Top 10 myths about working with the FDA for an oncology drug approval

How can you establish a clinical strategy for scientific and commercial success? Understanding the FDA is a big part of that. With 80+ former regulators within our 1000+ strong consulting team, we speak from first-hand experience.

1 The FDA gives all new products accelerated approval based on a Phase 1/2 trial.

From 2016 to 2019,[1] there were 115 regular approvals versus 39 accelerated approvals for new molecular entities.

2 The FDA requires two randomized trials with overall survival as the endpoint, allows enrollment only of U.S. patients, and does not permit crossover within the pivotal trial.

Oncology is the exception to the two-trial rule. One pivotal trial is usually adequate. There is no requirement to conduct a trial in the U.S. population, provided the trial population is relevant to the U.S. population. The FDA does not prohibit crossovers; the sponsor assumes the risk.

3 The FDA and the industry agree that more is always better for dosing in an oncology setting.

Monoclonal antibodies and cellular therapies often do not reach a maximum tolerated dose (MTD) level. The FDA emphasizes that MTD designs are not optimal for biologics or small molecules and encourages adaptive designs with better biomarkers for dose-finding.

4 FDA advisory committees such as the Oncologic Drugs Advisory Committee (ODAC) are the final decision-makers in drug approval.

ODAC is solely an advisory body with no decision-making role.

1. Food & Drug Administration (FDA). Hematology/Oncology (Cancer) Approvals & Safety Notifications. Available at: https://www.fda.gov/drugs/resources-information-approved-drugs/hematology oncology-cancer-approvals-safety-notifications Accessed August 2020.

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5 The FDA determines the cost of anticancer agents.

The FDA reviews scientific data to determine drug approval based on the risk/benefit ratio and has no decision-making over drug cost.

6 The FDA will reject oncology clinical trials where there is discordance between the investigator's and the blinded independent review committee's (IRC) assessment of radiology scans.

The FDA recognizes that discordance is not unusual, with discordance regularly occurring in up to 30% of patients.[2, 3]

7 The FDA has never granted a marketing approval using real-world data (RWD) as evidence of efficacy for the investigational product.

While FDA guidelines are still forthcoming, there are multiple examples where RWD has influenced regulatory decision-making and supported both initial and supplemental marketing applications for new indications.

8 The FDA chooses to withhold its reasons for a negative review and non-approval of a drug.

While the FDA website does post reviews and letters for approved drugs, it is legally prohibited from sharing negative reviews.

9 The FDA does not use the expedited programs available to speed drug development for promising therapies.

The FDA offers several expedited approval programs for drugs that show promise in early trials.

10 Regulatory agencies act independently of one another.

The FDA holds a monthly teleconference with peers including the EMA, Health Canada, Swiss Medic, PMDA (Japan) and TGA (Australia).

To learn more, download our Oncology eBook.

2. Dodd LE, et al. "Blinded independent central review of progression-free survival in phase III clinical trials: important design element or unnecessary expense?" J Clin Oncol. 2008 Aug 1;26(22):3791-6. doi: 10.1200/JCO.2008.16.1711
3. Zhang JJ, et al. "Evaluation of blinded independent central review of tumor progression in oncology clinical trials: a meta-analysis." Drug Inf J 2013. 47.167-74. doi: 10.1177/0092861512459733

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