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

Published: 02-04-2020
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Primary Objective*To assess the safety and tolerability of Patidegib Topical Gel, 2% inpatients who have completed PellePharm Study Pelle-926-201 or Pelle-926-301Secondary Objectives*To assess the efficacy of Patidegib Topical Gel, 2% in patients...

Ethical review	Approved WMO
Status	Recruitment stopped
Health condition type	Skin neoplasms malignant and unspecified
Study type	Interventional

Summary

ID

NL-OMON49176

Source
ToetsingOnline

Brief title
PATIDEGIB Phase 3 OLE study in subjects with Gorlin syndrome

Condition

- Skin neoplasms malignant and unspecified

Synonym
Gorlin Syndrome; Basal Cell Nevus Syndrome

Research involving
Human

Sponsors and support

Primary sponsor: PellePharm, Inc.

Source(s) of monetary or material Support: PellPharm;Inc.

Intervention

Keyword: Gorlin Syndrome, Open-Label Extension Study, Patidegib Topical Gel

Outcome measures

Primary outcome

* The number of lesions on the face that are suspicious for BCC

* The number of lesions on the face that are suspicious for BCC and that are surgically removed

* The number of lesions on the face that were suspicious for BCC, removed and confirmed on histology to be BCC

* Quality of Life Assessments

Secondary outcome

* Safety Assessments

* Dermal Safety and Tolerability Events

* Physical examinations

* Pregnancy tests

Study description

Background summary

PellePharm, Inc. (PellePharm) is developing a topical formulation of patidegib for the mitigation of the disease burden of persistently developing basal cell carcinomas (BCCs) in patients with Gorlin syndrome and a high frequency of BCCs (HF-BCC). There are currently no approved therapies for Gorlin syndrome or HF-BCC. The continuous development of BCCs, especially on the face, can lead to seriously disfiguring scars and functional impairment, resulting in a significant decrease in quality of life. It is hoped that, by decreasing the number of surgeries that these patients require, their quality of life will be

greatly improved.

Study objective

Primary Objective
*To assess the safety and tolerability of Patidegib Topical Gel, 2% inpatients who have completed PellePharm Study Pelle-926-201 or Pelle-926-301

Secondary Objectives
*To assess the efficacy of Patidegib Topical Gel, 2% in patients whohave completed PellePharm Study Pelle-926-201 or Pelle-926-301

Study design

This is a multicenter, open label extension study evaluating the safety of Patidegib Topical Gel, 2%, applied topically twice daily to the face of adult subjects with Gorlin syndrome. The treatment duration is 12 months, with potential extension beyond this period. Enrollment will close approximately after the last patient in Study 926-301 has completed their Study 926-301-related procedures.

Intervention

Patidegib Topical Gel, 2% (w/w). The IP will be applied to the face defined as the area extending from the anterior hairline to the jaw line (except the eyelids).

Study burden and risks

Patidegib Topical Gel, 2 % has shown promise to potentially mitigate tumor burden, without the systemic toxicity of oral HHI (Hedgehog Inhibitors) in patients with Gorlin syndrome. The disease burden for these patients is significant, also compared to the potential risks of the Investigational Product (Based on the safety evaluation in previous clinical studies and the low systemic bioavailability of Patidegib Topical Gel, 2%,). The current benefit-risk profile is therefore deemed acceptable to support the continued development of patidegib topical gel in Gorlin patients.

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Trial sites

Listed location countries

Netherlands

Eligibility criteria

Age
Adults (18-64 years)
Elderly (65 years and older)

Inclusion criteria

1. The subject must be at least 18 years old.
2. The subject must provide written informed consent prior to any study procedures.
3. The subject must have completed PellePharm Study 926-201 or 926-301 with adequate compliance
4. Study 926-301 subjects must have completed the End of Treatment Visit in Study 301, prior to the Screening Visit in this study. They must also complete all Study 301 related procedures prior to the Baseline Visit of this study.
5. The subject must meet diagnostic criteria for Gorlin (basal cell nevus) syndrome, including major criterion #a plus 1 additional major criterion or plus 2 additional minor criteria listed in Appendix 17.2
6. The subject must be willing to abstain from application of a non-study topical medication (prescription or over the counter) to facial skin for the duration of the trial except as prescribed by the Investigator. Moisturizers and emollients are allowed. Subjects will be encouraged to use their preferred sunscreen with a sunscreen protection factor (SPF) of at least 30 daily on all exposed skin sites.
7. Female subjects must have a negative pregnancy test (serum pregnancy test at Screening Visit, urine pregnancy test at Baseline Visit). For Study 301

subjects, a negative serum pregnancy test result from Study 301 is acceptable if the test was done within 7 days of the Screening Visit of this study.
8. If the subject is a woman of child bearing potential (WOCBP), she must be willing to use birth control methods which may be considered highly effective (Appendix 17.1). Hormonal contraception must be supplemented with a barrier method (preferably condom). Birth control must start prior to Baseline, continue through the duration of the study, and for 30 days after last application of IP
9. If the subject is a male with a female sex partner who is a WOCBP, the subject must be willing to use condoms, even after a vasectomy, starting prior to Baseline, through the duration of the study, and for at least 3 months after the last application of IP.
10. The subject is willing for all facial BCCs to be evaluated and follow treatment recommendations made only by the Investigator
11. The subject is willing to forego treatment of facial BCCs with anything other than the study IP except when the Investigator believes that delay of treatment of a BCC potentially might compromise the health of the subject. In such instances, the only other allowed form of treatment is surgical.

Exclusion criteria

1. The subject has used topical treatment to the face or systemic therapies that might interfere with the evaluation of the study IP. Among these are use of the following:
a. 5-fluorouracil, imiquimod, or Ingenol mebutate (except as topical treatment to anatomical areas other than the face) systemically or topically to the skin within the 2 months prior to the Screening and Baseline Visit.
b. Systemic chemotherapy within 1 year prior to the Screening and Baseline Visit.
c. Known inhibitors of the Hedgehog signaling pathway (e.g., vismodegib, sonidegib, itraconazole) topically (except as topical treatment to anatomical areas other than the face) or systemically within 2 months prior to the Screening and Baseline Visit.
d. Photodynamic therapy (PDT) except to localized non-facial, individual BCCs within 2 months prior to the Screening and Baseline Visit.
2. The subject has a known hypersensitivity to any of the ingredients in the study IP formulation.
3. The subject is unable or unwilling to make a good faith effort to return to the study site for all study visits and tests.
4. The subject has current, recent (within five half lives of the experimental drug or if half life not known, within the past 6 months prior to the Screening Visit), or planned participation in an experimental drug study (excluding Study 926-301) while enrolled in this study.
5. The subject is a WOCBP who is unwilling or unable to comply with pregnancy prevention measures.

6. The subject is pregnant or breastfeeding.
7. The subject has any condition or situation which, in the Investigator*s opinion, may put the subject at significant risk, could confound the study results, or could interfere significantly with the subject*s participation in the study. This may include a history of other skin conditions (e.g., severe facial eczema) or diseases, metabolic dysfunction, physical examination (PE) findings, or clinical laboratory findings giving reasonable suspicion of a disease or condition that contraindicates use of an investigational drug or that might affect interpretation of the results of the study or render the subject at high risk from treatment complications.

Study design

Design

Study phase:	3
Study type:	Interventional
Masking:	Open (masking not used)
Control:	Uncontrolled
Primary purpose:	Treatment

Recruitment

NL

Recruitment status:	Recruitment stopped
Start date (anticipated):	21-07-2020
Enrollment:	10
Type:	Actual

Medical products/devices used

Product type:	Medicine
Brand name:	Not available
Generic name:	Patidegib

Ethics review

Approved WMO

Date:	02-04-2020
Application type:	First submission
Review commission:	METC academisch ziekenhuis Maastricht/Universiteit Maastricht, METC azM/UM (Maastricht)
Approved WMO Date:	17-06-2020
Application type:	First submission
Review commission:	METC academisch ziekenhuis Maastricht/Universiteit Maastricht, METC azM/UM (Maastricht)
Approved WMO Date:	07-07-2020
Application type:	Amendment
Review commission:	METC academisch ziekenhuis Maastricht/Universiteit Maastricht, METC azM/UM (Maastricht)
Approved WMO Date:	20-07-2020
Application type:	Amendment
Review commission:	METC academisch ziekenhuis Maastricht/Universiteit Maastricht, METC azM/UM (Maastricht)

Study registrations

Followed up by the following (possibly more current) registration

No registrations found.

Other (possibly less up-to-date) registrations in this register

No registrations found.

In other registers

Register	ID
EudraCT	EUCTR2020-000253-27-NL
CCMO	NL73056.068.20