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SCIENTISTS FIND NEW AGENT TO FIGHT GENETIC DISORDERS: ZORRO-LOCKED NUCLEIC ACID

New Study in* The FASEB Journal *describes how Zorro-LNA turns off harmful genes

Bethesda, MD—A study published 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."

The Editor-in-Chief of *The FASEB Journal* will be available for interviews about this or other articles at a press reception at this year's Experimental Biology meeting in Washington, D.C., on Tuesday, May 1, 2007, between 1:30–3:00 PM in the Experimental Biology press lounge. (Refreshments will be provided.) For more information about the meeting, visit www.eb2007.org. A fact sheet on this article is available at *The FASEB Journal*'s press room. Visit www.fasebj.org and click "Press Room" in the left column. *The FASEB Journal* is published by the Federation of American Societies for Experimental Biology (FASEB) and is consistently ranked among the top three biology journals worldwide by the Institute for Scientific Information. FASEB comprises 21 nonprofit societies with more than 80,000 members, making it the largest coalition of biomedical research associations in the United States. FASEB advances biological science through collaborative advocacy for research policies that promote scientific progress and education and lead to improvements in human health.

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