MedicalXpress

Scientists find new agent to fight genetic disorders -- Zorro-Locked Nucleic Acid

April 30 2007

A study to appear in the June 2007 issue of The FASEB Journal describes a new agent, called "Zorro-LNA," which has the potential to stop genetic disorders in their tracks. In the study, researchers from the Karolinska Institute in Stockholm, Sweden, describe how they developed Zorro-LNA to bind with both strands of a gene's DNA simultaneously, effectively disabling that gene.

This development has clinical implications for virtually every human condition caused by or worsened by dominant defective genes. Examples include: Huntington's disease, familial high cholesterol, polycystic kidney disease, some instances of glaucoma and colorectal cancer, and neurofibromatosis, among others.

"Zorro-LNA is a new substance that targets DNA and turns off genes," said co-author Edvard Smith of the Karolinska Institute in Sweden. "It has the potential of becoming a new drug for the treatment of human genetic disease."

The findings described in this article significantly raise the possibility that new therapies could arise where defective DNA is deactivated more completely and more thoroughly than ever before. For instance, Zorro-LNA could be used in combination with "RNA interference" (RNAi). Like Zorro-LNA, RNAi has the ability to deactivate genes, but does so by degrading the gene's RNA. In addition, Zorro-LNA could be used to deactivate certain genes in stem cells, which could eventually lead to the development of new cells, tissues, or organs. The discovery of RNAi was

recognized by a Nobel Prize award in 2006 to two American scientists.

"This is a major development in the treatment not only of genetic diseases, but also of acquired diseases when microbes or toxins cause genes to go awry" said Gerald Weissmann, M.D., Editor-in-Chief of The FASEB Journal. "One might say these researchers have found a gene-hunter's Holy Grail for which scientists have been hunting for many years. Zorro-LNA should give us a new, safe way of blocking the effects of errors in our genetic repertoire."

Source: Federation of American Societies for Experimental Biology