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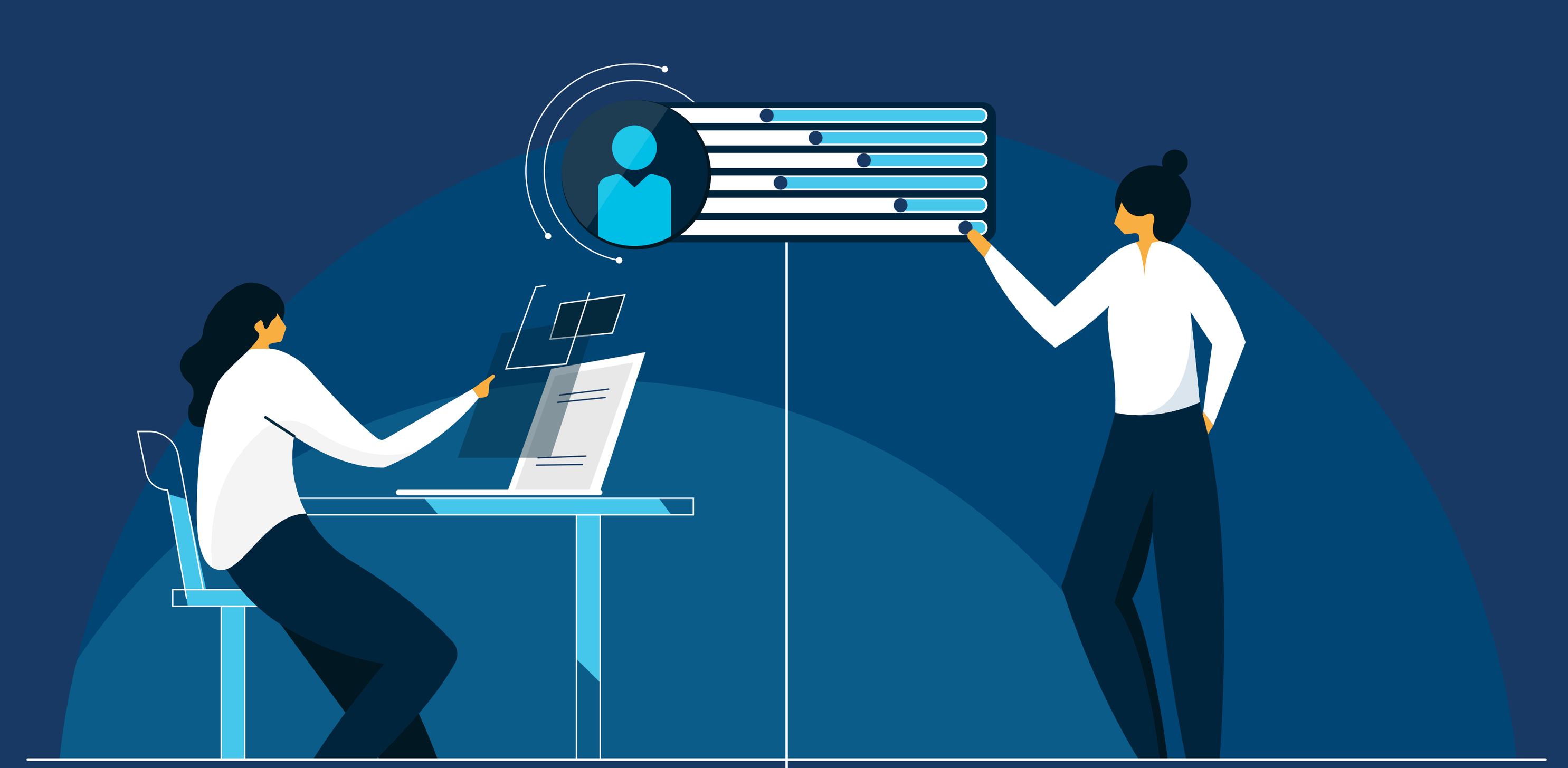
Real-World Evidence (RWE)

Why It's Here to Stay and How to Embrace It

Defining RWD & RWE



Real-World Data (RWD)

Data relating to patient health status and delivery of healthcare routinely collected from a variety of sources

- Electronic Health Records (EHR)
- Medical Claims and billing activity
- Disease and Product registry data
- Patient-generated data (in home, mobile devices)
- Data pools collected by public sector, not-for-profit, and commercial organizations

Real-World Evidence (RWE)

Clinical evidence about the usage and potential benefits or risk of medical products derived from the analysis of RWD

- Real-world evidence is the insight derived from the analysis of real-world data
- RWE can be generated by different study designs or analyses including but not limited to randomized trials, pragmatic trials, and observational studies

Drivers of the Growing Influence of RWE

90% of life science company executives have or are planning to invest in RWE capabilities

2016

21st Century Cures Act passed

- Accelerate medical product development, bring new innovations and advances to patients faster

2018

FDA Framework for Real-World Evidence released

- Encourage use of additional data sources
- Accelerate drug development through use of RWE
- Increase role of observational studies
- Assess reliability and relevance of RWD
- Examine potential gaps in RWD sources

2019

FDA's Sentinel System Broadened

- Accelerate access to and broaden use of RWD for RWE

2020

COVID-19 Pandemic

- Unprecedented challenge to public health
- Need for rapid development of vaccines and treatment
- RWE-enabled fast-tracking of preventive measures, treatments, and diagnostics to battle COViD-19
- RWE used to study existing drugs to treat COVID-19
- RWE study from CDC showed mRNA vaccines were 94% effective
- Israeli RWE study led to third booster shot for Pfizer vaccine

2021

The FDA Real-World Evidence (RWE) Framework and Considerations for Use in Regulatory Decision-Making released

- Assessing fitness-for-use of RWD in regulatory decision-making
- Potential for study designs using RWD to support effectiveness
- Regulatory considerations for study designs using RWE
- Appropriate data standards for integration and submission to FDA

Recent Approvals Using RWE

Efficacy

- BioMarin's **Brineura** (Late infantile neuronal ceroid lipofuscionsis type 2)
- Provepharma's **ProVay Blue** (Acquired methemoglobinemia)
- Fresenius Kabi's **Omegaven** (Pediatric parenteral cholestasis)
- Jazz Pharmaceuticals' **Defitelio** (Severe hepatic venoocclusive disorder)
- Merck's **Zostavax** (Prevention of shingles in persons 50 years or older)
- TB Alliance's **Pretomanid** (Drug-resistant tuberculosis)

Label Expansion

- Pfizer's **Ibrance** (Breast cancer in men)
- Amgen's **Blincyto** (Acute lymphoblastic leukemia)
- Wellstat's **Vistogard** (5-FU overdose)
- Novo Nordisk's **NovoSevenRT** (Glanzmann's thrombasthenia)
- Vertex' **Kalydeco** (Cystic Fibrosis)
- Astellas Pharma's **Prograf** (Prevent lung transplant rejections)

Rare Diseases

- Genzyme's **Lumizyme** and **Myozyme** (Pompe disease)
- Recordati/Orphan Europe's **Carbaglu** (Hyperammonemia)
- Asklepion's **Cholbam** (Bile acid synthesis disorders)
- BTG's **Voraxaze** (Methotrexate toxicity)
- Aegerion's **Myalept** (Lipodystrophy)
- Novartis' **Lutathera** (GEP-NET)
- D Serono/Pfizer's **Bavencio** (Merkel cell carcinoma)
- Novartis' **Lutathera** (Gastroenteropancreatic neurodoctrine tumours)
- Novartis' **Zolgensma** (Children with spinal muscular atrophy)

Use of RWE Across Healthcare

Physicians/Providers

- Conduct physician-led clinical research
- Monitor quality of care
- Check on treatment adherence
- Evaluate treatment effectiveness
- Assess treatment patterns and drug utilization

Payers

- Improve care affordability through claims analysis
- Support coverage decisions
- Monitor value and effectiveness of providers
- Create guidance and support for clinical practice
- Support value-based partnerships

Regulators

- Provide input to drug and device approval process
- Monitor post-market safety, complications & adverse events
- Accelerate decision-making in areas where randomized controlled trials (RCTs) are impractical

Device Manufacturers

- Conduct studies to generate innovative medical products
- Accelerate time-to-market
- Enhance testing in design phase
- Uncover safety issues sooner

Pharma

- Improve drug development process
- Optimize clinical trials
- Identify new therapy targets
- Understand and measure patient populations
- Increase understanding of disease
- Compare drug effectiveness
- Aid in outcome-based reimbursements

Integration of RWE by Life Sciences

Research and Development

- Identify diseases and indications with significant burden in population
- Optimize trial design by understanding patients, diseases, comorbidities, and concomitant treatments
- Create external control arms
- Shape target product profile and market potential

Epidemiology

- Provide scientifically-valid studies to inform benefits and risks
- Enable rapid response to regulator requests for disease information
- Identify incidence and prevalence
- Conduct study feasibility assessments
- Evaluate drug safety and effectiveness pre/post market
- Assess treatment patterns

Commercial

- Understand patient journeys
- Conduct product market forecasting
- Understand off label use of products
- Monitor product uptake and market share
- Determine market size
- Target payers/providers
- Stratify markets based on patient populations

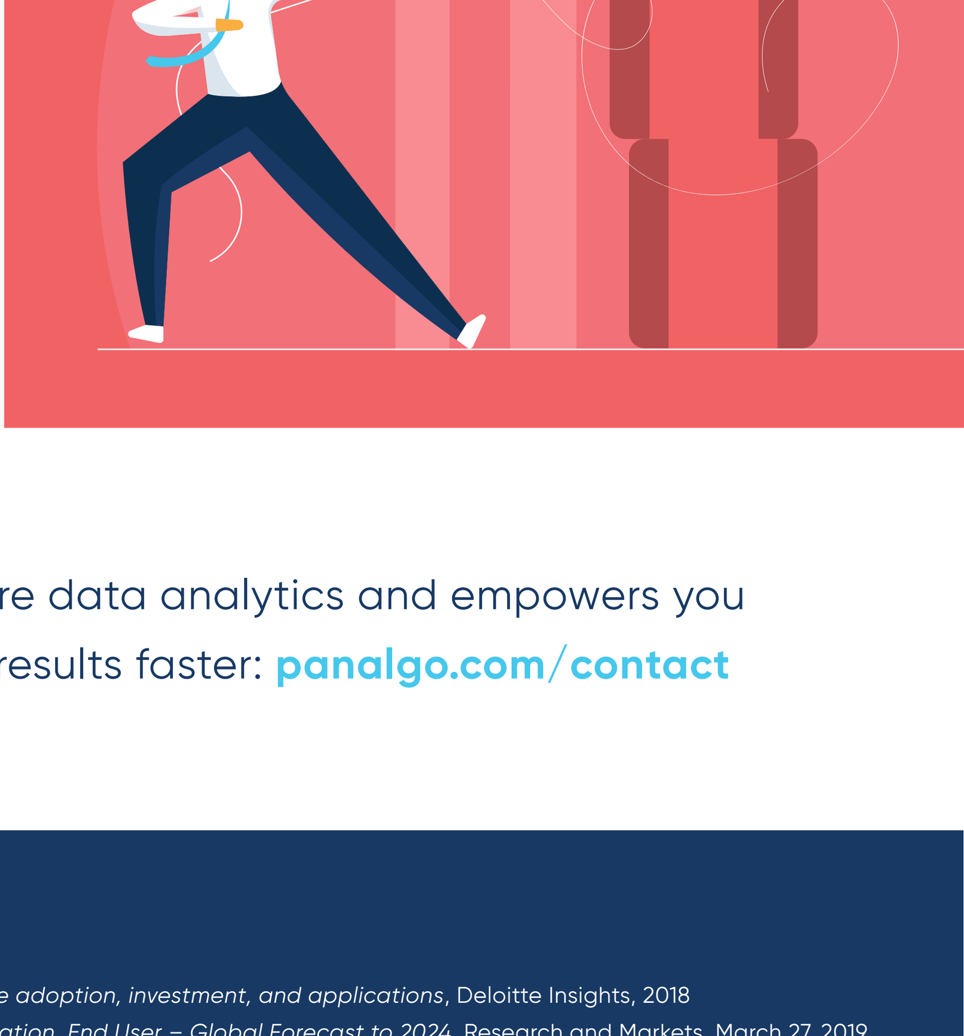
HEOR

- Conduct comparative effectiveness analysis
- Analyze healthcare resource use and costs
- Evaluate patient outcomes
- Conduct patient safety analyses
- Identify patient adherence and utilization patterns
- Generate evidence for drug access and reimbursement
- Inform and execute value-based contracts

Leveraging the Power of RWE with Panalgo's IHD Analytics

Panalgo's IHD platform enhances the power of RWE

- Streamlines the analytics process by removing complex programing – 85% faster
- Ease of use allows non-technical staff to analyze data and enables teams to scale capabilities
- Supports life sciences teams from pipeline to prescription
- Allows users to run unlimited customized queries
- Provides the ability to conduct ad hoc analyses in real time



Learn how IHD streamlines healthcare data analytics and empowers you to generate and share trustworthy results faster: **panalgo.com/contact**

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About Panalgo

Panalgo provides software that streamlines healthcare data analytics by removing complex programming from the equation. Our Instant Health Data (IHD) software empowers teams to generate and share trustworthy results faster, enabling more impactful decisions. To learn more visit us at *www.panalgo.com*.