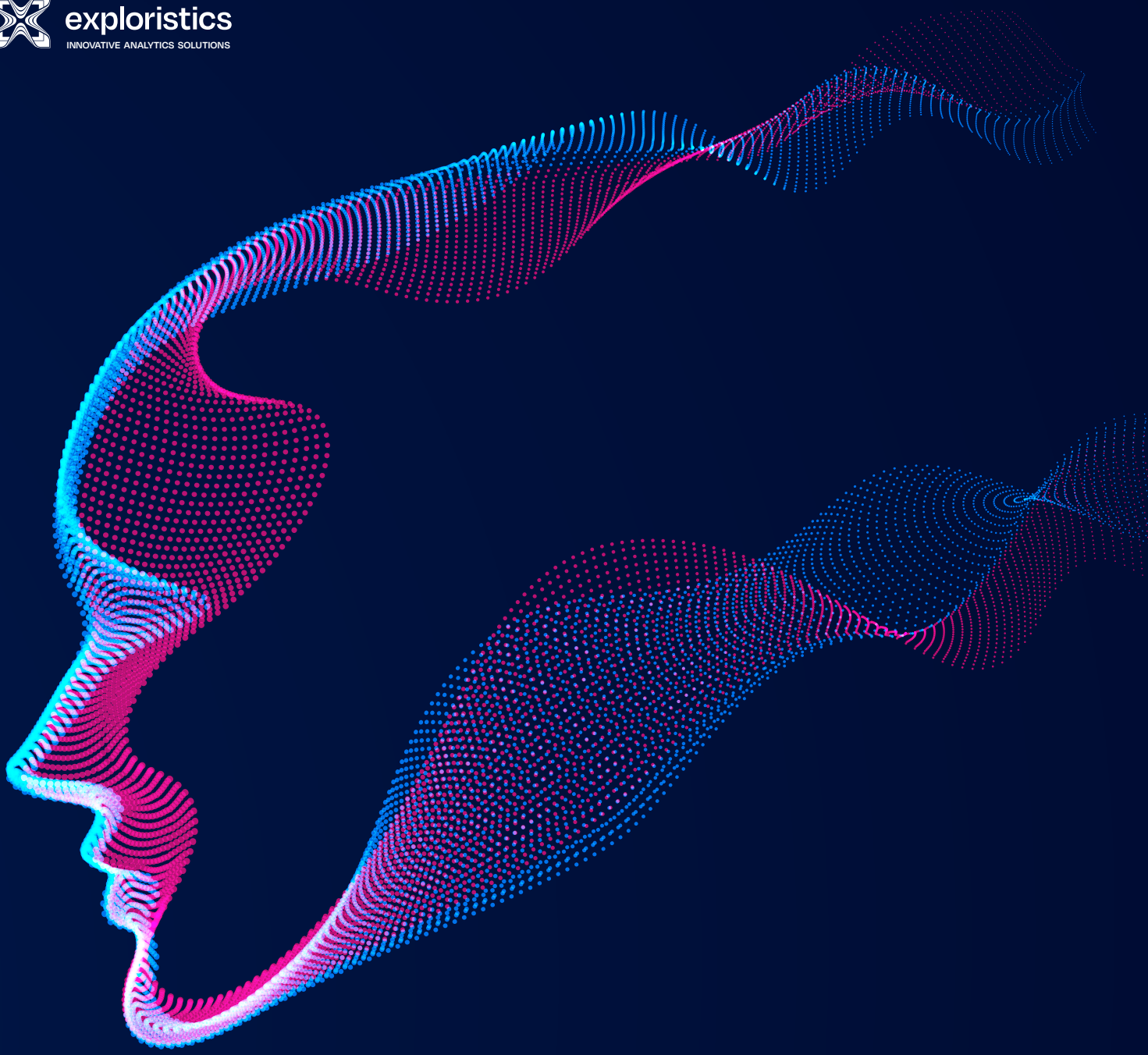
exploristics
INNOVATIVE ANALYTICS SOLUTIONS



Statistical Insights

The Liberation of Real-World Data

Meeting the challenges of harnessing clinical insights from the real world.

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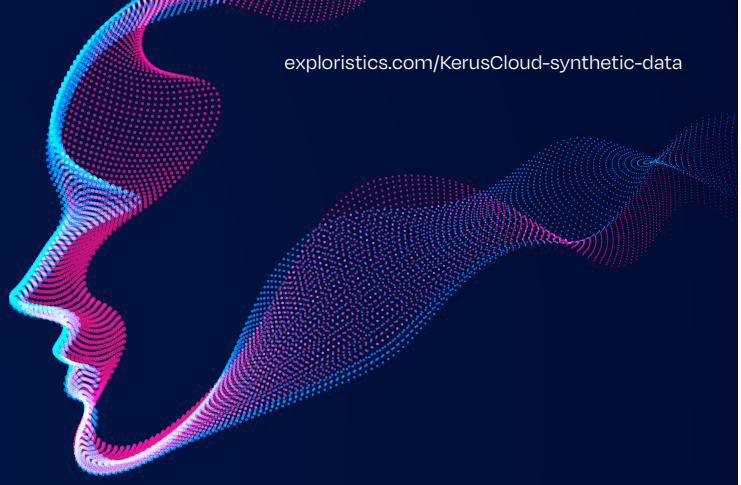


The costs and complexities associated with drug development are fuelling the drive for more efficient healthcare delivery. Using real-world data resources leads to better- informed projects, improved patient outcomes and a more effective evaluation of success.

This 'liberation' of real-world data is an overarching requirement that needs to become standard practice. Aiden Flynn, CEO of clinical trial data and design experts, Exploristics, explores how the collection and analysis of real-world patient data can be an invaluable asset in clinical development.

From clinic to the real world

Randomised clinical trials (RCTs) have long been the benchmark for evaluating a new treatment's safety and efficacy profile to meet requirements for regulatory approval. However, they offer limited insight into a drug's long-term therapeutic effectiveness and value post-authorisation. Since they are conducted under optimised conditions using small, well-defined patient populations and short-term end-points, RCTs do not reflect a treatment's impact in an everyday setting, in which treatments are given over long periods to heterogeneous patient populations in diverse healthcare environments. Consequently, there is a clear need to understand the effectiveness of new treatments in real-life settings where the value and outcomes can be measured over longer time scales.

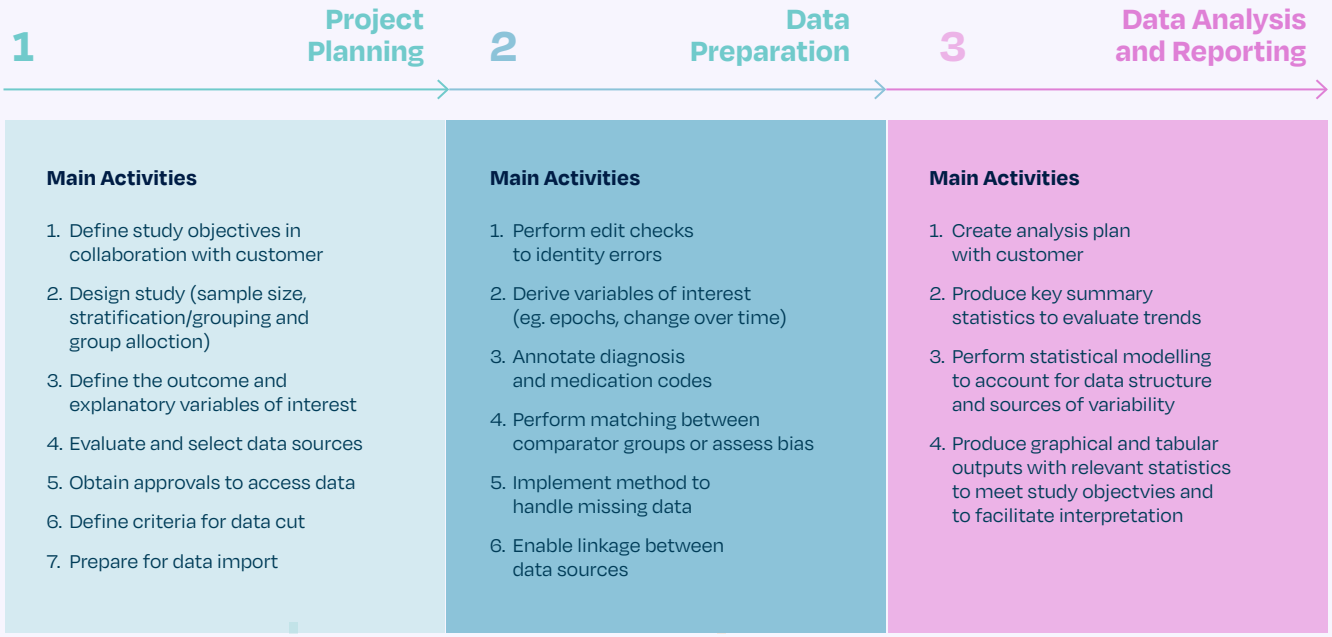
Currently, the long-term impact of a clinical treatment is primarily evaluated using Phase IV (post-authorisation) clinical studies which monitor effectiveness in the general population, whilst amassing information about any adverse events associated with widespread use. In this context, the use of real-world data sources is common.

However, real-world data can also provide invaluable insight during the research and development of a new medicine. The emerging multitude of publicly available real- world data sources including electronic health records, mobile health apps, claims data bases, health surveys and patient registries, offer a new and evolving prospect for bringing real world insight into this earlier development stage. At this point, such sources can help to evaluate the commercial opportunity and clinical need as well as inform the feasibility of a development plan, clinical trial design, positioning and market access. But with many developers constrained by costs, time and expertise, the power of real-world data is often not fully exploited as an R&D resource.

Seeing the bigger picture

Failure to unlock the potential of real-world data throughout the development and post-authorisation process means that developers are restricted in their view of a treatment's full potential impact. Real-world data analysis that includes a wider range of observational healthcare data sources collected in real-life scenarios offers a more comprehensive, three-dimensional picture of how a treatment is used and its benefits throughout its lifecycle in clinic. This builds on the narrow information derived from RCTs.
The growing number of potential data sources as well as the plethora of novel analytics tools to mine them now offer an unmissable opportunity to glean useful clinical information for healthcare decision-makers, including developers, regulators, ethics boards, reimbursement

The planning preparation and analysis process required for real world data analysis



bodies, healthcare providers, health insurance companies, as well as public policymakers. With such large numbers of stakeholders involved in treatment and purchasing decisions, there is a considerable need to exploit these wide-ranging public healthcare data sources effectively.

Suitably captured and analysed, real-world data offers more than just an increased insight into the effectiveness, cost and safety of new clinical interventions. Appropriately harnessed, it can be used to explore new clinical questions, identify new uses for medicines, fill in knowledge gaps on the use of medicines in real world settings, identify and monitor unforeseen risks and adverse events associated with an approved medication or evaluate unstudied factors influencing a patient's outcome to improve patient care. Yet despite such clinical promise, we still face considerable challenges in finding the best way to reap these benefits.

The challenges of analysing real-world data

While there is growing consensus on the need for incorporating healthcare analytics using real-world data into our understanding of treatment outcomes, its delivery is complex. The challenge of analysing real-world data lies in the disorganised nature of the data involved. Real-world evidence is derived from a variety of disparate sources which need to be suitably aligned to enable meaningful information to be extracted. Unlike RCTs, where the nature and method of data collection is outlined at the outset, real-world data is collected in a more ad hoc fashion often with great variability in how, when and what is recorded even within a single data source. This makes it difficult to extract reliable information.

The difficulties arising from such data disparity, as well as missing data elements currently severely limit the use of real-world evidence. When data sources do utilise the same methods of recording and collection, data can be pooled to create a larger data set enabling more diverse patient group analyses. This can increase the likelihood of identifying rare events or patient subgroups, leading to useful new insights such as characterising new subsets of response or improved treatment targeting. However, given the variability in collection and diversity of sources for real-world evidence this is not always possible. Consequently,

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the utility of real-world data relies on the quality of the data source and methodology used to analyse it. Whilst the increasing use of electronic data collection (EDC) will undoubtedly improve the quality of data, there remains the need for all stakeholders to understand what simple steps we can already take to facilitate the analysis real-world data and so drive better healthcare decision- making.

Ensuring aligned high-quality data from the real world

Although challenges remain in harnessing real-world evidence successfully, with the growing desire to mine this resource, common data standards and best practices are beginning to crystallise. These emerging developments are improving the accuracy of the insights, the comparisons and conclusions drawn from real world data. As with any well-designed experiment, real-world data studies benefit from prescribed clear objectives, study design, methodology and endpoints drawn up with a data analysis plan. Successful analysis of real-world data is also highly dependent on the appropriate preparation and cleaning of data. This involves rigorous evaluation of any potential bias, and the use of robust statistical modelling approaches to handle the structure and sources of variability in the data. In most cases, this is an iterative process involving close collaboration between the domain experts and the data analysts or statistician. At present, the analysis of real-world data is currently difficult to automate as without the necessary quality control measures there is a substantial risk of drawing false conclusions and misinterpreting the data.

Despite the difficulties, there are ways to deliver insightful healthcare analytics using real-world data if appropriate project planning, preparation, and analysis are conducted. Within each step of this process (as outlined below) there are core activities that require the oversight of experts which, at present, largely precludes automation. Nevertheless, the time invested in each of these stages will determine the success of a project using real-world data. Indeed, typically the planning and data preparation steps prior to analysis require a greater investment of resources to complete than the data analysis itself, and yet many projects do not pay due attention or even ignore these important steps to their cost.

The world is full of alternative sources of real-world data that give us a window into how medicines are used in real life. The liberation of these real-world data could transform the development of new medicines and the delivery of healthcare. Whilst it may take some time to fully exploit the potential of real-world data, there is little doubt that it will become an integral part of clinical development.

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