Clinical trial results:
A phase II trial in patients with myelofibrosis (primary, post-ET or post PV-MF) treated with the selective JAK2 inhibitor Pacritinib before reduced-intensity conditioning allogeneic stem cell transplantation

Summary

EudraCT number	2015-000195-98
Trial protocol	NL BE
Global end of trial date	31 December 2024

Results information

Result version number	v1 (current)
This version publication date	03 December 2025
First version publication date	03 December 2025

Trial information

Trial identification

Sponsor protocol code	HO134

Additional study identifiers

ISRCTN number	-
ClinicalTrials.gov id (NCT number)	-
WHO universal trial number (UTN)	-

Notes:

Sponsors

Sponsor organisation name	HOVON
Sponsor organisation address	Dr. Molewaterplein 40, Rotterdam, Netherlands,
Public contact	HOVON, Erasmus MC - HOVON, HOVON@erasmusmc.nl
Scientific contact	HOVON, Erasmus MC - HOVON, HOVON@erasmusmc.nl

Notes:

Paediatric regulatory details

Is trial part of an agreed paediatric investigation plan (PIP)	No
Does article 45 of REGULATION (EC) No 1901/2006 apply to this trial?	No
Does article 46 of REGULATION (EC) No 1901/2006 apply to this trial?	No

Notes:

Results analysis stage

Analysis stage	Final
Date of interim/final analysis	02 February 2023
Is this the analysis of the primary completion data?	Yes
Primary completion date	01 February 2023
Global end of trial reached?	Yes
Global end of trial date	31 December 2024
Was the trial ended prematurely?	No

Notes:

General information about the trial

Main objective of the trial:

Improve allo-SCT transplant outcome using a uniform conditioning regimen and pacritinib pretreatment by means of the proportion of patients with a failure within 6 months post-transplant. Events that are considered a failure are: primary graft failure; secondary graft failure; acute GvHD grade 3-4; death whatever the cause is.

Protection of trial subjects:

Insurance and monitoring

Background therapy: -

Evidence for comparator: -

Actual start date of recruitment	01 January 2016
Long term follow-up planned	No
Independent data monitoring committee (IDMC) involvement?	Yes

Notes:

Population of trial subjects

Subjects enrolled per country

Country: Number of subjects enrolled	Netherlands: 51
Country: Number of subjects enrolled	Belgium: 10
Worldwide total number of subjects	61
EEA total number of subjects	61

Notes:

Subjects enrolled per age group

In utero	0
Preterm newborn - gestational age < 37 wk	0
Newborns (0-27 days)	0
Infants and toddlers (28 days-23 months)	0
Children (2-11 years)	0
Adolescents (12-17 years)	0
Adults (18-64 years)	33
From 65 to 84 years	28
85 years and over	0

Subject disposition

Recruitment

Recruitment details: -

Pre-assignment

Screening details:

All subjects gave written informed consent and were screened according to the inclusion- and exclusion criteria

Period 1

Period 1 title	Overall period (overall period)
Is this the baseline period?	Yes
Allocation method	Not applicable
Blinding used	Not blinded

Arms

Arm title	Experimental group

Arm description: -

Arm type	Experimental
Investigational medicinal product name	Pacritinib
Investigational medicinal product code	SB1518
Other name	
Pharmaceutical forms	Capsule, hard
Routes of administration	Oral use

Dosage and administration details:

200 mg twice daily Orally, 2 capsules twice per day at the same time of day with or without food for 28 days

after the first cycle, pacritinib dosage should be reduced to dose level -1
Dose level -1 : 300 mg/day
Dose level -2 : 200 mg/day

Number of subjects in period 1	Experimental group
Started	61
Completed	32
Not completed	29
Adverse reactions (combined)	7
Other	14
Lack of efficacy	8

Baseline characteristics

Reporting groups	
Reporting group title	Overall period

Reporting group description: -

Reporting group values	Overall period	Total	
Number of subjects	61	61	
Age categorical Units: Subjects			
Adults (18-64 years)	33	33	
From 65-84 years	28	28	
Age continuous Units: years			
median	64		
full range (min-max)	34 to 70	-	
Gender categorical Units: Subjects			
Female	19	19	
Male	42	42	

End points

End points reporting groups

Reporting group title	Experimental group

Reporting group description: -

Primary: Primary endpoint

End point title	Primary endpoint[1]

End point description:

End point type	Primary

End point timeframe:

See publication

Notes:

[1] - No statistical analyses have been specified for this primary end point. It is expected there is at least one statistical analysis for each primary end point.
Justification: Statistical data section from publication

End point values	Experimental group			
Subject group type	Reporting group			
Number of subjects analysed	61			
Units: Whole	61			

Attachments (see zip file)	Statistical data section from List of reported SAE's/saedata134-5Aug2025.pdf List of reported non-SAE's/nonsaedata134-5Aug2025.pdf

Statistical analyses

No statistical analyses for this end point

Adverse events

Adverse events information

Timeframe for reporting adverse events:

Adverse events will be reported from the first study-related procedure until 30 days following the last dose of any drug from the protocol treatment schedule or until the start of subsequent systemic therapy for the disease under study, if earlier.

Adverse event reporting additional description:

Adverse events occurring after 30 days should also be reported if considered at least possibly related to the investigational medicinal product by the investigator.

Assessment type	Systematic

Dictionary used

Dictionary name	CTCAE
Dictionary version	4

Reporting groups

Reporting group title	Experimental group

Reporting group description: -

Serious adverse events	Experimental group		
Total subjects affected by serious adverse events			
subjects affected / exposed	21 / 61 (34.43%)		
number of deaths (all causes)	24		
number of deaths resulting from adverse events			
Neoplasms benign, malignant and unspecified (incl cysts and polyps)			
Neoplasms benign, malignant and unspecified (incl cysts and polyps	Additional description: All combined		
subjects affected / exposed	1 / 61 (1.64%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 1		
Vascular disorders			
Vascular disorders	Additional description: All combined		
subjects affected / exposed	1 / 61 (1.64%)		
occurrences causally related to treatment / all	1 / 1		
deaths causally related to treatment / all	1 / 1		
General disorders and administration site conditions			
General disorders and administration site conditions	Additional description: All combined		
subjects affected / exposed	3 / 61 (4.92%)		
occurrences causally related to treatment / all	0 / 4		
deaths causally related to treatment / all	0 / 1		

Respiratory, thoracic and mediastinal disorders			
Respiratory, thoracic and mediastinal disorders	Additional description: All combined		
subjects affected / exposed	1 / 61 (1.64%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Investigations			
Investigations	Additional description: All combined		
subjects affected / exposed	2 / 61 (3.28%)		
occurrences causally related to treatment / all	1 / 2		
deaths causally related to treatment / all	0 / 0		
Cardiac disorders			
Cardiac disorders	Additional description: All combined		
subjects affected / exposed	2 / 61 (3.28%)		
occurrences causally related to treatment / all	0 / 2		
deaths causally related to treatment / all	0 / 0		
Nervous system disorders			
Nervous system disorders	Additional description: All combined		
subjects affected / exposed	4 / 61 (6.56%)		
occurrences causally related to treatment / all	1 / 4		
deaths causally related to treatment / all	0 / 1		
Eye disorders			
Eye disorders	Additional description: All combined		
subjects affected / exposed	1 / 61 (1.64%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Gastrointestinal disorders			
Gastrointestinal disorders	Additional description: All combined		
subjects affected / exposed	4 / 61 (6.56%)		
occurrences causally related to treatment / all	4 / 5		
deaths causally related to treatment / all	0 / 0		
Renal and urinary disorders			
Renal and urinary disorders	Additional description: All combined		
subjects affected / exposed	1 / 61 (1.64%)		
occurrences causally related to treatment / all	1 / 1		
deaths causally related to treatment / all	0 / 0		

Musculoskeletal and connective tissue disorders			
Musculoskeletal and connective tissue disorders	Additional description: All combined		
subjects affected / exposed	1 / 61 (1.64%)		
occurrences causally related to treatment / all	1 / 1		
deaths causally related to treatment / all	0 / 0		
Infections and infestations			
Infections and infestations	Additional description: All combined		
subjects affected / exposed	7 / 61 (11.48%)		
occurrences causally related to treatment / all	3 / 9		
deaths causally related to treatment / all	1 / 1		
Metabolism and nutrition disorders			
Metabolism and nutrition disorders	Additional description: All combined		
subjects affected / exposed	1 / 61 (1.64%)		
occurrences causally related to treatment / all	2 / 2		
deaths causally related to treatment / all	0 / 0		

Frequency threshold for reporting non-serious adverse events: 0 %

Non-serious adverse events	Experimental group		
Total subjects affected by non-serious adverse events			
subjects affected / exposed	55 / 61 (90.16%)		
Neoplasms benign, malignant and unspecified (incl cysts and polyps)			
Neoplasms benign, malignant and unspecified (including cysts and polyps)	Additional description: All combined		
subjects affected / exposed	1 / 61 (1.64%)		
occurrences (all)	1		
Vascular disorders			
Vascular disorders	Additional description: All combined		
subjects affected / exposed	14 / 61 (22.95%)		
occurrences (all)	16		
General disorders and administration site conditions			
General disorders and administration site conditions	Additional description: All combined		
subjects affected / exposed	26 / 61 (42.62%)		
occurrences (all)	35		
Immune system disorders			

Immune system disorders	Additional description: All combined		
subjects affected / exposed	2 / 61 (3.28%)		
occurrences (all)	2		
Respiratory, thoracic and mediastinal disorders			
Respiratory, thoracic and mediastinal disorders	Additional description: All combined		
subjects affected / exposed	6 / 61 (9.84%)		
occurrences (all)	6		
Psychiatric disorders			
Psychiatric disorders	Additional description: All combined		
subjects affected / exposed	3 / 61 (4.92%)		
occurrences (all)	3		
Investigations			
Investigations	Additional description: All combined		
subjects affected / exposed	44 / 61 (72.13%)		
occurrences (all)	333		
Injury, poisoning and procedural complications			
Injury, poisoning and procedural complications	Additional description: All combined		
subjects affected / exposed	2 / 61 (3.28%)		
occurrences (all)	2		
Cardiac disorders			
Cardiac disorders	Additional description: All combined		
subjects affected / exposed	7 / 61 (11.48%)		
occurrences (all)	9		
Nervous system disorders			
Nervous system disorders	Additional description: All combined		
subjects affected / exposed	12 / 61 (19.67%)		
occurrences (all)	14		
Blood and lymphatic system disorders			
Blood and lymphatic system disorders	Additional description: All combined		
subjects affected / exposed	39 / 61 (63.93%)		
occurrences (all)	78		
Ear and labyrinth disorders			
Ear and labyrinth disorders	Additional description: All combined		
subjects affected / exposed	2 / 61 (3.28%)		
occurrences (all)	2		
Eye disorders			

Eye disorders	Additional description: All combined		
subjects affected / exposed	3 / 61 (4.92%)		
occurrences (all)	4		
Gastrointestinal disorders			
Gastrointestinal disorders	Additional description: All combined		
subjects affected / exposed	28 / 61 (45.90%)		
occurrences (all)	43		
Hepatobiliary disorders			
Hepatobiliary disorders	Additional description: All combined		
subjects affected / exposed	2 / 61 (3.28%)		
occurrences (all)	2		
Skin and subcutaneous tissue disorders			
Skin and subcutaneous tissue disorders	Additional description: All combined		
subjects affected / exposed	3 / 61 (4.92%)		
occurrences (all)	3		
Renal and urinary disorders			
Renal and urinary disorders	Additional description: All combined		
subjects affected / exposed	5 / 61 (8.20%)		
occurrences (all)	8		
Musculoskeletal and connective tissue disorders			
Musculoskeletal and connective tissue disorders	Additional description: All combined		
subjects affected / exposed	7 / 61 (11.48%)		
occurrences (all)	7		
Infections and infestations			
Infections and infestations	Additional description: All combined		
subjects affected / exposed	19 / 61 (31.15%)		
occurrences (all)	28		
Metabolism and nutrition disorders			
Metabolism and nutrition disorders	Additional description: All combined		
subjects affected / exposed	15 / 61 (24.59%)		
occurrences (all)	22		

More information

Substantial protocol amendments (globally)

Were there any global substantial amendments to the protocol? Yes

Date	Amendment
26 January 2017	Amendment 1 Reason for this amendment Protocol: Change in conditioning treatment for allogeneic stem cell therapy. Adjustments to exclusion criteria based on new insights from the manufacturer of the study medication (CTI, pacritinib). Adjustments in dose regulation based on new insights from the manufacturer. Patient information: For future research, patients may be asked to provide a saliva sample as a reference specimen. Participating hospitals: UMC Utrecht has withdrawn from the study
12 March 2018	Amendment 2 Reason for this amendment Protocol: Adjustments to exclusion criteria based on new insights from the manufacturer of the study medication (CTI, pacritinib). Clarification of dose regulation. Patient information: Several textual changes.
27 September 2018	Amendment 3 Reason for this amendment Protocol: Adjustments to exclusion criteria related to prior treatment with JAK2 inhibitors. Changes to conditioning schedule. Changes to sampling schedule. Patient information: Consent for bone marrow sampling.

15 April 2019	Amendment 4 Reason for this amendment Protocol: Adjustments to inclusion criteria. Dose adjustment in case of low platelet count. Changes in side studies and central lab procedures. Patient information: Clarification of bone marrow examination. Removal of MRI side study. EudraCT form: Address change for vendor due to Brexit. Inclusion criteria updated.
03 October 2019	Amendment 5 Reason for this amendment EudraCT and ABR form: Local investigator [Name] replaces [Name] in MUMC+ Maastricht. Submission of the annual progress report (APR) and annual safety report (DSUR), as well as version 8.0 of the IMPD.
28 August 2020	Amendment 6 The reason for this change is: Protocol: additions to the study protocol following the DSMB decision regarding interim analysis. In addition, we are submitting the new Investigator's Brochure (IB) for pacritinib (version 13) and the no consequences statement.
15 July 2024	Amendment 7 The reason for these changes is: Update of EudraCT and ABR in connection with a new principal investigator at VUmc [name], adjustment of HOVON contact details.

Notes:

Interruptions (globally)

Were there any global interruptions to the trial? No

Limitations and caveats

None reported