U.S. FOOD & DRUG ADMINISTRATION


# Public Meeting on Center for Drug Evaluation and Research Standard Core Sets: Clinical Outcome Assessments and Endpoints Grant Program

Friday, August 28, 2020

# Welcome and Introductory Remarks

# Disclaimer

The views expressed in the following presentations are those of the individual speakers and do not necessarily represent an official FDA position.

# Agenda

| | |
|---|---|
| 08:30 a.m. | Welcome and Introductory Remarks |
| 08:35 a.m. | Update on CDER Standard Core Sets: Clinical Outcome Assessments (COAs) and Endpoints Pilot Grant Program |
| 08:45 a.m. | Impacts and Shared Lessons of COVID-19<br>Audience Question and Answer |
| 10:00 a.m. | Migraine Clinical Outcome Assessment System (MiCOAS) Grant<br>Audience Question and Answer |
| 10:30 a.m. | Break |
| 10:45 a.m. | Northwestern University Clinical Outcome Assessment Team (NUCOAT) Grant<br>Audience Question and Answer |
| 11:15 a.m. | Clinical Outcome Assessments for Acute Pain Therapeutics in Infants and Young Children (COA-APTIC) Grant<br>Audience Question and Answer |
| 11:45 a.m. | CDER Standard Core Sets: Clinical Outcome Assessments and Endpoints Pilot Grant Program New Funding Opportunity<br>Audience Question and Answer |
| 12:20 p.m. | Closing Remarks |

# CDER Pilot Grant Program: Standard Core Clinical Outcome Assessments (COAs) and their Related Endpoints


# Questions?

**To ask a question, click on the "dialogue" icon in the lower right corner of the viewer.**

# Send us your comments!

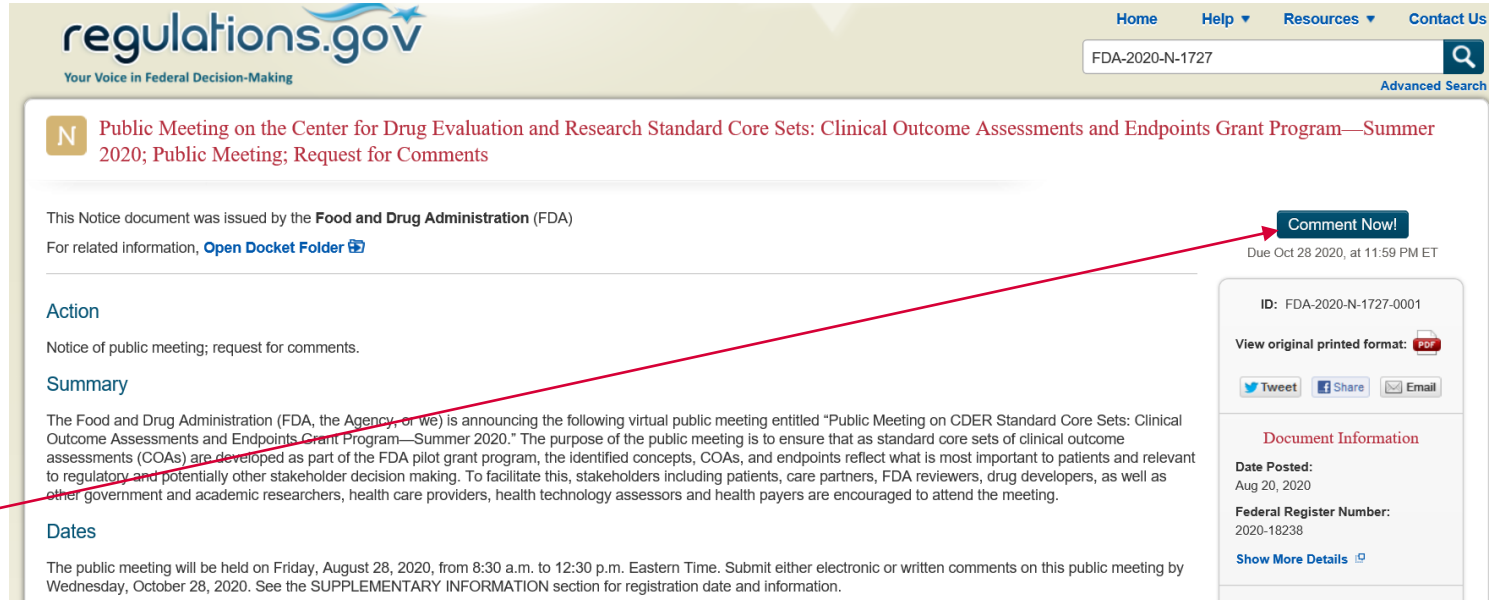
**You can also send us comments through the "public docket"**

- The docket will be open until October 28, 2020!

Visit: https://www.regulations.gov/document?D=FDA-2020-N-1727-0001

Or Search for our Docket # "2020-N-1727" on www.regulations.gov

And **Click Comment Now!**



# Updates on CDER Standard Core Sets: COAs and Endpoints Pilot Grant Program

**Robyn Bent, RN, MS**
Office of the Center Director
Center for Drug Evaluation and Research

# Grant Program Purpose

Aims to help make incorporating patient perspective more sustainable

Enable the development of publicly available standard core sets of measures of disease burden and treatment burden for a given area

Provide avenues to advance the use of patient input as an important part of drug development

# Awarded Grants

On September 11, 2019 the FDA made the following three awards:

**Migraine** Clinical Outcome Assessment System (MiCOAS)

Clinical Outcome Assessments for **Acute Pain** Therapeutics in Infants and Young Children

(COA APTIC)

Northwestern University Clinical Outcome Assessment Team (NUCOAT) – **Physical Function**

# UG3/UH3 Cooperative Agreement

The UG3/UH3 Phase Innovation Award Cooperative Agreement involves 2 phases:

- Milestone-driven planning phase **(UG3)** provides funding for 1 to 2 years to conduct planning activities.
- Implementation phase **(UH3)** provides funding for 3 to 4 years to projects that successfully complete the planning activities and reach the projected milestones set in the UG3 phase.

# Collaborative Effort

- Monthly Meetings with FDA Staff
- Teams made up of CDER Staff from
  - Review Divisions
  - Office of Biostatistics
  - Division of Clinical Outcome Assessments
  - Patient Focused Drug Development
- Other Centers

# Stakeholder Engagement

- **Public Meetings**
  - Twice yearly public meetings with an opportunity for stakeholders to ask questions and provide feedback both as part of the meetings or by submitting feedback to the public docket
- **External Technical Advisory Committee**
  - Made up of disease specific experts, COA experts, biostatisticians, patient experts, and other technical experts as appropriate who oversee and monitor the specific projects
- **Scientific Policy Board**
  - To bring a global perspective to the Standard Core COA development process

# PFDD Vision

1. **Ensure confidence** in reliability and accuracy of **methodologically sound** patient experience data for regulatory decision making
2. Promote rapid **consistent adoption**
3. **Increase predictability** for sponsors
4. Sustain incorporation of patient's experience in drug development and decision making—**make it standard practice**

https://www.fda.gov/drugs/development-approval-process-drugs/cder-patient-focused-drug-development

# Impacts and Shared Lessons of COVID-19

**Panel Discussion**

# Audience Q&A

# CDER Pilot Grant Program: Standard Core Clinical Outcome Assessments (COAs) and their Related Endpoints


# Questions?

**To ask a question, click on the "dialogue" icon in the lower right corner of the viewer.**

# Migraine Clinical Outcome Assessment System

Richard B. Lipton, MD

Albert Einstein College of Medicine

R. J. Wirth, PhD

Vector Psychometric Group, LLC

12% of the
Global Population

Pain
Nausea
Vomiting
Photophobia
Phonophobia
Aura

Pain
Nausea
Vomiting
Photophobia
Phonophobia
Aura

Is this all that is important to people living with migraine?

# MiCOAS Project: Phase 1

Develop endpoints and their measures that accurately reflect patients' experiences

Develop these endpoints and measures using patient input/collaboration and gold-standard psychometric methods.

# MiCOAS Project: Phase 1

- Aim 1:
  - Build a team of advisors
  - Develop initial list of endpoints
- Aim 2:
  - Conduct systematic literature reviews
  - Refine endpoint list
- Aim 3:
  - Talk to people with migraine
  - Make recommendations for new outcome(s)/endpoint(s)

# External Technical Advisory Committee

- **Robyn Bent, MS**: *Director, CDER PFDD Program, FDA*
- **Nicki Bush, MHS**: *Eli Lilly and Company, Director and Global Head of Patient-focused Outcomes Center of Expertise*
- **Roger Cady, MD**: *Lundbeck Pharmaceutical, Vice President, Neurology*
- **David Dodick, MD**: *Mayo Clinic College of Medicine & Science, Professor; Director of the Headache Program, Department of Neurology, Mayo Clinic*
- **Peter Goadsby, MD, PhD, DSc**: *UCSF and King's College London, Professor of Neurology; NIHR-Welcome Trust Clinical Research Facility, Director*
- **Katie Golden**: *Patient Advocate, Director of Patient Relations & Steering Committee Member for CHAMP*
- **Kelly McCarrier, PhD, MPH**: *Pharmerit International, Director and Qualitative Research Lead, Patient-Centered Outcomes Center of Excellence*
- **Buzz Stewart, PhD, MPH**: *Managing Member, HINT Consulting*; *John Hopkins Bloomberg School of Public Health, Adjunct Professor*

# CHAMP

## Coalition For Headache And Migraine Patients

Provide support to people with headache, migraine, and cluster diseases

Bring together stakeholders to more effectively help people

Identify unmet needs of those with headache, migraine, and cluster diseases and work to better support people with headache and migraine and their caregivers

# Literature Reviews

- Acute and Preventive Literature Review reports completed
  - Acute Review - 705 articles, in depth analysis of 451 publications
  - Preventive Review - 757 articles, in depth analysis of 268 publications
  - Both reviews found variability across publications in the outcomes used and a lack of standardization in the definitions of outcomes and endpoints

- Both reports will be submitted as manuscripts to *Headache* in September 2020

# Qualitative Study Update

- Initial recruitment has resulted in a participant pool of over 400 people with migraine who are eligible and willing to participate in the qualitative interviews

- Conducted initial set of interviews (n=4) and one group practice interview with patient advocates (n=2)

- Paused to assess:
  - Interview guide function
  - Participants' ability to differentiate between pre- and post-COVID-19 migraine experience
  - How COVID-19 has impacted participants' migraine
  - Implications COVID-19 has on our ability to collect usable data

# Key Points from First Wave Interviews (n = 4)

- Variation in impact of COVID-19 on participants' migraine experience
  - Two participants did not cite any notable changes in their migraine experience due to COVID-19
  - When changes occurred, included:
    - Increases in migraine frequency and severity attributed to triggers like stress, mask-wearing, and less opportunity to engage in preventive behaviors
    - Short-lived disruptions to in-office treatments like Botox
    - Fewer concerns about engaging in activities outside the home during migraine attacks
  - No participants cited fundamental changes in their symptom profile, disease impacts, or treatment priorities

# Key Points from First Wave Interviews (n = 4)

- Participants were able to characterize minor changes in their treatment priorities due to COVID-19

- Participants “usual” or “typical” experience with migraine was very much at the forefront of their mind, despite any changes they have experienced due to recent events
  - Conveyed by vivid migraine attack descriptions and the ability to clearly articulate shifts in their experience over time and from phase to phase of their attacks.

- Second wave of interviews (n=36) is in progress

# MiCOAS Project: Phase 2

- Aim 4:
  - Talk to people with migraines to determine how we can best capture the recommended outcomes/endpoints in a way that makes sense to patients
- Aim 5:
  - Conduct two rounds of data collection (acute & preventive) to study the psychometric quality of the new measure(s) using gold-standard methods
- Aim 6:
  - Disseminate, disseminate, disseminate

Thank You.

# Audience Q&A

# CDER Pilot Grant Program: Standard Core Clinical Outcome Assessments (COAs) and their Related Endpoints


# Questions?

**To ask a question, click on the "dialogue" icon in the lower right corner of the viewer.**

# Break

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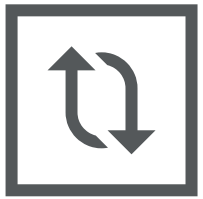
# Northwestern University Clinical Outcome Assessment Team (NUCOAT) Grant Program

*Public Meeting on Core Outcome Sets*

August 28th, 2020

# NUCOAT UG3 Aims

| | | *Status* |
|---|---|---|
| Aim 1 | To convene stakeholders, including patients, care partners, clinicians, measurement experts, payers, regulators, and industry representatives, around the topic of **physical function (PF)** as it relates to approval of new drugs. | ✓ |
| Aim 2 | To propose six (6) model conditions (3 sarcopenia; 3 rare disorders) in which to test measures of PF, covering a range of type and severity of limitation, and identify gaps, if any, in our proposed PF measures. |  |
| Aim 3 | To propose interim plans and final plans for refining and testing PF PROs based on the PROMIS PF bank v2.0, and PF PerfOs based on the NIH Toolbox and the Short Physical Performance Battery. | |

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✓: Completed Aim

 : In Progress Aim

# UG3 Candidate Conditions

- **Sarcopenia Comorbidities**
  - Osteoarthritis (OA)
  - Hip Fracture
  - Advanced Cancer
  - Chronic Obstructive Pulmonary Disease (COPD)
  - Heart Failure
  - Parkinson's disease
- **Rare Disorders** *(finalized 2/28/20)*
  - Hepatocellular Carcinoma (HCC)
  - Facioscapulohumeral Muscular Dystrophy (FSHD)
  - Myositis
  - Idiopathic Pulmonary Fibrosis (IPF)
  - Systemic Sclerosis

3 conditions will be selected from each column

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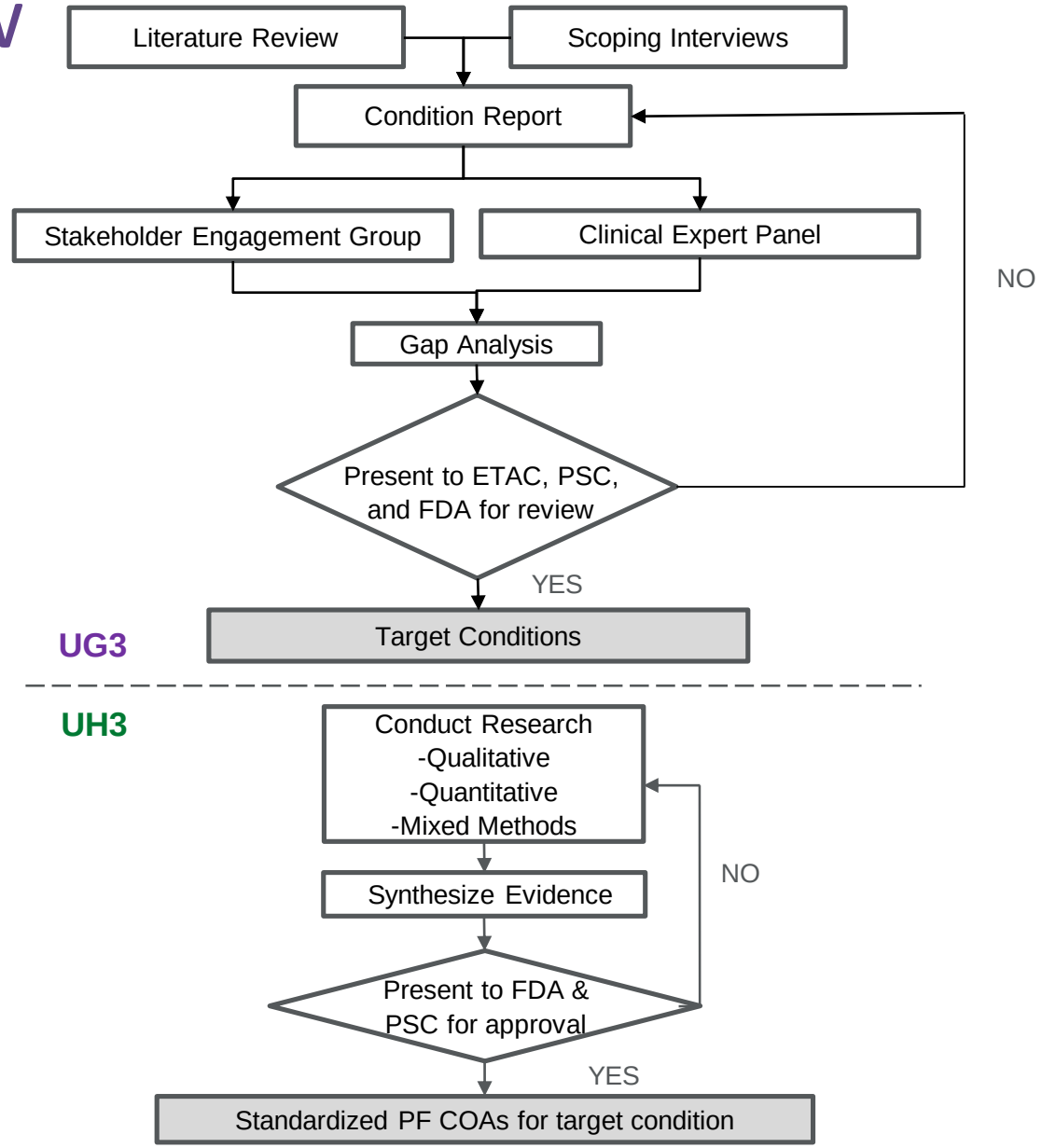
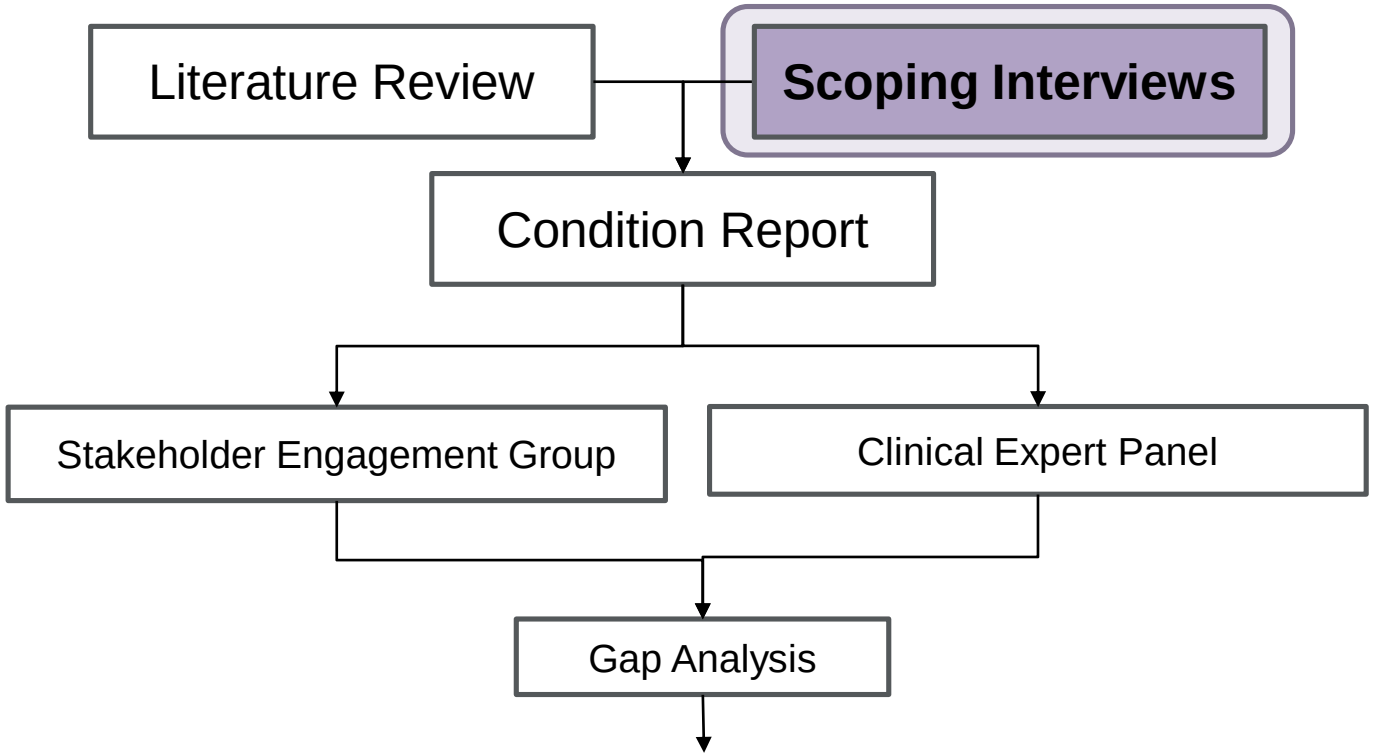
# Project Overview

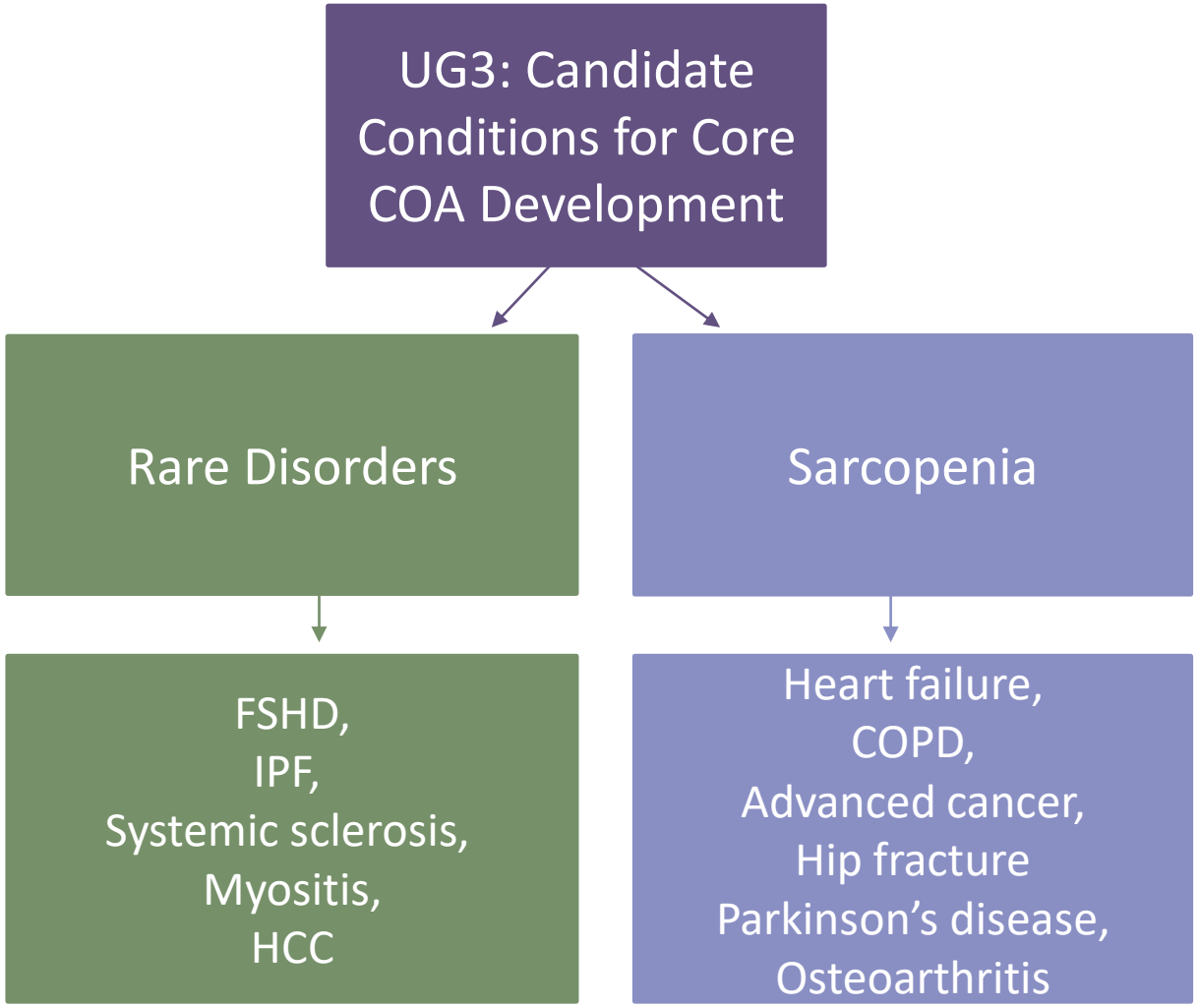


# Scoping Interviews



# Scoping Interviews

- **Objectives:**
  - Explore range of PF limitations, severity, and HRQL impact for candidate conditions (5 rare disorders, 6 sarcopenic)
  - Findings will complement the gap analysis and inform selection of up to 6 conditions for the development and validation work proposed in UH3

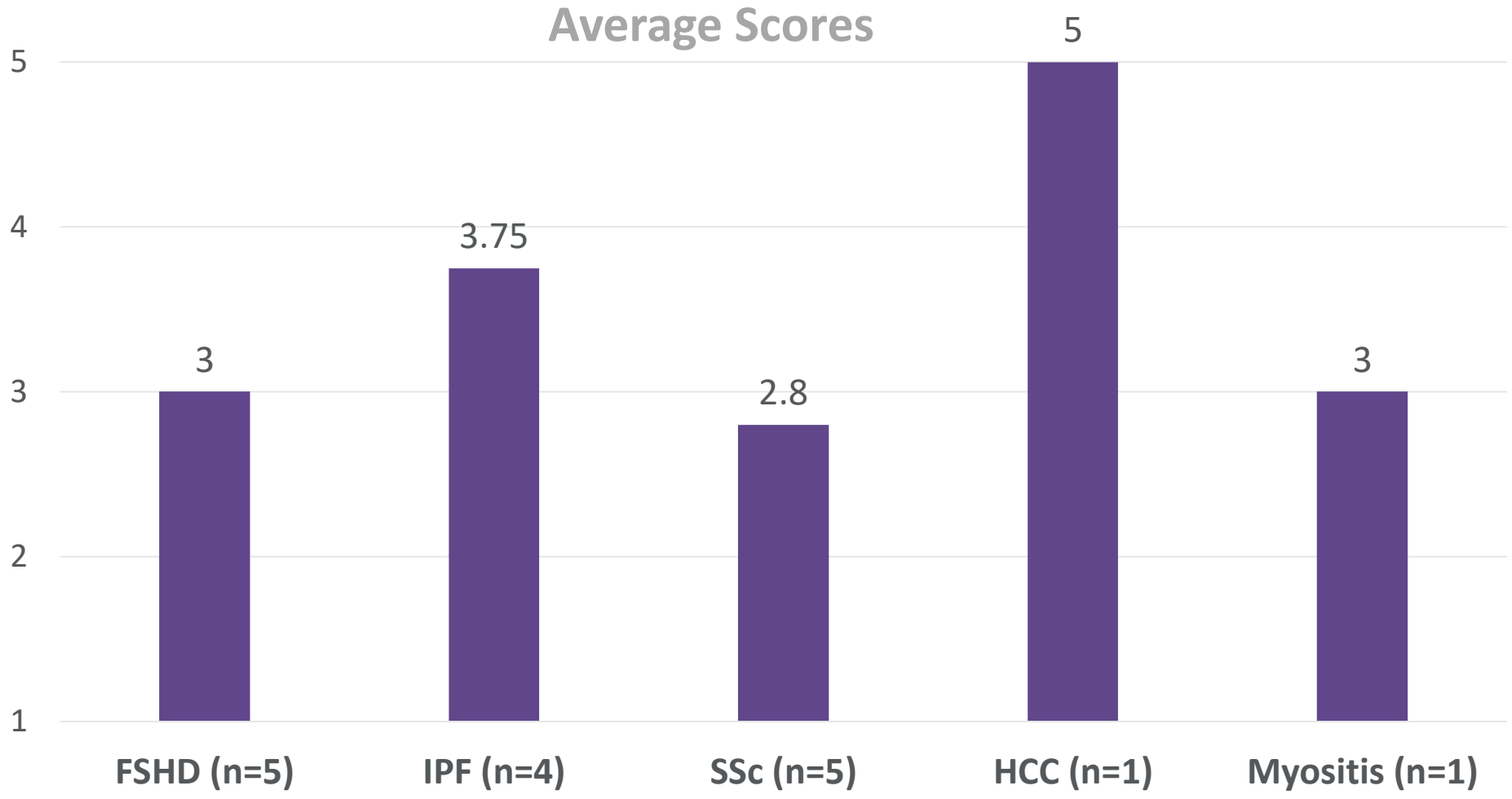


| Rare Disorder (RD) Participant Characteristics | FSHD (n=5) | IPF (n=4) | SSc (n=5) | HCC (n=1) | Myositis (n=1) |
|---|---|---|---|---|---|
| **Age, years** | | | | | |
| Average (range) | 46.2 (19-65) | 66.5 (60-72) | 57.0 (36-71) | 64.0 (--) | 55 (--) |
| **Gender** | | | | | |
| Female | 3 | 2 | 4 | 0 | 0 |
| Male | 2 | 2 | 1 | 1 | 1 |
| **Hispanic/Latino** | | | | | |
| Yes | 0 | 1 | 0 | 0 | 0 |
| No | 5 | 3 | 5 | 1 | 1 |
| **Race** | | | | | |
| White | 5[a] | 4 | 4 | 1 | 0 |
| Asian | 1[a] | 0 | 0 | 0 | 1 |
| American Indian/Alaska Native | 1[a] | 0 | 0 | 0 | 0 |
| Black or African American | 0 | 0 | 1 | 0 | 0 |

[a] *Identified as mixed race*

# Global06: **"To what extent are you able to carry out your everyday physical activities such as walking, climbing stairs, carrying groceries, or moving a chair?"**

**Scale Responses**
5 = *Completely*
4 = *Mostly*
3 = *Moderately*
2 = *A little*
1 = *Not at all*



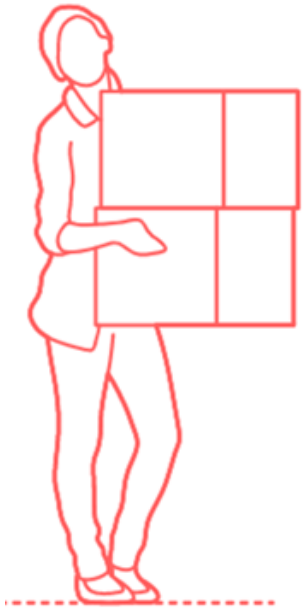
# Preliminary Findings: Physical Function Limitations

**Mobility**

**Central / Axial Function**

**Instrumental Activities of Daily Living**

**Dexterity**



**Facial Function**

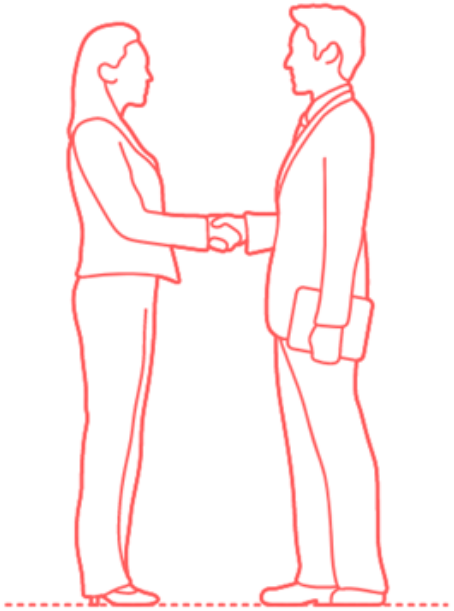


Figure source: https://www.dimensions.com/classifications/humans

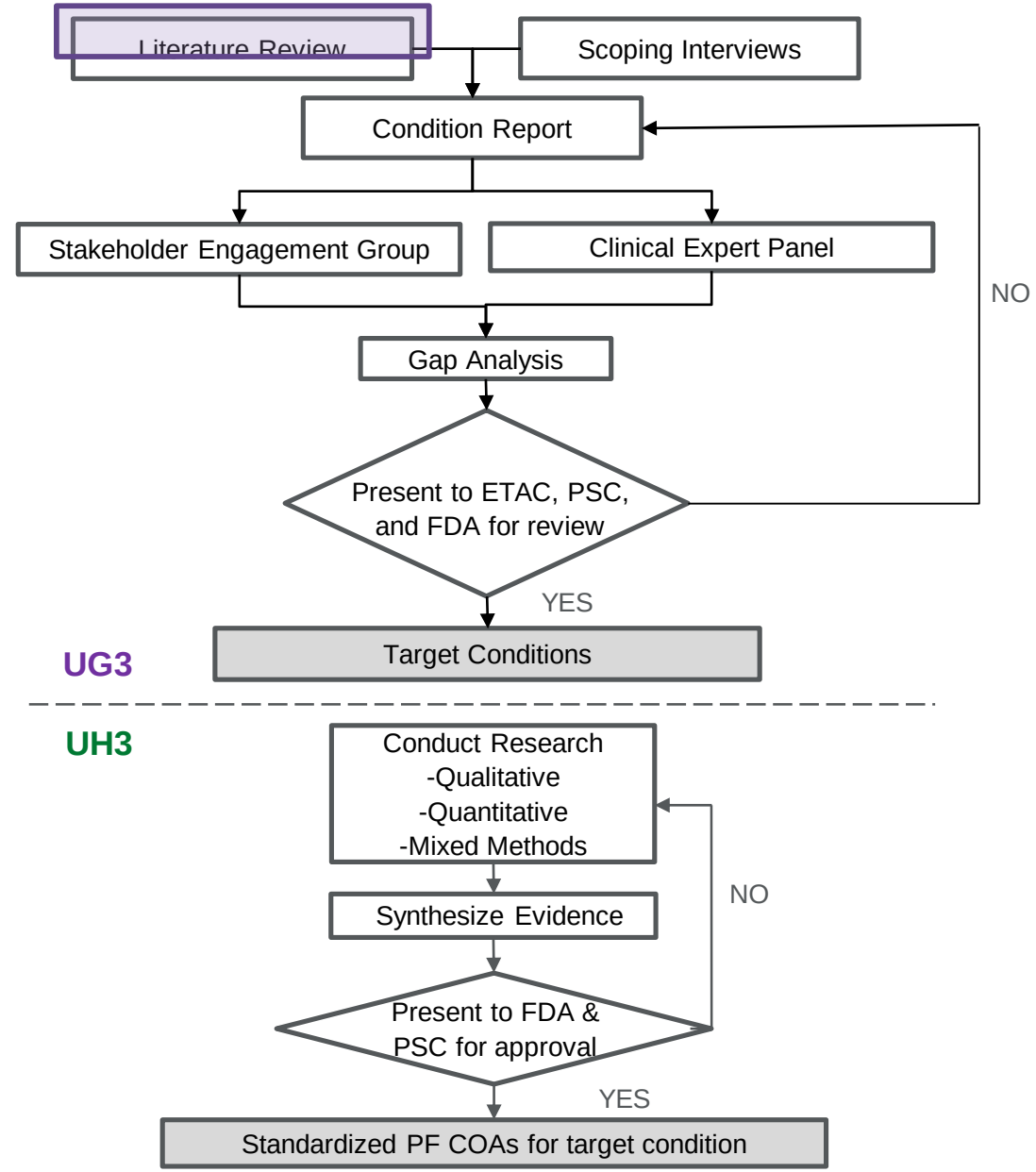
| RD Physical Function Limitations | FSHD (n=5) | IPF (n=4) | SSc (n=5) | HCC (n=1) | Myositis (n=1) | Total |
|---|---|---|---|---|---|---|
| **Mobility** | | | | | | |
| Walking | 5 | 4 | 4 | | 1 | 14 |
| Climbing stairs | 3 | 3 | 4 | | 1 | 11 |
| Running/jogging | 2 | 3 | 3 | | | 8 |
| Balance | 4 | 1 | 1 | | | 6 |
| Standing | 3 | 1 | 1 | | | 5 |
| Standing from a seated position | 3 | | 1 | | 1 | 5 |
| Walking on uneven surfaces | 3 | | 2 | | | 5 |
| Sitting down or rising from a low seat | 2 | | 2 | | | 4 |
| Descending stairs | 1 | 1 | 2 | | | 4 |
| Getting up from the floor | 2 | | 1 | | 1 | 4 |
| Walking uphill | 1 | 2 | 1 | | | 4 |

| RD Physical Function Limitations | FSHD (n=5) | IPF (n=3) | SSc (n=5) | HCC (n=1) | Myositis (n=1) | Total |
|---|---|---|---|---|---|---|
| **Dexterity** | | | | | | |
| Grasping/holding objects | 2 | | 5 | | 1 | 8 |
| Raising arms above shoulders/ reaching overhead | 3 | | 3 | | | 6 |
| Pushing an object (door, chair) | 1 | | 2 | | | 3 |
| Bringing hands to mouth/face/head | 1 | | 1 | | 1 | 3 |
| Typing, pressing buttons, or touchscreens | | | 2 | | | 2 |
| Writing | | | 1 | | 1 | 2 |
| Lifting arms | 1 | | | | | 1 |
| Reaching in front of body | | | 1 | | | 1 |
| Using bathroom tissue | | | 1 | | | 1 |

| RD Physical Function Limitations | FSHD (n=5) | IPF (n=4) | SSc (n=5) | HCC (n=1) | Myositis (n=1) | Total |
|---|---|---|---|---|---|---|
| **Facial Function** | | | | | | |
| Mouth movement (puckering, challenges speaking) | 3 | | 2 | | | 5 |
| Smiling | 3 | | | | | 3 |
| Facial expressions (general) | 2 | | 1 | | | 3 |
| Closing eyes | 2 | | | | | 2 |
| Raising eyebrows | 1 | | | | | 1 |

# Literature Review

- **UG3 Aim 2:**
  - Identify and describe respective impacts of candidate conditions (rare disorders & sarcopenic conditions) on the spectrum of PF
  - Identify existing PROs and PerfOs assessing these PF outcomes, and summarize evidence supporting their inclusion in a minimum core set of COAs

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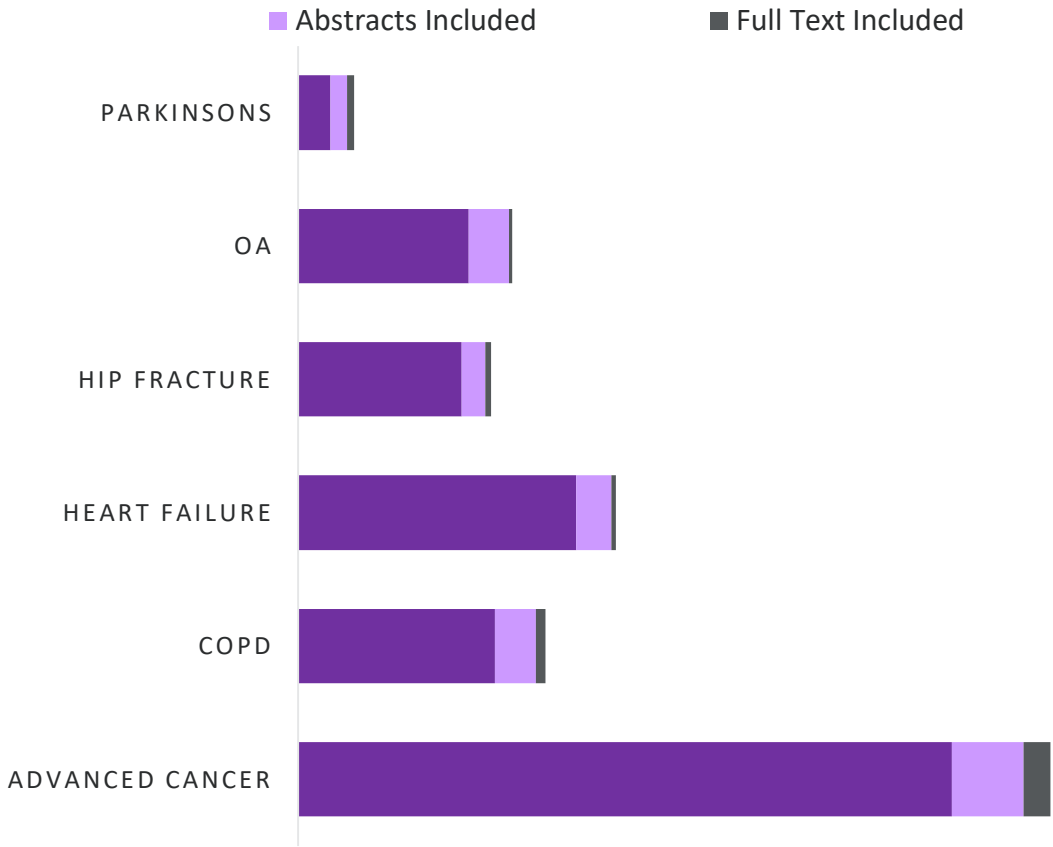
# Literature Review

- **Scoping Literature Review**
  - Map the key concepts on a particular topic or research area
  - Identify key concepts
  - Identify gaps in the research

  - Scoping literature reviews are particularly useful when a field is complex or has not been comprehensively reviewed

# Literature Review – Sarcopenia Articles

| Condition | Abstracts Reviewed | Abstracts Included | Articles Included |
|---|---|---|---|
| Parkinson's | 70 | 21 | 9 |
| OA | 267 | 50 | 4 |
| Hip Fracture | 234 | 30 | 7 |
| Heart Failure | 391 | 44 | 7 |
| COPD | 297 | 51 | 12 |
| Cancer | 940 | 89 | 34 |

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# Literature Review Observations

Advanced cancer: Frail advanced cancer patients who self-report lower physical activity demonstrate low physical performance on objective tests

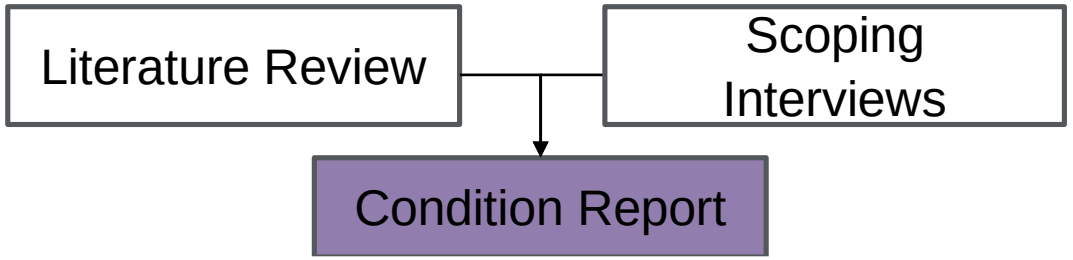
Parkinson's : Clinicians report that sarcopenic Parkinson's patients have increased difficulty with activities of daily living

Osteoarthritis: Sarcopenic obesity patients with OA display lower grip strength and poor physical performance (gait speed, Timed Up & Go test, Sit-to-Stand test, 6 m walk test)

COPD: Lung (respiratory) function is assessed with digital health technologies and is related to Physical Function assessments in COPD patients.

# Next Steps

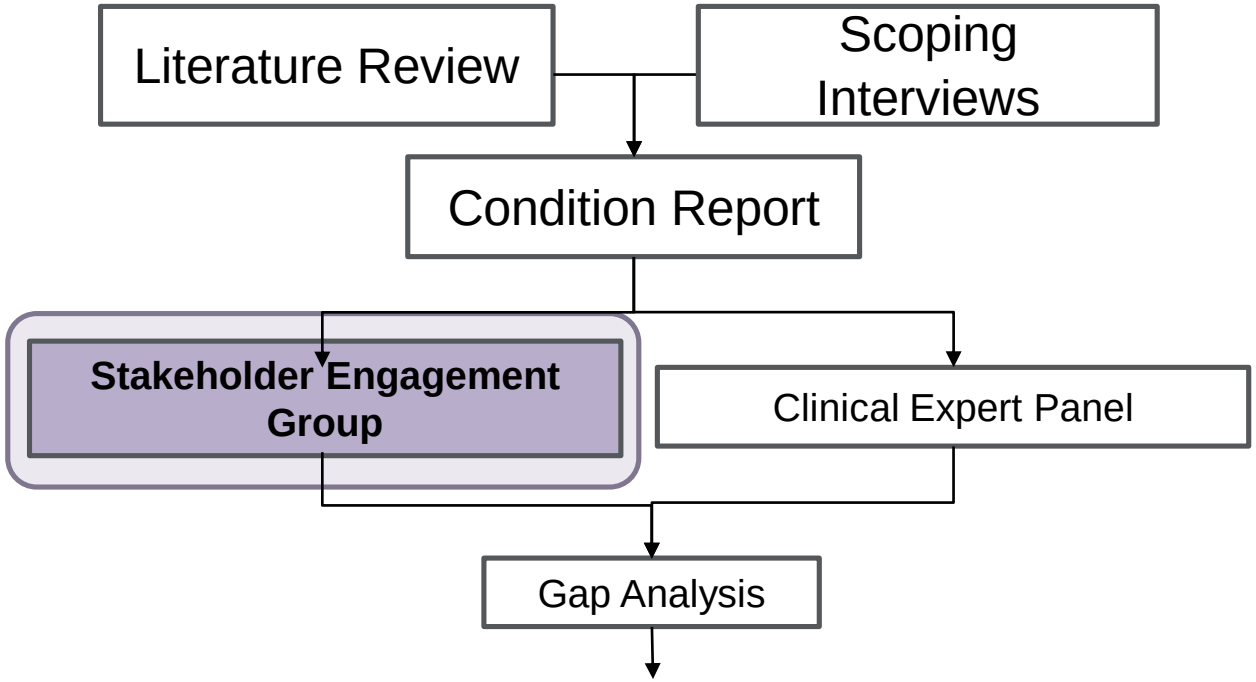
- Complete rare disorders scoping literature reviews (*September*)
- Conduct analysis of data extracted from searches (*October*)
- Integrate with scoping interview results in “Condition Report” (*November*)



# Stakeholder Engagement Group

- **UG3 Aim 1:** To convene stakeholders, including patients, care partners, clinicians, measurement experts, payers, regulators, and pharmaceutical industry representatives, around the topic of PF as it relates to approval of new drugs.

- **Goal:** To motivate, facilitate, and retain the active participation and involvement from all key stakeholder groups in this patient-focused outcome measurement initiative.

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# SEG Membership (N = 14)

| Name (Organization) | Group Represented |
|---|---|
| Lisa Autry<br>Kimberly Bennett-Eady<br>Chris Looby<br>Damian Santay | Patients |
| Derrick Bennett<br>Bunny Garthe<br>Carol Looby<br>Barbara Santay | Care Partners |
| Vanessa Boulanger (*National Organization for Rare Disorders*)<br>Ryne Carney (*Alliance for Aging Research*) | Patient Advocacy |
| Katy Benjamin (*AbbVie; SEG Co-Chair*)<br>Linda Deal (*Pfizer*)<br>James Shaw (*Bristol Myers Squibb*) | Pharma |
| Bob Lazarchik | Healthcare Payer |

# SEG Meetings

## Meeting 1
## 2/26/20

- **SEG Launch Meeting**
  - NUCOAT and SEG Member Introductions
  - NUCOAT Overview and Project Milestones
  - SEG Responsibilities and Co-Chair Election

## Meeting 2
## 6/8/20

- **SEG Full Member Meeting**
  - Feedback on NUCOAT Research Activities
  - Feedback on Project Website
  - Discussion on Further Engaging SEG in NUCOAT Committees and Decision Making

## Meeting 3
## 7/17/20

- **SEG Patients and Care Partners Meeting**
  - Feedback on NUCOAT Research Activities
  - Discussion of Impact of COVID-19 on Healthcare and Potential Research Engagement

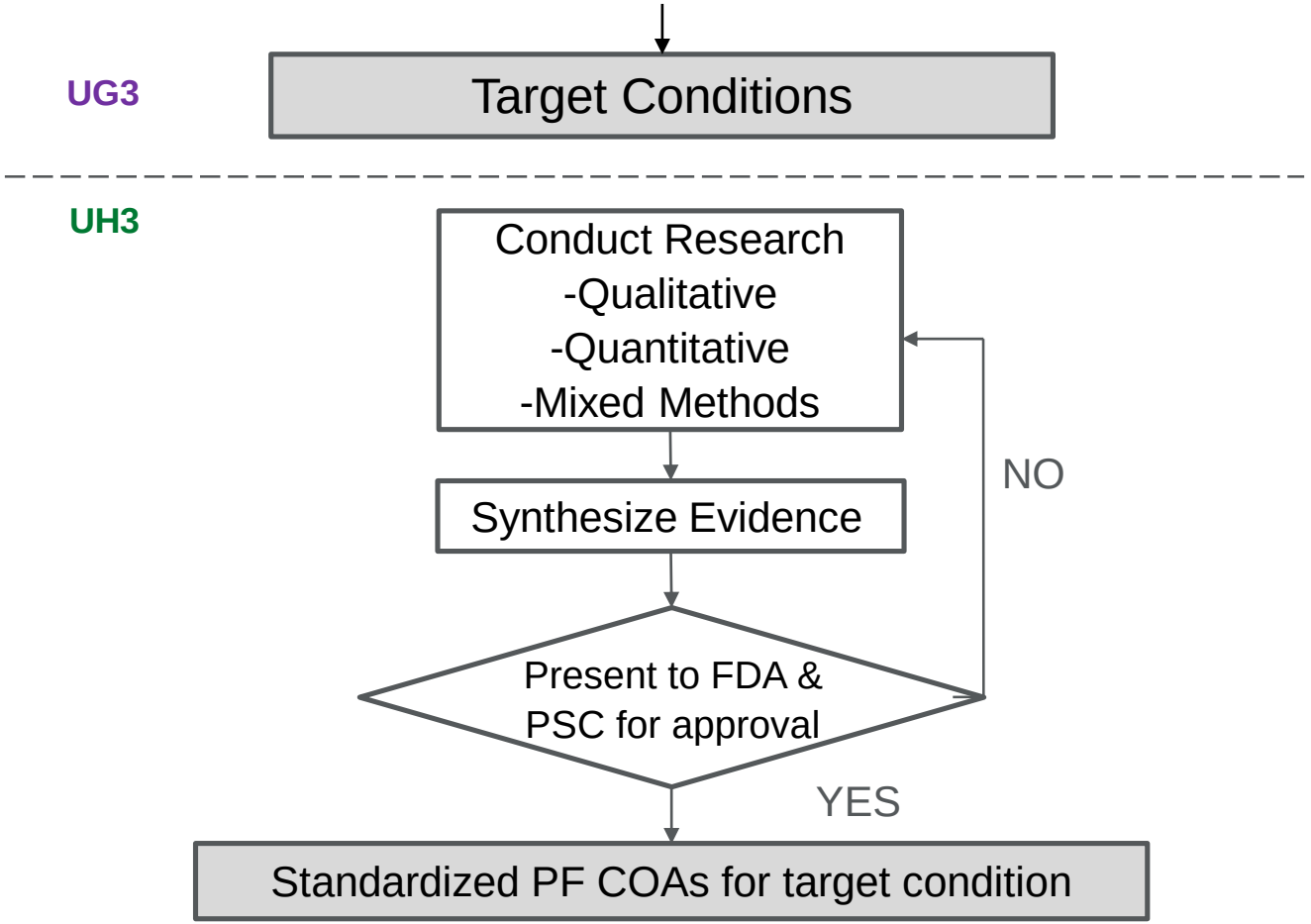
# SEG Meeting 3 Observations

## Impact of COVID-19 on Care Partners & Patients (Sarcopenia-Related Conditions and Rare Disorders)

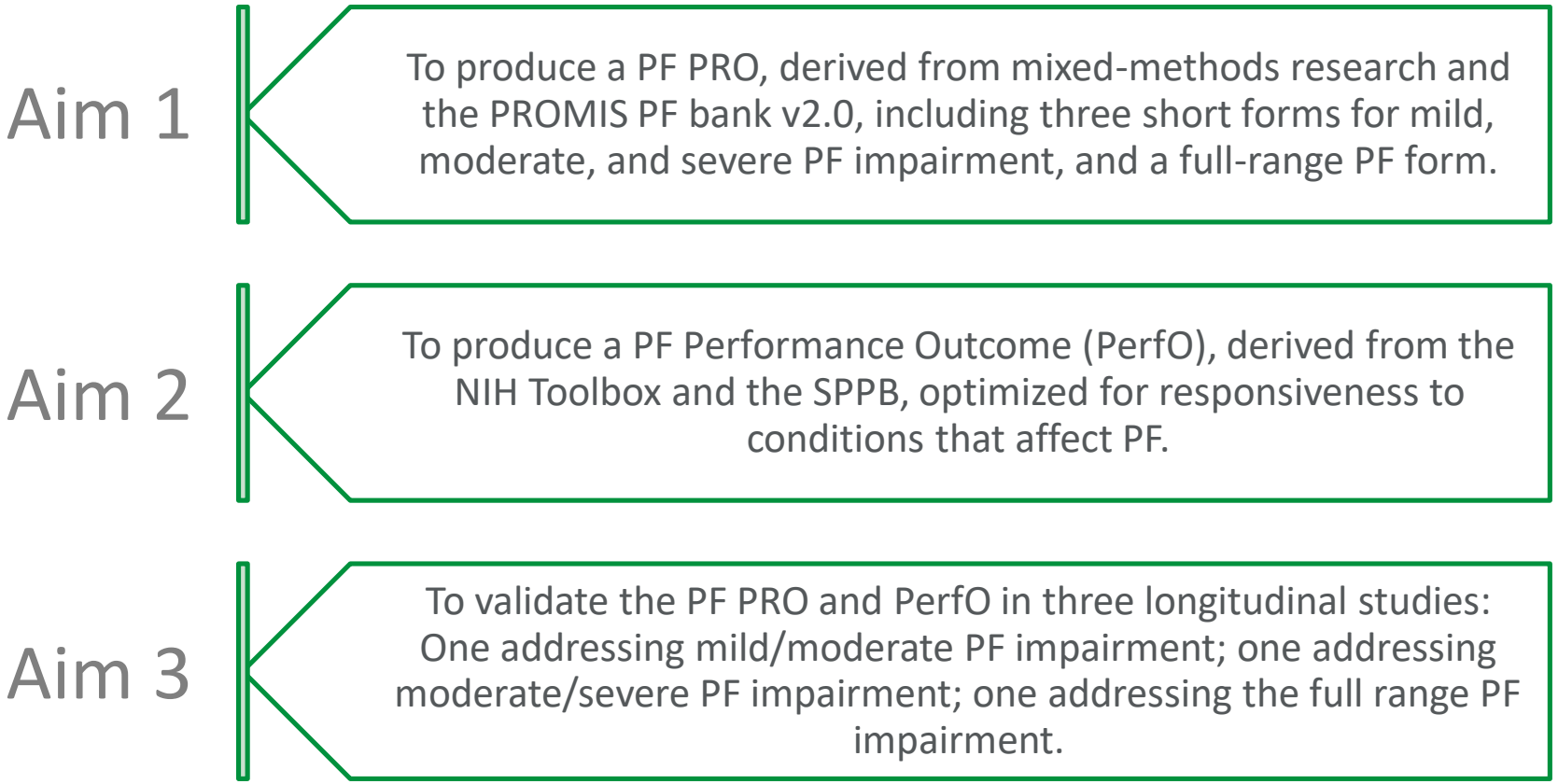
| *Healthcare Engagement* |
| --- |
| Cancelled in-person appointments (2) |
| Postponed in-person appointments (physical therapy, mammogram) (2) |
| Completed remote healthcare visits (4) |
| Completed in-person healthcare visits (chemotherapy, primary care) (2) |
| Provider cancelled/re-scheduled appointments (1) |

| *Potential Research Engagement* |
| --- |
| Would participate in person with comprehensive COVID-19 safety protocols (1) |
| Would participate remotely (2) |
| Would participate in home visits (2) |
| Would not participate in person (1) |

# Transition to UH3



# NUCOAT UH3 Aims



# Thank You

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# Audience Q&A

# CDER Pilot Grant Program: Standard Core Clinical Outcome Assessments (COAs) and their Related Endpoints


# Questions?

**To ask a question, click on the "dialogue" icon in the lower right corner of the viewer.**

# **C**linical **O**utcome **A**ssessments for **A**cute **P**ain **T**herapeutics in **I**nfants and Young **C**hildren

## Public Meeting

August 28, 2020

COA-APTIC

Bryce Reeve, PhD
Kanecia Zimmerman, MD, MPH
Duke University School of Medicine

# COA-APTIC Public Meeting Agenda

I. Overarching Goals and Objectives
   - Specific Aims 1-3

II. COA-APTIC Overview of Aims & Milestones
   - Identifying and Developing Milestone 1 & Milestone 2

III. Dissemination Plan

IV. Stakeholder Engagement

V. Next Steps
   - Upcoming Aims and Milestones
     - UG3 year 2
     - UH3

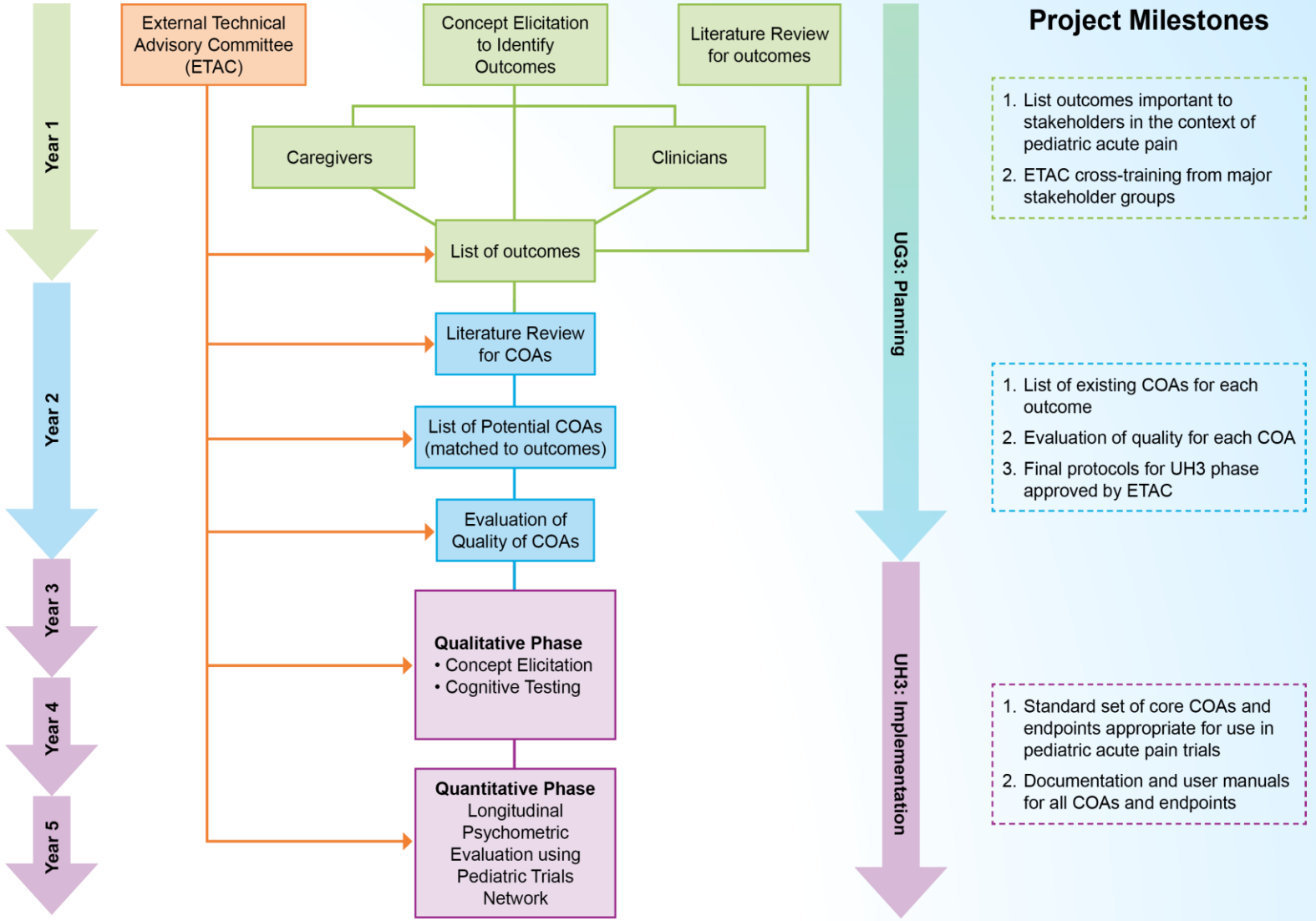
# Overarching Goals and Objectives

**Identify or develop core sets of high-quality Clinical Outcome Assessments (COAs) and endpoints for assessment of acute pain and other relevant outcomes for use in clinical trials of pain therapeutics in infants and young children.**

# Specific Aims

- **UG3 Aim 1**: Identify the core set of outcomes most pertinent to patients, caregivers, and clinicians for management of acute pain in infants and young children.
- **UG3 Aim 2**: Identify COAs and endpoints for each outcome, and formally evaluate each COA and endpoint's fit-for-purpose within the context of pediatric acute pain drug trials.
- **UH3 Aim 3:** Evaluate relevant psychometric properties of chosen COAs by leveraging an ongoing NIH-sponsored PTN master protocol of analgesics in infants and children.

# COA-APTIC Overview of Aims & Milestones




## Project Milestones

1. List outcomes important to stakeholders in the context of pediatric acute pain
2. ETAC cross-training from major stakeholder groups

1. List of existing COAs for each outcome
2. Evaluation of quality for each COA
3. Final protocols for UH3 phase approved by ETAC

1. Standard set of core COAs and endpoints appropriate for use in pediatric acute pain trials
2. Documentation and user manuals for all COAs and endpoints

# UG3 Phase: Approach to Identifying Key Outcomes, Measures, and Endpoints

1. External Technical Advisory Committee (ETAC)
2. Systematic Literature Review
3. Concept Elicitation Interviews with Key Stakeholders

# 1. External Technical Advisory Committee (ETAC)

**Robyn Bent**

*U.S. Food and Drug Administration (FDA)*

**Specialization: Regulatory**

**Sharon Brown**

*Parent Advocate*

**Specialization: Long-term effects of pediatric pain**

**Sarah Ciaccia**

*Parent Advocate*

**Specialization: Parental perceptions of acute pain**

**Ernest Kopecky, PhD**

*Teva Pharmaceutical Industries*

**Specialization: Industry**

**Meghan Pascal**

*Parent Advocate*

**Specialization: Parental perceptions of acute pain**

**Amy Ohmer**

*International Children's Advisory Network (iCAN)*

**Specialization: Patient advocacy and engagement**

**Frank Rockhold, PhD**

*Duke University*

**Specialization: Biostatistics**

**Bonnie Stevens, PhD**

*University of Toronto*

**Specialization: Management of pain in hospitalized preterm newborn infants**

**Gary Walco, PhD, ABPP**

*University of Washington School of Medicine*

**Specialization: Co-chair of Pediatric Pain Research Consortium within ACTTION**

**David Warner, MD**

*Mayo Clinic*

**Specialization: Pediatric anesthesiology**

**Leanne West**

*International Children's Advisory Network (iCAN)*

**Specialization: Patient advocacy and engagement**

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# ETAC Cross Training: March 6, 2020

- Parental Perceptions of Acute Pain
- Infant and Toddler Development
- Clinical Outcome Assessments (COA) Development Process
- Pain Assessment in Infants and Young Children
- Perioperative Pain Assessment and Management
- Designing Outcomes and Endpoints in Clinical Trials
- Regulatory Concerns Regarding Pain Trials in Infants and Young Children
- Industry Concerns Regarding Therapeutic Agents for Acute Pain in Infants and Young Children
- Engaging Patient Stakeholders in Research and Practice.

https://dcri.org/coa-aptic/

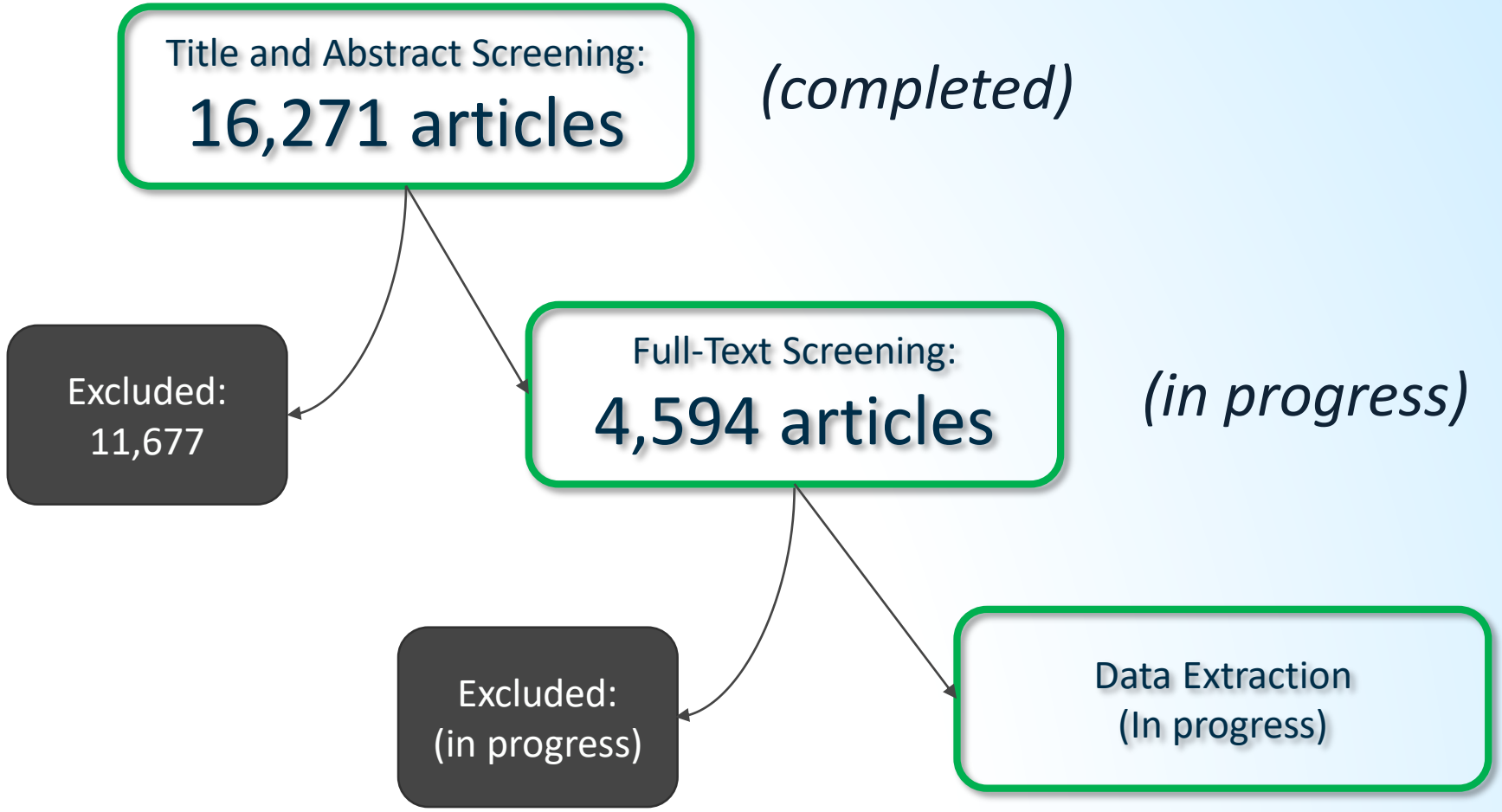
# 2. Systematic Literature Review

- **Goal:**
  - Identify relevant outcomes, measures, and endpoints to be captured within the context therapeutic studies of acute pain experienced by infants and young children (0 to <3 years old)

# Literature Review Methods

- Key concepts
  - Search terms to capture wide range of
    - painful events, procedures, and conditions
    - pain measurement and pain treatment
    - children 0 to <3 years old
  - Include pediatric studies published after 1980
- Databases searched
  - PubMed, PychINFO, Web of Science, Clinicaltrials.gov

# Literature Review

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# Literature Review Data

- Sample Size
- Population Demographics
- Study Type (Randomized Controlled Trial, Observational, etc.)
- Study Intervention
- Data Collection Time Points
- Study Outcomes
- Study Measures
- Study Endpoints
- Adverse Events Reported

# 3. Concept Elicitation Interviews with Stakeholders

- Goal: To identify important aspects of acute pain assessment, treatment, and response to treatment in children who are 0 to <3 years of age, from a clinician and caregiver perspective.
- Methods:
  - One-hour, phone based interviews
  - 27 Clinicians (Pediatric physicians, pharmacists, nurse practitioners, physician assistants and/or nurses)
  - 42 Caregivers/Parents of children with
    - Malignant or non—malignant visceral or hematologic disease
    - Surgery (or other procedure)
    - Trauma or injury
    - Congenital Conditions

# Clinician Interviews

- Topics of interest:
  - General approach to pain assessment
  - Pain expression
  - Differences in expression of pain and non-pain distress (e.g. fear / anxiety)
  - Interventions for pain and non-pain distress
  - Pain scales used
  - Additional concepts to measure alongside pain (e.g. sedative effect)
- Probed on three age sub-categories:
  - 0- under 2 months
  - 2 months to under 1 year
  - 1 year to under 3 years

# Caregiver Interviews

- Topics of interest:
  - Medical History and painful experiences
  - Pain expression
  - Distress expression vs pain expression
  - Interventions for pain or distress
  - Pain scales used
  - Additional concepts important to caregivers (e.g. side effects or sedative effects, medication cost)
- Caregivers/Parents of Children who experience acute pain and stratified by child's age
  - 0 to < 2 months, 2 to < 6 months, 6 to < 12 months, 12 to < 18 months, 18 to < 24 months, 24 to < 30 months, 30 to < 36 months

# Dissemination

**The COA-APTIC Website was made available to the public on March 1, 2020.**


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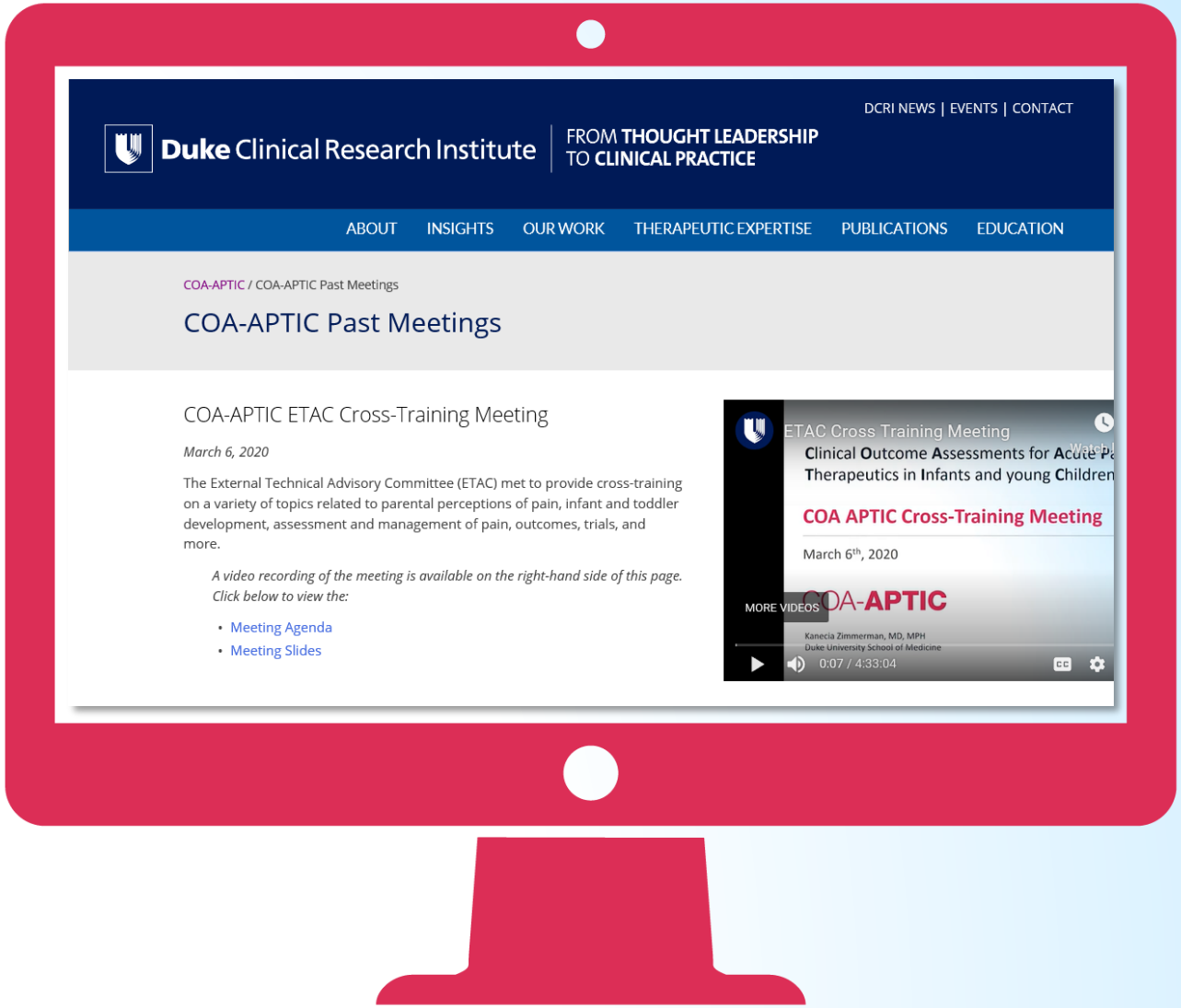
# Dissemination

**Meeting videos, agendas, and minutes can be found on the COA-APTIC website.**

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# Stakeholder Engagement

- National Institute of Health (NIH)
  - The NIH is collaborating with COA-APTIC by allowing COA-APTIC to recruit participants from PTN's ANA, DGX, and POP studies.
- Initiative on Methods, Measurement, and Pain Assessment in Clinical Trials (IMMPACT)
  - Provides reviews and recommendations for improving the design, execution, and interpretation of clinical trials of treatments for pain.
- Analgesic, Anesthetic, and Addiction Clinical Trial Translations, Innovations, Opportunities, and Networks (ACTTION)
  - COA-APTIC collaborates with ACCTION to expedite the discovery and development of improved analgesic for the benefit of the public health.
  - Dr. Bryce Reeve is an Advisory Committee Member for ACTTION.

# Next steps for COA-APTIC: UG3 Phase

| | |
|---|---|
| Jan 2021 | • Clinician Interviews completed |
| Mar 2021 | • Caregiver Interviews completed |
| Mar 2021 | • Literature Review Completed |
| Mar 2021 | • Final List of Outcomes Developed |
| Apr 2021 | • ETAC Feedback |

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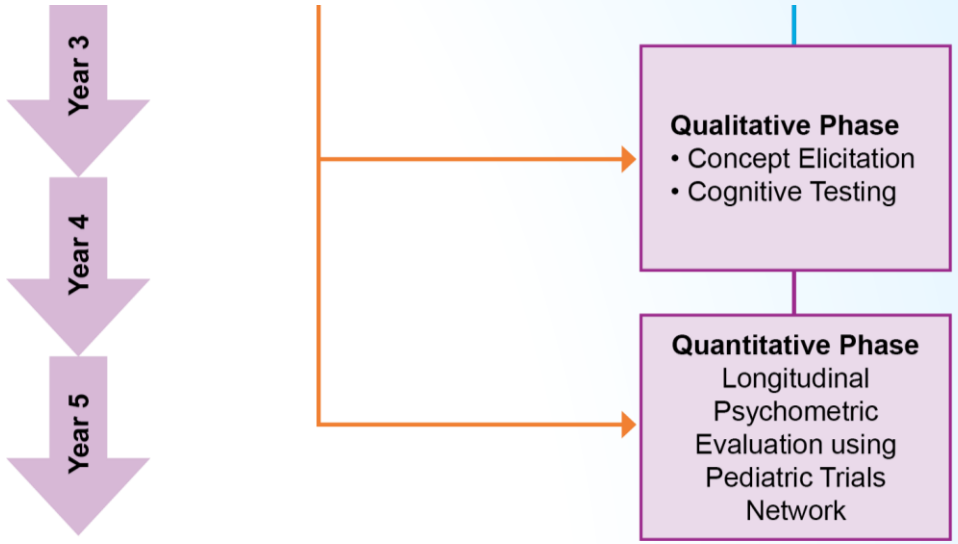
# Upcoming Aims and Milestones: UG3

- Identify characteristics of existing COAs for each outcome
- Evaluate quality for each COA
- Develop final protocols for UH3 phase

# Upcoming Aims and Milestones: UH3 Phase

- Develop a standard set of core COAs and endpoints appropriate for use in pediatric acute pain trials.
- Provide evidence to support COAs and endpoints.
- Provide documentation and user manuals for all COAs and endpoints.

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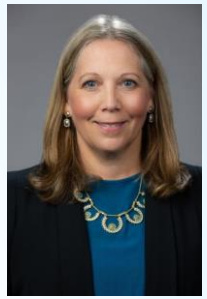


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# Questions?

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# Audience Q&A

# CDER Pilot Grant Program: Standard Core Clinical Outcome Assessments (COAs) and their Related Endpoints


# Questions?

**To ask a question, click on the "dialogue" icon in the lower right corner of the viewer.**

# CDER Standard Core Sets: Clinical Outcome Assessments and Endpoints Pilot Grant Program New Funding Opportunity

**Panel Discussion**

# Audience Q&A

# CDER Pilot Grant Program: Standard Core Clinical Outcome Assessments (COAs) and their Related Endpoints


# Questions?

**To ask a question, click on the "dialogue" icon in the lower right corner of the viewer.**

# Send us your comments!

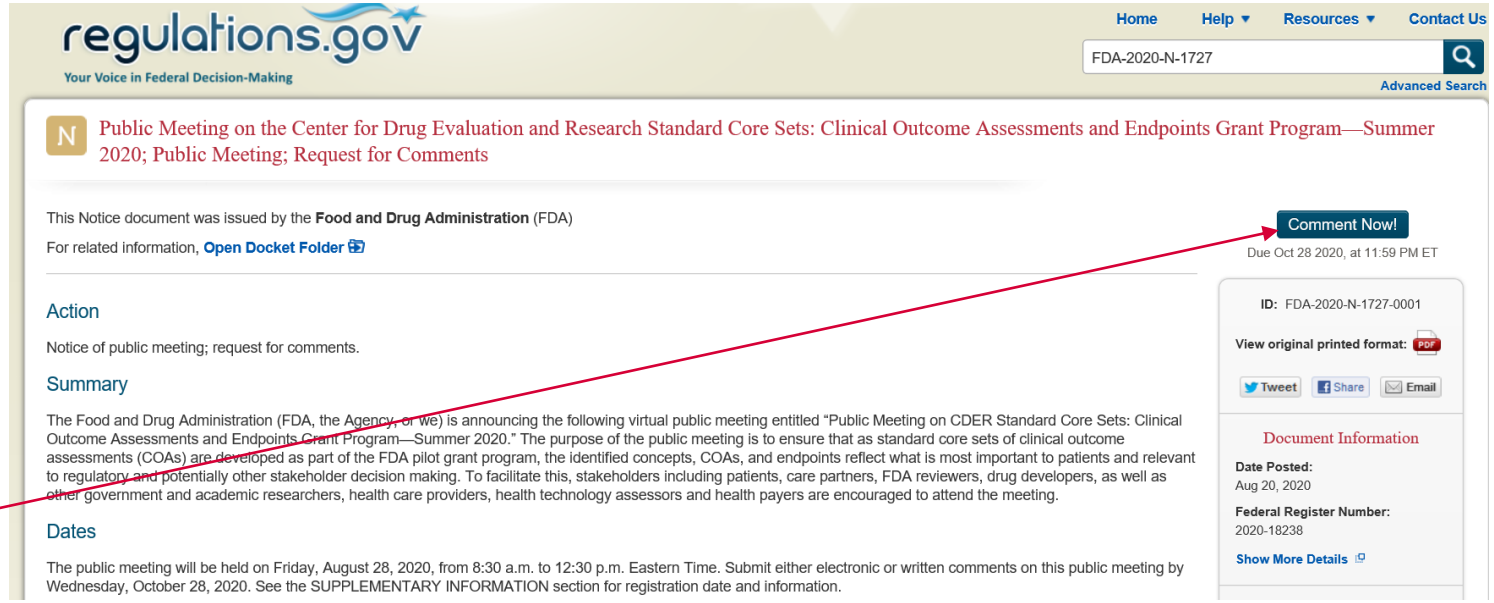
**You can also send us comments through the "public docket"**

- The docket will be open until October 28, 2020!

Visit: https://www.regulations.gov/document?D=FDA-2020-N-1727-0001

Or Search for our Docket # "2020-N-1727" on www.regulations.gov

And **Click Comment Now!**



# Closing Remarks

**Robyn Bent, RN, MS**
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