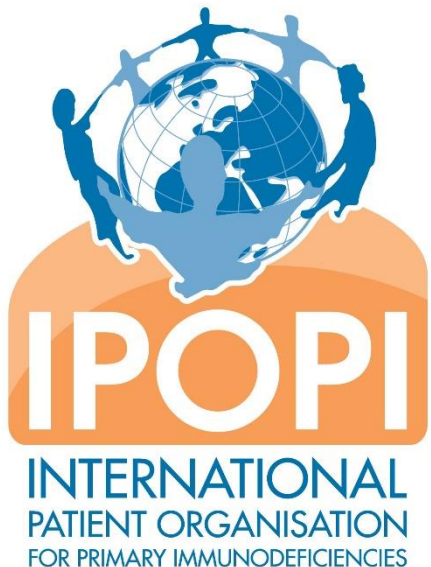


# Representing patients at Enpr-EMA

**Jose Drabwell**  
**President IPOPI**

**IPOPI 11<sup>th</sup> PID Forum**  
**European Parliament (Brussels), 27 June 2018**



# The Paediatric Regulation

- **Before the Paediatric Regulation (2007):** many medicines authorised in the EU were not studied adequately or authorised in children.
- **Children and babies often react differently to medicines than adults.** However, information on the use in children is not always available.
- **The Regulation introduced sweeping changes designed to better protect the health of children.**
- **The main change was the creation of the Paediatric Committee (PDCO) at the EMA to provide objective scientific opinions on Paediatric Investigation Plans (PIDs).**

# EMA supporting medicines for children

- In 2007, the Paediatric Regulation introduced a system of obligation, rewards and incentives to encourage manufacturers to research and develop medicines for children's specific therapeutic needs.
- Fosters the authorisation of more medicines for children, better information, better research and development.
- Some data:
  - 2007 and 2017 – 200 plus new medicines for children.
  - 2004-2006 – 30 medicines
  - 2014-2016 – 74 medicines

# European Network of Paediatric Research at EMA Enpr-EMA

- Created in January 2008
- Main objective: facilitate studies to increase availability of medicinal products authorised for use in the paediatric population, by:
  - Encouraging ethical research on the quality, safety and efficacy of medicines for use in children;
  - helping with the recruitment of patients for clinical trials;
  - enabling collaboration between networks and stakeholders;
  - avoiding unnecessary duplication of studies;
  - building up scientific and administrative competence at a European level;
  - promoting European Commission framework programme applications.

# Enpr-EMA's role

- Identify problems and challenges for individual networks
- Act as a European voice to raise awareness on the challenges and difficulties on a European level (i.e. response to the Paediatric Regulation consultation)
- Propose solutions – as some of the ad-hoc Enpr-EMA working groups have done
- The network does not perform clinical trials or fund studies or research or decide on areas for paediatric research (*responsibility of Member States, the European Commission or each individual member organization*).

# Enpr-EMA's membership

- A network of more than 40 research networks, investigators and centres with recognised expertise in performing clinical studies in children.
- Members of Enpr-EMA are divided into 4 different categories depending on various levels of criteria fulfillment.
- Members perform research in children (newborns to adolescents), in many therapeutic areas, ranging from pre-clinical studies to pharmacovigilance.
- During the last few years an European Young Persons Network has also been established to involve young people at the Paediatric Committee.

# Enpr-EMA's added value

- Provide expert advice (treatment options, paediatric needs, feasibility of a paediatric CT)
- Speeds up the recruitment for a CT, by pooling together patients
- Platform for multi-stakeholder meetings (investigators, networks, patient – patient reps, regulators)
- Enpr-EMA allows networking and collaboration with members from within and outside the European Union (EU), including academia and the pharmaceutical industry.
- Access to academic partners through collaboration with EMA's SME office

# Enpr-EMA activities

- Annual face-to-face meetings.
- Two yearly conference call.
- Feedback from the working groups, such as Ethics, Antibiotics, Young people advisory group, Public-Private Partnership.
- Feedback from the various networks, EBMT, European Cystic Fibrosis, German Neonatal Network etc.



# IPOPI at the EMA

- PID is a multiple condition disease and represented in many of the networks.
- Represent PCWP at these meetings.
- Participate in discussion regarding patients issues such as informed consent and assent.
- Introduction of possible networks and experts.
- Involvement of ERNs in future developments.
- Registry for off-label use.

See you  
in  
Madrid!

an IPOPI event



# IPIC2019

INTERNATIONAL PRIMARY  
IMMUNODEFICIENCIES  
CONGRESS

---

6-8 NOVEMBER  
MADRID, SPAIN

---

[www.ipic2019.com](http://www.ipic2019.com)

# Thank you!



[facebook.com/IPOPIPID](https://facebook.com/IPOPIPID)



[@IPOPI\\_info](https://twitter.com/IPOPI_info)



[TV.IPOPI.org](https://TV.IPOPI.org)