

An Evaluation of PMR/Cs for Assessing Data in Racial and Ethnic Populations Underrepresented in Pivotal Trials Supporting Novel Oncology Approvals, 2012-2023

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Objectives

Identify trends in approval and trial characteristics for drugs with and without race and ethnicity postmarketing requirements/commitments (R/E PMR/C).

Methods

- **Compiled PMR/Cs** included in novel oncology drugs' approval letters 2012-2023.
- **Identified R/E PMR/Cs** by searching PMR/C descriptions containing the terms "race", "racial", "ethnicity", "ethnic", and/or "represent."
- **Collected approval characteristics** for all drugs approved 2021-2023.
- **Analyzed data** to identify trends based on approval characteristics.

Sources: Approval letters and review documents found on Drugs@FDA and CBER's page of novel biologic approvals with supporting documents.

Conclusions

- Prior to 2020, FDA issued no PMR/Cs specifying the need for improved representation based on race and ethnicity. Between 2021-2023, more than half of drugs approved had a R/E PMR/C.
- Trials supporting approvals 2021-2023 underrepresented racial and ethnic groups.
- Underrepresentation in clinical trials is not the only factor driving R/E PMR/Cs.
- Certain approval characteristics (i.e., single-arm trials, AA, lower U.S. participation, potential safety signals) may contribute to the issuance of a R/E PMR/C.
- PMR/Cs may be used for characterizing optimal use of a drug in a more representative population; however, stakeholders should prioritize ensuring premarket clinical trials are inclusive.

It is important to identify factors that may influence whether a R/E PMR/C will be issued, which can inform more optimal trial designs and help to ensure clinical trials provide information to characterize use in a diverse and representative patient population. In addition, it may be necessary to ensure clinical trials are sufficiently powered to evaluate differences in safety and efficacy, especially when known disparities are present.

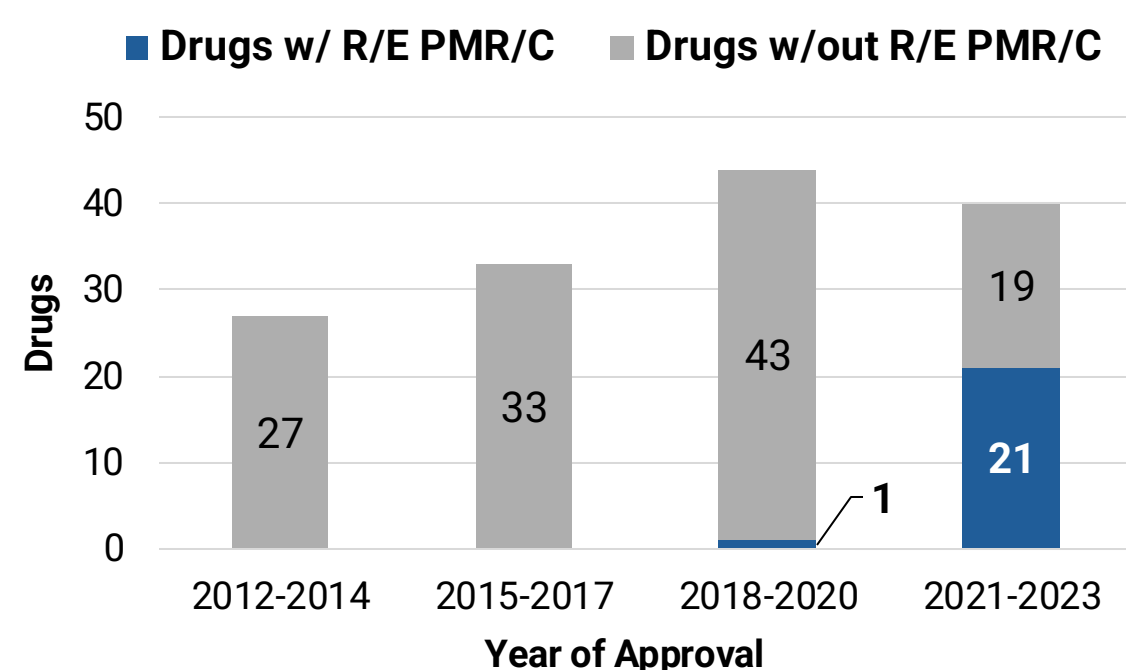
Disclosures

The authors have nothing to disclose.

Results

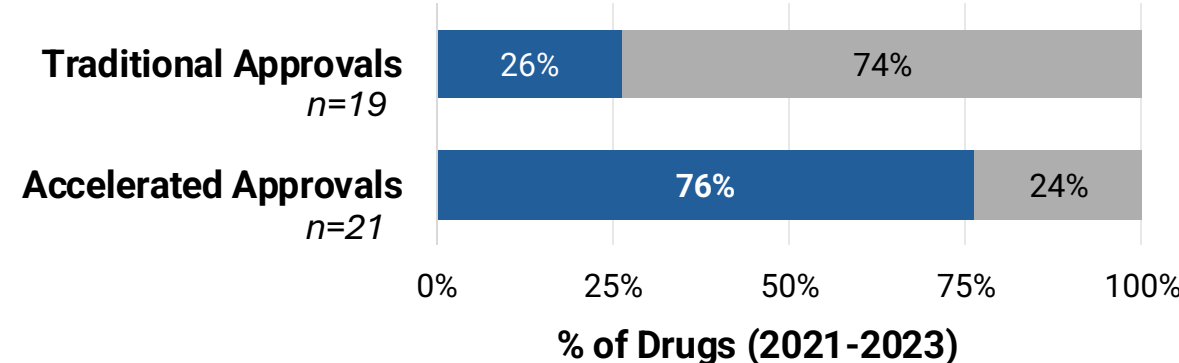
Year of Approval

More than half of drugs approved 2021-2023 (21/40, 53%) had a R/E PMR/C. Prior to 2021, only 1/104 drugs approved had a R/E PMR/C.



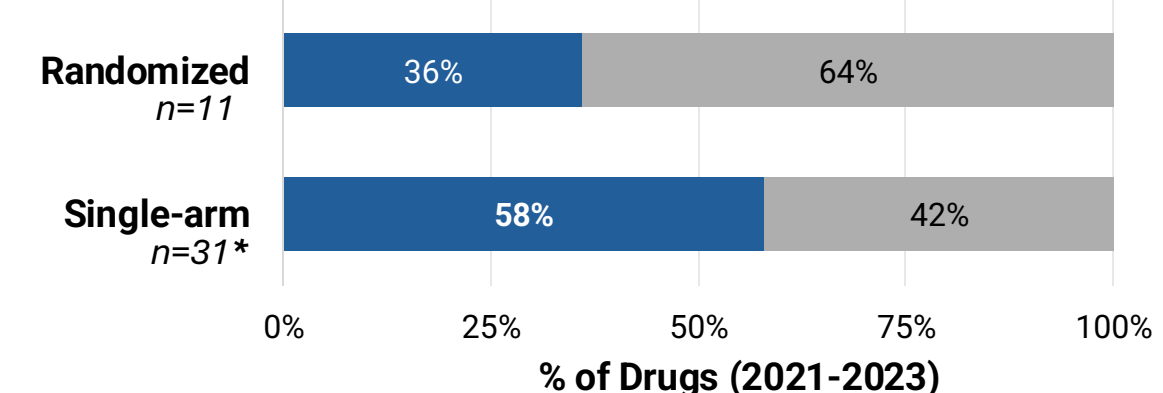
Approval Pathway

Most accelerated approvals (16/21, 76%) had a R/E PMR/C.



Pivotal Trial Design

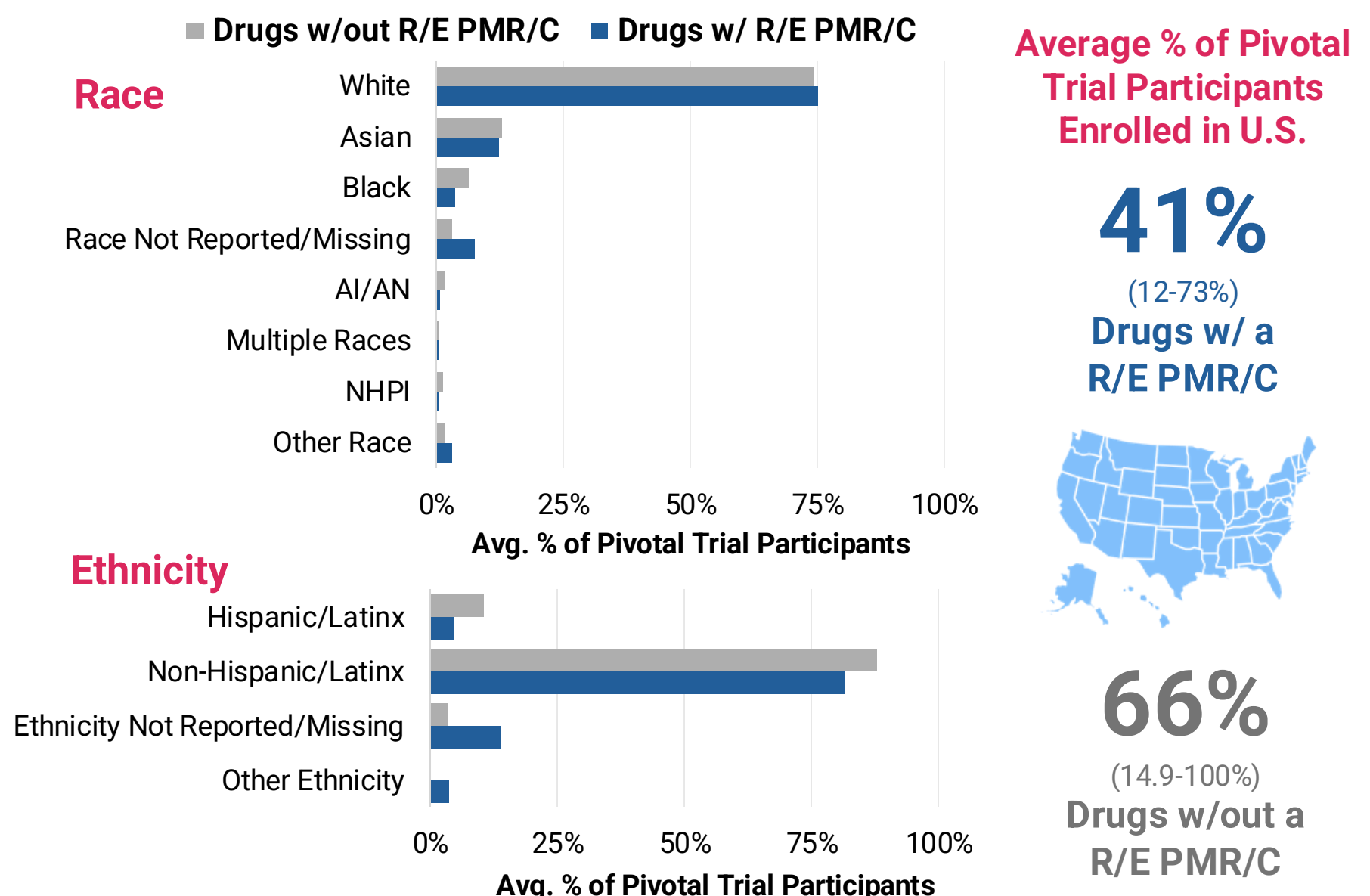
Most approvals supported by a single-arm pivotal trial(s) (18/31, 58%) had a R/E PMR/C.



*Two approvals were supported by one randomized trial and one single-arm trial and are included in the counts for both trial design categories.

Pivotal trials supporting drugs with a R/E PMR/C included fewer patients who are Hispanic/Latinx (4.8% vs. 10.5%), Black (3.7% vs. 6.6%), American Indian and Alaska Native (AI/AN) (.9% vs. 1.6%), Native Hawaiian and Pacific Islander (NHPI) (.5% vs. 1.3%), and enrolled in the U.S. (41% vs. 66%), and reported more missing/not reported data for race (7.6% vs. 3.3%) and ethnicity (14% vs. 3.5%) compared to trials supporting approvals without a R/E PMR/C.

Trial Demographics



Average % of Pivotal Trial Participants Enrolled in U.S.

41%
(12-73%)
Drugs w/ a R/E PMR/C



66%
(14.9-100%)
Drugs w/out a R/E PMR/C

Note: To allow for comparison, the analysis of trial demographics focuses on indications for which there was at least 1 approval with a R/E PMR/C and at least 1 approval without a R/E PMR/C (i.e., NSCLC, NHL, MM, Gynecologic Cancers) (n=23 drugs; 15 w/ R/E PMR/C; 8 w/ out R/E PMR/C)

Efficacy, safety, dosing, and PK subgroup analyses indicated a potential difference for more drugs with a R/E PMR/C compared to those for drugs without. Potential safety differences were most common: 7 drugs with a R/E PMR/C & 5 drugs without a R/E PMR/C had a potential safety difference.

Subgroup Analyses

