



ORAL PRESENTATION

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# Colchicine trial in PFAPA Syndrome and MEFV-negative patients

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From 8th International Congress of Familial Mediterranean Fever and Systemic Autoinflammatory Diseases Dresden, Germany. 30 September - 3 October 2015

## Introduction

PFAPA Syndrome (Periodic Fever, Aphthous stomatitis, Pharyngitis, and cervical Adenitis) is the most common periodic fever in childhood; the diagnosis is based on clinical criteria. Familial Mediterranean Fever (FMF) is a monogenic autosomal recessive autoinflammatory disease, whose diagnosis is based on clinical elements, supported by MEFV genetic mutations. When there is only a mutation or no one, the patient undergoes a trial with colchicine for 4-6 months, and diagnosis is confirmed in case of clinical response and fever early recurrence after suspension. Current treatment of PFAPA is symptomatic. Febrile episodes show a rapid response to the administration of one or two doses of prednisone (1-2 mg/kg) or betamethasone (0.1-0.2 mg/kg). Total requirement of steroid increases over time, and the frequency of attacks worsens the quality of life of patients. In literature, the prophylaxis of PFAPA febrile attacks with colchicine (0.5-1 mg/day) has been tested only on a few patients, with controversial results.

## Objectives

Considering the similarities between FMF MEFV-negative patients (MEFVneg) and PFAPA patients, we aimed to demonstrate that colchicine is effective in PFAPA too: positive response was evaluated in terms of reduction in frequency >50% and severity of attacks >50%.

## Materials and methods

We conducted a prospective cohort study (from September 2012, still ongoing), comparing two groups: 67 MEFVneg and 51 PFAPA patients. 36 of the latter group underwent colchicine trial, after obtaining informed consent.

## Results

We assessed the response of PFAPA patients to colchicine preventive treatment: good response was observed in 75% (27 patients of 36), and a non-response in 25% (9 pts). The effective treatment rate of MEFVneg is 100%, by definition. The average dose of colchicine administered in PFAPA was 1.14 mg/day, compared to MEFVneg (1.34 mg/day). The dose per kilogram of body weight is 0.020 mg/kg/day in both groups. We can state that the colchicine dose requirement in PFAPA coincides to the one of FMF patients.

## Conclusion

Our study showed that colchicine regimen is effective in 75% of cases. Prophylaxis with colchicine should be offered to all PFAPA patients, instead of steroids or other symptomatic therapy (as paracetamol or ibuprofen), before the treatment with anti-IL1 $\beta$  biologic drugs, with considerable savings in pharmacoconomics.

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Published: 28 September 2015

doi:10.1186/1546-0096-13-S1-O5

Cite this article as: Kadhim et al.: Colchicine trial in PFAPA Syndrome and MEFV-negative patients. *Pediatric Rheumatology* 2015 **13**(Suppl 1):O5.

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