Evaluating Efzofitimod in a Subset of Sarcoidosis with the Restrictive Phenotype

Vis Niranjan, MD[1]; Pavithra Ramesh, MS[1], Sanjay Shukla, MD[2]; Nelson Kinnersley PhD[3]; Dan Culver, DO[4]

[1]RxMD, Chennai, India; [2]aTyr Pharma, San Diego, California, US; [3]Octa Consulting Services, Ltd., London, England; [4]Department of Pulmonary and Critical Care Medicine, Integrated Hospital Care Institute, Cleveland Clinic, Cleveland, OH, USA

INTRODUCTION

EFZO-FIT (ATYR1923-C-004) is a randomized (1:1:1 placebo or efzofitimod 3 or 5 mg/kg), trial evaluating efficacy and safety of a 48-week (12 doses) treatment with efzofitimod in pulmonary sarcoidosis. A total of 264 patients were enrolled and treated (intent-to-treat, ITT). Here we present data for a subset of patients of pulmonary sarcoidosis with the restrictive phenotype (FVCpp ≤ 80%, FEV1/FVC ≥ 0.7).

OBJECTIVE

To evaluate the efficacy, safety of efzofitimod in the restrictive phenotype patients from the EFZO-FIT trial.

METHODS

In this posthoc analysis, the Week 48 change from baseline in FVC and Patient Reported Outcomes was analyzed using a Random Coefficient Regression Model (RCRM) to supplement the Mixed Model for Repeated Measures (MMRM) analysis performed for the ITT data.

RESULTS

There were 16, 16 and 12 patients in the placebo, 3 mg/kg and 5 mg/kg arms respectively. These baseline characteristics were generally balanced with some minor imbalances between treatment arms, consistent with the small sample size. Weight was overall higher in the restrictive subset than the ITT. Mean FVC was expectedly lower in the restrictive subset than that for the ITT population.

Overall, the magnitude of steroid reduction in the 5 mg/kg was comparable to that in the placebo arm.

In the 5 mg/kg efzofitimod arm, the placebo adjusted week 48 change from baseline for FVC by RCRM was 123.8 ml. The placebo adjusted week 48 change from baseline by RCRM showed improvement for the 5 mg/kg arm versus placebo for King's sarcoidosis questionnaire-Lung (KSQ-L), KSQ-general health (KSQ-GH), fatigue assessment scale (FAS), Leicester cough questionnaire (LCQ).

Efzofitimod was generally well tolerated, consistent with the ITT population.

RESULTS (cont.)

Table 1. Baseline demographics, disease, and pulmonary characteristics

	Placebo N=16	Efzofitimod 3 mg/kg N=16	Efzofitimod 5 mg/kg N=12
Baseline Demographics			
Gender - Female, n (%)	6 (37.5)	7 (43.8)	5 (41.7)
Race - black, n (%)	5 (31.2)	3 (18.8)	3 (25)
Weight in kg, mean (sd)	88.9 (19.1)	86.6 (24.4)	95.9 (20.3)
BMI in kg/m², mean (sd)	30.2 (4.5)	29 (5.4)	32.4 (8.8)
Age in years, mean (sd)	52.4 (10.9)	54.6 (12.3)	57.8 (8.1)
Baseline Characteristics			
Baseline OCS in mg/day mean (sd)	10.88 (4.7)	11.72 (3.38)	11.88 (4.54)
OCS duration, n (%) 0 to < 2 years ≥ 2 years	 8 (50) 8 (50)	 10 (62.5) 6 (37.5)	 5 (41.7) 7 (58.3)
Concomitant immunosuppressant, n (%)	9 (56.3)	7 (43.8)	6 (50)
Baseline Pulmonary Characteristics			
FVC pp in %, mean (sd)	70.82 (8.78)	68.48 (7.24)	71.32 (4.29)
FEV1 pp in %, mean (sd)	69.33 (8.63)	68.46 (8.64)	73.01 (6.62)
Baseline PROs			
KSQ-L, mean (sd)	46.69 (12.73)	49.7 (7.01)	48.26 (10.01)
KSQ-GH, mean (sd)	55.40 (11.59)	52.23 (14.41)	49.46 (15.85)
LCQ, mean (sd)	15.09 (4.76)	16.40 (3.58)	14.02 (4.96)
FAS, mean (sd)	27.75 (8.30)	29.69 (9.25)	29.67 (9.99)

Table 2. Efficacy

	Placebo N=16	Efzofitimod 3 mg/kg N=16	Efzofitimod 5 mg/kg N=12
OCS			
OCS CFB at Week 48 (mg/day) mean (sd)	-6.350 (10.420)	-8.126 (3.838)	-7.661 (6.371)
FVC			
FVC, baseline in ml, mean (sd)	2851 (839)	2710 (819)	2795 (467)
Placebo adjusted annual rate of change (RCRM)		32.2	123.8
PROs[2]			
KSQ-L Placebo adjusted Annual rate of change (RCRM)		-0.81	2.14
KSQ-GH Placebo adjusted Annual rate of change (RCRM)		-0.25	2.32
LCQ Placebo adjusted Annual Rate of change (RCRM)		0.5826	0.2427
FAS Placebo adjusted annual rate of change (RCRM)[1]		-2.2955	-4.9447

[1] Lower score indicates fatigue is getting better

RESULTS (cont.)

Figure 1. FVC

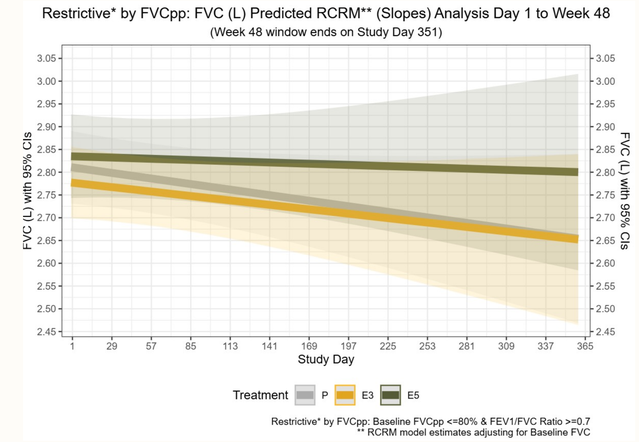


Figure 2. KSQ-Lung

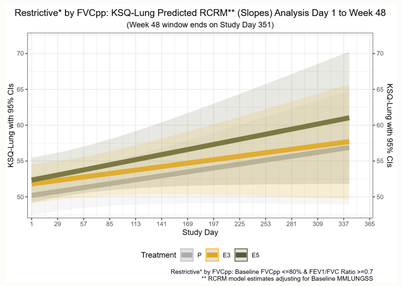


Figure 3. KSQ-GH

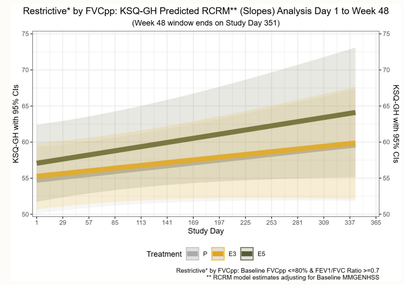


Figure 4. LCQ

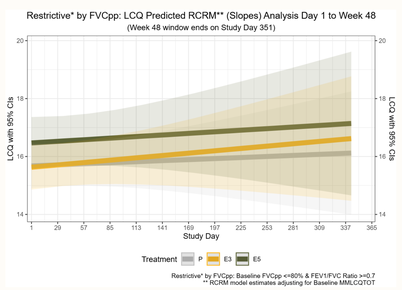
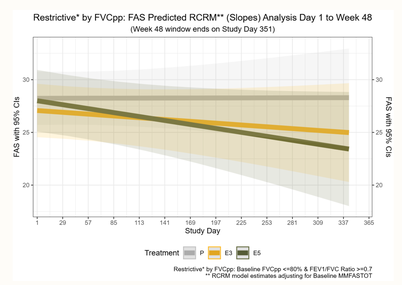


Figure 5. FAS



CONCLUSION

The study results warrant further investigation in patients with pulmonary sarcoidosis and the restrictive phenotype.

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