CLINUVEL

Company Announcement

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

Australian TGA approves SCENESSE®

CLINUVEL's drug authorised for adults with the metabolic disorder erythropoietic protoporphyria (EPP) in Australia

Executive summary

- SCENESSE® approved by Australian TGA under Priority Review
- First approved therapy for erythropoietic protoporphyria (EPP) in Australia
- SCENESSE® indicated for the prevention of phototoxicity in adult EPP patients, to be administered every two months
- Follow up of patients as part of CLINUVEL's commitment

Melbourne, Australia, 27 October 2020

CLINUVEL PHARMACEUTICALS LTD today announced that the Australian Therapeutic Goods Administration (TGA) has approved the registration of its drug SCENESSE® (afamelanotide) for the prevention of phototoxicity in adult patients with erythropoietic protoporphyria (EPP).[1] SCENESSE® is the first treatment approved for EPP patients in Australia.

TGA approval for registration

The TGA evaluated the SCENESSE® dossier under Section 25 of the Therapeutic Goods Act (1989) and notified CLINUVEL in line with Section 25(3) of its decision to approve the drug's registration on the Australian Register of Therapeutic Goods (ARTG). The notification completes a nine-month review by the TGA following validation of the SCENESSE® dossier.

SCENESSE® will be registered in Australia for the indication "*the prevention of phototoxicity in adult patients with EPP".* The drug will be available as a prescription medication in Australia, to be administered by trained and accredited healthcare professionals every two months. CLINUVEL will implement a comprehensive training and accreditation program, ensuring that healthcare professionals are provided with information in line with the approval.

As an innovative chemical entity, SCENESSE® will be subject to additional safety monitoring, as well as a formal risk management plan and regular pharmacovigilance (drug safety) reporting to the TGA. This is in line with the drug's European and US marketing authorisations, received in 2014 and 2019 respectively, and allows for long-term follow up of patient safety.

Following inclusion of SCENESSE® on the ARTG, CLINUVEL is legally allowed to supply the product within Australia. CLINUVEL is engaging with Pharmaceutical Benefits Advisory Committee to make the drug available on the Pharmaceutical Benefits Scheme in Australia.

SCENESSE® was granted an orphan drug designation in 2010, recognising the potential of the drug to treat a rare (affecting fewer than 2,000 individuals in Australia) metabolic disease. This designation – a regulatory mechanism

to encourage development of medicinal products for patients with severe and neglected diseases – entitled CLINUVEL to a waiver of registration fees as well as review under the TGA's priority registration pathway.

First systemic photoprotective drug for erythropoietic protoporphyria (EPP)
SCENESSE® is the first approved treatment for EPP patients in Australia and the only drug to have been evaluated for safety and effectiveness in randomised placebo-controlled clinical trials in this disease anywhere in the world.

EPP is a poorly characterised rare metabolic disorder causing lifelong absolute light intolerance. Due to a genetic defect, EPP patients suffer debilitating acute phototoxic reactions (anaphylactoid reactions and second-degree burns) after just a few minutes of exposure to visible light (including sun and artificial light). Burns and reactions may last days to weeks. Without treatment patients must withdraw from light exposure to prevent phototoxicity, leading to lifelong social isolation.

SCENESSE® has been shown to reduce the incidence and severity of phototoxic reactions and increase the amount of time patients can expose to light without incurring phototoxicity. The drug is administered as a controlled-release subcutaneous injectable implant in an outpatient setting. Each SCENESSE® implant contains 16mg of the active ingredient afamelanotide which is released into the body over a period of approximately 10 days. Afamelanotide – an analogue of the naturally occurring hormone alpha-melanocyte stimulating hormone – binds to melanocortin-1 receptors on cells and provides total body protection from light and UV (systemic photoprotection) to EPP patients. Over 10,000 doses of afamelanotide have been administered to over 1,400 individuals worldwide, with a positive safety profile maintained to date.

Commentary
"It is deeply satisfying to share with all involved that SCENESSE® – an innovative, Australian-developed drug – has been approved to treat Australian EPP patients," CLINUVEL's Chief Scientific Officer, Dr Dennis Wright said. "Our team is particularly grateful to individuals and families within the Australian EPP patient community who have supported our development program over the past 15 years as we work towards enabling treatment access.

"The TGA is the third global regulatory agency to evaluate and approve SCENESSE® for EPP. Our stepwise approach to regulatory engagement continues to be validated by today's news while we progress discussions in other regions where EPP patients lack access to treatment," Dr Wright said.

"Our consistency, persistence in the dialogue with the TGA over more than 10 years has led to this landmark decision," CLINUVEL's Regulatory Affairs Manager, Dr Monique Baldwin said. "Our full attention now turns to actively progressing discussions with the Pharmaceutical Benefits Advisory Committee to have SCENESSE® included in the Pharmaceutical Benefits List and enabling access for Australian EPP patients."

- End -

[1] SCENESSE® (afamelanotide 16mg) is approved in the European Union as an orphan medicinal product for the prevention of phototoxicity in adult patients with erythropoietic protoporphyria (EPP). SCENESSE® is approved in the USA to increase "pain- free" light exposure in adult EPP patients with a history of phototoxicity. Information on the product can be found on CLINUVEL's website at www.clinuvel.com.

Authorised for ASX release by the Board of Directors of CLINUVEL PHARMACEUTICALS LTD

About CLINUVEL PHARMACEUTICALS LIMITED
CLINUVEL PHARMACEUTICALS LTD (ASX: CUV; NASDAQ INTERNATIONAL DESIGNATION ADR: CLVLY; XETRA-DAX: UR9) is a global and diversified biopharmaceutical company focused on developing and commercialising treatments for patients with genetic, metabolic, and life-threatening disorders, as well as healthcare solutions for the general population. 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 systemic photoprotection, DNA repair and acute or life-threatening conditions. 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.

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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, the COVID-19 pandemic affecting the supply chain for a protracted period of time, 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, China 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 2020 Annual 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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