ERKNet
The European Rare Kidney Disease
Reference Network

ERKReg
The European Rare Kidney Disease Registry

Onboarding to EMA catalogue for RWD sources and studies: A user experience

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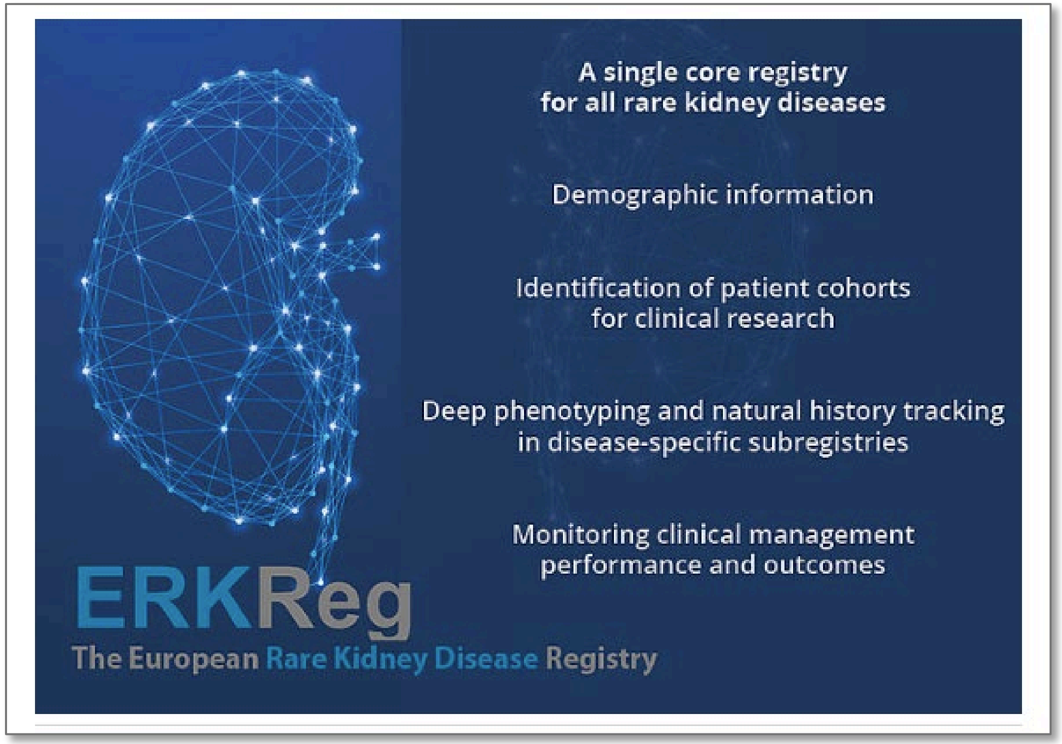


A single core registry
for all rare kidney diseases

Demographic information

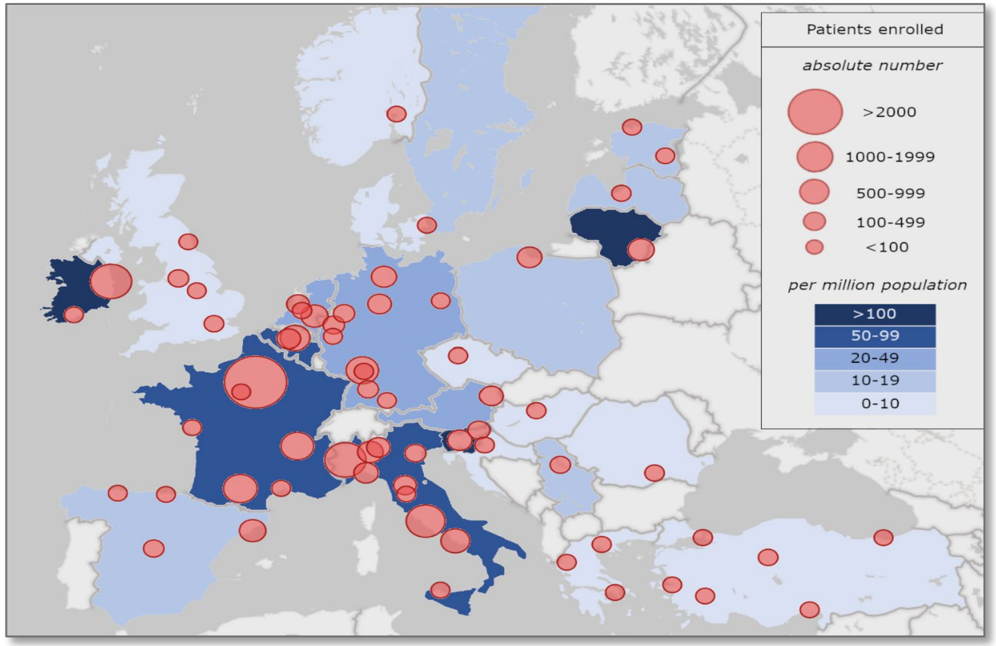
Identification of patient cohorts
for clinical research

Deep phenotyping and natural history tracking
in disease-specific subregistries

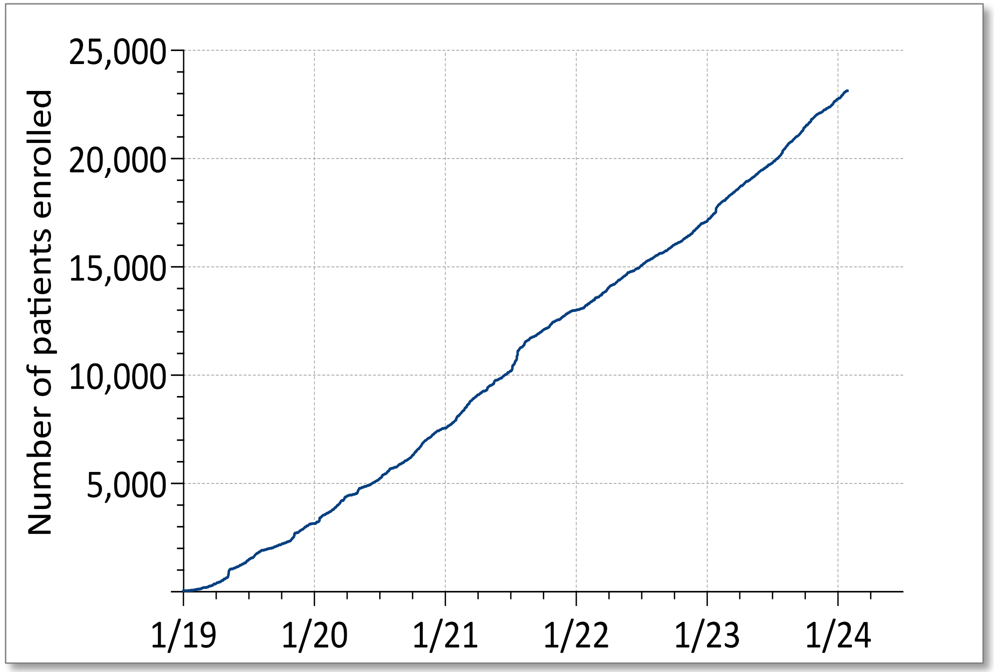
Monitoring clinical management
performance and outcomes

ERKReg

The European Rare Kidney Disease Registry



- Centralized online registry
- 23,000 patients enrolled since 1/2019
  at 80 specialized units in 24 countries
- 7 disease-specific sub-registry extensions
- No. of data items collected:
  Core registry: 231 basic, 215 follow-up
  Subregistries: 72 basic, 278 follow-up



ERKReg Onboarding to EMA RWD Catalogue

Oct. 2022

Preliminary phase:

- First contact with IQVIA
- Presentation of RWD catalogue initiative

Nov. 2022

Population of Data Capture Sheet:

- Sharing of metadata dictionary
- Pre-population of Data Capture Sheet
- Pre-populated ~90% of elements
- Completed of Data Capture Sheet

Dec. 2022

Validation:

- 1st metadata validation
- Feedback from IQVIA
- 2nd metadata validation

Feb. 2023

Follow-up:

- Clarification of some elements required
- Metadata refresh

Our collaboration - Summary

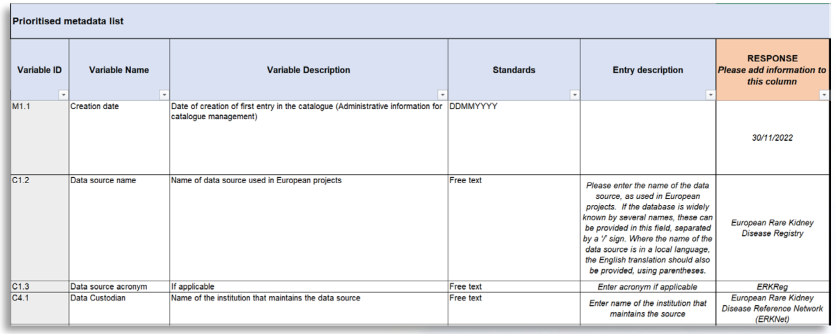
Main requirement:

- Populate the **Data Capture** Spreadsheet, covering elements as defined in EMA **list of metadata for Real World Data catalogues**

Process:

- **Most elements pre-filled** by IQVIA, using ERKReg data dictionary and other available information
  → showcases benefits of comprehensive registry documentation
- **Validated pre-populated sheet**
- **Populated remaining elements**, some requiring additional data processing:
  - Up-to-date contact information
  - Details on family linkage
  - Metrics (e.g. records by country/age, median observation time, patient activity, etc.)
- Defined initial **methodology** for metadata updates and their **frequency** (we agreed on 6-monthly)

Data Capture Sheet (excel format)



Prioritised metadata list

Variable ID	Variable Name	Variable Description	Standards	Entry description	RESPONSE *Please add information to this column*
M1.1	Creation date	Date of creation of first entry in the catalogue (Administrative information for catalogue management)	DDMMYYYY		*30/11/2022*
C1.2	Data source name	Name of data source used in European projects	Free text	*Please enter the name of the data source, as used in European projects. If the database is widely known by several names, these can be provided in this field, separated by a '/' sign. Where the name of the data source is in a local language, the English translation should also be provided, using parentheses.*	*European Rare Kidney Disease Registry*
C1.3	Data source acronym	If applicable	Free text	*Enter acronym if applicable*	*ERKReg*
C4.1	Data Custodian	Name of the institution that maintains the data source	Free text	*Enter name of the institution that maintains the source*	*European Rare Kidney Disease Reference Network (ERKNet)*

Our collaboration - Takeaways

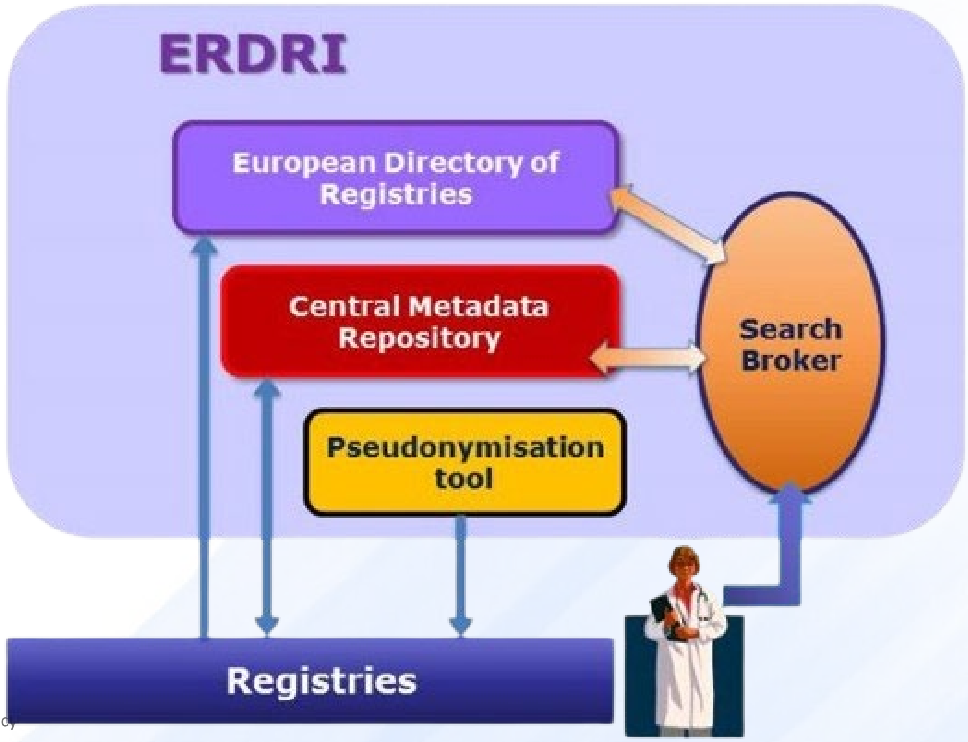
- **Participation** in the EMA RWD catalogue inititative involved streamlined, **easy-to-follow steps**
- After initial kick-off meeting, successive exchanges and validations performed through e-mails
- **Very positive experience** in working with IQVIA colleagues
- ERKNet looks forward to continuing to participate in the EMA RWD catalogues and supporting the needs of all involved stakeholders.

ERDRI

European **R**are **D**isease **R**egistry **I**nfrastructure

European
Commission
Joint Research Centre

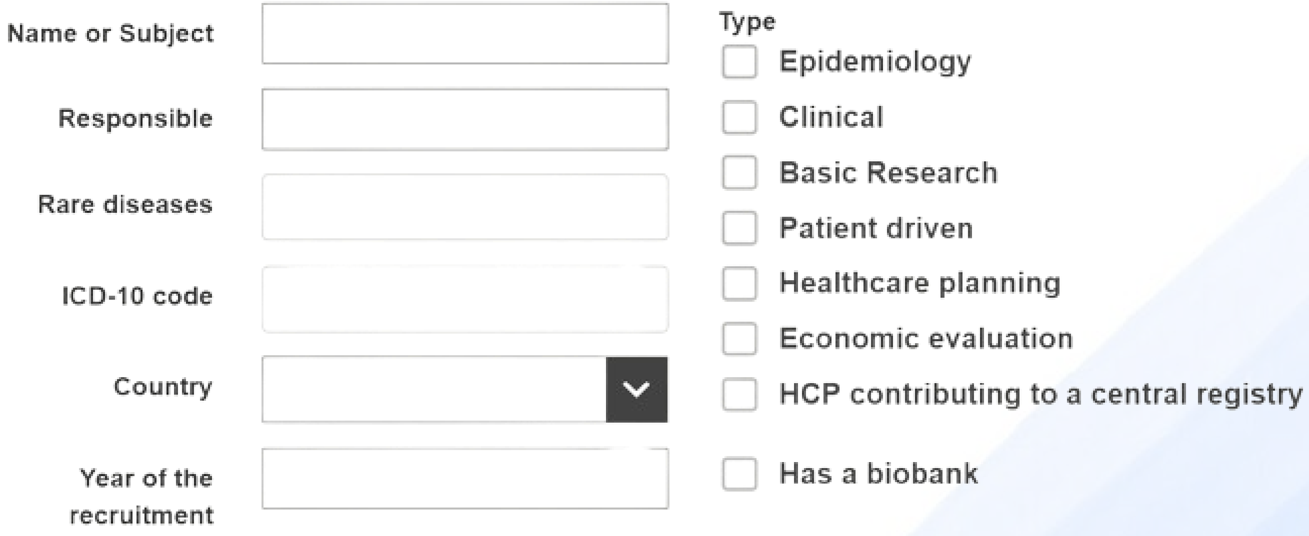
- **ERDRI** is part of the **EU RD Platform**, offered by the **Joint Research Centre** of the EC.
- **ERDRI** assists in making metadata/data from rare disease registries **searchable** and **findable**.
- Provides **multiple** components and **tools**:
  - European **D**irectory **of** **R**egistries (ERDRI.dor)
  - Central **M**eta**da**ta **R**epository (ERDRI.mdr)
  - **Se**arch **bro**ker (ERDRI.sebro)
  - Pseudonymisation Tool (ERDRI.spider)
- ERDRI currently includes **129 RD registries**



ERDRI.dor

Directory of Registries

- **ERDRI.dor** provides an overview of the main characteristics and descriptions of participating rare disease registries.
- Data input and curation is managed by **registry holders**.
- Allows **filtered search** to find registries of potential relevance to specific research questions.



Name or Subject

Responsible

Rare diseases

ICD-10 code

Country

Year of the recruitment

Type

- ☐ Epidemiology
- ☐ Clinical
- ☐ Basic Research
- ☐ Patient driven
- ☐ Healthcare planning
- ☐ Economic evaluation
- ☐ HCP contributing to a central registry

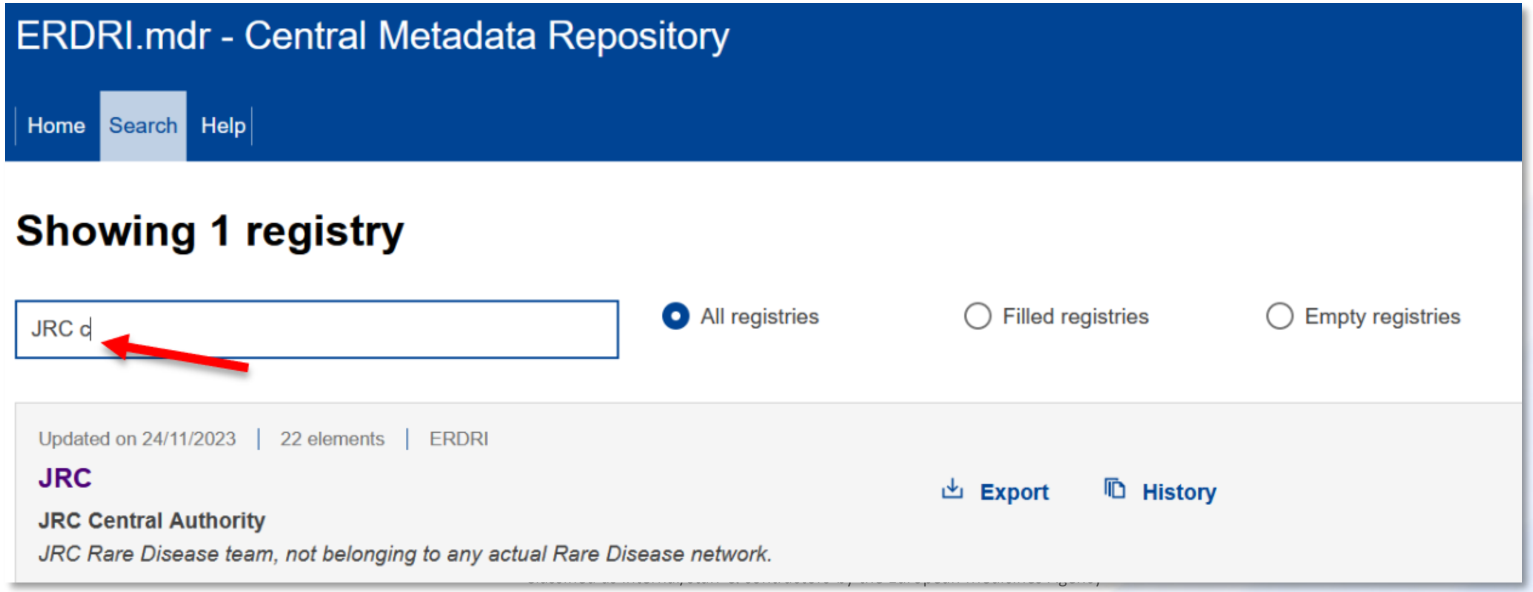
☐ Has a biobank

ERDRI.mdr

Metadata Repository

European Commission
Joint Research Centre

- **ERDRI.mdr** serves as metadata storage and **facilitates semantic interoperability** between RD registries.
- Includes data element **designations** and their **definitions**.
- **Metadata** items can be uploaded **automatically** or inserted **manually**.



ERDRI.mdr - Central Metadata Repository

Showing 1 registry

JRC c

● All registries ○ Filled registries ○ Empty registries

Updated on 24/11/2023 | 22 elements | ERDRI

JRC

JRC Central Authority

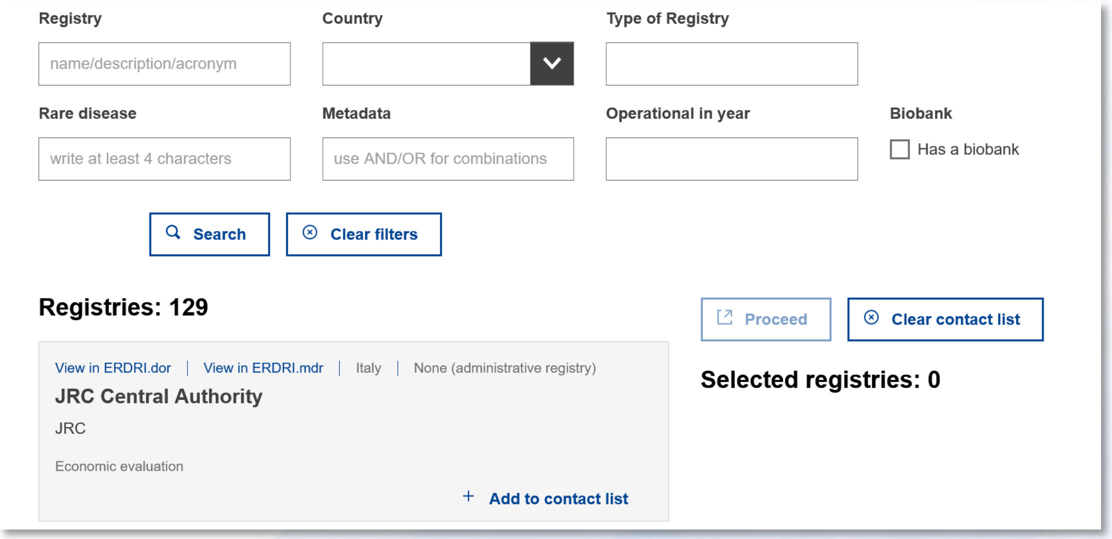
JRC Rare Disease team, not belonging to any actual Rare Disease network.

ERDRI.sebro

Search Broker

European Commission
Joint Research Centre

- **ERDRI.sebro** allows authenticated users to retrieve metadata of their interest from ERDRI-participating registries
- Its search function facilitates the **identification of registries** containing **relevant metadata**
- After identification, a contact form through the EU RD Platform allows the user to **directly communicate** to the registries.



Registries: 129

Italy | None (administrative registry)

JRC Central Authority

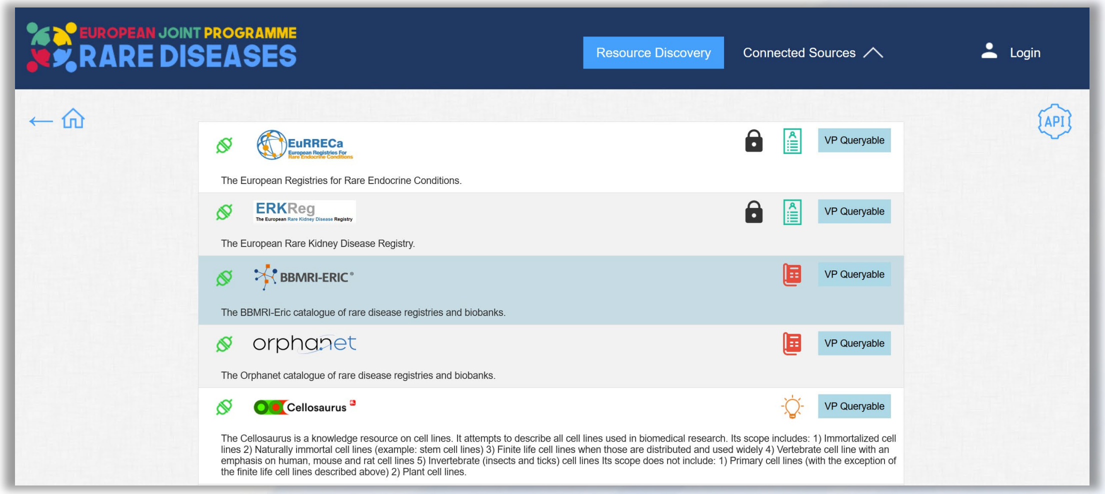
JRC

Economic evaluation

Selected registries: 0

EJP RD - Virtual Platform

- The **Virtual Platform** (VP) is a growing **federated ecosystem** of Findable, Accessible, Interoperable and Reusable (FAIR) resources, aimed at serving the RD research community.
- It includes catalogues of resources, **registries**, biobanks, knowledge bases and tools **compliant with agreed standards**.
- The **VP Portal** allows users to query all VP network resources to find those of interest to their research.



The European Registries for Rare Endocrine Conditions.

The European Rare Kidney Disease Registry.

The BBMRI-Eric catalogue of rare disease registries and biobanks.

The Orphanet catalogue of rare disease registries and biobanks.

The Cellosaurus is a knowledge resource on cell lines. It attempts to describe all cell lines used in biomedical research. Its scope includes: 1) Immortalized cell lines 2) Naturally immortal cell lines (example: stem cell lines) 3) Finite life cell lines when those are distributed and used widely 4) Vertebrate cell line with an emphasis on human, mouse and rat cell lines 5) Invertebrate (insects and ticks) cell lines Its scope does not include: 1) Primary cell lines (with the exception of the finite life cell lines described above) 2) Plant cell lines.

EJP RD - Virtual Platform

EUROPEAN JOINT PROGRAMME RARE DISEASES

- The **VP network** defines **3 levels** of connection:
  - **Level 1:** Resource discovery → **Using resource-provided metadata**
  - **Level 2:** Content discovery →Aggregated data
  - **Level 3:** Data Analysis → Record-level data
- Resources must present their **metadata** in a **harmonised, machine-readable** schema based on the ‘**Data Catalogue Vocabulary**’ (DCAT)
- **Variations** of the metadata model are provided for **each resource type** (e.g. registries, biobanks, catalogues)
- Metadata is then provided **directly by resources** using **FAIR Data Points** (FDPs)