

Fundamentals of real world evidence

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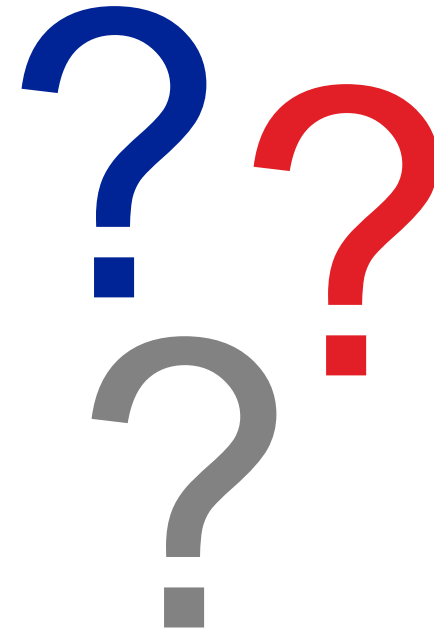


Questions for you

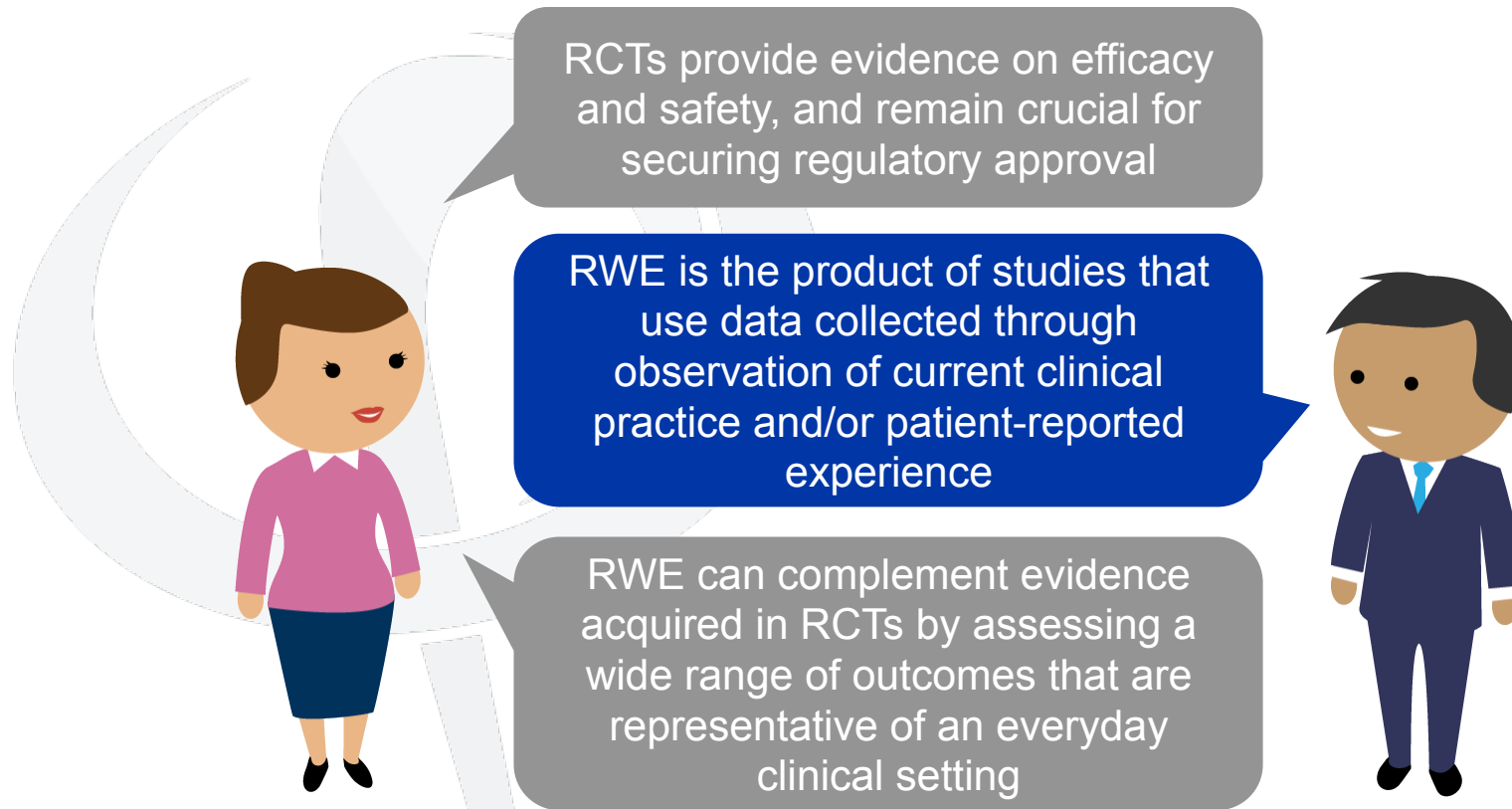
A large, light gray magnifying glass graphic is positioned on the left side of the slide. The handle of the magnifying glass is at the bottom, and the lens is at the top. Two blue speech bubbles are attached to the lens area.

What is RWE?

Do we need RWE if we have good RCT data?



Types of evidence



Efficacy is the intrinsic effect of an intervention measured under pre-specified conditions

Effectiveness measures the beneficial effect in routine clinical practice



Efficacy versus effectiveness: an analogy



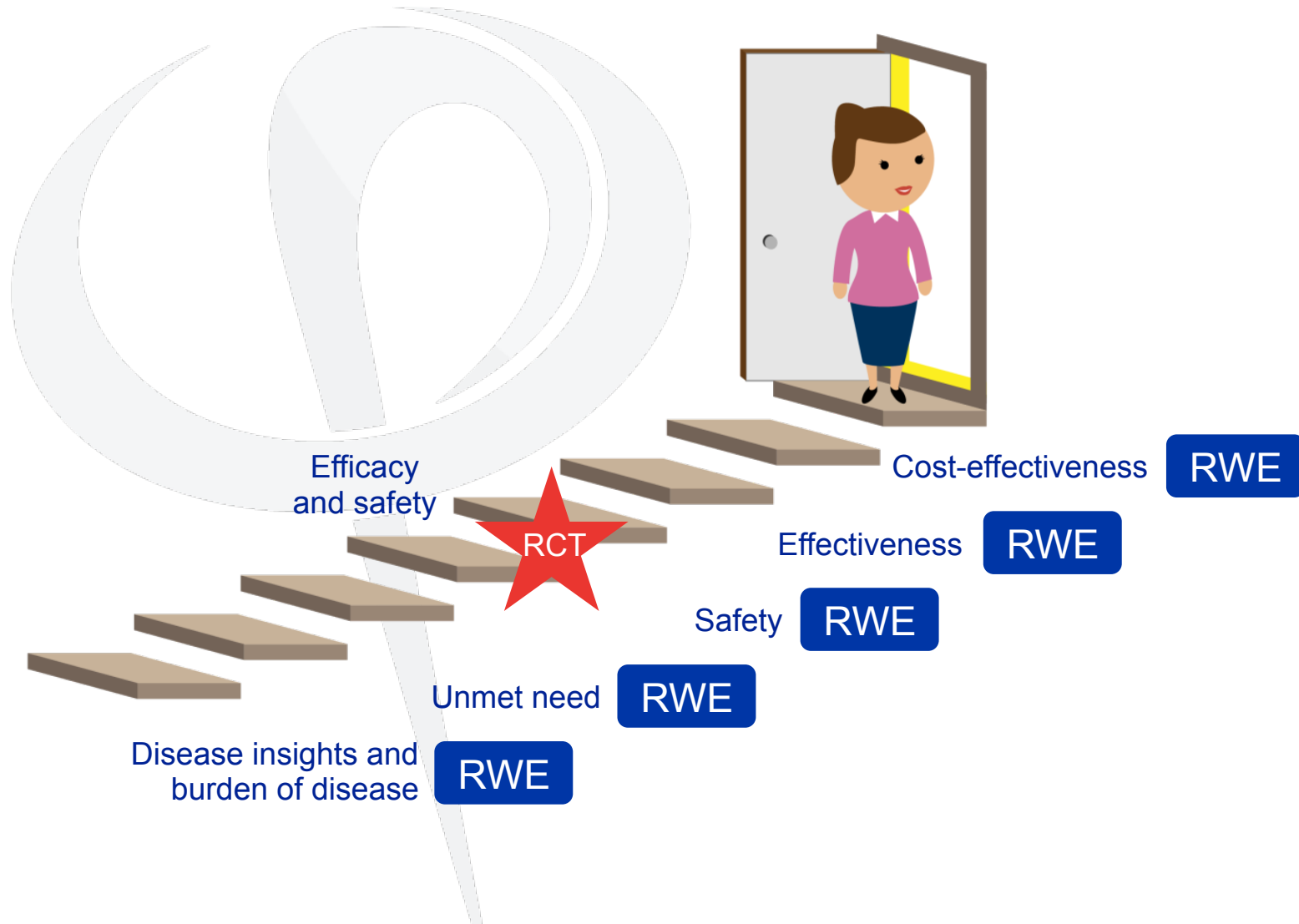
Standing quarter mile:
12.5 seconds



Standing quarter mile:
> 12.5 seconds!



Steps to success




RWE complements data from RCTs

	RCT	RWE study
Objective	Can it work?	Does it work?
Purpose	To gain regulatory approval	To influence clinical practice
Setting/design	Ideal conditions	Real world conditions
Intervention	Fixed regimen	Flexible regimen
Compliance	High	Low to high
External validity	Low to medium: homogeneous populations	High: heterogeneous populations
Internal validity	High	Variable



A question for you

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Where does RWE
come from?



RWE data sources

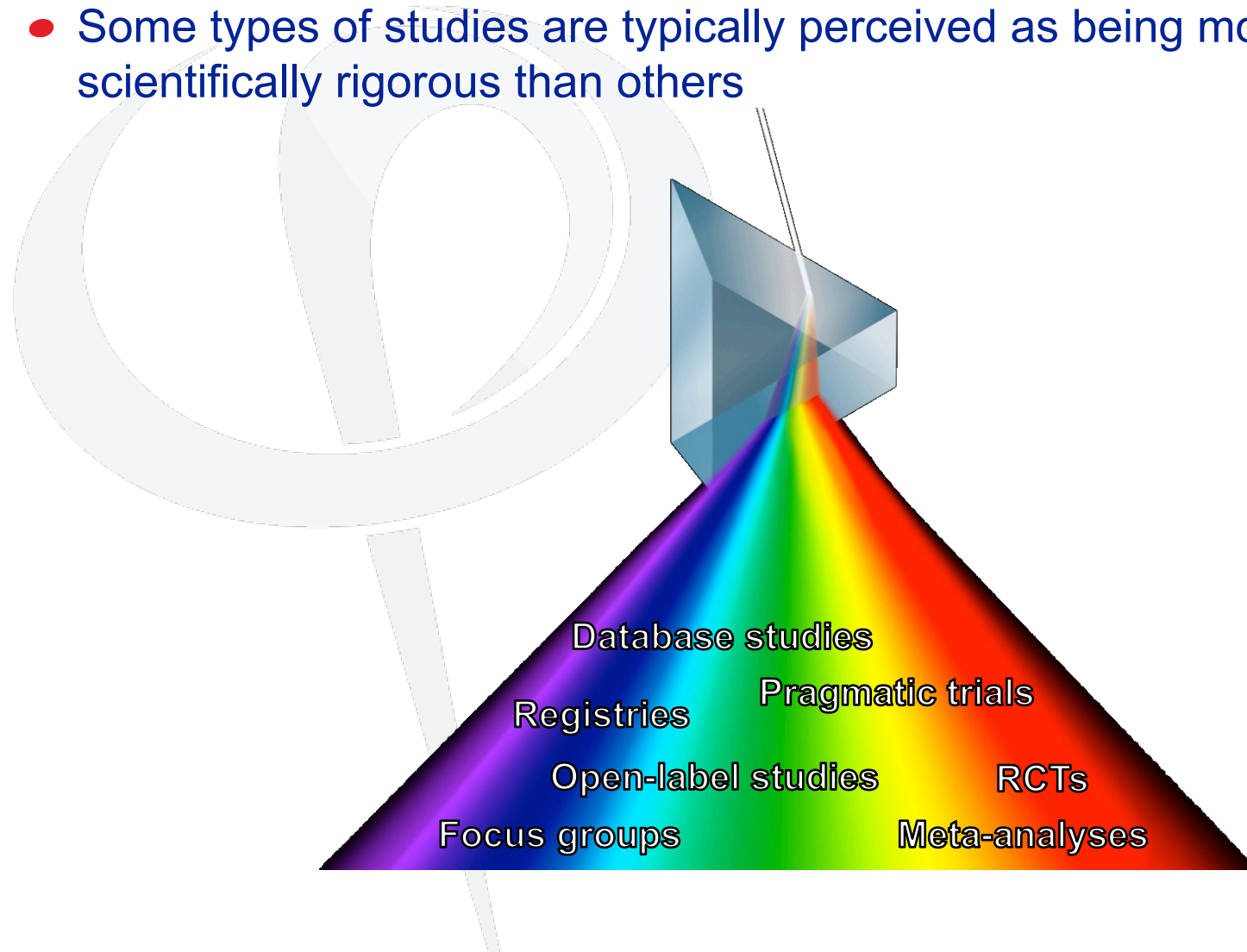
- Evidence can be generated from primary or secondary data sources
 - Primary data are newly generated by an investigator during a study
 - Secondary data refer to patient-level data that were collected for other purposes and are being used again to answer another research question or hypothesis

Primary data sources	Secondary data sources
RCTs	Administrative claims databases
Pragmatic clinical trials	Electronic medical records
Prospective cohort studies	Disease registries
	National and regional registries



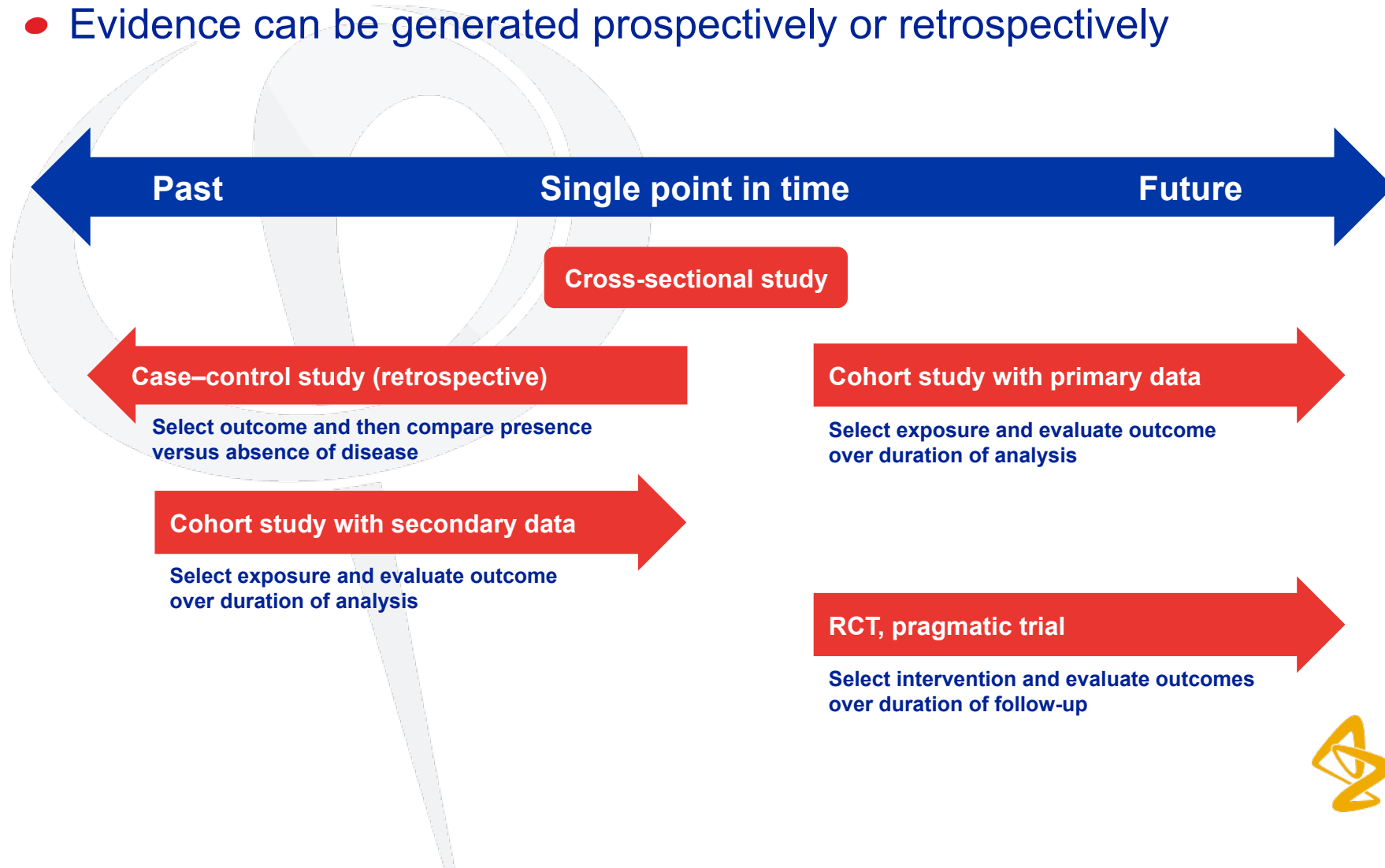
Validity of evidence

- Some types of studies are typically perceived as being more scientifically rigorous than others



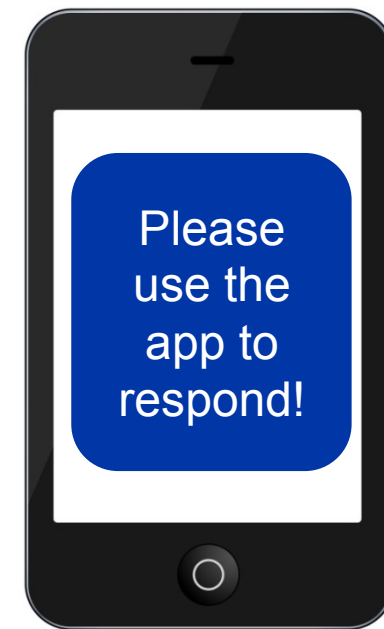
Direction of research

- Evidence can be generated prospectively or retrospectively

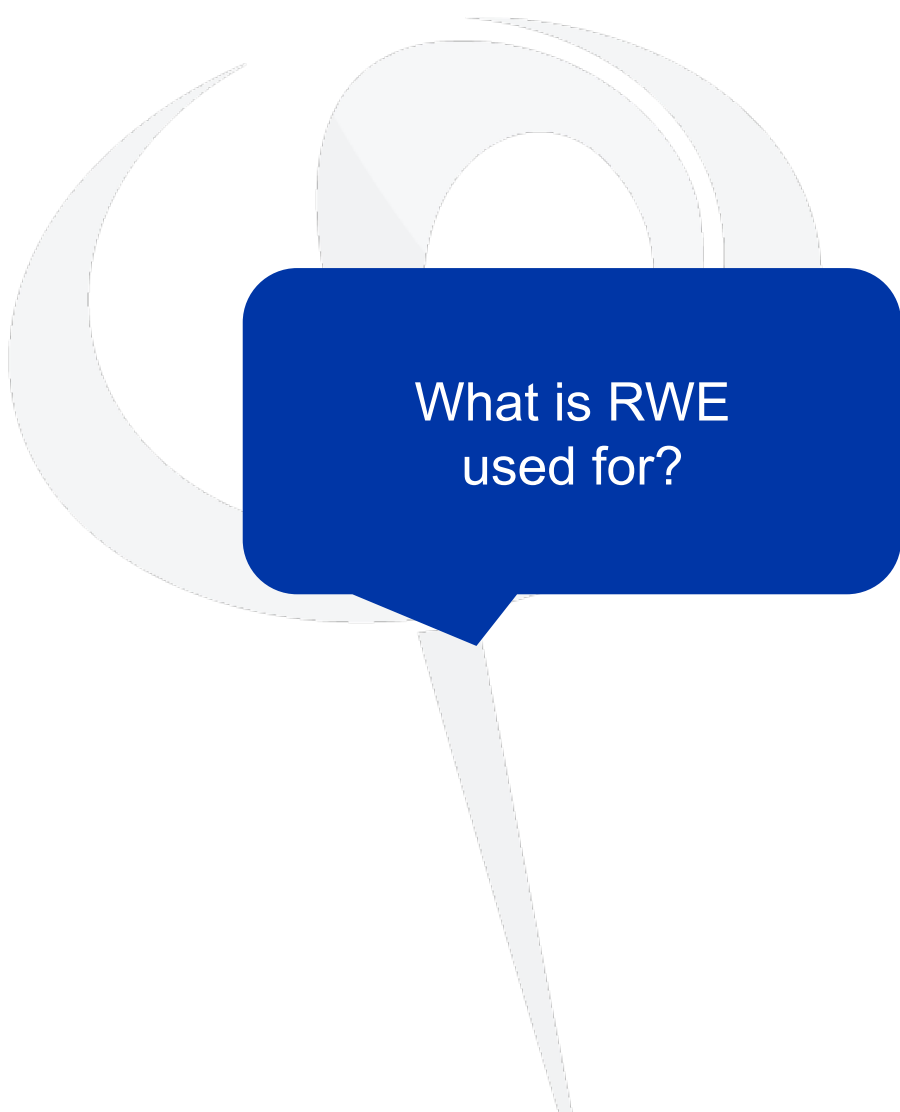


A question for you

- How important do you think RWE is?
 1. It is a critical element of evidence-based medicine
 2. It is helpful for informing clinical decisions but it is not essential
 3. It is not important at all, RCTs give us all the evidence we need



A question for you

A large, light gray question mark icon with a speech bubble pointing to the text.

What is RWE
used for?



Healthcare decision-makers need evidence

- Healthcare decision-makers are interested in clinical decisions and the evidence that supports those decisions
- There are a variety of healthcare decision-makers



- Understanding their different perspectives is key to generating the right evidence

Does this treatment work? How safe is it?

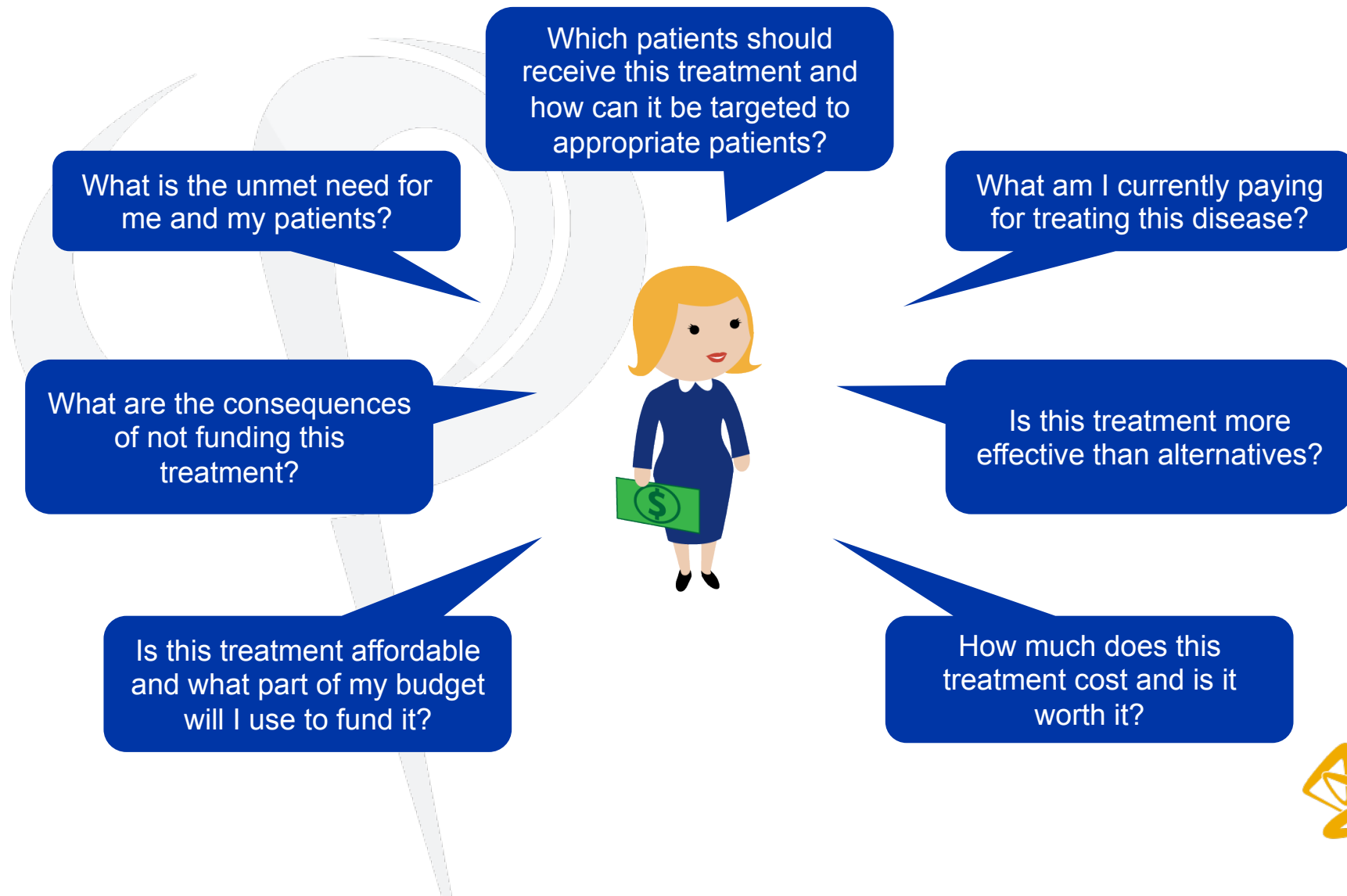
How much does this treatment cost?
Is it worth it?

How are other healthcare professionals using this treatment? What results are they seeing?

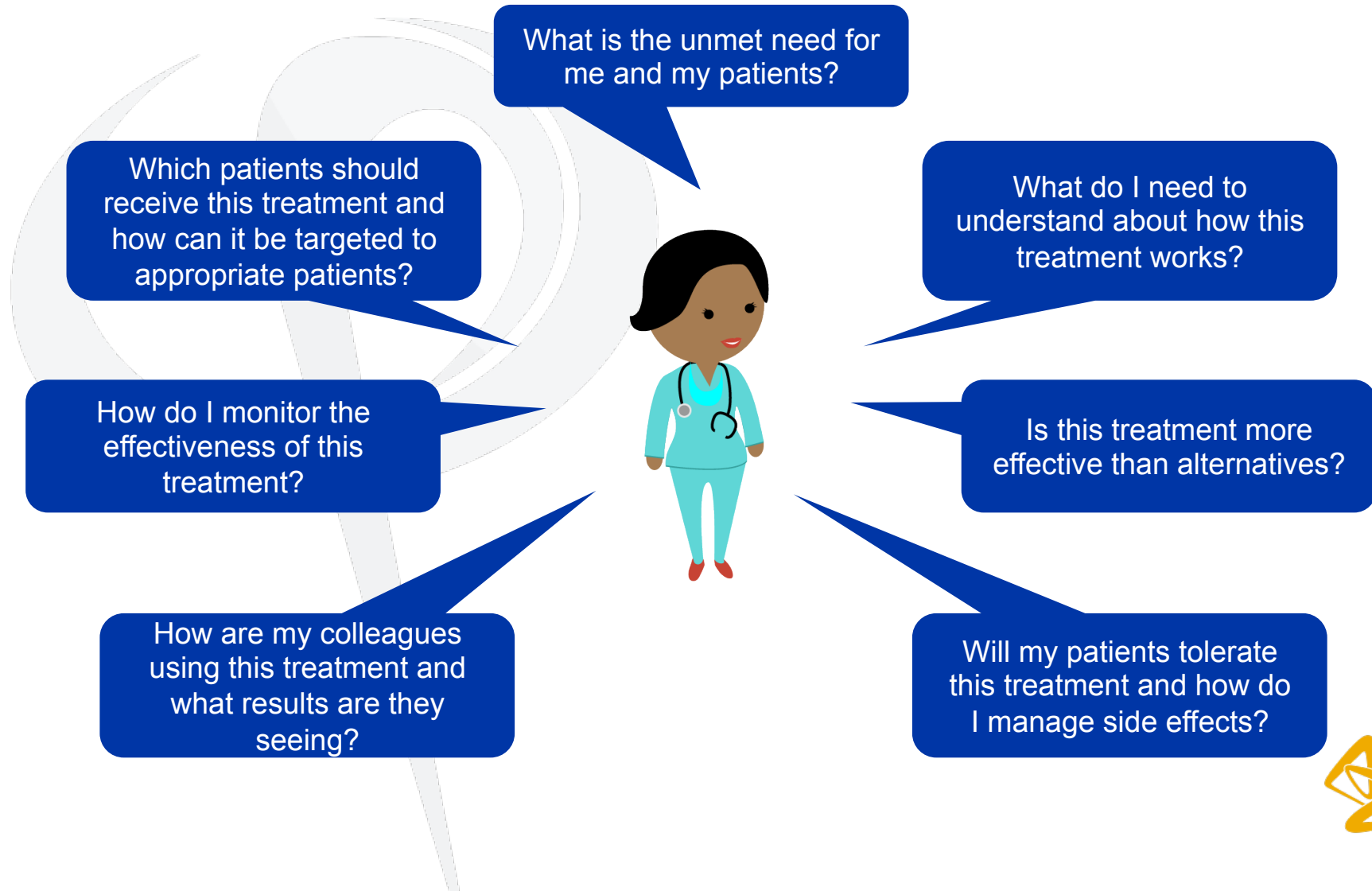
Is this the best treatment for me?



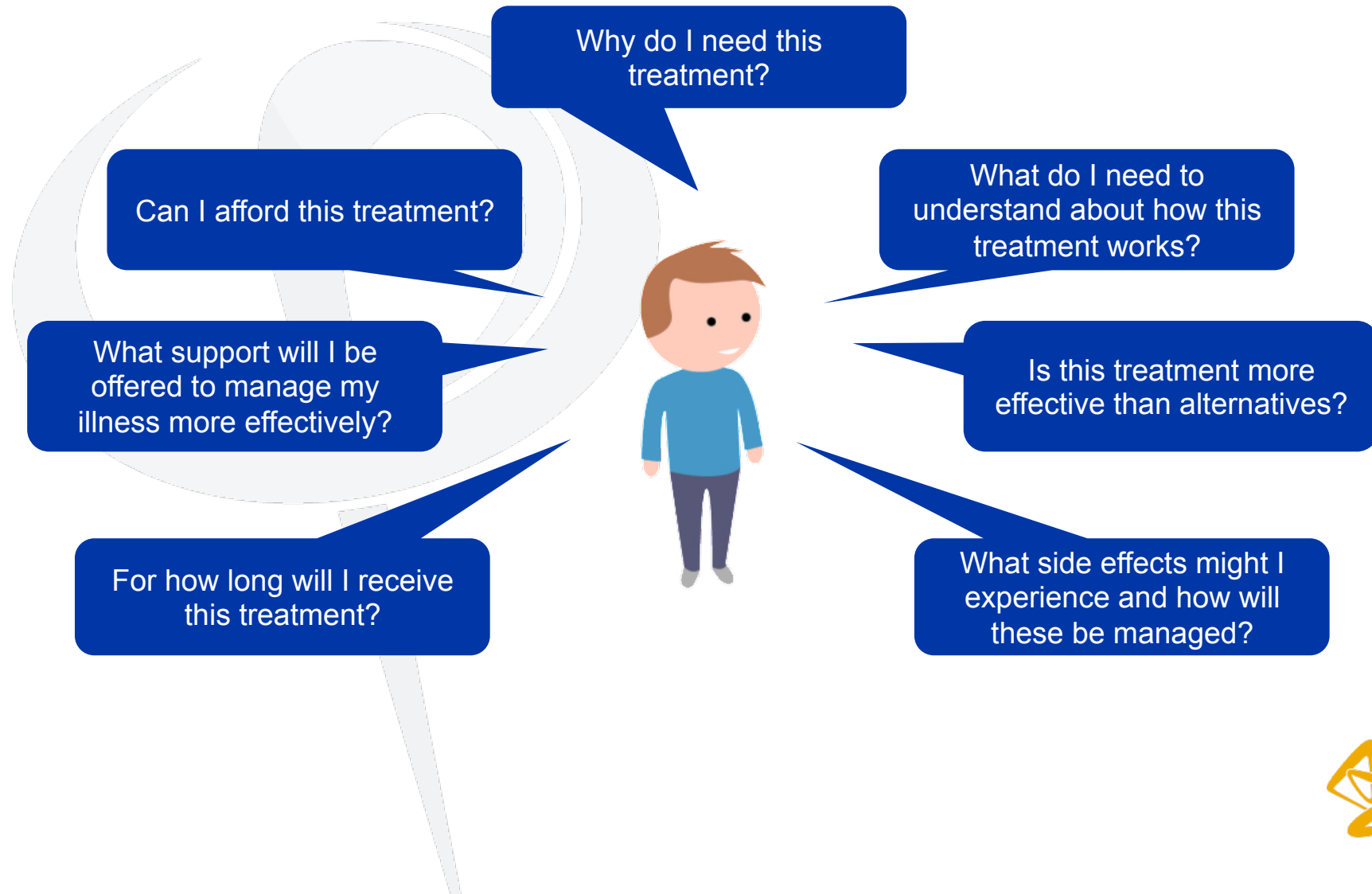
Payer evidence needs



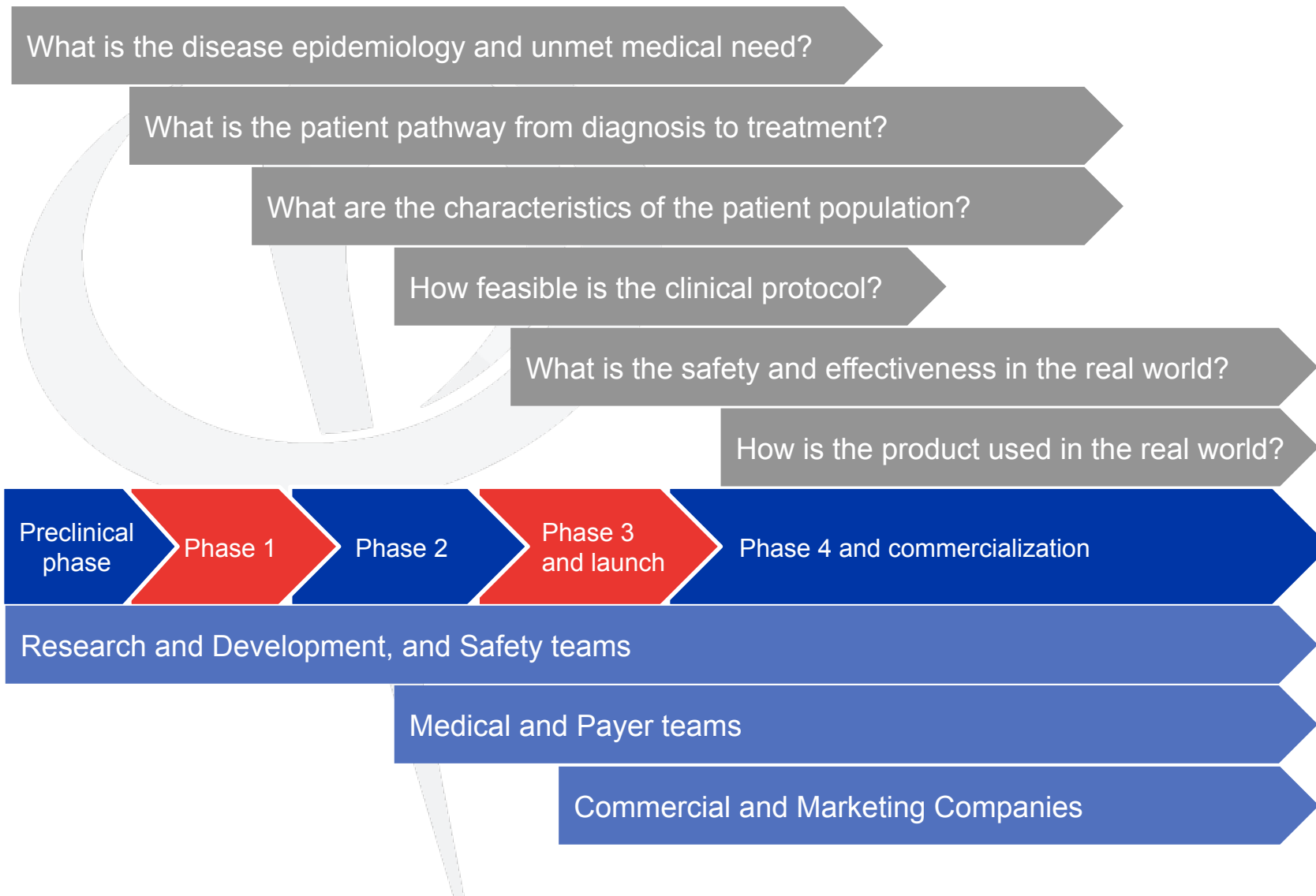
Healthcare professional evidence needs



Patient evidence needs



Evidence is needed throughout the product life cycle



Summary: what is RWE?

Where does RWE come from?

- Observational studies
- Current clinical practice
- Patient-reported experiences

What does RWE do?

- Complements evidence from RCTs
- Describes outcomes representative of an everyday clinical setting

What information can RWE provide?

- Burden of disease and unmet medical need
- Treatment reality
- Comparative clinical effectiveness and safety



Summary: why is RWE important?

