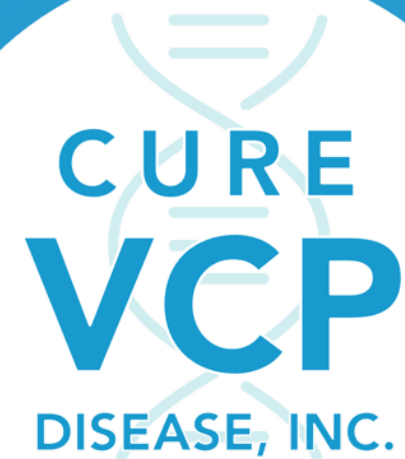


# Clinical Trial Readiness Programs

September 21, 2023



# We must use data to strengthen the patient voice

Patients and patient organizations require ownership of patient research data



Global Patient Registry  
CoRDS Registry at Sanford

**AIM: Global Patient  
Identification & Disease  
Understanding**

1. Used NIH Healthmeasures.net Surveys
2. Valuable insights to daily life of patient
3. Global
4. Used in the development of SOC and launching point for other natural histories



Retrospective  
Natural History Study

**AIM: Unlock the power of  
patient records for disease  
understanding and future  
clinical trials**

1. Partner with AllStripes
2. Patient Medical Records
3. Patient-Owned
4. US and Canada Only



Prospective  
Natural History Study

**AIM: Can we measure  
change in one year with  
existing measures?**

1. Partner with Alfano Lab at Nationwide Children's Hospital
2. Data Sharing Agreement
3. In-clinic and Remote with expert PT



Sensitivity of Measures  
Video Study

**AIM: Can we use videos for  
clinical endpoints?**

1. Partner with Emmes Endpoint Solutions
2. Data Sharing Agreement
3. Video Training with expert PT
4. Secure Submission
5. Video Scorecard

# How Cure VCP Disease assisted Emmes

Emmes is developing a custom Video Scorecard for VCP patients on 14 independence tasks



- Provided VCP patient insight & perspective on “independence” tasks and measure selection
- Funded project
- Encouraged patient participation
- Feedback on technology execution



Nov 2021

May 2022

Nov 2022

# Nationwide NHS Study Design

Leveraging outcomes established for similar diseases  
 - Limb-Girdle (GRASP)    - sIBM    - ALS

North Star Assessment for limb-girdle type dystrophies (NSAD)

Timed Up & Go (TUG)

On-site Only:

- 100-meter walk test

ALS-FRS

IBM-FRS

Global Health Scale

Neuro-QOL Upper & Lower Extremity Function

Neuro-QOL Cognitive Function

Upper Limb 2.0 (PUL)

Hand Grip Strength

9 Hole Peg Test

**+ Cognition Testing**

Forced Vital Capacity (FVC),

Forced Expiratory Volume in 1 (FEV1)



# Components of the NSAD and PUL 2.0

NSAD
1. Stand
2. Walk
3. Stand up from chair
4. Stand on one leg -Right
5. Stand on one leg -Left
6. Climb box step –Right
7. Climb box step -Left
8. Descend box step -Right
9. Descend box step -Left
10. Gets to sitting
11. Rise from floor
12. Lifts head
13. Stands on heels
14. Jump
15. Hop right leg
16. Hop left leg
17. Run (10m)

Rating Scale	
2	"Normal" - no obvious modification of activity
1	Modified method but achieves goal independent of physical assistance from another
0	Unable to achieve independently

PUL 2.0
1. Shoulder abduction both arms above head
2. Shoulder abduction to shoulder height
3. (No weight) Shoulder flexion to shoulder height
4. (500g) Shoulder flexion to shoulder height
5. (500g) Shoulder flexion above shoulder height
6. (1kg): Shoulder flexion above shoulder height
7. Hands to mouth
8. Hands lap to table
9. (100g ) Move weight on table
10. (500g) Move weight on a table
11. (1kg) Move weight on table
12. Lift heavy can diagonally
13. (three cans) Stack cans
14. (five cans) Stack cans
15. Remove lid
16. Tearing paper
17. Tracing path
18. Push on light
19. Supination
20. Pick up coins
20. Pick up coins
22. Pick up 10g finger pinch

Rating Scale	
2	Able
1	With Compensation
0	Unable

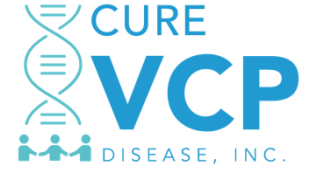
# How Cure VCP Disease assisted Nationwide

- Provided VCP patient insight & perspective on outcome measure selection
  - Hand grip, spirometry, lower & upper extremities
  - Heterogeneous disease progression
- Funded the project – Initial 12-month study + funded second year
- Encouraged patient participation – recruited 40+ participants
  - Enrolled 5 new patients & conducted measures at recent patient conference
- Purchased and assembled remote kit materials
- Mailed remote kits to patients
- Conducted technology and equipment introductory call
  - Establish familiarity with kit contents
  - Spirometry device setup
  - Phone vs. laptop setup
  - Space requirements
- Provided travel stipends to Columbus, Ohio



# Exciting results!

## Nationwide Natural History Study



- Finishing Year 2 now, added cognitive assessments
- Quantitative data and video evidence
- Meaningful endpoints have been identified!
- Will not need a third year!
- Publication by end of 2023
- Statistically insignificant between in-clinic & remote measurements
  - Standardization of equipment is key
- Useful for entire neuromuscular community



A close-up photograph of microscope objective lenses, with a blue color overlay. The lenses are labeled with magnification and numerical values: '4x', '10X/0.25', and '160/0.17'.

# Forward, Together.

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