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FDA grants Orphan Drug Designation to ASLAN003 for the treatment of acute myeloid leukemia

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On 20 August 2018, the US Food and Drug Administration (FDA) granted Orphan Drug Designation to ASLAN003, a potent inhibitor of human dihydroorotate dehydrogenase (DHODH), for the treatment of patients with acute myeloid leukemia (AML).[1]

DHODH is an essential enzyme of *de novo* pyrimidine biosynthesis of DNA and RNA. David Skyes *et al.* have shown in a study that the inhibition of DHODH enables myeloid differentiation in human and mouse AML models.[2] ASLAN003 is an orally active, potent inhibitor of DHODH, which have been shown to have the ability to differentiate AML blast cells into granulocytes in a variety of AML cell lines that do not respond to ATRA.

A phase IIa dose optimization study (NCT03451084), assessing the safety and efficacy of ASLAN003 as monotherapy in patients with AML is currently ongoing. The primary objective of the study is overall complete remission. The secondary objectives are the incidence of complete remission, relapse-free survival, clinical benefit rate and tumor percentage change.

References

1. Nasdaq: ASLAN Pharmaceuticals Granted Orphan Drug Designation by the FDA for ASLAN003 for the Treatment of Acute Myeloid Leukaemia. 2018 Aug 20.https://www.nasdaq.com/press-release/aslan-pharmaceuticals-granted-orphan-drug-designation-by-the-fda-for-aslan003-for-the-treatment-of-20180820-00056 [Accessed 2018 Aug 20].
2. Skyes D. *et al.* Inhibition of Dihydroorotate Dehydrogenase Overcomes Differentiation Blockade in Acute Myeloid Leukemia. Cell. 2016 Sep 22;167(1):171-186.e15. DOI: 10.1016/j.cell.2016.08.057. Epub 2016 Sep 15.