

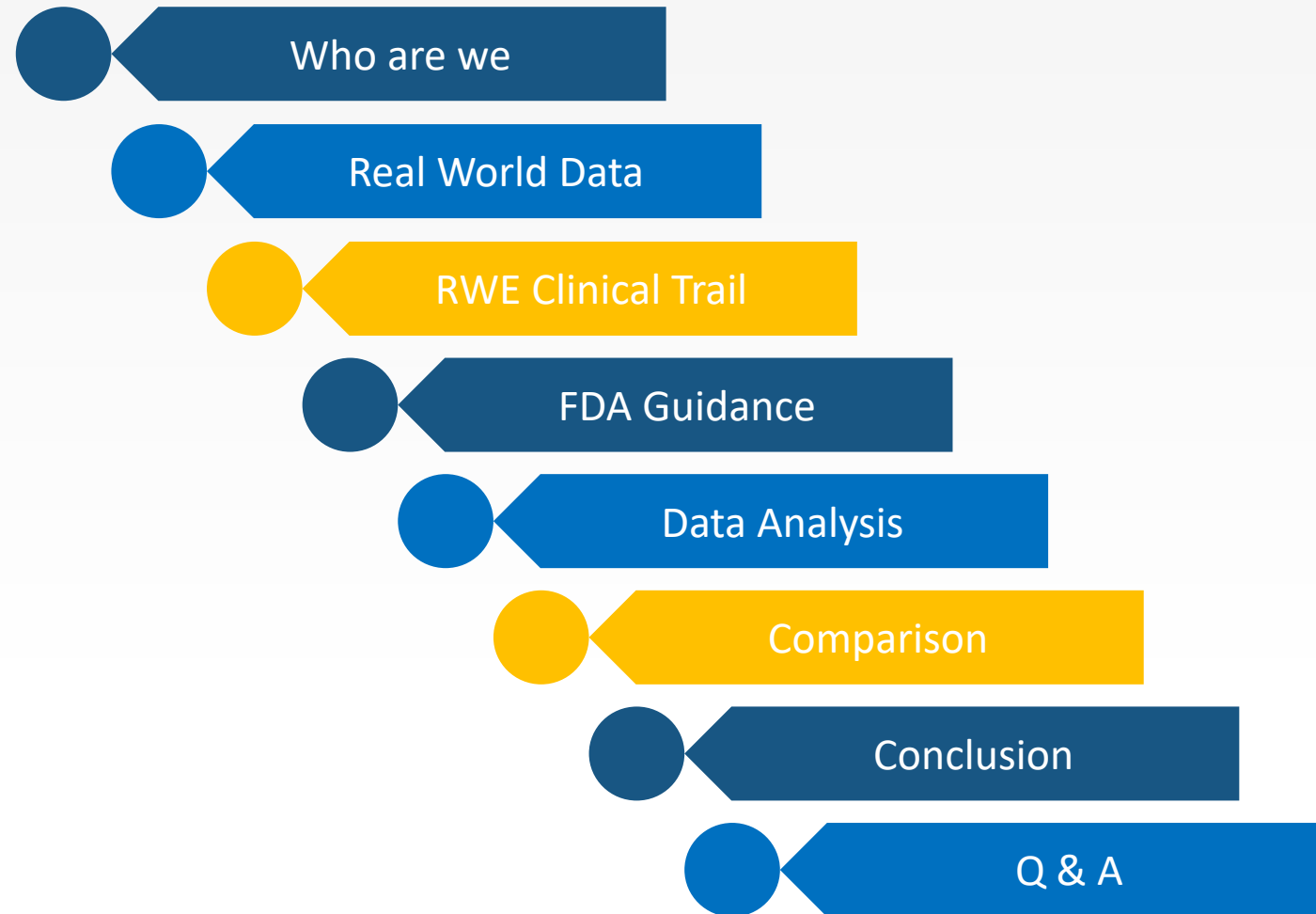
The Real Benefit to Using **Real-World Evidence** ! Studies

Presenter: **Arthur Sargsyan**

Role: **Head of Recourse Planning Department**



Agenda



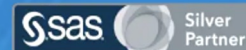
Who we are?



First generation of
Clinical SAS
programmers



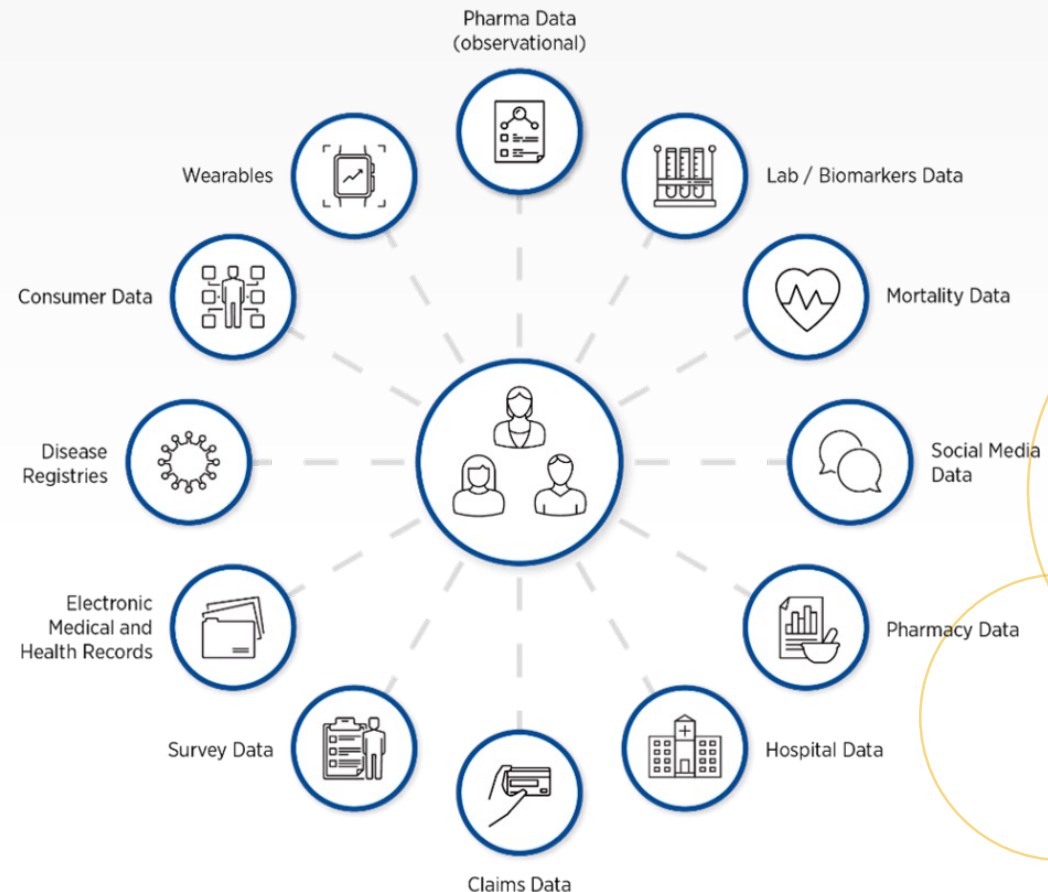
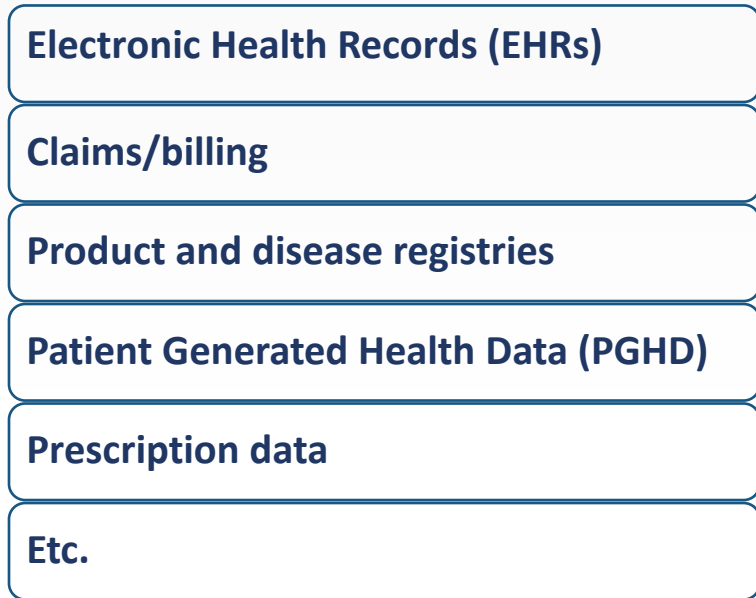
The Gurus LLC



Real World Data

Real World Data (RWD) are the data relating to patient health status and/or the delivery of health care routinely collected from a variety of sources.

Heterogeneous patient population in real-world settings



Electronic Health Records (EHRs)

Electronic Health Record (EHR) is a digital version of a patient's paper chart

Contain a patient's medical history, diagnoses, medications, treatment plans, immunization dates, allergies, radiology images, laboratory and test results, etc.



Patient Generated Health Data (PGHD)

Patient Generated Health Data (PGHD) is health data created by Patient, Family members or other Caregivers

Contain a patient's health history, treatment history, Biometric data, Symptoms, Lifestyle choices etc.



Real World Evidence

Real-world evidence (RWE) is the usage and potential benefits/ risks of a medical product derived in medicine based on the clinical evidence.

Complementing the knowledge gained from traditional clinical trials

Used to evaluate the effects of medical products



Data Analysis Challenges

Combining Data from multiple sources

Poor Data Quality

Data Size

Lack of expertise

Analysis tools

Data Analysis Tools



Excel

Python



R

SAS



JMP

Power BI



FDA

Draft Published in
September 2021

The 21st Century Cures Act

FDA Guidance is intended to provide clarity to the public regarding existing requirements under the law

Protocols submitted to FDA should identify all data sources proposed for the study maximize the completeness and accuracy of the RWD

Populations

Exposures

Outcomes of Interest

Covariates

Defining the time periods

Validation of Outcomes

Descriptive analyses should be included to characterize the missing data

Benefits

and

Pitfalls

Provide insights on Long term safety

Identify potential risk groups

Cost effective

Can help pharma companies to understand safety, effectiveness, off-label use, etc.

Potential limitations

Reliability and accuracy

Incomplete Data



Real World Evidence

Real-life Application

Fewer Inclusion/exclusion

No restriction on population

Flexible data

Open label

Long term Application Data

Reduced cost

VS

Randomized Controlled Trail

Protocol based Intervention

Strict Inclusion/exclusion

Defined sample size

Fixed Arm/Rand

Blinded/Unblinded

Short term Application Data

High Cost

Example of RWE

Ibrance (palbociclib) approvals

In April 2019, the FDA expanded the approval of Ibrance (palbociclib) to treat certain types of breast cancer in men. Before the approval, Ibrance was only approved for use in women. In this case, the manufacturer used real-world data from three databases to provide evidence for approval. Real-world evidence was useful in this instance since breast cancer isn't as common in men. So, recruiting enough participants for RCTs would be difficult. Now, men with certain types of breast cancer have an approved treatment option.

Orencia (abatacept)

In December 2021, the FDA approved Orencia (abatacept) to prevent graft versus host disease (GVHD). GVHD occurs when a stem cell or bone marrow donor's immune cells attack the recipient's body. Orencia became the first FDA-approved medication to prevent GVHD. Researchers reviewed real-world data from a registry. They looked at outcomes of stem cell transplant recipients. People who received Orencia

Questions





Thanks for **ATTENTION**

