European Federation of Pharmaceutical Industries and Associations

Outcomes of EFPIA survey on registries and qualification performed in February 2024






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On behalf of EFPIA

Objectives

EFPIA acknowledges there are key learnings and challenges to share with EMA based on industry's experience on the use of registries (qualified and non-qualified) for use in drug development

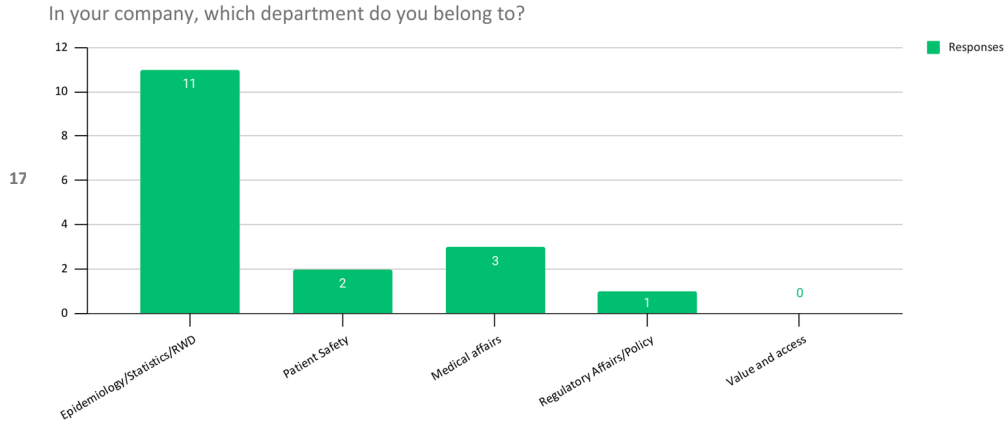
In this presentation, we will:

- Share results from the EFPIA survey that highlight industry experience on qualified and non qualified registries
- Highlight discussion points that may increase clarity and streamline process

Introduction survey

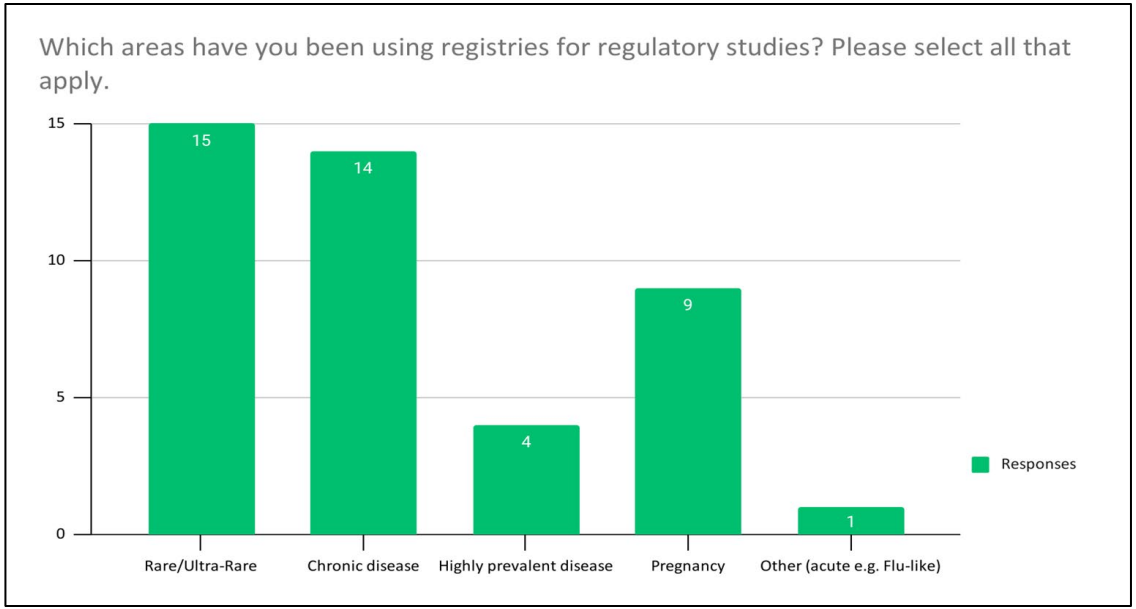
In preparation of this workshop, Efpia performed a survey amongst industry members to obtain insights in industry experience on planning and/or performing registry-based studies

- target audience survey: Industry
- survey closing date: January 26, 2024
- number of responders: 17



17 responders

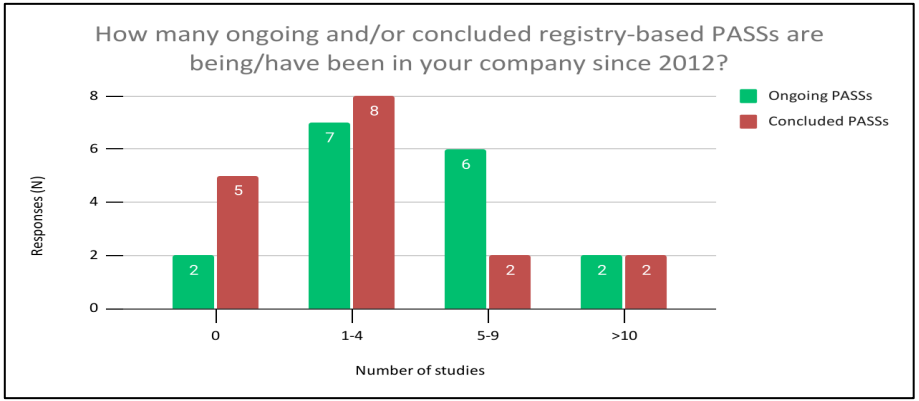
Disease areas where registries are used for regulatory studies



17 responders

Registries are broadly used for several disease areas, mostly in rare/ultra rare diseases, chronic diseases and pregnancy studies

Experience of responders on registry-based PASSs and PAESs since 2012



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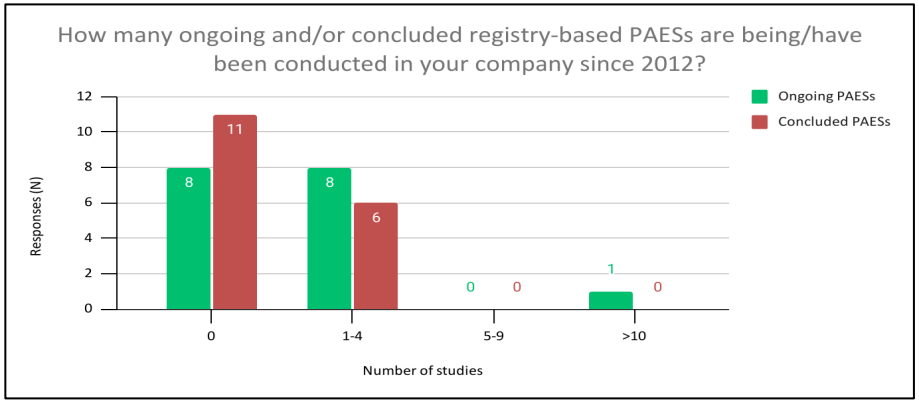
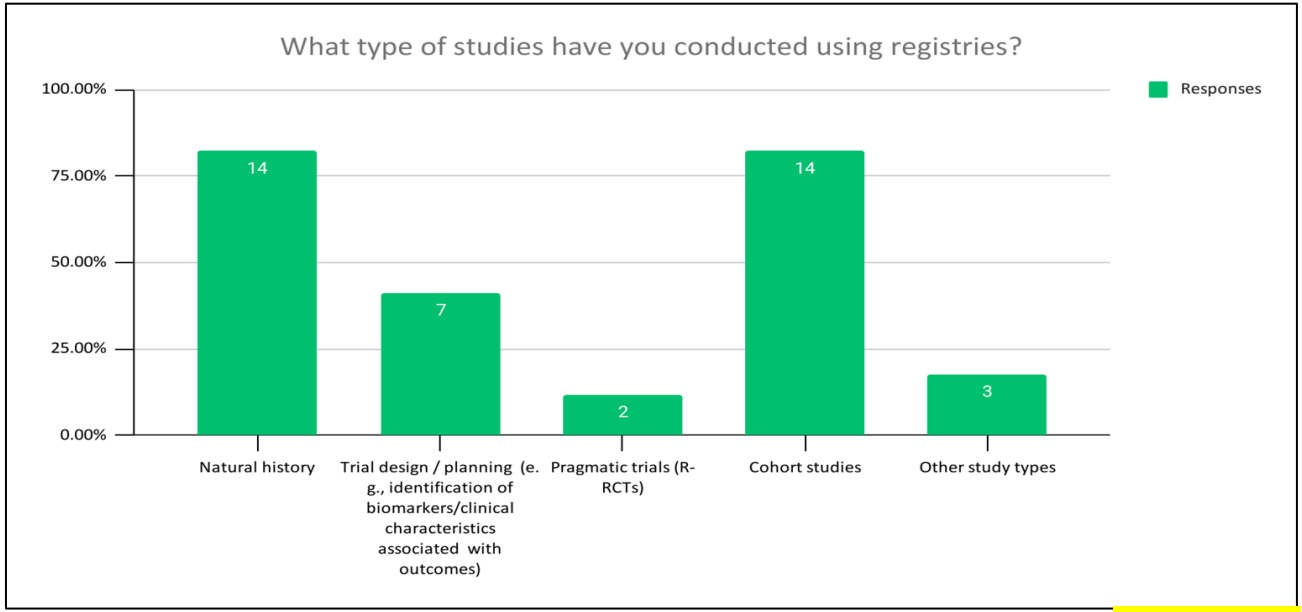


17 responders

Industry has extensive experience with registry studies as PASS and PAES in regulatory submissions since 2012

PASS postauthorisation safety studies
PAES postauthorisation efficacy studies

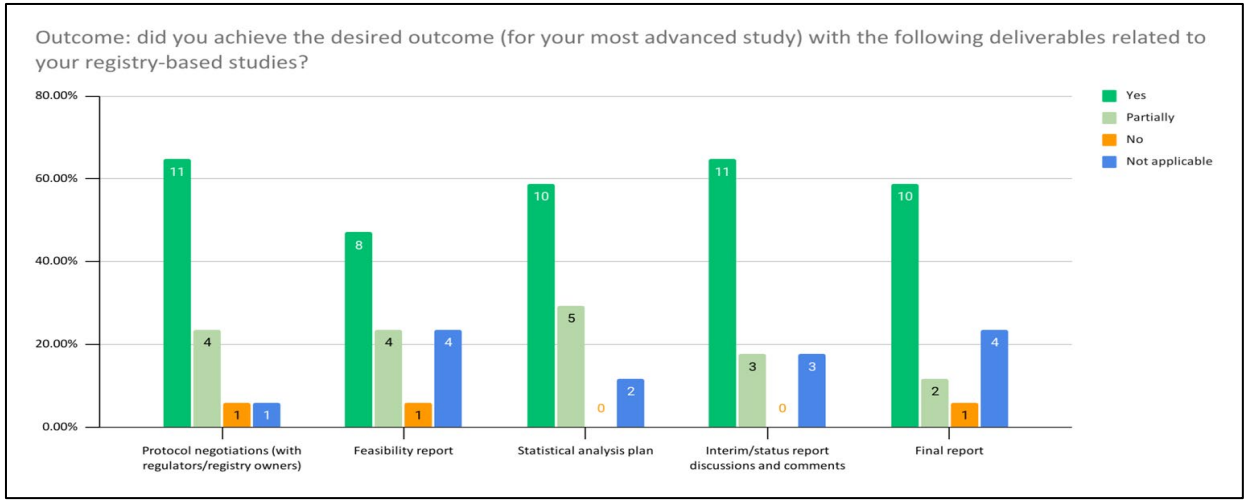
Industry utilized registries for several types of studies



17 responders

Registries are mainly utilized by industry for cohort studies and natural history studies

Industry experience on registry-based studies



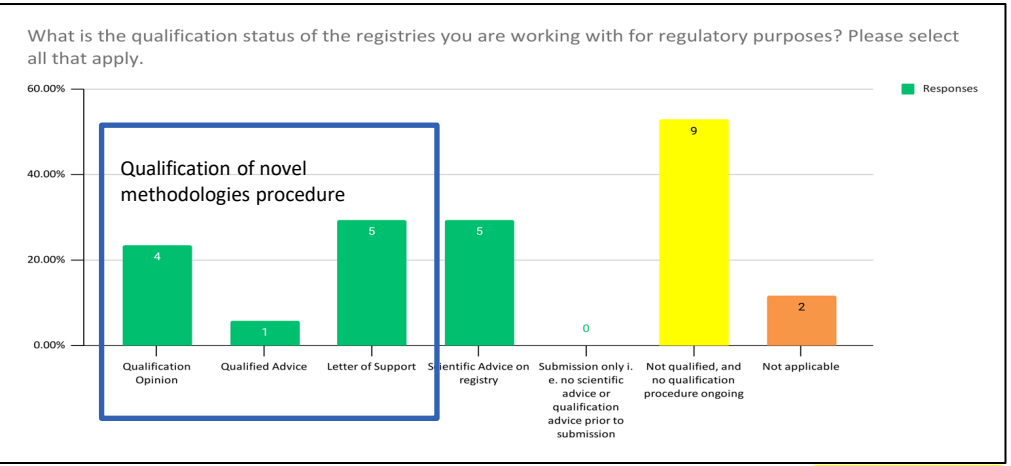
17 responders

Breakdown of the deliverables related to registry-based studies show successful outcomes at various milestones along the study process

Industry observed some challenges related to registry-based studies including:

- Some registries may not achieve the rigor required for regulatory submissions
- Registries may experience slow recruitment
- Some registries may have data quality issues causing complexities for regulatory use of data

Different EU regulatory procedures are used for registries



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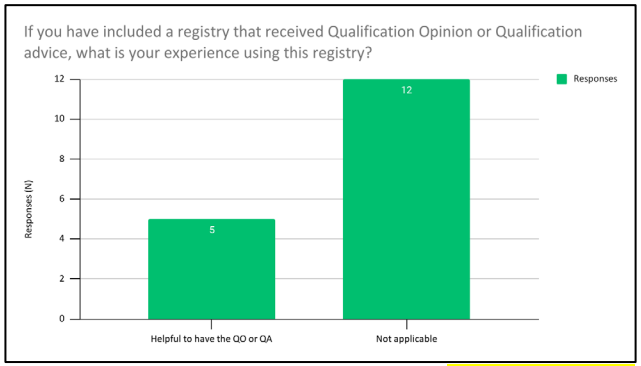

17 responders

Qualified registries: Although the number is relatively limited, they are used for regulatory purposes

Various regulatory engagements for registries:

- Registry specific advice through qualification procedure
- Product specific scientific advice
- Regulatory submissions with no specific scientific advice on registries

In rare diseases, it is expected that the number of data sources/registries are limited. Therefore, registries need to be used by multiple participants

Responding companies who included a registry (5/17) with QO/QA found it helpful.

Summary of EFPIA survey findings on registry-based studies

Registry-based studies are widely used by industry

Advice during qualification of registries is considered helpful

Extensive experience on registry-based studies used as PASSs and PAESs

Different EU regulatory procedures can be followed

Registries are mainly utilized by industry for cohort studies and natural history studies

Number of qualified registries are relatively limited

An approach to further the discussion about registries and qualification

EFPIA recognized that qualification may ensure a certain level of regulatory acceptability and encourage the use of data by many stakeholders. During the workshop, we would like to share our experiences with registries and explore:

- The value of qualification for different purposes and stakeholders
- Qualification as one option for fitness-for-purpose assessments and how the process will be informed by emerging tools such as the data quality frameworks
- Moving towards a future state where quality frameworks and maturity models foster a sustainable “quality by design” approach to registries that may help reduce the need for qualification over time

Opportunities to increase clarity and streamline processes, and plan for the future

Value of qualification

- How does it serve different purposes for different stakeholders?
- Are the potential advantages and value clear to all stakeholders?
- Transparency: Is the information accessible on the EMA website?

Ecosystem: Qualification is one tool within this landscape

- How does qualification interact with other "tools" available such as Data Quality framework; registry-based study guideline, Kahn framework etc?
- Interdependencies: Can the DQ framework be used to facilitate the qualification process?
- Going forward will there still be place for 'non-qualified' registries being used for regulatory decision making?

Lifecycle management of qualified registries

- Can tools such as DQ framework and registry-based study guideline help ensure that standards of qualified registries are sustained over time?
- How to ensure that the registry remain fit for purpose over time?

Procedural aspects

- Guidance to future applicants like the checklist
- Trialogue between regulator-applicant-registry holder

Thank you for the opportunity to share our perspective