Evaluating Trends in the Use of RWE in FDA New Drug Approvals

RWD60

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Making Real-World Impact

BACKGROUND

Introduction

- A 2022 study demonstrated the proportion of drugs approved by the FDA containing real-world evidence (RWE) is increasing.[1]
- The FDA released the framework for its real-world evidence program in 2018, yet there is continued debate about the role of RWE in drug approvals.[2]

Objective

- Evaluate the use of RWE in recent FDA drug approvals by assessing the frequency, purpose, and acceptability of the RWE.

METHODS

- Publicly available FDA application review files for new drug application (NDA) approvals during 2022 were systematically reviewed for the use of RWE.
- Sources included the FDA's 2022 Center for Drug Evaluation and Research (CDER) Drug and Biologic Calendar Year Approvals and the FDA's Drugs@FDA: FDA-Approved Drugs.
- Search terms included: RWE, real-world evidence, external control, non-interventional, registry, observational, retrospective, and cohort studies.
- Results were classified by therapeutic area (TA) and FDA submission classification to evaluate current trends in RWE use.
- RWE in approvals was categorized by frequency (percentage of approvals containing RWE), purpose (benchmark/disease history, formal comparison, or post-authorization request), and acceptability (did the FDA consider the RWE acceptable as support for the approval).

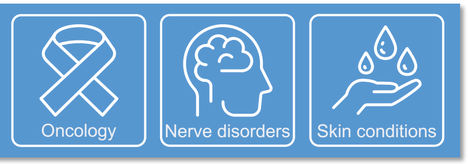
CONCLUSIONS

- In 2022, RWE was included in less than half of NDAs to support the approval of medicines by the FDA.
- The purpose of the RWE varied across the TAs evaluated, as well as submission classifications, impacting the acceptability of the RWE.
- Given high unmet patient needs, and the FDAs willingness to consider RWE in NDAs, pharmaceutical companies should consider how RWE can support rapid approval of drugs for patients.

RESULTS

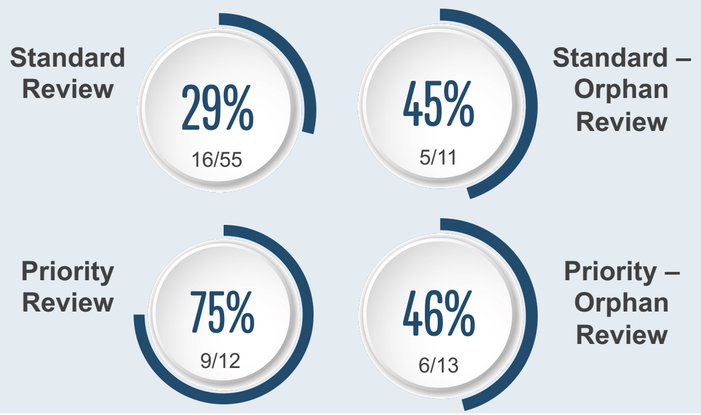
- The FDA's CDER included 92 NDA approvals for the year 2022 (91 with information available for assessment).
- Of the 91 NDA approvals available for assessment, **40%** (36/91) contained RWE within the application review files.

TOP 3 TAs with submissions containing RWE



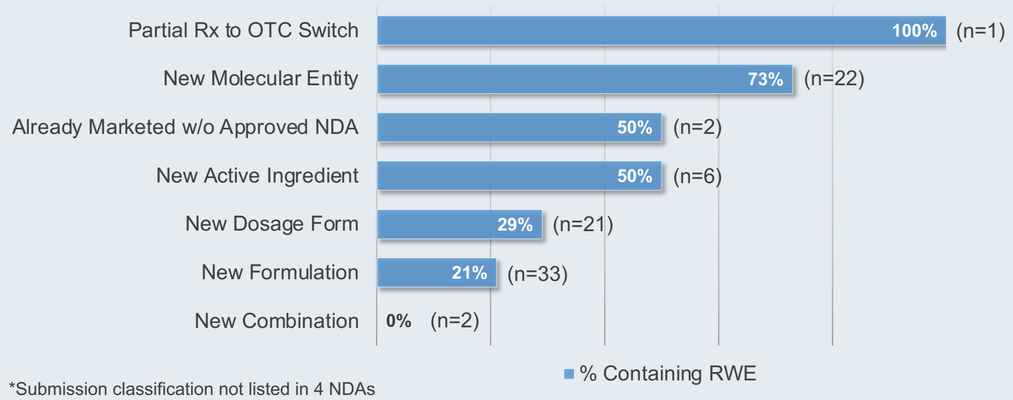
- A higher percentage of NDA's with RWE was seen in approvals categorized as priority or orphan (Figure 1).

Figure 1. NDA Approvals Containing RWE in 2022 by Review Priority



- Seven (7) submission classifications were identified within the NDA approvals, the percent of RWE included by each classification are presented in Figure 2.

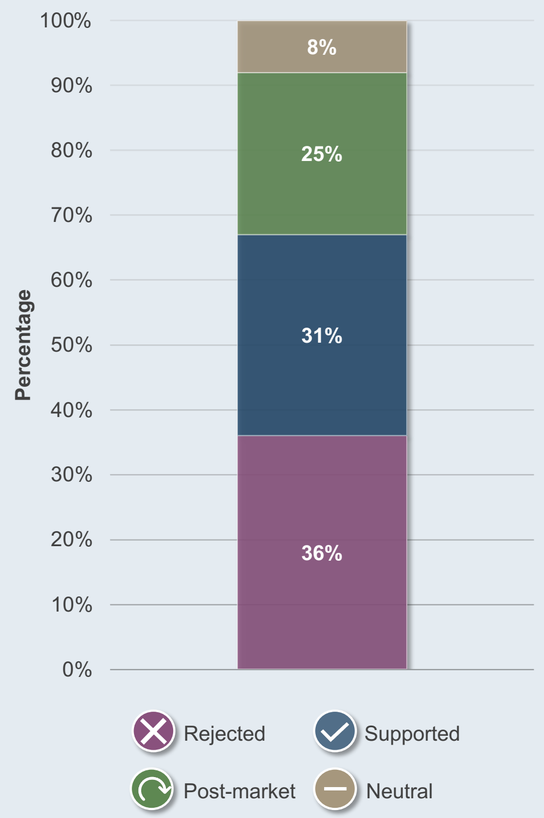
Figure 2. NDA Approvals Containing RWE by Submission Classification* (N=91)



*Submission classification not listed in 4 NDAs

- RWE was included as a benchmark or to establish disease history in **67%** of the NDA approvals.
- A retrospective study design (**69%**) was most used in evaluating RWE with data commonly sourced from registry or chart data.
- RWE was most often used to support product safety (**72%**), followed by efficacy and safety together (**25%**).
- RWE was rejected as useful in **38%** of NDA approvals, accepted as supporting the approval in **31%**, and as a post-marketing requirement in **25%** (Figure 3).

Figure 3. Acceptability of the RWE (N=36)



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REFERENCES, SUPPLEMENTAL INFO, & CONTACT