

## UCSD Scientists Receive Prestigious Hartwell Biomedical Research Awards

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Three scientists at the University of California, San Diego School of Medicine are among this year's 12 winners of Hartwell Individual Biomedical Research Awards, honoring researchers whose work advances children's health.

The UC San Diego recipients are Jack Bui, MD, PhD, assistant professor in the Department of Pathology; Pamela Itkin-Ansari, PhD, assistant professor in the Department of Pediatrics; and Adriana Tremoulet, MD, assistant adjunct professor in the Department of Pediatrics. Each will receive a \$100,000 grant annually from the Hartwell Foundation for three years to cover direct research costs.

It is only the second time in the history of the Hartwell awards that a single institution has had three winners in the same year. The Hartwell Foundation also ranks UC San Diego among the top ten centers for biomedical research in the country.

"This year, UC San Diego made an extraordinary effort to nominate a terrific group of researchers for the Hartwell Individual Biomedical Research Award," said Frederick Dombrose, PhD, president of The Hartwell Foundation. "We applaud their diligence in identifying such innovative science with the potential to benefit children."

While Bui, Itkin-Ansari and Tremoulet share a common goal of improving the understanding and treatment of childhood diseases, their particular pursuits are notably diverse. Bui is interested in helping the body's immune system better recognize and kill tumor cells. Itkin-Ansari is looking for ways to improve treatment of Type-1 diabetes. And Tremoulet is focusing on finding biomarkers for Kawasaki disease, a mysterious vascular disorder of unknown cause.

"The Hartwell Foundation has a rigorous evaluation process to select individuals with novel, early stage research projects that are high risk but have a high potential reward for improving children's health," said Gordon Gill, MD, dean for scientific affairs at the School of Medicine. "We are proud that Drs. Bui, Itkin-Ansari and Tremoulet have received this recognition and much-needed support to move their research to a new level."

**Jack Bui** In the United States, cancer is the leading cause of disease-related death in children under the age of 20. Each year, thousands of children are diagnosed with cancer. Despite massive investments of time and resources, the need for new, innovative cancer therapeutics for this population remains acute.

In a paradigm shift, Bui suggests a solution may lie within. The idea is based on spontaneous remission from terminal cancer, cases in which it's believed the body's own immune system attacked and destroy cancerous cells. Although such documented remissions are quite rare, it's believed the phenomenon happens more frequently, typically in individuals whose cancer is cured before they are even diagnosed.

Bui proposes to understand how spontaneous remission of terminal cancer can be duplicated; how the body's innate immune system recognizes developing tumor cells, then isolates and destroys them. His working hypothesis is that certain genes expressed in cancer cell lines can be used to therapeutically to stimulate the immune system to eradicate cancers like sarcoma.

**Pamela Itkin-Ansari** Type-1 diabetes is a disease in which normal pancreatic cells are destroyed by the patient's own immune system causing the loss of insulin production, a hormone essential to regulating carbohydrate and fat metabolism in the body. Children are notably impacted: Of the estimated 850,000 to 1.7 million Americans who have Type 1 diabetes, 125,000 are 19 years and younger. An additional 30,000 Americans develop Type 1 diabetes each year, roughly 13,000 are children.

With collaborators at the Sanford/Burnham Institute for Medical Research, Itkin-Ansari has shown in a mouse model that transplanted pancreatic beta cells can be shielded from the immune system when encapsulated in a synthetic device. In theory, such a device could provide essential protection to transplanted pancreatic beta-cells in overcoming the autoimmune response that characterizes Type 1 diabetes. If successful, her encapsulated cell technique will not require tissue matching and will not have a requirement for immunosuppression, thus transforming the health and quality of life for diabetic children

**Adriana Tremoulet** Kawasaki disease is a rare condition in children that involves inflammation of blood vessels. It is the leading cause of pediatric acquired heart disease in the United States, yet there is no diagnostic test for KD. Current diagnoses are limited to overt clinical signs, such as rash and fever, but these symptoms overlap with other infections and conditions, many of which require no treatment. In up to a third of KD cases, many of the clinical signs are not even present.

Finding single, tell-tale biomarkers for KD has been problematic. Tremoulet proposes to identify a novel panel of biomarkers that will differentiate KD patients from children with benign rash-fever illnesses. If successful, she expects a point of service test for diagnosing KD will be available in every emergency room, pediatric office, and urgent care center in the U.S. within 5-10 years.

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