Regulatory strategies for value maximisation of early clinical stage assets




The Goal

To strengthen your partnering negotiation position, reduce the time to the clinic and to help you determine value-adding regulatory milestones by applying integrated regulatory strategies, ultimately optimising the value of your asset at the point of exit.

The challenges

In speaking with numerous small and mid-sized biotech companies, we at NDA understand that there are many important reasons why a company would choose not to develop and commercialise a product on their own. The cost of development increases exponentially, making the final stages impossible to finance for many companies. Building a commercial organisation covering vast territories can be an equally daunting task. Companies we work with have a clear definition of what it is they want to do and often their strategy is limited to discovering and developing proofs of concepts of products in a certain region; the rest is simply not part of their focus.

Whatever the reason, the goal for many companies we work with is often to develop the product to a stage where partnering or out-licensing becomes feasible. The value of the product is however not a binary matter of reaching a particular point of development. The value of your asset is also built iteratively and how well you have conducted and documented your development programme will decide the ultimate value of your asset when you negotiate with your future partners.

Regardless of your point of exit, the longer-term goal of corporate partners for any product is commercialisation. The perceived risks and success probabilities will strongly influence whether they will choose to partner with you or someone else. This seems obvious, but it is sometimes neglected by companies whose goal it is to accelerate the product towards the first key milestone: the start of clinical development.

Various studies have shown that taking advantage of regulatory opportunities, such as fast-track and orphan drug designations is reflected in increased stock prices for pharmaceutical companies and this can provide positive signals to investors that the drug may have financial value in the future.[1, 2, 3]

Commercialisation of a product requires regulatory approval, issued by the respective competent authority and companies should make use of an integrated regulatory strategy throughout product development to meet this long-term goal. Identification of critical activities, milestones and decision points as well as risk management tools that support the efficient advancement of a product towards gaining regulatory approval in each region, are integral parts of the approach.

How to maximise value during early product development?

Defining the path towards critical milestones and its timely execution while balancing risks and resources should be a key strategic focus for small to medium enterprises. Integrating project specific development strategies and the respective milestone deliverables in the overall corporate objectives provides a strategic roadmap to valuable exit points and allows acceleration of high value projects.

Such a regulatory strategy can optimise the efficiency of product development and prevent any unnecessary backward steps. A risk-based approach should also be sufficiently flexible to allow for future changes in the market and the regulatory environment.

Developing a global regulatory strategy is an important value driver of a product as it enables a global approach to maximise return on investment. Although regulatory harmonisation is proceeding through the ICH process, there remain many differences owing mainly to the legislative basis in the respective jurisdictions. These differences must be considered at all stages of development to optimally achieve registration in all regions of interest.

To maximise your partnering opportunities, the product should be developed for as broad a market as possible. Differentiated products that satisfy the requirements of the major regulatory authorities in all relevant regions will be attractive to single partners focusing on world-wide rights, but can also more easily be used to attract strategic partners for specific regions. Such products will also maximise the opportunity for gaining additional funding and provide attractive exit opportunities for SMEs.

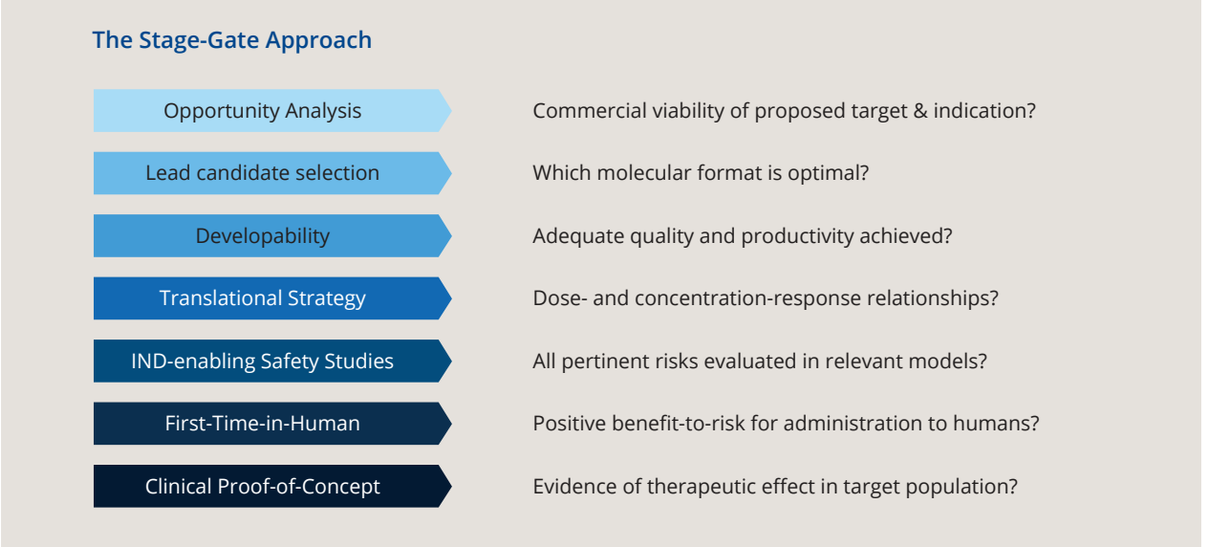
Implementing a global and value-driven perspective in early-stage development is a key driver in positioning the product towards successful global approval and commercialisation.

How to start?

Defining a Target Product Profile for the potential drug candidate is a great starting point to delineate an early development strategy. This can be initiated as early as preclinical development and can then be updated at the major development milestones.

Go/No-Go criteria as well as milestones and deliverables can be mapped out as part of a stage-gate approach.

Figure 1: Stage-Gates for Early Development



Developed by Paul Chamberlain, member of the NDA Advisory Board

Mapping the risks and the value inflection points

Starting from the available data (in-house and external), a stage-gate approach can be applied using critical input and assessment of alternative routes for product development. This results in a clear and acceptable product profile that allows proactive mitigation of challenges and gaps. In addition, an early assessment of benefit/risk can be conducted which is further developed through the development life cycle. Consistent, clear product messages, identification of supporting data and the identification of common (core) requirements will result in a core dossier for the EU and US. This will in turn achieve acceleration of development timelines, focussing of resources and increased value of the product.

Selecting the most effective tools

Product-specific risk identification, allied to understanding of the regulatory environment, facilitates prioritisation of resource allocation to the development and validation of an optimal analytical and bioanalytical toolbox. Choices made early in development can have a critical influence on effectiveness of both the product quality control strategy and monitoring of efficacy and safety endpoints in preclinical and clinical studies. Good choices made early in development can minimise costs, avoid time-consuming back-fill of gaps and lock-in incremental asset value.

Conclusion

Defining an early development strategy and implementing it into an overall strategic roadmap is a very valuable component of overall product development.

This roadmap encompasses the scientific and clinical pull to generate products that fulfil unmet clinical needs and provide benefit to patients. The roadmap also provides the regulatory push to successfully navigate regulatory requirements for product approval and to generate a dossier supporting favourable pricing reimbursement discussions. The endpoint is the maximised commercial value for a potential drug product to a partner, licensor or acquirer. This in turn maximises the corporate value through the achievements of value inflection milestones as well as iterative value creation through robust documentation and regulatory interactions, and provides potential for corporate financing and exit strategies.

The NDA Approach

Combining NDA's experiences from industry and agencies, working across multiple regions and multiple sets of requirements allows us to support companies all the way through the translational stages of product development and throughout the product lifecycle.

We will help you to define integrated project deliverables and input data for informed decisions at critical stages of product development to prioritise your development activities, identify resource and investment needs, resulting in added commercial value and minimised regulatory risk.

References:

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