Predicting Colchicine Response In Patients With Undefined Autoinflammatory Diseases

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Background/Purpose:
Autoinflammatory diseases (AIDs) are a rare group of illnesses characterized by unprovoked episodes of fever and systemic inflammation. An understanding of their pathophysiology has led to the development of effective treatment guidelines. Unfortunately, many patients with recurrent fevers have symptoms that do not match any of the known AIDs. There is an unmet need to provide effective treatment to these patients with undefined AIDs (uAIDs).

Colchicine, a treatment for patients with familial Mediterranean fever, is sometimes used to treat patients with uAIDs. We examined the efficacy of colchicine in patients with uAIDs and identified clinical factors that predicted a good colchicine response.

Methods
We conducted a retrospective chart review of patients with a clinical diagnosis of uAIDs who tolerated colchicine. A total of 184 patients with uAIDs were identified and 68 had used colchicine. 33 (45%) were good colchicine responders. 44% were partial responders. 7.4% were non-responders.

Patient characteristics based on colchicine response are shown in Table 1; ethnicities are shown in Figures 1 and 2. Clinical characteristics of febrile episodes, based on colchicine response, are shown in Table 2.

Results
184 patients with uAIDs were identified and 68 had used colchicine. 33 (45%) were good colchicine responders. 30 (44.1%) were partial responders. 5 (7.4%) were non-responders.

Patient characteristics based on colchicine response are shown in Table 1; ethnicities are shown in Figures 1 and 2. Clinical characteristics of febrile episodes, based on colchicine response, are shown in Table 2.

Conclusions
Colchicine was effective treatment for most patients with uAIDs, with 48% and 44% of patients having a good or partial response, respectively.

• Patient characteristics, including the presence of mutations in AID genes (eg. MEFV), a family history of recurrent fevers, and age of disease onset did not predict colchicine response.

• On the other hand, features of febrile episodes were useful in predicting colchicine response: patients were more likely to have a good response if they had vomiting during flares; abdominal pain approached statistical significance.

• Although prior studies have shown colchicine to be beneficial for patients with recurrent aphthous stomatitis (eg. Behcet’s), our cohort of patients with uAIDs and aphthous ulcers were less likely to benefit from colchicine than patients without aphthous.

Table 1. Patient characteristics based on colchicine response.

Table 2. Clinical characteristics of febrile episodes.

Figure 1. Ethnicity of good colchicine responders.

Figure 2. Ethnicity of partial and non-responders.