It’s 2022. What does this mean for the life sciences industry?

How real-world data, technology and innovative approaches will contribute

IBM **Watson Health**®

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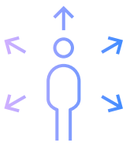

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Explore why accessible, actionable, integrated data is one of the biggest life sciences trends to watch in 2022.

Introduction

The life sciences industry has reached an inflection point. Transformational shifts, including advances in precision medicine, new cell and gene therapies, and innovation in rare disease research, are driving the need for solutions that support faster, more efficient discovery as well as streamlined clinical development and commercialization.

In addition, the need for agility and speed have never been more critical to inform business strategies during the current pandemic and enable companies to reduce risk associated with future global events. And market forces continue to require rigorous cost controls and demand new levels of flexibility in the core of life sciences businesses.

Biologics, complex research areas such as oncology, and the growing demand for real-world evidence are resulting in increasingly complex trial designs, complex patient stratification needs, more genomics data, and an overall increase in data volume and complexity of data sources (including real-world and e-source). In turn, these design complexities contribute to execution challenges, that lead to a higher risk for trial failure and an overall lower rate of return. For example, oncology only has a 38% product-specific probability of technical and regulatory success[20]. Addressing these challenges requires a more collaborative focus to cultivate the expertise and solutions required to translate, aggregate, and curate data in order to maximize usability and minimize human effort.

How can your life sciences organization keep up with these shifts and trends? The first step is to learn what to look for. From innovations in real-world data (RWD) to decentralized clinical trials (DCTs), here are the major trends our research and data experts at IBM® Watson Health® will be watching closely in 2022.

Developments in patient data use

Since the sequencing of the human genome in 2003, personalized precision medicine has advanced in many exciting ways due to the abundance of individualized medical data available.

Patients and researchers have access to more RWD on factors that affect health than ever before[1], drawing from the proliferation of genetic testing, as well as data from smart watches, cell phones, and connected in-home medical devices. Turning that data into actionable real-world evidence (RWE) will be critical for advancing precision medicine. Here are some developments to look for as the year unfolds.

Pursuing intelligent patient health records

The depth and breadth of healthcare data to which researchers, drug developers, clinicians, and medical device manufacturers have access is expanding[2]. From insurance claims to clinical labs, imaging studies to data from wearables, there is more data collected as people live their lives than can be reasonably utilized in clinical development. All of this new information will likely lead to the creation of a new kind of patient health record called the intelligent patient health record. Ideally, intelligent patient health records will offer a 360° view of key health indicators for a patient that includes their current and prior therapeutic interventions, therapeutic efficacy, allergies, adverse events, and other data that can be used to inform their care. As technology progresses and more patient data becomes available in real time, creating intelligent health records could emerge as a key resource for moving precision medicine forward.

Integrating disparate data

Although intelligent health records could represent an important step in personalizing medicine, creating them, and maintaining them requires overcoming existing obstacles related to data access and integration. Bringing siloed data sources together is a complex task. However, it is so critical to driving down the overall cost of healthcare that government legislation, like the 21st Century Cures Act in the United States, is being used to move healthcare data interoperability forward. The interoperability trend will be the first critical step towards building intelligent health records and opportunities for researchers to access data that was previously hard to acquire and effectively use.

Encouraging data sharing

Promoting data interoperability isn't the only trend necessary to advance precision medicine. Data ownership and use rights often constrain access and create gaps that stall data integration. Use rights are typically negotiated on a case-by-case basis as data aggregators work to amass individual agreements from health systems and providers. Fortunately, there are solutions and services emerging that can accelerate data sharing by centralizing much of the data curation and rights management. It's likely these data collections will continue to grow and become a vital part of delivering on the promises of precision medicine.

A move toward decentralized clinical trials

The limitations of traditional clinical trials, amplified during the pandemic, have shown there is a need for study formats that reach people where they are, provide more equitable access, allow more flexible study designs, and accommodate more direct data sources.

Decentralized clinical trials (DCTs) have emerged as a way to address those limitations[3]. These trials, which augment or replace traditional in-clinic approaches with direct to patient and general practitioner interactions and data-driven alternatives, have seen increasing adoption due to the pandemic along with the increasing emphasis on in-home care.

In the current realization of DCTs, data collected direct from patients via devices, in-home and in-clinic visits, or extracted from the EHR/EMR, are fed into an electronic data capture (EDC) platform or clinical data management system (CDMS), translated into study reports, and prepared for regulatory review. The range of digital enablement tools that have emerged to facilitate these trials has created a continuum of DCT designs that range from site-less trials to direct-to-patient and hybrid models. The growth of DCTs, along with the increasing complexity of regular trials, has generated more individual data points than ever before[4]. Managing the data collected through DCTs, whether from multiple trial sites or through digital technologies, will ultimately require intuitive, fit-for-purpose technologies and expertise.

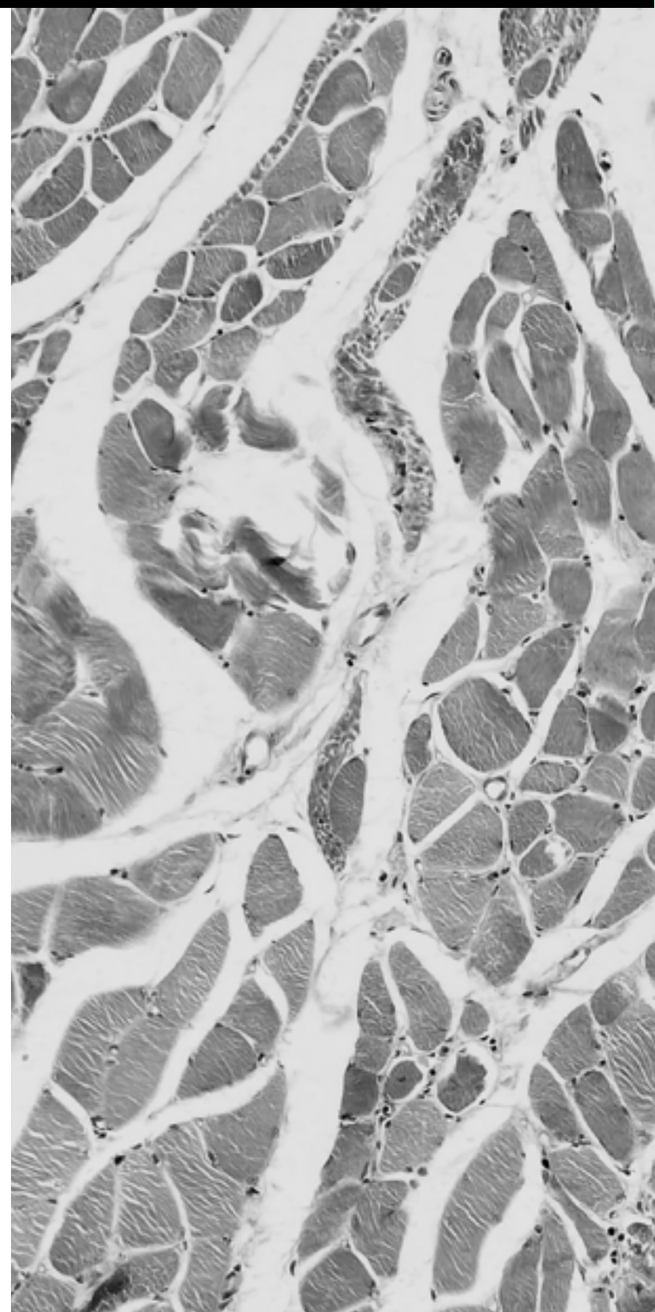
Combatting data noise in DCTs

In addition to lab results and vital statistics, the feedback clinical trial participants provide is essential to form a comprehensive picture of their health. Although digital tools like telehealth portals can offer sponsors access to patients and provide a wealth of data, this data is often unstandardized, and difficult to use. Making that data usable for reporting purposes will require technologies that can provide translation and formatting that is both consistent and accurate. Natural language processing (NLP) technologies are one potential way to standardize this unstructured data[5]. By translating information found in sources like clinical notes into a machine-readable format, NLP could help make unstructured data more usable for researchers. Purpose-built, fully-integrated clinical management systems can also help maintain high-quality, reliable data as part of a DCT.

Encouraging patient participation in DCTs

Although there are some early indications that DCTs have an easier time recruiting patients and a slightly higher retention rate, the complexities of individual trials will ultimately determine how people can participate[6]. As DCTs are more broadly adopted, the need for trial designs that center the patient experience will likely become a critical factor in their success and keeping their trial interactions simple and positive will be key to ensuring compliance as more hands-off paradigms become common. Protocol and trial designs that are increasingly focused on remote access, flexibility, and digital collaboration will also require sponsors, providers, and others to develop expertise in how DCTs can be structured and conducted to ensure success.

Making DCTs successful requires a more open, information-sharing ecosystem worldview involving public and private data resources, incorporating a sophisticated data governance framework, and embracing a willingness to integrate different solutions and complex data sources. The ability of DCTs to drive improved clinical development outcomes is dependent on a company's ability to modernize their operations with AI and related cognitive solutions at scale. Companies can benefit from open cross-industry collaboration, integrate with a broad ecosystem of partners, and efficiently use data and analytics to inform their research portfolio and business strategy. As seen in success stories from other industries like retail, where engagement is critical and competitors are continually evolving, investment in digital transformation can help improve life sciences resilience and deliver strategic returns.



RWD opportunities in clinical development and approvals

As sources of healthcare data expand and new analytical capabilities emerge, regulatory standards related to RWD have begun to shift.

For years, researchers have been exploring the use of RWD for health outcomes research, post-marketing safety, comparative effectiveness studies, and other types of epidemiological studies[7]. Now, more regulatory agencies are recognizing the promise of RWD, and they support finding responsible ways to incorporate RWD into clinical research[8].

Generating operational efficiencies from RWD

One of the most promising areas where RWD could make a difference is in the study of rare diseases, which can be difficult or impossible to pursue within a traditional clinical trial framework. There may be as many as 7,000 rare diseases, but only a small percentage of those diseases can be viably studied in clinical trials because of low patient numbers[9]. RWD could help researchers by providing additional data that would help them achieve their data collection goals and obtain usable results despite the small numbers of available patients.

Moving towards using RWD in approvals

As more comprehensive data sets provide researchers a better understanding of the impacts of their therapies on specific populations, a paradigm that emphasizes longitudinal data analysis as part of the regulatory landscape is starting to emerge. Although the shift toward using RWD for first-time approvals is still a long way off, one of the primary ways RWD could be used to support regulatory approval is through external control arms (ECAs)), which are studies that use patient-level matched, unexposed controls to create a comparator cohort[10]. By using data collected from previously-conducted randomized control trials (RCTs), insurance claims, electronic medical records (EMRs), patient registries, and elsewhere, ECAs can either supplement the control group in a randomized trial or replace the control group entirely, depending on the circumstances. RWD is also used to support post-approval safety studies, which could help drug developers prove the safety of their therapies for specific patient populations or applications after receiving initial regulatory approval[11]. This approach is particularly useful for representing patients, like pregnant women, who are historically underrepresented in clinical trials, by amassing existing data on the real-world impacts of existing therapies.

Accessible, actionable, integrated data: the coming trend

The huge volumes of data produced in human health will continue to be an increasingly complex challenge for researchers across life sciences and healthcare.

As clinical trials continue to ramp back up to pre-pandemic volumes and beyond, the value of this data will hinge on the industry's ability to create accessible, intuitive platforms for curating and sharing it.

Advancements in artificial intelligence (AI) and machine learning (ML) as well as an increasing focus on enabling data interoperability through technology solutions that drive faster, more meaningful insights are at the forefront of solving these big data challenges. At IBM Watson Health, we're helping move this trend forward with experts who have decades of experience building retrospective healthcare databases and using real-world data across the treatment lifecycle. The clients we collaborate with will benefit from IBM's proprietary real-world data assets as well as researchers and data scientists who possess the technical expertise needed to identify, vet, access, link, and analyze external data sources.

As life sciences organizations continue to look for solutions to cut through data noise in 2022, technologies that harness the power of RWD will continue to evolve in unprecedented ways. By focusing on fit-for-purpose data platforms, patient-centered clinical trial designs, and collaborations that uncover RWD-driven insights, we will likely see emerging solutions that help organizations expedite treatment development, uncover new uses for existing treatments, and reach more patients.

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