A RWD Roadmap in China: Challenges and Opportunities for IVD

Industry Perspective

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Application of RWD in IVD Industry

In vitro diagnostic products **are those reagents, instruments, and systems intended for use in the diagnosis of disease or other conditions, including a determination of the state of health, in order to cure, mitigate, treat, or prevent disease or its sequelae**

	1 R&D/Medical	2 Regulatory	3 Business/Market Access	4 Clinical Decision Support
Activities	Understand disease epidemiology/burden/unmet medical needs Describing patient journey & treatment pathways	Describing test use in populations not studied in clinical trials Justify proposed clinical trial endpoints and patient selection criteria	Monitoring adoption trends of new tests for business strategy Evidence generation to justify medical value/Impact of test use on treatment selection & outcomes	Clinical decision supporting software display, analyze, or print medical information about a patient, and support or provide recommendations to a health care professional
Value	New product concepts targeting unmet medical needs Prioritization of investments for new test development Inform clinical trial design	Enable faster product registration & claim extension Reduce costs for clinical evidence generation	Influence reimbursement & pricing decision by payers Enable inclusion of diagnostics in clinical guidelines	Support data-driven clinical decision

RWE-related Guidance Issued by FDA in 2017

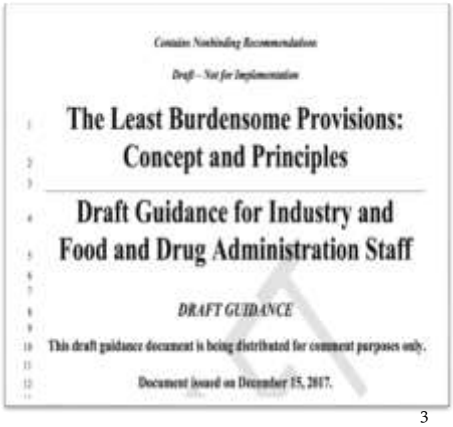
Contains Nonbinding Recommendations

Use of Real-World Evidence to Support Regulatory Decision-Making for Medical Devices

Guidance for Industry and Food and Drug Administration Staff

Document issued on August 31, 2017.

The draft of this document was issued on July 27, 2016



Contains Nonbinding Recommendations

Draft – Not for Implementation

The Least Burdensome Provisions: Concept and Principles

Draft Guidance for Industry and Food and Drug Administration Staff

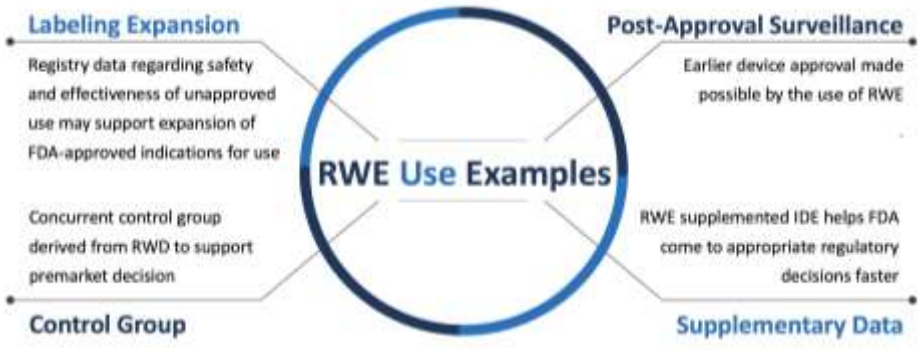
DRAFT GUIDANCE

This draft guidance document is being distributed for comment purposes only.

Document issued on December 15, 2017.

3

Examples that RWE May Play a Role in Supporting Regulatory Decision



Use of Real-World Evidence to Support Regulatory Decision-Making for Medical Devices. Guidance for Industry and FDA Staff. August 31, 2017

27

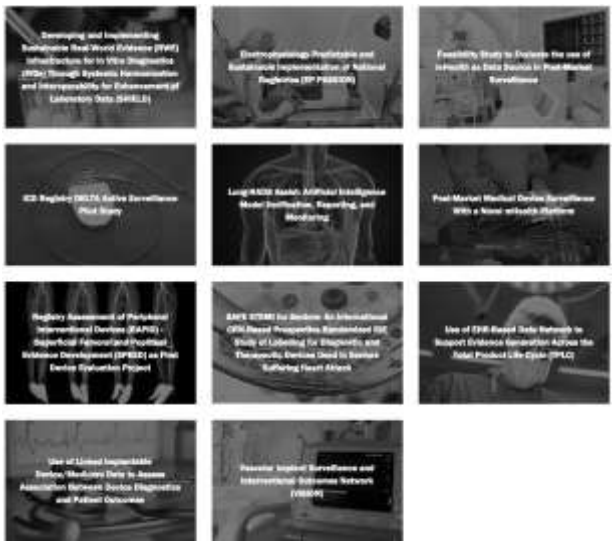
MDIC
MEDICAL DEVICE INNOVATION CONSORTIUM

Public-Private Partnership to Advance Medical Device Regulatory Science

- Members: FDA, NIH, CMS, industry, no-profits, patient organizations
- Mission: Faster, safer, more cost-effective innovation for patient benefit
- Real-World Evidence for IVDs (Clinical Diagnostics)
  - Develop framework to address challenges of RWE for IVD manufacturers, and use RWD to support regulatory & reimbursement decision-making (Deliverable: Q4 2018)

NEST
National Evaluation System for health Technology

11 Demonstration RWD Projects on Medical Devices



https://nestcc.org/demonstration-projects/

Medical Value of Intensive Testing Case Example:

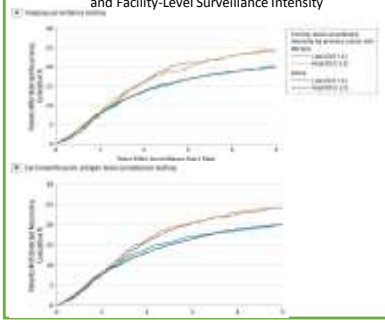
Dose intensive surveillance testing improve patient outcome?

Methods

- **DESIGN**: Retrospective cohort study
- **DATA**: National Cancer Database
- **PATIENT**: 8529 stage I-III CRC patients diagnosed 2006-2007 with follow-up through 2014
- **EXPOSURE**: Intensity of imaging and carcinoembryonic antigen (CEA) surveillance testing
- **OUTCOME**: time to detection of CRC recurrence

Results

Cumulative Incidence of Recurrence by Disease Site and Facility-Level Surveillance Intensity



Conclusion

Among patients treated for stage I, II, or III CRC, there was no significant association between surveillance intensity and detection of recurrence.

Snyder, R.A., et al., *Association Between Intensity of Posttreatment Surveillance Testing and Detection of Recurrence in Patients With Colorectal Cancer.* Jama, 2018. **319**(20): p. 2104-2115.

FDA Indication Expansion Case Example:

Leveraging a RWD Database to Enable Pre-Market Claims

FDA cleared two 510(k)s for sequencing assays for variant/variant combinations associated with cystic fibrosis using a public next-generation sequencing (NGS) database.

Traditional Studies: Full clinical trials/summary of information available in peer-reviewed literature to provide evidence of the test's clinical validity.

Study Using Public Database – An established publicly-maintained database hosted by the academic institution was used to support clinical validity of the test in lieu of clinical trials.

- Database used as a source of valid scientific evidence to establish which variants/ variant combinations were causal for the target disease.
- Additional relevant patient information, e.g. sweat chloride, lung function, pancreatic status, and Pseudomonas infection rate, associated with each variant/variant combination were included in the evaluation.

https://www.accessdata.fda.gov/cdrh_docs/reviews/K124006.pdf
https://www.accessdata.fda.gov/cdrh_docs/reviews/K132750.pdf
https://www.fda.gov/downloads/MedicalDevices/DeviceRegulationandGuidance/GuidanceDocuments/ucm509837.pdf

Michael Waters. Harnessing Real-World Data (RWD) for and from in vitro Diagnostics (IVDs). ISPOR – Baltimore, MD 5/23/2018

Challenges of Use of RWE in IVD Industry in China

- Understanding and acceptance of RWE
- Meaningful data at scale
  - Data accessibility
  - Data quality
    - Completeness
    - Longitudinal data
    - Representativeness
- Multi-sector, multidisciplinary collaboration

Thank You!