Desmoid Tumor Research Foundation PRO Development Study
Clinical Outcome Assessments (COA)

- Patient Reported Outcome (PRO)
  - 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.

- Clinician Reported Outcome (ClinRO)
  - An assessment that is determined by an observer with some recognized professional training that is relevant to the measurement being made.

- Observer Reported Outcome (ObsRO)
  - 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.

- Performance Rated Outcome (PerfO)
  - Based on a task(s) performed by a patient according to instructions that is administered by a health care professional.

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

Step 1
• Characterize the patient experience of desmoid tumors via a targeted literature review for concepts and instruments
• Develop a conceptual model

Step 2
• Perform interviews with patients and clinicians
• Refine the conceptual model

Step 3
• Develop PRO items

Step 4
• Perform interviews with patients to determine ability to understand the draft items and modify items as needed

Step 5
• Communicate with FDA regarding the development and qualification of a new PRO instrument in desmoid tumors
COA Instrument Selection

Approach to COA instrument selection

1. Define the target patient population (who)

2. Identify concepts (what)

3. Identify target audience (who)

4. Concepts into messages (what)

5. Identify COA instruments (how)

6. Instrument review and selection (how)

7. Instrument administration (how)

Evidence Generation:
- a) Patients
- b) Literature
- c) Experts

Who will be influenced by these findings:
- a) Scientific community
- b) Patients
- c) Payers

Messages incorporate concepts, patient population, and expected treatment benefit.
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Instrument Review 1: Review of all potential PRO instruments for content validity

Instrument Review 2: Assessment of psychometric properties

Measurement strategy, endpoints, and mode of administration
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

<table>
<thead>
<tr>
<th>Permission</th>
<th>Location and Method</th>
<th>Topics Covered</th>
<th>Reporting and Confidentiality</th>
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</thead>
<tbody>
<tr>
<td>• Before we talk with patients we gather basic health information</td>
<td>• A research phone call will be about an hour long</td>
<td>• Experience of desmoid tumors in general</td>
<td>• Report on how patients feel with desmoid tumors</td>
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<tr>
<td>• We also ask permission to talk with them (informed consent)</td>
<td>• Patients should be in a quiet place where they feel comfortable sharing information</td>
<td>• Symptoms experienced</td>
<td>• Help other people learn about desmoid tumors</td>
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<td>• The researcher takes notes during the call</td>
<td>• How symptoms change or impact patient’s life</td>
<td>• Raise awareness of desmoid tumors</td>
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<td></td>
<td>• The conversation is recorded so the researcher can listen and make sure we did not miss anything</td>
<td>• How desmoid tumors impact family, friends, and work life</td>
<td>• Patient information is confidential, researchers will not share it with anyone and it will stay anonymous</td>
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<td>• What patients think a treatment should be able to do</td>
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