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Activating frataxin expression by single-stranded siRNAs targeting the GAA repeat expansion

Xiulong Shen, Audrius Kilikevicius, Daniel O'Reilly, Thazha P. Prakash, Masad J. Damha, Frank Rigo, David R. Corey

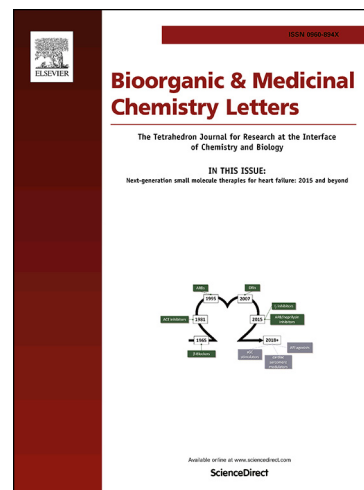
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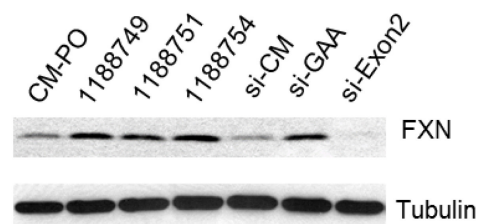
Graphical Abstract

Activating frataxin expression by single-stranded siRNAs targeting the GAA repeat expansion

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No.	Sequence (5'-3')	EC ₅₀ (nM)
1188749	5'-P-T _s U _s UC _s UU _s CU _s UC _s UU _s CU _s U _s C _s U _s U _s C _s -A _s A _s	5.0 ± 0.8
1188751	5'-P-T _s C _s UU _s CU _s UC _s UU _s CU _s UC _s U _s U _s C _s U _s -A _s A _s	6.2 ± 0.6
1188754	5'-P-T _s U _s CU _s UC _s UU _s CU _s UC _s UU _s C _s U _s C _s U _s -A _s A _s	3.2 ± 0.7



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