# SCENESSE® attains historic breakthrough European Marketing Authorisation

*European Medicines Agency CHMP approves novel drug for erythropoietic protoporphyria patients under exceptional circumstances (ART 14 (8) OF REGULATION (EC) NO 726/2004)*

Zug, Switzerland and Melbourne, Australia, October 27, 2014

**EXECUTIVE SUMMARY**

- SCENESSE® attains an historic breakthrough EMA approval for adult EPP patients extremely intolerant of light and UV
- Marketing authorisation in 31 European States
- Australian listed Clinuvel (ASX: CUV) has taken SCENESSE® from discovery to commercialisation
- SCENESSE® as the first drug for which patients' and physicians' clinical experiences were integrated EMA's formal decision process
- Investor telephone conference-call Wednesday 29 October:
  1800 Melbourne = 0900 Zurich = 0300 New York = 1500 Singapore
  Details will be released to the ASX on Tuesday October 28.

Clinuvel Pharmaceuticals Limited (**ASX: CUV; XETRA-DAX: UR9; ADR: CLVLY**) is proud to announce that the European Medicines Agency's (EMA's) Committee for Medicinal Products for Human Use (CHMP) has voted in favour of marketing authorisation (MA) of Clinuvel's drug SCENESSE® (afamelanotide 16mg) for adult patients with erythropoietic protoporphyria (EPP).

SCENESSE® is a first-in-class drug (belonging to a new drug category). EPP is a debilitating, rare genetic disorder clinically regarded as extreme intolerance to light and UV (phototoxicity). An estimated 10,000 EPP patients are affected worldwide, 45% of whom live within Europe.

Clinuvel conducted five trials testing SCENESSE® in approximately 350 adult EPP patients across Australia, Europe and the USA. The first EPP patient received the drug in 2006. Clinuvel submitted its dossier for EMA evaluation on 6 February 2012.

In September 2014, it was announced that the EMA - for the first time in its history - was incorporating patients' and physicians' clinical experiences with SCENESSE® in the CHMP's decision process.

**EMA Decision**

On 23 October 2014 in its plenary session the CHMP voted in favour of marketing authorisation of SCENESSE®. This decision takes into account the challenges and limitations of conducting clinical trials in rare and severe disorders and allows access to treatment for patients without alternative medcine.[1] Marketing authorisation is given for distribution of SCENESSE® across 31 European states.[2] The CHMP's decision will now be sent to the European Commission for formal ratification, expected within 67 days.

Clinuvel and the EMA have agreed to a comprehensive post-authorisation pharmacovigilance plan to monitor patients' safety long term. SCENESSE® will be distributed through academic and specialised centres.

**Comment**

"On behalf of the Board, I congratulate shareholders and patients, experts in the field and Clinuvel's teams around the world," Clinuvel's Chair, Stan McLiesh said. "I have witnessed from our staff a decade long dedication and an enormous zeal which has come together for patients and all stakeholders in a marvellous outcome, one rarely accomplished in pharmaceutical development and which is now unique to Australia."

"Medical innovation requires an exceptional focus with a consistent strategy, passionate team and long term trust from patients, expert physicians and regulatory authorities worldwide," Clinuvel's CEO, Dr Philippe Wolgen said.

"Today I am excited but mostly proud that Clinuvel overcame a number of hurdles from having identified an unmet clinical need and taken a molecule from discovery to final commercial product. I congratulate our Acting Chief Scientific Officer Dr Wright and his entire team for the extraordinary achievement."

"This outcome greatly impacts Clinuvel, our further operations and intrinsic value. The immediate goal is to distribute the drug to European patients in the coming months, while we owe it to our American and Asia-Pacific patient community to accelerate the regulatory process for them to gain access to SCENESSE®," Dr Wolgen said.

- End -

Investor note: Clinuvel will host a teleconference with shareholders on Wednesday October 29 at 1800 Melbourne time. Details will be released to the ASX on Tuesday October 28.

[1] According to Article 14(8) of Regulation (EC) No 726/2004. Further details of Approval under Exceptional Circumstances can be found on the EMA's website at:
http://www.ema.europa.eu/docs/en_GB/document_library/Regulatory_and_procedural_guideline/2009/10/WC500004883.pdf
[2] The 28 EU member nations as well as Iceland, Liechtenstein and Norway.

**Investor enquiries**
Clinuvel Pharmaceuticals Ltd (Australia)
+61 3 9660 4900
investorrelatons@clinuvel.com

**About SCENESSE®**
SCENESSE® is an injectable controlled-release implant formulation containing 16mg of afamelanotide, a novel molecule from a family of drugs known as melanocortins. SCENESSE® is the first melanocortin approved by a leading regulatory authority for the prevention of skin burns and symptoms in EPP. The drug provides anti-oxidative effects and activates the skin's dark pigment, eumelanin, providing EPP patients with a biological barrier between their skin and the various wavelengths of light triggering phototoxic reactions.

The clinical relevance of SCENESSE® is to facilitate patients to expose themselves to light and UV, preventing them from incurring the characteristic burns seen in EPP. SCENESSE® has been clinically trialled in over 900 patients, including 350 adult EPP patients. In October 2014 the European Medicines Agency recommended approval for SCENESSE® under exceptional circumstances.

**About erythropoietic protoporphyria (EPP)**
EPP is characterised by severe phototoxicity (absolute intolerance to light) of the skin resulting in intolerable pain, swelling, scarring and a state of distress. During phototoxic episodes patients experience long-term swelling of the exposed body surfaces such as the face, hands and feet. A severe reaction – triggered by exposure to light, particularly UV 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 known risk to light and UV, patients often lead lifelong an isolated indoor life deprived of normal activities.

**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 patient populations with a clinical need for photoprotection and another population with a need for repigmentation. These patient groups range in size from 5,000 to 45 million. Clinuvel's lead compound, SCENESSE® (afamelanotide 16mg), a first-in-class drug targeting erythropoietic protoporphyria (EPP), has completed Phase II and III trials in the US and Europe, and has been recommended for marketing authorisation under exceptional circumstance by the European Medicines Agency. Based in Melbourne, Australia, Clinuvel has operations in Europe, the US and Singapore.
For more information go to http://www.clinuvel.com.

- 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

Level 14 / 190 Queen Street
Melbourne, Victoria 3000
Australia

T +61 3 9660 4900
F +61 3 9660 4999

www.clinuvel.com