MONTHLY DEEP FOCUS:

What are some unique examples of the applications of Real-World Evidence (RWE) in the biopharmaceutical industry?

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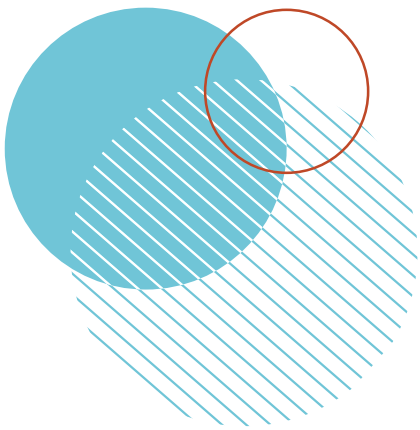
Over the past decades, with increased usage of Electronic Health Records (EHR), adoption of wearable devices computers along with electronic health insurance data across the global patient population have enabled us to capture vast volume and variety of data very fast. It is not necessarily a problem of data scarcity but a problem of plenty!

The real-world data powered by "right" tools & analytics has potential to impact every aspect of drug development and launch. Today it is a well-recognized fact that drug development has become very expensive as well as time consuming. Yet, often, the desired outcomes are still not achieved.

Often patients, providers, and payers lack answers to fundamental questions like: "What treatment is best for me or my patient?", "How do patients treated in the 'real world' perform on this therapy?", and "What is the differential value of this therapy relative to other treatment options?"



The evidence gap persists despite a richness of available data, novel analytic methods, and inexpensive computing and genomic sequencing power. The insights generated from data collected during routine clinical practice i.e. Real-World Data (RWD)—provides a platform which can close the gap from bench (clinical research) to bedside (clinical practice).

Figure 1 outlines the time and percentage of expenditure on each phase of drug development.

Early Discovery & Pre-Clinical Research: By leveraging RWD powered by "right" analytics tools that leverage life-sciences knowledge, we can reveal connections and relationships among genes, drugs, diseases, and other entities. RWE analyses can identify biomarkers of therapeutic response and resistance to optimize a drug development strategy. For example, clinico-genomic database with tumor sequencing information from large patient dataset can help identify and characterize genomic profiles of patients with rapid progression or otherwise poor prognosis. Researchers can generate new hypotheses backed with evidence-based predictions. This approach can cut down risk in some elements of early discovery by focusing on identification of high-responding patient cohorts.

Leveraging Geo-Mapping as a Valuable Tool to Recruit and Design Phase II and III Trials: A few key challenges that impact cost as well as timing of the trials are: ensuring that trial design especially patient population is reflective of real world, identifying 'right" patients for recruitment in trials, and identifying site locations. RWE can help answer these question and geo-tag patients. In certain conditions, especially in oncology, it may not be possible to have a control arm or a control arm can be difficult to recruit – prolonging the trial. Historic Real-World Data can be leveraged to create or simulate a control arm that may reduce trial size (that is, required number of patients), duration, and cost.

Drugs for conditions with high unmet need: Today FDA's Breakthrough Therapy Designation Pathway allows stakeholders to get many essential drugs to patients faster. In these situations, RWE provides an alternative means of satisfying regulatory requirement that is faster, cheaper, and more representative of real-world populations. This is a major win-win for patients and pharmaceutical manufacturers!

RWE has seen a widespread use in early understanding of positioning, pricing, and market size. In a crowded space where many drugs exist for an indication, it's important for companies to identify in

Fig. 1

Problem

Drug Research/ Discovery	Development/ Pre Clinical	Clinical Trials	FDA Review	Phase IV Studies
Basic research	Laboratory and animal testing	Patient testing	1 Drug approved	
10,000 compounds screened	250 compounds tested	5 compounds tested		
5 years	**1.5 years**	**6 years**	**6 months - 2 years**	**3+ years**
27% of R&D spend		**54%** of R&D spend	**5%** of R&D spend	**14%** of R&D spend

Solution

Preclinical/ Phase I	Phase II	Phase III	Market	Phase IV
PK/PD driven drug development	Curate and standardize biomarker data for clinical trial participants	Better trial design Targeted point recruitment Precicely target highest levels of unmet need	Medication adherence Engage payors much earlier in R&D Combine health, social and behavioral patient data	Combine RWE with FDA adverse event reporting system Include social media for signal validation Conduct studies with RWE cohorts

what population sub-set the drug works best and what is the differential value.

Indication expansion is an important part of lifecycle management and has tangible benefits for patients. Label expansion is often very important for ensuring clinical guidelines, inclusion, and treatment coverage. Recently, the FDA launched Friends of Cancer Research's efforts program to identify older products needing updates in labels specifically for generic therapies where labels no longer fully reflect how a therapy is used in practice. Although the pilot was terminated due to complexities related to execution, if used with right framework RWE has potential to provide evidence required for such initiatives.

As healthcare landscape becomes more complex coupled with ever escalating cost of care, RWE has potential to provide valuable insights and bring efficiencies that are much needed by all the stakeholders: be it pharmaceutical companies, patients, providers, or payers. There is a need to democratize the data and tools so that the RWE is readily available to small and mid-sized companies. With advancement of machine learning and Artificial Intelligence, we will see wider adoption and usage of real-world data (RWD).