

OCE's Patient-Focused Drug Development and Outreach Efforts

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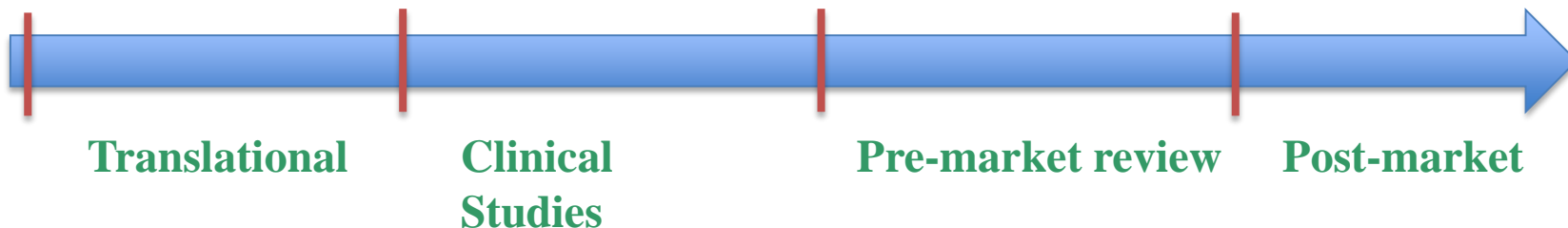
Patient-Focused Drug Development

What matters most to patients and how can it be measured?

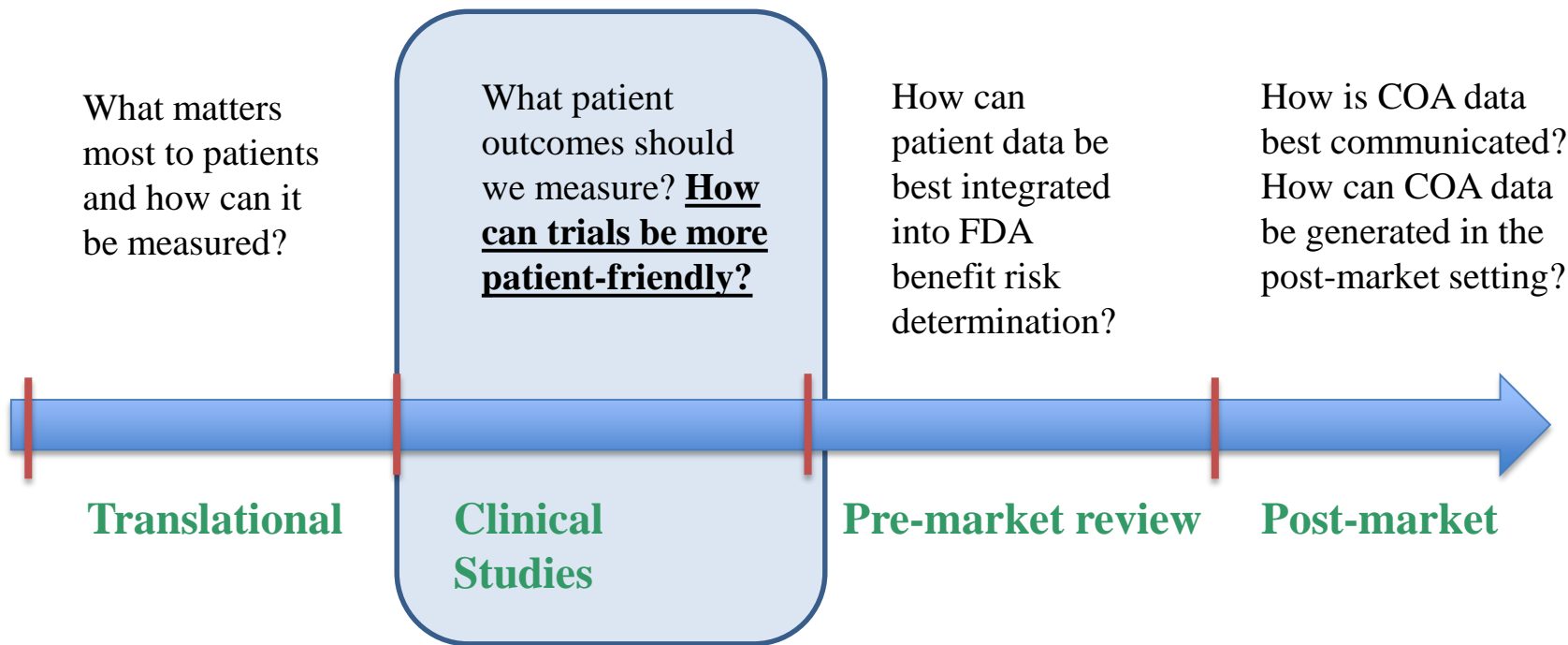
What patient outcomes should we measure? How can trials be more patient-friendly?

How can patient data be best integrated into FDA benefit risk determination?

How is COA data best communicated? How can COA data be generated in the post-market setting?



Patient-Focused Drug Development

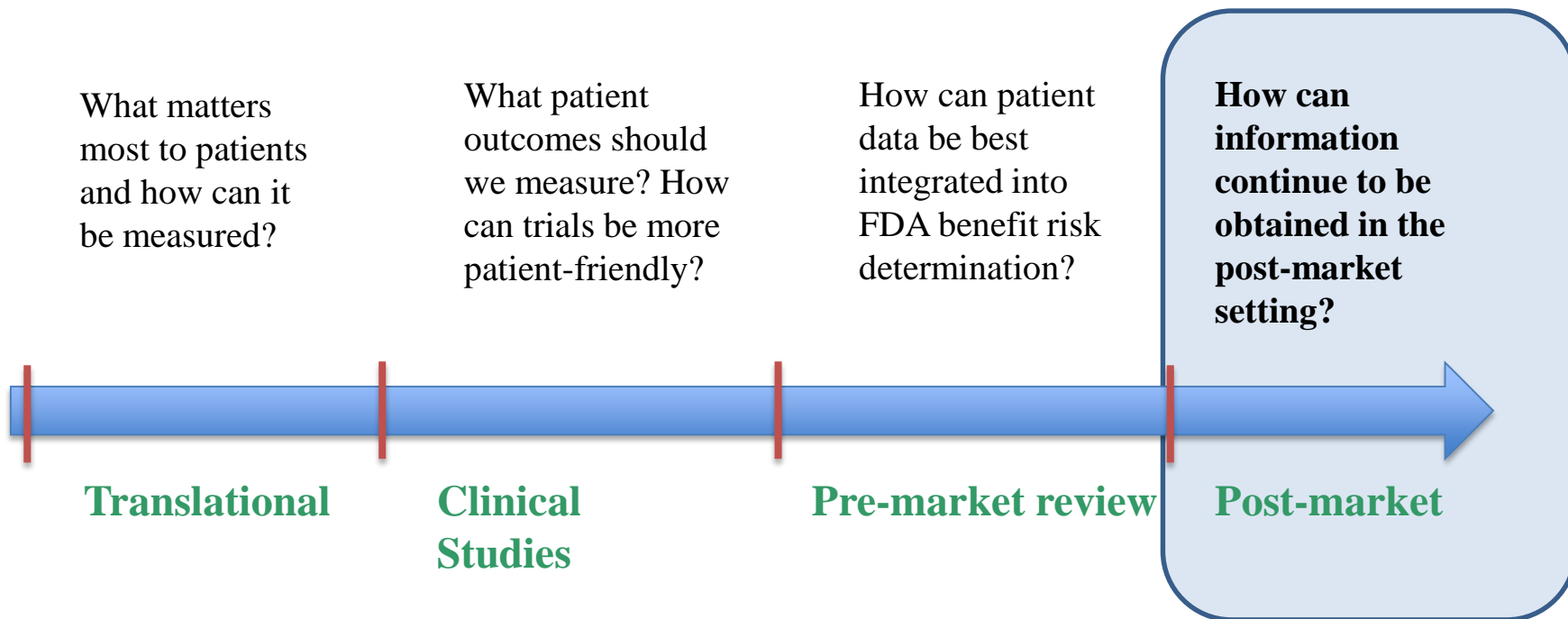


Patient-Focused Drug Development- We are getting there...

How can trials be more patient friendly?

- Broadening eligibility criteria
- Improving trial access and reducing disparities
- Decentralized trials
- Digital health technology tools for remote data acquisition
- Learning healthcare systems and pragmatic / practical trials

Patient-Focused Drug Development



“Real-World Data” What is It?

Real-world *data* are the data relating to patient health status and/or the delivery of health care routinely collected from a variety of sources.

- Electronic health records (EHRs)
- Claims and billing activities
- Product and disease registries
- Patient-generated data including in home-use settings
- Data gathered from other sources that can inform on health status, such as mobile devices

Real World Data Real World Evidence

Real-world *evidence* is the clinical evidence regarding the usage and potential benefits or risks of a medical product derived from analysis of RWD.

- Study Designs
- Analyses
- Randomized trials
- Observational studies (prospective or retrospective)

How Might We Use RWE?

- Understand how patients use different kinds of cancer therapies in the general population
- Learn more about safety of a cancer product in “real patients” once the cancer drug has been approved
- Better understand the natural history of a particular patient population throughout their cancer journey
- Generate information about how cancer drugs may work in different kinds of cancers



Summary



American Society of Hematology
Helping hematologists conquer blood diseases worldwide



We will continue to engage patients, advocacy groups, international regulators, companies and the academic community to improve measurement of symptoms and function in the clinical trial and real-world settings, and to make trials more patient-friendly...



And Many Other Collaborations!