SCENESSE® receives US FDA orphan designation for cutaneous porphyrias

Afamelanotide recognised as potential treatment for rare metabolic disorders

Melbourne, Australia and Leatherhead, UK, February 12 2016

Clinuvel Pharmaceuticals Limited (**ASX: CUV; XETRA-DAX: UR9; ADR: CLVLY**) today announced that its lead drug SCENESSE® (afamelanotide 16mg) has received an additional orphan drug designation (ODD) from the US Food and Drug Administration (FDA) for the treatment of cutaneous variants of porphyria. The ODD recognises the potential of afamelanotide to treat or prevent symptoms in rare forms of porphyria and offers incentives to Clinuvel to develop the drug for these patients.

SCENESSE® (AFAMELANOTIDE 16MG) AND CUTANEOUS PORPHYRIAS

SCENESSE® has been clinically evaluated as a photoprotective drug in one severe form of porphyria – erythropoietic protoporphyria or EPP – for which it has received marketing authorisation in Europe.[1] It is proposed that SCENESSE® may also have a photoprotective effect for patients with other rare forms of cutaneous porphyria: variegate porphyria (VP), hereditary coproporphyria (HCP) and congenital erythropoietic porphyria (CEP).

Porphyrias are a family of seven genetic metabolic disorders which cause malfunctions in the haem biosynthetic pathway. While classically grouped together, each of the five cutaneous porphyrias has clinically distinct symptoms, generally characterised as acute dermal reactions – affecting the skin and anaphylactoid in nature – which are caused by the accumulation and storage of phototoxic molecules (porphyrins) in the body.

When patients with cutaneous porphyria expose their skin to *visible light* – both sunlight and certain artificial lights – the porphyrins react and cause symptoms such as oedema and incapacitating deep burns leading to damage of soft tissue and eventually scarring. Patients are conditioned from childhood to recognise the source of their symptoms and withdraw from light and sun exposure to prevent phototoxicity, resulting in light starvation and social isolation.

SCENESSE® has previously been granted orphan designation by the FDA for EPP and CEP. While no further trials are currently planned in cutaneous porphyrias, Clinuvel is working to make SCENESSE® available to several patients with CEP on a name-patient basis due to the extreme severity and progressive character of this disorder. Only a few hundred cases of CEP have ever been recorded in the literature.

US ORPHAN DRUG DESIGNATION (ODD)

Orphan-drug designation is granted by the FDA's Office of Orphan Products Development to drugs which have the potential to diagnose or treat rare conditions, defined as those affecting less than 200,000 individuals in the United States. The additional orphan designation entitles Clinuvel to technical assistance throughout the development process for cutaneous porphyrias, potential fee reductions and tax credits, and seven years' market exclusivity if approved for marketing by the FDA.

"Beyond EPP we have always recognised that patients with other forms of cutaneous porphyria are deeply affected by their condition," Clinuvel's Acting Chief Scientific Officer, Dr Dennis Wright said. "The FDA's designation acknowledges the potential of afamelanotide to assist those patients."

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[1] SCENESSE® (afamelanotide 16mg) is approved as an orphan medicinal product in Europe 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.

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About Clinuvel Pharmaceuticals Limited
Clinuvel Pharmaceuticals Ltd (ASX: CUV; XETRA-DAX: UR9; ADR: CLVLY) is a global biopharmaceutical company focused on developing drugs for the treatment of a range of severe disorders. With its unique expertise in understanding the interaction of light and human skin, the company has identified patient populations with a clinical need for photoprotection and for repigmentation. The worldwide prevalence of these patient groups range from 5,000 to 45 million. Clinuvel's lead compound, SCENESSE® (afamelanotide 16mg), was approved by the European Commission in 2014 for the prevention of phototoxicity in adult patients with erythropoietic protoporphyria (EPP).
Headquartered in Melbourne, Australia, Clinuvel has operations in Europe, Switzerland, the US and Singapore.
For more information go to http://www.clinuvel.com.

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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