

**Lay summary of research project grant application**

Project title	<b>IMPROVEMENT (improving the outcome in myositis spectrum diseases: core set variables harmonization and use from children to adulthood) project</b>
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**1. Objective of the project**

To harness the international strength of worldwide myositis centres to collect biological and quality of life information widely across the globe, which will increase the number of myositis-related adult and childhood cases that are studied. Collecting such information, over time, will help clinicians better recognise and treat these diseases which is anticipated to result in increased survival and decreased disability – while very importantly learning about the unmet needs of patients which is anticipated to improve their quality of life.

**2. Background**

Myositis is an inflammation of the muscles that causes weakness. Patients affected by myositis often cope with additional major health problems related to myositis, such as disease of the lungs, joints and skin, hence the term “myositis spectrum disorders” (MSD). The onset of myositis and MSD can be very variable and be very difficult to diagnose – such as when patients lack obvious muscle involvement, and may have other features to start off with such as arthritis or lung disease, so the risk of making the wrong diagnosis can be high. The myositis expert community recognises that we have much to learn and to teach about the different ways the diseases present (in adulthood and in childhood), the blood tests related to the diagnoses and the best treatment options to improve survival and increase quality of life, with special attention to the vulnerable period of chronic illness when the teenage patient is transitioning to an adult patient.

**3. Methods and approach**

A group of physician experts in MSD (rheumatology, neurology and pulmonary disease specialists) and patients, will work to identify an essential group of tests and questionnaires that are most important for all aspects of MSD (including muscle, joints, lung, skin as well as psychosocial impact and patient perceived needs). This will be accomplished by first reviewing all the relevant literature and all the data from existing registries. Once this is done, we will get agreement from experts and patients on the final group tests and questionnaires that work best in MSD. The final group of tests and questionnaires will be used globally to collect information from thousands of patients. The study is keen to allow use of electronic devices for patient questionnaires which can be used also to maintain connection with adolescent patients during the transitional period of chronic care.

Data is currently being collected by two international groups with an interest in MSD, The American and European Network of Antisynthetase Syndrome (AENEAS) collaborative group and the Euromyositis Registry. Euromyositis have an established web-based data entry and would like to share expertise and technology with AENEAS.

#### **4. Primary and secondary aims**

*Primary aim (the most important questions to be answered in the study).*

To harmonize the international registries with MSD: EUROMYOSITIS and AENAS with national registries and hospital records to create a longitudinal data base to improve follow up of patients and to improve treatment and outcome of disease.

*Secondary aims. (Other important questions to be answered in the study).*

- To agree upon a set of measures that can be used to assess MSD patients across all aspects of their disease spectrum
- To study MSD patients over time to look for trends in their disease patterns
- To help with patient-recorded outcome measure collection by using electronic smartphone applications to collect information outside of the clinic
- To help collect information about patients by extracting information from hospital electronic health records into Euromyositis and AENEAS

#### **5. Recruitment of participants**

Participants are invited through myositis centres and through already existing registries for myositis

#### **6. Inclusion and exclusion criteria**

- Patients with positive myositis specific antibodies who have arthritis and/or lung disease
- Patient with a diagnosis of myositis according to the new Classification criteria (which can be found by searching: Lundberg PMID27320359)

#### **7. Expected benefits for patients**

To have a better knowledge of these diseases, earlier detection, improve survival and quality of life.

#### **8. Expected benefits for society**

With greater knowledge we can reduce death and disability related to MSD.

#### **9. Burden for patients participating in this study**

This study is observational only and except for standard of care questionnaires, there is no burden for participating patients.

#### **10. Patient involvement in the design and conduct of the study**

Patients are involved in every phase of the project. The project is without risks.