The generalizability and transportability of FDA drug approvals in breast cancer from 2010 - 2025

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Abstract

Dr. Yedjou's "Health and Racial Disparity in Breast Cancer" reported that while there is only a 2.6% difference in the incidence rate of breast cancer between White and Black women, Black women are 42% more likely to die from breast cancer than White women. While there may be other factors that play a role in these statistics, current trial designs often fail to account for the differences present amongst race and ethnic groups, leading to regulatory approvals based on non-generalizable data. In order to further evaluate the generalizability of breast cancer drugs from the past decade (2015 to 2025), we reviewed the FDA's "Oncology (Cancer) /Hematologic Malignancies Approval Notifications" webpage. It was found that 9 out of 33 of the clinical trials associated with FDA approvals met the minimum criteria for clinical trial excellence. We advocate for the exclusive use of trials meeting the minimum standards for "clinical trial excellence" as the basis of FDA approvals ensuring equitable benefits for all patients.

Introduction

Clinical trial generalizability remains an unmet goal in oncology clinical research. Generalizability refers to the ability to infer the average treatment effect from a sample to the entire target population. This issue is particularly evident when evaluating FDA drug approvals for breast cancer in the U.S. In order to further assess the representativeness of drugs in breast cancer from 2015 to 2025, we reviewed the FDA's "Oncology (Cancer) /Hematologic Malignancies Approval Notifications" webpage and assigned a DRIVE score to each. We have previously defined a minimum DRIVE score of 3 as the benchmark for clinical excellence and relevance based on racial representation.

Methods and Materials

Between February 3, 2015, and January 27, 2025, we identified 33 breast cancer drug approvals and used the DRIVE calculator to evaluate the generalizability of drug approval for target populations for which they will be used. Each study was examined for the inclusion of demographic data and reporting of the racial/ethnic composition of its participants. Following this review, a DRIVE score, ranging from 0 to 5, was assigned to each study, with a designation of "0x" or "0*" given if the racial/ethnic data was not reported or was reported separately, respectively. We have previously defined a minimum DRIVE score of 3 as the benchmark for clinical excellence and relevance based on racial representation. Demographic information was recorded as it was available at the time of abstract submission, based on the NCI clinical trial results section.

Results

A total of 9 breast cancer clinical trials met the criteria for clinical trial excellence, revealing an overwhelming lack of generalizability within these FDA drug approvals. Over this ten-year period, 24 clinical trials failed to meet the minimum requirements for clinical trial excellence. The full score breakdown is as follows: 3 received a score of 0x, 2 received a score of 0, 5 received a score of 0*, 13 received a score of 1, 1 received a score of 2, 8 received a score of 3, 1 received a score of 4, and 0 received a score of 5.

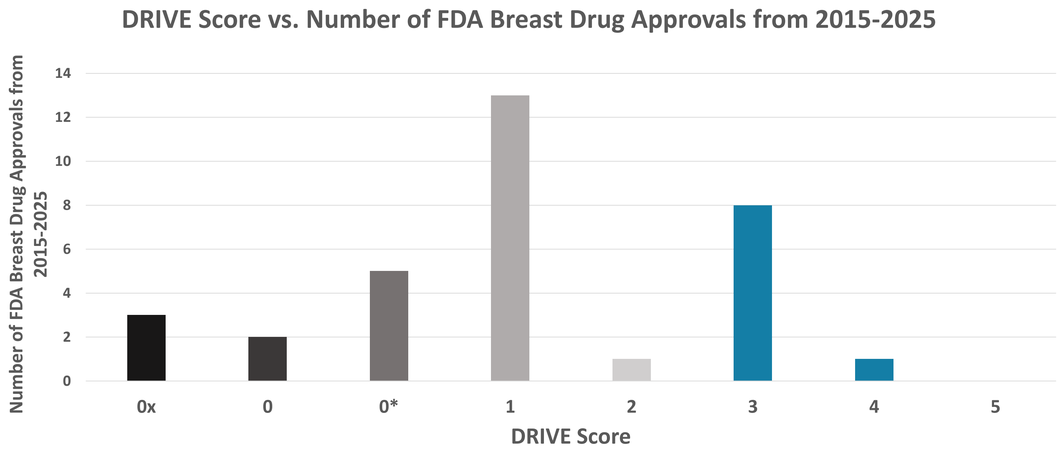


Figure 1.

Graph of DRIVE Scores vs the number of FDA Lymphoma Drug approvals from 2015 – 2025. A score of 3 or above indicates the trial achieved clinical trial excellence. A score below 3 indicates a failure to achieve clinical trial excellence. A score of 0* is assigned to studies who reported racial demographic data, but in a format incompatible with the DRIVE Calculator and updated FDA guidelines. A score of 0x indicated that no racial demographic data was reported.

Conclusions

Our findings reveal that 9 out of 33 of the clinical trials associated with FDA approvals met the minimum criteria for clinical trial excellence. Although all the breast cancer drugs evaluated have received FDA approval, 72.5% do not accurately represent the intended patient populations. This lack of representation exacerbates disparities, particularly among minoritized populations, and leaves critical gaps in understanding potential adverse reactions or side effects. The aim of this analysis is to emphasize the need to minimize therapeutic misconceptions, the assumption that approved drugs with promising clinical trial data have demonstrated effectiveness across all patient populations. A clear understanding of the DRIVE Score Ranking and its purpose can assist clinicians and patients identify which drugs are supported by generalizable and transportable data, ultimately guiding more inclusive and equitable clinical research practices. For these reasons, we advocate for the exclusive use of trials meeting the minimum standards for "clinical trial excellence" as the basis of FDA approvals ensuring equitable benefits for all patients.

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