Clinical trial results:
A Phase 3, Multicenter, Open-Label Extension Study of Patidegib Topical Gel, 2% in Subjects with Gorlin Syndrome (Basal Cell Nevus Syndrome)

Summary

EudraCT number	2020-000253-27
Trial protocol	GB FR NL DK ES BE IT
Global end of trial date	14 July 2021

Results information

Result version number	v1 (current)
This version publication date	06 July 2023
First version publication date	06 July 2023

Trial information

Trial identification

Sponsor protocol code	Pelle-926-301E

Additional study identifiers

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

Notes:

Sponsors

Sponsor organisation name	PellePharm, Inc. / Sol-Gel Technologies Ltd
Sponsor organisation address	7 Golda Meir St, Ness Ziona, Israel, 7403650
Public contact	VP, Clinical Development, Sol-Gel Technologies Ltd, ofral@sol-gel.com
Scientific contact	VP, Clinical Development, Sol-Gel Technologies Ltd, ofral@sol-gel.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	14 July 2021
Is this the analysis of the primary completion data?	Yes
Primary completion date	14 July 2021
Global end of trial reached?	Yes
Global end of trial date	14 July 2021
Was the trial ended prematurely?	No

Notes:

General information about the trial

Main objective of the trial:

The primary objective of the study is to assess the safety and tolerability of Patidegib Topical Gel, 2% in subjects who have completed PellePharm Study Pelle-926-201 or Pelle-926-301.

Protection of trial subjects:

This study was conducted in accordance with the ethical principles originating from the Declaration of Helsinki, ICH guidelines, GCP, and in compliance with local regulatory requirements. The protocol, informed consent form and other information provided to subjects, and all appropriate amendments were properly reviewed and approved by the IRB/EC/REB. The IRB-approved informed consent form followed the Protection of Human Subjects regulations listed in 21 Code of Federal Regulations Part 50, and had to be signed and dated by each subject prior to conduct of any study procedures. The background of the study and the benefits and risks of the procedures and study had to be clearly and understandably explained to the subjects. Subject data was protected by ensuring that no captured data contained subject names, addresses, telephone numbers, email addresses, or other directly identifying personal information.

Background therapy: -

Evidence for comparator: -

Actual start date of recruitment	04 June 2020
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	Netherlands: 8
Country: Number of subjects enrolled	Spain: 14
Country: Number of subjects enrolled	United Kingdom: 10
Country: Number of subjects enrolled	Belgium: 2
Country: Number of subjects enrolled	Denmark: 2
Country: Number of subjects enrolled	France: 8
Country: Number of subjects enrolled	Germany: 8
Country: Number of subjects enrolled	Italy: 5
Country: Number of subjects enrolled	United States: 51
Worldwide total number of subjects	108
EEA total number of subjects	47

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)	83
From 65 to 84 years	25
85 years and over	0

Subject disposition

Recruitment

Recruitment details: -

Pre-assignment

Screening details:

To be recruited, the subjects had to have completed PellePharm Study 926-201 or 926-301, abstain from application of facial non-study topical medication, could not be pregnant or breastfeeding, and willing to use effective contraception if the they or their partner was a woman of childbearing potential.

Period 1

Period 1 title	Recruitment period
Is this the baseline period?	No
Allocation method	Not applicable
Blinding used	Not blinded

Arms

Arm title	Recruited patients

Arm description: -

Arm type	No intervention

No investigational medicinal product assigned in this arm

Number of subjects in period 1	Recruited patients
Started	108
Completed	105
Not completed	3
No treatment received	3

Period 2

Period 2 title	Treatment period
Is this the baseline period?	Yes[1]
Allocation method	Not applicable
Blinding used	Not blinded

Blinding implementation details:

Not applicable.

Arms

Arm title	Patidegib
Arm description: -	
Arm type	Experimental
Investigational medicinal product name	Patidegib
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Gel
Routes of administration	Topical

Dosage and administration details:

Patidegib Topical Gel, 2% (w/w) was applied to the area extending from the anterior hairline to the jaw line at the clinic on study day 1 (baseline).

Notes:

[1] - Period 1 is not the baseline period. It is expected that period 1 will be the baseline period.

Justification: Period 1 was the recruitment period. Baseline information was summarised only for the patients who received any treatment (Period 2).

Number of subjects in period 2[2]	Patidegib
Started	105
Completed	3
Not completed	102
Consent withdrawn by subject	4
Other	1
Sponsor decision	95
Lost to follow-up	1
Lack of efficacy	1

Notes:

[2] - The number of subjects reported to be in the baseline period are not the same as the worldwide number enrolled in the trial. It is expected that these numbers will be the same.

Justification: Baseline information was summarised for the patients who received any treatment. Three patients in the recruitment period (worldwide number) did not receive any treatment and baseline data was therefore not summarised for these patients.

Baseline characteristics

Reporting groups

Reporting group title	Treatment period

Reporting group description: -

Reporting group values	Treatment period	Total	
Number of subjects	105	105	
Age categorical Units: Subjects			
In utero		0	
Preterm newborn infants (gestational age < 37 wks)		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)		0	
From 65-84 years		0	
85 years and over		0	
Age continuous Units: years			
arithmetic mean	53.9		
standard deviation	± 13.88	-	
Gender categorical Units: Subjects			
Female	46	46	
Male	59	59	

End points

End points reporting groups

Reporting group title	Recruited patients
Reporting group description: -	
Reporting group title	Patidegib
Reporting group description: -	

Primary: Safety

End point title	Safety[1]
End point description:	
End point type	Primary
End point timeframe:	
From start of treatment until 30 days after last dose.	

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: The study ended prematurely and no statistical analyses were performed.

End point values	Patidegib			
Subject group type	Reporting group			
Number of subjects analysed	105			
Units: % of subjects with at least one AE				
number (not applicable)	43.8			

Statistical analyses

No statistical analyses for this end point

Adverse events

Adverse events information[1]

Timeframe for reporting adverse events:

From start of treatment until 30 days after last dose.

Assessment type	Systematic

Dictionary used

Dictionary name	MedDRA
Dictionary version	21.1

Reporting groups

Reporting group title	Treated patients

Reporting group description: -

Notes:

[1] - There are no non-serious adverse events recorded for these results. It is expected that there will be at least one non-serious adverse event reported.
Justification: There were no non-serious adverse events that affected 5% or more of the patients. Overall, 46 patients had at least one adverse event.

Serious adverse events	Treated patients		
Total subjects affected by serious adverse events			
subjects affected / exposed	3 / 105 (2.86%)		
number of deaths (all causes)	0		
number of deaths resulting from adverse events			
Neoplasms benign, malignant and unspecified (incl cysts and polyps)			
Breast cancer			
subjects affected / exposed	1 / 105 (0.95%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Injury, poisoning and procedural complications			
Craniocerebral injury			
subjects affected / exposed	1 / 105 (0.95%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Cardiac disorders			
Atrial fibrillation			
subjects affected / exposed	1 / 105 (0.95%)		
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	Treated patients		
Total subjects affected by non-serious adverse events subjects affected / exposed	0 / 105 (0.00%)		

More information

Substantial protocol amendments (globally)

Were there any global substantial amendments to the protocol? Yes

Date	Amendment
30 January 2020	The study received a new EudraCT number. This occurred before the recruitment of any patient.

Notes:

Interruptions (globally)

Were there any global interruptions to the trial? No

Limitations and caveats

None reported