FDA – eSource and RWD/RWE: An Update

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The views and opinions expressed in the following slides are those of the individual presenter and should not be attributed to the FDA.
“eSource” to “RWD” – It’s *All* Electronically Captured Health Data!

<table>
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<th>Randomized interventional</th>
<th>Interventional non-randomized</th>
<th>Non-randomized / non-interventional</th>
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<tbody>
<tr>
<td><strong>Traditional Randomized Trial Using RWD Elements</strong></td>
<td><strong>Trials in Clinical Practice Settings</strong></td>
<td><strong>Observational Studies</strong></td>
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<td>RWD to assess enrollment criteria / trial feasibility</td>
<td>eCRF + selected outcomes identified using EHR/claims data</td>
<td><em>RCTs - Leveraging RWD</em></td>
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<td>RWD to support site selection</td>
<td>Mobile technology used to capture supportive endpoints (e.g., to assess ambulation)</td>
<td>RCTs with pragmatic design elements using claims/EHR data</td>
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<td>“eSource” uses</td>
<td>Single arm study using external control</td>
<td>Prospective data collection</td>
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<td>Registry trials/study</td>
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<td>Prospective Cohort Study</td>
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<td>Using existing databases</td>
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<td>Case – Control</td>
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<td>Retrospective Cohort Study (HC)</td>
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<td>“RWD” uses</td>
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Why Expand RWD/E Use?

• Provide new opportunities to close the divide between research and clinical care
  • Additional settings, access to more diverse populations, larger data sets

• Big data – potential for detection of infrequent events, long-term but infrequent outcomes

• Lower resource intensity – more questions answered

• Understand how medications are used in practice and value
FDA’s Real-World Evidence Program

https://www.fda.gov/science-research/science-and-research-special-topics/real-world-evidence
Today - Limited Existing FDA Guidance

What does FDA recommend?

Reflects limited relevant RWD considerations

Providing Regulatory Submissions in Electronic Format — Standardized Study Data

Guidance for Industry


Use of Electronic Health Record Data in Clinical Investigations

Guidance for Industry

How does FDA currently request it?

Does not directly reflect RWD considerations
What’s Underway?

• Scan for relevant data standards for EHR and Claims data – “what exists”
• RWE requirements in FDA – “what must be true”
• Line of sight of FDA requirements across existing (CDISC and HL7) standards – “where’s the opportunity”

Objective:
A Roadmap to a future state of standards that support RWD capture to regulatory submissions
Coordinate, Collaborate And Leverage!

- CERSI
- I-SOY Trials
- VULCAN HL7 FHIR
- TransCelerate Biopharma Inc.
- Reagan-Udall Foundation for the Food and Drug Administration
- Friends of Cancer Research
- COVID-19 Evidence Accelerator
- eSource for Clinical Trials
- National COVID Cohort Collaborative (N3C)
- Society for Clinical Data Management
- NIH
- The Office of the National Coordinator for Health Information Technology
- NSF
- U.S. Food & Drug Administration
- cdisc
- Pfizer
- UAMS
- OneSource
- eSource Implementation Consortium