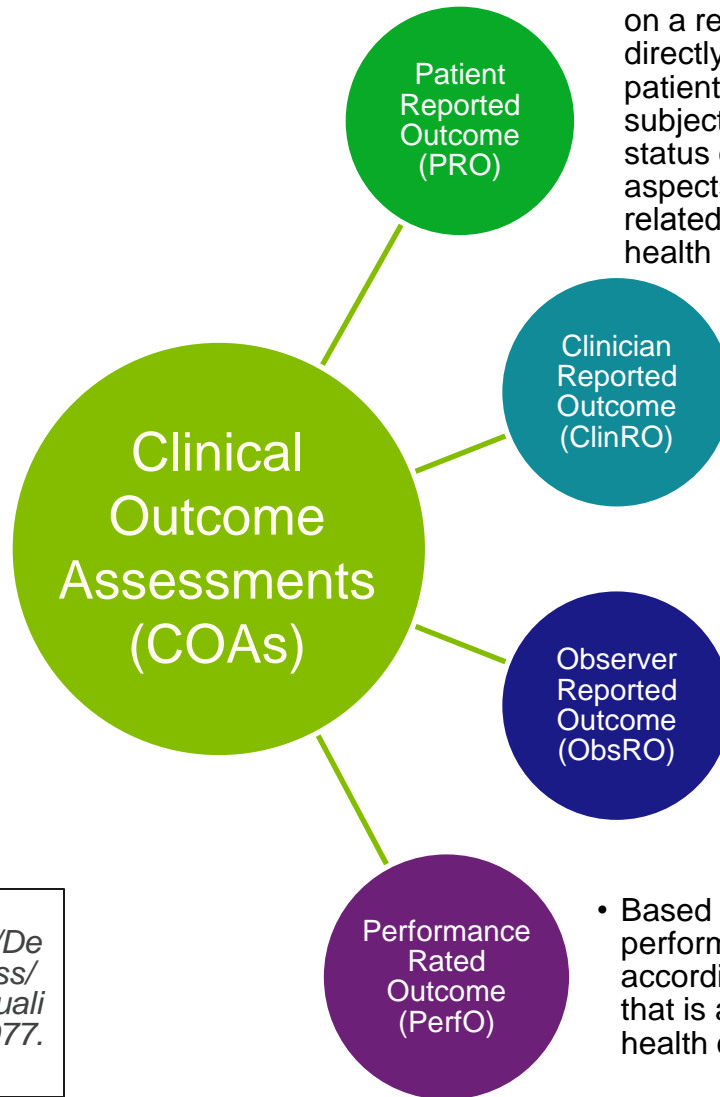


Desmoid Tumor Research Foundation PRO Development Study

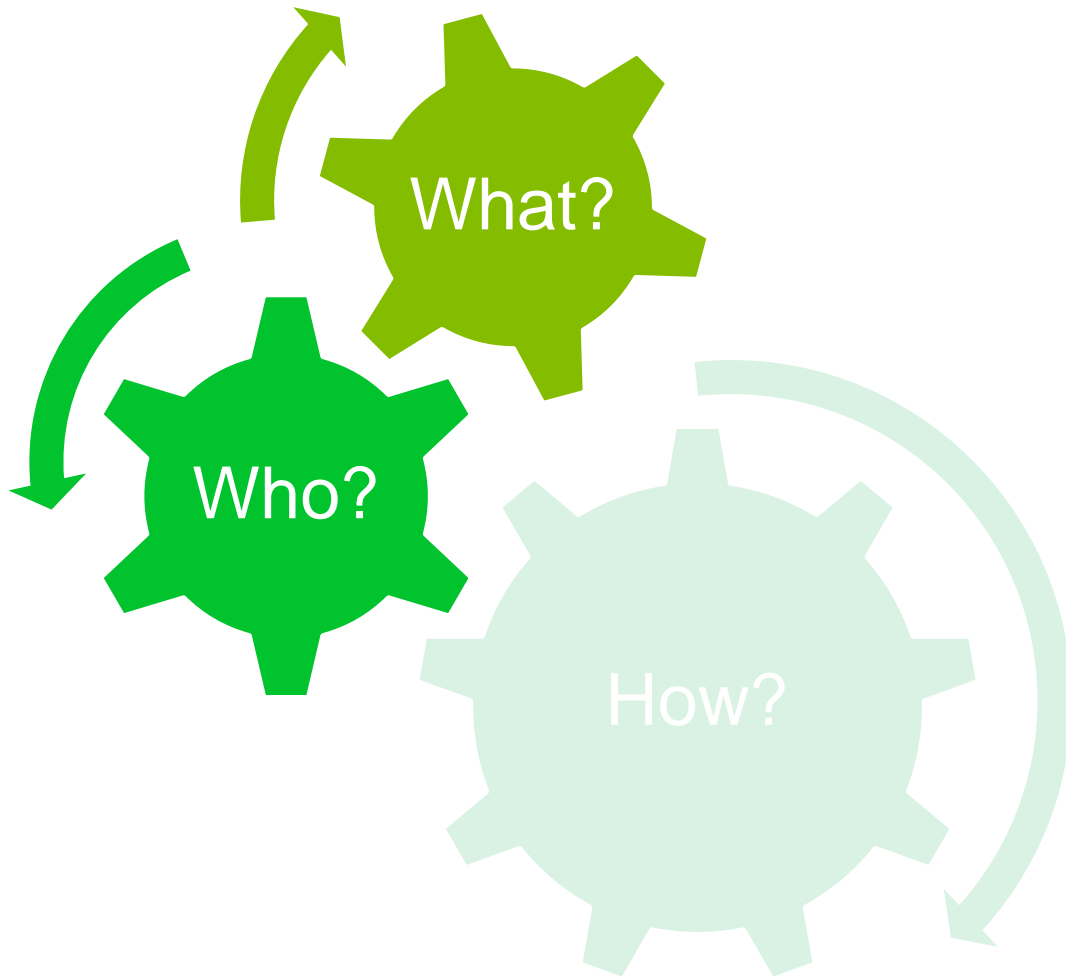
Clinical Outcome Assessments (COA)



- A measurement based on a report that comes directly from the patient (i.e., study subject) about the status of particular aspects of or events related to a patient's health condition.
- An assessment that is determined by an observer with some recognized professional training that is relevant to the measurement being made.
- An assessment that is determined by an observer who does not have a background of professional training that is relevant to the measurement being made, i.e., a non-clinician observer such as a teacher or caregiver.
- Based on a task(s) performed by a patient according to instructions that is administered by a health care professional.

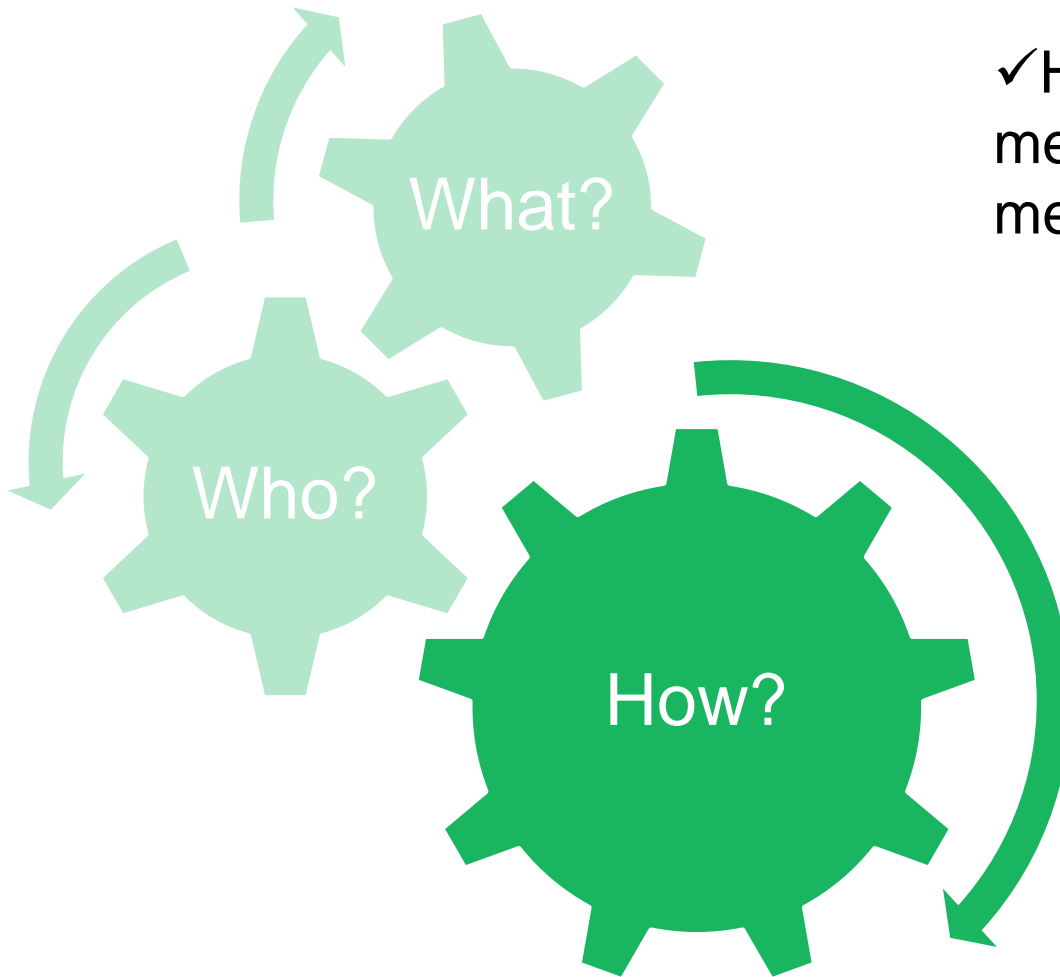
Taken from FDA website:
<http://www.fda.gov/Drugs/DevelopmentApprovalProcess/DrugDevelopmentToolsQualificationProgram/ucm284077.htm>

What, Who, How



- ✓ What do you want to say: target claims/objectives & concepts
 - ✓ Target claims/promotional claims/publication goals:
 - ✓ Identify the concepts, or what you want to measure, that will support your messages
- ✓ What to measure depends upon:
 - ✓ Who is in your target patient population
 - ✓ Who you want to influence with your results – regulators, payers, clinicians, and/or patients themselves

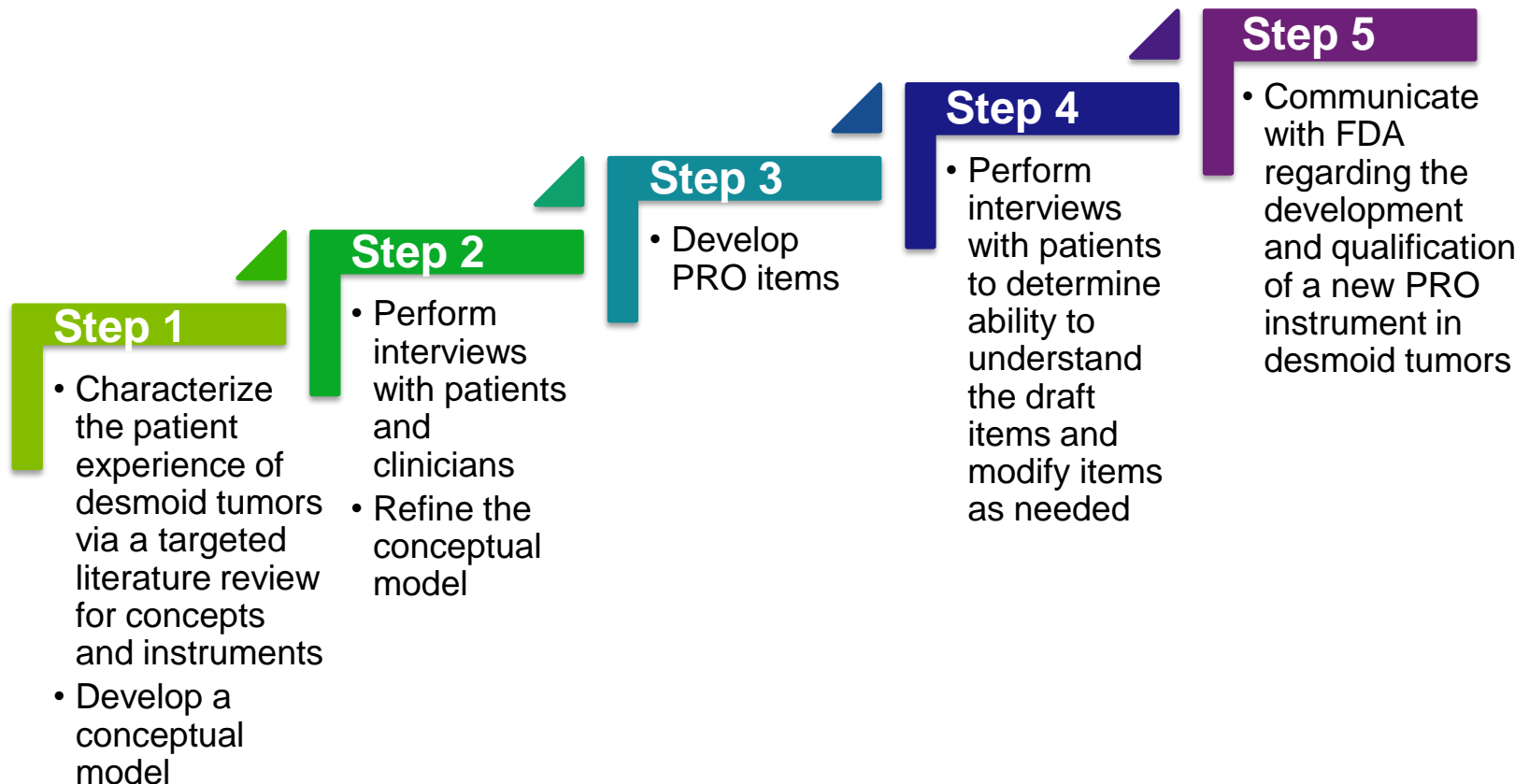
What, Who, How



- ✓ How do we capture that message: instruments, measurement strategy
 - ✓ A means to capture data (i.e., a questionnaire) plus all the information that supports its use
 - ✓ What instruments can be used to measure the 'what', and what is the risk of use?

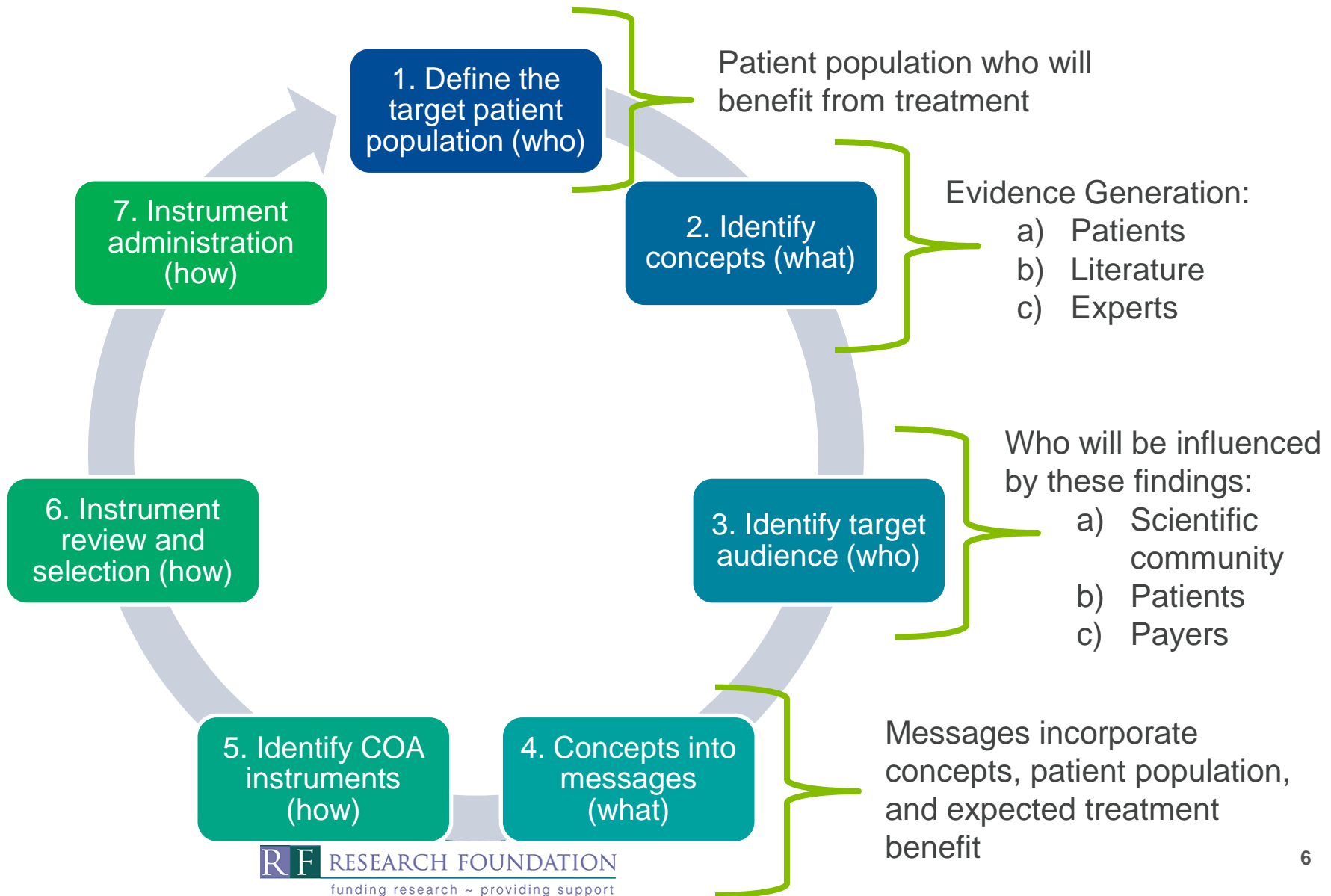
Overview of Approach

- Portray the patient experience of living with desmoid tumors via qualitative evidence and patient reported outcome (PRO) item generation
- Achieve objectives by following steps below



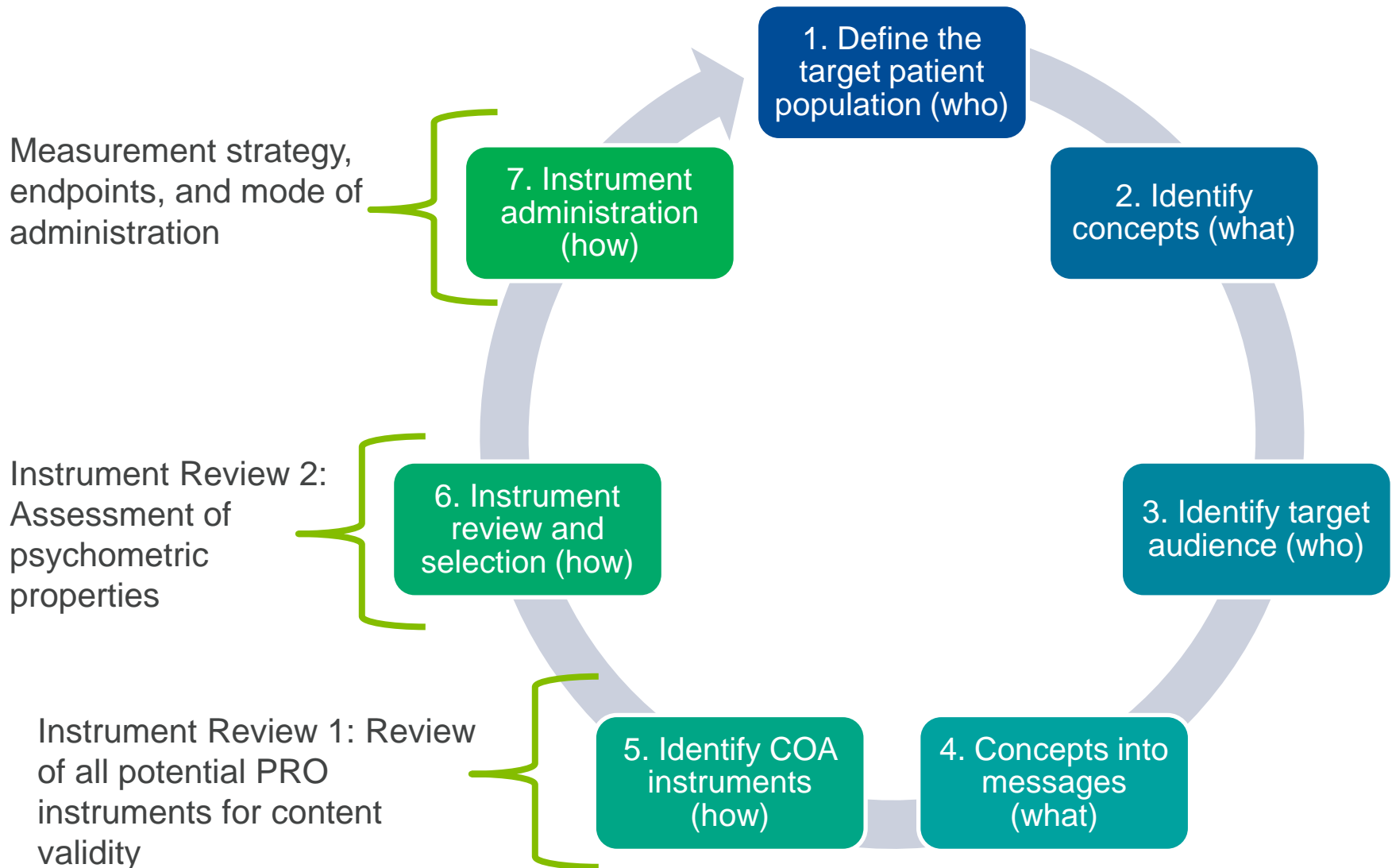
COA Instrument Selection

Approach to COA instrument selection



COA Instrument Selection

Approach to COA instrument selection



The Patient's Voice Counts



Patient insights can help develop new ways of studying conditions such as desmoid tumors

- To help develop new ways to treat patients, DTRF wants to better understand what patients go through every day in living with desmoid tumors

The patient's voice counts

- Patient voice is important, by having a conversation about personal experience of living with desmoid tumors, patients can support new research



Researchers are here to listen

- The interviews are an opportunity for patients to describe what they experience and go through

The Patient Interview Process

Permission

- Before we talk with patients we gather basic health information
- We also ask permission to talk with them (informed consent)

Location and Method

- A research phone call will be about an hour long
- Patients should be in a quiet place where they feel comfortable sharing information
- The researcher takes notes during the call
- The conversation is recorded so the researcher can listen and make sure we did not miss anything

Topics Covered

- Experience of desmoid tumors in general
- Symptoms experienced
- How symptoms change or impact patient's life
- How desmoid tumors impact family, friends, and work life
- What patients think a treatment should be able to do

Reporting and Confidentiality

- Report on how patients feel with desmoid tumors
- Help other people learn about desmoid tumors
- Raise awareness of desmoid tumors

- Patient information is confidential, researchers will not share it with anyone and it will stay anonymous