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Friedmann Named 2015 Japan Prize Winner

Prestigious award honors his role in development of gene therapy

Theodore Friedmann, MD, professor in the Department of Pediatrics at University of California, San Diego School of Medicine was named today one of three recipients of the 2015 Japan Prize, a prestigious international award honoring laureates whose "original and outstanding achievements in science and technology have advanced the frontiers of knowledge and served the cause of peace and prosperity for mankind."

Theodore Friedmann, MD

Friedmann is being recognized for his pioneering research and contributions to the development of gene therapy, a new field of medicine which in significant ways originated at UC San Diego. The sponsoring Japan Prize Foundation describes Friedmann as "the father of gene therapy."

Sharing the 2015 Japan Prize "in the field of medical science and medicinal science" with Friedman is Alain Fischer, MD, PhD, director of immunology at the Necker Hospital in Paris, France. Fischer is credited with demonstrating the clinical efficacy of gene therapy by successfully treating children suffering from a severe genetic disorder that renders them extremely vulnerable to infections.

The third 2015 Japan Prize laureate is Yutaka Takahasi, PhD, professor emeritus at the University of Tokyo, who is being honored in the "field of resources, energy and social infrastructure" for his contributions to river basin management and reducing water-related disasters.

Each laureate will receive a certificate of recognition and commemorative gold medal. A cash award of approximately $416,600 will also be given to each prize field. Since its inception in 1985, 83 laureates from 13 countries have received the Japan Prize in a variety of fields and

disciplines. Several have subsequently become Nobel Prize laureates as well.

In 1972, Friedmann, then a visiting scientist at the Salk Institute for Biological Sciences in La Jolla, and Richard Roblin, also at the Salk Institute, published a foundational article in the field, a paper in the journal *Science* under the heading “Gene therapy for human genetic disease?”

The idea of gene therapy, which quickly captured the public imagination, was fueled by its appealingly straightforward approach and what Friedmann has described as its “obvious correctness”: Disarm a potentially pathogenic virus to make it benign. Stuff these viral particles with normal DNA. Then inject them into patients carrying abnormal genes where they will deliver their therapeutic cargoes inside the defective target cells. In theory, the good DNA replaces or corrects the abnormal function of the defective genes, rendering previously impaired cells whole, normal and healthy. End of disease.

It’s not quite that simple, of course, something Friedmann and Roblin cautioned in their 1972 paper. Despite progress in the understanding of cellular functions, the roles of DNA and a series of experimental and clinical advances, the history of gene therapy has been marked by distinct highs and lows.

In 1968, Friedmann, working at the National Institutes of Health in Bethesda, Maryland with the late Jay Seegmiller (a founding faculty member of the UC San Diego School of Medicine) and others, showed that by adding foreign DNA to cultured cells from patients with Lesch-Nyhan syndrome they could correct genetic defects that caused the rare but devastating neurological disorder. The condition was first described by William Nyhan, MD, a UC San Diego professor of pediatrics, and medical student Michael Lesch in 1964.

The feat was a powerful proof-of-concept, but subsequent efforts to advance the work to human clinical trials stalled. “We began to realize that it would be very complicated to take this idea and make it work in people,” Friedmann said, who joined the UC San Diego School of Medicine faculty in 1969.

In recent years, the promise of gene therapy has finally translated into some clinical success stories, including Fischer’s work with children suffering from severe combined immunodeficiency or SCID. Friedmann and colleagues have continued their own work, including how to use current genetic knowledge and stem cell models to understand the basis for Lesch-Nyhan and identify more accessible treatment targets for gene-based and other forms of therapy.

“Technology has gotten better,” said Friedmann. “New kinds of viruses, such as the lentiviruses (a more efficient and safer gene delivery vector) were created. Disease models expanded. The science got more rigorous. I think the Japan Prize Foundation’s decision to honor Dr. Fischer and me is even greater recognition of the field itself, how far it has come and how much promise it holds.”

Friedmann said he hoped the prize would further spur the field of gene therapy, accelerating research and development at UC San Diego and other centers around the world.

“We’re well past the stage of having to prove the concept of gene therapy and have finally overcome our history of perhaps promising too much too soon. We’re at the point where we can truly begin to deliver real treatments to real people.”

A formal awards ceremony is scheduled for April 23, 2015 in Tokyo.
