Media release

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

First patient dosed in SCENESSE® DNA Repair Program

Xeroderma pigmentosum (XP) patient receives SCENESSE® treatment

Note to editors: CLINUVEL is releasing in-depth infographics and a video explaining the mechanisms of action in XP and the overall DNA repair process. For updates, follow:
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Melbourne, Australia, 15 September 2020

In the search for a preventative treatment of skin cancers, including melanoma, it is imperative to understand and treat DNA damage caused by ultraviolet (UV) radiation. Following the treatment of a patient suffering from xeroderma pigmentosum (XP), a disease characterised by an inborn insufficiency to repair DNA damaged by sun exposure, Australian based CLINUVEL PHARMACEUTICALS is the first company worldwide to use a systemic therapy to repair DNA.

CLINUVEL's drug SCENESSE® (afamelanotide 16mg) belongs to a group of hormones which have been shown to reduce UV-induced damage to DNA (photoproducts) and assist in DNA regeneration.[1] The Company is running a staged clinical program to confirm the ability of SCENESSE® to repair the DNA in skin cells, focusing initially on XP.

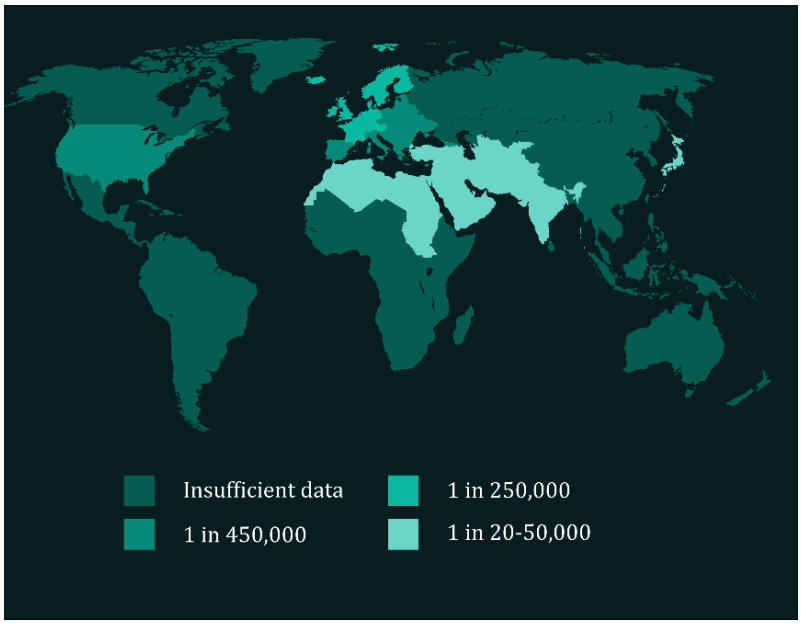

Figure 1 The worldwide incidence of xeroderma pigmentosum

SCENESSE® IN XERODERMA PIGMENTOSUM

XP is a genetic disease which has served as a human model for studying the insufficiency of human DNA repair. Patients develop frequent skin cancers from an early age – most experience their first malignancy before adolescence – and must avoid all forms of UV exposure. The disease has a high mortality rate, with a median life expectancy of thirty years. XP treatment is limited to management of symptoms, in particular regular surgery to remove cancerous lesions. An estimated 1 in 450,000 individuals in Europe suffer from XP.

Today CLINUVEL announced that SCENESSE® has been administered for the first time to a patient diagnosed with XP under a Special Access Program, whereby the patient's safety will be evaluated over six weeks of treatment. Following confirmation of safety of the drug product in this patient, CLINUVEL will conduct two further studies as part of the DNA Repair Program. Both studies – an open-label Phase II study involving six XP-C patients (CUV150) and a control study enrolling 10 healthy volunteers (CUV151) – will evaluate the impact of treatment with SCENESSE® on DNA damage and restoration.

Commentary

"We seek to provide meaningful benefit to XP patients, and these results will serve a wider population of fair-skinned individuals at risk of developing skin cancers," CLINUVEL's Clinical Operations Manager, Dr Pilar Bilbao

said. “The next 12 months will be exciting for many patients, their families, the clinical experts and our own teams.”

CLINUVEL is developing a range of products based on its very targeted research with SCENESSE®, including topical formulations of its proprietary drugs and over-the-counter products, and has recently opened a new Research and Development Centre in Singapore to accelerate this work. First results from the use of SCENESSE® in XP patients are expected in 2021. The scientific data from SCENESSE® has led to further products for general use.

Program	Objectives
Special Access Program 1 XP-C patient	Safety Clinical observations – expert clinician
CUV150 (Phase IIa pilot) 6 XP-C patients	Safety DNA repair – biopsies and assays to evaluate photoproducts and repair mechanisms Disease severity and quality of life assessments
CUV151 (Phase IIa) 10 healthy volunteers	DNA repair – biopsies and assays to evaluate photoproducts and repair mechanisms

Figure 2: SCENESSE® DNA Repair Program – studies 2020/21

- 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 biopharmaceutical company focused on developing and delivering treatments for patients with a range of severe genetic, skin, and systemic disorders. As pioneers in photomedicine and understanding the interaction of light and human biology, CLINUVEL’s research and development initially 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. CLINUVEL is advancing its portfolio of melanocortins, among which is PRÉNUMBRA® for the treatment of several critical disorders. Headquartered in Melbourne, Australia, CLINUVEL has operations in Europe, Singapore, and the USA. For more information please go to http://www.clinuvel.com.

Media enquiries
Monsoon Communications
Mr Rudi Michelson, 61 411 402 737, rudim@monsoon.com.au
Level 39, 55 Collins Street, Melbourne, Victoria, Australia 3000

Head of Investor Relations
Mr Malcolm Bull, CLINUVEL PHARMACEUTICALS LTD

Investor enquiries
https://www.clinuvel.com/investors/contact-us

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 2019 Annual Report and 2020 Preliminary Final 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.

www.clinuvel.com
Level 11 T +61 3 9660 4900
535 Bourke Street F +61 3 9660 4999
Melbourne
Victoria, Australia, 3000