Stephanie J. Lee
sjlee@fredhutch.org

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

Stephanie J. Lee,[1] Franco Locatelli,[2] Francis Ayuketang Ayuk,[3] Tsila Zuckerman,[4] Kentaro Fukushima,[5] Carlos Vallejo,[6] Joseph Pidala,[7] Nada Hamad,[8] Ivan Moiseev,[9] Francesca Bonifazi,[10] Shashikant Apte,[11] Robert Zeiser,[12] Valkal Bhatt,[13] Maanasa Gowda,[14] Jackie Han,[14] Tommaso Stefanelli,[15] Mike Zuurman,[15] Takanori Teshima[16]

[1]Fred Hutchinson Cancer Research Center, Seattle, WA; [2]Dipartimento di Oncoematologia Pediatrica, IRCCS, Ospedale Pediatrico Bambino Gesù, Sapienza, Università di Roma, Rome, Italy; [3]Universitaetsklinikum Hamburg Eppendorf, Hamburg, Germany; [4]Rambam Health Care Campus Technion, Haifa, Israel; [5]Department of Hematology and Oncology, Osaka University Graduate School of Medicine, Suita, Japan; [6]Hospital de Donostia, San Sebastián, Spain; [7]Moffitt Cancer Center, Tampa, FL; [8]Haematology Department, St Vincent's Hospital, Sydney, Australia; [9]St. Petersburg State Medical University, Pavlov, St. Petersburg, Russia; [10]IRCCS Azienda Ospedaliero-Universitaria di Bologna, Bologna, Italy; [11]Sahyadri Speciality Hospital, Pune, India; [12]Department of Medicine I, Faculty of Medicine, Medical Center, University of Freiburg, Freiburg, Germany; [13]Incyte Corporation, Wilmington, DE; [14]Novartis Pharmaceuticals Corporation, East Hanover, NJ; [15]Novartis Pharma AG, Basel, Switzerland; [16]Hokkaido University Hospital, Sapporo, Japan

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https://bit.ly/LeeSJ3909?r=qr

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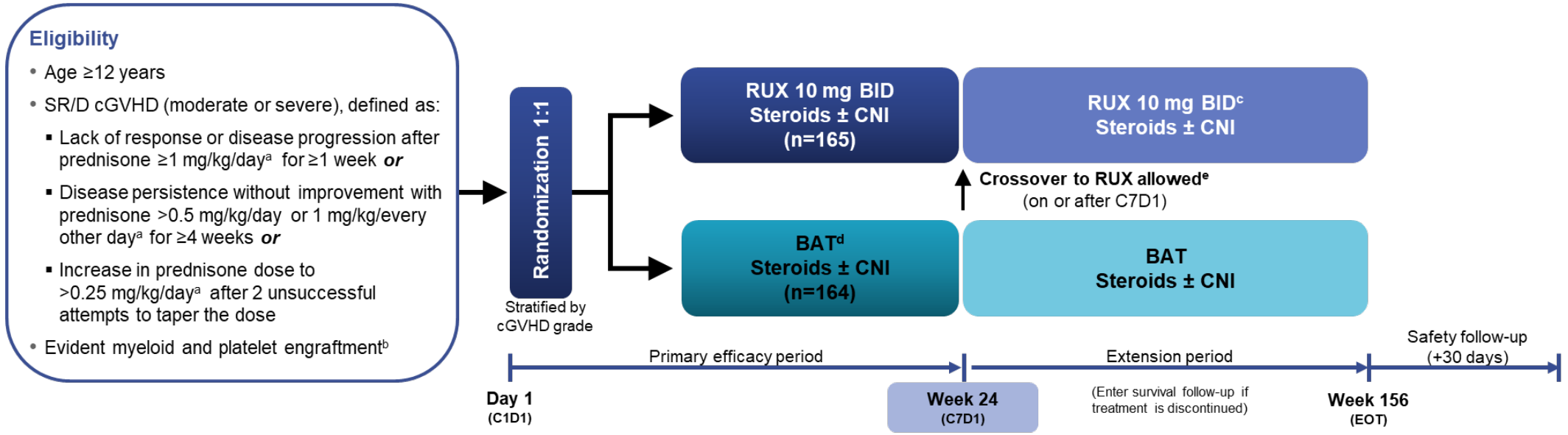
Methods—Study Design



- **Primary endpoint**: ORR (complete response + partial response) at week 24 using NIH consensus criteria for response
- **Key secondary endpoints**
  - Failure-free survival
  - Modified Lee symptom scale response at week 24 (≥7-point reduction from baseline in the summary symptom)
- **Other endpoints:** additional PROs (FACT-BMT, EQ-5D-5L, PGIS, PGIC) collected at baseline and every 4 weeks through week 24 or until treatment failure or discontinuation from the main study period

Zeiser, et al. *N Engl J Med*. 2021;385:228-238. BAT, best available therapy; BID, twice daily; C, cycle; cGVHD, chronic graft-versus-host disease; CNI, calcineurin inhibitor; CR, complete response; D, day; EOT, end of treatment period; FFS, failure-free survival; mLSS, modified Lee Symptom Scale; NRM, nonrelapse mortality; ORR, overall response rate; PR, partial response; RUX, ruxolitinib; SR, steroid refractory/dependent. [a] Or prednisone equivalent. [b] Absolute neutrophil count >1000/mm^3 and platelet count ≥25,000/mm^3. [c] Chosen by the investigator at randomization and could include extracorporeal photopheresis, low-dose methotrexate, mycophenolate mofetil, everolimus, sirolimus, infliximab, rituximab, pentostatin, imatinib, or ibrutinib. [d] RUX tapering was permitted after C7D1 for responding patients. [e] On or after C7D1, patients randomized to BAT who progressed, had a mixed or unchanged response, developed toxicity to BAT, or experienced a cGVHD flare were allowed to cross over from BAT to ruxolitinib.

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Baseline Characteristics

Variable	Ruxolitinib (N=165)	BAT (N=164)
Age, median (range), years	49.0 (13.0-73.0)	50.0 (12.0-76.0)
Female, n (%)	56 (33.9)	72 (43.9)
Prior aGVHD, n (%)	92 (55.8)	88 (53.7)
cGVHD severity, n (%)[a]		
Mild	1 (0.6)	1 (0.6)
Moderate	67 (40.6)	74 (45.1)
Severe	97 (58.8)	89 (54.3)
Total mLSS score, median (range)	18.67 (0-79.6)[b]	18.54 (0.7-54.4)[c]
Baseline mLSS score <7, n (%)	23 (13.9)	19 (11.6)
Donor type, n (%)[b]		
Related/unrelated	91 (54.5)/76 (45.5)	87 (52.1)/80 (47.9)
Prior systemic therapy for cGVHD, n (%)[c]		
Glucocorticoid only	70 (42.4)	81 (49.4)
Glucocorticoid + CNI	68 (41.2)	69 (42.1)
Glucocorticoid + CNI + other systemic therapy	10 (6.1)	4 (2.4)
Glucocorticoid + other systemic therapy	14 (8.5)	9 (5.5)
Missing	3 (1.8)	1 (0.6)
T-cell depletion, n (%)	22 (13.2)	16 (9.6)
Organ involvement, n (%)		
Mouth	97 (58.8)	103 (62.8)
GI	39 (23.6)	36 (22.0)
Lung	74 (44.8)	67 (40.9)
Eyes	97 (58.8)	92 (56.1)
Joints and fascia	48 (29.1)	42 (25.6)
Liver	42 (25.5)	40 (24.4)
Skin	121 (73.3)	113 (68.9)
Genital tract	14 (8.5)	17 (10.4)
Missing	0	1 (0.6)

aGVHD, acute graft-vs-host disease; BAT, best-available therapy; cGVHD, chronic graft-vs-host disease; CNI, calcineurin inhibitor; GI, gastrointestinal; mLSS, modified Lee Symptom Score.

[a] Severity was graded according to National Institutes of Health consensus staging criteria at screening. Enrollment of patients with mild cGVHD was considered a protocol deviation. [b] n=149. [c] n=141.

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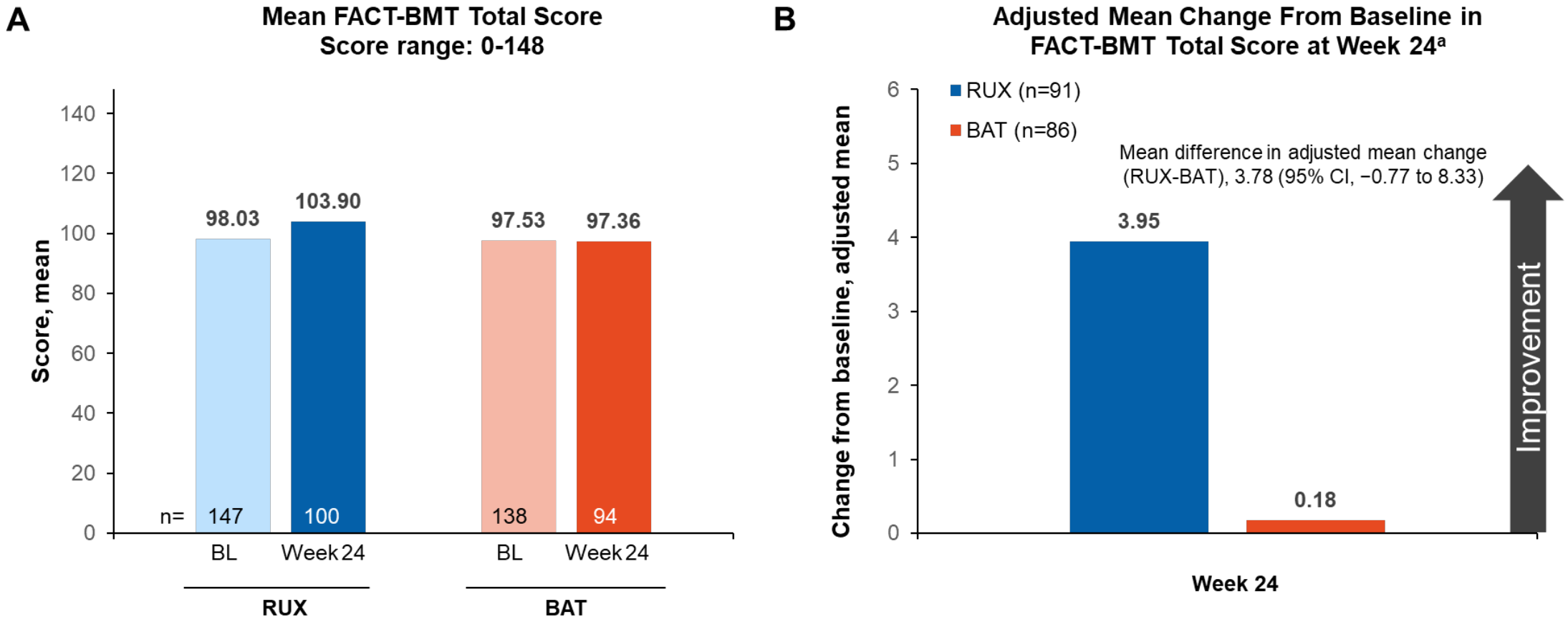
Mean Change From Baseline in FACT-BMT Total Score at Week 24



BAT, best available therapy; BL, baseline; FACT-BMT, Functional Assessment of Cancer Therapy-Bone Marrow Transplantation; RUX, ruxolitinib.

[a] n is the number of patients with observation at both baseline and week 24. Adjusted means and CIs are from a mixed linear repeated measures model that includes treatment, visit, treatment × visit, stratification factors, and baseline 5-level EQ-5D index score as fixed effects, and patient as random effect. The correlation of repeated measures within a patient is estimated with an unstructured covariance matrix.

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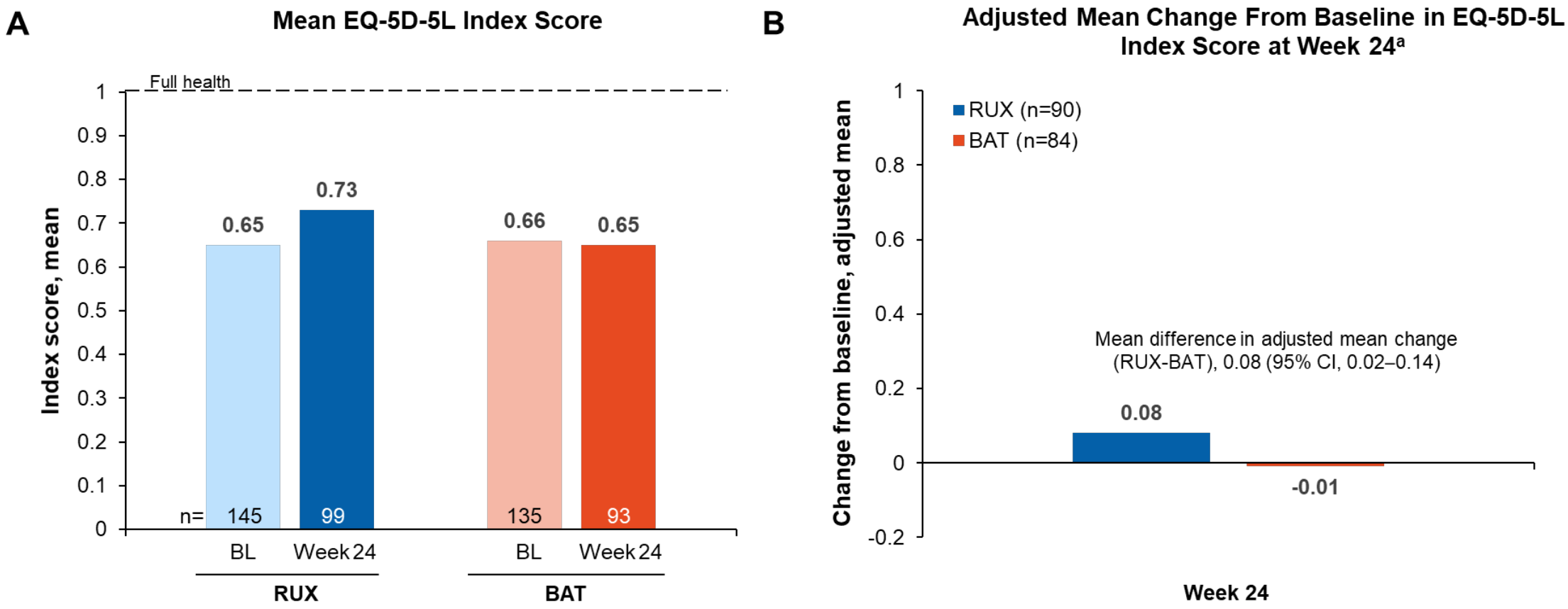
Mean EQ-5D-5L Index Score at Baseline and Week 24



BAT, best-available therapy; BL, baseline; EQ-5D-5L, 5-level EQ-5D; RUX, ruxolitinib; VAS, visual analog scale.

[a] n is the number of patients with observation at both baseline and week 24. Adjusted means and CIs are from a mixed linear repeated measures model that includes treatment, visit, treatment × visit, stratification factors, and baseline 5-level EQ-5D index score as fixed effects, and patient as random effect. The correlation of repeated measures within a patient is estimated with an unstructured covariance matrix.

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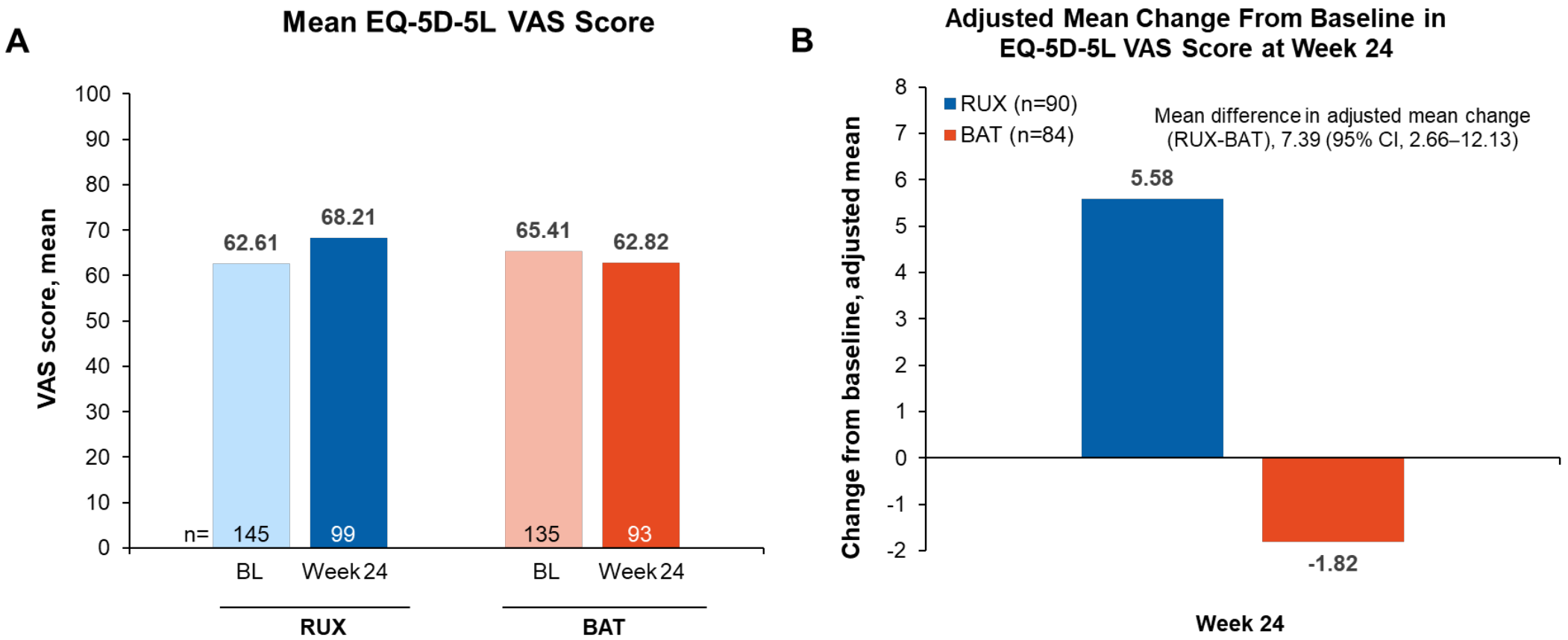
Mean EQ-5D-5L VAS Score at Baseline and Week 24



BAT, best-available therapy; BL, baseline; EQ-5D-5L, 5-level EQ-5D; RUX, ruxolitinib.

[a] n is the number of patients with observation at both baseline and week 24. Adjusted means and CIs are from a mixed linear repeated measures model that includes treatment, visit, treatment × visit, stratification factors, and baseline 5-level EQ-5D index score as fixed effects, and patient as random effect. The correlation of repeated measures within a patient is estimated with an unstructured covariance matrix.

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