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REAL-WORLD EVIDENCE (RWE):
HOW IT'S CHANGING THE WAY LIFE SCIENCES WORKS

DEFINING RWD & RWE

REAL-WORLD DATA (RWD):
Data relating to patient health status and delivery of healthcare routinely collected from a variety of sources

REAL-WORLD EVIDENCE (RWE):
Clinical evidence about the usage and potential benefits or risk of medical products derived from the analysis of RWD

SOURCES OF RWD

- **ELECTRONIC HEALTH RECORDS** (EHR)
- **MEDICAL CLAIMS AND BILLING DATA**
- **DISEASE AND PRODUCT REGISTRY DATA**
- **PATIENT-GENERATED DATA** (IN HOME, MOBILE DEVICES)
- **DATA POOLS COLLECTED BY PUBLIC SECTOR, NOT-FOR-PROFIT, COMMERCIAL ORGANIZATIONS**

DRIVERS OF RWE ADOPTION

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

2016 — 21ST CENTURY CURES ACT PASSED

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

2018 — FDA FRAMEWORK FOR REAL-WORLD EVIDENCE RELEASED

- Encourage use of additional data source
- 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

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

PAYORS
- 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 RCTs are impractical

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

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

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, concomitant treatment
- 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

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

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

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)

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)

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)

LEARN HOW IHD STREAMLINES HEALTHCARE DATA ANALYTICS AND EMPOWERS YOU TO GENERATE AND SHARE TRUSTWORTHY RESULTS FASTER AT:

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About Panalgo

Panalgo, formerly BHE, 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.*