What are the critical success factors for a biotech's first commercialization in Europe?

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INTRODUCTION

BACKGROUND

Smaller biotechnology companies have pioneered transformative discoveries and therapeutic tools, such as gene therapies, CAR Ts, and antisense DNA.

On the precipice of commercialization, biotechnology company's have an important decision to make: ***"Should we commercialize in Europe as well as the USA?"***

The $246 billion potential European market offers substantial opportunity for smaller biotech companies however, navigating its highly localized HTA systems is daunting.

OBJECTIVE

Explore European **commercialization outcomes** for smaller biotechnology companies, & **identify critical questions & success factors** to be considered to support the **delivery** of in-demand **therapies** to **HCPs & patients**

METHODOLOGY

STEP 1: BIOTECHNOLOGY COMPANY SCREENING

Identified 150+ biotechnology companies *(not top-50 pharmaceutical companies)* who have successfully, or attempted to, launch in Europe with HQs in the USA or Asia. Filtered company list from **150+ to 25 companies** based on criteria in **Fig. 1**.

Figure 1: Biotechnology company list filtration criteria

- **Biotechnology definition**
  Company based in USA or Asia, with no established EU presence
- **Launch attempts**
  Company's >1 launch attempts were included & non-commercialized outcomes assessed
- **Type of launch asset**
  Biosimilar & generic companies were excluded
- **Timeline**
  Launch attempt within last 10 years
- **EMA opinion**
  Assets with negative EMA opinions were excluded

STEP 2: COMPANY STRATIFICATION

Companies were grouped into one of three categories (**examples** below):

- **Go-it-alone:** BluebirdBio, Blueprint Medicines, Rhythm Pharmaceuticals
- **Partnership:** Esperion, Myovant sciences, Atara Bio
- **Acquisition:** Kite Pharma

STEP 3: EVALUATION FRAMEWORK

Retrospective structured secondary research across themes and criteria to uncover pillars for success across the three business models (**Fig 2.**):

Figure 2: Biotech European launch evaluation framework overview

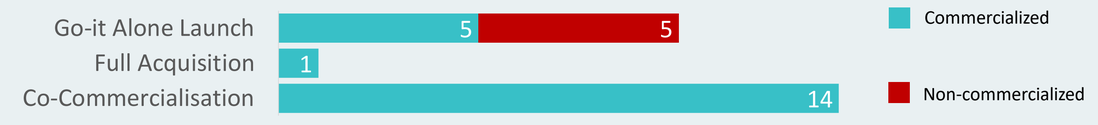
Theme		Criteria overview	
Company	Biotech	Mission, vision & company goals	Company portfolio & therapeutic area strategy vs launch asset
		Location including where they established EU HQ	Leadership including EU specific hires for launch
		Financial resources including market cap & cash runway	Team structure & evolution for EU market
		Estimated headcount at time of EU launch	
	Partner	Type of Partner (e.g., VC, pharmaceutical company)	Partner portfolio & therapy area strategy focus
		Partner mission, vision, values & goals	Value of & published detail of deal
		Partner prior EU launch experience	Success of deal & partnership
Asset		Asset modality & mechanism of action	Level of unmet need in EU population & size of addressable population
		EU launch indication(s)	Competitiveness of EU market
		Data package (e.g., no. of trial patients, trial sites, endpoint buy-in, quality of data)	
Launch readiness		Market shaping activities (e.g., conferences, publications, partnerships with KOLs, PAGs,)	EU countries where they successfully gained access
		Experienced market access professionals to navigate negotiations	Commercialized or non-commercialized outcome of future EU launches (post first launch)
		Manufacturing managed in-house or contracted	

RESULTS

BIOTECH EUROPEAN LAUNCH MODEL OUTCOMES

Successful commercialization was linked to leveraging partnership expertise. **Non-commercialization occurred in 50% of biotechs assessed who pursued '*go-it-alone*', ** indicating greater risk (**Fig. 3**):

Figure 3: Partnership model outcomes (n = 25)



BIOTECH EUROPEAN COMMERCIALIZATION CONSIDERATIONS

Analysis identified a biotechs first European commercialization is **contingent on a combination of factors.** It is **unlikely one factor will determine the outcome.**

Figure 4: Commercialization considerations overview

Disease burden & unmet need	How high is the level of unmet need within the indication(s)? How burdensome is the disease & current treatment on patient quality of life, across the patient journey?
Clinical trial sites & endpoints	Have trials been initiated in European markets? If not, is the demographic of patients similar in the trial market(s)? Have discussions with European regulatory authorities occurred ensuring they will accept data from other regions for this disease? Are trial endpoints widely accepted by the main stakeholders?
Asset level of innovation	Is the asset mechanism of action & modality accepted by the clinical community, payers, & current clinical guidelines?
HTA & access knowledge	How well defined are HTA pathways for the indication & modality? How challenging has it been for analogous company's/ assets to gain access & negotiate an approved price?
HCP sales force engagement	Are target patients & HCPs located in concentrated specialized centers or distributed across hospitals/ primary care practices? Given this, what size of sales force is required to achieve target engagement?
Manufacturing	What size & complexity of manufacturing facilities are required to meet demand?
Supply chain	What is the ease of delivering the therapeutic to the patient?

Answers to these key considerations will **<u>establish the level of risk to 'go-it-alone'</u>** based on the likely experience, resource & financial funding needed to achieve European commercialization

Gene therapy case study – value of a partnership when the barrier to commercialization is high

A more challenging modality to launch given the height of the barriers for success including stakeholder unfamiliarity, evolving clinical guidelines & practice, navigating difficult payer negotiations & supply chain

- **Commercialized outcome:** Regenxbio partnered with Novartis to launch onasemnogene abeparvovec
- **Non-commercialized outcome:** BluebirdBio attempted to 'go-it-alone' for betibeglogene autotemcel

CONCLUSION

EARLY PREPARATION IS EQUALLY AS IMPORTANT AS EXECUTION

Learnings from the retrospective analysis, combined with the European biotech launch framework criteria, enables biotechs to identify the right questions & considerations **early** when making critical commercialization decisions. This should support biotechs to take the optimal commercialization route for their company & asset, enabling the delivery of therapeutics to patients.

References

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