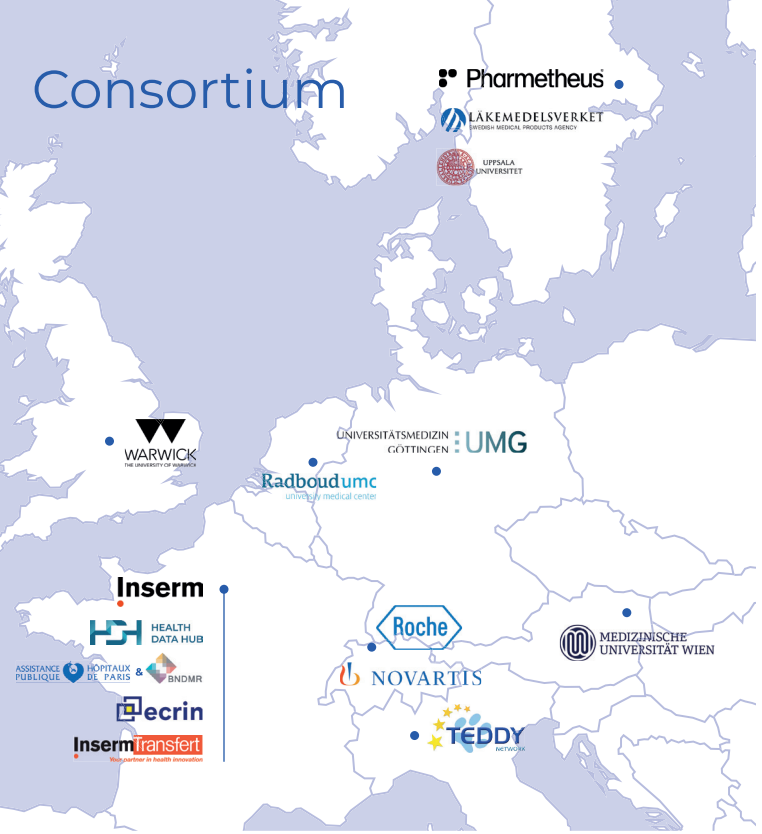
Consortium



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

MPA – LÄKEMEDELSVERKET
Uppsala, Sweden

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

IT – INSERM TRANSFERT
Paris, France

RUMC – STICHTING RADBOUD UNIVERSITAIR MEDISCH CENTRUM
Nijmegen, Netherlands

UMG – UNIVERSITY MEDICAL CENTER GÖTTINGEN
Göttingen, Germany

AP-HP – ASSISTANCE PUBLIQUE – HÔPITAUX DE PARIS, BANQUE NATIONALE DE DONNÉES MALADIES RARES (BNDMR)
Paris, France

PMX – PHARMETHEUS AB
Uppsala, Sweden

ECRIN – EUROPEAN CLINICAL RESEARCH INFRASTRUCTURE NETWORK
Paris, France

NOVARTIS – NOVARTIS PHARMA AG
Basel, Switzerland

UOW – THE UNIVERSITY OF WARWICK
Coventry, United Kingdom

Learn more

Find out more about this groundbreaking study with its extraordinary consortium of international teams, online at:

invents-he.eu

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Contact

Sarah Zohar
Scientific coordinator
INSERM U1346, France

Vincent Damotte
Scientific Project Manager
INSERM U1346, France

invents

Innovative designs, extrapolation, simulation methods and evidence-tools, for rare diseases addressing regulatory needs.

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

- 5 Years duration
- 9 Countries
- 15 Partners
- 27 Clinical trials
- 9 Real world data sets
- 8.4 M€ EU, UK and Swiss funding*

**€6 million European Commission funding, CHF 2 million of subsidy funding by SERI, and £411,804 UKRI funding*

The INVENTS project brings together researchers from universities and industry and patients representatives to improve the development of treatments for rare diseases. By using extensive data from a range of use cases provided by our industrial partners Roche (tocilizumab) and Novartis (secukinumab, fingolimod), and along with real-world data from the 'Banque Nationale de Données Maladies Rares' (BNDMR) and 'Système National des Données de Santé' (SNDS) and advanced computational models and innovative trials designs, INVENTS will enhance evidence from small sample sizes.

Funded by the European Union's Horizon Europe Framework programme under grant agreement 101136365
Funded by the Swiss State Secretariat for Education, Research and Innovation (SERI)
Funded by the UKRI Innovate UK under their Horizon Europe Guarantee scheme

Context

Up to 36 million people in the EU live with one of the more than 7000 distinct Rare Diseases (RD). The evaluation of new medicines for RD and paediatric RD presents several challenges:

- **small patient sample sizes,**
- **variability among patients and diseases,**
- **and differences in disease knowledge.**

As a result, access to effective treatments and the availability of treatment options for RD are often limited.

Concept

The project's main goal is to provide clinical trial stakeholders, trialists and regulators with generalizable methods, designs and evidence assessment criteria for the future evaluation of treatments for RD and paediatric RD.

This evidence-based framework will allow (i) clinical trials' stakeholders to choose the most appropriate approach(es) for developing treatments for both well-investigated or less-studied RD, and (ii) help regulators make informed decisions about small sample evidence in RD with the help of evidence-tools, such as, workflows and guidelines.

Objectives

1. Improving **robustness** of model-based treatment effect estimation and extrapolation methods
2. Developing **in silico trials workflow** using modelling and simulation, clinical trial and Real-World Data (RWD) to cope with missing knowledge
3. Increasing **robustness** of small population confirmatory trials using validated and credible models
4. Proposing **evidence synthesis approaches** using computational models, clinical studies, RWD and virtual cohorts
5. Developing **evidence-tools for regulatory decision-making** in RD
6. Integrating **patient engagement and regulatory perspectives**

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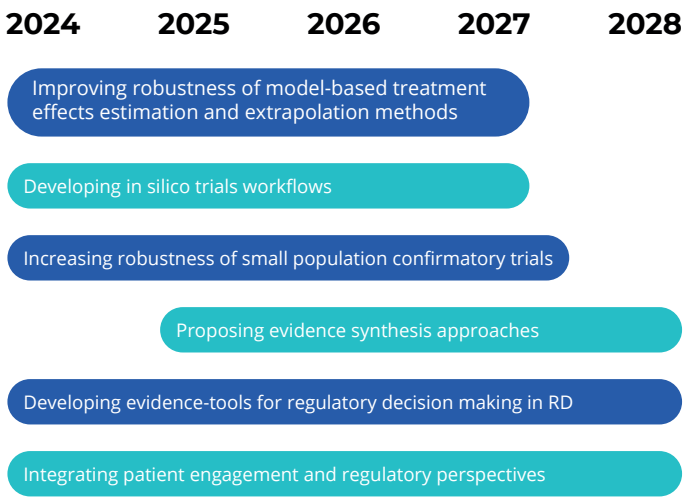
Work plan



Anticipated impact

Access to advanced modeling and simulation tools that accelerate the development of orphan and paediatric medicines.

Accurate computational models to enhance statistical robustness and guide cost-effective trial designs for small populations.

Precise in silico tools for assessing the use of real-world data and estimating risk-benefit effects in small population trials.

Guidance for the use of validated computational models to support a robust extrapolation framework and facilitate the safety and efficacy assessment in the process of regulatory appraisal of orphan and/or paediatric medicinal products.