



**Katherine A. High, M.D.**  
**President and Head of Research and  
Development**



Dr. Kathy High, an accomplished hematologist with a longstanding interest in gene therapy for genetic disease, began her career studying the molecular basis of blood coagulation and the development of novel therapeutics for the treatment of bleeding disorders.

Her pioneering bench-to-bedside studies of gene therapy for hemophilia led to a series of studies that characterized the human immune response to adeno-associated viral (AAV) vectors in a variety of target tissues. Kathy's work has evolved to encompass clinical translation of potential gene therapies for multiple inherited disorders. As the director of the Center for Cellular and Molecular Therapeutics at the Children's Hospital of Philadelphia (CHOP), Kathy assembled a multidisciplinary team of scientists and researchers working to discover new gene and cell therapies for genetic diseases and to facilitate rapid translation of preclinical discoveries into clinical application.

As president at Spark Therapeutics, she has led the development and regulatory approval of the first gene therapy for a genetic disease in the United States. Spark has shown human proof-of-concept of its leading gene therapy platform in both the retina and the liver, and has received breakthrough therapy designations for three different therapeutics across two different tissue targets, the eye and the liver. In addition to her operational role, Kathy serves as a member of the Board of Directors of Spark.

Kathy was a long-time member of the faculty at the University of Pennsylvania and of the medical staff at CHOP, where she was also an Investigator of the Howard Hughes Medical Institute. She served a five-year term on the FDA Advisory Committee on Cell, Tissue and Gene Therapies and is a past president of the American Society of Gene & Cell Therapy (ASGCT). She received her A.B. in chemistry from Harvard University, an M.D. from the University of North Carolina School of Medicine, a business certification from the University of North Carolina Business School Management Institute for Hospital Administrators and an M.A. from the University of Pennsylvania.