Institute of Biostatistics and Analyses

UNLOCKING THE POWER OF REAL-WORLD DATA IN CLINICAL RESEARCH: A COMPELLING VALUE PROPOSITION

healthy data

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PREFACE

At the Institute of Biostatistics and Analyses, we're on a mission to promote **HEALTHY DATA**. Our team started as a spin-off of Masaryk University, and our specialization is **managing** and **analyzing data** for **clinical research projects**. Not only do we possess advanced data analysis capabilities, but we also excel at project management, allowing us to provide comprehensive **value-added services.**

We can proudly say that we've successfully brought research and development out of the academic sphere and get it into the real world. One of our biggest accomplishments is the creation of our very own **electronic solution for data management**: **CLADE-IS**. This powerful tool includes a **module for risk-based monitoring** that uses machine learning algorithms to keep your data safe and sound.

Our company is built around four key departments: project management, data management, software development, and data analysis. Each one plays a vital role in helping us conduct clinical research projects in the real world.

Our mission is to deliver accurate and reliable data to the pharmaceutical and biotech sectors, supporting their efforts in developing life-saving solutions that meet the highest standards of safety and efficacy.

UNLOCK THE SECRETS OF REAL-WORLD DATA

Time is precious, and we know that better than anyone. That's why we want to get straight to the point and explain why you need to read this white paper.

This article is essential reading for anybody with a stake in clinical research in the healthcare industry, pharmaceutical industry, medical device manufacturing, clinical data management, or project management in clinical studies. You will gain an in-depth comprehension of the present landscape of **real-world data in healthcare** and how it may **transform clinical research** and **patient care**. However, that is not all.

1

In this document, you will learn about the equipment and individuals needed to carry out actual real-world evidence projects.

2

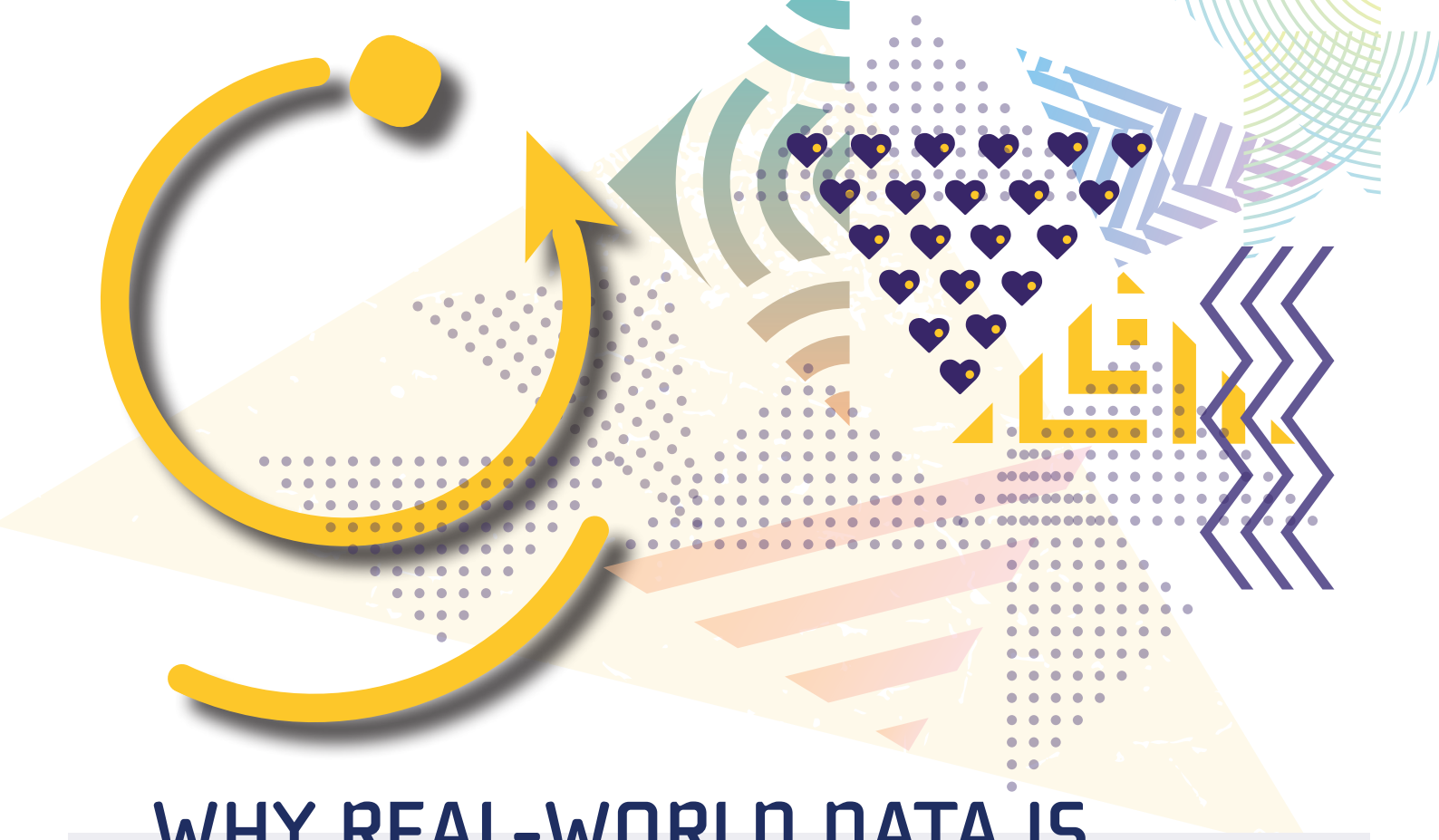
You'll get all the information you need to maximize the use of your clinical research, including a focus on the significance of data quality and management.

3

In a nutshell, the information in this white paper could revolutionize your approach to clinical research.

4

Don't stop reading this helpful content created by IBA experts and miss out on all the benefits it has to offer!



WHY REAL-WORLD DATA IS A GAME-CHANGER: EVERYTHING YOU NEED TO KNOW

Let's get concrete about real-world data. If you're involved in drug or medical device development, you know that data is everything. But do you know the difference between real-world data and experimental trial data? It's important to understand both, from the early phases of clinical trials with strong inclusion and exclusion criteria, to real-world data collected in everyday life.

Now, you might be thinking that real-world data is only used for pharma market access, but that's not the whole story. Initiatives like EHDEN (European Health Data Evidence Network) and OMOP (Observational Medical Outcomes Partnership) are gathering data from patient registries and non-interventional studies to unlock the potential of this type of data. When used properly, real--world data has the power to dramatically improve drug development and clarify the value of new therapies.

So, whether you're working in preclinical or clinical phases, post-market observations and monitoring using real-world data forms an integral part of the drug development cycle. Don't miss out on the opportunity to learn more about the value of real-world data and how it can benefit your work in the healthcare industry.

UNLOCKING THE POWER OF REAL-WORLD MEDICAL DATA: A COMPREHENSIVE GUIDE

Patient Registries

Introducing the game-changing solution for medical professionals and pharmaceutical companies: Patient Registries. Patient Registries are the ultimate tool for collecting and analyzing uniform data on patients defined by disease, condition, or exposure. This invaluable data includes demographic information, medical history, diagnostic results, treatment information, and outcomes.

Why is this kind of data so important? It allows medical professionals to better understand the safety and effectiveness of drugs and treatments, ultimately improving patient outcomes. It's also a critical factor in product development and pharmaco-economics.

But that's not all. Patient Registries can also help track the natural history of a disease or condition, identify trends over time, and even identify patients who may be eligible for clinical trials. They can also identify potential risk factors for diseases or conditions, improve communication and coordination of care among healthcare providers, and identify disparities in healthcare access and outcomes for certain patient populations.

Biobanks

Unlike any other registry, biobank offers a complete package - from storing patient data, such as demographic and medical history, and family history from a group of individuals to preservation of biological samples for comprehensive research.

To achieve optimal results, biobank requires a robust EDC (electronic data capture) system that seamlessly integrates with laboratory warehouse management systems. With this technology, we can unlock the full potential of biobank's data and gain invaluable insights in medicine, medical devices, and in vitro diagnostics.

Biobank data is a goldmine for the medical industry, offering critical information for disease prevention, product development, and advanced medical research. This cutting-edge tool provides medical professionals with a powerful resource to unlock new insights and drive innovation.

Product Registries for Medical Devices and In Vitro Diagnostics

Product registries are similar in terms of methods and management to patient registries and biobanks. They are usually used by medical device and in vitro diagnostics manufacturers, to better understand the behavior of their products in real life conditions.

These collected data are important not only for tracking safety and effectiveness, identifying potential issues or complications associated with a device, but also for evaluating the performance of a device compared to other similar devices, identifying trends in device use and adoption, providing information to healthcare providers and manufacturers improving the design.

In addition, collecting post-CE clinical data for medical devices is required by the new EU legislation bill, the so-called Medical Device Regulation, as part of CE mark approval and maintenance, mainly for classes IIa, IIb and III.

Non-Interventional Trials

Non-interventional studies (NIS) represent a critical component of the clinical development program for novel pharmaceuticals. This category encompasses a range of study types, including post-marketing surveillance, post-authorization safety, register, case-control, and cohort studies. Cohort studies are particularly valuable as they allow researchers to follow groups of individuals over time and gather data on human health.

Additionally, it is important to note that medical devices also undergo early-phase development and post-marketing clinical follow-up, which are primarily focused on safety observation.

By incorporating non-interventional studies into clinical development programs, pharmaceutical and medical device companies can more effectively gather real-world data and enhance their understanding of product safety and efficacy.

NIS provide valuable real-world data that are often not captured in clinical trials, as these studies tend to exclude certain populations. NIS are particularly well-suited for investigating key outcomes such as prognosis, life expectancy, quality of life, benefits, and adverse events under real-world conditions.

Prospective non-interventional studies represent a type of observational study in which a group of individuals is followed over time, without manipulation of the exposure or intervention being studied. Unlike randomized controlled trials, participants are not randomly assigned to different groups, and exposure or intervention is determined by usual care or the participant‘s choice.

In contrast, retrospective non-interventional studies collect data retrospectively, looking back in time to study the relationship between an exposure and an outcome. Retrospective observational studies, often known as case-control studies, are another name for these kinds of research.

Researchers and pharmaceutical businesses can learn more from NIS than from conventional clinical trials alone, leading to more effective drug development, better patient outcomes, and improved public health. Medical decision-making relies heavily on solid evidence bases, and the knowledge gathered from such investigations can help strengthen and expand such bases.

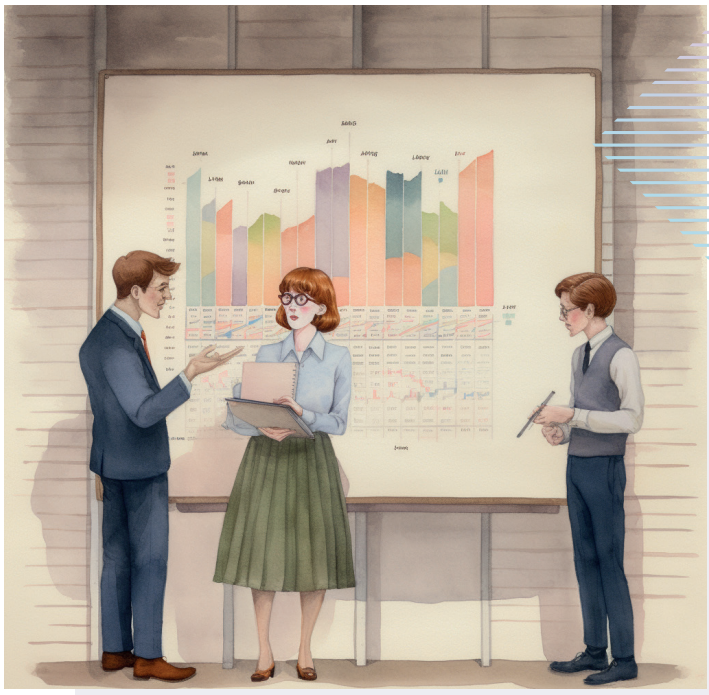
Cohort studies and other forms of observational research are crucial for deducing the effects of exposures on health outcomes. Exposures, such as treatments or environmental variables, and outcomes, such as illness development, can be potentially linked when a group of people are followed over time.

However, it must be emphasized that observational studies cannot prove a cause-and-effect relationship.

In recent years, the emerging field of Exposomics has gained attention for its focus on understanding how environmental exposures can impact human health. Exposomics seeks to investigate the cumulative effects of environmental factors, including pollutants and chemicals, on health outcomes, while also considering genetic and epigenetic factors.

Post authorization safety studies are a vital subgroup of post-marketing studies, focusing on identifying potential adverse events associated with a particular treatment or intervention. These studies aim to identify the frequency of known adverse events, as well as potential rare adverse events that may not have been previously identified. Additionally, safety studies analyze probable risks in special patient populations.

If you are interested in conducting observational studies or safety studies, our team of experts can provide you with the necessary tools and expertise to ensure your study is a success.



THE DRIVING FORCE BEHIND REAL-WORLD EVIDENCE: TOOLS AND EXPERTS YOU NEED TO KNOW

Essential Tools You Need to Succeed

What are the tools for achieving or ensuring good quality of data in real-world settings?

We have selected four very basic but necessary tools: Electronic Data Capture System (EDC system), Clinical Trial Management System (CTMS), Electronic Case Report Forms (eCRFs), and Electronic Trials Master File (eTMF).

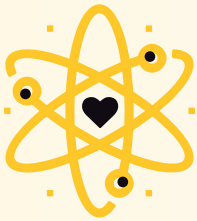
The interactions between these software can be described as follows: Subject's visit triggers data entry into an eCRF within the EDC system. If this is, for example, the first patient visit, then it triggers an important milestone within the CTMS system. All the milestones are connected to documents, which together form eTMF of a study. While CTMS and eTMF are mainly used by project managers, the EDC/eCRF is where all the data usually originates, and this belongs to the Clinical Data Managers.

As a company that primarily focuses on data quality, we have built our EDC system that we provide as a service. If you would like to know more about what makes a great EDC system, contact us, and we will send a checklist that will help you.

The People Factor: Key to Successful Real-World Evidence Projects

The successful execution of clinical research in real-world settings heavily relies on the contribution of personnel in the data collection, management, and analysis processes.

Project Management:



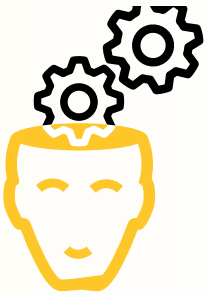
Clinical trial project managers are key players in ensuring the success of clinical trials. They are responsible for guiding the study from its initiation, including the development of the clinical research plan and budget proposal, to its execution. Effective study execution in the early stages requires project managers to contribute to the creation of informed consent, site selection, submission to state authorities, and ethical committees, as well as ensuring protocol compliance.

As the study progresses, project managers primarily focus on managing sites and patient enrollment to ensure timely and efficient completion of the trial. With their extensive expertise in clinical trial management, project managers are essential for sponsors to achieve their research objectives and ensure the highest quality standards of research conduct.

Data Management:

In contemporary clinical research, Data Managers have emerged as indispensable agents for ensuring optimal data quality. These professionals are charged with designing and developing user-friendly electronic Case Report Forms (eCRFs) that minimize the time that investigators spend entering patient data. Additionally, Data Managers conduct extensive data quality checks, such as data verifications and validations, to ensure the integrity and accuracy of the collected data. They are also responsible for overseeing all data management documentation, providing investigator training, and offering help desk support. Through their contributions, Data Managers ensure that clinical trials are conducted with the highest standards of data quality and efficiency.

Data Analysts:



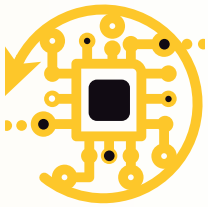
Choosing the right methodology and study design to achieve study objectives is just the tip of the iceberg of what data analysts do. They also review the protocol, ensuring that all necessary variables are included to answer primary and secondary objectives. Data analysts work closely with data managers on randomization if required.

Data analysts can also improve the smoothness of the monitoring process by using statistical methods and data reporting. On-site monitoring can be partially and effectively replaced by statistical monitoring. The combination of on-site and regular statistical monitoring yields the highest possible data quality.

Their main scope of work, however, lies in statistical programming of analyses and output creation, including contributions to publications.

Over time, we have learned that there is still a lot of confusion among stakeholders in the healthcare industry. Therefore, we have been running our statistical courses focused on clinical research for several years.

IT Team:



In the realm of clinical research, one might assume that the IT team does not play a crucial role. Contrary to this belief, however, the IT team is a crucial component in the success of any clinical research endeavor.

At IBA, we are fortunate to have a dedicated team solely focused on developing our proprietary EDC software, CLADE-IS, which is now available as a service. With our team's assistance, we are capable to design and implement study-specific features, including the integration of various hardware and mobile applications.

Moreover, we can create and design websites for registries that include POWER-BI charts to display their research findings.

The Unifying Force Across Departments: Data Science.

Data Science is the fundamental component that unifies all departments at IBA, and this is what distinguishes our organization from others. It may seem unclear how data science is related to clinical research.

However, data science is essential to clinical research because it provides the methods for dissecting the massive, complex datasets generated by clinical trials. The pharmaceutical industry, the medical device industry, and the in vitro diagnostics industry can all benefit from data science in clinical research.

1

Pattern recognition: recognition of patterns in clinical data, such as disease risk factors or patient subgroups with different therapy responses.

2

Data science as a predictive modeling: identification of high-risk individuals for a disease or the forecasting of the probability of a positive treatment outcome.

3

Evidence on the safety and effectiveness of therapies: evidence on the safety and effectiveness can be gained from real-world scenarios through the application of data science and the examination of real-world data obtained from electronic health records, claims data, and other sources.

4

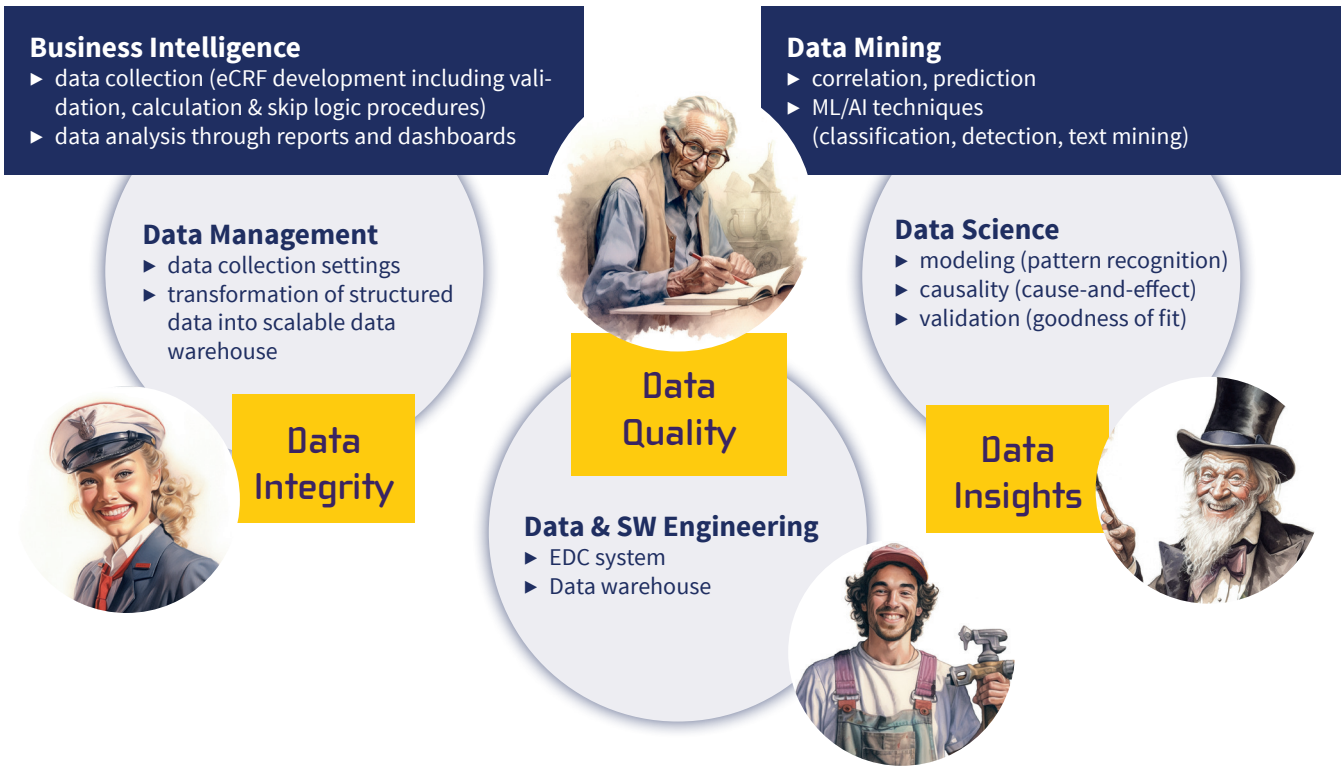
Clinical trial design: data science can be used in conjunction with other techniques to optimize clinical trial design, including the selection of the appropriate patient group and endpoint.

5

Visualization: data science methods allow for the visualization of clinical data, which helps researchers and clinicians better understand and communicate the results of clinical trials.

In conclusion, data science is an indispensable part of medical investigation. Insights gained from data science‘s examination of vast and complicated datasets can be used to improve clinical trial design, execution, and analysis. Therefore, it is crucial for firms to invest in data science skills and infrastructure in order to carry out high-quality and effective research.

PEOPLE behind data-driven clinical research



Let's examine the people-oriented aspect of NIS, which necessitates rigorous data collection, management, and analysis. Data is obtained from a variety of sources and in various formats, including EHR systems, EDC systems, patient diaries, wearable electronics, and others.

Upon considering this situation, you may wonder what a plumber, flight attendant, writer, and magician have in common. They appear to have more differences than similarities. However, these professions can represent different data-driven positions within a CRO conducting NIS or any data-driven late-phase clinical research.

While there may be many overlaps among a data manager, data engineer, data analyst, and data scientist, their roles can differ significantly. Can we define these differences? And how can they contribute to the success of a NIS?

The **data manager** serves as a flight attendant, providing comfort during long and short data journeys.	The **data engineer** operates like a plumber, ensuring that data pipes are secure and free of leaks.	The **data analyst** is like a writer, creating content that makes scientists famous.	And the **data scientist** is like a magician, conjuring unexpected insights from data.

For example, at IBA, data managers place a high value on data integrity, while data scientists prioritize data insights. Our software developers and data engineers aim to keep data organized and tidy, and all members of our team share a common value of data quality.

THE ADVANTAGES OF COLLECTING REAL-WORLD DATA: A COMPELLING CASE

There are many advantages of obtaining clinical data from the actual world, we decided to name few:

1

Better patient outcomes: by analyzing real-world data you can learn how effective treatments and interventions really are at improving health outcomes.

2

Patient access: real-world data can be used to pinpoint specific regions in which treatment is lacking.

3

Drug development: real-world data can support drug research by providing information on how well and how safely medications and therapies are used in clinical settings.

4

Clinical trial design: real-world data can help improve clinical trial design by giving essential information on patient groups, treatment patterns, and outcomes.

5

Conformity: real-world data collection is often required by regulatory bodies as part of post-marketing surveillance to guarantee that the product‘s safety and efficacy are closely monitored after approval.

6

Cost-effective: because it may be done during regular clinical practice, collecting real-world data can be less expensive than performing randomized controlled trials.

Now you may wonder how real-world investigations stack up against clinical trials. Each type of research has its own advantages and disadvantages and is essential at different stages of the product life cycle. High-quality information on the efficacy and safety of interventions can be found in clinical trials, whereas real-world research sheds light on the efficacy and safety of interventions in their natural habitats. Both sorts of research are essential for enhancing our knowledge of medical practices and the lives of patients. Below is a table comparing the benefits and drawbacks of RWE research and clinical trials.

Aspect	NIS & RWD/RWE projects	Clinical trials (RCTs)
COSTS	low costs	very high costs
SUCCESS	high success rate	low certainty
ABILITY TO ANSWER RESEARCH QUESTION(S)	can answer	answers only the product-related questions, fails in answering more general research questions
ROLE IN DRUG DEVELOPMENT	significant	significant
REGULATORY ISSUES	easy procedures	complex and tedious procedures
RECRUITMENT OF SUBJECTS	easy and fast	difficult, long-term, multiple challenges
EVIDENCE OF CLINICAL VALUE	very helpful	limited

In summary, real-world data collected in a clinical setting can provide considerable benefits, such as enhancing patient outcomes, assisting in drug development, and guiding the planning of clinical trials.

Get in touch with us if you have any further questions or concerns about the procedure.

We will be by your side every step of the way to help and advise you. In order to provide you with the skills and information you'll need to gather data in the real world, we provide training and educational resources in addition to our expert advice. Get in touch with us right now to find out more about how we can assist you with your clinical research endeavors.

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CONCLUSION

Because of its potential to improve our understanding of patient outcomes, treatment efficacy, and drug development, real-world data has gained prominence in clinical research. The development of data science and related technologies has made it easier and cheaper to gather and analyze data from the real world.

Organizations can enhance patient care and clinical trial design by using real-world data to make educated decisions.

It is becoming increasingly important for businesses to collaborate with real-world data professionals who can help them successfully implement their strategies. When it comes to helping our customers succeed in their real-world data projects, nobody does it better than IBA.

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