

“Optimizing dysphagia assessments using MBSImP in adults with nephropathic cystinosis”

Progress Report: January 2023

Executive Overview

As of May 2021, we completed MBSImP analysis of all 60 swallow studies collected during our previous clinical trial readiness study. Our findings were presented at the Muscle Study Group (MSG) Annual Scientific Meeting in October 2021 and published in *Muscle and Nerve* in June 2022.

The personnel have remained the same throughout the study, with Dr. Reza Seyedsadjadi as the principal investigator, Dr. Florian Eichler as the co-investigator, Stacey Sullivan as the speech pathologist, and Natalie Grant as the study coordinator.

Aim 1: Characterize oral, lingual and pharyngeal swallowing physiology for future treatment development. (a) Retrospective analysis of 60 video swallowing studies using MBSImP analysis (b) isolate specific muscles impacting swallow function as potential treatment targets for strengthening (c) Correlate these measures to other clinical and patient-reported outcome measures.

In our previous clinical trial readiness study, we collected video fluoroscopic swallow studies in 20 patients at 3 timepoints. The Penetration-Aspiration-Scale (PAS) was used to characterize dysphagia during the initial clinical trial readiness study and did not prove sensitive enough to support identification of possible interventions to improve function. Therefore, in the current study, Stacey Sullivan retrospectively reviewed these swallow studies using the MBSImP, a validated tool used to score swallowing across 17 physiologic domains. Use of this tool allows for a more specific characterization of oropharyngeal dysphagia in terms of exact muscles implicated in dysphagia, allowing for improved identification of specific treatment targets.

Our findings highlight a range of physiological changes in both the oral and pharyngeal phases of swallowing. Specifically, analysis in $\geq 50\%$ of the patients demonstrated difficulties of tongue control during bolus hold, reduced lingual motion, presence of oral residue, reduced laryngeal elevation, reduced tongue base retraction, and presence of pharyngeal residue. These findings highlight oral tongue, base of tongue, pharyngeal, and laryngeal weakness impacting swallow safety and efficiency. Targeted treatments aimed to improve function of these muscle groups individually or in tandem is of interest for future studies.

There were significant correlations between the oral total score and two patient-reported dysphagia outcome measures, the 10-Item Eating Assessment Tool (EAT-10) and the M.D. Anderson Dysphagia Inventory (MDADI). There was also a significant correlation between the pharyngeal total score and the EAT-10. We did not observe any significant changes in swallowing function over the one-year period.

Key milestones:

- 12/10/2020: Notified of grant approval
- 2/3/2021: IRB approval obtained to apply MBSImp analysis to the 60 swallow studies collected in the previous study
- 5/5/2021: Stacey completes initial analysis of all 60 swallow studies
- 7/1/2021: Submitted abstract to Muscle Study Group (MSG) for presentation at the Annual Scientific Meeting. Abstract title: “Longitudinal dysphagia assessment in patients with cystinosis using MBSImp”
- 8/2/2021: Abstract accepted for presentation at MSG
- 9/23/2021: Manuscript submitted To Muscle and Nerve
- 10/3/2021: Findings presented at MSG Annual Scientific Meeting
- 11/24/2021: Reviewer comments received from Muscle and Nerve
- 5/20/2022: Manuscript accepted for publication
- 6/26/2022: Manuscript published in Muscle and Nerve (*Sullivan S, Grant N, Hammond C, David WS, Eichler F, Sadjadi R. Longitudinal dysphagia assessment in adult patients with nephropathic cystinosis using the Modified Barium Swallow Impairment Profile. Muscle Nerve. 2022 Aug;66(2):223-226. doi: 10.1002/mus.27642.*)