Clinical trial results:
An Open label, Compassionate Use Study of Tenalisib (RP6530) in Patients currently receiving treatment on Tenalisib trials in Hematological Malignancies

Summary

EudraCT number	2020-001663-90
Trial protocol	PL BG
Global end of trial date	

Results information

Result version number	v1 (current)
This version publication date	12 October 2022
First version publication date	12 October 2022

Trial information

Trial identification

Sponsor protocol code	RP6530-1803

Additional study identifiers

ISRCTN number	-
ClinicalTrials.gov id (NCT number)	NCT03711604
WHO universal trial number (UTN)	-

Notes:

Sponsors

Sponsor organisation name	Rhizen Pharmaceuticls SA
Sponsor organisation address	Grosspeteranlage 29,, Basel, Switzerland, 4052
Public contact	Ajit Nair / SVP & Head Clinical R&D, Rhizen Pharmaceuticals SA, +41 4033241041, an@rhizen.com
Scientific contact	Ajit Nair / SVP & Head Clinical R&D, Rhizen Pharmaceuticals SA, +41 4033241041, an@rhizen.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	Interim
Date of interim/final analysis	15 September 2022
Is this the analysis of the primary completion data?	No

Global end of trial reached?	No

Notes:

General information about the trial

Main objective of the trial:

To evaluate the safety and tolerability of Tenalisib as single agent or in combination until the withdrawal of subject from the study due to disease progression, unacceptable toxicity or any other reason including consent withdrawal or investigator’s decision.

Protection of trial subjects:

The study was conducted in accordance with the ethical principles stated in the Declaration of Helsinki and the International Conference on Harmonization (ICH) Guideline for Good Clinical Practice (GCP). Confidentiality of patient’s personal data was protected in accordance with the Health Insurance Portability and Accountability Act of 1996 (HIPAA) and national data protection laws, as applicable. During the conduct of the study, all subjects were closely monitored for all adverse events regularly as mentioned in the protocol.

Background therapy: -

Evidence for comparator: -

Actual start date of recruitment	28 November 2018
Long term follow-up planned	No
Independent data monitoring committee (IDMC) involvement?	No

Notes:

Population of trial subjects

Subjects enrolled per country

Country: Number of subjects enrolled	United States: 10
Country: Number of subjects enrolled	Georgia: 6
Country: Number of subjects enrolled	Poland: 1
Worldwide total number of subjects	17
EEA total number of subjects	1

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)	5

From 65 to 84 years	11
85 years and over	1

Subject disposition

Recruitment

Recruitment details:

This was an open-label, compassionate use study in patients who have completed a clinical trial of Tenalisib. This trial offers an opportunity to patients who have responded (either have CR, PR, or SD on Tenalisib treatment) to receive Tenalisib as compassionate medication following their completion of the previous study.

Pre-assignment

Screening details:

Patients continued to receive Tenalisib (schedule and dose) as they received in previous protocols unless dose adjustments or delays are necessary for toxicity management. In case of rollover from a combination study, the investigator has a choice to continue with the combination therapy or to maintain the patient on Tenalisib monotherapy.

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	Tenalisib (RP6530)
Arm description: -	
Arm type	Experimental
Investigational medicinal product name	Tenalisib
Investigational medicinal product code	RP6530
Other name	
Pharmaceutical forms	Tablet
Routes of administration	Oral use

Dosage and administration details:

Subjects received either 400 mg BID or 800 mg BID orally in a 28-day cycle.

Number of subjects in period 1	Tenalisib (RP6530)
Started	17
Completed	17

Baseline characteristics

Reporting groups

Reporting group title	Overall study (overall period)

Reporting group description: -

Reporting group values	Overall study (overall period)	Total	
Number of subjects	17	17	
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)	5	5	
From 65-84 years	11	11	
85 years and over	1	1	
Age continuous Units: years			
median	70.99		
full range (min-max)	45.55 to 91.05	-	
Gender categorical Units: Subjects			
Female	6	6	
Male	11	11	

End points

End points reporting groups

Reporting group title	Tenalisib (RP6530)

Reporting group description: -

Primary: Adverse Events (AEs), Grade 3/ 4 AEs and Serious Adverse Event (SAEs)

End point title	Adverse Events (AEs), Grade 3/ 4 AEs and Serious Adverse Event (SAEs)[1]

End point description:

End point type	Primary

End point timeframe:

2 years

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 statistical analyses for this endpoint

End point values	Tenalisib (RP6530)			
Subject group type	Reporting group			
Number of subjects analysed	17			
Units: subjects				
Subjects with AEs	12			
Subjects with grade 3/4 AEs	5			
Subjects with SAEs	4			

Statistical analyses

No statistical analyses for this end point

Adverse events

Adverse events information

Timeframe for reporting adverse events:

2 years

Assessment type	Systematic

Dictionary used

Dictionary name	MedDRA
Dictionary version	25

Reporting groups

Reporting group title	Tenalisib (RP6530)

Reporting group description: -

Serious adverse events	Tenalisib (RP6530)		
Total subjects affected by serious adverse events			
subjects affected / exposed	5 / 17 (29.41%)		
number of deaths (all causes)	1		
number of deaths resulting from adverse events	1		
Neoplasms benign, malignant and unspecified (incl cysts and polyps)			
Renal cell carcinoma			
subjects affected / exposed	1 / 17 (5.88%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Nervous system disorders			
Seizure			
subjects affected / exposed	1 / 17 (5.88%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Haemorrhage intracranial			
subjects affected / exposed	1 / 17 (5.88%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Infections and infestations			
COVID-19			
subjects affected / exposed	2 / 17 (11.76%)		
occurrences causally related to treatment / all	0 / 2		
deaths causally related to treatment / all	0 / 1		

COVID-19 pneumonia			
subjects affected / exposed	1 / 17 (5.88%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		

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

Non-serious adverse events	Tenalisib (RP6530)		
Total subjects affected by non-serious adverse events			
subjects affected / exposed	12 / 17 (70.59%)		
Neoplasms benign, malignant and unspecified (incl cysts and polyps)			
Basal cell carcinoma			
subjects affected / exposed	1 / 17 (5.88%)		
occurrences (all)	1		
Vascular disorders			
Haematoma			
subjects affected / exposed	1 / 17 (5.88%)		
occurrences (all)	1		
Surgical and medical procedures			
Nephrectomy			
subjects affected / exposed	1 / 17 (5.88%)		
occurrences (all)	1		
General disorders and administration site conditions			
Fatigue			
subjects affected / exposed	2 / 17 (11.76%)		
occurrences (all)	2		
Obstruction			
subjects affected / exposed	1 / 17 (5.88%)		
occurrences (all)	1		
Oedema			
subjects affected / exposed	2 / 17 (11.76%)		
occurrences (all)	2		
Respiratory, thoracic and mediastinal disorders			
Epistaxis			
subjects affected / exposed	1 / 17 (5.88%)		
occurrences (all)	1		

Pneumonitis subjects affected / exposed occurrences (all)	1 / 17 (5.88%) 1		
Psychiatric disorders Insomnia subjects affected / exposed occurrences (all) Mental status changes subjects affected / exposed occurrences (all)	 1 / 17 (5.88%) 1 1 / 17 (5.88%) 1		
Investigations Aspartate aminotransferase increased subjects affected / exposed occurrences (all) Blood alkaline phosphatase increased subjects affected / exposed occurrences (all) Neutrophil count decreased subjects affected / exposed occurrences (all) Platelet count decreased subjects affected / exposed occurrences (all) Thrombocytopenia subjects affected / exposed occurrences (all) Troponin increased subjects affected / exposed occurrences (all)	 2 / 17 (11.76%) 3 2 / 17 (11.76%) 5 2 / 17 (11.76%) 7 2 / 17 (11.76%) 9 2 / 17 (11.76%) 3 1 / 17 (5.88%) 1		
Injury, poisoning and procedural complications Wound subjects affected / exposed occurrences (all)	 1 / 17 (5.88%) 1		
Cardiac disorders Myocardial infarction			

subjects affected / exposed	1 / 17 (5.88%)		
occurrences (all)	1		
Supraventricular extrasystoles			
subjects affected / exposed	1 / 17 (5.88%)		
occurrences (all)	1		
Nervous system disorders			
Dysgeusia			
subjects affected / exposed	1 / 17 (5.88%)		
occurrences (all)	1		
Neuropathy peripheral			
subjects affected / exposed	1 / 17 (5.88%)		
occurrences (all)	1		
Post herpetic neuralgia			
subjects affected / exposed	1 / 17 (5.88%)		
occurrences (all)	1		
Spinal cord compression			
subjects affected / exposed	1 / 17 (5.88%)		
occurrences (all)	1		
Gastrointestinal disorders			
Abdominal discomfort			
subjects affected / exposed	1 / 17 (5.88%)		
occurrences (all)	1		
Constipation			
subjects affected / exposed	1 / 17 (5.88%)		
occurrences (all)	1		
Diarrhoea			
subjects affected / exposed	2 / 17 (11.76%)		
occurrences (all)	2		
Nausea			
subjects affected / exposed	1 / 17 (5.88%)		
occurrences (all)	1		
Vomiting			
subjects affected / exposed	1 / 17 (5.88%)		
occurrences (all)	2		
Skin and subcutaneous tissue disorders			

Actinic keratosis subjects affected / exposed occurrences (all)	1 / 17 (5.88%) 0		
Erythema subjects affected / exposed occurrences (all)	1 / 17 (5.88%) 1		
Exfoliative rash subjects affected / exposed occurrences (all)	1 / 17 (5.88%) 1		
Pruritus subjects affected / exposed occurrences (all)	1 / 17 (5.88%) 1		
Rash subjects affected / exposed occurrences (all)	1 / 17 (5.88%) 1		
Rash pruritic subjects affected / exposed occurrences (all)	1 / 17 (5.88%) 1		
Renal and urinary disorders Renal disorder subjects affected / exposed occurrences (all)	1 / 17 (5.88%) 1		
Musculoskeletal and connective tissue disorders Arthralgia subjects affected / exposed occurrences (all)	1 / 17 (5.88%) 1		
Back pain subjects affected / exposed occurrences (all)	1 / 17 (5.88%) 1		
Infections and infestations Covid-19 subjects affected / exposed occurrences (all)	1 / 17 (5.88%) 1		

More information

Substantial protocol amendments (globally)

Were there any global substantial amendments to the protocol? No

Interruptions (globally)

Were there any global interruptions to the trial? No

Limitations and caveats

Limitations of the trial such as small numbers of subjects analysed or technical problems leading to unreliable data.

This is not an interim analysis, it's more an update of ongoing study data. The global trial is still ongoing with one patient.

Notes: