

**2020 KFP in Medicine Laureate
Professor Stuart Holland Orkin
Acceptance Speech**

To His Majesty:

I am truly honored to receive the King Faisal Prize in Medicine in behalf of the many extraordinary trainees who have worked in my laboratory to understand and develop new treatments for the hemoglobin disorders, notably sickle cell anemia and beta-thalassemia. This award brings international recognition to these disorders, which exert an enormous global health burden, disproportionately in just those areas of the world where medical resources are often scarce. After years of study with the most contemporary methods of genetics and molecular biology, we have a deep understanding of how to apply gene therapy or gene editing to provide effective, long-lasting benefit to patients with hemoglobinopathies. Yet, much remains to be done to bring genetic therapies to the many affected individuals and to develop drug therapy that will be more readily adopted in larger numbers of patients. This award will serve as a signal to all of medicine that we are on the verge of transformational changes in the ways with which we can help patients with hemoglobin disorders. This is truly an exciting era.

The accomplishments of my laboratory, particularly those that have occurred over the past nearly 15 years to reinvigorate research in the hemoglobinopathies, would not have taken place if it weren't for the extraordinary trainees that I have had the distinct honor of mentoring during this time. I specifically wish to mention Drs. Vijay Sankaran and Dan Bauer, two remarkable physician scientists, and Dr. Jian Xu, an outstanding molecular biologist. These 3 trainees established the foundational studies of BCL11A and its role in regulating the production of fetal hemoglobin. Each made singular contributions to the body of work.

In closing, I express my gratitude to those at Boston Children's Hospital, the Dana Farber Cancer Institute and Harvard Medical School who have supported my career and provided the environment in which to do our research. Again, I am indebted to you for recognition of our research with the distinguished King Faisal Prize in Medicine.