RWE4Decisions
REAL WORLD EVIDENCE
Public Webinar

From Policy to Practice:

EHDS Implementation to Support Better Real-World Evidence for HTA/Payers

9 April 2025

15:00 - 16:30 CEST

on Zoom

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WHO WE ARE

HTA/Payer-led multi-stakeholder learning network

OUR PRINCIPLES

- Transparency
- Collaboration

OUR WORK

CHALLENGE

Highly innovative medicines often have immature clinical evidence (and high prices).

LEADING QUESTIONS

- How **fit-for-purpose Real-World Evidence** (RWE) can be generated over the life cycle of highly innovative medicines to inform HTA/Payer decisions?
- Can **requirements be aligned** across stakeholders and health jurisdictions/Payers?

RWE4Decisions REAL WORLD EVIDENCE 2025 STEERING GROUP

HTA bodies / Payers

Jo De Cock

Senior Advisor, **INAMI-RIZIV**

Francis Arickx

Advisor, **INAMI-RIZIV**

Niklas Hedberg

Chief Pharmacist, **TLV**

Piia Rannanheimo

Chief Specialist, **Fimea**

Cláudia Furtado

Head HTA, P&R Div. and Information & Strategic Planning, **INFARMED**

Christian Dehlendorff

Biostatistical Chief Consultant, **Danish Medicines Council**

Carlos M. Saborido

Director of the HTA Agency, **ISCIII**

Shaun Rowark

Associate Director for Data Access and Analysis, **NICE**

Elena Lungu

Director, CDA Data Systems and Analytics, **CDA**

Industry

EUCOPE

astellas

AstraZeneca

GILEAD

MSD

Roche

Takeda

International Org.

Eric Sutherland

Senior Health Economist, **OECD**

Patient Representatives

Chris Sotirelis

Patient Advocate for **Thalassemia**

Julien Delaye

Patient Engagement Manager **EURORDIS**

Insurer

Hans-Georg Eichler

Consulting physician, **Austrian Social Insurance Inst.**

Clinician

Matti Aapro

Director, **Genolier Cancer Centre**

Analytics Expert

Ashley Jaksa

Market Access Scientific Strategy Lead, **Aetion, US**

Academia

Entela Xoxi

Pharmacologist, **Uni. Cattolica Sacro Cuore**

Facilitators

FIPRA International

Karen Facey, Senior Adviser (HTA)

François Meyer, Special Adviser (HTA)

Secretariat provided by FIPRA funded by EUCOPE and member companies

RWE4Decisions REAL WORLD EVIDENCE contribution to the EHDS

Calling for a robust EHDS framework (2020-2022)

2020 - Announcement of the European Data Strategy, including the EHDS

- Call for a **RWE Learning Network** within the EHDS

2022 - EHDS proposal

- Call to include **RWE and HTA/Payer considerations** in the EHDS

RWE4Decisions

Call to Action

RWE4Decisions calls for Multi-Stakeholder EU Learning Network on Real-World Evidence within the European Health Data Space

Real-World Data (RWD) collection and Real-World Evidence (RWE) generation can play a critical role in assessing the value of treatments for patients and healthcare systems, as RWD can fill evidence gaps that might exist in clinical development programmes and bring insights into effectiveness in 'real life' settings.

RWE is essential to support decision-making and is particularly relevant when 'highly innovative technologies', which are potentially transformative (such as immunotherapies and cell & gene therapies), come to market early via expedited regulatory approvals to benefit patients and often only limited evidence from traditional clinical development programmes is possible. This is particularly relevant in oncology and treatment of patients with rare diseases.

The COVID-19 outbreak has highlighted the importance of a digital infrastructure to enable the use of RWD for rapid decision-making in our healthcare and political systems. There has never been more of a need for a "learning healthcare system" that gathers local, real-life evidence to inform complex models about future possibilities.

Payers and HTA bodies need to find new ways to evolve their decision-making processes, collaborating with regulators and other stakeholders to share learnings and best practice on the use of RWE in order to address the uncertainties regarding the value of new technologies across the lifecycle of the technology.

RWE4Decisions calls for the creation of a **multi-stakeholder EU Learning Network on Real-World Evidence**, which is based on a transparent governance mechanism. This Learning Network, designed for Member States to implement evidence-based decision-making, should be supported by EU funding, and:

1. clarify **when, by whom and how** real-world data should be collected in order to generate real-world evidence that **meets the needs of patients and healthcare systems**;
2. be based on a **voluntary mechanism**;
3. be underpinned by **robust methodologies** in alignment with other initiatives.

Stakeholder Statement (2025)

Call for Effective Stakeholder Engagement and Capacity Building during the Implementation of the EHDS

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RWE4Decisions REAL WORLD EVIDENCE

A **multi-stakeholder learning network** that supports **open dialogues** to determine how fit-for-purpose RWE can be generated over the **life cycle of highly innovative medicines** and inform HTA/Payer decisions:

- Systems to identify medicines most likely to need RWE and anticipate RWE generation (early planning).
- Clarifying how RWE should be generated by Health Technology Developers and how it will be assessed by HTA and used by Payers/decision-makers.
- Improving the execution of effective Post Launch Evidence Generation (PLEG) studies and aligning them across jurisdictions where possible.

Housekeeping

Today's webinar is scheduled for **1.5 hours**

This meeting will be **recorded** and made available at *rwe4decisions.com*

Please **use the Q&A function** to ask a question or make a comment
You can **upvote** other participant's comments

When asking a question, please **rename yourself** on Zoom
to show name and affiliation

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CO-MODERATORS

ERIC SUTHERLAND

Senior Health Economist, OECD

KAREN FACEY

RWE4Decisions Facilitator

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PANELLISTS

KEYNOTE

DAVID ASTURIOL
Policy Officer, DG SANTE, European Commission

NATIONAL IMPLEMENTATION

MAARI PARKKINEN
Development Manager, Findata
Project Manager, FinHITS

JØRGEN SCHØLER KRISTENSEN
Chairman of the Pan-European Observatory, Health Outcomes Observatory (H2O); Former Chairman, Danish Medicines Council

DISCUSSANTS

INGRID MAES
Managing Director & Founder, Inovigate; RWD4BE Multi-stakeholder initiative

ALEXANDER NATZ
Secretary General, EUCOPE

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KEYNOTE ADDRESS

DAVID ASTURIOL

Policy Officer, ‘Digital Health’ Unit,
DG SANTE, European Commission

EHDS – Secondary uses of data

David Asturiol
Policy officer on the EHDS

European Comission
DG for Health and Food Safety
Unit C1 – Digital Health

EHDS in a Nutshell – what is it about?

Primary use = use of data for the delivery of healthcare

Improving patients' access to their health data;

Ensuring seamless exchanges for continuity of healthcare.

Secondary use = use of data for research and public interest purposes

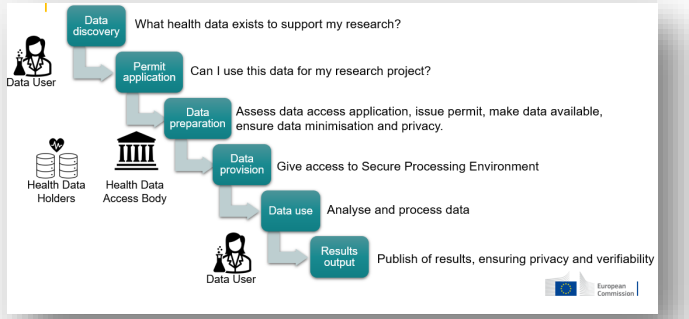
Making data available for research, policy-making etc. in a safe and secure way.

Requirements for electronic health record (EHR) systems

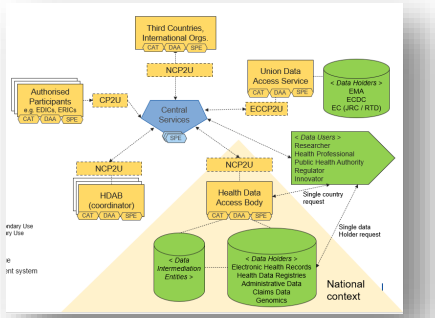
Creating a single market for electronic health records systems, supporting both primary and secondary use.

EHDS in a Nutshell – Secondary Use

- **Common European rules on**:
  - Who has to make data available, → Art 2(1t) & Art 50
  - Which data have to be made available, → Art 51
  - For which purposes, → Art 53&54
  - Under which conditions → Art 68
- **Establishes obligations for:**
  - Health Data Access Bodies,
  - Data holders,
  - Data users
- **Establishes an EU infrastructure to share data**
  - HealthData@EU



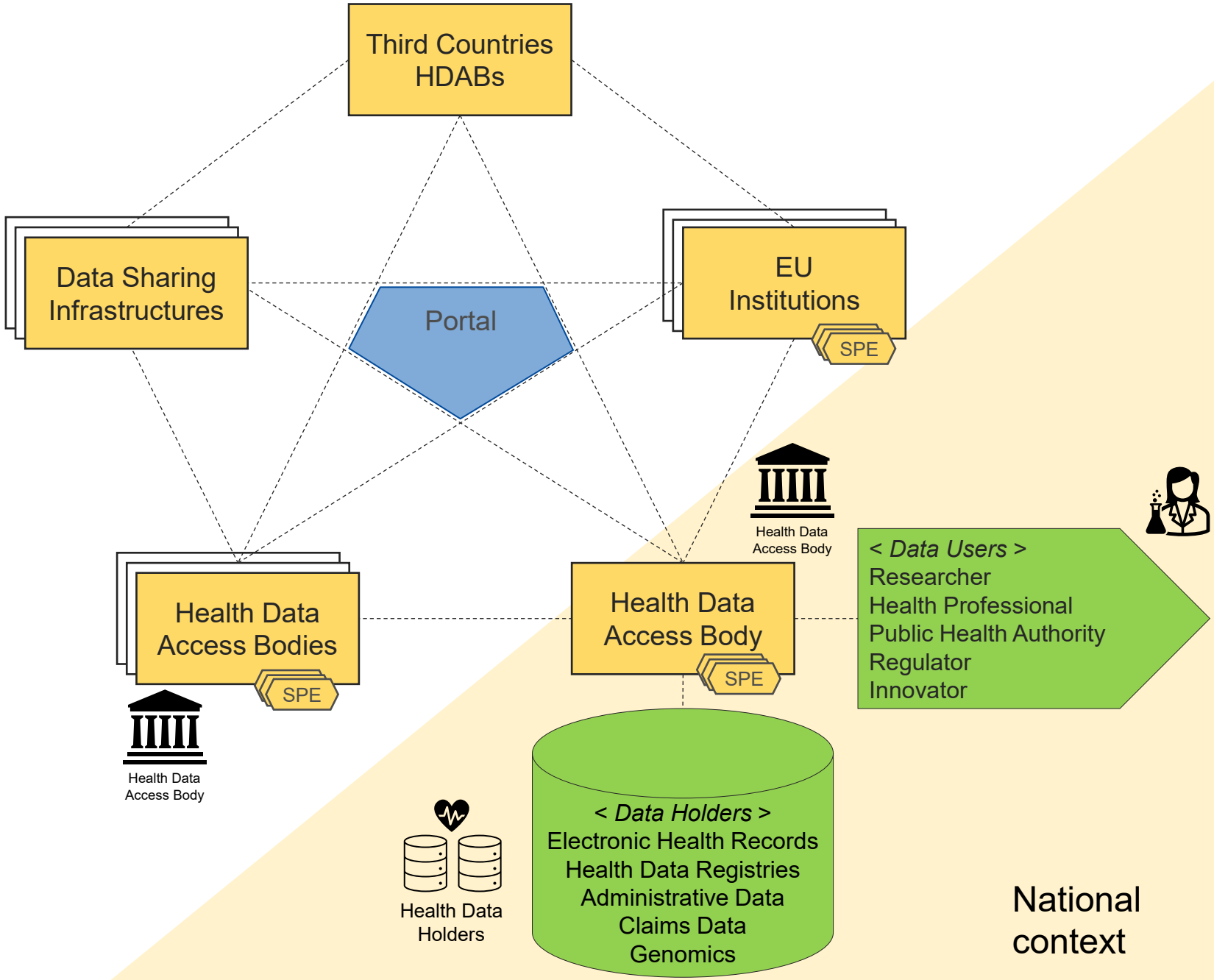
Making (more) data available for research, policy-making etc. in a safe and secure way



European Commission

Cross-border secondary use infrastructure

HealthData@EU



Central support services provided by EC

National data management services provided by authorised participants

SPE

Secure Processing Environments

Local services provided by/to local partners

EHDS in a Nutshell – Secondary Use

- **Common European rules on**:
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- **Establishes obligations for:**
  - Health Data Access bodies,
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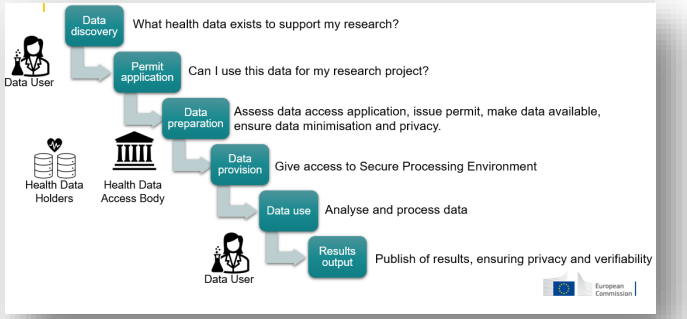
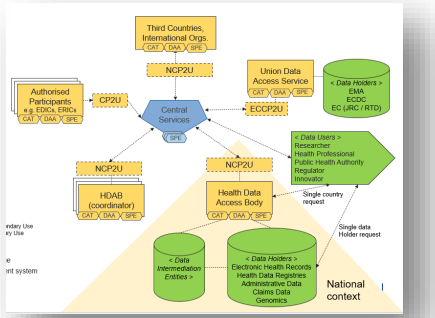
  - Data users
- **Establishes an EU infrastructure to share data**
  - HealthData@EU
- **Common procedure to access data**

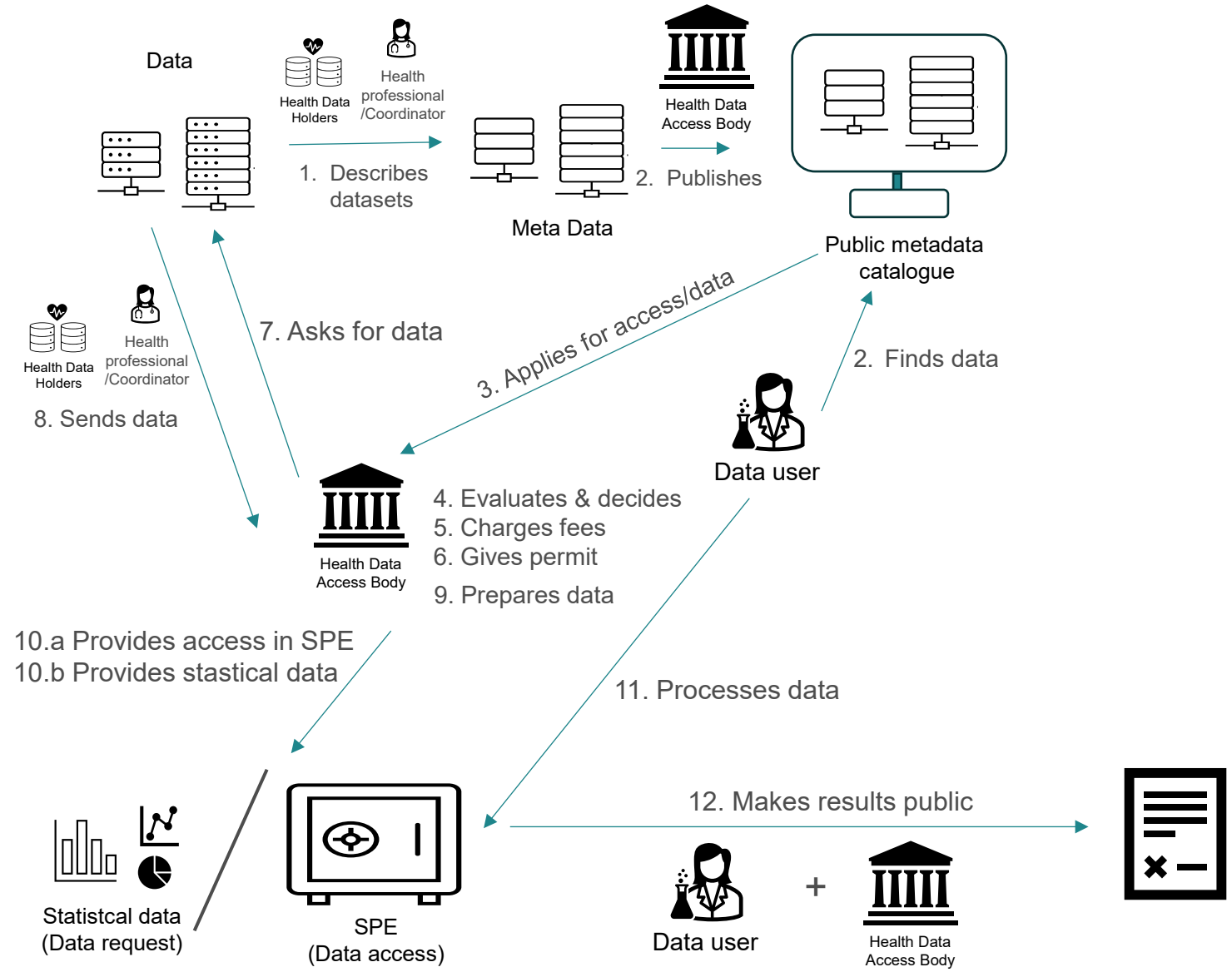
Making (more) data available for research, policy-making etc. in a safe and secure way





European Commission

User Journey EHDS



Main Duties

Health professional /Coordinator, Health Data Holders

1. Describe datasets (DQUL)
2. Provide datasets to HDAB

2.1 Receive fees

Data user

1. Find datasets
2. Submit applications
3. Pay fees
4. Process data according to EHDS
5. Make results public

Health Data Access Body

1. Publish metadata records
2. Evaluate and take decisions on access
3. Charge fees
4. Give permit
5. Ask data to data holders
6. Prepare data (e.g. link, anonymise)
7. Place data in SPE (no opted-out)
8. Provide access to data
9. Monitor processing in SPE
10. Make process transparent (e.g. Publish application, permit, results)

EHDS in a Nutshell – Secondary Use

- **Common European rules on**:
  - Who has to make data available,
  - Which data have to be made available,
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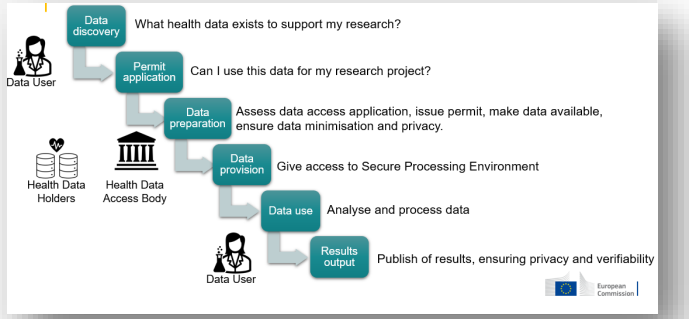
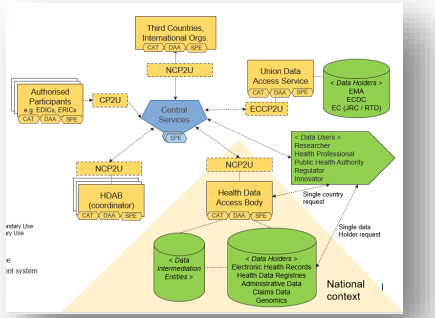
  - Under which conditions
- **Establishes obligations for:**
  - Health Data Access bodies,
  - Data holders,
  - Data users
- **Establishes an EU infrastructure to share data**
  - HealthData@EU
- **Common procedure to access data**
- **Streamlined process & safeguards for privacy of data subjects**:
  - Data access application – evaluation - permit process,
  - Anonymisation/pseudonymisation of data,
  - SPE,
  - Opt-out

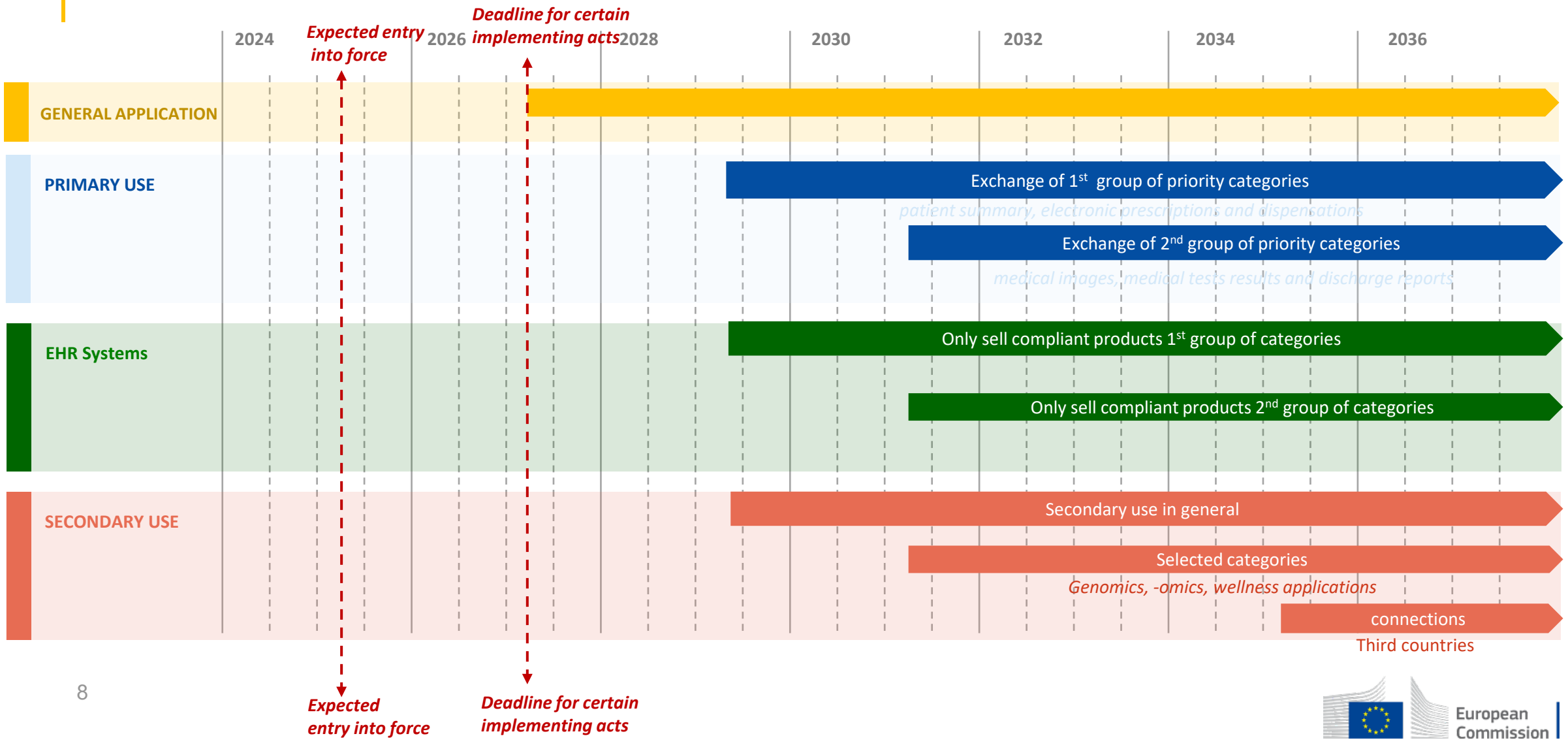
Making (more) data available for research, policy-making etc. in a safe and secure way





European Commission

EHDS – Overall timeline for application



European Commission

Thank you

European Commission

EHDS in a Nutshell – Secondary Use

- **Common European rules on**:
  - Who has to make data available,
  - Which data have to be made available,
  - For which purposes,
  - Under which conditions

Making (more) data available for research, policy-making etc. in a safe and secure way

Making decisions detrimental to individuals or groups based on electronic health data, qualifying as decisions if they have legal, social, or economic impacts.

- Making employment-related decisions or offering less favorable terms in goods or services based on health data, including discriminatory decisions affecting insurance, credit, or loans.
- Conducting advertising or marketing activities.
- Developing products or services that could harm individuals, public health, or society, including illegal drugs, alcohol, tobacco, weaponry, or addictive products.
- Engaging in activities that conflict with ethical standards set by national law.

- **Public interest in the area of public and occupational health**, such as activities for protection against serious cross-border threats to health and public health surveillance or activities ensuring high levels of quality and safety of healthcare, including patient safety, and of medicinal products or medical devices;
- **Policy making and regulatory activities** to support public sector bodies or Union institutions, agencies and bodies, including regulatory authorities, in the health or care sector to carry out their tasks defined in their mandates;
- **Statistics**, such as national, multi-national and Union level official statistics defined in Regulation (EU) No 223/2009 related to health or care sectors;

Reserved for public sector bodies *and Union institutions, offices,*

- **education or teaching activities** in health or care sectors at the level of vocational or higher;
- **scientific research** related to health or care sectors, **contributing to public health** or health technology assessment, or ensuring high levels of quality and safety of health care, of medicinal products or of medical devices, **with the aim of benefitting the end-users** including : development and innovation activities for products or services; training, testing and evaluating of algorithms, including in medical devices, in-vitro diagnostic medical devices, AI systems and digital health applications;
- **improving delivery of care, treatment optimization and providing healthcare**, based on the electronic health data of other natural persons.

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PRESENTER

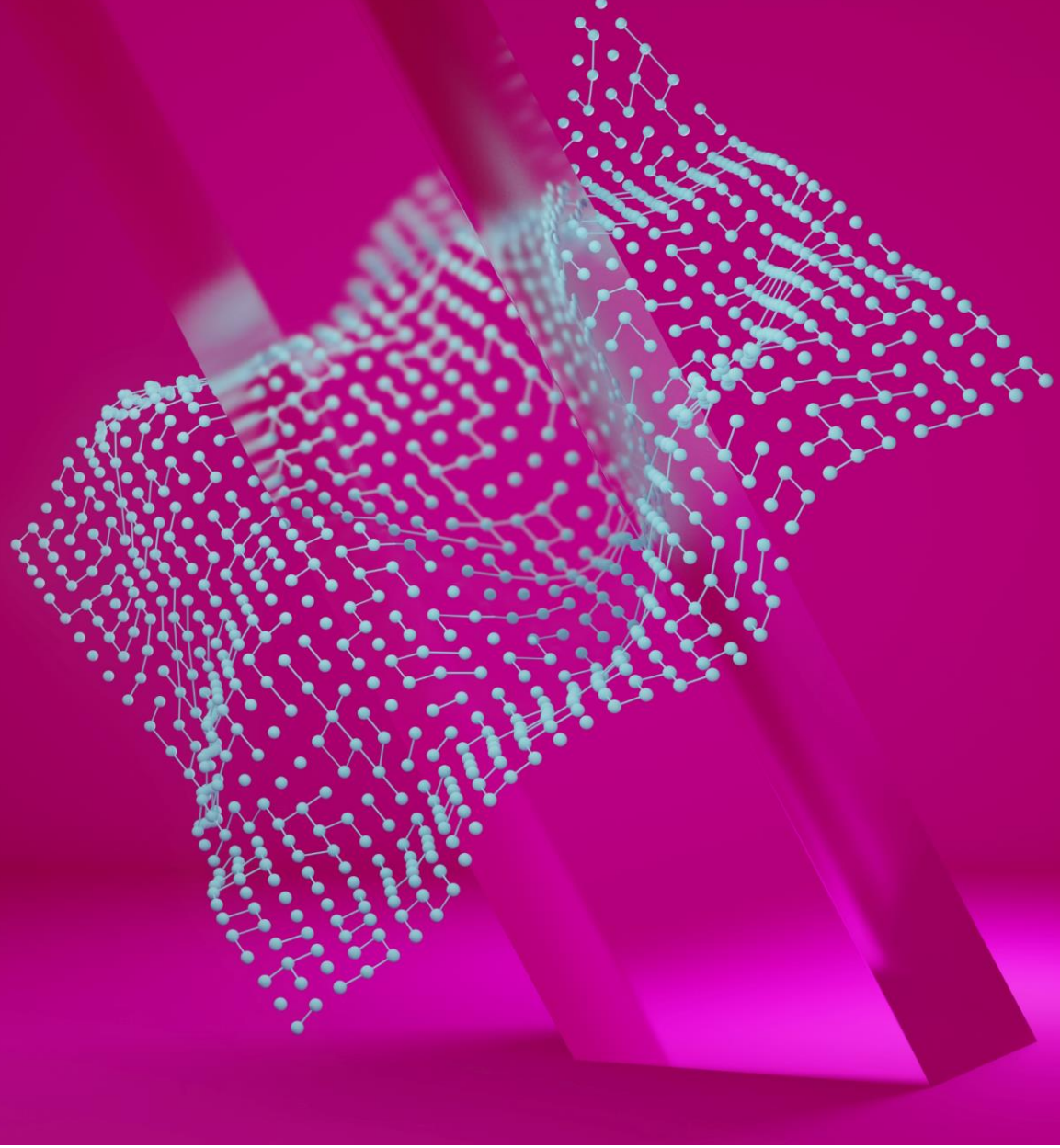
MAARI PARKKINEN

Development Manager, Findata

Project Manager, FinHITS

FinHITS – Towards the Future of Secondary Use of Health Data

Maari Parkkinen, Development Manager
Finnish Social and Health Data Permit Authority Findata



FINDATA

Co-funded by the European Union

Finland's strengths in the secondary use of health data

- **Personal identity code**
  - Key for linking personal information from various registers
- **Rich national data registers** with long time series
  - In practice 100% coverage of Finnish population
- **"Opt-out" principle** in secondary use of social and health data
  - Consent not required for registry-based research
- **High trust** on
  - Government and authorities, transparent operation
  - Benefits from research (as better healthcare)
  - Data security and data protection
- **Legal basis**
  - Act on Secondary Use of Health and Social Data

Introducing Findata – Finnish social and health data permit authority

A centralized data permit authority for secondary use of national social and health data. **Founded in 2019**, operation based on the national Act.

Positioned in Finnish Institute for Health and Welfare, steered by the Ministry of Social affairs and health.

Data from national registers, public and private social and health care service providers and Kanta services.

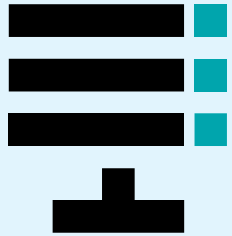
MISSION: Improve access to rich nation-wide datasets and **enhance** data security and the data protection of individuals.

FINDATA

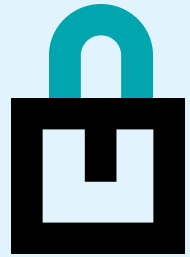
Findata's core services

Help Desk & Metadata Catalogue

Grants access to data, provides aggregated data



Collects and pre-processes data



Provides Kapseli® SPE and transfer services

FINDATA

FinHITS – Strengthening Finnish Health Data ICT for Secondary use

Funding: EU4Health / Direct grants to Member States: for setting up services by Health Data Access Bodies - Secondary use of health data.

Budget: Approx. 2.5 million € (37 million € available in the call) Funding approx. 1,5 million € (60 %)

Duration: 1.11.2023–31.10.2027

More information: findata.fi/finhits

- **Objectives:**
  - Strengthen the Finnish infrastructure to ensure efficient and secure secondary use of health data.
  - Enable Finland's smooth participation in the EHDS
  - Support other member countries by bringing information about practical experiences.
- **Development work based on the**
  - **European Health Data Space** (EHDS) regulation
  - **Practical experiences, feedback, and insights** from national stakeholder groups and customers
  - **EU projects and collaborations**, including HealthData@EU Pilot, Tehdas2, Quantum, and the Community of Practice
- **Main challenge:** project timeline and uncertainties concerning the content of implementation acts and national implementation.

Key areas of development

1. **Data access application management system**: Improvements in collaboration for defining needed data and information flow to support more efficient processes, fewer errors and better anticipation.
2. **National dataset catalogue**: Improve user-friendliness to enhanced discoverability and prepare for standards coming from EHDS.
3. **Secure processing environment:** Support for more advanced methods and large datasets, efficiency in verifying anonymity of results.
4. **Cross-border gateway:** Joining in the HealthData@EU infrastructure.
5. **Data quality**: Support for data quality, data quality and utility label & two ready-made datasets.

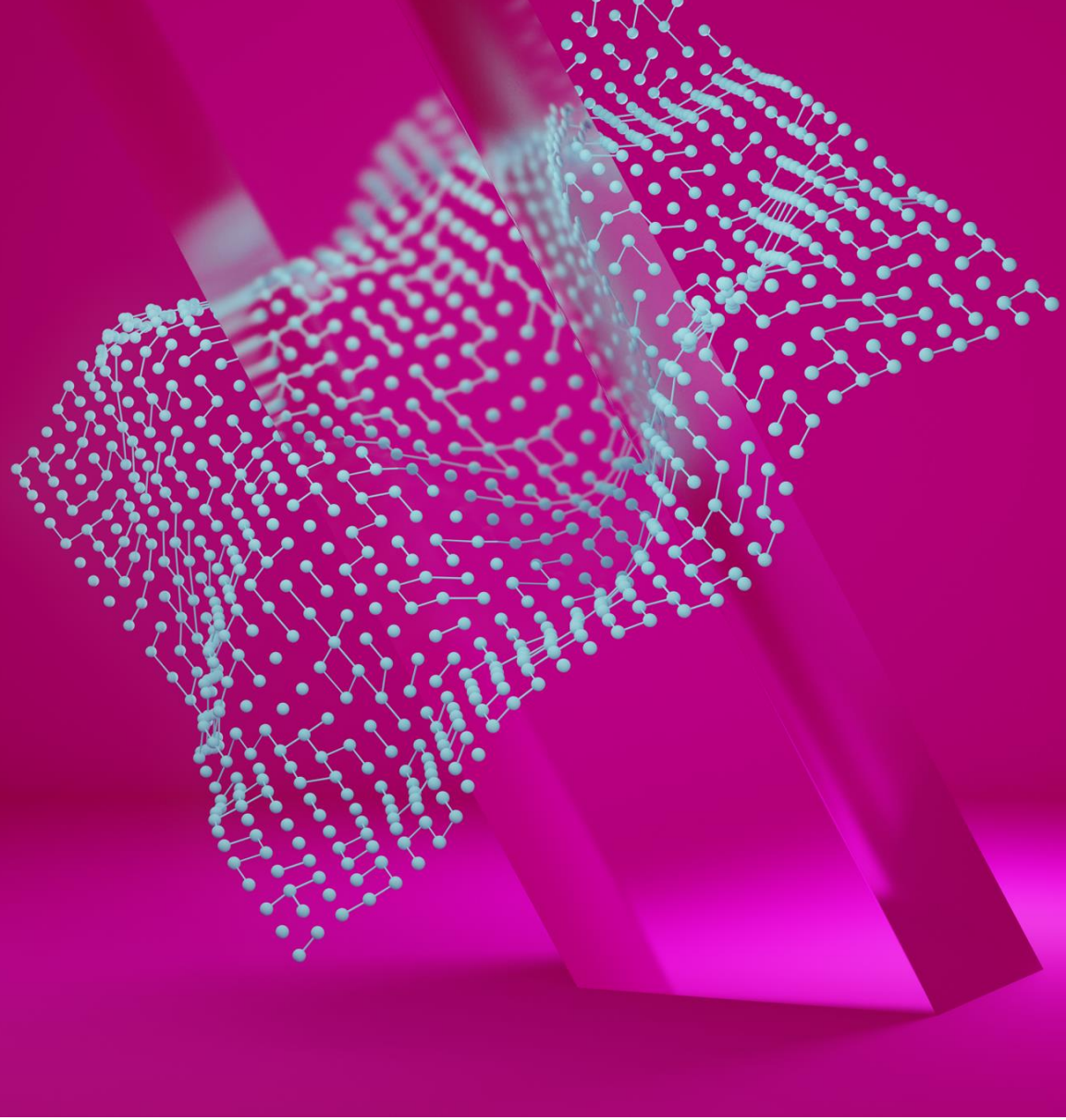
\+ **Service desk:** Expand the available advisory content and guidance.

Challenges and solutions

Work package	Challenges	Solutions
WP 5 – Data Access Application Management System	• Doesn't support smooth data description or communication between different parties. • Real-time information flow.	• Data description tool connected to National Dataset Catalogue and structured data processing, collaborating with controllers. • Improved portal to facilitate information flow. • *Reporting and templates, connection to the HealthData@EU*
WP 6 – National Dataset Catalogue	• Does not meet the needs of growing user base and international clients. • Not EHDS compliant.	• Improving usability and addressing needs of international clients. • Integration with Findata's e-service • *Standards for metadata descriptions, Health-DCAT*
WP 7 – Secure Processing Environment	• Does not support the growing number of users, handle very large datasets, or utilize advanced methods.	• Increasing computing power (GPU) and larger data transfers • Enhanced license management. • Tools for anonymisation and verification • Federated analysis
WP 8 – Cross-border gateway	• No technical solution for the cross-border transfer of health data for secondary use.	• *Connect to the HealthData@EU infrastructure to enable cross-border workflows at EU-scale.*
WP 9 – Health data quality	• Tools and guidelines need to be updated for improving data quality.	• Produce guidelines and instructions (HDAB supporting data quality) • Two ready-made datasets. • *HDAB and data quality and utility label*

Key takeaways

- **Trust:** Transparent communication and guidance for all stakeholders is a must and builds trust.
- **Collaboration:** Efficient processes require close collaboration with ecosystem stakeholders and customers, developing digital capabilities to serve everyone involved.
- **Agile operations:** Learning by doing and learning fast, focus on solving problems one by one



findata.fi/finhits

info@findata.fi | @FindataFi

FINDATA

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PRESENTER

JØRGEN SCHØLER KRISTENSEN

Chairman of the Pan-European Observatory,
Health Outcomes Observatory (H2O)
Former Chairman, Danish Medicines Council

FROM POLICY TO PRACTICE:
EHDS IMPLEMENTATION TO SUPPORT BETTER REAL-WORLD EVIDENCE FOR HTA/PAYERS

H2O HEALTH OUTCOMES OBSERVATORY

The Health Outcomes Observatory (H2O) project

Jørgen Schøler Kristensen
MD, D.Med.Sci
Chairman Pan European Observatory PEO
Former Chairman of the Danish Medicines Council (National HTA)

This project has received funding from the Innovative Medicines Initiative 2 Joint Undertaking under grant agreement No 945345-2. This Joint Undertaking receives support from the European Union's Horizon 2020 research and innovation programme and EFPIA and Trial Nation and JDRF.

How Digitally Captured Standardized Patient Voice can transform health care

H2O HEALTH OUTCOMES OBSERVATORY

Patients wish for their voice to be heard and ensure shared decision making with their HCPs. They wish avoiding unnecessary visits to the hospitals whilst maintaining high quality of diagnoses, treatment and outcomes. They are also keen to remain in control of their data.

Health Care Providers hospitals struggle to deliver to an expanding population of elderly and chose among treatment possibilities while not being able to expand the number of employees. At the same time there is high demand for research.

Health Technology Agencies and European Medicines Agency require more evidence as new more advanced medicines for ex. rare diseases enter market and there are increasing challenges on allocating limited resources.

Pharmaceutical Industry struggles to provide the evidence needed for comparing new treatments to the standard of care.

H_2O | One of the main drivers of this growth is the specific demand from the HTA authorities to see patient outcomes

HTA agencies are looking for the following:

Compound/ drug name	Disease	Outcomes in PICO (QoL measures used)	Advice
Daratumumab, carfilzomib, isatuximab	**Multiple myeloma**	• Progression-free survival • Time to progression	Price negotiation
Ciltacabtagene autoleucel	**Multiple myeloma**	• Cost effectiveness	Price negotiation/ pay-for-performance arrangement
Ripretinib	**Colon cancer, stomach cancer**	• Overall survival • QoL (EQ5D, EORTC – QLQ) • Adverse effects & drop-out because of AE	Price negotiation
Daratumumab	**Amyloidose**	• Overall survival • Organ response • QoL (SF -36) • Adverse effects & drop out because of AE	Negative (flawed cost effectiveness model
Brexucabtagene-autoleucel	**Acute lymphoblastic leukemia**	• Overall survival • Quality of life (EQ5D, EORTC – QLQ) • Adverse events	Negative after conditional reimbursement (missing QoL data)
Efanesoctocog alfa	**Haemophilia A**	• Annual no. of haemorrhages • % of patients without haemorrhages • QoL (Haem –A-QoL physical health) • Adverse effects & drop out because of AE	Positive
Risankizumab	**Colitis ulcerosa**	• Clinical remission • Endoscopic improvement • QoL (IBDQ) • Adverse events & drop out because of AE	Positive
Lisocabtagene maraleucel	**Large B-cell lymphoma**	• Overall survival • QoL (EORTC –QLQ, FACT –lymS)	Price negotiation

However, electronic health records (EHRs) do not capture the data needed for assessing the value of drugs:

- **HTA agencies require specific RWE –** they want insights on overall survival, quality of life, adverse effects, treatment dropouts, and reasons for changing treatment
- **This type of data to assess the value of drugs is not captured in EHRs,** even if they were interoperable – pharma companies must collect this data through a registry
- HTA agencies rely on **registries and observational studies**, but these are **costly, fragmented, and inefficient**

H_2O provides an alternative:

A scalable, registry-quality RWE solution that is more cost-effective, standardized, and patient-centric, making it easier for HCPs to participate

H2O works within the framework of a Pan European Health Outcome Observatory (PEO)

PAN-EUROPEAN OBSERVATORY

- The PanEuropean Health Outcome Observatory (PEO) is an independent legal entity led by **a multi-stakeholder Board** comprising senior clinicians, the European Patient Forum and leaders of HTA agencies (the European authorities assessing the value of drugs)
- The PEO **coordinates an ecosystem** comprising several leading European hospitals, patient organizations and academic institutions who are eager to support this initiative.

Patients

Data collection & consent based on standardized Patient-Reported Outcome (PRO) measures and digital tools

Initial disease areas:

- Inflammatory bowl disease
- Lung and breast cancer
- Diabetes

PRO data & consents

National observatories

AUSTRIA | GERMANY | NETHER-LANDS | SPAIN

Data collection layer, protected under local/national legal and ethical frameworks

Patient-reported data

Provider-reported data

Statistics & analyses

Researchers

Data access as appropriate according to legal/ethical governance, reports, decision support

Benefitting from data access:

- **Patient Organizations**
- **Public/Academic Research**
- **Private Research**
- **Health Authorities**

Patient Solution Companies

Providers

Improved care through improved patient-HCP conversations and patient solutions provided

H2O | The value H2O provides is: Being the unique base for PRO's in Europe

1

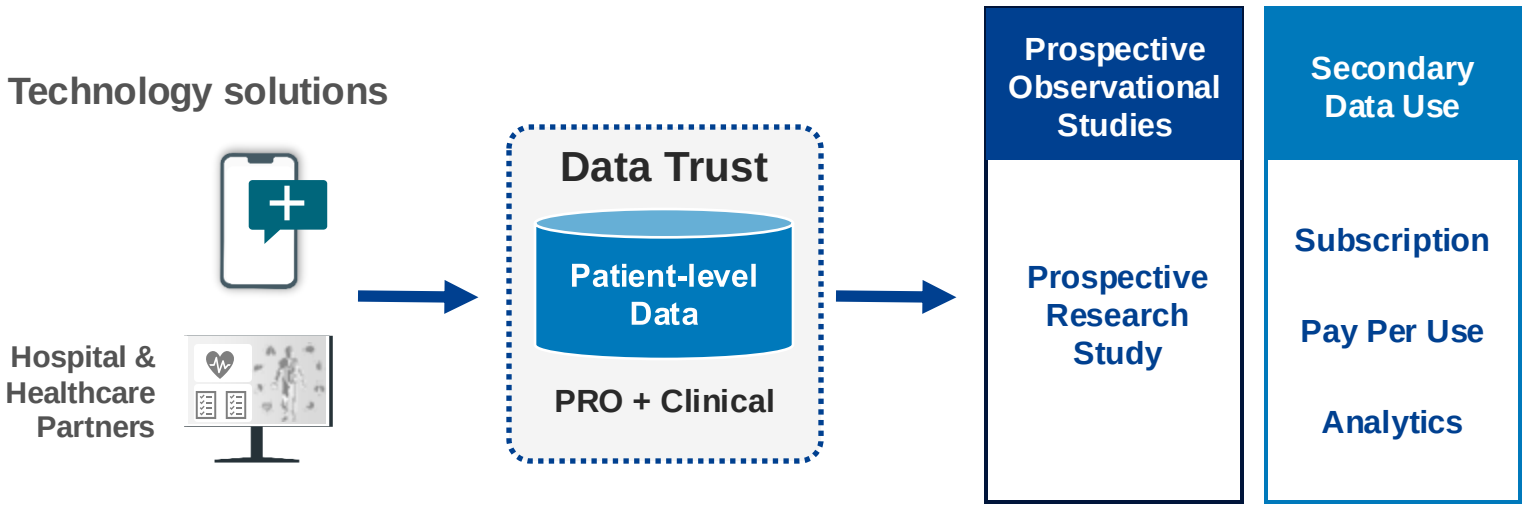
Technology companies develop solutions that are standardized and can be deployed across countries

2

Linked data generated through routine clinical practice is brought into the Data Trust

3

Data Trust allows access in two ways



- Unique Core dataset: connection of both Electronic Health Record data with Patient Reported Outcomes (PRO)
- Automation potential: Automating processes to streamline study setup and execution, reducing costs and timelines
- PRO innovation: Ability to create and implement new PRO core outcome sets to ensure standardization of data collection

H_2O | More work on

1. Need to create better patient and health care provider incentives so most of a population of patients is using PROs as a way of communication between patient and HCP.

2. Standardization on patient outcomes, both clinically captured, and PROs **is critical** but can be done through multistakeholder consensus.

3. Keep trust high with data linking in each country and analysing data at EU level in a federated model for secondary use.

4. More investment is necessary while business model needs to offer value to all stakeholders.

H2O HEALTH OUTCOMES OBSERVATORY

THANK YOU!

health-outcomes-observatory.eu

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DISCUSSANTS

INGRID MAES

Managing Director & Founder, Inovigate
RWD4BE Multi-Stakeholder initiative

ALEXANDER NATZ

Secretary General, EUCOPE

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PANEL DISCUSSION

DAVID ASTURIOL

Policy Officer, DG SANTE,
European Commission

MAARI PARKKINEN

Development Manager, Findata
Project Manager, FinHITS

JØRGEN SCHØLER KRISTENSEN

Chairman of the Pan-European Observatory,
Health Outcomes Observatory (H2O)
Former Chairman, Danish Medicines Council

INGRID MAES

Managing Director & Founder, Inovigate
RWD4BE Multi-stakeholder initiative

ALEXANDER NATZ

Secretary General, EUCOPE

Thank you for your contributions!

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Or get in touch via email at **secretariat@rwe4decisions.com**

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