

CRAIG C. MELLO, PHD

Craig C. Mello, PhD, is the *Blais University Chair in Molecular Medicine* at the University of Massachusetts Medical School (UMMS) in Worcester, Massachusetts. He was also designated an Investigator of the Howard Hughes Medical Institute in 2000, the third HHMI researcher selected at UMMS. HHMI is a \$13 billion medical research organization that employs more than 350 eminent researchers at 72 medical schools, universities and research institutes worldwide.

Dr. Mello and his colleague Andrew Fire, PhD, of Stanford University, received the 2006 Nobel Prize in Physiology or Medicine for their discoveries related to RNA interference (RNAi).

Mello holds his BS in biochemistry from Brown University and his PhD in Cellular and Developmental Biology from Harvard University. He was a postdoctoral fellow at the Fred Hutchinson Cancer Research Center before coming to UMMS in 1994. He was also a 1995 Pew Scholar in the Biomedical Sciences.

Mello and Dr. Fire, formerly of the Carnegie Institution of Washington, demonstrated that a certain form of RNA had the unanticipated property of silencing—or interfering with—the expression of a gene whose coding sequence of DNA was similar to that of the RNA they tested. The RNAi mechanism—a natural response of an organism to double-stranded RNA, of which many viruses are comprised—destroys the gene products that a virus needs to replicate itself, essentially halting the progression of the invading viral infection.

The discovery, which offers astounding potential for understanding and manipulating the cellular basis of human disease, has had two extraordinary impacts on biological science. One is as a research tool: RNAi is now the state-of-the-art method by which scientists can knock down the expression of specific genes in cells, to thus define the biological functions of those genes. But just as important has been the finding that RNAi is a normal process of genetic regulation that takes place during development. Thus, RNAi has provided not only a powerful research tool for experimentally knocking out the expression of specific genes, but has opened a completely new and totally unanticipated window on developmental gene regulation.

Published in the February 19, 1998 issue of *Nature*, Mello and Fire's work in RNAi, considered the formative paper on the topic, was hailed as “an electrifying discovery” and named the 2002 “Breakthrough of the Year” by *Science* magazine. In 2003, Mello and Fire received the prestigious National Academy of Sciences Award in Molecular Biology for their pioneering work as well as the Wiley Prize in the Biomedical Sciences from Rockefeller University; the Fourth Annual Aventis Innovative Investigator Award at the Drug Discovery Technology World Conference; and, in 2004, the Warren Triennial Prize, the highest research honor bestowed by Massachusetts General Hospital. Also in 2004, Fire was elected a member of the National Academy of Sciences, an honor bestowed upon Mello a year later.

In 2005, Mello and Fire were recipients of several additional honors including Brandeis University's Lewis S. Rosenstiel Award for Distinguished Work in Medical Research, the Canadian government's Gairdner International Award and the Massry Prize. In 2006, the pair flew to Germany to accept the Paul Ehrlich and Ludwig Darmstaedter Prize, one of the highest and most internationally renowned awards conferred in the Federal Republic of Germany in the field of medicine. Mello was named the inaugural recipient of The Dr. Paul Janssen Award for Biomedical Research by Johnson & Johnson in 2006.

Given the fundamental and broad-based impact of RNAi, a patent, “Genetic Inhibition by Double-Stranded RNA,” (US Patent 6,506,559 B1) issued to UMMS and Carnegie, is expected to have far-reaching licensing potential both in the lab and in drug development. Because both institutions were eager to bring RNAi to bear as broadly as possible to hasten genetic research, they developed a licensing policy by which companies can readily obtain, for a basic fee, a wide-ranging and non-exclusive license for scientists to use the technology for research. A significant number of companies have already licensed the invention and additional companies have expressed interest.