Stanley T. Crooke, M.D., Ph.D.

Founder, Chief Executive Officer and Chairman of the Board

Dr. Stanley Crooke is founder, chairman and chief executive officer of n-Lorem, a nonprofit foundation focused on providing treatments for patients with nano-rare disease patients (1 to 30 patients worldwide), which he initiated in January 2020.

Prior to n- Lorem, Dr. Crooke founded and was Chairman and Chief Executive Officer and Lead Scientist of Ionis Pharmaceuticals. During his tenure at Ionis, he led the scientific development of a new platform for drug discovery, antisense technology and the creation of one of the largest and more advanced development pipelines in the biotechnology industry, and commercialized several antisense drugs including, SPINRAZA® (nusinersen), TEGSEDI™ (inotersen) and others. Early in Dr. Crooke's career, he led the creation of the first broad anticancer program in the industry at Bristol-Myers, bringing numerous anticancer drugs to the market in the first five years of his career. He then assumed responsibility for worldwide R&D (president) at SmithKline Beckman (now GSK). During his tenure at SKB, Dr. Crooke led the restructuring of R&D and the development of several drugs that were commercialized.

Dr. Crooke has also contemporaneously led a successful academic career becoming a full professor at Baylor College of Medicine and the University of Pennsylvania Medical School where he trained a number of Ph.D. students and won several teaching awards. Dr. Crooke has been an active scientist throughout his career as well.

Dr. Crooke has received a number of awards, most recently, the Prix Galien Roy Vagelos Pro Bono Humanum Award, the American Chemical Society's E.B. Hershberg Award for Important Discoveries in Medicinally Active Substances, the Lifetime Achievement Award presented by the Oligonucleotide Therapeutics Society, the Scrip Lifetime Achievement Award and the 2019 Massry Prize.

Dr. Crooke received his M.D. and Ph.D. degrees and house staff training at Baylor College of Medicine, where he currently serves on the Board of Advisors. In 2021, Dr. Crooke has been named Distinguished Alumnus of both Baylor College of Medicine's Graduate and Medical schools and named one of the 20 of the most influential biopharma R&D executives by Endpoints News. He has published nearly 600 scientific publications, edited more than 20 books, has numerous patents, and led the development of more than 23 drugs that have been commercialized.

C. Frank Bennett, Ph.D.

Chief Technical Officer

Dr. Frank Bennett is the chief technical officer of n-Lorem Foundation. He also is a founding member of Ionis Pharmaceuticals and serves as the company's chief scientific officer. He is responsible for preclinical antisense drug discovery and antisense technology research. Dr. Bennett is also the franchise leader for neurological programs at Ionis. He has been involved in the development of antisense oligonucleotides as therapeutic agents, including research on the application of oligonucleotides for inflammatory, neurodegenerative diseases and cancer, oligonucleotide delivery, pharmacokinetics and medicinal chemistry.

Dr. Bennett is a co-recipient of the 2019 Breakthrough Prize in Life Sciences for his contributions to the discovery and development of SPINRAZA® (nusinersen) and the 2018 Hereditary Disease Foundation's (HDF) Leslie Gehry Brenner Prize for Innovation in Science for his leadership and continued commitment to developing antisense therapies for Huntington's disease (HD).

Dr. Bennett has published more than 200 papers in the field of antisense research and development and he is an inventor on more than 175 issued patents.

Prior to joining Ionis, Dr. Bennett was associate senior investigator in the Department of Molecular Pharmacology at SmithKline and French Laboratories, currently, GlaxoSmithKline.

He received his Ph.D. in Pharmacology from Baylor College of Medicine, Houston, Texas and his B.S. degree in Pharmacy from the University of New Mexico, Albuquerque, New Mexico. He performed his postdoctoral research in the Department of Molecular Pharmacology at SmithKline and French Laboratories.

Dr. Bennett serves on the Advisory Board for the Experimental Therapeutics Centre in Singapore and the Hereditary Disease Foundation.

Sarah Glass, Ph.D.

Chief Operating Officer

Dr. Sarah Glass is the Chief Operating Officer of n-Lorem Foundation. Sarah received her Ph.D. in Molecular Genetics at Ohio State University where she trained in rare inherited cancer syndromes. She has over 20 years of experience in clinical development and research across academia, pharmaceutical companies, and CROs. Sarah brings significant strengths and experience as an accomplished research geneticist, rare disease drug developer, and clinical trialist. She is acclaimed for forging key strategic partnerships across rare disease sectors and has driven efficiencies to decrease patient/ caregiver burden in clinical research. Most notably, Sarah combines the professional expertise and training with the perspective of a parent. This allows Sarah to not only personally understand the challenges faced by our patients and their families, but also to translate this understanding into n-Lorem's paradigm shifting platform solution for the ultra-rare community. Outside of n-Lorem, Sarah volunteers as chair of the DYRK1A Medical and Scientific advisory board and is engaged in many rare disease organizations to ensure collaboration in reaching our common goals.

Joe Gleeson, M.D.

Chief Medical Officer

Dr. Joe Gleeson is the Chief Medical Officer of n-Lorem Foundation. Joe graduated from the University of Chicago Pritzker School of Medicine, then trained in pediatrics, neurology and neurogenetics at Children's Hospital, Boston and Harvard Medical School. He has been a faculty member at University of California San Diego School of Medicine and an attending physician at Rady Children's Hospital San Diego since 1999. From 2008-2019, he was an investigator with the Howard Hughes Medical Institute. From 2014-2015, he was the Hess Professor and head of the Laboratory for Pediatric Brain Disease at Rockefeller University, and director of mendelian sequencing at the New York Genome Center. Since 2015, he has held the Rady Children's Hospital auxiliary endowed professorship of neuroscience and served as director of neuroscience research at the Rady Children's Institute for Genomic Medicine. He is an investigator with the Simons Foundation for Autism Research Initiative, and an elected member of the National Academy of Medicine.

Amy C. Williford, Ph.D.

Sr. Director of Communications and Donor Relations

Dr. Amy Williford is the senior director of communications and donor relations at n-Lorem Foundation. Amy is a seasoned communications professional who is well versed in translating complex scientific concepts and ideas into concise and easy-to-understand concepts.

A scientist by training, Amy supported multiple high-profile communications programs as the associate director of corporate communications at Ionis Pharmaceuticals. She also managed and built relationships with pharmaceutical partners, institutional investors and private shareholders.

Prior to joining Ionis, Amy worked at a public relations agency, where she managed and led communications for a range of companies across the biotechnology industry, and built long-standing relationships with key media, investors and analysts.

Amy received her Ph.D. in chemistry from The Scripps Research Institute, where she studied the selective binding of transcription factors to DNA.

Kim Butler

Sr. Director of Operations

Kim Butler is the senior director of operations at n-Lorem Foundation. Kim is a broadly experienced administrative assistant who has been employed by Ionis Pharmaceuticals since 2014 . During her tenure with Ionis she has been promoted several times and has assumed substantial managerial responsibilities. Additionally, she is accountable for leading the training of all administrative personnel and leads the management of administrative activities for Ionis. Currently, Kim is the senior administrative assistant to the Chairman and CEO of n-Lorem and has played an instrumental role in the formation of the foundation.

Prior to joining Ionis, Ms. Butler served in senior administrative positions in several organizations and has help found and participated in a number of charitable efforts. She is active in a wide range of civic service groups such as the American Legion and a frequent volunteer committed to serving the needy empathetically.

Tracy Cole, Ph.D.

Sr. Director of Research

Dr. Tracy Cole is the senior director of research at n-Lorem Foundation. Tracy has expertise in all aspects and stages of drug discovery and development. Most recently at Neurocrine Biosciences, Tracy led internal preclinical efforts to advance AAV gene therapy-based drug discovery. Prior to joining Neurocrine, Tracy was an integral part of Ionis Pharmaceutical's neuroscience group where she coordinated with clinical, pharmacokinetic, toxicology, in vitro screening, regulatory, legal, and translational medicine groups to bring multiple ASO therapies to the clinic.

Tracy received her Ph.D. in neurobiology and behavior at the University of California, Irvine, where she focused on the involvement of inflammation in Alzheimer's disease, specifically one aspect of the innate immune response called the complement cascade. Tracy has contributed to nine clinical development compound patents and authored/co-authored over ten top-tier journal publications.

Julie Douville, Ph.D.

Sr. Director of Pre-clinical Development

Dr. Julie Douville is the senior director of pre-clinical development at n-Lorem Foundation. Prior to n-Lorem, Julie was at Charles River Laboratories from 2007 to 2022 as the director of infusion, parenteral administration and neurotoxicology, where she specialized in cerebrospinal administration, and in the development of antisense oligonucleotides, gene therapies and other therapeutic entities. In this role, Julie oversaw several IND-supporting programs for indications including ALS, Alzheimer's Disease, Parkinson's, Angelman's Syndrome, Duchenne's Muscular Dystrophy, and many others. Working with academic investigators, she personally oversaw the toxicology program for milasen, the first personalized ASO treatment that was customized for a mutation in a single patient, which was approved for clinical use by the FDA in 2018. Julie led several additional n=1 preclinical programs, in addition to being involved on many other rare disease programs for which she developed strategies for acceleration of the preclinical phase. She has taken part in key discussions with regulatory authorities in the FDA and EMA, helping to establish precedents

for the U.S. guidelines around individualized antisense oligonucleotide treatment. Julie carries a strong background in GLP preclinical development, and in-depth knowledge of ASO-associated toxicities, especially in the central nervous system. Julie received her Ph.D. in Microbiology and Immunology from the University of Montreal.

Jeff Caroll, Ph.D.

Scientific Advisor

Dr. Jeff Carroll is a scientific advisor for n-Lorem. Jeff began assisting n-Lorem's access to treatment committee at a very early stage, given his passion for helping families with rare genetic diseases. This passion emerges from a personal connection – Jeff's mother died after suffering from Huntington's Disease, placing her 6 children at 50% risk of developing the disease. In 2003, Jeff learned that he inherited this mutation from his mother, sparking a passion to help understand the disease, and to help hasten the development of novel treatments for all HD patients.

Jeff is now an associate professor in the Department of Neurology at the University of Washington. Jeff's primary scientific focus is on conducting translational studies in support of "Huntingtin Lowering" approaches to Huntington's Disease, primarily through the use of antisense oligonucleotides. Jeff's Ph.D. and postdoctoral studies all involved using mouse models to understand basic mechanisms of HD and preclinical testing of therapeutic interventions in these models. During his Ph.D., he trained with Michael Hayden (UBC) and his postdoctoral studies were conducted under the supervision of Marcy MacDonald (MGH, Harvard Medical School).

Cedrik Ngongang, M.D.

Sr. Director, Medical Geneticist

Dr. Cedrik Ngongang is the senior director medical geneticist at n-Lorem Foundation. Prior to joining n-Lorem, Dr. Ngongang was a scientist at the National Human Genome Research Institute, National Institutes of Health.

Dr. Ngongang's clinical and research experience in genetic medicine spans various aspects of the diagnostics and management of rare disorders, including clinical assessment of genomic variants, elucidation of the genetic basis of Mendelian phenotypes in individuals from ancestrally diverse backgrounds, and application of machine learning approaches to the clinical and genomic diagnosis.

He received his M.D. from the University of Yaoundé 1 Faculty of Medicine, and his Master of Medicine in medical genetics from the University of Cape Town. He completed his postdoctoral training in molecular genetics at the National Institutes of Health, following his board-certification in clinical genetics in South Africa.