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The Role of Medical Affairs in Lifecycle Management

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MAPS 2030 VISION FOR MEDICAL AFFAIRS

Medical Affairs will be a strategic leader at the center of clinical development and commercialization efforts, addressing unmet patient, payer, policymaker, and provider needs that advance clinical practice and improve patient outcomes.

Introduction

New treatments aim to improve patient outcomes. New treatments also drive a company's ability to invest in the development of future therapies that continue to improve patient outcomes. But what happens when a new treatment is no longer "new"?

Historically, at the end of a product's period of exclusivity when generics are allowed to enter the market alongside a company's product, innovator biopharmaceutical companies would pivot to producing an authorized generic while focusing on approval of novel assets in the company's pipeline.

More recently there has been a paradigm shift in how companies plan for and deliver on the "long tail" of a drug's impact even after Loss of Exclusivity (LoE) – a process known as lifecycle management (LCM). In this new paradigm, Medical Affairs is an essential partner in ensuring that approved treatments benefit new and expanded patient populations beyond the initial indicated population.

This article leverages the input of 21 biopharmaceutical and MedTech leaders from the MAPS Strategic Leadership Council to describe how to have effective lifecycle management.

Primary Goal of LCM: Meeting Unmet Need

Drugs are approved based, in large part, on their ability to address unmet needs in patients studied as part of registrational clinical trials. However, there are often additional types of patients not included in these clinical trials or even patients with related conditions that could benefit from a new treatment. And a drug initially approved for one indication in a fairly narrow patient population may extend its period of exclusivity if approved for use in additional patient populations or for other uses.

This process of identifying, testing and earning approval for new post-approval indications is generally referred to as "label expansion." Successful label expansion provides HCPs with new, on-label uses of a drug, thus increasing patient access. Label expansion may also include maximizing a product's impact within the initial indicated population, for example by identifying characteristics of super-responders or non-responders to differentiate patient sub-populations where a drug is especially appropriate in comparison with others in the same class.

Whether a company's approach to label expansion focuses on new, approved indications or differentiation within the approved population/condition will depend on many factors unique to a company's size, pipeline, therapeutic area, etc.

Most companies will include any planned (or hypothesized) label expansion opportunities in both the Target Product Profile (TPP) and the Integrated Evidence Generation Plan (IEGP), which defines the studies needed to earn first indication approval as well as approval for any new indications. This usually takes the form of laying out a series of clinical trials from Phases 1-3 to earn initial approval, and then plans for additional clinical trials or studies needed to extend approval to new populations and/or indications.

When clinical trials are used for label expansion, a company's Clinical Development team within Research & Development will most often lead these studies. However, post-approval studies may also

include Phase 4 trials and studies that generate Real-World Evidence (RWE), both of which are often led by Medical Affairs, sometimes in collaboration with Health Economics & Outcomes Research (HEOR) and/or Epidemiology teams. In fact, with the U.S. Food & Drug Administration (US FDA) and European Medicines Agency (EMA), among other agencies, increasingly taking RWE studies into account when making regulatory decisions, the role of Medical Affairs in label expansion is becoming even more critical and pronounced.

Alternately, the potential for new indications may arise unexpectedly – or at least in a way that isn't foreseen when developing the initial TPP and IEGP. This provides a major role for Medical Affairs: Medical Affairs professionals in the field are uniquely positioned to gather insights identifying potential new opportunities or benefits not included in initial product planning. Medical Affairs may also support investigator-sponsored studies where an investigator can independently explore potential new uses for a marketed drug. These studies may in turn provide rationale for larger, company-sponsored studies, which are often led by R&D. In this way, Medical Affairs input is essential, not only in creating the initial TPP and IEGP, but in revising it to include the clinical data, RWE studies, patient-reported outcomes and other evidence that may inform expansion of a label into new indications to address additional unmet patient needs.

It's worth noting that patient experience and even outcomes may also be improved by new drug formulations, such as developing a drug initially approved as an oral medication into an injectable, patch or extended-release formulation. New formulations may also result in an additional opportunity for exclusivity. Medical Affairs can be instrumental in generating and communicating patient data to determine preferred formulations that maximize convenience, adherence and/or address other quality-of-life issues. Like new indications, new formulations will require Medical Affairs to collaborate with colleagues in R&D, Commercial and Regulatory to update the IEGP with any needed clinical or RWE studies.

Additional Ways Medical Affairs Can Inform LCM

Medical Affairs may also play a role in strategies that do not necessarily extend a drug's period of exclusivity but may help organizations maximize the impact of their products later in the lifecycle in other ways. For example, through interactions with the external healthcare ecosystem, Medical Affairs may identify the potential for follow-on products such as companion diagnostics, supportive care products, or even biosimilars that leverage much of the clinical development work required for the existing product. Medical Affairs' understanding of the patient journey and competitive landscape may also contribute strategically important insights to guide decisions regarding when a company should release its own generic.

Regional Considerations

Due to differences in regulatory and legal frameworks across countries and regions, a product will face different degrees of protection and competition at different times in different places. (If only LCM were as easy as planning for one global LOE date!) The "rolling" nature of patent expirations and loss of regulatory protection requires Medical Affairs teams and their counterparts in Commercial, R&D and Regulatory to tailor evidence generation planning to prepare for the types and timelines of studies needed to achieve realistic goals in each region, per unique regulatory, reimbursement and legal requirements. Evidence generation may also be needed to meet regulatory requirements that allow products to expand into new geographies/regions (e.g., China).

Timing of Medical Affairs Involvement in Lifecycle Planning

Planning for label expansion and other aspects of LCM will vary based on a company's size, pipeline, therapeutic area, and other unique factors. For example, a biotech company with a product targeting a niche population in rare disease may have limited resources and may thus be forced to delay the bulk of LCM planning until after earning first indication approval. At the other end of the spectrum, a large pharmaceutical company developing a product likely to impact a broad patient population may "bake" LCM into the earliest stages of the product planning process, even during disease area awareness or when defining the Target Product Profile (in which planned additional indications can be developed as sub-profiles of the TPP).

In some cases, initial approval may only be a first step on a "roadmap" that paves the way for approval in additional indications, making label expansion nearly parallel to initial approval in terms of company strategy. This approach is especially common in oncology and other therapeutic areas (e.g., immunology) in which the mechanism of action of the drug targets various disease conditions (e.g. psoriasis and inflammatory bowel disease), providing clear target populations for additional approvals. Early planning for label expansion is also common in extending the use of drugs approved for adults to pediatric patients or to other populations not necessarily included in the registrational clinical trials but who would be expected to have similar benefit.

A Note on LCM in MedTech

Once a drug is approved, it is essentially a static entity. Yes, new formulations, dosing, or indications may emerge, but the evolution of pharmaceutical products tends to occur as better products supplant the old ones, rather than through adaptation of existing products. The opposite is true in MedTech, where approved products are continuously evolving, and device modifications may be an essential piece of LCM that allows devices to retain differentiation. Improving features and usability may protect MedTech products in the market long after their pharmaceutical product cousins have been put out to pasture. In MedTech, LCM also includes planning for end of product life and/or end of service: When will software updates be needed? How about hardware updates? How can industry partner with customers and health systems to manage end-of-service for equipment and transition to new solutions? Medical Affairs insights stemming from interactions with real-world users of a device are essential in both evolving and retiring products.

All this said, many pharmaceutical companies bring Medical Affairs into LCM once dosing is established and stage-gate decisions are made to continue development in a way that implies optimism for approval and thus the eventual need for LCM. However, even within companies who follow this model, considerable variation exists in the degree of LCM investment in early (pre-phase 3) development stages. Overall, the question of LCM timing and the pace of investment seems to hinge on a company's resources (of course), its confidence in product approval, the broad-vs-narrow scope of initial submission (how much label expansion is required/expected), and a company's vision for the impact of LCM activities.

Measuring the Impact of LCM

Because "final" results of LCM may not be evident for years (if then), many companies seek to measure steps along the way that reinforce or suggest revision to current strategies. Many companies are moving toward patient-centric endpoints to measure the impact of their initiatives, including but certainly not limited to LCM – for example, measuring the overall number of patients impacted by a treatment, or the ability of a new indication to address unmet medical need and improve patient outcomes.

Another focus of Medical Affairs success metrics that applies to LCM strategy/tactics is the move to measure impact rather than actions – in other words, we have been "counting things but are these the things that count?" For example, it may be that earning 15 new indications is less impactful than earning one new indication for a broad patient population (or, as is widely cited in Medical Affairs, counting 15 MSL/HCP engagements may be less impactful than recognizing one interaction that results in an HCP updating their treatment approach to match recognized guidelines).

One key in measuring impact rather than actions in LCM is asking how the knowledge gained from a metric could drive actions or decisions – in other words, not only "measuring to measure" but measuring to provide validation or correction for strategic directions. If a company is managing LCM across a global portfolio that includes approved and pipeline products, it is also important to standardize the use of success metrics to allow apples-to-apples comparisons across product lines.

Finally, it is, of course, also important to take into account practicality. If an organization chooses to under-resource Medical's involvement in LCM, it may in fact be limited to counting actions like engagements, publications, and phase 4 or RWE studies initiated or completed rather than using more sophisticated impact metrics such as the measurement of how Medical actions result in closing gaps in patient care.

Conclusion

Medical Affairs involvement in LCM is essential to ensure sustained and broad patient benefit from new health technologies. Medical insights may identify unmet need that provides direction for label expansion. And Medical-led RWE and Phase 4 studies now sit alongside clinical trials in the IEGP to achieve this expansion. At the same time, Medical's impact in LCM goes beyond label expansion to touch areas including access, adherence, and patient-centricity that may all provide a competitive advantage for a company's authorized generic even when placed on a shelf (or on payer's formulary) alongside other generics. Done right, the result of including Medical Affairs as a strategic partner in LCM can broaden the number and type of patients who benefit from biopharmaceutical and MedTech innovations while providing revenue sustainability.

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