Novel microRNA inhibitor combines with MDM2 inhibition for AML

THE PROBLEM

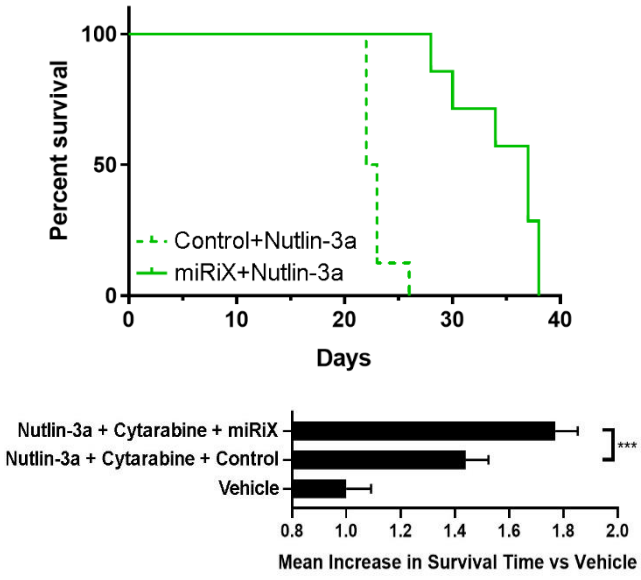
Acute myeloid leukaemia (AML) is a lethal malignancy with an overall 5-year survival rate of just 25%. Cytarabine-based chemotherapy has been the standard-of-care for AML for decades despite poor outcomes, and as such new therapeutics are urgently required. Pharmacological inhibition of MDM2/4 has shown promise in this role, with a number of organisations currently in phase I-III clinical trials with their lead candidates. Initial data has shown that combining this approach with cytarabine can lead to durable responses, however only in an as yet undefined subset of patients. Further increasing the efficacy of this therapeutic approach using an AML-specific sensitizing compound would significantly expand the general applicability of this approach.

THE TECHNOLOGY

Our compound, miRiX, is a novel microRNA inhibitor which modulates the MDM2/4 pathway, thereby increasing the anti-leukaemic activity of MDM2/4 inhibitors such as Nutlin derivatives. miRiX is effective *in vivo* as a combination therapy, expected to synergize with any MDM2/4 inhibitor together with the current gold standard chemotherapy cytarabine, resulting in improved treatment efficacy and patient outcomes.

DEVELOPMENT STAGE

In vivo efficacy has been demonstrated in an aggressive AML mouse xenograft model. A combination of MiriX, MDM2 inhibitor (Nutlin-3a), with or without cytarabine, significantly increased mean survival time compared to Nutlin-3a and/or cytarabine alone. Further *in vivo* validation testing is currently underway.



SCIENTIFIC PUBLICATIONS

Vu T, Wang K, Stoelzel F, Ehninger G, Molloy TJ, Ma DDF. *miR-10a as a therapeutic target and predictive biomarker for MDM2 inhibition in acute myeloid leukemia*. Leukemia. (link)

IP POSITION

This technology is the subject of an Australian provisional patent application and developed by Prof David Ma, and Dr Timothy Molloy of the St Vincent's Centre for Applied Medical Research.

COMMERCIAL OPPORTUNITY

This is an opportunity to acquire a novel microRNA inhibitor, designed to work in combination with cytarabine chemotherapy and MDM2/4 inhibitors. This combination therapy could provide significant decreases in the patient mortality rates associated with AML.

We are currently seeking industry partners with the resources and capabilities for the rapid co-development and licensing of our therapeutic.

CONTACT

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