

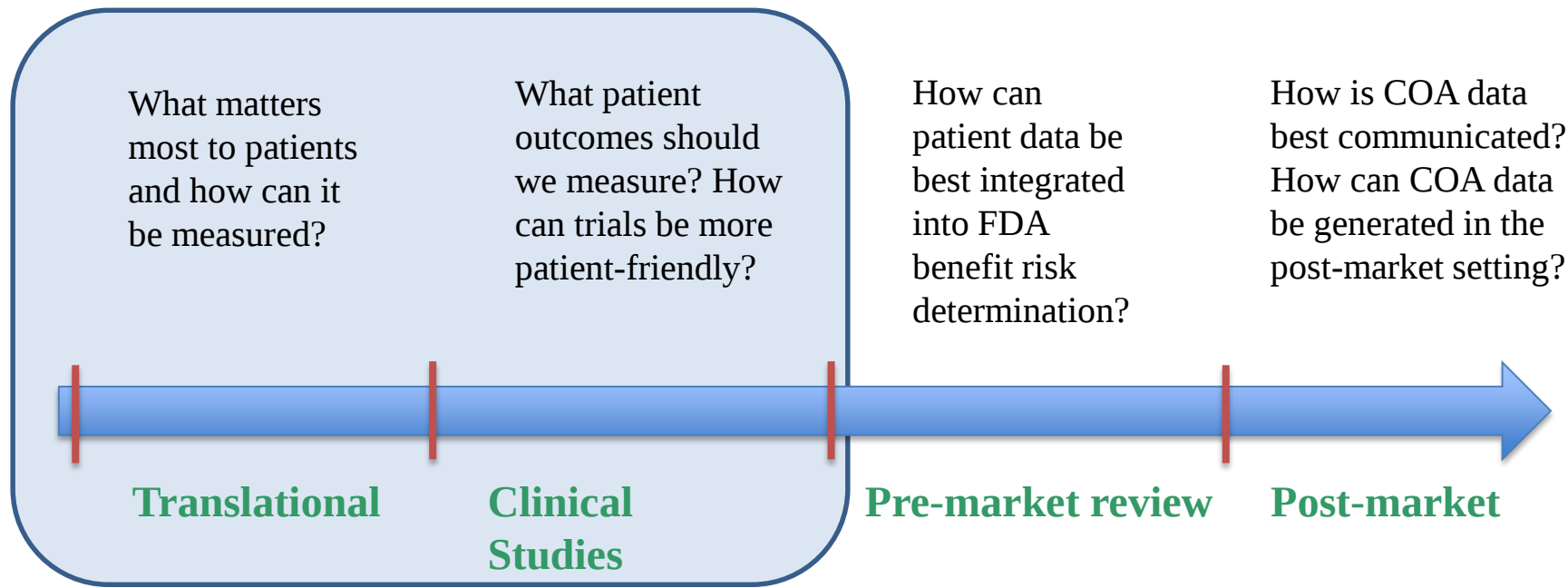
Assessing Physical Function in Cancer Patients

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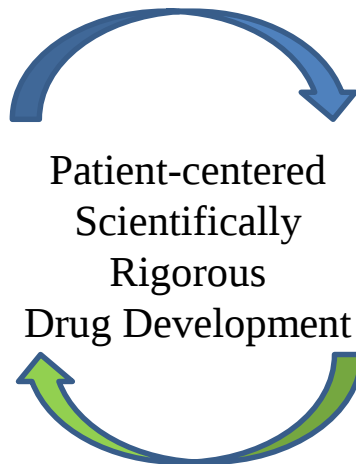
Patient-Focused Drug Development



Successful Patient-Focused Drug Development Must be a **Dialogue**

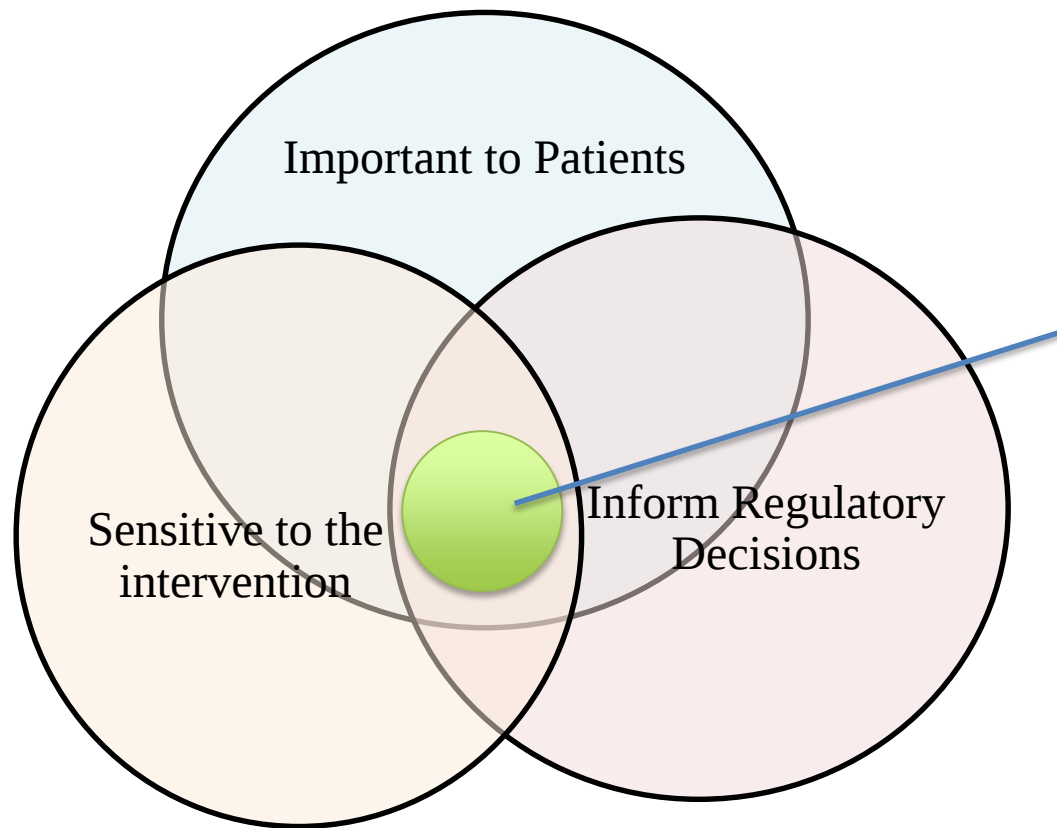
Patients

- Experts in how they experience their disease
- Identify what matters most to patients
- Identify areas to make clinical trials more patient-friendly



Clinicians/ Trialists/ Health Policy Leaders

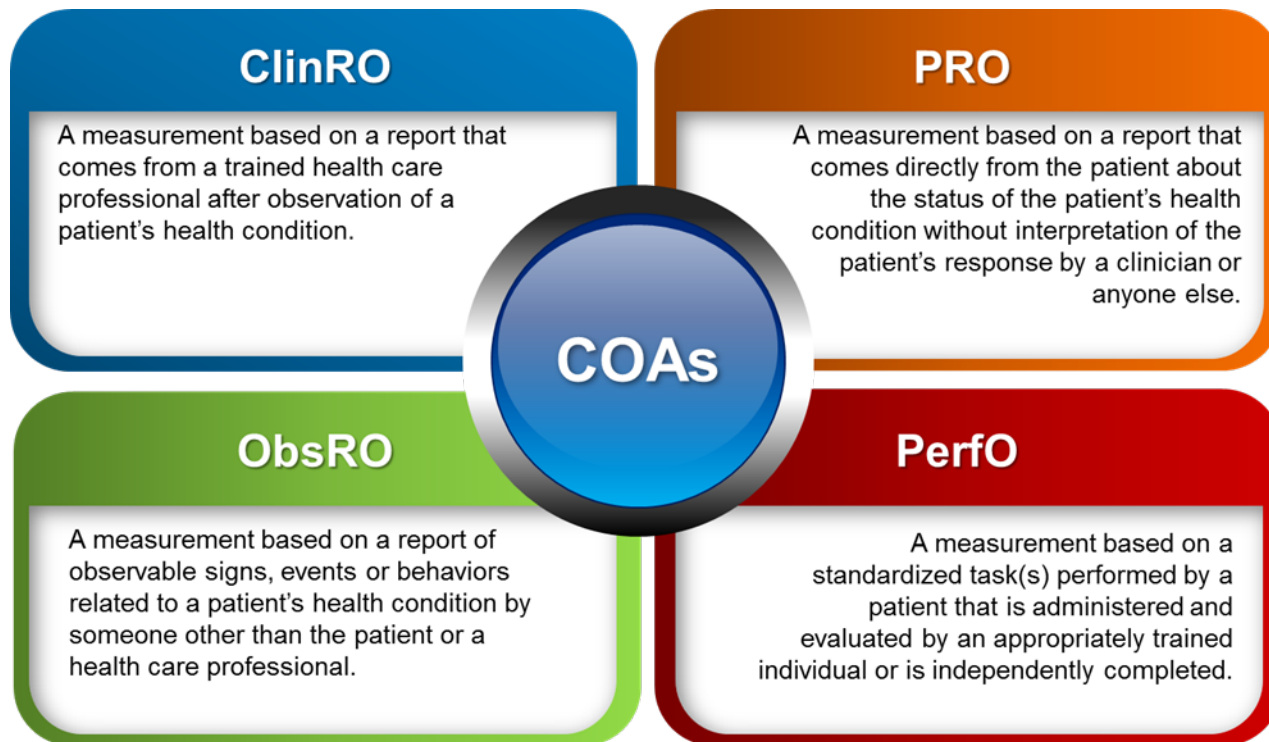
- Experts in clinical trial design and conduct
- Medical expertise
- Assess feasibility of trial modifications and outcome measures



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[Core Clinical Outcomes](#)

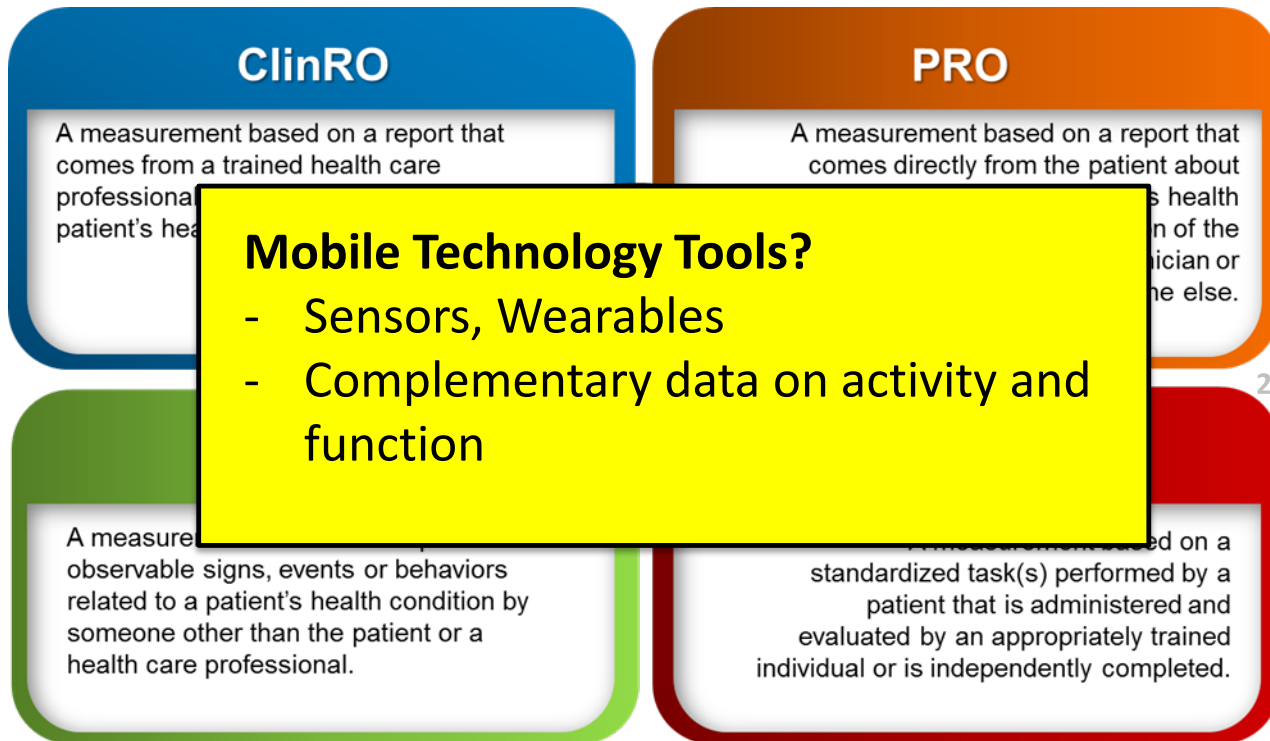
- Disease Symptoms
- Symptomatic Adverse Events
 - Global Side Effect Summary
- **Physical Function and Ability to Perform Activities**

Ways to Measure Physical Function



Clinical Outcome Assessments (COAs)

COA: Assessment of a clinical outcome made through report by a clinician, a patient, a non-clinician observer or through a performance-based assessment



OCE is Interested in Exploring Physical Function in Cancer Populations

- **Broad Agency Announcement- OCE research funding**
 - **Measurement focus-** Wearable device, PRO, Clinician
 - **Setting-** academic center / like a clinical trial
 - **What we hope to learn:** how wearable devices work compared to PRO and clinician assessment

Questions:

- Measurement characteristics (sensitivity, reliability)
- What is a meaningful difference in function?
- How might function relate to toxicity? Disease progression?

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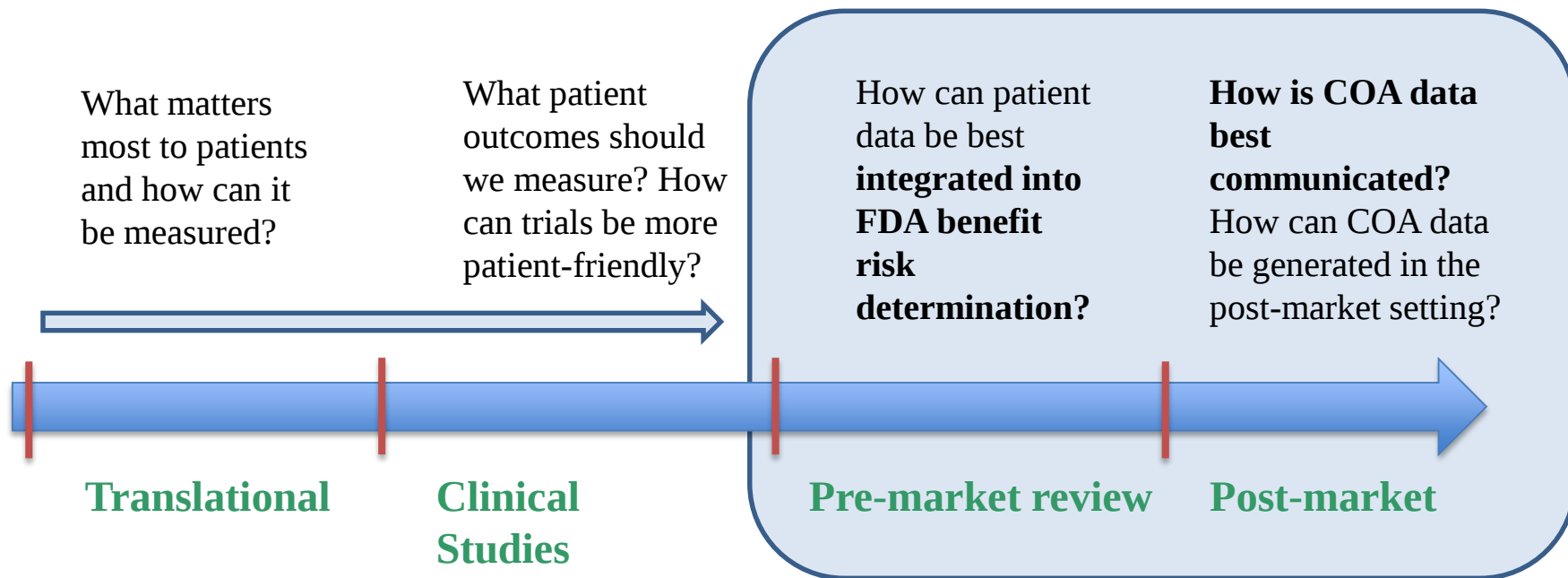
- **Research Collaboration- Kaiser Permanente**

- **Measurement focus-** PRO physical function
- **Setting-** “Real World” clinic
- **What we hope to learn:** Natural history of patient-reported physical function in newly diagnosed advanced pancreatic cancer patients

Questions:

- What are measurement characteristics of PROMIS physical function PRO measures?
- How does physical function change over time and through treatment?
- How is physical function associated with treatment? hospitalization? and cancer progression?

Patient-Focused Drug Development



How can physical function inform regulatory review and patient choice?

How is a patient's physical function affected while they are benefitting from a treatment?

Can patients work and perform their usual activities while the treatment is controlling their disease?

Which patient group functioned better at one year in a clinical trial?

Summary

- Physical function and ability to care of oneself is important to cancer patients and can be measured in multiple ways
- Physical function and ability to work and perform leisure activities are a focus of FDA's work with PRO and wearable devices to improve measurement of the "patient experience"
- FDA Oncology is developing common physical function outcomes to answer questions that can complement our risk:benefit assessments

Key References:

1. FDA-NIH Biomarkers, Endpoints, and other Tools Resource
<https://www.ncbi.nlm.nih.gov/books/NBK338448/>
2. FDA Guidance for Industry- PRO Measures
<https://www.fda.gov/downloads/drugs/guidances/ucm193282.pdf>
3. Kluetz, P.G., et al., Informing the Tolerability of Cancer Treatments Using Patient-Reported Outcome Measures: Summary of an FDA and Critical Path Institute Workshop. *Value in Health*, 2017.
<https://www.sciencedirect.com/science/article/pii/S1098301517335209>
4. Kluetz, P.G., et al., Focusing on Core Patient-Reported Outcomes in Cancer Clinical Trials: Symptomatic Adverse Events, Physical Function, and Disease-Related Symptoms. *Clin Cancer Res*, 2016. **22**(7): p. 1553-8. <https://clincancerres.aacrjournals.org/content/22/7/1553.short>
5. Kim, J., et al. Use of PRO measures to inform tolerability in oncology trials: implications for clinical review, IND safety reporting, and clinical site inspections. *Clinical Cancer Research*, 2018. **24**(8), 1780-1784. <https://clincancerres.aacrjournals.org/content/24/8/1780.abstract>

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