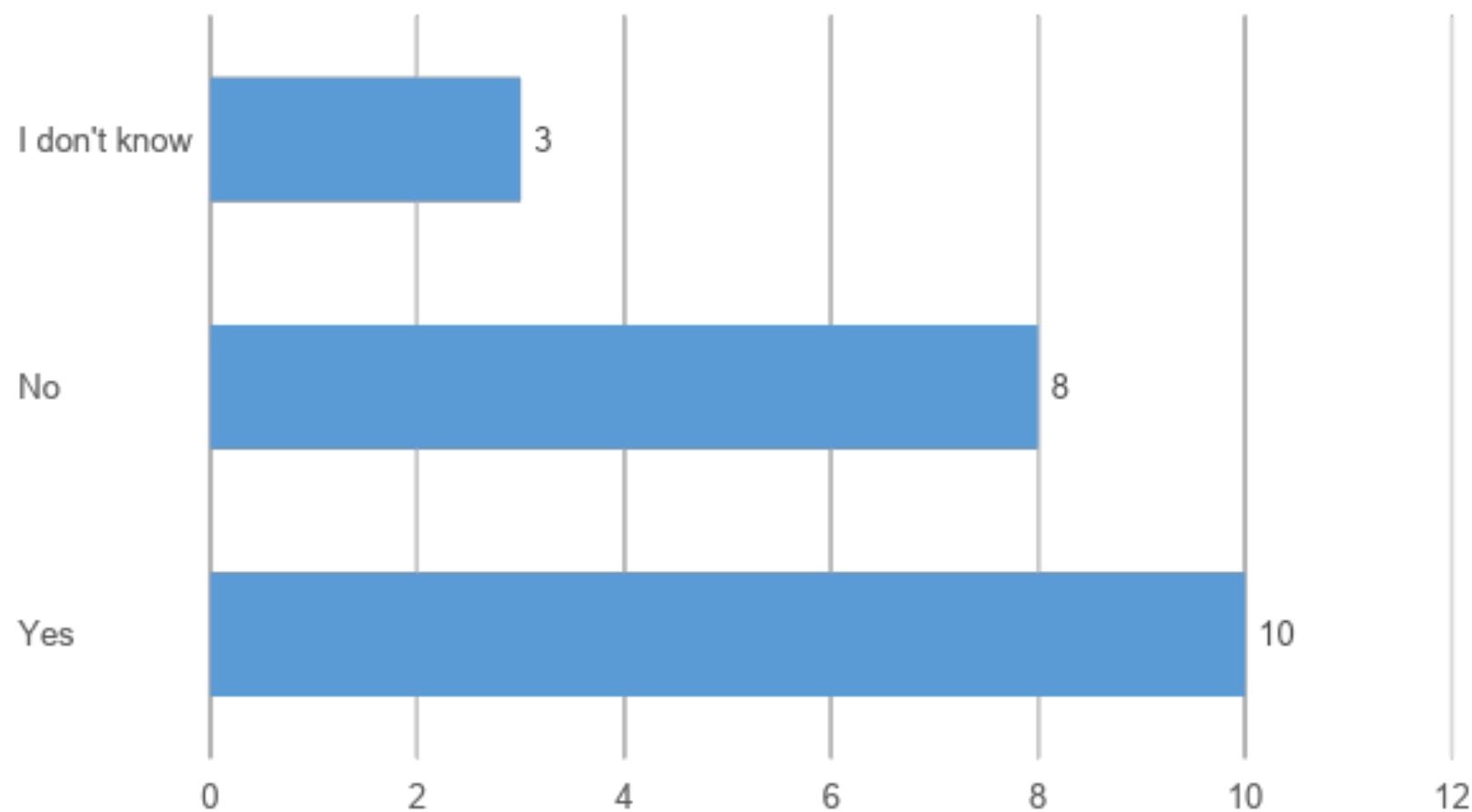


# Reasons given to patient organisations for the Ig shortage

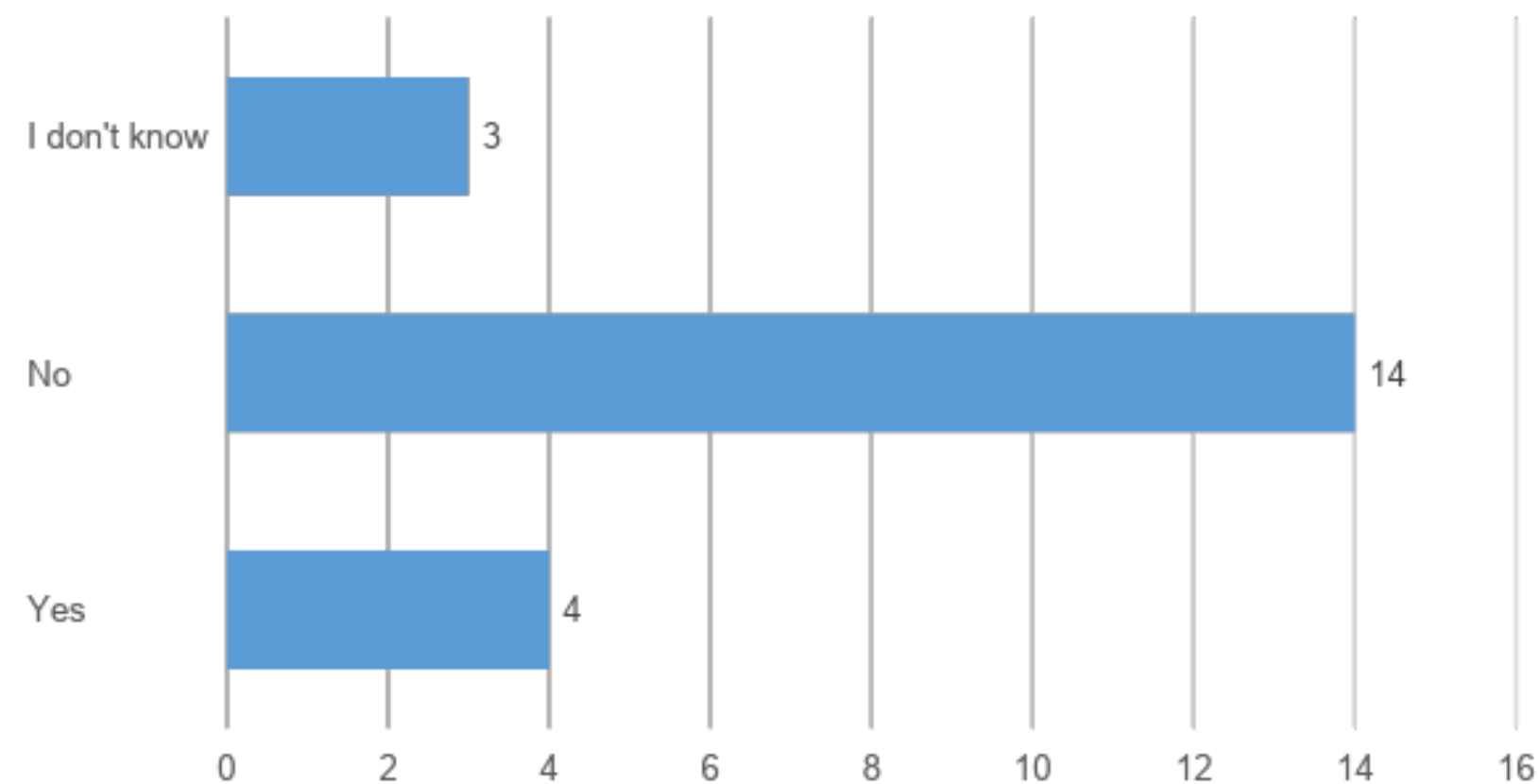


# Dialogue with patient organisations

Was the patient organisation contacted by manufacturers during the shortage? (n=21)



Has the national patient organisation been contacted by public authorities/regulators on the reasons for the shortage? (n=21)



# Key take aways

---

- Shortages of immunoglobulin therapies are a challenge for patients with PIDs in many countries.
- Ig shortages have direct consequences in the well-being of the patients and in their QoL.
- Reasons for the Ig shortage are multifaceted and sometimes not disclosed.
- Different levels of engagement with patient organisations from the manufacturers and from the national / regional governments – improvement needed

Thank you for your attention

PID FORUM

# Exploring the impact of medicine shortages on PID patients

AN IPOPI EVENT



**5 Nov 2024**

**13.00 - 15.00**

**ASP3H1**

Hosted by

MEP Vytenis Andriukaitis (S&D, Lithuania)

MEP Tomislav Sokol (EPP, Croatia)





## ***How to overcome medicine shortages in the EU***

**Klaus Kruttwig**, European Medicines Agency (EMA),  
Medicines and Medical Devices Shortages Specialist

# How to overcome medicine shortages in the EU

Exploring the impact of medicines  
shortages on PID patients

5 November 2024

Klaus Kruttwig, European Medicines Agency (EMA),  
Medicines and Medical Devices Shortages  
Specialist





The owner of copyright and other intellectual property rights for this presentation is EMA. The information made available in this presentation may be reproduced in accordance with the [EMA Legal Notice](#) provided that the source and the author is acknowledged.





Improving the availability of medicines authorised in the EU is a key priority for the **European Medicines Regulatory Network (EMRN)**



Regulatory authorities - within and outside Europe - are increasingly **working together** to prevent shortages and to limit their impact whenever they occur



The joint **HMA/EMA Task Force on the Availability of Authorised Medicines** for Human and Veterinary Use (TF-AAM) provides **strategic support** to tackle disruptions in medicine supply and ensure availability



The EMA's role in **crisis preparedness and management** in reference to availability of medicinal products has increased significantly following the outbreak of the Covid-19 pandemic. **Regulation 2022/123** formalises the structures and processes established during the pandemic.



Provides a framework for activities established by the European Medicines Agency to monitor and **mitigate potential and actual shortages of medicines**



Sets **processes/tools for shortages reporting** and coordinates **responses** of EU countries to shortages of critical medicines during crisis and for monitoring of events which might lead to a crisis situation

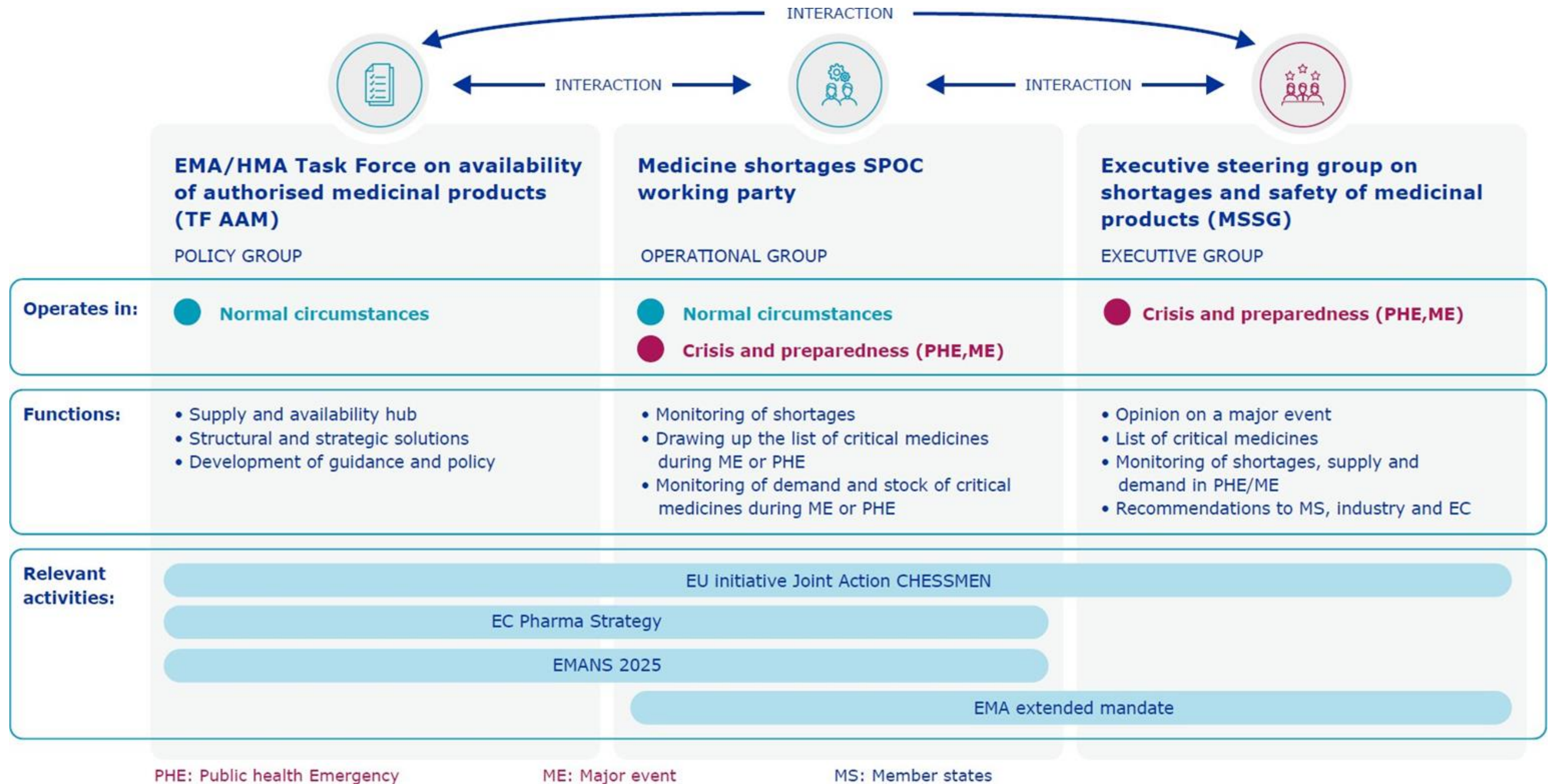


Established the "**Medicines Shortages Steering Group**" (MSSG) supported by the **SPOC Working Party** and a Network of contact points from pharmaceutical companies (MAH i-SPOCs)



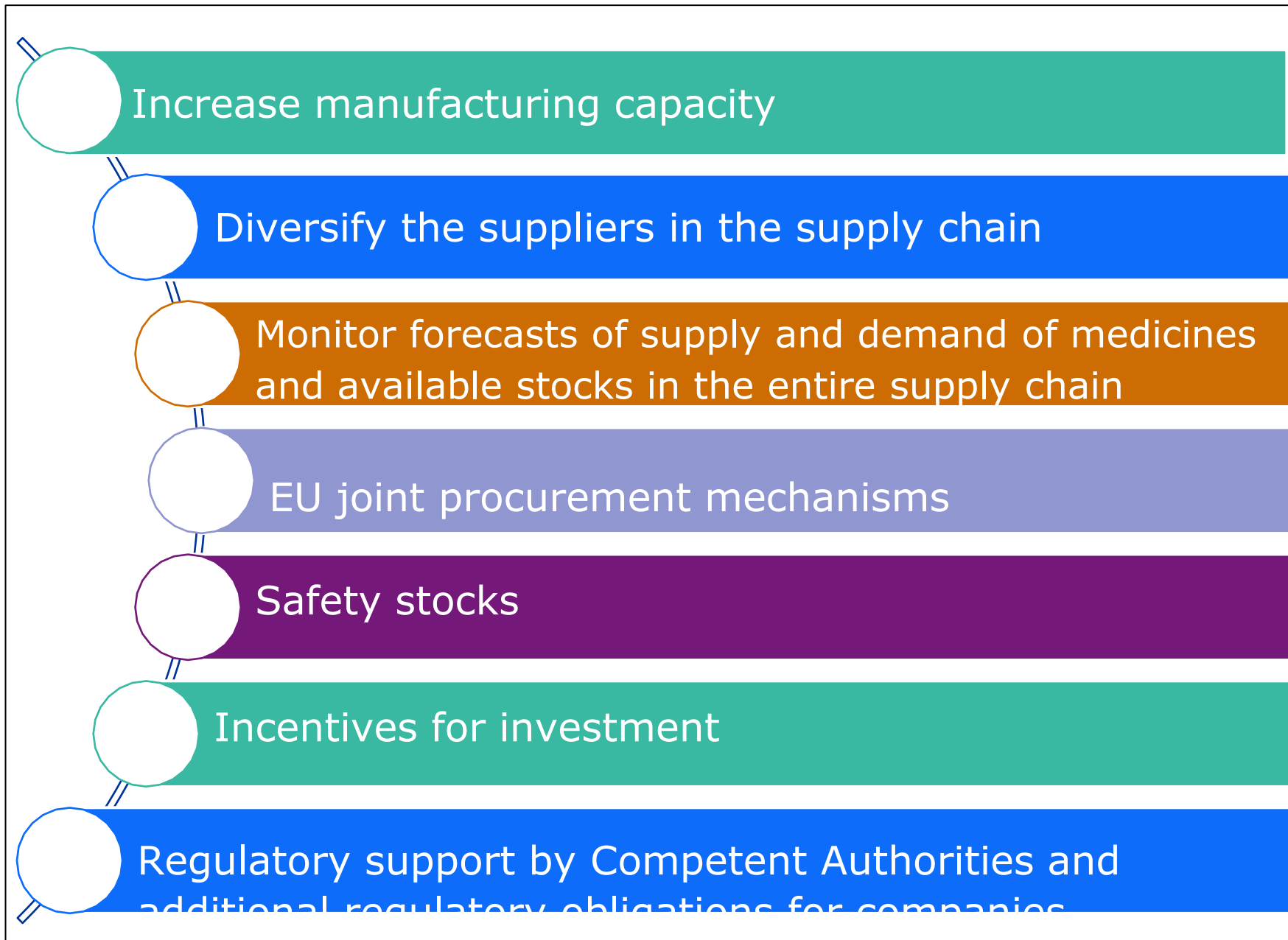
Foresees the development of the **European Shortages Monitoring Platform** (ESMP) by February 2025

# Availability of medicines: EU level coordination



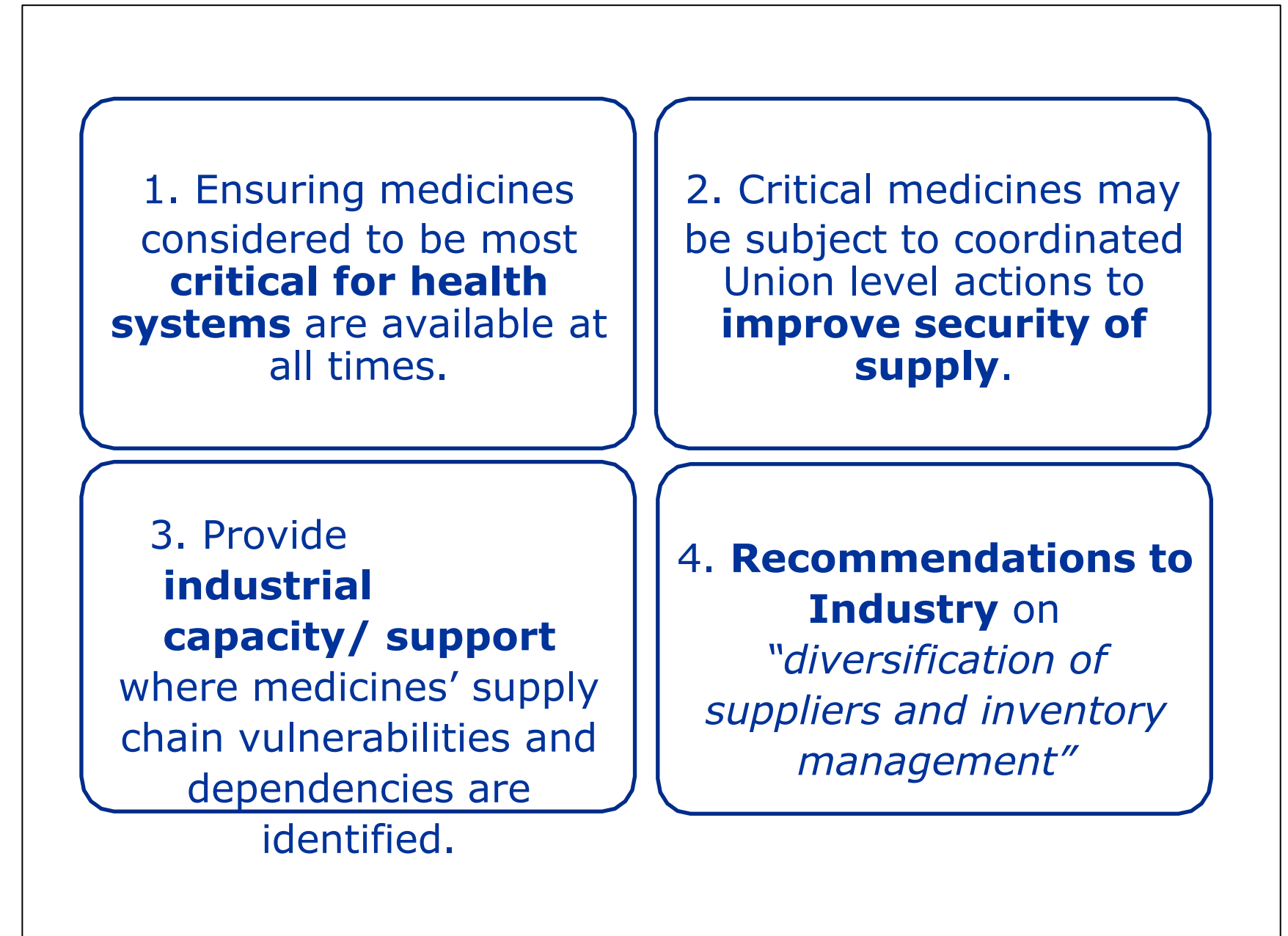


## Recommendations to strengthen supply chains of critical medicinal products



[MSSG recommendations to strengthen supply chains of critical medicinal products](#)

## Union List of critical medicines



[First version of the Union list of critical medicines agreed to help avoid potential shortages in the EU | European Medicines Agency \(EMA\)](#)

## International cooperation

### Global Regulatory Working Group on Drug Shortages

- Meets every quarter and share information about relevant drug supply issues (exchange of information and support each other)
- International cooperation on Drug Shortage Reporting, Signal Detection and Signal Assessment
- International cooperation and alignment on shortage mitigation and prevention strategies

### Bilateral exchanges with e.g. FDA, HC, TGA, etc

- Shortages of concern to both regions
- Exchanges of best practices to mitigate and prevent shortages

## MSSG Solidarity Mechanism

The VSM is used as a **last resort** to **temporarily alleviate critical shortages in a Member State.**



Allows a MS to **request assistance** from other Member States in obtaining stocks of a medicine **during critical shortages**

4 VSM procedures concluded, all successfully

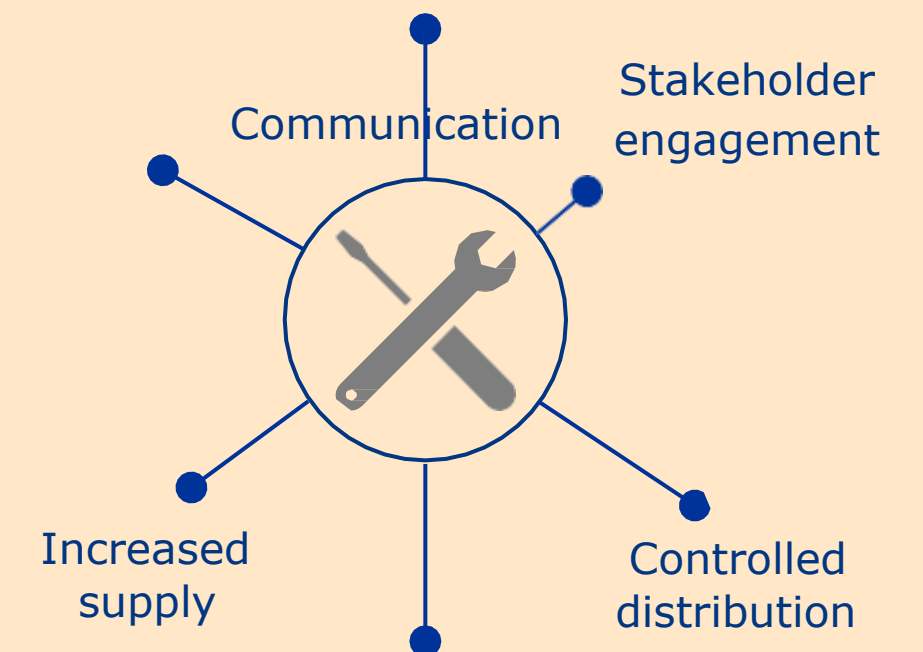
Conditions:

1. EMA already notified of the shortage (SPOC WP)
2. No or insufficient therapeutic alternatives available
3. Insufficient quantities to treat critical indications
4. No or insufficient relief from short-term measures
5. Urgency (e.g., < 1 month supply)

[MSSG Solidarity mechanism process \(europa.eu\)](https://european-council.europa.eu/media/en/press-operations/infographic-1142020.pdf)

## MSSG Toolkit

### European and International cooperation



### Regulatory flexibilities

- Labelling exemptions
- Extension of shelf life
- Accelerating supply-critical variations
- Liaison with EDQM (CEP acceleration)

[MSSG recommendations toolkit \(europa.eu\)](https://european-council.europa.eu/media/en/press-operations/infographic-1142020.pdf)

- The MSSG, supported by the SPOC WP, continues to ensure a robust response to medicine supply issues under **preparedness activities or during a major events/public-health emergencies**. It coordinates urgent actions within the European Union (EU) to manage medicine supply issues.
- **Shift** from a reactive (**management**) approach to a proactive (**prevention**) approach to ensure supply of medicines.
- **New EU pharmaceutical legislation** proposal currently under negotiation, together with the initiatives foreseen in the **European Commission Communication on tackling medicine shortages in the EU, including the CMA** will further reinforce security of supply for critical medicines and prevention of shortages.
- EMA continues to monitor shortages of immunoglobulins through the SPOC WP and the MSSG is currently developing a set of recommendations for Rho(D) immune globulins/ Anti-D immunoglobulins.

# Any questions?

## Further information

---

[Klaus.Kruttwig@ema.europa.eu](mailto:Klaus.Kruttwig@ema.europa.eu)

**Official address** Domenico Scarlattilaan 6 • 1083 HS Amsterdam • The Netherlands

**Telephone** +31 (0)88 781 6000

**Send us a question** Go to [www.ema.europa.eu/contact](http://www.ema.europa.eu/contact)

Follow us on  **@EMA\_News**







## ***Panel discussion: the impact of medicine shortages on rare disease patients***

- ***Karliën Claes***, *International Nursing Group for Immunodeficiencies (INGID), Director of Education*
- ***Klaus Kruttwig***, *European Medicines Agency (EMA), Medicines and Medical Devices Shortages Specialist*
- ***Dr. Nizar Mahlaoui***, *Necker-Enfants Malades University Hospital*
- ***Otilia Stanga***, *Romanian Association for Patients with Primary Immunodeficiencies (ARPID), President & IPOPI, Treasurer*



# Open Floor Discussion



## ***Closing Statement***

**MEP Vytenis Andriukaitis**

(S&D, Lithuania)



## ***Closing Statement***

**MEP Tomislav Sokol**

(EPP, Croatia)

**THANK YOU FOR  
ATTENDING**

**THE PID FORUM!**

**Stay tuned for more...**

PID FORUM

# Exploring the impact of medicine shortages on PID patients

AN IPOPI EVENT



**IPOPI**

**5 Nov 2024**  
**13.00 - 15.00**  
**ASP3H1**

Hosted by  
MEP Vytenis Andriukaitis (S&D, Lithuania)  
MEP Tomislav Sokol (EPP, Croatia)