

Clinuvel successful in US Phase III trial of SCENESSE® in rare skin disorder

US study in erythropoietic protoporphyria (EPP) confirms new drug safe, effective

Melbourne, Australia and Baar, Switzerland, November 11 2013

Clinuvel Pharmaceuticals Limited (ASX: CUV; XETRA-DAX: UR9; ADR: CLVLY) today announced results from its confirmatory Phase III US study of the novel drug SCENESSE® (afamelanotide) in patients diagnosed with the rare light intolerance disorder erythropoietic protoporphyria (EPP). Eighty seven adult EPP patients completed the six-month, randomised, multicentre, double-blind, placebo-controlled study (CUV039) with results showing that treatment with SCENESSE® improved patients' ability to expose their skin to light and improved their quality of life (QoL). CUV039 was conducted in seven specialist porphyria centres (Alabama, California, Michigan, New York, North Carolina, Texas and Utah) across the USA.

"This program is the first to fully and rigorously evaluate a therapy for EPP, a disease which is poorly understood globally and presents uniquely in the clinic," Dr Robert J Desnick, Dean for Genetic and Genomic Medicine and Professor and Chairman Emeritus of the Department of Genetics and Genomic Sciences at Mount Sinai School of Medicine, New York, and a lead investigator on the CUV039 study said.

"The results reflect numerically what our patients reported in the clinic: when treated with afamelanotide they can spend more time outside, experience less pain, and lead more normal lives. Professionally this is satisfying, as we may now, finally, be able to tell EPP patients that we can manage or prevent their painful symptoms and give them a freedom never before experienced," Dr Desnick said.

EPP is a rare genetic disease found mainly in fair-skinned people. It is characterised by severe phototoxicity (intolerance to light) of the skin, resulting in intolerable pain, swelling and scarring, usually of exposed areas such as the face, hands and feet. Symptoms can vary from mild to extreme lasting pain requiring hospitalisation. Patients often lead an indoor and sheltered life, avoiding light exposure to prevent symptoms.

The primary study endpoint was to determine the extent to which patients could expose their skin to direct sunlight between 10am and 6pm. A strong trend towards greater direct sunlight exposure was seen in patients receiving afamelanotide, compared to placebo. Median total direct sunlight exposure was 64.13 hours (range 0 - 650.5 hours) in the active group compared with 47.5 hours (range 0 - 224 hours) for placebo-recipients ($p=0.107$). The distribution of the number of days with sun exposure of various blocks of time was significantly different between the treatment groups ($p<0.001$). As an example, SCENESSE® recipients reported more days when they had pain-free exposure of 60 minutes or more (the time of greatest risk of burns). Further data analyses, including objective photoprovocation testing, confirmed the ability of patients on active treatment to tolerate greater light exposure and spend more time outdoors.

As a secondary endpoint, QoL was evaluated using the validated EPP-Quality of Life ("EPP-QoL") questionnaire. Following commencement of treatment the QoL of EPP patients improved, and was significantly better for active drug recipients.

The safety profile of the drug was good. Headaches and nausea were the most common adverse events, with slightly more reports of these in the treatment group.

"We have focused on delivering a therapy for these patients for more than eight years," Clinuvel's CEO, Dr Philippe Wolgen said. "Overall these results indicate that afamelanotide provides an effective treatment for patients with EPP and add to the body of evidence currently being reviewed for marketing authorisation by European regulatory authorities."

In 2011 results were announced from Phase II US and Phase III EU trials (CUV029 and CUV030) of SCENESSE® in EPP. These showed SCENESSE® could reduce the severity of EPP symptoms and enable patients to lead more normal lives. A marked improvement in QoL was also reported. Thus far, no serious safety concerns have been identified from the use of afamelanotide in more than 900 patients, including over 300 EPP patients, involved in

various clinical trials. Clinuvel is awaiting a response from the European Medicines Agency on a marketing authorisation application for SCENESSE® for EPP. SCENESSE® has been granted Orphan Drug Status in the US and Europe for EPP. Clinuvel is seeking a meeting with the US Food and Drug Administration to discuss a New Drug Application in the first quarter of 2014.

- End -

Note to journalists: Clinuvel has also released a full technical announcement on the CUV039 study, which is available on the Company's website.

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Appendix I (Following Code of Best Practice, ASX)

Name of trial

CUV039: A Phase III, Multicentre, Double-Blind, Randomized, Placebo-Controlled Study to Confirm the Safety and Efficacy of Subcutaneous Bioresorbable Afamelanotide Implants in Patients with Erythropoietic Protoporphyrria (EPP).

Primary endpoint

Determine whether afamelanotide can enable EPP patients to expose themselves to sunlight without incurring pain and phototoxic reactions, measured by duration of direct sunlight exposure between 10:00 and 18:00 hours on days when no pain is experienced (Likert pain score of 0).

Secondary endpoints

1. Determine whether afamelanotide can:
 - Increase the duration of time patients can be exposed to direct sunlight between 10:00 and 18:00 hours with no or mild pain (Likert scores of 0 to 3) and overall;
 - Improve the quality of life of patients;
 - Reduce the susceptibility to provocation with a standardized light source (minimum symptom dose).
2. Evaluate the safety and tolerability of afamelanotide implants by measuring treatment-emergent adverse events (TEAEs).

Blinding status

Double-blind.

Product development status

Good Manufacturing Practice (GMP) Standard.

Treatment method, frequency, dose levels

This was a randomised placebo-controlled study conducted in two parallel study arms for a six month period (three doses) in months when sunlight is most intense. Eligible patients received afamelanotide (16 mg implants) or placebo according to the following dosing regimen:

- Group A: administered afamelanotide implants on Days 0, 60 and 120;
- Group B: administered placebo implants on Days 0, 60 and 120.

Number of trial subjects

93 patients enrolled, 87 (93.5%) completed all patient visits.

Subject selection criteria

The participants had to fulfil all of the following criteria for study participation:

- (a) Male or female subjects with a clinical diagnosis of EPP of sufficient severity that they have requested treatment to alleviate their symptoms;
- (b) Aged 18 years old and above;
- (c) Written informed consent prior to the performance of any study-specific procedures.

Trial location

Seven trial sites across the United States of America.

Expected duration of the trial

Six month treatment period for an individual patient. Patients returned for a long term treatment follow up visit three months after the completion of the study.

Trial standard

In compliance with Good Clinical Practices (GCP) and ICH guidelines.

Appendix II About SCENESSE® (afamelanotide 16mg)

SCENESSE® is a first-in-class therapeutic being developed by Clinuvel, with the generic name (or INN) afamelanotide. An analogue of α -MSH, afamelanotide is a linear peptide which activates eumelanin of the skin, the dark pigment which is known to provide photoprotective properties (offering skin protection against light and UV radiation). SCENESSE® is administered underneath the skin as a dissolvable implant approximately the size of a grain of rice. For more information on SCENESSE® go to <http://www.clinuvel.com/en/scenesse>.

SCENESSE® is a registered trademark of Clinuvel Pharmaceuticals Ltd.

About Clinuvel Pharmaceuticals Limited

Clinuvel Pharmaceuticals Ltd (ASX: CUV; XETRA-DAX: UR9; ADR: CLVLY) is a global biopharmaceutical company focused on developing drugs for the treatment of a range of severe skin disorders. With its unique expertise in understanding the interaction of light and human skin, the company has identified three groups of patients with a clinical need for photoprotection and another group with a need for repigmentation. These patient groups range in size from 10,000 to 45 million. Clinuvel's lead compound, SCENESSE® (afamelanotide), a first-in-class drug targeting erythropoietic protoporphyria (EPP), has completed Phase II and III trials in the US and Europe.

In February 2012 SCENESSE® was filed for review by the European Medicines Agency for EPP. A confirmatory six month Phase III US EPP trial commenced in May 2012. Presently, there is no known effective treatment for EPP and SCENESSE® has been granted orphan drug status. Based in Melbourne, Australia, Clinuvel has operations in Europe and the US. For further information please visit www.clinuvel.com

For more information on EPP go to <http://www.clinuvel.com/en/erythropoietic-protoporphyria/>

Clinuvel is an Australian biopharmaceutical company focussed on developing its photoprotective drug, SCENESSE® (afamelanotide) for a range of UV-related skin disorders resulting from exposure of the skin to harmful UV radiation. Pharmaceutical research and development involves long lead times and significant risks. Therefore, while all reasonable efforts have been made by Clinuvel to ensure that there is a reasonable basis for all statements made in this document that relate to prospective events or developments (forward-looking statements), investors should note the following:

- actual results may and often will differ materially from these forward-looking statements;
- no assurances can be given by Clinuvel that any stated objectives, outcomes or timeframes in respect of its development programme for SCENESSE® can or will be achieved;
- no assurances can be given by Clinuvel that, even if its development programme for SCENESSE® is successful, it will obtain regulatory approval for its pharmaceutical products or that such products, if approved for use, will be successful in the market place

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