

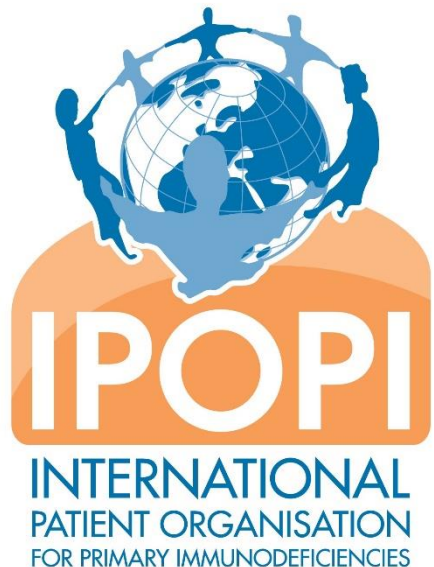
IPOPI 11th PID Forum

Access to paediatric medicines: the case of PIDs Towards the revision of Paediatric Regulation

Setting the scene

Martice Pergent

IPOPI Vice-Chair



European Parliament (Brussels), 27 June 2018

- Thank you Mr Faria and Dr Gentile for hosting this event and your commitment to IPOPI's activities
- Thank you Mr Zdechovský for your support.
- Thanks to participants, patients and experts who accepted to join and will contribute to the discussions.
- Thanks to CSL Behring, Grifols and Shire for supporting this IPOPI initiative.



Reserved right

IPOPI in a few words

- Association of national organisations of patients with PIDs.
- IPOPI's 4 main objectives:
 - To promote early diagnosis & ensure optimal access to care
 - To develop, strengthen and support National Member Organisations
 - To raise PID awareness globally
 - To stimulate stakeholder collaboration
- Established in 1992.
- Today counts with 63 members

Reasons & Objectives of the Forum

- Reasons:

- The Paediatric Regulation has been in place for 11 years (2007).
Revision envisaged starting this year with a consultation in Q3 2018.
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- Objectives:

- We want to **hear**:
 - experts in the various disciplines;
 - policy makers;
 - Individual patients
- To better **understand** the situation for therapies for children with PIDs;
- To **shape** our position for when the legislation starts getting discussed.

Paediatric regulation: adoption and status

- In force since January 2007. At the time, 50 to 90% of paediatric medicines had not been tested and evaluated
- Objective:
 - improve children's health by facilitating the development & availability of medicines for children (aged 0 to 17)
 - Avoid unnecessary trials in children
 - Avoid delays in the authorisation of medicines for adults
- Pillars of the Regulation
 - Establishment of European Medicines Agency Paediatric Committee (PDCO)
 - The Paediatric Investigation Plan (PIP)
 - A set of rewards and incentives (new and on-patent & off-patent)
 - Tools to increase information, transparency and stimulation of research

Primary immunodeficiencies & children

- Large and complex group of more than 350 rare & chronic diseases.
- Caused by an ineffective or inexistent immune system.
- PIDs often appear in childhood but can also manifest in adults.
 - So far, only one PID is recognised to affect adults only (Good syndrome)
- PIDs are treatable and in some cases curable (paediatric cases). If untreated, serious or fatal.

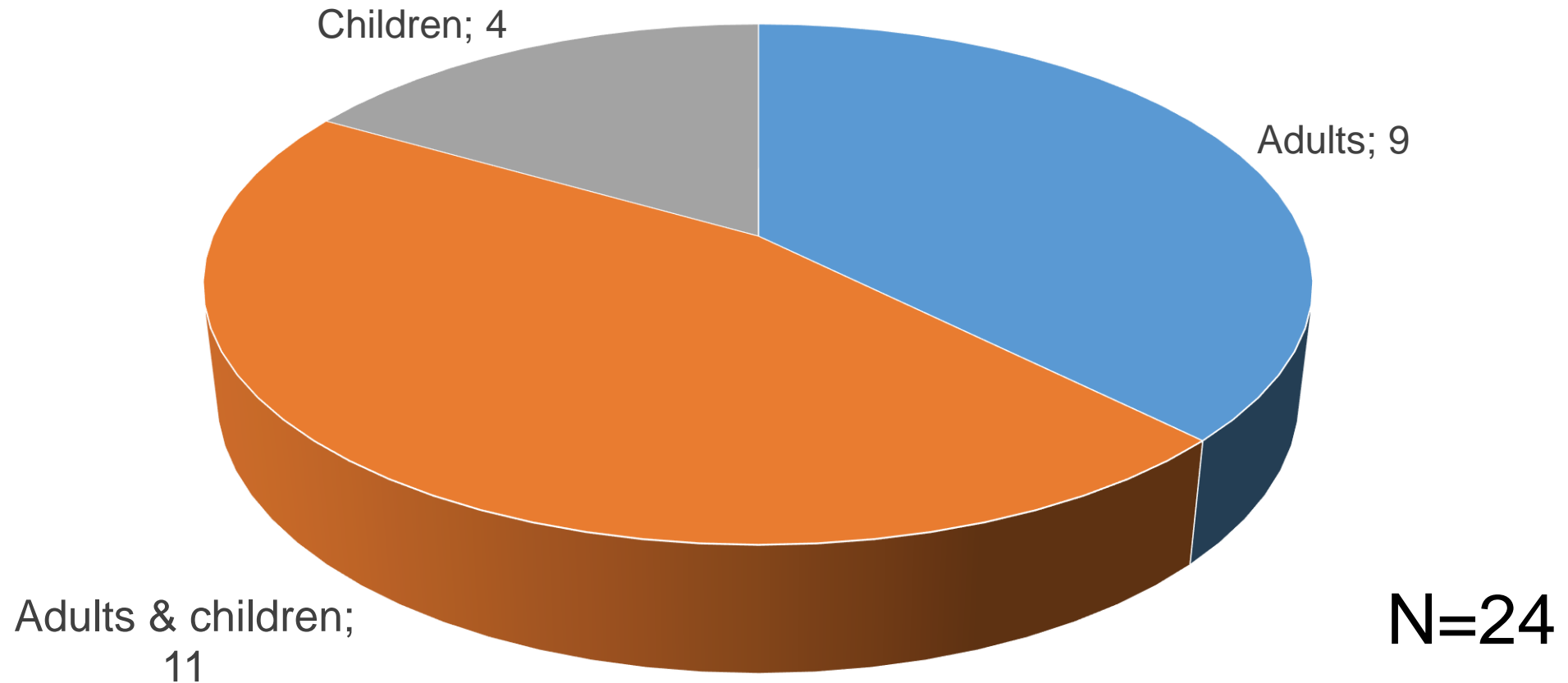
Treatments for children with PID

- Treatment options:
 - Immunoglobulin replacement therapy
 - Anti-infectious (antibiotic, antifungal, antiviral...), gamma interferon, granulocyte-colony stimulating factor (G-CSF) and others
 - Bone marrow transplantation / HSCT
 - Gene therapy
- But PIDs are complex conditions: some of them have additional manifestations that are not linked to the immune system and for which we have no treatment
 - Hyper IGE, Ataxia telangectasia, ...

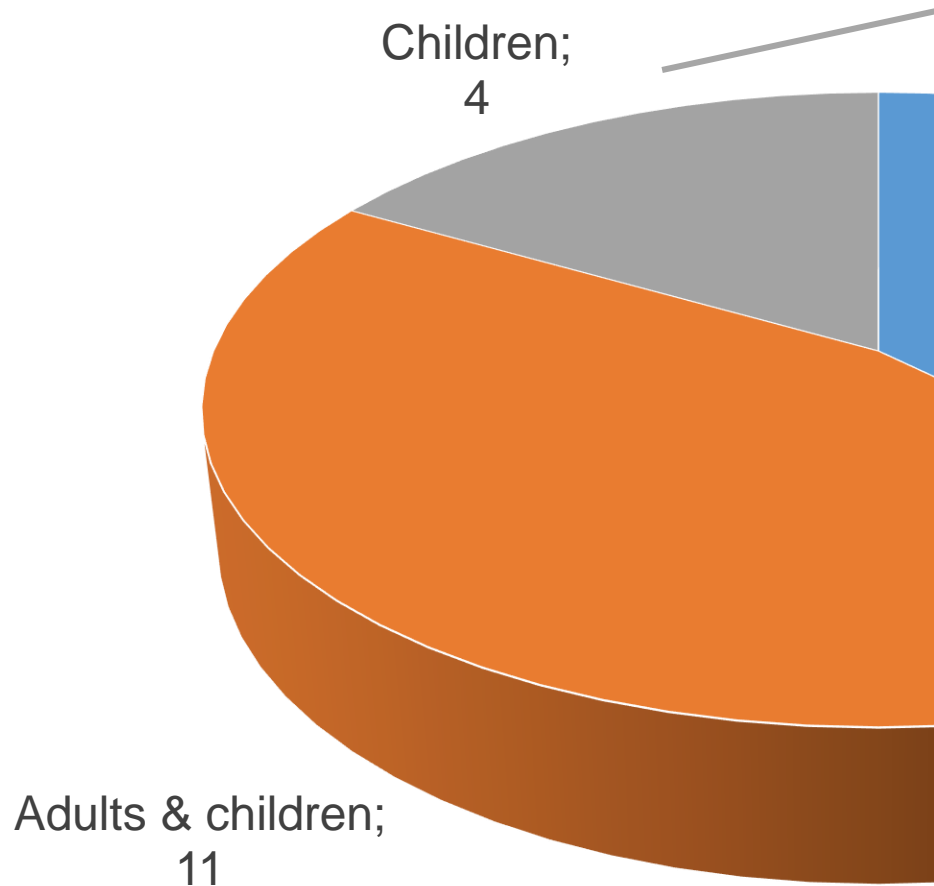
Treatments for children with PID (2)

- In spite of large range of treatments, there are **unmet medical needs** that require investment in research and development of new therapies and conditioning methods to improve the life of children and adults with PIDs.

Number of CTs for PIDs according to European CT Register



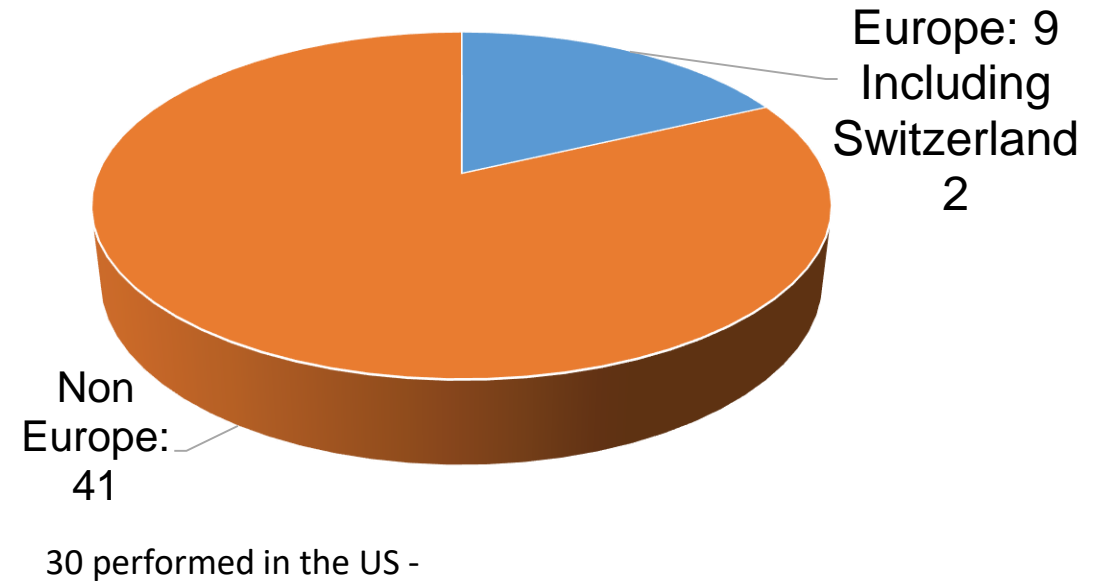
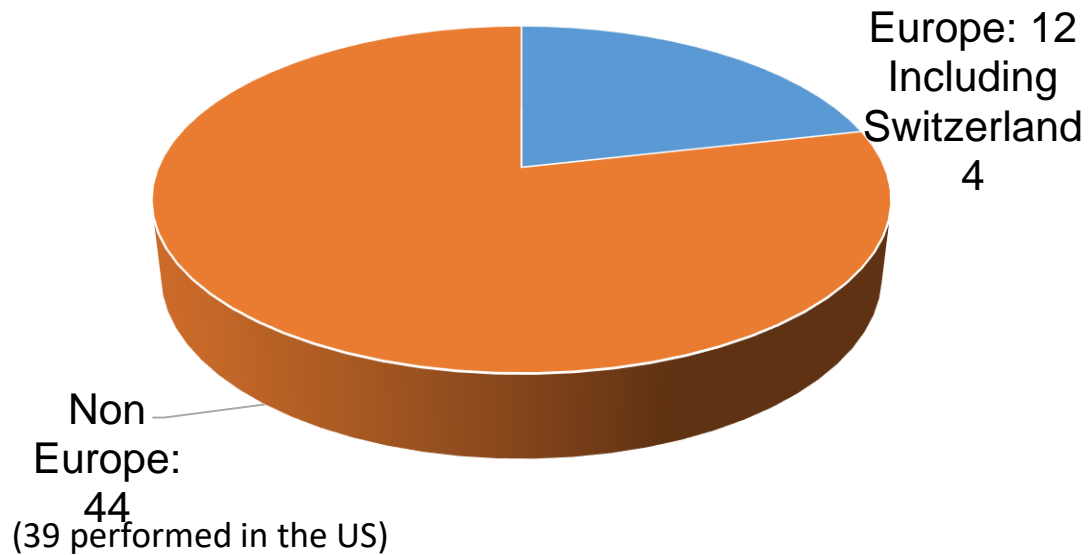
Number of CTs for PIDs according to European CT Register



- Evaluation of Treosulfan pharmacokinetics (PK) in children undergoing allogeneic haematopoietic stem cell transplantation (HSCT) – Great Ormond Street Hospital (UK)
- Clinical phase II trial to compare Treosulfan-based conditioning therapy with Busulfan-based conditioning prior to allogeneic haematopoietic stem cell transplantation (HSCT) in paediatric patients - medac Gesellschaft für klinische Spezialpräparate mbH (Germany)
- Phase I/II clinical trial of T cell suicide gene therapy following haploidentical stem cell transplantation - Great Ormond Street Hospital (UK)
- A multi-center phase I/II safety and feasibility study using CliniMACS TCR α/β and CD19 depleted stem cell grafts from haploidentical donors for haematopoietic progenitor cell transplantation in children - Miltenyi Biotec GmbH (Germany)

Number of CTs for PIDs according to ClinicalTrials.gov

- 56 studies including children (from birth to 17y) currently on-going worldwide
- 50 studies involving children with PIDs have been completed worldwide



Let's ensure that there is more European-based research performed.

Wishes from patients on paediatric therapy development (1)

- Optimal and effective treatments
- Curative therapies for children with PIDs
- Consider also quality of life improvement
 - Diversification of the administration route for IG (choice improves adherence and QOL)
 - Knowledge/medicine travelling, not patients
- Transparency & increased information:
 - On the new developments currently on the pipeline;
 - On current on-going CTs;
 - Help avoiding the conduct of unnecessary CTs in paediatric populations
 - *ERNs & Centres of Reference to partner with patient groups*
- Involvement of patients in CT design and information development

Wishes from patients on paediatric therapy development (2)

- Support to post-marketing data collection to increase knowledge and safety
 - *Importance of registries*
- Early access to CTs without jeopardising safety and efficacy
 - *Consideration of supportive measures such as SCID NBS for BMT & gene therapy!*
- We are aware that marketing authorisation does not mean availability on the market
 - *Legislation should ensure that patients enrolled in CTs are not left untreated after the CT (if successful)*
 - *Patient-centred approach to availability of therapies for children with PIDs and RDs*
- Increased cooperation between EMA and FDA & other international bodies
 - *Speeding up the process, simplification of CT conduct*



Thank you!

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