

Patient advocacy collaboration in a remote natural history study for valosin-containing protein (VCP) associated multisystem proteinopathy (MSP)

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**MOTIVATION:** Prospective longitudinal natural history is urgently needed to establish clinical trial readiness for the ultra-rare VCP disease population.

**AIM 1:** Define the natural history of VCP disease

**AIM 2:** Validate data collected via remote versus in-person clinic methods.

**AIM 3:** Identify any divergent disease trajectories

METHODS

1. Partnered with Alfano Lab at Nationwide Children's Hospital to design and conduct functional measures study
2. Conducted phase 1 study with 5 diverse patients using tele-health methods
3. Integrated learnings and patient feedback into phase 2 protocol design

PATIENT ADVOCACY ROLE

1. Provided patient perspective in study design
2. Advertised study in the patient community
3. Assembled remote patient kits to standardize equipment for measurement
4. Provided patient prep and technology training meeting for remote study
5. Provided study funding and travel stipends to participants for travel to Columbus, OH

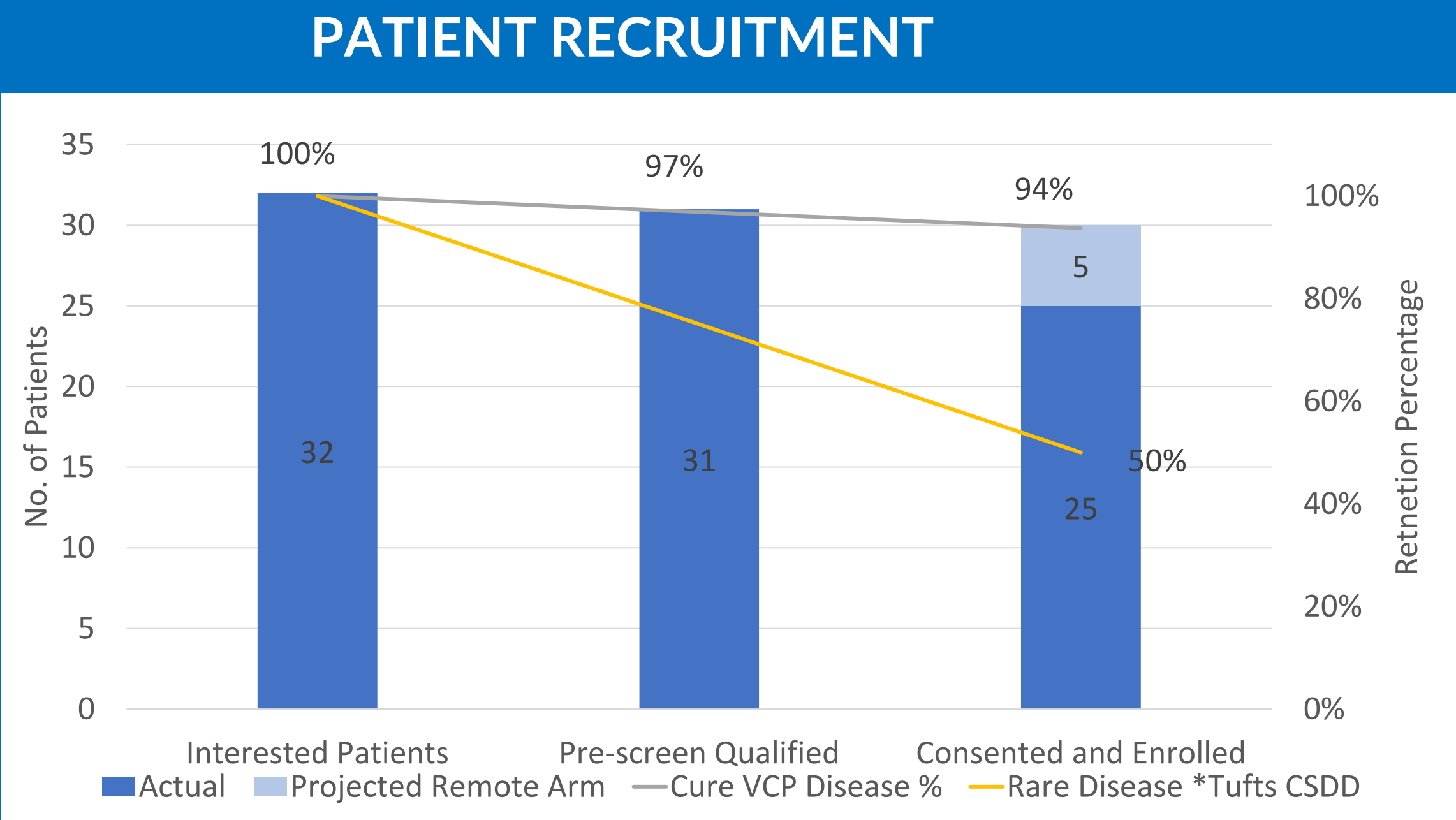
SPRING 2021 RESULTS

- Patient recruitment began at IRB approval
- Baseline remote & in-clinic visits launched 3 weeks from start
- Exceeded patient enrollment goal at 8 weeks
- Exploring expansion of study using remote only

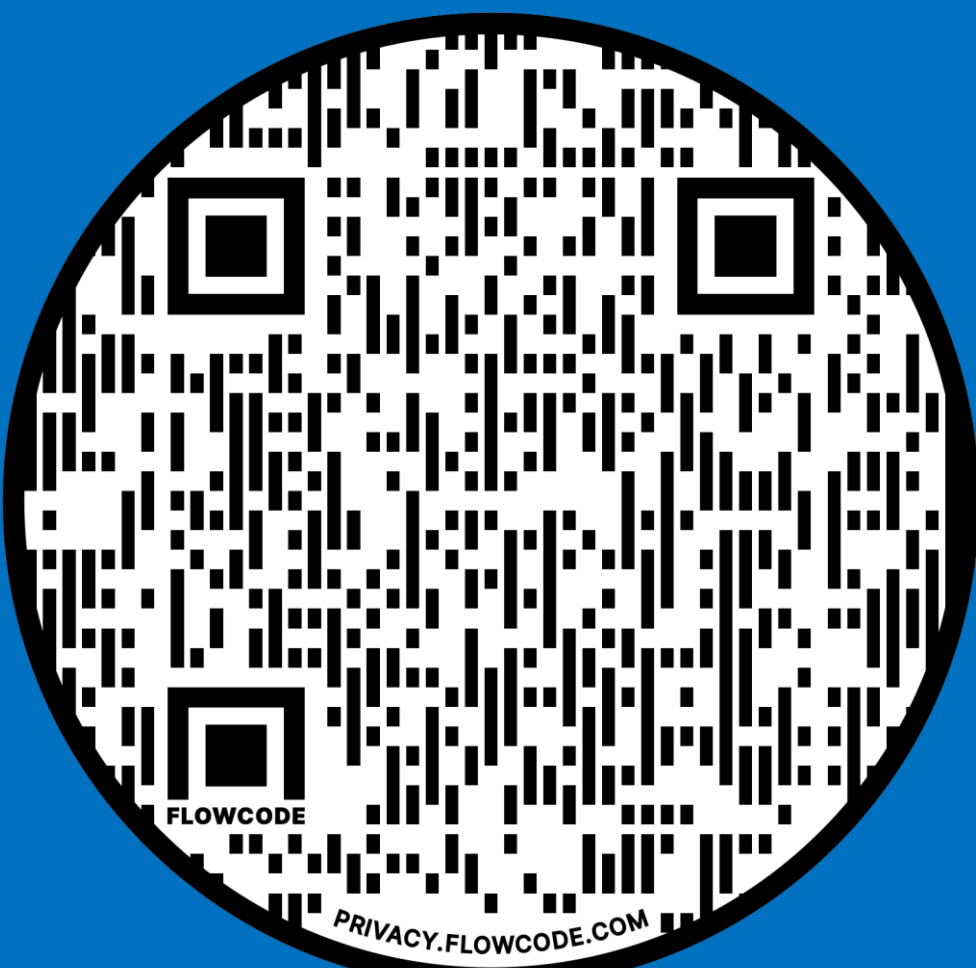
Successful collaboration between researchers and patient advocacy results in:

1. Effective Study Design
2. Integrated Patient Perspectives
3. Efficient Patient Recruitment and Study Startup

For remote & in-clinic natural history study of rare disease



Gaps in current research areas  
<https://www.centerwatch.com/articles/24543-recruitment-rates-rising-but-retention-rates-fall-according-to-new-study>



Take a picture to download the full abstract

PATIENT ENROLLMENT DETAILS (as of Sept 14, 2021)

| No. of Patients | Further Enrollment Details       |
|-----------------|----------------------------------|
| 24              | Consented and enrolled           |
|                 | Pending remote only study        |
| 4               | -Unwilling to travel             |
| 1               | -Pending scheduling              |
| 2               | Not interested or didn't qualify |

STUDY DESIGN & MILESTONES

PHASE 1  
AUG 2020 – NOV 2020

- Design – 2 months
- Study visits – 1 month
- Analysis – 1 month

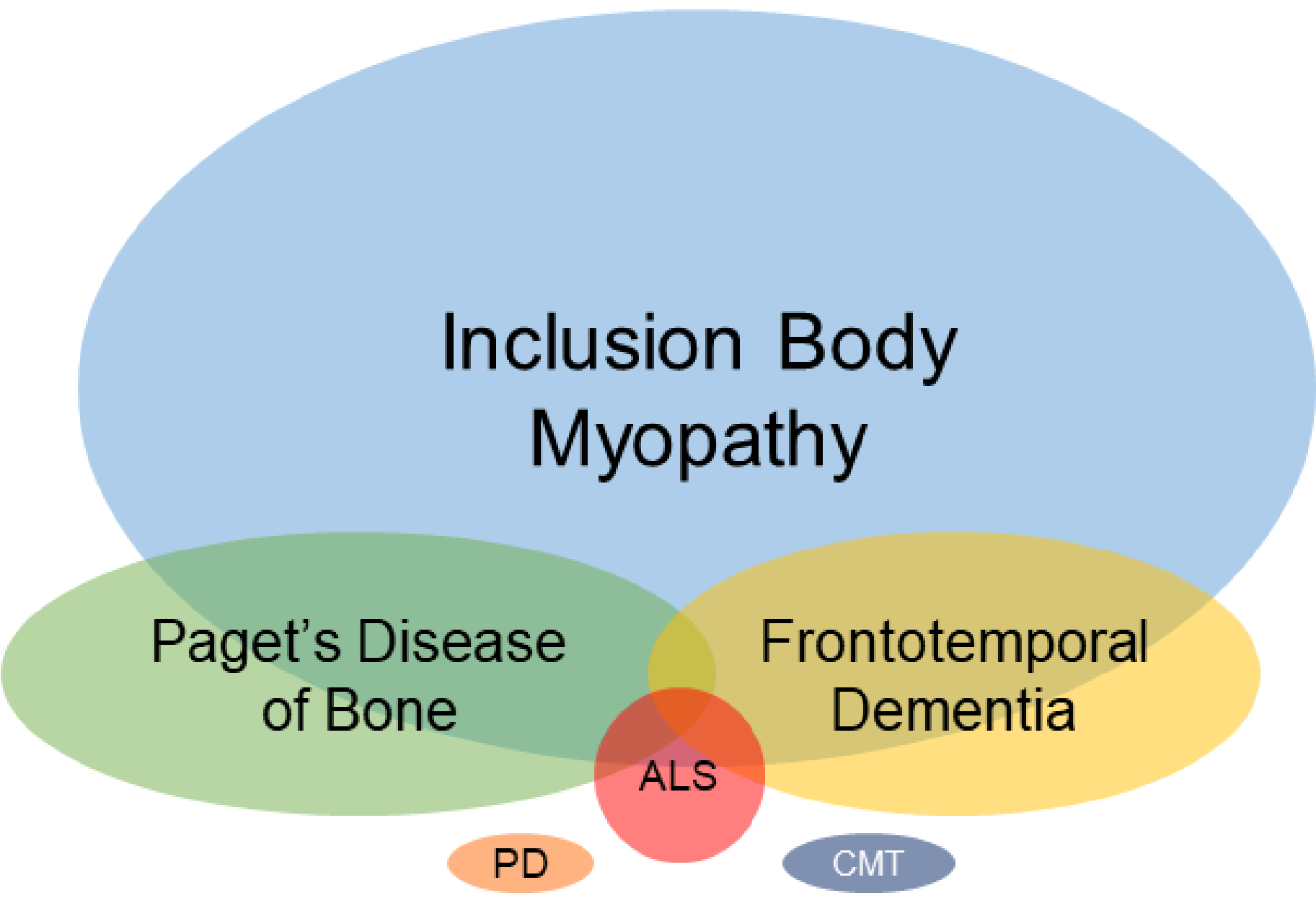
PHASE 2: Design  
DEC 2020 – MAR 2021

- Contract and IRB approval – 3 months

PHASE 2: Launch & Enrollment  
MAR 2021 – SEPT 2021

- 24 baseline remote & onsite visits completed by July 2021
- Six-month remote only visits scheduled to begin in October 2021
- Launching arm of study – remote only with no travel involved

Phenotypes of VCP associated multisystem proteinopathy (MSP)



Study Measures

UPPER EXTREMITY:

- Performance of Upper Limb 2.0 (PUL)
- Hand Grip Dynamometry
- Nine-Hole Peg Test
- ACTIVE WSV (in-clinic only)

LOWER EXTREMITY:

- North Star Assessment for limb-girdle type dystrophies (NSAD)
- Timed Up & Go (TUG)
- 100-meter walk (in-clinic only)
- 4-stair climb (in-clinic only)

SPIROMETRY:

- Forced Vital Capacity (FVC)
- Forced Expiratory Volume in 1 sec (FEV1)
- MIP/MEP (in-clinic only)

PATIENT REPORTED OUTCOMES:

- IBM-FRS
- Rasch Overall ALS Disability Scale (ROADS)
- PROMIS assessments
- Global Impression of disease severity
- Communicative Participation Item Bank
- Speech Handicap Index
- EAT-10

