4 REAL-WORLD EVIDENCE TRENDS
The Future of Real-World Evidence Studies

Trend 1: Improved Clinical and Commercial Collaboration

Trend 2: Repurposing Clinical Trial “Leftovers”

Trend 3: Real-World Evidence Centers of Excellence

Trend 4: Way “Beyond the Pill”

There’s No Substitute for Uncommon Expertise

The Future of Real-World Evidence Studies

About Worldwide

Last Update: 29042019
We’re in a truly transformative period for Real-World Evidence, in which increased stakeholder acceptance is being achieved through advances in technology, improved methodology, regulatory endorsement, and an overall recognition of the importance of the real-world in drug development and commercialization.

Jeff Trotter, M.B.A.
Senior Vice President, Worldwide Evidence

PREPARING FOR REAL-WORLD EVIDENCE?
Worldwide unlocks four trends to consider before you take the first step.
TREND 1: Improved Clinical and Commercial Collaboration

Starting with a prediction that’s both controversial and difficult to measure, 2019 will build on trends observed in 2018 for considerably greater cooperation and alignment between the two traditional organizational landmasses that exist in life-sciences companies. Although Clinical will always be the scientific and operational steward of drug development and Commercial will always be laser-focused on acceleration of product acceptance, a key by-product of the 21st Century Cures Act in the US was the recognition that findings from controlled clinical trials aren’t optimal for supporting medical decision-making under actual practice conditions. As a result, this policy-based validation of the need to challenge RCTs as the methodological gold standard means Clinical Development professionals should continue to be considerably more open to accommodating measures and outcomes within clinical trials that go well beyond what’s strictly necessary for regulatory approval. Anticipate advances in incorporating real-world clinical realities, economic measures, and patient-centric assessments (such as quality of life) for establishing product value in the eyes of key stakeholders and, in doing so, working collaboratively with colleagues.

Of course, even if mandated from the C-suite down and actively embraced by Clinical and Commercial professionals alike, perspectives, training, skill sets, and comfort zones vary considerably. Clients will continue to expect us to ensure balance in the design of clinical trials so that neither science nor strategy is compromised. In doing so, we will continue to referee truly passionate discussions about study end points, supporting analyses and data sources and critical operational considerations.

In this effort to address needs associated with both regulatory approval and market access, the common element is evidence: different types of evidence for different external stakeholders that allows us to ensure the right targets, data, and supporting processes are in place. We will be leveraging our unique multidisciplinary experience in supporting this shift toward increased organizational collaboration.
TREND 2: Repurposing Clinical Trial “Leftovers”

There were varying and increasingly innovative efforts to identify, access, recruit, and enroll subjects into clinical trials across disease states in 2018, and despite more patient-friendly study designs, screen failure rates continued to be much higher than actual enrollment percentages. Although we’ll continue to see improvements in the “funnel,” progressive clinical trial sponsors will also increasingly leverage the screen failures themselves (and study completers) by presenting opportunities for considerably more benign participation in observational studies and/or disease “communities.” Among the benefits achievable through such processes are:

- Compiling real-world patient experience data to support longitudinal research and natural history studies
- Compiling data to simulate a control arm for a controlled clinical trial and for ongoing safety surveillance
- Maintaining direct relationships with patients and advocacy groups in support of protocol optimization for future clinical research
- Establishing a pool of potential enrollees in future clinical (and market) research initiatives

In short, the “leftovers” from the clinical trial recruitment process represent an asset that sponsors can leverage across the product development and commercialization life cycle. In addition, sponsors will be using advanced technology to capture and compile data from the former screen failures and to maintain important patient relationships in the most responsible manner. At the same time, organizational “ownership” of such an asset will be another issue with its own set of policy, legal, privacy, and ethical implications.
Directly related to the previous predictions is the continued trend for life-sciences companies to establish Centers of Excellence (COE) for real-world evidence and, acknowledging the multiple purposes for real-world data, to place these teams organizationally somewhere between Clinical and Commercial, most often as an extension of Medical Affairs. The COEs are accessing an increasing number of real-world data sources (from EMRs, medical claims databases, pharmacy records, and wearables and other patient-centric datasets) to support needs along the product development and commercialization continuum, often saving companies the expense of multiple departments accessing the same data but for different reasons. More importantly, the COEs are employing AI, blockchain, and machine learning techniques to align disparate datasets and to create new insights that can augment (and eventually replace) traditional development and commercialization activities. As a result, the COEs are being staffed with an interesting array of professionals who have skill sets that include epidemiology, outcomes research, data management, statistical analysis, and information technology.

And the COEs are being established with fairly high expectations for liberated thinking and creativity, with budgets to match! One trend to watch out for: technology-based approaches for systematically capturing patient-reported outcomes. This goes beyond approaches available today, such as smartphones capable of fielding questionnaire responses and other user data (e.g., screen taps or activity monitoring not requiring any user interaction). Think about the capture of changes in patient quality of life transmitted directly and wirelessly from brain to device. Expect to hear about advances along these potentially very dicey lines.
As recent as just a few years back, “bundling” referred to drug/device combinations, but now we’re seeing fascinating pairings of drugs and technology specifically designed to maximize the chances of the drug truly achieving its therapeutic potential through adherence management, behavior modification, and real-time outcomes tracking.

In one form or another, the technological support will be designed (as naturalistically as possible) to maximize a product’s therapeutic benefit while providing alerts to events that might affect the outcome so that an intervention can be immediately implemented. Outcomes will also be tracked, which is an often overlooked, albeit critical, aspect of value-based contracts.
THERE’S NO SUBSTITUTE FOR UNCOMMON EXPERTISE
MEET YOUR PARTNERS

Michael Murphy, M.D., Ph.D.
Chief Medical and Scientific Officer

Dr. Murphy’s professional career has spanned 25 years, and his positions within the pharmaceutical industry emphasize the integration of medical and scientific acumen with operational excellence. He is board-certified in psychiatry and has a doctorate in pharmacology, with training at Tulane University, Stanford University, and the Mt. Sinai School of Medicine.

Dr. Murphy worked with Dr. Cutler to articulate Worldwide’s vision when the company was established as a global CRO in 1995 and was responsible for consulting services for protocol and program design and executive oversight for the execution, analysis, and interpretation of clinical trials across multiple therapeutic areas.

His supervisory responsibilities as Chief Medical & Scientific Officer at Worldwide are international in scope and include the design and implementation of protocol feasibility assessments, protocol development for phases I–IV including non-interventional research, the provision of medical monitoring and drug safety services, medical writing, and coordination of rater certification and surveillance activities for clinical trial assessments.

Jeff Trotter, M.B.A.
Senior Vice President, Worldwide Evidence™

Jeff Trotter was named Senior Vice President, Worldwide Evidence following the company’s acquisition of Continuum Clinical’s Late Stage (Observational Research and HEOR) division that completed in January 2018. Jeff served as president of Continuum since 2014, supporting clients with the critical transition from clinical development to commercialization through research excellence, responsible communications, and a constant focus on business strategy.

As an entrepreneur, researcher, consultant, and innovator with more than 30 years of experience, Jeff has been a pioneer in the evolving health economics and outcomes research community and is an industry leader in the design and implementation of patient registries and observational studies. He has spoken widely in front of varied industry audiences and has published over 50 original articles and research papers — including a book for the American Hospital Association, The Quest for Cost-Effectiveness in Healthcare: Achieving Clinical Excellence While Controlling Cost.
Matthew J. Gordon  
**Vice President, Real-World Evidence, Worldwide Evidence**

Matthew has over 20 years’ experience designing and managing observational non-interventional studies and other scientific research approaches; he has developed and led numerous successful global programs and has particular expertise in rare-disease programs. Matthew has been involved in studies that have objectives ranging from developing a better understanding of the natural history of a disease to supporting a global safety-surveillance mandate. His technical expertise includes program strategy and operational structure in disease and product registries, prospective pharmacoeconomic studies, and systematic reviews of scientific literature. Matthew is frequently published in the field of observational research and often speaks at scientific meetings about the continually evolving field of real-world research.

Les L. Noe  
**Vice President, Health Economics and Outcomes Research, Worldwide Evidence**

Les has worked in the health economics and outcomes research (HEOR) field for more than 25 years and has worked for Worldwide Evidence, a business unit of Worldwide Clinical Trials, since March 2018.

Les leads the HEOR practice at Worldwide Evidence, bringing more than 30 years of healthcare research experience in HEOR, pharmacy practice, and clinical research. Prior to joining Worldwide Evidence, he held similar positions at inVentiv Health Clinical, ICON Clinical Research, and Ovation Research Group. He has also worked within the pharmaceutical and biotechnology industries in both clinical research and health economics.

His areas of emphasis include strategic planning and consulting, prospective trial-based research, trial end point selection, patient-reported outcomes (PRO) research, cost-effectiveness and budget impact modeling, retrospective data analysis, observational research/registries, and dossier development. Les has experience in a wide range of therapeutic areas, including hematology/oncology, urology, rheumatology, neurology, gastroenterology, immunology, pulmonology, cardiology, psychiatry, wound care, and infectious disease.
The real world is real important but can be real messy, as well. Although an increasing array of datasets are becoming available, it’s critical to ask the right questions and to understand both the sources and limitations of the data. Whereas the RCT is to drug development what the automotive test track is to automobiles, the real world represents the actual traffic conditions in which “your mileage may vary.” Still, that’s where patient care and outcomes really happen and, accordingly, what really matters!

We truly are in a transformational period in which the demand for data is not only being met through advanced technology but also with considerable appreciation for the skill sets required to make sense of oft-murky data. Moreover, the future for prospective observational studies and registries looks brighter than ever, considering the multiple ways in which these research initiatives can be leveraged. At the same time, “driving” in the real world - operationalizing prospective non-interventional studies - requires unique capabilities and vision that differ dramatically from those employed in running traditional interventional clinical trials. CROs charged with designing and implementing real-world research studies should always be challenged for sensitivity to the underlying research purpose and their understanding of the critical operational nuances and organizational impact: only when executed appropriately can robust evidence of clinical, economic, and humanistic value be efficiently generated and make a true difference in the real world!
ABOUT WORLDWIDE

Worldwide Clinical Trials employs more than 1,600 professionals around the world, with offices in North and South America, Eastern and Western Europe, Russia, and Asia. Founded by physicians committed to advancing medical science, Worldwide is out to change how the world experiences CROs – in the best possible way. From early phase and bioanalytical sciences through late phase, post-approval and real-world evidence, we provide world-class, full-service drug development services.

With infrastructure and talent spanning 60 countries, we execute predictable, successful studies with operational excellence across a range of therapeutic areas, including central nervous system, cardiovascular, metabolic, immune-mediated inflammatory disorders (IMID), oncology and rare diseases. We never compromise on science or safety. We’re never satisfied with the status quo. We’re the Cure for the Common CRO.

For more information, visit http://www.worldwide.com or contact us at +1 610 964 2000.