THE VALUE OF REAL WORLD EVIDENCE IN PAYER DECISION-MAKING

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Health Economics and Outcomes Research Generates Evidence to Demonstrate Value, Inform strategy, And Gain insight across the product life cycle

**Optimizing Value**
Applying RWE to develop, support, and sustain a compelling value story for approved therapeutic interventions – and to inform new opportunities for therapeutic discovery and development via effective end-to-end RWE management

**Generating Value**
Maximizing potential for clinical and commercial success, leveraging real-world data (RWD) to segment patient populations for optimal therapeutic response and safety, and assessing category dynamics to support payer adoption and market access strategies

**Discovering Value**
Enhancing preclinical and clinical research through precise target and patient cohort identification

- Therapeutic Value Optimization and Value-Based Healthcare Strategy
- Economic Value, Evidence Translation, and Application
- Payer Adoption, Reimbursement Strategy, Market Intelligence, and Due Diligence Analyses
- Cost Effectiveness, Comparative Effectiveness, and Budget Impact Modeling and Assessment
- Evidence Generation
- Population Segmentation and Cohort Identification
- Clinical Outcomes Assessment and Trial Input
- Hypothesis Generation and Validation
External Trends Drive Demand and Demonstrate Importance of Real-world Evidence

Increased Access, Demand and Acceptance Of Real World Data, Real World Evidence, Linking Capabilities, and Technology.

Changing Payment Paradigms Emphasizing Value and Outcomes

Government Investment in Advancing Science and Applications of RWE and RWD

Heightened Regulatory Oversight on Scientific Rigor and Patient Privacy
Traditionally HEOR Primarily Focused on Generating Post-Market Evidence to Secure and Maintain Coverage and Payment of Technologies
Payers and Regulators are Advancing Applications of RWE as New Data, Methodologies, and Stakeholder Requirements Innovate and Expand across the Product Lifecycle

- Ensuring study design generates evidence relevant to payers and providers
- Supplementing clinical trial data with payer and provider data for indication expansion
- Using claims data to conduct comparative effectiveness research as a condition of coverage
- Modeling real world utilization to inform and reconcile value-based contract arrangements
- Synthesizing evidence to design care pathways to ensure optimal patient outcomes
- Evaluating the utility of claims data to conduct post-market surveillance
- Leveraging payer and provider data to help characterize patient population and develop payer evidence generation strategy
- Using claims data to conduct comparative effectiveness research as a condition of coverage

RDN Test Case

NEST

CMS Micra CED Study

Demand for Health Economic and Outcomes Research Evidence

Pre-Clinical Trial | Clinical Trial | Regulatory Approval | Post-Market Surveillance | Payer Coverage and Payment | Market Adoption

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Real World Evidence and Payer Decision Making: MitraClip Case Study

• MitraClip is an implantable transcatheter device used to repair the mitral valve in cases of mitral regurgitation (MR)
• CE Mark in 2008
• Approved by FDA in 2013 as first TMVR device for prohibitive risk patients with primary / degenerative MR
• Approved by FDA in 2019 for heart failure patients with secondary / functional MR
Real World Evidence and Payer Decision Making: MitraClip Case Study

- CMS covered under Coverage with Evidence Development (CED) in 2014
- French national registry showing “promising results” for patients ineligible for surgery (n = 62)
- German TRAMI registry showing “elderly and younger patients have similar benefits” (n = 1064)
- ACCESS-EU registry showed “significant reductions in MR and improvements in clinical outcomes” (n = 117)

Real World Evidence and Payer Decision Making: MitraClip Case Study

• CMS—
  • TMVR may improve health outcomes in very highly selected, well-informed, patients (...) in facilities that furnish an appropriate environment with data collection, as allowed through CED (...). We believe a well-designed registry could carefully monitor a clinical study for this purpose.

• RWE on MitraClip continues to be collected in the TVT registry as condition of National Coverage Decision

• November 2013 - September 2017
  • 12,334 MitraClip procedures
  • 275 sites

Using Real World Evidence to Inform Decision-making

MDIC Annual Public Forum 2019
Perry Bridger, VP Global Value and Access
Edwards Lifesciences
STS/ACC TVT Registry Implemented as Transcatheter Valves Entered the Market

Initial FDA Approval Conditional on Post-marketing Study Conducted through TVT Registry

“2. Newly Enrolled Study: ...The objectives of this study are to evaluate: (1) the **neurological and vascular outcomes** at 30 days and annually through five years post-implant, (2) the **learning curve** among surgical teams placing the device at 50 geographically disbursed sites with high, moderate and low volumes of potential patient participation, and (3) **composite safety and effectiveness endpoints** at 30 days and annually through five years post-implant... The data collection for this study... must be nested within the National Transcatheter Aortic Valve Replacement (TVT) registry housed jointly by the American College of Cardiology and Society for Thoracic Surgeons within four months of its initiation.”
Registry Reporting a Condition of Medicare Coverage

- The heart team and hospital are participating in a prospective, national, audited registry that:
  1) Consecutively enrolls TAVR patients
  2) Accepts all manufactured devices
  3) Follows the patient for at least one year, and
  4) Complies with relevant regulations relating to protecting human research subjects, including 45 CFR Part 46 and 21 CFR Parts 50 & 56

- The following outcomes must be tracked by the registry and the registry must be designed to permit identification and analysis of patient, practitioner and facility level variables that predict each of these outcomes:
  
  i. Stroke
  ii. All-cause mortality
  iii. Transient Ischemic Attacks (TIAs)
  iv. Major vascular events
  v. Acute kidney injury
  vi. Repeat aortic valve procedures
  vii. New permanent pacemaker implantation
  viii. Quality of Life (QoL)

Source: CMS National Coverage Determination (NCD) for Transcatheter Aortic Valve Replacement (TAVR). 2019
Comprehensive Registry Data can *Facilitate* and *Accelerate* Clearance and Approval

Registry Data Used for Indication Expansion for TAVR Valves

Alternate access (non-transfemoral) approval, expanding original indication for transfemoral access only

2015

Bicuspid precaution removed

2016

Mitral valve-in-valve

2017

FDA expands use of Sapien 3 artificial heart valve for high-risk patients: Expanded use approval relies on real world evidence
Insights/Observations from Transcatheter Valve RWE Experience

### Pros

- Complete, real time **assessment of device performance** in virtually all patients
- Linkage to CMS data for **long-term outcomes analysis**
- Facilitates **post-market surveillance** and timely **indication expansion**
- Utilization for refining **coverage policy**

### Cons

- **Expensive** to maintain and oversee registry data
- **Burdensome** for hospital programs to fulfill registry requirements, especially if unfunded for hospitals
- **Inequitable access** to the data may lead to biased/early evaluation

### Other considerations

- Mandatory vs. voluntary participation?
- Transparency and public reporting?
- Justifying long-term funding?