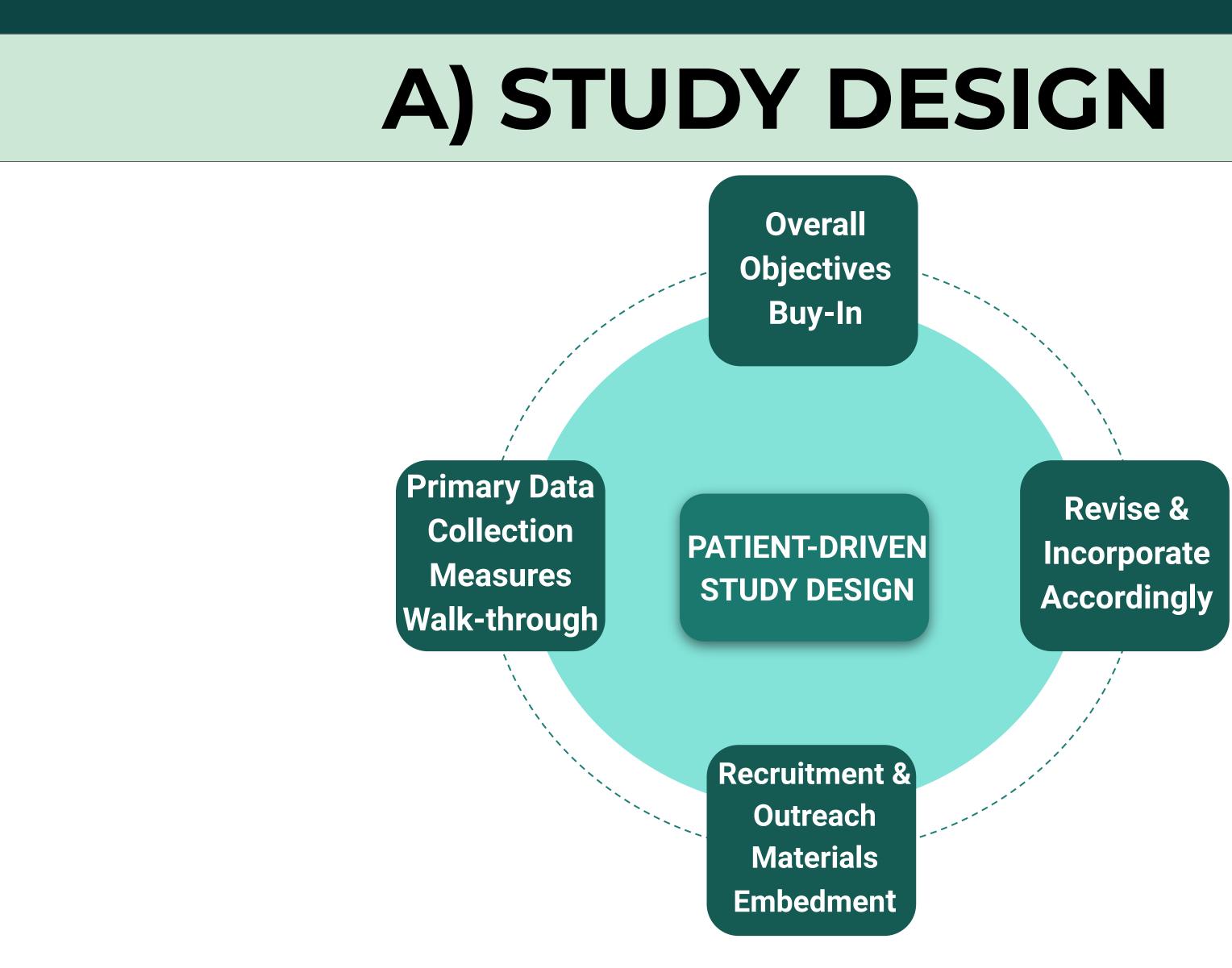
Patient-Driven Real World Data Outside of the Clinic: Demystifying the Capture of Home-Reported Outcomes in Rare Diseases $\sqrt{2}f$

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OBJECTIVES

The inaugural home-reported outcomes (HROs) study in a rare disease population must be focused on achieving important objectives: 1) Develop an Outcome Inventory of outcomes-of-interest to that population, including less-understood symptoms & behaviors 2) Understand the value of home-tracking and ideal features for the population in order to better design and implement future studies; and 3) Describe the relationship between home-reported vs. traditional outcomes in this population (ie. relationship between home-tracked treatments and claims-reported prescriptions).



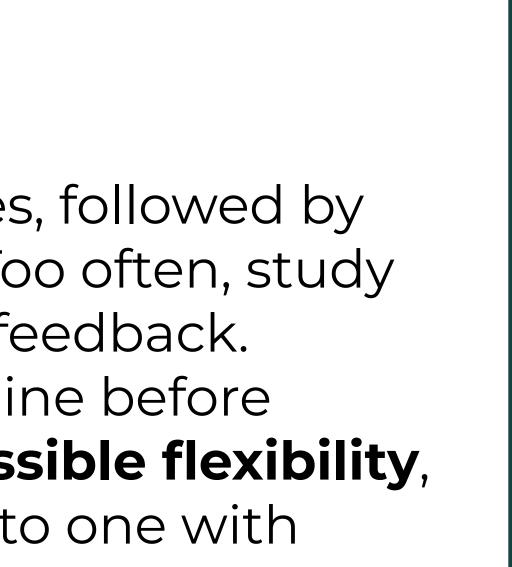
Study Design must begin with assessing study objectives, followed by the **immediate inclusion of participant perspectives**. Too often, study design is nearly complete prior to soliciting community feedback. Starting with a simpler draft of objectives and study outline before engaging with the community enables the greatest possible flexibility, resulting in a truly patient-driven approach, as opposed to one with patients included as an addendum.

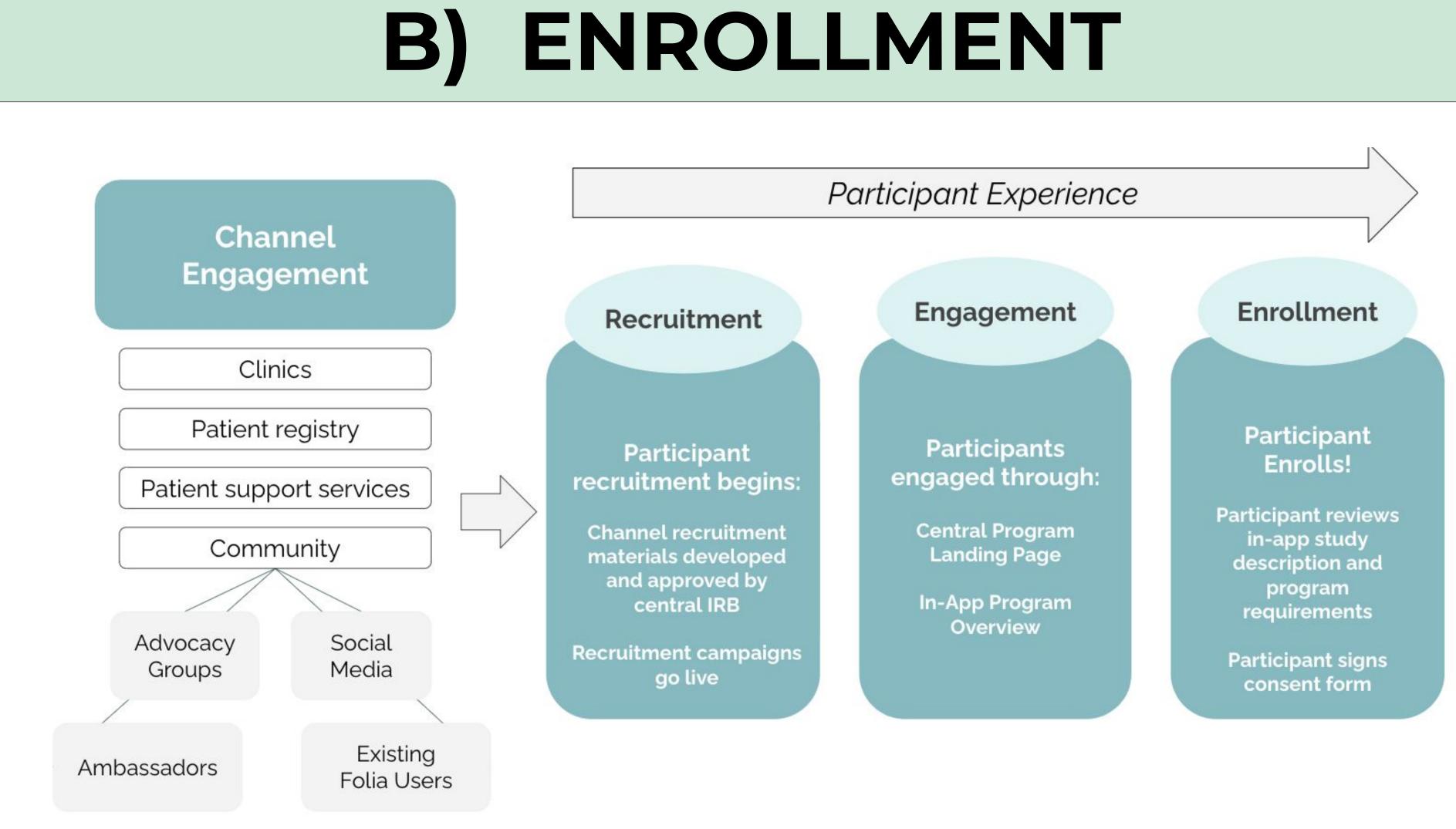
Observational studies can benefit from following patient-centric best practices, to ensure that researchers and participants are deriving full value.

METHODS

In launching HRO studies in multiple diseases, we have identified a set of best-practices for consideration by research teams: A) Study Design

- B) Enrollment
- C) Onboarding





- To dramatically reduce time-to-launch, it is important to utilize a central IRB and obtain waivers from clinical site IRBs.
- There is strong utility in **hybrid recruitment methods,** inclusive of community channels and traditional methods.
- It is possible for participants to complete **digital enrollment** without clinician walkthrough.

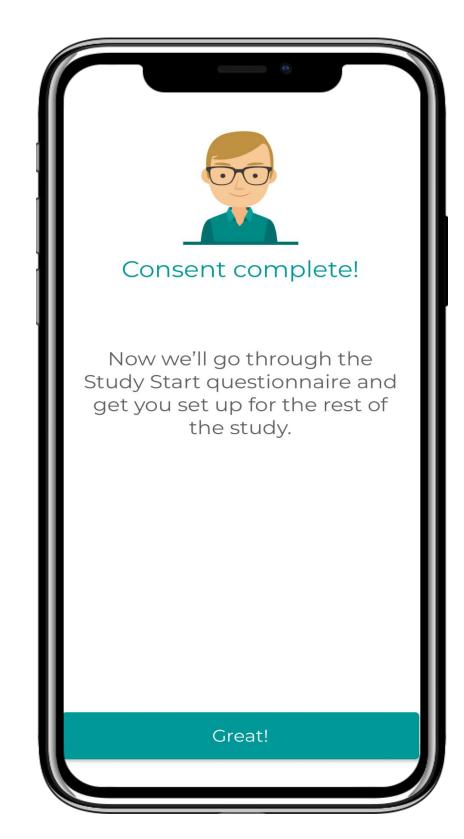
CONCLUSION





Patient Sharing: A simple PDF comprised of the participant's tracking history can be shared with their care team

Successful onboarding includes a well-oiled platform that guides participants through the first phases of the study; live support staffed by research professionals; and frequent positive reinforcement points, during the first stages of the study to ensure success.



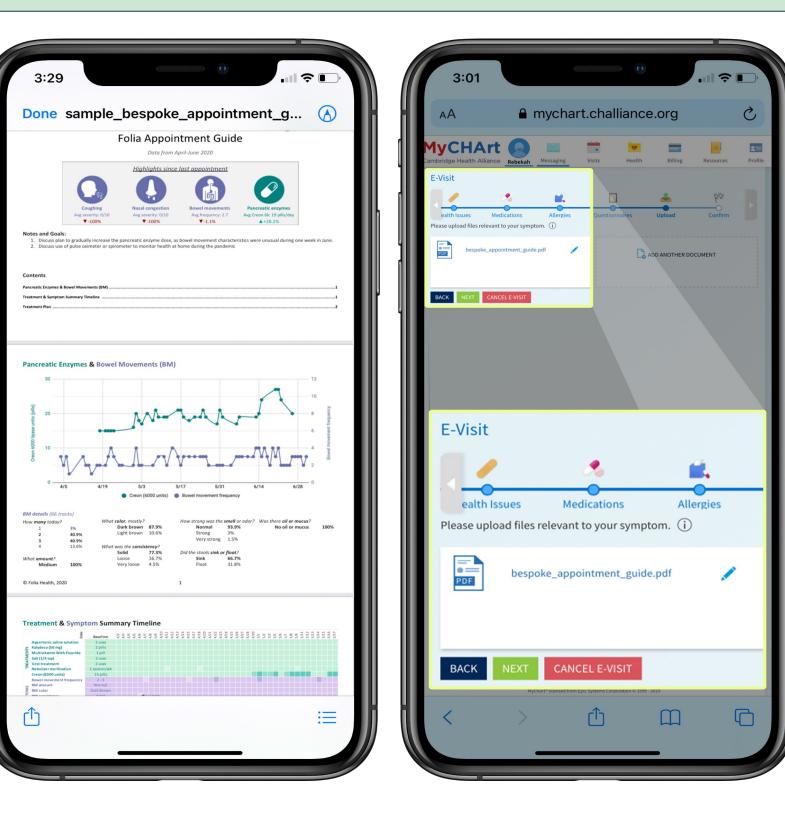
Phase 1: Consent & Study Star

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THE VALUE OF HOME TRACKING



C) ONBOARDING

Onboarding participants is a key part of study launch, requiring significant care and resourcing. In our experience, onboarding successfully will drive retention and long-term engagement.

