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Boston Children's Hospital selects Vijay G. Sankaran to receive Rising Star Award

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Saluting his spectacular track record to date of innovative research on red blood cell disorders and his future promise as a physician/scientist, Boston Children's Hospital has selected Vijay G. Sankaran, MD, PhD, of Dana-Farber/Boston Children's Cancer and Blood Disorders Center to receive the Rising Star Award at the hospital's third annual Global Pediatric Innovation Summit, *Taking on Tomorrow* (#PedInno15).

The Rising Star Award recognizes the outstanding achievements of an up-and-coming innovator or team of innovators under the age of 45 in the field of pediatric health care. The award recipient receives \$25,000 to be used to advance the work for which they are recognized. The presentation of the Rising Star Award and companion Lifetime Impact Award in a ceremony at the Seaport World Trade Center on November 10 marked the culmination of a two-day summit that brought together innovators and thought leaders from across the globe to examine clinical, informatics and business opportunities in pediatric innovation.

"Vijay's work exemplifies why the Rising Star was created—he's a shining example of the fact that the best in pediatric innovation is yet to come," said Boston Children's Hospital President and CEO Sandra L. Fenwick. "What makes his work with blood stem cells so significant isn't the impact that it could have on children at Boston Children's Hospital; it's the impact it could have on patients of all ages, worldwide."

Sankaran has risen remarkably quickly to become one of today's leading young scientists in hematology and oncology. He received his doctorate and medical degree from Harvard Medical School in 2009 and 2010, respectively, followed by simultaneous medical and postdoctoral research training at Boston Children's Hospital, Boston Medical Center, the Whitehead Institute for Biomedical Research and the Broad Institute. A board-certified pediatric hematologist/oncologist, Sankaran both established his laboratory at Dana-Farber/Boston Children's and was appointed an assistant professor of pediatrics at Harvard Medical School in January 2014.

"Vijay is extremely unusual for a scientist at his career stage in that he can both conceptualize an entire field of investigation and define a focused set of studies that get to the heart of a difficult problem," the Whitehead Institute's Harvey Lodish, PhD, one of Sankaran's mentors, wrote seconding him for the award. "He is a physician-scientist who is expert in fields as diverse as human genetics and computational analysis of genetic and molecular data; he is also a skilled diagnostician and is already considered a world expert in hematologic diseases."

Starting with his work with Lodish and mentors Stuart H. Orkin, MD, of Dana-Farber/Boston Children's—recipient of the hospital's 2015 Lifetime Impact Award—and Eric Lander, PhD, of the Broad Institute, Sankaran has established an ambitious platform of research focused on the genetics, genomics, cellular biology and physiology of red blood cell development and red blood cell disorders such as Diamond-Blackfan anemia (DBA), sickle cell disease (SCD) and thalassemia. For instance, over a period of four years, Sankaran combined human genetics and whole genome mapping to identify the molecular switch *BCL11A* as a possible treatment target for SCD and thalassemia (both diseases related to hemoglobin mutations). His discoveries are fueling current efforts to affect the gene's activity therapeutically using gene editing tools (e.g., CRISPR).

In addition, Sankaran's molecular research on DBA (a severe, congenital form of anemia) has directly resulted in a unified model of DBA biology that integrates seemingly conflicting genetic discoveries about the condition into a comprehensive explanation of its biochemical underpinnings.

Most recently, Sankaran's laboratory found they could triple blood stem cells' production of red blood cells by shutting off the gene *SH2B3*, a target they identified by combing data from genome-wide association studies. His group believes their genetic approach could lead to better, cheaper methods of manufacturing red blood cells for transfusion and for use as drug delivery vehicles.

In 2015 Sankaran received a prestigious Young Investigator Award from the Society for Pediatric Research for his efforts to unravel red blood cell disorder genetics and development.

"[Vijay] has already established a vibrant laboratory that is using human genetics to provide clues to the defects and pathophysiology of hematologic disorders," Orkin wrote in support of Sankaran's nomination. "[I]t is hard to

conceive of another young pediatric investigator who has accomplished so much in such a short time and is poised to make truly innovative discoveries going forward."

Source:

Boston Children's Hospital
