

Vertex taps Entrada for another try at myotonic dystrophy



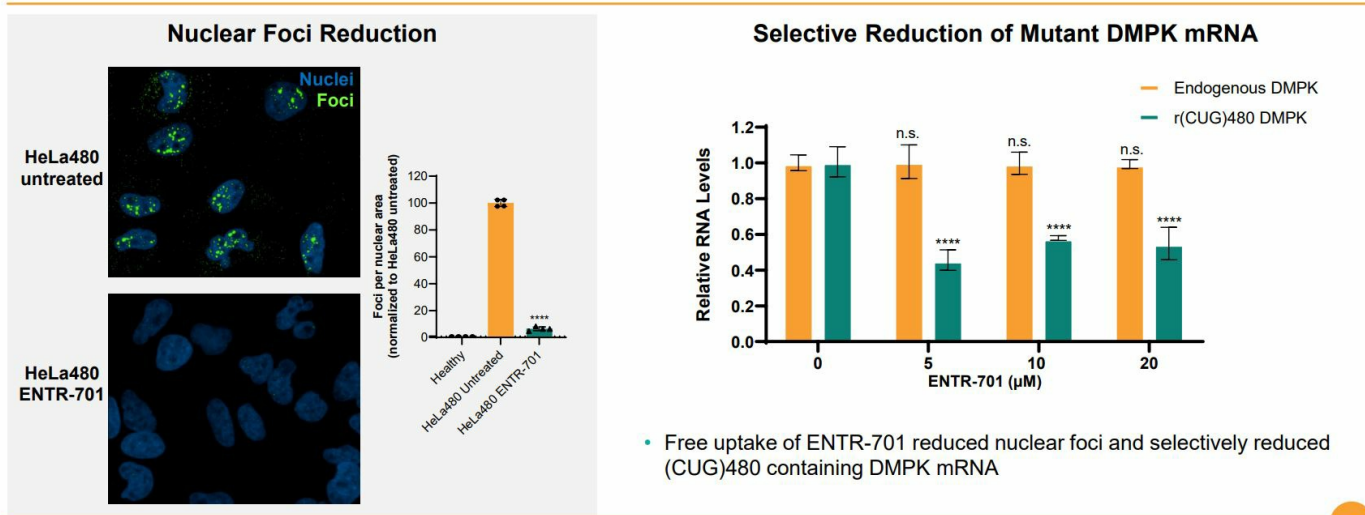
Amy Brown

This year has not seen many really chunky licensing deals, but [the closing days of the year are proving fruitful](#). Today brings news of a \$224m up-front payment by Vertex for Entrada's myotonic dystrophy type 1 (DM1) project, ENTR-701 - a healthy sum considering work remains preclinical. Nothing is available for the progressive muscle wasting disease, which, as Stifel analysts describe it, represents one of remaining "white spaces" in the orphan field. DM1 is more prevalent than cystic fibrosis, on which Vertex has built a \$8bn-and-counting business. The agreement also covers a four-year research collaboration in the DM1 space, and sees Entrada also getting a \$26m equity investment from Vertex and \$485m in potential milestone payments. The small biotech's technology is based on peptide conjugates; ENTR-701 targets what is thought to be the driver of DM1, correcting the expression of toxic proteins and restoring normal muscle function. This is not the first move Vertex has made in this space - deals with [Crispr Therapeutics and Exonics in 2019](#) and [Affinia in 2020](#) all named DM1 as a focus. No clinical projects are listed in Vertex's pipeline, however, so perhaps this Entrada move is a signal that a fresh approach was required.

ENTR-701 IN DM1 MODEL CELL LINE WITH REPEAT EXPANSION



DM1 clinical candidate, ENTR-701, showed reduction of nuclear foci and selective reduction of repeat expansion-containing DMPK transcript in the HeLa480 cell line



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ENTR-701 is the clinical candidate selected for DM1, composed of CUG-repeat blocking PMO conjugated to EEV; HeLa480 cell line was constructed by integrating (CTG)₄₈₀ and (CTG)₀ containing DMPK transgenes (Reddy, K. et al. *Proc. Natl. Acad. Sci.* 2019); ****p<0.0001, n.s., not significant; shown as mean ± standard deviation.

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Source: Entrada corporate presentation.

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Evaluate HQ
44-(0)20-7377-0800

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