



Clinical trial results:

An open-label, phase IIa study of the safety, tolerability, pharmacokinetics and pharmacodynamics of oral GB2064 (a LOXL2 inhibitor) in participants with myelofibrosis (The MYLOX-1 study).

Summary

EudraCT number	2020-003087-45
Trial protocol	DE IT
Global end of trial date	01 August 2024

Results information

Result version number	v1 (current)
This version publication date	18 July 2025
First version publication date	18 July 2025
Summary attachment (see zip file)	CSR_synopsis_20May2024 (MYLOX-1 CSR_synopsis_20May2024.pdf) Extension CSR Addendum Summary_V1.0_22APR2025 (Mylox-1_Extension CSR Addendum Summary_V1.0_22APR2025.pdf)

Trial information

Trial identification

Sponsor protocol code	TheMYLOX-1study
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Additional study identifiers

ISRCTN number	-
ClinicalTrials.gov id (NCT number)	NCT04679870
WHO universal trial number (UTN)	-

Notes:

Sponsors

Sponsor organisation name	Galecto Biotech AB
Sponsor organisation address	COBIS Science Park, Ole Maaloes Vej 3, Copenhagen, Italy, DK-2200
Public contact	Dipartimento Medico, OPIS s.r.l., 0039 03626331, info.studiclinici@opisresearch.com
Scientific contact	Dipartimento Medico, OPIS s.r.l., 0039 03626331, info.studiclinici@opisresearch.com

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	22 April 2025
Is this the analysis of the primary completion data?	Yes
Primary completion date	01 August 2024
Global end of trial reached?	Yes
Global end of trial date	01 August 2024
Was the trial ended prematurely?	No

Notes:

General information about the trial

Main objective of the trial:

To assess the safety, tolerability, pharmacokinetics, pharmacodynamics and clinical effects of 1000 mg BID GB2064 in participants with PMF or SMF.

Protection of trial subjects:

This study was conducted in compliance with the standards of Good Clinical Practice as defined in the ICH E6 (R2) 'Guideline for Good Clinical practice', including the archiving of essential documents.

Background therapy: -

Evidence for comparator: -

Actual start date of recruitment	09 June 2021
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	Germany: 5
Country: Number of subjects enrolled	Italy: 9
Country: Number of subjects enrolled	Australia: 2
Country: Number of subjects enrolled	United States: 2
Worldwide total number of subjects	18
EEA total number of subjects	14

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)	7
From 65 to 84 years	11

85 years and over	0
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Subject disposition

Recruitment

Recruitment details:

19 patients enrolled, though only 18 initiated treatment with GB2064

Pre-assignment

Screening details:

Screening period: patients were assessed for eligibility based on inclusion/exclusion criteria prior to receiving study treatment

Pre-assignment period milestones

Number of subjects started	18
Number of subjects completed	18

Period 1

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

Arms

Arm title	GB2064 1000 mg BID
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Arm description:

Participants received GB2064 at a dose of 1000 mg BID administered orally

Arm type	Experimental
Investigational medicinal product name	GB2064
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Film-coated tablet
Routes of administration	Oral use

Dosage and administration details:

1000 mg BID orally (4 x 250 mg tablets, twice daily)

Number of subjects in period 1	GB2064 1000 mg BID
Started	18
Completed	7
Not completed	11
Consent withdrawn by subject	3
Adverse event, non-fatal	3
Death	1
Progressive disease	3
Lack of efficacy	1

Baseline characteristics

Reporting groups

Reporting group title	Overall study
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Reporting group description: -

Reporting group values	Overall study	Total	
Number of subjects	18	18	
Age categorical			
Units: Subjects			
In utero	0	0	
Preterm newborn infants (gestational age < 37 wks)	0	0	
Newborns (0-27 days)	0	0	
Infants and toddlers (28 days-23 months)	0	0	
Children (2-11 years)	0	0	
Adolescents (12-17 years)	0	0	
Adults (18-64 years)	7	7	
From 65-84 years	11	11	
85 years and over	0	0	
Age continuous			
Units: years			
arithmetic mean	66.2		
standard deviation	± 8.43	-	
Gender categorical			
Units: Subjects			
Female	8	8	
Male	10	10	
Race (NIH/OMB)			
Units: Subjects			
White	18	18	
Region of Enrollment			
Units: Subjects			
United States	2	2	
Italy	9	9	
Australia	2	2	
Germany	5	5	

End points

End points reporting groups

Reporting group title	GB2064 1000 mg BID
Reporting group description:	
Participants received GB2064 at a dose of 1000 mg BID administered orally	

Primary: Safety and Tolerability of GB2064

End point title	Safety and Tolerability of GB2064 ^[1]
End point description:	

End point type	Primary
End point timeframe:	
9 Months	

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: No formal statistical analyses were planned.

The data has been listed and summarized using descriptive statistics. Continuous variables have been summarized using the number of observations, mean, standard deviation, median, and range as appropriate. Categorical values have been summarized using the number of observations and percentages as appropriate. Time-to-event endpoints have been estimated using Kaplan-Meier methodology. Graphical displays were also produced.

End point values	GB2064 1000 mg BID			
Subject group type	Reporting group			
Number of subjects analysed	18			
Units: Number of subjects with adverse events	17			

Statistical analyses

No statistical analyses for this end point

Secondary: Plasma concentrations

End point title	Plasma concentrations
End point description:	
Plasma concentrations of GB2064 throughout the study	

End point type	Secondary
End point timeframe:	
9 months	

End point values	GB2064 1000 mg BID			
Subject group type	Reporting group			
Number of subjects analysed	18			
Units: Plasma concentrations of GB2064 (ng/mL)				
arithmetic mean (standard deviation)				
Day 1 Pre-Dose	0 (± 0)			
Day 1 2h post-dose	5987.8 (± 1684.09)			
Month 1 Pre-dose	403.3 (± 1368.53)			
Month 1 2h post-dose	6313.8 (± 2375.99)			
Month 3 Pre-dose	108.0 (± 96.53)			
Month 3 2h post-dose	5757.7 (± 1317.27)			
Month 6 Pre-dose	49.8 (± 36.66)			
Month 6 2h Post-dose	5958.0 (± 2853.70)			
Month 9 Pre-dose	52.2 (± 57.94)			
Month 9 2h Post-dose	6217.1 (± 1312.03)			

Statistical analyses

No statistical analyses for this end point

Secondary: Bone Marrow Fibrosis - Reticulin Fibrosis

End point title	Bone Marrow Fibrosis - Reticulin Fibrosis
End point description:	
Degree of reticulin fibrosis throughout the study	
End point type	Secondary
End point timeframe:	
9 months	

End point values	GB2064 1000 mg BID			
Subject group type	Reporting group			
Number of subjects analysed	10			
Units: Number of participants showing a benefit	2			

Statistical analyses

No statistical analyses for this end point

Secondary: Bone Marrow Fibrosis - Collagen Fibrosis

End point title	Bone Marrow Fibrosis - Collagen Fibrosis
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End point description:

Degree of collagen fibrosis throughout the study

End point type	Secondary
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End point timeframe:

9 months

End point values	GB2064 1000 mg BID			
Subject group type	Reporting group			
Number of subjects analysed	10			
Units: Number of participants showing a benefit	6			

Statistical analyses

No statistical analyses for this end point

Secondary: Anemia response

End point title	Anemia response
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End point description:

Patients with an Anemia response to GB2064

End point type	Secondary
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End point timeframe:

9 months

End point values	GB2064 1000 mg BID			
Subject group type	Reporting group			
Number of subjects analysed	10			
Units: Number of participants showing a benefit	1			

Statistical analyses

No statistical analyses for this end point

Secondary: Symptom response

End point title	Symptom response
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End point description:

Patients with a symptom response (TSS50) to GB2064

End point type	Secondary
End point timeframe:	
9 Months	

End point values	GB2064 1000 mg BID			
Subject group type	Reporting group			
Number of subjects analysed	10			
Units: Number of participants showing a benefit	2			

Statistical analyses

No statistical analyses for this end point

Secondary: Spleen Response

End point title	Spleen Response
End point description:	
Patients with a spleen response to GB2064	
End point type	Secondary
End point timeframe:	
9 Months	

End point values	GB2064 1000 mg BID			
Subject group type	Reporting group			
Number of subjects analysed	10			
Units: Number of participants showing a benefit	1			

Statistical analyses

No statistical analyses for this end point

Adverse events

Adverse events information

Timeframe for reporting adverse events:

After initiation of study drug, all AEs will be reported until 4 weeks after the last dose of study drug and 90 days for SAEs or AESIs

Adverse event reporting additional description:

Investigators will seek information on AEs at each patient contact. All AEs, whether reported by the patient or noted by study personnel, will be recorded in the patient's medical record and on the AE pages of the eCRF.

Assessment type	Systematic
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Dictionary used

Dictionary name	MedDRA
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Dictionary version	26.0
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Reporting groups

Reporting group title	All subjects treated with GB2064
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Reporting group description:

All noxious and unintended responses to an IMP (i.e., where a causal relationship between an IMP and an AE is at least a reasonable possibility) related to any dose should be considered adverse drug reactions.

An unexpected adverse drug reaction is defined as an adverse reaction, the nature or severity of which is not consistent with the applicable product information (Investigator's Brochure).

All AEs will be reported until 4 weeks after the last dose of study drug and 90 days for SAEs or AESIs.

Adverse events requiring hospitalisation should be considered serious. In general, hospitalisation signifies that the participant has been detained (usually involving an overnight stay) at the hospital or emergency ward for observation and/or treatment that would not have been appropriate at the Clinical Research Unit (CRU).

Serious adverse events	All subjects treated with GB2064		
Total subjects affected by serious adverse events			
subjects affected / exposed	7 / 18 (38.89%)		
number of deaths (all causes)	1		
number of deaths resulting from adverse events			
Injury, poisoning and procedural complications			
Fall			
subjects affected / exposed	1 / 18 (5.56%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Post procedural haemorrhage			
subjects affected / exposed	1 / 18 (5.56%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		

Cardiac disorders			
Acute myocardial infarction			
subjects affected / exposed	1 / 18 (5.56%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
General disorders and administration site conditions			
Generalised Oedema			
subjects affected / exposed	1 / 18 (5.56%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Renal and urinary disorders			
Renal Colic			
subjects affected / exposed	1 / 18 (5.56%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Infections and infestations			
COVID-19pneumonia			
subjects affected / exposed	1 / 18 (5.56%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Pneumonia			
subjects affected / exposed	1 / 18 (5.56%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Sepsis			
subjects affected / exposed	1 / 18 (5.56%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 1		

Frequency threshold for reporting non-serious adverse events: 5 %

Non-serious adverse events	All subjects treated with GB2064		
Total subjects affected by non-serious adverse events			
subjects affected / exposed	17 / 18 (94.44%)		

Neoplasms benign, malignant and unspecified (incl cysts and polyps) Basal Cell Carcinoma subjects affected / exposed occurrences (all)	1 / 18 (5.56%) 1		
Vascular disorders Deep vein thrombosis subjects affected / exposed occurrences (all) Haematoma subjects affected / exposed occurrences (all) Hypertension subjects affected / exposed occurrences (all) Skin ulcer subjects affected / exposed occurrences (all)	1 / 18 (5.56%) 1 2 / 18 (11.11%) 2 1 / 18 (5.56%) 1 1 / 18 (5.56%) 1		
General disorders and administration site conditions Asthenia subjects affected / exposed occurrences (all) Chest Pain subjects affected / exposed occurrences (all) Fatigue subjects affected / exposed occurrences (all) Gait disturbance subjects affected / exposed occurrences (all) Malaise subjects affected / exposed occurrences (all) Oedema peripheral subjects affected / exposed occurrences (all)	4 / 18 (22.22%) 4 1 / 18 (5.56%) 1 2 / 18 (11.11%) 2 1 / 18 (5.56%) 1 1 / 18 (5.56%) 1 2 / 18 (11.11%) 2		

Pyrexia subjects affected / exposed occurrences (all)	1 / 18 (5.56%) 1		
Respiratory, thoracic and mediastinal disorders Asthma subjects affected / exposed occurrences (all) Atelactasis subjects affected / exposed occurrences (all) Cough subjects affected / exposed occurrences (all) Dyspnoea subjects affected / exposed occurrences (all) Dyspnoea exertional subjects affected / exposed occurrences (all)	1 / 18 (5.56%) 1 1 / 18 (5.56%) 1 1 / 18 (5.56%) 1 1 / 18 (5.56%) 1 1 / 18 (5.56%) 1		
Psychiatric disorders Depression subjects affected / exposed occurrences (all)	1 / 18 (5.56%) 1		
Investigations Alanine aminotransferase increased subjects affected / exposed occurrences (all) Blood alkaline phosphatase increased subjects affected / exposed occurrences (all) Blood bilirubin increased subjects affected / exposed occurrences (all) Gamma-glutamyltransferase increased subjects affected / exposed occurrences (all)	1 / 18 (5.56%) 1 1 / 18 (5.56%) 1 1 / 18 (5.56%) 1 1 / 18 (5.56%) 1		

Lymphocyte count decreased subjects affected / exposed occurrences (all)	1 / 18 (5.56%) 1		
Neutrophil count increased subjects affected / exposed occurrences (all)	1 / 18 (5.56%) 1		
Platelet count decreased subjects affected / exposed occurrences (all)	1 / 18 (5.56%) 1		
Weight decreased subjects affected / exposed occurrences (all)	1 / 18 (5.56%) 1		
Cardiac disorders			
Atrial Fibrillation subjects affected / exposed occurrences (all)	1 / 18 (5.56%) 1		
Tricuspid valve incompetence subjects affected / exposed occurrences (all)	1 / 18 (5.56%) 1		
Nervous system disorders			
Disturbance in attention subjects affected / exposed occurrences (all)	1 / 18 (5.56%) 1		
Dizziness subjects affected / exposed occurrences (all)	1 / 18 (5.56%) 1		
Dysgeusia subjects affected / exposed occurrences (all)	2 / 18 (11.11%) 2		
Hepatic encephalopathy subjects affected / exposed occurrences (all)	1 / 18 (5.56%) 1		
Migraine subjects affected / exposed occurrences (all)	1 / 18 (5.56%) 1		
Somnolence			

subjects affected / exposed occurrences (all)	1 / 18 (5.56%) 1		
Blood and lymphatic system disorders			
Leukocytosis			
subjects affected / exposed	1 / 18 (5.56%)		
occurrences (all)	1		
Thrombocytopenia			
subjects affected / exposed	1 / 18 (5.56%)		
occurrences (all)	1		
Gastrointestinal disorders			
Aphthous ulcer			
subjects affected / exposed	1 / 18 (5.56%)		
occurrences (all)	1		
Ascites			
subjects affected / exposed	1 / 18 (5.56%)		
occurrences (all)	1		
Constipation			
subjects affected / exposed	1 / 18 (5.56%)		
occurrences (all)	1		
Diarrhoea			
subjects affected / exposed	2 / 18 (11.11%)		
occurrences (all)	2		
Dry Mouth			
subjects affected / exposed	1 / 18 (5.56%)		
occurrences (all)	1		
Dyspepsia			
subjects affected / exposed	2 / 18 (11.11%)		
occurrences (all)	2		
Epigastric discomfort			
subjects affected / exposed	1 / 18 (5.56%)		
occurrences (all)	1		
Gastritis			
subjects affected / exposed	2 / 18 (11.11%)		
occurrences (all)	2		
Gastrointestinal haemorrhage			

subjects affected / exposed	1 / 18 (5.56%)		
occurrences (all)	1		
Gastrooesophageal reflux disease			
subjects affected / exposed	1 / 18 (5.56%)		
occurrences (all)	1		
Nausea			
subjects affected / exposed	8 / 18 (44.44%)		
occurrences (all)	8		
Oesophagitis			
subjects affected / exposed	1 / 18 (5.56%)		
occurrences (all)	1		
Vomiting			
subjects affected / exposed	3 / 18 (16.67%)		
occurrences (all)	3		
Hepatobiliary disorders			
Hepatic cirrhosis			
subjects affected / exposed	1 / 18 (5.56%)		
occurrences (all)	1		
Liver Disorder			
subjects affected / exposed	1 / 18 (5.56%)		
occurrences (all)	1		
Skin and subcutaneous tissue disorders			
Dry skin			
subjects affected / exposed	1 / 18 (5.56%)		
occurrences (all)	1		
Eczema			
subjects affected / exposed	2 / 18 (11.11%)		
occurrences (all)	2		
Night sweats			
subjects affected / exposed	1 / 18 (5.56%)		
occurrences (all)	1		
Pruritis			
subjects affected / exposed	3 / 18 (16.67%)		
occurrences (all)	3		
Rash			

subjects affected / exposed	2 / 18 (11.11%)		
occurrences (all)	2		
Skin laceration			
subjects affected / exposed	1 / 18 (5.56%)		
occurrences (all)	1		
Renal and urinary disorders			
Dysuria			
subjects affected / exposed	1 / 18 (5.56%)		
occurrences (all)	1		
Haematuria			
subjects affected / exposed	1 / 18 (5.56%)		
occurrences (all)	1		
Nephrolithiasis			
subjects affected / exposed	2 / 18 (11.11%)		
occurrences (all)	2		
Strangury			
subjects affected / exposed	1 / 18 (5.56%)		
occurrences (all)	1		
Musculoskeletal and connective tissue disorders			
Arthralgia			
subjects affected / exposed	1 / 18 (5.56%)		
occurrences (all)	1		
Back pain			
subjects affected / exposed	1 / 18 (5.56%)		
occurrences (all)	1		
Bone pain			
subjects affected / exposed	1 / 18 (5.56%)		
occurrences (all)	1		
Joint stiffness			
subjects affected / exposed	1 / 18 (5.56%)		
occurrences (all)	1		
Myalgia			
subjects affected / exposed	4 / 18 (22.22%)		
occurrences (all)	4		
Pain in extremity			

subjects affected / exposed	1 / 18 (5.56%)		
occurrences (all)	1		
Infections and infestations			
Bronchitis			
subjects affected / exposed	1 / 18 (5.56%)		
occurrences (all)	1		
COVID-19			
subjects affected / exposed	3 / 18 (16.67%)		
occurrences (all)	3		
Nasopharyngitis			
subjects affected / exposed	1 / 18 (5.56%)		
occurrences (all)	1		
Pneumonia Aspiration			
subjects affected / exposed	1 / 18 (5.56%)		
occurrences (all)	1		
Staphylococcal sepsis			
subjects affected / exposed	1 / 18 (5.56%)		
occurrences (all)	1		
Upper Respiratory Tract Infection			
subjects affected / exposed	1 / 18 (5.56%)		
occurrences (all)	1		
Urinary Tract Infection			
subjects affected / exposed	1 / 18 (5.56%)		
occurrences (all)	1		
Metabolism and nutrition disorders			
Decreased appetite			
subjects affected / exposed	4 / 18 (22.22%)		
occurrences (all)	4		
Hyperglycaemia			
subjects affected / exposed	1 / 18 (5.56%)		
occurrences (all)	1		
Iron overload			
subjects affected / exposed	1 / 18 (5.56%)		
occurrences (all)	1		

More information

Substantial protocol amendments (globally)

Were there any global substantial amendments to the protocol? Yes

Date	Amendment
26 August 2020	Protocol version 02, dated 26 August 2020 Clarification to match dose rationale, Clarification of dose prediction, Correction of hyperlink from Appendix 3 to Appendix 2:
21 October 2020	Protocol version 03, dated 21 October 2020 The purpose of this amendment was to implement comments received from the Agency.
07 January 2022	Protocol version 04, dated 04 January 2022 The purpose of this amendment was to provide information on the extension study.
12 January 2022	Protocol version 05, dated 12 January 2022 The purpose of this amendment was to make corrections regarding the length of the extension study.
17 February 2022	Protocol version 06, dated 17 February 2022 The purpose of this amendment was to include provisions for steroid allowance for participants previously on JAK inhibitors and disease progression following spleen enlargement following discussions with a few PI's and DSMB chair. Editorial changes for better clarity have been included as well
02 February 2024	Protocol version 07, dated 02 February 2024 The protocol has been consolidated to be able to transition the MYLOX-1 trial from Clinical Trials Directive to Clinical Trials Regulation. Country-specific requirements from German authorities have been included to the protocol, such as excluding women of childbearing potential from the study sites in Germany. Protocol Clarifications issued separately previously have also been included within. Sponsor contact and principal investigator are changed.

Notes:

Interruptions (globally)

Were there any global interruptions to the trial? No

Limitations and caveats

None reported