# Final report of the pilot project based on OMOP harmonization and federated analyses

Real-world data and evidence from new medicines

TYKS

HUS

fimea

Pirkanmaan hyvinvointialue

Tays

SITRA

medaffcon

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# Summary (1)

## OBJECTIVES

- Test and develop Observational Medical Outcomes Partnership (OMOP) databases and a federated analysis-based operating model for producing real-world data (RWD) and real-world evidence (RWE) about the usage and outcomes of new medicines
- Evaluate the usability of the operating model in health technology assessment (HTA) of medicines
- Timeline: 04/2023–12/2023

## IMPLEMENTATION

- The key participants in the pilot were OMOP centers belonging to the FinOMOP consortium, members of the analytics team, clinical experts, and Finnish Medicines Agency Fimea as the client requesting data for evidence needs specific to HTA
- Data was requested for three case examples:
  (1) treatment for multiple myeloma, (2) CAR-T treatments,
  (3) treatments for SMA (spinal muscular atrophy)
- The OMOP centers used the OMOP databases of three university hospitals and delivered the extracted and aggregated statistical data to Fimea
- The Finnish Innovation Fund Sitra funded the work of the OMOP centers and the analytics team as part of the broader Health Data 2030 project

## KEY RESULTS

- A 10-step operating model was used in the pilot
- Not all data requested by Fimea was stored in the OMOP databases. The centers standardized the missing data into the OMOP Common Data Model (OMOP CDM).
- The most time-consuming steps were the standardization of missing data and the centers' contract processes, as well as the evaluation of the quality of the extracted data
- The OMOP centers provided Fimea with anonymous aggregated, center-specific results for each case example separately. Before the data was provided to Fimea, Finnish Social and Health Data Permit Authority Findata verified the anonymity of the results.
- Findata was also asked for guidance for producing anonymous results. According to Findata, the results from aggregated statistical data should be masked when there are 1–3 observations.
- Fimea pooled the center-specific results and evaluated the usability of the statistical data from the perspective of HTA related evidence needs. The evaluation used a classification: usable, partially usable, not available/unusable.

# Summary (2)

## KEY OBSERVATIONS

- For the first time, OMOP databases were used in a joint pilot between the FinOMOP consortium and Fimea, representing a client. The pilot produced valuable information on the development needs related to the operating model based on federated analysis and OMOP common data model.
- The success of the pilot was greatly influenced by collaboration among participants and the sharing of expertise. These factors played a crucial role in achieving positive outcomes.
- The indications for new medicines are typically very precisely defined, for example, by the stage of the disease, biomarkers, previous treatments, or treatment responses. The lack of structured electronic health record (EHR) data corresponding to specific indications limited the usability of the data stored in the OMOP databases.
- The usability of pooled results using data extracted from individual OMOP databases is limited, if every OMOP center masks observations ranking from 1–3
- In Finland, the legislation on secondary use of social and health data does not recognize the operating principles of federated analyses

## DEVELOPMENT NEEDS

- In the development of service operations aimed at utilizing OMOP databases and federated analytics, it is essential to define key customer groups and their data and evidence needs and to ensure that resources are allocated appropriately
- The development of the service operations of OMOP centers should continue in pilot projects
- The development efforts related to OMOP databases and structured health care data should be undertaken as a separate project, with a focus of prioritizing the needs of specific customer groups
- The quality work for the secondary use of social and health data should be initiated and quality requirements should be specified using international frameworks
- In local data permits, data extractions and statistical data production, it is crucial to ensure that results can be reported precisely according to the authorized indications for the medicine. In addition, clinical documentation practices should be developed so that reporting can be done with sufficient accuracy.

# 1. Introduction

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# 1.1. Secondary use of social and health data as part of the managed entry of new medicines

- The decision-making process related to the reimbursement and managed entry of new medicines has traditionally been based on the outcomes of clinical trials on the efficacy and safety of treatments. After market entry, the treatment outcomes have not been systematically monitored in healthcare, despite potential significant uncertainties concerning the treatment's benefits, cost-effectiveness and budget impacts.
- The need for post launch evidence generation is emphasized by the fact that pharmaceutical development has increasingly focused on personalized medicine, advanced therapies, and rare diseases. This means that medicines are coming to the market at an earlier stage of development. As a result, decisions are based on increasingly limited clinical evidence. However, the prices of new treatments are almost always high, despite the uncertainties related to the therapeutic and economic value of the treatment.
- Uncertainties also involve financial risks, which is why pharmaceutical companies and healthcare payers negotiate managed entry agreements. The agreements may involve the need to utilize systematically collected RWD on the use and outcomes of the treatment.
- Some of the managed entry agreements have been successful, but they also have weaknesses such as the labor-intensive administration, non-disclosed prices and other details of the agreements, and failures in generating relevant data and evidence post-launch
- Rapid developments in data management and advanced analytics would facilitate more efficient use of healthcare data in the managed entry of new medicines

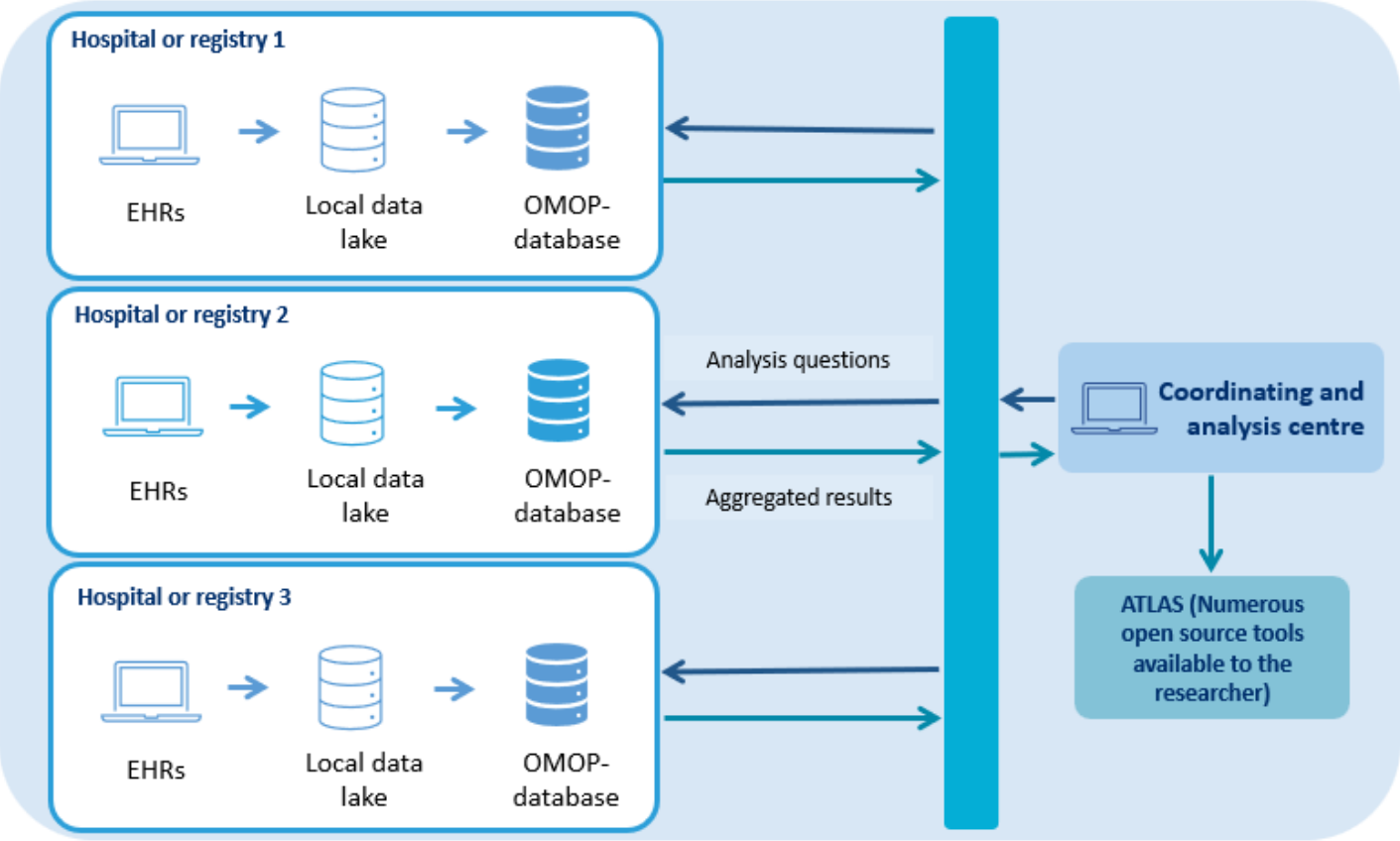
# 1.2. Authorities’ data and evidence needs in the managed entry of new medicines

- RWE based on the secondary use of social and health data is needed in various stages of managed entry of new medicines. The need is often related to disease- or patient group-specific data.
- Pharmaceutical companies produce and submit evidence for regulatory and HTA processes. The totality of evidence consists of clinical trials, observational studies, evidence syntheses, and decision-analytical modeling. The goal is to make the product available to patients as part of publicly funded reimbursement system or hospitals' medicine selection.
- Regulators and HTA bodies can also generate RWE themselves. They also provide advice to support the planning of studies and evidence generation.
- Currently, the broader secondary use of healthcare data encounters limitations stemming from several factors. These include availability, reusability, appropriateness (fit-for-purpose), data quality, and timeliness issues, as well as associated costs. Furthermore, the use of RWE studies in decision making faces limitation arising from various sources of bias and confounding.
- The competence, processes, information systems and technologies used by the regulators and HTA-bodies can also limit the use of RWD and RWE

**Tasks of authorities in managed entry of new medicines**

- Early dialogues / scientific advice
- Horizon Scanning
- Marketing authorisation
- Health Technology Assessment (HTA)
- Price negotiations and managed entry agreements
- Appraisal (reimbursement, publicly funded health care)
- Value-based steering
- Health technology reassessment

# 1.3. OMOP and federated analysis



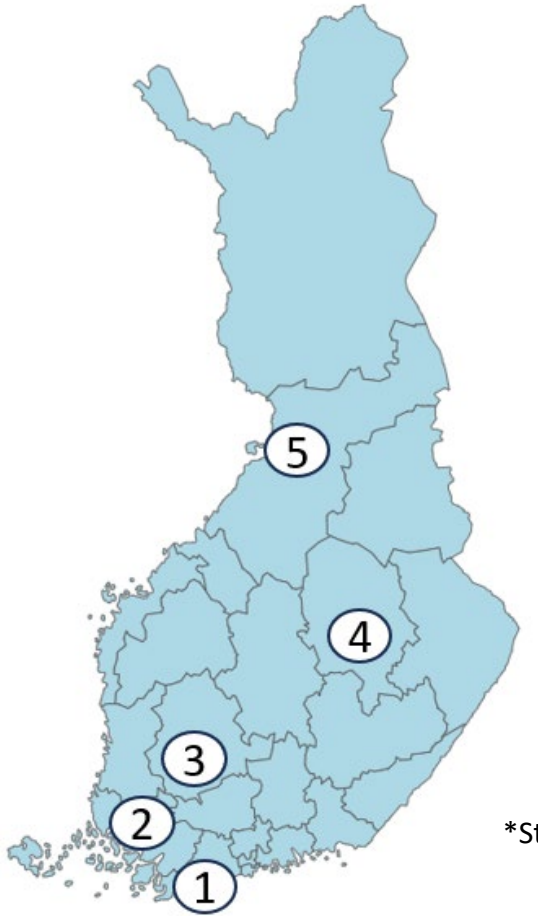

Modified from Laitinen, Virkki, Porkka: Duodecim 2022

- OMOP = Observational Medical Outcomes Partnership
- The same research is conducted across different OMOP centers, after anonymization data between centers is pooled
- For success, it is essential that health data is harmonized and conforms to a commonly agreed data model (OMOP CDM)
- A common extraction and analysis algorithm is utilized in each center
- The approach is faster, partially automated, less expensive, of higher quality and more scalable than traditional data extraction methods
- OMOP harmonization requires continuous development and quality assessment
- Allows extremely fast comparisons between university hospital treatments such as those seen during the Covid-19

# 1.4. FinOMOP consortium

- Members
  - Five university hospitals and their OMOP centers operating on their wellbeing services counties of Helsinki (HUS), Southwest Finland (Varha), Pirkanmaa (Pirha), North Savo (PSHVA) and North Ostrobothnia (Pohde)
  - Finnish Institute for Health and Welfare (THL)
  - FinnGen research project, University of Helsinki
  - Cooperative of Finnish Biobanks (FinBB)
- The members are jointly committed to
  - the harmonization of patient information according to the international OMOP criteria
  - common operating and contract models in data production, analytics, contract practices as well as customer service
- The goal of the members is to
  - enable Finnish researchers to participate in national and international research projects in the era of Act on the Secondary Use of Health and Social Data and the European Health Data Space (EHDS)
  - develop Finland as an international actor in registry research

**University hospitals shown on a map* of Finland**




**1.** Helsinki University Hospital (HUS)

**2.** Turku University Hospital (TYKS)

**3.** Tampere University Hospital (TAYS)

**4.** Kuopio University Hospital (KYS)

**5.** Oulu University Hospital (OYS)

*Statistics Finland. GeoStat.

# 1.5 FinOMOP consortium as a data partner of DARWIN EU®

- The European Medicines Agency (EMA) promotes the use of RWD and RWE, among other initiatives, through it's Data Analysis and Real World Interrogation Network (DARWIN EU®)
- DARWIN EU® is a network of data, experts, and information services that supports better decision-making throughout the life cycle of a medicinal product. The goal is to produce reliable evidence from real healthcare data, especially for the decision-making needs of the EMA and the European Medicines Regulatory Network.
- The FinOMOP consortium is a DARWIN EU® data partner from Finland
- DARWIN EU® uses the OMOP Common Data Model (CDM) and federated analyses

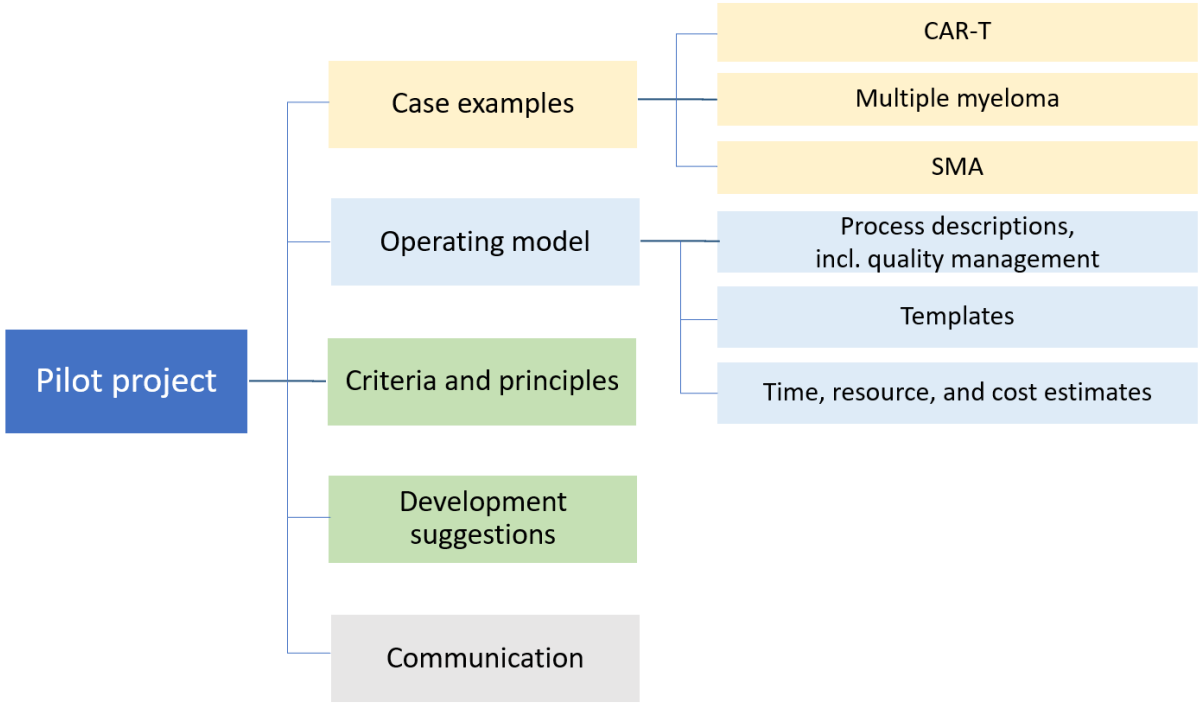
# 2. Objectives of the pilot

Image created using MS Copilot

# Objectives

- To test and develop the use of OMOP databases and federated analysis-based operating model for producing RWD and RWE about the usage and outcomes of new medicines
- To evaluate the usability of the federated analyses-based operating model for evidence needs related to HTA and managed entry of new medicines
- To define the issues requiring further clarification or development
- Three case examples were used in the pilot

**Division of the pilot project**



# 3. Implementation of the pilot

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# 3.1. Roles and participants

- Nine distinct roles were identified in the pilot. These roles represent the key participants for the pilot.
- The following slides present a more detailed summary of the roles and participants in the pilot
- Collaboration and knowledge exchange among the participants were crucial for the pilot's success
- Clearly defined responsibilities among the participants help to streamline communication and operations and enable the achievement of common goals

**ROLES OF PARTICIPANTS IN THE PILOT PROJECT**

1. Member of FinOMOP consortium
2. Coordinating OMOP center
3. Involved OMOP center
4. Data holder
5. Client
6. Clinical team
7. Analytics team
8. Data permit authority
9. Sponsor

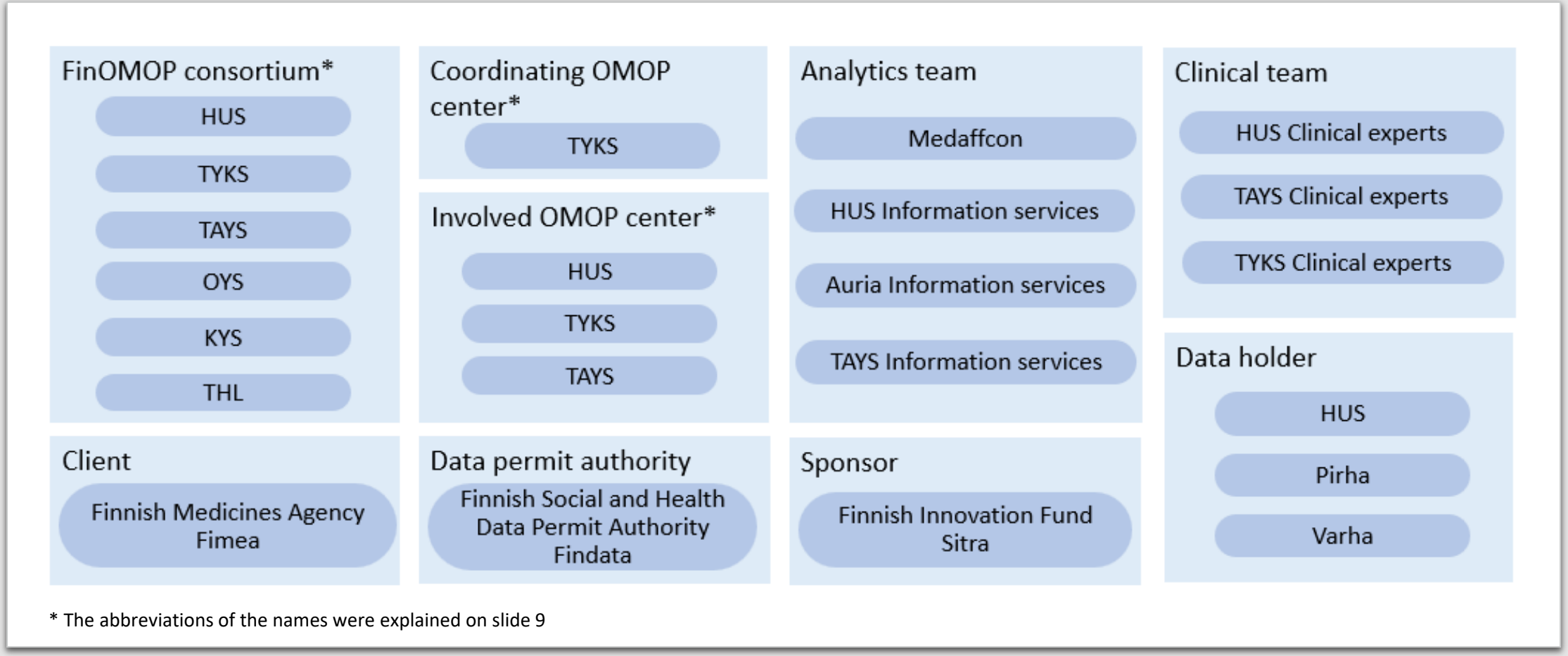
# Description of roles (1)

| ROLE | DESCRIPTION |
|---|---|
| 1. Member of FinOMOP consortium | The members (see slide 9) of the consortium:<br>• develop, validate, and maintain their OMOP databases<br>• systematically structure and harmonize data from electronic health records (EHRs) as part of the OMOP database development<br>• commit to common quality management |
| 2. Coordinating OMOP center | • Prepares the necessary contracts, other documents, and budgets<br>• Conducts a feasibility assessment and defines the final research plan in collaboration with the client<br>• Leads the research and is responsible for coordinating the participating OMOP centers and other key participants (analysts and clinical experts)<br>• Is responsible for funding between participating OMOP centers |
| 3. Participating OMOP center | Extracts data from the OMOP databases on the client's specific data and evidence needs and then contributes to the analyses in a predefined way |
| 4. Data holder | The data holder of the source data (e.g., a wellbeing services county), which defines the purpose and method of processing personal data |
| 5. Client | A participant (in the pilot, Finnish Medicines Agency Fimea) who requires data or evidence for their own tasks. The client defines the questions and variables and submits the research protocol and research contract to the coordinating OMOP center. |

# Description of roles (2)

| ROLE | DESCRIPTION |
|---|---|
| 6. Clinical team | Extensive expertise in the clinical field is required to effectively address complex data requirements. For example, the clinical team can identify patterns in EHR data entry and data-related anomalies, which should be considered when defining algorithms for data extraction and analysis. In addition, the clinical team can assess the validity of the produced statistical data by leveraging their clinical expertise and understanding of previous research. |
| 7. Analytics team | In each study, common OMOP data extraction, analysis, and reporting algorithm is defined and shared with all participating centers. The analytics team, in collaboration with the OMOP centers, ensures the identification of identical patient groups and the extraction of similar data from the OMOP databases. Subsequently, the results are reported to the client as specified in the protocol. |
| 8. Data permit authority | The data permit authority (in the pilot, Finnish Social and Health Data Permit Authority Findata) verifies the anonymity of the published results if the research has been based on a data permit in accordance with the Finnish Act on the Secondary Use of Health and Social Data |
| 9. Sponsor | A participant (in the pilot, The Finnish Innovation Fund Sitra), who funds the work of the OMOP centers and analytics team |

# Map of roles and participants in the pilot




* The abbreviations of the names were explained on slide 9

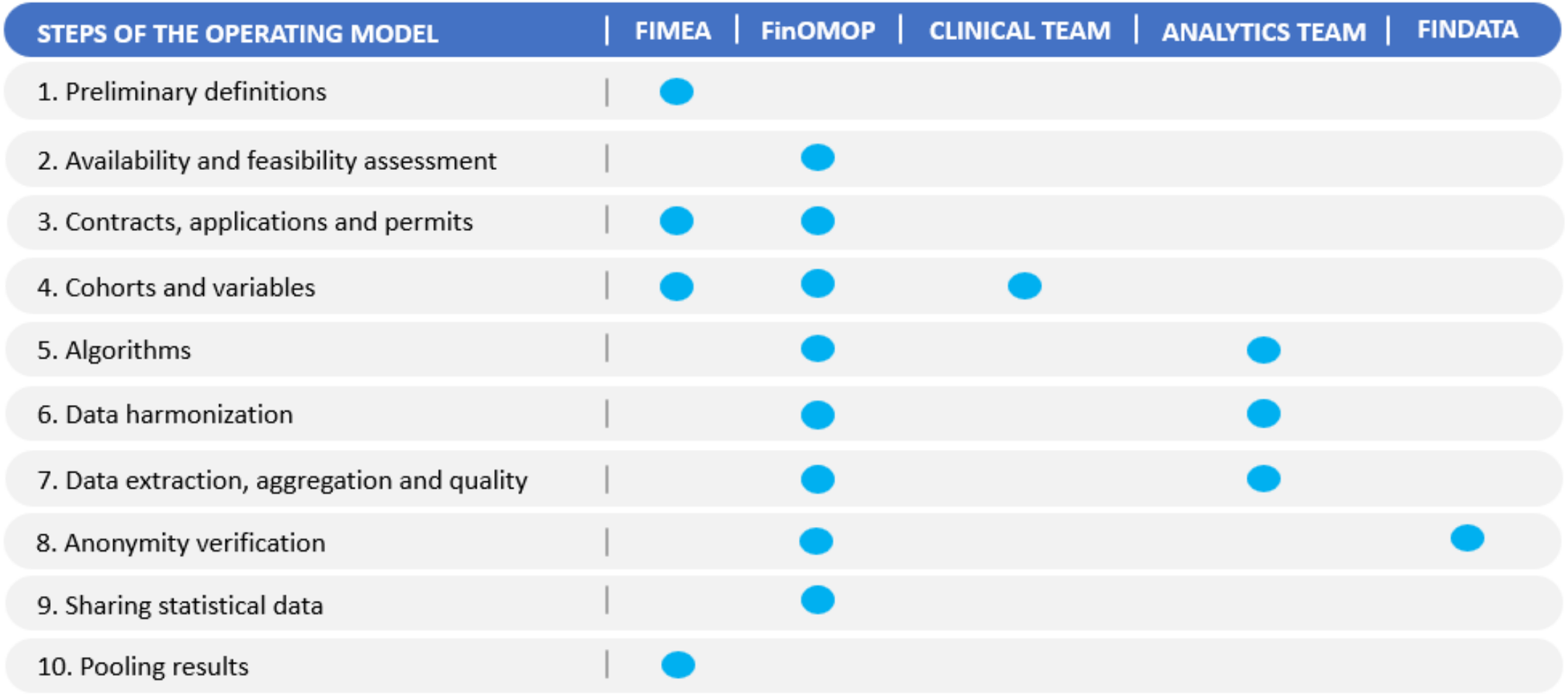
# 3.2. Operating model

- The operating model is a description of how RWD was produced in the pilot for new medicines using OMOP databases and federated analysis
- The operating model consists of 10 steps, in which the participants of the pilot collaborated in different ways
- The following slides present a more detailed summary of the different steps of the operating model used in the pilot
- In the operating model, the longest lead times and the greatest development needs were identified in contracts and permits (stage 3), supplementing OMOP databases with missing data (stage 6), and assessment the quality of the extracted data (stage 7)

**STEPS OF THE OPERATING MODEL**

1. Preliminary specifications
2. Availability and feasibility assessment
3. Contracts, applications and permits
4. Cohorts and variables
5. Algorithms
6. Data harmonization
7. Data extraction, aggregation and quality
8. Anonymity verification
9. Sharing statistical data
10. Pooling results

# Steps of the operating model



# Description of the operating model (1)

| STEP | DESCRIPTION |
|---|---|
| 1. Preliminary definitions | Fimea defined the data and evidence needs (questions and lists of variables) and submitted a description of the needs to the coordinating OMOP center |
| 2. Availability and feasibility assessment | The assessment of data availability and feasibility was carried out in cooperation with the OMOP centers |
| 3. Contracts, applications and permits | Fimea drafted a research plan and the data permit applications in cooperation with the OMOP centers. Data permits were processed and granted separately at each university hospital. |
| 4. Cohorts and variables | The detailed definition of the target population and variable list corresponding to Fimea's data request was done in cooperation with the clinical team, analytics team, Fimea and OMOP centers |
| 5. Algorithms | The analytics team produced common data extraction, analysis and reporting algorithms, which were then utilized by all OMOP centers. These common algorithms enabled independent execution of standardized analytics at each OMOP center. |

# Description of the operating model (2)

| STEP | DESCRIPTION |
|---|---|
| 6. Data harmonization | OMOP centers supplemented the OMOP databases by mapping missing data into the OMOP common data model |
| 7. Data extraction, aggregation and quality | Each OMOP center independently extracted data and conducted analyses using the common algorithms. Additionally, the OMOP centers were responsible for data aggregation and ensuring the quality of center-spesific data. |
| 8. Anonymity verification | At the request of the OMOP centers, Findata verified the anonymity of the center-specific datasets (intermediate results) before they were handed over to Fimea (see slide 27) |
| 9. Sharing statistical data | The OMOP centers delivered anonymised case-specific (related to multiple myeloma, CAR-T treatments, SMA treatments) aggregated statistical datasets to Fimea |
| 10. Pooling results | Fimea pooled the case-spesific results (related to multiple myeloma, CAR-T treatments and SMA treatments) from the OMOP centers and assessed their usability from the perspective of HTA |

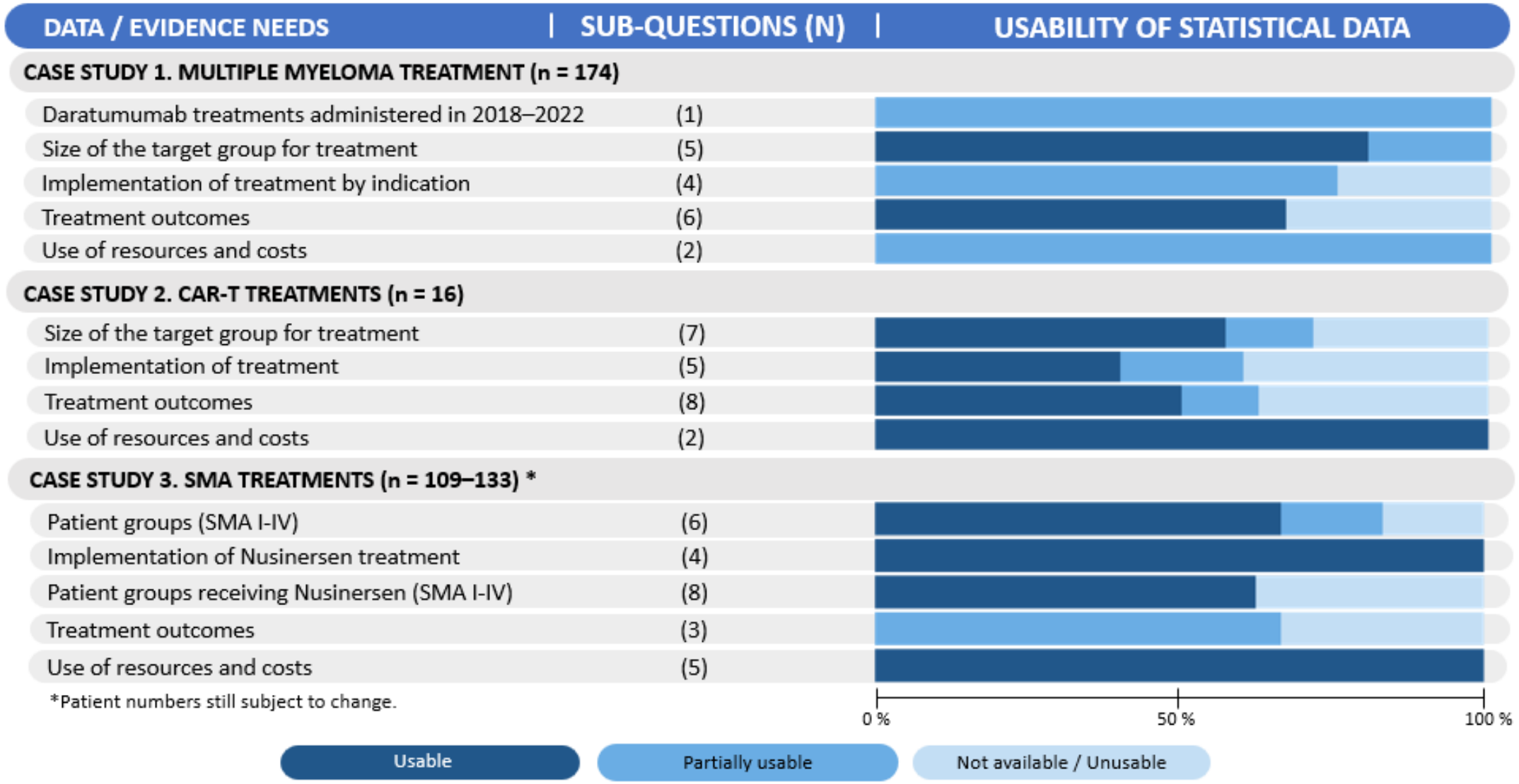
# 4. Produced statistical data

Image created using MS Copilot

# 4.1. Statistical data according to data and evidence needs

- In the pilot, the OMOP centers provided Fimea aggregated, anonymous statistical data for each case study separately. Before OMOP centers provided the data, Findata verified the anonymity of the results.
- There were differences between OMOP centers in how the purpose of data use was defined in contracts. These differences affected contract processes and subsequently influenced the timeframes, which varied across centers.
- The next slide provides a summary of the questions posed to the OMOP centers and an evaluation of the usability of the statistical data
  - The number of patients in the case examples was calculated from the statistical data provided by the OMOP centers
  - Topics related to data or evidence needs (such as treatment outcomes) have multiple sub-questions. The table indicates the number of these sub-questions. A comprehensive list of the sub-questions is provided in the appendices.
  - The usability of the data from the perspective of HTA was assessed with the following classification: usable, partially usable, not available/unusable

# Usability assessment of provided statistical data




Final report of the pilot project based on OMOP harmonization and federated analyses, May 2024

# 4.2. Observations from the usability assessement

- Data corresponding to data and evidence needs was mostly available
- The main delays in delivering the data occurred, because not all the data requested by Fimea was available in the OMOP databases. The centers standardized some of the missing data to the OMOP common data model.  In the SMA case example, however, data was compiled from patient records without OMOP standardization. Consequently, the accuracy of the centrally compiled SMA data varied, and there were challenges in pooling the data.
- Aggregated statistical data, which is generated using OMOP CDM and federated analysis can currently be used in HTA for purposes such as:
  - budget impact analysis (number of patients)
  - characterization of target population
  - to a limited extent in monitoring outcomes and resource use
- The indications for new medicines are typically very precise, for example considering disease stage, biomarkers, prior treatments, or treatment responses. The lack of structured EHR data corresponding to specific indications limited the usability of the data stored in the OMOP databases.
- In the future, the secondary use of healthcare data should enable more precise assessment of treatment utilization, outcomes, and costs within well-defined target populations

# 5. Criteria and principles



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# 5.1. Production of anonymous aggregated data in federated analytics

- Evidence generation after licensure or launch of a new medicine often involves using data from small patient groups. For example, the number of patients treated in Finland might range from a few to several dozen.
- In the pilot, Findata was asked for a guidance for producing anonymous results. According to Findata, the results should be masked in aggregated statistical data when there are 1–3 observations.
  - Findata's definition of anonymous data is based on the fact that it should not be possible to directly or indirectly identify an individual person based on the results. More detailed information can be found on Findata's website.
- In the pilot, each OMOP center sent the center-specific intermediate results to Findata for anonymity verification. After that, the OMOP centers transferred the results to Fimea. The intermediate results were masked according to the agreed practice:
  - Observations 0: marked as 0
  - Observations 1–3: marked as 1–3
  - Observations >3: marked as the number of observations
- In the next projects that involve the development of federated analytics-based operating models, the need to mask intermediate center-specific aggregated results must be reassessed:
  - Masking intermediate results diminishes the usability of pooled result datasets
  - Findata's role is to verify the anonymity of the results intended for publication, not the anonymity of intermediate results
- Further studies could evaluate the appropriateness of, for example, the following steps:
  - The data holder produces centrally aggregated anonymous data from a secure operating environment. These intermediate results may be sufficiently anonymous even without masking observations of 1–3.
  - The client pools the received intermediate results and applies the minimum frequency principle to the statistical data. If necessary, Findata verifies the anonymity of the statistical data.

# 5.2. Preliminary principles for sharing the results of federated analytics in HTA use cases

- Fimea shares the statistical data compiled in the pilot, upon request, with those authorities and HTA bodies whose tasks are related to HTA or managed entry of new medicines
- Since the summary data is anonymous aggregated statistical data, it can be published by properly mentioning the source, for example, in HTA reports
  - Suggested reference: Fimea. Real World Data (RWD) on medicine x / patient group y. Compiled by the FinOMOP consortium [date of data delivery]
- The preliminary principles concern data
  - that Fimea has requested from the FinOMOP consortium for the HTA of medicines
  - of which anonymity has been verified by Findata

# 6. Proposals for action and development needs



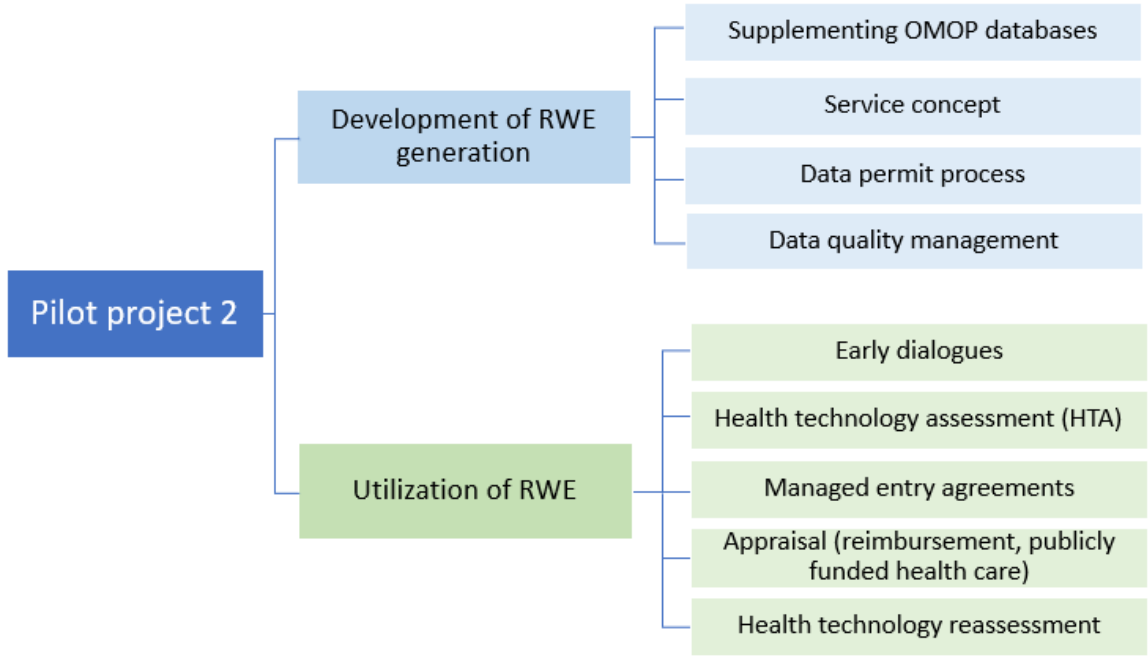
Image created using MS Copilot

# 6.1. Proposals for action

- To continue development of the OMOP databases and the federated analysis-based operating model for generating RWD and RWE from new medicines, launch a new collaborative project
- Expand the project to cover both development of the operating model and utilization of generated evidence in the managed access of new medicines
- Assess if national registry data, for example from Kanta Prescription center or Care Register (Hilmo and avoHilmo) can be utilized in evidence generation. Based on the assessment decide wheather to incorporate these data sources into the collaboration project.
- Invite a wider group of stakeholders to join the project, e.g. Council for Choices in Health Care in Finland (COHERE), Pharmaceutical Pricing Board (Hila), The Social Insurance Institution of Finland (Kela), National Assessment Network, representation of the National Advisory Committee on Medicines, the pharmaceutical industry, and patient representation
- The following slides provide a more detailed description of the development needs

**Areas of the follow-up pilot project**

*Development and utilization of data production*



# 6.2. Development needs (1)

## INFORMATION

- **HTA bodies and FinOMOP:** When defining data and evidence needs and planning data extraction and analysis, ensure that the results can be reported with precision corresponding to the indications of the treatment. Avoid excessive aggregation of data related to patient groups and treatment lines, as this can negatively impact the usability of statistical data in HTA.
- **Finnish Institute for Health and Welfare, health information system users and vendors:** Clinical documentation should be developed so that the basic population of the statistic can be formed with a precision corresponding to the indication of the medicine. In cancer treatments, for example, consider factors like number of prior treatment lines and medicine combinations used.
- **FinOMOP and HTA bodies:** Research protocols and the results of feasibility assessments should be published so that they are openly available and easy to find

## OMOP DATABASE

- **FinOMOP and Finnish Institute for Health and Welfare:** Creating a centralized national OMOP database could improve data harmonization, speed up data extraction and analysis. When the number of observations in a single center is only 1–3, a national OMOP database improves the possibility for accurate reporting of data.
- **FinOMOP:** Initiate separate development projects to standardize data missing from the OMOP databases
- **Finnish Institute for Health and Welfare and health information system users and vendors:** The quality of data in OMOP databases is conditional on high-quality and uniform clinical documentation. OMOP databases and the experiences gained from their use should also be used when developing clinical documention and data sharing.

# 6.2. Development needs (2)

## PROCESSES

- **Ministry of Social Affairs and Health:** During the development of HTA activities**,** integrate post launch evidence generation into HTA and decision-making processes. Ensure appropriate clinical documentation and evidence generation following the launch of new medicines.
- **FinOMOP and Fimea:** Anticipate data and evidence needs related to new medicines prior to HTA. Engage in early dialogues to identify requirements for standardizing data into the OMOP format, facilitating timely evidence generation for decision-making.
- **FinOMOP:** Develop the various steps of the operating model to ensure that lead times  match client requirements.

## ACTORS

- **HTA bodies:** Include interaction with other key data users (such as the pharmaceutical industry, decision-makers, and payers) during the process of defining data and evidence needs. The goal is to ensure the usability of data and evidence for multiple purposes across stakeholders.
- **FinOMOP:** Quality work for secondary use of social and health data should be initiated and minimum quality requirements defined. The data quality management should be developed to meet the requirements of key stakeholders such as the European Medicines Agency (EMA).
- **Data holders:** Develop uniform practices for issuing permits and implementing services for federated analytics

# 6.2. Development needs (3)

## COMPETENCE

- **Ministry of Social Affairs and Health:** During the development of HTA activities, focus also on enhancing the competence and processes of HTA bodies, decision-makers, and payers. This ensures their ability to define decision-relevant questions and effectively utilize the generated evidence in decision-making and procurement.
- **FinOMOP and Finnish Institute for Health and Welfare:** When developing operations, it is necessary to ensure sufficient competence and resources for the required service production, including data extraction, reporting, analytics, algorithm production, quality management, and coordination of the network of stakeholders.

## LEGISLATION

- **Ministry of Social Affairs and Health:** The need for legislative changes that would streamline federated analytics and enable the development and resources of a national OMOP database should be assessed. The national database should enable data extractions for needs such as research, assessment, knowledge-based management, and value-based steering.

# 7. Additional information

Image created using MS Copilot

# Concepts (1)

| CONCEPT | DESCRIPTION |
|---|---|
| **Aggregated data** | According to Findata's definition, aggregation is a statistical procedure in which data is combined and added together. Aggregated data describes a group of people instead of an individual person. The data for these groups of people has been formed in such a way that individuals cannot be identified. |
| **Anonymisation of Data** | According to Findata's definition anonymisation is a process in which the data is processed in a way that<br>• an individual person cannot be directly or indirectly identified<br>• conclusions cannot be made about just one individual person<br>• data about an individual cannot be combined with other data<br>Anonymous data should be impossible or exceedingly difficult to revert to a form where an individual person is identifiable. According to Act on the Secondary Use of Social and Health Data, the results must be anonymous. |

# Concepts (2)

| CONCEPT | DESCRIPTION |
|---|---|
| **Federated Data Analysis** | An approach in which aggregated statistical data is produced locally. Afterwards, the statistics produced by different regions/centers are combined, analyzed, and reported in a centralized manner. |
| **Horizon Scanning** | According to HTA Clossary Horizon Scanning in the context of HTA is about the systematic identification of health technologies that are new, emerging or becoming obsolete and that have the potential to effect health, health services and/or society.<br><br>Note 1: Related terms include early awareness and alert system |
| **OMOP CDM** | The Observational Medical Outcomes Partnership (OMOP) Common Data Model (CDM) is an open community data standard for healthcare. It is designed to standardize the structure and content of observational data and to enable efficient analyses that can produce reliable evidence. See more on OHDSI (Observational Health Data Sciences and Informatics) webpage. |

# Concepts (3)

| CONCEPT | DESCRIPTION |
|---|---|
| **Real-World Data (RWD) and Real-World Evidence (RWE)** | According to HTA Glossary:<br>• RWD is data collected during the routine delivery of health care<br><br>Note 1: Sources may include observational data, administrative data, research data, patient-generated data or professional-generated data. These data may be collected in administrative datasets, case notes, surveys, product and disease registries, social media, electronic health records, claims and billing datasets, or mobile health applications.<br><br>• RWE is evidence derived from the analysis of real-world data |
| **Secondary use of Social and Health Data** | The secondary use of social and health data refers to the utilization of customer and register data for a purpose other than that for which they were originally stored (primary purpose) in health and social service sector activities. The scope of the Act on the Secondary Use of Social and Health Data (552/2019) is defined as: statistics; scientific research; development and innovation activities; teaching; knowledge management; steering and supervision of social and health care by authorities; and the planning and reporting duties by authorities. See more on the Ministry of Social Affairs and Health webpage. |

# Appendices

- Draft template for feasibility assessment
- Case Example 1. Treatment for Multiple myeloma
- Case Example 2. CAR-T treatments
- Case Example 3. Treatments for SMA
- Project team



Image created using MS Copilot

# Draft template for feasibility assessment

**1. Submitted by**

1.1. Name

1.2. Organization

1.3. E-mail

1.4. Phone

**2. Data request**

2.1. Title

2.2. Objective and study questions (detailed study questions in appendix)

- Primary objective:
- Secondary objective:

2.3. Purpose

2.4. Population

- Inclusion criteria:
- Exclusion criteria:

2.5. Variables (minimun dataset in the appendix)

2.6. Geographic requirements

2.7. Timelines

**3. Feasibility feedback**

3.1. Data request is feasible (yes/partially)

3.2. Data request is not feasible (e.g. low number of observations within the database, indication of interest cannot be identified)

3.3. Preliminary feasibility assessment calculations concerning the amount of center-specific data (not patients) available

3.4. Recommendations on choice of OMOP database partners

3.5. Other recommendations concerning the application of the data (objectives, study population, choice of study design, etc.)

# Case Example 1. Treatment for Multiple myeloma

- Daratumumab treatments administered in 2018–2022
- Size of the target group for Daratumumab treatment
  - How many patients were treated?
  - Basic information about the treated patients
  - For what indications was the treatment used?
  - How many patients underwent a stem cell transplant prior treatment with Daratumumab?
  - How many patients underwent a stem cell transplant after treatment with Daratumumab?
- Implementation of Daratumumab treatment by indication
  - In which line of treatment was it used?
  - In what combinations was it used?
  - What previous treatments had the patients received?
  - What follow-up treatments did the patients receive?
- Daratumumab treatment results
  - What response was achieved in patients?
  - Time until relapse or next treatment?
  - What was disease-related mortality?
  - What was the survival time of patients (both from diagnosis and treatment initiation)?
  - How soon did the disease progress?
  - What adverse effects did patients experience?
- Use of resources and costs
  - What was the total cost of treatment after receiving Daratumumab?
  - What was the number of hospital days for patients?

# Case Example 2. CAR-T treatments

- Size of the target group for the treatment
  - How many patients belonging to the CAR-T treatment target group were treated in 2019–2022?
    - B-cell acute lymphoblastic leukemia (ALL)
    - Diffuse large cell B-cell lymphoma (DLBCL)
    - Primary mediastinal large B-cell lymphoma (PMBCL)
    - Mantle cell lymphoma (MCL)
    - Follicular lymphoma (FL)
    - High-grade B-cell lymphoma (HGBL)
- Implementation of treatment
  - For how many patients were preparations for CAR-T treatment started 2019–2022?
    - Tisagenlecleucel (L01XX71)
    - Aksicabtagene ciloleucel (L0100XX70)
    - Brexucabtagene autoleucel (L01XL06)
  - How many patients have received CAR-T infusion in 2019–2022?
    - Tisagenlecleucel (L01XX71) – ALL, DLBCL, FL
    - Aksicabtagene ciloleucel (L0100XX70) – ALL, PMBCL, FL, HGBL
    - Brexucabtagene autoleucel (L01XL06) – ALL, MCL
  - Has CAR-T treatment been targeted at individuals in good condition (ECOG 0–1)?
  - How many treatments had the patients received before CAR-T treatment? Which treatments?
  - How soon after diagnosis was CAR-T therapy administered?
- Treatment results
  - What is the disease and treatment-related mortality rate of patients treated with CAR-T?
  - What is the survival time of treated patients, from diagnosis, from CAR-T treatment decision, and from receiving the treatment?
  - How many patients underwent a stem cell transplant after CAR-T treatment?
  - What response was achieved in patients with CAR-T therapy?
  - What follow-up treatments were given and how soon after CAR-T therapy?
  - What is the survival time for patients receiving follow-up treatment after CAR-T therapy (from the start of follow-up therapy)?
  - What were the most common serious adverse events in patients during/after CAR-T therapy?
  - How many patients received Tocilizumab?
- Use of resources and costs
  - What were the total costs of CAR-T therapy from the time of infusion onwards?
  - What was the number of hospital days for patients?

# Case Example 3. Treatments for SMA

- Patient groups*
  - How many new SMA patients have been diagnosed (incidence) in 2018–2022?
    - Number by SMA type (0-IV)
    - Number according to SMA 2 copies (0, 1, 2, 3, 4, > 4)
  - What is the age of patients at the time of symptom onset?
  - What is the age of patients at the time of diagnosis?
  - What is the gender distribution of patients?
- Implementation of treatment
  - How many patients have received any of the following treatments:
    - Nusinersen
    - Risdiplam
    - Zolgensma
  - How many doses of Nusinersen per year have been administered?
- Patient groups receiving Nusinersen*
  - What is the
    - SMA type (I-IV) of the patients who received treatment?
    - number of SMA 2 copies (0, 1, 2, 3, 4, > 4)?
  - What was the age of the patients when the treatment started?
  - How long was the time from the onset of symptoms to the start of treatment?
- In what proportion of patients treated with Nusinersen has treatment been discontinued?
  - What was the average duration of treatment for the patients treated?
  - What was the reason for discontinuation of treatment? (permanent ventilation, death, adverse events, lack of efficacy, other reason)?
- Treatment results*
  - What was the time from diagnosis to death or permanent ventilation for treated patients?
  - What was the time from the start of treatment to death or permanent ventilation for treated patients?
  - What are the motor function-related treatment outcomes by type (e.g., type I: sitting, standing without support; type II: separate indicators, such as HINE, CHOP INTEND)?
- Use of resources and costs*
  - What was the number of hospital days for patients?
  - What kind of treatment did the patients receive?
    - Respiratory treatment
    - Digestive system and nutritional treatment
    - Orthopedic treatment and rehabilitation
    - Palliative care

*The information in the sections is distributed by SMA types I to IV

# Project team

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