Young patients' engagement in the European Joint Programme on Rare Diseases – EJPRD

Mariangela Lupo

European Network of Excellence for Paediatric Clinical Research

Via Luigi Porta 14, 27100 PAVIA - ITALY • VAT 01825900184

www.teddynetwork.net • info@teddynetwork.net

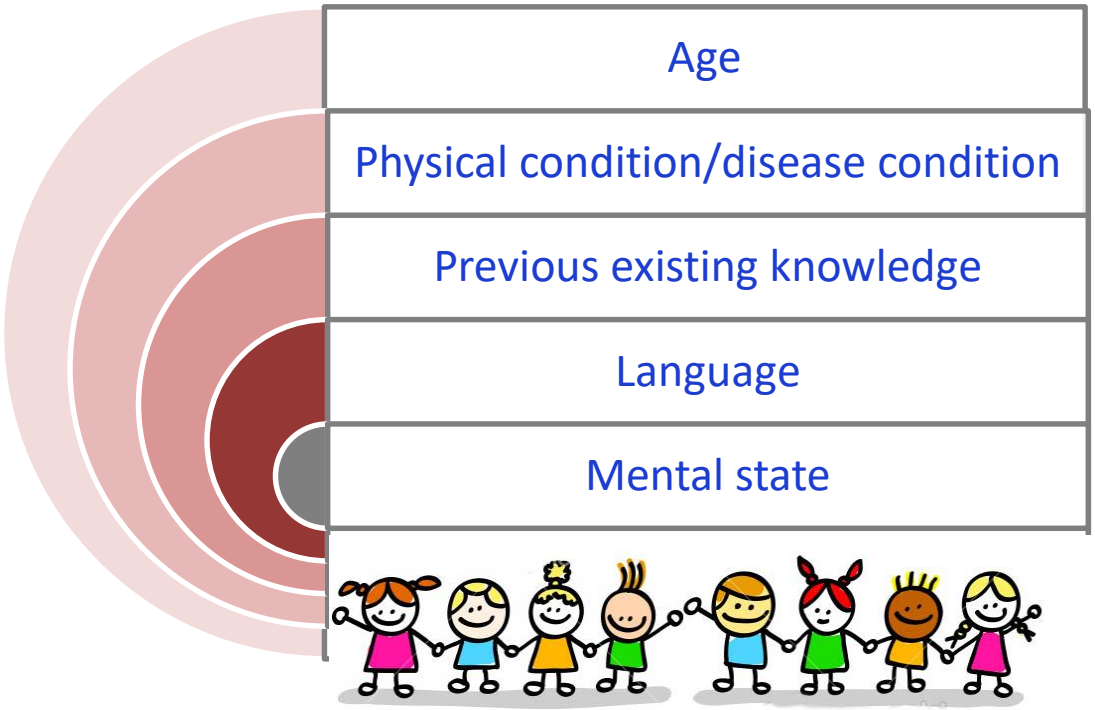
Can children be involved in this process?

The UN Convention on the Rights of the Child, Article 12 states Children and Young People have a right to have their views heard in all matters affecting them and for these to be taken seriously

TEDDY NETWORK
European Network of Excellence for Paediatric Clinical Research

Challenges in the engagement of children

Some key factors influence children comprehension of the information and the consequent involvement in clinical research process

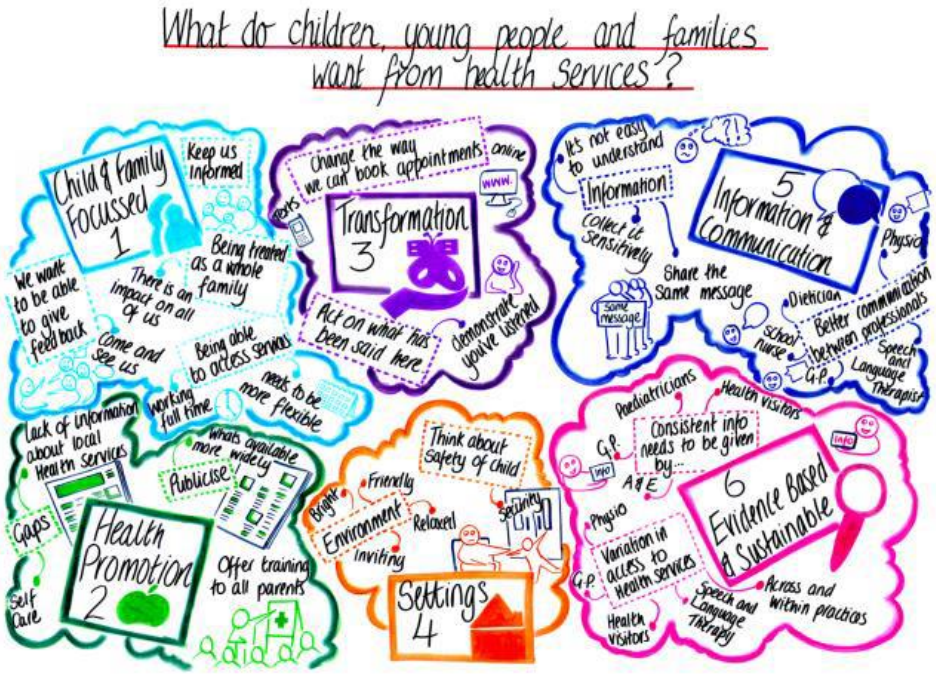


TEDDY NETWORK
European Network of Excellence for Paediatric Clinical Research

Young patients engagement

- Engaging children early on in the research process and educating them about the importance of participating in clinical research, could help in reducing the patients' retention and fostering treatments compliance.

- A patient-centred approach promotes the collaboration between the different actors of the research process and should be included in the best practices of the paediatric research in order to enhance quality and relevance of the research itself.



TEDDY NETWORK
European Network of Excellence for Paediatric Clinical Research

Specifically tailored methods should be applied to the training and empowerment process of paediatric patients

- Due to the paediatric specific developmental characteristics, specifically tailored methods should be applied to the training and empowerment process of paediatric patients.
- Educate and empower the paediatric patients in the specific topic of the project for which their help is requested. For example, improve the language, content and format of the assent document.
- Dynamic interactivity to collect feedback aiming to improve the project by using the best methods to collect the information. For example, focus groups, questionnaires or surveys, personal interviews, etc.

European Joint Programme on Rare Diseases

EJP-RD aims to create a research and innovation pipeline "from bench to bedside" ensuring rapid translation of research results into clinical applications and uptake in healthcare for the benefit of patients.

TEDDY NETWORK
European Network of Excellence for Paediatric Clinical Research

European Joint Programme on Rare Diseases

- **Union contribution:** 55 M€ (70% reimbursement rate)
- **Total budget (min. submitted):** 101 M€ (→ expected > 110 M€)
- **Number of partners:** 88
- **Number of participating countries (beneficiaries and LTPs):** 35 including 27 EU MS (AT, BE, BG, CZ, DE, ES, EE, FI, FR, GR, HU, HR, IE, IT, NL, LT, LV, LU, MT, PL, PT, RO, SE, SK, SL, SV, UK), 7 associated (AM, CH, GE, IL, NO, RS TK) and third countries (CA)
- **Timeline:** Jan 2019 – Dec 2023

- **Types of partners:**
  - 33 research funding bodies/ministries
  - 12 research institutes
  - 22 universities/hospital universities
  - 11 hospitals
  - 5 EU infrastructures (BBMRI, EATRIS, ECRIN, ELIXIR, INFRAFRONTIER) + EORTC
  - EURORDIS & ePAGs
  - 6 charities/foundations (FTELE, AFM, FFRD, FGB, BSF, IT)

European Joint Programme on Rare Diseases

The specific objective of EJP-RD is 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

The whole programme is based on the mixture/integration of already existing activities/programs/tools/trainings and the creation of new ones (new added value)

EJP RD STRUCTURE

COORDINATION & TRANSVERSAL ACTIVITIES

INTEGRATIVE RESEARCH STRATEGY

SUSTAINABILITY

ETHICAL & REGULATORY

COMMUNICATION

1 FUNDING

2 COORDINATED ACCESS TO DATA & SERVICES

3 CAPACITY BUIDLING & EMPOWERMENT

4 ACCELERATING TRANSLATION OF RESEARCH & THERAPY DEVELOPMENT

WP1 COORDINATION & MANAGEMENT

WP2
STRATEGY

WP3
SUSTAINABILITY

WP4
ETHICS, LEGAL, REGULATORY & IPR

WP5
COMMUNICATION & DISSEMINATION

WP6
Joint Transnational Calls

WP7
Networking scheme

WP8
RDR Challenges

WP9
Monitoring of funded projects

WP 10
User-driven strategic planning for P2

WP 11
Virtual Platfform for data & resources

WP 12
Enabling sustainable FAIRness

WP 13
Holistic approaches for rare disease diagnostics and therapeutics

WP 14
Training on data management & quality

WP 15
Capacity building and training of patients and researchers

WP 16
Online Academic education course

WP 17
ERN RD training and support programme

WP 18
Development and adaptation of training activities

WP 19
Facilitating partnerships and accelerating translation

WP 20
Validation , use and development of innovative methodologies for clinical studies

TEDDY NETWORK
European Network of Excellence for Paediatric Clinical Research

Inserm
La science pour la santé
From science to health

Pillar 1: funding collaborative research on RD

The Pillar will foster joint transnational calls for collaborative research projects **resulting in financial support** to third parties encompassing various aspects of rare diseases.

In addition, the financial support actions will be extended to the Networking Scheme (to encourage sharing of knowledge on rare diseases between clinicians, (biomedical) scientists and patient organizations) and the Rare Disease Research Challenges (innovative funding scheme to foster private-public partnerships and multistakeholder collaborations).

TEDDY NETWORK
European Network of Excellence for Paediatric Clinical Research

Pillar 2: Innovative coordinated access to data and services for transformative RD research

The Pillar will **develop a FAIR virtual platform for RD information** encompassing research data, samples, tools and standards to support and accelerate rare diseases research. In addition, **pilot and proof-of-concept projects will be put in place** to enable multidisciplinary, holistic approaches for rare disease diagnostics and therapeutics and fostering creation of complete diseases pathways.

TEDDY NETWORK
European Network of Excellence for Paediatric Clinical Research

Pillar 3: capacity building and empowerment

The pillar will be raising the level of knowledge and know-how within the RD research and care community, including through ERNs and RD patient representatives and advocates. The pillar foresees the development of:

- a **comprehensive programme of training courses** will tackle areas such as research data management, orphan drug development, HTA & regulatory processes, best practice guidelines and ERN cross-cutting themes & needs.
- training courses will be developed and adapted to address the specific needs of EU13 countries
- first-in-Europe online academic course to provide an EU-wide **streamlined education programme on RD** research to all interested stakeholders.

TEDDY NETWORK
European Network of Excellence for Paediatric Clinical Research

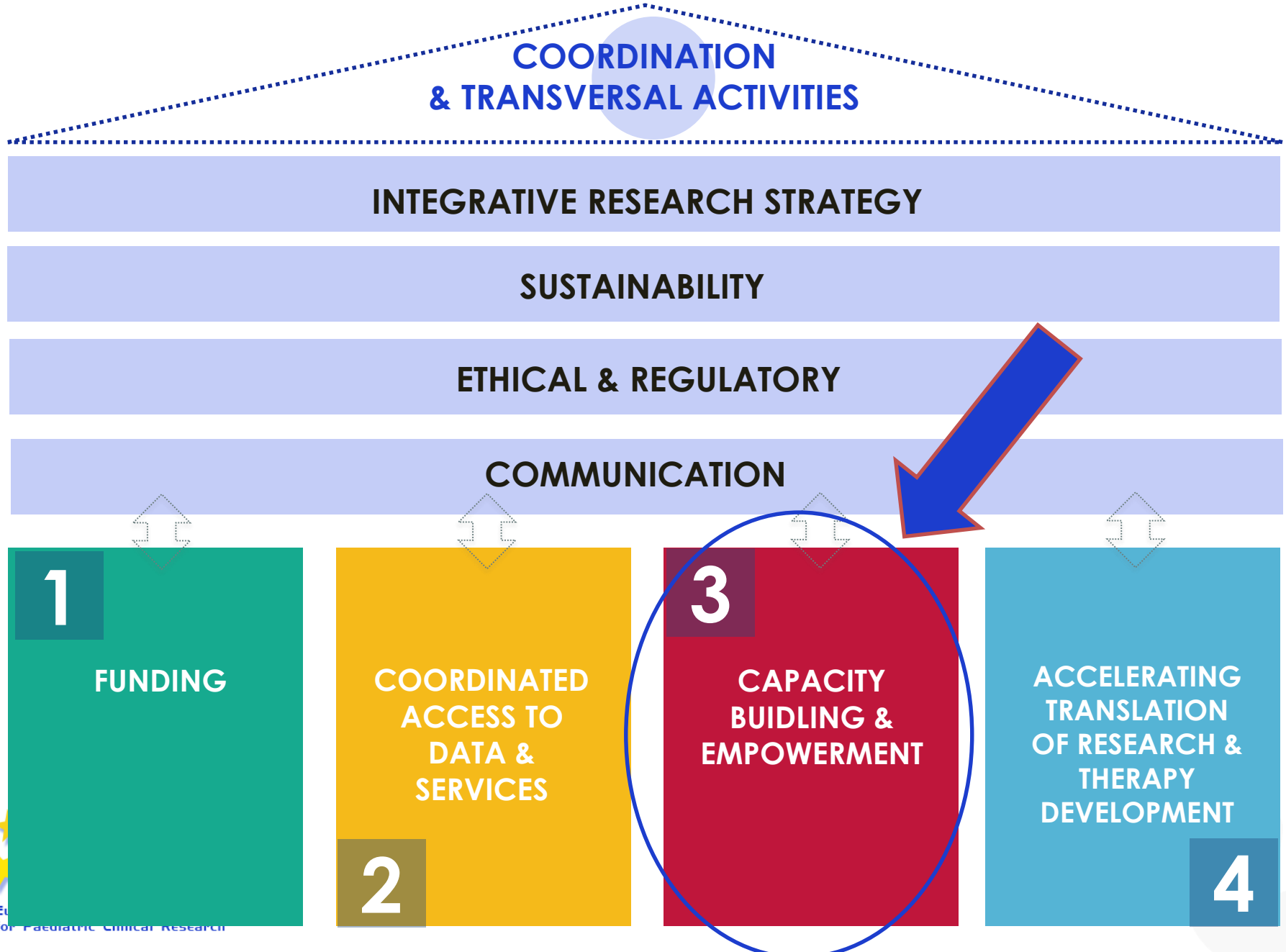
Pillar 4: accelerating the translation of high potential projects and improving outcomes of clinical studies in small populations

By creating a bridge between basic research and medical innovation, otherwise known as the “bench to bedside” approach, this pillar aims to support the RD community to more effectively translate high quality research into high impact interventions for the RD patient community. To this aim:

- Research projects funded through Pillar 1 and previously by E-Rare will be selected for their translational potential and specifically supported and guided throughout this process.
- Pillar 4 will foster the development of innovative methodologies tailored for clinical studies in RDs by specifically supporting ERNs to use the most adapted methodologies improving trial studies in RDs.
- Demonstration studies will be funded to validate existing and most recently presented innovative methodologiesas well as most promising new statistical methodologies;

TEDDY NETWORK
European Network of Excellence for Paediatric Clinical Research

TEDDY INVOLVEMENT



PILLAR 3

The overall objective of this WP is to address one key component of the EJP on RD that is to improve RD research and innovation and to enhance the uptake of research results by building the capacity of the patient community and other key stakeholders.

WP15: Capacity building & training of patients and researchers in rare diseases research and processes

Expert Patients and Researchers EURORDIS Summer school – scientific innovation and translation research aspects in RDs for patient advocates – leadership & communication skills for patient advocates and representatives – eduocation material and activities for paediatric patients

WP16: Online academic education course

Based on assessed needs of the RD community – in collaboration with universities – 10 to 12 modules with accreditation – e-learning format open to all – Future Learn platform

WP17: ERN RD training & support programmes

NETWORK
European Network of Excellence for Paediatric Clinical Research

TEDDY LEADS TASK 15.4

Educational materials and activities for paediatric patients

The planned activities include short workshops in collaboration with Fundaciò Sant Joan de Deu, EURORDIS and the eYPAGnet (the European Young Persons' Advisory Group network) for 15 patients' representatives focusing on the following areas:

- Genetic /rare disease characteristics and evolution
- Role for paediatric patients and relevance of clinical and translational research (including patients' engagement in clinical trials, the consent issue, the patient-reported outcomes in the context of paediatric rare diseases)
- Orphan Medicinal Products availability and access for paediatric patients on the EU market, pharmacovigilance and off-label use in paediatric setting

TEDDY NETWORK
European Network of Excellence for Paediatric Clinical Research

TEDDY LEADS TASK 15.4

Educational materials and activities for paediatric patients

The Face to Face 'paediatric patient experts training course' will be hosted in Italy (TEDDY), Spain (JSJD) and France (EURORDIS).

KiDS Bari

KiDS Albania

KIDS BARCELONA

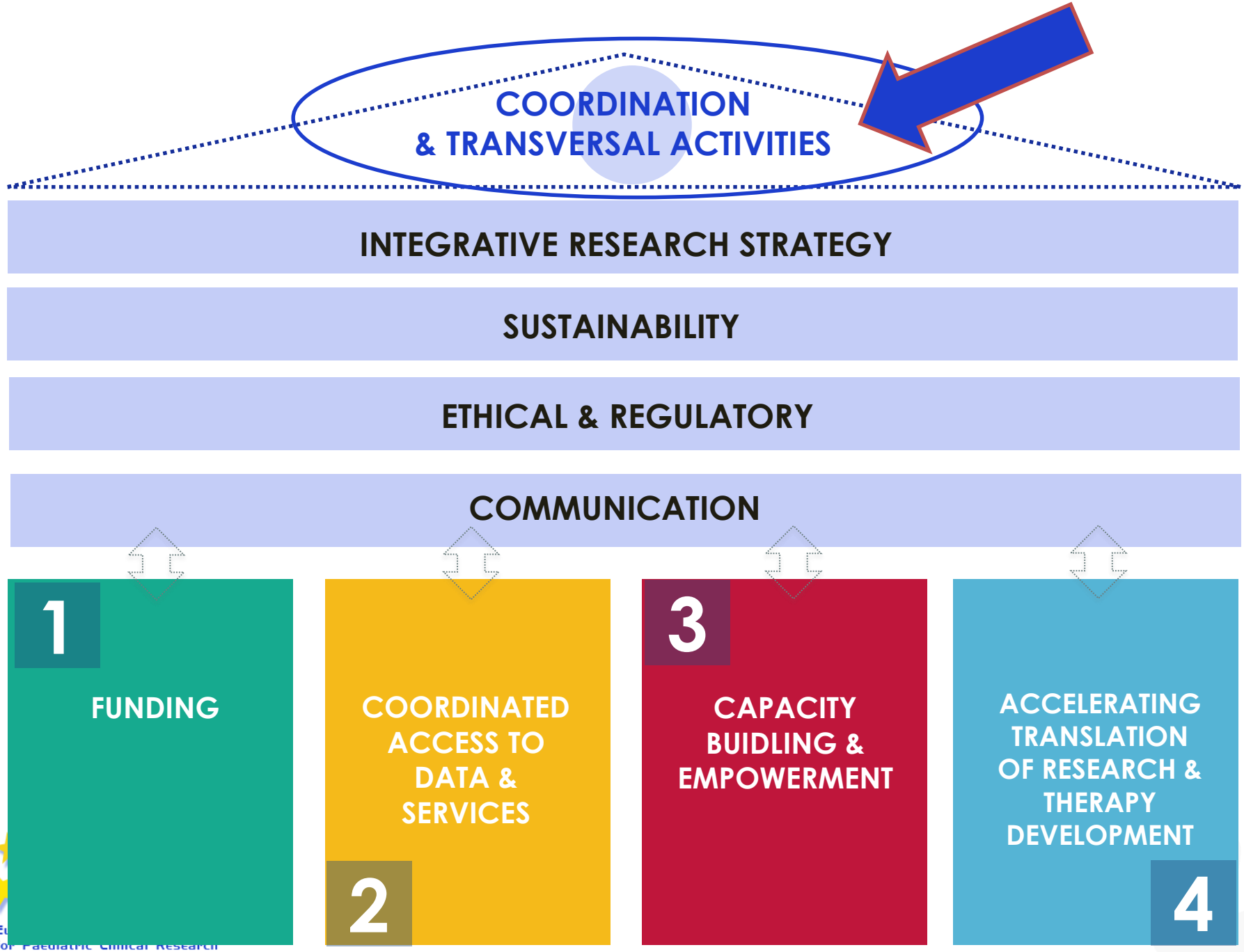
EURORDIS RARE DISEASES EUROPE

TEDDY NETWORK
European Network of Excellence for Paediatric Clinical Research

TEDDY as contributing entity

- TEDDY is among the few organisations whose members have been considered in the Consortium Agreement as Contributing Entities and have rights to access to the Background and the Results of the project at the conditions set out in the Consortium Agreement.
- This is a very important achievement as it means that TEDDY members are considered in the project as EJPRD Linked Third parties, in the same way as the members of the involved ERICs and ERNs.

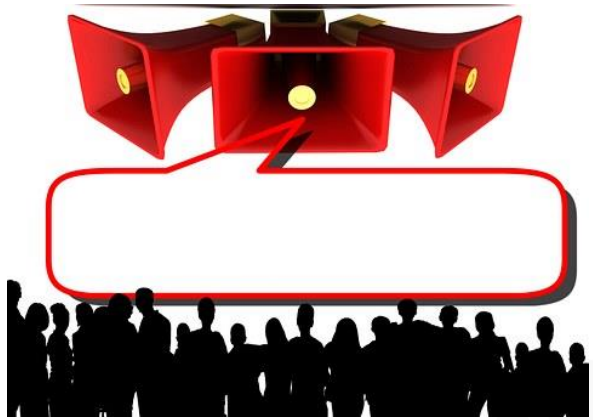
TEDDY NETWORK
European Network of Excellence for Paediatric Clinical Research


COORDINATION
& TRANSVERSAL ACTIVITIES
INTEGRATIVE RESEARCH STRATEGY
SUSTAINABILITY
ETHICAL & REGULATORY
COMMUNICATION
1
FUNDING
2
COORDINATED ACCESS TO DATA & SERVICES
3
CAPACITY BUIDLING & EMPOWERMENT
4
ACCELERATING TRANSLATION OF RESEARCH & THERAPY DEVELOPMENT

COORDINATION & TRANSVERSAL ACTIVITIES

- PROGRAMME MANAGEMENT & COORDINATION
- INTEGRATIVE RESEARCH & INNOVATION STRATEGY
- SUSTAINABILITY
- ETHICS, LEGAL, REGULATORY & IPR
- COMMUNICATION & DISSEMINATION

TEDDY will be involved in the project in the communication activities. It will provide its experience in the field of paediatrics, ensuring the right awareness on the importance of the paediatric aspects in the field of the rare disease research.



TEDDY NETWORK
European Network of Excellence for Paediatric Clinical Research

CONCLUSIONS

TEDDY will be central to further engage and empower rare disease patients into research and provide them with the tools required to apprehend and actively contribute to innovation and therapy development.

There will also be relevant links and synergies with the ongoing H2020 project EPTRI European Paediatric Translational Research Infrastructure, coordinated by CVBF, the H2020 project PEDCRIN Paediatric Clinical Research Infrastructure Network coordinated by ECRIN and with the IMI2 C4C project.

TEDDY NETWORK
European Network of Excellence for Paediatric Clinical Research