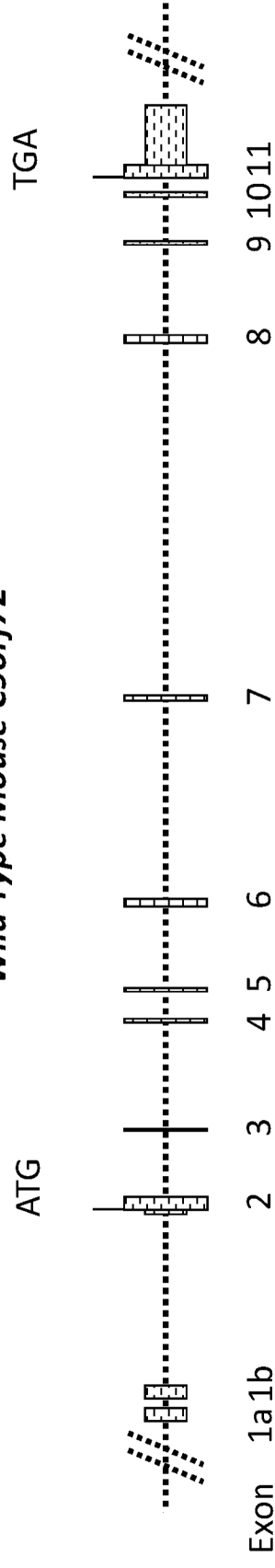
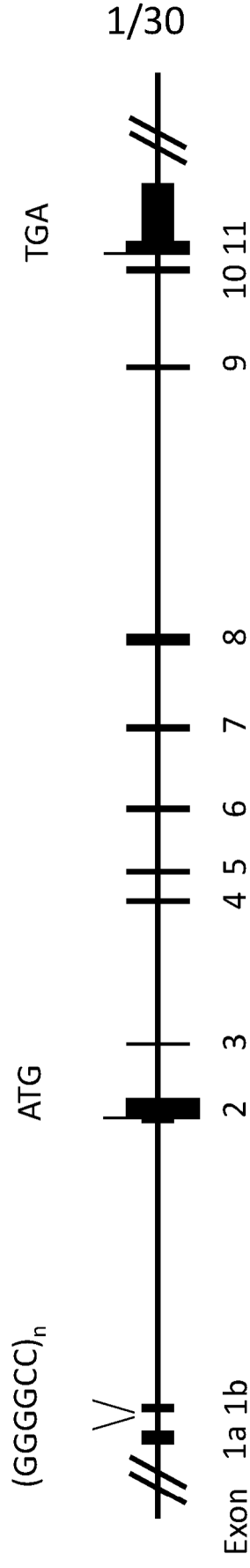


Wild Type Mouse C9orf72



Wild Type Human C9ORF72



Humanized Mouse C9orf72

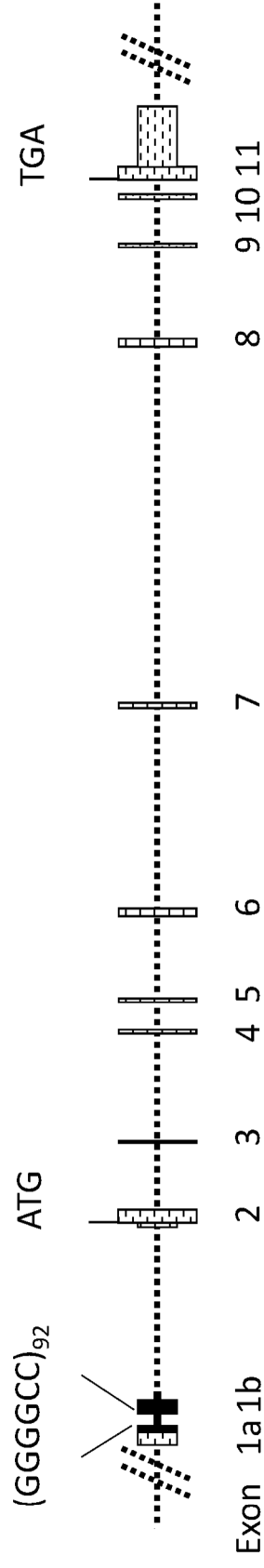


FIG. 1

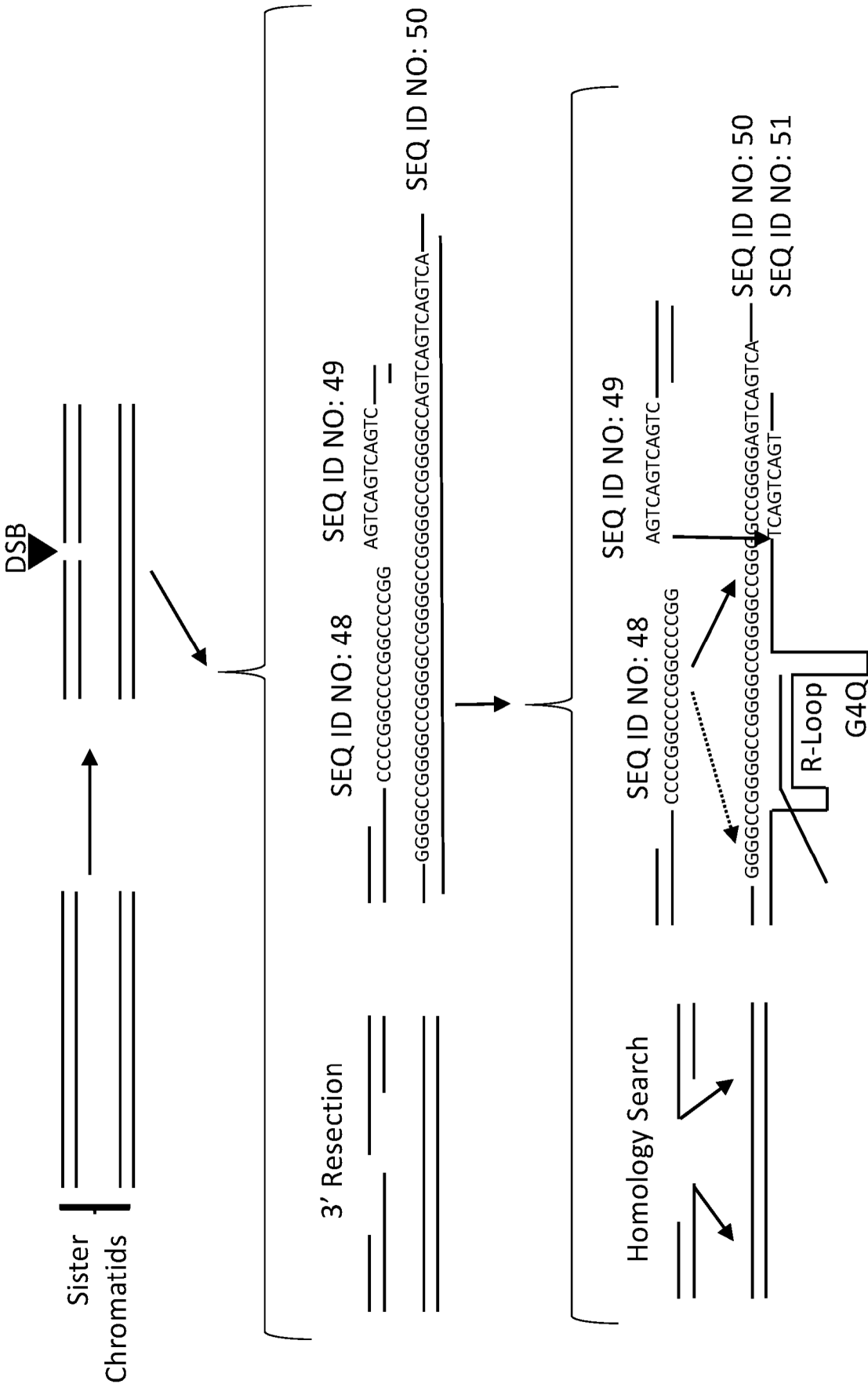


FIG. 2

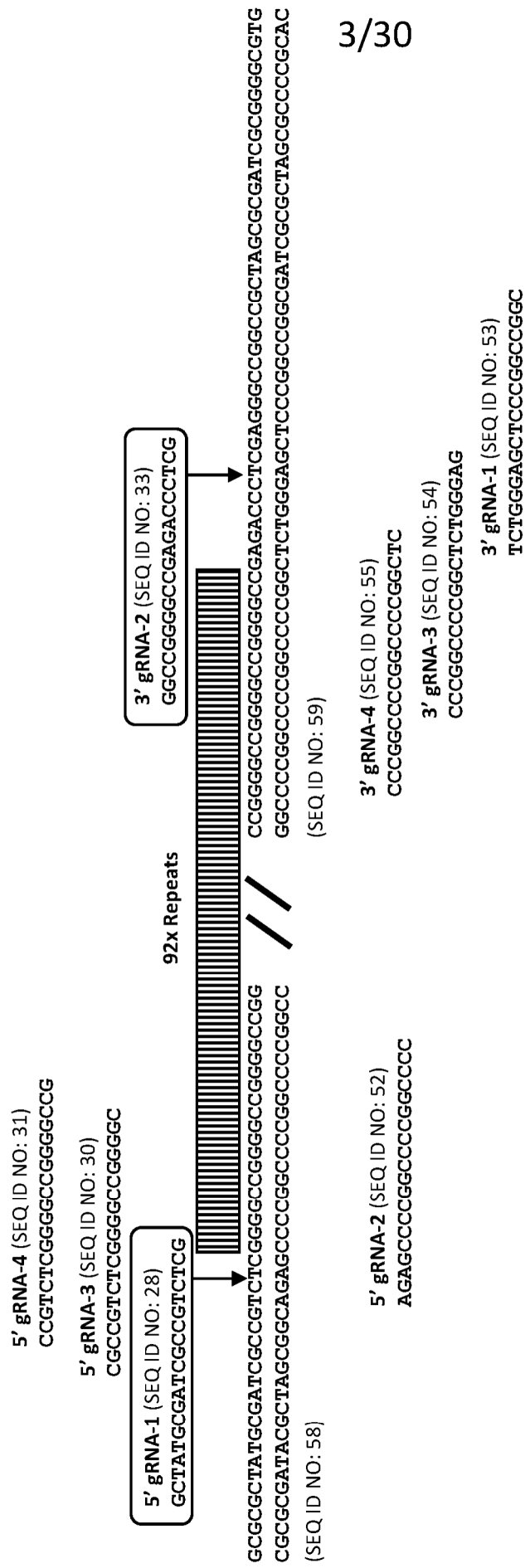


FIG. 3

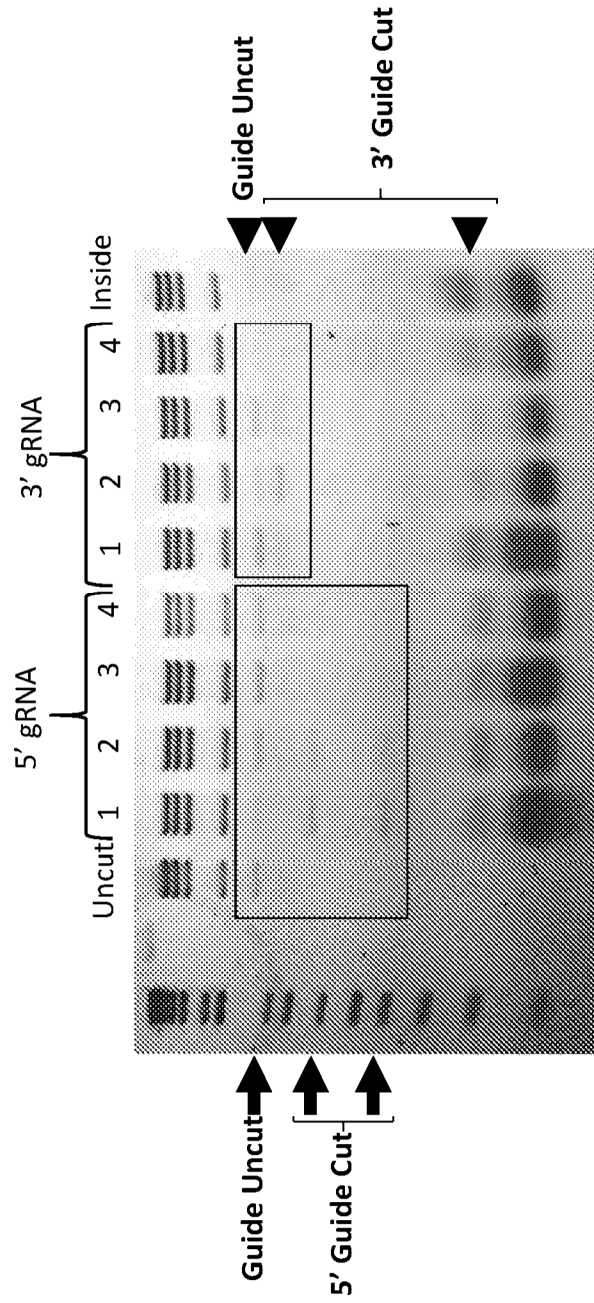


FIG. 4

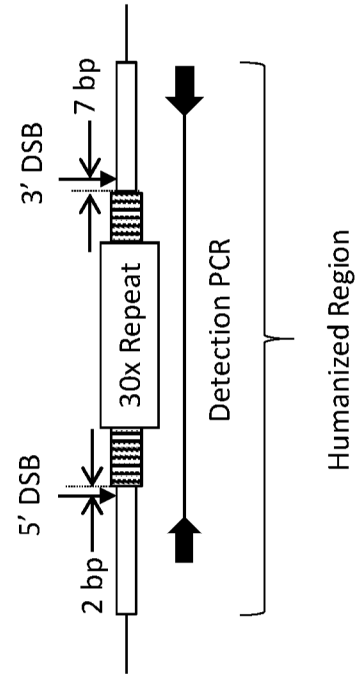


FIG. 5

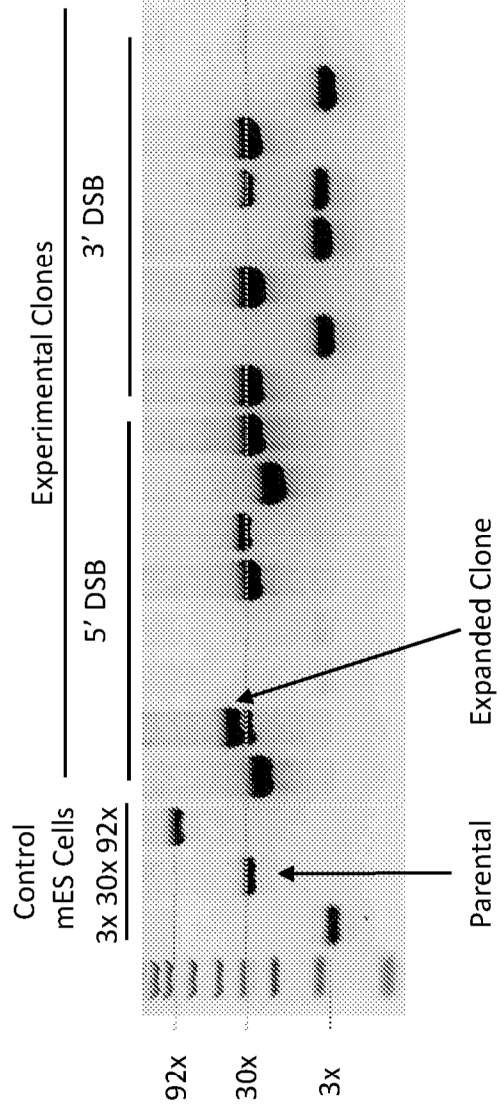


FIG. 6A

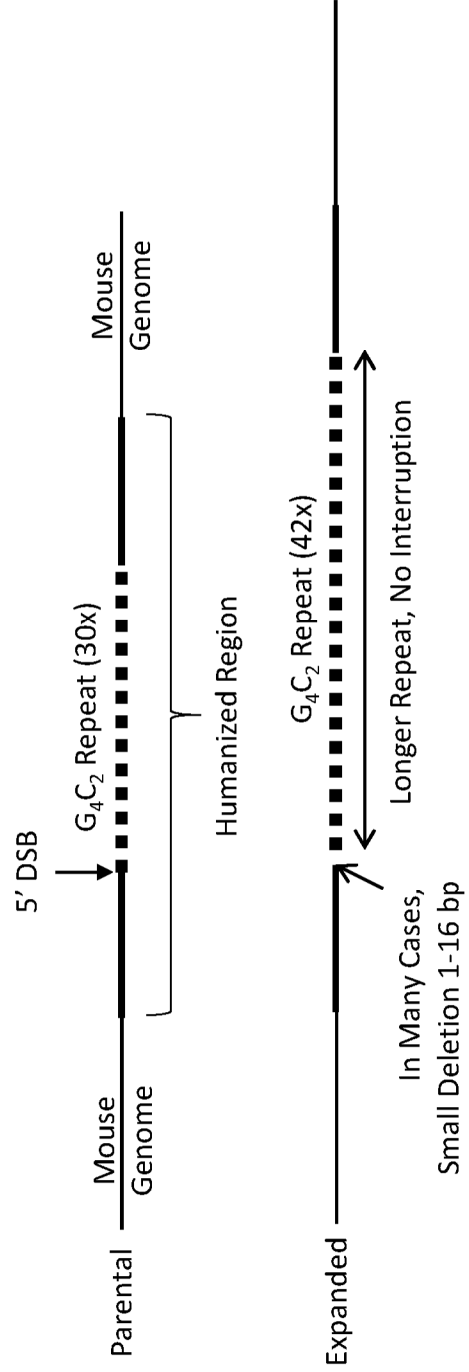


FIG. 6B

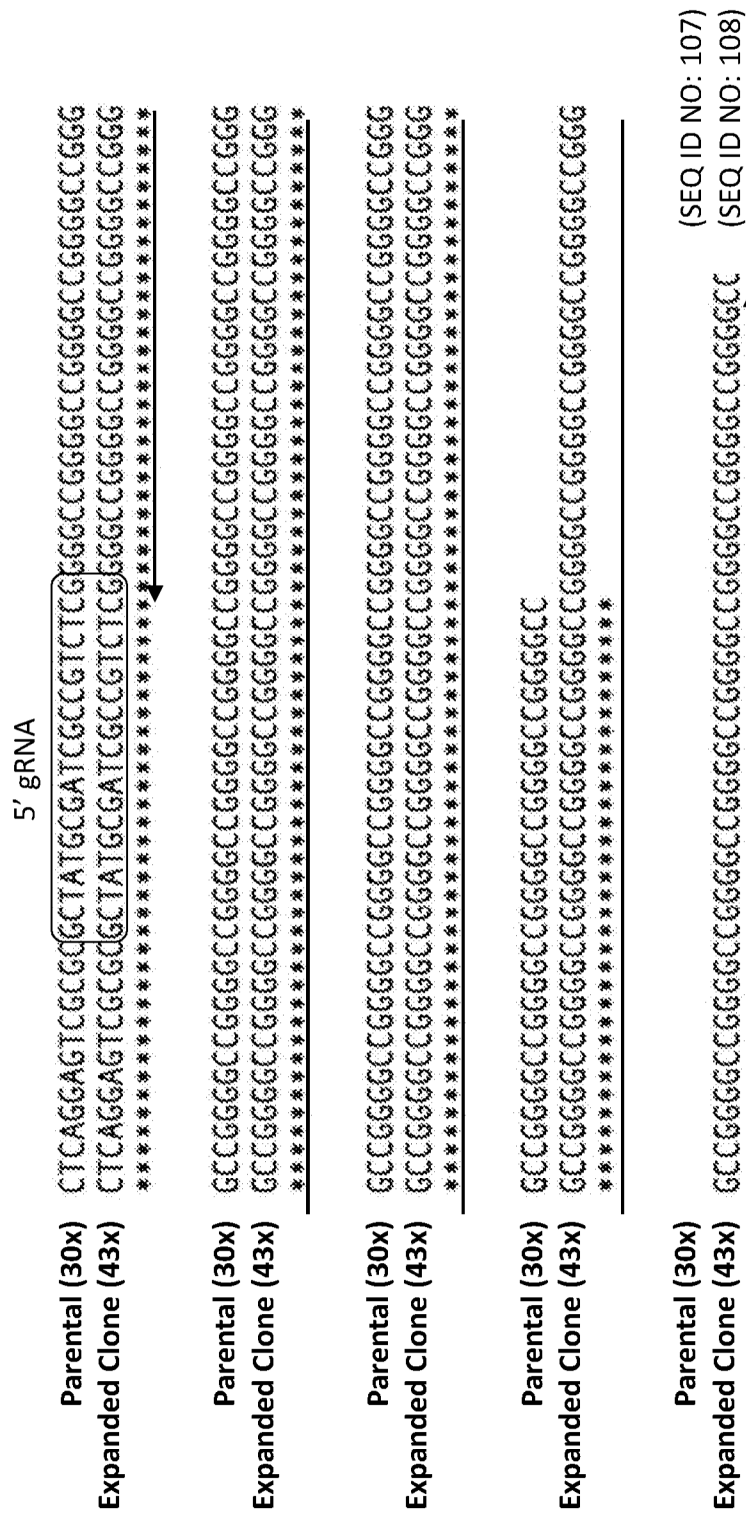


FIG. 6C

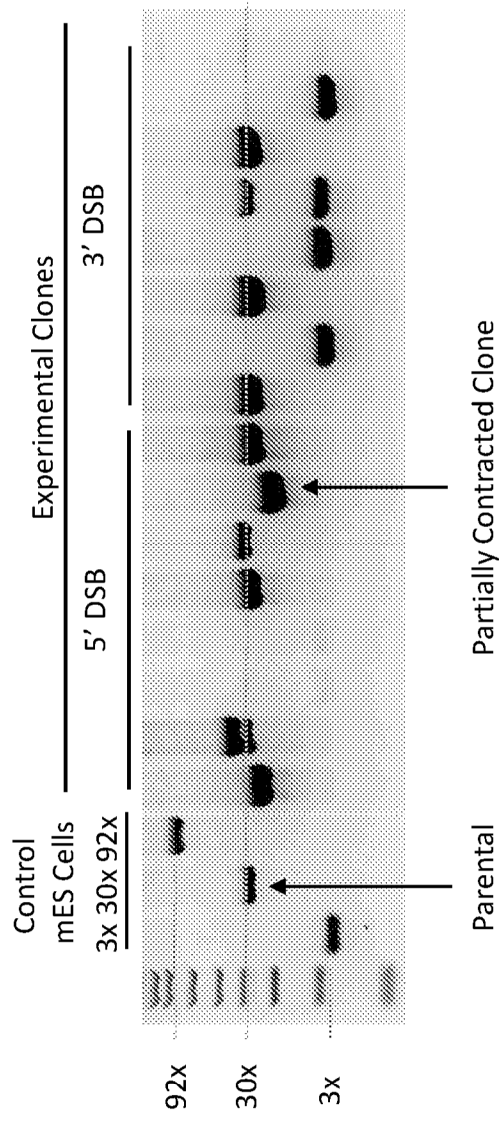


FIG. 7A

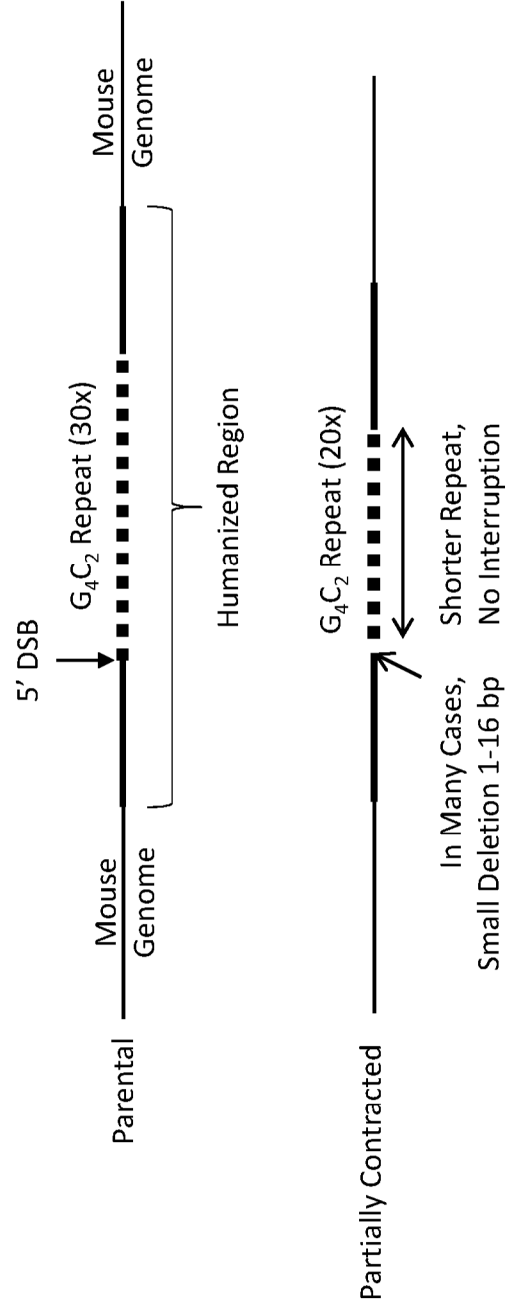


FIG. 7B

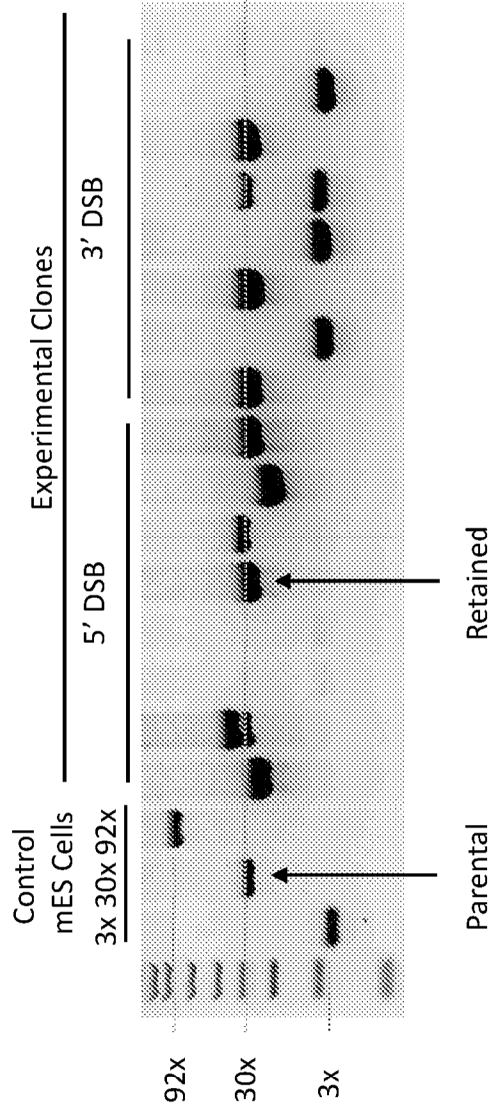


FIG. 8A

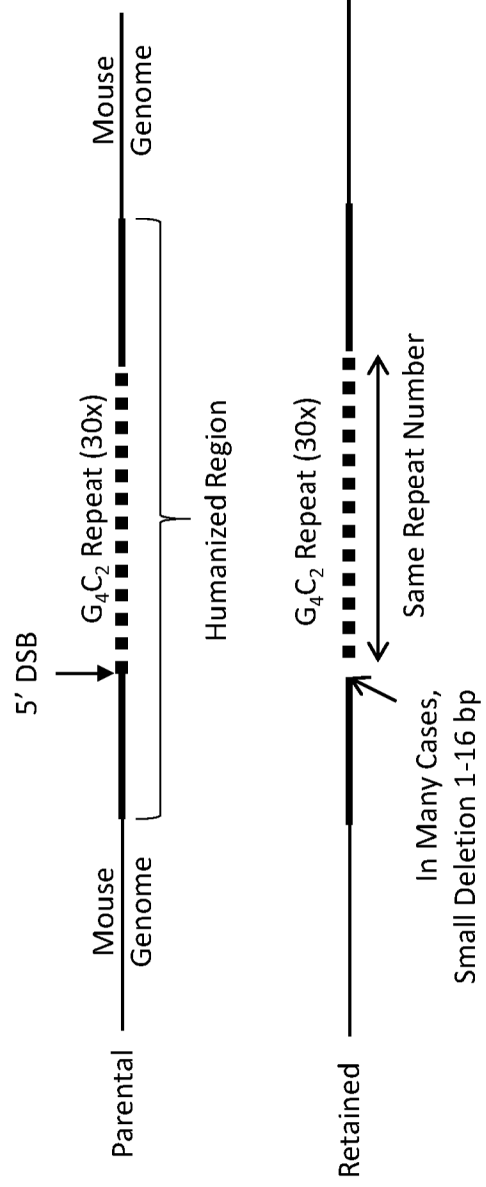


FIG. 8B

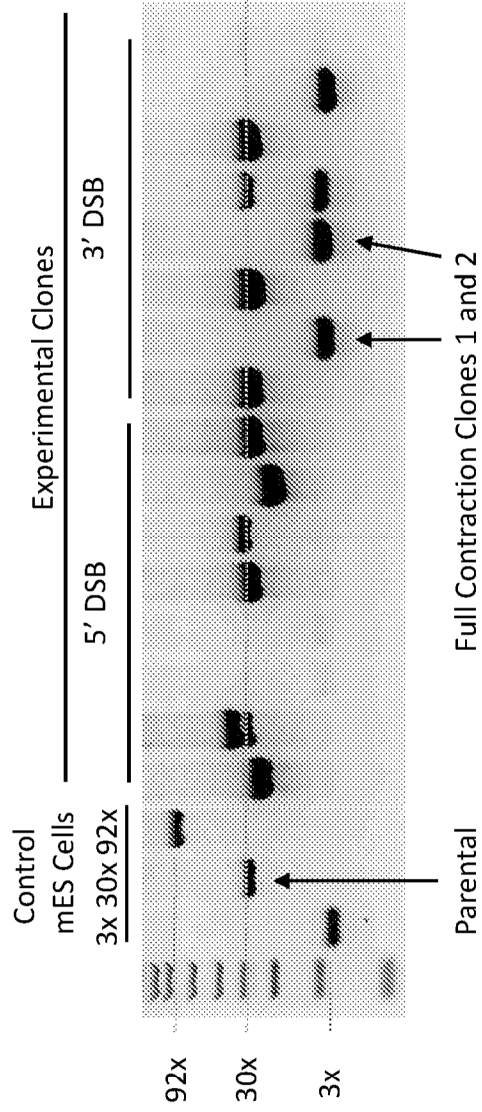


FIG. 9A

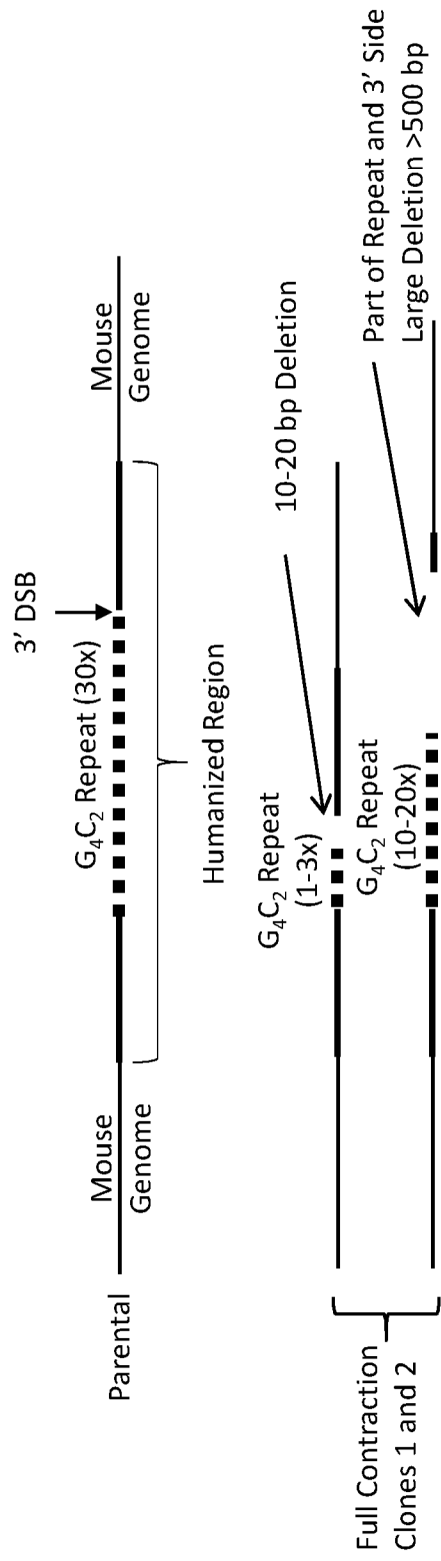


FIG. 9B

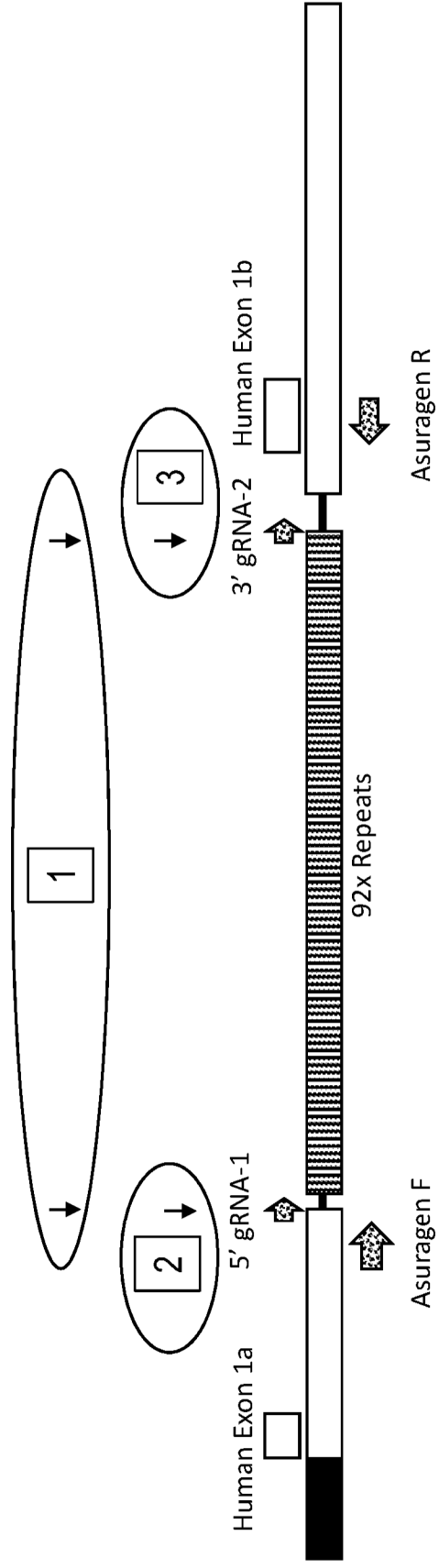
**FIG. 10**

FIG. 11A

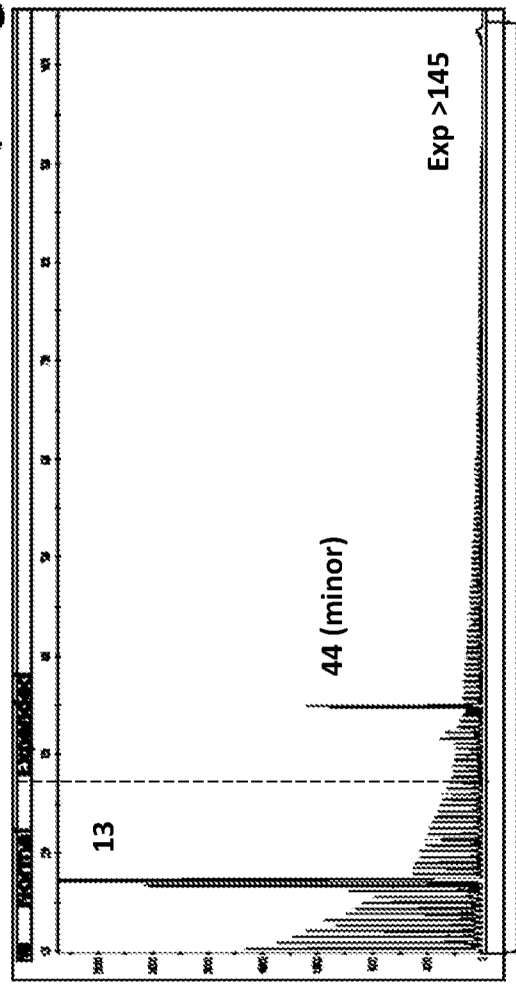
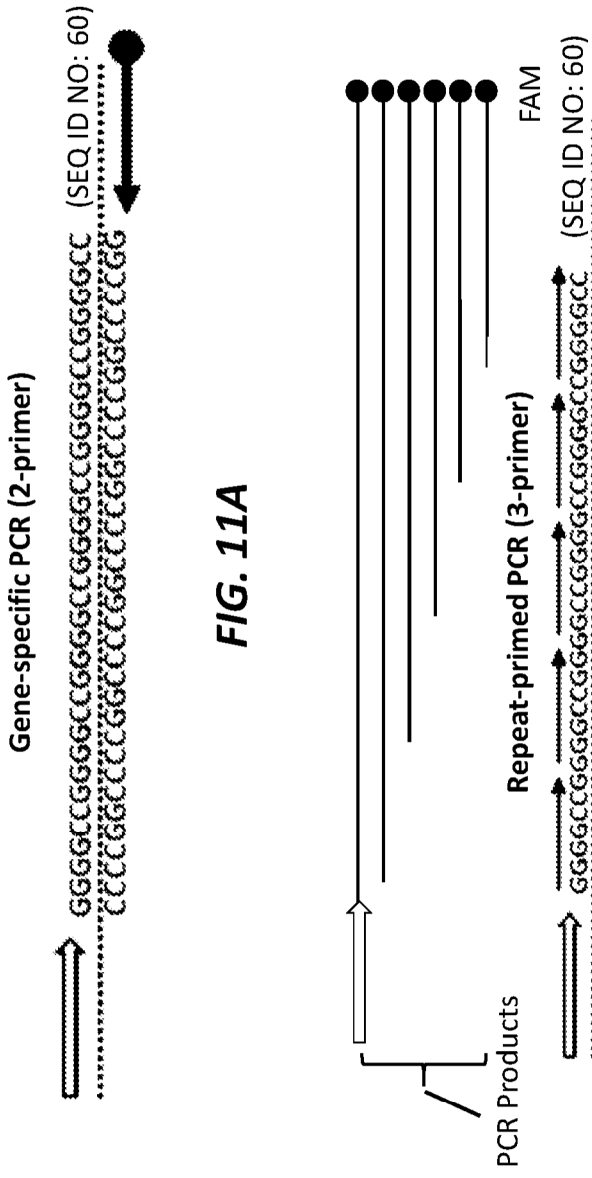


FIG. 11B

Repeat primed peaks

2 5' Single DSB

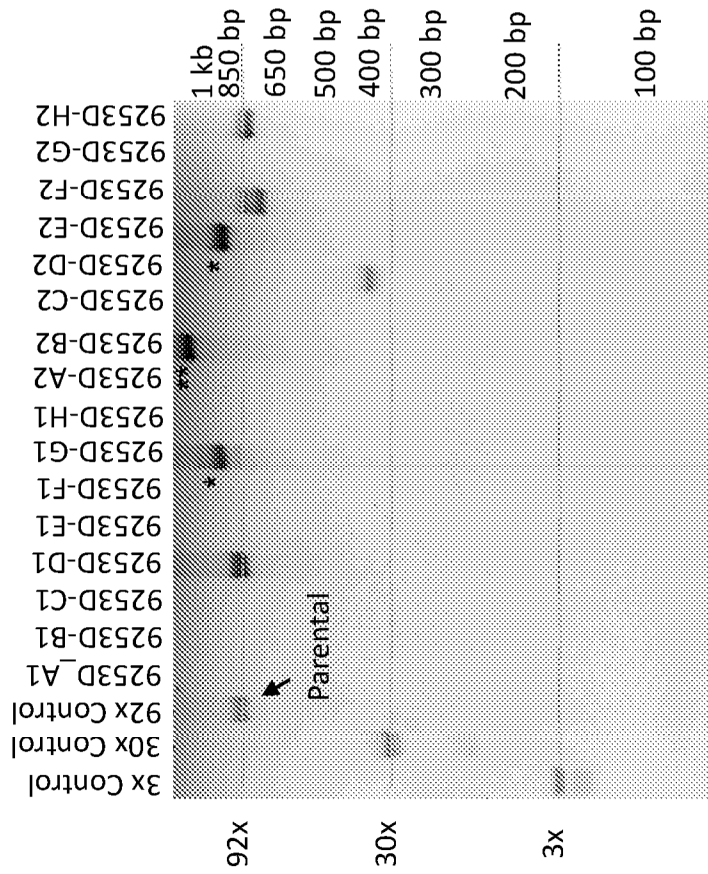


FIG. 12A

3 3' Single DSB

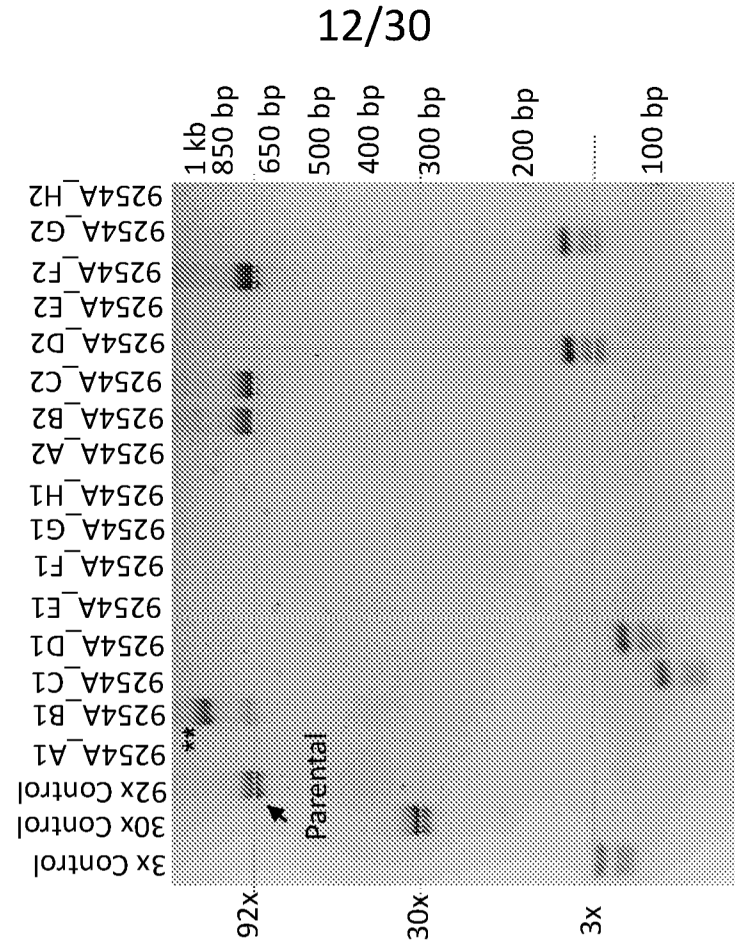


FIG. 12B

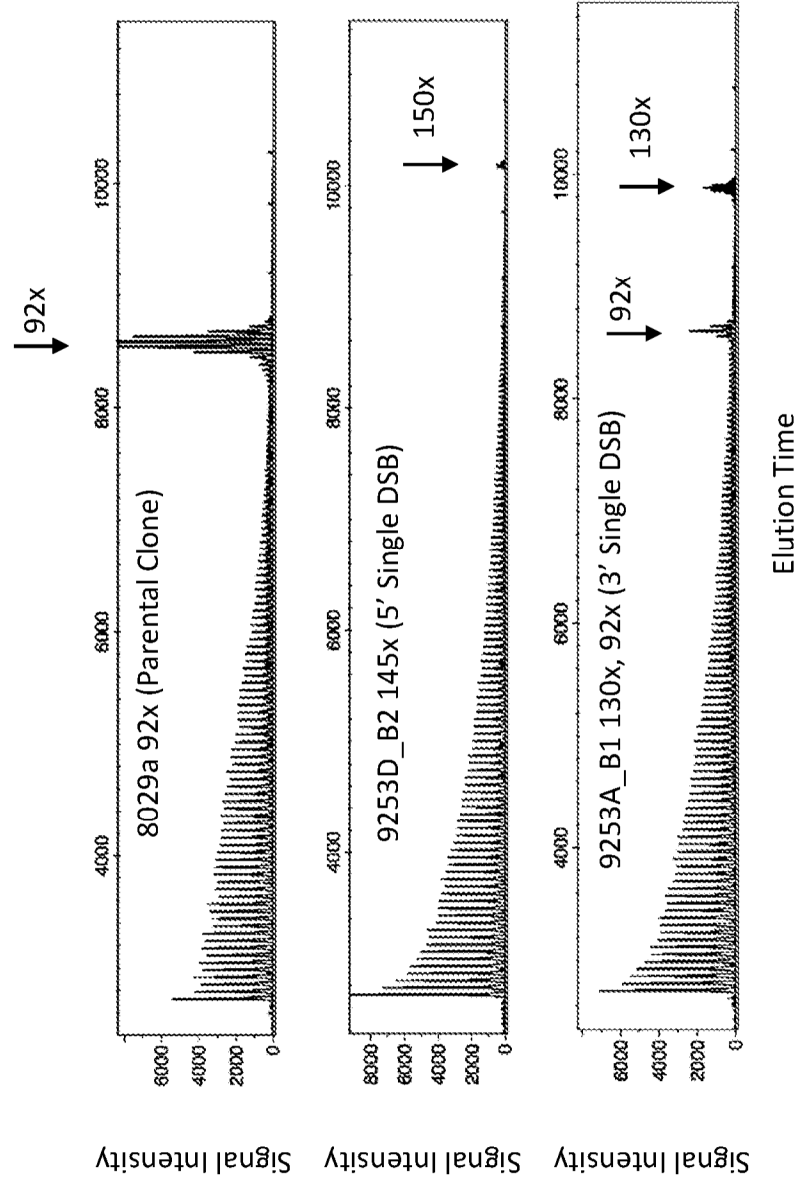


FIG. 13

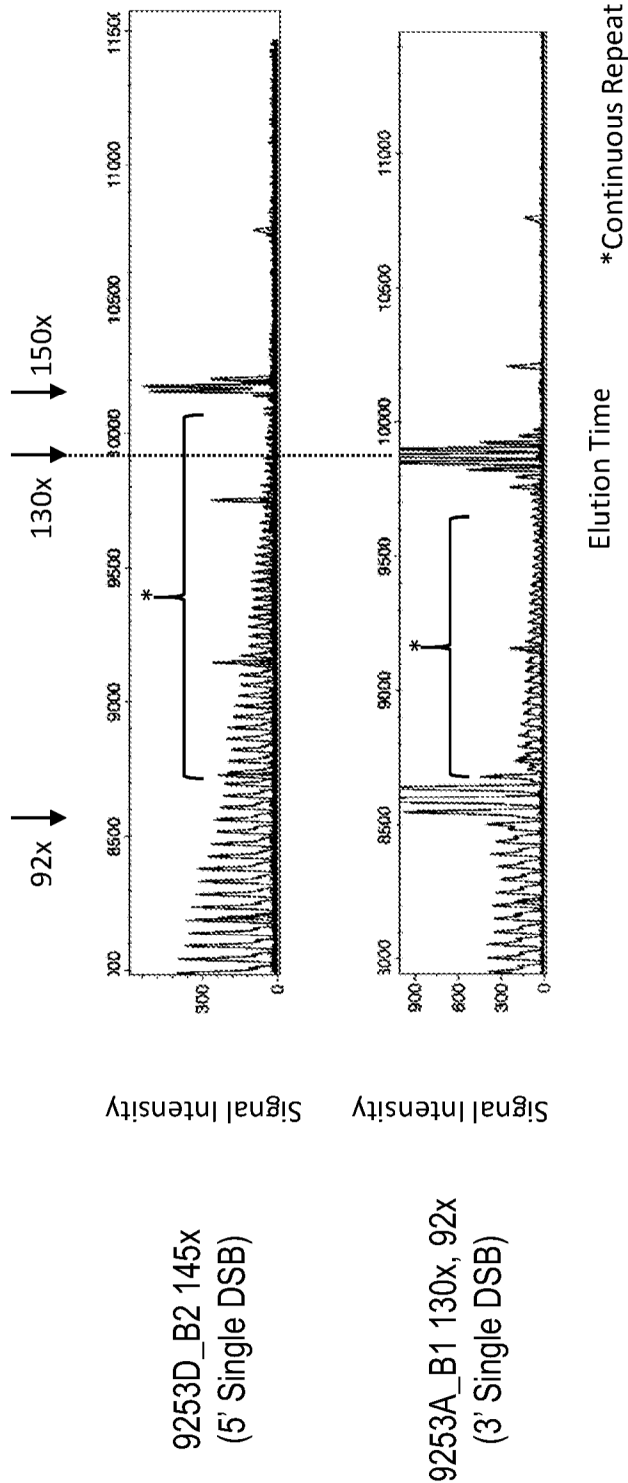


FIG. 14

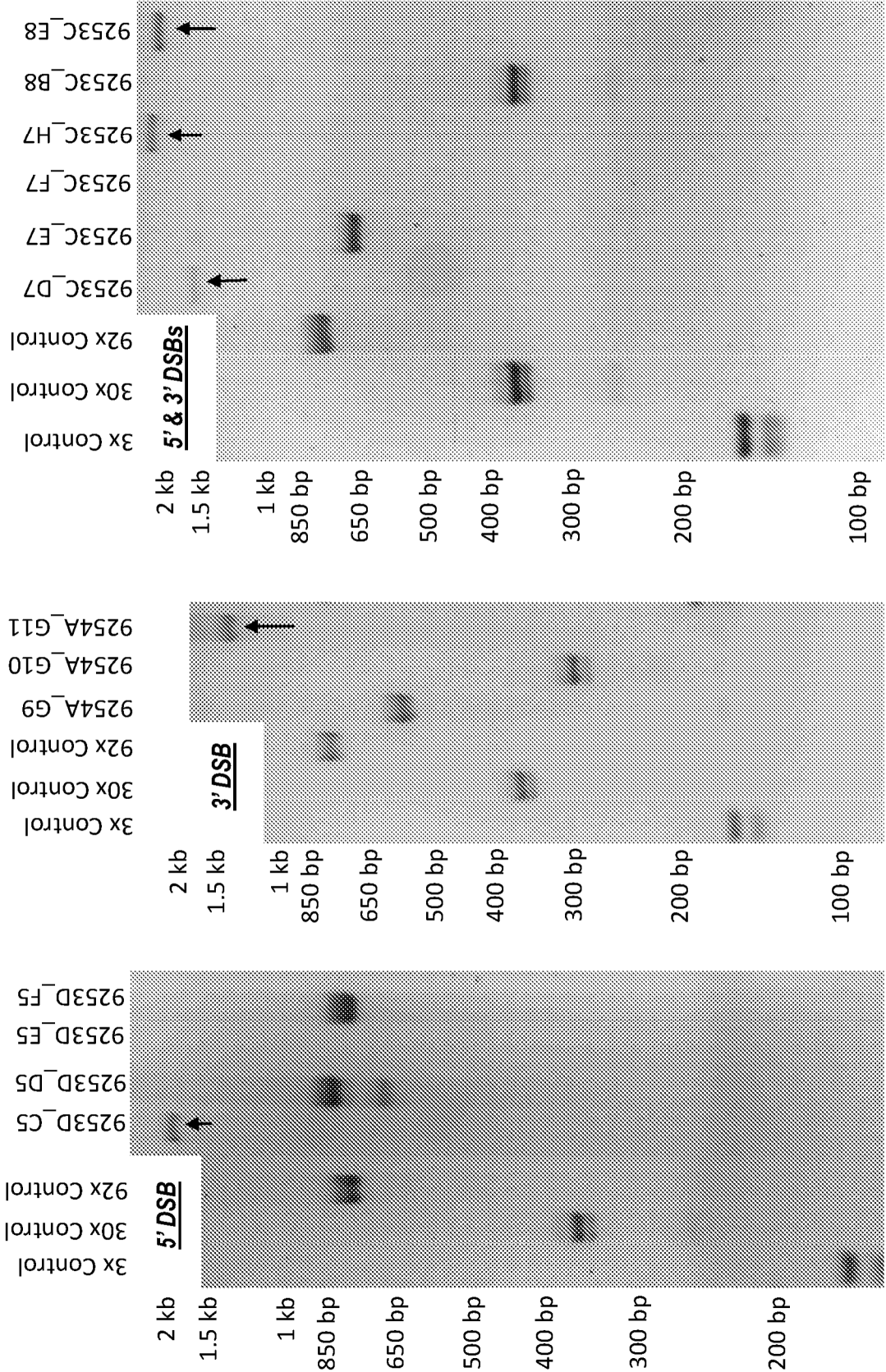


FIG. 15

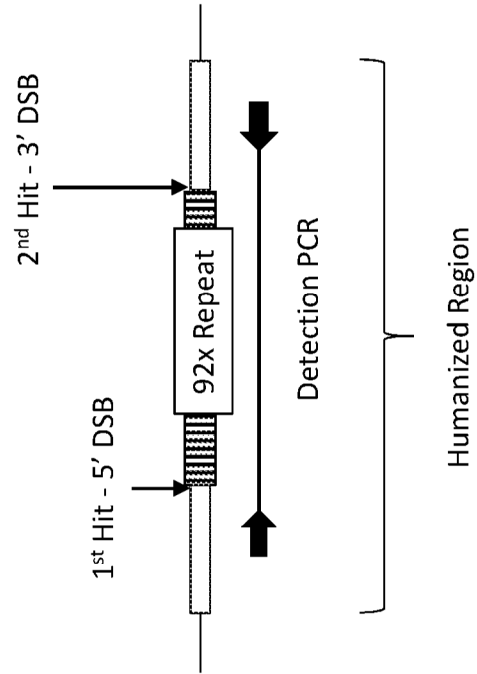


FIG. 16A

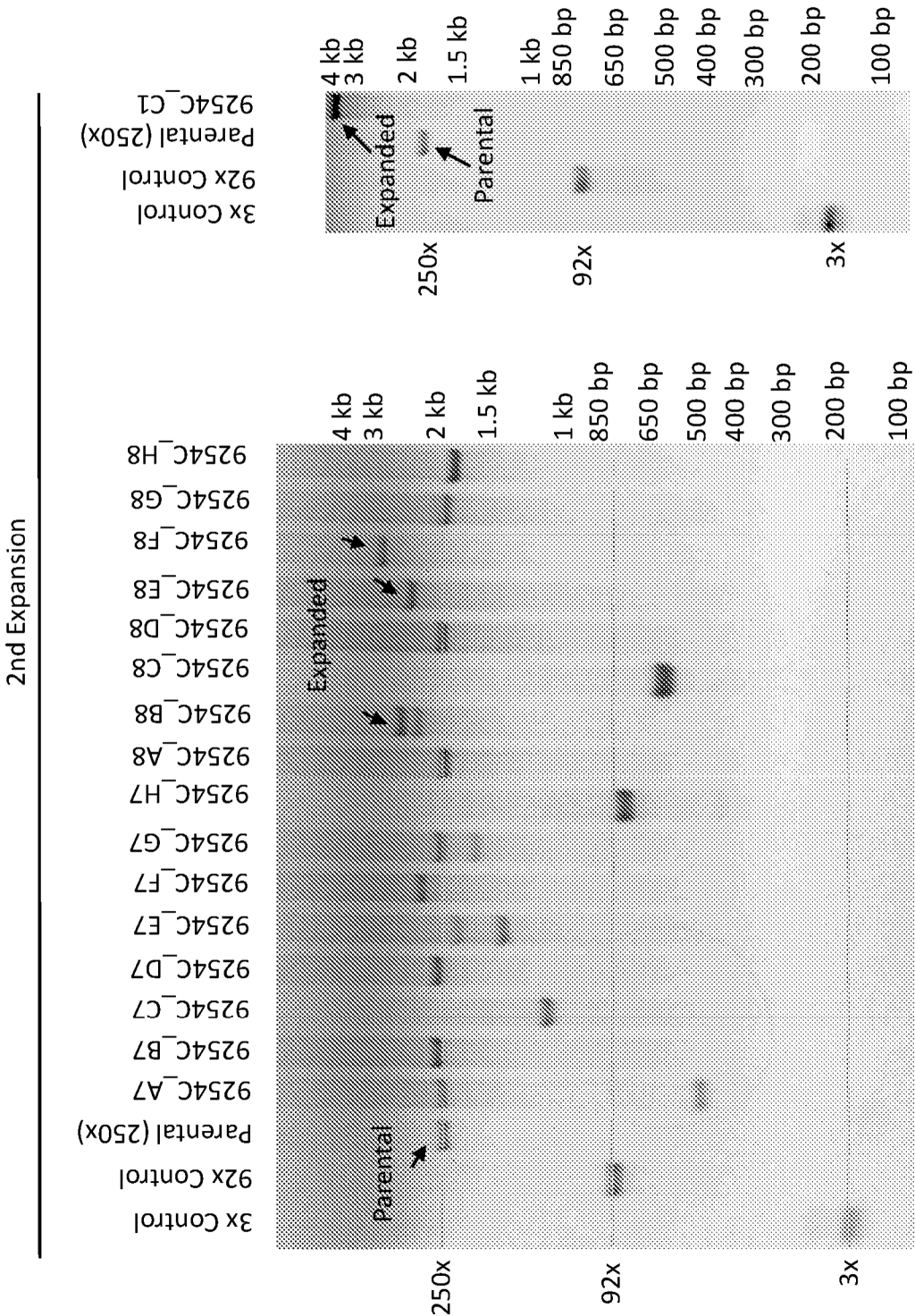


FIG. 16B

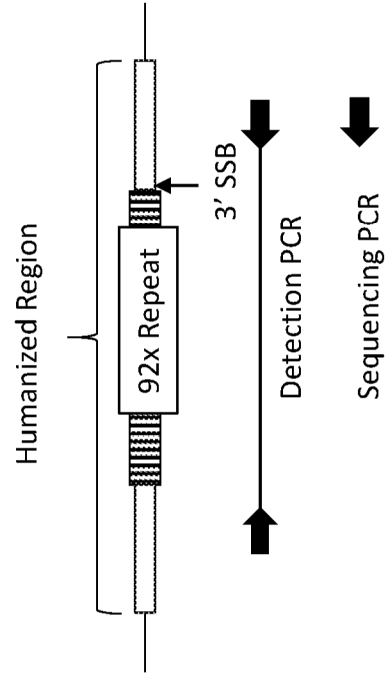


FIG. 17A

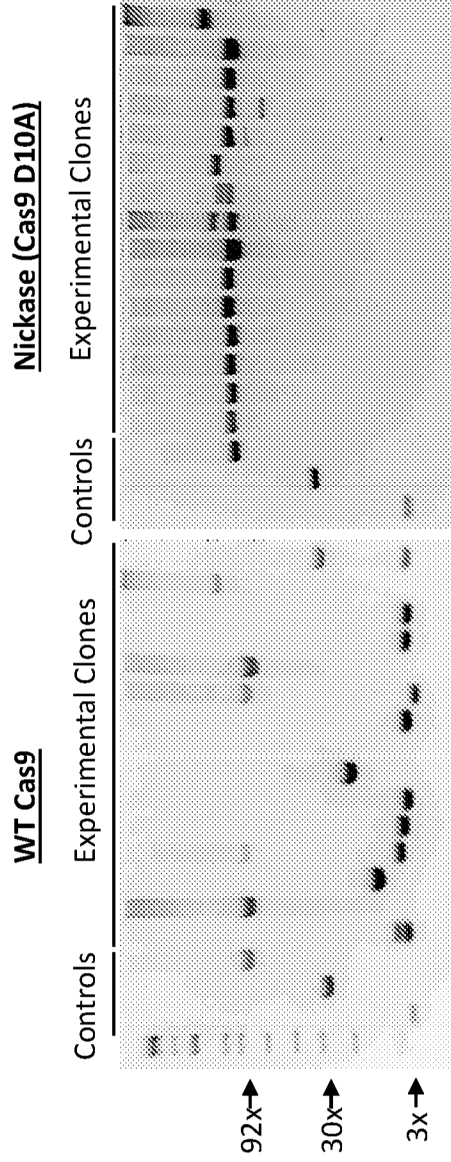


FIG. 17B

WT Cas9

Expanded Clones [GGGCCGGGGCCGGGGCCGGGGCCGGGGCCGGAGACCCCTCGAGGGCCGGCCCGCTAGCGCGATCGCG (SEQ ID NO: 109)
GGCCCGGGGGCCGGGGCCGGGGCCGGGGCCGGAGACC-TCGAGGGCCGGCCCGCTAGCGCGATCGCG (SEQ ID NO: 110)
GGCCCGGGGGCCGGGGCCGGGGCCGGGGCCGGAGAC-----GAGGGCCGGCCCGCTAGCGCGATCGCG (SEQ ID NO: 111)
GGCCCGGGGGCCGGGGCCGGGGCCGGGGCCGGAGACCC--GAGGGCCGGCCCGCTAGCGCGATCGCG (SEQ ID NO: 112)
GGCCCGGGGGCCGGGGCCGGGGCCGGGGCCGGGGCC--G-GGGCCGGCCCGCTAGCGCGATCGCG (SEQ ID NO: 113)
GGCCCGGGGGCCGGGGCCGGGGCCGGGGCCGGAGACCCCTCGAGGGCCGGCCCGCTAGCGCGATCGCG (SEQ ID NO: 109)
-----GGCCGGGGCCGGAGACCCCTCGAGG----- (SEQ ID NO: 114)

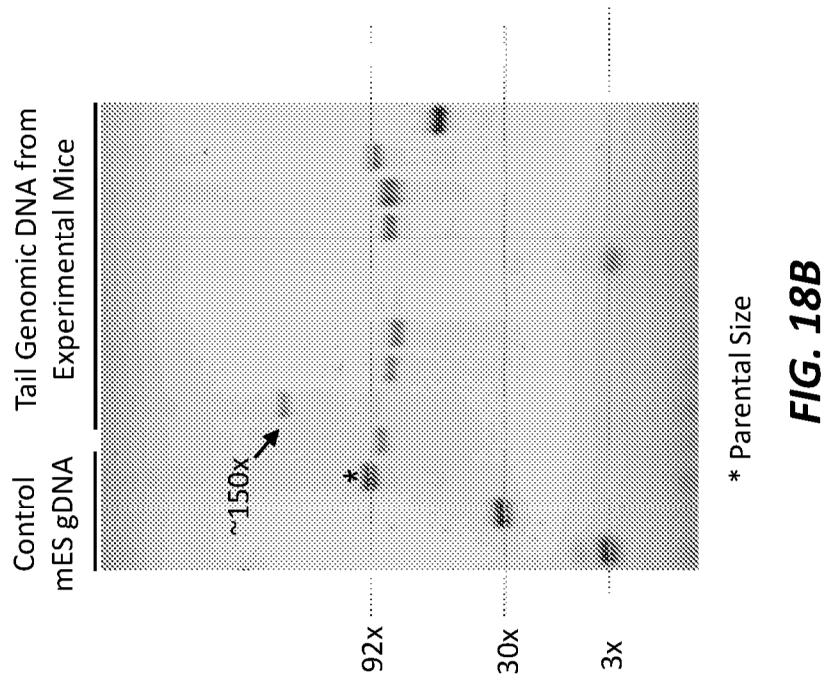
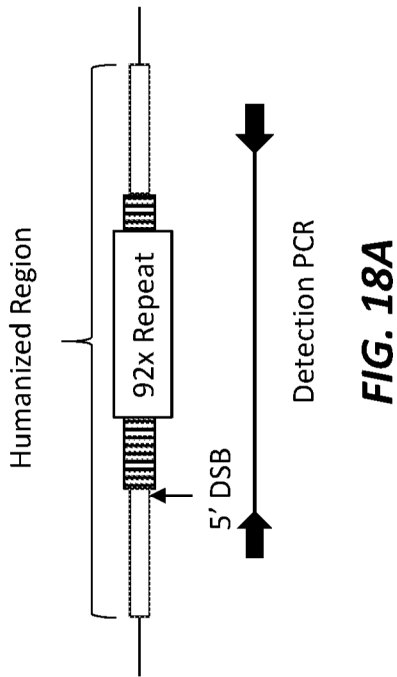
gRNA Target Sequence + PAM

Cas9 Nickase (D10A)

Expanded Clones [GCCGGGGCCGGGGCCGGGGCCGGGGCCGGCCGCTAGCGCGATCGCGGGGCGGTGGTCGGGGCGGG (SEQ ID NO: 115)
GCCGGGGCCGGGGCCGGGGCCGGGGCCGGCCGCTAGCGCGATCGCGGGGCGGTGGTCGGGGCGGG (SEQ ID NO: 115)
GCCGGGGCCGGGGCCGGGGCCGGGGCCGGCCGCTAGCGCGATCGCGGGGCGGTGGTCGGGGCGGG (SEQ ID NO: 115)
GCCGGGGCCGGGGCCGGGGCCGGGGCCGGCCGCTAGCGCGATCGCGGGGCGGTGGTCGGGGCGGG (SEQ ID NO: 115)
GCCGGGGCCGGGGCCGGGGCCGGGGCCGGCCGCTAGCGCGATCGCGGGGCGGTGGTCGGGGCGGG (SEQ ID NO: 115)
GCCGGGGCCGGGGCCGGGGCCGGGGCCGGCCGCTAGCGCGATCGCGGGGCGGTGGTCGGGGCGGG (SEQ ID NO: 115)
GCCGGGGCCGGGGCCGGGGCCGGGGCCGGCCGCTAGCGCGATCGCGGGGCGGTGGTCGGGGCGGG (SEQ ID NO: 115)
GCCGGGGCCGGGGCCGGGGCCGGGGCCGGCCGCTAGCGCGATCGCGGGGCGGTGGTCGGGGCGGG (SEQ ID NO: 115)
-----GGCCGGGGCCGGAGACCCCTCGAGG----- (SEQ ID NO: 114)

gRNA Target Sequence + PAM

FIG. 17C



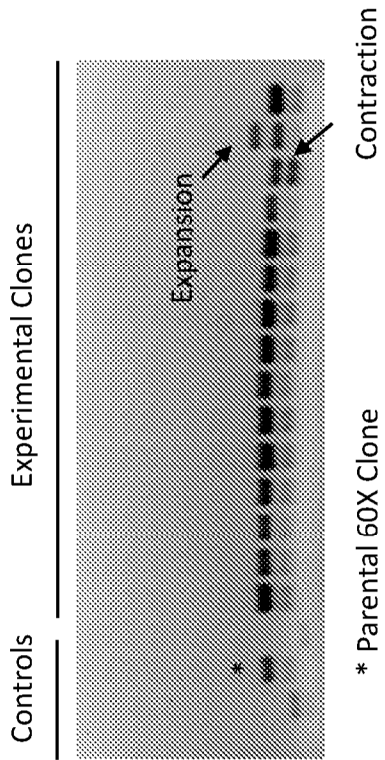
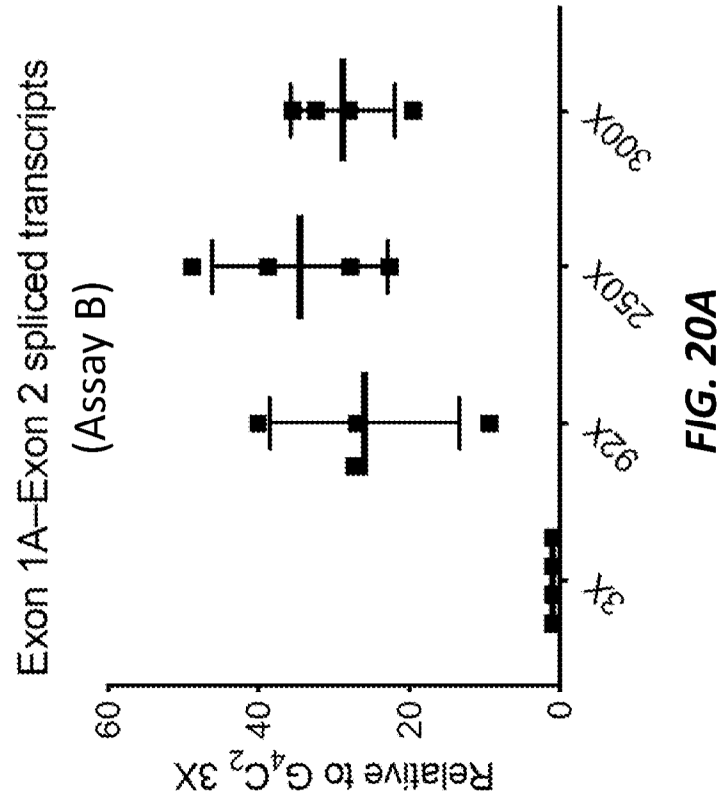
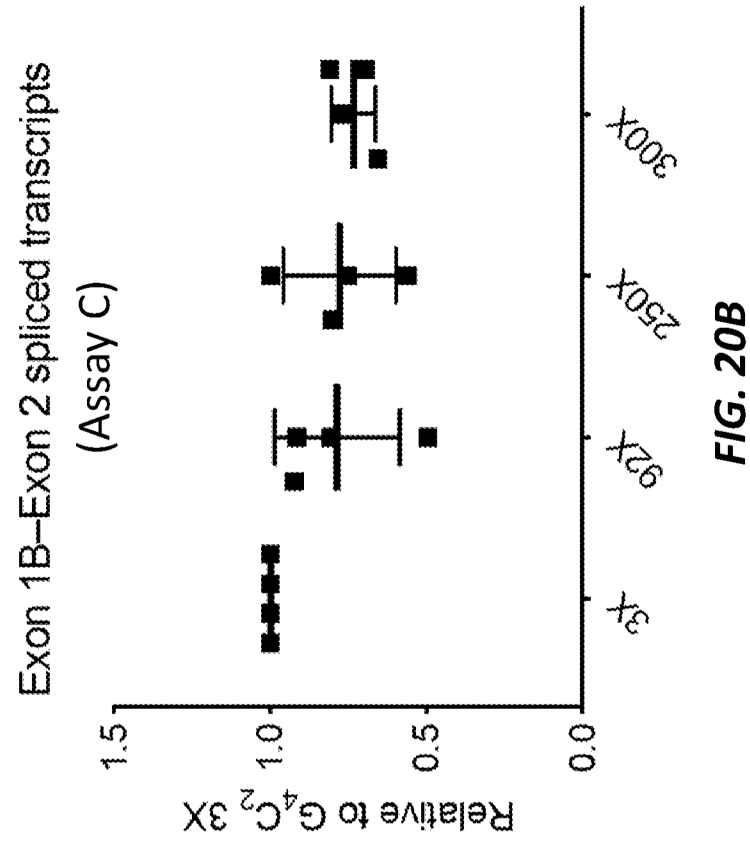


FIG. 19



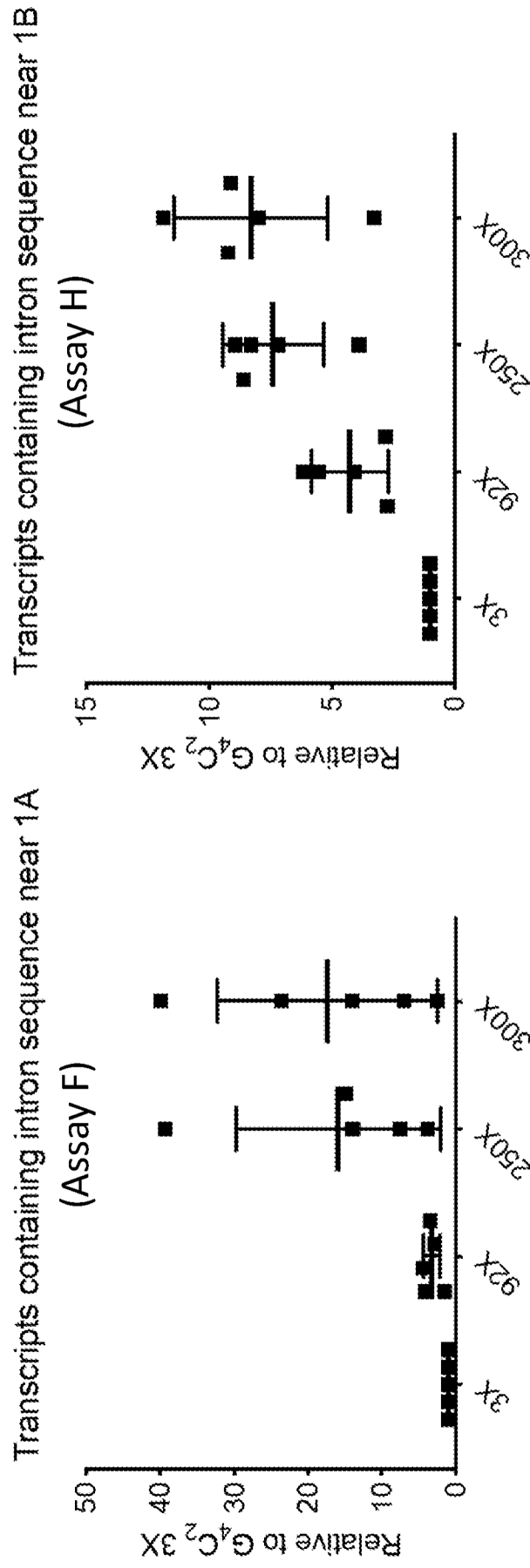


FIG. 20D

FIG. 20C

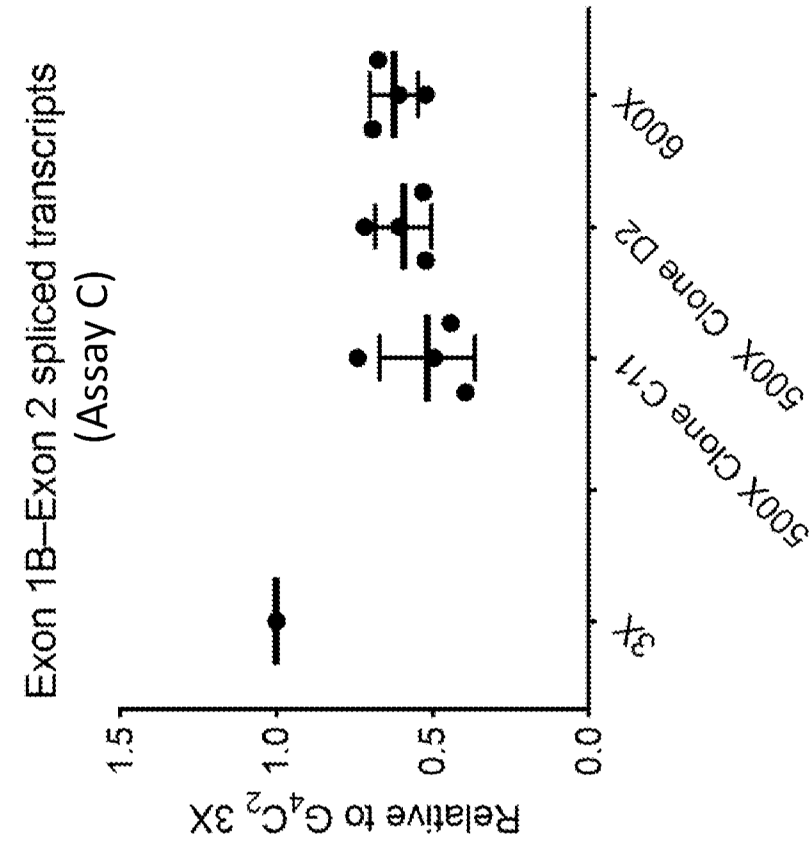


FIG. 20F

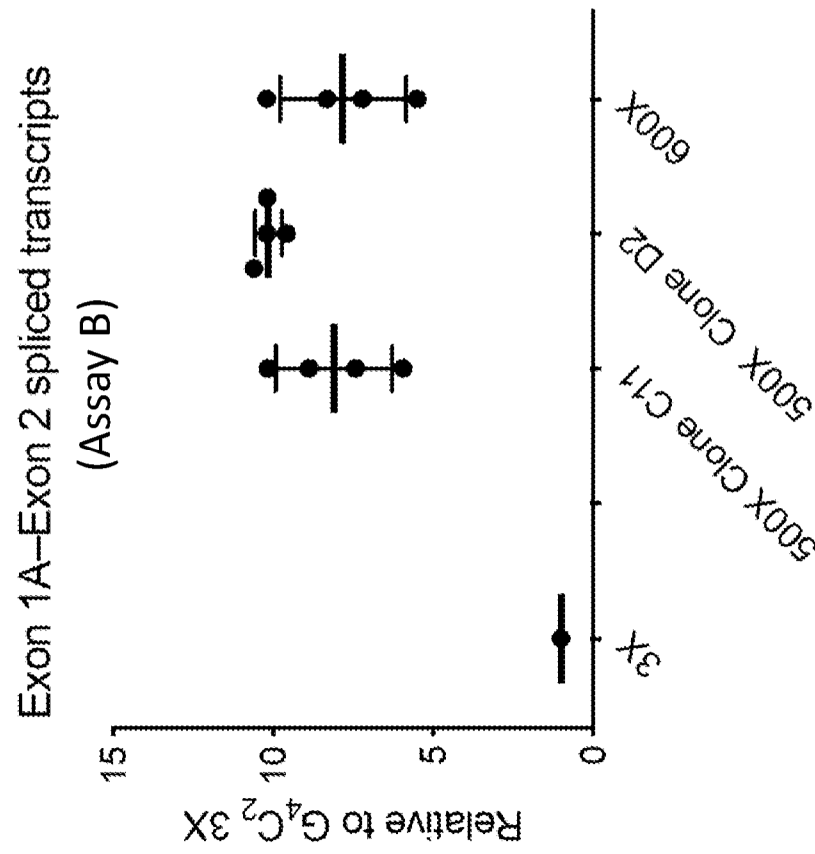


FIG. 20E

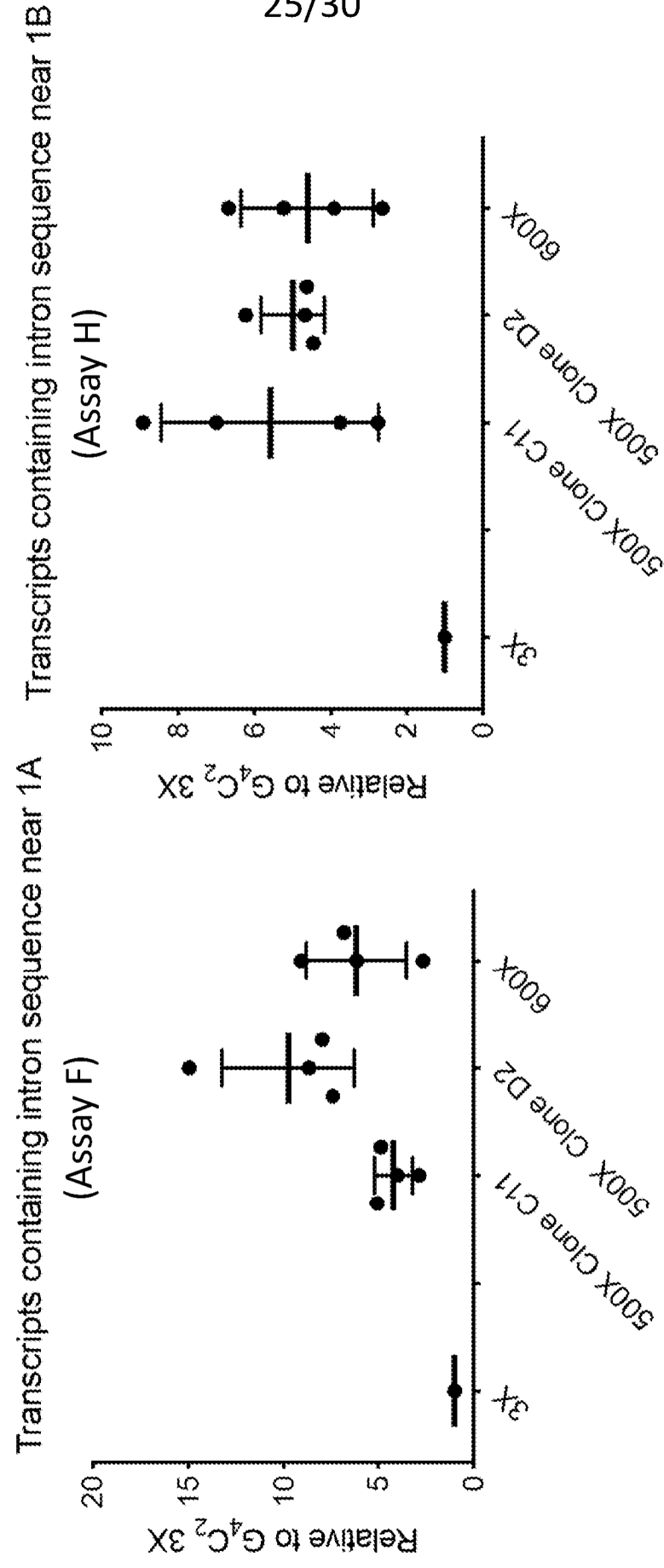


FIG. 20G

FIG. 20H

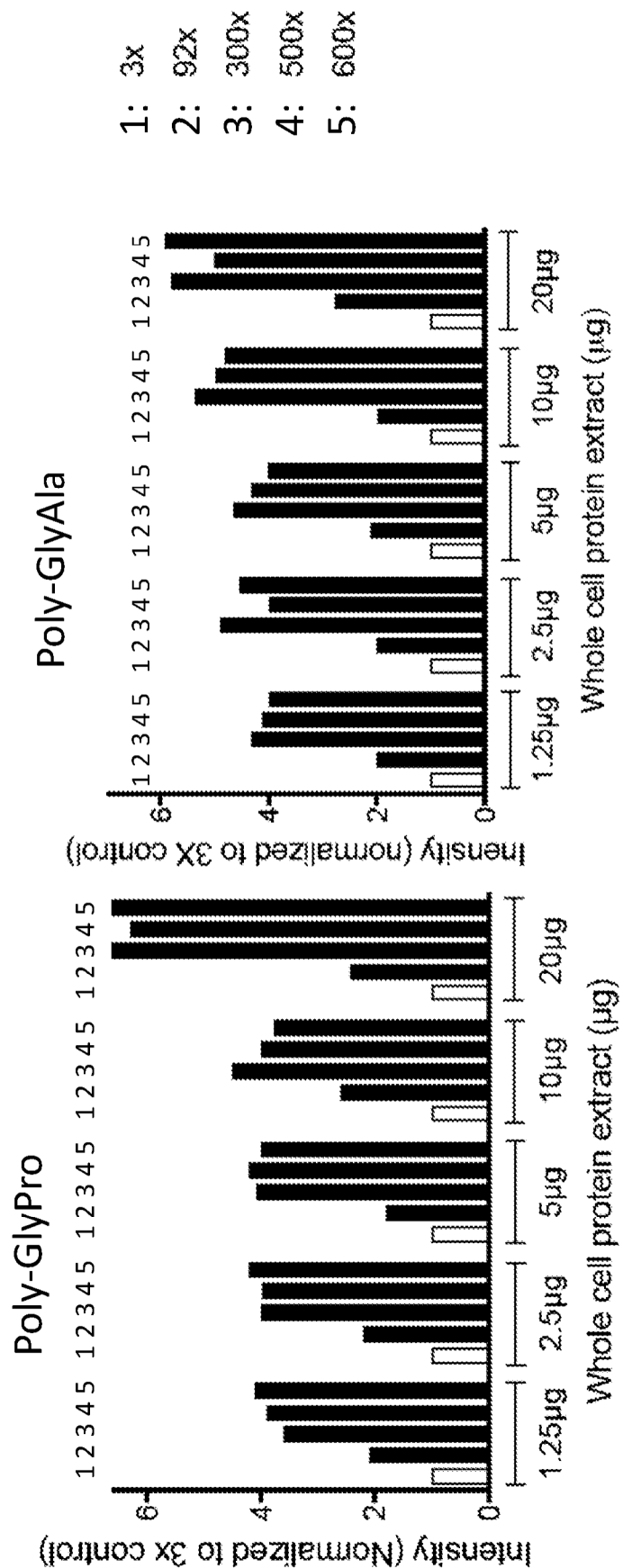
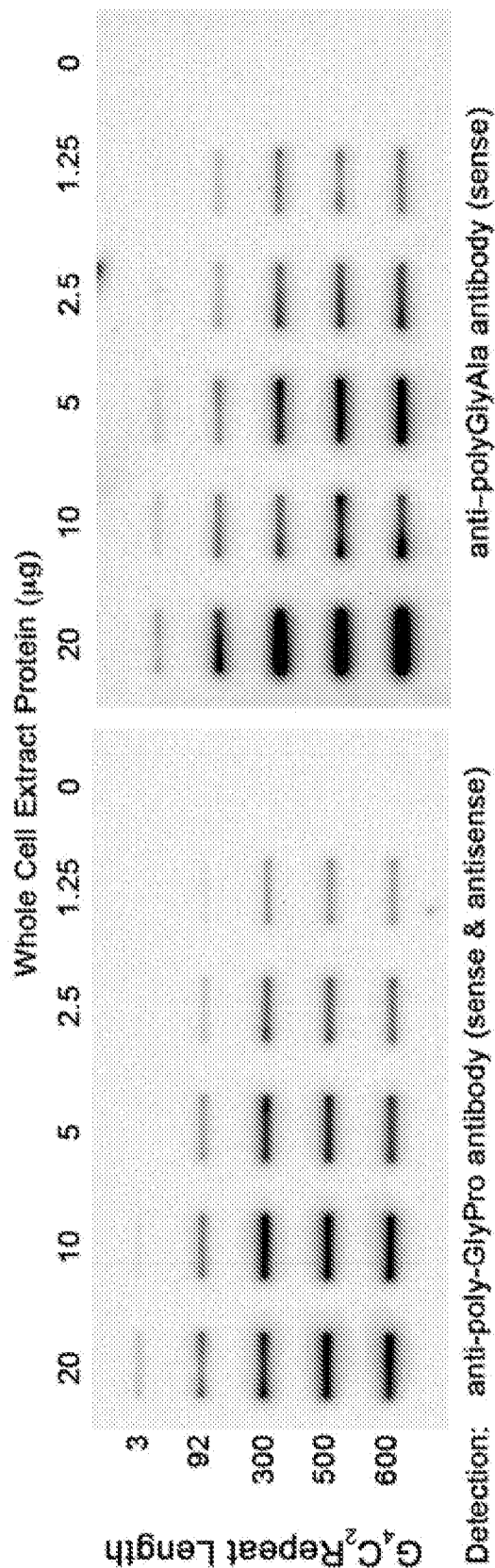


FIG. 21

Transcripts Containing Intron Sequence Near 1A

ES-Cell-Derived Motor Neurons

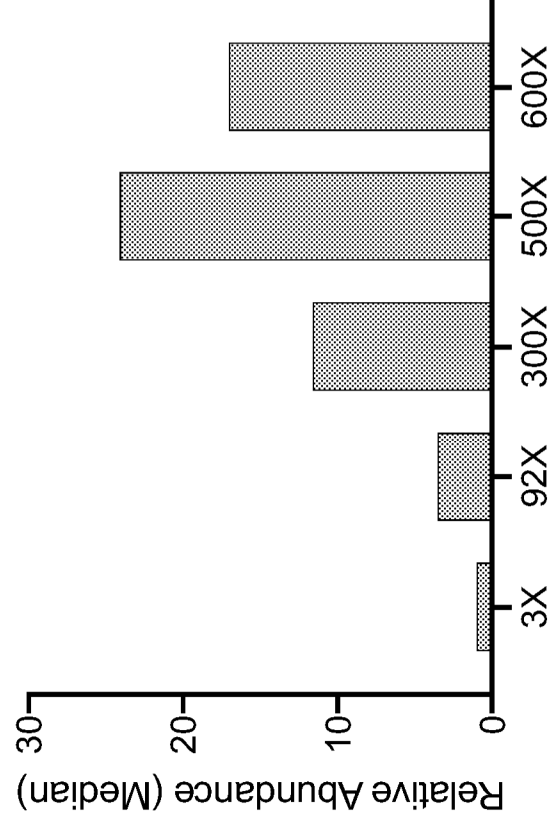


FIG. 22A

C9orf72 300x Repeat Mice

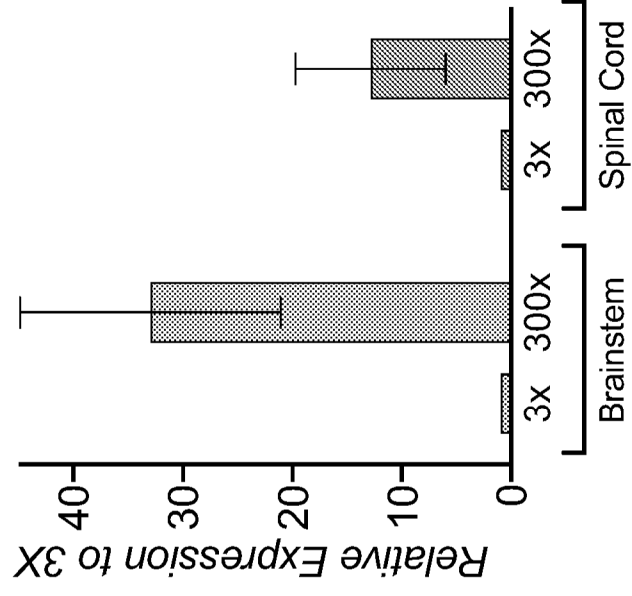


FIG. 22B

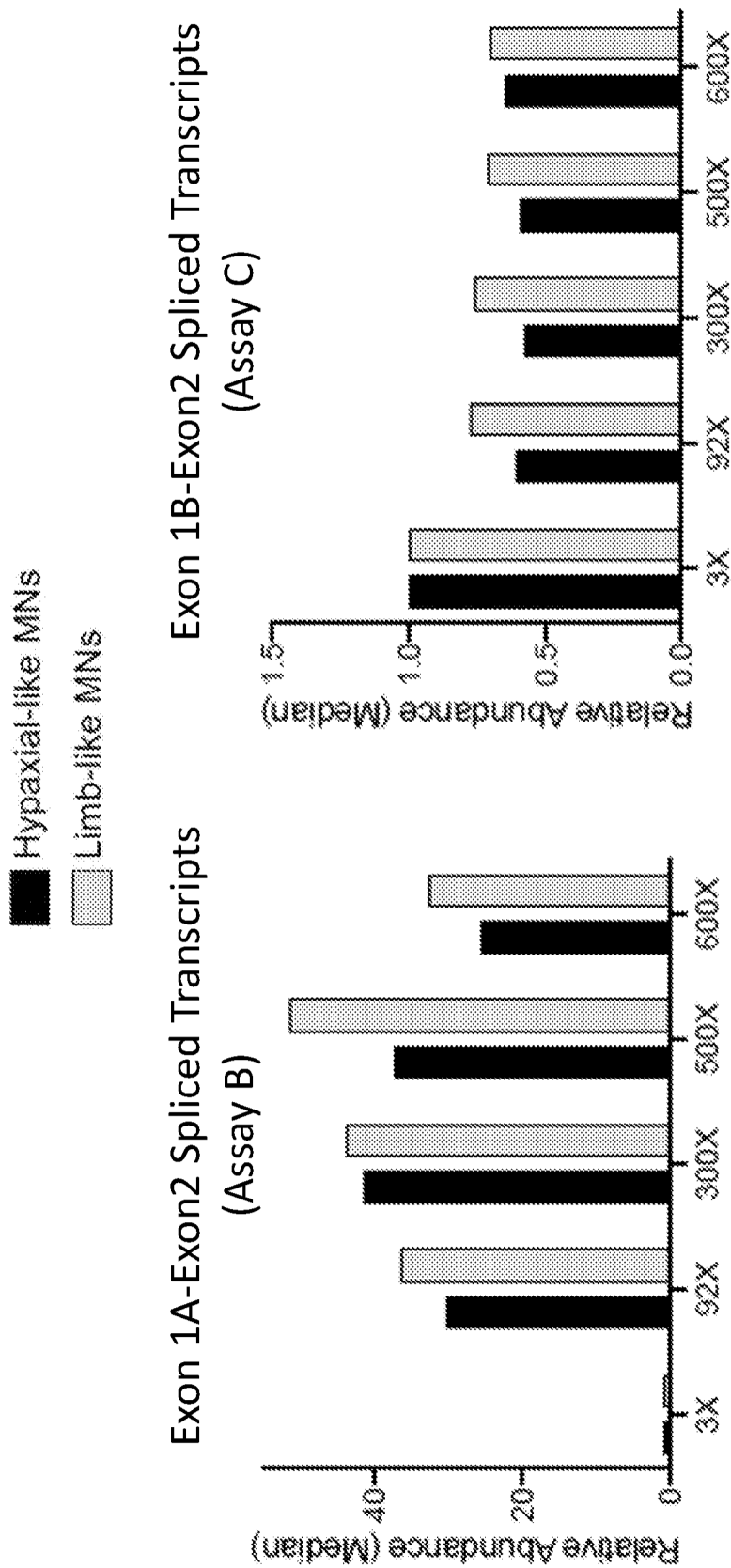


FIG. 23B

FIG. 23A

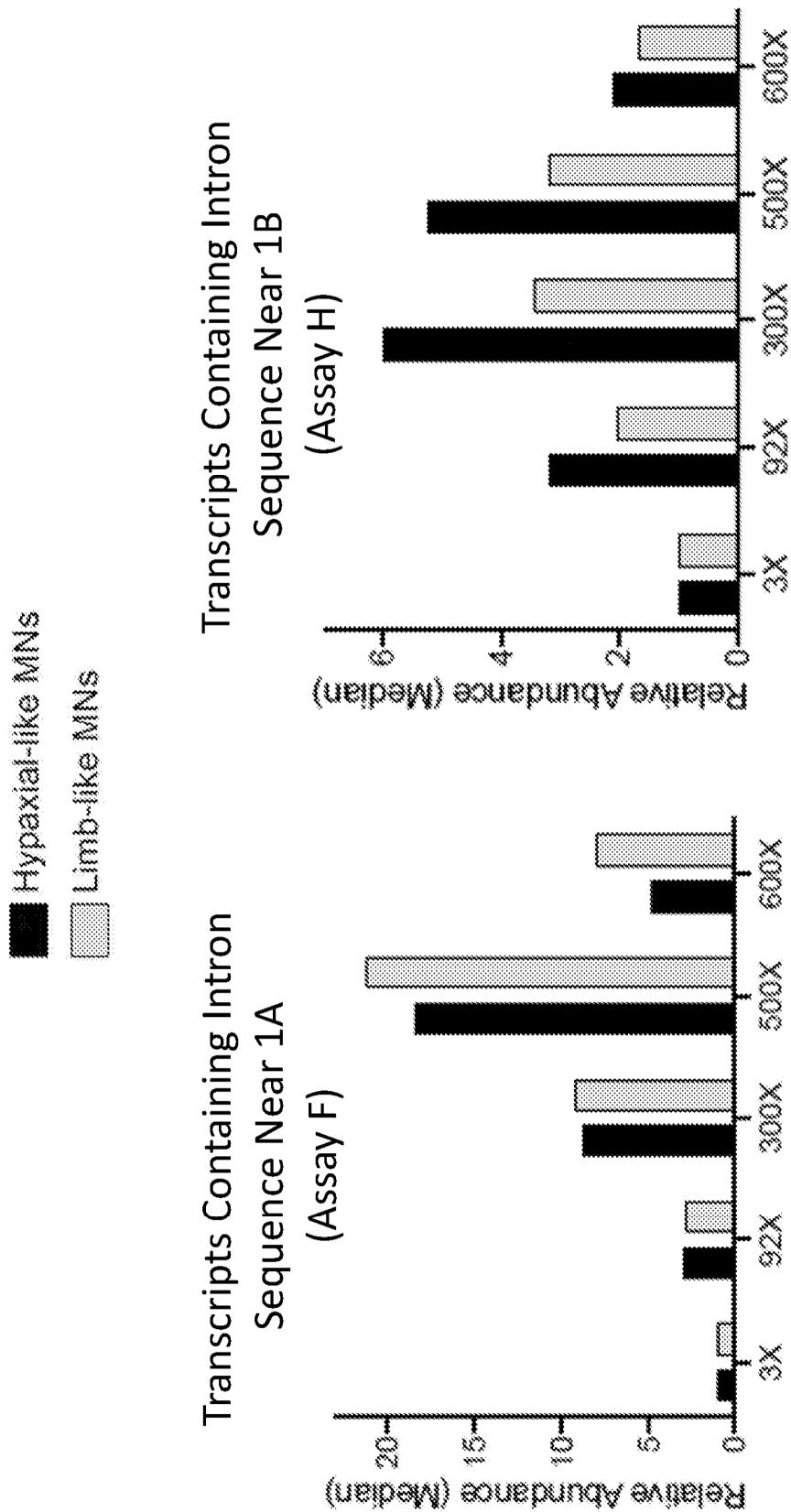


FIG. 23C

FIG. 23D

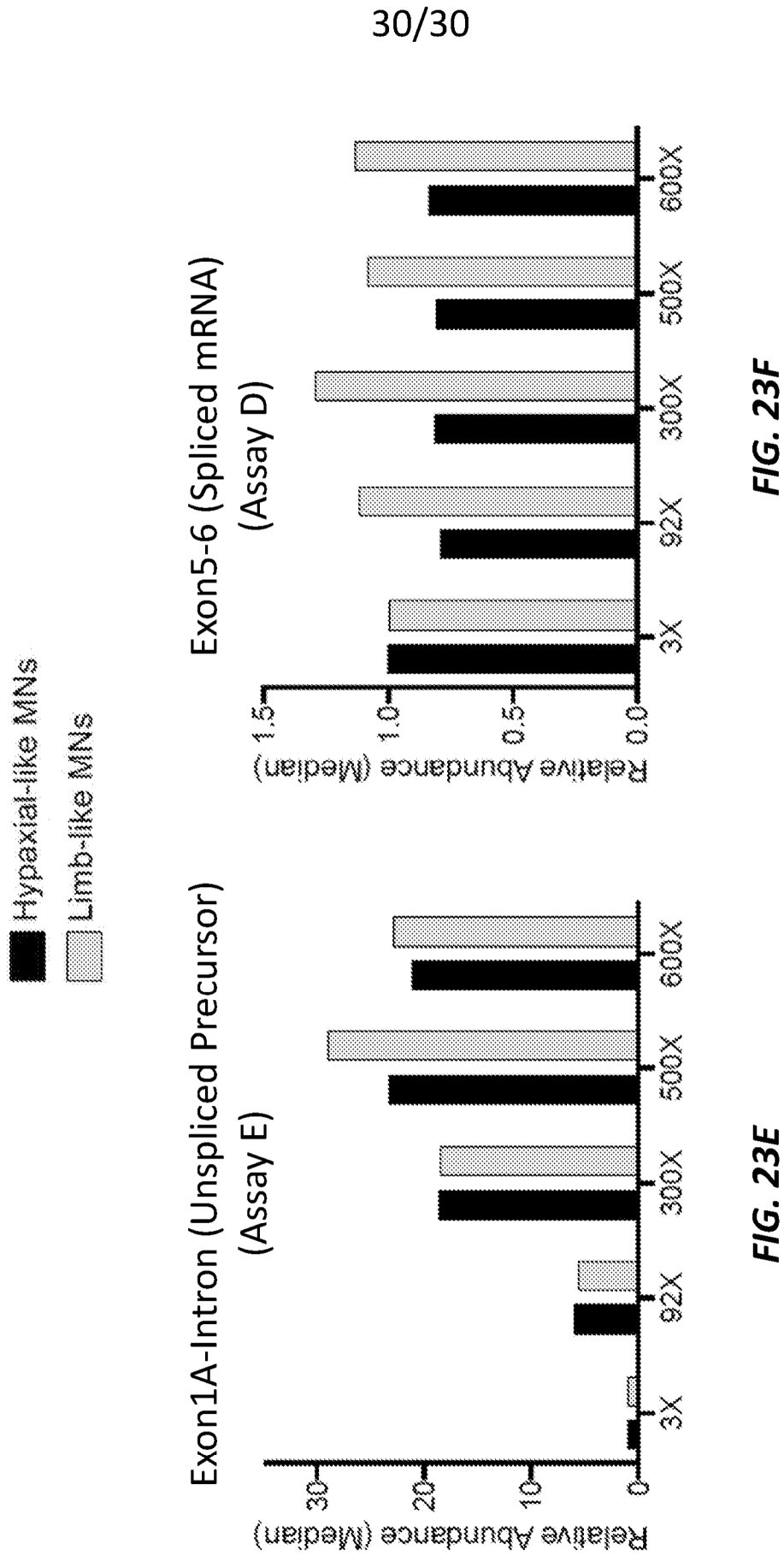


FIG. 23F

FIG. 23E