

Assessing Symptoms and Functioning in Clinical Cancer Research to Evaluate Treatment Benefit

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Clinical Benefit

A positive, clinically meaningful effect of an intervention, in terms of how an individual...

...feels, functions, or survives.



“What we observe is not nature in itself but nature exposed to our method of questioning.”

-Werner Heisenberg, 1958

FDA's Patient-Focused Drug Development (PFDD) Guidance Series for Enhancing the Incorporation of the Patient's Voice in Medical Product Development and Regulatory Decision Making.



Collecting Comprehensive and Representative Input



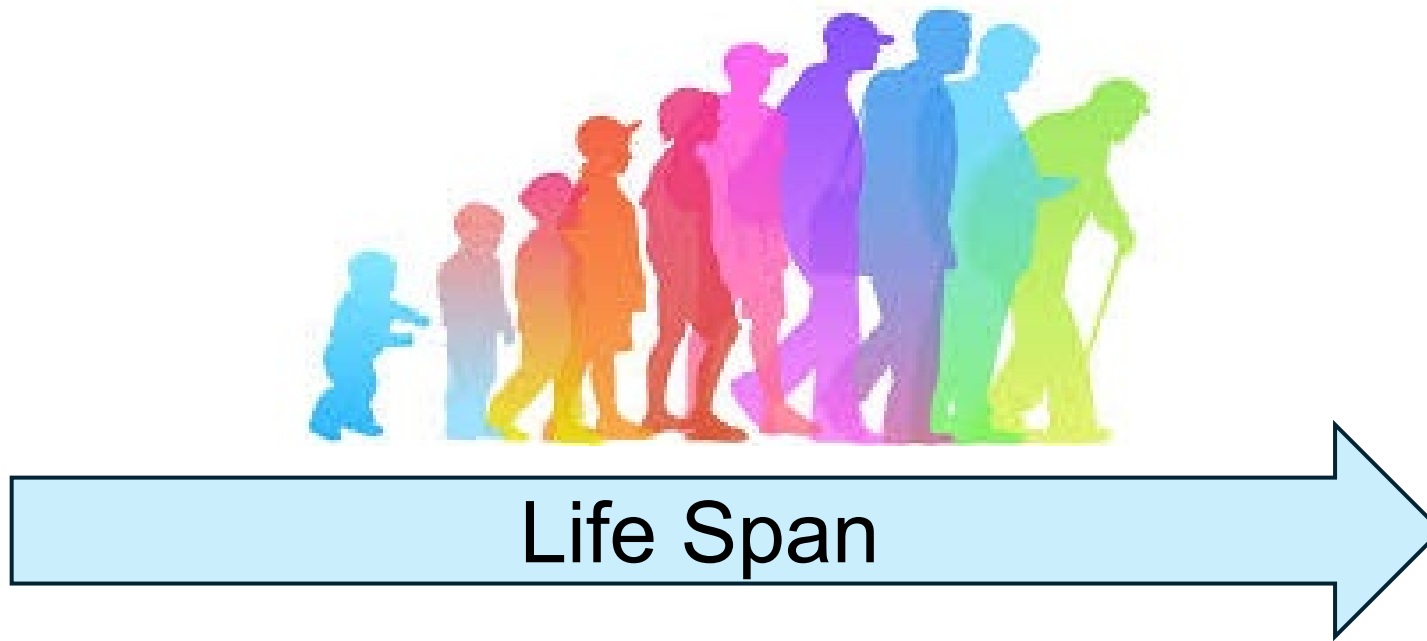
Methods to Identify What is Important to Patients



Selecting, Developing, or Modifying Fit-for-Purpose COAs



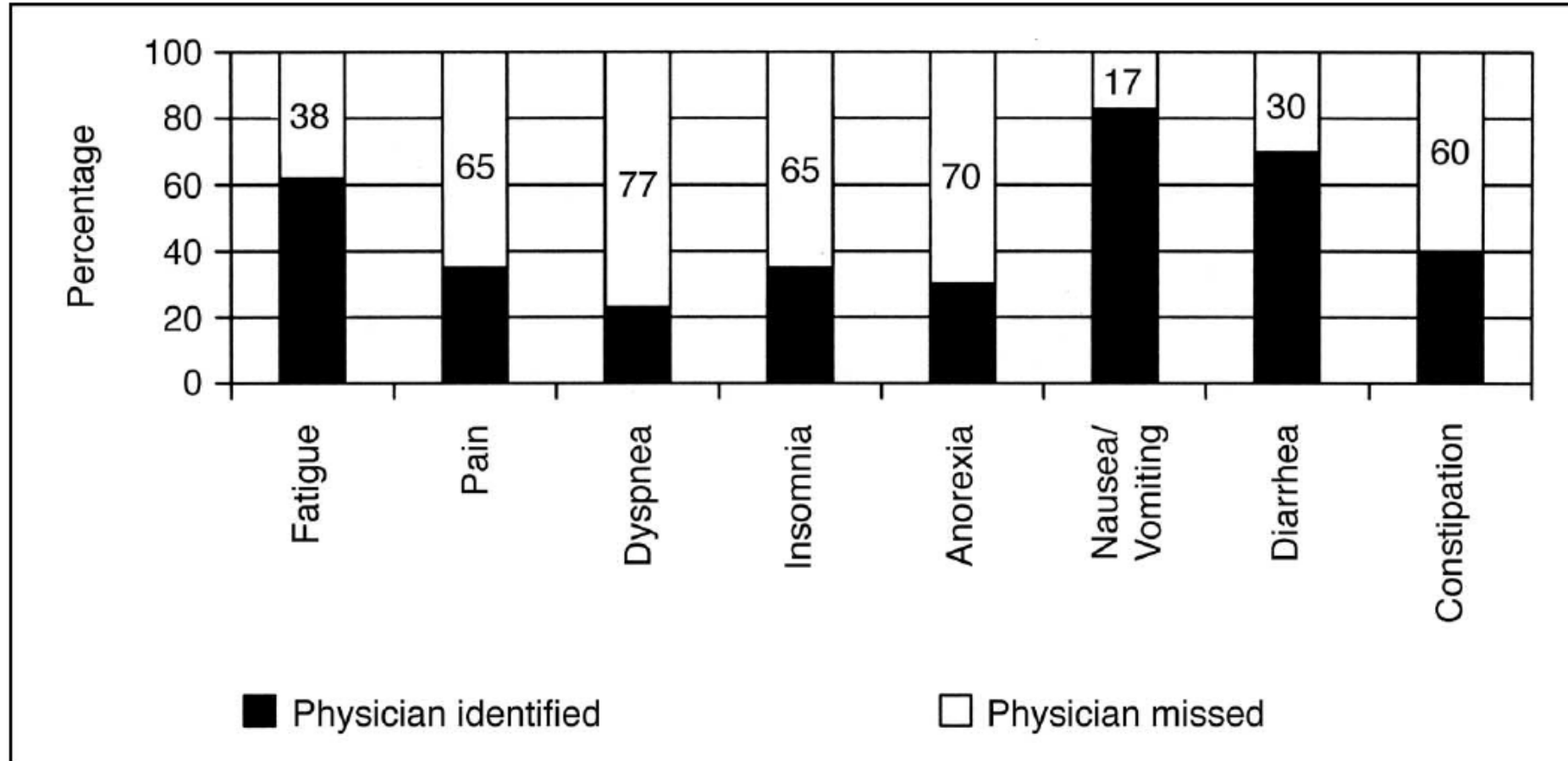
Incorporating COAs into Endpoints for Regulatory Decision Making



1. What symptoms and functional outcomes should we be assessing?
2. Who is the best source for this information?
3. Is there an available fit-for-purpose COA?
4. How to integrate the COA in the study to maximize data quality and value?
5. How do we summarize findings and make decisions?

Clinician Reports Don't Reflect Patient Reports

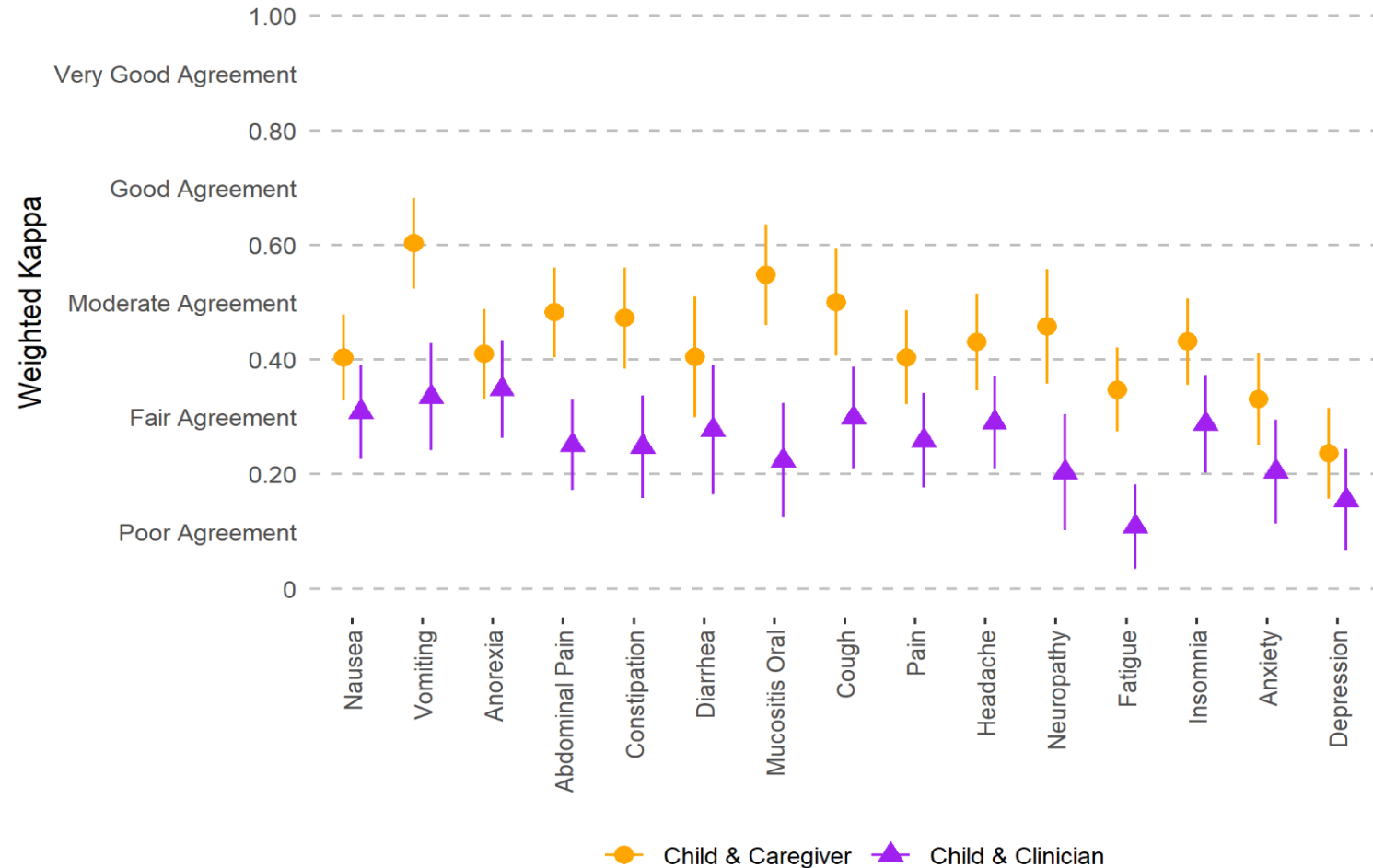
Phase II trial of weekly calcitriol and docetaxel in 37 adult men with metastatic prostate cancer



Source: Fromme EK, Eilers KM, Mori M, et al. How accurate is clinician reporting of chemotherapy adverse effects? A comparison with patient-reported symptoms from the Quality-of-Life Questionnaire C30. Journal of Clinical Oncology 2004;22:3485-3490.

Clinician Reports Don't Reflect Patient Reports

Observational study of 438 children (7-18 years), their parent/caregiver, and their clinician. Children received chemotherapy or radiation.



Patient-Reported Outcomes Measurement Information System®

- Assesses a broad range of symptoms and functioning.
- Applicable for a broad range of populations.
 - Variety of diseases and health conditions
 - Children and adults
 - Multiple languages
- Offers flexibility for investigators.
 - Select relevant patient-centered outcomes
 - Select length of measure



Key Points



- Patients inform the evaluation of treatment benefit
- There is guidance on assessing patient-centered outcomes
- Contemporary measures give investigators more freedom in what and how they capture patient-centered outcomes
- Patients and patient advocates can serve many roles, such as: investigators, advisors, and participants
 - *“Nothing about us, without us.”*