Exploring INVENTS Work Packages: Building the Foundations for Future Collaboration with the experts

Viviana Giannuzzi

INVENTS paediatric Patient Expert Group Parallel Session

19/09/2026

https://invents-he.eu/

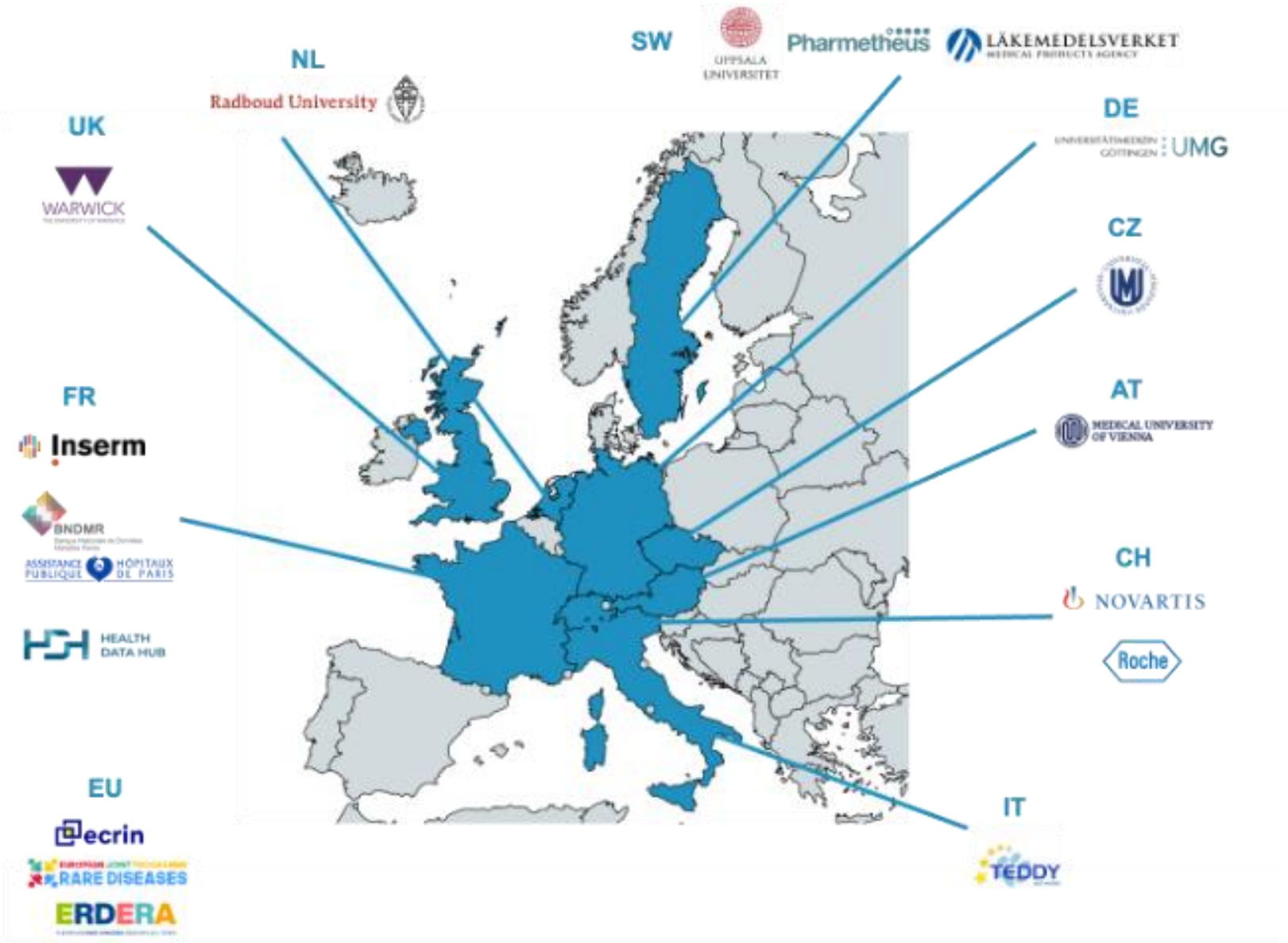
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INVENTS Consortium

1. **Funded by European Union, Switzerland & United Kingdom**
2. **9 Countries**
3. **16 Partners**



The Problem

Clinicians are facing challenges in designing clinical trials for rare diseases

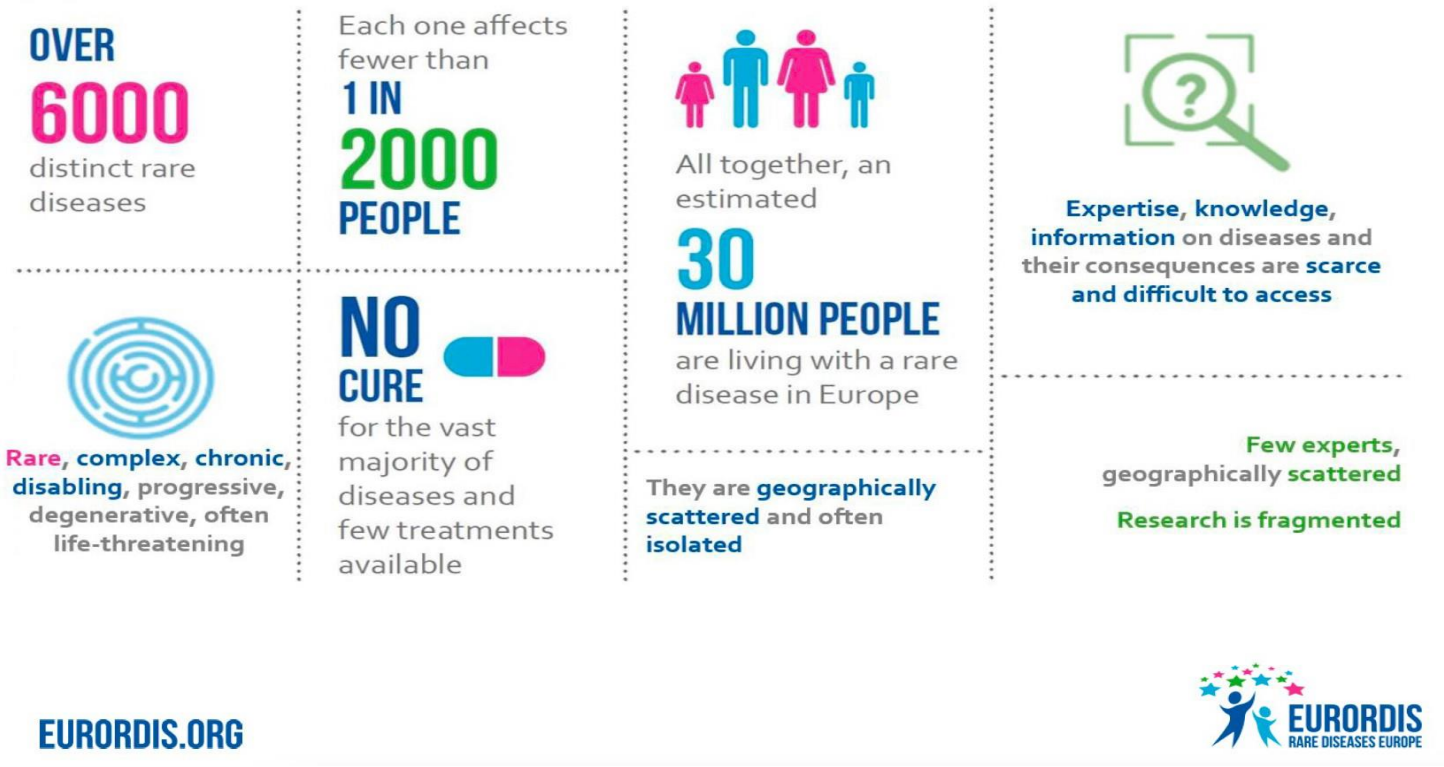


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Rare Diseases



70%

OF RARE DISEASES START IN CHILDHOOD

Image copy right : Raquel Castro, Social Policy Senior Manager , EURORDIS-Rare Diseases Europe

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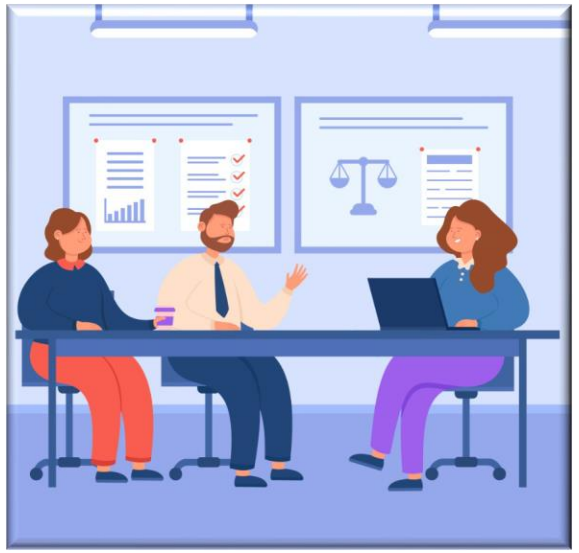
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Our solutions

New methods for clinical trials design in rarediseases



Make european and local regulatory agencies used those methods



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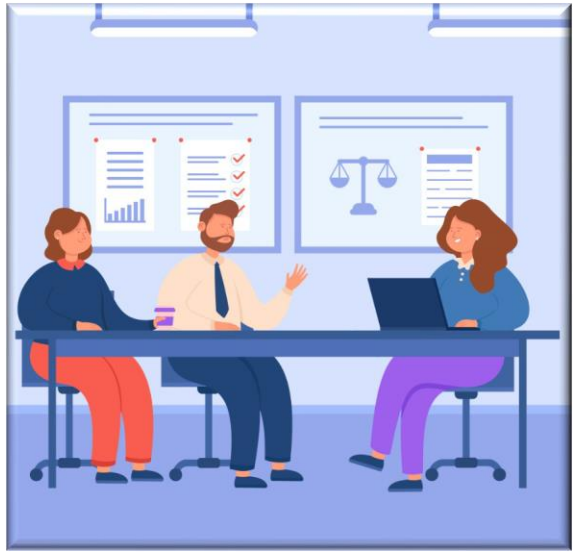
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Type of data



Make european and local regulatory agencies used those methods



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Type of data



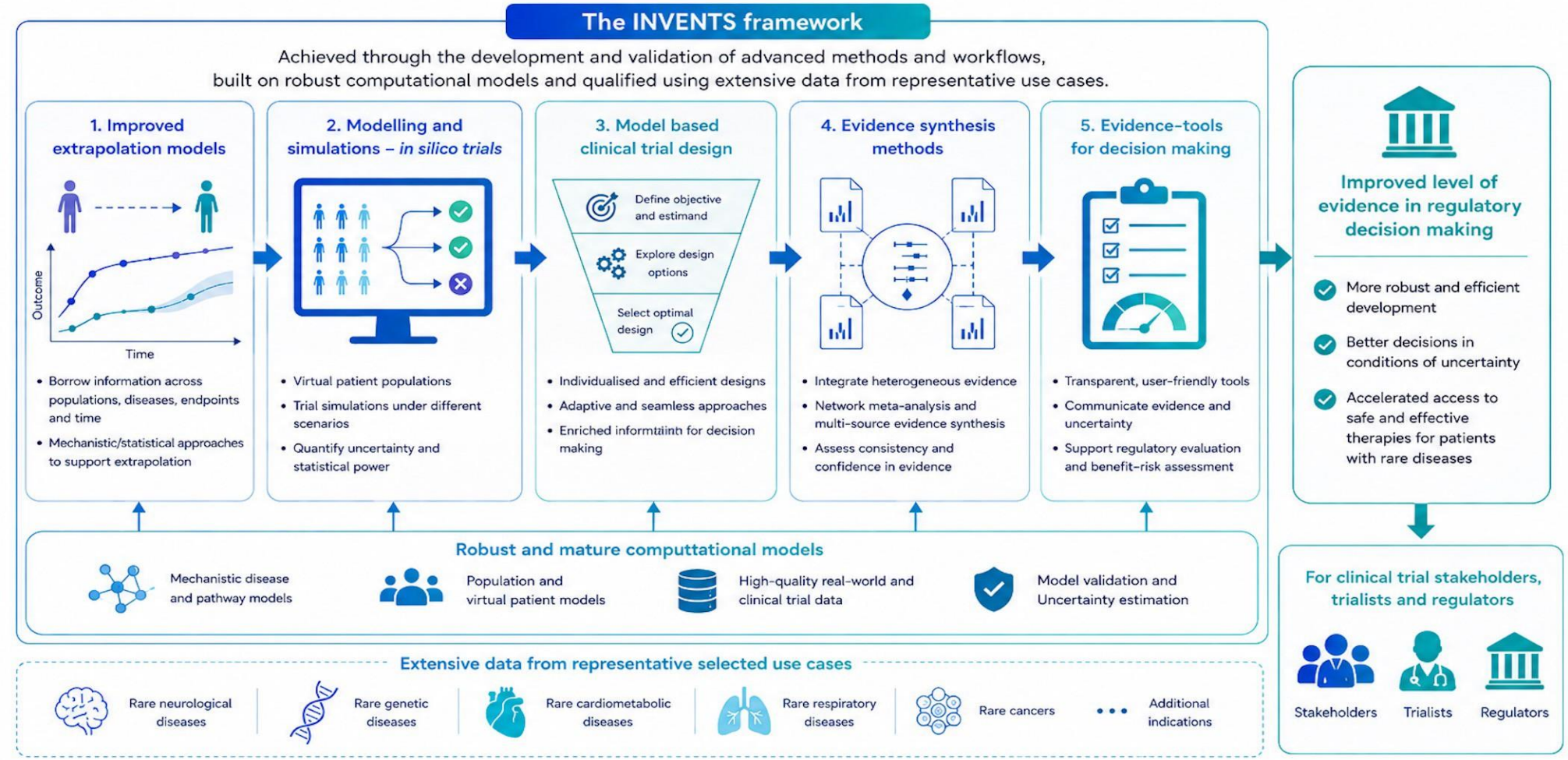


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INVENTS Overall Objective



- To provide clinical trial stakeholders, trialists and regulators with a **generalisable framework encompassing methods, workflows and evidence-tools to improve the level of evidence in regulatory decision making in rare diseases**.
- This will be achieved through the development and validation of **improved extrapolation models, simulation and in silico trials, model based clinical trial design and evidence synthesis methods**, all based on robust and mature computational models and qualified on extensive data from representative selected use cases.

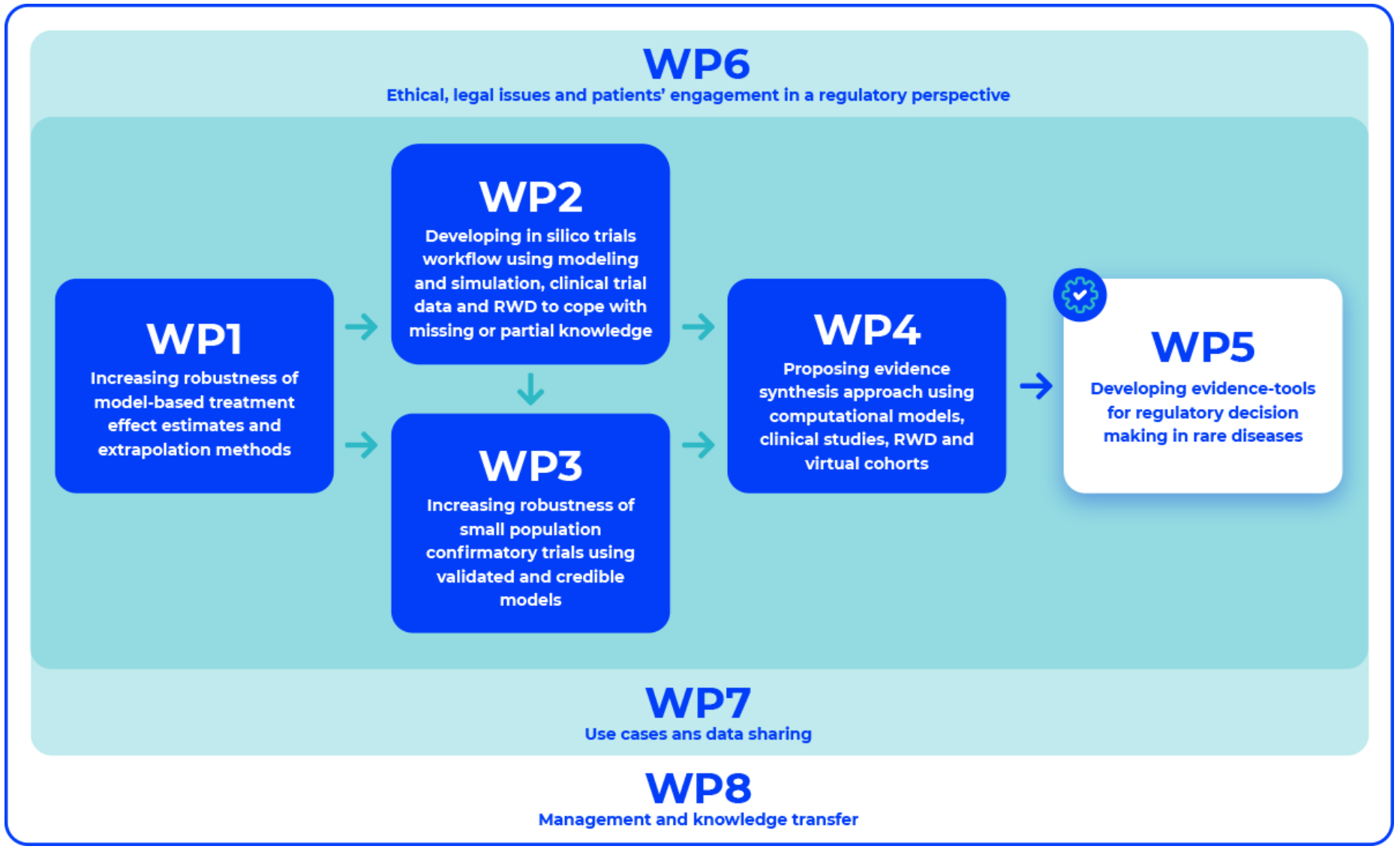
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Ready to discover INVENTS Work Packeges

INVENTS Project



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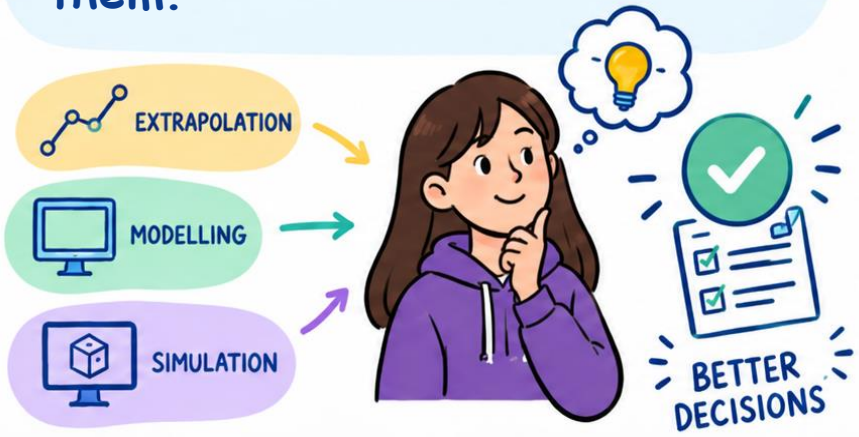
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WP1 - Objectives

Overall Objective

Improve the robustness of extrapolation, modelling and simulation methods, to propose tools that help in the decision-making process and to evaluate them.



Specific Objectives

1. Review methods and tools for longitudinal analyses used in the proposed case studies.
2. Increase robustness of extrapolation, modelling and simulation.
3. Provide robustness and accuracy assessment criteria to evaluate the extrapolation, modelling and simulation, methods based on longitudinal data.



UPPSALA UNIVERSITET

Mats Karlsson
Pr of Pharmacometrics
UPPSALA University



Roche

Yumi Cleary
Clinical Pharmacometrician
F. Hoffmann-La Roche

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WP2 - Objectives

Overall Goal

Find the best way to use different types of health data and computer models to create "virtual patients" and study how they could help us understand treatments.



Health data
- medical records
- test results
- genetic data
- and more...

Computer models
- simulation
- data analysis
- algorithms

Virtual patients
Digital versions of real patients

Better research
Helps us understand how treatments may work

Specific Goals

1 **EXPLORE**



Explore different ways to create virtual patients using health data and computer models.

2 **COMPARE**



Compare these methods to see which ones create the most realistic and reliable virtual patients.

3 **EVALUATE**



Identify how we can check whether virtual patients are accurate and useful for research.



Inserm

Sarah Zohar
Director of Research
Inserm

NOVARTIS



Marina Savelieva
Group Head
Pharmacometrics
Novartis

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WP3 - Objectives

Overall Objective

Develop and assess new ways to design clinical trials using computer models, such as PK/PD and in silico trials, to make trials more efficient and reliable.



Specific Objectives

1. **Design better trials**
   Develop flexible clinical trials using computer models to help make decisions during the trial.
2. **Use computer models**
   Develop ways to use in silico models in new types of clinical trials.
3. **Extrapolate results**
   Develop ways to design clinical trials when the results will be used to make predictions for other situations.
4. **Compare models and patients**
   Improve trial designs to compare different models, virtual patients and groups of patients.

MEDIZINISCHE UNIVERSITÄT WIEN



Martin Posch
Pr of Medical Statistics



Franz Koenig
Pr of Medical Statistics
Medical University of Vienna

UNIVERSITY OF WARWICK



Nigel Stallard
Pr of Medical Statistics
Univeristy of Warwick

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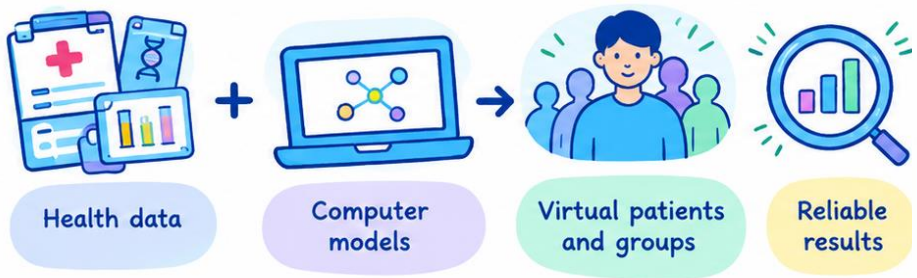
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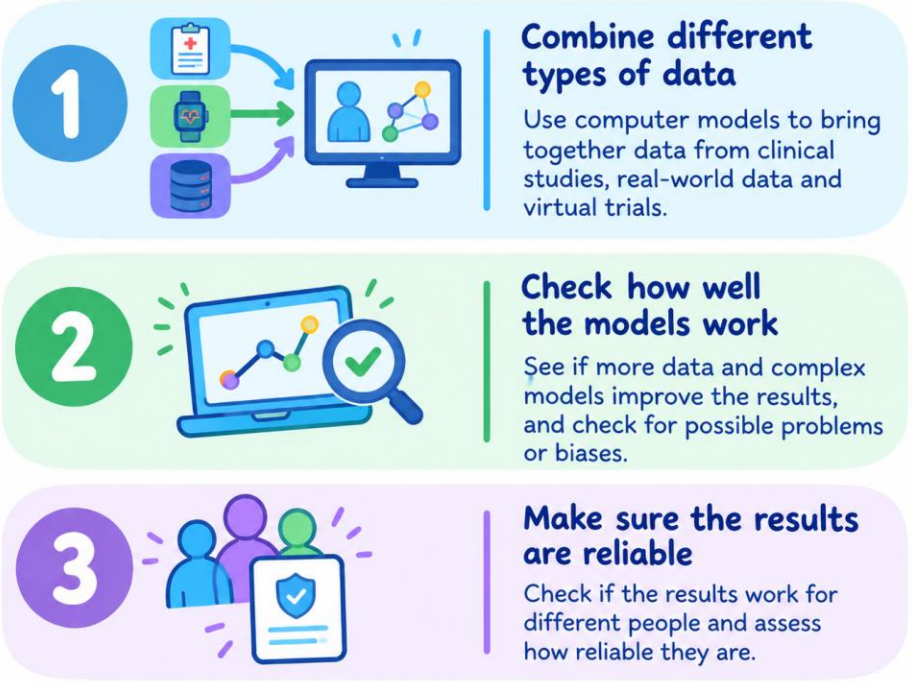
WP4 - Objectives

Overall Objective

How information originating from different data sources should be synthesised when in silico trials generating virtual patients and cohorts are involved, and check if the models give reliable results.



Specific Objectives:



UNIVERSITÄTSMEDIZIN GÖTTINGEN : UMG



Tim Friede
Pr of Biostatistics
Medical University of Goettinger



Christian Röver
PhD Statistics
Medical University of Goettinger

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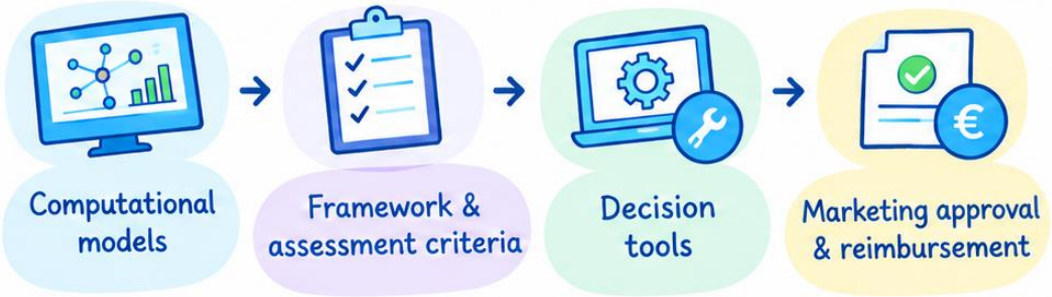
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WP5 - Objectives

Overall Objective

Provide applicants and assessors with a framework, assessment criteria and associated decision tools for the use of computational models with impact on decision for marketing approval and reimbursement for small populations.





Specific Objectives:

1. **Check if the models are reliable**
   Define criteria to assess whether the models give correct and useful results. This is especially important for hybrid and model-based trial designs in small populations.

2. **Use computer models for clinical trials**
   Apply computer models to support late-stage clinical trials in small populations.

3. **Find evidence for small populations**
   Use data from different sources and trial designs – including in silico trials, hybrid designs and real-world data.

4. **Compare and set standards**
   Use clear and trusted rules, tools and standards to show that the methods work and can be used for health and regulatory decisions.

Radboudumc university medical center



Kit Roes
Prof of Biostatistics
Radboud UMC

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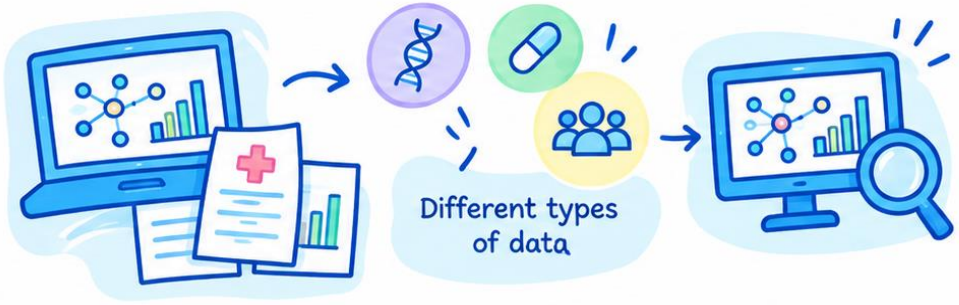
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WP6 - Objectives

Overall Objective

Ensure the Ethical, Legal and Regulatory requirements and Patients' perspective implementation.



Specific Objectives

1. To provide a regulatory and ethical overview and recommendations to support the work on development and modelling approaches.
2. To build and enhance the capacity of RD & paediatric patients and their representatives to contribute to the development and evaluation of modelling approaches and increase their acceptability.
3. To design and propose the integration of new tools allowing the objective quantification of patients' experience in RD/paediatric drug development decision-making by competent authorities.

Inserm



Daria Julkowska
Pr of Genetics Inserm Scientific coordinator of the European Programme on Rare Diseases

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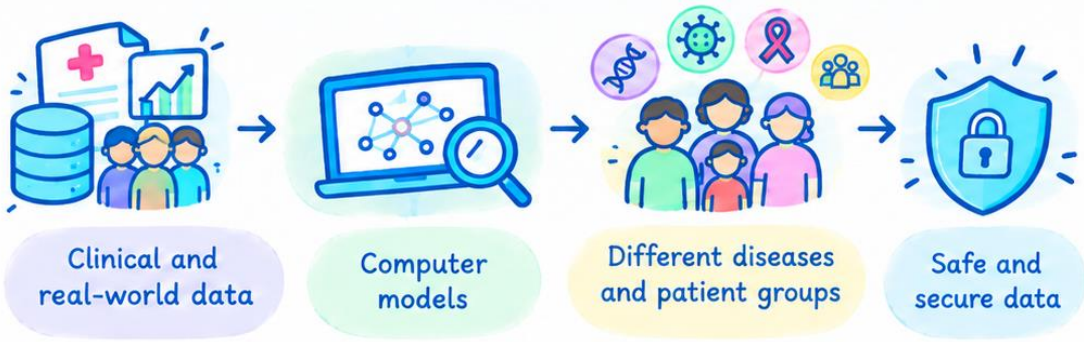
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WP7 – Objectives

Overall Objective

Use clinical and real-world data from different diseases and patient groups to test computer models and better understand how treatments work, while keeping data safe and secure.



Specific Objectives

1. Data scope, sharing and quality standards of clinical trial data for Secukinumab (Fingolimod) (Novartis).
2. Data scope, sharing and quality standards of clinical trial data for Tocilizumab (Roche).
3. Data scope, sharing and quality standards for real world data and French National Health Data System (SNDS).
4. Data scope, linking data from SNDS and French National Registry of Rare Diseases (BNDMR).

ASSISTANCE PUBLIQUE HÔPITAUX DE PARIS



Anne-Sophie Jannot
PhD in Statistical Genetics
MD in Public Health - APHP

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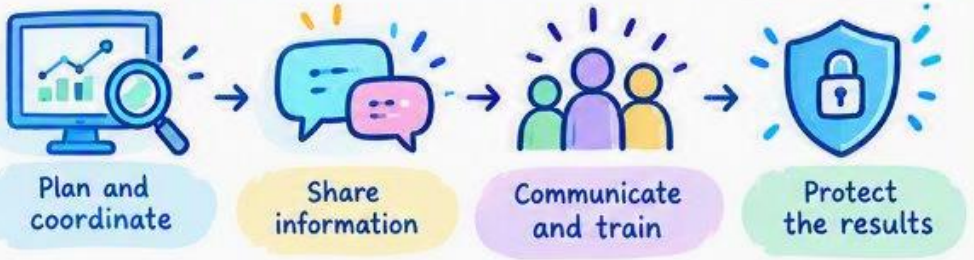
WP8 - Objectives

Overall Objective

To make the project work well, by **managing and coordinating** the project and the team, and by making sure the **results are achieved** as planned.

The WP will also share information, communicate and train everyone, and **protect the project results.**





Specific Objectives

1. Provide efficient management and administration of the project, while fulfilling all legal requirements.
2. Act as the interface between the consortium and the EC and establish a functional management structure to ensure smooth interaction among the partners.
3. Provide assistance and advice to the project partners regarding administration and reporting.
4. Develop and maintain the Data Management and Dissemination, Communication & Exploitation plans.
5. Maximise the dissemination of the project's results via experts/regulators/clinical trial stakeholders training.

Inserm



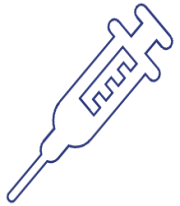
Sarah Zohar
Director of Research
Inserm

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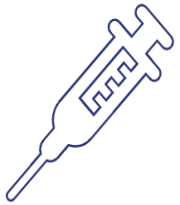
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Uses Cases



Secukinumab (Novartis) | Plaque-type Psoriasis, Juvenile Idiopathic Arthritis



Tocilizumab (Roche) | Rheumatoid arthritis, Polyarticular & Systemic Juvenile Idiopathic Arthritis, Systemic Sclerosis, Giant Cell Arteritis



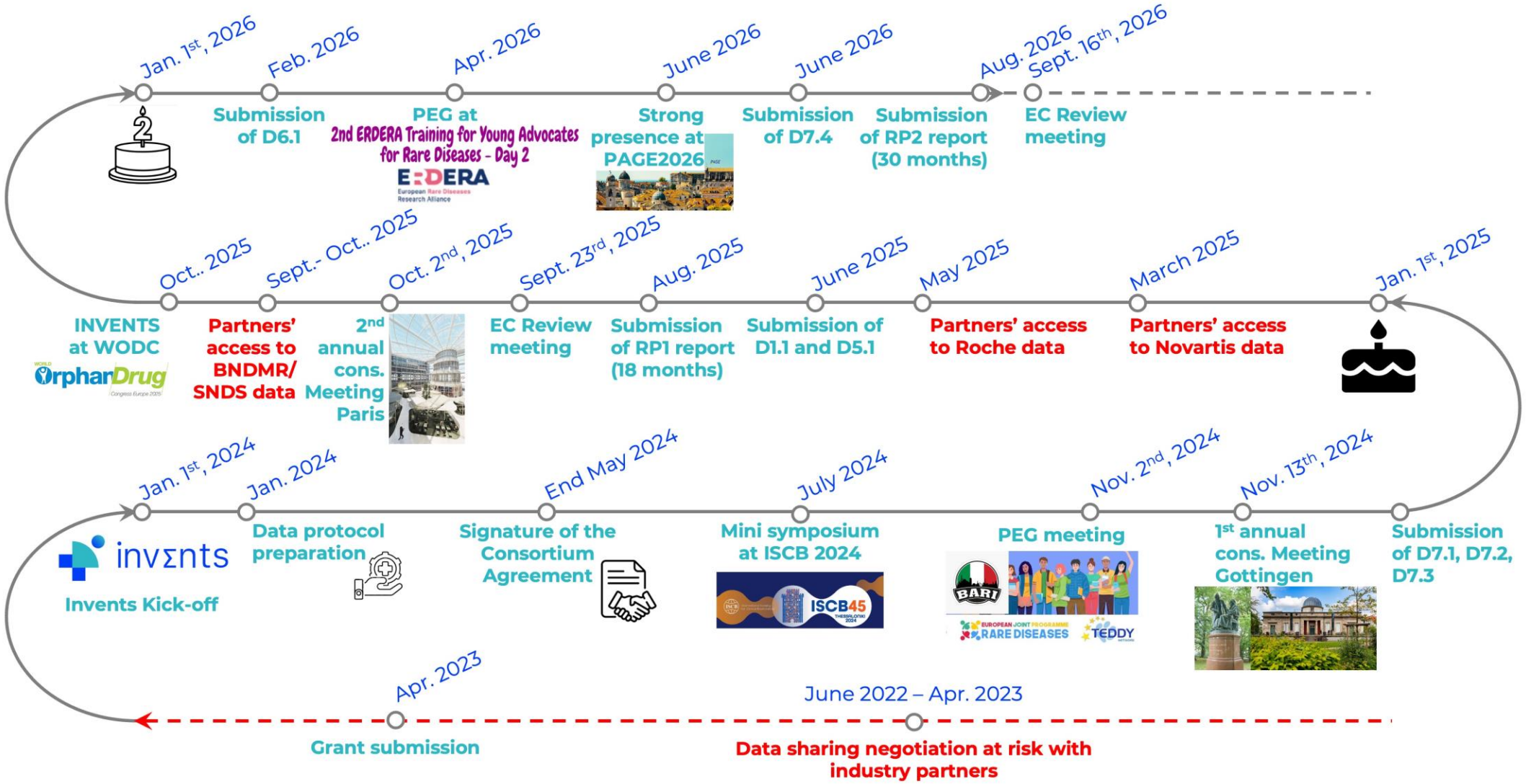
Fingolimod (Novartis) | Multiple Sclerosis

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It has been quite a journey…



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The Role of Patients Expert Group in INVENTS



Why it's important for you to give your opinion?

When you talk about what works well or what could be improved, you help doctors and researchers better understand what it's like to live with your illness



- **Create better medications**
- **Better target research on medications**
- **Improve the way care is given**
- **Change the hospital experience**

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Your Voice in the Next Project Steps

As a next step, we would like to involve you in working together with experts from the different Work Packages (WPs). What do you think about this opportunity?



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Thank you for your attention

SPEAKER'S DETAILS

Name
Title
Coordinates

Name
Title
Coordinates

Contact
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