



## Pediatric Patients Expert Group Training – Day 2

### Minutes

**Date:** November 3<sup>rd</sup>, 2024 | 9h00-12h

**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)

The meeting was attended by 8 PEG participants.

#### **1. New methods for prospective studies involving children – Tim Friede**

The 3 following innovative designs in paediatrics were discussed:

- Adaptive designs
- Non-randomized studies (RWD)
- Combining randomized controlled trials with RWD

Reminders on the PARADIGMS trial (from yesterday session) and the concept of randomization were given.

Then the concept of the size (number of patients and length of treatment or follow-up) of the trials was discussed: The larger the number of patients and the longer the treatment / follow-up the more informative the trial is. The concept of adaptive design was then explained, in which we can check if assumptions that were made when designing the trial are correct using the data from the trial as they occur. The example of PARADIGMS trial was used to illustrate this concept.

An introduction on the use of observational data (RWD) in clinical research was then given with an emphasis of the difficulties to analyze them. The example of a study in paediatric cardiology was used as an illustration.

Then the pyramid representing the hierarchy of evidence was explained

Then the concept of using observational data to support small randomized controlled trials was introduced (combination of RWD and RCT) and illustrated with the early PRO-TECT study.

In conclusion, these methods help to make clinical research in very rare diseases more efficient leading to faster clinical developments of new treatments.

#### **2. New methods using data – Yanis Mimouni**

First, an explanation about what are and what is the purpose of health data was given.

The concept of Digital Health Records and Smart Check-Ups was then introduced as well as the role of Wearable Health Tech for Young Patient.

Then was introduced the concept of Predictive Models for Preventing Illness (to predict someone's future health.)

The 3 steps to build predictive models to predict health outcome were explained (Data collection, Patterns identification, models building). Today there are advanced tools helping in analyzing mountains of data, recognizing patterns, forecasting Health and speeding Up Medical Research.

The concepts of modelling and simulation in healthcare and virtual clinical trials were then introduced. Particularly, a focus was made on the steps of a virtual clinical trial (Design & Simulation Setup, Data Collection & Integration, Digital Twin Creation, Simulated Treatment



Testing, Data Analysis & Optimization, Outcome Validation) and on the benefits of virtual clinical trials for young patients (Faster Access to Treatments, Reduced Sample Sizes, Personalized Therapies, Lower Risk Exposure...).

As an ending note, an emphasize was made on the importance of including the patient perspective and the role of the PEG in INVENTS.

**End of second day**