Adrian R. Krainer

Cold Spring Harbor Labs

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Adrian Krainer is the St. Giles Foundation Professor at Cold Spring Harbor Laboratory. He received a B.A. cum laude in Biochemistry from Columbia University in 1981, and a Ph.D. in Biochemistry from Harvard University in 1986, working in the laboratory of Prof. Tom Maniatis. He joined CSHL in 1986 as a CSH Fellow, and was promoted to Assistant Professor in 1989, Associate Professor in 1990, and Professor in 1994. The Krainer lab studies the mechanisms and regulation of messenger RNA splicing in human cells, and the role of splicing dysfunction in genetic diseases and cancer. They are also engaged in the preclinical development of antisense-oligonucleotide drugs that target RNA splicing or other RNA-processing steps. Together with Ionis Pharmaceuticals and Biogen, the Krainer lab developed nusinersen (Spinraza), which was approved by the FDA in 2016 as the first treatment for spinal muscular atrophy, a neurodegenerative disease that was the leading genetic cause of infant mortality. Adrian is a member of the U.S. National Academy of Sciences, the National Academy of Medicine, the National Academy of Inventors, and the American Academy of Arts & Sciences. He has received multiple scientific awards, including among others the Life Sciences Breakthrough Prize (2019), the Wolf Prize in Medicine (2021), the International Prize for Translational Neuroscience (2019), the RNA Society's Lifetime Achievement Award (2019), the Ross Prize in Molecular Medicine (2020), the Peter Speiser Award in Pharmaceutical Sciences (2019), the Albany Prize in Medicine & Biomedical Research (2024), and the Heinrich Wieland Prize (2025). Adrian served as President of the RNA Society in 2014, and currently serves on the advisory boards of several scientific centers and networks, non-profit foundations, and biotechnology companies in the U.S., Europe, and Latin America. He is a co-founder and Director of Stoke Therapeutics.

Abstract: "Antisense Oligonucleotides for Rare-Disease Therapy"

In collaboration with Ionis Pharmaceuticals and Biogen, we previously developed nusinersen (Spinraza), an antisense oligonucleotide (ASO) that modulates alternative splicing of SMN2 exon 7, restoring normal levels of functional SMN protein in the context of spinal muscular atrophy (SMA). The long duration of action of CNS-administered ASOs like Spinraza allows infrequent dosing by lumbar puncture, providing a feasible and effective approach to treat neurological disorders. Consequently, many ASOs are being developed against relevant targets in neurology and neuro-oncology, in addition to other targets in the liver and the eye. Splice-switching ASOs, in particular, are highly versatile, because of the pervasiveness of pre-mRNA splicing. They can be designed to upregulate or downregulate the expression of target genes, to change the relative levels of alternatively spliced isoforms, to correct defective splicing, or to bypass frame-disrupting mutations. I will describe specific applications of this powerful technology.