Use of Real-World Evidence in 2023 Novel Drug Approvals

Fifty-five novel drugs were approved by FDA in 2023. Only 5 applications included real-world evidence (some with multiple studies). Highlights:

- 2 external controls
- 7 extant data cohorts (6 with EHR and 3 with billing claims)
- 3 included some pediatrics (youngest age 8)

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01 Background

- There is an increasing use of data from real-world settings to provide evidence supporting regulatory decision making and approvals of medical products.
- The use of real-world evidence (RWE) can complement or serve as an alternative to evidence yielded by randomized controlled trials (RCTs).
- The US Food and Drug Administration (FDA) was required to evaluate potential use of RWE to support approval of new indications or support post-approval requirements for medicines under the 2016 21st Century Cures Act.
- FDA drafted guidance on its regulatory expectations regarding the use of RWE in drug approvals.

02 Objectives

- To assess the proportion of novel medication approvals which included RWE in their premarket submissions
- To quantify the types of RWE used
- To explore variation across factors including therapeutic area (TA), age of indicated population, review priority, or whether the RWE supported efficacy or safety

FDA Websites

List of approvals: https://www.fda.gov/drugs/new-drugs-fda-cders-new-molecular-entities-and-new-therapeutic-biological-products/novel-drug-approvals-2023
Approvals by month:
https://www.accessdata.fda.gov/scripts/cder/daf/index.cfm?event=reportsSearch.process

03 Methodology

- Identified the 2023 novel drug approvals and associated clinical reviews on FDA website
- For each drug approval, abstracted:
  - Drug name, active ingredient, approval date, approved use, review priority, TA, indicated population, indicated age, and whether any RWE was included in the review of efficacy or safety
- If RWE was part of the clinical review, also abstracted
  - Number of RWE studies in the approval package along with specifics of study design
  - If RWE included extant data, denoted type of data (EHR or insurance claims)
- Descriptive statistics calculated overall and by therapeutic area
- NOTE: use of RWE for expanded indications was out of scope for this review

04 Results

One of the 55 approvals did not have publicly available clinical review data (and, thus did not have sufficient detail for determination of RWE)

- Most common therapeutic areas: hematology (n=11, 20.4%), neurology (n=8, 14.8%), oncology (n=8, 14.8%), genetics (n=5, 9.3%), and infectious disease (n=5, 9.3%)
- Most approvals were only for treatment of adults (18+ years, 79.6%)

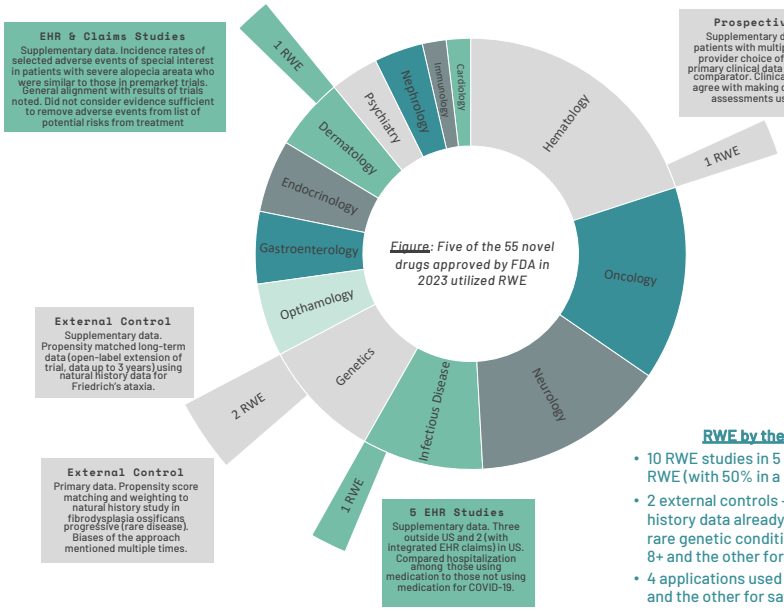

Figure: Five of the 55 novel drugs approved by FDA in 2023 utilized RWE

RWE by the Numbers

- 10 RWE studies in 5 applications using RWE (with 50% in a single application)
- 2 external controls – both using natural history data already collected; both for rare genetic conditions; one for ages 8+ and the other for 16+ years
- 4 applications used RWE for efficacy and the other for safety

05 Conclusion

- Use of RWE for novel premarket applications in 2023 remains minimal (<10%) and varied across therapeutic areas, indicated ages, and types of RWE studies
- RWE was mostly used to support efficacy (4 of 5 applications with RWE)
- FDA generally supports the use of RWE, though there are consistent remarks in reviews of new drug applications that results from RWE need to be interpreted with caution