RWE Generation: Blueprint for Drug Development and Commercialization

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Presenters

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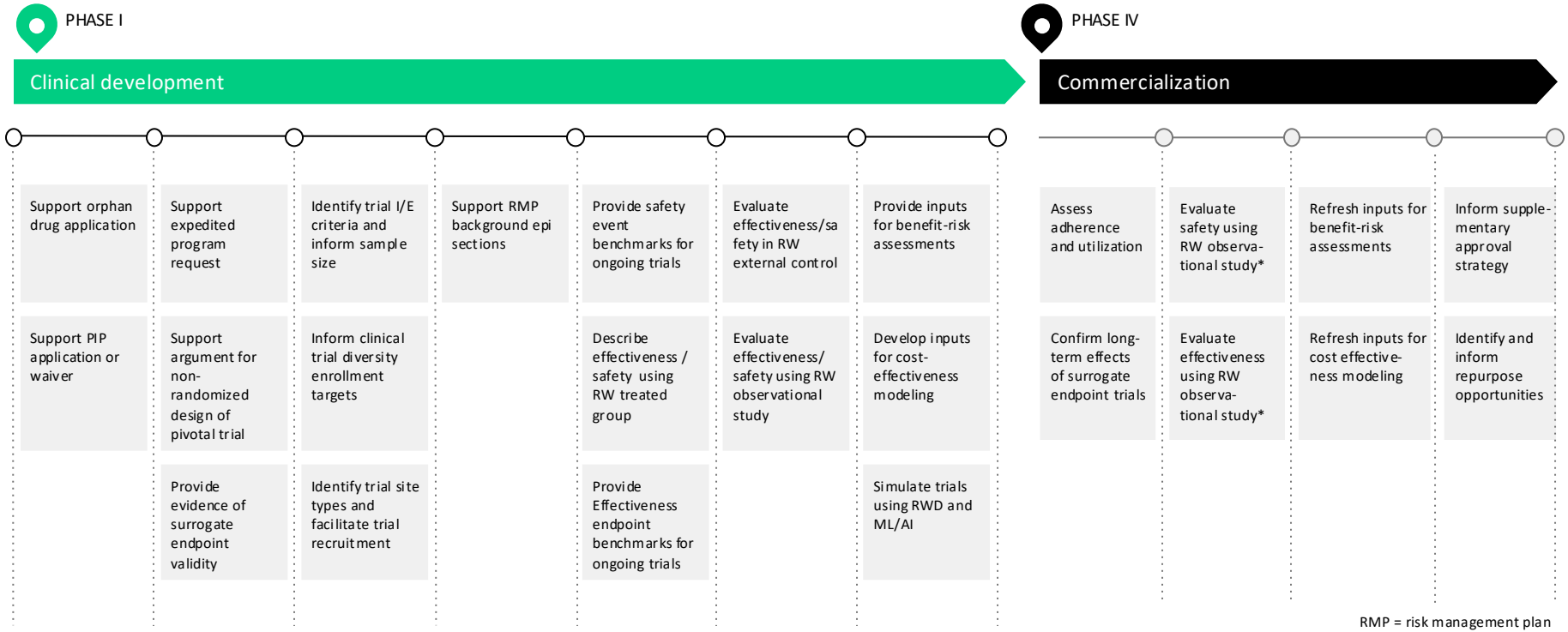
Agenda

Welcome and introductions	5 min
Back to basics - defining RWE “homework studies”	5 min
Role of robust descriptive RWE studies in clinical development	5 min
Role of robust descriptive RWE studies in commercialization	5 min
Q+A	10 min

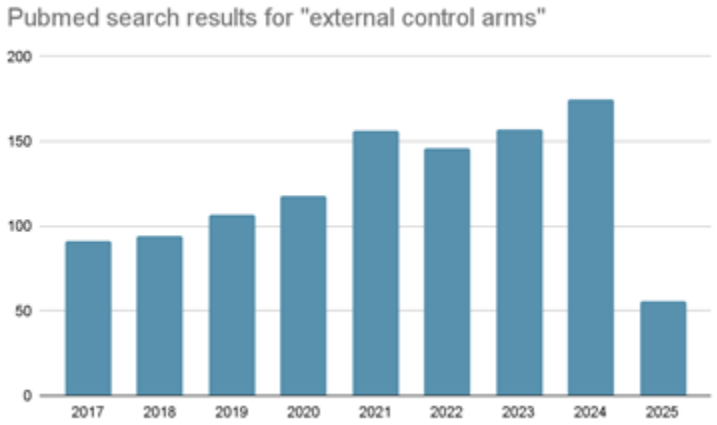
RWD provides key insights for development and commercialization



RMP = risk management plan
PIP = pediatric investigational plan
ECA - external comparator/control arm
*Voluntary or to fulfill: US post-marketing requirements (PMR), post-marketing commitments (PMC); EU post-authorisation safety studies (PASS), EU post-authorisation efficacy studies (PAES) for effectiveness.

Robust descriptive RWE studies are often overlooked

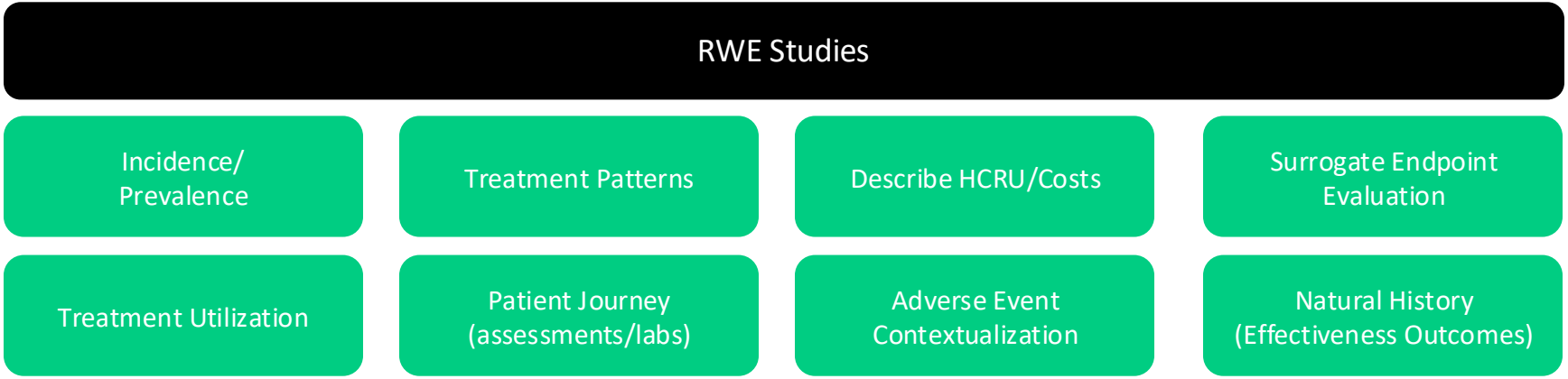
Recent focus has been on complex and often rarer use cases for RWD (e.g., ECAs)



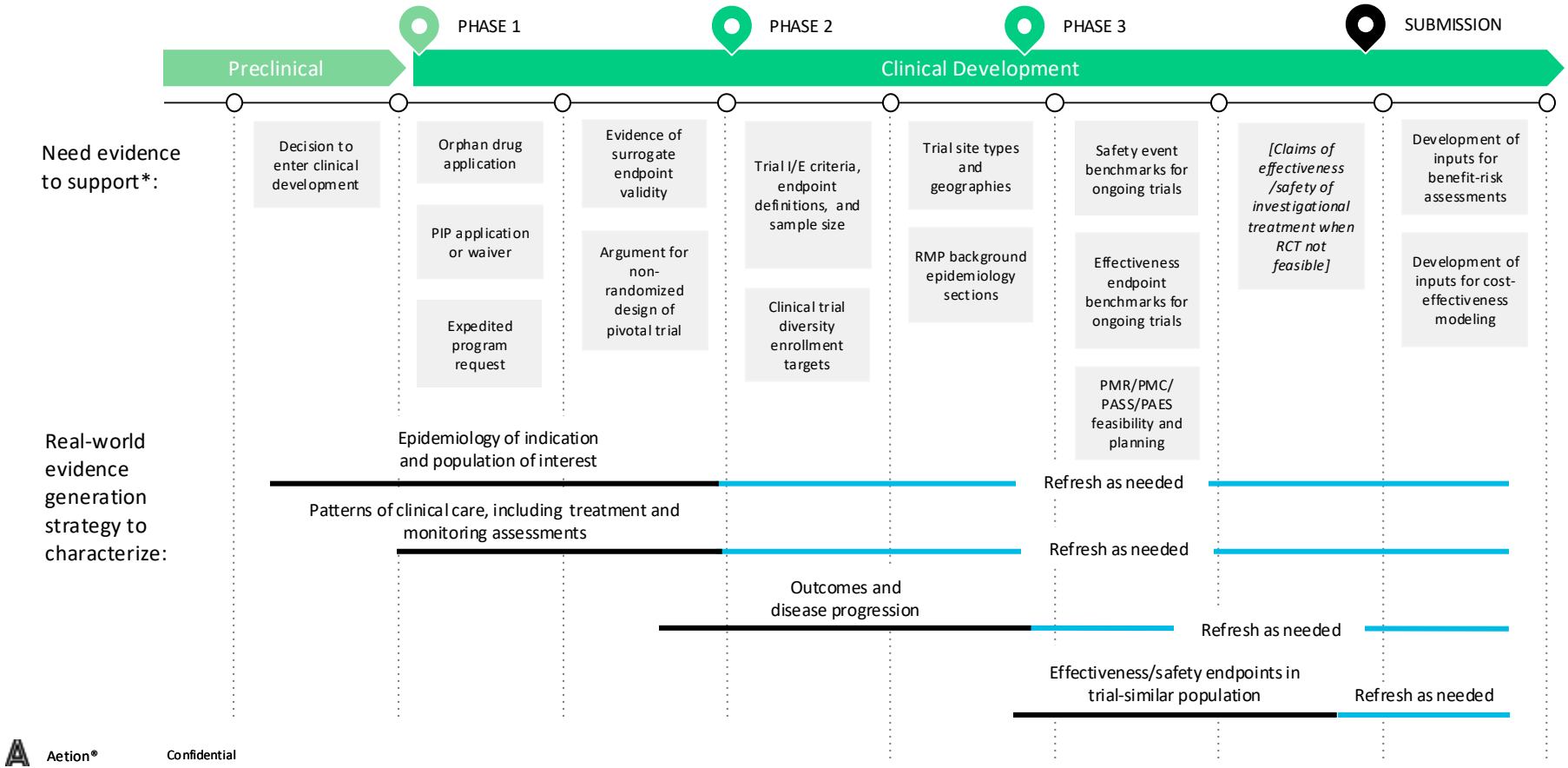
Robust descriptive RWE studies play a key role in regulatory and HTA decision-making

- In a review of 111 EPAR documents from the EMA, RWE was used in 98% of EPARs to describe the epidemiology of the disease including burden of disease, disease features, and identifying the right patient population (Eskola et al, 2022)
- In a review of 52 HTAs for melanoma drugs, 56% cited RWE studies for prevalence/incidence, 48% cited RWE for cost estimates (Makady et al, 2018)

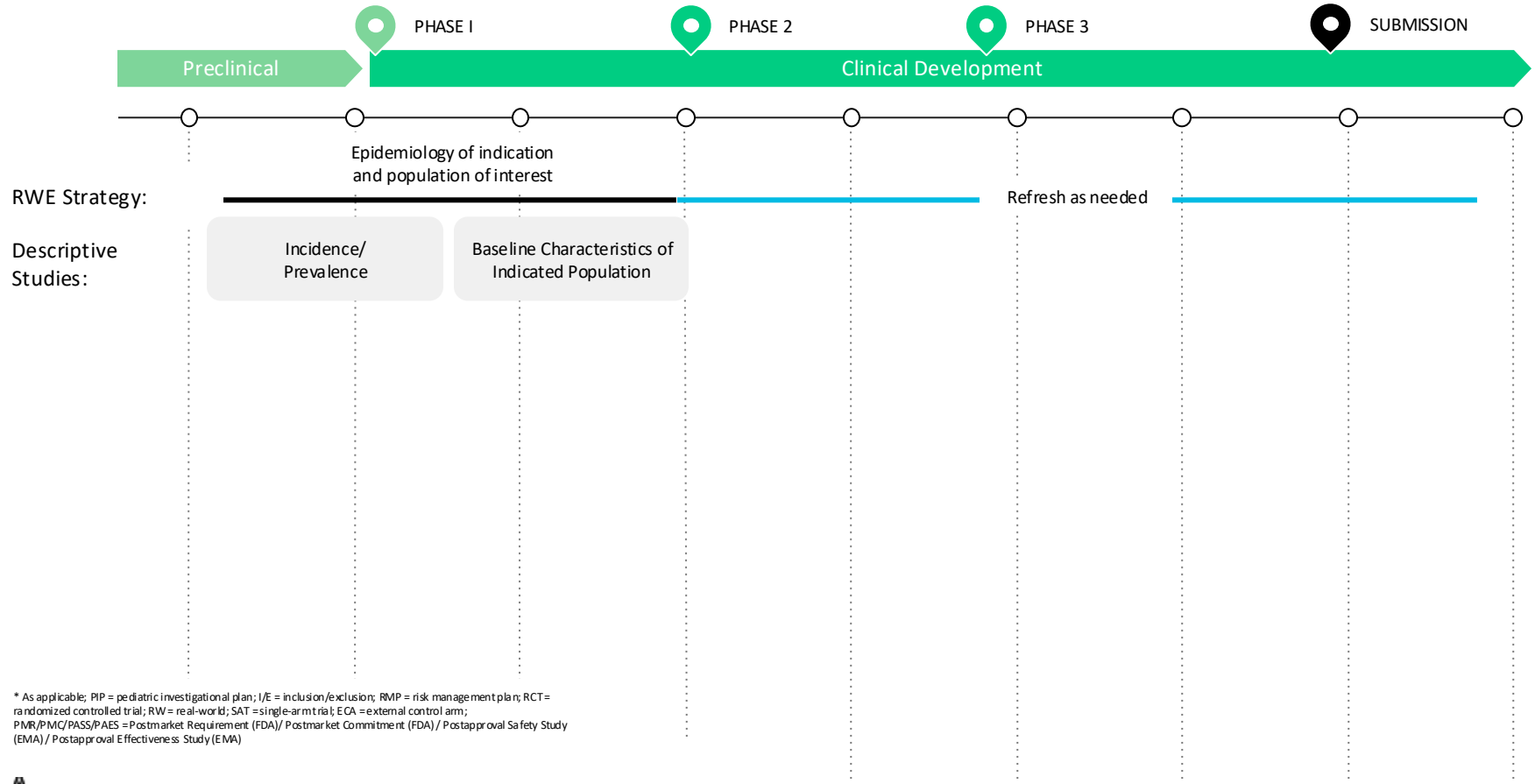
Descriptive RWE studies serve as the foundation for decisions across development and commercialization



Early & robust RWE supports evidence-based decision making



Suite of Descriptive RWE Studies Empowers Your Organization to Make Timely and Robust Clinical Development Decisions



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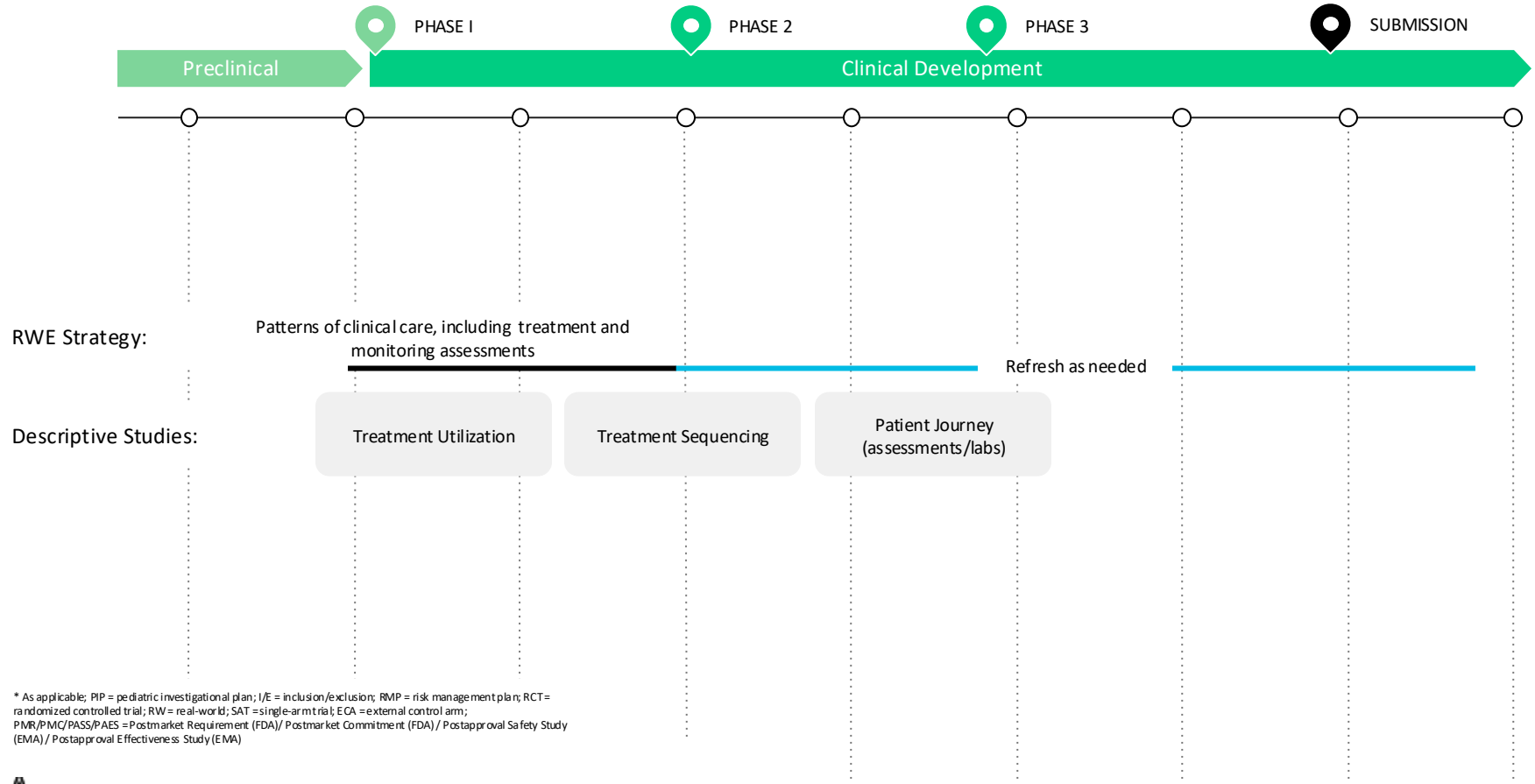

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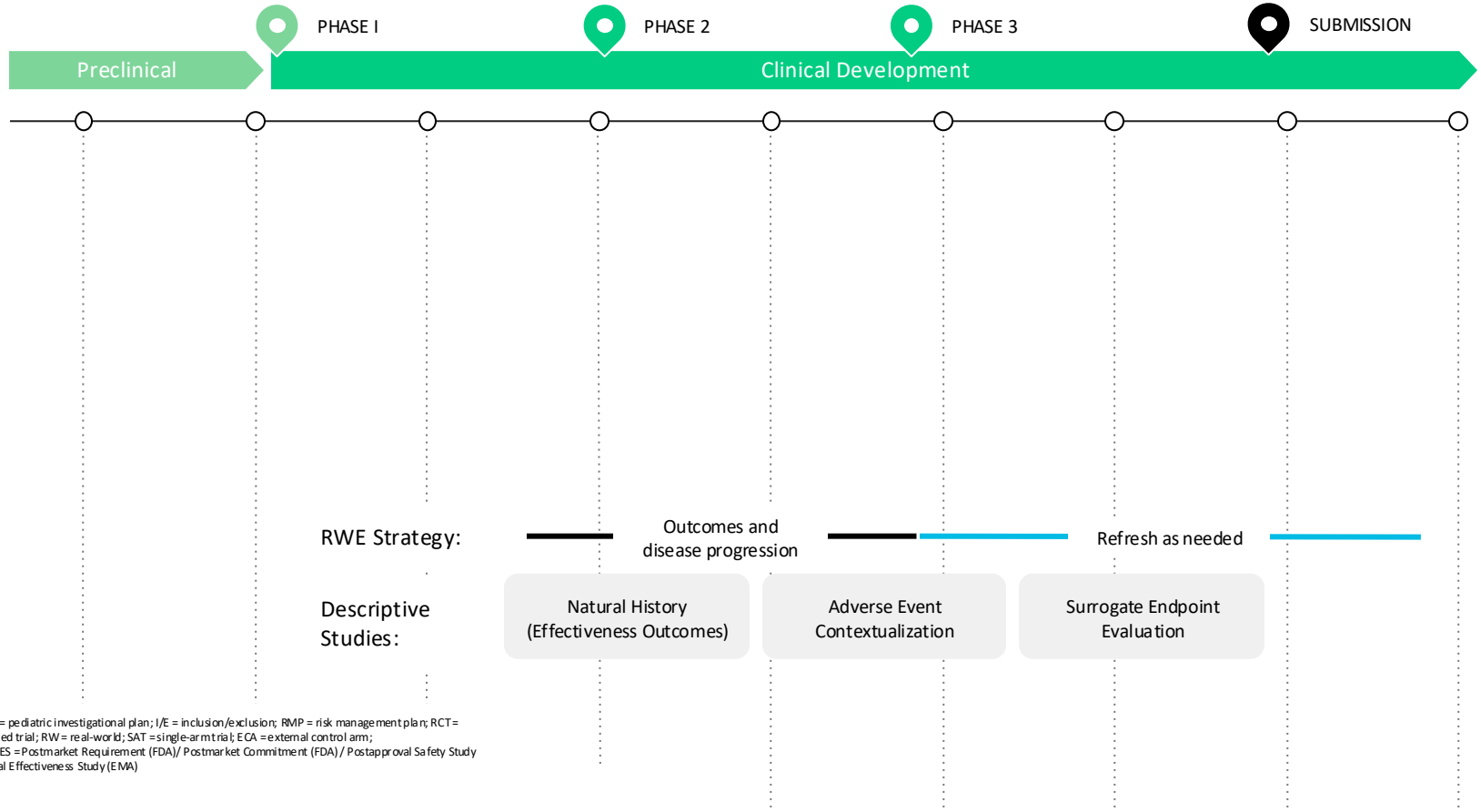

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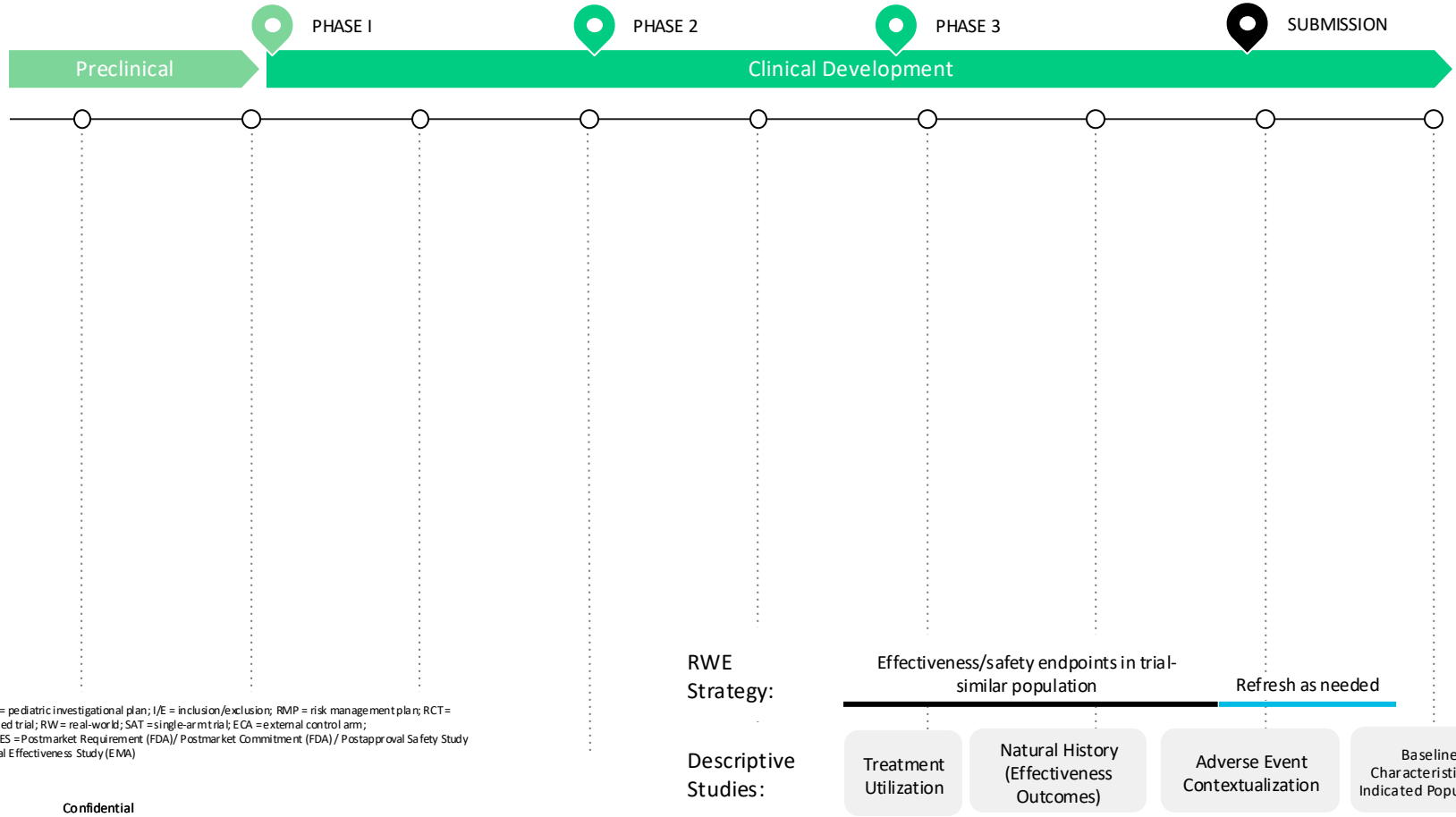

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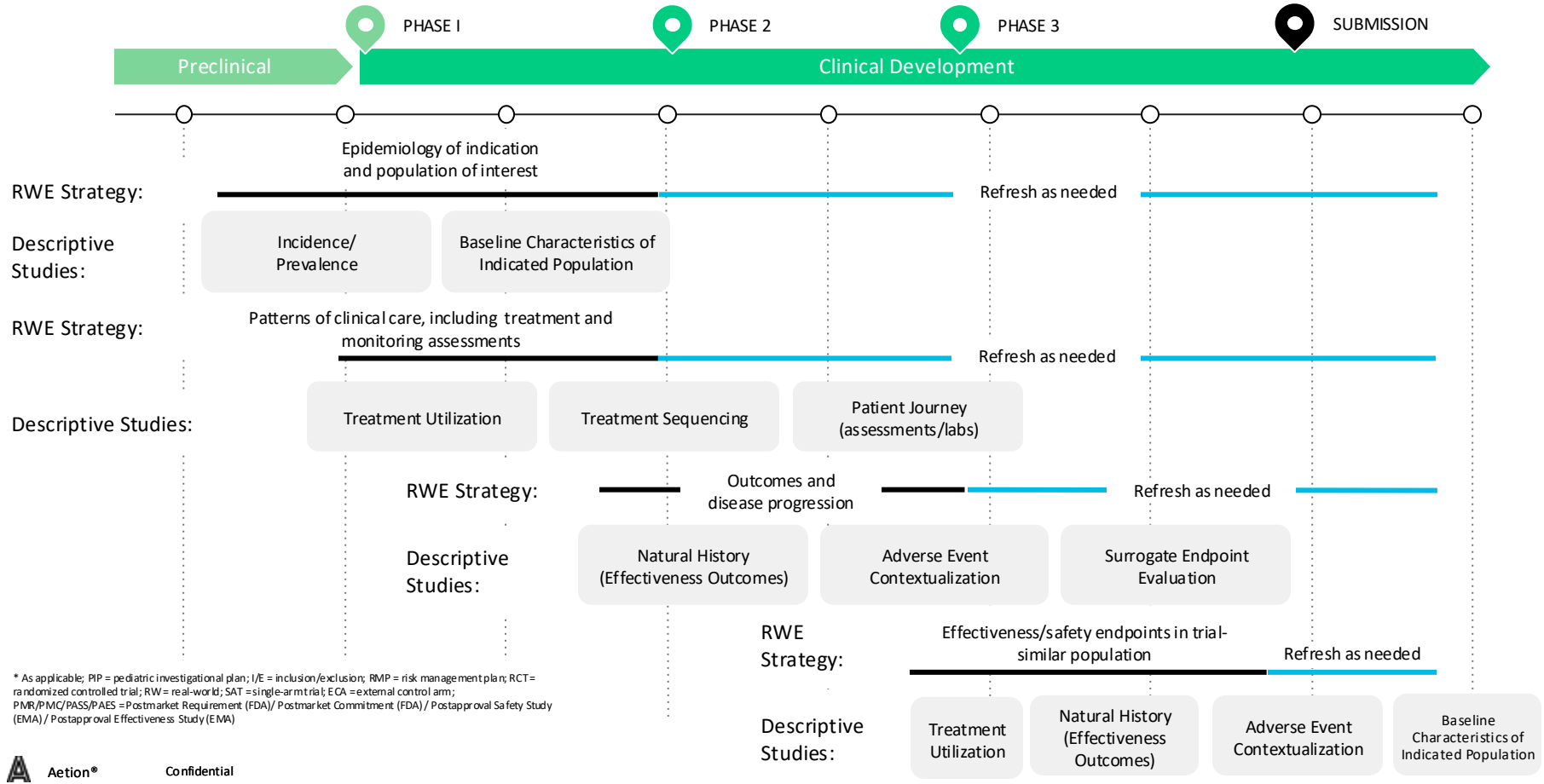

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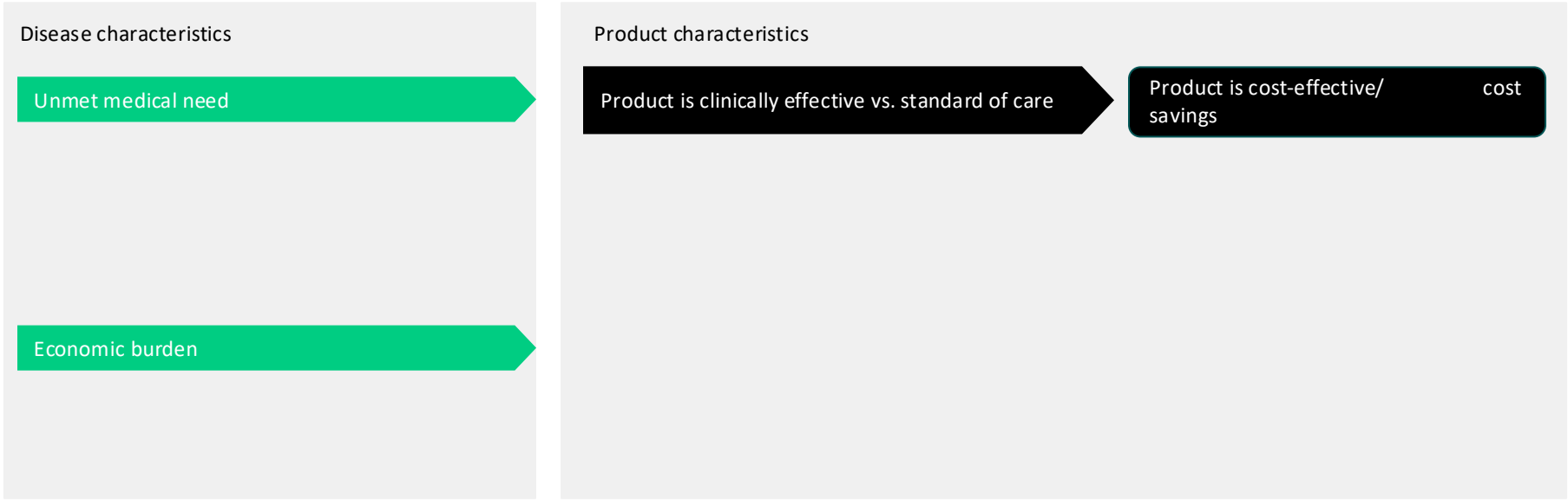
RWE’s foundational role in demonstrating value

To justify reimbursing a new market entry, sponsors must tell a value story that demonstrates:



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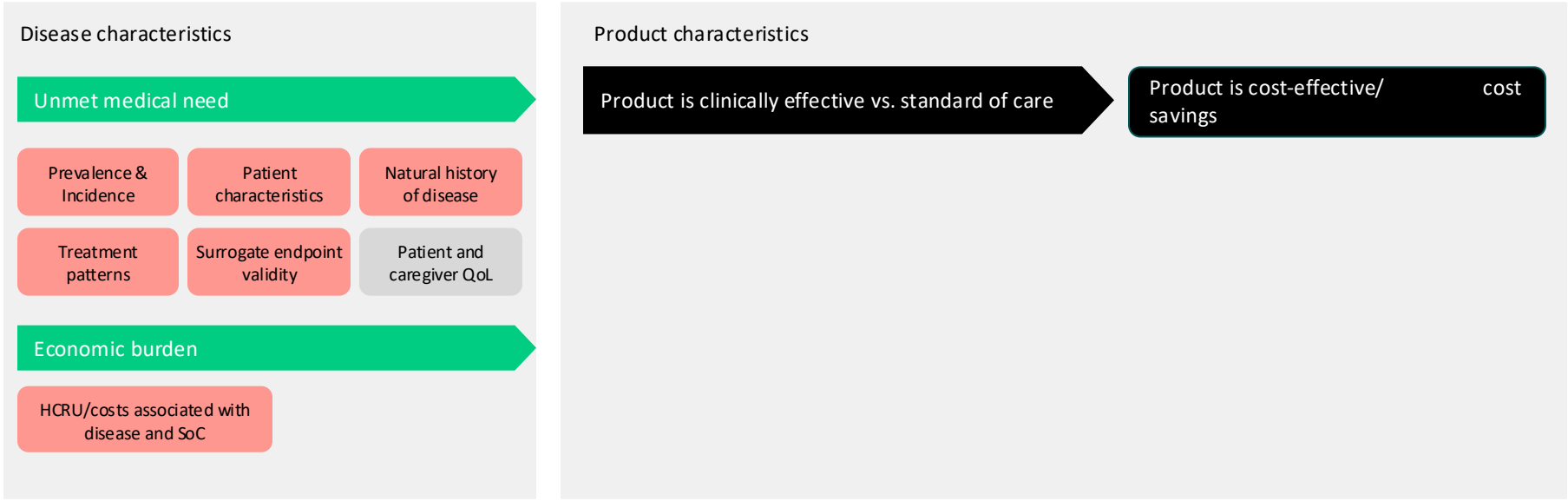

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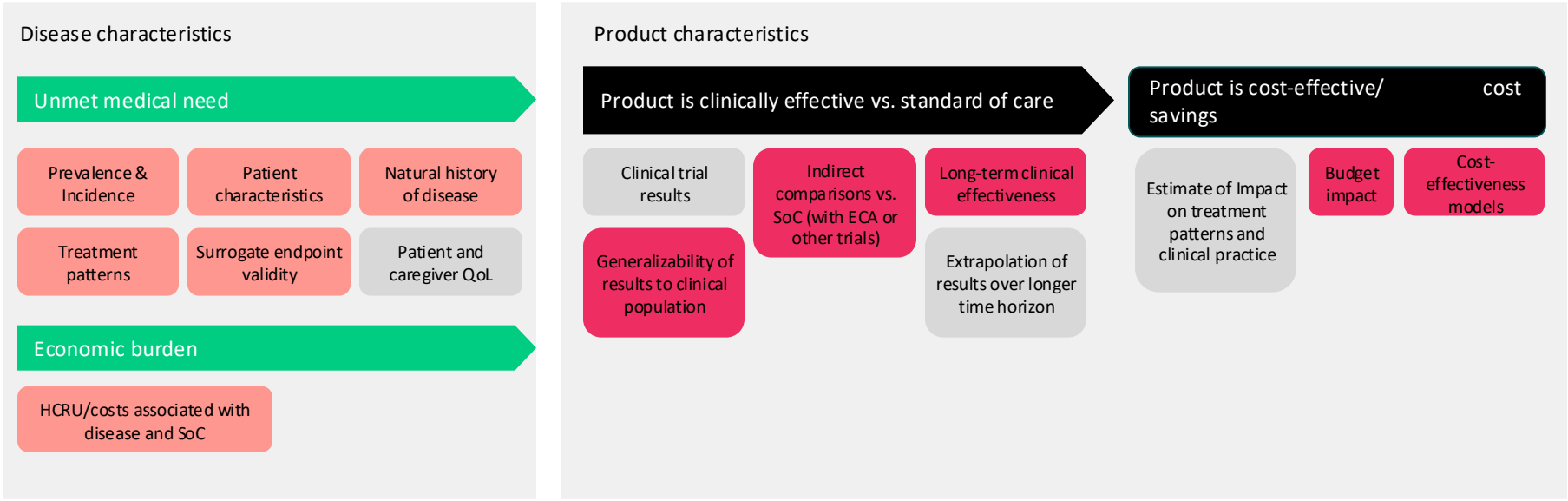

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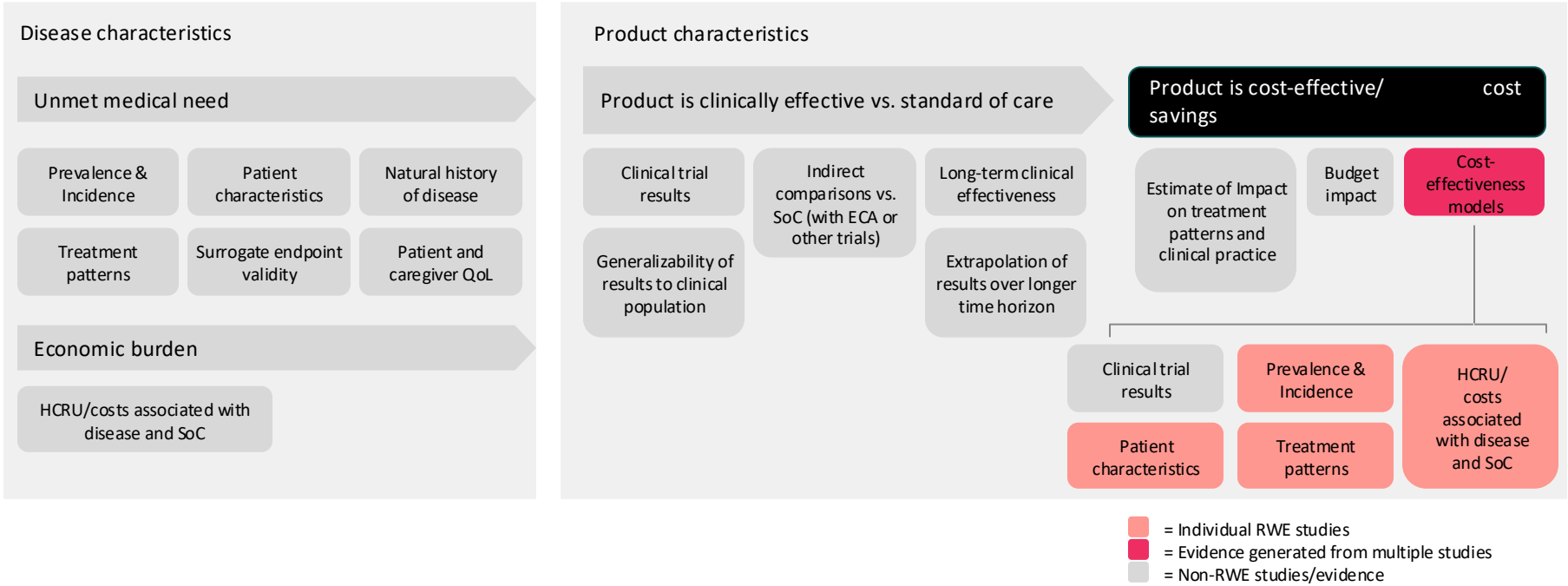

CASE STUDY: How RWE studies enabled future value story discussions for rare autoimmune product

SCENARIO

- Customer developing product for rare autoimmune condition
- Standard of care can control symptoms for some patients, but inadequate symptom control leads to exacerbations which can require hospitalization and expensive therapies like IVIG
- Customer needs to demonstrate unmet need with current available therapies to warrant reimbursement for a targeted immunotherapy

RWE STRATEGY

- Descriptive studies of treated patients to demonstrate clinical and economic burden of disease associated with exacerbations
- Initiated the study in early phase III

ROBUST DESCRIPTIVE RWE STUDIES

- HCRU/costs associated with disease and SoC
- Natural history of disease
- Treatment patterns

RWE IMPACT

- Directly links future RCT results (i.e., reduction in exacerbations) to cost savings for health systems
- Addressed evidence gaps observed by payers/HTA agencies and supports future cost-effectiveness model inputs
- Data used to justify new market entry and price point during pre-launch discussions with payers

CASE STUDY: RWE studies to support launch strategy and optimize reimbursement in a crowded IBD market

SCENARIO

- Crowded IBD market and limited differentiation between sponsor's product and current standards of care
- Needed to identify unmet need in the crowded market to justify new market entry and price point

RWE STRATEGY

- Multiple descriptive studies in claims and registry RWD
- Used RWE to develop model to identify high-risk patients not sufficiently managed on existing treatments
- Initiated in early phase III (>2 years before launch)

ROBUST DESCRIPTIVE RWE STUDIES

- Patient characteristics
- Natural history of disease
- Treatment patterns
- HCRU/costs

RWE Impact

- Analysis identified unmet need in a crowded IBD market
- Informed launch strategy to optimize reimbursement
- RWE studies were used to facilitate pre-launch conversations with payers

Q&A

Thank you

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