



POSTER PRESENTATION

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# The familial Mediterranean fever (FMF) 50 score: does it work in a controlled clinical trial? Re-analysis of the trial of riloncept for patients with colchicine resistant or intolerant FMF

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## Background

The familial Mediterranean fever 50 score (FMF50) was recently devised to define response to treatment and as an outcome measure for clinical trials of FMF.

## Objectives

To examine the performance of the FMF50 score in a previously published trial of riloncept [1] for patients whose FMF was resistant or intolerant to colchicine.

## Methods

We reanalyzed the data from the controlled trial of riloncept vs. placebo in 14 patients with colchicine-resistant or intolerant FMF using the FMF50 score as the primary outcome. The FMF50 score required improvement by  $\geq 50\%$  in five of six criteria (attack frequency, attack duration, global patient assessment, global physician assessment, frequency of attacks with arthritis, and levels of acute-phase reactants) without worsening of the sixth criterion.

## Results

In the original trial riloncept was considered effective according to the primary outcome measure (differences in the attack frequency) with eight analyzable patients considered responders and four as non-responders. According to the FMF50 score, only two participants would have been considered responders to riloncept, and one to placebo. Only two participants had  $\geq 50\%$  differences between riloncept and placebo in five criteria.

The major explanation for non-response to treatment was that with riloncept the duration of attack decreased by  $\geq 50\%$  in only 2 participants and 5 participants had no attacks of arthritis either during screening (before randomization) or during treatment with riloncept.

## Conclusions

The proposed FMF50 score did not differentiate well between responders and non-responders compared to the a priori defined primary outcome measure in this successful controlled study and should be revisited prior to adoption as a primary outcome measure in multinational FMF trials.

## Authors' details

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## Reference

1. Hashkes PJ, Spalding SJ, Giannini EH, Huang B, Johnson A, Park G: Riloncept for colchicine-resistant or -intolerant familial Mediterranean fever: a randomized trial. *Ann Intern Med* 2012, **157**(8):533-541.

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