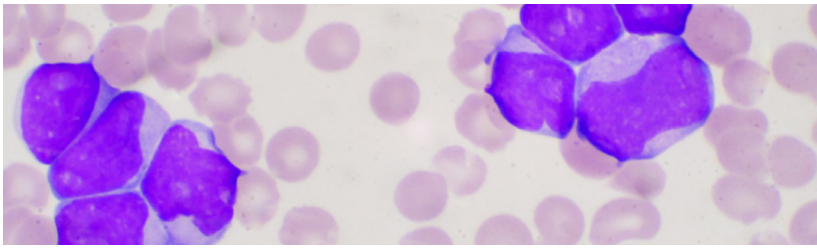
Identifying Potential Treatments for Leukemias and Other Cancers

INVESTIGATING ABNORMAL GENE ACTIVATION

Abnormal activation of SET binding protein 1 (SETBP1) gene has been found to occur frequently in a variety of myeloid neoplasms, including myeloid leukemias, and other solid tumor cancers. Activation of this gene may play a role in disease initiation or progression and is associated with poor prognosis, driving an urgent need to develop effective targeted therapies for patients.

A PROMISING DISCOVERY FOR TARGETED THERAPY

USU and HJF have identified compounds to inhibit the activity of SETBP1. Blocking the interaction between SETbp1 and XPO1 may represent a promising therapeutic strategy for treating myeloid neoplasms. Researchers have identified small molecules that could be promising therapeutic intervention strategies and may be effective in treating cancers associated with SETbp1 overexpression or missense mutations.



APPLICATIONS

USU and HJF researchers have identified compounds that target a biomarker associated with poor prognosis and survival and may be effective for treating a variety of human myeloid neoplasms and solid tumors:

- Primary acute, secondary, chronic, or atypical chronic myeloid leukemias
- Chronic and juvenile myelomonocytic leukemia
- Chronic myeloid leukemia blast crisis
- Myelodysplastic syndrome
- Chronic neutrophilic leukemia
- Targeted therapy for cancers with mutated or over-expressed SETBP1, which is associated with poor prognosis.

SOLUTION ADVANTAGES

Further, development of these small molecule leads should lead to highly targeted therapies. The currently identified compounds tested in preclinical studies show promising results, including:

- **Reduced transcription:** Targeted therapy reduces levels of SETbp1 expression at 12 hours post-treatment.
- **Colony inhibition:** Treatment with small molecule inhibitors reduced cell survival and levels of the SETbp1 overexpressed from a variety of solid tumor types.
- **Prolonged survival:** Small animal studies demonstrated that this targeted therapy prolonged survival and decreased the spleen size in mice with leukemia induced by SETbp1 missense mutation.

DEVELOPMENT STATUS

Preclinical proof of concept completed

PATENT STATUS

Patent pending

LICENSING OPPORTUNITIES

HJF is seeking development partners for this technology.

CONTACT INFORMATION

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TRACK CODE

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