From Molecule to Market: Leveraging Patient Insights to Optimize Drug Delivery

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BACKGROUND: DRUG AND DEVICE DEVELOPMENT PROCESS

The current pathway for pharmaceutical companies to develop a new Drug is complex, expensive and lengthy. Deciding to bring a newly discovered molecule to the market can be even more daunting, especially if the new Drug is part of a combination product that involves the use of a Class 2 or greater Drug Delivery Device. To help companies understand what is required for a launch in the Unites States, the FDA has broken out the process into separate steps:

- **Drug**
  - Discovery and Development
  - Preclinical Research
  - Clinical Research
  - FDA Review
  - FDA Post-Market Safety Monitoring

- **Device**
  - Device Discovery and Concept
  - Preclinical Research – Prototype
  - Pathway to Approval
  - FDA Review
  - FDA Post-Market Safety Monitoring

Within these seemingly few steps lie multiple rounds of development, prototyping, research, and evidence-generation meant to establish the effectiveness and safety of both the Drug and the Drug Delivery Device such as:

- Biocompatibility Testing
- Performance Testing
- Environmental Condition Testing

- Materials Testing
- Shelf-Life Stability Testing

Depending on the class of the device, pharmaceutical companies can take various paths through FDA review, with Class 2 and 3 devices requiring the most involved processes ending, ideally, with a Premarket Approval (PMA) or with a successful 510(k) application. This process is fraught with uncertainty at nearly every major milestone and can require 10-15 years to complete. Notably, the FDA continues to monitor the performance of the Drug and the Drug Delivery Device once approved and requires companies to take swift action when issues are discovered post-launch. Similar or even more stringent processes are in place for Drugs intended for use globally.

Pharmaceutical companies often rely on external partners at various stages of the development process to assist with regulatory strategies or required testing. Recently, many companies are deciding to include additional forms of research or move standard testing upstream. For example, early identification of Human Factor considerations can accelerate and derisk downstream development and begin the process of providing an ideal experience for end users. Taking a user-centered research and design approach along with comprehensive regulatory testing facilitates cross-functional, internal and external endorsement of the selected device at launch.

VARIETY IN DRUG DELIVERY DEVICE USE CASES

Throughout the development process, leading pharmaceutical companies continually strive to innovate and develop ways to set themselves and their new product apart. To accomplish this goal, and be commercially successful, both the Drug and Drug Delivery Device must meet the real and perceived needs of the end users. This is applicable whether the molecule is novel for a rare disease, an incremental innovation into a competitive market, or an additional Indication for Use. Additionally, complex generics and Biosimilars have recently opened the door for new companies to enter historically isolated markets. While these newer companies may not have the resources of a larger, established competitor they are highly motivated to provide an ideal experience for their potential users.

From day one of development, pharmaceutical companies have the opportunity to focus on the ideal user experience with the Drug Delivery Device and take an approach that accurately identifies and meets their unique user needs. This allows for the patient to feel like the Drug Delivery Device was designed "with them in mind" despite the platform or type of administration required. Taking a multi-disciplinary approach linking user experience, human factors, design ideation, characterization testing and onboarding solutions is the ideal way to accelerate and derisk the overall product development while maintaining a user-centric philosophy.

A strategy used by many pharmaceutical companies to differentiate their newly developed Drug is through the use of a preferred Drug Delivery Device based on various platforms available for administration. This can include intravenous or subcutaneous infusion systems, vial and syringe solutions, single or multi-dose autoinjectors, On-body devices or options for a nasal/oral administration. Often, the platform for administration is decided by the clinical profile of the Drug, however the specific Drug Delivery Device within that category will still need to be selected. Additional considerations by the pharmaceutical company include choosing a Drug Delivery Device manufacturer that has the ability to provide configurable delivery devices and "off-the-shelf" platforms for early drug development testing and data gathering.

How does a pharmaceutical company decide what needs to be considered when selecting a Drug Delivery Device? Ideally by exploring the unique needs of their patients or healthcare providers and focusing their Drug Delivery Device design on the needs identified.

Initial factors to consider include:

- *"Will this be administered primarily by a healthcare provider or self-administered?"*
- *"Will healthcare providers be influenced by device-specific, published literature?"*
- *"Will this be used by patients who have dexterity or fine-motor movement issues?"*
- *"Is this an acute use requiring 'in the moment' resources or a routine administration allowing for more time for training and onboarding?"*
- *"Where does the intended user administer this medication?"*
- *"What is the cognitive ability of the intended patient population/caregiver?"*
- *"How can the Device provide a competitive edge over the current offerings for this condition?"*
- *"What additional tactics or resources may be needed during self-administration?"*

While these sorts of factors are crucial to consider, they in no way encompass all of the real and perceived needs that may exist for the intended users of the Drug Delivery Device. When the user is intended to be a patient (i.e., a self-administered injectable) then additional research and considerations beyond what is required by governing bodies needs to be identified and undertaken. Leading pharmaceutical companies utilize a comprehensive patient and user-centric research strategy alongside their regulatory strategy to move important development insights upstream.

INITIATING A USER-CENTRIC INSIGHTS STRATEGY FOR DEVICE SELECTION

When it comes to the subject of Drug Delivery Device Selection often pharmaceutical companies will work with partners to enhance their internal strategy. Facilitating and overseeing research with multiple external partners while balancing the needs of the pharmaceutical company and the Drug Delivery Device manufacturer can be daunting to a company's commercial, regulatory and market access teams. Choosing the right partner is key as some do not have the experience of working alongside the pharmaceutical company early in the development process. Conversely, the company must consider a partner who is agile enough to pivot along the course of Drug development while keeping the ideal commercial launch representation of the Drug in mind.

In order to comprehensively identify, validate and meet the unique user needs the following types of research should each be considered by pharmaceutical companies to guide market strategy and provide a competitive edge:

- Device Preference and Comparative Studies
  - Research to compare and contrast currently available and bespoke Drug Delivery Devices
  - Additional considerations include device and formulation scenarios as well as varying markets and geographic scope
- User Experience and Acceptability Research
  - Focused user research around usability and overall acceptance of proposed Drug Delivery Device
  - Options to explore routes of administration changes such as transitioning from intravenous to subcutaneous injection, or from vial-and-syringe to an On-body Device
  - Designed to help guide pharmaceutical companies claims for publication and commercial messaging
- Human Factor Studies
  - Device Use, Use Environment and User Interface Research
  - Formative and Summative Studies
  - Threshold and Comparative Analyses
  - Use-Related Risk Analysis (URRA) and Validation Testing
- Market Scouting, Journey Mapping and Discovery Research
  - Identification of key points along the user's journey from initial diagnosis through treatment adoption and adherence
  - Primary research with patients, healthcare providers or other relevant stakeholders to understand device-specific needs, barriers and preferences
  - Competitive landscape reviews and comparison to market analogues
  - Identification of preferred tactics and solutions for Drug Delivery Device onboarding and training such as:
    - Needleless Demonstration Devices
    - Autoinjector and Pre-Filled Syringe Device Accessories
    - Digital Health and Connected Devices
    - Injection Mats and User-Centric Packaging
- Persona Development and Device Characterization Testing
  - User and Device feature identification and quantification
  - Lab-based testing to quantify force requirements, feature performance, functions and use steps
    - Cap removal/closure
    - Effort required to set dosing
    - Actuation forces
    - Needle attachment and removal torque
    - Auditory feedback decibel levels
    - Injection delivery time
- Ergonomic and Design Review
  - Customized Design Services
    - Bespoke devices and training solutions
    - Tailored components and features using currently marketed devices
  - Expert review and opinion based on Anthropometric and Human Performance Databases
  - Anthropometric – hand length and width, finger length, etc.
  - Coordination or function alterations caused by condition or age
    - Grasping/Dexterity
    - Strength/Precision
    - Sensory Impairments (neuropathy, hearing/vision loss)
  - Cognitive and Emotional considerations
  - Environmental factors

SUMMARY/KEY TAKEAWAYS

- The development of combination Drug products involving Drug Delivery Devices is a lengthy, expensive and complex process, often taking years to complete
- Early identification of Human Factor and User Experience design needs can accelerate the development and reduce the risks associated with bringing a new Drug to market
- Patient and stakeholder research (such as Market Scouting, Journey Mapping, Discovery Research) helps to identify key points along the journey to better understand the needs, barriers and preferences around onboarding and device training
- Device Characterization focused on unique user needs (dexterity, cognitive ability, etc.) can facilitate the development of features and functions to provide a competitive advantage
- Ergonomics reviews based on anthropometric and human performance databases ensure the device is designed to accommodate various user needs
- Crafting optimized commercial messaging through Acceptability research is a strategy utilized by leading pharmaceutical companies