America’s Best In Medicine: Gerald F. Cox, MD, PhD, For Advancing Clinical Development In Rare Genetic Disease Therapies

Board-Certified Clinical Geneticist and Founder of Gerald Cox Rare Care Consulting Advises Biotechnology Companies While Serving at Boston Children’s Hospital and Harvard Medical School

Boston, Massachusetts Mar 31, 2026 (Issuewire.com) - Boston, Massachusetts – Gerald F. Cox, MD, PhD, is a Board-Certified Clinical Geneticist with more than 25 years of experience dedicated to advancing therapies for rare genetic diseases. As the Founder of Gerald Cox Rare Care Consulting, LLC, Dr. Cox provides expert guidance to biotechnology companies across all phases of clinical development for investigational therapies, helping translate emerging scientific discoveries into meaningful treatments for patients with rare disorders.

In addition to his consulting work, Dr. Cox remains actively involved in patient care and academic medicine. He serves as a part-time Staff Physician in the Genetics Clinic at Boston Children's Hospital and as an Instructor in Pediatrics at Harvard Medical School, where he continues to contribute to clinical education and the advancement of genetic medicine.

Dr. Cox earned both his MD and PhD from the University of California, San Diego. He completed his internship and residency in Pediatrics, followed by Clinical and Research fellowships in Genetics at Boston Children's Hospital and Harvard Medical School. He is board-certified in Clinical, Biochemical, and Molecular Genetics by the American Board of Medical Genetics and Genomics and was previously board-certified in Pediatrics by the American Board of Pediatrics.

Dr. Cox began his career as a physician-scientist at Boston Children's Hospital, where he combined patient care with laboratory-based research in human genetics. After several years in academic medicine, he transitioned into the biotechnology industry, joining Genzyme—now part of Sanofi—where he spent more than 16 years in roles of increasing leadership responsibility. He ultimately served as Vice President of Rare Disease Clinical Development.

During his tenure at Genzyme, Dr. Cox played a pivotal role in the global development and approval of treatments for several lysosomal storage disorders. His work contributed to enzyme replacement therapies for Mucopolysaccharidosis types I and II, Gaucher disease, Niemann-Pick B disease, and infantile Pompe disease. In addition to enzyme replacement therapies, he also helped advance substrate reduction therapies and other innovative treatment strategies designed to address complex metabolic disorders.

From 2016 to 2018, Dr. Cox served as Chief Medical Officer at Editas Medicine, where he led the clinical development of genome-editing therapies based on CRISPR technology. During his time there, he contributed to the development of the first approved investigational new drug (IND) application for an in vivo CRISPR therapy designed to treat Leber congenital amaurosis type 10, a rare genetic form of inherited blindness. This work represented a significant milestone in the advancement of gene-editing technologies for human disease.

Beyond his leadership in clinical development, Dr. Cox has remained deeply engaged in patient advocacy and scientific research. He served on the Board of Directors for the National Tay-Sachs and Allied Diseases (NTSAD) Organization, where he chaired the Research Committee. In this role, he helped families understand the importance of clinical trials and how participation contributes to the advancement of potential new therapies for rare conditions.

Dr. Cox has authored more than 100 publications focused on genetic disorders, clinical trials, and emerging genomic therapies. His work reflects a long-standing commitment to advancing scientific knowledge while improving the lives of individuals and families affected by rare diseases.

Over the course of his career, Dr. Cox has successfully integrated clinical medicine, scientific research, biotechnology leadership, and mentorship. His contributions have helped shape the development of

innovative therapies that are transforming care for patients with rare genetic disorders around the world.

Among his many professional recognitions are Who's Who Recognition in 2025 for excellence and leadership in clinical genetics and rare disease research, and the Lifetime Achievement Award in 2026, honoring decades of pioneering contributions to the development of therapies for rare genetic conditions and his enduring commitment to patient care.

Learn more about Dr. Gerald F. Cox:

Through his America's Best in Medicine profile, https://americasbestinmedicine.com/connect/gerald-cox, through his profile on Boston Children's Hospital, https://www.childrenshospital.org/providers/gerald-cox or through his website, https://www.geraldfcox.com/

America's Best in Medicine

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