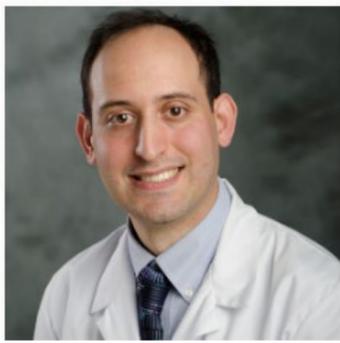


Using Steroids to Improve Outcomes in Rhabdomyolysis in Pediatric Patients



Natasha Shur, MD
Attending Clinical Geneticist



Seth Berger, MD
Attending Clinical Geneticist



Sophia Wu, MS3
Medical Student



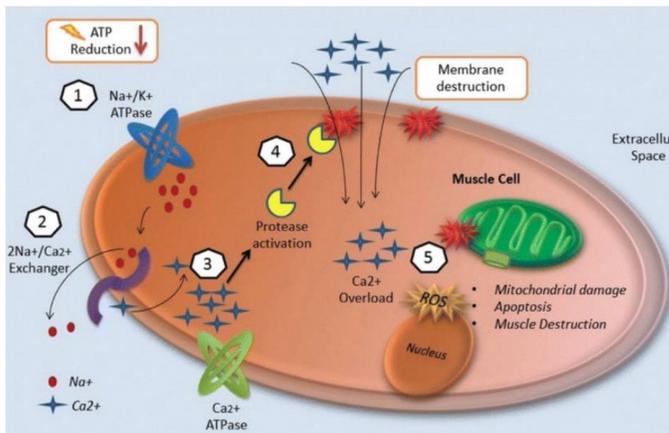
Nathan Oh
Research Assistant

PROPOSED TREATMENT

Using the steroid dexamethasone in conjunction with standard therapy of fluids and supportive care to improve clinical care practices and guidelines.

Rhabdomyolysis is a complex medical condition involving excess inflammation that leads to muscle breakdown. While steroids have been used off-label to treat rhabdomyolysis on a case-by-case basis, no formal clinical trials investigating steroids in rhabdomyolysis have been conducted. This lack of published clinical trial data has resulted in hesitation by clinicians to treat patients with steroids and a lack of standard clinical recommendations for steroid use, such as dose, efficacy, and when to initiate.

Dexamethasone was first approved by the FDA in 1958 and is commonly used to treat asthma. It has also been used to treat a variety of inflammatory conditions as well as other conditions, including severe COVID-19. In rhabdomyolysis, dexamethasone's anti-inflammatory effect could help reduce the symptoms, improve recovery rate, and decrease serious complications such as acute kidney injury.



<https://doi.org/10.1002/ajmg.a.62000>

Our clinical trial will evaluate and characterize the tolerability and clinical effectiveness of dexamethasone in improving the standard care for rhabdomyolysis.

SUMMARY STATEMENT

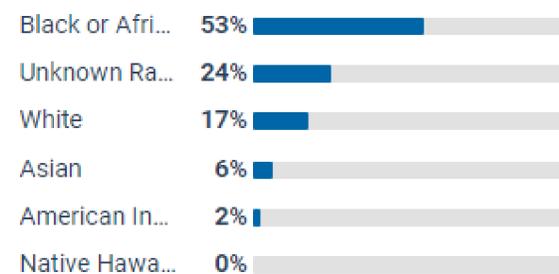
Adding dexamethasone to current therapy to improve standard care for children, adolescent and young adult patients with rhabdomyolysis

DISEASE/CONDITION

Rhabdomyolysis is a syndrome common in adolescents and adults characterized by muscle breakdown and the release of muscle components into the blood. Clinically, rhabdomyolysis is exhibited by muscle aches, weakness, and muscle breakdown. Patients are also at risk for kidney injury, acute liver failure, and subsequent multiorgan failure.

This disease affects minority populations at significantly higher rates than other populations nationally.

Race



CURRENT TREATMENT

Standard care of rhabdomyolysis is largely supportive care with fluids, rest and symptom management, with the goal of preventing acute kidney injury. However, there is no active treatment or cure to date.

Steroids are used in inflammatory neuromuscular disorders, and supplementary steroid treatment in rhabdomyolysis has shown some off-label success.

PROJECT

A single center, open label, prospective, randomized study for rhabdomyolysis patients age 0-21, comparing dexamethasone added to standard care versus standard care alone.

Patients will be randomized to dexamethasone for 5 days in addition to standard care or to standard care alone. Patients will be evaluated clinically at baseline before starting treatment and at the end of the study period, for a total of 2 telemedicine visits. Enrolled patients will be surveyed on symptoms, clinical course, and their experience.

We expect to obtain new knowledge that potentially improves existing practice and fills important gaps in understanding this understudied condition. We believe that this pilot study will be easy to expand to a multi-site follow-on study, eventually allowing dexamethasone to be incorporated into rhabdomyolysis standard care.



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