Using Real-World Data to Drive Market Access and Stakeholder Engagement
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Introduction

The reimbursement landscape is changing and national policymakers and payers increasingly demand solid, value-based evidence. Alongside clinical trial data, real-world data (RWD) play a central role in generating evidence of a drug’s safety and health economic profile that are key to market access decisions.

The reality for pharmaceutical companies is that the evidence requirements for regulatory approval and those for market access are increasingly disconnected. As Nigel Hughes, Director for Integrative Healthcare Informatics at Janssen R&D (Johnson & Johnson), explains in a recent FirstWord report, RWD may provide new insights in addition to the data from placebo-controlled clinical trials, that can support the evidence story for market access.

“One key challenge for the industry over the last few years has been a change from viewing efficacy and safety, for regulatory purposes, as the final hurdle before approving and utilising the drug. Increasingly, RWD are considered to take an equally, if not more, important role, especially post-approval,” adds Hughes.

Figure 1: Collecting, processing and analysing RWD

**Key:** RWD = real world data; EMR = electronic medical records; PRO = patient reported outcomes.


Real-world data in drug development

Analysis of RWD can help identify potential new drug candidates and promote their development into effective and, importantly, reimbursable medicines. Intelligent use of RWD can also aid progress in realising the potential of stratified medicine, making a significant improvement to the cost-effectiveness and accuracy of the R&D process.

Pharmaceutical companies can leverage RWD to assess and determine the most appropriate direction for their R&D strategies, to ensure they are developing the right medicines. Shailja Dixit, Executive Director, Medical Affairs, Allergan suggests, “By having good real-world data and doing good modelling, this wastage can be avoided because you may be able to see the prospective value of the product early on.” Adoption of an early RWD-based evidence generation strategy can inform every strategic drug development decision. Dixit explains, “Right now, the go/no-go decisions as the molecule goes through the lifecycle are probably based on a single dimensional data source, which is coming from the molecule itself. Real-world data provide you with that additional dimension for more informed decision making.”


As companies shift their resources towards the development of more personalised medicine, particularly for cancer and cardiovascular disease, traditional RWD resources are increasingly being enhanced by genetic and biomarker data to gain a better understanding of disease pathways and to establish associations with phenotypic expression. The convergence of these data with existing big data sources, such as electronic medical records, prescription, patient-reported outcomes databases, enables more sophisticated approaches to R&D that are more focused on individual genotypes and phenotypes and have the potential to drive significant change across the R&D pipeline.

The integration of genomic data with RWD can enable patient populations to be identified and stratified based on their risk of disease, as well as disease progression and severity. These data can also be used to inform clinical trial design, site selection and patient selection.

With regulators beginning to demand more evidence based on RWD, companies need to find ways to generate this evidence at an earlier stage of development than has traditionally been the case. For example, patient-reported outcomes during clinical trials can generate data that is more attuned to the real world. Patient-reported outcomes can be derived from questionnaires and diaries, and include data such as ability to function, quality of life, and the impact of symptoms and adverse effects on daily life from the patient’s perspective. As Hughes remarks, “It's now clear that even the regulatory approach is potentially going to change in the future to respond to these kind of requirements. But clearly how we're doing it to date is now no longer as applicable to current needs.”

The adaptive pathway developed by the European Medicines Agency (EMA) demonstrates how RWD can feed back into the development process, refining the drug or the structure of clinical trials. The aim of the pathway is to use an iterative development approach to improve patient access to new drugs, primarily where there is high medical need and where traditional data collection routes are difficult. Drugs are approved for an initial restricted patient population, or receive conditional approval based on surrogate endpoints. Companies can also involve stakeholders, including patients and HTAs, in informal discussions. The real-world use provides additional data that is used to support the final approval, as well as building the value story for stakeholders.

**Gaining market access through strategic use of real-world data**

When making decisions about new drugs, payers increasingly want to know what they are paying for, how the drug’s cost effectiveness will shape up compared with other drugs on the market, and whether it will improve outcomes for patients. Comparative effectiveness research is not, however, a regulatory requirement for new drugs and the cost of adding this type of research on top of RCTs is likely to be unsustainable.

The increasing availability of RWD can potentially fill the gap by providing insights into treatments, outcomes and costs in clinical practice, thereby improving the results of health economic analyses and reducing the reliance on modelling assumptions. However, the expansion of RWD presents challenges in determining the best datasets to inform health economic models. A recent study recommends that the choice of data should be informed by its potential to answer the research question, the therapeutic area, the size of the patient population and the ability to link databases.

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7 IMS Health (January 2016) Op cit.
Because RWD is based on more realistic populations than clinical trials, it provides feedback on how the drug is likely to change patient outcomes after launch, and how it is likely to perform in the longer term. This will include information on risks and benefits in people who have co-morbidities and are taking other prescription and OTC medications, and what the impact is likely to be on costs for the healthcare system.

RWD also provides a core resource for teams responsible for pricing strategies, and according to Florent Richy, Head of Global Epidemiology, Merck Serono, forms an intrinsic part of the reimbursement process. This is because pricing is moving away from fixed prices to value-based models, where payments are based on the value provided by the drug or treatment, and on the drug’s impact on the healthcare system as a whole in comparison with its competitors.

Post-approval, this shift to value-based care places an even greater emphasis on RWD and outcomes, according to Hughes: “Payers want to know if your outcomes are consistent over the next few years. If they are, fine. If they’re not, they might reduce the price, the reimbursement, or position in the formulary. Payers are requiring improved health technology assessments and other evidence bases before granting a price... the reality is that eventually the business model will change, where big pharma will be paid for the outcomes that it’s able to produce.”

RWD and patient/physician engagement
RWD has enormous potential for patient and physician engagement. The healthcare adoption of the Internet of Things (IoT) is still at a relatively early stage but already there are numerous apps and wearables that can both inform and assist patients and physicians, from simply reminding patients to take their medication on a regular basis to monitoring blood sugar levels, blood pressure or lung function. Digital technology has the power to assist patients to take a more active part in their healthcare, and to feed vital real-world information directly to their physicians and/or pharmacies.

A recent study suggests that the proliferation of wearables and connected devices will drive healthcare in the future, and that consumers are drawn to companies that offer the latest devices, such as smart-watches. These devices allow physicians to exchange patient data and messages in a secure and compliant manner. This technology is enabling physicians to capture more information about their patients, such as exercise levels, sleep patterns, weight and symptoms, which can help them to make informed decisions and encourage better outcomes.

The potential for wearables to revolutionise healthcare is highlighted by one observer, who suggests that in the future companion wearables or companion devices will be aligned to medicines in a similar way to companion diagnostics. For pharma companies, digital technology will be seen as a differentiator between products and the services and data wrapped around them, providing value to both patients and physicians, and resulting in increased patient compliance and better outcomes.

The value of RWD from digital technology extends far beyond individual patient-physician engagement, as the collection of aggregated data increasingly feeds into datasets that can be accessed and analysed by healthcare providers, payers and pharmaceutical companies alike.

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9 FirstWord (January 2016) Op cit


Challenges to building real-world data

Apps, devices and sensors generate vast amounts of raw data that have the potential to provide valuable insights, particularly if these data are cross-referenced with other RWD datasets. The challenge, however, is collating datasets in a format that enables them to be cross referenced and interrogated. Without advanced analytics capabilities, the conversion of raw data into actionable insights that can drive better business decisions and support improved outcomes for patients simply cannot happen.

Dixit would like to see the data and analytics companies coming together and making RWD more readily available. “This might be done with some kind of technology, possibly cloud based, and having some easy platforms so that at least some level of data is readily available under a licence or a system that is inexpensive for more companies.”

Robin Munro, Director, Real-world Evidence and Outcomes, Zephyr Health agrees that, “Without good analysis and interpretation, data are just facts and figures without useful meaning or insight.” He suggests that companies are looking to leverage advanced predictive analytics and machine learning, to have a concise and predictive way of extracting the data, turning it into evidence and then having actual results from that.

Predictive analytics is increasingly being used by physicians and insurance companies to interrogate massive amounts of data in order to predict outcomes for individual patients. Predictive analysis can also reveal associations in data that humans may not suspect, such as responses to particular medicines, predicting infections from a specific medical procedure or determining the likelihood of future wellness or of developing disease. Prediction modelling, based on a prediction profile or algorithm from past individuals, can be deployed to obtain a prediction for a new individual.12

The future of RWD

As technology improves and the breadth and depth of RWD increases in the coming years, pharmaceutical companies will be able to use predictive analytics to target medicines to patients who are more likely to benefit from them. Insights from RWD will be leveraged as companies continue to shift away from bulk medicines towards those that help smaller, more targeted patient populations.

There will be a greater use of RWD in drug development, both to inform clinical trial design and to identify and recruit patients. It has been suggested that R&D can only be improved by integrating insights from RWD, and that the traditional animal-based model could be replaced by a new R&D paradigm that would include a combination of data from mobile health (mHealth) technology wearables and biomarkers. Data from these sources would help to more precisely show the value of medicines.13

According to Dixit, in the next 10 to 15 years, pharma will use RWD more and earlier for evidence creation and to aid better decision making. She adds that in the future she would like to see RWD used more extensively in clinical trial design, making it more precise. “I hope that more registries – which I feel have limited value – will be replaced by more prospective data collection or observational data collection using RWD. We should see more RWD being used in phase IV observational studies, replacing registries.”14

As access to real world data grows, and as capabilities in predictive analytics and machine learning advance, there will be more opportunities for companies to gain valuable insights, and these insights will be a source of fuel for pharma’s drive towards new outcomes-based models. The integration of digital technology with medicines will both enhance the services provided to patients and provide growing and more accurate RWD resources, which in turn will provide further opportunities to improve stakeholder engagement in the future.