

**Unlock the potential of real-world data of rare adult solid cancers:
the EURACAN data ecosystem to reduce disparities in cancer**



Annalisa Trama
annalisa.trama@istitutotumori.mi.it



**European
Reference
Network**
for rare or low preva
complex diseases

EU JOINT ACTION
JANE
NETWORKS OF EXPERTISE
ON CANCER



How?



MEDICAL

MEDICAL

MEDICAL

Health Care
Sector
Hospital
Pharmacist
Nurse
Dentist
First Aid
Surgeon
Emergency

MEDICAL

MEDICAL

MEDICAL

MEDICAL

MEDICAL

MEDICAL

MEDICAL

MEDICAL

MEDICAL

Health Care
Doctor
Hospital
Pharmacist
Nurse
Dentist
First Aid
Surgeon
Emergency

MEDICAL

MEDICAL

MEDICAL

MEDICAL

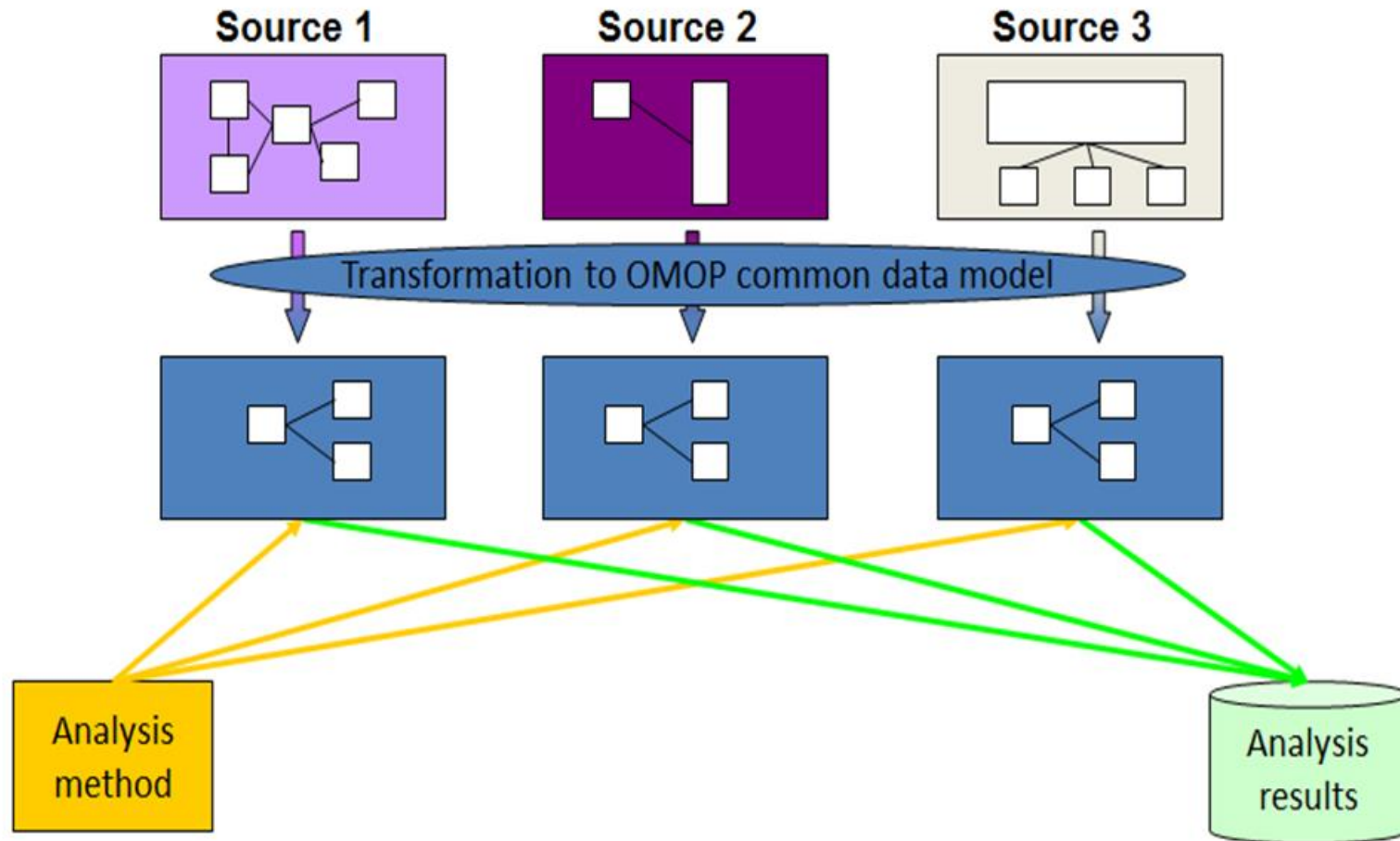
MEDICAL

MEDICAL

MEDICAL

MEDICAL

The Observational Medical Outcomes Partnership (OMOP) Common Data Model





OHDSI

OBSERVATIONAL HEALTH DATA SCIENCES AND INFORMATICS

Open-source process

- Join the collaborative
- Propose a study to the open collaborative
- Write protocol
 - <http://www.ohdsi.org/web/wiki/doku.php?id=research:studies>
- Code it, run it locally, debug it (minimize others' work)
- Publish it: <https://github.com/ohdsi>
- Each node voluntarily executes on their CDM
- Centrally share results
- Collaboratively explore results and jointly publish findings



EURACAN

Power in numbers: overcoming the scarcity of rare cancer data by harmonizing European sarcoma registries

Standardizing European sarcoma registry data to the OMOP Common Data Model: the retroperitoneal sarcoma use case

Background: Research in rare cancers is hampered by low patient numbers, dispersed clinical data and tumor samples, and a limited number of experts per rare cancer diagnosis. The ERN EURACAN (European Reference Network for Rare Adult Solid Cancers) was established to bring together data and knowledge of European Healthcare professionals.

Result 1: # patients of each of the retroperitoneal sarcoma types in each registry

Sarcoma Type	Approximate Patient Count
Well-differentiated liposarcoma	250
Dedifferentiated liposarcoma	200
Leiomyosarcoma	150
Solitary fibrous tumor	100
Malignant peripheral nerve sheath tumor	50
Undifferentiated/unclassified sarcoma	100
Other sarcoma	100

Result 2: Examples of survival curves generated with the CohortSurvival package.

Methods

- 7 European Data Partners
- Data conversion to OMOP-CDM
- Federated analysis

Limitation: Conversion of each of the data sources to the OMOP-CDM and setting up a node for federated analysis requires a lot of technical expertise. The OHDSI tools have a steep learning curve.

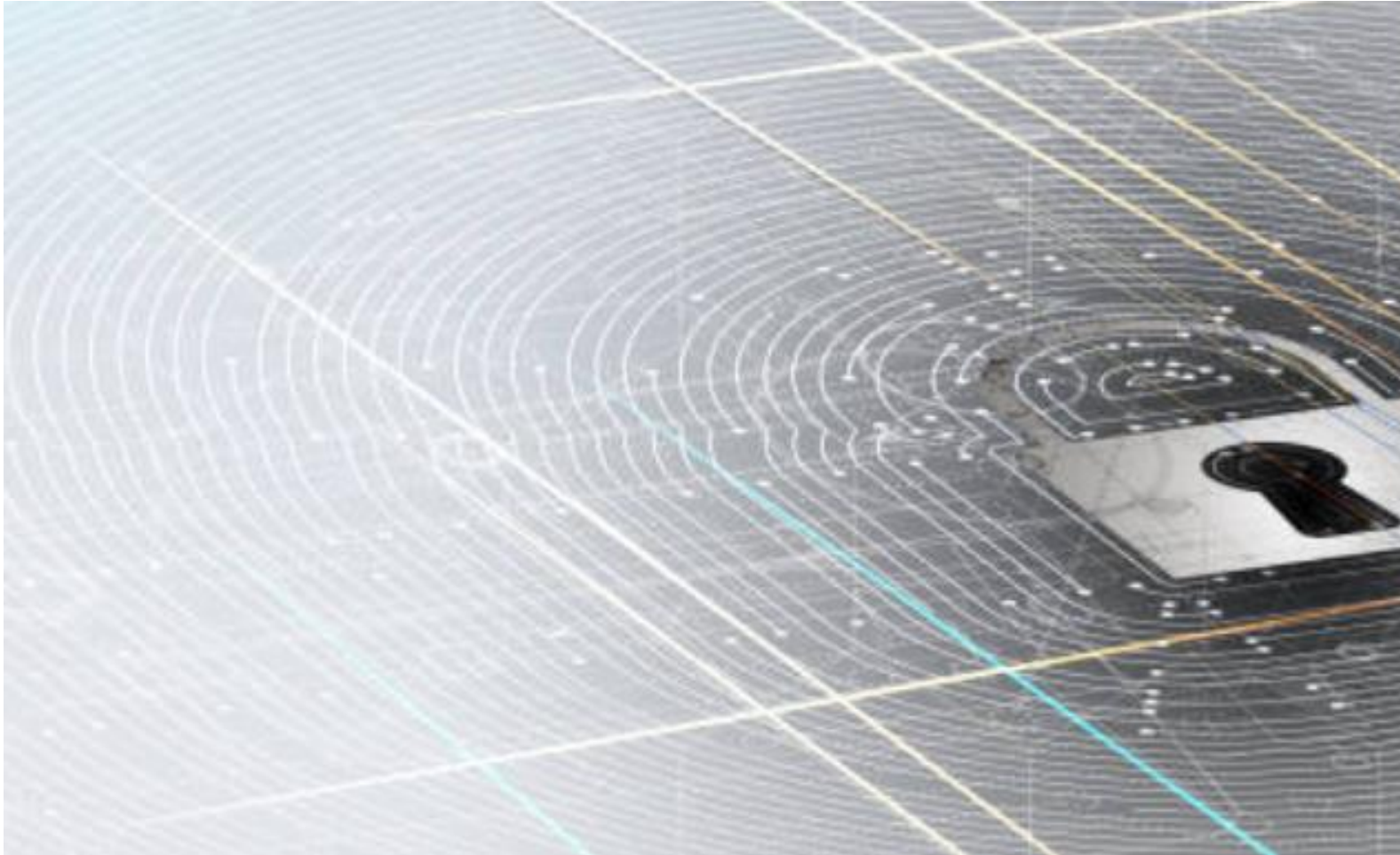
Logos: BlueBerry, OHDSI, BIOMERIS, KNL, BLISS BLUMIS, Cancer.

Authors: Peter Prinsen, Paolo Lasalvia, Roberto Lilini, Vittoria Ramella, Anna Aloni, Joanna Szabandera, Espen Enerly, Masako van Swieten, Siri Larømmingen, Julien Boffard, Audrey Ponce, Thomas Gaudin, Claire Clermont-Arbaud, Aïme Sans, Jean-Yves Blay, Arnaud Malafaire, Danielle Newby, Gips Geleynse, Annalisa Trama.

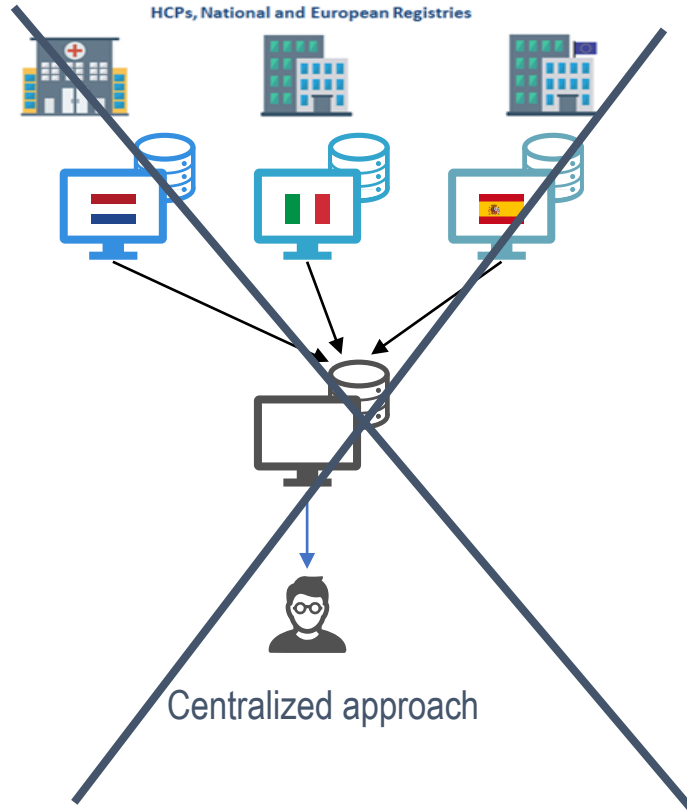
SARCOMAS



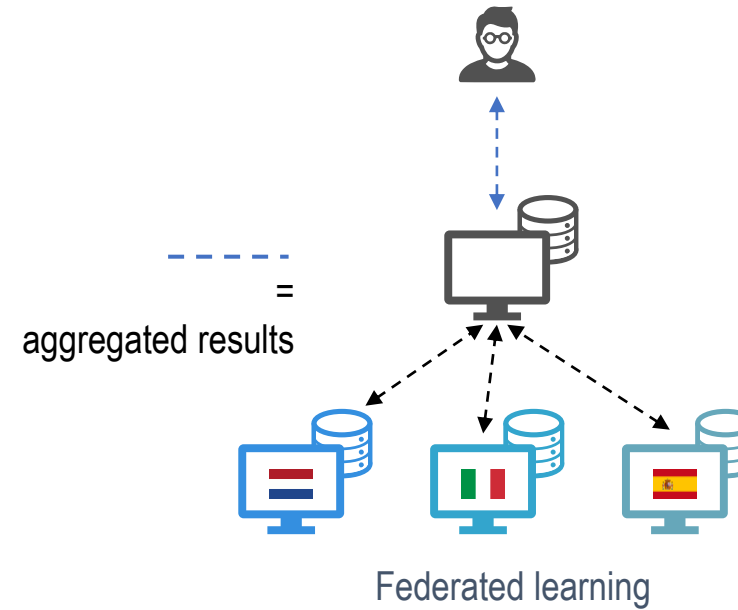
Privacy enhancing technologies



Federated Learning



Performing an analysis across multiple decentralized data sources, without exchanging their data.



Why?



**European
Reference
Network**
for rare or low prevalence
complex diseases

- to help describe the natural history;
- to evaluate factors that influence prognosis (e.g. mortality, survival, progression free survival and treatment response);
- to assess treatments effectiveness (systemic, radiotherapy, surgery, target therapy, immunotherapy and possible combinations);
- to measure indicators of quality of care (diagnostic and staging procedures, treatment strategies, follow-up etc.).

The registry aims to collect information on the storage of biological samples and imaging at the participating centres

Core dataset + ad hoc add on variables study specific



The European Medicines Agency (EMA) and the European Medicines Regulatory Network established a coordination centre to provide **timely and reliable evidence on the use, safety and effectiveness of medicines for human use, including vaccines, from real world healthcare databases across the European Union (EU).**

This capability is called the **Data Analysis and Real World Interrogation Network (DARWIN EU®).**

DARWIN EU delivers **real-world evidence** from across Europe on diseases, populations and the uses and performance of medicines.

This enables EMA and national competent authorities in the the European medicines regulatory network to use these data whenever needed throughout the lifecycle of a medicinal product.

DARWIN EU supports regulatory decision-making by:

- establishing and expanding a **catalogue of observational data sources** for use in medicines regulation;
- providing a source of high-quality, validated real world data on the uses, safety and efficacy of medicines;
- addressing specific questions by carrying out high-quality, **non-interventional studies**, including developing scientific protocols, interrogating relevant data sources and interpreting and reporting study results.

The range of approved **healthcare databases** enabling distributed data access via DARWIN EU will evolve and expand over time.

Registry based studies

1. To provide comparator groups of patients for a single arm trial where RCT is not feasible or unethical
2. To support registry-based RCT for patient recruitment
3. To supplement the evidence generated in the pre-authorisation phase
 - (information on standards of care for the disease, determinants of disease outcomes in clinical practice, validity of a surrogate endpoint used in the evaluation)
4. To contextualise the results of uncontrolled trials
5. To provide data sources or infrastructure for post-authorisation evidence generation

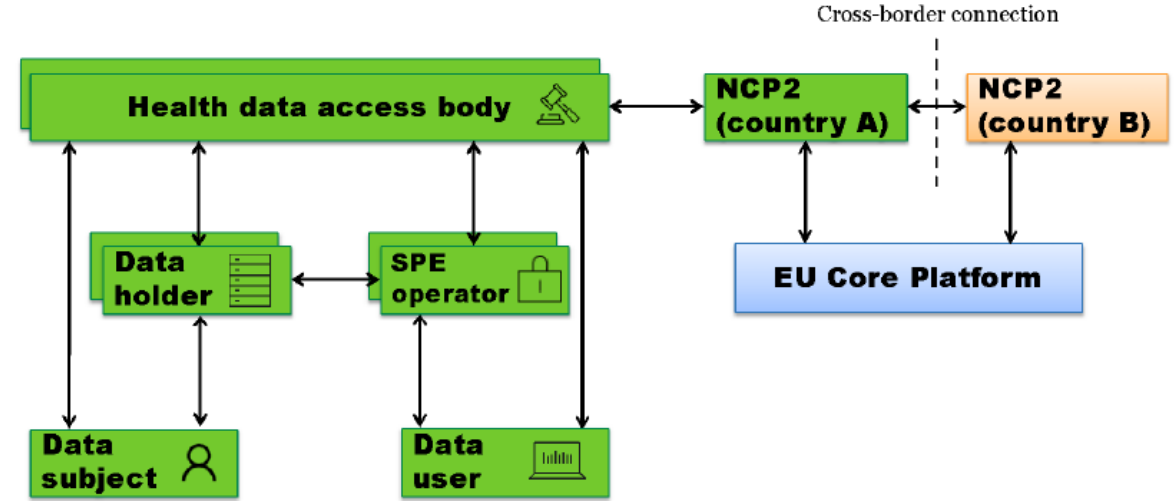


Strasbourg, 3.5.2022
COM(2022) 197 final
2022/0140 (COD)

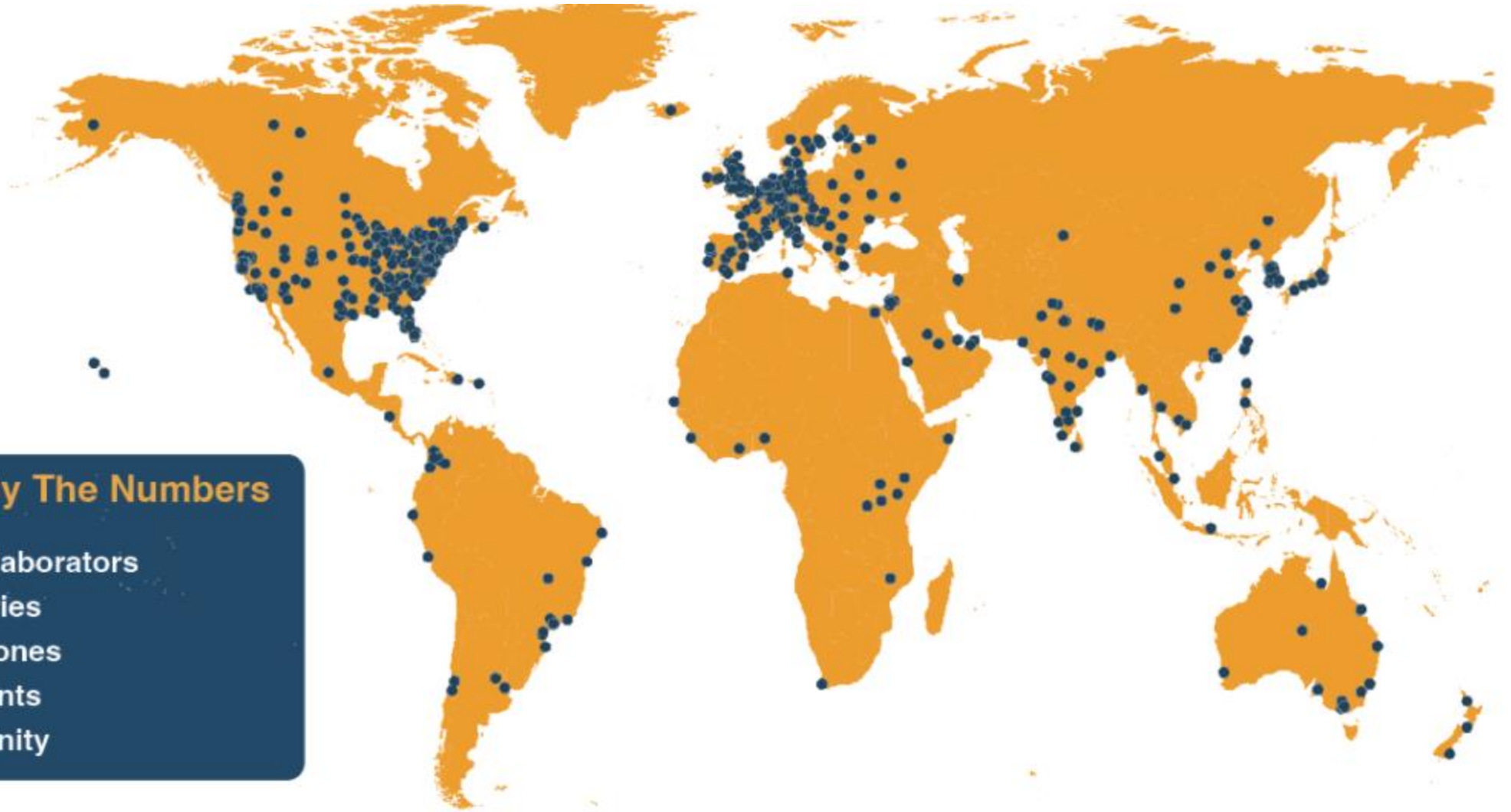
Proposal for a
REGULATION OF THE EUROPEAN PARLIAMENT AND OF THE COUNCIL
on the European Health Data Space

(Text with EEA relevance)

{SEC(2022) 196 final} - {SWD(2022) 130 final} - {SWD(2022) 131 final} -
{SWD(2022) 132 final}



HealthData@EU simplified architecture



OHDSI By The Numbers

- 3,266 collaborators
- 80 countries
- 21 time zones
- 6 continents
- 1 community



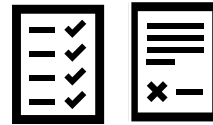
The challenges



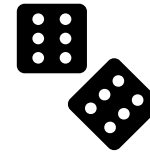
Limited interoperability



Different data quality



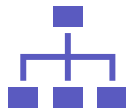
Different data standard



Lack of structured data

A	B	C	D	E	F	G	H
À	BE	CE	DE	E	EF	GE	HA
à	bè	cè	dè	é	ef	gè	há
[a:]	[be:]	[ke:]	[de:]	[e:]	[ef]	[ge:]	[ha:]
I	K	L	M	N	O	P	Q
I	KA	EL	EM	EN	O	PE	QV
I	ká	el	em	en	ó	pé	qù
[i:]	[ka:]	[el]	[em]	[en]	[o:]	[pe:]	[k'u:]
R	S	T	V	X	Y	Z	
ER	ES	TE	V	EX	I GRAECA	ZETA	
er	es	tè	ù	ex	I Graeca	zèta	
[er]	[es]	[te:]	[ù:]	[eks]	[i:'grajka]	[zeta]	

Lack of multilingual NLP model



Different governance



Different legal framework



Lack of trust



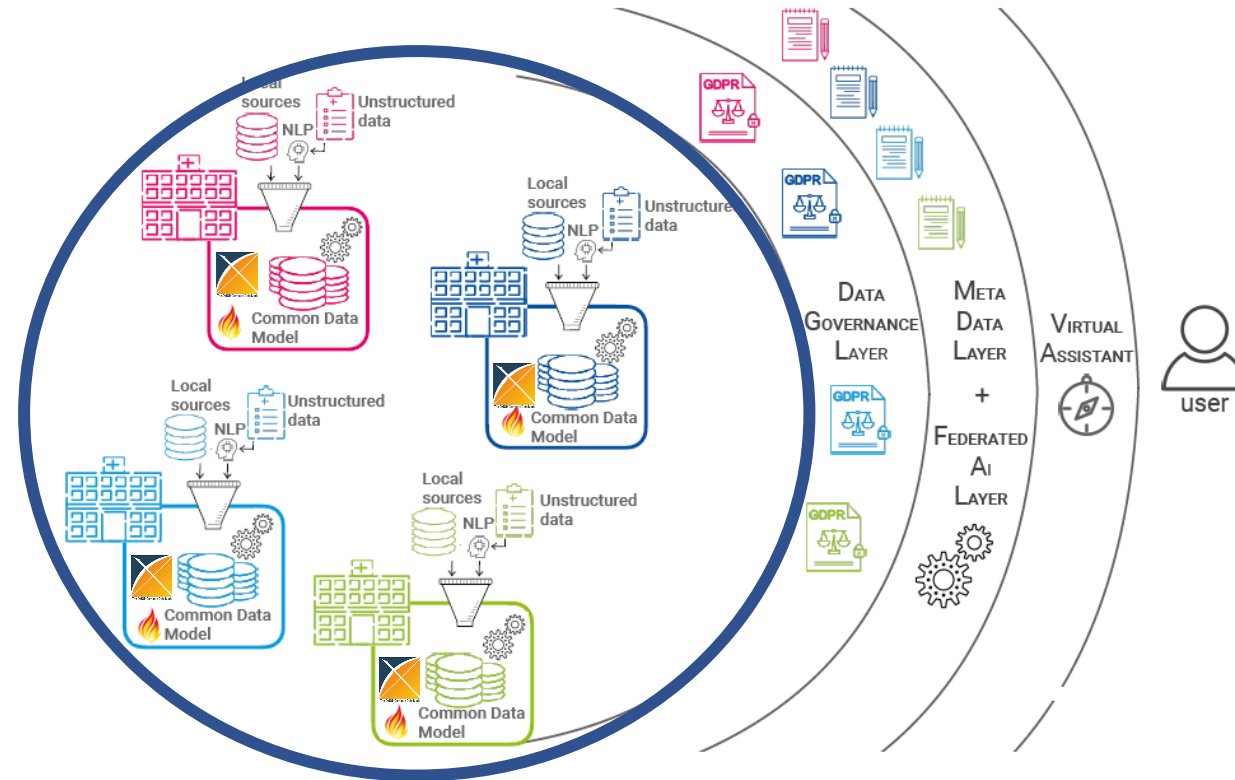
Lack of incentives for data sharing



Human and financial resources for sustainability

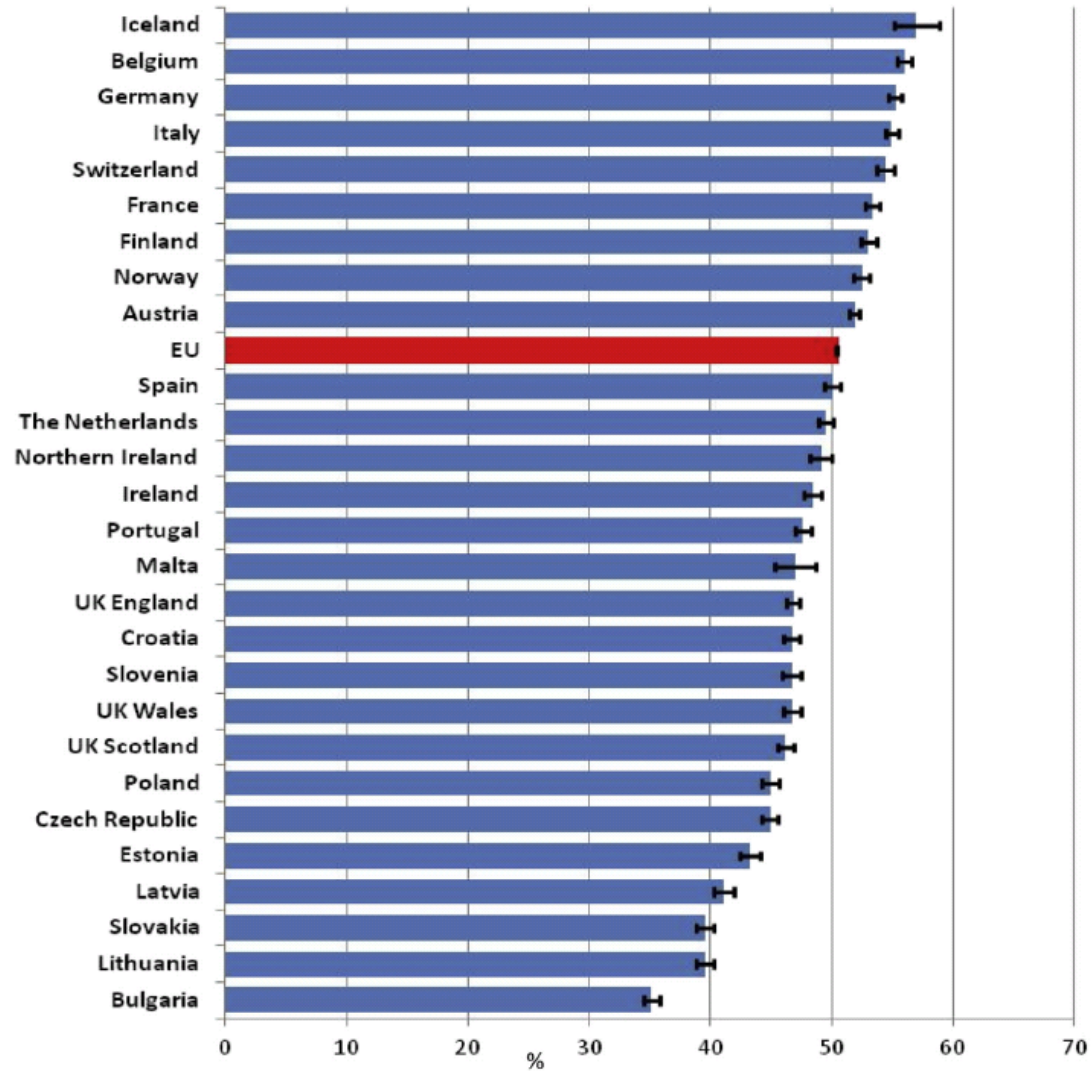
The federated data ecosystem

*a platform that combines data from different data providers and **creates value** through the usage of the data*





Rare cancers survival across european countries



Thank you

