Grinnell Madison (Orcid ID: 0000-0003-3183-5185)
Keyes Emily R (Orcid ID: 0000-0003-4336-7323)
Vazquez Thomas (Orcid ID: 0000-0003-1721-5617)
Werth Victoria (Orcid ID: 0000-0003-3030-5369)

Mycophenolate Mofetil and Methotrexate Efficacy in Dermatomyositis

Madison Grinnell,[1,2] Emily Keyes,[1,2] DeAnna Diaz,[1,2] Thomas Vazquez,[1,2] Rui Feng[3] and Victoria P. Werth[1,2]

1 Department of Dermatology, Perelman School of Medicine, University of Pennsylvania, Philadelphia, PA, USA
2 Corporal Michael J. Crescenz VAMC, Philadelphia, PA, USA
3 Department of Biostatistics, University of Pennsylvania, Philadelphia, PA, USA

Correspondence: Victoria P. Werth, MD

Email: werth@pennmedicine.upenn.edu

Funding sources: United States Department of Veterans Affairs (Veterans Health Administration, Office of Research and Development and Biomedical Laboratory Research and Development) and National Institute of Health [R01AR076766] (VPW)

Conflicts of Interest: We have no conflicts of interest to disclose.

Treatment of dermatomyositis (DM) typically follows a stepwise sequence starting with methotrexate (MTX) or mycophenolate mofetil (MMF) after inadequate antimalarial response (1). However, data is scarce regarding the effectiveness of MTX and MMF. A cohort of 24 patients with currently skin-predominant DM was identified from a prospective database at the University of Pennsylvania Perelman School of Medicine. Included patients took MTX or MMF and had at least two study visits within a retrospective observation period from October 2008 to February 2021. The Cutaneous Dermatomyositis Disease Area and Severity Index (CDASI), a validated disease scoring tool (2), was used to assess severity and outcomes. Patients with mild

This article has been accepted for publication and undergone full peer review but has not been through the copyediting, typesetting, pagination and proofreading process which may lead to differences between this version and the Version of Record. Please cite this article as doi: 10.1111/bjd.21235

disease activity, defined as a CDASI activity (CDASI-A) score <14 (maximum sub-score of 100), were excluded as were any patients on medications used to treat DM aside from MTX or MMF, with the exception of chronic antimalarials or topical medications.

For both MMF (n=13) and MTX (n=11), there was no baseline difference in CDASI-A scores at treatment initiation. There was no significant difference in the degree of improvement on either medication, with a mean difference in daily CDASI-A change between MTX and MMF of -0.0028 ± 0.0024 (p=0.2400) using a mixed linear effects model. For MTX, the median percentage change in CDASI-A between the first and last study visit was -74%. For MMF, the median percentage change was -76%. A decrease of 40% or greater in the CDASI-A score has previously been associated with a meaningful change in quality of life (3). Defining responders as having a 40% or greater improvement in their CDASI-A score between their first and second observations (see Figure 1), 27% of the patients taking MTX were responders while 54% of patients taking MMF were responders. The range of time varied between the first and second visits with 50% of patients having a second study visit within 150 days. By last follow-up, 55% of patients taking MTX were considered responders and 77% of patients taking MMF met criteria to be considered responders. For MTX, the median follow-up for the second visit was 178 days and for the last visit was 776 days. For MMF, the median follow up was similar—147 days for the second visit, and for the last visit was 787 days. Six patients taking MMF had previously been treated with MTX and one patient using MTX had previously used MMF.

While there was virtually no difference between the daily change in the MTX and MMF groups, the small sample size and non-randomized design preclude definitive conclusions. However, it

can provide a preliminary reference for future study design. Small observational studies are also subject to confounders and other biases. To overcome these shortcomings, large randomized clinical trials are needed. Either MMF or MTX may be added to treatment plans for patients with DM who have not responded to antimalarials. Our data suggest that responders continued to improve over many months while most non-responders showed little improvement at first follow-up during the observation period.

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Figure Legend

Figure 1. MMF Takers and MTX Takers CDASI Activity Scores, Inset showing first 180 days of therapy

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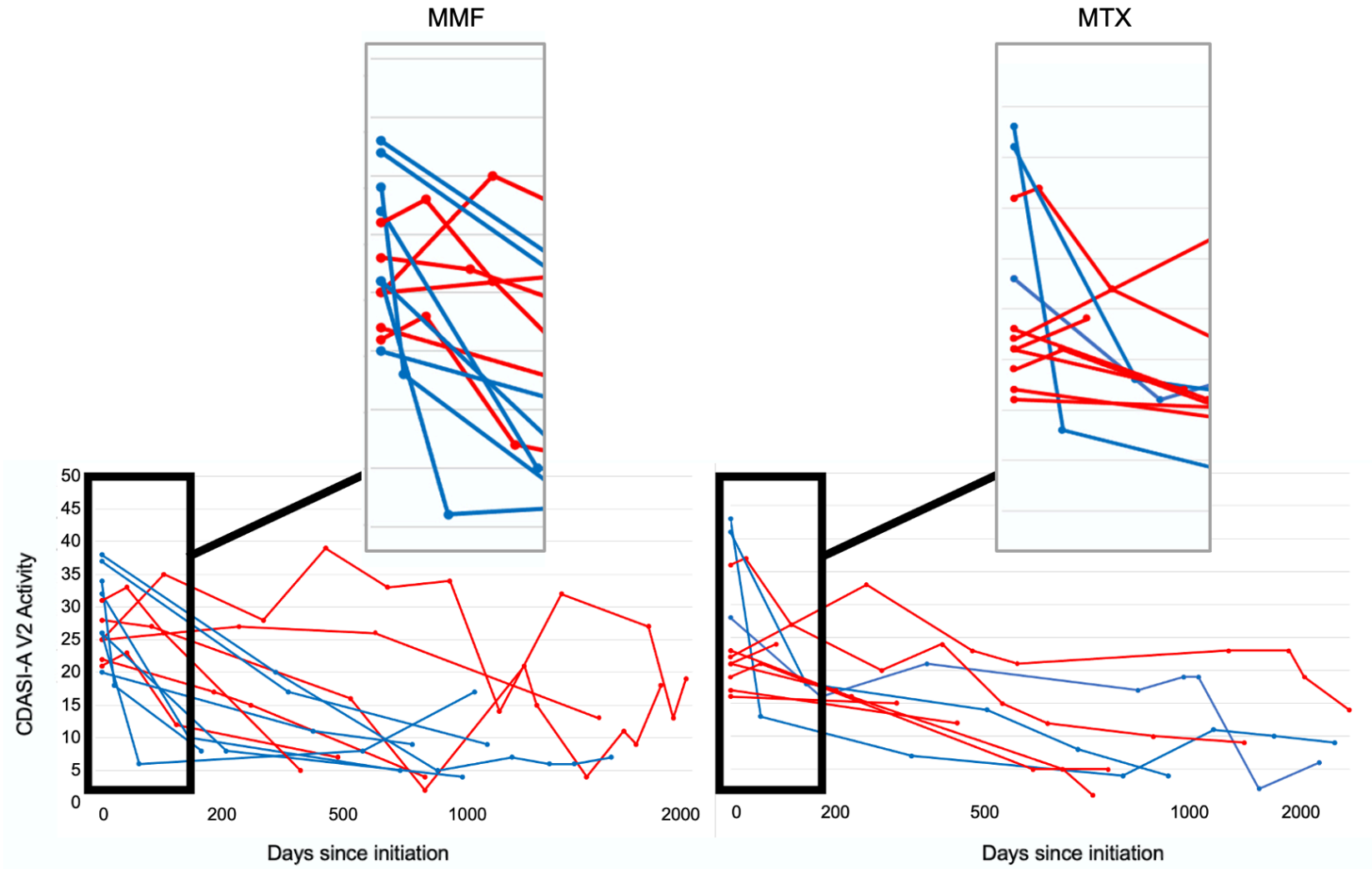

*Responders at first follow up displayed in blue, nonresponders displayed in red