

Background

Helping Early-Stage Biotech to Better Characterize Commercial Opportunity



Situation

- Since 2017, we have been working with an early-stage biotech that is working on therapeutics for rare genetic disorders
- Over the past years, the company needed support in developing a winning strategy and communicating the potential commercial opportunity to investors
- In particular, there was a need to define the portfolio strategy more clearly by
 1. **Defining Addressable Market:** Research and analytics to explore different scenarios for company's lead asset
 2. **Identifying Growth Trajectory:** Identifying strategies to expand addressable market and drive demand post launch
- Exploring Portfolio Expansion: Primary market research to facilitate go/no-go decision-making for adding additional indications or assets to the portfolio



Challenges

- The company needed to clearly communicate the commercial opportunity to investors throughout several rounds of funding (pre-IPO)
- Target condition was ultra-rare genetic disease that is not well understood or characterized (eg, when it comes to role of different genotypes)
- Company was considering diversifying their portfolio of assets but needed additional primary research to better understand disease background and opportunity across different therapeutic areas
- Company only has small commercial team in-house and needed strategic partner to conduct primary and secondary research to support content development and decision-making



Questions

- Addressable Market:**
 - What peak sales could be expected after product launch?
 - How should we expect sales to develop based on different scenarios?
- Growth Trajectory:**
 - What were the prevalence and relevance of genetic subtypes in genes relevant to company's lead asset?
 - Could the addressable market be expanded (eg, by redefining which genotypes were relevant)?
- Portfolio Expansion:**
 - What were the addressable markets in different disease areas of interest?
 - What were the unmet needs from a treater perspective in those disease areas?

To learn more, please contact:

Ken Howie | Partner

☎ 412 / 979 . 4916

✉ khowie@magnoliainnovation.com

Diego Rodriguez | Partner

☎ 848 / 333 . 9780

✉ drodriguez@magnoliainnovation.com

2 Hudson Place, 6th Floor | Hoboken, NJ 07030

☎ 646 / 586 . 3080

✉ info@magnoliainnovation.com

🌐 magnoliainnovation.com

Approach

Combination of Primary and Secondary Research to Support Decision-Making



Defining Addressable Market

- We leveraged different sources, including qualitative interviews with treaters/likely treaters, scientific publications, and analyst reports, to better describe addressable market
- We used the in-house production team to develop concise and impactful communication materials, including visualizations of disease mechanism

Research revealed key insights that informed materials which were leveraged in different ways:

- Board of director presentations
- Website and SEC Form S-1 materials
- Investor presentations (eg, J.P. Morgan Annual Healthcare Conference)



Identifying Growth Trajectory

- Combination of desk research using a variety of scientific sources (eg, PubMed, ClinicalTrials.gov, ClinVar, OMIM) and direct outreach to academic and commercial genetic testing laboratories
- Goal was to better understand prevalence and relevance of different mutations relevant to company's lead indication

Research findings helped lead to understanding of how recent scientific analysis might translate into greater commercial implications for lead asset by:

- Collecting evidence to support idea that broader population than originally assumed could benefit from treatment with lead asset
- Providing adjusted ranges for addressable market based on information collected from genetic testing labs



Exploring Portfolio Expansion

- Qualitative interviews with Key Opinion Leaders across several potential target conditions
- Pressure test assumptions around prevalence, addressable population, unmet need, and competitive landscape

Research insights informed executive-level decision-making in two ways:

- Identifying potential additional indications for lead asset
- Enabling go/no-go decisions on purchase of new assets

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Outcomes

Long-Term Partnership Between Company and Magnolia



Successful Series Funding and IPO

- We worked directly with CEO and leadership team to prepare materials for investors and ahead of IPO
- Company successfully raised funding and eventually took the company public

Scenario	Addressable Market							
	I		II		III		IV	
Patient Group Conversion	On Tx 20%	Not on Tx 10%	On Tx 30%	Not on Tx 15%	On Tx 40%	Not on Tx 20%	On Tx 50%	Not on Tx 25%
Patient Number	480	870	720	1,305	960	1,740	1,200	2,175
\$ 100,000	\$ 153,000,000	\$ 229,500,000	\$ 306,000,000	\$ 382,500,000				
\$ 200,000	\$ 306,000,000	\$ 459,000,000	\$ 612,000,000	\$ 765,000,000				
\$ 300,000	\$ 459,000,000	\$ 688,500,000	\$ 918,000,000	\$ 1,147,500,000				
\$ 400,000	\$ 612,000,000	\$ 918,000,000	\$ 1,224,000,000	\$ 1,530,000,000				
\$ 500,000	\$ 765,000,000	\$ 1,147,500,000	\$ 1,530,000,000	\$ 1,912,500,000				
\$ 600,000	\$ 918,000,000	\$ 1,377,000,000	\$ 1,836,000,000	\$ 2,295,000,000				
\$ 700,000	\$ 1,071,000,000	\$ 1,606,500,000	\$ 2,142,000,000	\$ 2,677,500,000				

Business Case Construct	Indication #1 Opportunity*	Indication #2 Opportunity*
Market Size <i>What is the size of the market?</i>	~7,800 to 70,200 (Global) ¹	~10,000 to 15,000 (US) ²
Room in Market <i>To what extent is there room in the market for new entrants?</i>	High	Low
Ability to Differentiate <i>How competitive could asset be against current SOC therapies?</i>	Moderate	Moderate
Unmet Need <i>Is there a clear and recognizable unmet need?</i>	High	High
Use Case(s) <i>Are there clear use cases for asset that fit pricing model?</i>	Moderate	Moderate
MOA Alignment <i>How well is pathophysiology understood/aligned to asset?</i>	Low	High
Trial Feasibility <i>How feasible would it be to conduct a proof-of-concept study?</i>	Low	High



Portfolio Development

- Providing company with evidence to support argument to broaden addressable market by indication expansion
- Supporting due diligence activities ahead of purchase of additional assets to strengthen company's rare disease portfolio



Strategic Partnership Between Company and Magnolia

- We have become a key thought partner to executives and support strategic decision-making through research and consulting activities
- Agreement in place that allows us to support company on ad hoc requests and as needed

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