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Industry Perspectives in Modern Drug Research and Development Strategies

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Keywords: Pharmaceutical industry, research and development, challenges, new trends, digital

1. Introduction

The pharmaceutical industry is a major driver in addressing unmet medical need and has been a key asset to the world's economy. Its global market, currently valued at approximately 1.2 trillion USD, has been increasing in the past decade. With the global population growing and average life expectancy increasing, taken together with the increasing prevalence of major diseases such as cancer, diabetes and cardiovascular conditions, unmet needs continue to grow. Recent trends, however, suggest that the rate of growth in the pharma industry has decreased and is expected to slow further in the coming years. Furthermore, despite major advances in biotechnology and medicine, there is still no curative therapy for most chronic diseases; some diseases have no treatment at all.

The present review highlights the difficulties the pharmaceutical industry is currently facing and outlines new trends and selected key research and development strategies that are being implemented to meet the challenges.

2. Key Challenges for the Pharmaceutical Industry

Innovative drug development is getting more expensive overall and in some cases, less efficient. A recent report by Deloitte that has tracked a cohort of 12 leading global biopharma companies over several years reveals a systemic, cross company, decade-long decline in the productivity of research and development. At the same time, payers and customers increasingly demand cost efficient therapies, together with supportive real world clinical evidence.

The conventional model of chronic, high priced treatment is no longer sustainable as payers and health care stakeholders, including patient group

representatives, are demanding short-term and more affordable prevention strategies or treatments that can offer a cure. In parallel, the repeated use of self-administered and inexpensive over-the-counter drugs is increasing.

Furthermore, certain diseases are very difficult, either scientifically or commercially for pharma to invest in. For instance, psychiatric diseases such as schizophrenia, depression or autism have complex pathology without translatable preclinical models and objective clinical endpoints or biomarkers. Another example is infectious diseases. Similarly, ultra orphan conditions are often neglected by research and drug development and as a result, such diseases have limited or no treatment. This is a challenge not only for the pharmaceutical industry but also for the whole society and most importantly, for the patient.

Furthermore, the pharmaceutical industry now has the worst reputation compared to all industries according to a recent survey by Gallup including 1,525 adults in all 50 states in the US. Despite all these challenges, drugs with new modalities are coming to the clinic at an increasing rate and new technologies are emerging.

3. Current Trends and Strategies in Pharma

Big Data refers to extremely large data sets analyzed computationally to reveal patterns, trends, and associations. Their use in the pharma sector is rapidly evolving and expected to facilitate drug discovery, research and development. Similarly, Artificial Intelligence (AI) applications, together with cloud computing have also been increasingly used by pharma companies to help drive fast data-driven decisions for greater efficiencies, cost-savings and competitive advantages.

The revolution of digital technologies is transforming multiple elements of drug development,

such as drug discovery, trial design, and endpoints. For example, randomized clinical trials have shown the effectiveness of selected mobile applications in reducing symptoms of depression. In asthma, self-monitoring of respiratory symptoms with a connected mobile spirometer has been shown as a feasible, safe and satisfactory tool for patients.

Pharma has clearly become more patient centric: endpoints, real world evidence and experiences that matter to patients are increasingly used in trials and discussions with regulators, payers and governments.

Novel study designs pioneered in oncology are being implemented in general medicine to investigate multiple hypotheses through concurrent sub-studies. Those include multiple treatments (umbrella design) or populations (basket study) or adding/removing arms during the trial (platform study). These approaches, now increasingly supported by regulators, may offer enhanced efficiency.

Personalized and tissue-agnostic therapies are now a reality. The strategy of using the same drug to treat different disease types that have the same molecular abnormality (e.g., genetic mutation) targeted by the drug has been introduced in oncology. This novel approach also has potential in complex autoimmune diseases, where a homogeneous endotype of different conditions identified by a common biomarker has a better chance to respond to a targeted therapy than simply treating unselected patients.

Not only discussions but also active collaborations between pharma, health care, governments and patient representatives are increasing. A prime example is the Innovative Medicine Initiative (IMI), a European Union-led public-private partnership where the relevant stakeholders including academia, industry, small enterprises, regulators, payors, and patients work together to facilitate health research and innovation.

Finally yet importantly, transformative innovation has resulted in new complex modalities such as cell and gene therapies. Novel therapies are emerging based on pioneering technologies such as nanoparticles, anti-sense oligonucleotides, organoids, digital therapeutics, or 3D printing. These innovations often come from small biotech firms whose contribution to big pharma is now essential.

4. Summary and Conclusion

The pharma industry is experiencing challenging but exciting times today. Scientific challenges,

price pressures, and regulatory demands contribute to a major transformation (**Table 1**) of the pharma industry. Overall, pharma companies must be alert and have the capability and willingness to fundamentally reshape their business model to remain competitive and grow in order to deliver new medicines. The opportunities and remaining unmet medical needs are both enormous.

Table 1 Key trends and their scope

Trend	Impact
Transformative scientific innovation	Efficiency, new therapies
Big data and digital technologies	Broad impact to improve efficiency
World wide evidence	Patient focus, reimbursement strategies
Novel study design	Efficiency, ethical considerations
Personalized medicine	Increased efficacy
Public private partnerships	Collaboration

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