Team devises ingenious method to attack cancer at its genetic source

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MicroRNAs may be tiny—as few as 20 genetic letters, compared to 3 billion in the DNA of a human—but they play a major role in biology, helping to determine which genes are expressed or silenced. In the last 10 years, researchers at Yale and elsewhere have shown they play a major role in formation and spread of tumors.

However, their potential as a target for cancer therapy has not been realized because of a daunting problem that has held back clinical applications of gene therapy: How can you target minute pieces of genetic material locked safely inside the membranes of billions of cells?

Now a multi-disciplinary team of Yale researchers has solved the problem by designing a therapeutic molecule that both targets the acidic microenvironments of tumors and penetrates cells to deliver a therapeutic cargo. The new delivery system effectively killed advanced tumors in mice, the team reports in the Nov. 17 issue of the journal *Nature*.

"This strategy opens up a new pathway to therapy, not just for the treatment of cancer but for a host of other diseases as well," said Donald Engelman, a co-author of the paper and the Eugene Higgins Professor of Molecular Biophysics and Biochemistry at Yale.

Provided by Yale University