Patient-Reported Outcomes Among Patients With Steroid-Refractory or -Dependent Chronic Graft-vs-Host Disease Randomized to Ruxolitinib vs Best Available Therapy

**INTRODUCTION**

- Ruxolitinib (Jakafi) (JAK1/2) inhibitor is currently being used in the US for the treatment of patients aged ≥12 years with chronic graft-vs-host disease (cGVHD) who have failed first-line therapy by 28 days after randomization (BAT) or have not received a first-line therapy (BAT). This study compared ruxolitinib to BAT in patients with steroid-refractory or -dependent (SR/D) cGVHD.
- The primary outcome was the Stroke Symptom Score (SSS) at week 24, as measured by the Lee Severity Score (Lee-SSS).
- Secondary outcomes included change in the primary and key secondary endpoints.
- A larger proportion of patients treated with ruxolitinib than BAT were mLSS responders (≥7-point reduction from baseline in the summary symptom score) at week 24 and at any visit up to week 24.

**RESULTS**

**Patients**
- Baseline characteristics, including symptom burden, were balanced between arms (Supplementary Material).
- mLSS response
  - **Figure 1. mLSS Response and Mean Summary Symptom Score Over Time**
  - **Figure 2. mLSS Responders by Baseline cGVHD Severity, Overall Response at Week 24, and Steroid Dose at Week 24**
  - **Figure 3. Association Between Organ Response and Change in Symptom Score (mLSS)**

**Organ and mLSS response**
- **Figure 3A. Mean Change From Baseline in Summary Score at Week 24 by mLSS Subcategory**
- **Figure 3B. Individual Organ Response at Week 24**
- **Figure 3C. Linear Regression Analysis of Organ Response and mLSS Subcategory at Week 24**
- **Figure 4. PGIS and PGIC at Week 24**

**Additional PROs**
- **Figure 5. Mean Summary Score Baseline to Week 24 by Orphan Disease**
- **Figure 6. EQ-5D-5L and FACT-BMT at Week 24**

**CONCLUSIONS**
- In REACH3, ruxolitinib treatment led to greater improvements in both physician-assessed cGVHD outcomes and PROs compared with BAT.
- Among patients with available steroid data, a greater percentage of patients treated with ruxolitinib vs BAT were receiving steroid doses <7.5 mg/day at week 24 (54.2% vs 43.1%).
- No difference between arms was observed in the FACT-BMT (ruxolitinib, n=118; BAT, n=116).
- EQ-5D-5L scores were numerically higher with ruxolitinib than with BAT (0.66 vs 0.65).
- As demonstrated by reductions on the psychological, energy, and nutrition subscales, overall symptom burden not directly tied to organ responses was also better with ruxolitinib than with BAT.

**Abbreviations**
- cGVHD: Chronic Graft-vs-Host Disease
- CR: Complete response
- EQ-5D-5L: EuroQol-5D-5L
- FACT-BMT: Functional Assessment of Cancer Therapy-Bone Marrow Transplantation
- EQ-SD: EQ-5D-5L score
g- FACT: Functional Assessment of Cancer Therapy
- HCV: Hepatitis C virus
- HLA: Human leukocyte antigen
- JLRS: Japanese Language Revamp Scale
- mCR: Major clinical response
- mLSS: Modified Summary Symptom Score
- PGIC: Patient Global Impression of Change
- PGIS: Patient Global Impression of Severity
- PRO: Patient-reported outcome
- REACH: Ruxolitinib Evaluation of Adaptive Chances for Chronic GVHD
- SD: Slight or moderate symptoms
- SE: Severe or very severe symptoms
- SR: Severe or very severe symptoms
- SR/D: Steroid-refractory or -dependent
- SSS: Stroke Symptom Score
- T: Total
- U: Uncertainty
- V: Very
- VA: Very much better
- VAS: Visual Analog Scale
- VASG: Visual Analog Scale Global
- VASR: Visual Analog Scale Range
- VCS: Visual Choice Scale
- VRS: Visual Rating Scale
- %: Percentage
- **Methods**
- **Figure 4A. Mean Summary Score Baseline to Week 24 by Orphan Disease**
- **Figure 4B. EQ-5D-5L and FACT-BMT at Week 24**

**References**