New methods for quantifying antisense drug delivery to target cells and tissues

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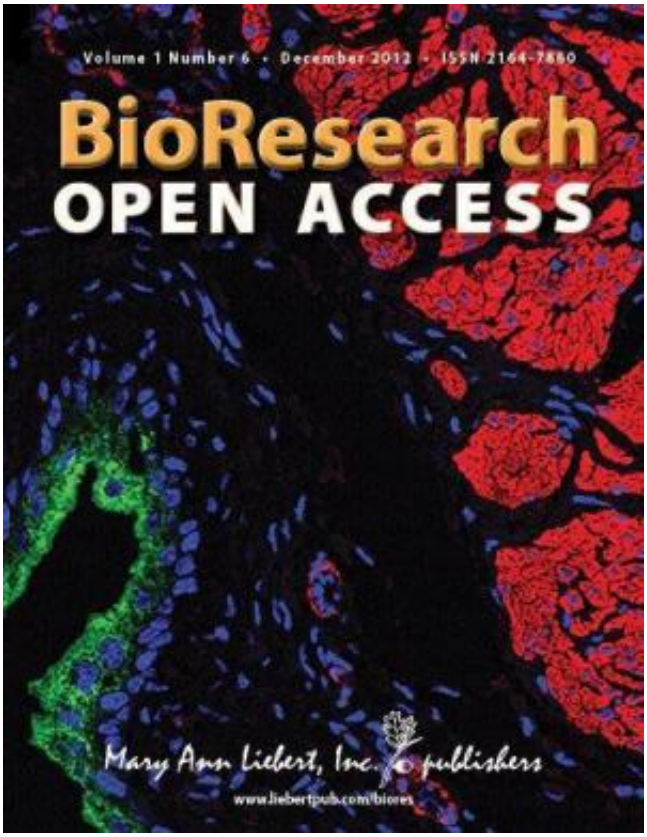

Powerful antisense drugs that target disease-associated genes to block their expression can be used to treat a broad range of diseases. Though antisense therapy has been proven effective, challenges remain in ensuring that the drugs reach their intended targets. Two new methods

for detecting and measuring the levels of antisense drugs in cells that could accelerate the development of improved antisense drugs are described in an article in *BioResearch Open Access*.

In the article "Development of Novel Bioanalytical Methods to Determine the Effective Concentrations of Phosphorodiamidate Morpholino Oligomers in Tissues and Cells," Frederick Schnell, Stacy Crumley, Dan Mourich, and P.L. Iversen, from Sarepta Therapeutics and Oregon State University (Corvallis, OR), describe two fast and sensitive methods for detecting a promising type of antisense therapeutic called a phosphorodiamidate morpholine oligomer, or PMO. Using these novel methods they were able to detect PMO delivery to individual cells and quantify how much PMO resides in a particular tissue in the body, such as the lung. For example, the authors describe the measurement of intranasally delivered PMO in lung tissue and, more specifically, in different cell types in the lung. They were able to measure the clearance kinetics of the PMO and determine whether it stayed in the lung tissue.

"The development of novel, rapid PMO detection techniques such as these will advance the field of PMO research in a significant way, providing valid alternatives to the current time-consuming and labor-intensive methods," says Editor-in-Chief Jane Taylor, PhD, MRC Centre for Regenerative Medicine, University of Edinburgh, Scotland.

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