Potential New Treatments For Leukemia

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Asian Scientist (Dec. 10, 2013) - Two international research teams led by scientists in Singapore have identified potential new treatments for a particular subtype of acute myeloid leukemia (AML).

One study identified the *Sox4* gene as a potential therapeutic target in certain patients with acute myeloid leukemia (AML) while the other study found that a class of anti-cancer drugs, histone deacetylase (HDAC) inhibitors, may be potential candidates for the treatment of certain types of AML.

AML is a group of heterogeneous diseases with considerable diversity in terms of genetic abnormalities. Both teams of researchers studied a type of AML that harbored mutations in the *CEBPA* tumor suppressor gene or had characteristics associated with altered *CEBPA* function.

The first study, published in *Cancer Cell*, found that *Sox4* is a molecular target for *CEBPA* mutations and demonstrated that targeting *Sox4* may be an effective strategy for treating AML patients harboring *CEBPA* mutations.

Although *CEBPA* mutations have been studied for decades, how the promote tumor-formation and the role of their downstream targets are still poorly understood. This is the first study that identified a molecular target downstream of *CEBPA* mutations.

The second study, published in *Haematologica*, examined the blood samples of more than 500 patients who were newly diagnosed with AML and found that a group of genes, known as the *CEBPA* signature, was not properly expressed in 20 per cent of the blood samples.

The scientists further demonstrated that drugs known as HDAC inhibitors were able to reactivate expression of these genes and as such, could potentially be used as drugs in the treatment of AML characterized by this signature.

The discoveries suggest potential strategies for the treatment of such leukemia and is a significant step forward as there are currently limited therapeutic options for this subtype of AML.

> "Up to now, therapeutic options for AML are very limited. By understanding oncogenic pathways and the signatures which respond to specific enzyme inhibitors, we can build up our knowledge and understanding towards the development of more efficient drugs," said Professor Daniel Tenen, who is a senior author of both studies.

The articles can be found at:

Zhang H et al. (2013) Sox4 Is a Key Oncogenic Target in C/EBP? Mutant Acute Myeloid Leukemia.

Liss A et al. (2013) The Gene Signature In CCAAT-Enhancer-Binding Protein ? Dysfunctional Acute Myeloid Leukemia Predicts Responsiveness To Histone Deacetylase Inhibitors.

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