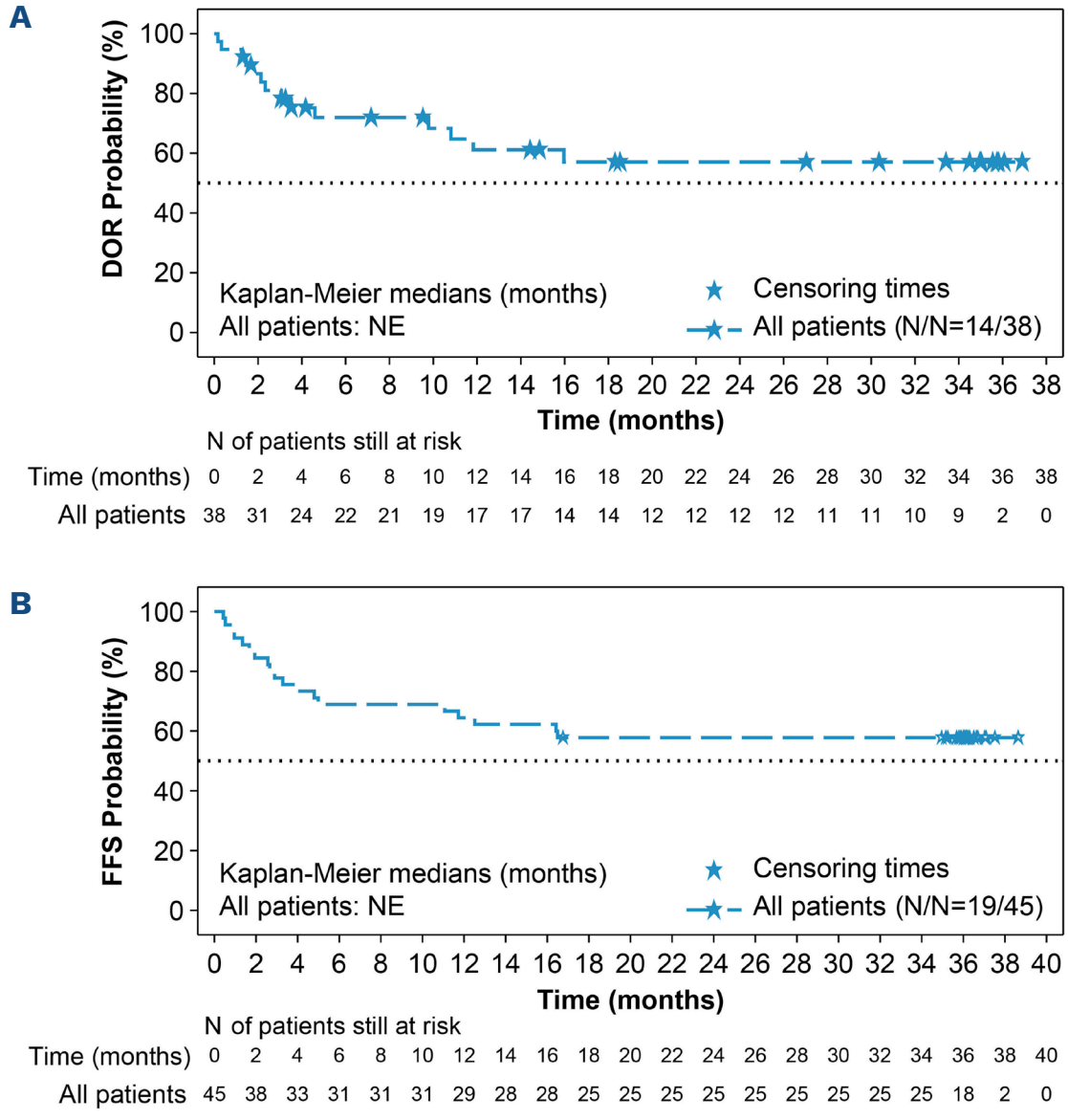
Ruxolitinib in treatment-naive or corticosteroid-refractory pediatric patients with chronic graft-*versus*-host disease: final analysis of the phase II REACH5 trial

Chronic graft-*versus*-host disease (cGVHD) remains a major limitation for the success of allogeneic hematopoietic stem cell transplantation (allo-HSCT) and can affect 30% to 70% of patients.[1] Although corticosteroids (CS) are the standard-of-care first-line treatment for GVHD,[2] up to 50% of patients become steroid refractory (SR).[3,4] In second line, evidence for pediatric patients is limited and largely from adult cohorts. Ruxolitinib, a selective JAK1/JAK2 inhibitor, is approved for patients ≥12 years across many countries and regions,[5,6] supported by data in patients with both acute GVHD (aGVH-DH,[7]) and cGVHD.[1] In the European Union, the indication was expanded to pediatric patients ≥28 days (aGVHD) and ≥6 months (cGVHD) with inadequate response to CS or other systemic therapies (with a 5 mg/mL oral solution available for children with difficulty swallowing), based on REACH4[8] and REACH5[9] outcomes. The REACH5 study (*clinicaltrials gov. Identifier: NCT03774082*) was a phase II, open-label, single-arm, multicenter study of ruxolitinib added to CS in pediatric patients with moderate to severe cGVHD after allo-HSCT. A pre-specified interim analysis demonstrated that ruxolitinib was active and well tolerated in both treatment-naive and SR patients aged ≥2 to <18 years with cGVHD.[9] In this letter, we report the final analysis results from REACH5 with a median follow-up of 36 months. Overall, long-term outcomes confirm ruxolitinib efficacy in patients aged 2 to <18 years with treatment-naive or SR-cGVHD, improving overall response rate (ORR), duration of response (DOR), best overall response (BOR), failure-free survival (FFS), and CS-free response, and with no new safety concerns.

The REACH5 study design, patient baseline characteristics and primary analysis results have been published.[9] The study complied with the Declaration of Helsinki and International Conference on Harmonization Good Clinical Practice Guidelines and the protocol was approved by independent ethics committees/review boards. Parents or legal guardians provided written consent for patient participation.



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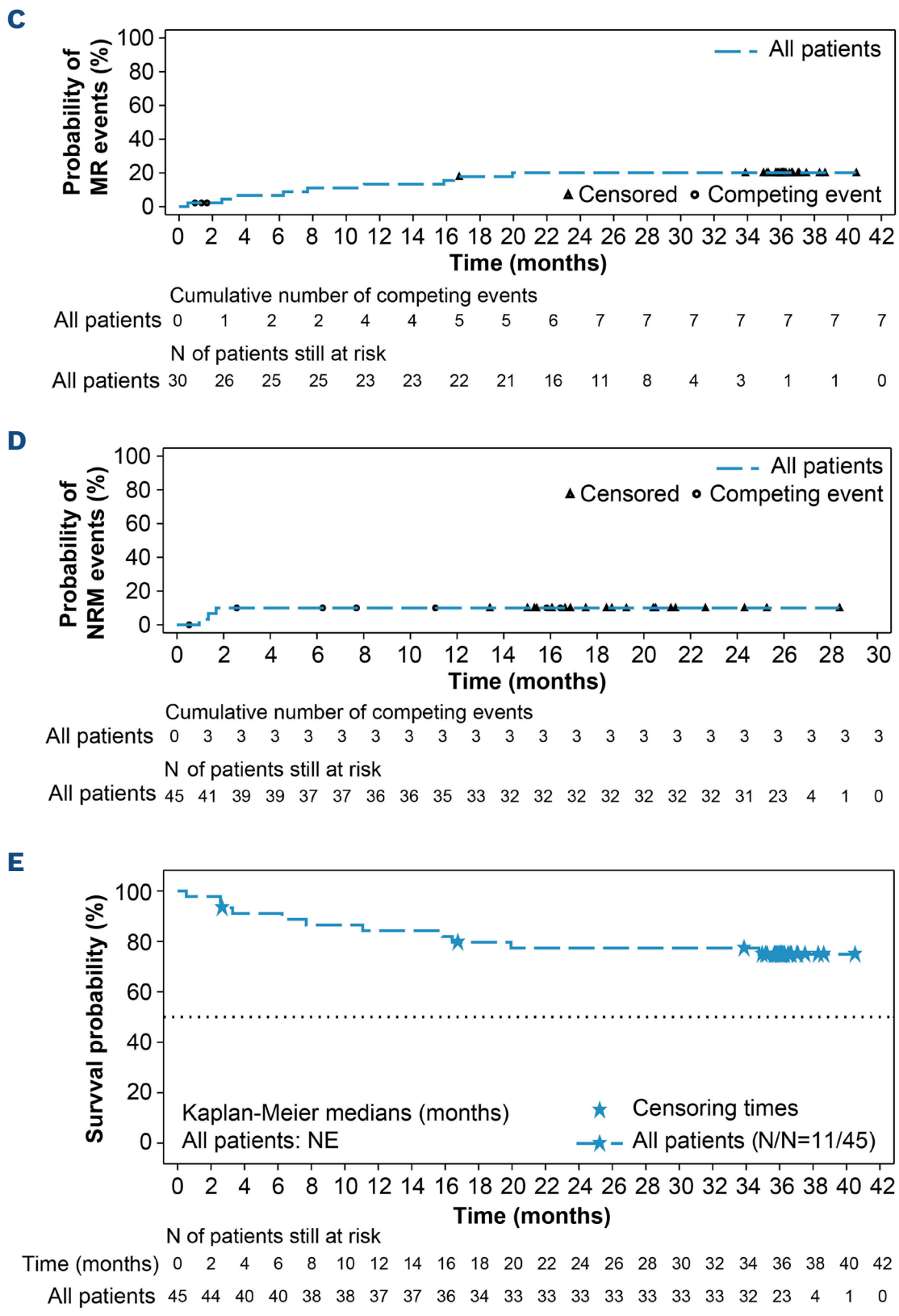

Figure 1. Secondary endpoints. (A) Duration of response (DOR). (B) Failure-free survival (FFS). (C) Malignancy relapse/recurrence (MR). (D) Non-relapse mortality (NRM). (E) Overall survival (OS). (A) DOR, which was assessed for responders only, was defined as the time from first response until chronic garft-*versus*-host disease (cGVHD) progression, death, or the date of additional systemic therapy for cGVHD. (B) FFS was defined as the time from date of treatment to any of the following: relapse/recurrence of the underlying disease, death due to underlying disease, NRM, or addition of another systemic therapy for cGVHD. (C) MR was defined as time from date of treatment assignment to the date of relapse or recurrence of the underlying disease. The competing risk included death due to NRM. (D) NRM was defined as the time from date of treatment assignment to the date of death not due to underlying disease relapse/recurrence. The competing risk included hematologic MR. (E) OS was defined as time from the date of treatment assignment to date of death due to any cause. NE: not evaluable.

In brief, 45 patients were enrolled and received at least one dose of ruxolitinib, per age-based treatment group assignment (≥12 to <18 years: 10 mg twice daily [bid]; ≥6 to <12 years: 5 mg bid, ≥2 to <6 years 4 mg/m^2 bid). Ruxolitinib was administered in 28-day cycles until approximately 36 months (39 cycles) unless interruption or reduction was required to manage toxicity or for progressive disease. Ruxolitinib tapering due to complete response (CR) or partial response (PR) was permitted on/after cycle 7 day 1. Study visits occurred weekly during cycle 1, once every 28 days from cycle 2 day 1 up to cycle 7 day 1, on cycle 9 day 1, and every 12 weeks thereafter until cycle 39 day 1. *Online Supplementary Table S1* summarizes the patient disposition. BOR, DOR, FFS, incidence of malignancy relapse/progression (MR), non-relapse

mortality (NRM), overall survival (OS), graft failure, CS-free response rate, and safety were analyzed up to 36 months post treatment initiation and are reported here.

Baseline characteristics were generally balanced across age groups, except for a greater proportion of patients with SR-cGVHD in the ≥6 to <12-year group (75.0%) compared with the ≥12 to <18-year group (54.5%) and the ≥2 to <6-year group (57.1%); a lower proportion of treatment-naive patients had severe cGVHD (47.1%) compared with SR-GvHD patients (67.9%). A significant proportion of patients had lung (31.1%) or liver involvement (22.2%), driven mainly by the ≥12 to <18-year group (lung: 50.0%; liver: 27.3%)

In this final analysis, the BOR rate was 84.4% (90% confidence interval [CI]: 72.8-92.5), with 14 patients (31.1%) showing CR and 24 patients (53.3%) showing PR as best responses. Compared to BOR previously reported up to cycle 7 day 1 (82.2%; 90% CI: 70.2-90.8),[9] one additional patient achieved PR after six cycles of treatment, and eight patients improved their response from PR to CR.

The median DOR was not reached for responders (95% CI: 9.8-not evaluable [NE]) (Figure 1A). Fourteen of 38 responders (36.8%) lost response; the most common event was the addition of new systemic therapy (7 patients, 18.4%). The estimated probability to still be in response after 12 months and 18 months was 61.2% (95% CI: 42.2-75.6) and 57.1% (95% CI: 37.9-72.3), respectively, with this percentage remaining

Table 1. Summary of daily systemic corticosteroid reduction and systemic corticosteroid-free response at study end.

Reduction of daily systemic CS dose by the end of study (safety analysis set)			**All patients N=45 N (%)**
Patients taking CS at baseline			40 (88.9)
Patients completely tapered off*			28 (70.0)
Patients with ≤0.2 mg/kg/day at least once			31 (77.5)
Patients with ≥50% reduction from baseline at least once			33 (82.5)
Systemic CS-free response in treatment-naive and SR-cGVHD (full analysis set)			
	Treatment naive N=17 N (%)	**SR-cGVHD N=28 N (%)**	**All patients N=45 N (%)**
Overall response among CS-free responders			
CR	8 (47.1)	5 (17.9)	13 (28.9)
PR	0	6 (21.4)	6 (13.3)
Overall response among CS-free non-responders			
CR	1 (5.9)	0	1 (2.2)
PR	1 (5.9)	9 (32.1)	10 (22.2)
Unchanged response	2 (11.8)	2 (7.1)	4 (8.9)
Mixed response	0	0	0
Progression	0	1 (3.6)	1 (2.2)
Other†	5 (29.4)	4 (14.3)	9 (20.0)
Unknown	0	1 (3.6)	1 (2.2)
Death	0	1 (3.6)	1 (2.2)
Early discontinuation	0	0	0
Missing visits	0	0	0
Overall CS-free response rate (ORR: CR+PR)	8 (47.1)	11 (39.3)	19 (42.2)
90% CI	26.0-68.9	23.8-56.5	29.7-55.5

Reduction of daily systemic corticosteroid (CS) dose analysis: CS dose (mg) was defined as the sum of prednisone, prednisolone, and methylprednisolone (converted to prednisone by multiplying with factor 1.25). Patients without CS at baseline were not considered for this analysis. Percentages were calculated on the number of patients taking CS at baseline, whereas the percentage in the first line is based on all patients in the safety analysis set. Systemic CS as documented in the dose administration electronic case form. Dose reductions and tapering were considered regardless of the reason. Analysis was performed on the safety analysis set, defined as all patients who received at least 1 dose of study treatment. Patients were analyzed according to the study treatment received, where the treatment received was defined as the assigned dose level of ruxolitinib if the patient took at least 1 dose of that treatment or the first dose level received if the assigned dose level was never received. *Includes patients who stopped CS. Systemic CS-free analysis: systemic CS-free response rate was defined as the proportion of patients taking no systemic CS therapy for at least 1 month and demonstrating a complete response (CR) or partial response (PR) at any time post baseline. Initiation of any new systemic therapy after chronic graft-*versus*-host disease (cGVHD) flare or recurrence led to the patient being considered as a non-responder. The analysis included those in the full analysis set (defined as all patients to whom ruxolitinib was assigned and who received at least 1 dose of ruxolitinib) and includes patients without any systemic CS treatment at baseline. The two-sided 90% confidence interval (CI) for the response rate was calculated using the Clopper Pearson exact method. †Other: patient with additional systemic therapies along with CR/PR per investigator assessment. ORR: overall response rate; SR: steroid refractory.

then unchanged until 36 months.

The median FFS was not reached (95% CI: 11.7-NE) (Figure 1B). Nineteen patients (42.2%) had experienced failure, compared to 18 patients reported in the interim analysis.[9] The most frequent event was the addition of new systemic therapy (24.4%), followed by death (11.1%) and underlying disease relapse/recurrence (6.7%). The 12-month FFS probability was 64.4% (95% CI: 48.7-76.5), and the 18-month FFS probability was 57.8% (95% CI: 42.1-70.6), which was sustained until 36 months.

The estimated cumulative incidence of MR among patients with underlying hematologic malignancy was low (10.0%; 95% CI: 2.5-23.9) at 1 year. Notably, only three events occurred, all within the first 2 months of treatment. No additional relapses were observed during long-term follow-up (Figure 1C). Nine patients (20.0%) had an event of NRM. The estimated cumulative incidence (95% CI) at 12 months and 18 months was 13.3% (95% CI: 5.3-25.0) and 17.8% (95% CI: 8.2-30.3), and remained at 20.1% (95% CI: 9.8-32.9) from 24 to 36 months (Figure 1D).

The median OS was not reached (95% CI: NE-NE). The 12-month and 36-month survival rates were 84.2% (95% CI: 69.7-92.2) and 74.9% (95% CI: 59.3-85.3), respectively.

Of 40 patients (88.9%) taking CS at baseline, 33 patients (82.5%) had ≥50% reduction from baseline at least once and 28 patients (70%) had been completely tapered off (Table 1). The overall CS-free response rate was 42.2% (90% CI: 29.7-55.5), for all patients, with 13 patients (28.9%) showing a CS-free CR and six patients (13.3%) showing a CS-free PR (Table 1). The overall CS-free response rate was slightly higher in

Table 2. Summary of adverse events.

	≥12 to <18 years Ruxolitinib 10 mg bid N=22		≥6 to <12 years Ruxolitinib 5 mg bid N=16		≥2 to <6 years Ruxolitinib 4 mg/m² N=7		All patients N=45	
	All grades N (%)	Grade ≥3 N (%)	All grades N (%)	Grade ≥3 N (%)	All grades N (%)	Grade ≥3 N (%)	All grades N (%)	Grade ≥3 N (%)
AE Treatment-related	22 (100.0) 17 (77.3)	17 (77.3) 11 (50.0)	15 (93.8) 6 (37.5)	9 (56.3) 4 (25.0)	7 (100) 4 (57.1)	5 (71.4) 1 (14.3)	44 (97.8) 27 (60.0)	31 (68.9) 16 (35.6)
SAE Treatment-related	15 (68.2) 8 (36.4)	12 (54.5) 7 (31.8)	7 (43.8) 1 (6.3)	5 (31.3) 0 (0.0)	4 (57.1) 0 (0.0)	4 (57.1) 0 (0.0)	26 (57.8) 9 (20.0)	21 (46.7) 7 (15.6)
Fatal SAE Treatment-related	0 (0.0) 0 (0.0)	0 (0.0) 0 (0.0)	2 (12.5) 0 (0.0)	2 (12.5) 0 (0.0)	1 (14.3) 0 (0.0)	1 (14.3) 0 (0.0)	3 (6.7) 0 (0.0)	3 (6.7) 0 (0.0)
AE leading to discontinuation Treatment-related	5 (22.7) 2 (9.1)	5 (22.7) 2 (9.1)	1 (6.3) 1 (6.3)	0 (0.0) 0 (0.0)	1 (14.3) 0 (0.0)	1 (14.3) 0 (0.0)	7 (15.6) 3 (6.7)	6 (13.3) 2 (4.4)
AE leading to dose adjustment/interruption	7 (31.8)	6 (27.3)	3 (18.8)	3 (18.8)	2 (28.6)	2 (28.6)	12 (26.7)	11 (24.4)
AE requiring additional therapy	20 (90.9)	14 (63.6)	14 (87.5)	9 (56.3)	7 (100)	4 (57.1)	41 (91.1)	27 (60.0)
AE regardless of study treatment relationship by preferred term (>10%)*								
N of patients with at least 1 event	22 (100)	17 (77.3)	15 (93.8)	9 (56.3)	7 (100)	5 (71.4)	44 (97.8)	31 (68.9)
Anemia	7 (31.8)	6 (27.3)	2 (12.5)	2 (12.5)	2 (28.6)	1 (14.3)	11 (24.4)	9 (20.0)
COVID-19	5 (22.7)	2 (9.1)	3 (18.8)	0	2 (28.6)	0	10 (22.2)	2 (4.4)
Pyrexia	5 (22.7)	0	4 (25.0)	0	1 (14.3)	0	10 (22.2)	0
Decreased neutrophil count	5 (22.7)	5 (22.7)	2 (12.5)	2 (12.5)	2 (28.6)	2 (28.6)	9 (20.0)	9 (20.0)
Upper respiratory tract infection	1 (4.5)	0	4 (25.0)	0	4 (57.1)	0	9 (20.0)	0
Neutropenia	2 (9.1)	1 (4.5)	4 (25.0)	3 (18.8)	2 (28.6)	1 (14.3)	8 (17.8)	5 (11.1)
Headache	5 (22.7)	0	2 (12.5)	0	0	0	7 (15.6)	0
Hypertension	3 (13.6)	1 (4.5)	3 (18.8)	1 (6.3)	1 (14.3)	0	7 (15.6)	2 (4.4)
Decreased platelet count	5 (22.7)	4 (18.2)	2 (12.5)	2 (12.5)	0	0	7 (15.6)	6 (13.3)
Cough	3 (13.6)	0	3 (18.8)	0	0	0	6 (13.3)	0
Pneumonia	4 (18.2)	2 (9.1)	2 (12.5)	1 (6.3)	0	0	6 (13.3)	3 (6.7)
Increased ALT	3 (13.6)	2 (9.1)	1 (6.3)	1 (6.3)	1 (14.3)	1 (14.3)	5 (11.1)	4 (8.9)
Cytomegalovirus infection reactivation	3 (13.6)	0	2 (12.5)	0	0	0	5 (11.1)	4 (8.9)
Diarrhea	2 (9.1)	0	2 (12.5)	0	1 (14.3)	0	5 (11.1)	0
Nasopharyngitis	1 (4.5)	0	3 (18.8)	0	1 (14.3)	0	5 (11.1)	0
Thrombocytopenia	3 (13.6)	2 (9.1)	1 (6.3)	1 (6.3)	1 (14.3)	0	5 (11.1)	3 (6.7)
Vomiting	3 (13.6)	0	2 (12.5)	0	0	0	5 (11.1)	0
Decreased white blood cell count	2 (9.1)	2 (9.1)	2 (12.5)	2 (12.5)	1 (14.3)	0	5 (11.1)	4 (8.9)

A patient with multiple severity grades for an adverse event (AE) was only counted under the maximum grade. "Treatment related" refers to relationship to ruxolitinib. MedDRA version 27.0, CTCAE version 4.03. *AE occurring during treatment or within 30 days of the last study medication are summarized. ALT: alanine aminotransferase; bid: twice daily; COVID-19: coronovirus disease 2019; CTCAE: Common Terminology Criteria for Adverse Events; MedDRA: medical dictionary for regulatory activities; SAE: serious AE.

treatment-naive patients (47.1%, 90% CI: 26.0-68.9) than in patients with SR-GVHD (39.3%, 90% CI: 23.8-56.5) (Table 1). There were no further cases of graft failure following the two (4.44%) previously reported.[9]

The median duration of exposure to ruxolitinib was 12.7 months (range, 0.5-37.6 months). The median duration of exposure was highest in the ≥2 to <6 years group (15.8 months), followed by the ≥6 to <12 years group (13.6 months), and lowest in the ≥12 to <18 years group (9.5 months). Safety observations are shown in Table 2. Almost all patients in the study (97.8%) had at least one adverse event (AE); 31 patients (68.9%) had a grade ≥3 AE. AE leading to discontinuation occurred in seven patients (5 in ≥12 to <18 years, 1 in ≥6 to <12 years, and 1 in ≥2 to <6 years groups, respectively), including alveolar proteinosis, *Aspergillus* infection, COVID-19, herpes zoster, retinal vein occlusion, thrombocytopenia and transplant failure (1 patient each), and were all serious AE, except thrombocytopenia. The most common AE of special interest were infections excluding tuberculosis (75.6%), which were higher in ≥6 to <12 years and in ≥2 to <6 years groups than in the ≥12 to <18 years group, with COVID-19 reported as the most frequent infection. On-treatment deaths occurred in three patients (2 in ≥6 to <12 years and 1 in ≥2 to <6 years groups) all due to AE but not related to ruxolitinib. Post-treatment deaths (≥30 days after the last ruxolitinib dose) occurred in eight patients (6 in ≥12 to <18 years, 1 in ≥6 to <12 years, and 1 in ≥2 to <6 years groups) due to thrombotic microangiopathy, cardiac arrest, multiple organ dysfunction syndrome, COVID-19, fungal pneumonia, transplant (graft) failure, acute lymphoblastic leukemia (ALL), and recurrent leukemia (1 patient each). One additional patient died compared with the interim analysis (N=7),[9] due to ALL. These overall efficacy and safety observations confirm the previously reported findings.[9] Ruxolitinib was effective in patients aged ≥2 to <18 years with either treatment-naive or SR-cGVHD. Benefits were seen for BOR, ORR, DOR, FFS, and CS-free response, considering the severity of the disease in these patients. The estimated probability of being in response at 12 and 36 months was 61.2% and 57.1%, respectively; similar to the DOR observed in the REACH3 study, which was conducted in adults and adolescents with moderate to severe cGVHD (68.5% at 12 months[10] and 59.6% at 36 months).[11] Two noteworthy observations from the current analysis are; over time, a proportion of patients with PR reached CR, and, treatment-naive patients had a higher likelihood of attaining CS-free CR than those with SR-cGVHD. These promising results, however, are based on a limited sample size and, therefore, further investigation is needed to fully evaluate the role of ruxolitinib as front line therapy in children with cGVHD. Our trial demonstrates the benefit of ruxolitinib at 4 mg/m^2; however, European Union labeling recommends 8 mg/m^2 in patients aged 6 months to <6 years[6] in light of study findings showing lower ruxolitinib systemic exposure in this age group,[9] and subsequent modeling and simulation results (*data not published*). In this final analysis, no new safety concerns emerged with longer exposure to ruxolitinib and extended follow-up, and no additional risk identified for treatment-naive patients as compared with SR patients. Overall, these findings support ruxolitinib as a valuable treatment option for pediatric cGVHD.

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Disclosures

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Contributions

Conceptualization, methodology and supervision by FL and CD-dH. Data curation by TS, SP, YS and KS. Formal analysis by SP. Investigation by FL, BA, HJK, KK, YT, AK, MGADM, YC, SB, HJI, TG, M-Y L and CD-dH. Project administration by TS, SP, YS and KS. Visualization by SP. Writing of the original draft by FL, BA, HJK, KK, YT, AK, MGADM, YC, SB, HJI, TG, M-Y L, TS, SP, YS, KS and CD-dH. Writing review and editing by FL, BA, HJK, KK, YT, AK, MGADM, YC, SB, HJI, TG, M-Y L, TS, SP, YS, KS and CD-dH.

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Data-sharing statement

Novartis is committed to sharing with qualified external researchers access to patient-level data and supporting clinical documents from eligible studies. These requests are reviewed and approved by an independent review panel on the basis of scientific merit. All data provided are anonymized to respect the privacy of patients who have participated in the trial, in line with applicable laws and regulations. This trial data availability is in accordance with the criteria and process described on www.clinicalstudydatarequest.com.

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