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ARTICLE

One target for amyotrophic lateral sclerosis therapy?

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Abstract

Repeat expansion mutations cause a range of developmental, neurodegenerative, and neuromuscular disorders. The repeat sequences generally comprise a 3- to 6-base pair repeat unit that expands above a critical threshold, leading to disease. Expanded repeats cause disease via a range of mechanisms, including loss of function of the repeat-containing protein and production of toxic repeat RNAs and proteins, making the disorders difficult to treat. In 2011, a hexanucleotide repeat expansion in the *C9orf72* gene was identified as the most common cause of frontotemporal dementia and amyotrophic lateral sclerosis (termed c9FTD/ALS) (1, 2). On page 708 of this issue, Kramer *et al.* (3) report that targeting a single factor, Spt4, reduced production of *C9orf72* repeat expansion-associated RNA and protein, and ameliorated neurodegeneration in model systems.

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