ABSTRACT NUMBER: 7L

A Phase 2 Study of Safety and Efficacy of Anabasum (JBT-101), a Cannabinoid Receptor Type 2 Agonist, in Refractory Skin-Predominant Dermatomyositis

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SESSION INFORMATION

Date: Tuesday, November 7, 2017

Session Title: ACR Late-Breaking Abstract Poster

Session Type: ACR Late-breaking Abstract Session

Session Time: 9:00AM-11:00AM

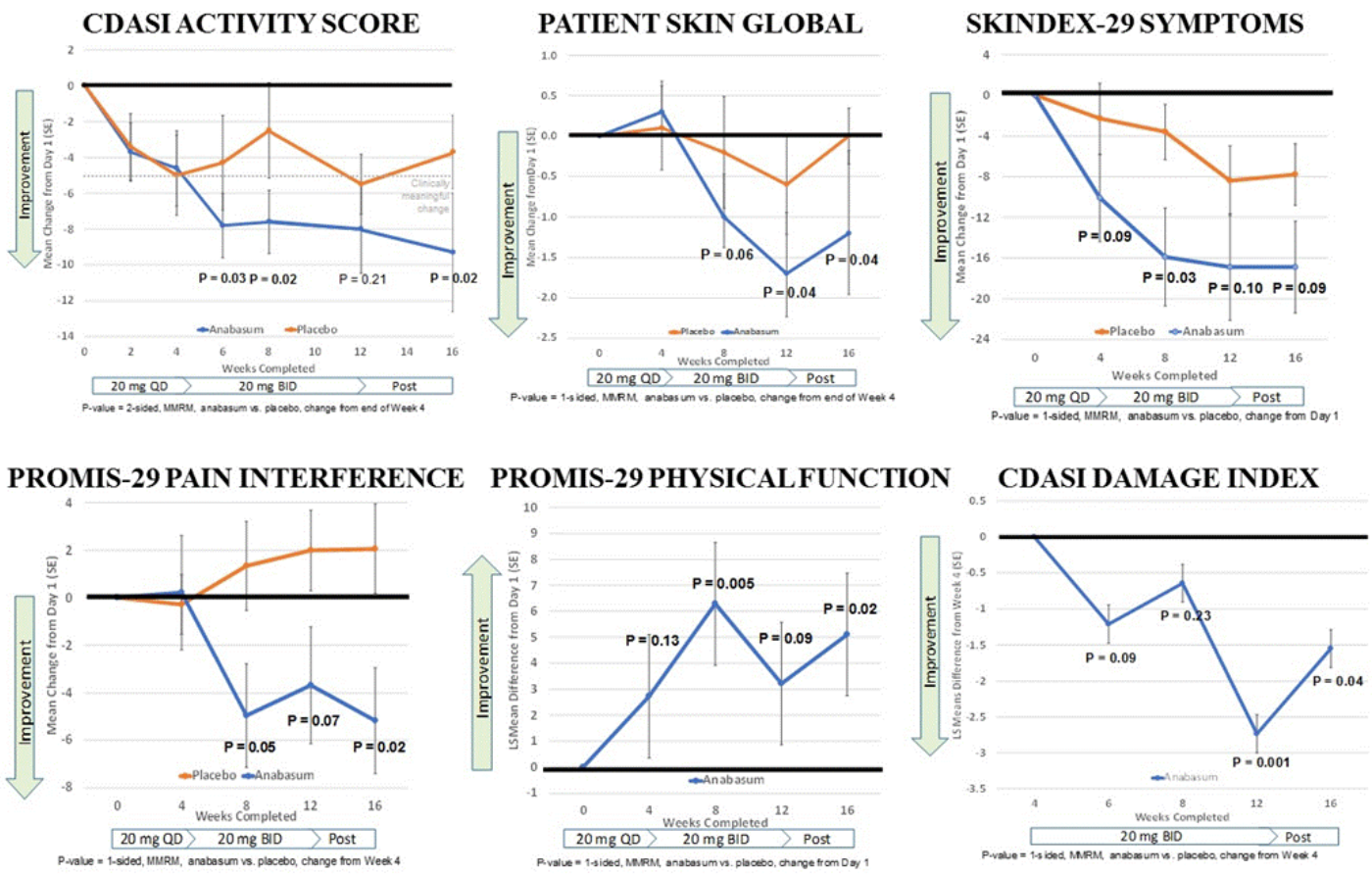
Background/Purpose: Effective treatment options are limited for refractory skindisease in dermatomyositis (DM). Anabasum is a non-immunosuppressive,synthetic, oral preferential CB2 agonist that triggers resolution of innateimmune responses and reduces cytokine production by PBMC from DM patients. Thispurpose of this study was to test safety and efficacy of anabasum in DMsubjects with refractory, moderate-to-severely active skin disease.

Methods: A double-blind, randomized placebo-controlled16-week Phase 2 trial (JBT101-DM-001) enrolled adults with documented DM and a Cutaneous Dermatomyositis Disease Area and Severity Index (CDASI)activity score ≥ 14, minimal active muscle involvement and failure orintolerance to hydroxychloroquine and stable DM medications including immunosuppressants. Subjects received 2 escalating doses of anabasum (20 mg QD X 4 weeks, then 20mg BID X 8 weeks) or PBO X 12 weeks. Subjects were followed off study drug X 4weeks. Safety and efficacy outcomes were assessed from Day 1 through end ofstudy at Week 16. The primary efficacy objective was to assess efficacy ofanabasum using CDASI activity score.

Results: 11 adults eachreceived anabasum and PBO (N = 22). Demographic and disease characteristicswere similar in both cohorts. Both cohorts had mean CDASI activity scores inthe severe range (33-35) despite immunosuppressants (19/22 subjects). Anabasum subjects had clinically meaningful improvement inCDASI activity scores with mean reduction ≥ 5 points at all visits after4 weeks. Improvement had statistical significance at end of study (Fig. 1, P =0.02, 2-sided MMRM) that first became apparent after 4 weeks. Anabasum provided greater

improvement than placebo in CDAIdamage index, patient-reported global skin disease and overall diseaseassessments, skin symptoms including photosensitivity and itch, fatigue, sleep,pain interference with activities, pain, and physical function (examples in Fig.1). Improvements in secondary efficacy outcomes reached statisticalsignificance (P ≤ 0.1, 1-sided MMRM) at multiple visits after week 4(Fig. 1). There were no serious, severe or unexpected adverse events (AEs)related to anabasum. Tolerability of anabasum was excellent with no studydrop-outs. Subjects in the anabasum cohort had numerically more mild AEs thanplacebo subjects (29 vs. 19) and fewer moderate AEs (4 vs. 7). AEs in ≥3 subjects in any cohort were diarrhea, dizziness (lightheadedness), fatigueand dry mouth.

Figure 1. Effects of Anabasum on Efficacy Outcomes in Refractory, Skin-Predominant DM



Conclusion: Anabasum demonstrated consistentevidence of clinical benefit across multiple efficacy outcomes and had acceptablesafety and tolerability in this Phase 2 trial in refractory skin disease in DM. Further evaluation of anabasum in the treatment of DM is warranted.

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