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Precision medicine for all common diseases with the SCIO℠ method

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Abstract

This article examines the advancements in scientific research and the challenges associated with translating them into clinical practice. To address the challenges the pharmaceutical and biotechnology industry faces, it highlights the success of innovative approaches in rare disease drug development, particularly in overcoming obstacles specific to orphan indications. More importantly, the concept of Strategic Clinical Innovation Organization℠ (SCIO) was introduced as a method to tackle the challenges in translational medicine as it has proven effective for orphan indications and holds potential for broader application in clinical trial strategy design. It emphasizes that SCIO℠ method's integration of early clinical and regulatory strategies, non-clinical evidence, and the utilization of AI and data analytics enhances trial efficiency and optimizes trial design and execution. In addition, this article stressed the significance of SCIO℠ utilizing biomarker validation in guiding precision medicine interventions and improving patient stratification and clinical trial design. Overall, the transition to precision medicine holds promise for delivering more individualized and personalized care to patients care, improving treatment outcomes, minimizing adverse events, and transforming healthcare.

Keywords: SCIO; Strategic Clinical Innovation Organization; Clinical Strategy; Regulatory Strategy; AI; Clinical Trial Strategy; Precision Medicine.

1. Introduction

The last 30 years have witnessed unparallel advancements in scientific research developing treatments that impact the course of diseases in many areas of medicine. Despite these advances, their translation from bench to bedside is lagging and clinical research remains outpaced. The productivity of pharmaceutical development has been declining due to high failure rates in clinical research. Clinical trials are expensive and often burdensome on patients. The resources, time, and funding grow with the clinical development moving from pre-clinical to phase III. Thus, the cost of a failed phase III trial is not just the cost associated with the trial itself but the cost of all prior research and trials, as well as the cost of lost time pursuing a potentially viable alternative. Clinical research strategy and design represents a fulcrum for the development of safe and effective drugs in a timely manner.

Regarding how to improve the efficiency of clinical trials, the rare disease drug model may provide an answer to the industry. Rare diseases drug development embraced an innovative approach to overcome the challenges specific of orphan indications which proven to be successful by a higher percentage of the FDA approved drugs 55% of orphan designated versus 45% non-orphan designated [1].

Strategic Clinical Innovation Organization℠ (SCIO) applies the rare disease model and achieved a great success with clinical trials for common diseases with a significate increase in patient safety and chance to success while reducing time and capital inputs. This article will explain how SCIO℠ may help achieve the efficiency of a safety trial with the use of orphan drug development model.

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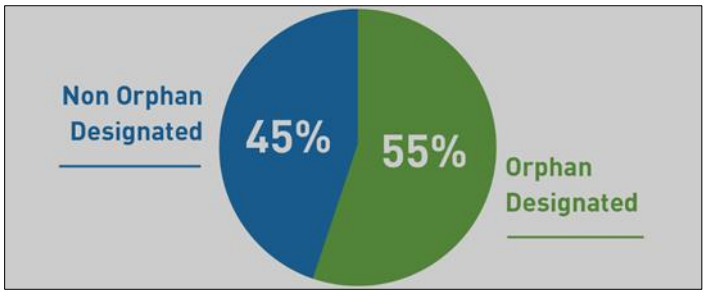

Figure 1 The percentages of Approved Orphan and Non-Orphan drugs [1]

2. Precision Medicine: The Paradigm Shift and Challenges

A transition to precision medicine will replace buster medicine to provide more targeted, effective, and personalized patient care. This paradigm shift can improve treatment outcomes, minimize adverse events, optimize resource utilization, and transform healthcare.

Buster medicine, also called the "one-size-fits-all" approach, does not suit the needs of the current medical world. Buster medicine created two significant problems in treating patients: ineffective treatment of individuals with different genetic and environmental backgrounds (lack of efficacy) and increased adverse events due to the failure to consider patient differences (safety concerns). Contrary to buster medicine, precision medicine customizes treatments to a subpopulation with a common susceptibility to a particular disease or similar response to a specific drug [2].

The precision medicine transition involves a departure from the one-size-fits-all approach and represents a shift in healthcare towards tailoring treatments to the molecular alterations present in an individual's disease, and aims to provide more targeted, effective, and efficient care. That's why precision medicine can be more effective and have fewer side effects compared to traditional, broad-spectrum treatments.

Precision medicine, despite its benefits, faces several challenges that need to be addressed for its successful implementation. One of the most critical challenges is to identify reliable and validated biomarkers. Choosing the most relevant biomarkers for a particular disease or treatment response prediction is a critical step. Identifying biomarkers that are specific, sensitive, and clinically meaningful requires extensive understanding of diseases complex genetic interactions, and the interplay between genetics, epigenetics, and environmental influences affecting diseases' prediction, risk assessment, and treatment selection. Furthermore, biomarker validation is essential to the success of developing precision medicine. Biomarkers can include genetic mutations, gene expression patterns, protein levels, or other molecular indicators. We can determine drug assets' clinical utility and development potential by identifying relevant biomarkers associated with specific diseases, treatment responses, or adverse events [3]. In addition, biomarker validation also plays a vital role in clinical trial design and patient stratification [4]. By identifying biomarkers associated with treatment response or disease progression, clinical trials can be designed with appropriate patient population stratification to increase the chances of detecting treatment effects and improving trial outcomes. Finally, validated biomarkers can be used to monitor treatment efficacy and safety over time. Tracking changes in biomarker levels allows adverse events to be closely monitored and potentially predicted. This proactive monitoring approach allows for early intervention and adjustment of treatment plans as needed [5].

Another critical challenge facing precision medicine is the decline of clinical research effectiveness. Over the recent years, many technologies were adopted, which were considered critical for delivering more simplicity and convenience and addressing the decline of clinical research effectiveness. Unfortunately, the deployment of many technology initiatives has proven unable to reduce challenges like late failure and rising costs. It is needed to have targets and strategic goals, coupled with a comprehensive understanding of relevant operations and linked processes, for the technology to bring the most significant value.

The successful implementation of precision medicine relies on the integration and analysis of large volumes of diverse data, including genomic data, clinical data, lifestyle information, and environmental factors. Artificial intelligence (AI) and machine learning algorithms are employed to extract meaningful insights from these complex datasets, enabling better patient stratification, treatment predictions, and decision-making.

3. Strategic Clinical Innovation Organization (SCIO℠) to Streamline, De-Risk, and Accelerate Clinical Research

SCIO is the acronym for "Strategic Clinical Innovation Organization", and in Latin “Scio” means to know, to understand, and the knowledge of "How-to." The concept of SCIO was developed to address cost efficiency and risk management from pre-clinical to market drug development. It involves an innovative method to establish specific clinical and regulatory strategies and roadmap as part of a pre-IND and IND submission before starting tactical clinical activities (i.e., CRO).

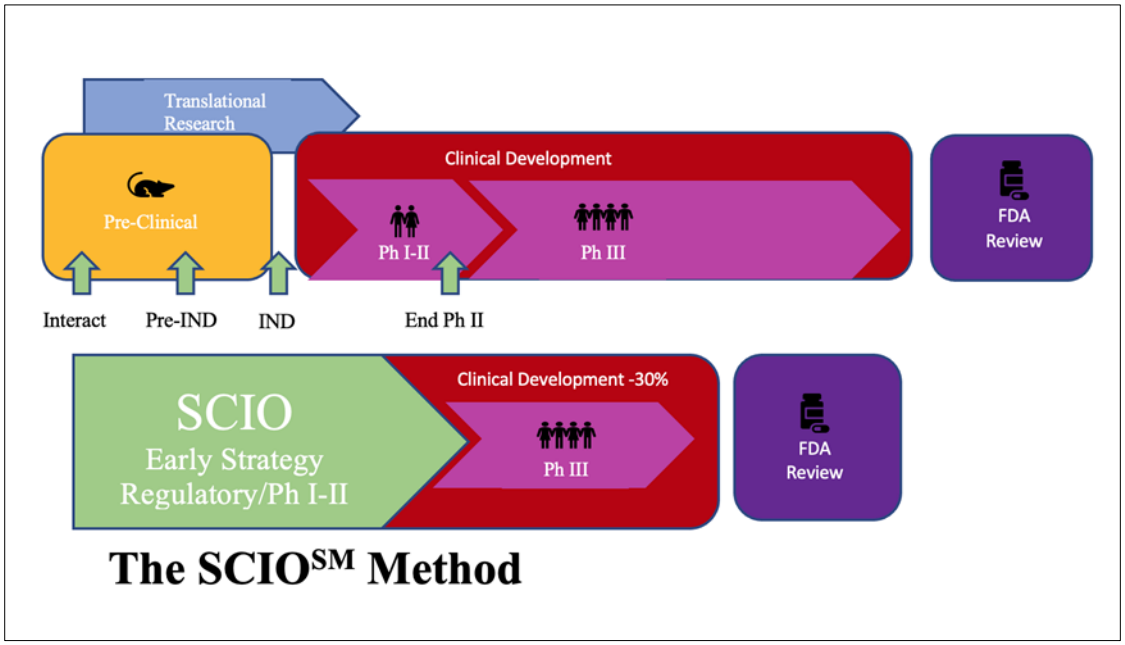

Figure 2 The Strategic Clinical Innovation Organization (SCIO℠) Method

The SCIO method finds its innovative and strategic approach from the development of rare diseases therapeutics. Clinical trials for a rare disease require a tailored approach, collaboration, flexibility, and patient engagement which were proven to be critical for the successful conduct of trials for orphan population. These rare diseases drug development model anticipated and paved the way to precision medicine. Thus, the SCIO method starting from the rare diseases experience is an innovative and strategic method to drug development for precision medicine in common diseases. The SCIO method includes strategy and technologies adoption to overcome the challenges of precision medicine drug development, and the adoption of artificial intelligence (AI) has been the latest update on the concept of SCIO.

The SCIO objective is to innovate and advance the clinical development process, improving clinical trials' efficiency, effectiveness, and outcomes. SCIO employs strategic approaches and adopts innovative practices to overcome the challenges associated with traditional clinical development. Leveraging artificial intelligence (AI), data analytics, and a deep understanding of regulatory affairs and medical knowledge to streamline processes, enhance patient recruitment and engagement, and optimize trial design and execution.

The core of the SCIO method is to innovate. Thus, SCIO has been evolving alongside advancement with new technologies and cutting-edge scientific concepts. That is why SCIO tackled the challenges coming with traditional clinical development and transformed itself to meet products' specific needs.

The clinical trial process has stayed the same for decades. Although we are witnessing the adoption of multiple tools and technologies, they cannot solve the core problems of high expense or failure. Current challenges such as patient enrolment, lower patient retention rate, complexities around Clinical Research Organizations (CROs) selection and management, and protocol complexities severely delay or restrict small and medium biotech companies from introducing a product to market at the right time. SCIO devises an innovative translation and early development method, which integrates nonclinical evidence into clinical research, thus supporting the regulatory strategy and building the efficacy and safety evidence since the first man to reduce risk in late clinical trials while preparing the product for

market positioning. SCIO overcomes the silos and, at the same time, promotes a perfect match between the product mechanism of action (MOA) to target indications.

4. SCIO's Integration of Rare Disease Drug Development Model

Designing clinical trials for rare diseases requires choosing an appropriate trial design based on the disease characteristics and available patient population. Traditional randomized controlled trials may not always be feasible due to limited patient numbers. careful consideration of the unique characteristics which involves flexibility, and creativity to overcome the challenges posed by small patient populations. Identify the target patient population based on specific disease characteristics, such as genetic mutations, biomarkers, or clinical phenotypes. Collaborate with patient advocacy groups, disease registries, and healthcare providers to facilitate patient identification and recruitment. Determine the appropriate control group or comparators for the trial. This can include historical controls, external control groups, or within-patient comparisons if applicable. Select endpoints that are clinically meaningful and can demonstrate the efficacy of the intervention. Consider surrogate endpoints, patient-reported outcomes, biomarkers, or other measures that provide clinically meaningful information about treatment response.

The traditional clinical development process for new therapies presents many challenges that slow the trial timeline and increase costs. Without an early clinical and regulatory strategy before deploying the start of CRO tactic activities and study enrolment, sponsors may face difficulties that will impact trial execution and results. They can experience challenges in recruiting and enrolling patients due to suboptimal indication selection; getting lost in a complex regulatory process and misaligned with agencies due to the lack of a strategic regulatory roadmap; trial delay due to the mismanagement of CROs and unexpected risks happening throughout the trial conduct, and difficulties in interpreting data due to a lack of clinical strategy with anticipation of how the data might look like during the planning phase. In addition, any of the issues that arise during the conduct of the study will also yield an increase in trial cost.

Based on the belief that there is no goal without a strategy, any drug development needs a strategy to generate quality data. SCIO is designed to help pharmaceutical innovators address their concerns, maneuver around evolving challenges, identify time and cost efficiencies, and relieve risk management on their journey to market approval. The SCIO method aims to learn and predict so better decisions can be made for a successful drug opportunity. The foundation of SCIO method is to know the potential of each preclinical asset and indicate the level of certainty for each strategic option.

5. Conclusion

Drug development requires deep experience. However, a common mistake we often must correct about the experience is using yesterday's assumptions for tomorrow's drugs. Today's technological molecules require innovation in the development process. Data also shows that early strategy (from indication selection and validation into phase I-II trial) leads to a competitive advantage that a late development redirection of optionality and decisions cannot replace. It indicates that with different strategies, one product may have different destinies. That is why weighing options before starting regulatory activities and ensuing clinical execution is essential, which means bringing together the right population with meaningful endpoints (clinical and surrogate) and negotiating with regulatory agencies.

SCIO clinical research method customizes strategy and roadmap for each pharmaceutical and biological candidate. It allows for robust clinical data generation and informed decision-making during the clinical development stage. AI can contribute to assessing the potential of preclinical assets and predicting the level of certainty for strategic options by analyzing various data sources and applying machine learning algorithms during patient enrollment and recruitment, trial design and optimization, predictive modeling, and risk assessment/mitigation, drug repurposing, real-time monitoring and adverse event detection and analysis, data analysis and decision support.

Compliance with ethical standards

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Disclosure of conflict of interest

We have no conflicts of interest to disclose.

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