# FDA awards SCENESSE® Fast Track designation for treatment of EPP

Executive summary:

- FDA awards Fast Track Designation[1] to expedite review of SCENESSE® in EPP
- NDA filing allowed on a rolling basis
- FDA hosting a Scientific Workshop[2] on EPP to learn more about disease and drug treatment

Melbourne, Australia and New York, USA, July 6 2016

Clinuvel Pharmaceuticals [**ASX: CUV; Nasdaq International Designation ADR: CLVLY; Xetra-DAX: UR9**] today announced that the US Food and Drug Administration (FDA) has granted SCENESSE® (afamelanotide 16 mg) Fast Track designation for the treatment of erythropoietic protoporphyria (EPP). This designation recognises the severity of EPP and the unmet medical need of the disorder in the US. SCENESSE® is commercially available for EPP patients in Europe.[3]

**US FDA REVIEW PROCESS**

Clinuvel has been in regular contact with the FDA since 2009 to discuss its clinical trial program, the complexity of EPP and the unique nature of SCENESSE® as a new molecular entity. Fast Track designation has been recommended following an initial FDA review of the Clinuvel data sets requested by the relevant scientific division of the FDA in early 2016.

The Fast Track designation enables Clinuvel to file a New Drug Application (NDA) on a rolling basis for US regulatory assessment. A pre-NDA meeting will be scheduled shortly with the FDA to discuss the timing of the first filing of the scientific dossier which will also be eligible for priority review, allowing an abbreviated review time of approximately six months.

The FDA intends to host a Scientific Workshop[2] on EPP on October 24 to learn more about the impact of EPP on patients and the proposed drug treatment.

In 2008, the Office for Orphan Products of the FDA awarded SCENESSE® orphan designation for EPP. Significant advantages of the orphan designation include substantially lower NDA filing fees and market exclusivity for seven years following first market access in the US.

**US EPP COMMUNITY**

Since the commencement of a US clinical program for EPP in 2009 Clinuvel has received a large number of US patient requests to access SCENESSE®. Clinuvel has communicated to patients and patient associations that it must await US regulatory clearance for the drug.

There is a strong network of expert EPP treatment centres in the US, coordinated by the US Porphyrias Consortium. Expert centres will be notified of the expedited US regulatory process to make the treatment available to their patients.

**COMMENTARY**

"This is excellent news for US EPP patients, as Clinuvel has been in productive discussion with the US FDA for a considerable time directed towards making the drug available," Clinuvel's Chair, Stan McLiesh said.

"The FDA has shown the vision to first look at Clinuvel's scientific data and agree to a swift review process and involve patients and expert physicians for a comprehensive meeting to learn more about the disease and the potential of the innovative treatment," Mr McLiesh said.

"Looking ahead, we can expect that the FDA will ask Clinuvel to implement strict pharmacovigilance measures once the drug is available in the US, similar to the systems we have now put in place in Europe," Clinuvel's Acting Chief Scientific Officer, Dr Dennis Wright said. "We see increasing harmonisation between EMA and FDA when it comes to managing the risks of novel treatments made available to the public. The common regulatory thinking is reflected in

an approval process where the voice of patients and physicians is given weight in decision making. Clinuvel's regulatory team is now being expanded to ensure that the NDA dossier is to a high standard and that we move through this review process swiftly," Dr Wright said.

- End -

[1]Food and Drug Administration Modernization Act, 1997.

[2]FDA Scientific Workshop: 24 October 2016: http://www.fda.gov/drugs/newsevents/ucm501389.htm.

[3] SCENESSE® (afamelanotide 16mg) is approved in Europe as an orphan medicinal product for the prevention of phototoxicity (anaphylactoid reactions and burns) in adult patients with EPP. The innovative nature of the therapy in the orphan disorder, the lack of available scientific instruments to adequately measure the therapy, ethical considerations and the drug's positive safety profile were some of the factors which led to the European marketing authorisation of SCENESSE®. Information on the product can be found on Clinuvel's website at www.clinuvel.com.

**About erythropoietic protoporphyria (EPP)**
Erythropoietic protoporphyria (EPP) is a rare genetic storage disorder that causes severe anaphylactic-like reactions to light (phototoxicity). Patients complain of burning under the skin, usually within minutes of exposure to bright lights, especially sunlight. The first sensation – phototoxicity – is the result of a chemical reaction taking place underneath patients' skin which results in burns and damage of the tissues.
EPP symptoms can be acute, or delayed (subacute), most often expressed as generalised oedema, effusion in tissues and distortion of the skin. As little as a few minutes of light outdoors (even when it is overcast or transmitted through a window) or artificial light exposure may be sufficient to evoke EPP symptoms.
Phototoxicity is unresponsive to traditional pain and burn management techniques and patients can be incapacitated for days before reactions subside. Most patients withdraw from light exposure in order to manage their phototoxic symptoms.

**About Clinuvel Pharmaceuticals Limited**
Clinuvel Pharmaceuticals Ltd (ASX: CUV; NASDAQ INTERNATIONAL DESIGNATION ADR: CLVLY; XETRA-DAX: UR9) is a global biopharmaceutical company focused on developing drugs for the treatment of a range of severe disorders. With its unique expertise in understanding the interaction of light and human skin, the Clinuvel has identified patient populations with a clinical need for photoprotection and for repigmentation. The worldwide prevalence of these patient groups range from 5,000 to 45 million. Clinuvel's lead compound, SCENESSE® (afamelanotide 16mg), was approved by the European Commission in 2014 for the prevention of phototoxicity (anaphylactoid reactions and burns) in adult patients with erythropoietic protoporphyria (EPP).
Headquartered in Melbourne, Australia, Clinuvel has operations in Europe, Switzerland, the US and Singapore.
For more information go to http://www.clinuvel.com.



**Media enquiries**

| | | |
|---|---|---|
| Lachlan Hay, Clinuvel (UK) Ltd. | +44 1372 860 765 | Lachlan.Hay@clinuvel.com |
| Nick Miles, Cabinet Privé de Conseils s.a. | +41 22 321 4540 | miles@cpc-pr.com |
| Ted Agne, The Communications Strategy Group Inc. | +1 718 631 3117 | edagne@comstragroup.com |

**Investor enquiries**
InvestorRelations@clinuvel.com

**Forward-Looking Statements**
This release to the Australian Securities Exchange and to press may contain 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 advance its vitiligo programs; 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.

Level 5, 160 Queen Street
Melbourne, Victoria 3000
Australia

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

www.clinuvel.com