

## Clinuvel and FDA discuss EPP indication and regulatory pathways for SCENESSE®

*Type C meeting provides guidance for follow up and further discussions between FDA and Clinuvel*

Leatherhead, UK and Melbourne, Australia, October 5, 2015

Clinuvel Pharmaceuticals Limited (**ASX: CUV; ADR:CLVLY; XETRA:DAX**) today announced that on September 30 it met in Silver Spring, USA, with the US Food and Drug Administration's (FDA's) Division for Dermatology and Dental Products (DDDP) and representatives of the Center of Drug Evaluation and Research (CDER). The objective of the meeting was to discuss the US regulatory review of SCENESSE® (afamelanotide 16mg) to be made available to American erythropoietic protoporphyria (EPP) patients. In 2014 SCENESSE® was granted marketing authorisation by the European Medicines Agency as a prophylactic photoprotective drug for adult EPP patients.

The DDDP stated that it was seeking a regulatory pathway to make SCENESSE® available in the US, and the regulatory avenue of Accelerated Approval was suggested, pending FDA's review, analyses and further discussions on available photoprovocation and data on quality of life in EPP patients.

A discussion was held on the drug's benefits to patients who had received SCENESSE®. Expert European and US porphyria clinicians were invited to share their experience in the treatment of EPP patients and the use of SCENESSE® in their patients. Further discussions will be held with the DDDP following the review of photoprovocation and quality of life data.

- End -

### References

Langendonk J et al (2015). Afamelanotide for Erythropoietic Protoporphyria. *NEJM*. 373(1):48-59.

Minder EI et al (2010). Patient-recorded outcome to assess therapeutic efficacy in protoporphyria-induced dermal phototoxicity: a proposal. *Health Qual Life Outcomes*. 8:60.

Rufener EA (1987). Erythropoietic protoporphyria: a study of its psychosocial aspects. *Brit J Dermatol*. 116:703-8.

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### About erythropoietic protoporphyria (EPP)

EPP is characterised by phototoxicity (absolute intolerance to light) of the skin resulting in severe reactions, swelling, scarring and a state of distress. During phototoxic episodes patients experience long-term swelling of exposed body surfaces such as the face, hands and feet. A severe reaction – triggered by exposure to light – may result in hospitalisation. Patients do not respond to any analgesics or medication and following light exposure are typically unable to function. Due to the lifelong risk posed by light and UV, patients are often forced to lead an isolated indoor life deprived of normal activities.

### **Photoprovocation and Quality of Life data**

Due to a genetic defect EPP patients are intolerant to light and sun. 'Photoprovocation' is a standardised methodology of provoking limited EPP symptoms using specific controlled beams of light. In all five clinical studies of SCENESSE® patients were subjected to photoprovocation under laboratory conditions.

'Quality of life' assessments utilise patient surveys to capture and analyse the impact of disease and treatment. Clinuvel has conducted quality of life surveys in all five EPP studies. Over the past eight years a disease-specific EPP Quality of Life questionnaire, developed by Clinuvel in association with expert EPP physicians, was used during clinical trials because other assessment tools were found to be unsuitable for evaluating the quality of life of EPP patients

### **Forward-Looking Statements**

This release to the Australian Securities Exchange and to press contains forward-looking statements, including statements regarding future results, performance or achievements. These statements involve known and unknown risks, uncertainties and other factors which may cause Clinuvel's actual results, performance or achievements to be materially different from any future results, performances or achievements expressed or implied by the forward-looking statements. These statements reflect our current views with respect to future events and are based on assumptions and subject to risks and uncertainties. Given these uncertainties, you should not place undue reliance on these forward-looking statements. Some of the factors that could affect the forward-looking statements contained herein include: that the FDA may require additional studies beyond the studies planned for product candidates or may not provide regulatory clearances, including for SCENESSE®; that the FDA may not provide regulatory approval for any use of SCENESSE® or that the approval may be limited; that Clinuvel may never file an NDA for SCENESSE® regulatory approval in the US; that the Company may not be able to access adequate capital to move its vitiligo programs forward; that the Company may not be able to retain its current pharmaceutical and biotechnology key personnel and knowhow for further development of its product candidates or may not reach favourable agreements with potential pricing and reimbursement agencies in Europe and the US.

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