

New treatment offers hope for children with debilitating skin and muscle disease

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The results of a UK study presented today at the European League Against Rheumatism Annual Congress (EULAR 2016) showed that tumour necrosis factor inhibitor (anti-TNF) treatment is effective at improving both muscle and skin involvement in children with juvenile dermatomyositis (JDM). These findings bring new hope to JDM patients who have failed to respond to multiple drug treatments and who, as a result, have a greater risk of painful complications and premature death.

JDM is a rare <u>chronic autoimmune disease</u> of <u>children</u> characterised by inflammation of the muscles, skin and other organs., In the UK, the reported incidence of JDM is two to three cases per million children younger than 16 years old, with a median age at onset of 6.8 years; JDM is more common in girls than boys, by a ratio of approximately 2.3:1. Characteristic findings include skin rash, skin ulceration, and muscle weakness.

In general, children with JDM are able to lead normal lives; however, some patients with JDM have a <u>disease</u> course that is refractory to multiple drug treatments. For those children where treatments have failed, prolonged disease activity has been shown to be associated with increased mortality and complications, such as scarring, pain due to trapped nerves, and shortening of the muscles causing joints to stay bent.

"High levels of the cell signalling protein TNF have been reported in JDM patients with a long disease course, suggesting this immune cell regulator may play a significant role in refractory disease," said Dr



Raquel Campanilho-Marques of the Institute of Child Health, University College London, UK. "There are no published clinical trials (only case reports) of this therapy, but some are in progress. Our study is one of the largest to explore the efficacy and safety of anti-TNF therapy in a large independent cohort of JDM patients."

Evaluation of 66 patients with JDM, recruited from the UK JDM Cohort and Biomarker Study and actively treated with anti-TNF agents, showed significant improvements in muscle and skin involvement, as well as in overall disease activity. There were significant changes in the median values of two standard <u>muscle</u> measurements: the Childhood Myositis Assessment Scale and Manual Muscle Testing (p

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