PLANNING FOR SUCCESSFUL AND EFFICIENT ATMP DEVELOPMENT

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1. BUSINESS STRATEGY AND INVESTORS

Building a solid business case early is a strong prerequisite for being successful in partnering with investors and co-developers. And as such it is likely to be considered a solid proposition for investment if you're planning both for development of your product into clinical stages as well as market approval in the end.

- Company organization
- Partnerships & collaborations
- Roles and responsibilities (MT & board)
- IP and freedom to operate
- Competitor analysis

2. PRODUCT DEFINITION (TPP)

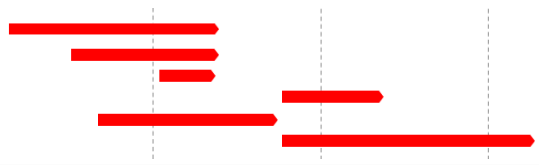
Begin planning of product development with the ultimate goal in mind – product labelingTPP: Format for a summary of a drug development program described in terms of labeling concepts. (FDA definition adapted from Guidance: Target Product Profile – a strategic development process tool)

- Strategic planning tool,
- Facilitates interaction with regulatory agencies,
- Facilitates interaction with investors and stakeholders
- Facilitates planning for a successful non-clinical and clinical development as well as design of manufacturing plans

3. CMC DEVELOPMENT

ATMPs are usually manufactured using complex biological and technological processes for which many protocols are to be developed. Culturing, modification, harvesting, purification and formulation of the product all give rise to challenges regarding product quality characteristics like purity, potency, and safety. Furthermore, manufacturing processes often demonstrate an inherent variability causing significant heterogeneity between batches, which also relates to the challenges in testing, characterization and control.

EXAMPLE



4. NON-CLINICAL DEVELOPMENT

Due to the specific characteristics of ATMPs and differences in regulatory requirements, non-clinical development may not follow a "standardized" approach. Products used in non-clinical studies should be representative of the product that will be administered to humans in clinical studies. The final product should be based on the right data an non-clinical studies should be performed using the most relevant in vitro and in vivo models available, the rationale for the selection of these models needs a solid justification.

SCIENCE BASED RATIONALE → Animal model selection | Dose finding | Toxicity – immunogenicity | Insertional mutagenesis | Germline transmission | Pharmacokinetics | Biodistribution – Shedding | Genotoxicity and oncogenicity

5. CLINICAL DEVELOPMENT

Many ATMPs are first in man clinical trials and/or first in class medicinal products. Consequently, the clinical trial design harbours specific challenges like:

- The mode of delivery that should be described in an extensive TPP at an early stage of development
- Setting your inclusion and exclusion criteria right
- Selection of a starting dose and a staggered approach for patient enrolment

Given the unique character of ATMPs specific requirements for long-term follow up are demanded. The design of the long-term follow-up regimen needs to be determined on a case-by-case basis depending on the product and the trial population.

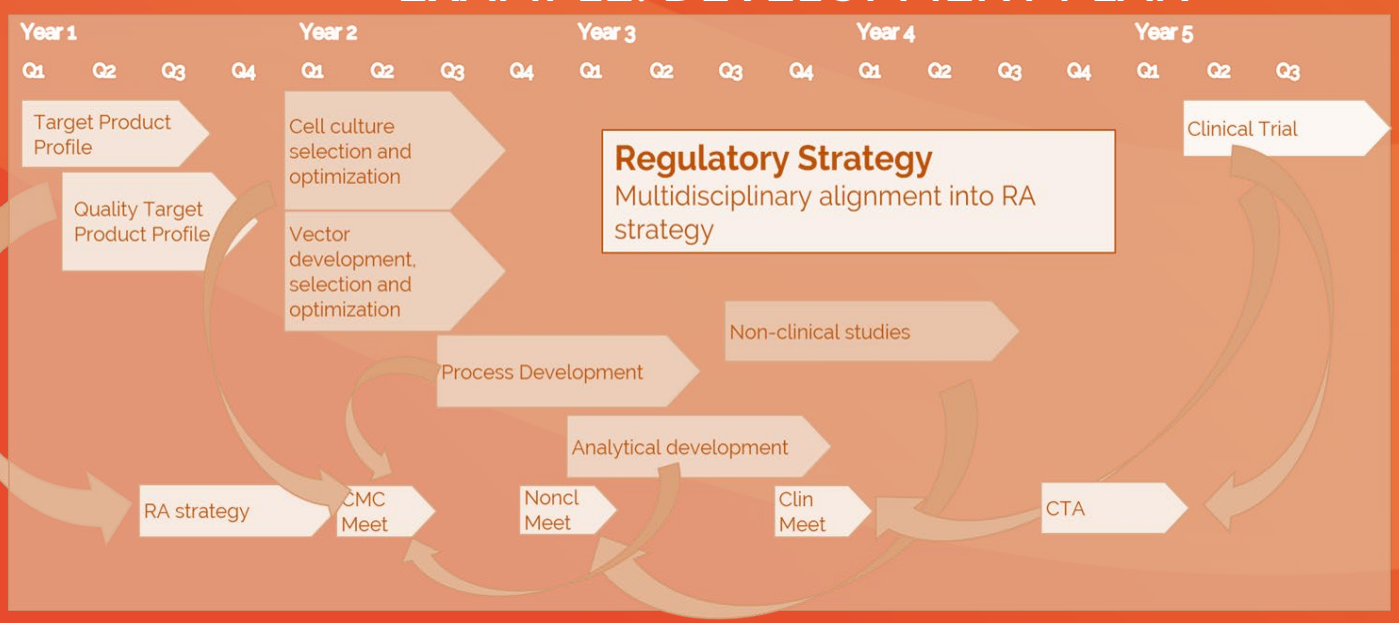
6. REGULATORY STRATEGY

A solid regulatory strategy will not only expose any regulatory challenges but also create regulatory opportunities. It will allow you to:

- Set milestones and deliverables
- Identify risks and mitigation strategies
- Set up a strategy for communication with Health Authorities

This will all be beneficial in guiding your ATMP through the regulatory maze and make sure opportunities turn into reality.

EXAMPLE: DEVELOPMENT PLAN



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