

Small molecule for treating multiple myeloma and increasing blood cell production

Knowing changes everything.™

SUNY Upstate Medical University is actively seeking partners interested in commercializing a class of small molecule compounds showing promise for treating various cancers, including multiple myeloma, and for increasing blood cell production after chemo or radiation therapy.

Treating multiple myeloma

This year over 20,000 new cases of multiple myeloma will be diagnosed, and an estimated 10,000 deaths will occur from this debilitating disease. While treatment of multiple myeloma has improved, there is still no cure, and the 70,000 people living with or in remission from multiple myeloma still have only a 35% chance of surviving five years.

Experiments at Upstate have shown that inhibiting the SHIP1 gene could be useful for treating multiple myeloma, and the researchers have found several small molecule SHIP1 inhibitors with therapeutic potential. These SHIP1 inhibitors have been shown to kill multiple myeloma and myeloid leukemia cells *in vitro*, and *in vivo* studies show that SHIP1 inhibitors provide a significant survival benefit out to four months past an initial challenge with multiple myeloma. Critically, multiple myeloma has been classified as an orphan drug in the US, potentially smoothing the path to regulatory approval for any drug treating it.

Increasing blood cell production

Chemo or radiation therapy for cancer is harsh, and patients undergoing either often suffer from myelosuppression (severe reductions in blood cell production). It is essential to the patient's recovery to restore all of his or her blood cell types to normal levels as soon as possible after chemo or radiation therapy. Unfortunately, with the exception of thrombopoietin, which experienced adverse events when tested clinically, no current drugs act to improve platelet production. This is a serious problem as an inadequate level of platelets frequently threatens the health of myelosuppressed patients.

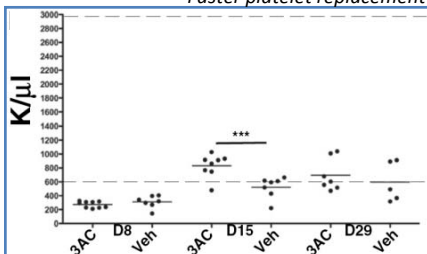
The SHIP1 inhibiting compounds under development have usefulness in enhancing the production of all blood cell types, potentially providing an easy-to-store and administer small molecule alternative to the current protein-based blood cell production enhancing drugs. *In vivo* data shows that Upstate's SHIP1 inhibitor is effective at increasing the speed of recovery of all blood cell types including platelets after the myeloablative damage that frequently occurs in cancer therapies.

Benefits

- Small molecule (easy to store and administer [orally], cheaper to produce)
- Targets novel pathway, SHIP1
- Interferes with cancer cell survival
- Increases production of all blood cell types, including platelets

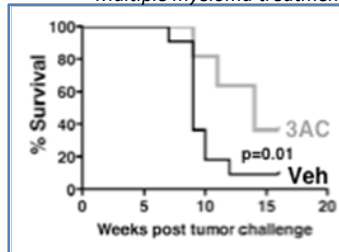
In vivo results

Faster platelet replacement



Increased recovery of platelets to the normal range (indicated by dotted lines) in mice administered Upstate's SHIP1 inhibitor 3AC

Multiple myeloma treatment



16 wks after challenge with multiple myeloma there is a significant survival benefit to mice administered 3AC

Tech ID: 1720-110

Inventors:

William Kerr- Professor,
Dept. of Microbiology and
Immunology; Upstate
Medical University

John Chisholm- Professor,
Dept. of Chemistry;
Syracuse University

Patent pending:
PCT /US2011/031930

Contact:

Scott Macfarlane
Tel. 315-464-7613
macfarls@upstate.edu