The INVENTS Patient Preference Questionnaire Validation

2nd ERDERA Training for Young Advocates for Rare Diseases Paris, France

23-25 April 2026

Donato Bonifazi – TEDDY Chair

INVENTS has received funding from the European Union's Horizon Europe Research and Innovation program under grant agreement 101136365.

Funded by the Swiss State Secretariat for Education, Research and Innovation (SERI).

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INVENTS

INNOVATIVE DESIGNS, EXTRAPOLATION, SIMULATION METHODS AND EVIDENCE-TOOLS FOR RARE DISEASES ADDRESSING REGULATORY NEEDS

Consortium

Coordinator - **INSERM – INSTITUT NATIONAL DE LA SANTE ET DE LA RECHERCHE MEDICALE**
Paris, France

INSERM – INSTITUT NATIONAL DE LA SANTE ET DE LA RECHERCHE MEDICALE
Paris, France

UU – UPPSALA UNIVERSITET
Uppsala, Sweden

MUW – MEDIZINISCHE UNIVERSITÄT WIEN
Vienna, Austria

HDH – PLATEFORME DES DONNEES DE SANTE
Paris, France

TEDDY – EUROPEAN NETWORK OF EXCELLENCE FOR PEDIATRIC CLINICAL RESEARCH
Pavia, Italy

MUNI – MASARYKOVA UNIVERZITA
Brno, Czechia

MPA – LÄKEMEDELSVERKET
Uppsala, Sweden

ROCHE – F. HOFFMANN-LA ROCHE AG
Basel, Switzerland

RUMC – STICHTING RADBOUD UNIVERSITAIR MEDISCH CENTRUM
Nijmegen, Netherlands

UMG – UNIVERSITY MEDICAL CENTER GÖTTINGEN
Göttingen, Germany

AP-HP – ASSISTANCE PUBLIQUE HÔPITAUX DE PARIS
Paris, France

PMX – PHARMETHEUS AB
Uppsala, Sweden

ECRIN – EUROPEAN CLINICAL RESEARCH INFRASTRUCTURE NETWORK
Paris, France

IT – INSERM TRANSFERT
Paris, France

NOVARTIS – NOVARTIS PHARMA AG
Basel, Switzerland

UOW – THE UNIVERSITY OF WARWICK
Coventry, United Kingdom

INVENTS Project

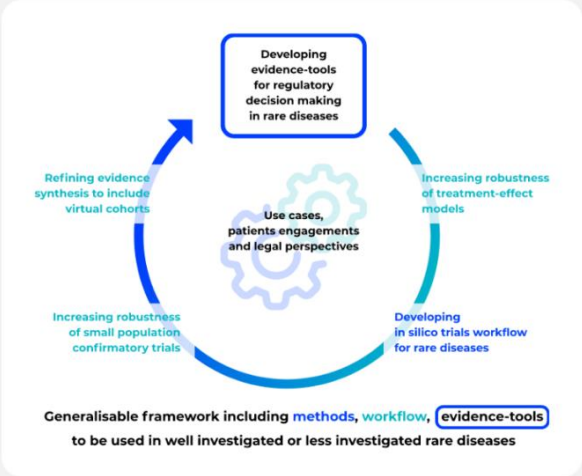
The INVENTS project focuses on advancing the future evaluation of treatments and the regulatory decision-making for rare diseases (RD) including paediatric rare diseases.

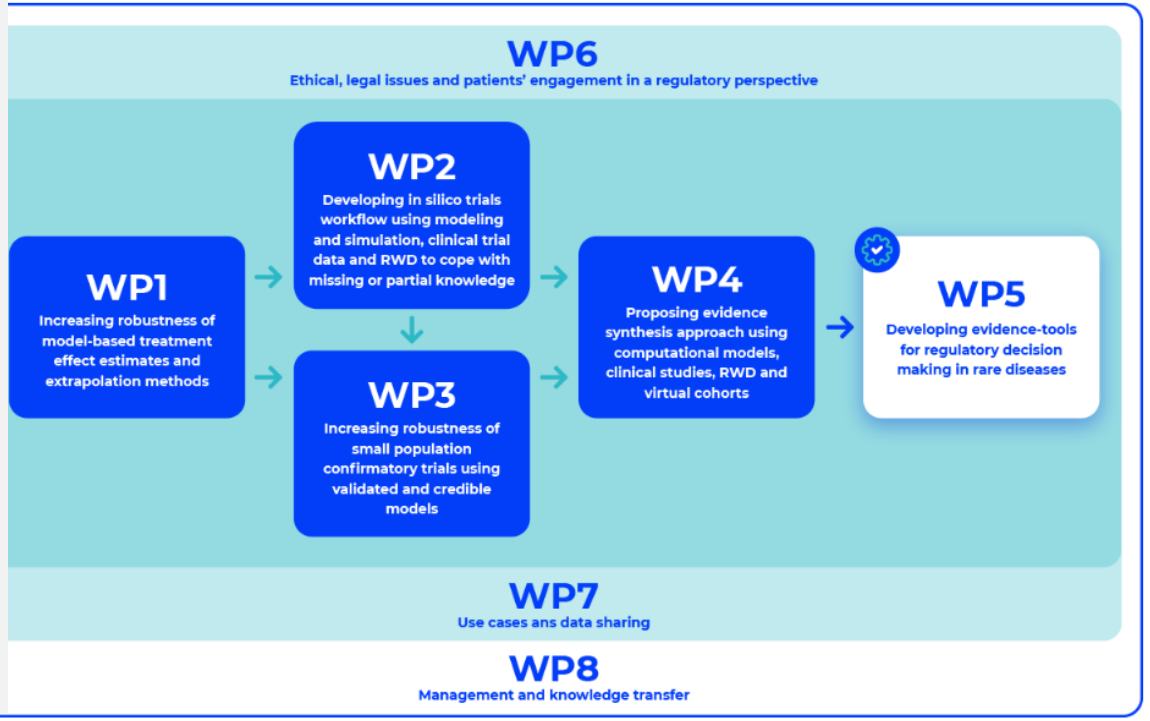
Challenges

Future assessments for rare diseases treatments face challenges :

1. A lack of clinical trials and sufficient evidence to decide for treatment
2. The heterogeneity of rare diseases
3. Outdated research infrastructures and approaches

In a 5 years project, INVENTS aims to enhance decision-making by incorporating patient experiences, creating in silico evaluation models from clinical trial or real-world data, and developing regulatory decision support tools for regulatory agencies.





TEDDY contributes its expertise in ethical and regulatory matters.
TEDDY has established a paediatric Patient Expert Group (PEG) to gather patient feedback and actively involve young members in in silico research and the use of real-world data.

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WP6

Ethical, legal issues and patients' engagement in a regulatory perspective

Overall Objective:

ensure the Ethical, Legal and Regulatory requirements and Patients' perspective implementation

Patient Preference Questionnaire

- This activity is part of the INVENTS project workshop for young patients with rare diseases.

- **The objective is to validate a new "Patient Preference Questionnaire"** designed to ensure clinical trials and treatment authorisations consider what matters most to young patients, not just medical data.



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Patient Preference Questionnaire

Scan the QR code with your phone and start providing your feedback!



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Thank you for your attention

https://invents-he.eu/

Dr. Donato Bonifazi TEDDY Network Chair
ACT-EU MSP Advisory Group, Enpr-EMA Coordinating Group, iCAN Advisoy Board member

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