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Practical Challenges in Real World Evidence (RWE) Studies

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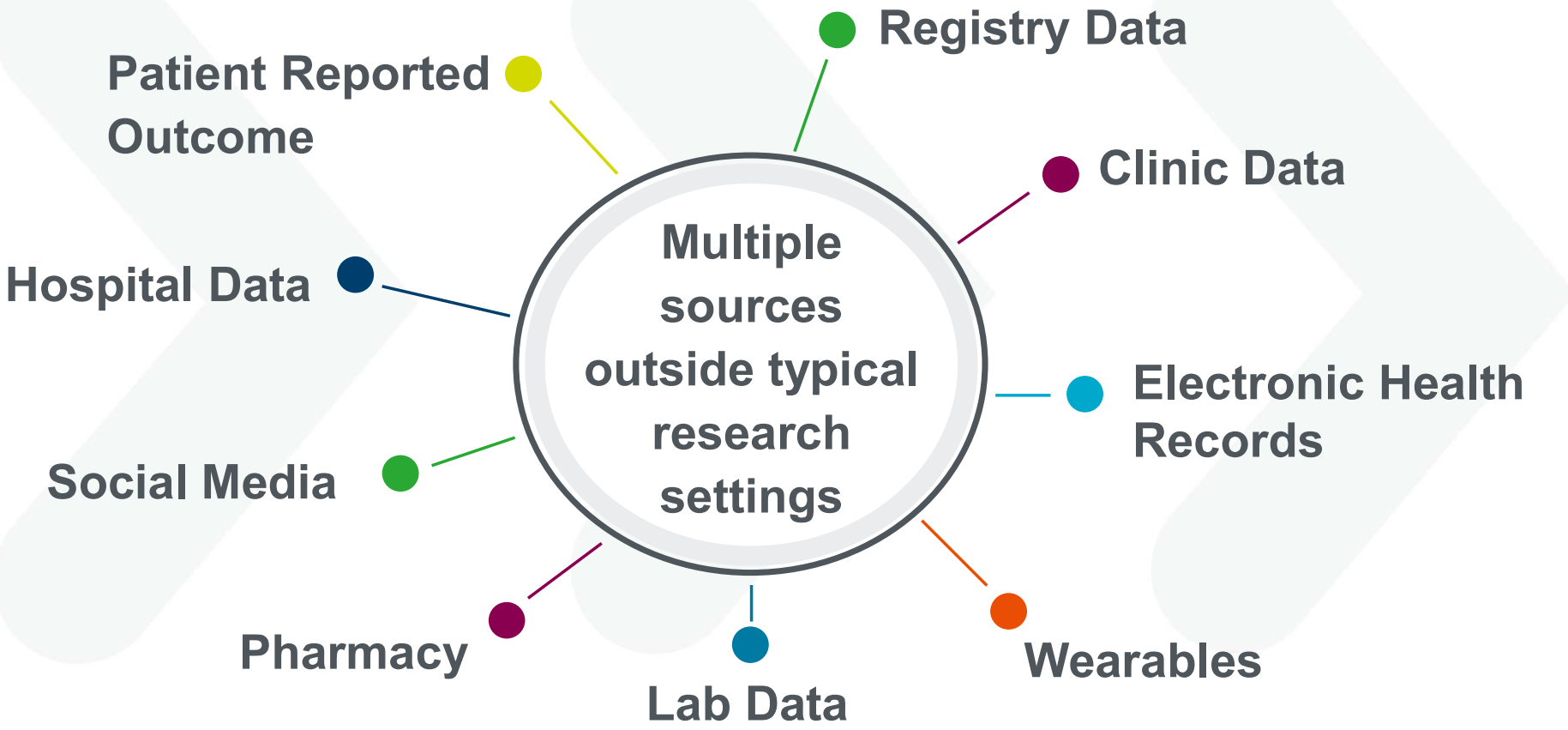
Disclaimer

The contents of this presentation are my own, and do not necessarily reflect the views and/or policies of Parexel.

Real-World Data (RWD)

"The data relating to patient health status and/or the delivery of health care routinely collected from a variety of sources"[1]



1 International Harmonisation of Real-World Evidence Terminology and Convergence of 4 General Principles Regarding Planning and Reporting of Studies Using Real-World Data, with 5 a Focus on Effectiveness of Medicines, 2023, quoted from FDA

Real-World Evidence (RWE)

- *"Clinical evidence regarding the usage and potential benefits or risks of a medical product derived from analysis of RWD"*[1]

- Turn the raw data into useful source for stakeholder
- Transform diverse types of evidence into information on health care
- Use RWD to utilise authorized medicines in routine medical practice
- Generate evidence that supports regulatory decisions on post-marketing safety and effectiveness of medicines

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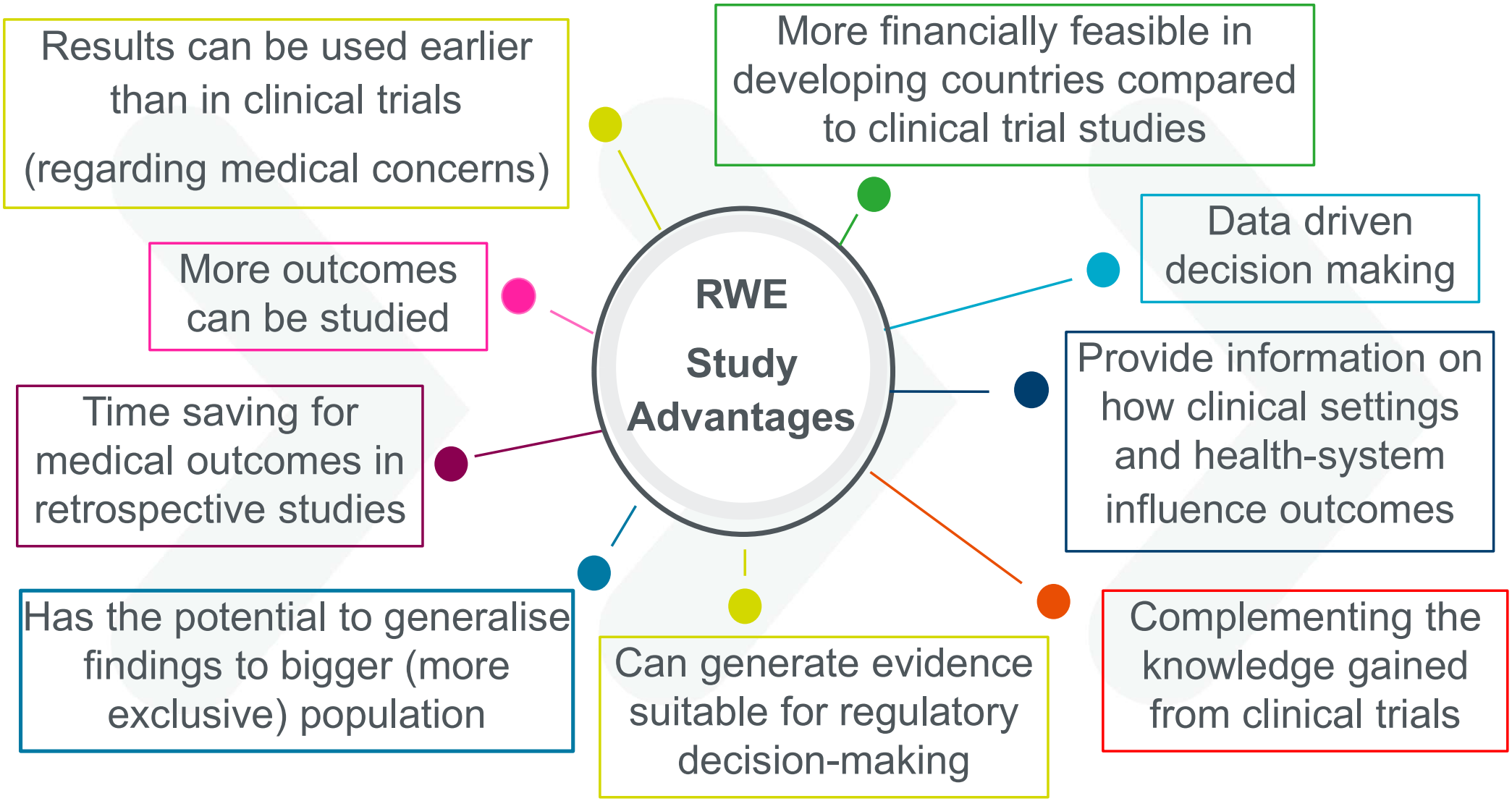


Challenges

The handling of data collection and analysis in real-world Evidence studies present significant challenges, mainly due to fewer regulations in place compared to clinical trial studies.

Many of which can potentially cause inaccuracy and biasness in final analysis.

Here only a handful of challenges that I became aware of in the two recent non-interventional studies I am working on.

RWE Study Challenges

Study Design

Inconsistent Regulations

Data Management

Programming & Analysis

- Scientific question may be lacking ==> check proxies to start with
- Scientific question is known but not collected
  - No guarantee what can be achieved
  - Quality of data not promising
- Retrospective:
  - No control of data availability
  - No easy to track missing data back
- Prospective: Can decide what to collect ==> expensive

RWE Study Challenges

Study Design

Inconsistent Regulations

Data Management

Programming & Analysis

RWE Retrospective → Consent Form required in some countries → Oncology: Person may not be alive anymore

↓

Not easy to ask families (emotional reasons) → Missing possibly important data → 

RWE Study Challenges

Study Design

Inconsistent Regulations

Data Management

Programming & Analysis

- In some countries ICF is not required if data is fully anonymised
  - Limits the type of access, site training and data record
  - Might end up changing endpoint
  - Can damage the research

RWE Study Challenges

Study Design

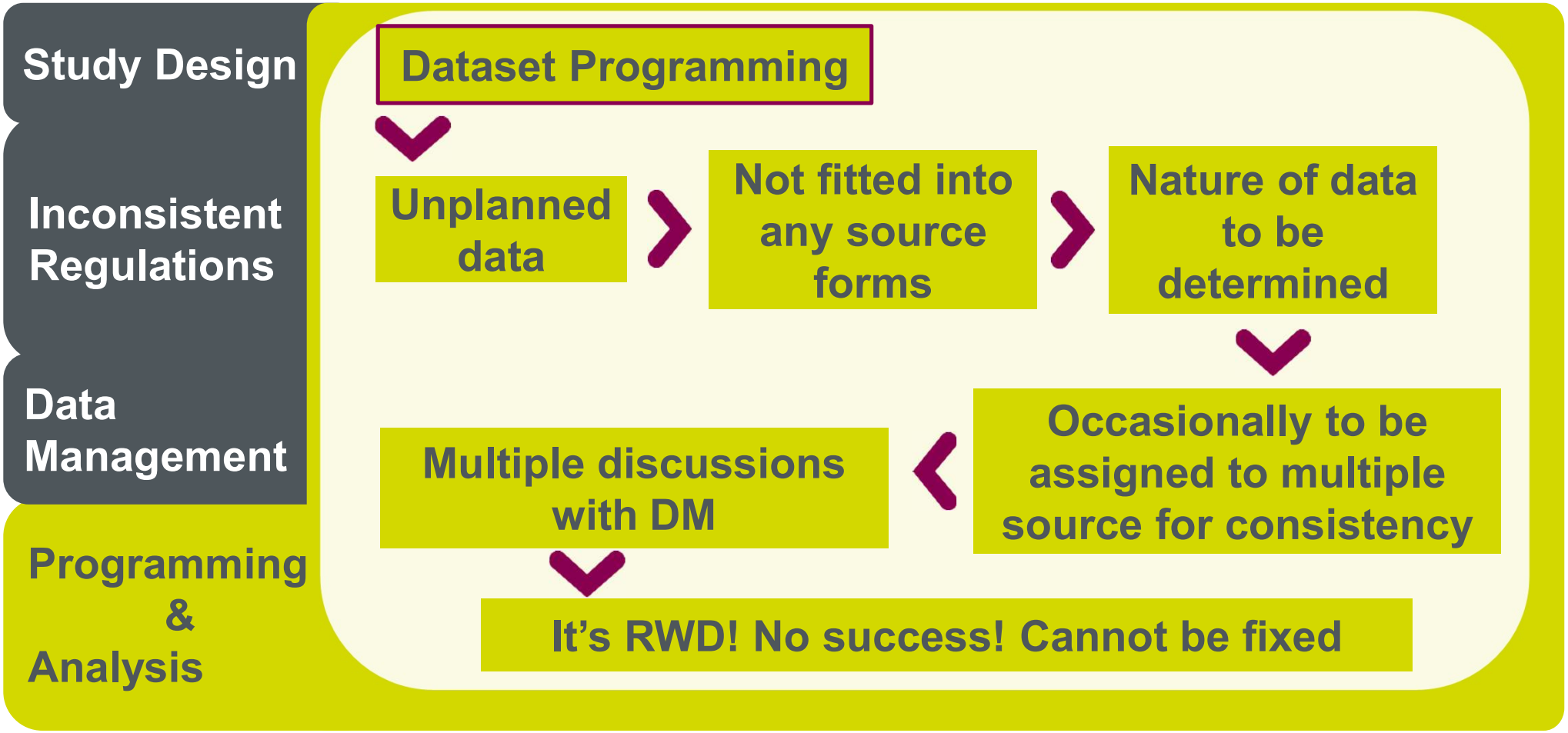
Inconsistent Regulations

Data Management

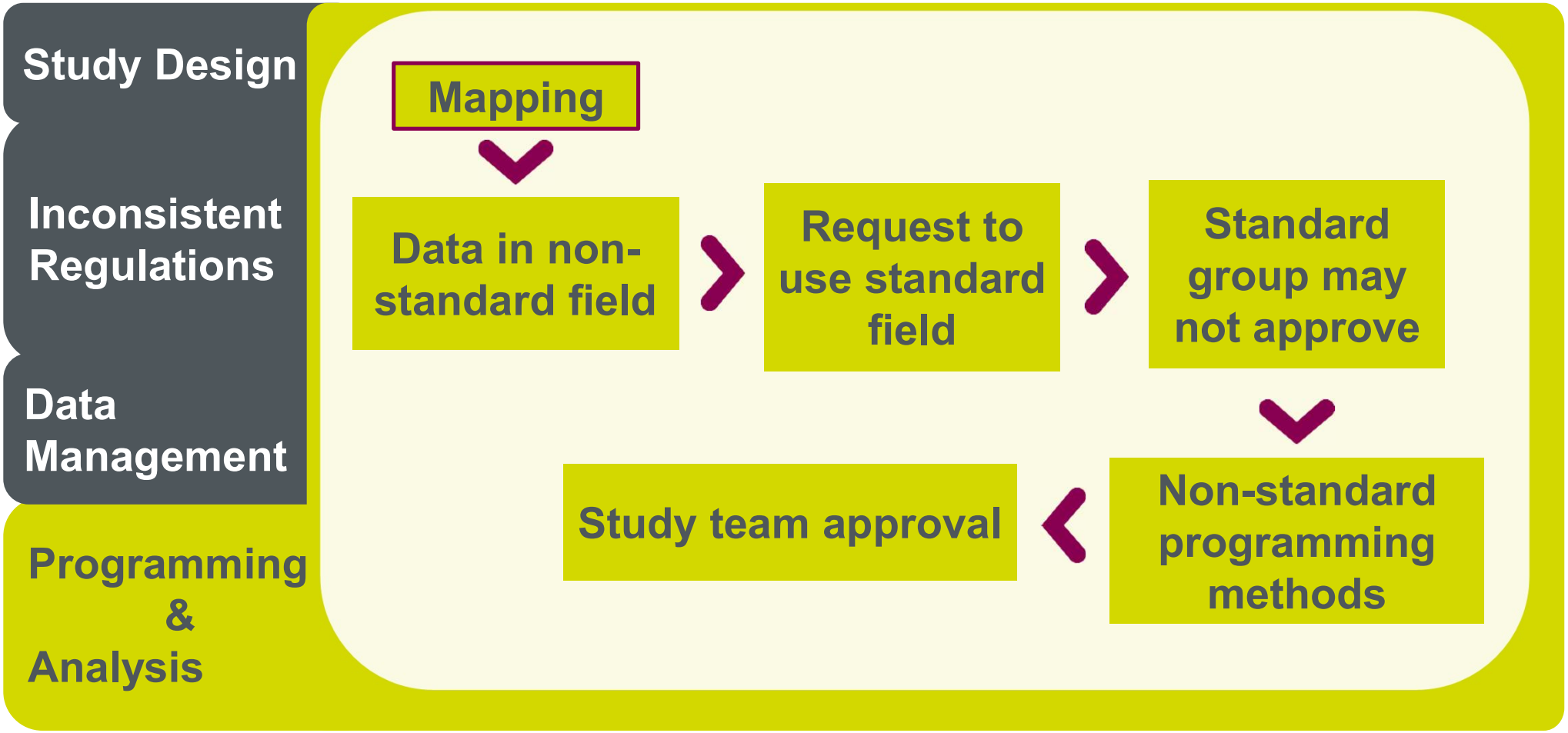
Programming & Analysis

- Non-standard approach to CRF design
  - Present database to accommodate the RWD setting
- Different standard of care
  - Data lost by patient
  - Data not documented
  - Patient moved to another area
  - Case by case need to be accommodated
- Staff availability and data varying scheduled visits ==> Delay in entry and timelines

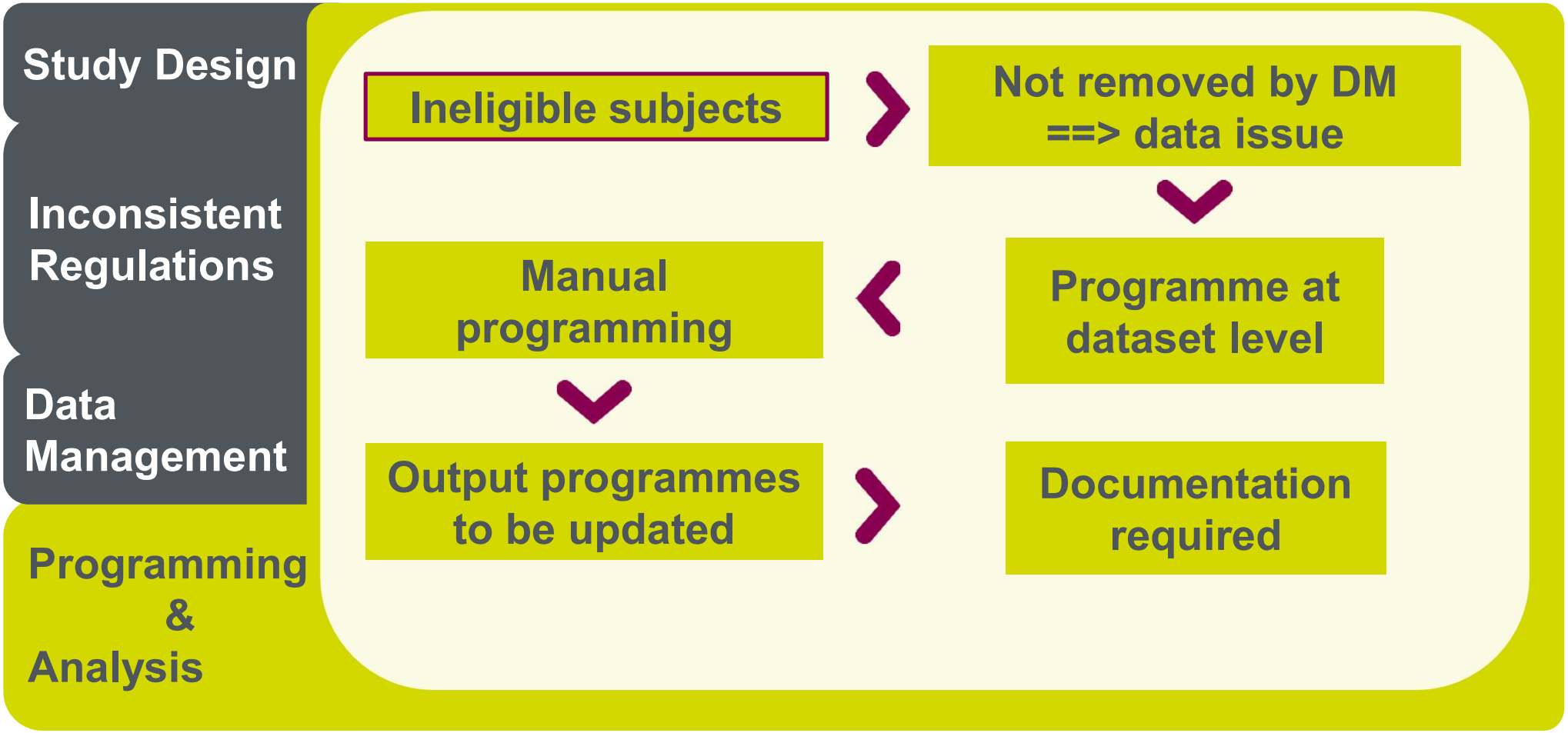
RWE Study Challenges



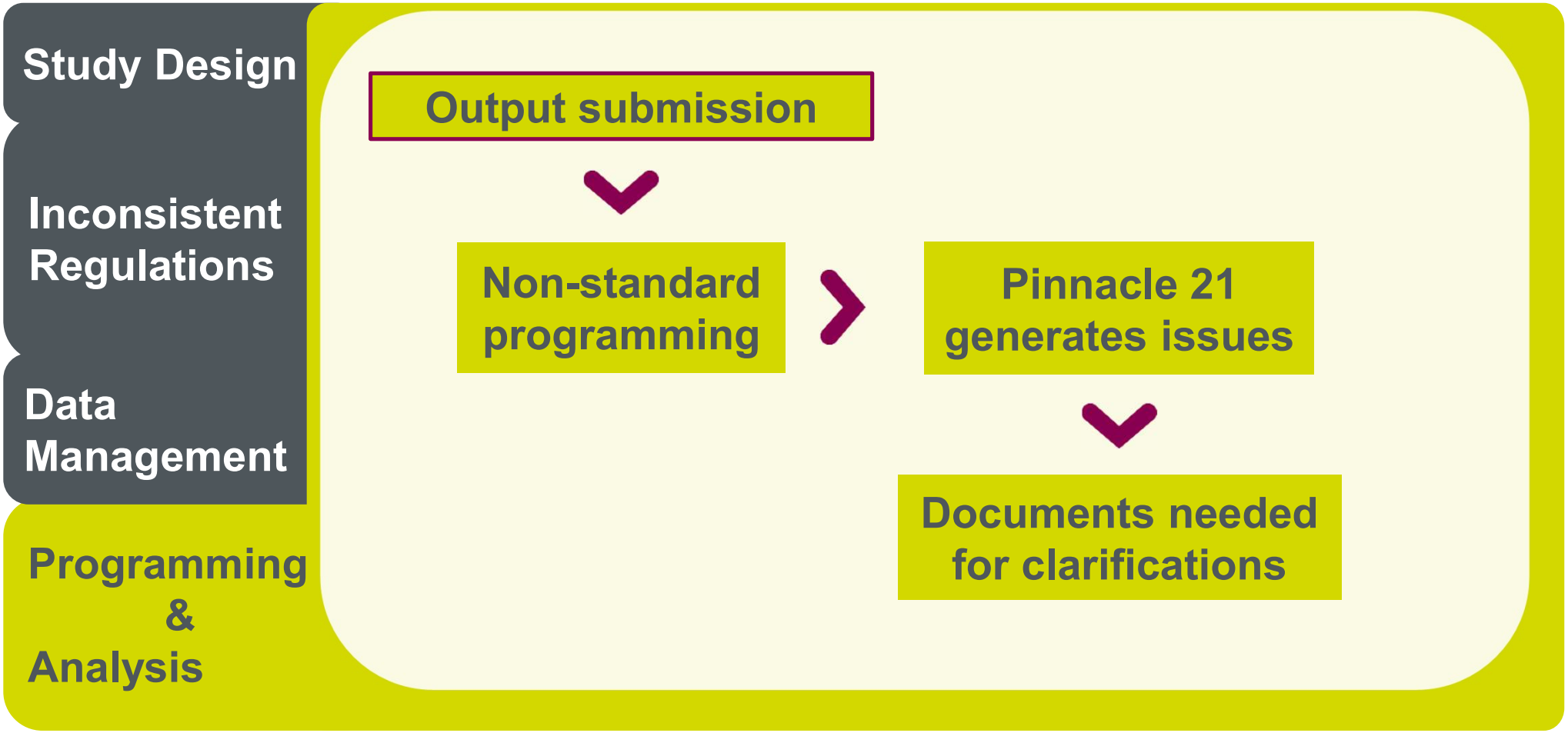
RWE Study Challenges



RWE Study Challenges



RWE Study Challenges



Remarks

- Fewer regulations in place for RWD can challenge RWE studies in all stages from study design all the way to providing analysis to stakeholders.
- RWE are financially feasible though to complement clinical trials and study more outcomes in bigger patients' population

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Thank you