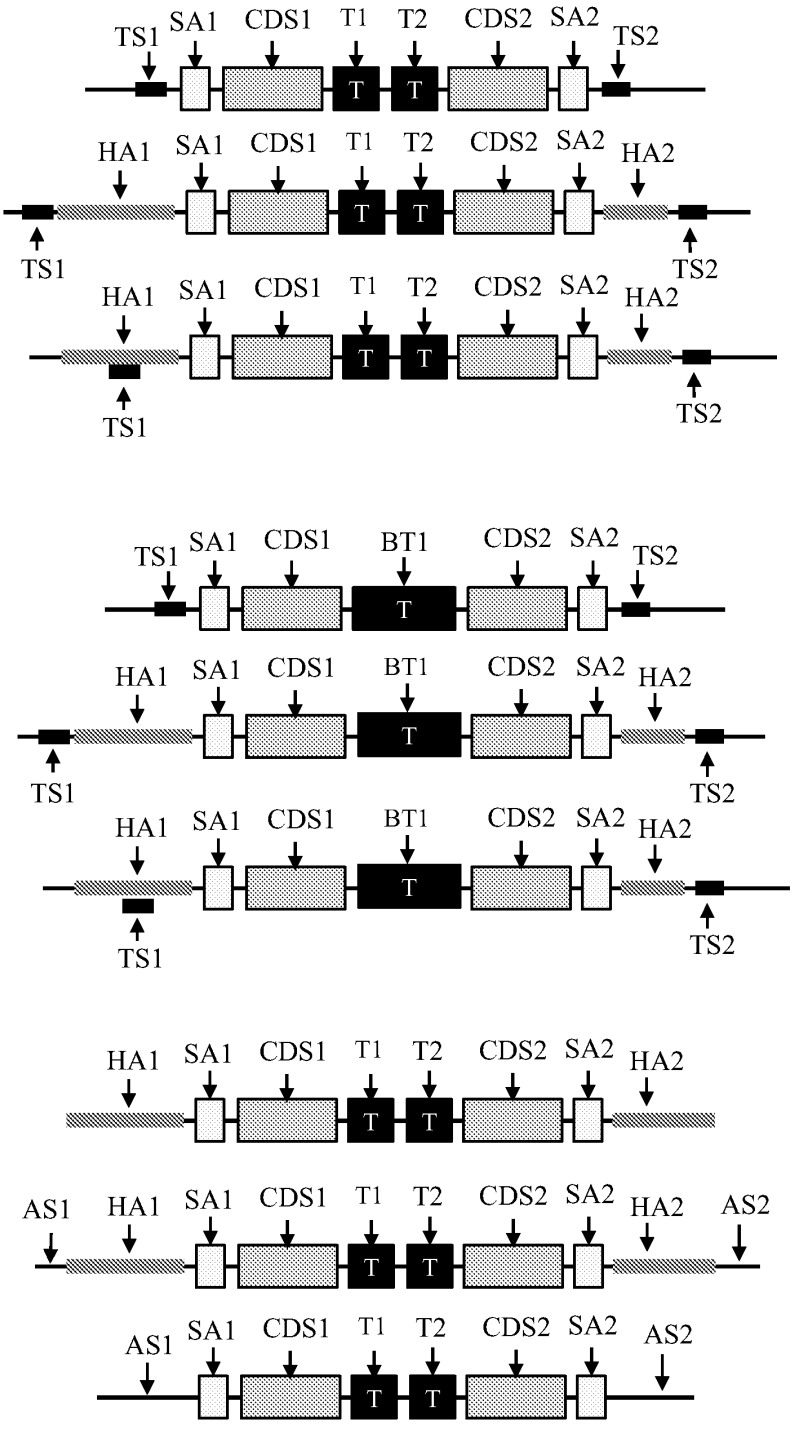


FIG. 1

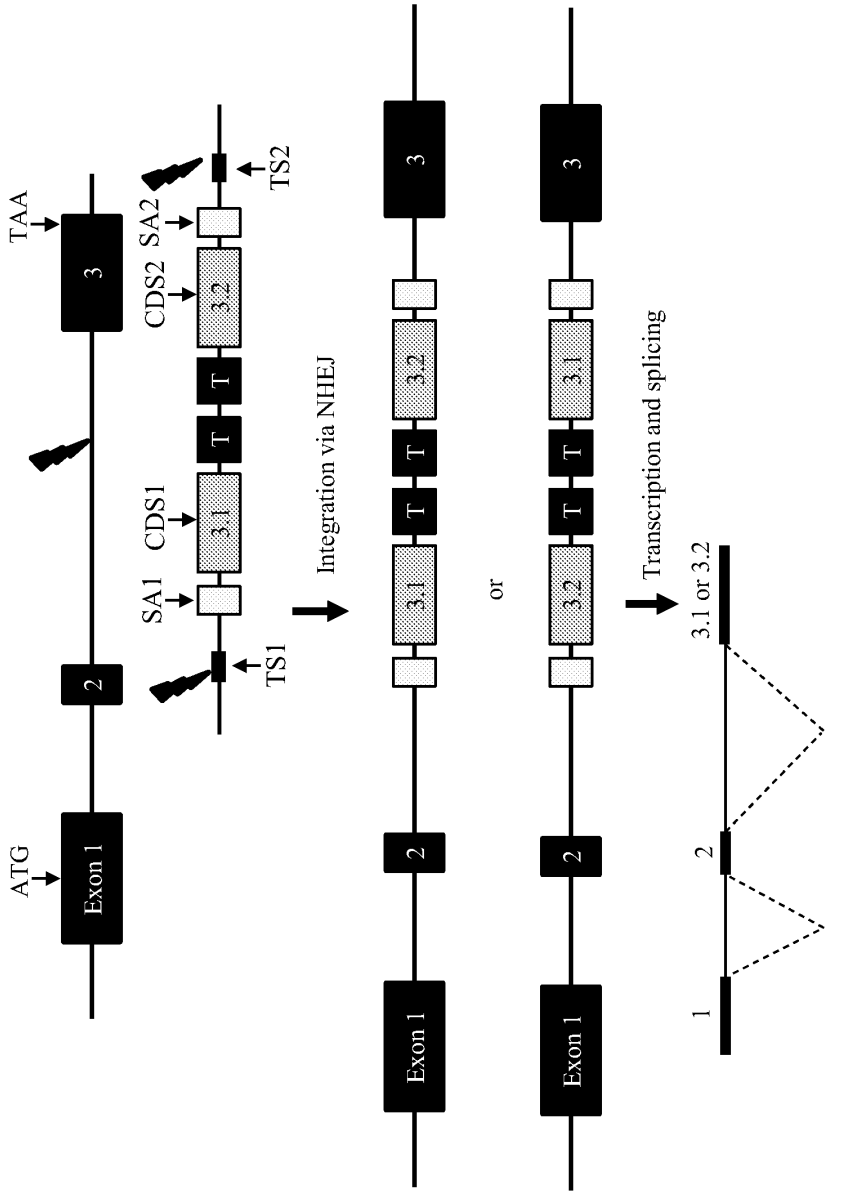

ATG
Exon 1
2
TAA
3
SA1
CDS1
3.1
T
T
T
TS1
CDS2
SA2
3.2
TS2
Integration via NHEJ
Exon 1
2
3.1
T
T
T
3.2
3
or
Exon 1
2
3.2
T
T
T
3.1
3
Transcription and splicing
1
2
3.1 or 3.2

FIG. 2

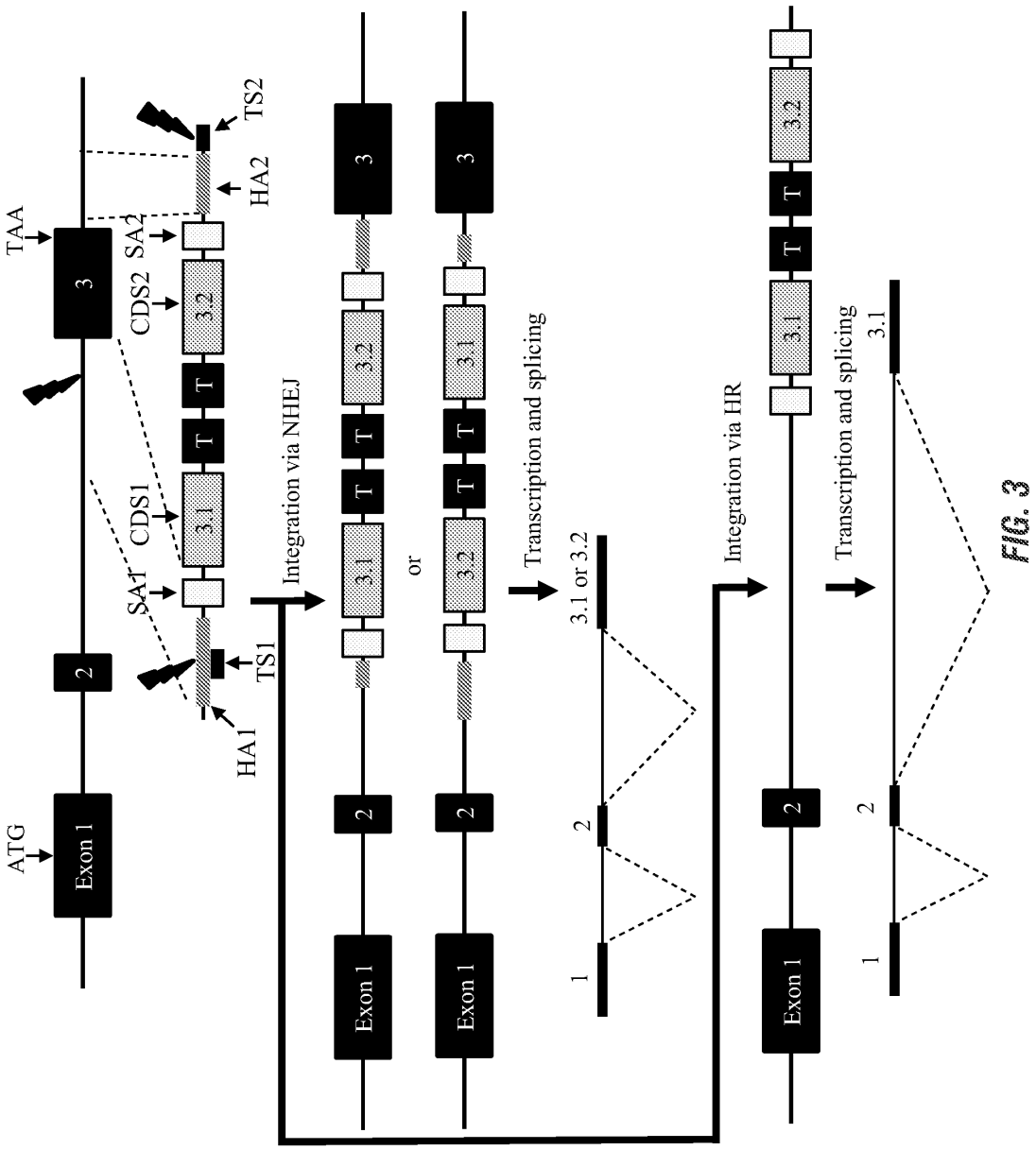

ATG
Exon 1
2
TAA
3
SA1
CDS1
SA2
CDS2
3.1
3.2
T
T
T
HA1
TS1
HA2
TS2
Integration via NHEJ
Exon 1
2
3.1
T
T
T
3.2
3
or
Exon 1
2
3.2
T
T
T
3.1
3
Transcription and splicing
1
2
3.1 or 3.2
Integration via HR
Exon 1
2
3.1
T
T
T
3.2
Transcription and splicing
1
2
3.1

FIG. 3

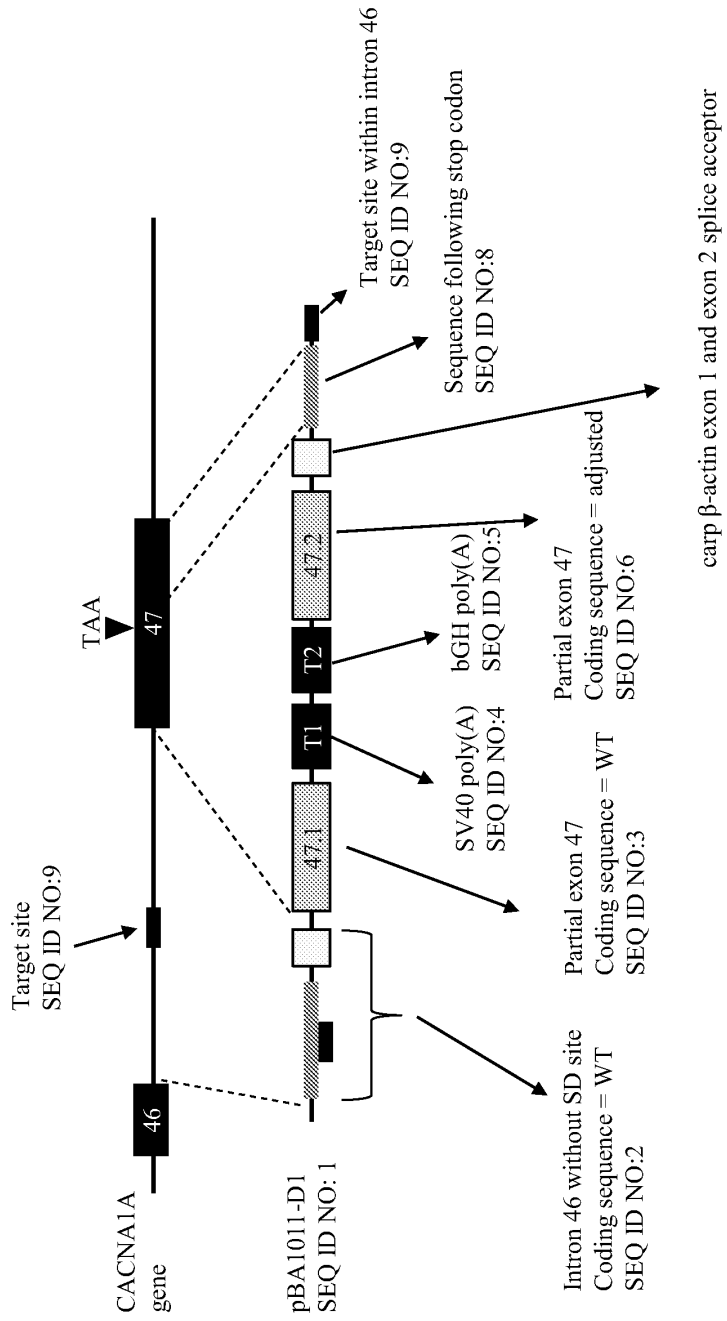

CACNA1A
gene
46
47
TAA
Target site
SEQ ID NO:9
pBA1011-D1
SEQ ID NO: 1
47.1
T1
T2
47.2
Target site within intron 46
SEQ ID NO:9
Sequence following stop codon
SEQ ID NO:8
SV40 poly(A)
SEQ ID NO:4
bGH poly(A)
SEQ ID NO:5
Intron 46 without SD site
Coding sequence = WT
SEQ ID NO:2
Partial exon 47
Coding sequence = WT
SEQ ID NO:3
Partial exon 47
Coding sequence = adjusted
SEQ ID NO:6
carp β-actin exon 1 and exon 2 splice acceptor
SEQ ID NO:7

FIG. 4

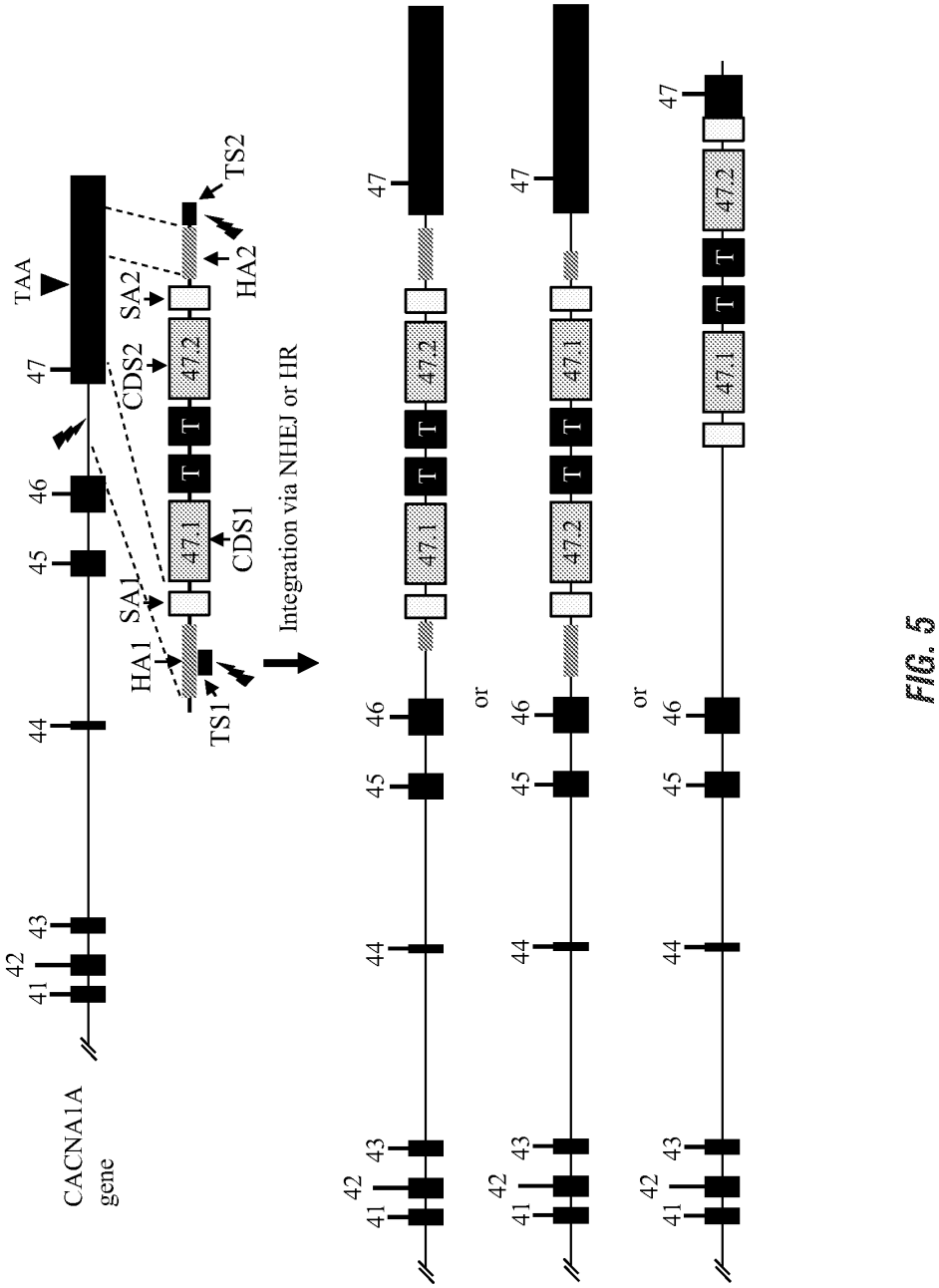
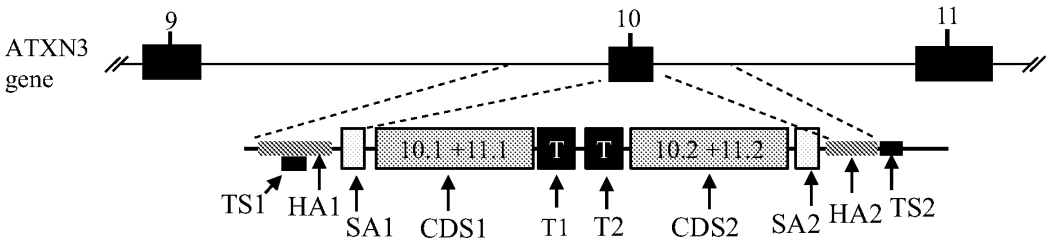

CACNA1A
gene
41
42
43
44
45
46
47
TAA
SA1
HA1
TS1
CDS1
47.1
T
T
SA2
CDS2
47.2
TS2
HA2
Integration via NHEJ or HR
or
or

FIG. 5



pBA1012-D1 (SEQ ID NO: 10); pBA1135 (SEQ ID NO:17)

FIG. 6

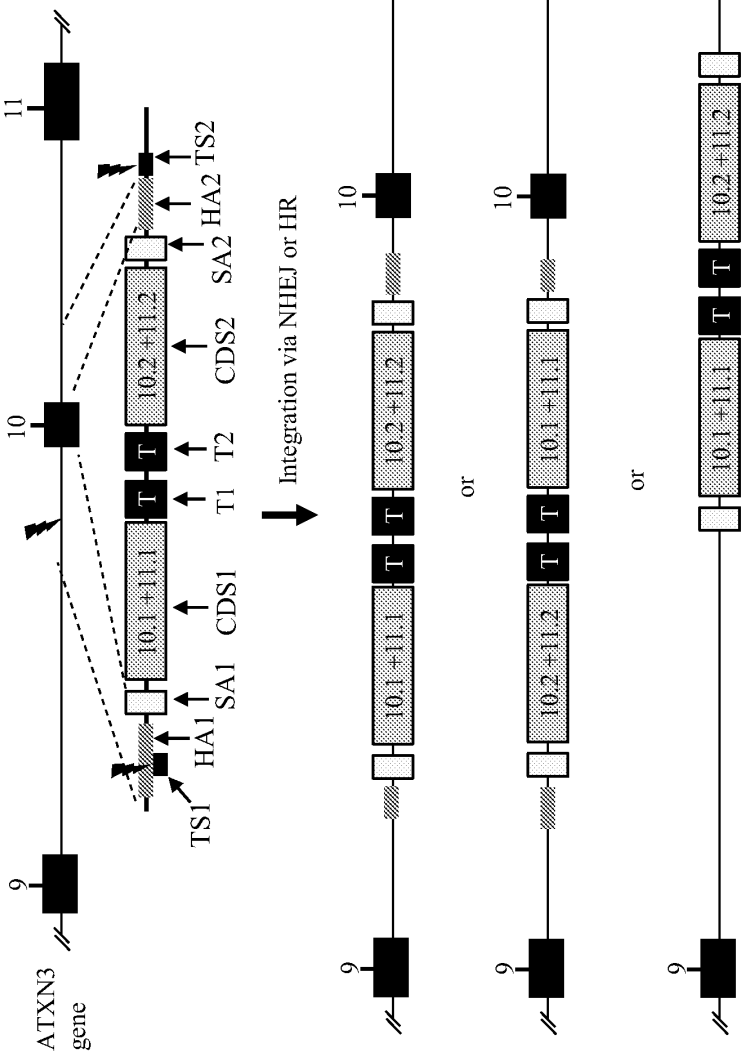

ATXN3
gene
9
10
11
TS1
HA1
SA1
CDS1
T1
T2
CDS2
SA2
HA2
TS2
T
10.1 +11.1
10.2 +11.2
Integration via NHEJ or HR
or
or

FIG. 7

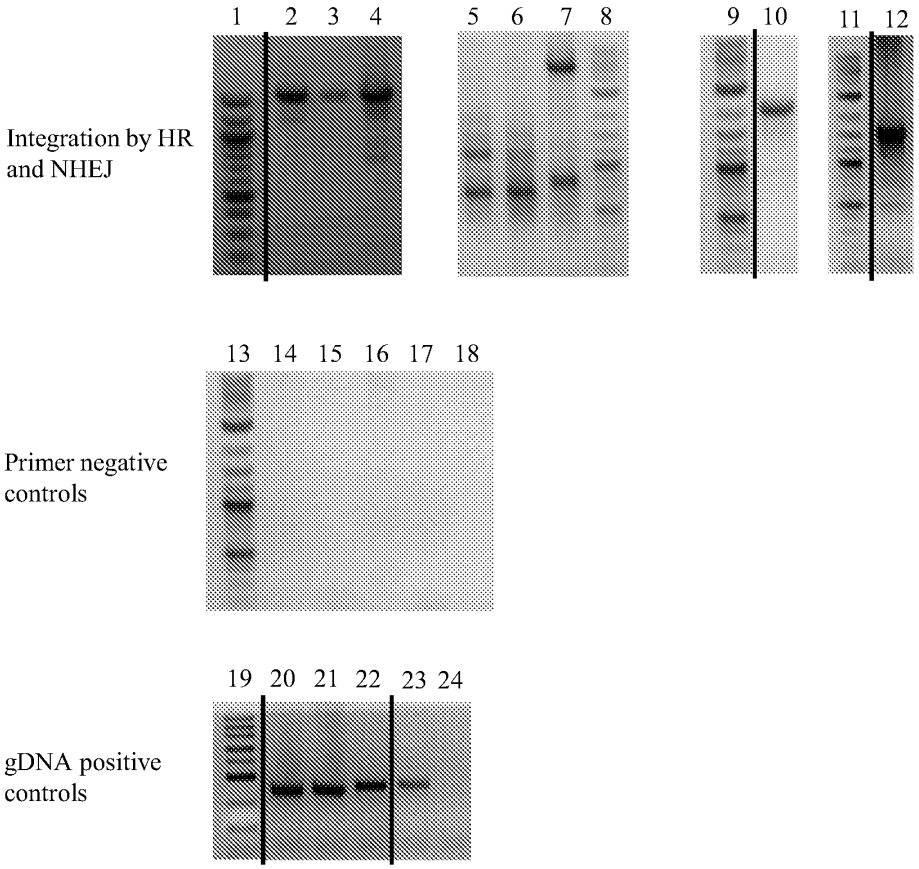

1 2 3 4
5 6 7 8
9 10
11 12
Integration by HR and NHEJ
13 14 15 16 17 18
Primer negative controls
19 20 21 22 23 24
gDNA positive controls

FIG. 8