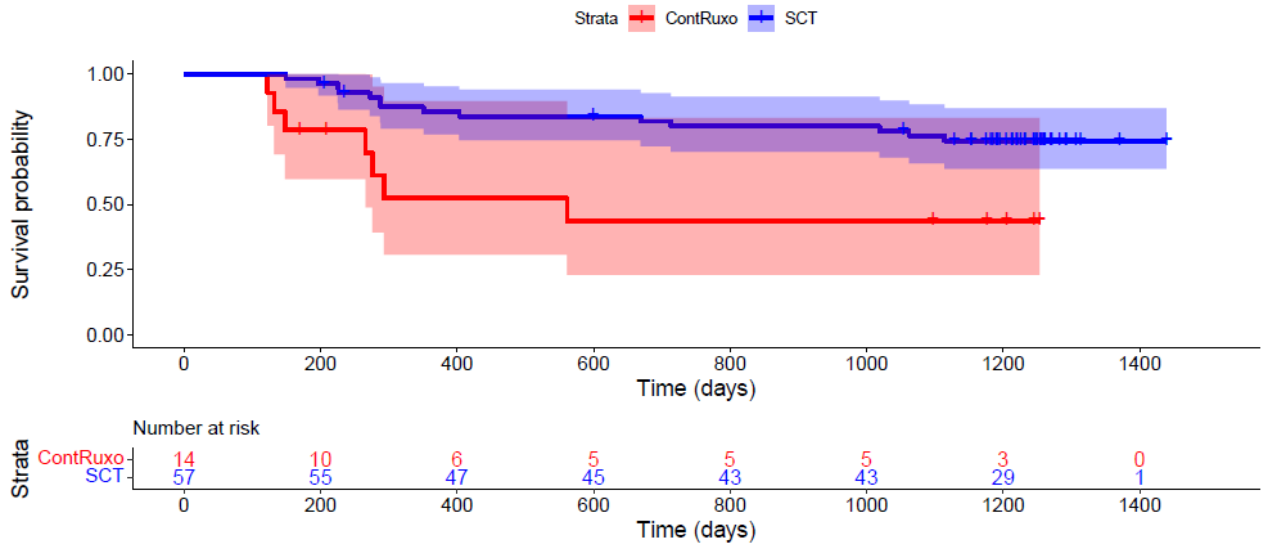
14.2 Efficacy Analysis

14.2.1 Primary endpoints

Figure 14.2.1.1: Event free survival by treatment arm (Kaplan-Meier) - Full Analysis Population



ContRuxo: Ruxolitinib continuous therapy. SCT: Allogeneic stem cell transplantation.

Table 14.2.1.2: Event free survival by treatment arm (Kaplan-Meier) - Full Analysis Population

Treatment arm	Time to event (days)	Survival function estimate	Standard Error	95% lower confidence interval	95% upper confidence interval
ContRuxo	122	0.929	0.069	0.803	1.000
	132	0.857	0.094	0.692	1.000
	148	0.786	0.110	0.598	1.000
	266	0.698	0.128	0.488	0.999
	276	0.611	0.138	0.392	0.952
	293	0.524	0.143	0.306	0.896
	561	0.437	0.144	0.229	0.832
SCT	149	0.982	0.017	0.949	1.000
	197	0.965	0.024	0.918	1.000

All numerical values are rounded to 3 decimal places. SCT: allogeneic stem cell transplantation. Standard error calculated using Greenwood's formula.
Reference- Cantor AB. Projecting the standard error of the Kaplan-Meier estimator. Stat Med. 2001 Jul 30;20(14):2091-7. doi: 10.1002/sim.856. PMID: 11439423.

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Table 14.2.1.2: Event free survival by treatment arm (Kaplan-Meier) - Full Analysis Population

Treatment arm	Time to event (days)	Survival function estimate	Standard Error	95% lower confidence interval	95% upper confidence interval
SCT	225	0.947	0.030	0.890	1.000
	226	0.929	0.034	0.865	0.999
	272	0.911	0.038	0.839	0.989
	287	0.893	0.041	0.815	0.978
	288	0.875	0.044	0.792	0.966
	351	0.856	0.047	0.769	0.954
	403	0.838	0.049	0.747	0.941
	669	0.819	0.052	0.724	0.927
	713	0.801	0.054	0.702	0.914

All numerical values are rounded to 3 decimal places. SCT: allogeneic stem cell transplantation. Standard error calculated using Greenwood's formula.
Reference- Cantor AB. Projecting the standard error of the Kaplan-Meier estimator. Stat Med. 2001 Jul 30;20(14):2091-7. doi: 10.1002/sim.856. PMID: 11439423.

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Table 14.2.1.2: Event free survival by treatment arm (Kaplan-Meier) - Full Analysis Population

Treatment arm	Time to event (days)	Survival function estimate	Standard Error	95% lower confidence interval	95% upper confidence interval
SCT	1,019	0.782	0.056	0.680	0.899
	1,062	0.763	0.058	0.658	0.885
	1,114	0.744	0.059	0.637	0.869

All numerical values are rounded to 3 decimal places. SCT: allogeneic stem cell transplantation. Standard error calculated using Greenwood's formula.
Reference- Cantor AB. Projecting the standard error of the Kaplan-Meier estimator. Stat Med. 2001 Jul 30;20(14):2091-7. doi: 10.1002/sim.856. PMID: 11439423.

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Table 14.2.1.3: Comparison of event free survival at 3 years after start of investigational treatment between SCT and ContRuxo - Full Analysis Population

Time to event	Treatment arm	Survival function estimate, S(t)	Standard Error	Absolute difference in S(t)	95% lower confidence interval	95% upper confidence interval	Z-value	P-value
3 years	ContRuxo	0.437	0.144	0.308	0.003	0.612	1.979	0.048
	SCT	0.744	0.059					

3 years defined as 3 years + 92 days after the date of inclusion to allow for Ruxolitinib induction period.
All numerical values are rounded to 3 decimal places. SCT: allogeneic stem cell transplantation. Standard error calculated using Greenwood's formula.
Reference- Cantor AB. Projecting the standard error of the Kaplan-Meier estimator. Stat Med. 2001 Jul 30;20(14):2091-7. doi: 10.1002/sim.856. PMID: 11439423.

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Table 14.2.1.4: Median survival time for event free survival - Full Analysis Population

Treatment arm	Median survival time [Days]	95% Lower confidence interval	95% Upper confidence interval
ContRuxo	561	266	-
SCT	-	-	-

Table 14.2.1.5: Parameter estimates of event-free survival (stratified Cox regression model) - Full Analysis Population

Number of subjects included	Number of events	Parameter estimate	Standard Error	95% lower confidence interval	95% upper confidence interval
62	18	-1.828697	0.5330278	-2.873412	-0.7839819

Parameter estimates, standard error and confidence intervals estimated via stratified Cox model. Strata are defined by age category (<=55 years vs >55 years), and by Dynamic International Prognostic Scoring System (Category_1 = DIPSS:Intermediate 1/ Intermediate 2 and Category_2 = DIPSS: High-risk).

Table 14.2.1.6: Hazard ratio of event-free survival for treatment "SCT" in comparison with treatment "ContRuxo" (stratified Cox regression model) - Full Analysis Population

Number of subjects included	Hazard Ratio	Standard Error	95% lower confidence interval	95% upper confidence interval	P-value (Wald)	P-value (Logrank)
62	0.161	0.533	0.057	0.457	0.001	0.00017

SCT: allogeneic stem cell transplantation. Strata are defined by age category (<=55 years vs >55 years), and by Dynamic International Prognostic Scoring System (Category_1 = DIPSS:Intermediate 1/ Intermediate 2 and Category_2 = DIPSS: High-risk).

Table 14.2.1.7: Parameter estimates of event-free survival (adjusted Cox regression model) - Full Analysis Population

Parameter	Parameter estimate	Standard Error	95% lower confidence interval	95% upper confidence interval
SCT	-1.857	0.510	-2.855	-0.858
Age (per year)	0.040	0.027	-0.014	0.093
DIPSS Category 2	0.254	0.768	-1.251	1.759

N = 62. SCT: allogeneic stem cell transplantation.
Parameter estimates, standard error and confidence intervals estimated via adjusted Cox model. Strata are defined by by Dynamic International Prognostic Scoring System (Category_1 = DIPSS:Intermediate 1 risk or Intermediate 2 risk; Category_2 = DIPSS: High-risk).

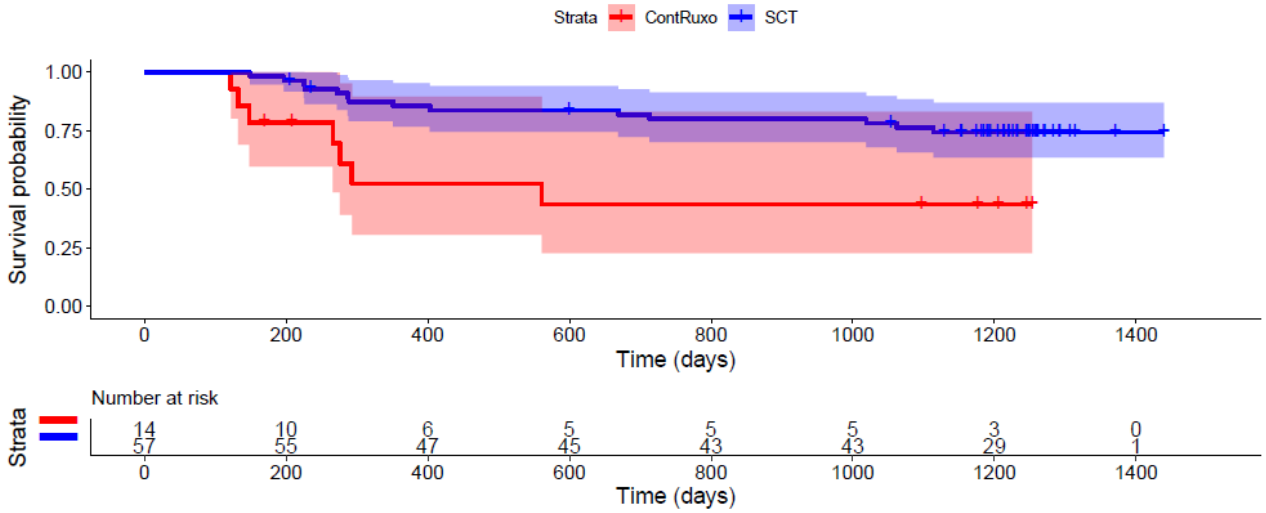
Table 14.2.1.8: Hazard ratio of event-free survival for treatment "SCT" in comparison with treatment "ContRuxo" (adjusted Cox regression model) - Full Analysis Population

Parameter	Hazard Ratio	Standard Error	95% lower confidence interval	95% upper confidence interval	P-value (Wald)	P-value (LogRank)
SCT	0.156	0.510	0.058	0.424	<0.0001	
Age (per year)	1.040	0.027	0.986	1.097	0.147	
DIPSS Category 2	1.290	0.768	0.286	5.809	0.740	
						< 0.0001

N = 62. SCT: allogeneic stem cell transplantation. Strata are defined by by Dynamic International Prognostic Scoring System (Category_1 = DIPSS:Intermediate 1 risk or Intermediate 2 risk; Category_2 = DIPSS: High-risk).

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Figue 14.2.1.9: Event free survival by treatment arm (Kaplan-Meier) - Per protocol population



Note that the Per Protocol Population does not differ from the Full Analysis Population.

ContRuxo: Ruxolitinib continuous therapy. SCT: Allogeneic stem cell transplantation.

Table 14.2.1.10: Event free survival by treatment arm (Kaplan-Meier) - Per protocol population

Treatment arm	Time to event (days)	Survival function estimate	Standard Error	95% lower confidence interval	95% upper confidence interval
ContRuxo	122	0.929	0.069	0.803	1.000
	132	0.857	0.094	0.692	1.000
	148	0.786	0.110	0.598	1.000
	266	0.698	0.128	0.488	0.999
	276	0.611	0.138	0.392	0.952
	293	0.524	0.143	0.306	0.896
	561	0.437	0.144	0.229	0.832
SCT	149	0.982	0.017	0.949	1.000
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All numerical values are rounded to 3 decimal places. SCT: allogeneic stem cell transplantation. Standard error calculated using Greenwood's formula.
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Table 14.2.1.10: Event free survival by treatment arm (Kaplan-Meier) - Per protocol population

Treatment arm	Time to event (days)	Survival function estimate	Standard Error	95% lower confidence interval	95% upper confidence interval
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	272	0.911	0.038	0.839	0.989
	287	0.893	0.041	0.815	0.978
	288	0.875	0.044	0.792	0.966
	351	0.856	0.047	0.769	0.954
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	669	0.819	0.052	0.724	0.927
	713	0.801	0.054	0.702	0.914

All numerical values are rounded to 3 decimal places. SCT: allogeneic stem cell transplantation. Standard error calculated using Greenwood's formula.
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Treatment arm	Time to event (days)	Survival function estimate	Standard Error	95% lower confidence interval	95% upper confidence interval
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	1,062	0.763	0.058	0.658	0.885
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All numerical values are rounded to 3 decimal places. SCT: allogeneic stem cell transplantation. Standard error calculated using Greenwood's formula.
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Table 14.2.1.11: Comparison of event free survival at 3 years after start of investigational treatment between SCT and ContRuxo - Per protocol population

Time to event	Treatment arm	Survival function estimate, S(t)	Standard Error	Absolute difference in S(t)	95% lower confidence interval for absolute difference	95% upper confidence interval for absolute difference	Z-value	P-value
3 years	ContRuxo	0.437	0.144	0.308	0.003	0.612	1.979	0.048
3 years	SCT	0.744	0.059					

3 years defined as 3 years + 92 days after the date of inclusion to allow for Ruxolitinib induction period.
All numerical values are rounded to 3 decimal places. SCT: allogeneic stem cell transplantation. Standard error calculated using Greenwood's formula.
Reference- Cantor AB. Projecting the standard error of the Kaplan-Meier estimator. Stat Med. 2001 Jul 30;20(14):2091-7. doi: 10.1002/sim.856. PMID: 11439423.
Note that the Per Protocol Population does not differ from the Full Analysis Population.

Table 14.2.1.12: Median survival time for event free survival - Per protocol population

Treatment arm	Median survival time [Days]	95% Lower confidence interval	95% Upper confidence interval
ContRuxo	561	266	-
SCT	-	-	-

Note that the Per Protocol Population does not differ from the Full Analysis Population.