

Gene Therapy Center endowed with \$2M gift

Horae Gene Therapy Center at UMMS named for Chinese benefactor

In the 1990s, gene therapy was first hailed as a potential cure for a myriad of diseases from sickle cell anemia to cystic fibrosis. It mesmerized the scientific community at the time—patients who participated in early clinical trials showed marked improvement, illustrating the treatment’s great promise. Sadly, that promise was hindered by the unfortunate and high-profile death of a patient during a trial in 1999. As a result, gene therapy was largely forgotten by the public and most of the scientific community.

Nearly two decades later, UMass Medical School is leading a resurgence in gene therapy treatment strategies that have had some notable successes—and are beginning to re-engage the public and attract the attention of prominent investors. In April 2015, the Medical School’s Gene Therapy Center, a global leader in adeno-associated virus (AAV) gene therapy, received a \$2 million endowment from the Horae Oriental Shenzhen Investment Company of Guangdong, China, a private company focused on developing the life sciences, medical research, medical education and medical care in Shenzhen, China. In recognition of the gift, the Gene Therapy Center was named the Horae (红瑞) Gene Therapy Center.



Front row, from left: Guangping Gao, PhD, P.J. “Chuck” Chen, Chancellor Michael Collins, Dean Terence Flotte and Yuet Chai appear with the staff of the Horae (红瑞) Gene Therapy Center in August 2015 to celebrate the endowment.

“The field of AAV gene therapy is attracting significant attention around the world and our scientists at the Gene Therapy Center are at the forefront of its discovery and development,” said Chancellor Michael F. Collins. “This gift supports our translational research into therapies for diseases that include cystic fibrosis, alpha-1 antitrypsin deficiency, lysosomal storage diseases, retinal and macular degeneration, and other genetic diseases.”

Guangping Gao, PhD, a top AAV researcher who has played a key role in the discovery and characterization of new AAV serotypes, is the director of the Horae (红瑞) Gene Therapy Center & Vector Core. Dr. Gao, the *Penelope Booth Rockwell Professor in Biomedical Research*, professor of microbiology & physiological systems and scientific director of the UMMS-China Program Office, said researchers at UMMS have a long history as pioneers in the field of AAV gene therapy.

“We are well known for the development of high quality AAV vectors for both academic researchers and

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industrial pharmaceutical companies around the world,” Gao said. “This gift will fuel our continuing discoveries and allow us to further expand our research. It will help us to translate many exciting preclinical discoveries and basic research into human clinical trials.”

“As a leading capital management group in China, we are not only seeking business opportunities in the area of life sciences, but we are also committed to scientific research,” said Yuet Chai, chairman of China Horae Capital Management Group, the holding company of Horae Oriental. “We greatly admire the world-class scientific achievements of UMass Medical School and are most honored to present this donation.”

Terence R. Flotte, MD, the *Celia and Isaac Haidak Professor of Medical Education*, executive deputy chancellor, provost and dean of the School of Medicine, was the principal investigator for the first use of recombinant AAV in humans, using the vector in cystic fibrosis trials beginning in 1995 before shifting his emphasis to alpha-1 antitrypsin deficiency, a genetic cause of emphysema. Dr. Flotte’s studies established important

safety data and contributed seminal observations regarding the molecular mechanisms of rAAV persistence and immune response in humans.

“Our Gene Therapy Center has also provided a platform technology to allow disease-focused researchers to directly translate knowledge about the genetic basis of diseases like ALS and Huntington’s disease into potential therapies for those diseases,” Flotte said. “The impact of this gift is multiplied by how thoroughly the center is interconnected with the RNA Therapeutics Institute and clinical departments like neurology and pediatrics.”

P.J. “Chuck” Chen, the CEO of the China Horae Group, said, “Our partnership with UMMS will be a perfect model of international collaboration between universities and companies, especially for the United States and China.

“A collaborative relationship between an academic research institution and a financial organization will generate great results beneficial to people, industry and science. We are looking forward to working with UMMS on developing biomedical markets in China for the long run.” ♦