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Health Solutions

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Market Access Evidence Roadmaps:
Maximizing Asset Value Through Evidence Generation Planning

Presenters

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Learning Objectives

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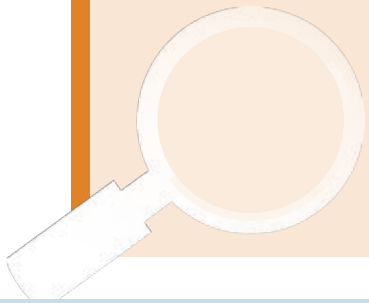
When should you develop a Roadmap and why?



What is an evidence planning Roadmap?

What can the Roadmap do for your asset?

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Abbreviations

		Slide Number(s)
EMA	European Medicines Agency	6, 23
FDA	Food and Drug Administration	6, 23
HTA	Health Technology Assessment	6, 7, 13, 19, 23, 28, 29
QUALY	quality-adjusted life-years	8, 25, 27
EQ-5D	EuroQOL-5 Dimension Questionnaire	8, 14
HEOR	health economics and outcomes research	10, 18
UK	United Kingdom	14, 19
P	payer	14
S	societal	14
CUA	cost-utility analysis	14
CEA	cost-effectiveness analysis	14
SF-36	36-Item Short Form Survey	14
US	United States	19
SLR	systematic literature review	20
Tx	treatment	20
TPP	total product price	24
CE	cost-effectiveness	25, 27, 28
HRQoL	health-related quality of life	26

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Introduction to why you Need a Roadmap

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Vice President,
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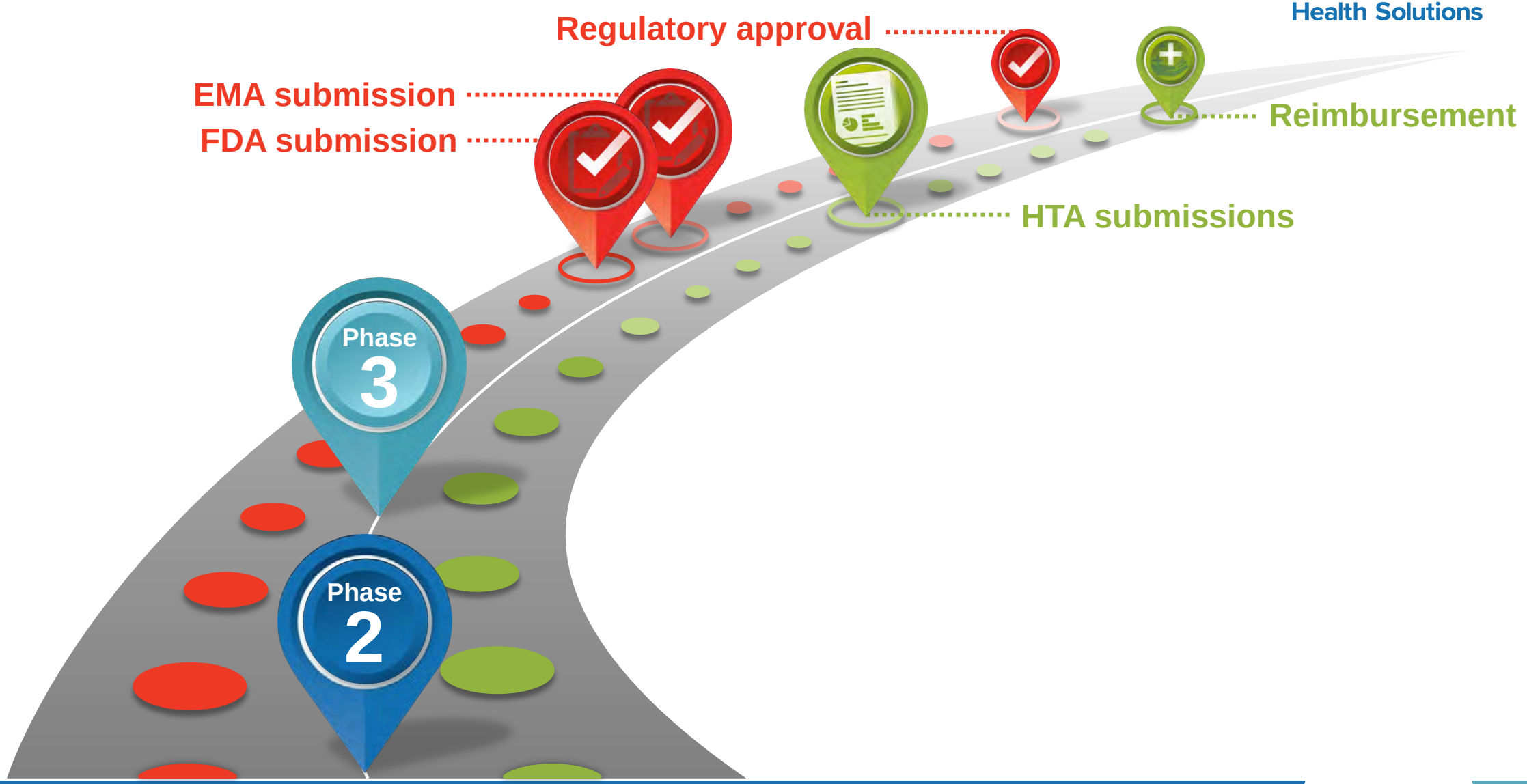
Journey to Market Access Success



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Maximising Value in the Market Place: *Considerations for Reimbursement*

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CLINICAL EFFECTIVENESS

1

- Effectiveness
- Safety
- Quality of life
- Compelling evidence of benefit ***vs. relevant comparators***

VALUE FOR MONEY

2

- Cost-effectiveness vs. comparators
- Value-based price

AFFORDABILITY

3

- Budget impact
- Size of patient population / budget impact can influence which HTA process applies

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Quality-Adjusted Life-Years

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Single, payer-relevant measure of patient health benefit, capturing differences in survival and quality of life, in all health conditions

A QALY is the product of the length of time spent in a particular health state and the utility weight (1 = full health; 0 = dead)

Utility can be measured in a variety of ways, the most common is the EQ-5D instrument

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What is an Evidence Planning Roadmap?

Kati Copley-Merriman
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Outcomes Strategy

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HEOR Roadmap Timing

- For products that might be approved based on phase 2 data, the HEOR Roadmap should start prior to beginning phase 2 trials
- Ideally for all other products, the HEOR Roadmap would begin prior to phase 3, in time to influence the study design

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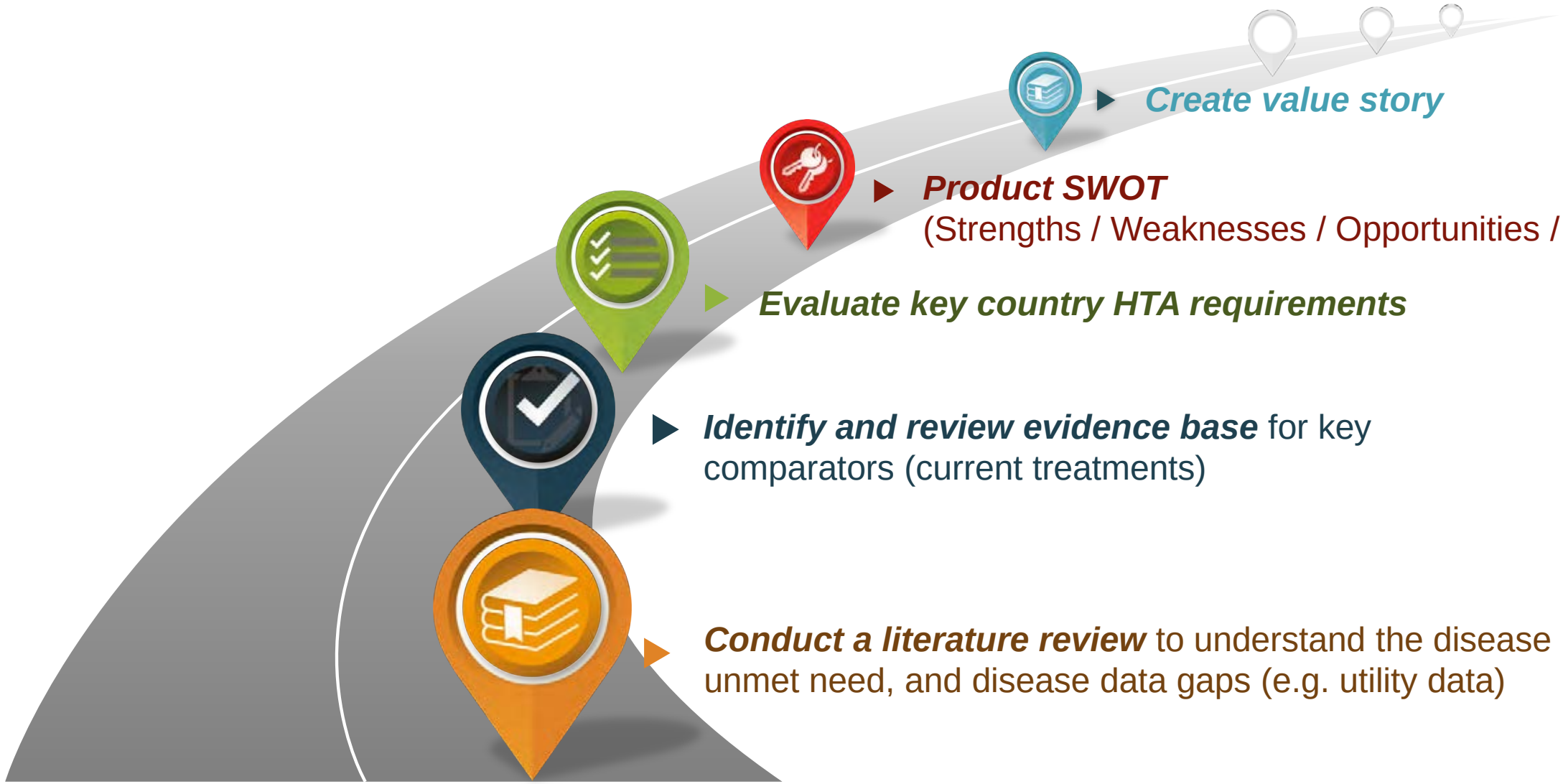
Market Access Evidence Plan Creation Process Overview



Create value story

Product SWOT
(Strengths / Weaknesses / Opportunities / Threats)

Evaluate key country HTA requirements

Identify and review evidence base for key comparators (current treatments)

Conduct a literature review to understand the disease burden, unmet need, and disease data gaps (e.g. utility data)

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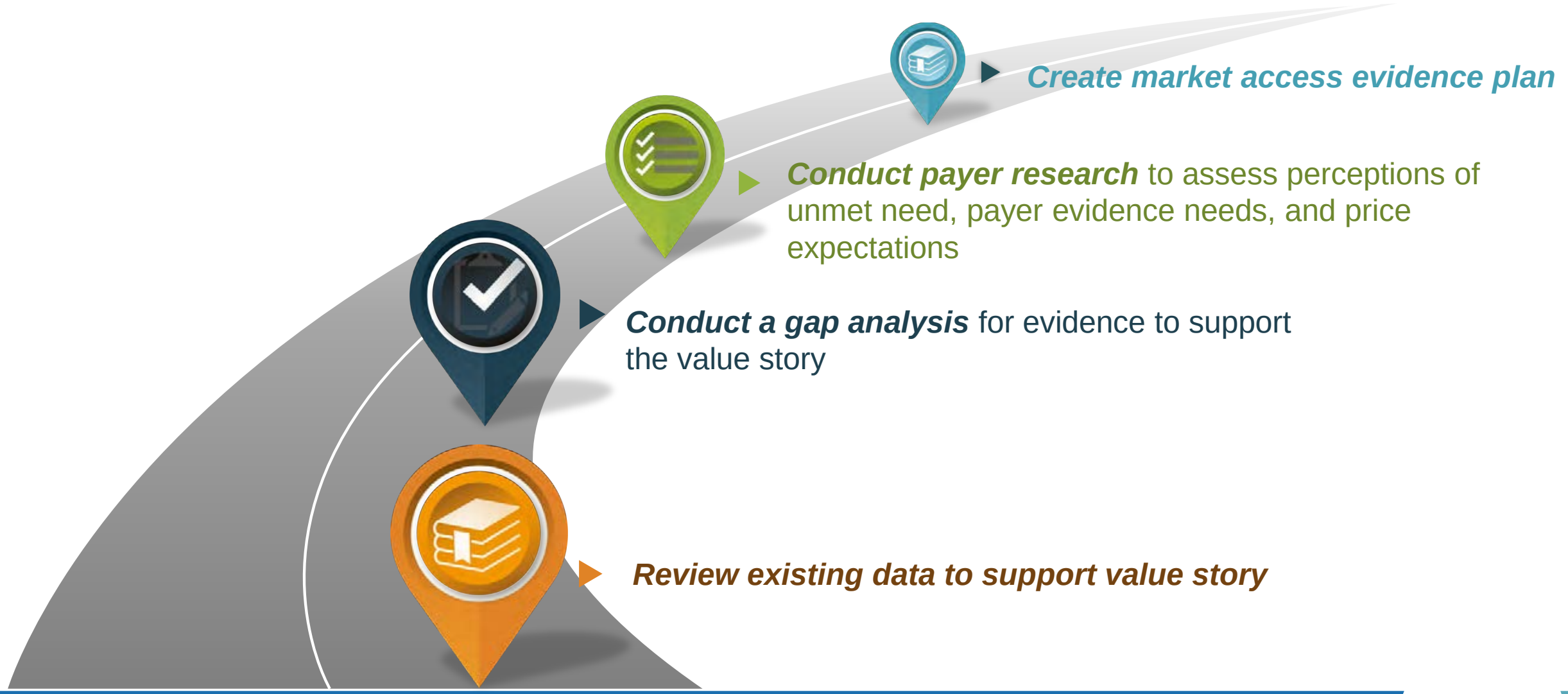
Market Access Evidence Plan Creation Process Overview



Create market access evidence plan

Conduct payer research to assess perceptions of unmet need, payer evidence needs, and price expectations

Conduct a gap analysis for evidence to support the value story

Review existing data to support value story

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Elements of HTA / Pricing & Reimbursement Submission (Reflecting the Value Story)

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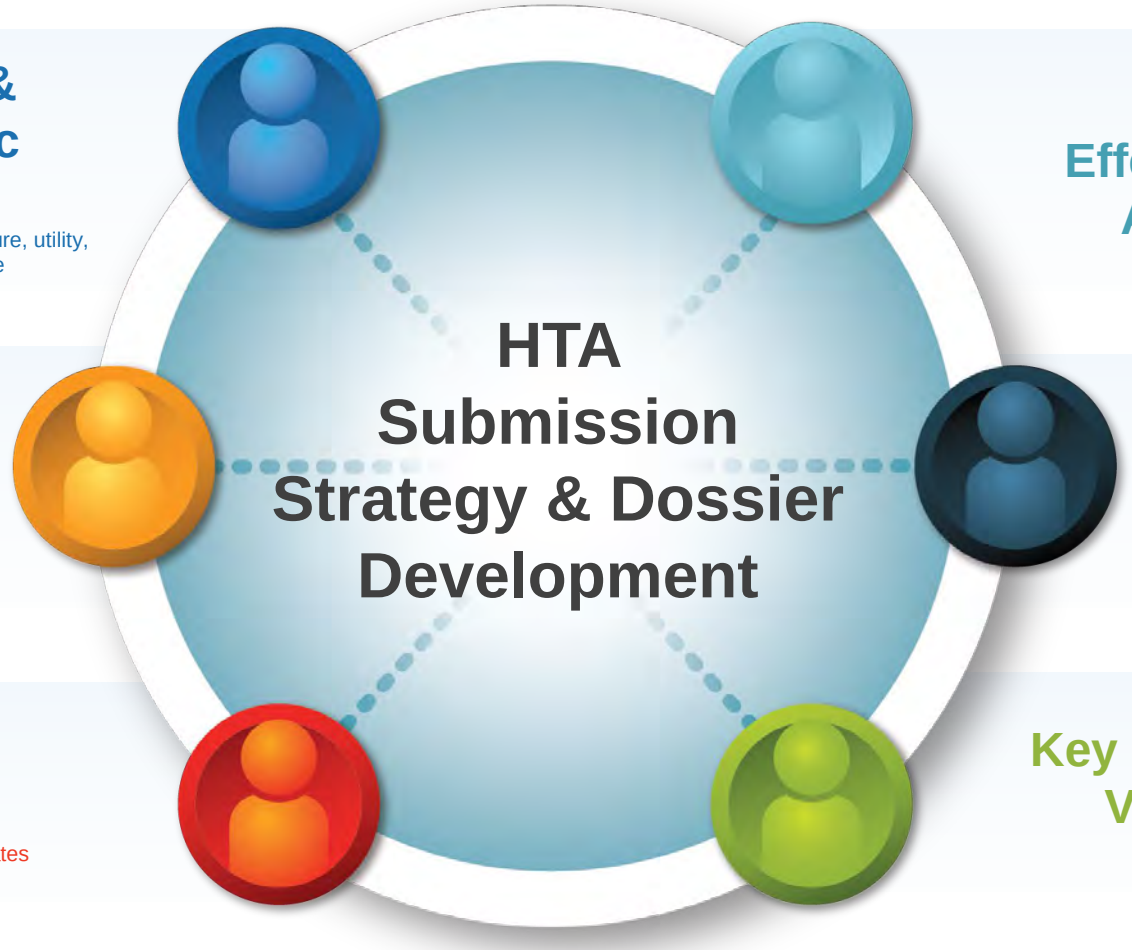
Targeted & Systematic Reviews
Burden of illness, clinical literature, utility, and economic literature

Network Meta-Analysis
Bayesian & frequentist methods

Utility Research
and resource use / cost estimates



Cost-Effectiveness Analysis

Budget-Impact Analysis

Key Stakeholder Validation

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Country-Specific HTA Requirements in Europe

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Assessment Criteria / Tools	Country						
	Germany	UK	France	Italy	Spain	Netherlands	Nordic Countries[a]
Assessment of therapeutic benefit	✓	✓	✓	✓	✓	✓	✓
Assessment of patient benefit	✓	✓	✓	✓	✓	✓	✓
Perspective	P	P	P	S/P	S/P	S	S
Cost-effectiveness model	—	CUA	CUA	CEA, CUA	CEA, CUA	CEA, CUA	CEA, CUA
Budget-impact model	Cost calculation	✓	✓	✓	✓	✓	Cost calculation
Therapeutic alternatives	✓	✓	✓	—	—	✓	✓
Systematic literature reviews	✓	✓	✓	✓	✓	✓	✓
Quality-of-life evaluation	—	EQ-5D	EQ-5D	EQ-5D	EQ-5D / SF-36	EQ-5D	EQ-5D
Dossier required	✓	✓	✓	✓	✓	✓	✓
Reference pricing required in dossier	Supportive information	No	Main criterion	Supportive information	Supportive information	Main criterion	Sweden: no Denmark, Finland, Norway: yes
Time from submission to reimbursement	0 (12-month free pricing)	180-250 days	180-250 days	180-250 days	180-400 days	90-400 days	180 days

[a] The Nordic countries include Denmark, Finland, Norway, and Sweden.

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SWOT Analysis: *Example*

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Strengths

- PRODUCT X is durable and has relatively quick onset and high response rate.
- PRODUCT X has a good safety profile similar to placebo.
- Phase 3 randomized trial with long-term follow-up.

Opportunities

- There is high unmet need.
- PRODUCT X will be the first approved treatment.
- Current treatment is a complex risky procedure.

Weaknesses

- PRODUCT X is rare and not well understood by payers.
- Epidemiology is not well studied and is affected by misdiagnosis.
- Natural history is not well understood.
- Economic burden had been studied in a few studies, but data are limited.

Threats

- PRODUCT X competitor has started phase 3 trials.
- PRODUCT X competitor is a once-daily oral tablet; PRODUCT X is intravenous administration.
- European markets have tougher reimbursement environments.

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Value Messages: *Example*

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Efficacy Messages

- The percentage of responders is higher after 2 weeks, 3 months, and 6 months of treatment with PRODUCT X, compared with placebo.
- Total body fat mass in overweight / obese patients is reduced after 3 and 6 months of treatment with PRODUCT X, compared with placebo, and is maintained for at least X months.

Safety Messages

- Treatment with PRODUCT X over 12 months shows no clinically significant findings in adverse events, compared with placebo.
- Treatment with PRODUCT X is well tolerated, and adverse events are mild.

Economic Value Messages

- PRODUCT X is cost-effective compared with placebo (no treatment).
- PRODUCT X has a low budget impact because the disease is rare.

HRQOL Improvement Messages

- Clinically meaningful improvement in quality of life has been shown after x weeks of treatment with PRODUCT X vs. a worsening with placebo.
- Time to deterioration is longer with PRODUCT X vs. placebo.

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Gap Analysis: *Example*

- Some epidemiology data are available but vary by study, possibly due to misdiagnosis; size of the population in different regions is uncertain
- Clinical burden is well established in the literature, but natural history and economic burden has not been well studied over time
- Longitudinal phase 2 / 3 trial design is robust, with several endpoints for reduction of primary disease and comorbidities, patient quality of life, and safety; utility values for economic models is unavailable
- Competitive trials designs are similar but have additional endpoints

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Objectives of Payer / Stakeholder Research

- In parallel to the outputs of the HEOR evidence plan for Product X, assess evidence-generation tactics and price expectations for key markets
- Stakeholder research can address key topics:
  - Market access considerations for the disease in target markets
  - Perceptions of disease burden and unmet need in target disease
  - Importance of clinical trial design endpoints for Product X
  - Pricing expectations based on target product profile
  - Evidence weaknesses and information gaps

Example Scope

United States

United Kingdom

Germany

France

Netherlands

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HEOR Plan Recommended Projects: *Example*

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Statements / Evidence Needed	Data Source	Country	Start Date / Study Length / Price Estimate	Strategy Objective Addressed
Early HTA advice	Letter of intent 3 months prior to building economic models	Europe	Q3, 2020 6-8 months $XX,XXX	Gain strategic input from country HTAs
Real-world burden of disease and treatment patterns	Database study or disease registry / partner with disease associations	US, UK, and others	Q3, 2020 $XX,XXX	Understand the burden of disease and current treatments
Early economic model	Economic model	US	Q4, 2020 $XX,XXX	Understand model data gaps and pricing implications
…	…	…	…	…

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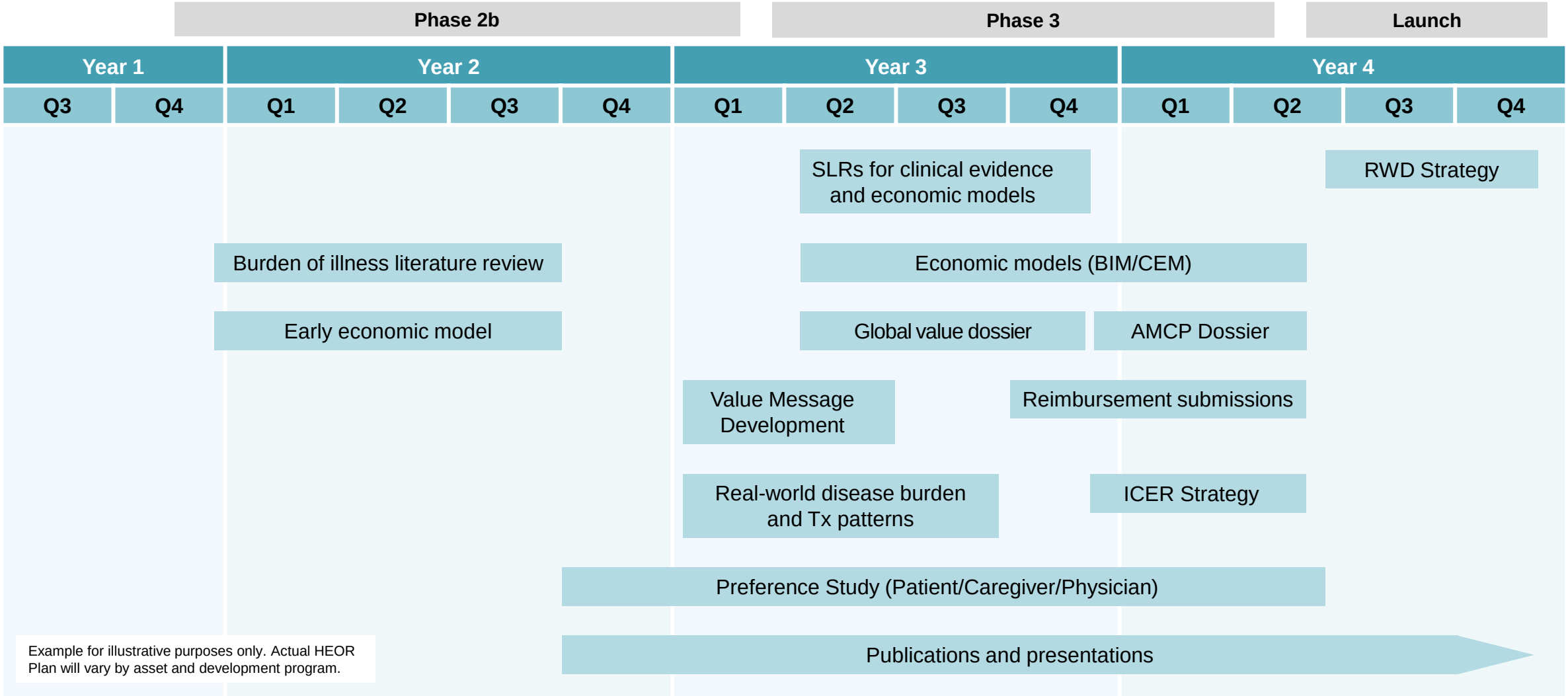
Timeline of Activities for the HEOR Plan: *Example*



Example for illustrative purposes only. Actual HEOR Plan will vary by asset and development program.

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Market Access Pitfalls and the Value of a Roadmap.

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How can a Market Access Evidence Plan Support the Value of a Pipeline Product?

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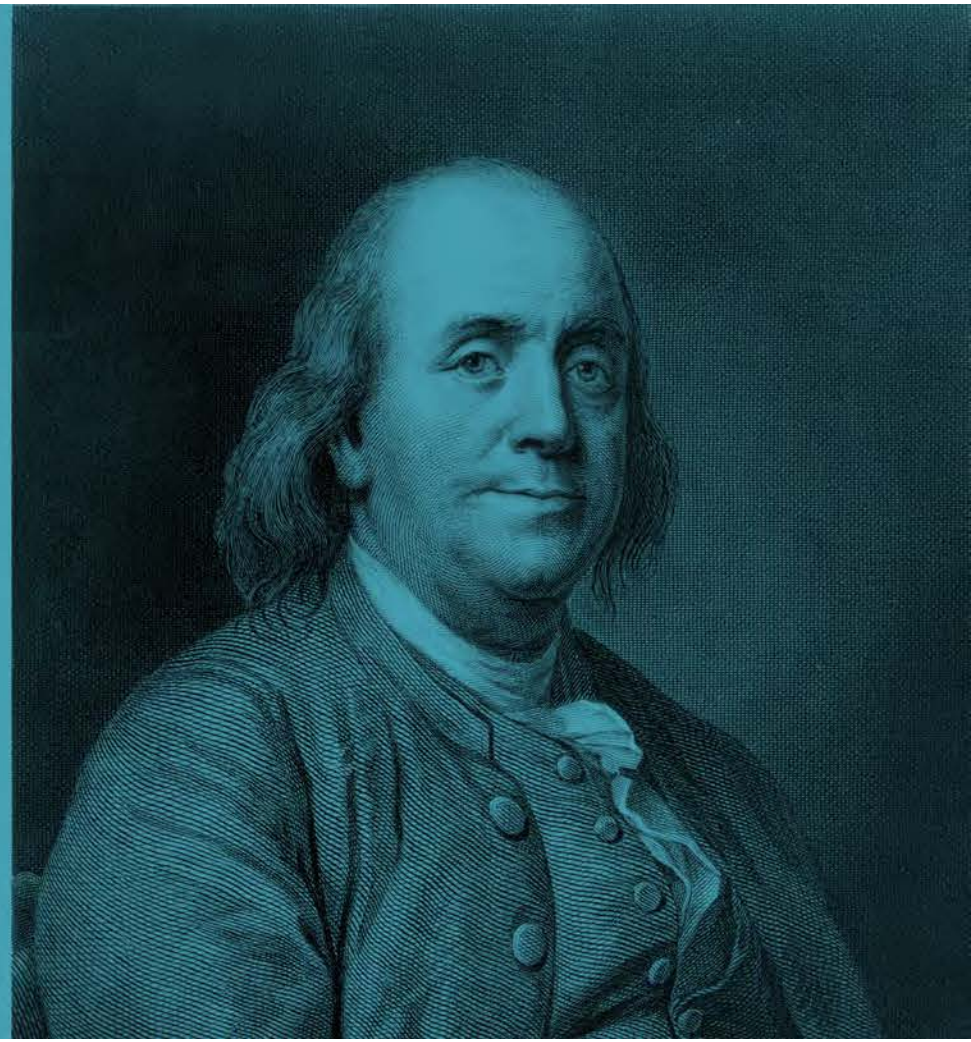
"If you fail to plan,
you are planning to fail!"

BENJAMIN FRANKLIN



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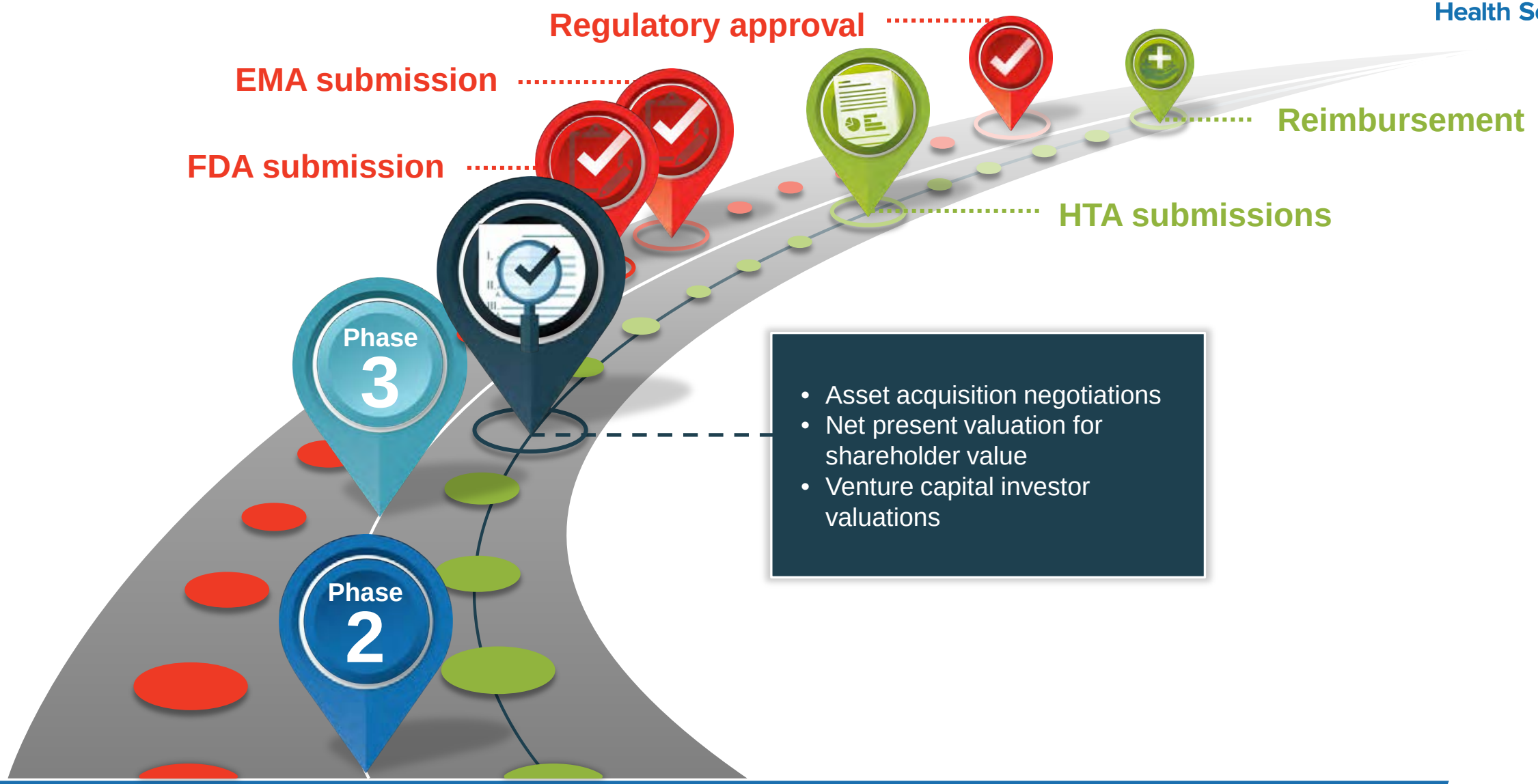
The Value of an Evidence Roadmap



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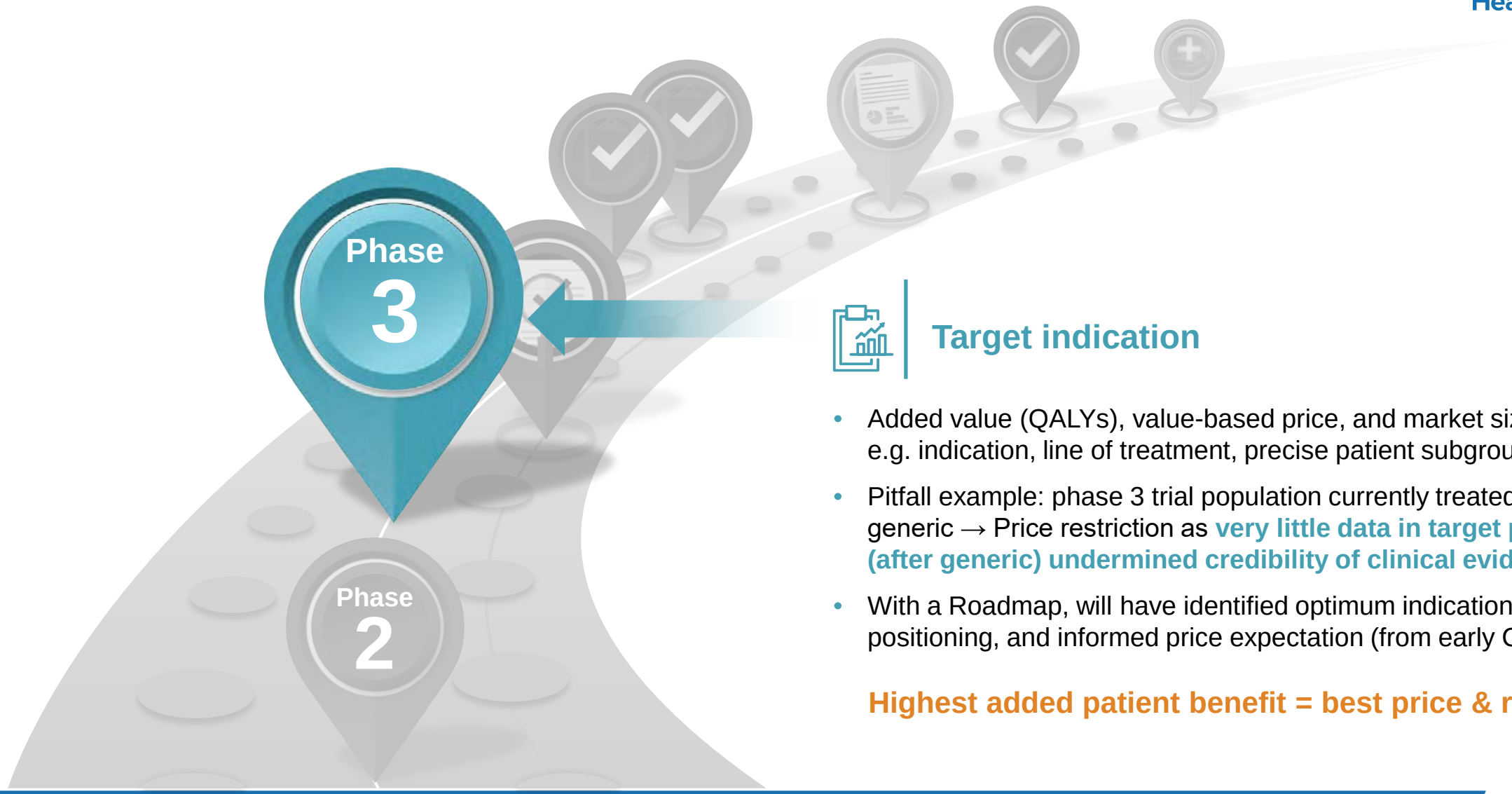

Target indication

- Added value (QALYs), value-based price, and market size differ by, e.g. indication, line of treatment, precise patient subgroup
- Pitfall example: phase 3 trial population currently treated with generic → Price restriction as **very little data in target positioning (after generic) undermined credibility of clinical evidence**
- With a Roadmap, will have identified optimum indication / positioning, and informed price expectation (from early CE model)

Highest added patient benefit = best price & reimbursement

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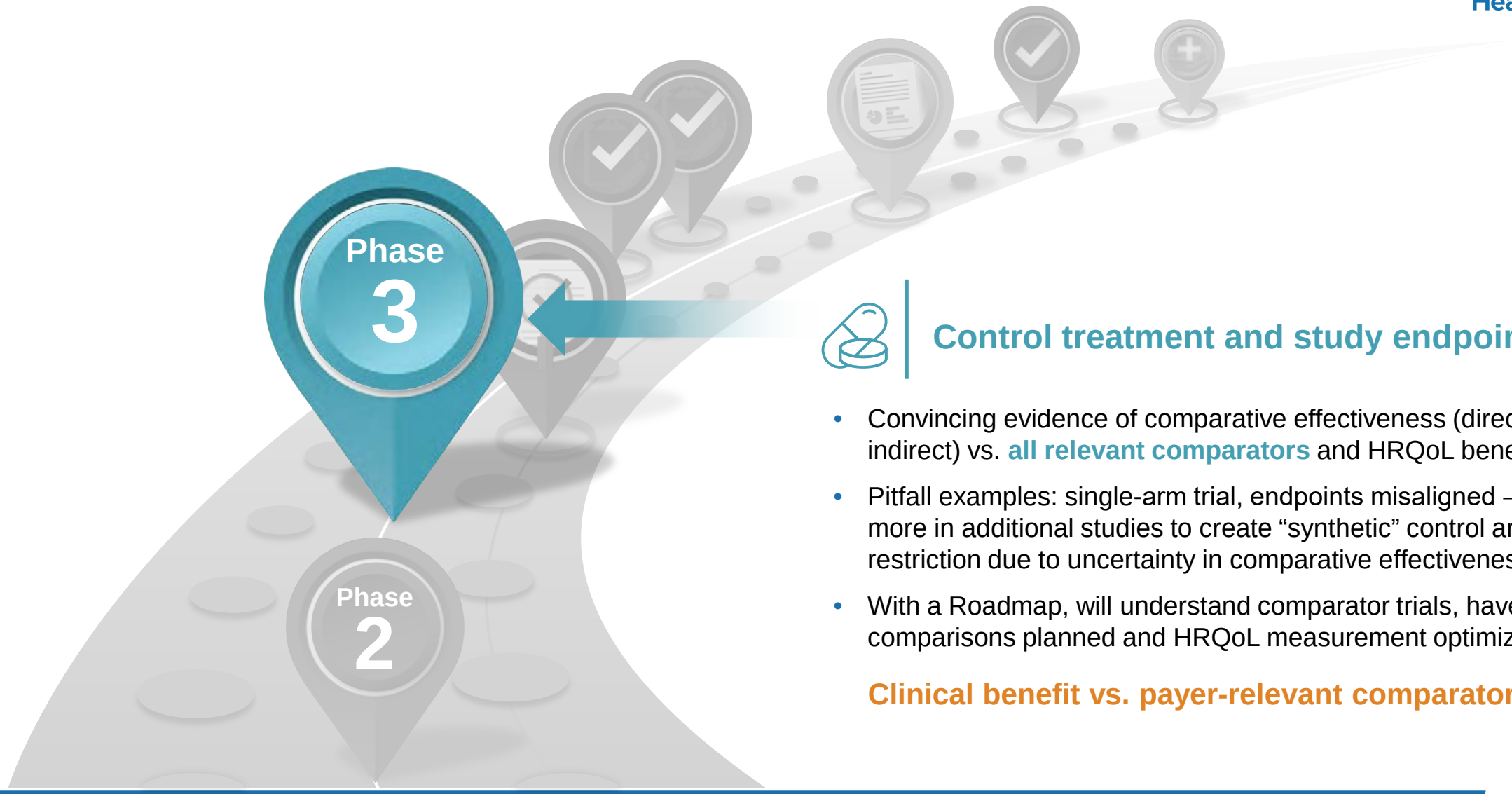
The Value of an Evidence Roadmap



Control treatment and study endpoints

- Convincing evidence of comparative effectiveness (direct or indirect) vs. **all relevant comparators** and HRQoL benefit
- Pitfall examples: single-arm trial, endpoints misaligned → Cost more in additional studies to create "synthetic" control arm; price restriction due to uncertainty in comparative effectiveness
- With a Roadmap, will understand comparator trials, have indirect comparisons planned and HRQoL measurement optimized

Clinical benefit vs. payer-relevant comparators

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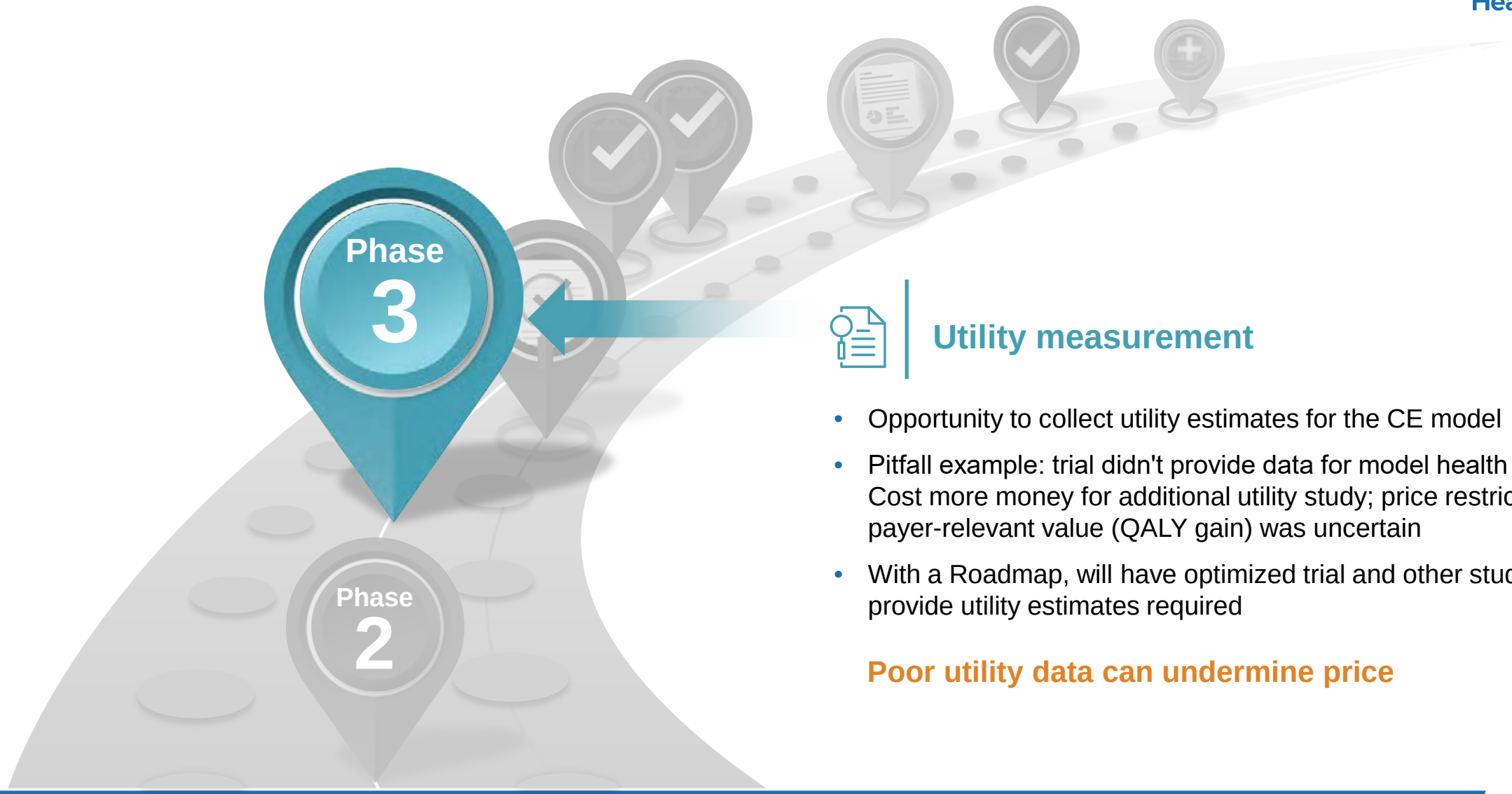

Utility measurement

- Opportunity to collect utility estimates for the CE model
- Pitfall example: trial didn't provide data for model health states → Cost more money for additional utility study; price restriction as payer-relevant value (QALY gain) was uncertain
- With a Roadmap, will have optimized trial and other studies to provide utility estimates required

Poor utility data can undermine price

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HTA research

- Systematic literature review, network meta-analysis, CE model, budget-impact model
- Pitfall example: utility and natural history data gap recognized too late
- With a Roadmap, will have research in good time for submissions

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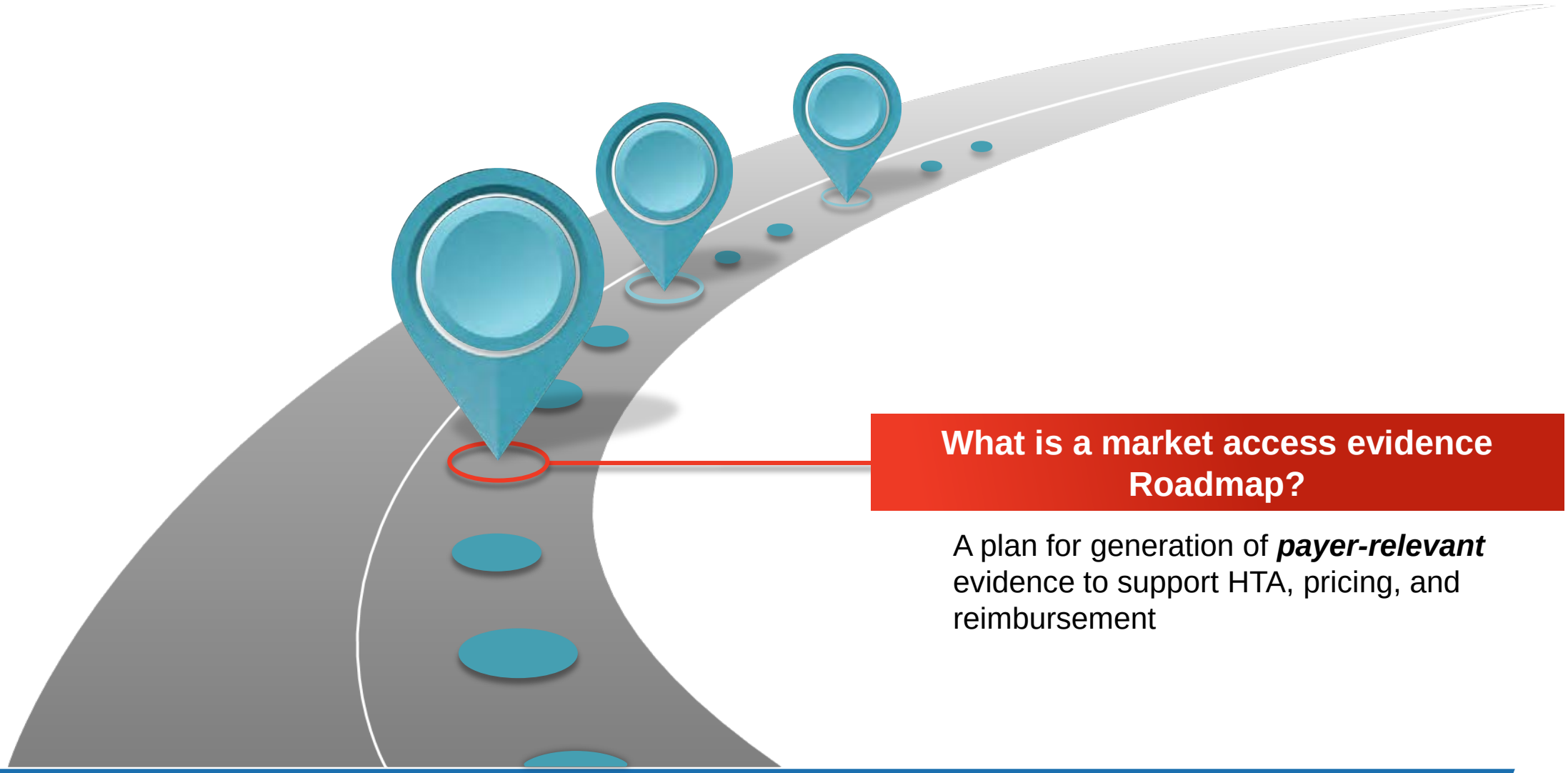
Key Take-Home Messages



What is a market access evidence Roadmap?

A plan for generation of ***payer-relevant*** evidence to support HTA, pricing, and reimbursement

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Key Take-Home Messages

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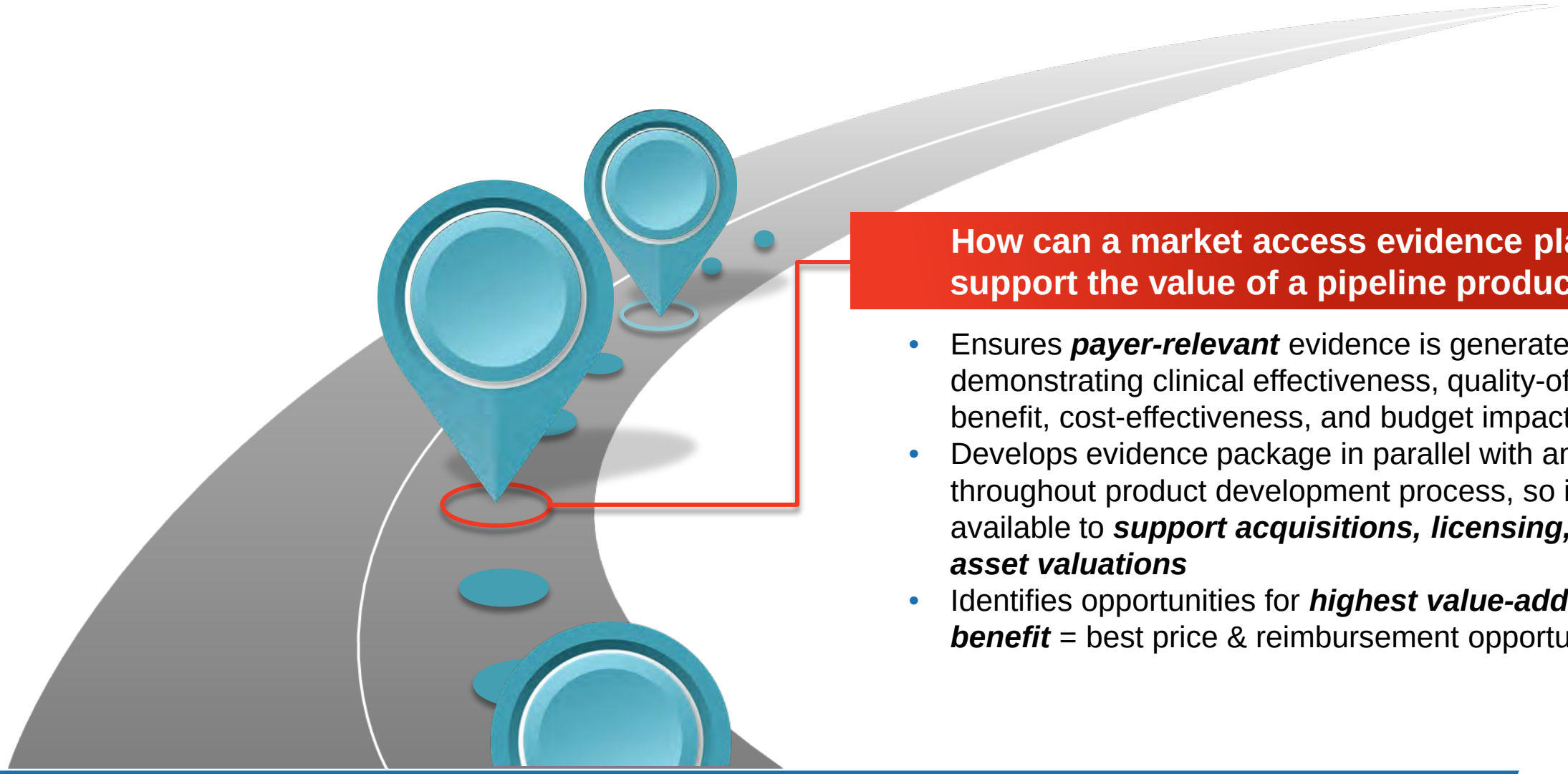

How can a market access evidence plan support the value of a pipeline product?

- Ensures ***payer-relevant*** evidence is generated demonstrating clinical effectiveness, quality-of-life benefit, cost-effectiveness, and budget impact
- Develops evidence package in parallel with and throughout product development process, so it is available to ***support acquisitions, licensing, and/or asset valuations***
- Identifies opportunities for ***highest value-added patient benefit*** = best price & reimbursement opportunity

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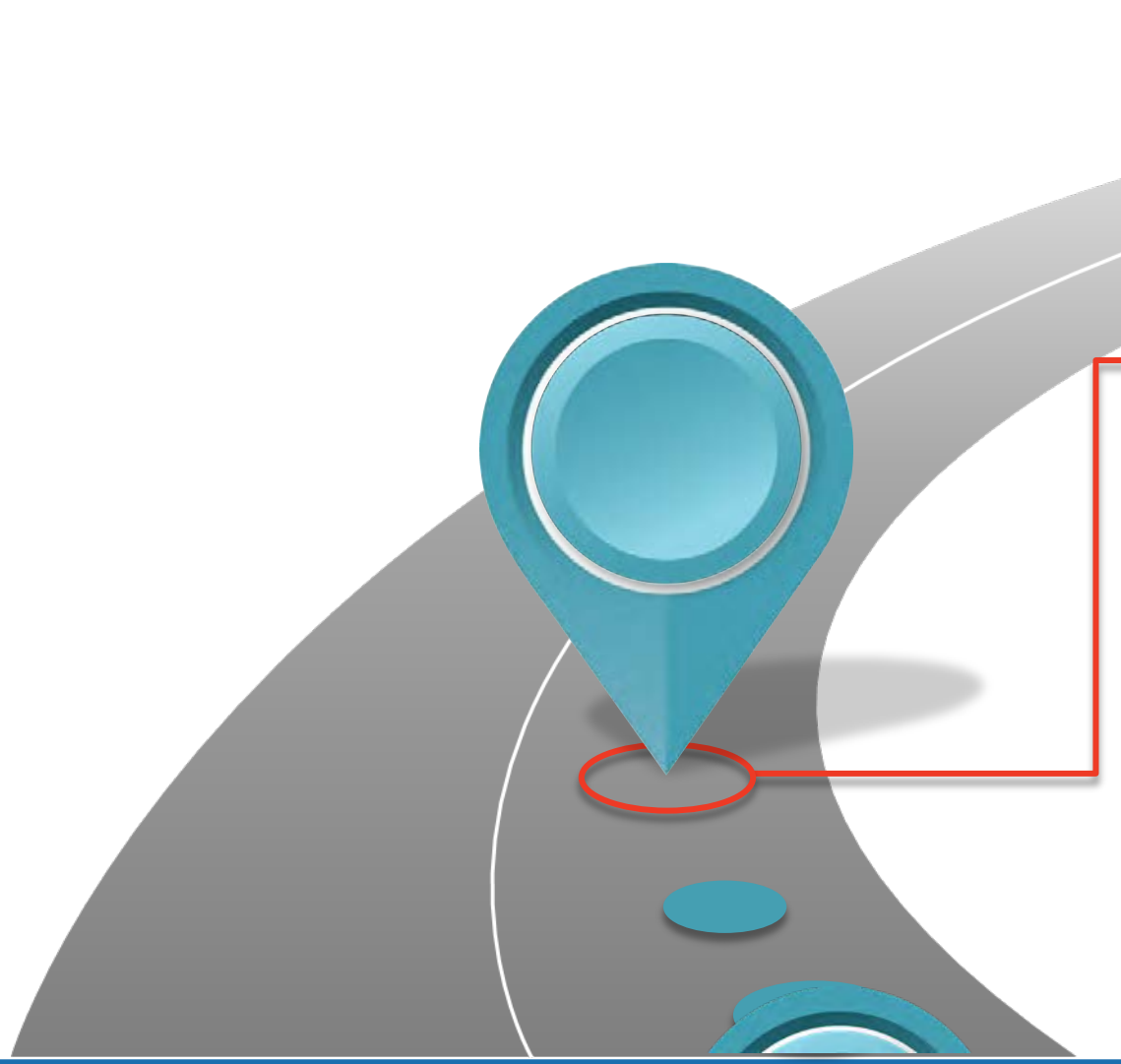
Key Take-Home Messages



When should a Roadmap be developed?

- Ideally start during phase 2 trials
- Still useful in early phase 3
- Update over time to adapt for any changes in the product profile / competitive landscape

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