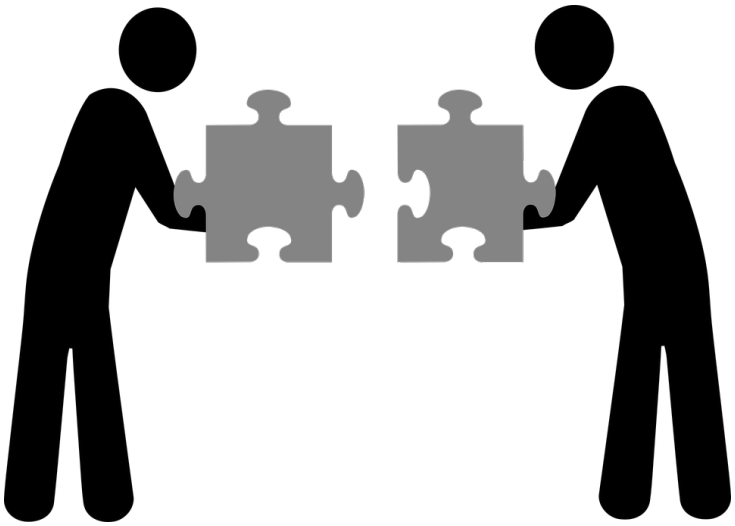
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Sangamo and Pfizer announce collaboration

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Sangamo Therapeutics, Inc. and Pfizer has announced a collaboration for the development of a potential gene therapy using zinc finger protein transcription factors (ZFP-TFs) to treat amyotrophic lateral sclerosis (ALS) and frontotemporal lobar degeneration (FTLD) linked to mutations of the C9ORF72 gene.

ALS and FTLD are part of a spectrum of neurodegenerative disorders caused by mutations in the C9ORF72 gene that involves hundreds of additional repetitions of a six base pair sequence of DNA. This ultimately leads to the deterioration of motor neurons, in the case of ALS, or neurons in the frontal and temporal lobes, in the case of FTLD.

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Dr. Sandy Macrae, Chief Executive Officer of Sangamo said, “We are excited to continue our collaborative relationship with Pfizer with this new program using Sangamo’s zinc finger protein technology to develop a potential gene therapy for patients with certain forms of ALS and FTLD, devastating diseases with very limited treatment options. The precision and flexibility of zinc finger proteins enables targeting of virtually any genetic mutation. Collaboration with the right partner for a given therapeutic application is a key component of our corporate strategy and enables us to pursue the vast opportunity set of our platform.”

Greg LaRosa, Senior Vice President and Chief Scientific Officer, Pfizer Rare Disease said, “We look forward to working with Sangamo on potential treatments for devastating diseases related to genetic mutations of the C9ORF72 gene. Pfizer is proud of the progress we have made in the area of gene therapy, which offers tremendous promise to patients and their families."

Sangamo’s ZFP-TF technology involves introducing an engineered zinc finger protein (ZFP) which is designed to identify and bind to a precise sequence of DNA. Once bound to the target sequence of DNA, a transcriptional repressor domain attached

to the ZFP suppresses expression of the gene. Under this collaboration, Sangamo and Pfizer will investigate allele-specific ZFP-TFs with the potential to differentiate the mutant C9ORF72 allele from the wild type allele and to specifically down-regulate expression of the mutant form of the gene.

Under the terms of the collaboration agreement, Sangamo will receive a $12 million upfront payment from Pfizer.

Sangamo will be responsible for the development of ZFP-TF candidates. Pfizer will be operationally and financially responsible for subsequent research, development, manufacturing and commercialization for the C9ORF72 ZFP-TF program and any resulting products. Sangamo is eligible to receive potential development and commercial milestone payments of up to $150 million, as well as tiered royalties on net sales.