

## ADAPT: Acthar in Dermatomyositis and Polymyositis Treatment Registry

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**Background:** Despite a wide variety of medications used to treat the inflammatory myopathies, including corticosteroids, immunosuppressive agents, intravenous gammaglobulin, and other biologics, the only FDA approved medications for polymyositis (PM) and dermatomyositis (DM) are corticosteroids and Acthar gel, a highly purified extract of porcine pituitary that contains adrenocorticotrophic hormone (ACTH)(1). ACTH is known to promote the release of endogenous corticosteroids as well as interacting at melanocortin (MSH) receptors. MSH receptors are comprised of five subtypes, four of which have distinct anti-inflammatory mechanisms distinct from steroidogenesis (2-5). Acthar, however, has not been widely studied in the treatment of PM/DM. One recent small retrospective case series suggested that Acthar may have utility in refractory PM/DM patients (6). Therefore, the purpose of this study is to better understand the patient characteristics that lead physicians to prescribe Acthar and the patient predictors of a beneficial response to Acthar.

**Objectives:** The goal of this project is to create a registry that will allow physicians to understand the clinical characteristics and outcomes of myositis patients treated with Acthar. Based on this goal our specific aims are as follows:

- 1. To create and maintain a registry linking clinical information with diagnosis, dosing, safety and clinical response to treatment with Acthar.*
- 2. To determine which patient characteristics lead physicians to prescribe Acthar to patients with myositis.*
- 3. To determine if sub-types of myositis, classified through muscle biopsy pathology, may predict response to Acthar.*

**Methods:** This is a non-interventional study collecting retrospective and prospective data on consented patients who are prescribed Acthar for PM or DM. Clinical exams, electrodiagnostic testing, muscle biopsies, and other diagnostic information that was collected or generated as part of routine clinical care, will be collected and analyzed. Follow up evaluations will be assessed by an independent adjudication committee to determine if patients had a beneficial response to therapy

**Results:** Data will be collected for 24 months and will be analyzed through exploratory models to see if there is evidence for a treatment effect with Acthar. Safety measures will be captured as well. If a beneficial effect is seen, then the data will be analyzed

to see if there are specific factors which predict a higher likelihood of response.

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