human B4GALT1 protein (P15291-1)
mouse B4galt1 protein (P15535-1)
p.352 on human
p.353 on mouse

FIG. 1A

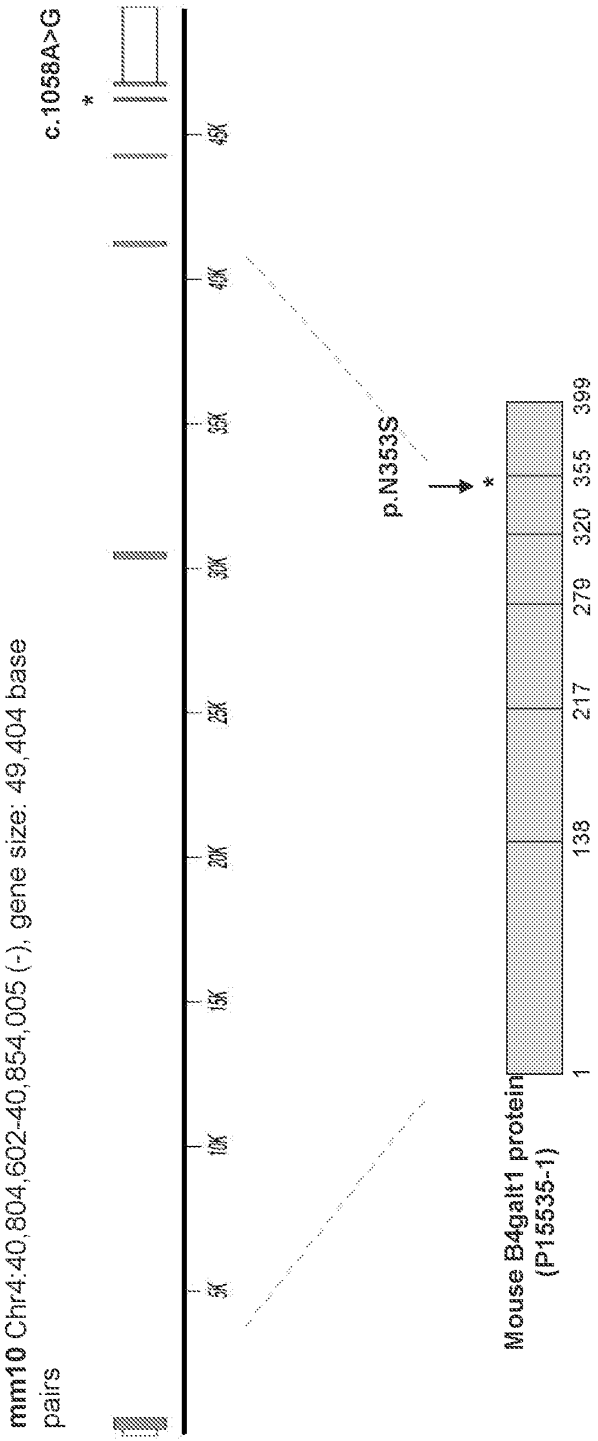

mm10 Chr4:40,804,602-40,854,005 (-), gene size: 49,404 base pairs
c.1058A>G
*
5K
10K
15K
20K
25K
30K
35K
40K
45K
p.N353S
*
Mouse B4galt1 protein (P15535-1)
1
138
217
279
320
355
399

FIG. 1B

gRNA
GGTTAGGAGTCTCCATGCAGGAG
WT sequence
ssODN sequence
AAT>AGT
p.N353S
CCT>CCC
p.P354P

FIG. 1C

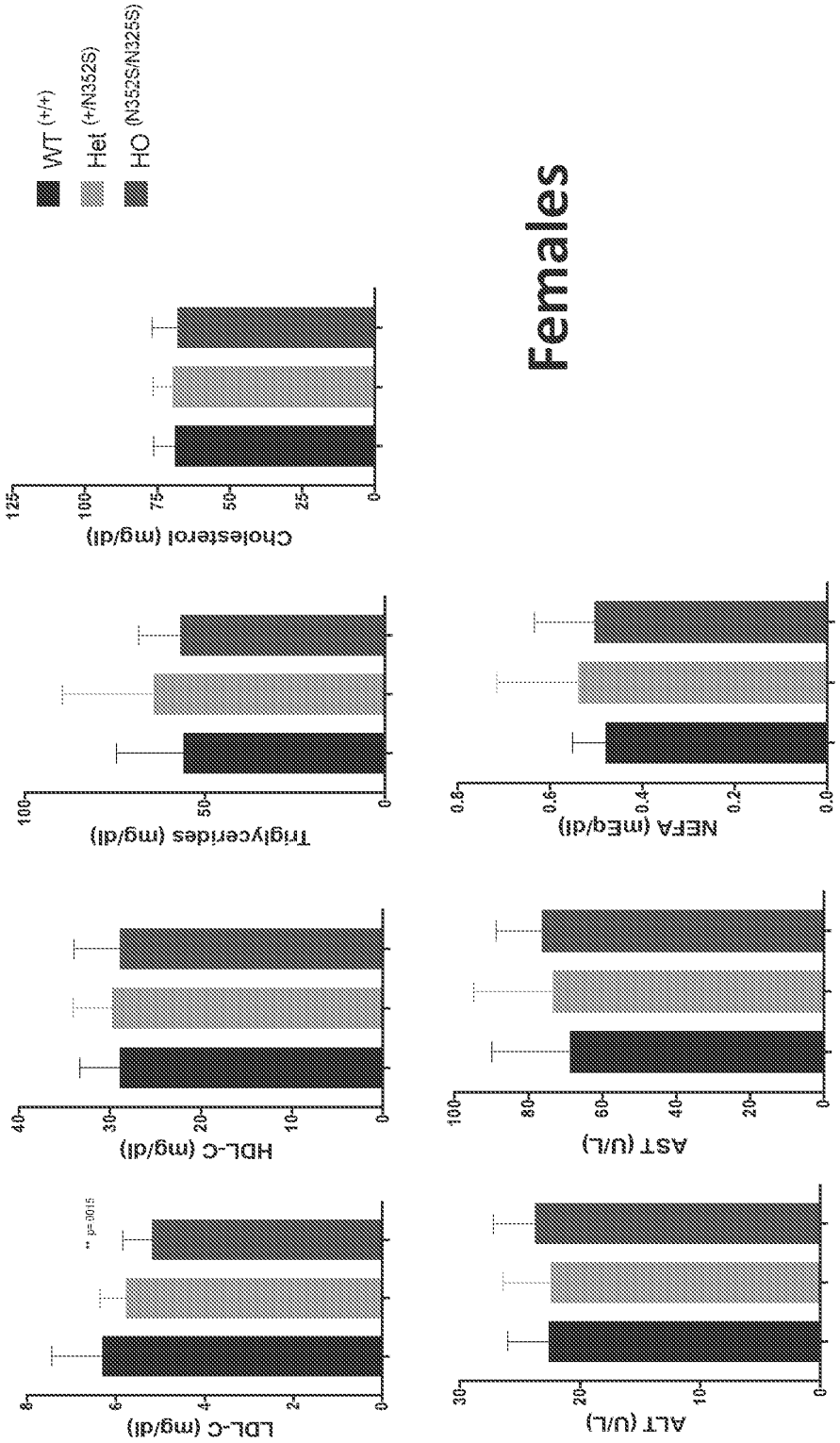

WT (+/+)
Het (+/N352S)
HO (N352S/N325S)
Females
Cholesterol (mg/dl)
Triglycerides (mg/dl)
NEFA (mEq/dl)
HDL-C (mg/dl)
AST (U/L)
LDL-C (mg/dl)
ALT (U/L)
** p= 0015

FIG. 2A

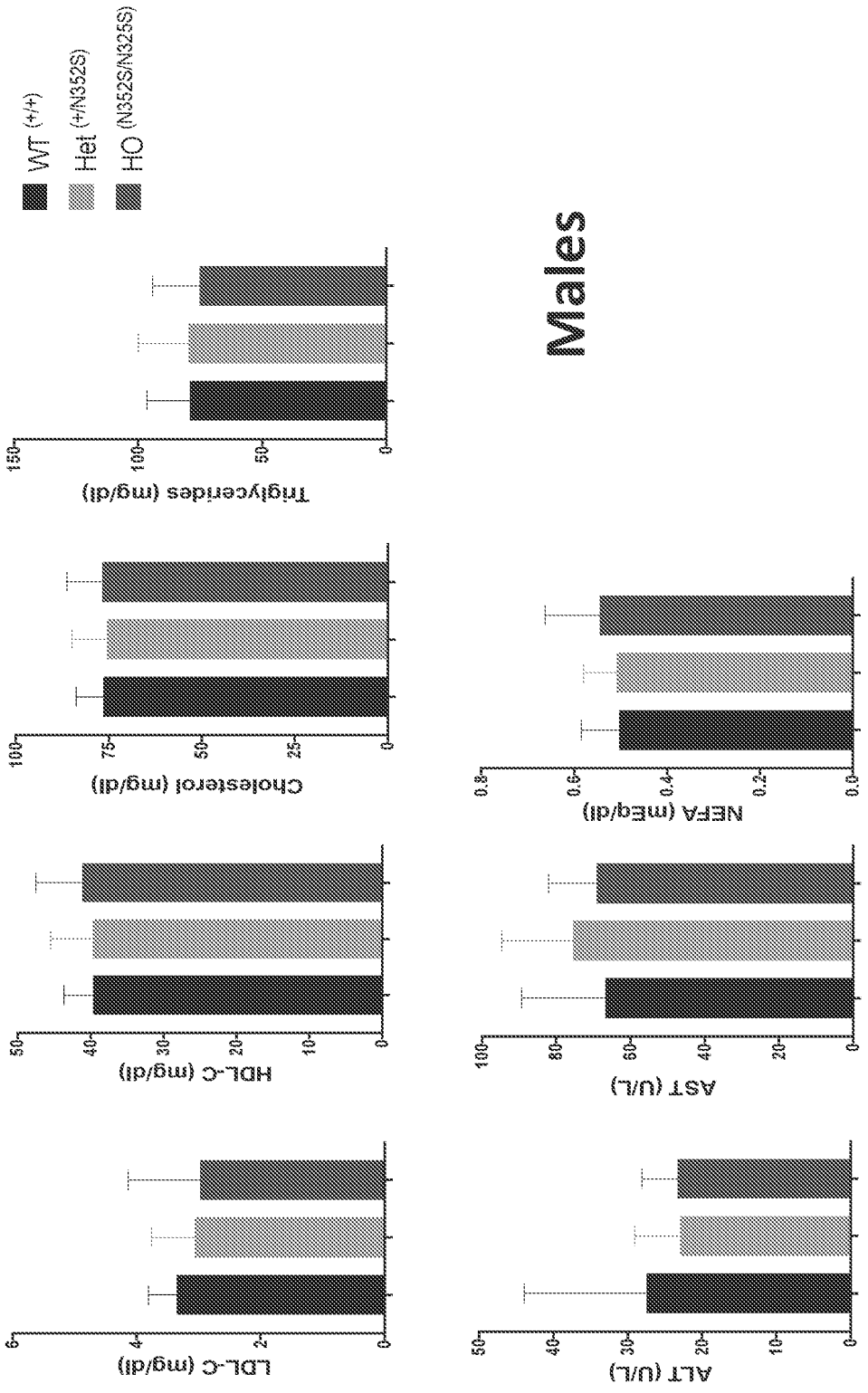

Males
WT (+/+)
Het (+/N352S)
HO (N352S/N325S)
Triglycerides (mg/dl)
Cholesterol (mg/dl)
HDL-C (mg/dl)
LDL-C (mg/dl)
NEFA (mEq/dl)
AST (U/L)
ALT (U/L)

FIG. 2B

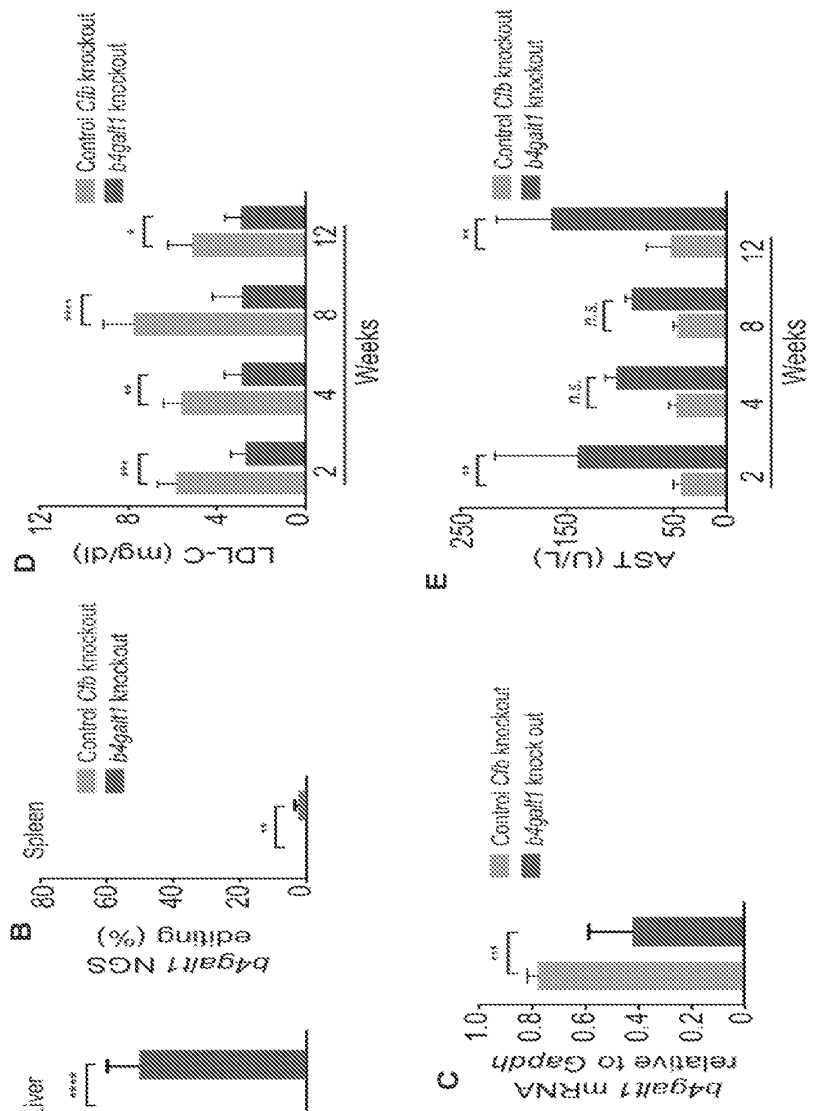

A
Liver
b4galt1 NGS editing (%)
80
60
40
20
0
****
B
Spleen
b4galt1 NGS editing (%)
Control C1b knockout
b4galt1 knockout
**
C
b4galt1 mRNA relative to Gapdh
Control C1b knockout
b4galt1 knock out
***
1.0
0.8
0.6
0.4
0.2
0
D
LDL-C (mg/dl)
12
8
4
0
***
**
***
*
2
4
8
12
Weeks
E
AST (U/L)
250
150
50
0
**
n.s.
*
n.s.
*
**
2
4
8
12
Weeks

FIG. 3A-3E

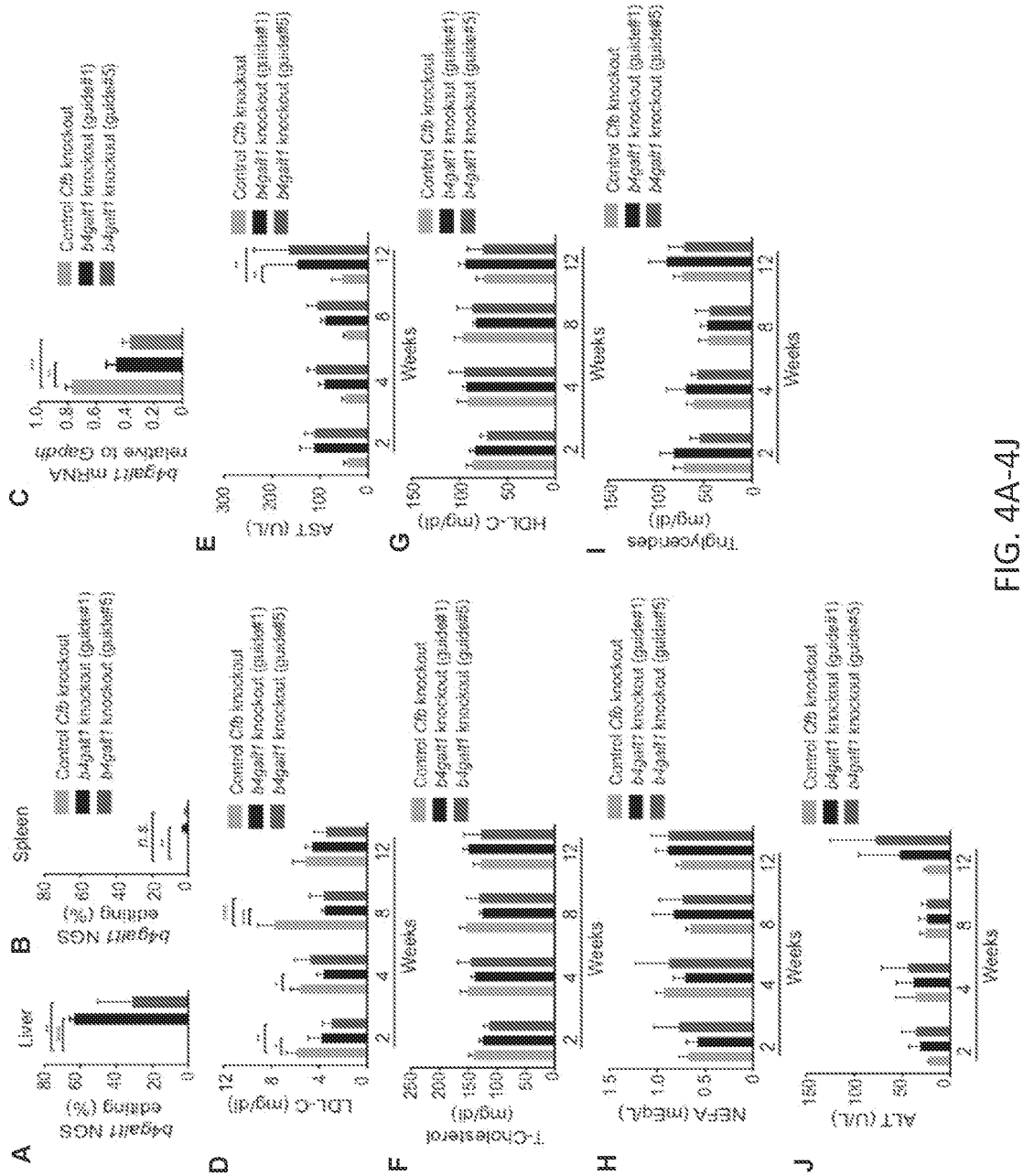

A
Liver
b4galt1 NGS editing (%)
B
Spleen
b4galt1 NGS editing (%)
n.s.
Control Cfb knockout
b4galt1 knockout (guide#1)
b4galt1 knockout (guide#5)
b4galt1 knockout (guide#6)
C
b4galt1 mRNA relative to Gapdh
D
LDL-C (mg/dl)
E
AST (U/L)
F
T-Cholesterol (mg/dl)
G
HDL-C (mg/dl)
H
NEFA (mEq/L)
I
Triglycerides (mg/dl)
J
ALT (U/L)
Weeks

FIG. 4A-4J