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(54) Title: NON-HUMAN ANIMALS HAVING A HEXANUCLEOTIDE REPEAT EXPANSION IN A C9ORF72 LOCUS

(57) Abstract: A non-human animal (e.g., a rodent) model for diseases associated with a C9ORF72 heterologous hexanucleotide repeat expansion sequence is provided, which non-human animal comprises a heterologous hexanucleotide repeat (GGGGCC) in an endogenous C9ORF72 locus. A non-human animal disclosed herein comprising a heterologous hexanucleotide repeat expansion sequence comprising at least one instance, e.g., repeat, of a hexanucleotide (GGGGCC) sequence may further exhibit a characteristic and/or phenotype associated with one or more neurodegenerative disorders (e.g., amyotrophic lateral sclerosis (ALS) and/or frontotemporal dementia (FTD), etc.). Methods of identifying therapeutic candidates that may be used to prevent, delay or treat one or more neurodegenerative (e.g., amyotrophic lateral sclerosis (ALS, also referred to as Lou Gehrig's disease) and frontotemporal dementia (FTD)) are also provided.