



ORAL PRESENTATION

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Colchicine trial in PFAPA Syndrome and MEFV-negative patients

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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 β biologic drugs, with considerable savings in pharmacoconomics.

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