US FDA UPDATE

Agency requests additional information on SCENESSE® new drug application for the treatment of EPP

Melbourne, Australia, and New York, USA, 05 September 2018

EXECUTIVE SUMMARY

- *FDA requests further documentation as part of the new drug application (NDA)*
- *Decision on Priority Review will be made following satisfaction of all agency requests*

CLINUVEL PHARMACEUTICALS LTD **(ASX: CUV; XETRA-DAX: UR9; NASDAQ INTERNATIONAL DESIGNATION: CLVLY)** today announced that the US Food and Drug Administration (FDA) has issued a request for further documentation to support CLINUVEL's new drug application (NDA) for SCENESSE® (afamelanotide 16mg). This additional information covers product manufacturing information and details from the European post-authorisation use of SCENESSE®.

FDA TIMELINE AND STATUS

SCENESSE® has been developed as a first-line pharmaceutical product aimed at treating patients with the orphan (rare) genetic disorder erythropoietic protoporphyria (EPP). The product was granted orphan drug designation by the FDA in 2008.

In July 2016 the FDA, having assessed the clinical data package for the main EPP studies, declared the data were ready for NDA submission, and in November 2016 a pre-NDA meeting was held. In October 2016, the FDA organised a first Scientific Workshop on EPP as part of a pilot scheme to involve patients and their families in the scientific review of disease and treatment solutions.

On 22 June 2018 CLINUVEL filed the final module of the NDA for SCENESSE® under "rolling review", with a request for Priority Review. The latest FDA documentation request represents a further step in the review of the submission, which was made under "rolling review" of the NDA. It is anticipated that after submission of the additional documentation, all information requested will be sufficient, after which the Division of Dermatology and Dental Products will take a decision on Priority Review.

CLINUVEL seeks regulatory approval for the same treatment dose and regimen in the United States as is currently approved in the European Union, where SCENESSE® is prescribed to EPP patients by clinical experts at specialised treatment centres.[1]

COMMENTARY

"We view the FDA's latest request as an integral part of the ongoing dialogue between CLINUVEL and the agency in our application to make SCENESSE® available in the United States," CLINUVEL's Chief Scientific Officer, Dr Dennis Wright said. "It is apparent during our interactions that in launching a first-in-class pharmaceutical and novel medicinal product, the agency is focussing on further technical information supporting SCENESSE®, as well as real world evidence in EPP patients currently coming on treatment in Europe.

"I am confident the FDA will analyse these data as a final step before it takes a stance on Priority Review. Since we are in frequent contact with the agency I believe that both parties are working towards the desired clinical outcome for US EPP patients, although at this stage further timelines have not been provided by the FDA," Dr Wright said.

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[1]SCENESSE® (afamelanotide 16mg) is approved in Europe as an orphan medicinal product for the prevention of phototoxicity in adult patients with EPP. Information on the product can be found on CLINUVEL's website at www.clinuvel.com.

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 and delivering treatments for patients with a range of severe genetic and skin disorders. As pioneers in photomedicine and understanding the interaction of light and human biology, CLINUVEL's research and development has led to innovative treatments for patient populations with a clinical need for photoprotection and repigmentation. These patient groups range in size from 5,000 to 45 million worldwide. 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). More information on EPP can be found at http://www.epp.care. Headquartered in Melbourne, Australia, CLINUVEL has operations in Europe, Switzerland, the US and Singapore, with the UK acting as the EU distribution centre.
For more information go to http://www.clinuvel.com.

About erythropoietic protoporphyria (EPP)
Erythropoietic protoporphyria (EPP) is a rare metabolic disorder that causes severe anaphylactoid reactions to light (phototoxicity). Patients incur physical burns and ulcers, and are in state of crisis following light exposure, summarised as phototoxicity. This usually occurs within minutes of exposure to bright lights, especially sunlight.
EPP symptoms can be acute, or delayed (subacute), most often expressed as generalised oedema, effusion in tissue 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.

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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; that the Company may incur unexpected delays in the outsourced manufacturing of SCENESSE® which may lead to it being unable to supply its commercial markets and/or clinical trial programs.

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