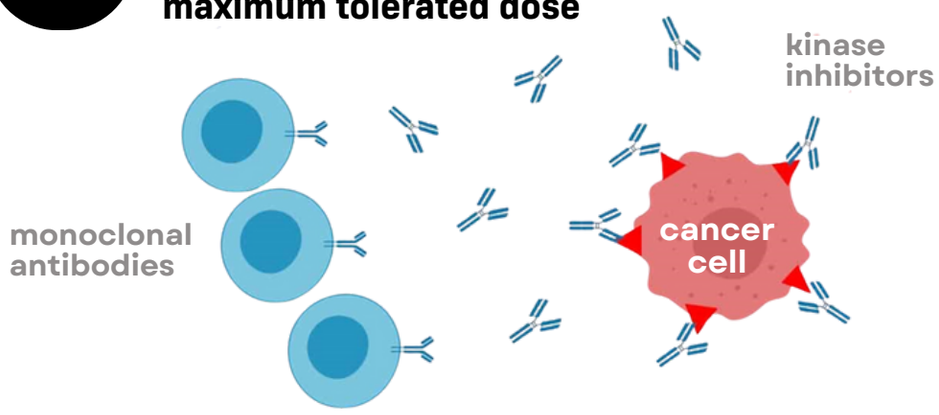
WHY CAPTURING PRO DATA MATTERS IN EARLY-PHASE ONCOLOGY TRIALS

SIGNANT HEALTH
THE EVIDENCE GENERATION COMPANY™

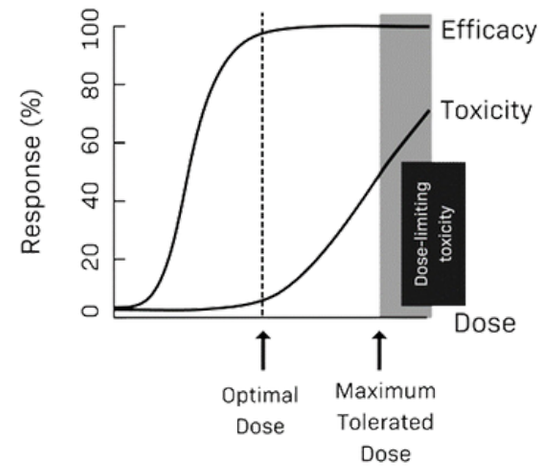
01

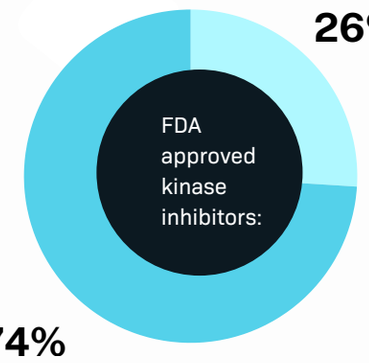
Newer, targeted treatments often show good efficacy at a range of doses below the maximum tolerated dose



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





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)

03

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

STANDARD MEASURES OF TOLERABILITY

CASE REPORT DATA	CLINICIAN-REPORTED OUTCOMES
• Dose modifications • Treatment pauses • Treatment discontinuation • Hospitalization • Death	• Adverse events (CTCAE)

04

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

05

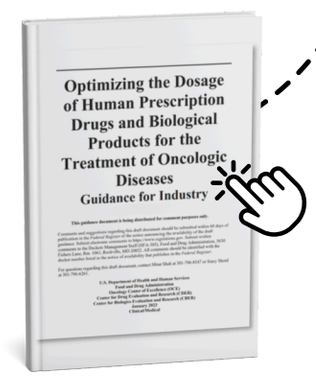
Only collecting clinician-reported data limits accurate tolerability assessment

> 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

Why use ePRO?

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**

READ: ePRO VS PAPER

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