The Chiari Surgical Success Scale (CSSS): Trust, Partnership & a Shared Vision of the Future

Background

The treatment of Chiari malformation (CM1) primarily relies upon surgical decompression of the cervicomedullary structures. Although CM1 is considered common, very little high-quality data exists allowing clinicians to confidently predict patient outcomes prior to treatment. Critical gaps in existing research and knowledge to provide that confidence include, but are not limited to: 1) the lack of a multi-center CM1-specific patient registry and 2) lack of understanding which clinical and radiographic factors predict surgical outcomes in CM1 patients. Previous efforts in this regard have been fragmented, relying mostly on single-center data or small patient registries focused primarily on more complex subpopulations.

There is a clear need for a reliable and valid Chiari Surgical Success Scale (CSSS) to identify the clinical and radiographic characteristics of CM1 and syringomyelia (SM) that can effectively predict patient outcomes. Measures such as this have a long history of assisting clinicians in successfully identifying key clinical parameters that predict patient outcomes for a wide variety of medical and surgical disorders. This will be the first scale that will use symptoms, clinical signs and medical imaging information to identify characteristics that might predict a good surgical outcome. The CSSS should help doctors, patients and loved ones face easier decisions when the time comes to consider surgery. It is also the first project in the project pipeline for the Chiari Clinical Research Consortium (CCRC).

Primary/Secondary Aims

Primary Aim: To develop and internally validate the Chiari Surgical Success Scale (CSSS), a prognostic model capable of identifying the probability that certain clinical and radiologic variables will predict a favorable response to surgery, defined by two simple outcomes questions, in patients with CM1.

Secondary Aims:

1. To determine the change in quality of life following surgery using the PROMIS-29 (adults) and PROMIS-25 (pediatric).
2. To determine the change in disability from headaches following surgery using the Migraine Disability Assessment (MIDAS) test (adults) and the PedMIDAS (pediatric).
3. To assess the complications from surgery in patients with CM1.
4. To evaluate any change in radiographic measurements at the final timepoint.

Methods

All consecutive surgical CM1 patients will be offered participation and followed for at least 1 year after surgery. Onboarding/enrollment is staggered, but each site is required to complete a 2-year enrollment period.

Because the presentation of CM1 is so disparate and dependent on individual characteristics, the inclusion criteria for this study as inclusive as possible to attempt to identify signals that may only be seen in large, multi-centered datasets. It is estimated that we will need at least 360 patients enrolled to complete our statistical analyses. All statistical analyses will be completed using Stata version 12 (StataCorp, College Station, Tx

Inclusion Criteria

- All individuals referred for CM surgical evaluation
- ≥5 years

Exclusion Criteria

- <5 years
- Not undergoing surgery (determine to not be a surgical Chiari case)
- Not undergoing surgery (other reason)
- All non-CM1 types (specifically defined as Chiari 2, Chiari 3, Chiari 4)*
- Chiari determined to be secondary (e.g., caused by CSF leak, craniosynostosis, increased intracranial pressure, brain tumor/other lesion, diagnosis of primary hydrocephalus, etc.)
- ·Prior cranial or spinal intradural neurosurgery
- Non-English speaking

*Chiari 0, Chiari 1.5, Chiari 0.5, etc. are purposefully *not excluded from this study*

The Chiari Surgical Success Scale (CSSS) is the first project of the Chiari Clinical Research Consortium (CCRC). Eighteen sites are involved, or becoming involved and over 1,200 participants have already been screened. This project will develop a practical tool that will improve neurosurgical care and patient outcomes. This project exemplifies what trust and true academic partnership with patient advocacy organizations is able to achieve.

Methods (cont.)

Data collection windows

- Baseline → 0-90 days prior to surgery
- Immediate Post-Op → within 7 days of surgery
- Initial Post-Op → 2-5 months after surgery
- Final Post-Op → 10-18 months after surgery

If a re-operation occurs before collection of the primary outcome questionnaire, additional data will be collected. At least the baseline and final follow up images will also be collected and added to the deidentified dataset for additional information.

Source Doc + CRF	Collected	Who Completes Survey?	Baseline	Post Operative	Initial Follow Up (optional study event)	Final Follow Up	At Time of Reoperation (if reop occurs)
Prescreening Survey	-Type of CM -Reasons for screen fail, if appl	PI/Coordinator	x				
Baseline Clinic Form	Collected during clinical encounter -Demographics/co-morbidities -Patient history -Headache matrix -Clinical exam findings	PI/Coordinator	x				
Imaging	Imaging findings taken from clinically appropriate brain, spinal MR scan	PI/Coordinator	x		x	x	x
Immediate Post-Operative Report	-Surgical date -Clinician reported surgical procedures and materials -Intraoperative findings	PI/Coordinator		x			x
Complications	-Clinician-reported surgical complications	PI/Coordinator			x	x	x
MIDAS	Self-reported headache disability	Participant (Adult)	x		x	x	
PedMIDAS	Self-reported headache disability	Participant (Pediatric)	x		x	x	
PROMIS29	Self-reported quality-of-life	Participant (Adult)	x		x	x	
PROMIS25	Self-reported quality-of-life	Participant (Pediatric)	x		x	x	
Primary Outcome Questionnaire	Improvement questions	Participant				x	

Preliminary Results

This study is still actively enrolling. As of this date, over **1,200 patients** have been screened. Of those, **232** were eligible and **203** have enrolled.

Only 40 participants have completed the study, so we cannot draw conclusions yet. However, we have been extremely pleased with the number of enrolling sites, the engagement with participants at each of those sites, and the impressive data completion we have achieved thusfar.

- 88% enrollment rate overall
- Only 2 sites are currently enrolling below 82%

A Novel Model

The Chiari Clinical Research Consortium (CCRC) was established with a goal of improving the quality of CM1 medical literature in the long-term. This model functions with each partner organization holding equal stake in the operations of the consortium.

The CSSS is simply the first project in a long pipeline of project proposals and exemplifies a sharp departure from the research models and partnerships that have been historically accepted.

Current Consortium members include:

- Univ. of Colorado
- Univ. of Alabama Birmigham
- Univ. of Michigan
- Univ. of Utah
- Washington Univ. St. Louis
- Wake Forest Univ.
- Texas Children's Hospital
- Johns Hopkins Univ.
- New York Univ.
- Metropolitan Neurosurg. Group
- Dayton Children's Hospital
- Rady Children's Hospital/UCSD
- Stanford Univ.
- Johns Hopkins All Children's
- Mayfield Clinic
- Macquarie Univ. (Australia)
- Brown Univ.

Future Directions

As this is the first project of the CCRC, further projects are being planned in the future. Becaus the organization functions as a true consortium, the final details of future projects are not yet finalized. Tentative plans include using the CSSS protocol as a baseline to expand out inclusion and exclusion criteria developing a large multi-center database as well as the implementation of machine learning and artificial intelligence to accelerate discovery and generate new hypotheses.

The first steps of these future directions are already underway. As any community-centered organization should, we are beginning with a needs assessment. We are scheduling structured and semi-structure interviews and surveys with diverse patients and caregivers living with these disorders to best direct how those future iterations are developed.