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Event – Scenesse waiting game could finally be over

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This year Clinuvel Pharmaceuticals should see validation of its lead pipeline drug, Scenesse, in erythropoietic protoporphyria, a rare light intolerance disorder. European approval is looming and readout from a confirmatory US trial is expected in the coming months.

However, the bigger money-spinner could be its use in the much larger indication of vitiligo, a loss of skin pigmentation. Confidence is already growing, with Scenesse producing positive results in a pilot study at the end of last year. A significant revenue stream would be welcomed by the Australian company, which has just $13m in cash following the long wait to approval, and its shares are in desperate need of a boost.

Company	Clinuvel Pharmaceuticals
Product	Scenesse
Market cap	$73m
Product NPV	$174m
% of market cap	238%
Event type	Phase III US results and European approval
Date	H1 2013

Into the light

Erythropoietic protoporphyria (EPP) is a metabolic disorder caused by a build-up in the skin of protoporphyrin IX, which reacts with sunlight, causing burning and blistering of the skin. Clinuvel claims that between 5,000-10,000 people worldwide suffer from the condition.

There are no specifically approved treatments and phototoxicity can only be avoided by staying out of sunlight. Back in 2008 both the EMA and FDA granted Scenesse orphan drug designation in EPP.

Scenesse is known generically as afamelanotide, and is an analogue of alpha-melanocyte stimulating hormone (a-MSH), which activates eumelanin, a dark pigment known to have photoprotective properties, in the skin. It is administered subcutaneously as a dissolvable implant and is slowly released over 10-15 days.

Encouraging data

In previous European trials Scenesse produced positive results, significantly reducing the severity and number of phototoxic reactions in EPP. The company filed for European registration in February last year, and approval is finally expected in the coming months (*Outlook brightening for Clinuvel, February 13, 2012*). Meanwhile, Scenesse is available on a compassionate basis in certain European countries.

But there has been little explanation as to why European regulators have not yet approved the drug, an event many had expected by the middle of last year. Shares in the group have drifted too, bobbing along between A$1.50 and A$2.00.

More transparent progress has been made in the US, with a confirmatory phase III US trial (CUV039) in 100 EPP patients starting last May. Clinuvel says data collection and preparation for the closure of trial centres has started, and depending on the results it will then discuss regulatory approval with the FDA.

Bigger indication

While European approval in EPP cannot come soon enough for the cash-strapped Clinuvel, the real prize will be approval in vitiligo, a condition in which light patches of skin appear on different parts of the body owing to the

loss of melanin.

Towards the end of last year Clinuvel released positive phase IIa pilot study results in 41 vitiligo patients. Scenesse was tested as an adjunct to the current treatment option, narrowband ultraviolet B light therapy (NB-UVB), and significantly improved repigmentation of lesions compared with NB-UVB monotherapy.

Nomura analysts estimate that the vitiligo affects 45 million people worldwide and that the market, currently made up of generic treatments such as topical creams, and UVB, is worth $1.4bn per year.

Brightening skies

If successful Scenesse would be the only branded treatment in vitiligo. Nomura expects approval in 2016. A phase IIb vitiligo trial is due to start later this year.

With a number of catalysts on the horizon, including potential European approval and confirmatory US results, the way could be paved for increased investor confidence in Scenesse. Validation and progress of the lead compound has been a long time coming.

Study name	Details	Trial ID
CUV039	US Phase III confirmatory study in EPP	NCT01605136
CUV102	US Phase II Scenesse and NB-UVB light vitiligo study	NCT01430195

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