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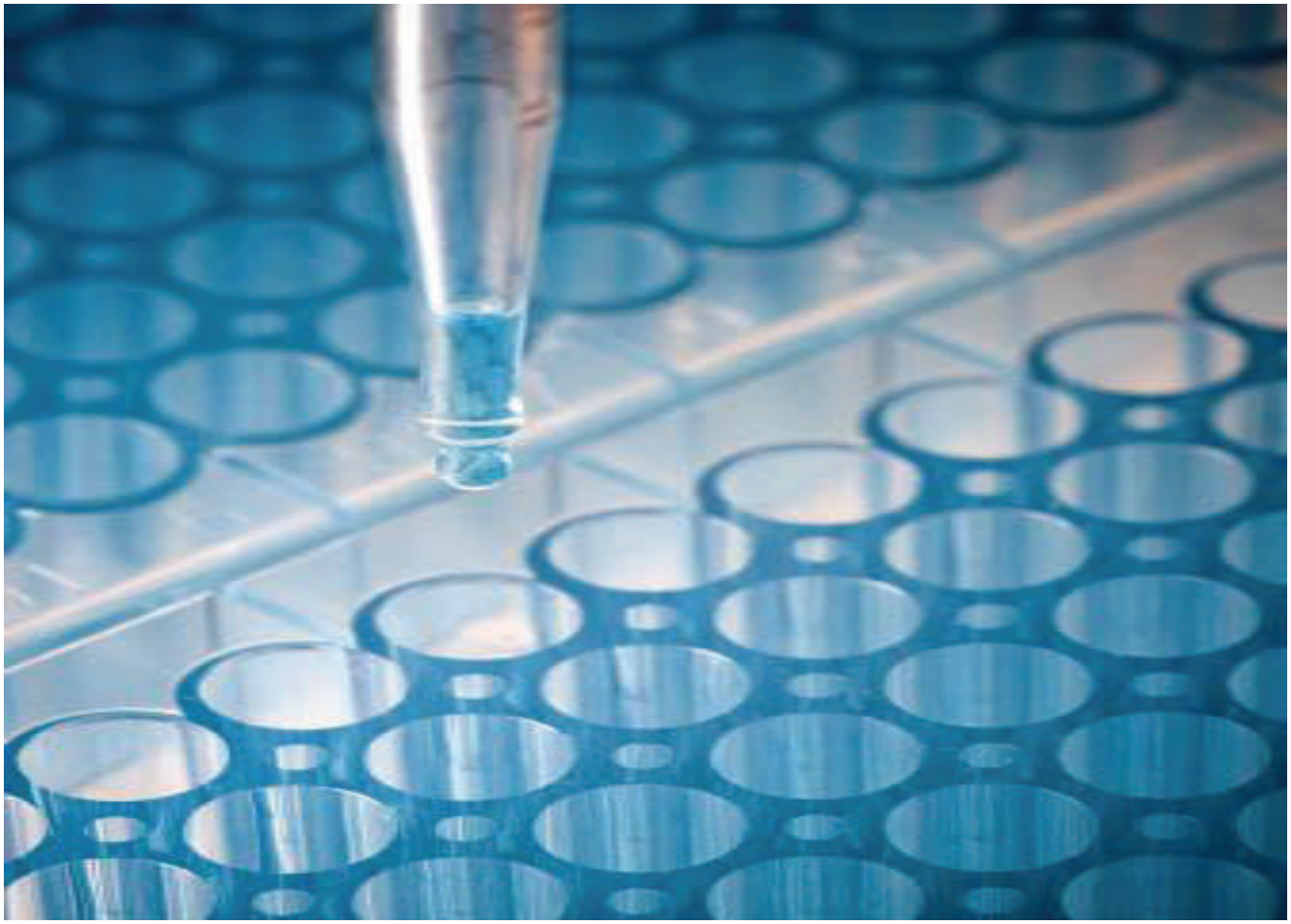
Discovery Platform

for ribozymes, antisense & RNAi based therapies

The development of RNA based therapies suffers from a bottleneck in identifying lead candidates that significantly knockdown target RNA *in vivo*. This is primarily due to the difficulties in predicting the dynamic secondary and tertiary structure of RNA. Currently, researchers begin by using software programs such as MFold and SFold. However, these programs do not fully take into account the dynamic and complex RNA structure. Once lead candidates are identified, traditional *in vitro* screening methods are used to determine RNA knockdown. These methods are costly, time consuming, and contribute to the lack of efficiency found in the preclinical process for RNA based therapeutics. After years of frustration over the slow pace of traditional methods, researchers at the University at Buffalo have developed a suite of optimizing software and screening tools to address these bottlenecks, with an eye towards increased likelihood of *in vivo* success.



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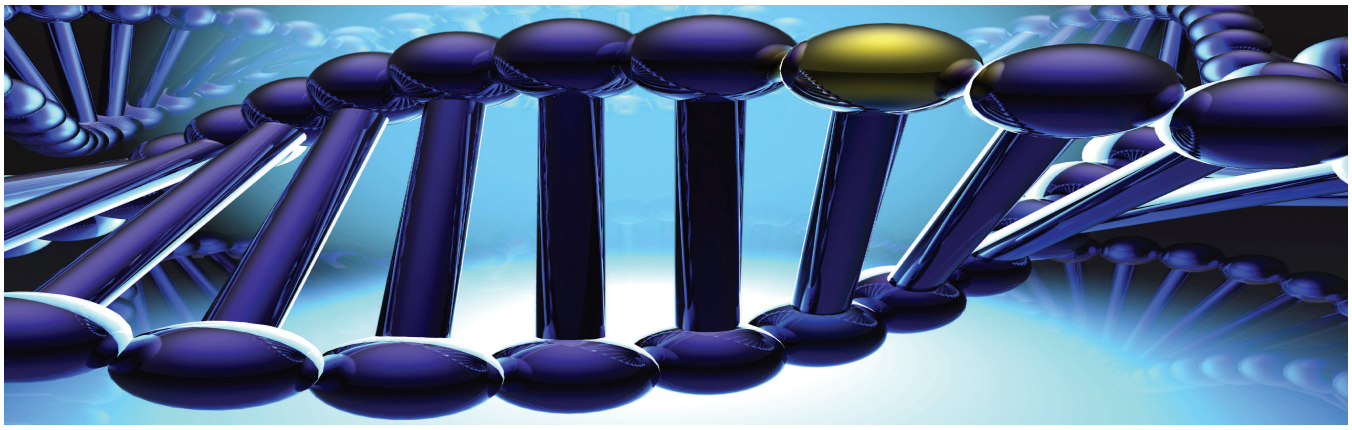

This platform is applicable to any RNA target in need of lead candidate development, optimization, and *in vitro* screening.

Proof of principle experiments for these technologies identified lead candidate hammerhead ribozyme sequences for human rhodopsin mRNA. Several of the best candidates were not identified by current methods. Furthermore, the high throughput assay associated with this platform showed a 28 fold increase in efficiency over traditional screening techniques, such as Western analysis.

RELATED PUBLICATION

Sullivan, J. M., Yau, E. H., Taggart, R. T., Butler, M. C. and Kolniak, T. A., "Bottlenecks in Development of Retinal Therapeutic Post-transcriptional Gene Silencing Agents", *Vision Research* 48:453-69 (2008).

Abdelmaksoud, H. E., Yau, E. H., Zuker, M. and Sullivan, J. M., "Development of Lead Hammerhead Ribozyme Candidates Against Human Rod Opsin mRNA for Retinal Degeneration Therapy", *Experimental Eye Research* Epub December 6, 2008.

PATENT STATUS

U.S. Patent Pending

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