INNOVATIVE TECHNOLOGIES UTILIZATION IN 21ST CENTURY NOVEL CLINICAL RESEARCH PROGRAMS TOWARDS GENERATION OF REAL-WORLD DATA AND ITS ARTICULATION FOR ANALYSIS & SUBMISSION

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INTRODUCTION

- It is well evident that safety and efficacy data collected from randomized controlled trials (RCT) provide high quality data on restricted patient populations and has been standard for determining market authorization. There are reasonable benefits in using RWE:

Less time and cost to produce meaningful data

Ability to capture additional information

Social determinants of health that can impact health outcomes

Detection of uncommon adverse events

Better utilization of innovative technologies such as AI, ML and Block Chain

Data articulation to achieve 21st century novel clinical research objectives.

Helpful in the generation of subjects pharmacogenomic information and pharmacokinetic differences between individual subjects.

Example of Differences in How Data from Conventional Randomized Controlled Trials (RCTs) & Real-World Evidence (RWE) are Utilized

Characteristics	RCTs	RWE
Standard of evidence	Gold standard	Complementary to RCTs
Cost	Costly to develop	Less Costly
Patient Population	Well-defined within constraints of specific inclusion criteria Results reflect outcomes in limited population	Broader and promotes evaluation of patients populations less often studied in clinical trials Patient data derived from other sources, including insurance claims
Sample size	Limited Requires sample size calculation to be performed in advance	Orders of magnitude larger
Efficacy	Randomized and blinding lead to minimized risk of data bias and confounding	Randomization and blinding may not be feasible Risk of unrecognized data bias and confounding greater
Adverse events	Only more frequently occurring adverse events revealed	Can reveal adverse events with much lower frequency and those requiring longer exposure to occur
Approval or Clearance of new medical products	Considered the gold standard necessary for new drug approval, and when feasible for new device approval	Not generally accepted for approving new drugs but can complement RCT findings, accepted for new device indications
Role in diabetes	Define efficacy and provide a preliminary safety profile in a well-defined and controlled population	Allows estimation of more realistic treatment effects of a wide range of diabetes interactions such as social determinants of heath and comorbidities
Other issues	May be less useful when strong signals are available form RWE or early-phase trials	Facilitates post marketing surveillance of adverse events and assessment of the product effectiveness Results may be less credible due when a control group is not included

Real World Evidence (RWE) And Real-world Data (RWD)

- Patient charts
- Laboratory reports
- Prescription refills

- Insurance claims
- Subject registries
- Patients treated on- and off-label

Real-world data (RWD) and real-world evidence (RWE) are typically used interchangeably. RWD usually refers to patient-level data gathered outside the conventional clinical trial setting. Examples include data from:

- Patients treated through expanded access
- Pragmatic clinical trials
- Mobile health device; wearables, sensors, adherence tools

- Surveys, social media platforms, online patient communities
- Secondary sources used to support decisions concerning safety, quality, care coordination, coverage and reimbursement.

Some Examples of Real-world data are shown below

Clinical
Demographics, EHR Data lab Test Results, Diagnoses, Procedures, Pathology/Histology Data, Radiology Images, Microbiology Data, Provider Notes, Admission/Discharge and Progress Reports, Performance Status

Medication
Medication Orders, Administration (Dose, Route, NDC/RxNorm codes), Concomitant Therapies, Point of Sale Data, (Prescription & OTC)
Prescription Refill, Allergies

Claims
Medical Claims, Prescription Drug Claims, Other Drug and Treatment Use Data

Molecular profiling
Genomic and Generic Testing Data (SNPs/Panels), Multi-Omnics Data (Proteomics, Transcriptomics, Metabonomics, Lipidomics), Other Biomarker Status

Family History
Historical Data on Health Conditions and Allergies Relating to Patient and Extended Family, Smoking Status, Alcohol Use

Mobile Health
Fitness Trackers, Wearable Devices, Other Health Apps Measuring Activity and Body Function

Environmental
Climate Factors, Pollutants, Infections, Lifestyle Factors (diets, stress), Other Environmental and Occupational Sources

Patient Reported
Patient Reported Outcomes, Surveys, Diaries (diets, habits), Personal Health Records, Adverse Event Reporting, Quality of Life Measures

Social media
Patient Communities, Twitter, Facebook, Blogs

Literature
Disease Burden, Clinical Characteristics, Prevalence/Incidence, Rates of Treatment, Resource Use and Costs, Disease Control, Quality of Life Measures

Rise Of Digital Health As Innovative Technology

Use of Technology	2010	2015	2020
World population (in Billions)	6.8	7.2	7.6
Devices (in Billions)	12.5	25	50
Devices (per Head)	1.8	3.5	6.5
Total No of Smart Phones, Worldwide (in Billions)	0.5	3	6

Digital technologies include a wide range of different technologies that can be used in trail processes including categories such as mobile applications, Information Technology (IT), wearable devices, sensors, platforms, and advanced analytics, such as connected devices, artificial intelligence, machine learning, and robotic processes.

As pharmaceutical industry and other organizations look to apply these technologies either individually or in combination to perform operational activities, such as recruiting patients, improving adherence, and capturing and analyzing data to wards generation of RWD.

Value Levers for Digital Technology in Clinical Development

ENGAGE

Patient-centric clinical development

- Collaborate with patients in the research process
- Ease patient access and reduce the burden of trial participation
- Transform patient care during a clinical trial

INNOVATE

Drive greater product value through new approaches and insights

- Improve adherence to therapy
- Increase statistical power and sensitivity of clinical studies
- Capture patient- centered endpoints and support product value propositions
- Get new insights from existing data
- Reduce the number of patients needed to study investigational treatments

EXECUTE

Gain efficiencies, optimize time and cost

- Expedite patient enrollment, improve retention, and increase the diversity of the study population
- Improve investigator productivity
- Identify unproductive sites for early intervention or shutdown and support risk-based monitoring
- Reduce manual effort on repetitive tasks

Any Questions?


THANK
YOU