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ASIA EDITION

FDA grants orphan drug status to ASLAN's treatment for AML

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Singapore - ASLAN Pharmaceuticals, a clinical-stage biopharmaceutical company targeting cancers that are both highly prevalent in Asia and orphan indications in the United States and Europe, announced that the US Food and Drug Administration (FDA) has granted ASLAN003 Orphan Drug Designation (ODD) as a treatment for acute myeloid leukaemia (AML).

ASLAN003 is an orally active, potent inhibitor of human dihydroorotate dehydrogenase (DHODH) that has the potential to be first-in-class in AML. AML is a cancer of the myeloid line of blood cells, characterised primarily by the rapid growth of abnormal white blood cells that build up in the bone marrow and interfere with the production of normal blood cells.

The US FDA grants orphan designation to drugs intended to treat a rare disease or condition that affects fewer than 200,000 individuals in the United States. AML patients that have failed on standard of care chemotherapy in AML or do not respond to chemotherapy are termed relapsed/refractory and represent the majority of the total AML population. In 2016, the annual incidence of relapsed/refractory patients was approximately 13,000 patients in the United States. ODD status can provide ASLAN certain development and commercial incentives including a seven-year period of market exclusivity in the US after product approval, FDA assistance in clinical trial design and an exemption from FDA user fees.

ASLAN is currently conducting a phase 2 clinical trial in Asia to develop ASLAN003 in AML and expects to report interim data in the second half of 2018. In previous clinical studies, ASLAN003 has demonstrated potent inhibition of DHODH (up to two orders of magnitude stronger than first generation DHODH inhibitors), lack of toxicities associated with first generation inhibitors and other novel AML therapies, and the potential to induce differentiation in blast cells and applicability in a broad range of AML patients.