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Supplemental Tables for:
FDA Approval Summary: Ruxolitinib for Treatment of Steroid-Refractory Acute Graft-Versus-Host Disease

Donna Przepiorka et al.

Table S1. Efficacy Endpoint Definitions

Endpoint	Definition
Complete Response (CR)	Stage 0 for each organ (skin, liver and GI tract), and no intervening additional therapy
Very Good Partial Response (VGPR)	Improvement by at least 1 stage in 1 or more organs and – Skin: No bullae and no residual erythematous rash involving < 25% of the body surface, and – Liver: Total serum bilirubin concentration < 2 mg/dL or < 25% of baseline at enrollment, and – Gut: Tolerating food or enteral feeding, predominantly formed stools, no overt gastrointestinal bleeding or abdominal cramping, and no more than occasional nausea or vomiting, and – No intervening additional therapy
Partial Response (PR)	Improvement by at least 1 stage in 1 or more organs without progression in other organs, and no intervening additional therapy
Duration of Response	Interval from the Day-28 response to the day of progression,[a] new systemic therapy[b] for aGVHD or death from any cause
Durability	Interval from the Day-28 response to the day of new therapy[b] for a GVHD or death from any cause

[a] Progression is defined as worsening by at least one stage in any organ without improvement in stage in other organs in comparison to the prior response assessment.
[b] New therapy is defined as a new systemic treatment for aGVHD or an increase in the dose of corticosteroids to methylprednisolone 2 mg/kg (+/- 10%) equivalent.

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Table S2. Subpopulation Analysis of Response

	Patients	ORR		CR	
	N	n (%)		n (%)	
Primary Analysis	49	28	(57.1%)	15	(30.6%)
Age					
< 65 Years	43	26	(60.5%)	14	(32.6%)
≥ 65 Years	6	2	(33.3%)	1	(16.7%)
Sex					
Female	26	14	(53.9%)	5	(19.2%)
Male	23	14	(60.9%)	10	(43.5%)
Race					
White	45	26	(57.8%)	13	(28.9%)
Black or African American	2	1	(50.0%)	1	(50.0%)
Asian	2	1	(50.0%)	1	(50.0%)
Ethnicity					
Hispanic	7	4	(57.1%)	3	(42.9%)
Not Hispanic	41	24	(58.5%)	12	(29.3%)
Not Reported	1	0	0	0	0
Donor					
Matched Related	17	10	(58.8%)	5	(29.4%)
Matched Unrelated	20	10	(50.0%)	5	(25.0%)
Mismatched	12	8	(66.7%)	5	(41.7%)
Baseline GVHD Grade					
Grade 2	13	13	(100%)	8	(61.5%)
Grade 3	27	11	(40.7%)	5	(18.5%)
Grade 4	9	4	(44.4%)	2	(22.2%)
Stem Cell Type					
Apheresis	39	20	(51.3%)	12	(30.8%)
Marrow	9	7	(77.8%)	3	(33.3%)
Cord Blood	1	1	(100%)	0	0
$\log_{10}$ ST2 ng/mL					
< 5.5	23	19	(82.6%)	12	(52.2%)
≥ 5.5	24	9	(37.5%)	3	(12.5%)
MB Score					
< 0.291	8	7	(87.5%)	3	(37.5%)
≥ 0.291	39	21	(53.9%)	12	(30.8%)

Abbreviations: CR, complete response; MB, MAGIC biomarker; ORR, overall response that includes CR + VGPR + PR.

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Table S3: Grouped Terms Used for FDA Analyses of Adverse Events

Adverse Reaction	**MedDRA[a] Level Used to Select Adverse Event Terms**
Bacterial infection	HLGT Bacterial infectious disorders
Diarrhea	HLT Diarrhoea (excl infective)
Dizziness	SMQ Vestibular disorders
Dyspnea	HLT Breathing abnormalities
Fatigue	HLT Asthenic conditions
Fungal infection	HLGT Fungal infectious disorders
Gastrointestinal pain	HLT Gastrointestinal and abdominal pains (excl oral and throat)
Hemorrhage	SMQ Haemorrhage terms (excl laboratory terms)
Infections	HLGT Infections - pathogen unspecified
Jaundice	HLT Cholestasis and jaundice
Edema	HLT Oedema NEC
Rash	HLT Rashes, eruptions and exanthems NEC
Renal injury	HLT Renal failure and impairment
Thrombosis	SMQ Embolic and thrombotic events
Viral infection	HLGT Viral infectious disorders

Abbreviations: HLGT, High Level Group Term; HLT High Level Term; SMQ, Standardized MedDRA Query

[a] MedDRA version 19.1 used

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Table S4: Infections As Reported in the Safety Population

Infection Group	Preferred Term	All Grades (N=71)	
Bacterial infection	Enterococcal infection	6	(8%)
	Staphylococcal infection	4	(6%)
	Klebsiella bacteraemia	2	(3%)
	Pseudomonas infection	2	(3%)
	Staphylococcal bacteraemia	2	(3%)
	Urinary tract infection bacterial	2	(3%)
	Cellulitis	1	(1%)
	Citrobacter infection	1	(1%)
	Clostridium difficile infection	1	(1%)
	Enterobacter bacteraemia	1	(1%)
	Escherichia urinary tract infection	1	(1%)
	Klebsiella infection	1	(1%)
	Otitis media bacterial	1	(1%)
	Pneumonia bacterial	1	(1%)
	Pneumonia legionella	1	(1%)
	Pseudomonal bacteraemia	1	(1%)
	Staphylococcal sepsis	1	(1%)
	Streptococcal bacteraemia	1	(1%)
	Streptococcal infection	1	(1%)
Protozoal infection	Cryptosporidiosis infection	1	(1%)
Fungal infection	Bronchopulmonary aspergillosis	3	(4%)
	Candida infection	2	(3%)
	Oral candidiasis	2	(3%)
	Hepatic infection fungal	1	(1%)
	Mycotic endophthalmitis	1	(1%)
	Oesophageal candidiasis	1	(1%)

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Infection Group	Preferred Term	All Grades (N=71)	
	Oral fungal infection	1	(1%)
	Sinusitis fungal	1	(1%)
Infections Unspecified	Sepsis	8	(11%)
	Urinary tract infection	6	(8%)
	Bacteraemia	5	(7%)
	Pneumonia	5	(7%)
	Device related infection	4	(6%)
	Lung infection	4	(6%)
	Septic shock	4	(6%)
	Skin infection	3	(4%)
	Upper respiratory tract infection	3	(4%)
	Cystitis	1	(1%)
	Enterocolitis infectious	1	(1%)
	Fungaemia	1	(1%)
	Gastroenteritis	1	(1%)
	Kidney infection	1	(1%)
	Peritonitis	1	(1%)
	Pharyngitis	1	(1%)
	Sialoadenitis	1	(1%)
	Sinusitis	1	(1%)
Viral infections	Cytomegalovirus infection	9	(13%)
	BK virus infection	5	(7%)
	Cytomegalovirus viraemia	4	(6%)
	Adenovirus infection	2	(3%)
	Corona virus infection	2	(3%)
	Respiratory syncytial virus infection	2	(3%)
	Cytomegalovirus chorioretinitis	1	(1%)
	Enterovirus infection	1	(1%)

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Infection Group	Preferred Term	All Grades (N=71)	
	Human herpesvirus 6 infection	1	(1%)
	Influenza	1	(1%)
	Parainfluenzae virus infection	1	(1%)
	Rhinovirus infection	1	(1%)
	Urinary tract infection viral	1	(1%)