(Case SD2021-201): Mechanism of action of a splicing modulator compound

Tech ID: 32260

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FULL DESCRIPTION

Researchers from UC San Diego have developed 17S-FD-895, a small molecule compound targeting SF3B1, which modulates mRNA splicing. To date, they have evaluated the effects of this splicing modulator on both self-renewal as well as pro-survival splice variants in CD34+ cells derived from both peripheral blood as well as bone marrow of pediatric AML patients. Splicing modulation induced MCL1 exon 2 skipping, producing pro-apoptotic MCL1-S transcripts. Hematopoietic progenitor assays demonstrated a dose-dependent reduction in LSC clonogenicity and self-renewal.

As a result of these studies, the researchers have demonstrated LSC splicing patterns in pediatric AML that may inform novel biomarker identification as well as development of 17S-FD-895 for pediatric AML.

7S-FD-895 may be a treatment for patients with adult AML that evolved from MDS or MPNs. Expanded clinical trials in other cancer types could prove that this agent may also have potential therapeutic applications in multiple myeloma and/or pediatric AML. In addition, biomarker studies have shown intron retention and exon skipping events detectable in AML disease-relevant and spliceosome-associated biomarkers after treatment with 17S-FD-895.

OTHER INFORMATION

KEYWORDS

splicing modulator, spliceosome, biomarkers, diagnostics, small molecule, inhibitor, leukemia, myeloma, cancer

CATEGORIZED AS

- **Materials & Chemicals**
  - Biological
- **Medical**
  - Disease: Cancer
  - New Chemical Entities, Drug Leads
  - Therapeutics

STATE OF DEVELOPMENT

Technology is patent-pending and available for licensing for commercial uses.

RELATED MATERIALS

- Van der Werf, I., Mondala, P., Diep, R., Balaian, L., Mason, C., Kaspers, G., ... & Fisch, K. (2020). Selective targeting of splicing deregulation in pediatric acute myeloid leukemia stem cells. - 05/15/2020

OTHER INFORMATION

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