Big Pharma: Kx Technology for Clinical Trials

By Kevin McGivern

The pharmaceutical industry is a data-intensive business that has been managing large amounts of diverse data for many years including: prescription and patient records, research and development statistics, commercial sales figures and data for drug formulation and manufacturing reporting. Big Data has impacted every sector of the global economy, and now at last, the era of Big Pharma is upon us - resulting in a dramatic increase in the volume, velocity and variety of data. This explosion of raw data is continuously encouraging the search for powerful and more efficient ways of organizing vast data sets. The industry has tremendous potential to be reinvented if it can efficiently unleash the power of Big Data.

At Kx, we have leading edge technology designed for ingesting, processing, validating, estimating and analyzing real-time, streaming and historical data. These capabilities can transform organizations across a number of different areas, whether it is using the latest simulation techniques for clinical trials, Big Data algorithms on prescription data or enabling predictive maintenance on the production floor, Kx can help deliver the insight that Big Pharma promises.

Recently, the United States Food and Drug Administration's decision to approve Sarepta Therapeutics' Duchenne muscular dystrophy drug has evoked highly polarized responses. Some laud its swiftness in providing access to timely treatment for a life-threatening disease. Others lambast the lack of evidence attesting to its efficacy, citing a 12-person trail as an inadequate population for drawing any conclusion. In the middle, are the emotions of sufferers, shareholder interests (Sarpeta's share price rose sharply on news of the approval) and the disdain of statisticians who bemoan perceived misinterpretations of the p-values, confidence intervals and false detection rates.

What it points to though is the ever increasing importance of simulation in determining the appropriateness and structure of clinical trials. Today it is driven mainly by two factors: cost and appropriateness. Both are facilitated by increasingly powerful simulation technologies, like Kx, to assess factors such as dosages, age, drug-drug interaction and adherence rates that determine the likelihood of success. As we approach a world of personalized medicine based on individual genome sequencing and bespoke formulation will we not reach a point where, by definition, the sample size is one - the target patient? At that point what is there other than simulations to assess the dosage, efficacy and formulation of a treatment?

In anticipation, more time and money should be spent on modeling and simulation technologies as has been done in so many other industries, from airline to auto testing, where in-human studies are simply not an option. And let's not forget that the Monte Carlo simulation itself, the foundation today of so many contemporary financial analytic techniques, was developed during the Manhattan project, where the risk of physical experiment was simply too high to proceed.

As an aside, an interesting question is what else will machine learning algorithms be able to determine from all the resulting simulation data. After all, the industry has a history of serendipitous discoveries: from penicillin as a chance finding in an overlooked Petri dish to Viagra as an unexpected side effect of a drug originally intended for chest pains. With all that data who knows what else might pop up?

In the meantime, David Grainger recently wrote an interesting article in Forbes on how clinical trial outcomes could be improved today simply by understanding the underlying statistics better, read it here.