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With help from Mother Nature: a novel new drug discovery may revolutionize treatment of inflammatory diseases and leukemia

If you found yourself on a run-away train, would you want to switch on the brakes? Two Vancouver Coastal Health Research Institute (VCHRI) and University of British Columbia investigators have found a way to turn on the brakes of a cell, and thus halt abnormal blood-cell growth in a range of inflammatory and autoimmune disorders and blood cancers.

The discovery was made by Drs. Alice Mui and Christopher Ong, two assistant professors in the Dept of Surgery at UBC and researchers in the VCHRI Immunity & Infection Research Centre and the Prostate Centre at VGH. It is highlighted in the Sept. 15 edition of *Blood, Journal of the American Society of Hematology*, the highest impact journal for hematology.

The immune system relies on white blood cells (leukocytes) to defend the body against infectious pathogens such as bacteria and viruses. In a healthy body, leukocytes are strictly controlled and turned off when no longer needed. This off-switch is controlled by a protein called “SHIP” – standing for SH2-containing inositol 5’phosphatase. SHIP, which is only present in blood cells, was discovered in 1997 by Dr. Gerald Krystal, a senior scientist at the BC Cancer Research Centre and a professor of Pathology and Laboratory Medicine at UBC. It regulates the PI3 kinase (PI3K) pathway which is essential for cell growth, survival and immune cell activation. Inappropriate or persistent activation of the PI3K pathway can result in serious inflammatory/immune diseases or blood cancers such as multiple myeloma, leukemia and lymphoma.

With the aim of finding new drugs for treatment of blood borne diseases, Drs. Mui and Ong collaborated with Dr. Krystal and Dr. Raymond Andersen, a renowned natural products chemist and professor of Earth and Ocean Sciences at UBC, to search for drugs that could modulate SHIP. The team screened Dr. Andersen’s library of sea sponge extracts for molecule compounds that can turn SHIP on.

“Sea sponges are a rich source of novel bio-active compounds, created by nature, to protect themselves against marine predators, and interestingly many of these compounds possess important medicinal properties,” says Dr. Christopher Ong. “Dr. Andersen’s library has

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already produced other agents with interesting biological properties on mammalian cells, some of which are in clinical development as potential drugs for treatment of human diseases, so we felt it would be an ideal place to look for drugs that activate SHIP.”

Drs. Mui and Ong identified a compound, now known as *AQX-MN100*. It is able to inhibit immune and blood cell activation both in the test tube and in mouse models of human inflammatory disease and lymphoma by activating SHIP. “This is an entirely new paradigm for controlling run-away cells,” says Dr. Alice Mui. “Previous research efforts were aimed at trying to control the cells through blocking stimulation signals. In the run-away train analogy, this would be like taking your foot off the accelerator and the train will eventually stop when it runs out of fuel vs. this new approach of directly applying the brakes. We are also excited because since SHIP is only found in immune/blood cells, side-effects of SHIP-based therapy on other cells of the body are expected to be limited.”

The AQX-MN100 discovery has been validated by proof-of-principal grants from the Canadian Institutes of Health Research (CIHR) aimed at translating basic research findings into clinically applicable therapy. This past spring, in one of the largest early-stage financings in B.C.’s recent biotech history, Vancouver based Aquinox Pharmaceuticals, the exclusive licensee of the SHIP and AQX-MN100 technology, raised 14.5 million US in venture capital for the continued development and first clinical trials of the drug for treatment of blood cancers and immune disorders.

The research team has also received \$250,000 from CIHR to support clinical development, as well as funding from VGH & UBC Hospital Foundation, through the fundraising efforts of the Sangara family of Vancouver, BC, to help determine whether AQX-MN100 is active against multiple myeloma. Over 50,000 people are living with incurable multiple myeloma in North America. New treatments for this disease are greatly needed.

“The work on SHIP promises to lead to great advances in the treatment of blood cancers and disorders of the immune system,” says Dr. Clay Smith, director of the Leukemia/BMT Program of VGH and British Columbia, and professor in the Department of Hematology and Bone Marrow Transplant at UBC. “It is doubly exciting that these advances came from a team of researchers right here in BC and hopefully this research will lead to better treatments not only in BC but throughout the world.”



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VCHRI is the research body of Vancouver Coastal Health Authority. In academic partnership with UBC, the institute advances health research and innovation across B.C., Canada, and beyond. www.vchri.ca

The Faculty of Medicine at UBC provides innovative programs in the health and life sciences, teaching students at the undergraduate, graduate and postgraduate levels, and generates more than \$200 million in research funding each year.

Aquinox Pharmaceuticals Inc., formed in 2004, is a privately held pharmaceutical company developing targeted small molecule therapeutics for the treatment of cancer and inflammatory disease. The Company has a strong intellectual property portfolio and library of pre-clinical drug candidates that target a unique biochemical enzyme called “SHIP” (SH2-containing inositol 5’phosphatase) which regulates the PI3 kinase (PI3K) pathway. The Company is based on the research of its four key scientific founders from the University of British Columbia, VCHRI, and the BC Cancer Agency in Vancouver. www.aqxpharma.com

CIHR is the Government of Canada's agency for health research. CIHR's mission is to create new scientific knowledge and to catalyze its translation into improved health, more effective health services and products, and a strengthened Canadian health care system. Composed of 13 Institutes, CIHR provides leadership and support to close to 10,000 health researchers and trainees across Canada.

VGH & UBC Hospital Foundation and its donors serve as a bridge between the essential health care governments provide and the most advanced health care possible. The Foundation raises critical additional funds on behalf of Vancouver General Hospital, UBC Hospital, G.F. Strong Rehab Centre and the Vancouver Coastal Health Research Institute so that every British Columbian has access to the latest and most sophisticated health care when they need it.