Addressing Unique Challenges Faced by Small to Medium Biopharma in Value Demonstration

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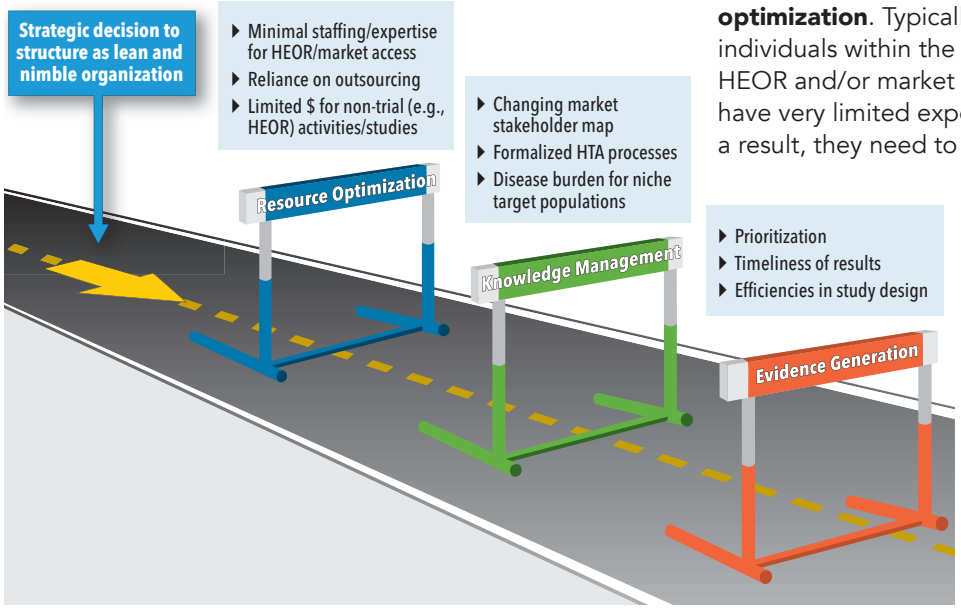
In recent years there has been a noticeable increase in the participation of small- to medium-sized biopharmaceutical companies in the development of new therapies. According to one recent analysis,[1] these companies increased their share of innovation origin (based on the number of new molecular entities) from 50% in 2004 to 73% in 2014. Statistics from the European Medicines Agency (EMA) indicate that 27% of new drugs introduced in the European market from 2010 to 2012 were from small to medium biopharma.[2] There has been a corresponding increase in the proportion of small to medium biopharma companies deciding to commercialize products themselves. This is due in part to the highly specialized nature of many therapies (e.g., orphan drugs) that may require a smaller scale as related to sales and marketing investment. As these companies move towards commercialization, they face unique challenges in meeting today's increasingly demanding evidentiary requirements and demonstrating the value of their new therapies. In this article, we describe these challenges - considering the business model that underlies them - and then summarize how Evidera engages with small to medium biopharma to address the challenges.

Figure 1: Unique Challenges Faced by Small to Medium Biopharma in Demonstrating Value of New Products

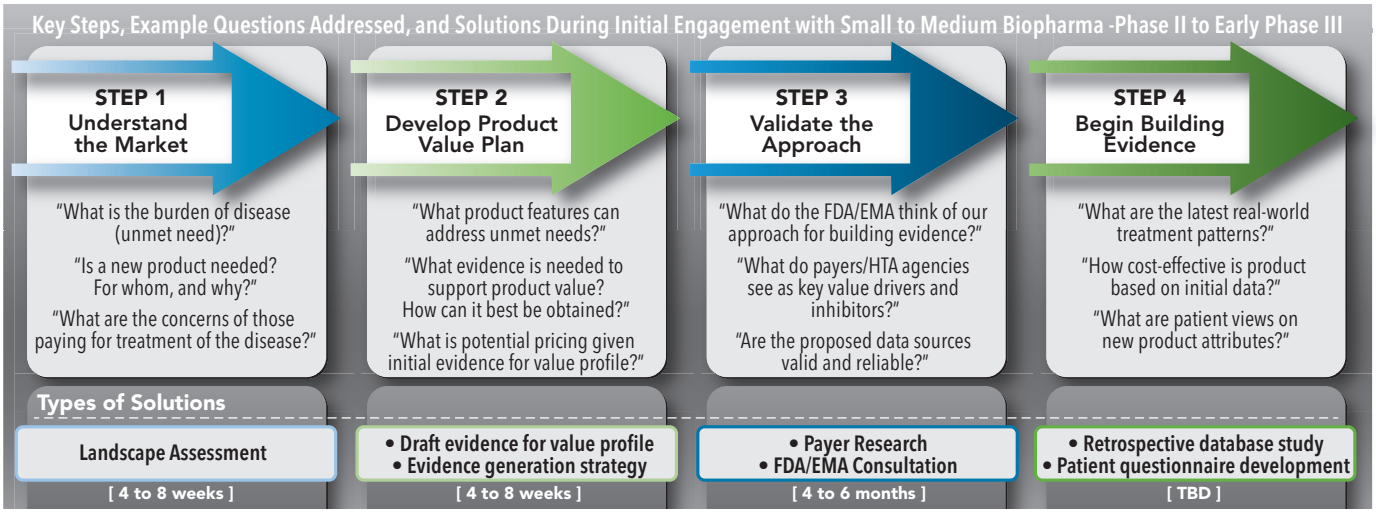


Value Demonstration Challenges for Small to Medium Biopharma

Small to medium biopharma companies face a business environment that is similar to that of most start-up companies: dependence on a single or very limited number of pipeline products, limited funding and the ongoing search to find additional investors, and pressure from investors to show a quick and high return on their investment. Given this environment, many of these companies (especially small biopharma) have made a strategic decision to be lean and nimble, hiring a limited staff that possess mainly scientific or entrepreneurial expertise.

As shown in Figure 1, the strategic decision by these companies to maintain a lean and efficient organizational structure leads to specific challenges vis-à-vis health economics and outcomes research (HEOR)/market access activities. One set of challenges relate to **resource optimization**. Typically there may be just one or two individuals within the company who are responsible for HEOR and/or market access, and often these individuals have very limited exposure to the needs in this space. As a result, they need to look outside their organization to obtain the necessary expertise. This expertise needs to cover a broad set of methods and approaches, and ideally address geographic variation. Given the individual(s)' responsibilities and workload, it is critical to identify an external partner who has the breadth and depth of HEOR/market access expertise. The HEOR/market access lead also faces a situation where the 'share of voice' is small relative to other functions within the organization.

Figure 2: Suggested Approach to Early Commercialization Support



Thus there are typically limited budgets for non-trial-related evidence generation (e.g., real-world studies) that may be important for market access considerations.

Small to medium biopharma companies also face key challenges in obtaining and maintaining an adequate knowledge base of the market landscape relevant to their product(s). There is constant change in the level of influence of the various stakeholders (e.g., providers, payers, patients) who will make decisions on the use and uptake of their product(s). Health technology assessment is becoming more formalized and increasingly complex, with significant variation in the processes being chosen by agencies in various countries. Due to the highly specialized nature of many new products in development, there is a dearth of information on the disease burden of the niche target populations – and this information will be critical for decision makers to understand the value proposition of these therapies.

A third set of challenges relates to the generation of the necessary evidence to demonstrate product value. Limited budgets mean that not every good HEOR study idea will be funded. There are some studies that will be viewed as necessary and others as 'nice to haves' – thus the rationale and justification for study prioritization are critical for small to medium biopharma executives who will have to make the case to their investors. The overall program of HEOR/market access activities will need to be designed in a thoughtful and integrated manner to ensure efficiencies and optimization of the available budget. And finally, studies that are funded will need to produce results in a timely manner to support critical go/no-go decisions and help justify additional funding from investors.

Addressing the Challenges with Early Commercialization Support

Figure 2 presents a suggested approach for providing small to medium size biopharma companies optimal early commercialization support that addresses the challenges just described. First, a partner such as Evidera, which has the broad expertise necessary to address the various questions included in each step of the process, offers the HEOR/market access lead a cost-effective outsourcing option. Second, Steps 1-3 in Figure 2 provide answers to some of the key knowledge management issues including 1) a detailed understanding of disease burden for the relevant target patient population, 2) in-depth perspectives from stakeholders on key value attributes, and 3) what evidence will regulators and payers need and expect to receive. Finally, a strong evidence generation strategy will result in a well-designed and cost-effective study plan. This will produce the right evidence for value demonstration that will ultimately get to the right audience at the right time.

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REFERENCES

[1] Rickwood S, Rosen D. 2020's top 20: Why the Top 20 Global Pharmaceutical Companies Matter So Much, Where They are Headed and What It Means for the Pharmaceutical Industry. *IMS White Paper.* 2015. Available at: http://www.imshealth.com/vgn-ext-templating/v/index.jsp?vgnextoid=a0839be195a5d410VgnVCM1000000e2e2ca2RCRD&vgnextchannel=6521e590cb4dc310VgnVCM100000a48d2ca2RCRD&vgnextfmt=default&vgnextcurrentchannel=1. Accessed October 2, 2015.

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