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Analysis of Real-World Evidence Use Across FDA Submissions in 2022 and 2023

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PRESENT STUDY SHOWS:

How RWE was used among FDA submissions over 2022-2023, mainly to support effectiveness claims in addition to efficacy and safety data coming from clinical studies.

REFERENCES:

1. U.S. Food & Drug Administration (FDA). Frameworks for FDA's Real-World Evidence Program. December 2018. Available at www.fda.gov
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3. FDA Guidance for Industry. Real-World Data: Assessing Electronic Health Records and Medical Claims Data To Support Regulatory Decision-Making for Drug and Biological Products. July 2024. Available at: www.fda.gov
4. FDA Guidance for Industry. Real-World Data: Assessing Registries To Support Regulatory Decision-Making for Drug and Biological Products. December 2023. Available at: www.fda.gov
5. FDA Guidance for Industry. Considerations for the Use of Real-World Data and Real-World Evidence To Support Regulatory Decision-Making for Drug and Biological Products. August 2023. Available at: www.fda.gov
6. FDA. Advancing in Real-World Evidence Program. Available at: www.fda.gov

BACKGROUND

- The US Food and Drug Administration FDA has a long history of using real-world data (RWD) to generate real-world evidence (RWE) with the aim of monitoring and evaluating the post-market safety of approved drugs. RWE has also been used historically to support effectiveness, but on a more limited basis[1]
- The 21st Century Cures Act, aimed to help accelerate medical product development and access to innovation, included the use of RWE to support medical products development and approval by FDA[2]
- Since then, FDA has published multiple guidance documents on the use of RWD (including claims, electronic health records and registries, among others) to help guide on the use of RWD and RWE in support of regulatory decision-making.[3-5] FDA also launched the Advancing in RWE Program in 2023 seeking to improve the quality and acceptability of RWE-based approaches in support of new intended labeling claims, including the approval of new indications of approved medical products, as well as post-approval study requirements[6]

OBJECTIVE

- The present study aims to describe the use of RWE among applications submitted to the FDA in 2022 and 2023

METHODS

- A primary structured review of FDA submissions was performed through the FDA website and complemented with a secondary targeted review of other sources to confirm findings and complete the data extraction
- Submissions including RWE during 2022 and 2023 were identified and the following data was extracted:
  - Year of approval
  - Therapeutic area
  - Type of submission [Biologics License Application (BLA)
  - Supplemental BLA (sBLA)
  - New Drug Application (NDA) and supplemental NDA (sNDA)]
  - Clinical studies and RWE (historical/external control, natural history, effectiveness, safety) included in the submission and RWD source
- The type of application and RWE included among submissions was descriptively analyzed

RESULTS

- Overall, 15 submissions were identified that included an RWE component during 2022-2023
- Most common therapeutic areas were neurology, (33.3%, N=5), oncology (20.0%, N=3) and immunology (20.0%, N=3) (Figure 1)
- Most submissions were either NDA (53.3%, N=8) or BLA (20.0%, N=3), whereas only 2 were sNDA (13.3%) and 3 were sBLA (13.3%) (Figure 2)
- Most submissions presented evidence from either randomized clinical trials (73.3%, N=11) or single arm trials (13.3%, N=2), whereas only 2 (13.3%) relied on effectiveness from RWE sources only (Figure 3)
- Most common type of RWD came from prospective non-interventional studies (73.3%, N=11), followed by registries (26.7%, N=4) and electronic health records (20.0%, N=3) (Figure 4)
- Effectiveness was the most common outcome assessed within the RWE component (73.3%, N=11)

Figure 1. Therapeutic Areas Where an RWE Component Was Included as Part of a Submission Between 2022 and 2023

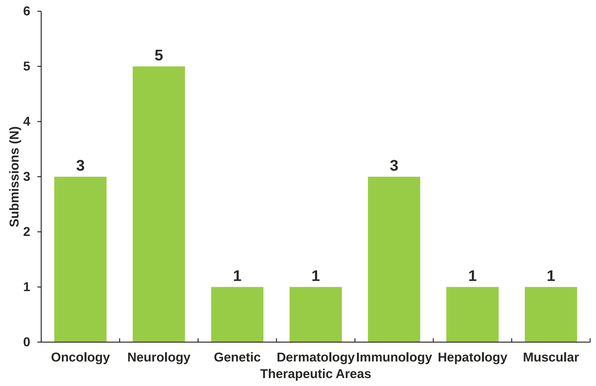


Figure 2. Types of Submission Including an RWE Component Between 2022 and 2023

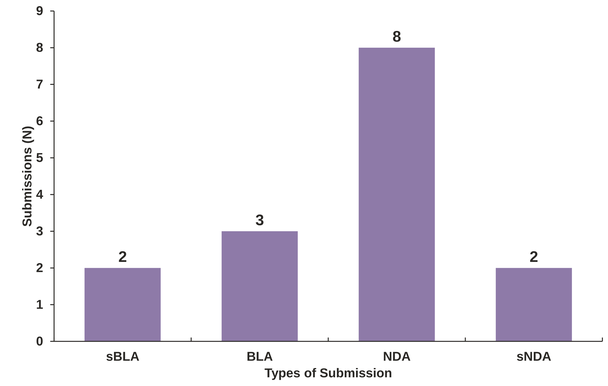


Figure 3. Type of Clinical Trial Evidence Submitted Within the Same Package of the RWE Component Between 2022 and 2023

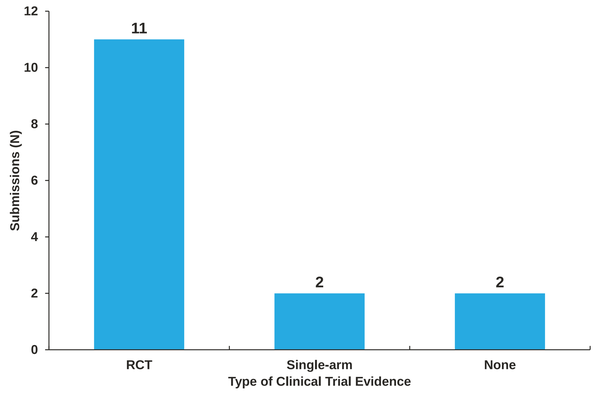
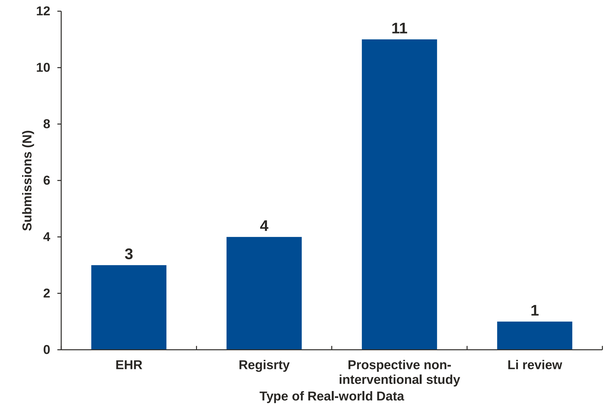


Figure 4: Type of Real-world Data Used in Submissions Including an RWE Component Between 2022 and 2023



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