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The FDA has taken the first step in approving VEGF-D diagnostics as a test to monitor LAM

The Australian based company Circadian Technologies Limited (ASX:CIR), the owner of an extensive patent portfolio covering VEGF-D and VEGF-D diagnostics, announced today that the FDA had granted its VEGF-D diagnostic test Humanitarian Use Device status for "the detection of circulating VEGF-D intended to monitor patients who have been diagnosed with lymphangioleiomyomatosis (LAM) for disease progression and response to therapeutic intervention."

Similar in concept to the FDA's designation of orphan drug status to therapeutic drugs, a HUD is defined by the FDA as a medical device intended to benefit patients in the treatment or diagnosis of a disease or condition that affects or is manifested in fewer than 4,000 individuals in the United States per year. HUD designation enables formal approval of a HUD using a regulatory approval process known as a Humanitarian Device Exemption (HDE).

The HDE process greatly accelerates marketing approval in the United States compared to conventional routes (PMA and 510(k)). Circadian is currently completing the development of kits to be manufactured under current Good Manufacturing Practice (cGMP) and plans to submit its HDE application before year end and hopes to have formal FDA approval in the first half of 2014.

Vascular endothelial growth factor-D (VEGF-D) is a protein that is involved in lymphatic vessel formation and growth and tumor metastasis. Most LAM patients have elevated serum (blood) levels of VEGF-D, and serum VEGF-D levels can help diagnose LAM without a lung biopsy in some cases. In a recent study[1], using data from the MILES Trial, the MILES team reported that high vascular endothelial growth factor D (VEGF-D) levels predict response to treatment with sirolimus.

Robert Klupacs, CEO of Circadian said "We are delighted to have had the opportunity to collaborate with The LAM Foundation as part of the ongoing process for FDA approval of our VEGF-D kit. We would specifically like to acknowledge the help of their members with the provision of data required for the submission, supplying samples for validation, assisting in conference calls with the FDA and numerous documentation reviews."

He added, "LAM is an incredibly debilitating condition with limited treatment options. Recent studies have shown that VEGF-D is a useful biomarker for LAM disease. We are proud that we have been able to develop the VEGF-D kit, which we believe can assist in improving the quality of life for LAM patients."

Laura Lentz, Board Chair of The LAM Foundation, said "This is an important first step toward making VEGF-D testing more accessible for all LAM patients. We are optimistic that use of the test will help physicians make better informed treatment decisions. We are delighted that Circadian is on the path to FDA approval."

VEGF-D testing is currently available through CLIA waiver through the clinical pathology lab at Cincinnati Children's Hospital Medical Center. The lab website link is:
https://research.cchmc.org/translationalcores/ttdsl

In addition to the FDA registration process, Circadian is also undertaking a similar registration path in the European Union and hopes to have European CE Mark approval for the test also by the first half of 2014.

[1] Young LR, Lee H-S, et al., Performance of serum VEGF-D as a biomarker of Lymphangioleiomyomatosis severity and treatment response: A prospective analysis from the MILES Trial. Lancet Respir. Med. 2013; doi:10.1016/S0140-6736(08)61345-8

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