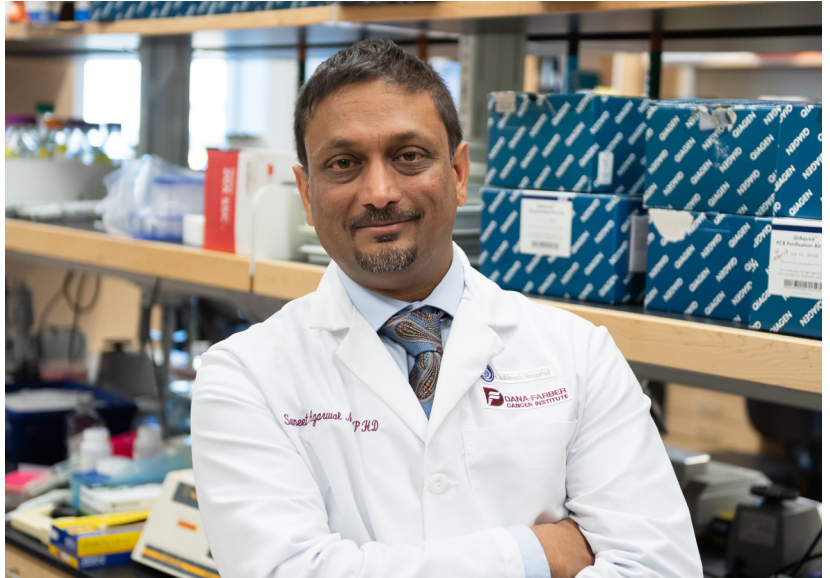


**SUNEET AGARWAL, MD, PhD**  
 Assistant Professor of Pediatrics  
 Harvard Medical School  
 Co-Program Leader for the  
 Stem Cell Transplant Center  
 Dana-Farber Cancer Institute and  
 Boston Children's Hospital  
 2018 Harrington Scholar-Innovator



## ANOTHER PLEASANT SURPRISE IN THE GENOME ERA

**FOCUS:** *Preventing dyskeratosis congenita (DC) and idiopathic pulmonary fibrosis (IPF) by restoring levels of a specific RNA molecule, as well as telomere homeostasis, in patient cells.*

Telomeres are caps at the ends of chromosomes that regulate how many times cells can divide. Problems that lead to diseases such as DC and IPF arise when there are mutations in the genes that regulate telomere biology. Dr. Agarwal's team appears to have found that when the PAPD5 enzyme is inhibited, telomere homeostasis in cells from IPF and DC patients is restored.

But that's only the beginning. Once you have agents that target fundamental pathways, often there are other conditions to which

your approach will apply. "We anticipate that these studies will yield novel telomerase modulators that could be useful in pulmonary fibrosis, bone marrow failure, and a range of degenerative disorders," Dr. Agarwal says. "Such links are increasingly being made, and validate focused efforts on rare diseases."

"I met DC patients at a family camp about eight years ago, and decided I wanted to fix this disease," Dr. Agarwal says. "The possibility of coming up with a breakthrough treatment for it is very

exciting. So is the notion that our work is leading to a broader body of knowledge that will apply to other diseases."

With support from Harrington, Dr. Agarwal is pursuing the goal of identifying small molecule modulators of PAPD5 activity, then testing their impact on telomere biology in patient cells.



FOR MORE INFORMATION, PLEASE VISIT:  
[harringtondiscovery.org/Videos](http://harringtondiscovery.org/Videos)

### IMPACT WISH:

*"We have high hopes that our work will yield novel telomerase modulators that could be useful in pulmonary fibrosis, bone marrow failure, and a range of degenerative disorders."*

**Harrington Discovery Institute**

 University Hospitals | Cleveland, Ohio