Media release

CLINUVEL

ASX: CUV
Nasdaq International Designation: CLVLY
XETRA-DAX: UR9

FDA APPROVES SCENESSE® FOR GENETIC DISORDER

New drug approved by US regulatory authorities for metabolic disorder erythropoietic protoporphyria (EPP)

Melbourne, Australia, 09 October 2019

The US Food and Drug Administration (FDA) has approved a new drug as the first ever treatment for a rare genetic metabolic disorder which causes absolute intolerance to light. SCENESSE® (afamelanotide 16mg), the first drug developed by Australian biopharmaceutical company CLINUVEL, has been approved to "*increase pain free light exposure in adult patients with a history of phototoxic reactions from erythropoietic protoporphyria (EPP)*".

"Today is a memorable day and victory for EPP patients, their families, and the global medical community who have all supported the development and US approval of the first ever treatment for this debilitating condition and the world's first systemic photo protective drug," CLINUVEL's Chief Scientific Officer, Dr Dennis Wright said.

"EPP patients are born with the disease and need to avoid all sources of light exposure, including sunlight throughout their life, or risk incapacitating burns. SCENESSE® is a novel drug and formulation, developed by CLINUVEL to provide photoprotection for EPP patients, enabling patients to expose to light, providing them a freedom they never had," Dr Wright said.

EPP is an inherited metabolic disorder of the heme biosynthesis pathway which causes lifelong phototoxicity due to the accumulation and storage of the compound protoporphyrin IX (PPIX) in the blood and tissues. When exposed to visible light and near-visible ultraviolet radiation, PPIX is activated, causing damage to surrounding tissue. Patients report they experience excruciating burning pain underneath their skin which can last days or weeks and forces them to avoid all further exposure to light. It is estimated that there are 5,000 to 10,000 EPP patients worldwide.

In 2014, SCENESSE® was approved by the European Medicines Agency for EPP. The drug binds to the melanocortin-1 receptor on skin cells and sets in motion a cascade of cellular events, one of which is the activation of the pigment melanin to provide a physical barrier to visible and invisible light in EPP patients. The drug is administered as a 16mg controlled-release injectable implant, designed to provide protection for up to 60 days.

"On this day, there are many professionals, patient organisations and stakeholders to be grateful to, but I should not forget the FDA's Divisional Director who led the review of SCENESSE® in EPP," CLINUVEL's Chief Executive Officer, Dr Philippe Wolgen said. "Our team is granted very little time to celebrate and now needs to shift its focus to facilitating drug product access for US EPP patients."

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Notes to editors:
A longer technical release has been issued to the Australian Securities Exchange and is available on CLINUVEL's website www.clinuvel.com.
SCENESSE® (afamelanotide16mg) is approved in the European Union as an orphan medicinal product for the prevention of phototoxicity in adult patients with EPP. The product was launched in Europe in 2016. Information on the product can be found on CLINUVEL's website at www.clinuvel.com.
Phase III clinical trials of SCENESSE® have been published in the *New England Journal of Medicine*, see:
Langendonk et al (2015). Afamelanotide for Erythropoietic Protoporphyria. *NEJM*. 373(1):48-59. Online at https://www.nejm.org/doi/10.1056/NEJMoa1411481.

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 and the US Food and Drug Administration in 2019 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, Singapore and the USA. For more information please go to http://www.clinuvel.com.

SCENESSE® is a registered trademark of CLINUVEL PHARMACEUTICALS LTD.

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Forward-Looking Statements
This release contains forward-looking statements, which reflect the current beliefs and expectations of CLINUVEL's management. Statements may involve a number of known and unknown risks that could cause our future results, performance or achievements to differ significantly from those expressed or implied by such forward-looking statements. Important factors that could cause or contribute to such differences include risks relating to: our ability to develop and commercialise pharmaceutical products, including our ability to develop, manufacture, market and sell biopharmaceutical products; competition for our products, especially SCENESSE® (afamelanotide 16mg); our ability to achieve expected safety and efficacy results through our innovative R&D efforts; the effectiveness of our patents and other protections for innovative products, particularly in view of national and regional variations in patent laws; our potential exposure to product liability claims to the extent not covered by insurance; increased government scrutiny in either Australia, the U.S., Europe and Japan of our agreements with third parties and suppliers; our exposure to currency fluctuations and restrictions as well as credit risks; the effects of reforms in healthcare regulation and pharmaceutical pricing and reimbursement; 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; any failures to comply with any government payment system (i.e. Medicare) reporting and payment obligations; uncertainties surrounding the legislative and regulatory pathways for the registration and approval of biotechnology based products; decisions by regulatory authorities regarding approval of our products as well as their decisions regarding label claims; any failure to retain or attract key personnel and managerial talent; the impact of broader change within the pharmaceutical industry and related industries; potential changes to tax liabilities or legislation; environmental risks; and other factors that have been discussed in our 2019 Preliminary Financial Report. Forward-looking statements speak only as of the date on which they are made, and the Company undertakes no obligation, outside of those required under applicable laws or relevant listing rules of the Australian Securities Exchange, to update or revise any forward-looking statement, whether as a result of new information, future events or otherwise. More information on the forecasts and estimates is available on request. Past performance is not an indicator of future performance.

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