

What is research-grade RWE and why does it matter?

Considerations in the generation of real-world evidence in the context of drug development

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Disclosures

Dr. Monda is an employee and stockholder of Amgen, Inc.

Emerging global trends present opportunities for RWE to deliver impact at scale

- The ever-expanding volume of connected healthcare data (linkage of EMR-claims-other) is paving the road to a birth-to-death digital healthcare record
- Cloud computing is revolutionizing data storage, processing, and access, creating disruption and opportunity
- Continued innovations in advanced RWE generation methods and applications including prediction and causal inference analytics are improving our understanding of disease and the value of therapeutic interventions
- The use of high-quality RWE to accelerate regulatory, reimbursement and provider acceptance continues to mature

The opportunities to generate credible, high-quality RWE to influence healthcare decision-making globally have never been greater

Principled design and analysis of RWD can answer important questions across the drug development lifecycle



Regulatory interactions

Payer/provider interactions

Standard of Care

- Treatment patterns
- Unmet need
- Benefits/risks of SOC

Healthcare Resource Utilization

- Disease burden
- Health economics
- Budget impact

Clinical/payer decisions

- Care pathways
- Impact of policy decisions
- Guideline attainment

Patient characterization

- Natural history
- Patient journey
- Comorbidity profile

Clinical program innovation

- Inclusion/exclusion criteria; endpoints
- Site/patient identification and recruitment
- Real-world comparators

CER/CSR

- Comparative effectiveness
- Comparative safety

Drug safety

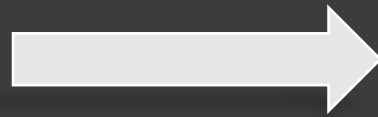
- Drug utilization
- Post-marketing commitments

Guiding principles for generating research-grade, decision-enabling, real-world evidence

- Clearly understand the question
- Select data that are fit-for-purpose
- Objectively assess whether traditional RWE is sufficient or whether advanced RWE is needed
 - Traditional RWE methods are well established and often, but not always, sufficient depending on the question at hand
 - Advanced RWE can include elements such as predictive models and machine learning, complex phenotype generation, linkage across multiple data source types
- Incorporate sensitivity/bias analyses to test robustness of findings

The key is to then apply real-world evidence analytics across the drug development lifecycle to unlock the value and reinforce as a key strategic element

Transitioning from one-off use cases to a multi-purpose, reusable and scalable RWE generation capability can help maximize the value



To do so requires a clear communication of the 'value chain' across the business



DATA

Think beyond the traditional data sources and access models



ANALYTICS

Consider strategy and scalability early on to advance adoption



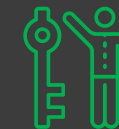
VISUALIZATIONS

Bringing the RWE story to life is key to gaining organizational buy-in



PARTNERSHIPS

No one is an expert at everything. Know your strengths and where you need to partner



ORGANIZATIONAL BUY-IN

Key to adopting advanced RWE analytics at scale. Includes risk-taking, sponsorship, strategic alignment



EXTERNAL ENGAGEMENT

Collaboration with external stakeholders (regulators, payers, clinicians) translates as 'value' to the business and, in turn, increases visibility and scalability

ENTERPRISE-WIDE CAPABILITIES

Concluding thoughts

- Given multiple advancements, the opportunities to influence decision-making using research-grade RWE has never been greater
- When generating research-grade RWE one must carefully consider
 - The study question
 - How fit-for-purpose the data are to answer the question
 - Analyses to test the robustness of the analytic plan
 - The decision-making context of the results
- Internal and external partnerships, strategic alignment, and clear communication of value is critical to building long-lasting and scalable RWE generation infrastructure