Real World Evidence – how patient groups can contribute and how to engage patient groups in clinical research

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What is real world evidence

Why we need real world evidence

Where can patients get involved in real-world evidence and clinical research generally

Questions and issues

Summary

What real world evidence is

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Real-World Evidence — What Is It and What Can It Tell Us?

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N Engl J Med 2016; 375:2293-2297 | December 8, 2016 | DOI: 10.1056/NEJMsb1609216

RWE definition. While the definition of **Real World Evidence** is still evolving, most proponents associate RWE with data that is derived from medical practice among heterogeneous sets of patients in **real life** practice settings, such as insurance claims data and clinical data from electronic health records.

https://www.nehi.net/writable/publication_files/file/rwe_issue_brief_final.pdf

What real world evidence is

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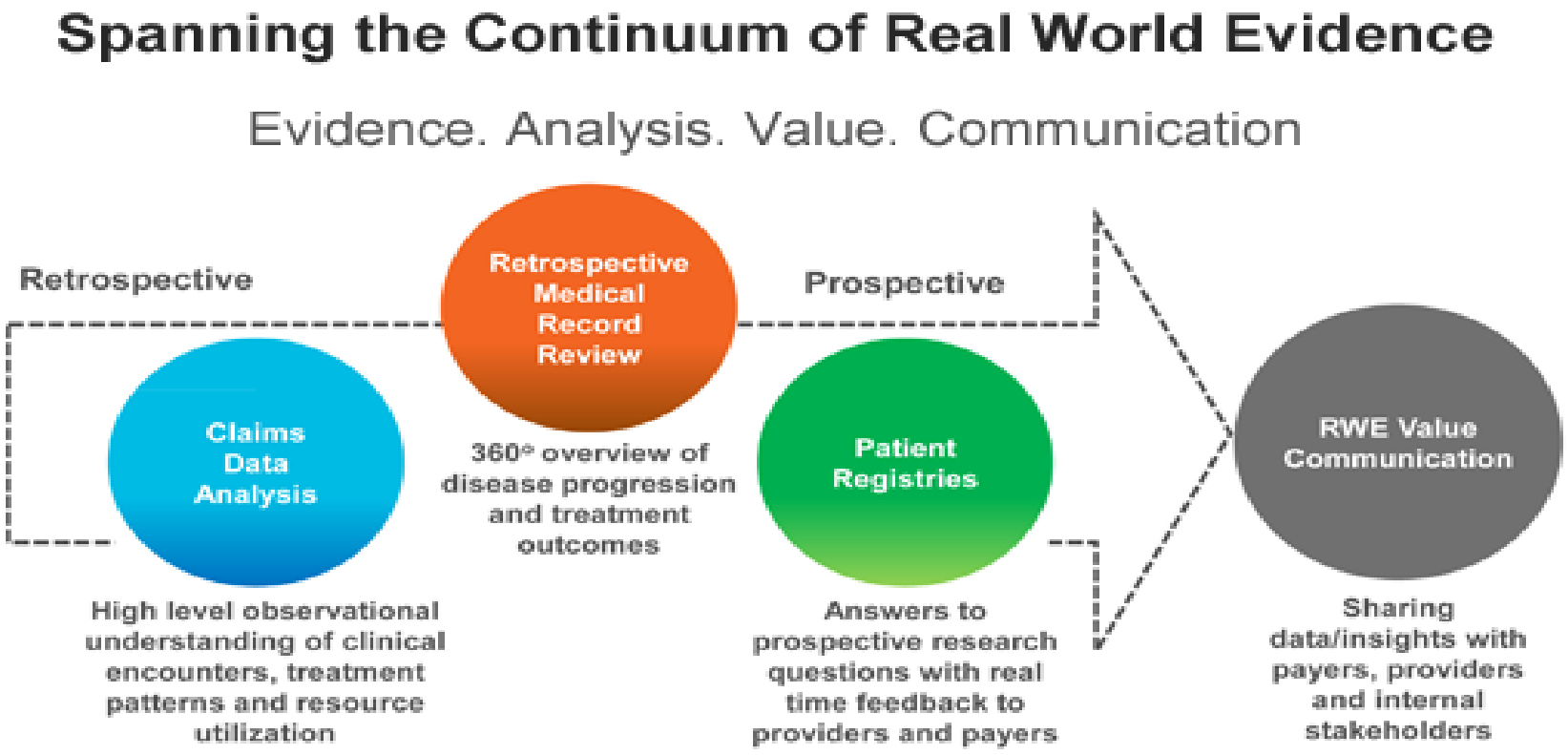
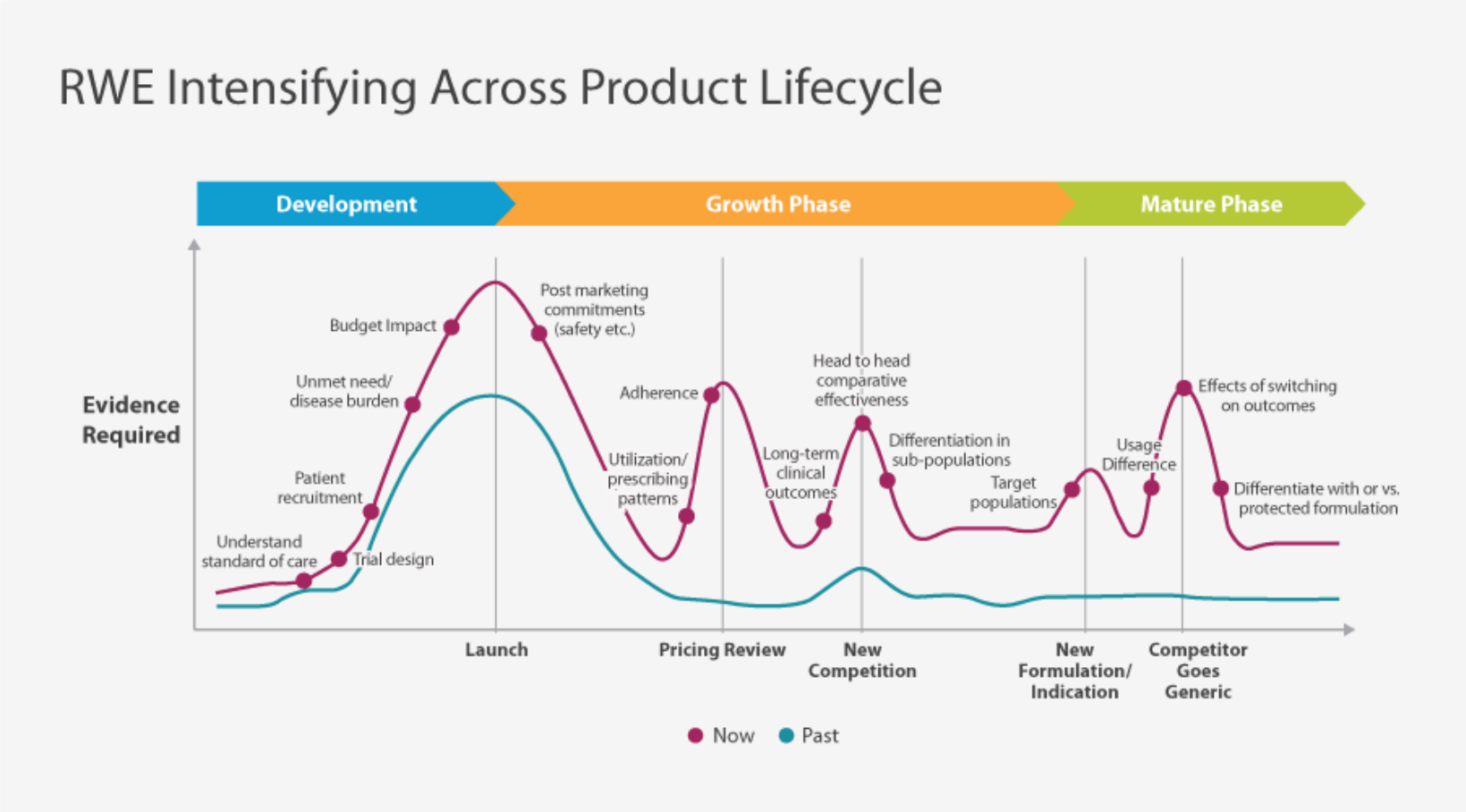


What real world evidence is



Why we need real world evidence

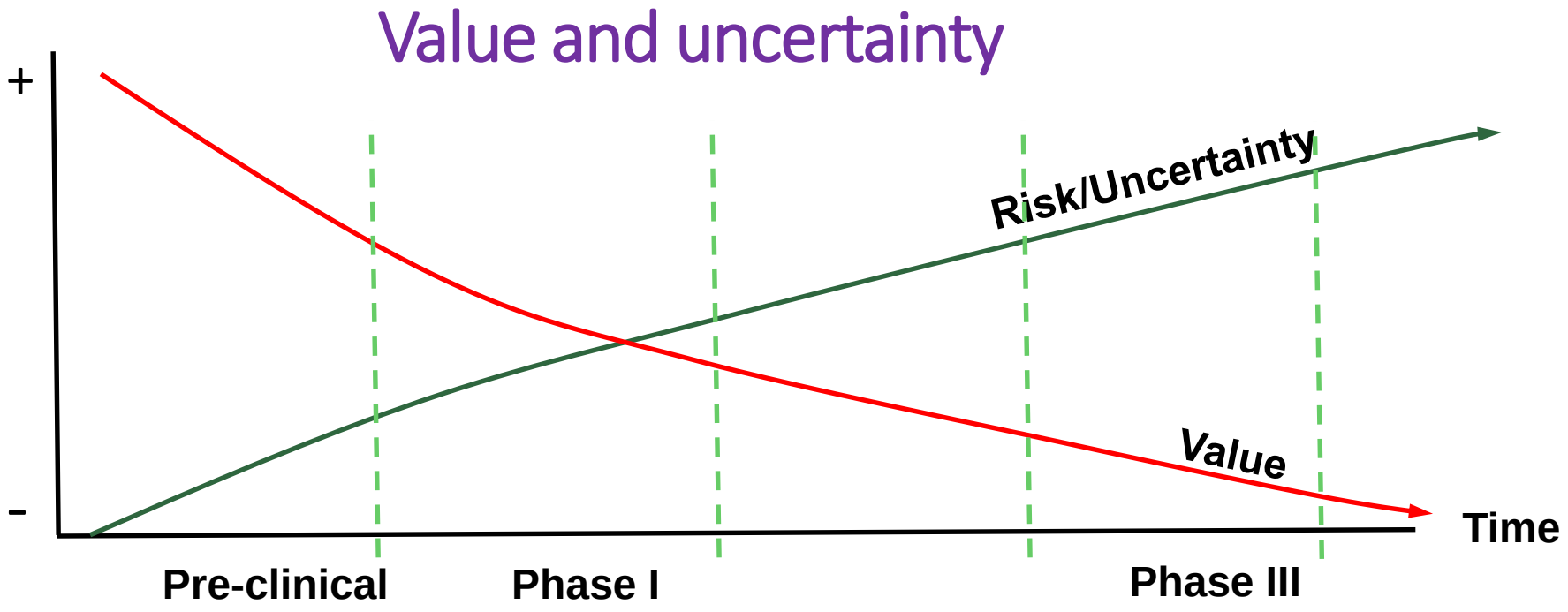
Clinical trial system broken……

The clinical trials system is "broken" and there needs to be new ways to collect and utilize patient data, Janet Woodcock, director of FDA's Center for Drug Evaluation and Research, told a workshop at the National Academies of Sciences, Engineering, and Medicine today.

https://endpts.com/fdas-janet-woodcock-the-clinical-trials-system-is-broken/

FDA U.S. FOOD & DRUG ADMINISTRATION

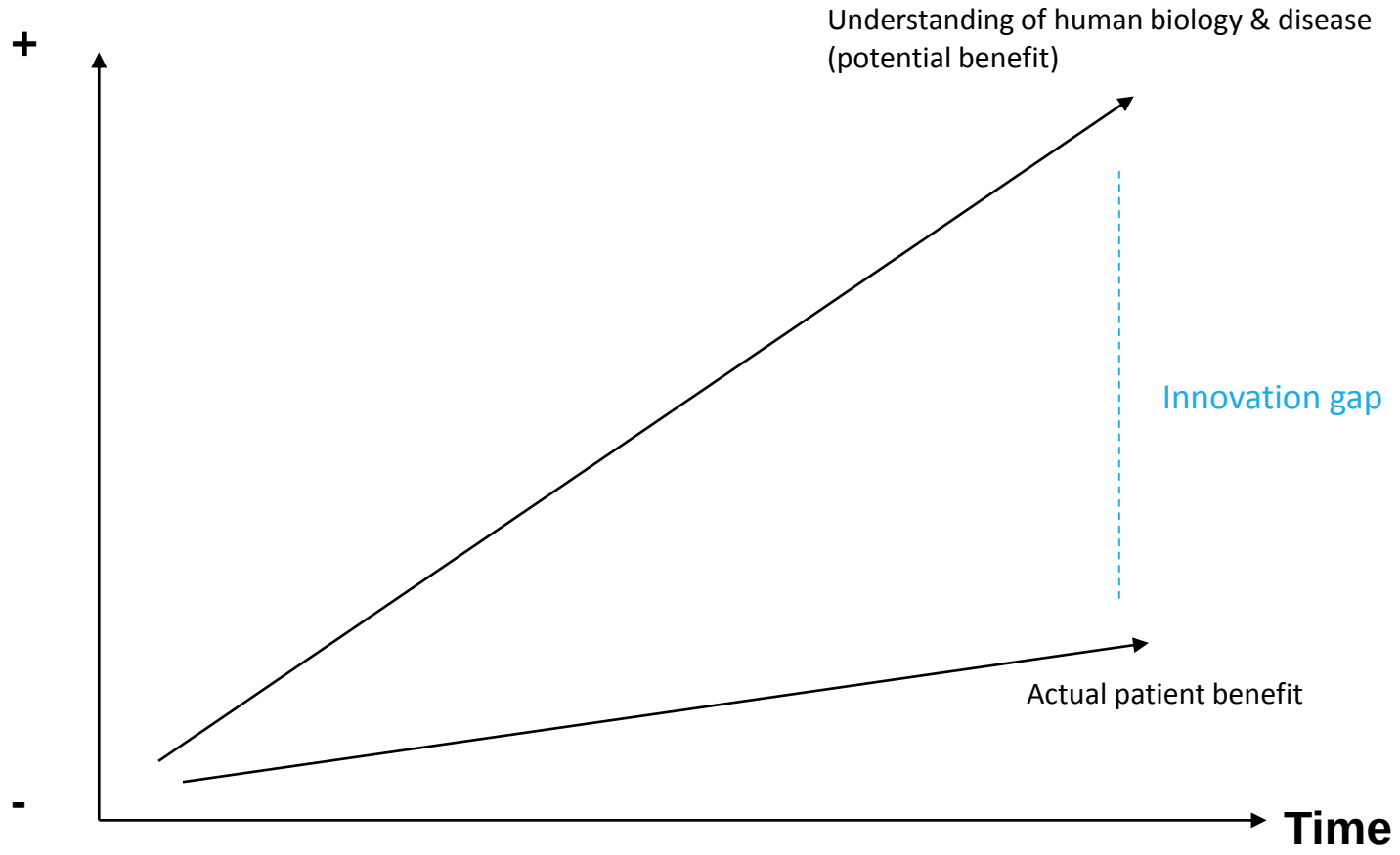
Why we need real world evidence



As a consequence of poor data

Why we need real world evidence

Bioscience: Lost in Translation? Richard Barker



How is RWE different?

WHAT DOES REAL WORLD EVIDENCE OFFER IN COMPARISON TO CONVENTIONAL RANDOMISED CONTROLLED TRIALS?		
RCTs		**RWE**
Prospective data collection		**Prospective and/or retrospective data collection**
Limited segment of the population is eligible for inclusion		**Broader and more representative of the patient population**
Good patient adherence and compliance		**Real world patient adherence and compliance**
Important for demonstrating efficacy and safety for drug licensing		**Demonstrates the benefits of a drug in everyday clinical practice**
Limited ability to investigate costs		**Ideal for showing value within local health economy**

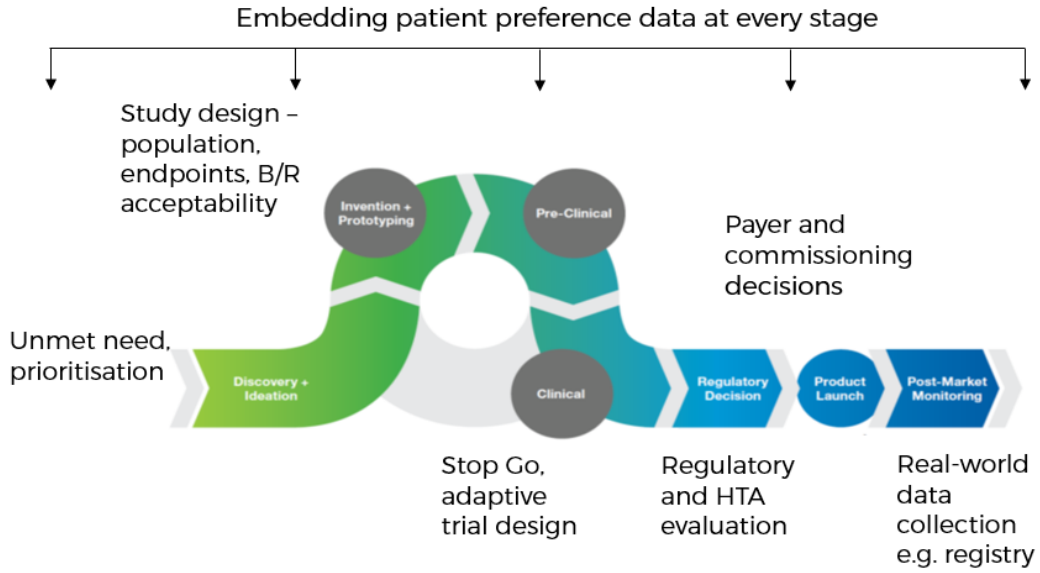
Where patients can get involved

Efficacy
Restricted patient group
Controlled environment
Value to regulators

Effectiveness
All patients
Real life environment
Value to payers

Where patients can get involved

- Health states tools
- Quality of life tools
- Patient reported outcome measures
- Patient preferences for treatment in the context of potential benefits and risks



Becoming increasingly important to articulate value proposition and to differentiate from other treatments especially where the clinical benefit is marginal.

"If we expect patients to comment on the benefits at the evaluation phase we need to ensure the endpoints to be meaningful to them in the first place".

Where patients can get involved

Myeloma UK Clinical Trial Network MUK *eight* phase IIb clinical trial to reduce uncertainty:

- Collaboration with Takeda (Millennium) Pharmaceuticals
- Critique of regulatory CPD identified risks that would likely lead to uncertainty
- Designed a phase IIb CTN study to generate evidence to mitigate risks and improve value proposition
- submitted jointly to NICE Scientific Advice as part of pilot
- Further similar studies and approaches in planning

NICE

MyelomaUK

Takeda ONCOLOGY

Natural history studies

What is different about rare diseases and Orphan drugs?

Diseases are usually poorly or incompletely understood

Generally, the lower the prevalence, the less well we tend to understand them – small populations

Limited opportunity for study and replication – highly heterogeneous group of diseases

7,000 different diseases

Often high phenotypic diversity within individual disorders

Usually little precedent for drug development within individual disorders

Often requires more (and more careful) planning than non-Orphan

Need a solid scientific base upon which to build an overall programme

Purpose: To inform drug development

Marketing approvals require design and conduct of adequate and well-controlled studies

Designing studies requires a scientific foundation upon which to build

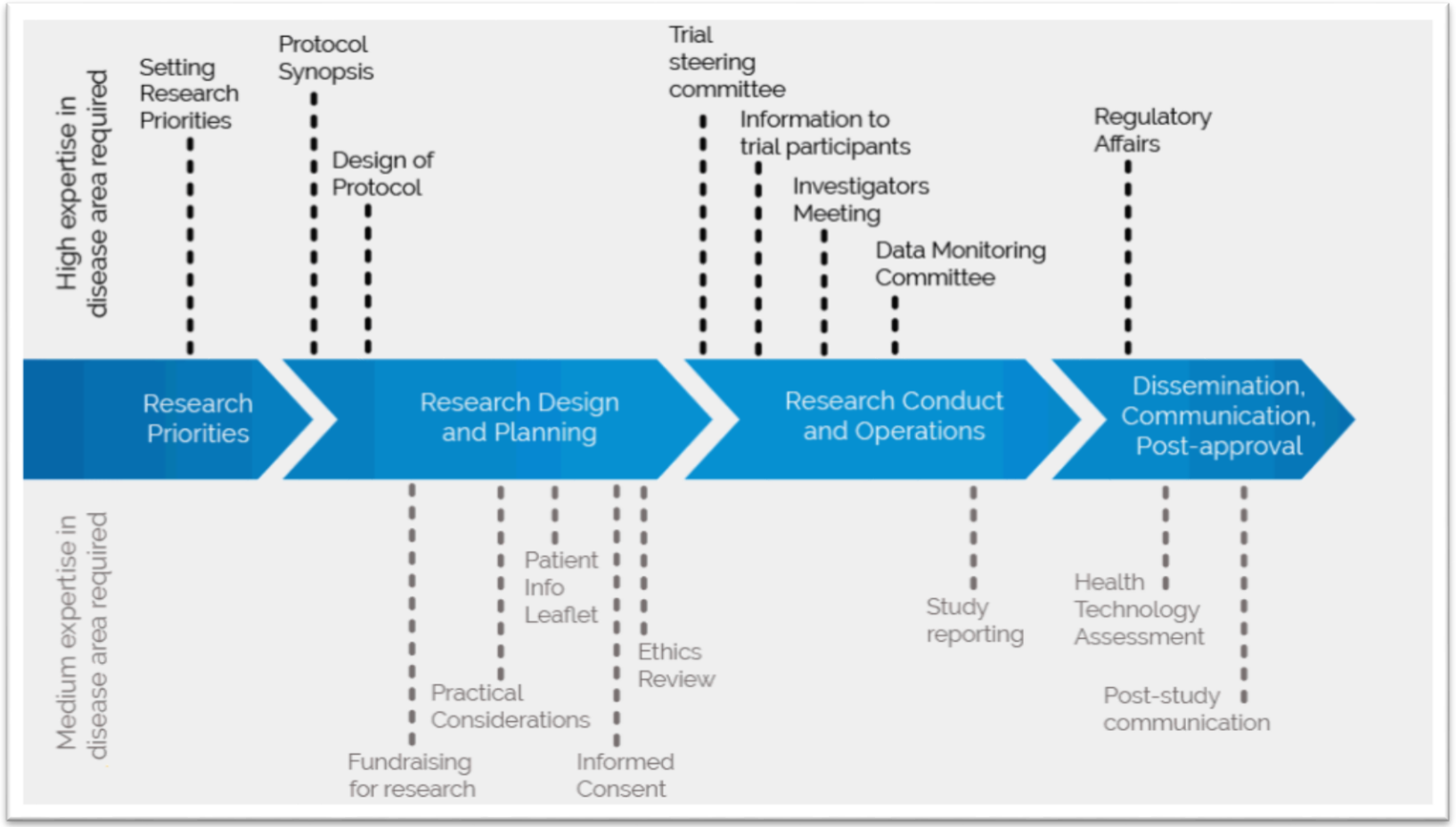
Knowledge of disease natural history is an essential element in the scientific foundation of any clinical development programme

Rare diseases, in general, are poorly understood

Important and essential role for natural history studies in rare disease drug development to facilitate efficient clinical development

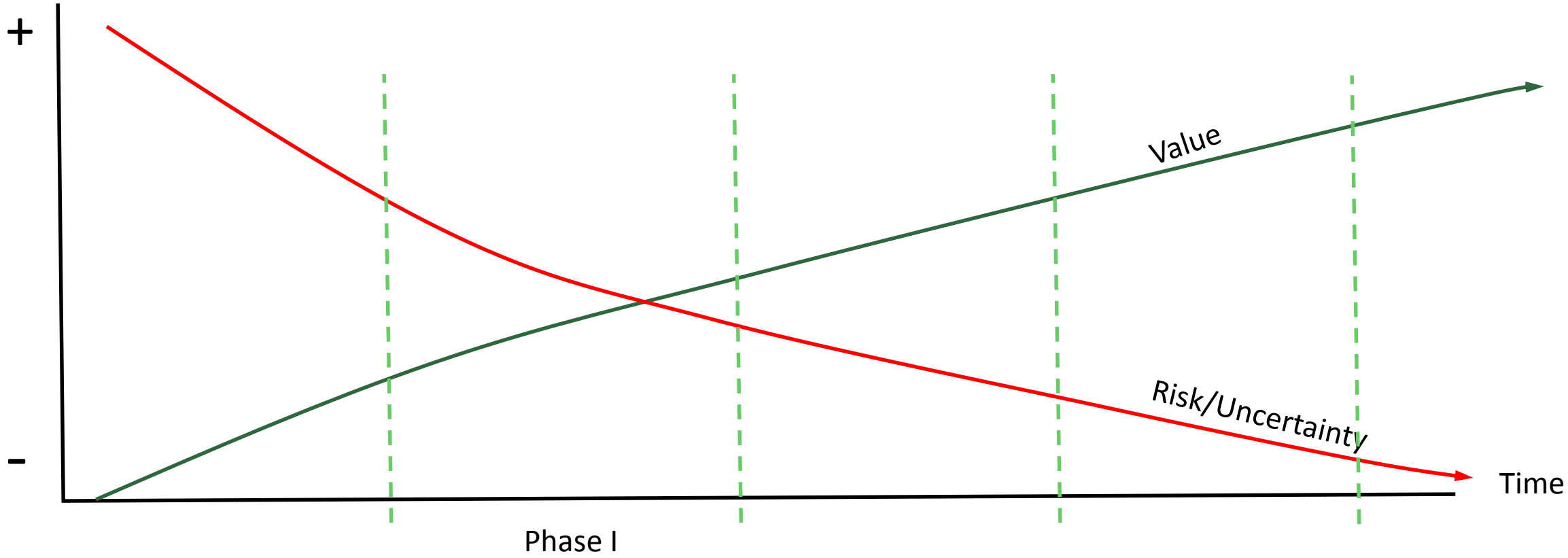
amyloidosis research consortium

Where patients can get involved



Source: Geissler, Ryll, Leto, Uhlenhopp EPALCO/EUPATI (2015, unpublished)

What success looks like



Better Research for Better Outcomes

Questions, risks and issues

Real world evidence on its own may not be the solution without improving underlying research.

Real world data may not be reliable and increasing uncertainty.

Who should pay for it and who owns it.

Potential challenges in rare disease in post marketing authorisation registries.

Potential issues if used to pay for outcomes or to shift price.

It could cause inequity in access to treatment and care where

Summary

Real world evidence has a role to play but cannot be used in isolation

Many issues and challenges not least that it could add to uncertainty

We need to improve clinical research and establish better alignment between the data needs of regulators and those of HTA bodies, payers and patients