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EARLY & HOLISTIC DEFINITION OF THE OPTIMUM PRIMARY PACKAGING STRATEGY: ORPHAN/RARE DISEASES FOCUS

In this article, Kate Hudson-Farmer, PhD, and Niels Kure, MBA, both Directors of Front-End Innovation at Medicom Innovation Partner (a Phillips-Medisize Company), reveal conflicting pressures on the product development process in orphan indications which potentially lead to products in this category – especially emerging biotech products – being left with suboptimal drug delivery systems and primary packaging, and the consequent negative impact on patients. The authors explain how overcoming these pressures and paying proper attention to the development of the primary packaging and delivery systems for orphan products early on in the development process is worthwhile, with benefits arising as early as during clinical development as well as in the longer term.

Development of a new therapy is a very complex process, and many of the important decisions taken in the earliest stages will eventually follow the drug all the way through its lifecycle, and subsequently also appear in the hands of the patient, caregiver and/or healthcare practitioner.

The complexities of many orphan diseases in terms of treatment options (or lack of), their chronic manifestation and the management of symptoms, make them among the most challenging diseases to manage. A growing number of potential orphan disease therapies are being developed as biologics requiring injection as the dominant route of administration. Moreover, a significant majority of these are likely to be delivered by some form of injection by either the patient themselves or the caregiver. Getting the drug on the market and into the hands of the patient as quickly as possible, as well as defending its position against future potential entrants, is paramount for pharmaceutical companies in order to secure both short and long-term return on investment. This time pressure on products for orphan indications means that it is often decided just to put the drug in a vial and give the patient a syringe, which gives rise to poor adherence, poor administration, lower safety and ultimately lower efficacy.

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This multi-layered complexity calls for strategic thinking where options for drug delivery solutions are considered against potential market needs, the strong scientific

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"Even for those orphan drugs that have achieved formulation into liquid preparations, the further application of prefilled syringes and even an auto injector, which is becoming more of the norm for many chronic diseases, is still lagging in the orphan arena."

drivers which normally characterise orphan drug development and the pharmaceutical company's own internal expectations. In order to retain a lead position over the longer term, thus creating competitive advantage beyond the orphan drug exclusivity period, one needs a thorough understanding of the lifecycle management strategies related to the drug packaging and delivery device options. It requires a "top down" and holistic approach to defining the treatment solution from a specific disease need perspective, capturing all the elements from therapeutic efficacy to optimal drug delivery to service-related benefits.

Ideally such a strategy process is carried out early enough in the drug development phase so that the required tests involving the primary packaging can be performed on the option that gives the required commercial and technical opportunities and positioning.

What is clear is that the drugs being developed to treat orphan diseases are not "off-the-shelf" but highly developed compounds tailored to the patients' needs. However, most of the drug delivery devices, which are supplied with the drug, are the complete opposite: off-the-shelf vials and syringes without any real thought as to the patients' needs. If the drug molecule is designed specifically to meet the needs of the patient, surely a drug delivery solution optimised to fit the needs of the target patient group and orphan disease area is of critical significance too?

Underestimating the importance of instilling confidence and wellbeing in such patient populations is crucial to long-term compliance with treatment. The healthcare world is shifting towards payment for outcomes and the orphan disease area is unlikely to escape this.

Carefully designed device solutions can be aimed at specific needs of a patient population and, vitally, to improving dosing consistency and accuracy. These factors lead to improving not only safety and efficacy but also the patients' adherence and compliance, aspects of utmost importance for improving outcomes. Improving outcomes leads to greater overall success of the drug both in clinical trials and on the market, justifying pricing decisions and creating a stronger, longer-term competitive position.

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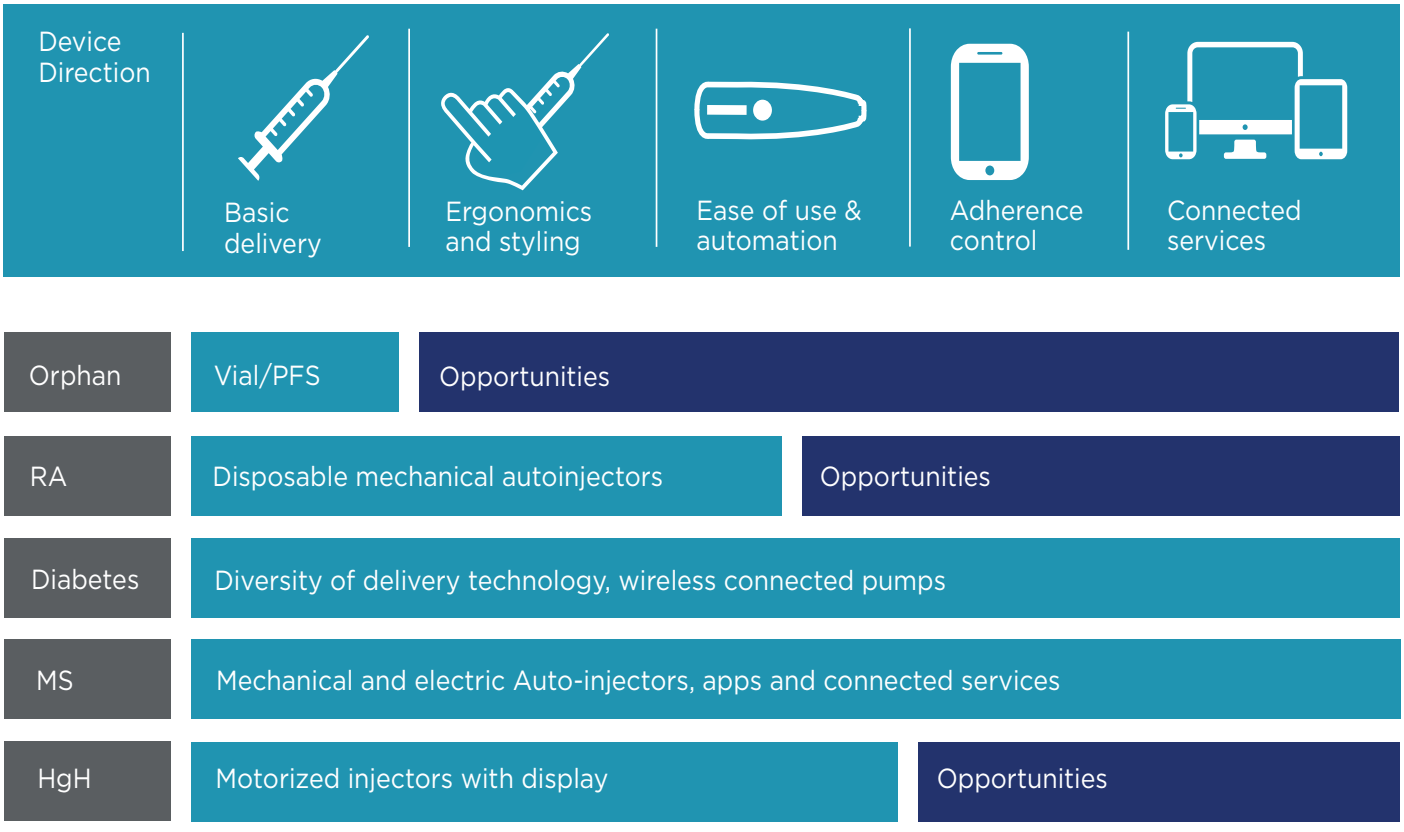

Figure 1: Examples of device and service maturity across therapies.

Fast-to-market approaches often involve formulating drugs that require reconstitution from lyophilised powders in a vial prior to injection. The reconstitution process requires the patient to carry out numerous steps whilst maintaining sterility and may potentially result in inconsistent reconstitution from dose to dose. Additionally, there are safety and emotional issues to contend with such as exposed needles not only posing a hazard to the user or caregiver, but creating fear and unease, often leading to lack of compliance in terms of continuing treatment. The arguments are strong for making self-injection as simple and user-friendly as possible. Indeed, there is growing evidence that reducing the number and complexity of steps required for self-injection improves not only consistency of dose delivered but also overall adherence to treatment.

> "...the overarching benefits that can be offered by such delivery systems to the orphan diseases sector are significant, and should be considered, as many have compound effects."

Using more advanced delivery systems to reconstitute drugs within the device, hide the needle from the user, as well as enabling a more consistent, easier and less fearful delivery is possible, but it is by far from being the norm.

Even for those orphan drugs that have achieved formulation into liquid preparations, the further application of prefilled syringes and even an auto injector, which is becoming more of the norm for many chronic diseases, is still lagging in the orphan arena.

Getting the drug on the market and into the hands of the patient as quickly as possible is a key driver for pharmaceutical companies, and this is extremely important for such orphan diseases where there is often such a lack of therapies. Unfortunately, thinking about the way such therapies are to be delivered is often not a dominant part of the total disease solution. Many view the utilisation of more advanced delivery systems as a diversification mechanism in a competitive market and thus do not see the need for such offerings for new market entrants where there may be a lack of significant competition. Additionally, due to the low volume of patients in any one orphan disease, a further perceived barrier is the ability to get a device manufacturer involved for such low volumes cost effectively.

However the overarching benefits that can be offered by such delivery systems to the orphan diseases sector are significant (see Figure 1), and should be considered, as many have compound effects. By their very nature orphan diseases have small patient numbers and clinical trial respondent numbers are allowed to reflect this. Consistent dosing within such small sample sizes, particularly. in later stage trials, where efficacy and improvements over other therapies is under scrutiny, is very important. Advanced delivery systems that can deliver doses repeatedly and accurately, in a user-friendly, non-threatening format, have the ability to provide results regarding actual efficacy more accurately for smaller populations, and are more likely to ensure adherence, compounding the effect of gaining accurate results.

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Coupling this with the ability to add connectivity to the device in addition to making it look and feel high quality, results in a range of extra values to patient, clinician and pharmaceutical company, both pre-market and post-launch. Training patients and caregivers to use such devices is simpler and cuts down time required with each patient. Devices can be made that offer obvious and simple user-steps that monitor usage, instil confidence and educate the user about the importance of certain aspects of their condition. All of these factors drive improvements in outcomes and the overall validated success of the treatment.

Complementing current early market access activities and programmes conducted in orphan drug development, the improved early interaction and careful understanding of care journey needs, together with strategic thinking about the drug delivery solution, will benefit patients, HCP, payers and pharmaceutical companies. Moving away from basic delivery mechanisms to more user-friendly, intelligent devices will add value to all stakeholders and must be the future for the orphan drug world in much the same way as the mainstream biopharmaceutical world.

ABOUT THE COMPANY

Medicom Innovation Partner (a Phillips-Medisize Company) is a leading global innovation, development and low-volume production provider focused on drug delivery devices and connected health solutions for the high-value rare disease and orphan drug market. Medicom Innovation Partner was established as a technology venture of Bang & Olufsen A/S back in 1989 and the company has been a dominant player within the drug device world for more than 25 years. Medicom holds a dedicated staff of more than 90 high-calibre innovation specialists, mechanical, hardware, software, quality assurance, regulatory and production engineers based in Struer, Denmark, and Cambridge, UK. Medicom has experienced considerable growth over the last five years.

As of May 31, 2016, Medicom is part of Phillips-Medisize Corporation. Phillips-Medisize is a leading global outsource provider of design and manufacturing services to the drug delivery and combination products, consumable diagnostics and medical device, and specialty commercial markets. The company has annual sales of over US$700 million with 80% of the total revenue coming from drug delivery, medical device, primary pharmaceutical packaging and diagnostic products such as: disposable insulin pens, glucose meters, specialty inhalation drug delivery devices, single-use surgical devices and consumable diagnostic components.

Together Phillips-Medisize and Medicom will become one of the leading players within the growing drug delivery device and connected health market.

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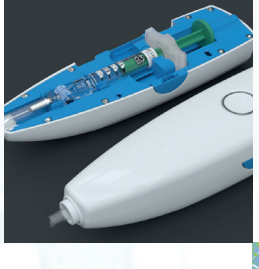
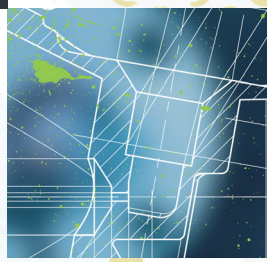
in patient centric
drug delivery systems





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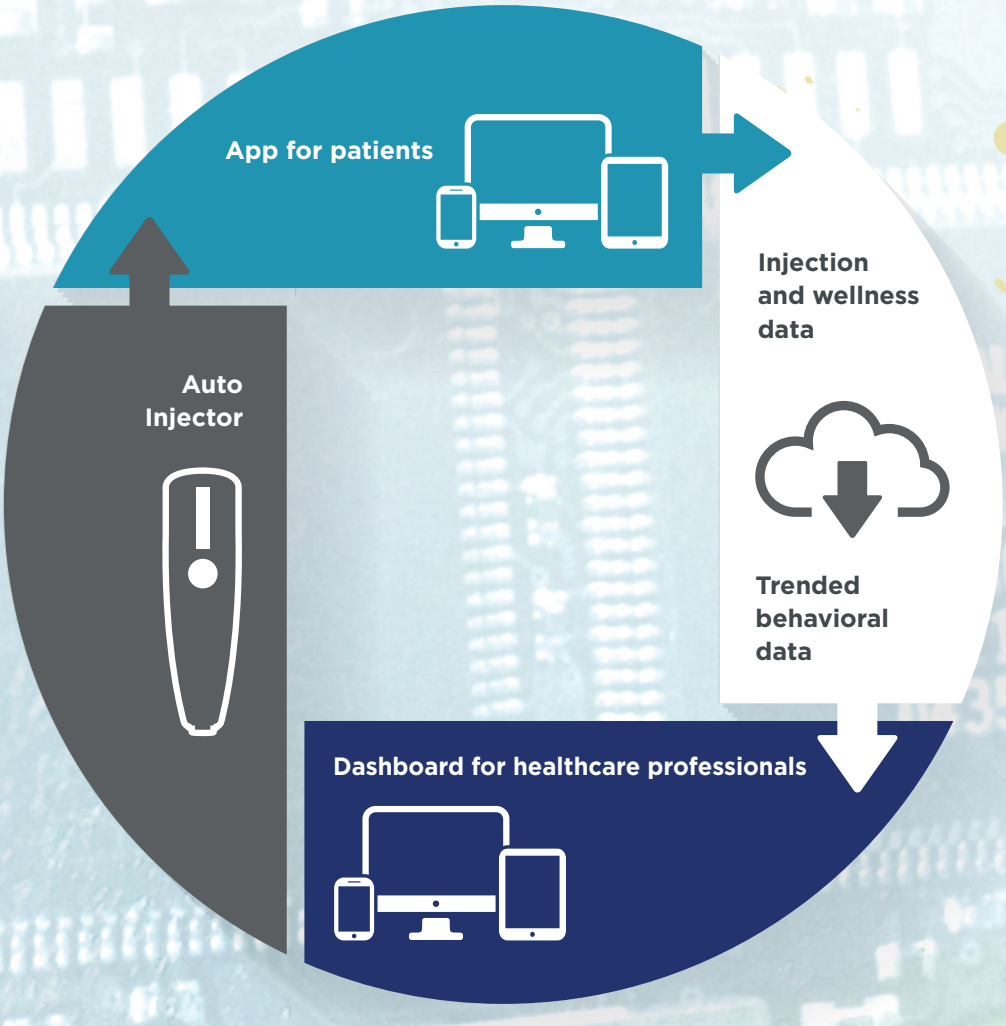

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