FDA SCHEDULES PRE-NDA MEETING FOR SCENESSE®

On 7 November the US Food and Drug Administration (FDA) will hold a meeting with CLINUVEL to discuss the submission a New Drug Application (NDA) for SCENESSE® (afamelanotide 16mg) in the treatment of erythropoietic protoporphyria (EPP)

Melbourne, Australia and New York, USA, 13 October 2016

EXECUTIVE SUMMARY

- The FDA scheduled a pre-NDA[1] meeting on 7 November to discuss CLINUVEL's planned US regulatory submission for SCENESSE®
- A key focus will be the establishment of Risk Evaluation and Mitigation Strategies (REMS) and post-authorisation safety measures
- Under Fast Track Designation[2] – one of FDA's Expedited Programs for Serious Conditions – submission of the NDA is allowed *on a rolling basis*
- Application fees waived for the orphan drug designated[3] product SCENESSE®
- On 24 October the FDA will hold an EPP Workshop with patients and physicians to gain a better understanding of the disease

CLINUVEL **[ASX: CUV; Nasdaq International Designation ADR: CLVLY; Xetra-DAX: UR9]** today announced that the FDA has confirmed a pre-NDA to be held on 7 November between the company and the FDA's Division of Dermatology and Dental Products (DDDP). The meeting will focus on the contents of the scientific dossier for SCENESSE® (NDA) with the aim of gaining FDA approval to treat US patients diagnosed with the rare genetic disorder EPP.

SCENESSE®, a New Molecule Entity (NME), has been granted both a Fast Track designation and Orphan Drug designation by the FDA. These regulatory mechanisms aim to encourage and expedite novel drug development and review for diseases with unmet clinical needs.

In 2014, SCENESSE® obtained marketing authorisation for the treatment of EPP from the European Medicines Agency (EMA)[4] and is currently being prescribed in a number of European countries.

PRE-NDA (TYPE-B) MEETING, 7 NOVEMBER 2016

During the meeting agreement is expected on the timing of the NDA filing and the establishment of Risk Evaluation and Mitigation Strategies (REMS) to ensure the safety and long term follow up of US EPP patients. Other issues to be discussed are chemistry, manufacturing and controls, non-clinical, the clinical effectiveness and safety sections of the company's planned NDA for SCENESSE® under Section 505(b)(1) of the Federal Food, Drug, and Cosmetic Act.

BENEFIT-RISK ASSESSMENT UNDER PDUFA V

For the product to be licensed and made available to US EPP patients, the FDA's Center for Drug Evaluation and Research (CDER) must arrive at a positive benefit-risk assessment of SCENESSE®.

The fifth Prescription Drug User Fee Act (PDUFA V)[5] provides the framework under which CDER make their benefit-risk assessment, taking into account the condition treated and the nature and extent of the unmet medical need.

PDUFA V also commits the FDA to Patient-Focused Drug Development. In this context, on 24 October the DDDP is hosting an EPP Workshop to obtain the patients' and physicians' perspective on the disease, its impact on daily life,

and their experiences with current treatment. The feedback gained from the workshop is expected to assist the FDA during the SCENESSE® review process.

Under the Fast Track designation – one of the FDA's 'Expedited Programs for Serious Conditions' – the FDA can review individual modules of a submission on a *rolling basis.* Pending the discussion with the FDA, it is expected that CLINUVEL will be able to file the first part of its scientific dossier in 2017.

The Fast Track designation also allows for an Advisory Committee to be called during the review process if the FDA wishes to incorporate additional professional opinions of independent porphyria experts, patients and other stakeholders. The orphan drug designation, granted under 21 CFR 316 Subpart C, waives all application fees for the NDA filing.

COMMENTARY
"I am pleased to have arrived at this most important juncture, having worked for nearly 12 years towards this goal with our clinical, regulatory, and finance teams," CLINUVEL's Acting Chief Scientific Officer, Dr Dennis Wright said.

"The 7 November meeting will inform the FDA of our intended timeline and sequence of filing of dossier sections and offers the opportunity to exchange views on content and format," Dr Wright said.

"In almost a decade of interaction with the FDA we have strived to maintain an open dialogue on our clinical program and data, and have been proactive in discussing the anticipated REMS for an innovative therapy," CLINUVEL's Global Director, Regulatory Affairs, Ms Nicoletta Muner said. "The DDDP's new leadership has shown a thoughtful approach by reviewing our clinical data and granting Fast Track designation. CLINUVEL's current distribution activities in Europe form the basis of our proposal to the FDA on how we plan to establish similar systems and processes in the US to optimise long-term follow up of EPP patients."

"Our task is to ensure that US patients secure access to SCENESSE® as soon as possible. We expect that EPP patients' willingness and ability to change their restricted light-avoidance behaviour will only become fully appreciated in a post-marketing environment under conditions of use," Ms Muner said.

- End -

Notes
[1] Type B meetings are held at specific points in the development of a new molecular entity, and include pre-new drug application meetings (21 CFR 312.47).

[2] Fast track is a process designed to facilitate the development, and expedite the review of drugs to treat serious conditions and fill an unmet medical need. See CLINUVEL's announcement 6 July, 2016.

[3] Federal Food, Drug & Cosmetic Act (2008), section 526.

[4] 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 an 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.

[5] The Food and Drug Administration Safety and Innovation Act (FDASIA; 2012). This law includes the reauthorisation of the Prescription Drug User Fee Act (PDUFA V) that provides FDA with the necessary resources to maintain a predictable and efficient review process for human drug and biologic products.

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

SCENESSE® is a registered trademark of CLINUVEL PHARMACEUTICALS LTD.

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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.

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