

**FD ID:** 1988**Requestor Name:** Richard Barohn; Xing Song**Requestor Institution:** University of Missouri**Project Title:** No More DROOLing - COmpariNg TReatments for SialOrrhea in people with ALS (DROOL-CONTROL)

**Background/Rationale:** Amyotrophic lateral sclerosis or ALS is a rapidly progressive and invariable fatal disease affecting motor nerves in the brain and spinal cord. ALS is a rare disease. Difficulty swallowing can lead to problems managing saliva and excessive pooling of saliva (sialorrhea) in patients with ALS. The excessive drooling/pooling of saliva (ED/PS) is not due to excessive saliva production but is the result of orofacial or lingual muscle weakness or the loss of the ability to swallow (dysphagia). This symptom is both a social and medical burden to the patient and their family and pooling of saliva can lead to choking episodes which can cause aspiration pneumonia. More importantly, it has been shown that Non-Invasive Ventilation (NIV) could significantly prolong survival and improve quality of life, while ED/PS has been identified as one of the key barriers that hinder the tolerance of NIV. While there are multiple standard-of-care (SOC) medications for managing saliva in ALS, there is a severe lack of high-quality evidence on the comparative effectiveness of these medications. To our knowledge, there are no studies that identify the most effective medication for managing ED/PS for ALS patients who have not yet reached the intractable stage.

**Specific Aims:** We propose a comparative effectiveness study (CER) to achieve the following specific aims:

Aim 1: To determine the effectiveness of the four SOC medications (scopolamine patch, glycopyrrolate, amitriptyline and atropine 1% sublingual drops) in controlling ED/PS.

Aim 2: To determine the tolerability of each of the four SOC medications for ED/PS in patients using clinical and patient-generated data (PGD).

Aim 3: To investigate the heterogenous treatment effect (HTE) of the four SOC medications for ED/PS and side effect profiles for subgroups of patients with ALS (bulbar onset versus limb onset, veteran vs. non-veteran, rural vs. urban, men vs. women, etc).

**Intervention (if applicable):** Four SOC medications for controlling ED/PS: scopolamine patch, glycopyrrolate, amitriptyline and atropine 1% sublingual drops

**Collaborators:** We will select 15 ALS or MDA clinics at PCORnet sites and 5 VA sites