COLCHICINE EFFICACY FOR UNDEFINED AUTOINFLAMMATORY DISEASES

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Purpose

Autoinflammatory diseases (AIDs) are a rare group of illnesses characterized by unprovoked episodes of fever and systemic inflammation. Patients with defined AIDs benefit from evidence-based treatment guidelines[1]. Unfortunately, many patients with AIDs do not have a genetic diagnosis and their symptoms do not match any of the known AIDs. There is an unmet need to provide effective treatment to these patients with undefined AIDs (uAIDs). We examined the efficacy of colchicine in patients with uAIDs and identified patient characteristics and 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. Responses were defined as follows:

- **Good response**: decrease in the frequency, severity, AND length of febrile episodes without requiring additional medications.
- **Partial response**: decrease in the frequency, severity, OR length of episodes; additional medicines may have been required.
- **No response:** no benefit was appreciated.

For statistical analyses, partial and non-responders were combined into a single group.

Results

184 patients with uAIDs were identified and 68 had used colchicine.

- 33 (48.5%) 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.

Figure 1. Ethnicity of good colchicine responders.

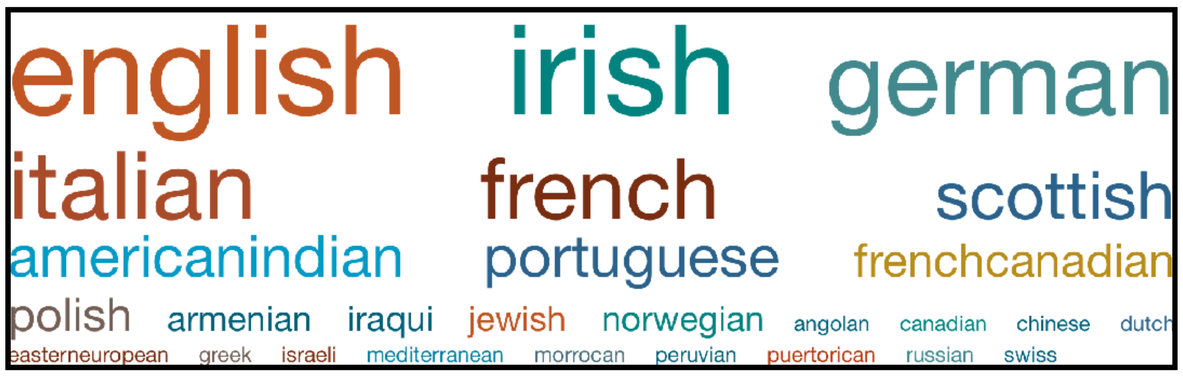

Figure 2. Ethnicity of partial and non-responders.

	Good responders N=33 (N,%)	Partial and non-responders N=35 (N,%)	p-value
Female	17 (52)	20 (57)	0.8078
Age at disease onset (months)	50.0	49.9	0.9908
Family history of recurrent fevers	2 (6.1)	7 (20)	0.1510
Mean colchicine dose (mg)	0.58	0.61	0.7184
Mean duration of colchicine follow-up (months)	37.8	32.4	0.4782
Periodic fever syndrome panel test sent	24 (73)	29 (83)	0.3866
Patients with heterozygous MEFV mutations	8 (24)	9 (26)	1.000
Periodic fever syndrome panel negative	11/24 (46)	16/29 (55)	0.5857

Table 1. Patient characteristics based on colchicine response.

	Good responders N=33 (N,%)	Partial and non-responders N=35 (N,%)	p-value
Abdominal pain	21 (64)	14 (40)	*0.058*
Aphthous stomatitis	6 (18)	18 (51)	**0.0054**
Arthralgia	16 (48)	10 (29)	0.1341
Chest pain	2 (6)	2 (6)	1.000
Diarrhea	7 (21)	3 (9)	0.1809
Fatigue	5 (15)	8 (23)	0.5415
Headache	12 (36)	12 (34)	1.000
Lymphadenopathy	5 (15)	8 (23)	0.5415
Myalgia	6 (18)	8 (23)	0.7669
Pharyngitis	2 (6)	2 (6)	1.000
Rash	6 (18)	8 (23)	0.7669
Vomiting	14 (42)	5 (14)	**0.0145**

Table 2. Clinical characteristics of febrile episodes.

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[2], Behcet's[3], and PFAPA[4], our cohort of patients with uAIDs and aphthous ulcers were less likely to benefit from colchicine than patients without aphthae.

References

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