What role does RWE play in FDA approvals?

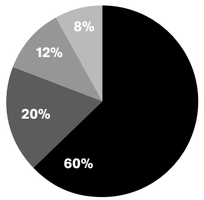
As we prepare for FDA's guidance on real-world evidence (RWE), we systematically assessed new drug and biologic approval documents in 2019 to understand how RWE influenced its decision-making.

RWE is prevalent in FDA approvals.

1 in 2 New Drug Applications (NDAs) and Biologics License Applications (BLAs) included an RWE study to support safety and/or effectiveness.*

FDA's decision on the RWE studies:

- Supportive evidence
- Inconclusive
- Substantial evidence or primary evidence
- Not addressed



RWE submission types included:

Ex-U.S. postmarket experience	12
Literature	8
Compassionate use	6
External control arm	6
Other	2

RWE informs prescribing.

61% of decisions' resultant package insert refer to the RWE studies & findings. *(11 of 18 applications)*

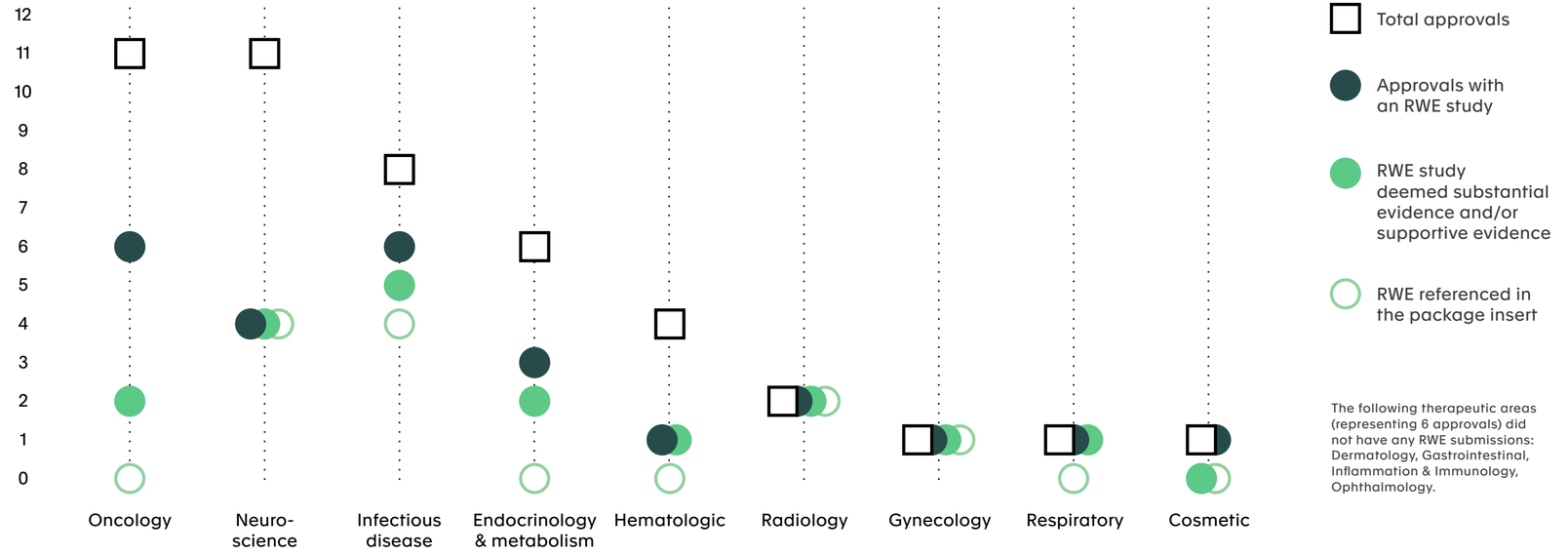
Principled database epidemiology is key to approval.

> Due to major methodological issues (**including immortal time bias, selection bias, misclassification, confounding, and missing data**), the FDA does not consider [the RWD] adequate to support regulatory decision making.

Recent example in which FDA identified several issues with an external control arm — many of these issues can be proactively addressed in study design and planning stages.

RWE spans therapeutic areas.

FDA approvals that included RWE studies were largely for treatments of serious conditions.



The following therapeutic areas (representing 6 approvals) did not have any RWE submissions: Dermatology, Gastrointestinal, Inflammation & Immunology, Ophthalmology.

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*Of the 72 NDAs and BLAs in 2019, we analyzed 51; we excluded the 21 assays, blood grouping reagents, and solutions.
Source: Aetion analysis; FDA approval documents.

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