



## Pediatric Patients Expert Group Training – Day 1

### Minutes

**Date:** November 2<sup>nd</sup>, 2024 | 9h10-16h

**Place:** Otranto meeting room @ Excelsior Hotel, Bari, Italy

#### **Participants:**

Vincent Damotte (Inserm), Yanis Mimouni (Inserm), Tim Friede (UMG), Donato Bonifazi (TedN), Maria Cavallo (TedN), Viviana Giannuzzi (TedN), Silvia Torretta (TedN), Francesco La Penna (TedN), Marco Gismondi (University of Bari), Marta Simone (University of Bari)

Remote participation of Stéphanie Gentile (Inserm) and Énora Le Roux (Inserm) for the session “INVENTS introduction”

The meeting was attended by 8 PEG participants.

#### **1. INVENTS Introduction – Vincent Damotte**

The first session was to introduce the INVENTS project to the pediatric Patients Expert Group and explain what will be the role of the pediatric PEG in this project.

An overview was given about the project, who is involved, what are the main objectives, the use cases, the type of data that will be used. Then the role of the PEG was introduced, emphasizing the importance of listening to pediatric patients in regulatory decisions.

#### **2. What is paediatric research, children's rights in health and research – Silvia Torretta**

An explanation was done about the different steps of medicines development and their testing process. The different types of research studies were introduced (in vitro, in vivo, in silico) and the role of clinical research as well as the phases of clinical trials were explained. Then a focus was done on the need of pediatric medicines. A focus was also done on the rights of children in health and research and the concepts of consent and assent were defined.

#### **3. Data collection and reuse - Tim Friede**

Before to start a clinical trial, we have to phrase the research question: Definition of the PICO(S) concept. An example of clinical trial was given (the PARADIGMS Trial in MS). Introduction of the concept of outcomes and randomization (2 groups: new treatment (fingolimod), versus standard treatment (interferon)). All parameters of the clinical trial are mentioned in the study protocol: what to do and when (concept of Assessment schedule). The Concept of length of the study was also introduced.

If participants are interested in finding clinical trials protocol that are of interest for them, they can find them on clinicaltrials.gov website. Local databases have also lay summaries in local languages. New law obliged us to provide lay summaries but it is important to talk to the doctor to have him explaining the clinical trials.

Then the concept of data reuse was explained, with the goal to make trials more in a more efficiently way. The concept of anonymization and pseudonymization was also introduced.

The observation was made that often patients don't want to talk openly about their disease with other patients. But real life stories are important. The concepts of quality of life/patients reported outcomes/ patients experience were explained.

#### **4. How innovation can improve paediatric clinical research, children's health, and well-being – Donato Bonifazi**

A description of innovation in general and innovation in pediatric research was made. Innovation improve paediatric health care by making tests, monitoring and treatments safer, faster, and more comfortable for kids.

Three groups of innovation in clinical research were introduced and discussed: Digital Health Technologies (DHTs), Innovative Trial Designs, Advanced Therapy Medicinal Products (ATMPs)

DHTs regroup Mobile Health, Telemedicine, Wearable Devices, Artificial Intelligence (AI), Electronic Health Records (EHRs), Consent (eConsent) and Patient-Reported Outcomes (ePRO).

ATMPs are mainly used to treat rare diseases especially in paediatric population. ATMPs by targeting specific genes can potentially cure or alleviate the symptoms of genetic disorders. The concept of personalized medicine was introduced.

Innovation:

- Enhance access to care and patient involvement and allows for real-time health monitoring
- Makes clinical research faster, more efficient, patient-centric, and data-driven
- Allows for personalized medicine to have precise, effective, and targeted interventions

#### **5. Rare Paediatric Arthritis in a nutshell, old, new, and upcoming medicines – Marco Gismondi**

Juvenile Idiopathic Arthritis (JIA) definition: Chronic auto-immune disease that affects the joints

The concept of autoimmunity was explained.

List of subsets of JIA: Oligoarthritis, Polyarthrtis, Systemic JIA

Manifestation : uveitis, gastro-intestinal problems

Diagnosis:

- clinical examination (physical examination)
- Ultrasonography
- Arthrocentesis + Synovial fluid analysis

Introduction of concept of 1st line and 2<sup>nd</sup> line treatments

Then there was a discussion about the medications that PEG participants are taking and there were practical activities of diagnosis.

#### **6. Paediatric onset Multiple Sclerosis (MS) in a nutshell, old, new, and upcoming medicines – Marta Simone**

Introduction on MS: common in adults, rare in children, explanation of the symptoms (weakness, aye problems, difficulties to walk ...). RR MS is the more common forms in children. Risk factors: environmental (EBV, vitamin D), immunological signature, genetic predisposition.

Pathogenesis (auto-reactive T cells in circulation)

Diagnosis (history and exam – symptoms, blood test, MRI, lumbar puncture, cognitive test...).

Treatments targets and list of treatments available (1<sup>st</sup> line vs 2<sup>nd</sup> line)

The current algorithm to treat children with MS was explained. But there is a change of paradigm, now neurologists want to treat patients with HET first



Overview of some ongoing clinical trials in pedMS (LEMKIDS, OPERETTA 2). Some research projects on personalized therapies with myelin regeneration as an objective are ongoing.

Then a quiz about MS was proposed to the PEG participants.

**End of first day**