CLINUVEL PROVIDES UPDATE ON SCENESSE® FDA FILING

CLINUVEL TO SUBMIT EUROPEAN POST-MARKETING DATA AS PART OF NDA FOR EPP

Melbourne, Australia, and Leatherhead, UK, 19 September 2017

- EUROPEAN POST-MARKETING SAFETY DATA TO BE SUBMITTED AS PART OF NEW DRUG APPLICATION (NDA), EXPECTED IN DECEMBER 2017
- NO RISK EVALUATION & MITIGATION STRATEGIES (REMS) EXPECTED BUT A SIMILAR POST-MARKETING PROGRAM AS IN EUROPE
- FDA ANSWER IS EXPECTED TO THE REQUEST FOR PRIORITY REVIEW
- FAST TRACK = ROLLING REVIEW

CLINUVEL PHARMACEUTICALS LTD [**ASX: CUV; NASDAQ INTERNATIONAL DESIGNATION: CLVLY; XETRA-DAX: UR9**] today announced an update on its submission of the New Drug Application (NDA) for CLINUVEL's drug SCENESSE® (afamelanotide 16 mg) to the US Food and Drug Administration (FDA).

SCENESSE® THERAPY FOR ULTRA-RARE DISORDER ERYTHROPOIETIC PROTOPORPHYRIA
CLINUVEL is seeking approval from the FDA to market SCENESSE® for the prevention of phototoxicity in adult patients with the rare genetic disorder erythropoietic protoporphyria (EPP) in the US. Patients with EPP experience severe anaphylactoid reactions and burns (phototoxicity) following brief exposure to visible light, both artificial and natural light sources, which can incapacitate patients for days or weeks. SCENESSE® activates melanocytic output (pigment cells) of the epidermis and the resulting eumelanin acts as a protective light barrier and potent antioxidant. In randomised placebo-controlled clinical trials SCENESSE® was shown to reduce the incidence and severity of phototoxic reactions in adult EPP patients. SCENESSE® treatment also enabled EPP patients to expose themselves to light, facilitating greater freedom and improving quality of life.

SCENESSE® was granted marketing authorisation for adult EPP patients in Europe in December 2014[1] and prior to the proposed therapy there have not been any medicinal remedies for EPP patients.

US REGULATORY STATUS: ORPHAN DRUG AND FAST TRACK DESIGNATION
The FDA has recognised that afamelanotide meets an unmet clinical need and treats a severe genetic condition for patients who are life-long deprived of light. SCENESSE® was granted orphan drug designation by the FDA in 2008 for the treatment of EPP patients and Fast Track Designation (FTD) in 2016, allowing for a 'rolling review' of the NDA. The orphan drug designation provides R&D and review incentives for drugs which may not otherwise be commercially viable to develop, while FTD assists to ensure that innovative drugs reach the patient population earlier than would be the case during a standard review process. The 'rolling review' under the FTD allows the FDA to start the review of the scientific dossier only when all modules have been submitted and have passed formal validation, a two-month process after submission of the final NDA module.

CLINUVEL obtained a positive FDA answer on acceptance of the current safety data, as the FDA issued a carcinogenicity waiver in 2017.

This year CLINUVEL applied for a Priority Review which would secure a maximum review period of six months, compared to the standard ten months. It is expected that the FDA will answer the request for Priority Review at the start of the review period.

The FDA has initiated a regulatory pilot scheme to involve patients in explaining the impact of diseases on their lives. The Division for Dental and Dermatology Products hosted a scientific workshop on EPP in October 2016.[2]

EUROPEAN POST-MARKETING SCENESSE® DATA
European distribution of SCENESSE® started in 2016 under a rigorous Risk Management Plan in which the ongoing administration of the drug in European EPP Expert Centres is closely monitored. This involves safety monitoring as well as data collection through the European EPP Disease Registry as part of a Post Authorisation Safety Study (PASS) protocol.

The safety profile of SCENESSE® has been positive to date and no safety concerns have been detected from the European distribution thus far. The feedback from the expert EPP treatment centres and patients has been very positive, whereby over 98 percent of patients have requested and received treatment for the second year running.

US POST-MARKETING SCENESSE® DATA
CLINUVEL has agreed with the FDA that safety data generated under the European PASS will form part of the NDA submission. The first full statistical analyses of the European post authorisation data are due to be available in December 2017.

In introducing a novel therapy for a rare and relatively unknown disease, it is usually expected that the post-licensing safety monitoring will be strict. Following discussion with the FDA, it is now unlikely that a Risk Evaluation and Mitigation Strategies (REMS) will be required for SCENESSE®. Instead, CLINUVEL has been given the choice to design a pharmacovigilance program akin to the current European program using one or two EPP disease registries.

COMMENTARY
"Given the novelty of afamelanotide as a systemic photoprotectant, our objective is to provide the FDA with a quality dossier in order to maximise the chances of regulatory approval," CLINUVEL's Acting Chief Scientific Officer, Dr Dennis Wright said.

"Paramount to success is our ability to demonstrate to the FDA that SCENESSE®, as an innovative and first-in-class treatment, is safe. A pivotal part of our NDA will be the inclusion of 12-month data analyses from European EPP patients. We keep working towards the best possible dossier to obtain marketing authorisation."

- End -

[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.

[2] Further details on this workshop are available at https://www.fda.gov/drugs/newsevents/ucm501389.htm.

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 product, 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.

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