What is Real World Evidence (RWE) and what does it bring to the pharmaceutical industry?

In Part I of the RWE series we introduce the ideas and concepts behind RWE, providing an overview of its role and how it fits into the industry. We explain why pharmaceutical companies are increasingly adopting RWE programmes.

This is the first part of a regular series available at svmpharma.com

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The Changing NHS

The NHS is going through a period of transition that looks set to continue for several years to come. In addition to changes in infrastructure and organisation, there has been a strategic shift with the NHS demanding more value from the resources it has available and the companies it works with. Controlling expenditure on drugs and managing the size of the UK Medicines Bill is a key area of action for the NHS, and this is apparent in a number of policies which have either been recently implemented by the NHS or are in an advanced stage of planning (1-4).

As a result of these changes in the NHS, both new and existing drugs are having their value called into question, placing certain pharmaceutical companies in a challenging position to remain competitive, and opening up new opportunities for others. Pre-launch randomised controlled trials (RCTs) are sufficient for establishing a drug’s efficacy, demonstrating safety and gaining a licence. However, it is becoming increasingly clear that drugs need to supplement this with Real World Evidence (RWE) in order to gain traction in the market and to then either grow or defend its share (5-7).

Defining Real World Evidence

RWE can be broadly defined as ‘data collected outside of conventional clinical trials in order to evaluate what is happening in clinical practice’. RWE and related methods seek to both enhance and complement existing methods of data collection (8).

This data is derived from sources including patient registries, routine administrative data, observational research datasets, electronic health records and population health surveys. It is with the technological evolution and broadening scope of these data sources in recent years that RWE is ready to fulfil its potential (9).
Data on a vast scale

RWE enables analyses of study populations at a scale far beyond what would be feasible in a clinical trial. Using powerful data tools, the filtering and analyses of millions of records can be undertaken efficiently on datasets such as Hospital Episode Statistics (HES) and GP Practice Prescribing Data. These large datasets are useful for identifying unmet treatment needs, targeting patient segments and highlighting prescribing patterns; these are insights which help both the NHS & the pharmaceutical industry (10-13).

Following the pseudonymous patient journey

Different records from a dataset such as HES or Clinical Practice Research Datalink (CPRD) can be linked via a unique identifier whilst maintaining the patient’s confidentiality. This allows the patient journey to be followed across hospitals visits, between primary and secondary care, and along treatment pathways. This can uncover valuable information such as motivators for seeking care, triggers for switching medicines and long term compliance issues. This can be used alongside qualitative datasets such as Patient Reported Outcome Measures (PROMS) (14).

Bespoke data collection methods answering the complex questions

Intelligent and customised RWE data collection programs can be designed alongside patient groups and healthcare professionals to focus on selected issues and fill gaps in knowledge. An online tool allows flexibility, validation and guidance in data input with real-time analysis. Complex patient histories can be streamlined and reduced to the pertinent information required, and real world scenarios can be analysed.

Judging true performance

It is difficult to predict how a drug will perform in the real world clinical setting. A variety of overriding factors come into play such as poor adherence, potential drug interactions due to polypharmacy, the impact of co-morbidities and how far the disease has progressed, all of which can diminish the impact of a drug or distort its effects. The collection and analysis of RWE enables stakeholders to make informed decisions throughout the product lifecycle (9, 15, 16).
A SELECTION OF RECENT UK RWE TRIALS

<table>
<thead>
<tr>
<th>Year</th>
<th>Type</th>
<th>Location</th>
<th>Size</th>
</tr>
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<tbody>
<tr>
<td>2013</td>
<td>Audit</td>
<td>Ten specialist centres across the UK</td>
<td>136</td>
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<tr>
<td>2014</td>
<td>Observational</td>
<td>Great Ormond Street Hospital, London</td>
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<tr>
<td>2014</td>
<td>Pragmatic RCT</td>
<td>Salford, Greater Manchester</td>
<td>7000+</td>
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Effectiveness of omalizumab in severe allergic asthma: a retrospective UK real-world study (17)

Efficacy and safety of canakinumab therapy in paediatric patients with cryopyrin-associated periodic syndrome: a single-centre, real-world experience (18)

Obtaining real-world evidence: the salford lung study (19)

RWE Success & Recognition

In recent years there has been a notable increase in the publication of RWE in leading journals worldwide; this shows an important change in the acceptance and recognition of real world data. In traditional RCTs, patient selection, randomisation to groups and other controlled conditions maximise internal validity. These RCTs are essential in evaluating the clinical effectiveness and safety of drugs but there are doubts on how it can be applied to the real world clinical setting (10, 16, 20-23). A move towards RWE shifts the focus towards increased generalisability of the findings and encompasses a wide range of study designs including pragmatic RCTs, modelling analyses and observational studies (17-19, 24, 25).

RWE consists of a broad and interrelated series of concepts; this introductory article has focused on insights from big data, the ability to follow a patient journey, customised data collection methods and the ability to judge true performance outside of a controlled trial.

Pharmaceutical companies need to adapt to meet the changing requirements and demands of the NHS, and to effectively show the value of their products. In this competitive environment, the collection, analysis and presentation of RWE can drive success and growth. RWE is growing in importance and is set to become an essential part of the marketing and medical strategy for a successful drug.

NEXT ON THE RWE SERIES JOIN US FOR PART II IN WHICH WE DETAIL THE DIFFERENT SOURCES OF RWE AND LOOK AT HOW THIS DATA CAN BE COLLECTED AND ANALYSED. VISIT SVMPHARMA.COM & FOLLOW US @ SVMPHARMA

2. Sculpher M. Value-based pricing to ensure cost effective drugs for the UK NHS: Will it work? Clinical Therapeutics.35(8):e129.


4. Wise J. NHS spending on drugs is frozen for two years under price deal 2013-11-07 15:12:47.


