Let's extend the conversation on estimands to Early Development

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Disclaimer

I am an employee of Johnson & Johnson and all opinions and information in this presentation are these of the presenter and do not necessarily reflect the views of J&J.

The BBS and Estimands

How it all started …

Joint EFSPI / BBS Webinar: Estimands addendum is final: Anything new for oncology?   **June 2020**

07.09.2020: BBS Webinar RCTs meeting causal inference: principal stratum strategy and beyond   **September 2020**

03.11.2020: Joint EFSPI / BBS Webinar: The application of estimands from a Neuroscience perspective   **November 2020**

… and developed …

Joint EFSPI & BBS virtual event
Addressing intercurrent events: Treatment policy and hypothetical strategies (day 1)   **December 2022**

Joint EFSPI & BBS virtual event
Addressing intercurrent events: Treatment policy and hypothetical strategies (day 2)   **December 2022**

Quantification of risk: ask the right questions or time to apply the estimand framework to safety   **April 2023**

Estimands in Early Development across Therapeutic Areas   **October 2023**

Estimands in ED

Something you *need* but may *not know* that you need (at least not yet!)

Challenges

Specific to ED

ED is fraught with

- High uncertainty

- Small sample sizes

- Very immature data, sometimes even at the decision-making time-points

- Few assumptions to rely on

- Intercurrent events (e.g. discontinuation, intra-patient dose escalation, *etc.*)

- Competing risks (e.g. death, progression, discontinuation)

By *design*, we are left with *adapting* our assumptions as much as we can, most of the times without prior specification.

The question we would like an answer to

What is the recommended Phase 2/3 dose/regimen?

How do we quantify the signs of *early* efficacy and, are they good enough?

And so

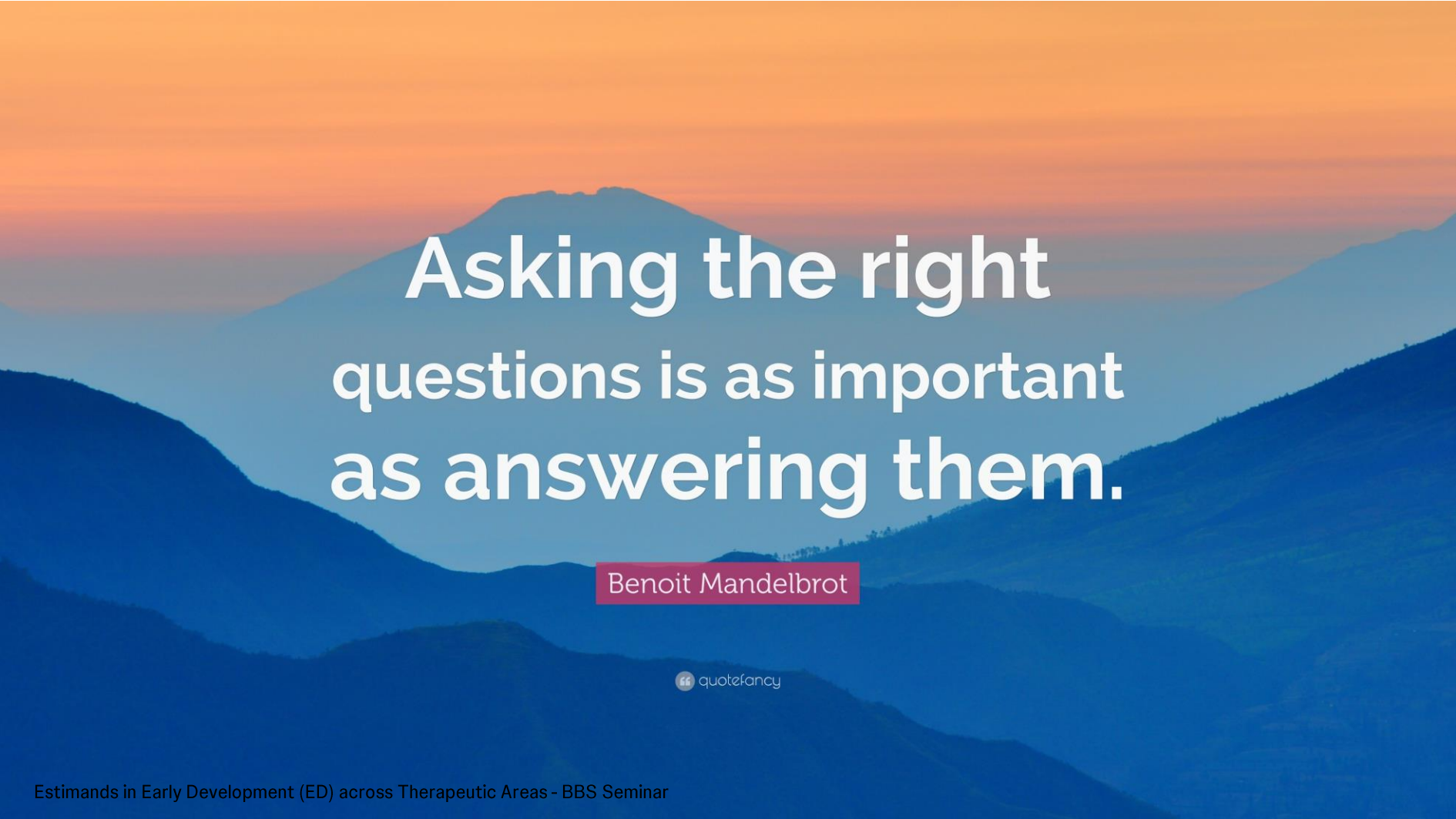Defining the estimand of interest for later stages

These questions involve both

- Safety

- Efficacy

Jointly at times.

Let's ask the questions and engage our partners

Setting the context

Thank you

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