

Strategic Considerations for Clinical Development Programs in Emerging Biopharma Companies

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Congratulations!



Your scientific hypothesis has been validated in animal studies



You've identified a lead compound or biologic (and back-ups)



You've understood some preliminary animal pharmacology and have negative preliminary toxicology findings



You've decided to start (or have begun) human clinical testing!



*... and your Investors are thrilled, but daunted
(because now the “fun” starts)*

Why are you here today?

What we will discuss:

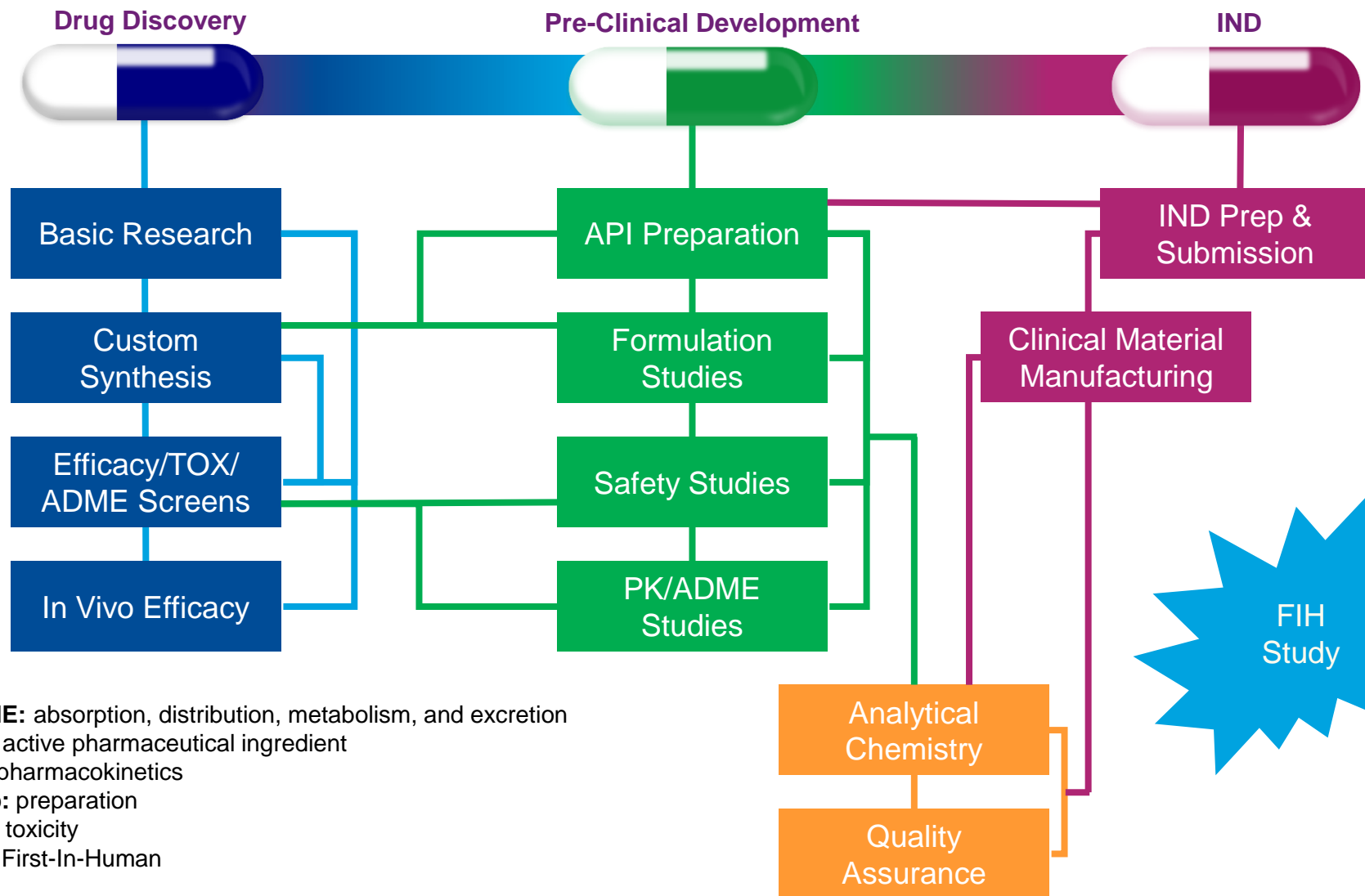
- The importance of designing your clinical program with the end in mind, from First-in-Man through approval and commercialization
 - Perspectives: Investors vs. stakeholders vs. market
 - Balancing risk with investment opportunity
 - Understanding the specific landscape and market for your product
 - Thinking ahead without investing major resources
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Key takeaways:

- Recognize the opportunity: Align clinical & commercial intentions
- Design to build evidence: Integrate key program elements to inform stakeholder requirements
- Optimize execution & operations: Balance current spend against future return by leveraging capabilities of a partner
- Deliver value: Planning for the future now helps optimize ROI

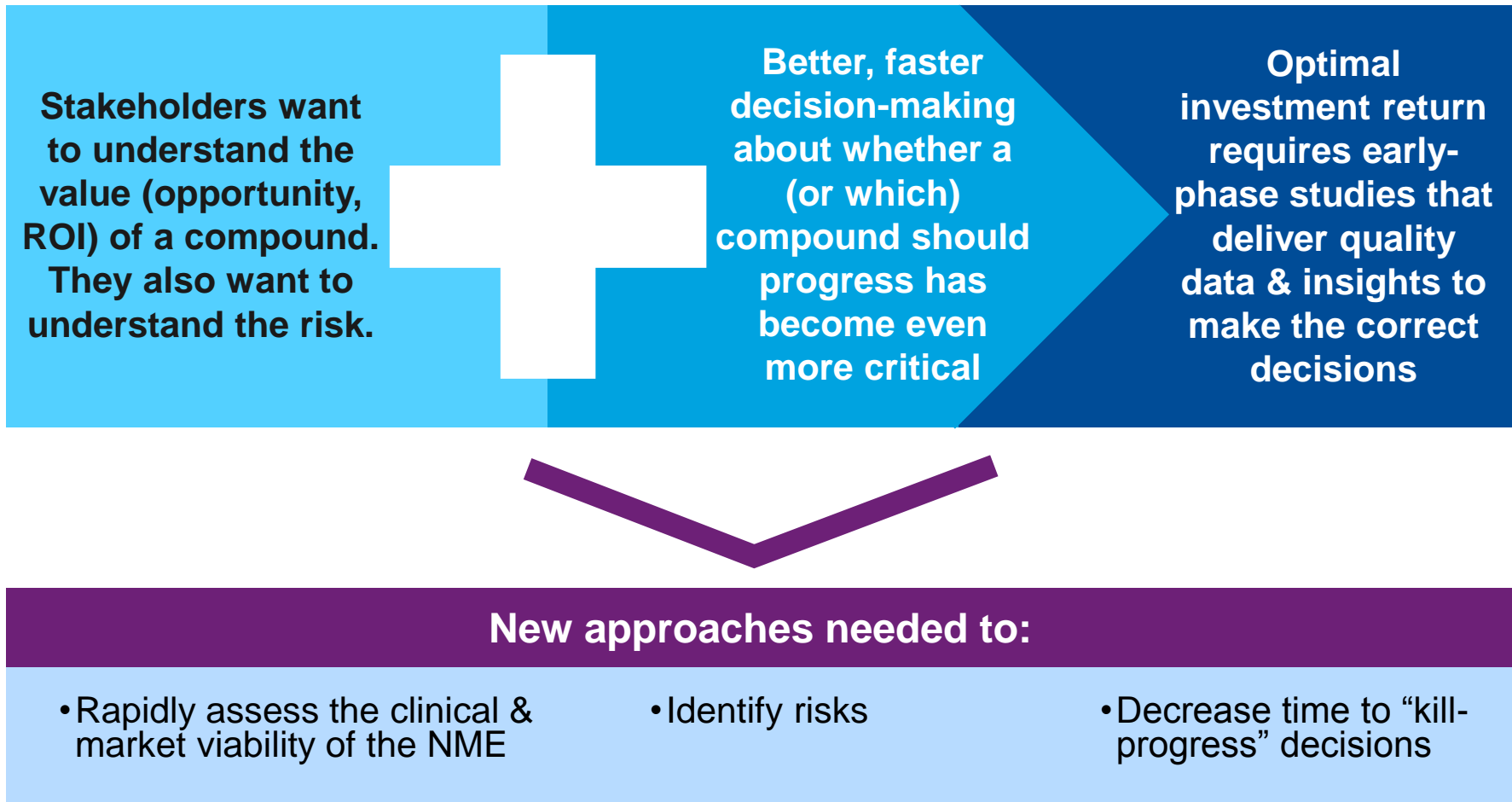
Activities of “Today”: Pre-clinical development

The next investment (\$7-10M) frontier...



Early clinical development realities

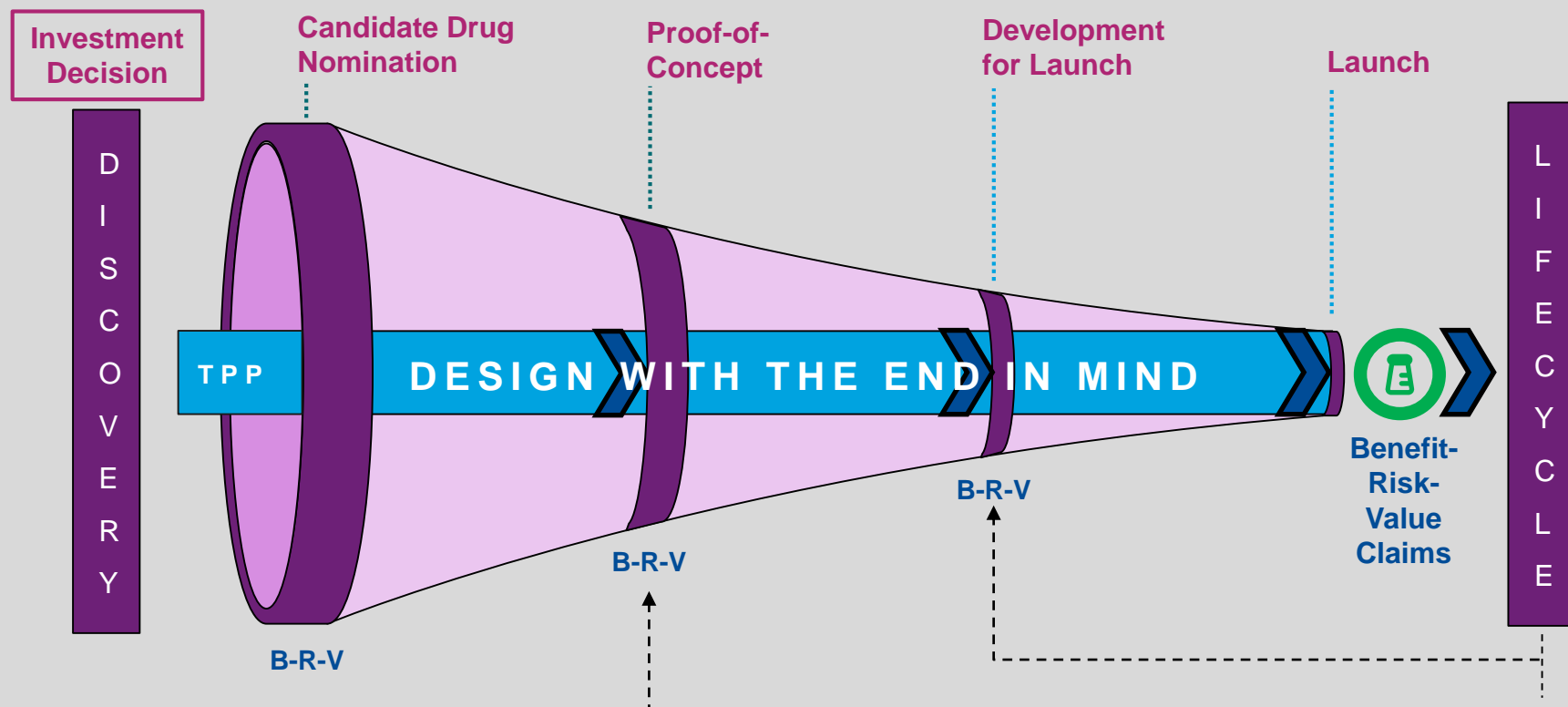
An investor's (and potential partner's) perspective



An investor's/partner's “long-haul” perspective

Capitalized costs per new drug approval: \$1460M

	Mean 80.8 mo.			16 mo.	
Phase	I	II	III	Submission-Launch	
Mean Out-of-Pocket Cost (\$M)	\$25.3	\$58.6	\$255.4	\$44M	\$965
Phase Transition Probability	59.5%	35.5%	61.9%	90.4%	11.8%

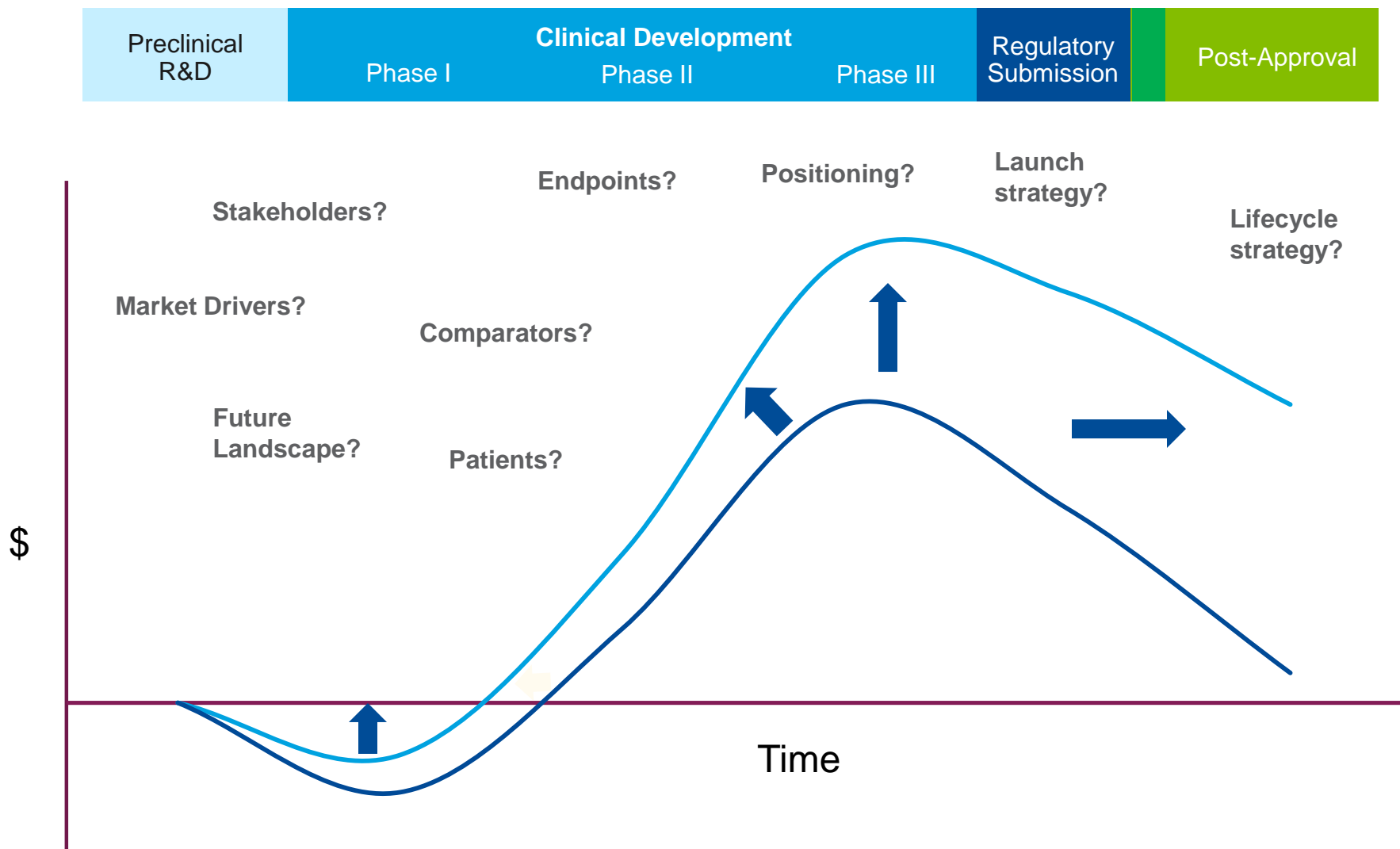


Polling question #1

Which statement below best fits your team's understanding of "long haul" point of view on your product and the incorporation a "design with the end in mind" principle in planning activities?

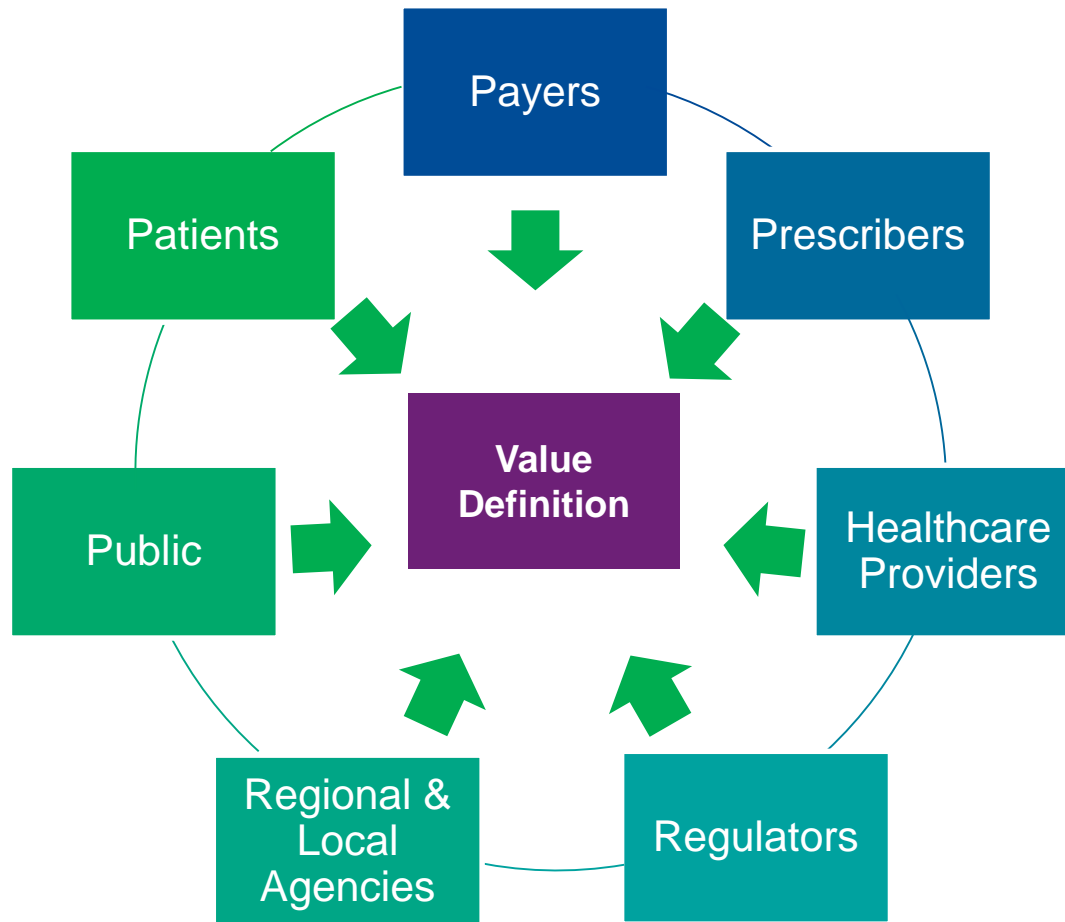
- › We have a strong understanding of the long haul view and actively incorporate it into our design principles and planning activities.
- › We have a strong understanding of the long haul view, but struggle to bring it into design principles and planning activities.
- › We are working towards better understanding of this view, and expect to incorporate it into our design principles and planning activities this year.
- › Our view of the long haul is limited and therefore not a part of our design principles and planning activities.

Value creation in biopharma

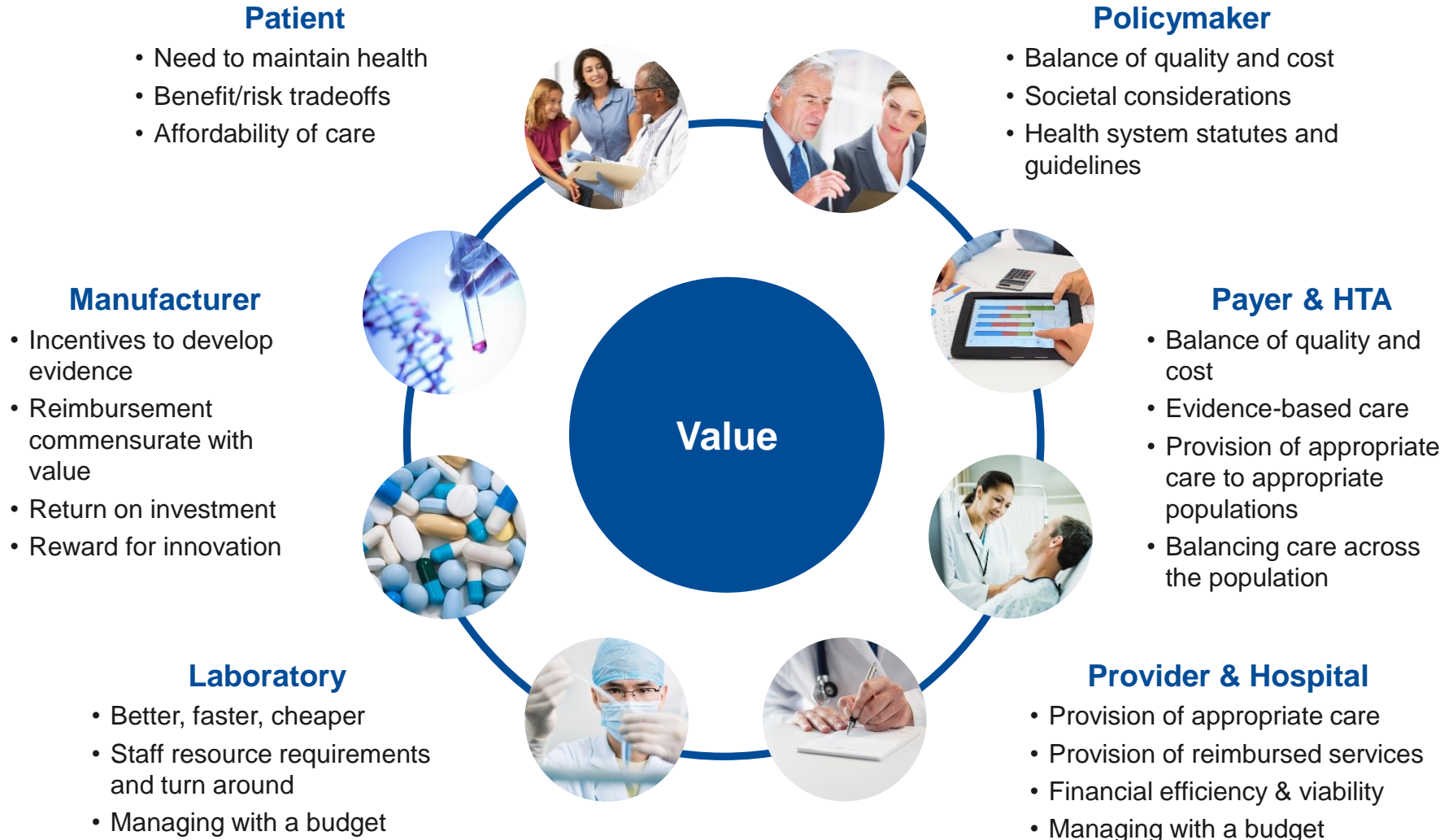


The Market Perspective: What shapes return?

Value is in the eye of the stakeholder / decision-makers

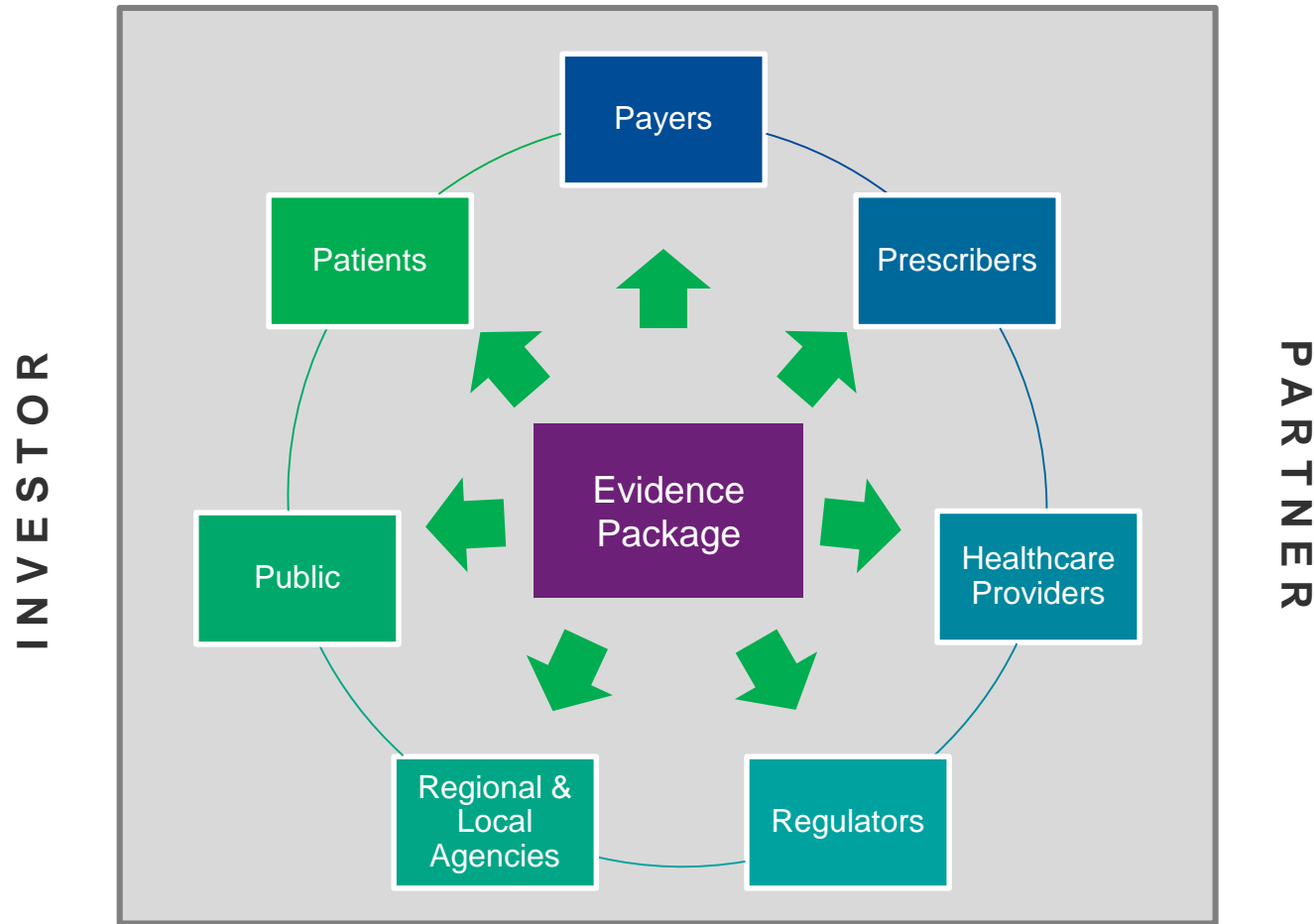


The challenge of meeting multiple stakeholder needs



Investment view: Can the molecule deliver value?

Value is built from the documented and expected performance of the molecule



Therefore, the “strategy” for development must focus on demonstrating the asset’s unique capabilities in the language of decision-makers

Polling question #2

Which statement below best aligns with your organization's abilities to address, understand, and incorporate the full range of stakeholder requirements into your product and program plans?

- › We're fully staffed and structured to address this today.
- › We have limited abilities to do this today, but are confident in the different options to get there.
- › We have limited abilities to do this today, and are uncertain how to approach it.
- › We haven't really considered this at all.

Defining the opportunity: Key early development questions

Deliverables and the tools used to answer them

	Question	Deliverable
Entering Pre-clinical	? What indication(s)?	✓ Landscape analysis
	? What patients?	✓ Patient pathway
	? What unmet need?	✓ Regulatory strategy
<hr/>		
Entering PH I-II	? What differentiation?	✓ HTA analysis
	? Which comparators?	✓ TTP gap analysis
	? What evidence requirements?	✓ Stakeholder analysis
	? What endpoints?	✓ CDP design & gap analysis

Early clinical strategy development

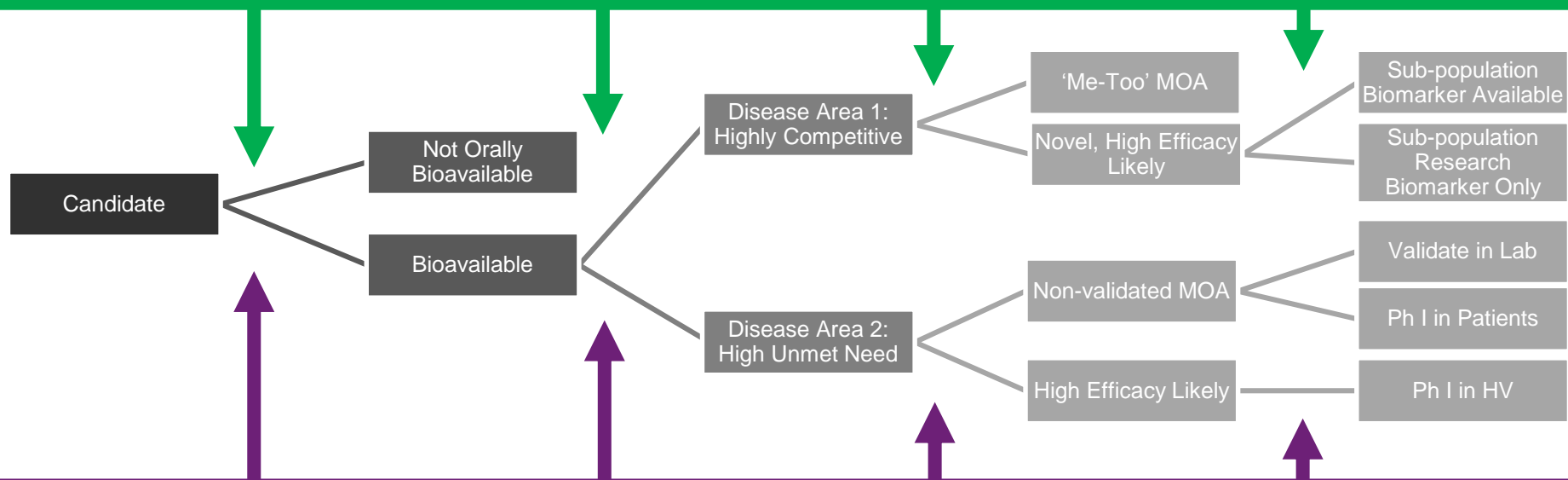
Strategic Question (What?)

Is it
'druggable'?
(Go/No-Go)

Is the Disease
Area Attractive?

Will this MOA
Compete?
(Go/No-Go)

What initial clinical focus
to drive differentiation?
(Go/No-Go)



PK/PD

- Landscape Assessment
- Disease TPP

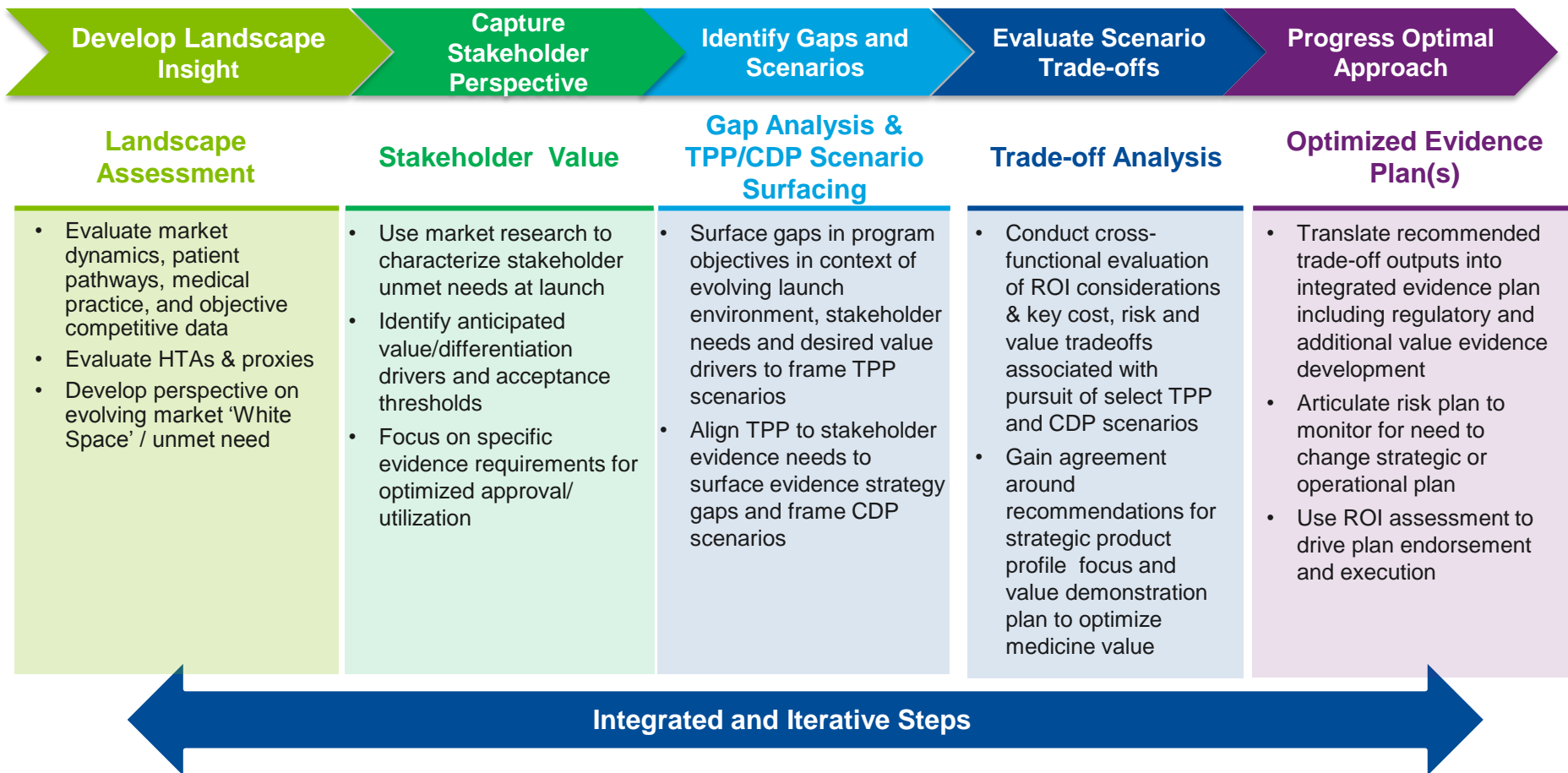
- Competitive Assessment
- SoC/Treatment Guidelines
- TPP Gap Analysis

- Patient Pathway
- Biomarker Assessment
- ROI Trade-off Analysis

Driving Execution Decisions (How?)

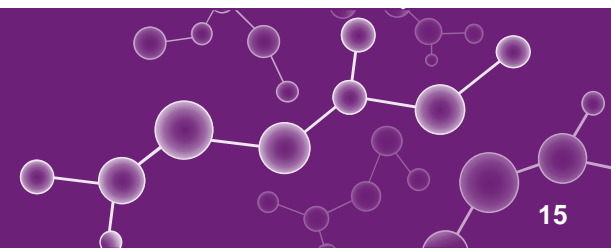
Building evidence: Optimizing evidence-generation

Building evidence requires addressing strategic questions using a stepwise approach to ensure next steps drive the highest program value



Improving your probability of success

- Systematically gathering and analyzing the information you need
- Supporting you in analyzing options to seamlessly integrate clinical strategy and execution



Building evidence: Integrated Asset Development

Demonstrating and delivering the full value of your therapy to the marketplace

Integrated Asset Development Plan delivers complete set of cross-functional strategic and operational outputs across the development of a pharmaceutical therapy

Integrated and iterative process drives optimized information and risk fidelity across the life cycle

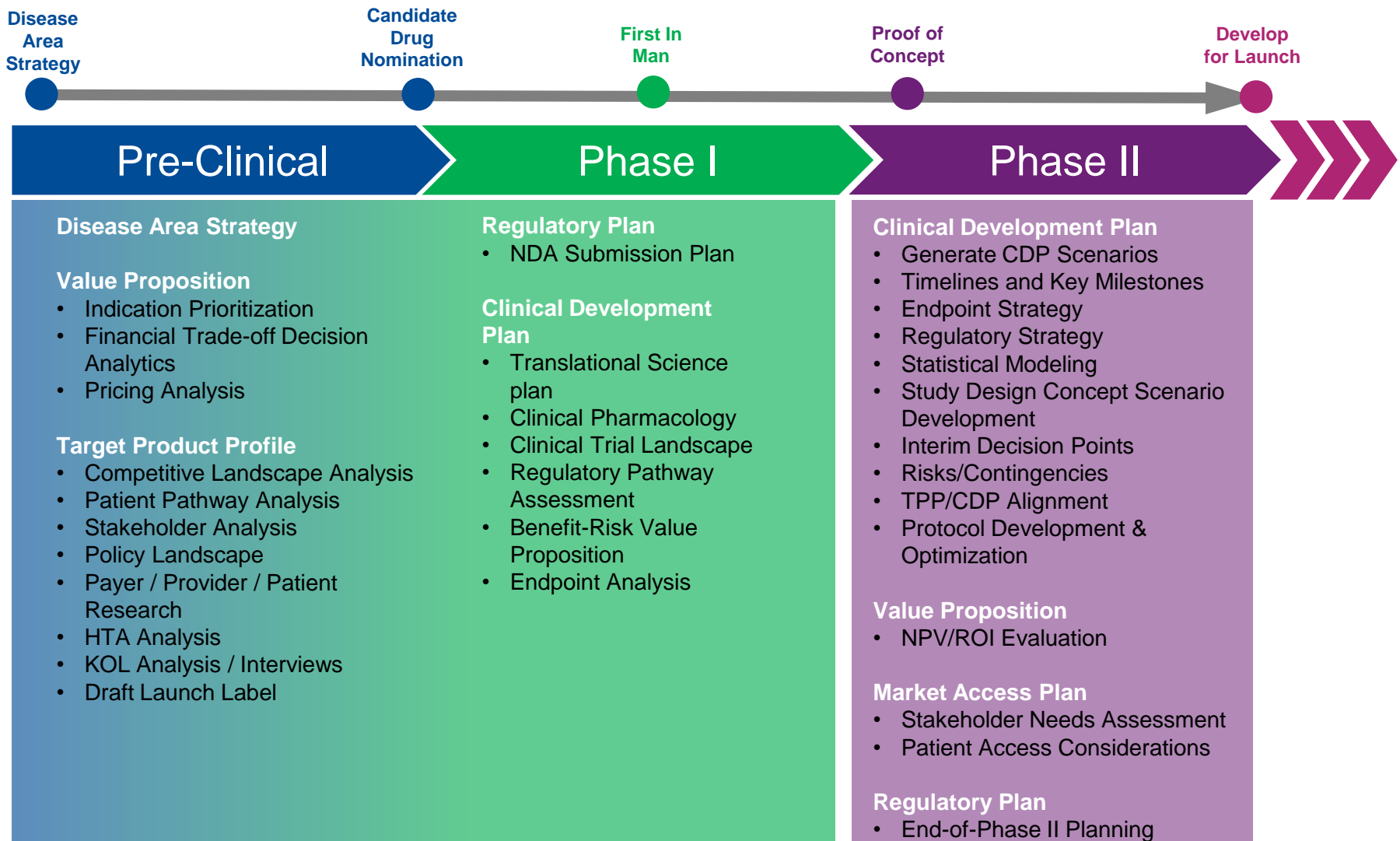
Process clarity provides efficiencies around cross-functional interfaces, dependencies and overlap

Cross-functional focus ensures that process drives optimized value to all critical stakeholders

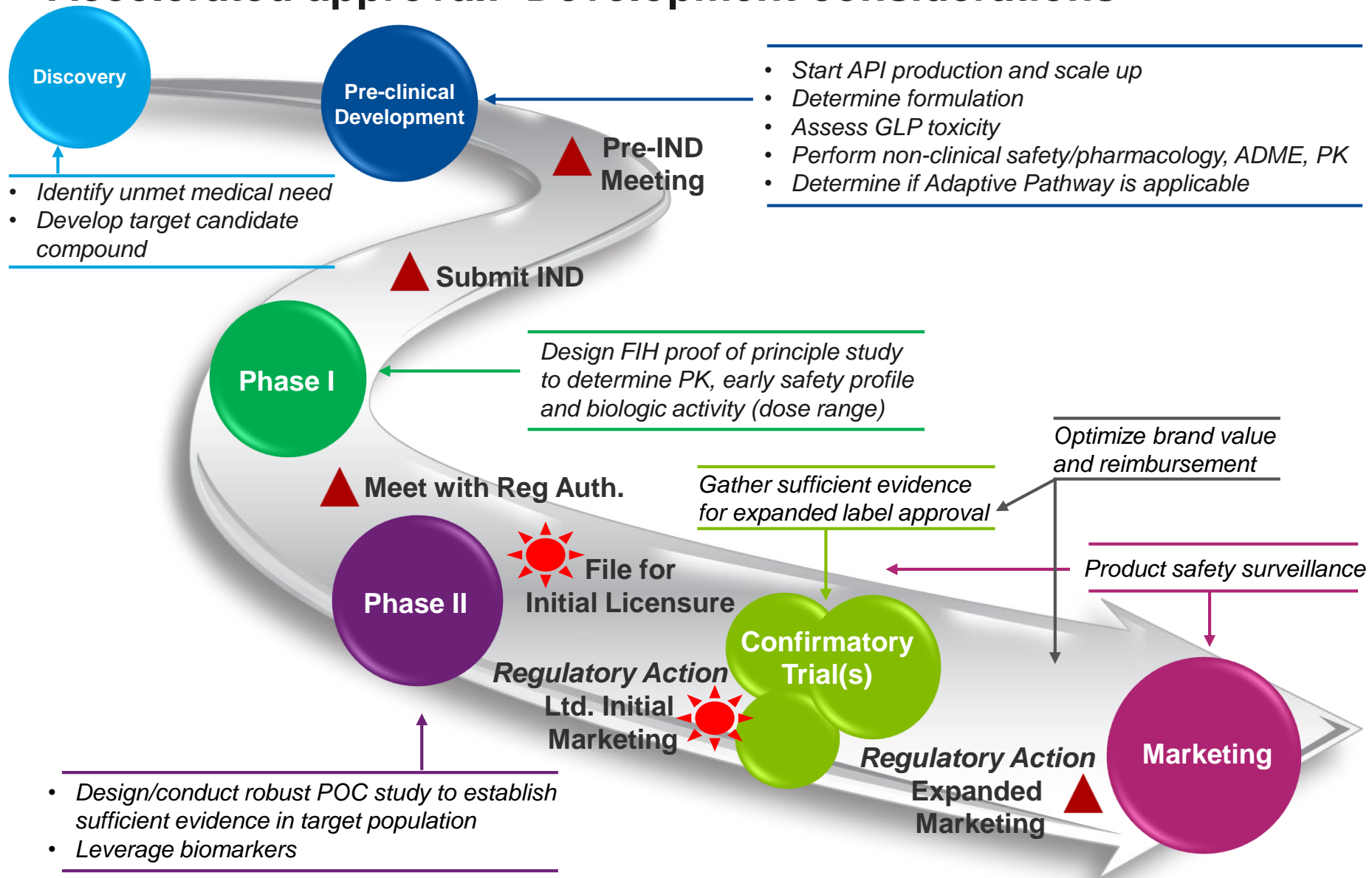


Building the early evidence-generation plan

Key deliverables and activities



Accelerated approval: Development considerations



Now what? Doing the work...

Optimizing execution & operations requires balancing internal cost with delivery

Keeping the “Foot in the Future” mindset while executing mission-critical activities is a challenging balancing act for a small, emerging enterprise

- Requires multi-disciplinary expertise (Large Pharma has New Products Planning)
- Requires senior-level, forward-thinking, strategic drug development expertise
- Requires good data to support cost, time, risk and return trade-offs
- Requires integration of multiple stakeholder-related planning efforts

This approach can be costly, especially if built through internal staffing

- Headcount would be added at a time of most significant product attrition risk
- Requisite expertise is expensive

Alternate approach: Consider a “virtual pharma model”

- Keep in-house decision-making expertise lean and focused on strategic intent
- Leverage capabilities of an integrated, end-to-end, full-service provider
- This requires a much more strategic, end-to-end approach to sourcing

Polling question #3

What is your organizations' primary approach to building infrastructure and operationalizing your key programs ?

- › We'll build out internal teams slowly overtime at a time of most significant product attrition risk.
- › We're partnering with other pharma organizations on this.
- › We're approaching a virtual model to keep a lean in-house model paired with expertise and services from an external service provider/partner (s)
- › Something in between

Leveraging your service provider

Comprehensive integrated clinical and regulatory sourcing support

Disease Area Strategy

Clinical Support for Lead Identification/Optimization or Due Diligence

Translation Science Strategy & Implementation (Lab)

Candidate Drug Nomination Package (Clinical/Reg/Commercial)

Animal-to-Human Dose Selection & Formulation (Clin Pharm/MBDD)

Preparation & Submission of IND Package/Agency meetings

Preparation of Phase I/PoC Protocols

Tech transfer & execution of GLP clinical assays (PK, PD , ADME, Genomic, etc,)

Early Patient & Endpoint Strategies

Ongoing evolution of Integrated Asset Development Plan

Operational Start-Up and Execution of ECD Program (Data Flow)

Preparation and transition to Phase II/III Clinical Program

**Seamless
Integration with
Early Development
Team**

**Integration with
Non-Clinical
Outsourced
Activities**

**Rigorous Project
Management
Across ECD
Portfolio**

**Integrated IT
infrastructure**

In Conclusion... Focus on Driving Value

Key Takeaways

The role of the Emerging Pharma leader is to balance the needs of “Today” with the requirements of “Tomorrow”:

- **Recognize the opportunity:**
 - › Align clinical & commercial intentions
- **Design to build evidence:**
 - › Integrate key program elements to inform stakeholder requirements
- **Optimize execution & operations:**
 - › Balance current spend against future return by leveraging capabilities of a partner/service provider
- **Deliver value:**
 - › Planning for the future now helps optimize the value of your asset

Thank you!



Q&A

For more information:



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