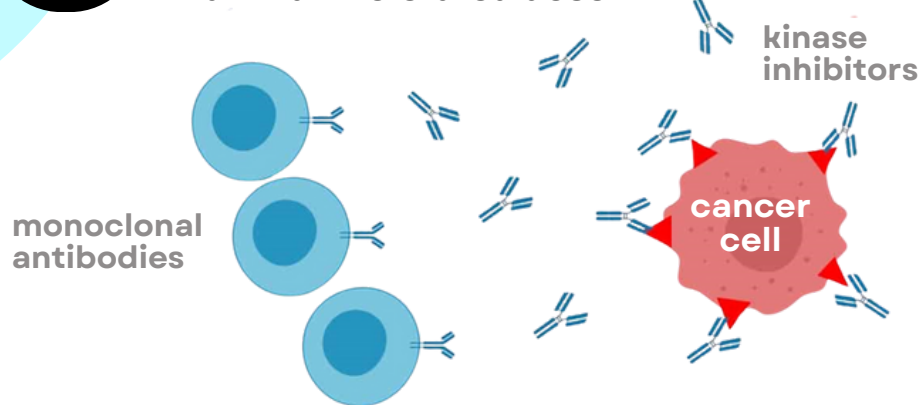


WHY CAPTURING PRO DATA MATTERS IN EARLY-PHASE ONCOLOGY TRIALS

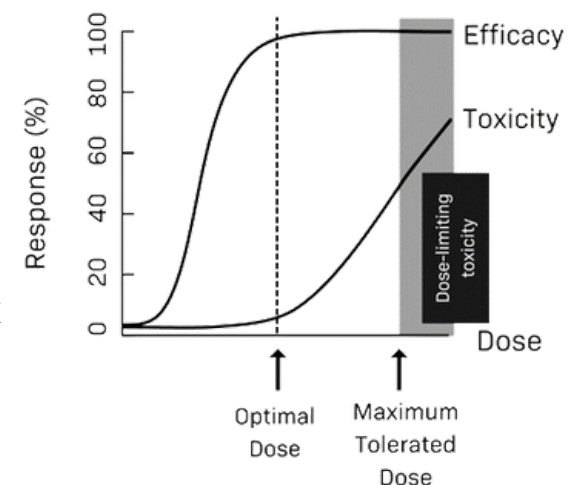
01

Newer, targeted treatments often show good efficacy at a range of doses below the maximum tolerated dose



02

Characterizing the dose-tolerability relationship is essential for optimal dose selection and limiting the risk of dose adjustments in later phases or post-marketing



03

Standard measures of tolerability are important, yet fail to account for the patient voice

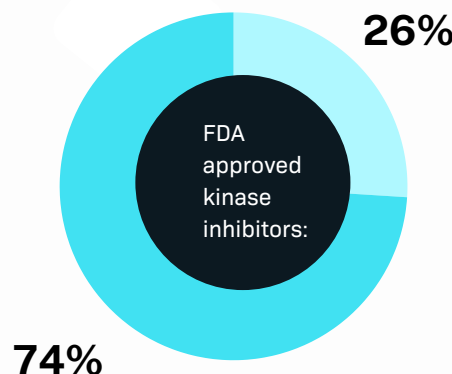
STANDARD MEASURES OF TOLERABILITY

CASE REPORT DATA

- Dose modifications
- Treatment pauses
- Treatment discontinuation
- Hospitalization
- Death

CLINICIAN-REPORTED OUTCOMES

- Adverse events (CTCAE)



26% of FDA-approved kinase inhibitors (2001-2015) were approved with post-marketing requirements (PMRs) or post-marketing commitments (PMCs) to study alternative doses

- Sorafenib (Renal cell carcinoma)
- Erlotinib (NSCLC)
- Vandetanib (Medullary thyroid cancer)
- Cabozantinib (Medullary thyroid cancer)
- Ponatinib (Chronic myelogenous leukemia)
- Dabrafenib (NSCLC)
- Lenvatinib (Differentiated thyroid cancer)

04

Clinicians systematically **under-report** and **under-score** adverse events compared to patient self-report

05

Only collecting clinician-reported data limits accurate tolerability assessment

The validity of AE reports erode when filtered through research staff and clinicians

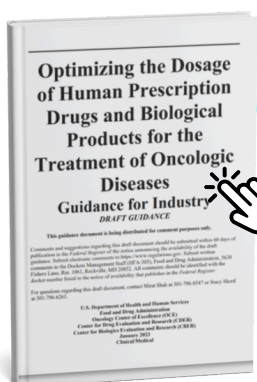
In future, tolerability assessment without patient-reported AEs will be considered incomplete

FDA 9th Annual Clinical Outcome Assessment in Cancer Clinical Trials (COA-CCT) Workshop – 25 June, 2024

06

Regulators request patient-reported outcomes data to complement traditional measures of tolerability

Inclusion of PROs should be considered to enhance the assessment of tolerability in early phase dosage finding trials



07

We recommend sponsors collect patient-reported AEs and the patient perspective on the impact of AEs on their functioning

AEs

+

Impact of AEs

- PRO-CTCAE

- Overall impact of side effects
- Physical function
- Role function

08

- 01 Ensure data quality
- 02 Simplify at-home completion
- 03 Drive on-time assessments
- 04 Facilitate effective remote patient monitoring
- 05 Enable complex measure implementation

Why use ePRO?

READ: ePRO VS PAPER