Data sharing for rare diseases in INVENTS: Going Beyond Conventional RCTs for Rare and Paediatric Diseases – the Roche perspective

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PSI conference

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

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A message from the Death of Data Sharing workshop in Basel: data sharing is getting harder, but it can still be done!

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The EU call HORIZON-HLTH-2023-IND-6.04

Modelling and simulation to address regulatory needs in the development of orphan and paediatric medicines

- Call linked to EMA's "**Regulatory Science Strategy to 2025**"
- **Expected outcomes**:
  - Developers and regulators have access to **robust modelling and simulation tools** to accelerate the effective development of orphan and/or paediatric medicinal products.
  - Developers and regulators use accurate simulation tools to **improve the statistical robustness in clinical trials intended for small populations and guide cost-effective clinical trials designs**.
  - Regulators have access to accurate **in-silico tools for assessing real world data (RWD) and patient reported outcomes** for optimizing the clinical endpoints in clinical trials for small populations.
  - **Regulators develop guidance** for a **robust extrapolation framework for the safety and efficacy** prediction during the regulatory assessment of orphan and/or paediatric medicinal products.
- Project is planned for 5 years with a budget of 8 million €

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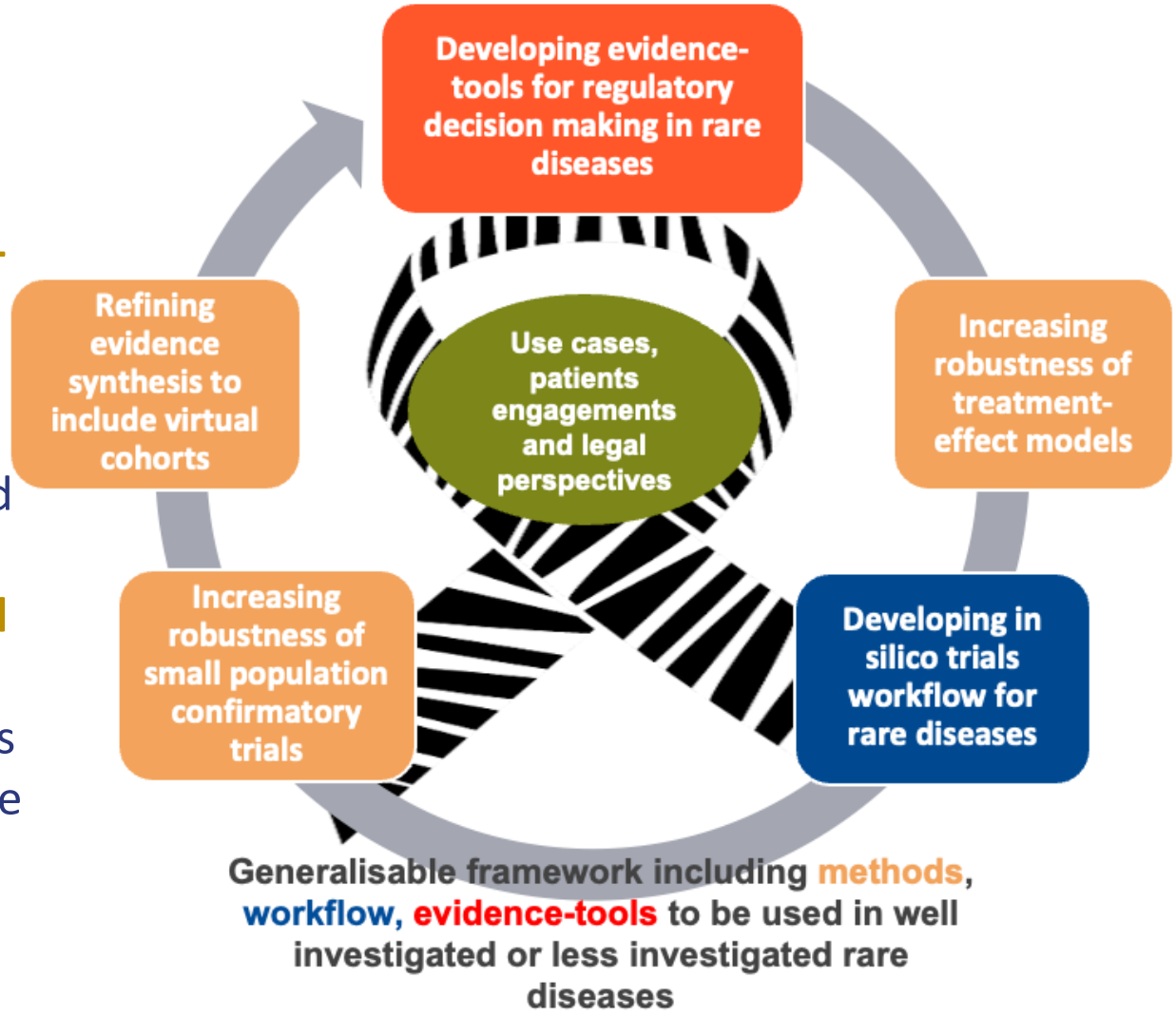
Objective of the INVENTS consortium

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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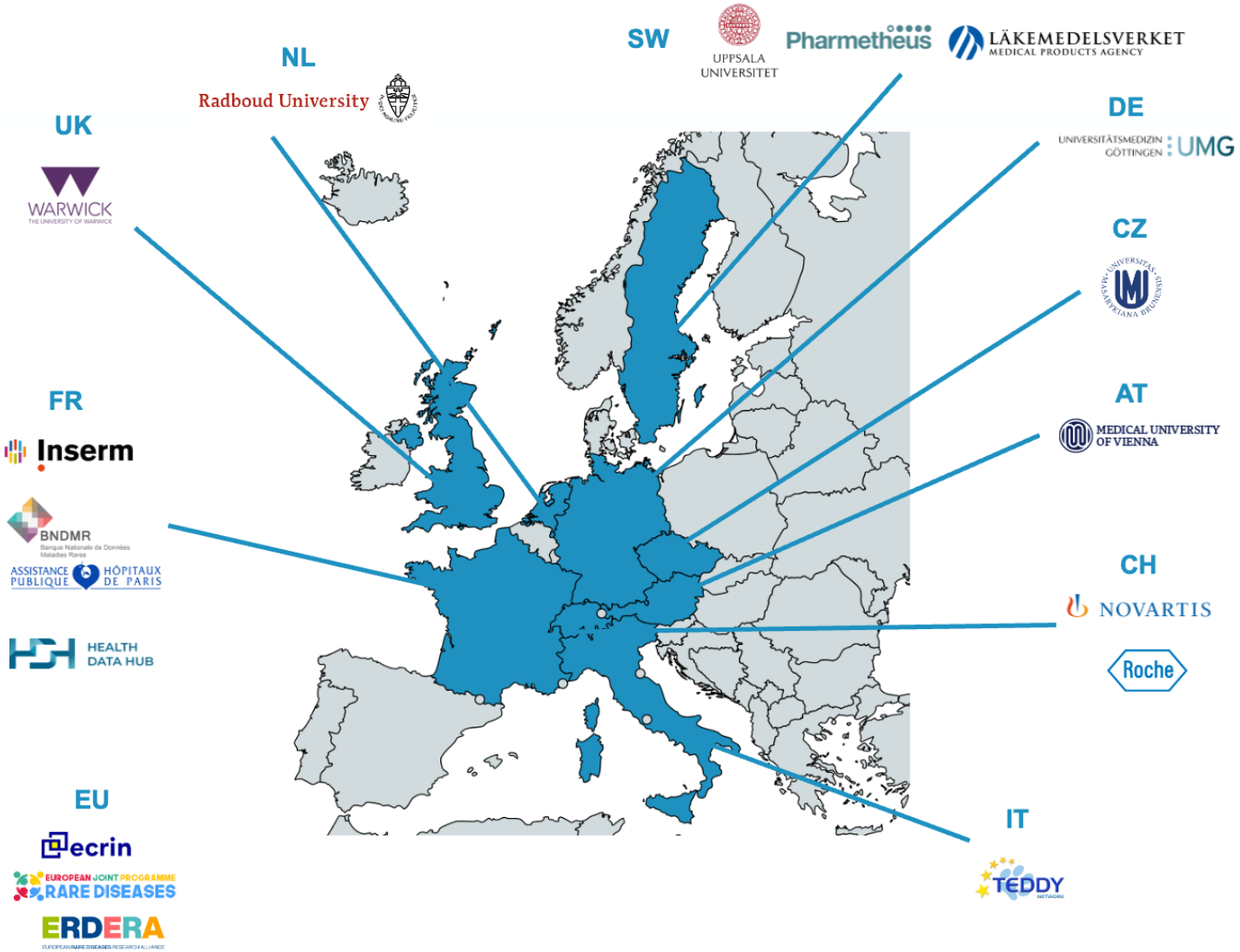
INVENTS as a pan-European partnership



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Key data sharing challenges

- Secondary data use rules vary between countries – some are incompatible (e.g. China)
- Risk-based de-identification – remove key identifiers (e.g. ethnicity, site), move to relative dates
- France does not recognise anonymisation. Can only pseudonymise – GDPR still applies.
- Data protection laws are a moving target. Some new interpretation every few months!
- Study ICFs are older, so need to be manually assessed for compliance with current law
- Ethics and privacy approvals are mandatory
- Patients need to be informed about data use, but no ability to contact patients directly
- HDH is a wonderful platform, but comes with rules and processes for security and data ingestion

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Timeline of the Roche data sharing – getting the grant

- September 2022: initial contact between INSERM and Roche to discuss a possible project
- Winter: preliminary internal approvals for patient privacy, intellectual property, and resources
- January 2023: commitment letter signed
- April 2023: grant proposal submitted – successful in August
- Summer 2023: define which studies to share and gain approval from the molecule team
- November 2023: grant agreement signed
- January 2024: project start
- April 2024: 1 million in funding from the Swiss SERI for data anonymisation and PhD students
- May 2024: consortium agreement signed

Schweizerische Eidgenossenschaft
Confédération suisse
Confederazione Svizzera
Confederaziun svizra

Swiss Confederation

Federal Department of Economic Affairs, Education and Research EAER
State Secretariat for Education, Research and Innovation SERI

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Timeline of the Roche data sharing – approvals

- January 2024: data anonymization process started, specs defined in summer 2024, initial programming in fall, then additional privacy review of results for paediatric and rare diseases
- March 2024: review of country ICFs for all studies
- April 2024: ethics submission to CESRESS (Comité éthique et scientifique pour les recherches, les études et les évaluations dans le domaine de la santé) – approved in April

CESREES
Comité Éthique et Scientifique pour les Recherches, les Études et les Évaluations en Santé

- April 2024: approval from Roche's Global Privacy Office
- July 2024: privacy submission to CNIL (Commission nationale de l'informatique et des libertés) – approved in October

CNIL.
COMMISSION NATIONALE INFORMATIQUE & LIBERTÉS

- September 2024: new SOP on external data sharing – additional review by Pharma Data Sharing Governance Committee
- December 2024: collective information to patients published
- April 2025: data anonymization completed

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Timeline of the Roche data sharing – making available

- Throughout 2024: workshops with HDH to prepare data sharing, work on security & compliance
- January 2025: HDH platform contract signed
- February 2025: create data documentation in HDH format
- March 2025: set up secure transfer channel to HDH
- April 2025: data sent to HDH together with data documentation
- April 2025: first users onboarded to HDH
- May 2025: data sharing agreements signed, ensure data subjects can exercise their rights
- Now: multiple partners able to work with data on HDH

HEALTH DATA HUB

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Three use cases to meet different modelling objectives

1. **Rheumatoid arthritis** is a very large, well understood adult disease with multiple large studies. Useful for modelling where a lot of patients are needed or where a good mechanistic understanding of the disease is helpful. Potential extrapolation to **giant cell arteritis** to explore additional evidence needs. Substantial additional French registry / real world studies are also available.
2. **Juvenile Idiopathic Arthritis** (polyarticular, systemic) has 4 pediatric studies that offer extrapolation from adult to pediatric or between related pediatric studies.
3. **Systemic sclerosis** has 2 large randomized trials with a negative primary endpoint and a (nominally) very positive secondary endpoint. This is an interesting example of a very high-quality replicated result without alpha control, which led to a label by FDA and a rejection by EMA.

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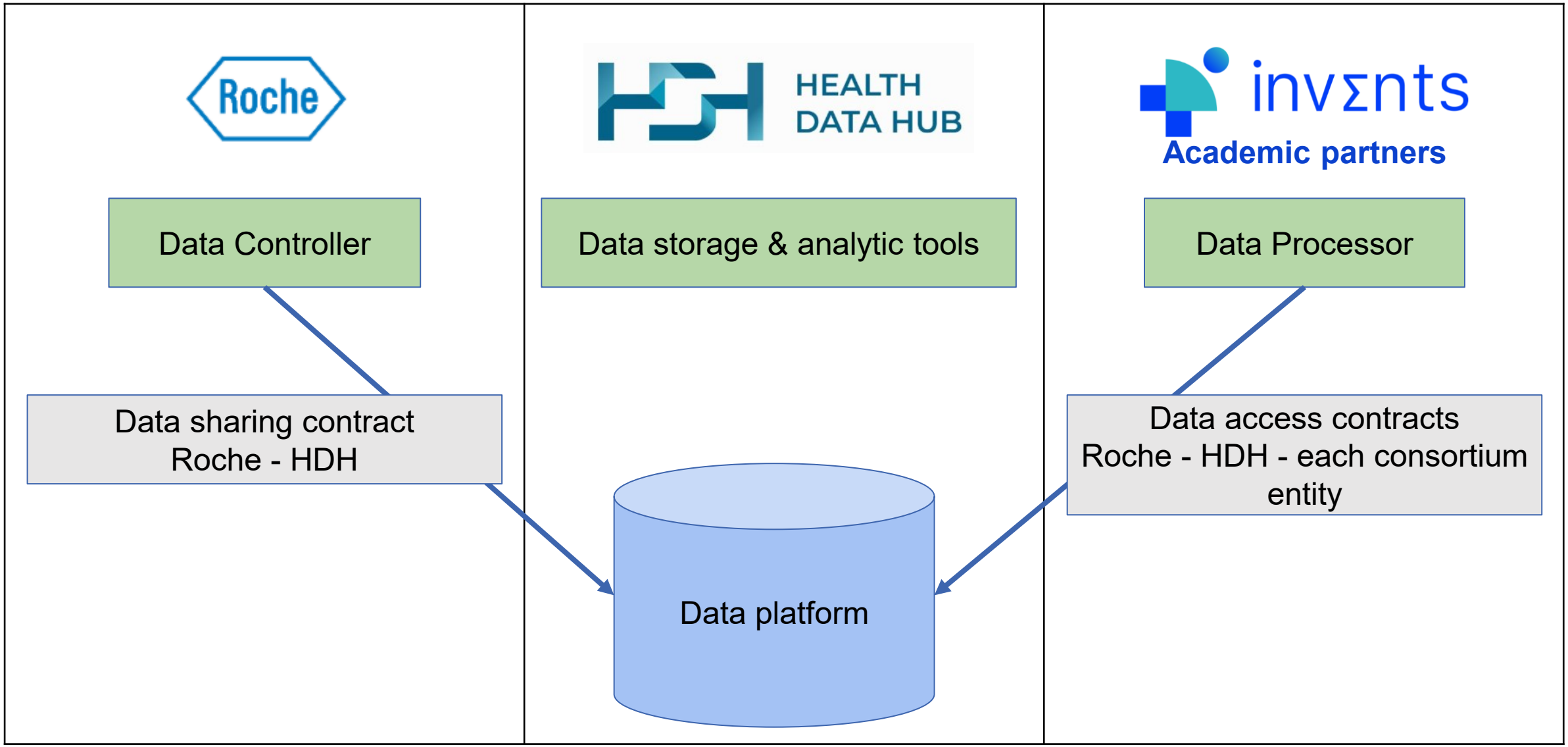
Sharing setup simplifies necessary privacy approvals



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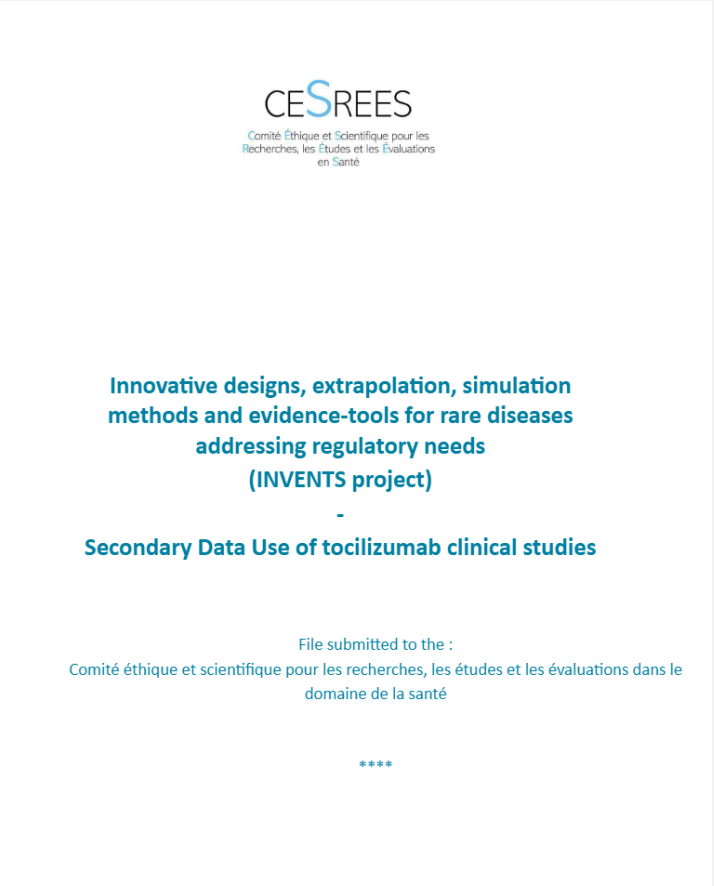
Protocol describes research objectives, methods, data protection and project roles

1. **Project participants**
2. **Objectives and aims**
3. **Methodology**
4. **Privacy, security, and confidentiality of data**

and…

- Ethics approval
- Informed consent
- Data documentation
- IT risk assessment


CESREES

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Innovative designs, extrapolation, simulation methods and evidence-tools for rare diseases addressing regulatory needs (INVENTS project)

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Secondary Data Use of tocilizumab clinical studies

File submitted to the :
Comité éthique et scientifique pour les recherches, les études et les évaluations dans le domaine de la santé

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De-identification to get privacy approval from the French National Commission on Informatics and Liberty (CNIL)

3.6. Preparation of data

Data collected from patients and their investigating physicians were originally pseudonymized and a patient number was randomly generated.

Before proceeding with the data sharing and analyses, preliminary work in 2 steps will be performed for each study:

1. Variables useful for the research will be selected and potentially identifying data will be removed, including:
    - Ethnicity information is needed for the purpose of pharmacokinetic modelling in work package 1, but will be limited to the most common ethnicities (others will be grouped as “other”) and access to ethnicity will be limited to situations where it is required for the purpose of the research
    - Patient names and initials will be removed
    - Site information will be removed
    - Country information will be removed, retaining only the geographic region (e.g. Europe)

2. Variables subject to modification are altered, including:
    - Patient number will be randomly re-allocated
    - All dates (enrolment, treatment administration, …) will be truncated (month/year) or removed

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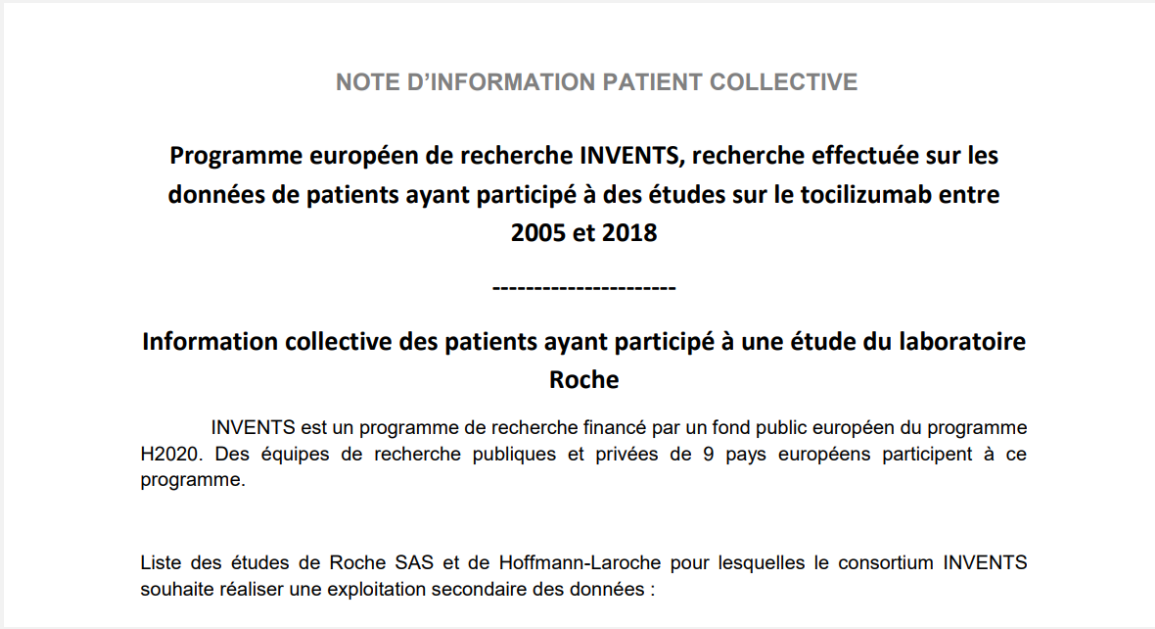
Collective information notifies study participants about research objectives and gives opportunity to opt out

1. **How is my data being used?**
2. **What is the research purpose?**
3. **How can I object?**



NOTE D'INFORMATION PATIENT COLLECTIVE

Programme européen de recherche INVENTS, recherche effectuée sur les données de patients ayant participé à des études sur le tocilizumab entre 2005 et 2018

Information collective des patients ayant participé à une étude du laboratoire Roche

INVENTS est un programme de recherche financé par un fond public européen du programme H2020. Des équipes de recherche publiques et privées de 9 pays européens participent à ce programme.

Liste des études de Roche SAS et de Hoffmann-Laroche pour lesquelles le consortium INVENTS souhaite réaliser une exploitation secondaire des données :

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