

# What is ACCORDS?

Adult and Child Center for Outcomes Research and Delivery Science

ACCORDS is a 'one-stop shop' for pragmatic research:

- A multi-disciplinary, collaborative research environment to catalyze innovative and impactful research
- Strong methodological cores and programs, led by national experts
- Consultations & team-building for grant proposals
- Mentorship, training & support for junior faculty
- Extensive educational offerings, both locally and nationally



# ACCORDS Upcoming Events – mark your calendars!

April 8-9, 2026	D&I Science for Researchers Workshop <i>Applications are open!</i>
April 24, 2026	ACCORDS & CCTSI Community Engagement Forum
May 20-21, 2026	Colorado Pragmatic Research in Health Conference <i>Pragmatic Research: Methods, Tools, and Technology for Rapidly Changing Contexts</i>  Registration is live! Visit <a href="http://coprhcon.com">coprhcon.com</a> for more info.  <b><u>*New to COPRH Con*</u></b> Pre-conference workshops: <ul style="list-style-type: none"><li>• Pragmatic Research Planning</li><li>• Planning for a Competitive Career Development Award: A Roadmap for Health Services Researchers</li><li>• Mixed Methods Design &amp; Integration Training for Health Services Research</li><li>• Design for Innovation: Practical Use of User-Centered Design</li><li>• AI Essentials for Health Services Researchers: Balancing Increased Productivity with Responsible Use</li><li>• Elevating Research Project Management Practice</li><li>• Dissemination in the Age of AI: Design Tools at Your Fingertip</li></ul>



# ACCORDS Fellowship Opportunities

## Scholars in Clinical Outcomes Research (SCORE)

- The Scholars in Clinical Outcomes Research (SCORE) Fellowship at ACCORDS is currently recruiting for the July 2026 cohort.
- Fellowship designed for junior and mid-career faculty at the University of Colorado Anschutz or affiliate institution with goal of translational or outcomes research
- Two-year program consists of weekly didactic training, weekly work-in-progress sessions, 1:1 mentoring, and intensive grant writing course
- Pilot awards available for competitive applicants
- Applicants must have support of home department/division; at least 50% protected time, and tuition support for program costs
- **Applications due March 15<sup>th</sup>, 2026!**

## ACCORDS Primary Care Research Fellowship

- Goal: Train post-doctoral professionals to become primary care research leaders
- Nationally renowned training in Implementation Science
- Fellows receive funds for pilot project support, tuition, travel for conferences and a standard NIH-level T32 stipend (options to negotiate for additional salary support)
- Expert support for fellows from ACCORDS cores, including: Biostatistics, Health Economics, Implementation Science, Qualitative/Mixed Methods, and Shared Decision Making
- Apply tuition toward an advanced degree tailored to your background (Master's Degree and/or Graduate Certificate in Implementation Science)
- **Applications open and reviewed on a rolling basis!**



# ACCORDS Highlights: Fellowship Edition

## Building Impactful Health Services Research Careers



**Emily Dunston, PhD, MS**  
Postdoctoral Research Fellow,  
University of Colorado, Division of  
Internal Medicine



**Megan Abbott, MD**  
Assistant Professor, Pediatric Epilepsy,  
University of Colorado, Department of  
Pediatrics, Division of Neurology



# **Beginning with the end in mind: An iterative prototyping of a cancer rehabilitation and exercise referral process in primary care**

*Emily R. Dunston, PhD, M.S.*

*Department of General Internal Medicine*

*University of Colorado Anschutz Medical Campus*



University of Colorado  
Anschutz Medical Campus

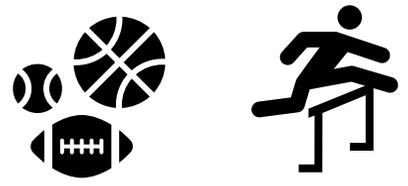
# Disclosures

No professional or personal conflicts of interest to report.

This project is supported by the Health Resources and Services Administration (HRSA) of the U.S. Department of Health and Human Services (HHS) as part of the award T32HP42016, totaling \$2,225,097 over 5 years, with 0% financed with non-governmental sources. The contents are those of the author and do not necessarily represent the official views of, nor an endorsement by HRSA, HHS, or the U.S. Government.

# Primary Care Research Fellowship

Background



Aims

**Sports Science**  
**Undergraduate & Masters Training**

Methods



**Exercise Oncology**  
**Doctoral Training**

Results

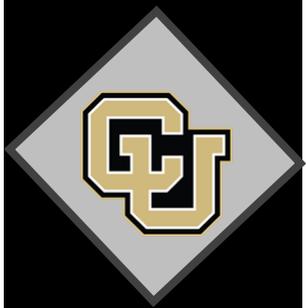


**Primary Care Research**  
**Implementation Science**  
**Postdoctoral Training**

Conclusions

I want to help everyone benefit from physical activity

These programs really help people, but reach is very limited



# Cancer Survivorship Care

Background

Aims

Methods

Results

Conclusions

**Table. Possible Late or Long-Term Side Effects of Cancer Treatment**

Chemotherapy	Radiation	Surgery
Bone and joint problems	Bone growth issues (in children)	Chronic pain
Dental problems	Cavities and tooth decay	Lymphedema
Digestion issues	Cognitive challenges	Phantom pain
Early menopause	Digestion issues	Scarring
Fatigue	Dry mouth	
Hearing loss	Early menopause	<b>Hormone Therapy</b>
Heart problems	Fatigue	Blood clots
Infertility	Heart and vascular problems	Hot flashes
Kidney and urinary problems	Hypothyroidism	Menopausal symptoms
Liver damage	Infertility	Osteoporosis
Loss of taste	Intestinal problems	Risk of other cancers
Lung disease	Lung disease	Sexual side effects
Nerve damage (neuropathy)	Lymphedema	
Osteoporosis	Memory problems	<b>Immunotherapy</b>
Reduced lung capacity	Osteoporosis	Late effects unknown yet
Risk of other cancers	Permanent hair loss	
Secondary cancers	Risk of stroke	<b>Targeted Therapy</b>
	Secondary cancers	Late effects unknown yet
	Skin sensitivity	
	Thyroid/adrenal gland problems	

**Meeting physical activity guidelines can attenuate cancer treatment related side-effects**

**66% of all cancer survivors are aged 65 years and older**

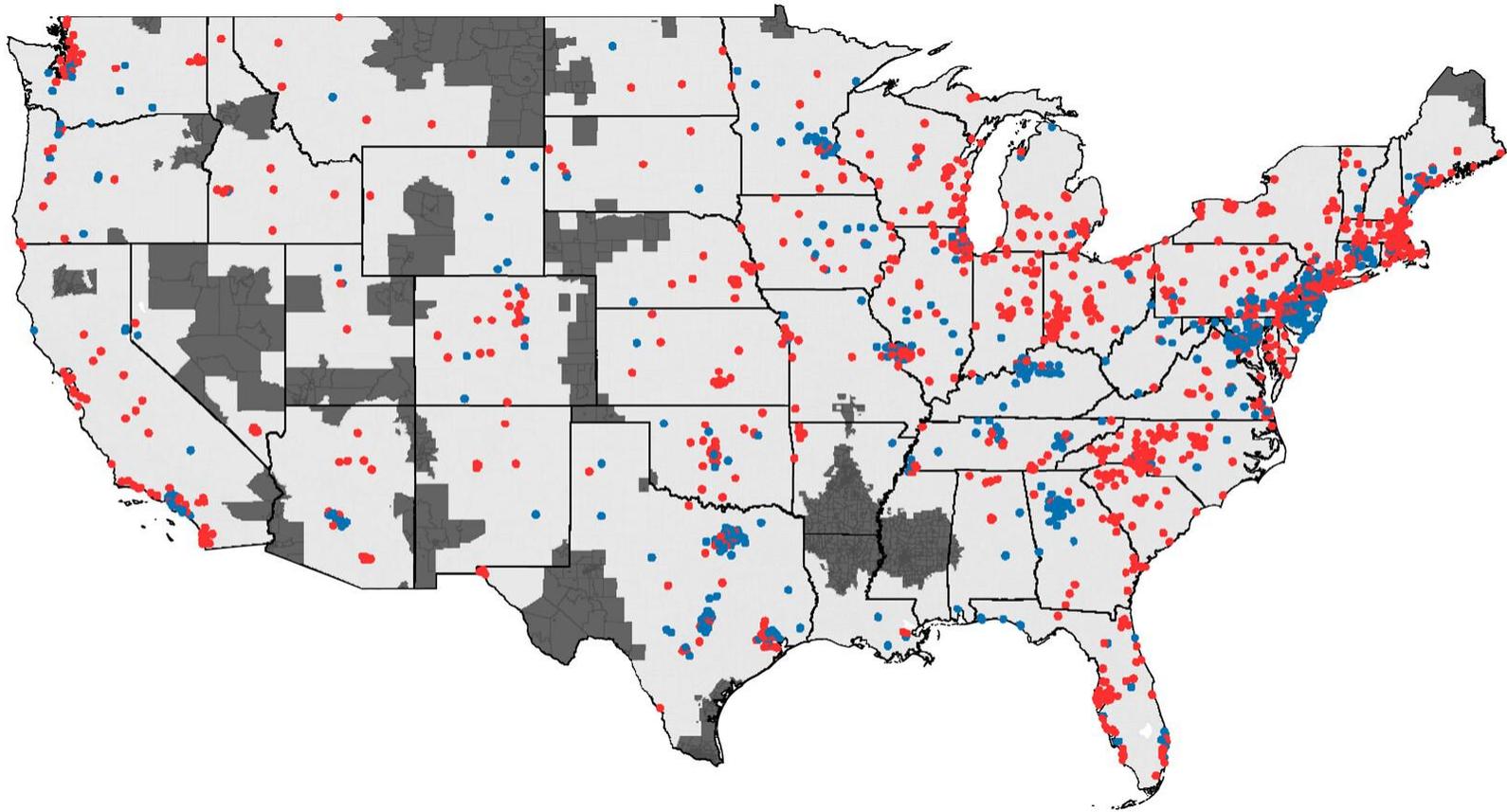


# Cancer Rehab and Exercise Services

Over 2,000 CaRES Across the United States

Background

- Aims
- Methods
- Results
- Conclusions



**Proximity to a program**

- Proximal to one or more programs
- Lack of proximity to any program

**Type of Program**

- Cancer rehabilitation
- Exercise oncology



# Benefits of CaRES

Background

Aims

Methods

Results

Conclusions



Outcome	Evidence Level	
	All Survivors	Survivors ≥ 65 years
↑ Physical Function	Systematic reviews across many cancer types	Two RCTs and two feasibility studies
↑ Quality of Life	Systematic reviews across many cancer types	One systematic review and several RCTs
↓ Fatigue	Systematic reviews primarily in breast and prostate cancers	Two systematic reviews and several RCTs
↓ Anxiety	Systematic reviews primarily in breast and prostate cancers	Insufficient evidence
↓ Depression	Systematic reviews across 4 cancer types	Insufficient evidence

Adeline et al., J Geriatr Oncol, 2020; Bourke et al., Eur Urol, 2014; Campbell et al., MSSE, 2019; Dittus et al., Support Care Cancer, 2020; Forbes et al., J Cancer Surviv, 2020; Luctkar-Flude et al., Cancer Nurs, 2007; Winters-Stone et al., J Geriatr Oncol, 2022

# CaRES Referrals

## Limited awareness of and access to CaRES

Background

Aims

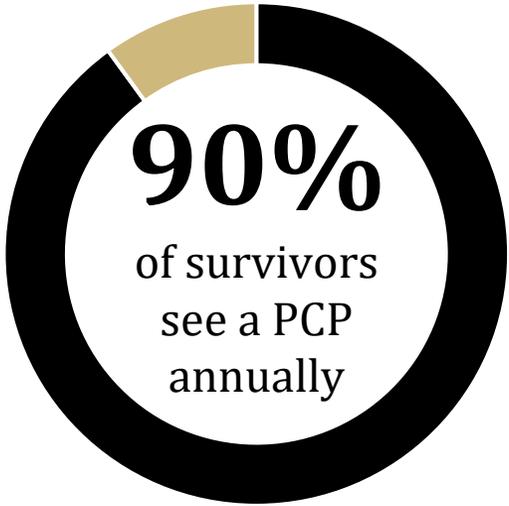
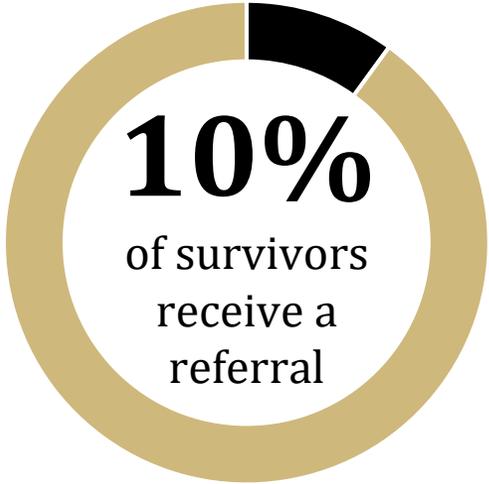
Methods

Results

Conclusions



Referral from a healthcare provider is a key facilitator to CaRES engagement



Efforts to improve CaRES referrals have been limited to the oncology settings

# Aims

Background

Aims

Methods

Results

Conclusions

Aim 1: Explore perspectives and characteristics of three key groups that are involved in referral of older cancer survivors to CaRES from primary care:

- 1) Older cancer survivors
- 2) Primary Care Providers
- 3) CaRES program directors

Aim 2: Iteratively refine a prototype of a CaRES referral workflow tailored to older adults for use in primary care clinics



# Conceptual Model

Background  
Aims  
Methods  
Results  
Conclusions

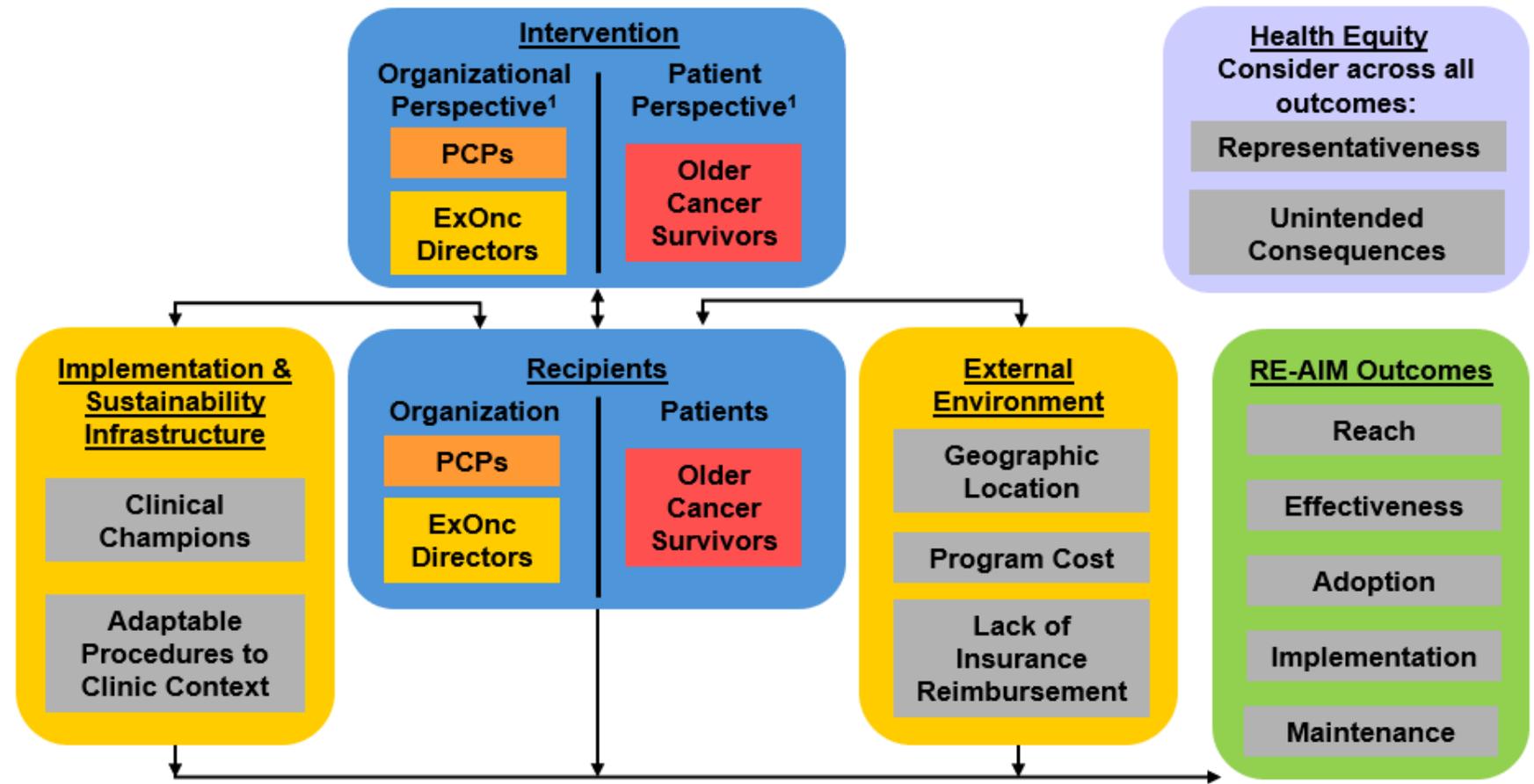


Figure 1. Conceptualization of the Practical, Robust Implementation Sustainability Framework  
Note: PCPs = primary care providers; ExOnc Directors = Exercise Oncology Program Directors;



# Target Populations and Recruitment

**Purposive sampling was used to recruit interview participants**

- Background
- Aims
- Methods**
- Results
- Conclusions

	<b>Older Cancer Survivors</b>	<b>Primary Care Providers</b>	<b>CaRES Directors</b>
<b>Description</b>	Adults ≥ 65 years who have received a cancer diagnosis	Physicians who care for older adults in their primary care practice	Individuals who direct or manage cancer-specific exercise programs
<b>Role</b>	Agree to referral and attend program	Place referral to exercise program	Receive referral and enroll participant



# In-depth Individual Interviews

Background

**Semi-structured interview guides were used to elicit perspectives and feedback on a referral workflow**

Aims

Methods



**Interviews were  $\leq 60$  minutes**



Results

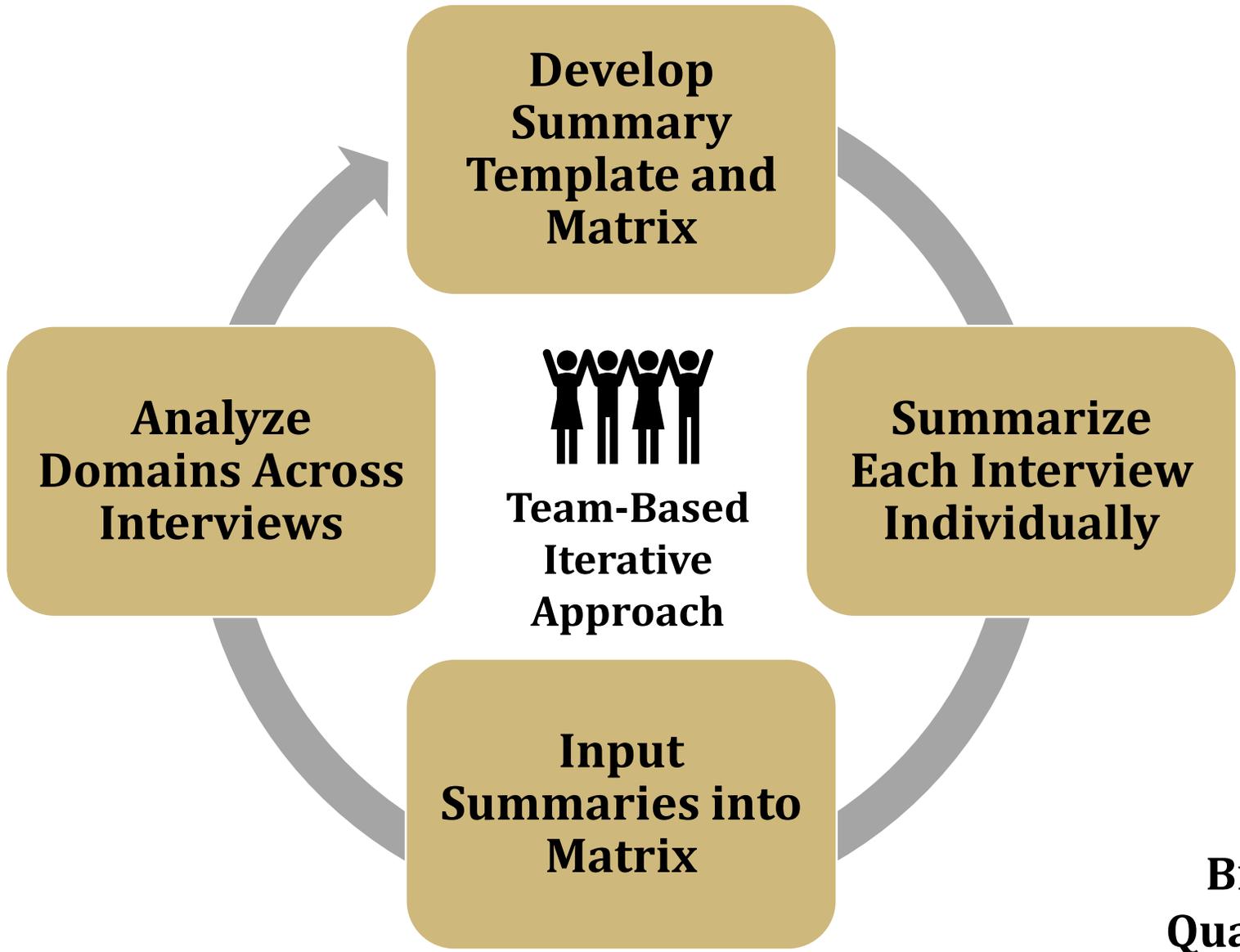
Conclusions

**Recorded, transcribed verbatim, and de-identified prior to analysis**



# Analysis

- Background
- Aims
- Methods**
- Results
- Conclusions

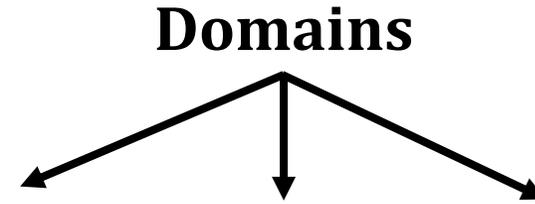


**Ashely Dafoe, MA**  
Qualitative Analyst



**Brooke Holliman, PhD**  
Qualitative Methodologist

# Example Matrix



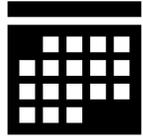
- Background
- Aims
- Methods**
- Results
- Conclusions

<b>Participant ID</b>	<b>Current Referral Processes</b>	<b>Potential PCP referrals</b>	<b>Determining Patient Eligibility</b>
PCP0			
Director1			
Patient2			
PCP3			



# Demographics

## Cancer Survivors (n=5)



Age Range:  
65 – 80 years



Cancer Types:

- Breast Cancer
- Colon Cancer
- Bladder
- Cardiac

## Primary Care Providers (n=6)



Clinic Types:

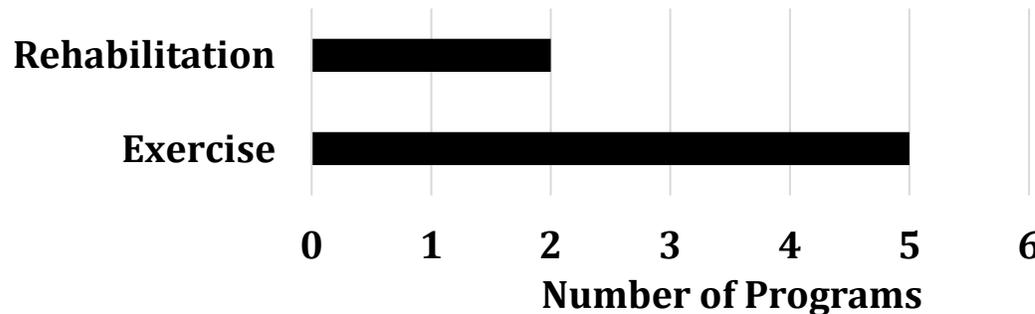
- General Internal Medicine
- Family Medicine
- Geriatrics



Time Practicing as a PCP:  
Average: 14.2 Years  
Range: 7 – 28 years

## CaRES Directors (n=7)

### CaRES Program Types



Background

Aims

Methods

Results

Conclusions



# Initial CaRES Referral Workflow

## Overview of Workflow

**Step 1:**  
Identify eligible patients

**Step 2:**  
Patient informed of CaRES

**Step 3:**  
Referral to CaRES

**Discussion with  
Primary Care Provider  
During Visit**

**Automated Messages to  
Patients**

**In After Visit Paperwork**

Background

Aims

Methods

Results

Conclusions



# Educating PCPs about CaRES

## Background

- PCPs and patients were not aware of CaRES programs

## Aims

- Initial education about CaRES for PCPs is needed

## Methods

- What are CaRES?
- What are the benefits of CaRES?
- Who is a good fit for CaRES?

## Results

- Education should be delivered through existing clinical communication channels
  - Clinic-wide meetings
  - Emails from clinic directors

## Conclusions

“First of all, you'd have to make sure that providers are aware of it... I wasn't aware of it. I was aware probably there was something, but it's not honestly on my radar to tell you the truth.” (PCP 3)



# Identifying Eligible Patients

Background

Aims

Methods

Results

Conclusions

- Information about cancer diagnoses are not always readily available in the EHR, especially for patient whose cancer care was in a different health system
- Eligibility process needs to be adapted to each clinics' workflow
- Patients wanted to be involved in considering their eligibility and readiness to participate

“I'd rather have a visit and talk to somebody than fill out a form.... Just because a question might pop into my head, and the questionnaire isn't gonna answer it.” (Patient 7)



# Informing Patients of CaRES

## Background

## Aims

## Methods

## Results

## Conclusions

- Directors, PCPs, and patients all highlighted the importance of a conversation during a visit
  - Personal connection
  - Opportunity to ask questions
- Both Directors and PCPs expressed that a resource with CaRES information would be beneficial
- A follow-up message after the visit may be a helpful reminder for patients

“my program, it's all about relationship building. With being able to add the primary care provider in person, being able to talk to them and understand like, "Hey, I saw you were diagnosed with this," it's a little bit more personal,” (Director 10)



# Referral

## Background

## Aims

## Methods

## Results

## Conclusions

- Current referrals to CaRES rely heavily on word of mouth
- PCPs and Directors felt both provider and self-referral options should be offered
  - Patients were concerned that they might not follow through on self-referrals
- PCPs preferred to place referrals in the electronic health record
  - May be challenging for community-based exercise programs

“I know that some individuals in speaking with their provider might not be ready in that moment, so being able to self-refer down the road or down the line, I think is valuable.” (Director 1)



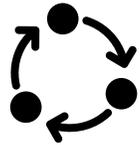
# Round 1 Conclusions

Background



Overall, CaRES referrals from primary care were acceptable across all three groups

Aims



However, PCPs already do so much the process must be seamlessly integrated to be feasible

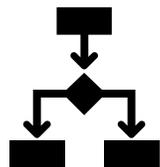
Methods

Results



Promoting awareness about CaRES and leveraging existing referral processes will be critical to implementation success

Conclusions



A clinical decision support tool is needed to help PCPs and patients navigate CaRES referral together



# Clinical Decision Support Tool

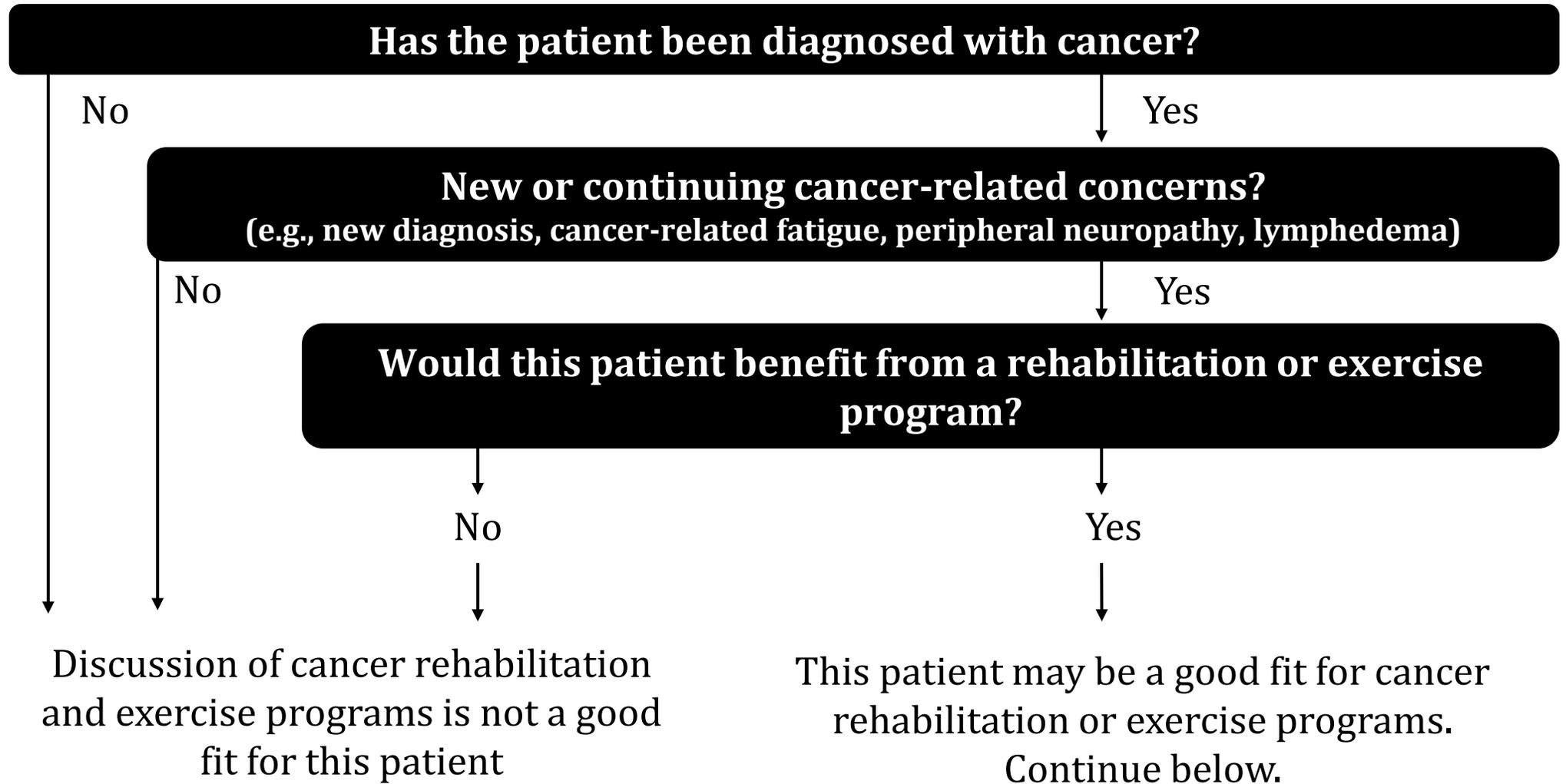
Background

Aims

Methods

Results

Conclusions



## Would the patient benefit more from rehabilitation or exercise?

### Rehabilitation is appropriate for:

- Poorly Controlled Lymphedema
- Moderate to severe neuropathy
- Moderate to severe hip/knee/back pain
- Substantial functional limitations

↓ Rehabilitation

**Is there a cancer rehabilitation program accessible to patient?**

Yes

No

Refer to a Cancer Rehab Program

Consider general PT/OT referral

### Exercise is appropriate for:

- Well-managed Lymphedema
- Mild neuropathy
- Fatigue
- Moderate functional limitations

↓ Exercise

**Is the patient meeting physical activity guidelines?**

Aerobic:  $\geq 30$  minutes of moderate physical activity 3x/week  
Strength training:  $\geq 2$  days per week

Yes

No

**Would the patient prefer to have professional support for exercise?**

No

Yes

Self-Management,  
No referral needed

Refer to an Exercise Oncology Program

# Feedback on the CDST

	Feedback	PCPs	Patients	Directors
Background	Clarify terms “moderate” and “substantial”	✓		✓
	Clarify term “professional support”	✓		
Aims	Remove jargon like “exercise oncology”	✓		
	Highlight psychosocial benefits of program		✓	✓
Methods	Unsure what counts as a “cancer-related concern”	✓		✓
	Ask patients if they have already participated in a cancer exercise or rehabilitation program		✓	
Results	Add a pathway to refer to an exercise program after completion of a rehabilitation program	✓	✓	
Conclusions	Educate on difference between exercise and rehab programs			✓
	Integrate tool into the electronic health record	✓		



# Round 2 Conclusions

Background



**Need to integrate the Clinical Decision Support Tool (CDST) into the EHR**

Aims



**Implementation of the CDST must be tailored to each clinic's individual workflow while retaining key functions**

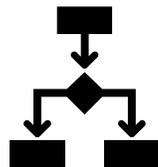
Methods

Results



**Need a supporting library of information about the CaRES programs available to a clinic's patient population**

Conclusions



**Next Steps: Create a high-fidelity prototype clinical decision support tool and prepare for EHR integration**



# Acknowledgements



**Amy Huebschmann, MD, MSc**  
**Primary Mentor**



**Brooke Holliman, PhD**  
**Qualitative Methodologist**



**Ashely Dafoe, MA**  
**Qualitative Analyst**

## **Additional Mentors:**

**Ryan Marker, DPT, PhD**  
**Russ Glasgow, PhD**  
**Elizabeth Bayliss, MD, MSPH**

**Romana Hasain-Wynia, PhD**  
**Mandy Allison, MAEd, MD, MSPH**

# Cancer Rehabilitation and Exercise Services (CaRES)

**Background**

**Cancer Rehabilitation**

**Cancer Exercise**

**Aims**

**Program Purpose**

Address a specific injury of condition

Improve overall fitness and physical function

**Methods**

**Participant Eligibility**

Complications of cancer treatment or substantial functional limitations

Moderate function limitations or deconditioning

**Results**

**Program Staff**

Physical and Occupational Therapists

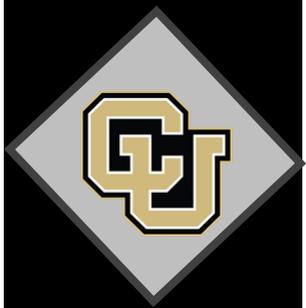
Cancer Exercise Trainers

**Conclusions**

**Program Delivery**

In-person

In-person or virtual



# Measuring Better: Outcome measures beyond epilepsy in Neurogenetic Conditions

Megan Abbott, MD

Assistant Professor, Pediatric Neurology  
and Epilepsy, University of Colorado

# Objectives

- Discuss challenges with epilepsy/seizures as the primary outcome measure in Neurogenetic Conditions
- Example of a novel clinical outcome assessment designed in CDKL5 Deficiency Disorder
- Walk through my current work modifying this scale for other DEEs
- Discuss the SCORE fellowship and impact on my work



## A little about me...

- Pediatric Epileptologist at Children's Colorado
- Medical Director of several gene specific clinics at Children's Colorado including SYNGAP1 Multidisciplinary clinic and SCN2A/SCN8A Multidisciplinary Clinic
- Clinical Researcher focussed on outcome measure/endpoint development in rare disease
- SCORE (Scholars in Clinical Outcomes Research) Fellow in my second year



# Challenges with current outcome measures for disease modifying clinical trials in rare disease:

- These are not epilepsy trials, therapies are aimed at improving disease overall, trial design is often longer (2-3 years)
- Need to specifically address top concerns noted by parents
- Need to be able to measure small but meaningful changes

In *SCN2A* the Vineland had limitations in showing growth or regression over time, with raw scores changing so slowly that standardized scores declined over time

- Lack of outcome measures specifically validated in Neurogenetic Conditions

Berg, AT, Palac, H, Wilkening, G, Zelko, F, Schust Meyer, L. *SCN2A*-Developmental and Epileptic Encephalopathies: Challenges to trial-readiness for non-seizure outcomes. *Epilepsia*. 2021; 62: 258–268. <https://doi.org/10.1111/epi.16750>



# Seizure frequency as primary outcome measure: potential problems in neurogenetic conditions

- Developmental delay precedes epilepsy much of the time, with patients achieving seizure remission at various time points
- Often seizure types are difficult to track (myoclonic, spasms), seizure frequency is inconsistent, many genetic conditions have mild/well controlled seizures

Rong M, Benke T, Zulfiqar Ali Q, Aledo-Serrano Á, Bayat A, Rossi A, Devinsky O, Qaiser F, Ali AS, Fasano A, Bassett AS, Andrade DM. Adult Phenotype of *SYNGAP1*-DEE. *Neurol Genet*. 2023 Nov 17;9(6):e200105. doi: 10.1212/NXG.0000000000200105. PMID: 38045990; PMCID: PMC10692795.

Vlaskamp DRM, Shaw BJ, Burgess R, Mei D, Montomoli M, Xie H, Myers CT, Bennett MF, XiangWei W, Williams D, Maas SM, Brooks AS, Mancini GMS, van de Laar IMBH, van Hagen JM, Ware TL, Webster RI, Malone S, Berkovic SF, Kalnins RM, Sicca F, Korenke GC, van Ravenswaaij-Arts CMA, Hildebrand MS, Mefford HC, Jiang Y, Guerrini R, Scheffer IE. *SYNGAP1* encephalopathy: A distinctive generalized developmental and epileptic encephalopathy. *Neurology*. 2019 Jan 8;92(2):e96-e107. doi: 10.1212/WNL.00000000000006729. Epub 2018 Dec 12. Erratum in: *Neurology*. 2019 Nov 12;93(20):908. doi: 10.1212/WNL.00000000000008352. PMID: 30541864; PMCID: PMC6340340.



# Patient Perspective: What should we measure?

- In *CDKL5* Deficiency Disorder, patients reported the following priorities in both a parent report and the natural history study:

<u>CDD PFDD Top Concerns</u>	<u>NHS 3 Top Concerns</u> (courtesy of Jeff Neul)
1. Global developmental delay	1. Epilepsy/Seizures
2. Epilepsy/seizures	2. Communication
3. Gastrointestinal and feeding problems	3. Sleep
4. Limited or absent speech	4. Lack of hand use
5. Behavioural disturbances	5. Abnormal walking/balance issues
6. Visual impairment	6. Constipation
7. Difficulty walking	7. Vision
8. Limited hand control	8. Teeth Grinding
9. Sleep	9. Repetitive hand movements
10. Scoliosis (curvature of the spine)	10. Poor weight gain



# Patient Perspective: What should we measure?

In a study of parents of children with Angelman Syndrome, parents identified their top priorities for a clinical trial to focus on<sup>5</sup>:

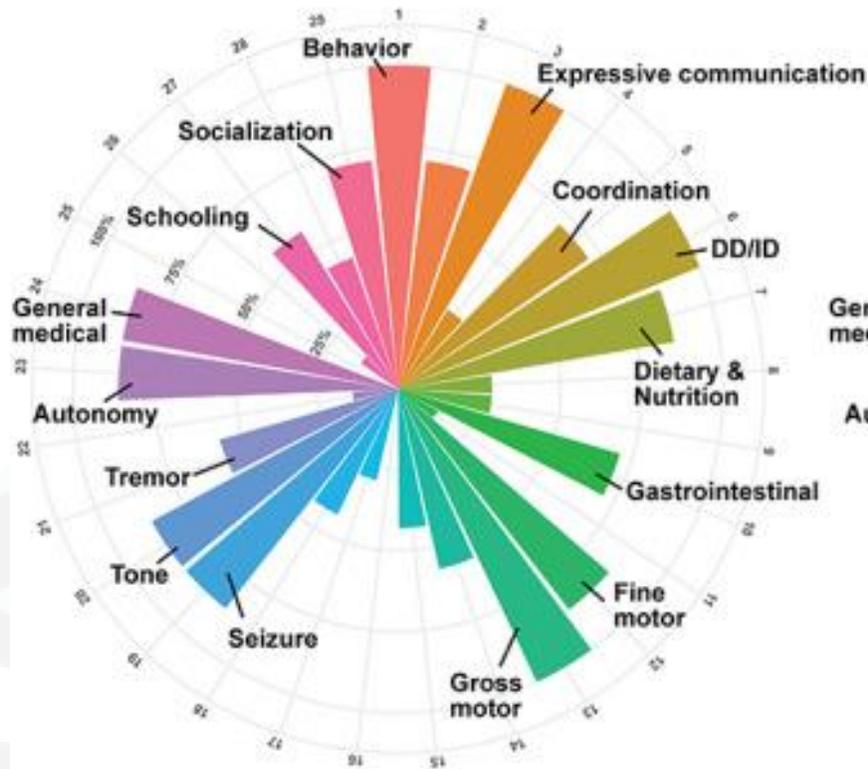
1. Neurology/Seizures/Epilepsy
2. Communication skills
3. Motor Skills
4. IQ/Cognitive function
5. Expressive speech
6. Sleep
7. Behavior

Adams, D, Roche, L, Heussler, H. Parent perceptions, beliefs, and fears around genetic treatments and cures for children with Angelman syndrome. *Am J Med Genet Part A*. 2020; 182A: 1716–1724. <https://doi.org/10.1002/ajmg.a.61631>

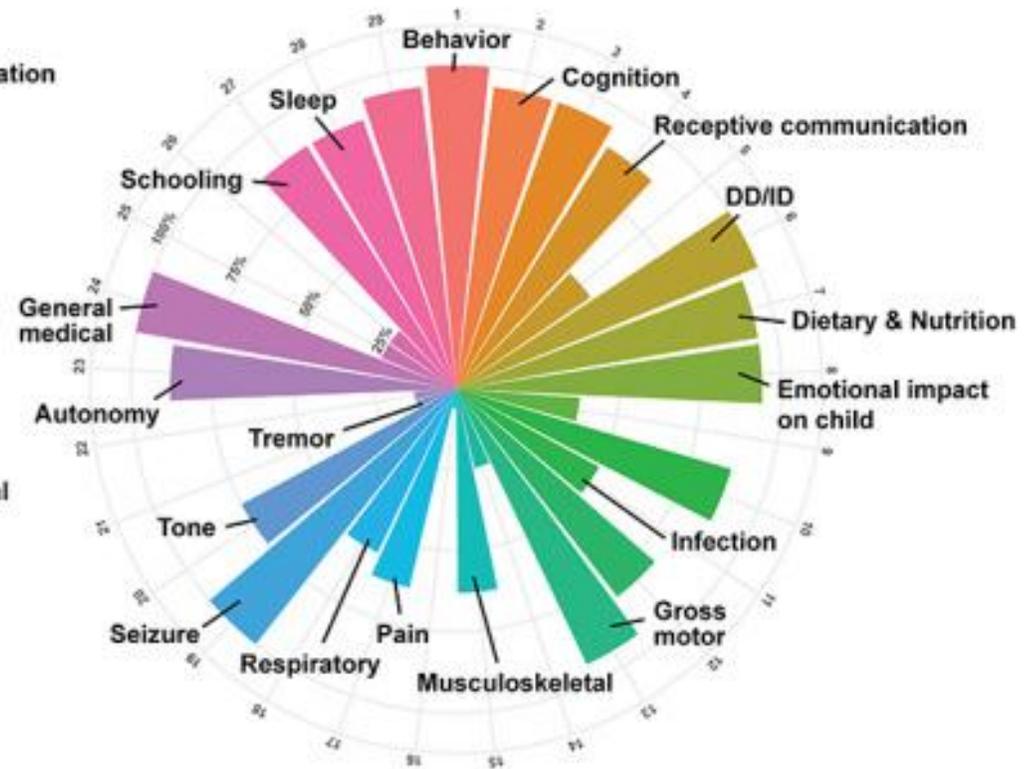


# STXBP1 Disease Concept Model

**A Healthcare providers (n=7)**



**B Caregivers (n=19)**



Sullivan, Katie R et al. "A disease concept model for STXBP1-related disorders." *Epilepsia open* vol. 8,2 (2023): 320-333. doi:10.1002/epi4.12688

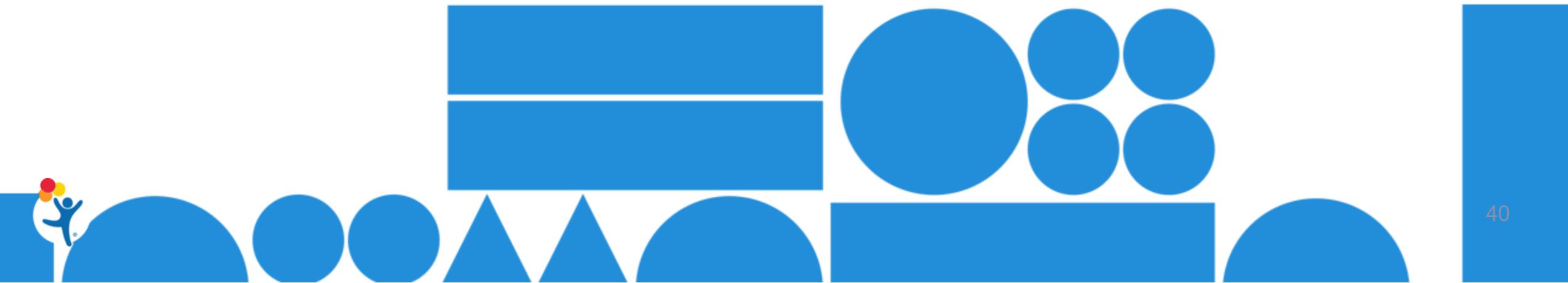


# Take aways:

- While epilepsy is a major concern, it is not the only concern
- Disease modifying therapies should be aimed at improving the majority of these concerns
- Will need outcome measures which consistently can measure these concerns:
  - Cognition
  - Speech/Motor
  - Sleep
  - Behavior
  - Vision



# Evaluating patients in the clinic: The Parent/Clinician CDKL5 Clinical Severity Assessment



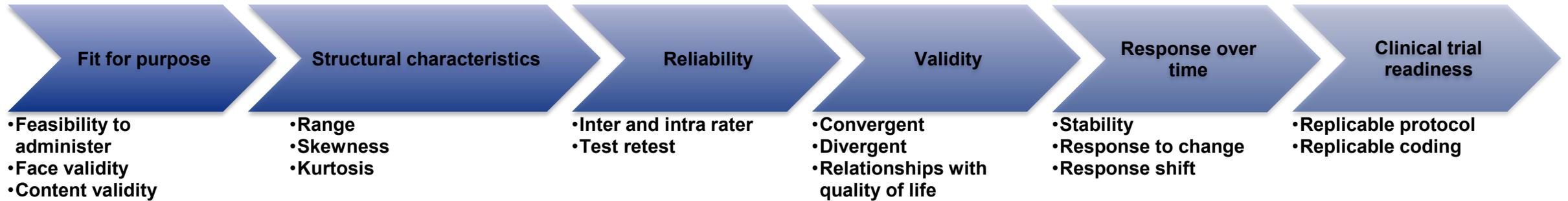
Multi-Site Validation of Biomarkers and Core Clinical Outcome Measures for Clinical Trials  
 Readiness in CDKL5 Deficiency Disorder (CDD): **NIH/NINDS U01NS114312**  
 (Feb2021-Jan2026) (Benke, PD)

## CDKL5 Outcome Measures Team



Affiliated with  
 University of Colorado  
 Anschutz Medical Campus

# Process of developing an outcome measure:



# Example of Motor CCSA Item:

## Gross Motor Function

- *You can ask a parent to show you these skills*
- *Ensure the child is tested for the duration of the activity*
- *Score on the highest level of assistance needed during the task*

### 3) (All ages) Head control in sitting - Support trunk in an upright position if required)

0, Full, active range of motion to look left or right

33.333, Limited range of motion to look left or right, lack of chin control

66.666, Can get head into alignment whether momentary or longer

100, No head control

### 4) (All ages) Supine lying to sitting

0, Moves from laying to sitting independently - good head and trunk control

20, Moves into sitting with some limitations - lag in head control, or uses own arm to assist

40, Minimal assistance to guide getting into sitting - a light touch, one hand held

60, Pull child into sitting, child holds head in line with body through a portion or all of the movement

80, Pull child into sitting, may momentarily tighten muscles through the neck but does not align head with body

100, No, minimal effort; full assist



Affiliated with

University of Colorado  
Anschutz Medical Campus

# Example of Communication CCSA Item:

## **21) (< 15 months) Speech (verbal)**

0, Purposeful vocalisation

50, Non purposeful vocalisation

100, No vocalization

## **22) (15-17 months) Speech (verbal)**

0, Single words - vocal - eg yes or no

33.333, Purposeful vocalisation or word approximation

66.666, Non purposeful vocalisation

100, No vocalization

## **23) (18 months+) Speech (verbal)**

0, Sentences or Phrases - 2 or more words - vocal, normal for age

20, Sentences or Phrases - 2 or more words - vocal, but not normal for age

40, Single words - vocal - eg yes or no

60, Purposeful vocalisation or word approximation

80, Non purposeful vocalisation

100, No vocalisation



Affiliated with

University of Colorado  
Anschutz Medical Campus

# Visual Components of CCSA score

1) Fixing and Following - *Follows in well-lit room, 10cm from examiner's face, without voice prompt.*

0- Fixes and follows examiner's face consistently

25- Fixes, occasionally/inconsistently follows examiner face

50- Fixes only, does not follow

75- Blinks to bright light (otoscope on maximum power)

100- Does not fix or follow

-

2) OKN - *OptOK app on ipad (minimum 10 inch) at full intensity in darkened room held 5-10 cm from the child's eyes for 30 seconds. Both directions tested.*

0- Normal OKN

33.33- Ignored OKN – notice then look away

66.66- Inconsistent OKN, Reduced OKN (movements present but reduced in amplitude, or loss of one visual field.

100- Very limited visual field. Absent OKN

3) Eye alignment

0 - Normal

50- Dysconjugate, intermittent

100- Dysconjugate, constant

Abnormal eye movements

4) Roving:

0- Not present

50- Intermittent

100- Persistent

5) Nystagmus

0- Not present

50- Intermittent

100- Persistent

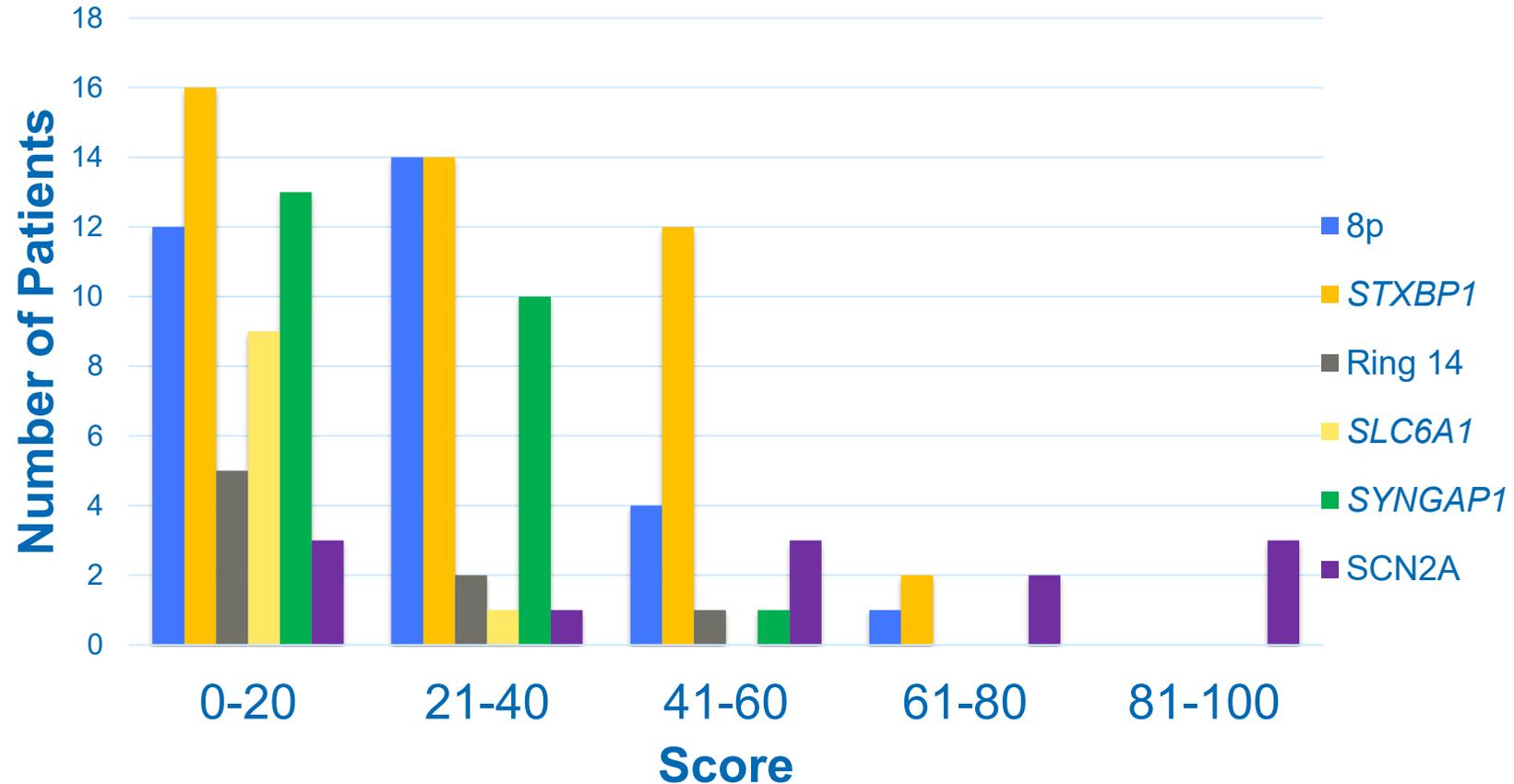


Affiliated with

University of Colorado  
Anschutz Medical Campus

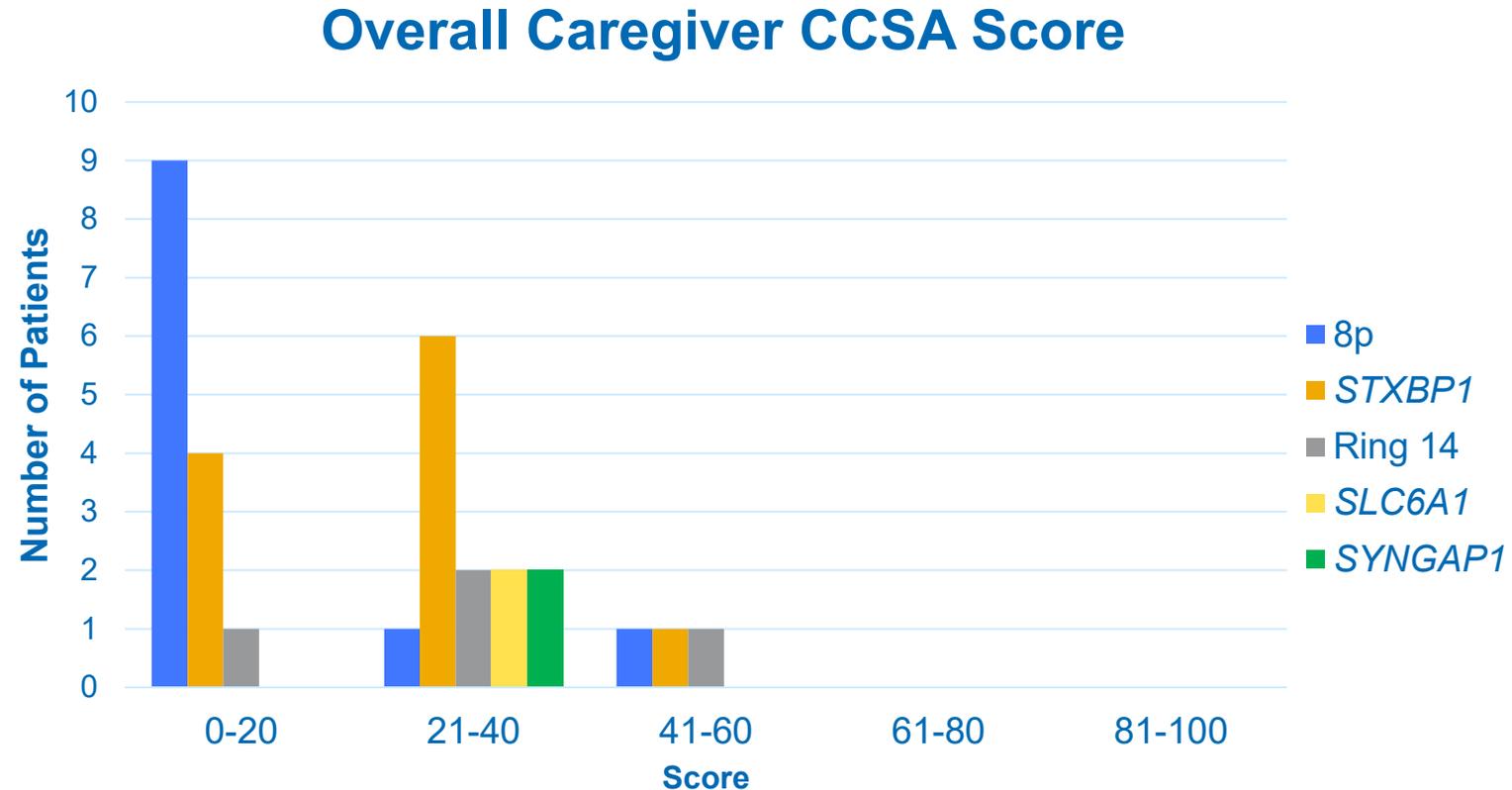
# Clinician Clinical Severity Assessment Scores:

## Overall Clinician CCSA Score



Affiliated with  
University of Colorado  
Anschutz Medical Campus

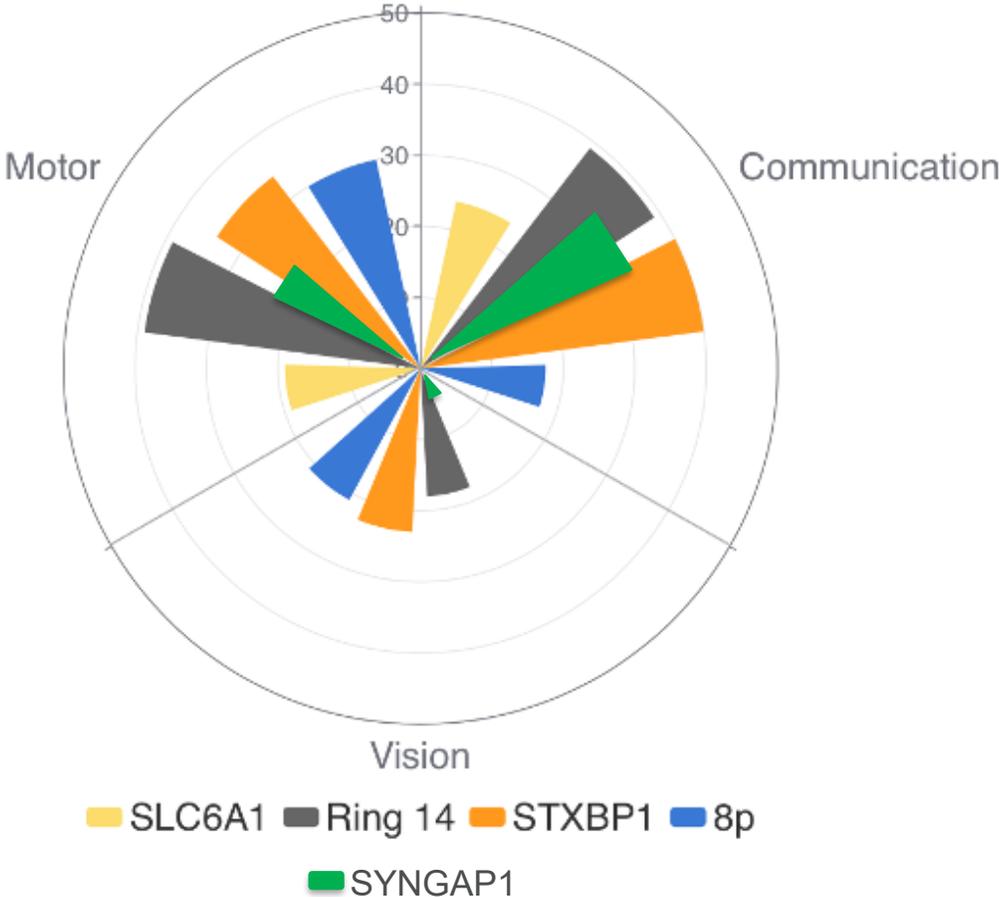
# Caregiver Clinical Severity Assessment Scores:



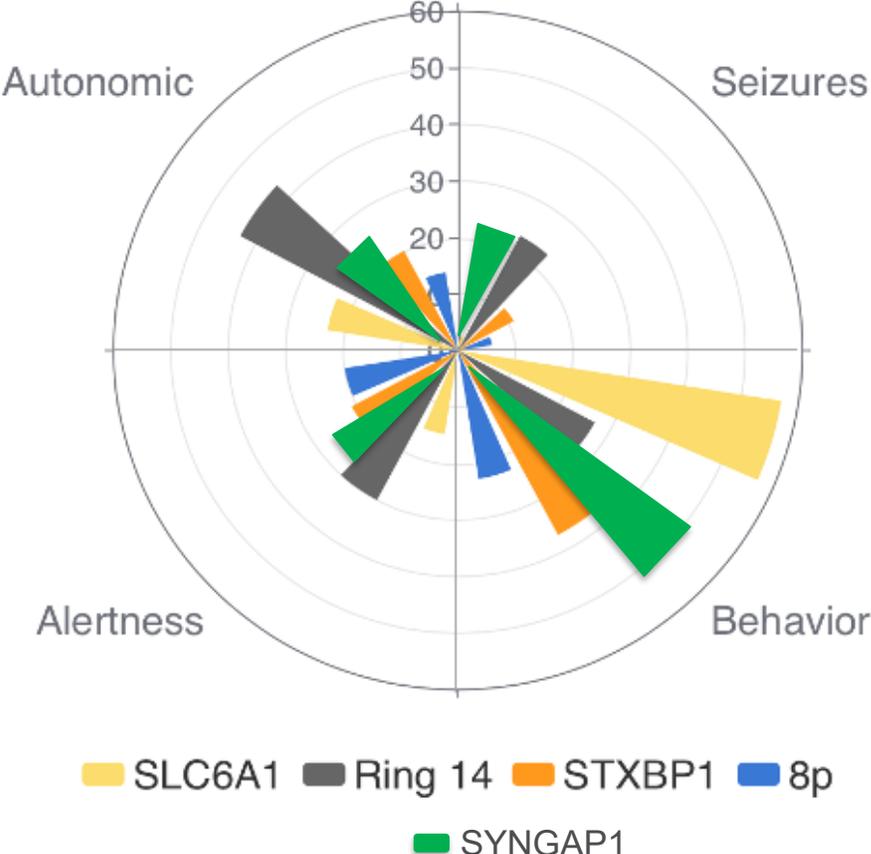
Affiliated with  
University of Colorado  
Anschutz Medical Campus

# Comparison of Scores Across Domains and Disorders

Clinician CCSA Domain Scores



Caregiver CCSA Domain Scores

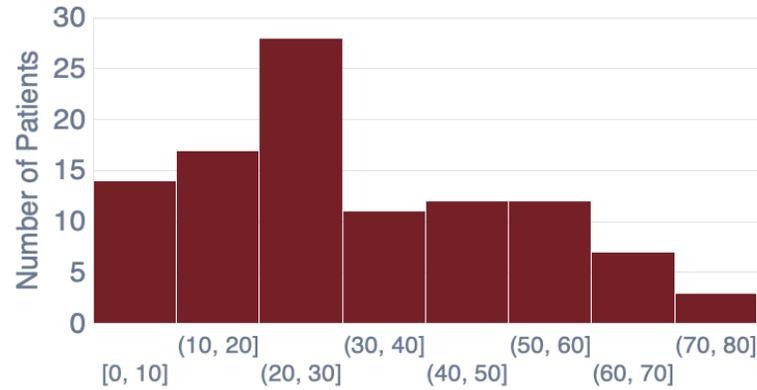


# Adapting CCSA for STXBP1...

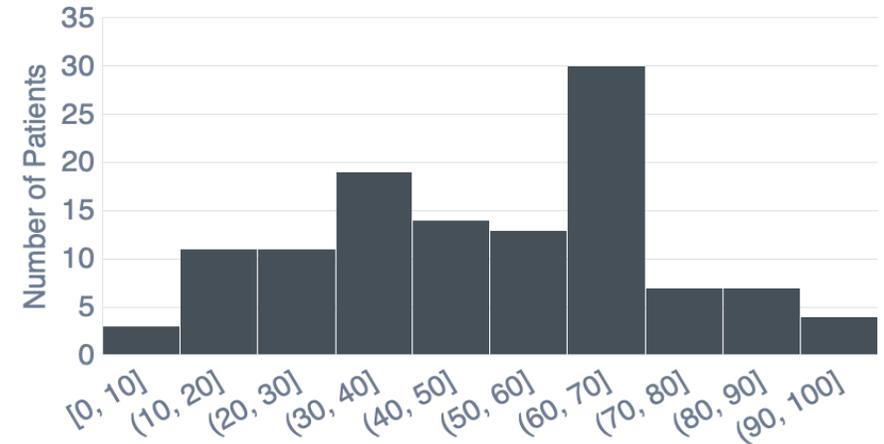


# Scores in 123 Patients with STXBP1

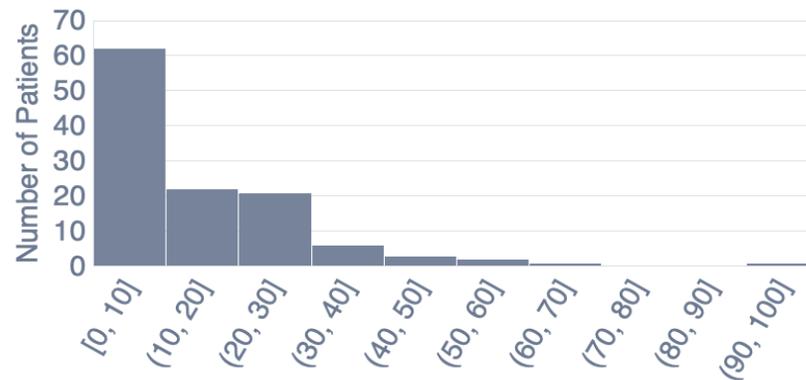
## Motor Domain Score



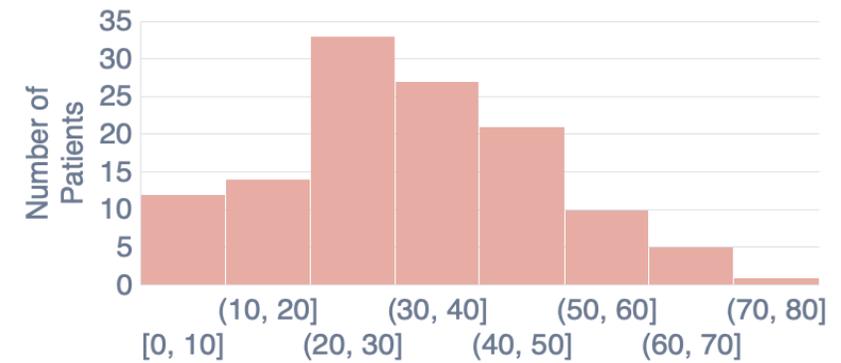
## Communication Domain Score

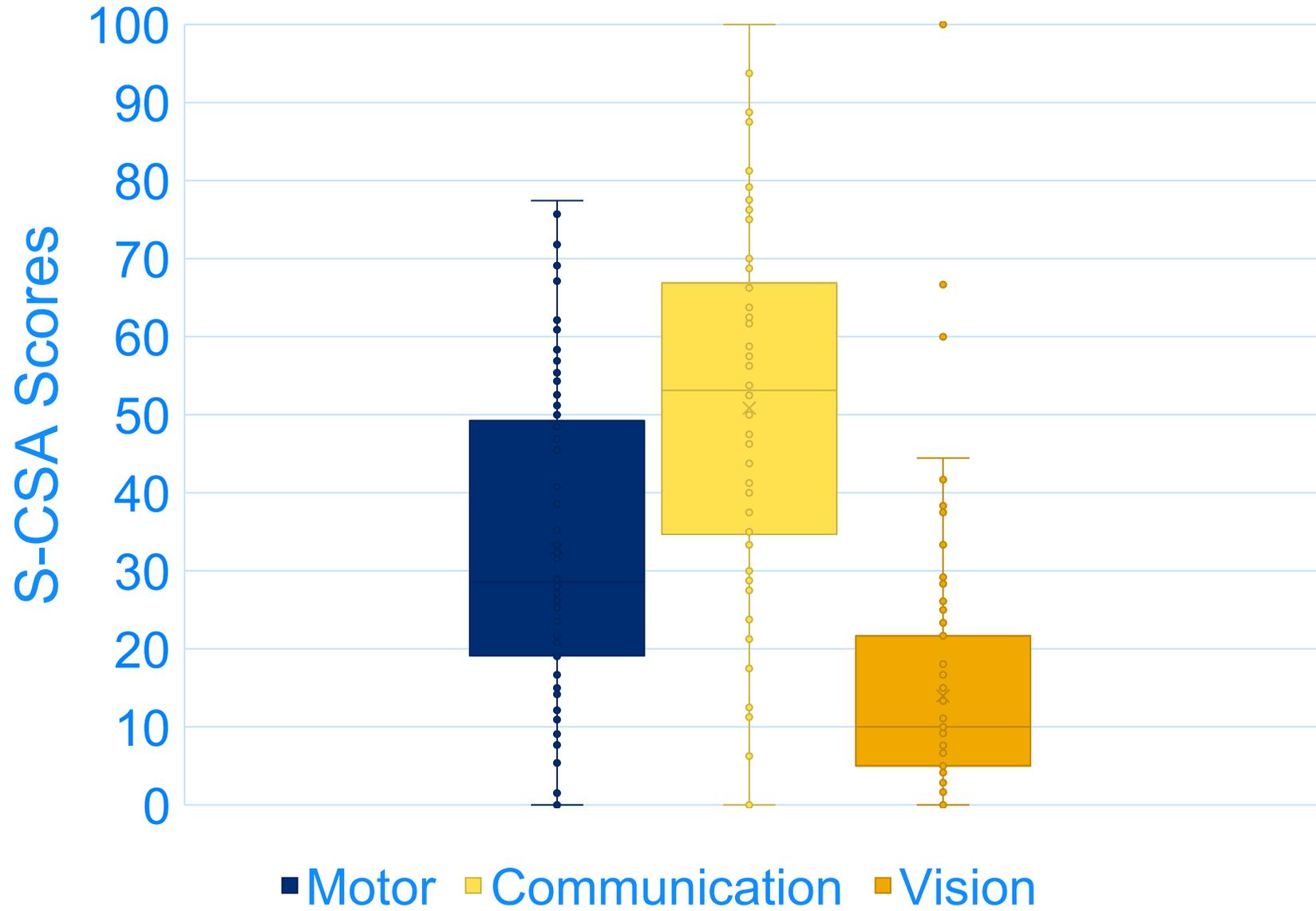


## Vision Domain Score



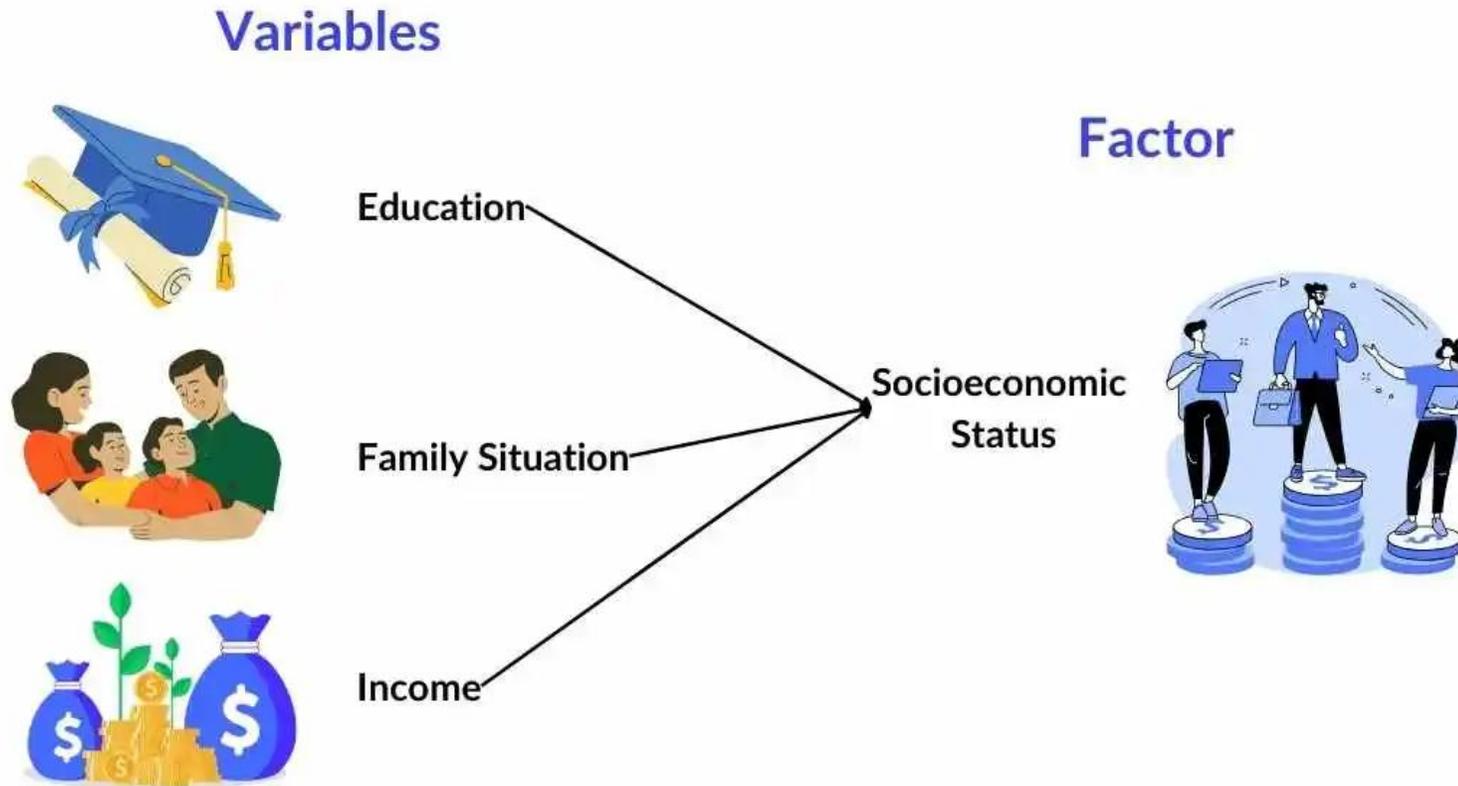
## Overall S-CSA Score





Affiliated with  
 University of Colorado  
 Anschutz Medical Campus

# Factor Analysis Explanation



# Factor Analysis

Summary of Psychometric Analyses & Accepted Thresholds					
Analysis Type	Description	Statistical Measure and Threshold	S-CSA Validation Values		
			Motor	Communication	Vision
Factor Analysis	Statistical method that finds patterns in test responses by grouping together items that seem to be measuring the same idea into factors	Factor Loading (>0.45)	>0.78	>0.66	>0.55
		RMSEA (<0.1)	0.095		
		Tucker-Lewis Index (>0.810)	0.975		
Internal Consistency	Indicates how well the questions on a test or survey all measure the same thing	Cronbach's Alpha (>0.630)	0.852	0.894	0.797
		Composite Reliability (>0.630)	0.958	0.880	0.865
Avg. Variance Extracted	Shows how much of the information in a group of items reflects the concept being measured, compared to random error	Average Variance Extracted (AVE) (>0.450)	0.820	0.648	0.576
Divergent Validity	Evaluates that a concept being measured is clearly different from other concepts.	Max Correlation Squared (<AVE + 10%)	0.517	0.517	0.349



## Results From Adaptation Process

- This clinician reported outcome measure demonstrates **a nice range of scores** in the three domains and overall score **without floor or ceiling effects**
- The initial way that items were grouped for CDKL5 **continues to work well** for STXBP1



# Overall take aways...

- Outcome measures beyond seizure frequency are necessary for future disease modifying clinical trials
- Standardized/ “off the shelf” measures have significant limitations in neurogenetic conditions
- Disease specific outcome measures may have applicability for conditions outside of what they were originally designed for



# My career development during SCORE fellowship...

## Career Development During SCORE Fellowship

### Research:

- Four first author publications on genetics & epilepsy
- Presenting author for AES and ILAE abstracts on severity assessment performance in other DEEs, manuscript now submitted
- Completing SCORE Qualitative Project on measure development

### Funding:

- Received 2 grants: NeuroNext Clinical Trials Research Fellowship, and Pediatric Epilepsy Research Foundation Bridge Grant
- Continued to be PI of several foundation grants focused on adaptation & pilot of severity assessment in specific DEEs (*SCN1A*, *SCN2A*)

### Clinical:

- *Clinical Assessment of Genetic Epilepsies*: Frequent participation in various neurogenetic multidisciplinary clinics
- *Outcome Measures*: Performed >100 severity assessments clinically in both CDD and other DEEs

### Leadership:

- Development & launch of the *SYNGAP1* and *SCN2A/8A* Multidisciplinary Clinics
- Serves on the Professional Advisory Board for the Epilepsy Foundation of Colorado



# Next Steps following SCORE:



Will continue to work on measure development and natural history study design in rare disease, am the PI on 4 current foundation grants and am a site PI/collaborator on several FDA R01 applications for natural history study development



I am applying for a K23, aiming for June submission



I am working on my Masters of Science in Clinical Science



# Thank you!

- To the wonderful patients and families I have the privilege of working with
- To my mentors and colleagues
- To the SCORE Fellowship and ACCORDS for excellent training and support



# ACCORDS Fellowship Opportunities

## Scholars in Clinical Outcomes Research (SCORE)

- The Scholars in Clinical Outcomes Research (SCORE) Fellowship at ACCORDS is currently recruiting for the July 2026 cohort.
- Fellowship designed for junior and mid-career faculty at the University of Colorado Anschutz or affiliate institution with goal of translational or outcomes research
- Two-year program consists of weekly didactic training, weekly work-in-progress sessions, 1:1 mentoring, and intensive grant writing course
- Pilot awards available for competitive applicants
- Applicants must have support of home department/division; at least 50% protected time, and tuition support for program costs
- **Applications due March 15<sup>th</sup>, 2026!**

## ACCORDS Primary Care Research Fellowship

- Goal: Train post-doctoral professionals to become primary care research leaders
- Nationally renowned training in Implementation Science
- Fellows receive funds for pilot project support, tuition, travel for conferences and a standard NIH-level T32 stipend (options to negotiate for additional salary support)
- Expert support for fellows from ACCORDS cores, including: Biostatistics, Health Economics, Implementation Science, Qualitative/Mixed Methods, and Shared Decision Making
- Apply tuition toward an advanced degree tailored to your background (Master's Degree and/or Graduate Certificate in Implementation Science)
- **Applications open and reviewed on a rolling basis!**

