Anifrolumab for treatment-resistant paediatric amyopathic dermatomyositis: report of two cases

Sir,

Amyopathic dermatomyositis (ADM) is a rare subtype of juvenile dermatomyositis (JDM), presenting with characteristic skin involvement but no clinical or laboratory evidence of myositis for at least six months (1). Skin involvement in ADM is often refractory to standard therapies. Anifrolumab, a fully human monoclonal antibody targeting the type I IFN receptor subunit 1, has demonstrated efficacy in systemic lupus erythematosus (SLE) (2). Given the overlapping pathophysiological features between SLE and ADM (3), we explored the off-label use of anifrolumab in two paediatric patients with treatment-refractory ADM.

Patient 1 was diagnosed at age 3 with classic JDM and later evolved into an amyopathic form. Despite multiple therapies, including corticosteroids (2mg/kg/die), methotrexate (10mg/m^2/week), hydroxychloroquine (5mg/kg/die), cyclosporine (3mg/kg/die), IVIG (2 g/kg), and baricitinib (4mg/die), cutaneous disease remained active. At age 16, monthly anifrolumab infusions (300 mg) were initiated alongside methotrexate (Fig. 1a). Significant clinical improvement was observed after the first dose, with near-complete resolution of skin lesions by the fourth infusion (Fig. 1b). The CDASI-A score decreased from 20 to 6, and levels of Siglec-1, a marker of IFN activity, also declined markedly from 65.4% to 21.6%.

Patient 2 was diagnosed with ADM at age 13, with malar rash and Gottron's papules but no muscle involvement. Initial treatment with hydroxychloroquine (5mg/kg/die) and methotrexate (10mg/m^2/week) failed. Anifrolumab infusions resulted in rapid clinical improvement, with CDASI-A decreasing from 15 (Fig. 2a) to 4 (Fig. 2b) within three doses, and a reduction in Siglec-1 expression from 33% to 7%.

Juvenile dermatomyositis (JDM) is a serious autoimmune condition and represents the most common inflammatory myopathy in childhood. Cutaneous involvement is often refractory to systemic therapies, with persistent active lesions or the development of new ones, even when muscular symptoms improve (3). Although the pathogenesis of dermatomyositis remains unclear, elevated levels of type I interferons have been demonstrated in both plasma and muscle tissue and it is known that increased interferon production is a hallmark of systemic lupus erythematosus (4). Anifrolumab, a human antibody against IFN1 receptor subunit 1 monoclonal antibody, is approved for use in SLE. Two phase III clinical trials (TULIP-1 and TULIP-2) involving patients with SLE demonstrated promising results in the treatment of cutaneous lupus (2, 4) and a recent 2023 EULAR recommendation supports the use of anifrolumab in patients with SLE who exhibit cutaneous manifestations that do not respond to first-line therapies, estimated at approximately 40% of cases (5). Considering the similarities between the skin manifestations of SLE and DMJ, and the strong efficacy of anifrolumab in SLE, the potential use of this drug in dermatomyositis has been proposed. A recent retrospective study analysed data

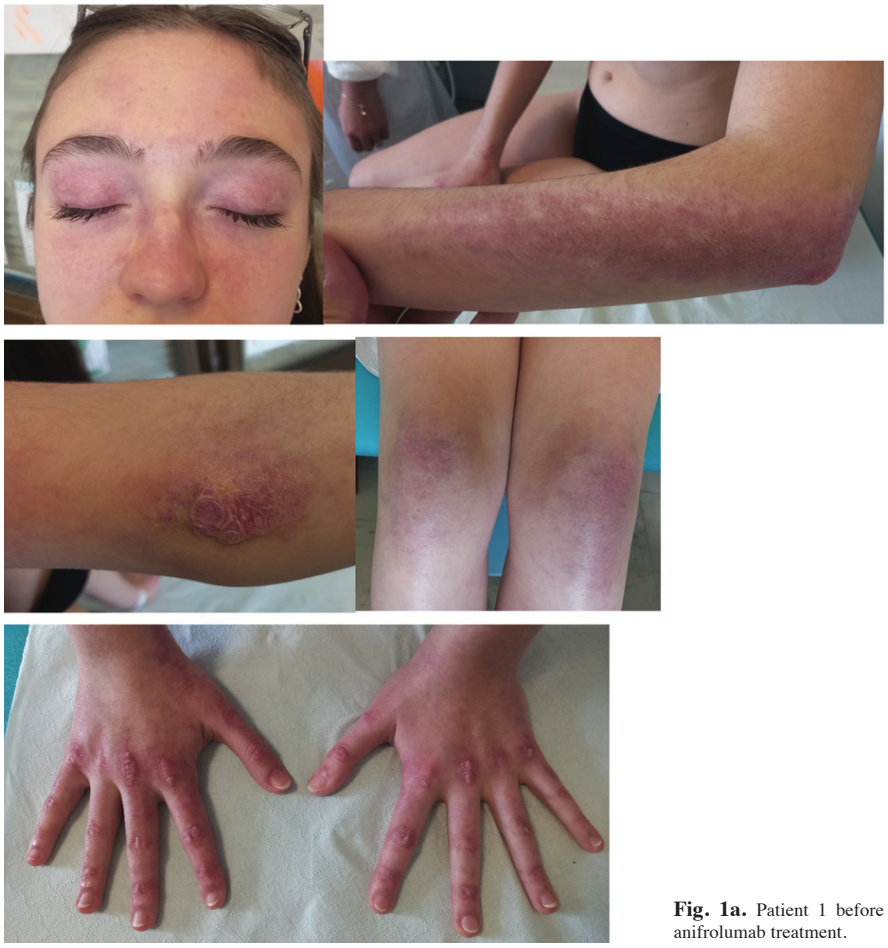


Fig. 1a. Patient 1 before anifrolumab treatment.

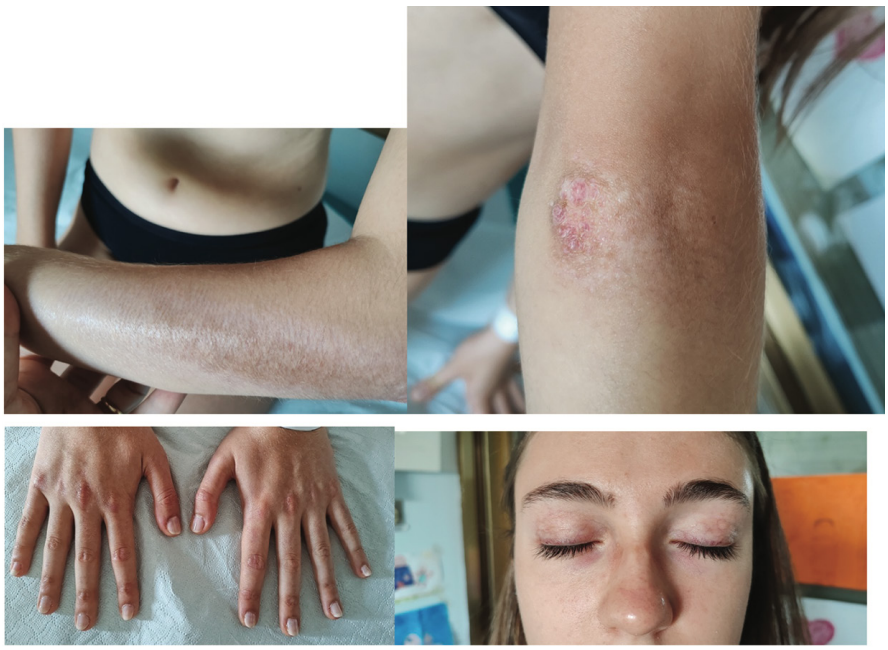


Fig. 1 b. Patient 1 before the fourth infusion of anifrolumab.

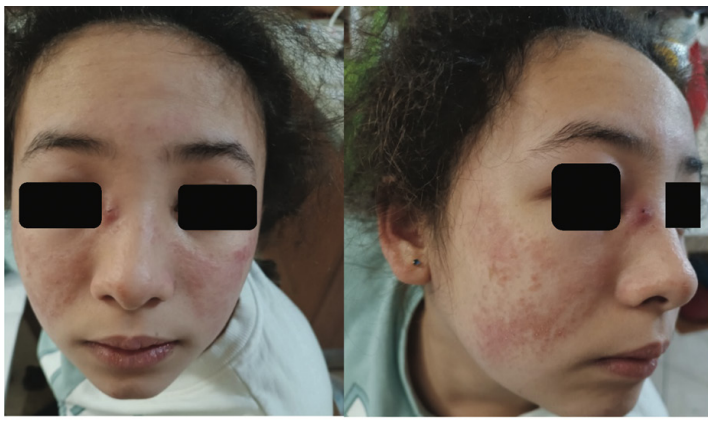
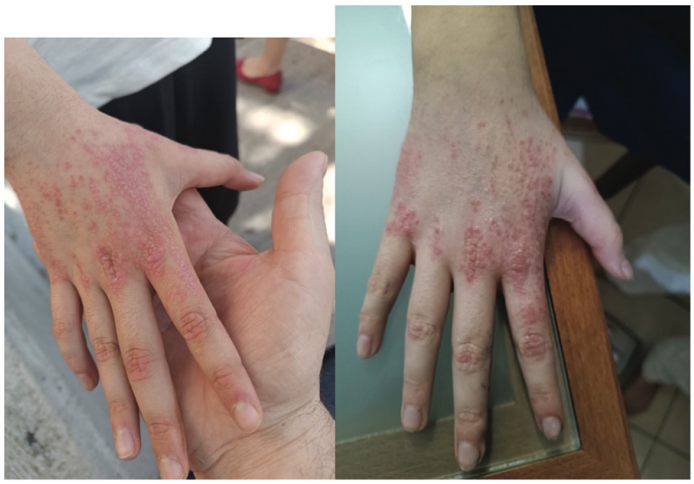


Fig. 2a. Patient 2 before anifrolumab treatment.



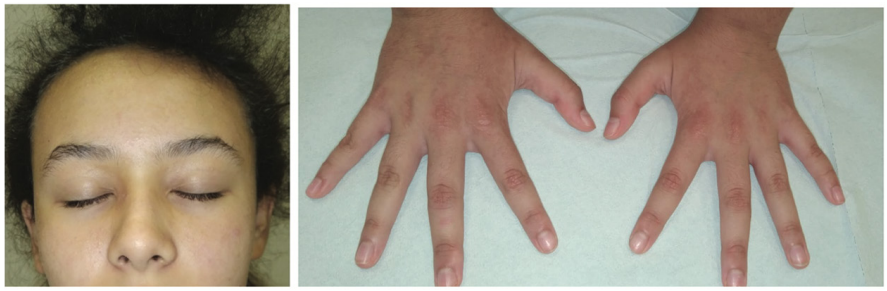


Fig. 2b. Patient 2 after the third infusion of anifrolumab.

from seven patients aged 14 to 66 years with dermatomyositis characterised by predominant cutaneous involvement that was refractory to standard therapies. All patients showed a nearly 50% reduction in CDASI-A scores observed after just two doses (6). Given the early, encouraging data, despite the limited number of patients, in May 2025 we used anifrolumab as off-label prescription to treat two paediatric patients with ADM, whose baseline characteristics are summarised in Table I, achieving excellent results on cutaneous manifestations. Patients did not experience any side effects in the following 6 months of follow up. Furthermore, in addition to the improvement of skin symptoms, we demonstrated a lower Siglec1 level in blood, showing a systemic improvement of the treatment. Finally, to our knowledge, this is the first report describing two cases of amyopathic dermatomyositis in the paediatric population treated with anifrolumab. Certainly, further studies are needed to demonstrate the efficacy of anifrolumab in the treatment of dermatomyositis resistant to first-line therapies.

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Competing interests: none declared.

Table I. Characteristics of patients at the time anifrolumab therapy was started.

	Patient 1	Patient 2
Sex	F	F
Age at disease onset	3 years	13 years
Disease duration (years)	13 years	1 year
Previous treatments	corticosteroids, methotrexate hydroxychloroquine, cyclosporine, IVIG, baricitinib	Hydroxychloroquine, methotrexate
Current treatments	weekly oral methotrexate	weekly oral methotrexate
Cutaneous manifestations	malar and heliotrope rash, Gottron's papules on the hands, knees and elbows. On the left forearm a skin lesion with reduced trophism, translucent in appearance and surrounded by erythema.	malar rash, Gottron's papules on hands, wrists, ankles, and elbows
Muscle enzymes	normal	normal
Siglec1	elevated	elevated

References

1. BAILEY EE, FIORENTINO DF: Amyopathic dermatomyositis: definitions, diagnosis, and management. *Curr Rheumatol Rep* 2014; 16(12): 465. https://doi.org/10.1007/s11926-014-0465-0
2. MORAND EF, FURIE R, TANAKA Y *et al*.: Trial of Anifrolumab in Active Systemic Lupus Erythematosus. *N Engl J Med* 2020; 382(3): 211-21. https://doi.org/10.1056/NEJMoa1912196
3. SHAW KS, REUSCH DB, CASTILLO RL *et al*.: Rapid Improvement in Recalcitrant Cutaneous Juvenile Dermatomyositis with Anifrolumab Treatment. *JAMA Dermatol* 2024; 160(2): 237-8. https://doi.org/10.1001/jamadermatol.2023.4744
4. HILE GA, WERTH VP: Understanding the Role of Type I Interferons in Cutaneous Lupus and Dermatomyositis: Toward Better Therapeutics. *Arthritis Rheumatol* 2025; 77(1): 1-11. https://doi.org/10.1002/art.42983
5. FANOURIAKIS A, KOSTOPOULOU M, ANDERSEN J *et al*.: EULAR recommendations for the management of systemic lupus erythematosus. *Ann Rheum Dis* 2024; 83(1): 15-29. https://doi.org/10.1136/ard-2023-224762
6. SHAW KS, HASHEMI KB, CASTILLO RL *et al*.: Anifrolumab in recalcitrant cutaneous dermatomyositis: A multicenter retrospective cohort study. *J Am Acad Dermatol* 2024; 91(6): 1217-9. https://doi.org/10.1016/j.jaad.2024.07.1491