Research We Fund

Project:
Development of new targeted therapies for acute myeloid leukaemia

Research team:
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Institution: Peter MacCallum Cancer Centre

Cancer type: Acute myeloid leukaemia

Years funded: 2019–2021

What is the project?

New therapies for acute myeloid leukaemia (AML) represent an area of urgent unmet clinical need. Mutations in a gene known as DNMT3A is common in AML and offers a promising opportunity for the development of new targeted drugs that specifically kill leukemic cells. We want to use advanced genetic and pharmacological tools to identify and validate new treatments for AML patients with a DNMT3A mutation.

What is the need?

AML is a low survival cancer with a five-year overall survival rate of less than 30%. It is also the most common acute leukaemia in adults with over one thousand new cases in Australia annually. Standard treatment for AML often involves high dose chemotherapy with significant side effects and options are limited for patients who do not respond or relapse. Mutations in the DNMT3A gene occur in around 25% of AML patients and are associated with poor prognosis. By understanding key cellular pathways that are affected by the mutations, we believe it’s possible to identify druggable targets that can be exploited to specifically eliminate leukemic cells.

What are you trying to achieve?

Our aim is to identify a new drug target that can be tested and then used in clinical trials aimed at achieving the complete cure of aggressive AML.

Project timeline

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<th>Timeline</th>
<th>2019</th>
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<td>Development and preliminary analysis of an AML model and a primary genome-wide screen.</td>
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<td>Validation of key oncogenic dependencies in mutant models and validation of selected hits from the screen</td>
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