EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Fit for purpose and Data Quality Framework

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The views expressed are personal views and not necessarily the views of CBG-MEB or EMA.

Overview

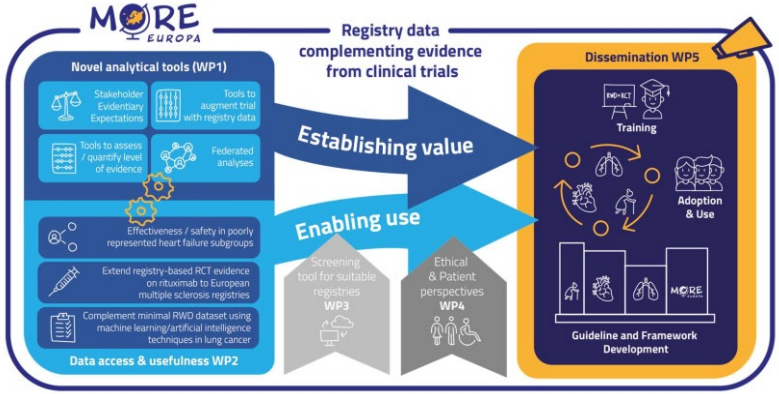
- Fit for purpose: Use of registries in drug development and regulatory context.
- Data Quality Framework highlights.
- A few take aways.

Areas of decision-making for which registries can be useful

Use case objective	Support the planning & validity of applicant studies	Understand clinical context	Investigate associations and impact
Use case category	Design and feasibility of planned studies	Disease epidemiology	Effectiveness and safety studies
	Representativeness and validity of completed studies	Clinical management	Impact of regulatory actions
		Drug utilisation	

Throughout the entire drug development life cycle

Richness of use of registries *illustrated*.



Early development

- Biomarker & clinical outcome associations to support probability of success & full development decisions.

Full development & decision making

- Contextualisation.
- Direct augmentation of clinical trial data (hybrid).
- Modeling efficacy / effectiveness in broad population based on clinical trial data.

Beyond Full development

- Target trial emulation for observational studies.
- Registry based clinical trials.
- Modelling for HTA support, including external controls.

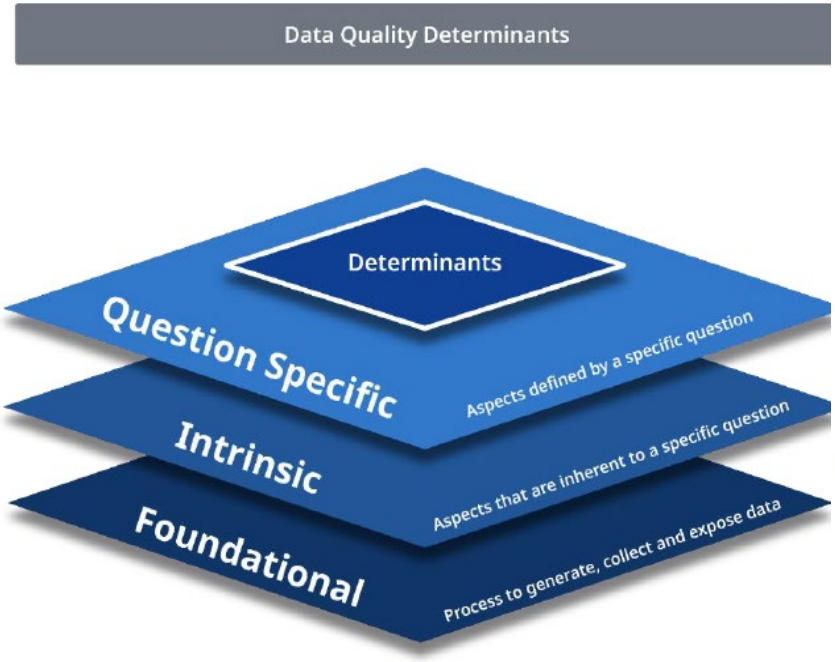
Using mutiple registries
Federated inference

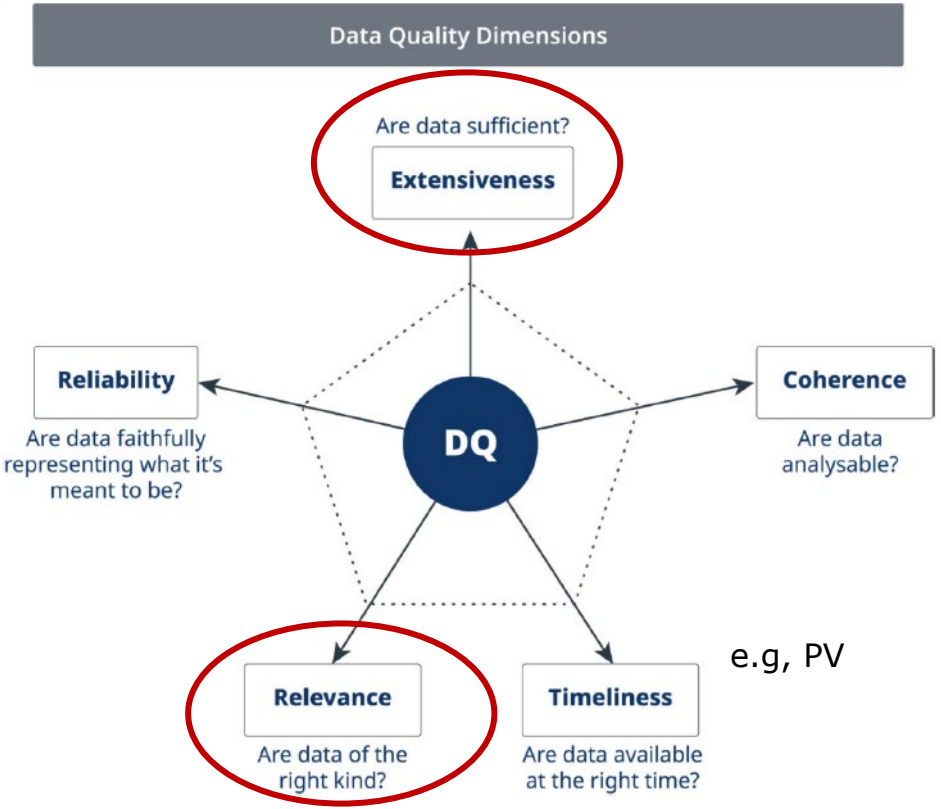
Richness of use of registries *illustrated*.

From a fit for purpose perspective of Quality,

these uses may impose (very) different quality requirements.

Data Quality Framework highlights.





e.g, PV

Registries serve mutliple research questions, many may not be known at the time of design.

	Is data correct? **Reliability**	**Is data enough?** **Extensiveness**	**Is data homogenous?** **Coherence**	**Is data timely?** **Timeliness**	**Is this the right data?** **Relevance**
Systems and processes	Determine reliability	Determine extensiveness	Enable coherence	Determines timeliness	
Metrics	Can assess reliability	Can measure extensiveness	Can measure and improve coherence		
Suitability to a research question	Defines "acceptable" reliability	Defines if data is sufficient	Defines if the level of coherence is adequate	Defines acceptable timeliness	Defines if the content of the data is what is needed

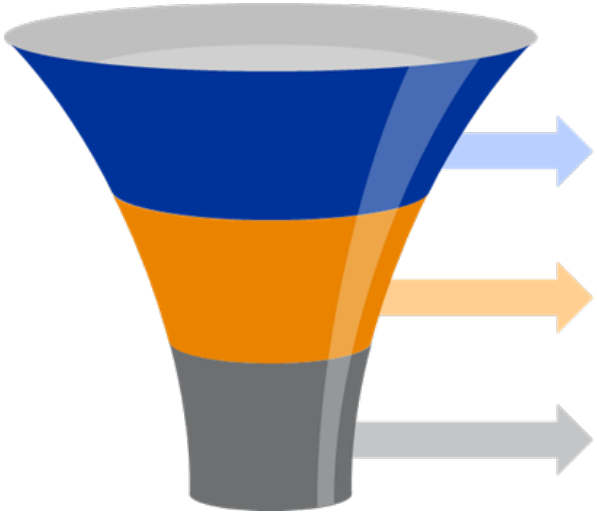
Framework

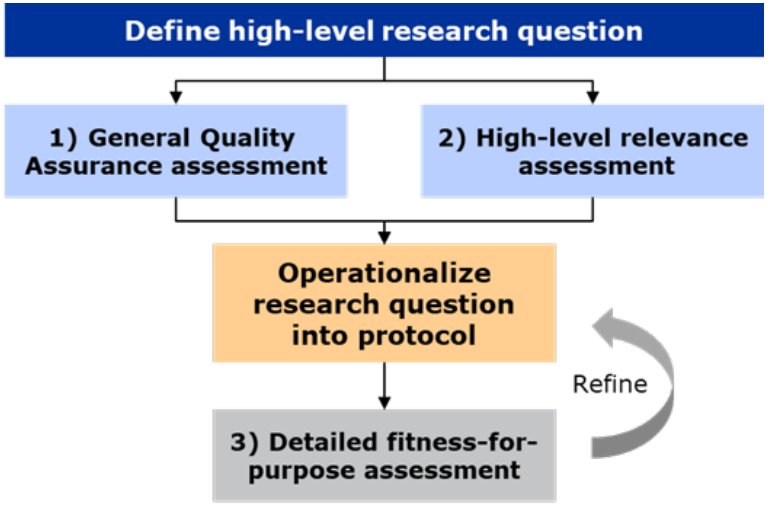
Key DQ features for Quality Assurance, Quality Reporting and DQ assessment.

	Reliability	Extensiveness	Coherence	Timeliness
Independent dataset checks				
Plausibility checks				
Conformance checks				
Comparison to other datasets				
Checks on dataset descriptors				

Test the completeness of test sets in use, identify gaps, redundancy, or complementary metrics.

Quality in relation to a specific research question.





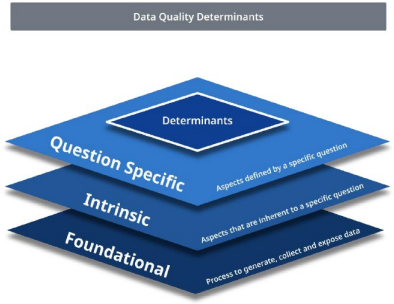
Quality in relation to a specific research question.

- Difficult to pre-specify thresholds or minimum criteria for data acceptance:
  - Depends on the type of study, and on disease-dependent and analysis-dependent factors
  - Other considerations, such as lack of other RWD sources in a therapeutic area or disease frequency.

- Conclusion on relevance requires an in-depth, systematic assessment of a RWD source against design elements.

- Metrics and acceptability may change with subset selection from the full source.
  - Extensiviness (e.g. sample size or granularity variables) and coherence may typically be affected.

A few take aways



- Given the breadth of secondary research questions that *registries* can serve: Aim high for Foundational dimension of quality.
- Qualification of registries to reflect these different levels of determinants.
- Dynamic: Use the systematic fitness for purpose assessment for new research questions to improve quality overall (efficiently).