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Targeted knock-up of endogenous genes using a long non-coding RNA chaperone to improve translation efficiency

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Abstract

The molecular repair toolbox has been augmented in the past year by the development of a technology that can specifically increase the amount of protein made by a targeted endogenous gene. This technology was first demonstrated in an elegant study by Carrieri et al (Nature 491:454). This paper describes a particular lncRNA containing a SINEB2 repeat that increases the efficiency of protein translation for a target gene to which the lncRNA specifically binds. In many ways the technique, dubbed SINEUP, is the antithesis of RNAi. For example, the effect is achieved in trans and SINEUP can act on endogenous genes. However, unlike RNAi, SINEUP does not interfere with mRNA levels, which remain constant. Moreover, SINEUP currently relies on a large, 1105nt effector domain (coupled to a 73 nt gene specific binding domain) making delivery of a therapeutic potentially more challenging than with RNAi. This exciting technology is still in its infancy and characterization of the SINEUP system is ongoing.

Biography

Michael Jones is CEO of Cell Guidance Systems, which he founded in 2010. Prior to this, he was COO of ReproCELL and headed Invitrogen's corporate development team in Asia Pacific. His research career focused on cancer genomics. Michael is the author of thirty papers and four patents.