WHITE PAPER

Reversing the Traditional Drug Development Model

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Introduction

For more than a decade, the pharmaceutical industry has been facing mounting public, government, and internal pressure to contain development costs, lower drug prices, and ramp up pipelines faster. At the same time, the cost, complexity and globalization of drug development continues to escalate. These challenges plus financial, political and scientific developments are driving a transformation in the traditional biopharmaceutical business model. Big Data technology and analytics are enabling new, more efficient drug development pathways.

Bringing a drug to market is a slow, expensive, and risky business. It takes about 10-20 years to develop a compound, and the failure rate is nearly 95%.[1] A report by the Tufts Center for the Study of Drug Development pegs the cost at $2.6 billion.[2] Getting a product to market in some cases can potentially cost more than $5 billion, according to an analysis by Forbes.[3] Industry companies can no longer bear to conduct business as usual.

Clearly the traditional "bench-to-bedside" drug development model is unsustainable. As drug sponsors seek new ways to meet the challenges of today's stringent business and industry climate, they are reinventing themselves, adopting new strategies to build pipelines more efficiently.

This paper discusses emerging strategies for making drug development more efficient while reducing costs and risk, such as in-licensing drugs for additional indications, leveraging existing compounds in the product portfolio for other indications (i.e. what Abbvie did with Humira), and reversing the standard steps in a traditional development model. Also discussed is how Big Data, outcomes research, predictive analytics, innovative study designs, and improved data accessibility are playing a major role in reshaping R&D practices to meet current demands and improve value in new product development.

New Strategies, New Business Model

As for new approaches to the selection of drug candidates, large companies have cut back on internal early development efforts and are relying more on in-licensing candidates or line extensions. There is a strong trend to grow revenue at lower costs by in-licensing approved products for additional indications. A strategy to speed the drug development process is new trial designs, such as adaptive clinical trials.

Coupled with these strategies, sponsors are reversing the traditional development pathway to reduce the high risk of failure, focusing on the economics of development and market acceptance at the outset. They begin by researching the marketability of the compound or in-licensed drug -- determining payer and provider acceptance, the chances of being added to formularies and at what level, and the drug price. Then they proceed to R&D (often shortening the process) and finally, submission for approval and delivery to patients. Big Data technology is fueling the new development process.

For example, assume a promising compound is available for in-licensing. The next steps might look like this:

- Analyze data from previous clinical trials using a visualization-based data discovery solution to form hypothesis of additional indications to address with the compound.
- Use an industry data service to look at existing therapeutics on the market for the additional indications. Review economic data, patient demographics, and sales of current therapies as well as products in development for the same indication to determine whether there is a market for the product.
- If there is already other medication on the market for the indication, review payer and provider data and the costs of similar medication for the same indication to assess whether payers are likely to put the new medication on the formulary.
- Leverage health economics outcomes research to determine the optimal price for the medication. Look at the state of care and costs for alternative treatments, such as hospitalization or surgery.
- Leverage translational medicine techniques to better match medications with patients by using biomarkers to stratify patients for treatment, resulting in improved therapeutic response and smaller, more efficient trial designs.

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Leveraging Big Data Technologies and Analytics

Applying Big Data technology and analytics to manage the explosion of real-world data available in the pharmaceutical industry can help sponsors make smarter decisions about which drugs to pursue and more efficient ways to develop them. Big Data is an emerging field that involves research in which a vast amount of information from multiple large, diverse data sets can be analyzed to gain new insight into products, processes, relationships, and outcomes. Used to quickly test new ideas or shape predictions, Big Data focuses on finding trends and patterns that would otherwise be difficult, expensive and even impossible to find using traditional information-gathering and information-processing methods.[4]

In contrast, real world data - including data from pharmacy claims receipts and data services such as IMS - involves information on actual patient contact and eventually, outcomes data. By understanding more about this process of care, researchers can employ advanced analytics to make medical care more targeted, efficient, and effective.

Analyzing real world data offers many advantages, such as enabling more precise design of clinical trials and gathering information about which subpopulations benefit the most from specific therapies. Mining this vast volume of valuable data can also help characterize diseases and patient populations, assess competitive products, target products, evaluate health economics, and identify subpopulations with superior product response.[5] Researching data related to previous trials of similar agents can also identify adverse effects on trial subjects, predict the likely profitability of the drug being tested, and understand trends in applicable regulations.

Life science companies are spending more and more on health economics and outcomes research but struggling with data volumes, data integration, long-running analyses and data visualization. Gaining the technological capabilities to benefit from Big Data is imperative to sustain pipeline growth.

Establishing the Optimal Technology Infrastructure

Leveraging Big Data technology can facilitate determining a drug's marketability and price. Advanced technology can combine historical and clinical data related to previous trials of similar agents, and help predict the likely profitability of a drug based on the historical analysis of similar agents and size of the potential market.

Acquiring these capabilities requires an investment in tools and technologies, including the infrastructure and related software, such as integration software that can aggregate and store data captured in disparate systems. Technology such as Apache Hadoop, and the Hortonworks Data Platform (HDP) combined with TIBCO Spotfire® can unify the data into a single data pool (aka "data lake") with centralized security, governance and operations to perform analyses of both structured and unstructured data. With Apache Hadoop, it is easy and cost effective to centrally collect, store and access unlimited quantities of diverse data, such as electronic health records, clinical lab results, imaging records, omics data, social media posts, blog articles, and publications.

With TIBCO Spotfire®, the easily accessible data provides a single point of access for advanced analytics on unified data. Users are empowered to quickly discover insights through meaningful visualizations, interactively visualizing outcomes data without requiring structural or semantic changes to that data. They gain access to their data in days compared to weeks or months with a standard enterprise data warehouse, and dynamically aggregate, filter and access micro-level data details for near real-time analytic visualizations. Predictive analytics using a platform that consolidates data, presents data on dashboards, and provides interactive visualizations and data analytics enables rapid interpretation and decision-making.

Advanced data analytics can shorten the development pathway. For example, a sponsor researching a drug for a second indication which enabled researchers to study the drug's impact on new biological pathways was able to understand the fundamental biology underlying the drug's mechanism of action and determine its effect on a new molecular pathway. The technology helped associate dosages with patient responses and demonstrate the drug's significant differentiation over the standard of care. The analytically supported results, along with a significant productivity improvement and avoidance of Phase 1-3 clinical trials helped the researchers file for drug approval in record time and save major costs.

Determining Optimal Trial Design

To improve efficiency and reduce the costs of the development process, one emerging approach is to use translational medicine techniques to better match drugs with patients. Several recent clinical studies used biomarkers to stratify patients for treatment, resulting in improved therapeutic response and smaller, more efficient trial designs. Thus translational medicine holds the promise not only to improve drug development efficacy and safety, but also to reduce clinical trial cost and success.

For example, for a drug being developed for a second indication, looking at data analyzed and subpopulations in previous trials can help determine the optimal subpopulations for the current trial. Subjects tested for the primary indication who have the same biomarkers and demographics required for the new study and those who have the secondary indication can conveniently be enrolled in the new study. Using these translational medicine techniques, researchers can access a more targeted study population and the study may require fewer subjects. By mining Big Data, the criteria for including patients in a trial could consider significantly more factors, enabling smaller, shorter, less expensive and more powerful trials.

Translational medicine technology platforms are being developed to provide the best Big Data solutions that will enable clinical and translational researchers to drive change and shape the future of medicine. The platforms are being designed to seamlessly integrate research data and clinical data to enhance the capability of clinical development programs by integrating a wide variety of assays - such as in-vivo and in-vitro imaging technologies, next generation sequencing, and multiplex enzyme-linked immunosorbent assays (ELISA) -- to the analysis of clinical data. Workflows for omics and imaging data analysis, including easy access to publicly available data repositories such as Gene Expression Omnibus (GEO) and tranSMART, are also being developed.

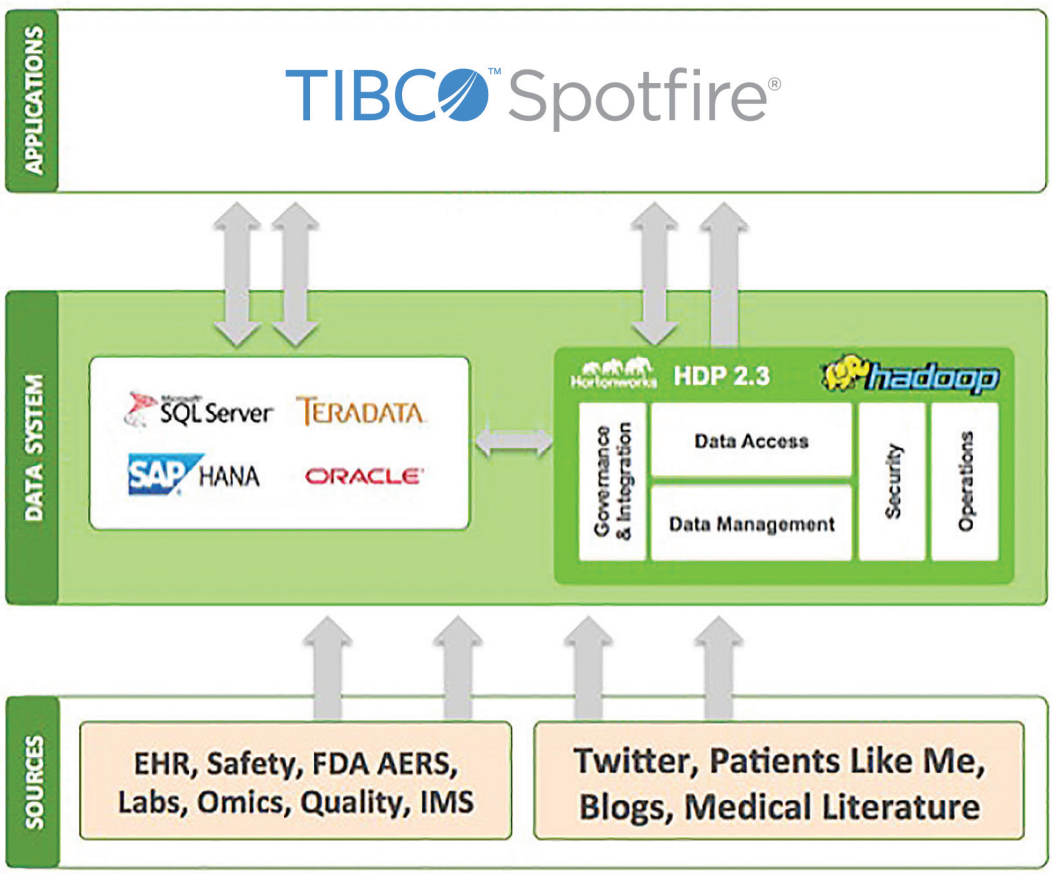
Innovative Strategies for Sustainable Pipeline Growth

Dramatic changes are on the drug development horizon as biopharmaceutical sponsors struggle to keep pace with the demands of today's stringent industry environment. To grow revenues and build pipelines, sponsors must bolster R&D efficiency and innovation. Current drug development models are inadequate to meet these demands. Some larger sponsors are beginning to apply new strategies and business models, leveraging Big real-world Data to achieve significant time- and cost-savings, improve decision-making and gain tremendous value in drug development.

The McKinsey Institute estimates that applying Big Data strategies can generate up to $100 billion in value annually across the U.S. healthcare system by optimizing innovation and improving the efficiency of research and clinical trials.[6] Leveraging Big Data can improve the ability to identify potential drug candidates and develop them into approved, reimbursed medicines more quickly.

Incorporating advanced technology to improve Big Data analytical capabilities and provide consistent, reliable, well-linked data is urgent, a survival tool for a sustainable future. Platforms with visual, interactive dashboards allow rapid decision-making and enable users to make deep examinations of data. The industry must adopt data systems that can quickly produce a lot of integrated data, and gather, analyze, store and respond to real-world outcomes and claims data. While industry adoption of new development models and technology platforms has been slow, as the industry is traditionally risk- and change-averse, sponsors are realizing the benefits of the Big Data opportunity in drug development and the necessity of improving their approach to manage and analyze data.

Example Architecture Diagram



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