Positive pilot data published on SCENESSE® in rare skin disorder

Physician-led pilot study successful in Hailey-Hailey disease, future Phase II in development

Baar, Switzerland and Melbourne, Australia, October 28 2013

Clinuvel Pharmaceuticals Limited (**ASX: CUV; XETRA-DAX: UR9; ADR: CLVLY**) today announced that results from a physician-led pilot study of SCENESSE® (afamelanotide 16mg implant) have been published in the journal *Clinical and Experimental Dermatology*. The pilot study, in two patients with the rare Hailey-Hailey Disease (HHD), indicates for the first time that afamelanotide may be of therapeutic benefit by offering long-term remission (disease free period).

Hailey-Hailey Disease

Hailey-Hailey Disease (HHD, also known as familial benign pemphigus) is a rare, chronic, inherited disorder where epidermal skin cells (keratinocytes) don't adhere correctly. This causes periodic eruption of plaque-like lesions and blisters on areas where skin folds, often on the neck, armpits or groin. HHD can be very distressing for patients, with outbreaks on the legs and groin leading to immobilisation due to the pain of friction, and a high risk of skin infection.

Current treatments, including topical corticosteroids and antibiotics, can manage minor outbreaks, but are seen as ineffective in severe cases, and there is no professional consensus on a first-line therapy as no remission has been achieved in these patients. Exact prevalence of HHD is unknown, however clinical reports to Clinuvel suggest that there are 20-30 HHD affected families in most Western European countries.

Published observations from preclinical and clinical pilot studies

The study authors hypothesised that an antioxidant effect of afamelanotide in keratinocytes may assist in reducing the effect of HHD and published several preclinical observations, based on laboratory work, to support their hypothesis, noting that the "clinical and laboratory results provided a strong rationale for the use of afamelanotide for the treatment of HHD".

A physician-led open-label observational study of SCENESSE® was then conducted in two HHD patients at San Gallicano Hospital in Rome. Both patients received two doses of SCENESSE®, one at the start of the study and the second after 30 days. In both patients lesions were reduced after 30 days and completely resolved after 60, with no disease recurrence until eight months after the withdrawal of treatment. Both patients reported an improvement in quality of life, measured by the SF-36 dermatology questionnaire, a common assessment tool in clinical studies. The drug was well tolerated by both patients.

Comment

"HHD is a poorly understood disease which has a chronic impact on the lives of patients and lacks an effective therapy," Clinuvel's Acting Chief Scientific Officer, Dr Dennis Wright said. "It's encouraging that afamelanotide may be able to benefit HHD patients."

"Clinuvel is well aware of the potential of afamelanotide to treat a number of skin disorders," Clinuvel's CEO, Dr Philippe Wolgen said. "This research proposes a new avenue for afamelanotide to help treat an expanded group of patients with severe, chronic and rare disorders. Based on the clinical observations to date, Clinuvel's team is now working with leading HHD physicians to arrive at a larger study designed to demonstrate a clinically meaningful effect for patients to achieve long term remission; a life without lesions. Perhaps most reassuring from this publication is that SCENESSE® was well tolerated by these patients, consistent with the drug's long standing safety profile."

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Publication

Biolcati G et al (2013). "Efficacy of the melanocortin analogue Nle4-D-Phe7-α-melanocyte-stimulating hormone in the treatment of patients with Hailey–Hailey disease." *Clin Exp Dermatol*. Epub 25 Oct.

References

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Le Donne M et al (2008). "Chronic vulvocrural dermatitis with burning and itching." *CMAJ*. 179(6): 555–556.
O'Neill MJF (2005). "Benign Chronic Pemphigus." *Online Mendelian Inheritance in Man*. Online: <http://omim.org/entry/169600>.

About SCENESSE® (afamelanotide)

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.

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 more information go to http://www.clinuvel.com.

Investor contacts:

Australia:	Clinuvel Pharmaceuticals Limited,	T: +61 3 9660 4900
Europe:	Clinuvel AG,	T: +41 41 767 45 45
E:	investorrelations@clinuvel.com	

Media contacts:

Lachlan Hay
Clinuvel AG
Baar, Switzerland
T: +41 41 767 45 45
Lachlan.Hay@clinuvel.com