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Del 15.7
Training plan for paediatric patients' in the EJP

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1. Background

The lack of paediatric medicines including innovative and advanced ones, is a long-lasting and well-known problem at European and international level. The need to include children in drug development programs has been largely recognised over the past few decades, and stringent legal and regulatory frameworks have been established all over the world with significant but insufficient results.

Paediatric clinical trials present many differences from clinical trials enrolling adults and they need to take into account some paediatric peculiarities such as organ maturation, different physiology and metabolism as well as samples variability based on the different age range.

All international texts and guidelines require that an appropriate risk/benefit ratio should be the basis of a clinical trial, especially for studies including children. To avoid exposing children to excessive risk through under-protection or missing opportunities for important advances through over-protection, strict attention should be paid to the criteria used for this evaluation, especially within clinical research phases in paediatrics.

Adult instruments are often inappropriate, and children have been poorly served by research, even though they have specific emotional and physical needs that must be met. In addition, the need for children to have equitable access to medicines will increase with the growth in advanced technologies and personalised medicine. As a result, children remain deprived of many therapeutics because of the challenges faced to appropriately study and tailor medicinal and other products for children.

It ***is therefore crucial to involve patients in the clinical trials*** to invert this paradigm. Patient engagement in clinical trials has in recent years emerged as an increasingly important aspect of successful clinical trials. Across all involved groups such as sponsors, academics, regulators, and patients, the primary goal of clinical trials is to more rapidly answer research questions and deliver safe, effective treatments to patients. In this sense, patient engagement is central to accomplishing this goal.

Patient engagement may indeed be defined as an effort to extend the benefits of patient insight and experiences, as well as desires and preferences, in both the research and development process, or, ‘‘from bench to bedside’’ through collaborative relationships, in order to conduct more efficient and patient-centered, high-quality research.

Since the UN Convention 1989, it is widely accepted that ***children have a right to express their views and to be heard in all matters that affect their lives***. The Convention on the Rights of the Child introduces for the first time in an international human rights treaty, the concept of the 'evolving capacities' of the child. Article 5 of the Convention states that direction and guidance, provided by parents or others with responsibility for the child, must take account of the capacities of the child to exercise rights on his or her own behalf. The concept of evolving capacities is central to the balance embodied in the Convention between recognising children as active agents in their own lives, entitled to be listened to, respected and granted increasing autonomy in the exercise of rights, while also being entitled to protection in accordance with their relative immaturity and youth.

However, it is very important to examine the inter-relationship between the concept of evolving capacity embodied in Article 5 and the concept of participation contained in Article 12 of the Convention. Article 12 asserts that States parties 'shall assure to the child who is capable of forming his or her own views the right to express those views freely in all matters affecting the child, the views of the child being given due weight in accordance with the age and maturity of the child'.

Children are thus rights-holders with a progressively evolving ability to make their own decisions. However, on matters concerning their health and general well-being, there is uncertainty as to how the increased recognition of their decision-making capacity should be addressed. Finding the right balance between autonomy and protection is a challenge when considering that children's rights are situated within a larger set of parental rights and responsibilities that also focus on their best interests

The young patients engagement early on in the research process and educating them about the importance of participating in clinical research, is not only a children's right but it is a fundamental strategy in reducing the patients' retention and fostering treatments compliance. The promotion of the collaboration between the different actors of the research process even when this process involves children should be included in the best practices of the paediatric research in order to enhance quality and relevance of the research itself.

However, some challenges have to be faced for the engagement of children, since it requires appropriate means and language to be adapted to key factors influencing children comprehension such as age, physical condition, previous knowledge and mental state. The child's autonomy can be conceptualised as 'the child's right to an open future', meaning a right to have one's future options kept open until one can make one's own decisions.

Due to the paediatric specific developmental characteristics, ***specifically tailored methods should be applied to the training and empowerment process of paediatric patients***. In particular, it is advisable to educate and empower them in specific tasks related to different therapeutic and research areas related to their condition and for which their participation is required. Adapted training modules, analysis and discussion of case studies, revision of assent documents (with appropriate language and content) should be developed also with the involvement of children and young people. Furthermore, the use of focus groups, questionnaires or surveys, personal interviews, constitutes a useful strategy to collect feedbacks aimed at improving treatments and research projects.

Starting from these premises and considering that ***training is crucial to facilitate the implementation of children rights to participation in decision regarding their health and to facilitate the involvement of paediatric patients in ethically sound rare diseases research***, the task 15.4 has foreseen the organisation of dedicated activities aimed at training expert young patients with rare diseases.

2. Methodology and timeline

The planned activities include short workshops 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 for 15 paediatric patients for each of the three years 2021-2022-2023.

The workshops aim at providing young rare disease patients with skills enabling them to get involved in rare diseases biomedical research and play a role in improving healthcare services for all patients by contributing with their specific experiences.

The workshop will be replicated each year according to the training plan described below and will address 15 pre-adolescence and adolescence, 12-18 years' patients with chronic rare diseases per year. A call for registrations will be launched by the beginning of October 2021 to identify the young participants and disseminated through several channels.

An online pre-test will be administered to the selected participants to verify their knowledge level and interest in healthcare and research fields. This information will allow to adapt the training plan and activities according to their specific training needs, expectations and interests. An authorisation/assent forms will be submitted to parents/children to comply with the legal requirements.

The workshops have been designed as face to face workshops but due to the pandemic situation the first edition will be organised as a virtual meeting. If the situation allows, in year 4 and 5 the training course will be developed face to face and will be hosted in the Partners' home countries (Italy, Spain, France or any other).

The workshops can be organised according to the timeline described in figure 1:

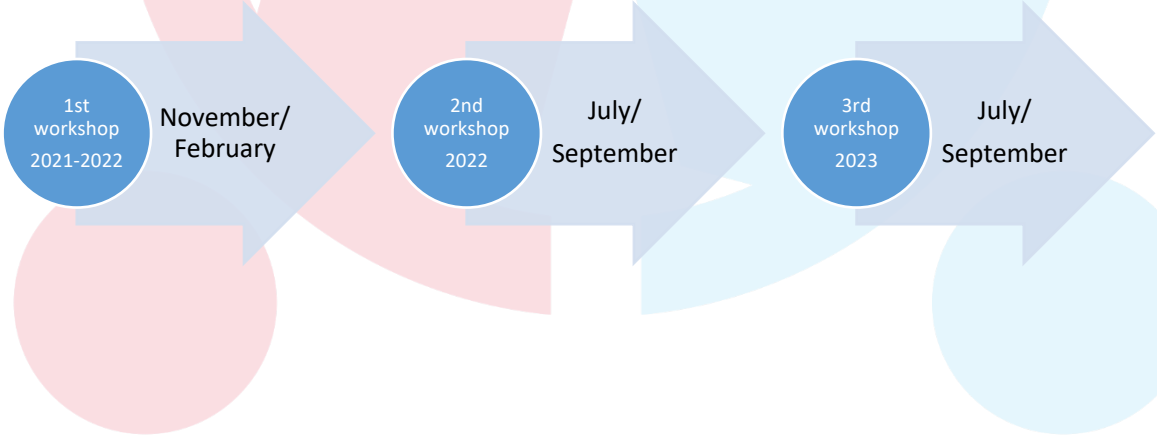

Figure 1. Timeline of the workshops

The first edition will be organised in distant and online mode. A session per month of maximum 2 hours and a half will be organised in order to avoid longer sessions that makes complicated during the school time. The first session will start in the second half of November 2021 and will end on the 28th of February with the organisation of a raise awareness activity to celebrate the worldwide Rare Disease Day (figure 2).

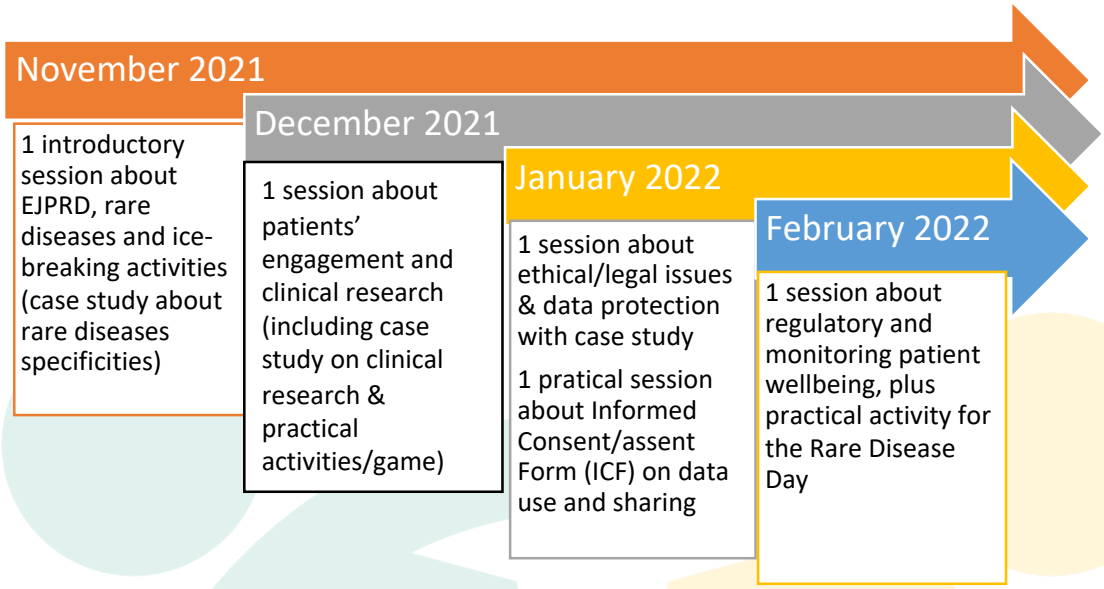

Figure 2. Programme of the workshops

The paediatric expert patients training workshop will foresee practical activities and case studies discussions after the classroom-taught lesson in order to make easier the comprehension of the topics discussed and to assess if the goals expected have been reached. Moreover, in order to increase the interaction, participants will be divided in small groups and will participate in parallel breaking out sessions, each coordinated by one moderator. At the end of each session, all groups will present the results of their work to the plenary session through posters presentation and speeches.

The face-to-face meetings have been planned to last three days and to be held in summer period in order to ensure that young patients are on vacation and have finished with the school duties. They might be held at the partner premises or in a location (research center/hospital/public institution) identified by TEDDY, EURORDIS and FSJD, possibly among EJP RD institutions, and potentially involving other Stakeholders, Networks, European/international institutions (i.e. Council of Europe) and International Organisations, i.e. in occasion of the iCAN (International Children's Advisory Network) Summit. iCAN is the worldwide Network of YPAGs, that organises yearly Research & Advocacy Summit with hundreds of children (both patients and healthy ones) coming from all the YPAGs of the world.

At the end of each meeting, short questionnaires will be distributed to participants to test their knowledge and get their feedback about the meeting so to eventually update the training plan.

The workshops will be performed in English and delivered by paediatricians, investigators, psychologists, representatives of patients associations, YPAGs facilitators, legal, ethical and regulatory experts, both internal and external to EJP RD, taking also into consideration and adapting the training contents already available and developed within other tasks of the project.

3. Programme Committee

In order to draft and finalise the training plan, a Programme Committee has been appointed. It will be in charge of developing and revising the training plan, revising all the materials developed for each workshop and approving the panel of the proposed speakers according to their expertise. It will also update the training plan according to the results of the workshop survey which will be aimed to collect feedbacks both from participants and speakers.

The Programme Committee is composed by the following members:

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

Moreover, a Young Programme Committee composed of patients with a rare disease has been created and will contribute in the revision of all materials developed for each workshop as well as in the design of the training activities. They have been selected among the members of the European YPAGs and is composed by the following members:

- 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.

3.1. Procedures to validate the training contents and materials

All the training contents and materials will be revised and validated by the members of the Programme Committees according to specific procedures.

The Programme Committee will receive from the task leader all the training materials by email and will be asked to revise them and provide feedback within 15 calendar days. A doodle pool will be sent to them with the aim to schedule a TC to present the materials and clarify the expected contributions. All the comments and feedbacks have to be sent in tracked changes and will be collected by the task leader that will integrate them in a consolidated version that will be send to the Programme Committee members for information at the end of the process. Tacit consent will be deemed granted if no comments are submitted by the due date. For specific topics, external experts or members from EJPRD might be involved according to their particular competences and skills.

Regarding the Young Programme Committee members, the task leader will send by email all the training materials asking for revision and implementation within 15 calendar days. The Team leader of each involved YPAG to which the young member belongs will be always copied in all the communications in order to facilitate their work and get their feedback on due time. In addition, detailed instructions will be sent to them with the aim to explain what is expected from them in terms of clarification of the contents, feasibility of the training project, children-friendly approach, adherence to the paediatric patients needs and interest. A TC might be organised prior to the delivery of the training materials in order to present them and clarify their expected contributions. All the comments and feedbacks will be sent by email by the related Team Leader in tracked changes and will be collected by the task leader that will integrate them in a consolidated version.

4. Selection of participants

For each workshop, a number of 15 pre-adolescence and adolescence, aged 12-18 years that are patients with chronic rare diseases will be selected. A call for registrations will be launched through social media, websites and newsletter of the following channels:

- Programme Committee members
- TEDDY, EURORDIS and FSJD (and its members, cascaded as widely as possible)
- EJPRD (and its members, cascaded as widely as possible)
- Young Persons Advisory Groups at EU level and eYPAGnet
- Patients associations in all the different countries (e.g. Feder in Spain, UNIAMO in Italy, etc.) and umbrella organisations such as European Patients Forum)
- Foundations (such as Telethon in Italy, Genethon in France, etc.) and not-for-profit organization

The call for registrations will be sent using a form created through the EJPRD MS Teams system for safety/confidentiality and will be returned duly filled by the minor that expressed the interest in being involved in the EJPRD training activities. Once the minor has been selected to participate in the workshop, a specific parental authorisation together with the minor assent form will be requested to use personal and sensitive information in accordance with GDPR requirements.

In the registration form, the following information will be requested:

- Name and surname
- Age
- Country of residence
- Rare disease affected (YES/NO, without specifying the kind of rare disease)
- English knowledge
- Availability to attend the meeting on Friday or Saturday and suggested timeline
- Availability to travel and overnight to attend the meeting (only for F2F meetings)
- Special needs and requirements (allergies, intolerance, visual impairment, deambulation problems) for face to face sessions.

The participants will be selected according to the following criteria:

- Good English proficiency
- Age (between 12 and 18)
- Country (in order to guarantee a fair geographic representation, no more than 2 minors per country will be selected
- Motivation and interest in improving health research
- Participation in specific advisory groups or paediatric patients organisations.

The application forms will be analysed and selected by TEDDY and FSJD representatives and leader of the task 15.4.

5. Training Plan

The training plan has been prepared with the following aims:

- develop a comprehensive set of educational materials and tools of empowerment on rare diseases, paediatric medicines development and clinical research, in order to have young patients trained with a common base of knowledge and specific expertise related to their own disease;
- train "expert" paediatric patients able to be an active part in all the decision-making processes related to health and research and help them to contribute in the definition of unmet needs to provide input in the design of the protocol, improve communication with the target population and brainstorm methods for dissemination of findings;
- fill an educational need, providing the tools of empowerment to the paediatric patients with rare chronic conditions to design better treatments and care for them;
- increase the awareness and promote the advocacy and advisory role of paediatric patients in rare disease research and drug development.

The training plan has been revised by the members of the Programme Committee as well as by the members of the Young Programme Committee. In order to engage as many young people as possible and ensure that the training plan could really meet the interest and the needs of the target audience, we have involved in the revision activities all the members of the European Young Persons Advisory Groups (YPAGs) to whom the members of the Young Programme Committee belong.

Please find attached as Annex 1 the evaluation forms received by KDS Bari and KIDS Albania as example of children participants. All their feedbacks have been included in the training plan and will be especially taken into consideration for the preparation of the training contents (that will be again reviewed by the Young people), and in the training plan that will be used for the next face to face sessions in 2022 and 2023.

Due to the pandemic situation, the first paediatric workshop will be held virtually. Therefore, a simplified version of the training plan will be adopted for this online edition, as described in the paragraph 5.1. This simplified structure takes account of difficulty keeping partecipants engaged for too many hours. In this case, a session per month of maximum 2 hours and a half starting from November 2021 and ending on February 2022 will be organised.

The training plan will be updated according to the specific topics and case studies proposed by the Programme Committee, and to address feedbacks received by the young patients and the speakers that participated in the previous editions of the paediatric expert patients training workshops.

TEDDY and FSJD will be responsible for the preparation of the training materials and the development of training tools according to the agreed training plan approved by the programme committee and, where relevant, with the involvement of YPAGs' members, organisation of the virtual/F2F meetings and selection of the young participants. Moreover, based on the training contents developed during the F2F courses, some educational webinars addressed to young patients (and their families) affected by rare diseases will be organised and disseminated through the patient organisations and relevant networks.

The ideal training plan, that has been prepared for the F2F workshops, is composed of:

1. **General modules**: dealing with general information about rare diseases and orphan drugs, ethical/legal aspects, patients advocacy, engagement that will be common to all the workshops;
2. To strengthen cooperation with ERNs, scientific **contents** will be adapted each year in accordance with the rare diseases identified in the following areas:
    - Cancers' diseases
    - Metabolic diseases

- Hematological diseases
- Neurodegerative diseases
- Other

3. **Case studies** and **practical activities.**

The training plan has been divided in several sessions according to the different topics covered.

For each workshop a rare disease will be identified, and specific case studies and scientific contents will be prepared and adapted by the selected speakers that will be experts in the field and members of the programme committee "on the basis of their expertise" for the relevant parts. For the first online edition, the training contents will be more general and not related to any specific disease area in order to widen the target audience and promote the participation of paediatric patients.

1st Session – Introduction on the EJP RD Project and ice-breaking activities

Learning objectives

The first session will be aimed to introduce the EJPRD project and the framework in which the activity has been organised. Some icebreaking activities will be organised as facilitation exercises intended to help the members of the group to begin the process of identifying themselves as a team as well as to help them to get to know each other.

In particular, the following topics will be addressed:

- *What is EJP RD?*
  EJP RD is an EU-funded project aimed to improve integration, efficacy, production and social impact of research on rare diseases through the development, demonstration and promotion of sharing of research and clinical data, materials, processes, knowledge and know-how, and an efficient model of financial support for research on rare diseases.
- *How EJP RD intends to involve children and young peoples in the project?*
  In order to make patients and laypersons ready to actively participate in scientific research, it is beneficial and important they are trained and properly educated on the main themes of scientific and biomedical research.
  One of the main goals of EJP RD is to raise the level of knowledge and know-how within the rare diseases (RD) research and care community, including adults and paediatric RD patient representatives and advocates and RD patients. One of the aims of EJP RD is to prepare rare disease patients to engage in scientific innovation and research through workshops, educational material and activities for paediatric patients.

2nd Session: Genetic /rare diseases characteristics and common challenges

Learning objectives

The second session will be aimed at providing an insight on what a rare disease is, and what the common challenges might be faced by people living with a rare condition:

In particular, the following topics will be addressed:

- *What is a rare disease?*
  A disease is defined as rare in Europe when it affects fewer than 1 in 2,000 people. There are over 300 million people living with one or more of over 6,000 identified rare diseases around the world. Rare diseases currently affect 3.5% - 5.9% of the worldwide population and each rare disease may only affect a handful of people, scattered around the world. 72% of rare diseases

are genetic starting in childhood, whilst others are the result of infections (bacterial or viral), allergies and environmental causes, or are degenerative. The main features of genetic rare diseases as well as the impact on growth and QoL will be analysed.

- *Common challenges*
  The lack of scientific knowledge and quality information on the disease often results in a delay in diagnosis. The broad diversity of disorders and relatively common symptoms which can hide underlying rare diseases can bring to an initial misdiagnosis, considering that symptoms differ not only from disease to disease, but also from patient to patient suffering from the same disease. The need for appropriate quality health care causes problems and difficulties in access to treatment and care and often results in heavy social and financial burdens on patients.
- *The importance of investing in paediatric research and it can make the difference.*

A case study on the rare diseases specificities will be developed.

3rd Session: Engagement, involvement and advocacy and the role for paediatric patients

Learning objectives

The awareness and involvement of paediatric patientsis important to improve and shape clinical research in order to address patients needs and priorities. With this aim, it is essential to increase the knowledge of children and their families about biomedical research and drug development by providing them with training with a child friendly methodology and adapted language using also case studies and practical activities. Thus, this session will be aimed to cover the following topics:

- *Patients' engagement, involvement and empowerment in rare disease research*. What does patient advocacy and *engagement* mean? What is the difference between empowerment, engagement and involvement? How patients can contribute in progressing paediatric research? How can patients advocate and inform researchers during all phases of research process? What is their role and why is so important their direct involvement in paediatric research?
- *Patient advocates at Institutional level.* How patients advocate within the European Medicines Agency (EMA) at different levels (e.g. as members of the COMP, PDCO, PCWP scientific committees and working party). How advisory groups can collaborate with other European/international institutions (e.g. European Parliament, Council of Europe), with the aim to strengthen children's participation in the decision-making process on matters regarding their health.
- *The ongoing scenario characterized by patients' associations and advocacy groups will be explicated and the opportunity of advocacy and engagement activities presented*
- *Example of social awareness campaigns and meetings will be presented, and practical activities organised.* Interactive session with the involvement of participants to create ideas for social media campaigns will be organised. Rare Disease Day (28th of February 2022) provides a fantastic opportunity to bring learning from the online course to life. Rare disease Day aims to raise awareness amongst the general public and decision-makers about rare diseases and their impact on patients' lives. The participants will develop a virtual campaign and a slogan and can also plan for a public event.

4th Session: Clinical research at a glance

Learning Objectives.

This session will be aimed at providing information on the differences between trials and other clinical studies (e.g. post-marketing observational studies), making aware the participants of the complexity of the process underlying the drug clinical development as well as providing information about the need of conducting clinical trials ad hoc for children.

The following topics will be addressed:

- Introduce what is clinical research and why it is so important
- Clinical research phases: from the first administration in human to observation of effects in real life.
- The difference between adult vs. children clinical trials and the clinical study design process
- What is a clinical protocol and who can participate in clinical trials (what is a blind, double-blind, double-dummy clinical trial? What is a randomized clinical trial? What is a Placebo?)

As case study activity a mock trial will be prepared.

Example of case study

1st phase: the play
The participants will be grouped in teams. Each team will consist of:
1 pharmacist
1 Doctor
2 Patients

The role will be identified by a post-it card attached on actors' clothes. The Pharmacist gives the medicines to the doctor, giving instructions on ***which*** *medicine will be given to* ***who.*** *It will be nice to replace medicine with candies with several tastes and shape or anything similar. If variety of ages in the group, best to give the older group members the tasks of playing pharmacist and doctor.*

OPEN-LABEL team:
Each pharmacist gives instruction to the doctor and writes on a blackboard which medicine will be received by the patient. The Doctor explains the shape, colour and taste of the medicine the patient will be receiving. The two patients will receive two ***different*** *medicines (candies) in shape, colour and taste. Each Patient from each team will be asked on their impressions on the shape, colour and taste of the medicines they have received.*

BLIND team*:*
Patients are now blinded with some scarfs or anything similar. The pharmacist gives instruction to the doctor and writes on a post it card the medicine the patient will receive, sticking the card on each patient's back. The doctor gives instruction on how to take the medicines and gives it to the patients. The two patients will receive the ***same*** *medicines (candies) in shape, colour and taste.*
Patients will be asked to answer on the expected shape, colour and taste of the medicine they have received. Now patients are asked on their impressions and the doctor reports them on each team's paper/blackboard.

DOUBLE BLIND team*:*
The Patient and Doctor are now blinded with some scarfs or anything similar. The pharmacist wites on a post-it card the medicine the patient will receive, sticking the card on each patient's back. The two patients will receive the ***same*** *medicines (candies) in shape, colour and taste. Patients will be asked to answer on the expected shape, colour and taste of the medicine they have received. Now patients are asked on their impressions and the doctor reports them on each team's paper/blackboard.*
In the end the shape, colour and taste of the given medicines will be unveiled and compared with the patients' impressions.

2nd phase: discussion

All Participants are asked to answer some questions (1 question at a time) on piece of papers which will be folded and put inside a pocket. Once every participant have answered, someone will read at loud the answers by pulling out answers from the pocket opening a discussion.

Questions asked to all participants:

- *Which are the main differences of the three scenarios?*
- *Which are the advantages of each scene?*
- *Which are the drawback of each scene?*

The practical rationale behind the blinding and unblind will be unveiled to the participants through a ppt presentation

5th Session: The regulatory framework

Learning Objectives

This session will be aimed to describe the specific legal framework that has been adopted at European and international level to facilitate the availability of the Orphan Medicinal Products. Thus, the following topics will be addressed:

- *What is an orphan drug?*
- *Orphan and Paediatric Regulations.* This regulation provides incentives to pharmaceutical companies to develop and market medicinal products to treat rare diseases. What is the current European regulatory framework?

6th Session: Monitoring patients safety

Learning Objectives

This session will be aimed at explaining the role of the pharmacovigilance (PV or PhV) that is the pharmacological science relating to the collection, detection, assessment, monitoring, and prevention of adverse effects with pharmaceutical products. Monitoring Patients safety is the most important and critical part of a clinical study. All the safety data should be reported in order to evaluate the risk-benefit ratio of the new treatment and guarantee patients wellbeing. This session will provide information on what it is necessary to do, in case of side effect possibly due to a treatment as well as how and where to communicate and report an adverse event. The session will explain in detail that the Adverse Event is an undesired effect that may occur which may or may not be related to the treatment.

During the session, a case study will be prepared.

7th Session: Paediatric research in an ELSI and data protection perspective

Learning Objectives:

Particular attention will be paid to the fundamental rights of children and the participation of children in the decision-making process on matters regarding their health. This will be aligned to the UN Convention on the rights of the child. To help learners understand why privacy and data protection compliance is of paramount importance within paediatric research this topic will be covered and will include: fundamental rights but also in common and agreed standards and practices. GDPR introduced many novelties to be considered in carrying out research involving personal data processing and data sharing and will be covered

This session aims at providing an overview of the importance and the implications of GDPR in research, of children rights in processing personal data for research and common tools and guidelines developed. The session will relate this topic to relevant ethical issues in the context of rare diseases.

The main elements of the informed consent and assent forms will be explained.

One case study including an assent form for children related to data processing will be prepared asking the participants to revise the informed consent forms and identify the best practice.

Example of case study

The participants will receive different types of informed consent form for the same clinical trial, (including the information/assent form for data processing, reuse and sharing). They have different structures, layouts and information. Participants are invited to assess the types of document and evaluate which fits best for the different age groups and identify the best practice to be used.

As a second activity, participants will receive the Patient Information Sheet and the Consent form, in which 5 elements are missing and 5 mistakes have been included. They have to identify the 10 elements to be corrected. Then the discussion will be open on the following questions:

Consider the Patient Information Sheet and the Consent form is there anything missing that is essential? Are they too long? Do they provide enough information? Do they explain the issues in taking and giving consent? Identify the strengths and weaknesses – listing things to be improved.

For the first edition, practical activities will be focused on the information/assent form for processing, reuse and sharing data in different age groups.

5.1. Simplified Training Plan for the online edition

Due to the pandemic situation, many adolescents all over Europe have had to attend the school lessons remotely. Therefore, in order to avoid to keep them engaged online for too many hours, it is decided to prepare a simplified version of the training plan for the first edition of the paediatric training workshop. In order to attract their interest and generate valuable outputs, several practical activities and case studies have been foreseen and a participatory methodology used.

To this aim, the following training plan has been drafted.

Meeting	Period	Activity	Sessions
1	November 2021	An introductory session about the EJPRD goals and activities and ice-breaking activities will be organised. A general introduction about rare disease peculiarities, with a practical case study will be given	• Introductory session about the EJPRD goals and activities (session 1 - 30 minutes). • Ice-breaking activities (session 2 - 30 minutes) • General introduction about the rare diseases peculiarities with a practical case study (session 3 - 60 minutes). • Conclusion (15 minutes)
2	December 2021	There will be a session dedicated to clinical research with the aim to introduce	• Session dedicated to clinical research (session 2 - 20 minutes)

		what is clinical research, the different phases of clinical research, the main elements of the clinical protocol. A session dedicated to patients' engagement will be organised to empower the participants on how patients can contribute in progressing paediatric research. As case study on clinical research, the "My Clinical Trial" serious game will be used. The digital game with an educational purpose has been created by TEDDY in collaboration with KIDS Bari and Albania young members. It aims to explain to children in a fun way what clinical trials are, how they work and why they are so important to developing drugs suitable for children. The game is extremely informative, as game-players will learn about clinical trial, study protocol, informed consent and assent in children, phases and procedures of clinical trials, data collection, and pharmacovigilance. It is also a positive example of patients engagement. During the meeting, the participants will start design the video that will be prepared for the Rare Disease Day.	• Session dedicated to patients' engagement (session 1 - 30 minutes). • Case study on clinical research: presentation of the "My Clinical Trial" serious game (session 3 – 50 minutes) • Introduction of the project of the video to be developed for the Rare Disease Day (session 4 - 15 minutes). Instructions will be sent by email to all participants to receive their feedback and start developing the video to be finalized in the last session.
3	January 2022	A special focus on the ethical aspects of paediatric research including an overview on a	• Session on the ethical aspects of the paediatric research (session 1 – 60 minutes)

		informed consent and assent form, and the types of consent/ assent forms that are used. Some examples of informed consent form and assent forms will be distributed to participants as pre-reading materials for the following meeting. A session aimed at providing an overview of GDPR rules and impact for research as well as of the children's rights in processing personal data for research data protection will be organised.	• Session on GDPR rules and impact for research as well as of the children's rights in processing personal data for research data protection (session 2 – 60 minutes) Both sessions will include case studies
4	January 2022	A practical session will be organised. For the first edition, practical activities will be focused on the information/assent form for processing, reuse and sharing data in different age groups. Participants will receive the Patient Information Sheet and the Consent form to identify the elements to be improved. Then, the discussion will be open on the following questions: Consider the Patient Information Sheet and the Consent form is there anything missing that is essential? Are they too long? Do they provide enough information? Do they explain the issues in taking and giving consent? Identify the strengths and weaknesses listing things to be improved.	• Practical session (session 1 - 2 hours)
5	February 2022	A session to present the regulatory framework of the paediatric research as well as the basic concepts of adverse events and monitoring. During the meeting, the	• Session on regulatory framework of the paediatric research (session 1 – 45 minutes) • Finalisation of the video (session 2 – 60 minutes)

		participants will finalise the video to be disseminated during the Rare Disease Day.	• conclusions and take-home messages (15 minutes)