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AI/ML is changing the game in drug discovery and development

In our previous post, we discussed how life sciences companies can increase real-world data's (RWD) effectiveness and value by developing a data strategy based on the analyses they want to conduct with that data.

In this follow-up article, we explain how using artificial intelligence/machine learning (AI/ML) presents a unique opportunity for companies to increase speed and agility in drug development and lower the cost of bringing lifesaving therapies to market.

The drug discovery and development process—early discovery, pre-clinical phase, clinical phase, and commercialization—is lengthy, expensive, and laborious. Bringing a new drug to market can take 10–15 years, and a recent estimate puts the cost for US manufacturers at about $1 billion.[1] What's even more shocking is that the success rate for experimental drugs to enter the market is only 14%.[2] Speed and efficiency, therefore, are critical at each stage of the process—the combination of the right data and advanced AI/ML technologies are key.

AI/ML needs span the drug life cycle and differ for each development phase, typically requiring pharma companies to marry RWD and internal data, such as clinical trial, genomics, and imaging data. Applying AI/ML to these voluminous datasets presents a huge opportunity for pharma R&D teams, as finding the right therapy can be similar to looking for a needle in a haystack.

Understanding AI/ML types and applications
AI/ML algorithms broadly fall into three categories:

- **Supervised learning.** These models require labeled datasets (i.e., ground truth). For example, CT scans or other medical images must be labeled with the true outcome, such as "disease" or "no disease," by medical experts. Supervised algorithms then train by learning from these labeled datasets and predict the outcomes for new datasets (i.e., not used for model development). Examples of supervised machine learning algorithms include logistic regression, random forests, support vector machines, and neural networks among others.

- **Unsupervised learning.** These models aim at detecting underlying relationships or patterns in the dataset. Unlike supervised models, they do not require labeled data. Some common unsupervised algorithms include k-means clustering, principal component analysis, and autoencoders.
- **Reinforcement learning.** Unlike supervised and unsupervised models, reinforcement learning doesn't learn from data; instead, it learns from experimenting. These models learn to solve a task by trial and error, getting rewarded for best actions, and getting penalized for errors. Reinforcement learning is a highly effective approach in early discovery for designing molecules with desired properties for various disease indications.

Numerous success stories have been emerging—so much so that 90% of large pharma companies launched AI/ML initiatives in 2020 alone.[3] The following AI/ML use cases illustrate the rich array of opportunities available to pharma companies across the drug development life cycle.

Early discovery phase. The first step in the drug development process is to identify a pipeline of candidates along with biological targets that might have roles in diseases to engineer novel therapies. Specific examples include:

- **Target discovery and validation.** This involves voluminous data, such as medical images, genomic data, chemical structures, and more. ML and deep learning (DL) can comb through these massive datasets to discover meaningful patterns for new drug development that may be overlooked by scientists using traditional approaches.
- **Lead screening.** Both supervised and unsupervised ML algorithms can accelerate the laborious process of discovering the best drug candidates that interact well with gene targets to reduce the failure rate during the clinical development phase.
- **Drug repurposing.** AI models applied to knowledge graphs have been employed for drug repurposing whereby existing drugs are used to treat challenging diseases, including COVID-19.[4]
- **De novo design.** Reinforcement learning is increasingly gaining traction in generating novel molecular structures and drug-like molecules to tackle critical diseases with unmet needs.

Pre-clinical phase. Drug safety is a major challenge in bringing new drugs to market. Pre-clinical evaluations are of the utmost importance to prevent toxic drugs from moving into the clinical trial phase. Despite this, high toxicity is still a major contributor to drug failure, accounting for two-thirds of post-market drug withdrawals and one-fifth of failures during clinical trials.[5] Accurate toxicity estimates are essential to maintain drug safety and help reduce the cost and development time of bringing new drugs to market. For example:

- **Predicting toxic outcomes.** A widely used method called QSAR (Quantitative Structure–Activity Relationships) uses ML models to predict the organ toxicity of drugs based on their structural properties. ML models are applied on massive chemical and molecular databases to help identify drug compounds with the least amount of toxicity for further testing.[6] Algorithms include random forest, k-means clustering, ensemble learning, and deep learning models.

Clinical phase. AI and ML can provide significant benefits across various aspects of the clinical development phase. Examples begin with protocol design and extend through site selection, trial conduct, and digitizing/optimizing data management and analysis.[7] A striking example is how COVID-19 vaccine developers used RWD and AI/ML to dramatically compress the traditional years-long drug development timeline.[8] Application areas include:

- **Site selection.** Using powerful ML and DL models to automate analysis of large amounts of data—both health-related (RWD) and non-health-related (e.g., foot traffic data, wearables, mask-wearing, and social distancing data)—scientists were able to predict COVID-19's outbreak in various countries/regions and the way it would spread. Identifying hotspots was crucial for opening clinical sites in these locations so enough patients could be recruited for trials.

- **Patient stratification.** Striving for patient diversity (race, ethnicity, and geographic location) in the recruiting process is important so that participants are representative of the real-world scenarios needed to run successful trials. Predictive models help identify such sub-groups. Models can also help researchers understand how different treatment courses may affect patient outcomes.

Commercialization phase. AI and ML models in combination with RWD and real-world evidence have the power to transform commercial analytics. Predictive analytics can help identify highly specific patient populations most likely to benefit from the drug, provide insights into health care provider (HCP) prescribing patterns, and maximize the impact of digital campaigns to boost sales. Their use in pre- and post-launch commercialization strategies may include:

- **Market segmentation and HCP targeting.** AI algorithms are particularly useful for patient segmentation in therapeutic areas like oncology, where patient populations are highly specific for a drug approved for a particular line of therapy. Supervised ML and reinforcement learning algorithms can help identify the right patient segments and identify the right HCPs to target, deriving better business value for the brand with less resources.
- **Omnichannel communication.** Pharma companies can leverage nontraditional datasets like social determinants of health and consumer behavioral data to determine how and when to communicate with patients, providers, and other stakeholders with personalized messages to drive successful marketing strategies.[9]

AI/ML is finally living up to its promise and changing the game in drug discovery and development. With unparalleled precision, power, and the ability to find patterns in vast amounts of data, advanced AI/ML techniques are leading to faster drug development timelines and a lower failure risk. Industry experts project that the pharmaceutical industry could boost earnings by more than 45% by making strong investments in AI/ML.[10] As with any advanced technology, adopting AI/ML comes with barriers that companies must overcome to reap benefits from their investments; however, potential benefits in treating patients—particularly in areas with little to no treatment options currently available—are strong incentives to keep moving forward.

Continue to follow our blog as we explore trends on this and other topics. If there is a specific subject you would like us to address, please reach out to Karla Feghali (kfeghali@deloitte.com) and Seshamalini Srinivasan (sesrinivasan@deloitte.com).

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Endnotes

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