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(54) Title: NUCLEASE-MEDIATED REPEAT EXPANSION

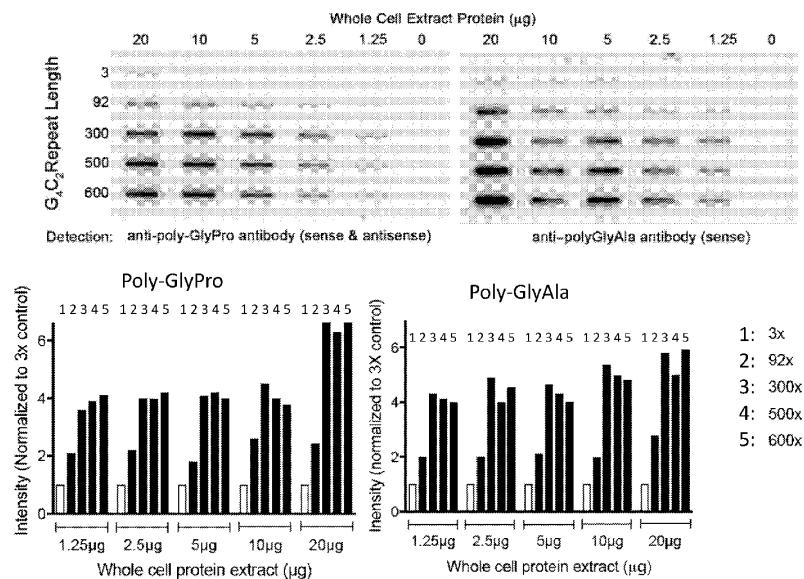


FIG. 21

(57) Abstract: Nuclease-mediated methods for expanding repeats already present at a genomic locus are provided. Non-human animal genomes, non-human animal cells, and non-human animals comprising a heterologous hexanucleotide repeat expansion sequence inserted at an endogenous *C9orf72* locus and methods of making such non-human animal cells and non-human animals through nuclease-mediated repeat expansion are also provided. Methods of using the non-human animal cells or non-human animals to identify therapeutic candidates that may be used to prevent, delay or treat one or more neurodegenerative disorders associated with repeat expansion at the *C9orf72* locus are also provided.

[Continued on next page]

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Declarations under Rule 4.17:

- *as to the identity of the inventor (Rule 4.17(i))*
- *as to applicant's entitlement to apply for and be granted a patent (Rule 4.17(ii))*
- *as to the applicant's entitlement to claim the priority of the earlier application (Rule 4.17(iii))*

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