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Report of the first paediatric patient experts training course

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Table of Contents

1. Introduction.....	3
2. Participants.....	3
3. Training plan and methodology	4
3.1. Detailed Training Plan	6
3.1.1. First meeting.....	6
3.1.2. Second meeting.....	6
3.1.3. Third meeting	8
3.1.4. Fourth meeting	9
3.1.5. Fifth meeting.....	10
4. Critical issues.....	10

1. Introduction

Within the task 15.4, the organisation of short workshops for 15 paediatric patients (pre-adolescence and adolescence, 12-18 years' patients with chronic rare diseases) for each of the three years 2021-2022-2023 is foreseen under the coordination of the TEDDY European Network of Excellence for Paediatric Research in collaboration with EURORDIS and the Sant Joan de Déu Research Foundation.

The workshops aim at providing young people living with rare diseases with the skills that would enable them to get involved in rare diseases biomedical research and to play a role in improving healthcare services for all patients while contributing with their specific experiences. The workshops have been designed as face-to-face workshops but due to the Covid-19 pandemic situation, this first edition has been converted into an online workshop.

If the pandemic situation allows, the second and the final training courses are expected to be organised as face to face and will be hosted in the partners' countries (Italy, Spain, France or any other still under discussion).

2. Participants

The first paediatric patient experts training course was delivered to 15 pre-adolescents and adolescents, aged from 12 to 18 years old who are patients with chronic rare diseases. In order to select the participants, a call for registrations (available via this link: <https://forms.office.com/r/gigibVSwaS>) was held from October 15th to November 23rd and disseminated through social media, websites and newsletters of the following channels:

- Programme Committee members (experts in the field of rare diseases)
- TEDDY, EURORDIS and FSJD (and its members, cascaded as widely as possible)
- EJP RD (and its members, cascaded as widely as possible)
- Young Persons Advisory Groups at EU level and the European YPAG Network
- Patients' associations in all the different countries (e.g., FEDER in Spain, UNIAMO in Italy, etc.) and umbrella organisations such as the European Patients Forum (EPF))
- Foundations (such as Telethon in Italy, Genethon in France, etc.) and not-for-profit organisations

The call's registration form was created through the EJP RD MS Teams system for safety/confidentiality and was returned duly filled by the minor who expressed his/her interest in being involved in the EJP RD training activities.

The online form is available via this [link](#).

Once the minor was selected to participate in the workshop, an information sheet (attached as [Annex 1](#)) and a parent consent form (attached as [Annex 2](#)) for the EJP RD training programme were requested before the start of the training workshops to use their personal and sensitive information in accordance with GDPR requirements.

Participants have been selected to participate according to the following call criteria:

- Good English proficiency

- Age (between 12 and 18 years old)
- Having a chronic rare condition
- Country (in order to guarantee a fair geographic representation)
- Motivation and interest in improving health research
- Participation in specific advisory groups or paediatric patients' organisations.

27 applications were received but only 14 participants were eligible to participate in the workshops were selected accordingly. They came from 7 different countries: Italy, Croatia, Russia, Albania, Spain, Romania, and Germany. The application forms were analysed and selected by TEDDY and FSJD representatives and the task 15.4 leader.

Moreover, an online pre-test was prepared and administered to the selected participants to verify their knowledge level and interest in healthcare and research fields.

The online form is available via this [link](#). This information allowed us to adapt the training plan and activities according to the specific training needs, expectations, and interests of the trainees

3. Training plan and methodology

For the first online edition of this training course, a simplified version of the training plan was prepared (for the version of the face-to-face training plan, please see Deliverable 15.7), where the training contents were more general and not related to any specific disease area in order to widen the target audience and promote the participation of paediatric patients.

The training plan of this first online edition was prepared following a participatory methodology that foresaw the contribution from the young people. To this aim, the following two Committees were appointed before the preparation of the training plan: a Programme Committee and a Young Programme Committee. They revised and validated all the training contents according to the specific procedures defined in the deliverable D.15.7.

The **Programme Committee** is composed of experts in the field of rare diseases and is in charge of developing and revising the training plan as well as revising all the materials developed for each workshop. The members will also be involved in the update of the training plan for the next two editions, according to the results of the workshop survey filled in by all the participants with the aim to collect their feedback and improve training contents.

The Programme Committee is composed of the following members:

- Task 15.4 Leader: Mariangela Lupo (TEDDY)
- One representative per each leading organisation (TEDDY, FSJD, EURORDIS): Annagrazia Altavilla (TEDDY), Begonya Nafria (FSJD), Virginie Bros-Facer (EURORDIS)
- One representative for paediatric patient organisations: Danielle Drachmann (Ketotic Hypoglycemia International)
- One representative for c4c: Becca Leary (Newcastle University)
- One representative for EPTRI: Adriana Ceci (Gianni Benzi Foundation)

The **Young Programme Committee** is composed of patients with a rare disease and is in charge of contributing to the revision of all materials developed for each workshop as well as in the design of the training activities. The members of the Committee have been selected among the members of the European Young Persons Advisory Groups (eYPAGs) as follows:

- KIDS Albania: A. M. (16 years old). He is living with McCune Albright syndrome
- KIDS Bari: V. G. (16 years old). She is living with the syndrome of Mainzer Smaldino
- KIDS Barcelona: N.N.R. (18 years old). She has spinal muscular atrophy.

In order to engage as many young people as possible and to ensure that the training plan really meets the interests and the needs of the target audience, TEDDY has been involved in the revision activities of the members of the TEDDY YPAGs in Italy and Albania, respectively KIDS Bari and KIDS Albania. Their feedback has been included in the training plan as well as considered in the preparation of the training contents of this first course (an example of their contribution is available in the deliverable D.15.7 where the evaluation forms received by KDS Bari, and KIDS Albania are attached).

The first edition of the paediatric expert patients training workshop started on November 26th and ended on March 23rd, according to the plan summarised below.

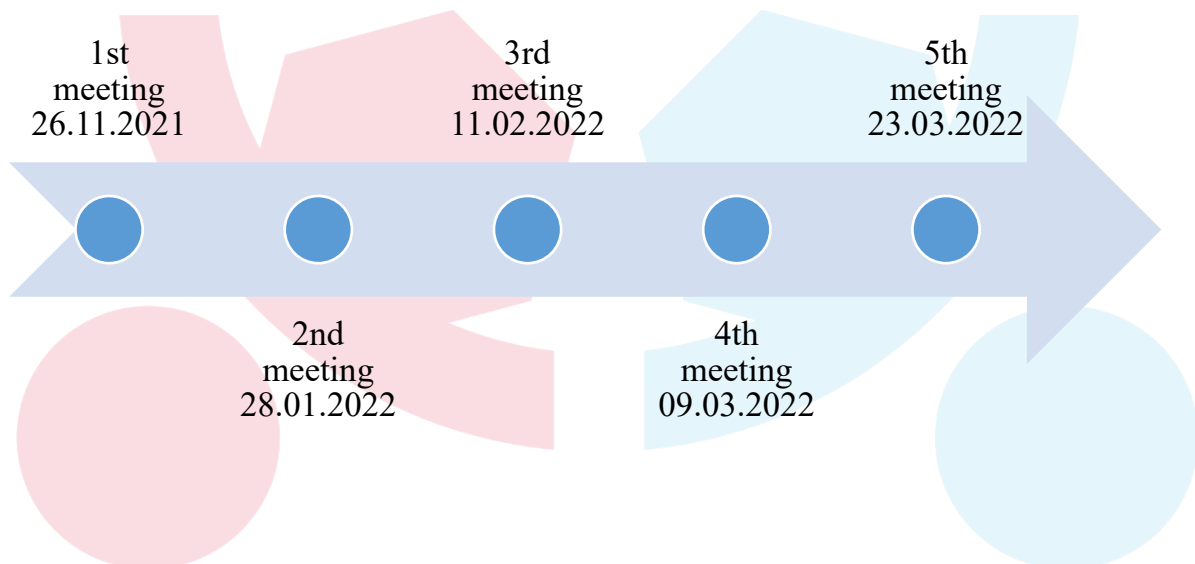


Figure 1. Programme of the workshops

Each meeting was organised as an online session of a maximum of 2,5 hours and was delivered through the EJP RD MS Teams platform. The workshops have been performed in English and delivered by selected experts, both internal and external to EJP RD. In order to engage as much as possible the participants and to make it easier for them to understand the topics discussed, each meeting has foreseen practical activities and case studies discussions.

3.1. Detailed Training Plan

3.1.1. First meeting

The first meeting was composed of a first introductory session about the EJP RD goals and activities, which was held by Clément Moreau as representative of the EJP RD Coordination Team. Moreover, an ice-breaker activity was organised by Mariangela Lupo (TEDDY) to get to know each other. This ice breaker activity was as follows:

Title: Where would you go, what would you bring with you?

Description: Each participant should start describing the place he/she would like to go and the three things that they would put in their luggage.

They have also to say who they would bring with them, like fantastic or real characters.

Everyone should select a picture from their mobile phone, show it to the group and then explain a little bit more about it.

The second part of the meeting was organised by Begonya Nafria (FSJD), who provided a general introduction about rare disease peculiarities. Her presentation is attached as [Annex 3](#). The first meeting lasted more than the expected 2,5 hours. Therefore, it was decided to move the case study activity to another session.

3.1.2. Second meeting

This meeting was dedicated to clinical research with the aim to introduce clinical research, its different phases and the main elements of the clinical protocol. It was held by Mariangela Lupo and Annagrazia Altavilla from TEDDY. Their presentation is attached as [Annex 4](#).

After introducing the key concepts of clinical research, the case study with the kids was organised using the serious game entitled “My Clinical Trial Center” which is available on both Android and iOS. The videogame was created by TEDDY KIDS Bari and Albania young members and is a digital game with an educational purpose aimed at explaining to children in a funny way what are clinical trials, how do they work and why they are so important to develop drugs suitable for children.

The game was extremely informative. Game-players had the opportunity to learn about clinical trials, study protocol, informed consent and assent for children, phases, and procedures of clinical trials and to test their knowledge about what we had explained to them previously. They concluded the game during the meeting and communicated the score once the whole level was completed, in order to create an internal rank. As the game was composed of questions with True/False and multiple answers, all the corrected answers to the activities were provided and revised with the whole group to check the errors and provide an explanation to them. The correct answers to the "My clinical Trial Center" serious game are attached as [Annex 5](#).

The second part of the meeting aimed at brainstorming about the video to be prepared for the rare disease day, held on February the 28th. We invited the trainees to participate in the #lightupforrare campaign launched by Rare Disease Day and explained in this video tutorial: <https://www.youtube.com/watch?v=tfqri6Dnaeg>.

Participants were asked to take multiple photos, first with cell phone in a horizontal position and then in a vertical position while they were illuminated by the colored screen of their computer, provided by the site www.rarediseaseday.org. Instructions for the preparation of the video were sent by email to all participants to receive their feedback and start developing the video to be finalized in the next session.

The final video was then developed and finalized by the TEDDY team in collaboration with the KIDS Bari young members and is available via this [link](#).

The young people also prepared the following slogan:

MY BEING SPECIAL HAS LIGHTED ME UP

Kids and Teens show their support to the #shareyourcolours campaign!

They are not their disease, they are not numbers... they are amazing and stunning teens in love with life and passionate about science!

Aren't they amazing? Their smiles are our hope for a better future!

#rarediseaseday #shareyourcolours #rarediseases #awareness #kidsbari #coloryourrare #coloriamoci #smile #showyourrare #giornatamalattierare #teddynetwork #ejprd #eptri #cvbf #icanresearch.



Figure 2. Preview of the video

The video was shared through the TEDDY social media channels and contacts.

3.1.3. Third meeting

The first part of the meeting was dedicated to the organization of the practical case study about rare diseases delivered by Begonya Nafria (FSJD). The second part of the meeting was dedicated to patients' engagement to empower the participants and to explain how patients can contribute to progress of paediatric research. She presented the slide about the EUPATI framework to involve patients in the drug development process (attached as [Annex 6](#)) and aimed to explain the following important topics:

- Patients can contribute throughout the drug development process and the sooner we involve them the better
- Patients provide a unique perspective that researchers can have: first person living with a specific condition
- Explain the different activities in which young people can be involved

The session was very practical and included at the end a Kahoot activity (Kahoot is a free game-based learning platform) to assess if they had achieved the contents.

3.1.4. Fourth meeting

This meeting was aimed to present an overview of GDPR rules and its impact in the research area as well as of the children's rights in processing personal data for research data protection. During the meeting, an overview on informed consent and assent form, and the types of consent/assent forms that are used has been provided. The presentation shown during the meeting by Annagrazia Altavilla from TEDDY is attached as [Annex 7](#).

On that occasion, the template of the informed consent for the adults that has been prepared by the ERNs in the framework of the ERICA project (available at this [link](#)), was shared with the kids.

The goal of the activity was to prepare a simplified version of the informed consent tailored to paediatric patients, by implementing the comments received from the kids through the questionnaires they filled and including a summary of the main relevant principles in the field (attached as [Annex 8](#)). The questionnaire aimed to collect feedback on what is unclear to them and what they would improve, such as: if they wanted to add images to clarify the data transfer stages (flow chart); if they want to use a simplified glossary of terms; if they understand what “commercial use” means, etc.

Moreover, the practical activity with the “colours methodology” (attached as [Annex 9](#)) was explained to them so that they could complete it at home and send their feedback within 10 days.

In particular, we asked them to analyse the ERN informed consent template for the adults and proceed with the exercise in the following way:

- Select the words that they don't understand (highlight them with red colour).
- If there is information that they don't understand (full paragraph), highlight them also with red colour.
- Select with green colour all the pieces of information that they easily understand without the explanations of an adult, and they consider are relevant for them in order to take the decision to give their agreement/assent.
- Finally, select in pink colour the pieces of information that although they understand they consider not relevant to be kept in the form or that should be included elsewhere or described in a different form.
- Detail any specific pieces of information that they think can be explained better with a scheme, flowchart, or other types of visual information.

The forms and the related documents were sent by email to the participants and the activity was completed in the last session of the workshop.

3.1.5. Fifth meeting

The first part of the workshop was presented by Annalisa Landi from the Gianni Benzi Foundation and aimed at providing an overview of the regulatory framework of the paediatric research as well as the basic concepts of adverse events and monitoring. The presentation is attached as [Annex 10](#). An interactive session was also organised and is attached as [Annex 11](#).

The second part of the session was managed by Annagrazia Altavilla and Mariangela Lupo from TEDDY and aimed at presenting the simplified form of the ERN template of the informed consent addressed to paediatric patients. The assent form was prepared by the TEDDY team with the participatory methodology based on the adults/parents' consent form elaborated within EJP RD for ERNs, and according to the comments received by the participants to the EJP RD course as well as the members of KIDS Albania and KIDS Bari, who have analyzed the new form for paediatric patients in a dedicated session. This paediatric assent form (attached as [Annex 12](#)) is to be considered as a part of a more comprehensive strategy to be adopted by registries to increase children's awareness of patient registries' activities and roles with the aim to foster their participation and engagement.

4. Critical issues

The first paediatric training workshop has been a great success even if some bottlenecks have been highlighted and can be improved in the next editions of the course.

In particular, the training course was addressed to 15 paediatric patients but the number of participants decreased along the course due to several factors:

- the difficulty to stay so many hours on an online platform considering that many of them had to attend school lessons remotely;
- the chronic condition of the participants and the pandemic situation which have in some cases hindered their participation due to health issues;
- the personal and school appointments of the young members;
- the linguistic barrier combined with the complexity of the topics.

In order to improve the quality of the next editions of the paediatric experts' training courses, an online evaluation form has been prepared by TEDDY and filled by the participants. This information will allow adapting the training plan and activities according to their specific training needs, expectations and interests. The evaluation form is attached as [Annex 13](#).