

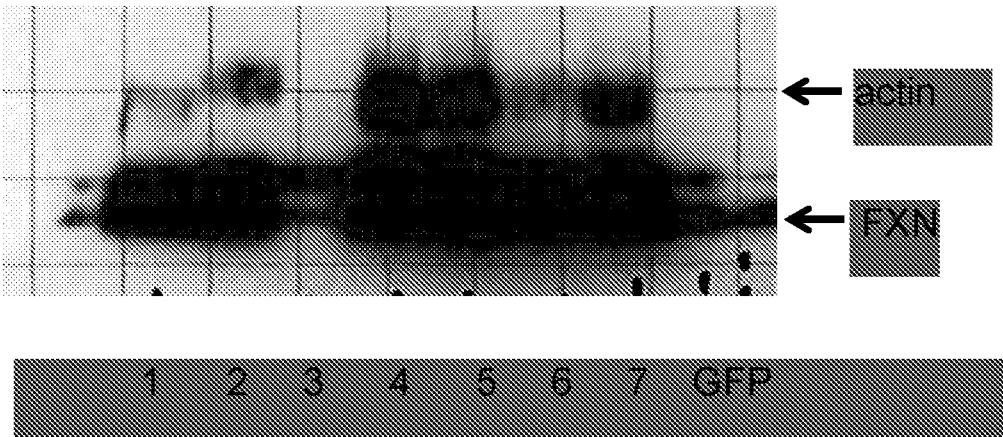


FIGURE 1A

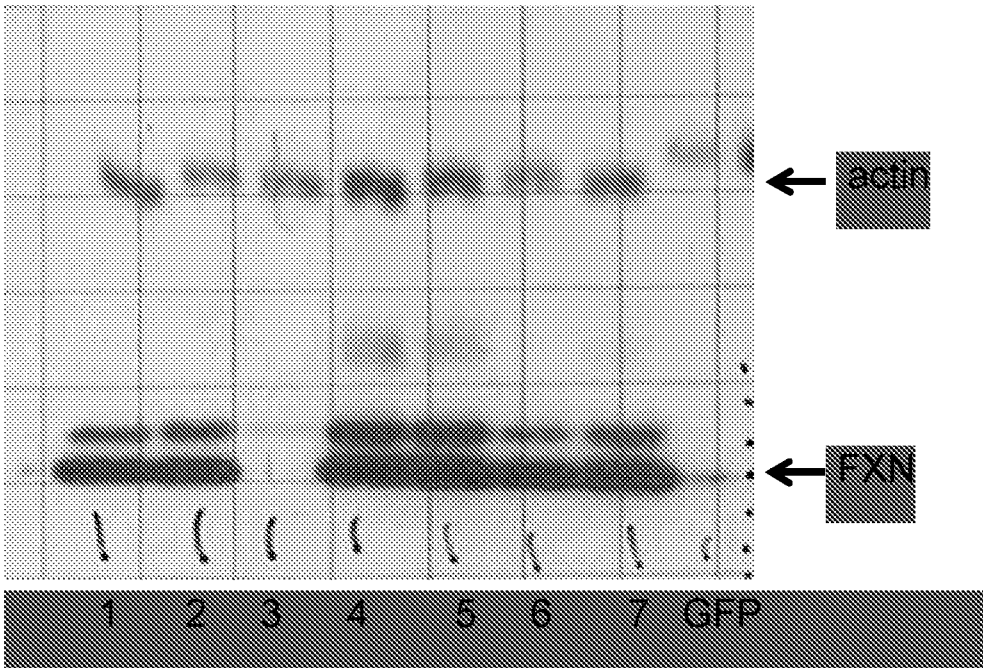


FIGURE 1B

FIGURE 2A

WT FXN (SEQ ID NO:19)

TAGAAG**ACCGGT**CGCCACCatgtggactctcgggcgccgcgagtagccggcctcctggcgt
caccagcccagcccaggcccagaccctcaccgggtcccgcggccggcagagttggcccca
ctctgcgccgcccgtggcctgcgccagacatcgatgcgacctgcacgccccgcccgcgaag
ttcgaaccaacgtggcctcaaccagatttggaatgtcaaaaagcagagtgctctatttgatga
atttgaggaaatctggaactttgggccaccaggctctctagatgagaccacctatgaaaga
ctagcagaggaaacgctggactcttttagcagagttttttgaagaccttgacagacaagccata
cacgtttgaggactatgatgtctcctttgggagtgggtgtcttaactgtcaaactgggtggag
atctaggaacctatgtgatcaacaagcagacgccaacaagcaaatctggctatcttctcca
tccagtggacctaagcgttatgactggactgggaaaaactgggtgtactcccacgacggcgt
gtccctccatgagctgctggccgagagctcactaaagccttaaaaaccaaactggacttgt
cttccttggcctattccgaaaagatgcttgaCGAGCGGCCGCTCCTAGGAGCAGTATCGAT
CCCAGCCCACTTTTCCCCAATACG**ACTAGT**ACTCGACTGTGCCTTCTAGTTGCCAGCCATCT
GTTGTTTGCCCTCCCCCGTGCCTTCCTTGACCCTGGAAGGTGCCACTCCCAGTGCCTTTC
CTAATAAAATGAGGAAATTGCATCGCATTGTCTGAGTAGGTGTCATTCTATTCTGGGGGGTG
GGGTGGGGCAGGACAGCAAGGGGGAGGATTGGGAAGACAACAGCAGGCATGCTGGGGATGCG
GTGGGCTCTATGGCTTCTGAGGCGGAAAGAACCAGCTTTGG**ACGCGT**CTTAAG

FIGURE 2B

IDT1 codon optimized FXN (SEQ ID NO:20)

TAGAAG**ACCGGT**CGCCACCatgtggactctgggtaggcgagcggtagggcggcctgttgcat
ctcctagtcctgcacaagctcaaacgctgactagagtccttcggccagcagaactggcgcca
ctttgcgccgcccggcgcggtcttcgcactgatattgatgccacttgacacccccggcgcgctc
cagtaatcagcggggacttaatacaattttggaatgtgaagaagcagtcctgtgtatcttatga
atctgcggaagagcgggaccctggggccaccctggtagccttgatgaaaccacctatgagcgc
ctggccgaagagacactggacagtcttgccgagttttttgaggatctggccgacaaacctta
tacttttgaggactatgacgtgtcctttggatctgggtgtattgaccgtaaaactcgggggag
accttgggacgtatgtaataaataagcagaccccaacaagcagatctggctcagctctcca
agtagtggtcctaagagatatgattggacggggcaagaactgggtctattcccatgatggcgt
ctctttgcatgaactccttgacgagagctgaccaaggccttgaagaccaaattggatctca
gcagcctcgcctatagtggcaaagatgcatagCGAGCGGCCGCTCCTAGGAGCAGTATCGAT
CCCAGCCCACTTTTCCCCAATACG**ACTAGT**ACTCGACTGTGCCTTCTAGTTGCCAGCCATCT
GTTGTTTGCCCTCCCCCGTGCCTTCCTTGACCCTGGAAGGTGCCACTCCCAGTGCCTTTC
CTAATAAAATGAGGAAATTGCATCGCATTGTCTGAGTAGGTGTCATTCTATTCTGGGGGGTG
GGGTGGGGCAGGACAGCAAGGGGGAGGATTGGGAAGACAACAGCAGGCATGCTGGGGATGCG
GTGGGCTCTATGGCTTCTGAGGCGGAAAGAACCAGCTTTGG**ACGCGT**CTTAAG

FIGURE 2C

IDT3 - low expresser (SEQ ID NO:21)

TAGAAG**ACCGGT**CGCCACCatgtggacactgggaaggcgccggtggccggtctgttggcat
caccatccccagcccaggtcagacactcaccagagtcccaagaccgcagagctggccct
ctgtgcgggcgccgaggccttcgcaccgatatcgatgctacatgcacgccacgcagagctag
ctcaaatcagaggggactcaaccagatatggaatgtcaagaagcaaagcgtgtatctcatga
acctccgaaaagcggcaccctgggacatcccgggtctctcgacgagaccacttatgaaaga
ctggcagaggagactcttgacagtctggcggagtcttcgaagacctcgctgacaagccata
taccttcgaagattacgacgtctccttcgggtctggtgctgactgtcaagcttggcggcg
acctggggacctacgtgatcaacaagcagactccaaacaagcaaactctggctcagcagtcca
agctccggacccaagagatacgattggacaggcaagaattgggtttactcccacgacggggt
gtccctccatgagctgctggccgctgagctgacgaaggccctgaagaccaagctggatctct
cctccctggcatacagtggtaaggacgttgacGAGCGGCCGCTCCTAGGAGCAGTATCGAT
CCCAGCCCACTTTTCCCCAATACG**ACTAGT**ACTCGACTGTGCCTTCTAGTTGCCAGCCATCT
GTTGTTTGCCCTCCCCCGTGCCTTCCTTGACCCTGGAAGGTGCCACTCCCCTGTCTTTT
CTAATAAAATGAGGAAATTGCATCGCATTGTCTGAGTAGGTGTCATTCTATTCTGGGGGGTG
GGGTGGGGCAGGACAGCAAGGGGGAGGATTGGGAAGACAACAGCAGGCATGCTGGGGATGCG
GTGGGCTCTATGGCTTCTGAGGCGGAAAGAACCAGCTTTGG**ACGCGT**CTTAAG

FIGURE 2D

IDT4 (SEQ ID NO:22)

TAGAAG**ACCGGT**CGCCACCatgtggactctgggcccggcgggcccgtagctggcttgctggcta
gcccaggtcccgcaggtcagactctcaccagggtagccaggcccgagagcttgctcca
ctctgcggacgcaggggtctgcaaccgatatcgacgcaacttgacgccgcggaggcctc
ttcaaacagagaggactcaatcaaatttggaatgtaaagaaacagagcgtgtatctcatga
acctccgaaagagtgggactcttgggcaccccgggtccctggacgagactacttacgagcgc
ctggccgaagaaaccttgattccctggcggagttttttgaagacttggcagacaagcctta
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tcttcgggtcccaaaaggtagcattggactggaaagaactgggtctacagtcacgacggtgt
ctccctgcacgaattgcttgccgctgagctgactaaggcgctcaaaacaaaactggatctgt
ccagccttgccatatagcgggaaggacgcatgaGAGCGGCCGCTCCTAGGAGCAGTATCGAT
CCCAGCCCACTTTTCCCCAATACG**ACTAGT**ACTCGACTGTGCCTTCTAGTTGCCAGCCATCT
GTTGTTTGCCCTCCCCCGTGCCTTCCTTGACCCTGGAAGGTGCCACTCCCCTGTCTTTT
CTAATAAAATGAGGAAATTGCATCGCATTGTCTGAGTAGGTGTCATTCTATTCTGGGGGGTG
GGGTGGGGCAGGACAGCAAGGGGGAGGATTGGGAAGACAACAGCAGGCATGCTGGGGATGCG
GTGGGCTCTATGGCTTCTGAGGCGGAAAGAACCAGCTTTGG**ACGCGT**CTTAAG

FIGURE 2E

GenScript (SEQ ID NO:23)

TAGAAG**ACCGGT**CGCCACCatgtggacactgggccggagagccgtcgctgggctgctggcat
caccatccccgcacaggcacagaccctgacaagagtccctcggccagcagagctggcccca
ctgtgctgggagagaggactgcaaccgacatcgatgctacttgtacccaaggcgagcaag
ctccaaccagcgagggctgaaccagattttggaatgtgaagaaacagctctgtctacctgatga
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caccttcgaagactatgacgtgagcttcggcagcggcgctgctgacagtcaaactgggcgggg
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tcaagcggccctaagaggtacgactggacagggaaaaactgggtgtatagtcacgatggcgt
ctcactgcatgagctgctggccgctgaactgactaaagccctgaaaactaaactggacctgt
cttccttggcatactctggcaaggacgcctgaCGAGCGGCCGCTCCTAGGAGCAGTATCGAT
CCCAGCCCACTTTTCCCCAATACG**ACTAGT**ACTCGACTGTGCCTTCTAGTTGCCAGCCATCT
GTTGTTTGCCCTCCCCCGTGCCTTCCTTGACCCTGGAAGGTGCCACTCCCAGTGCCTTTC
CTAATAAAATGAGGAAATTGCATCGCATTGTCTGAGTAGGTGTCATTCTATTCTGGGGGGTG
GGGTGGGGCAGGACAGCAAGGGGGAGGATTGGGAAGACAACAGCAGGCATGCTGGGGATGCG
GTGGGCTCTATGGCTTCTGAGGCGGAAAGAACCAGCTTTGG**ACGCGT**CTTAAG

FIGURE 2F

GenScript (low CpG) (SEQ ID NO:24)

TAGAAG**ACCGGT**CGCCACCatgtggactctgggccggagagcagtgccagagactgctggcaa
gtccatcacctgctcaggcacagactctgacaagagtcccaagacctgcagagctggctcca
ctgtgctgggagggcgcgactgagaacagacatcgatgctacatgtactcctcgacgggcaag
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cagtctgcatgagctgctggccgctgaactgaccaaggccctgaagacaaaactggacctgt
cctctctggcatatagcggaaaagatgcctgaCGAGCGGCCGCTCCTAGGAGCAGTATCGAT
CCCAGCCCACTTTTCCCCAATACG**ACTAGT**ACTCGACTGTGCCTTCTAGTTGCCAGCCATCT
GTTGTTTGCCCTCCCCCGTGCCTTCCTTGACCCTGGAAGGTGCCACTCCCAGTGCCTTTC
CTAATAAAATGAGGAAATTGCATCGCATTGTCTGAGTAGGTGTCATTCTATTCTGGGGGGTG
GGGTGGGGCAGGACAGCAAGGGGGAGGATTGGGAAGACAACAGCAGGCATGCTGGGGATGCG
GTGGGCTCTATGGCTTCTGAGGCGGAAAGAACCAGCTTTGG**ACGCGT**CTTAAG

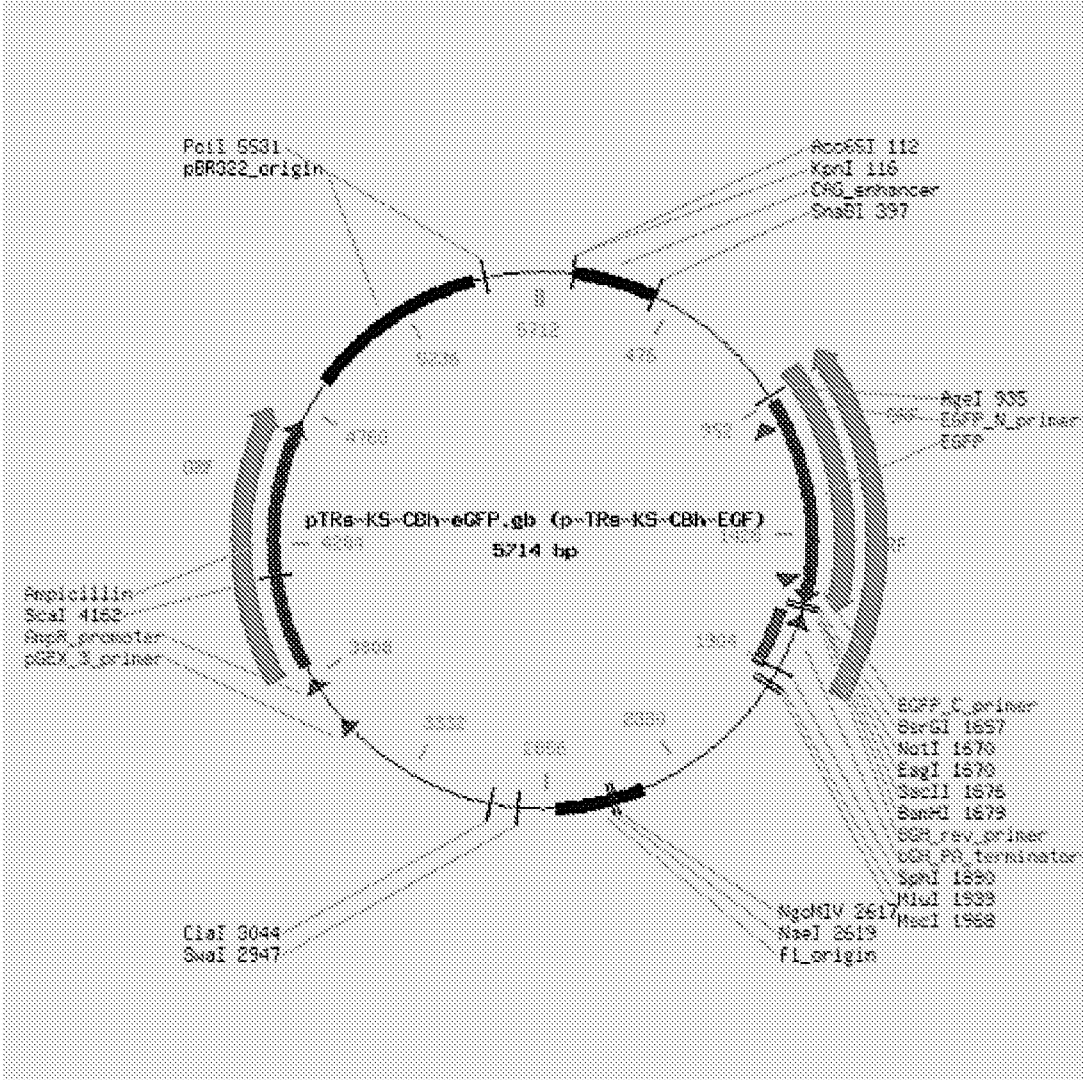
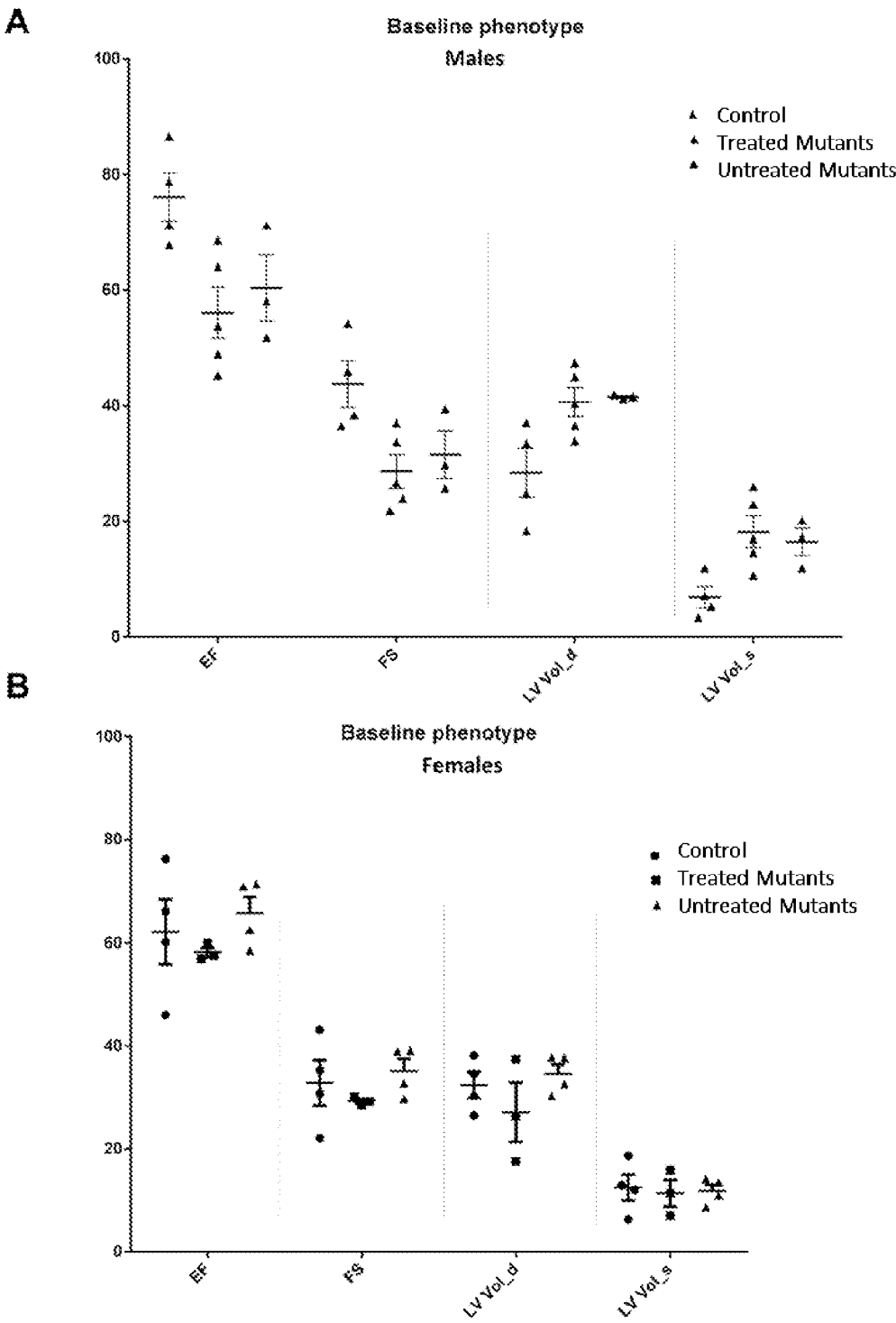
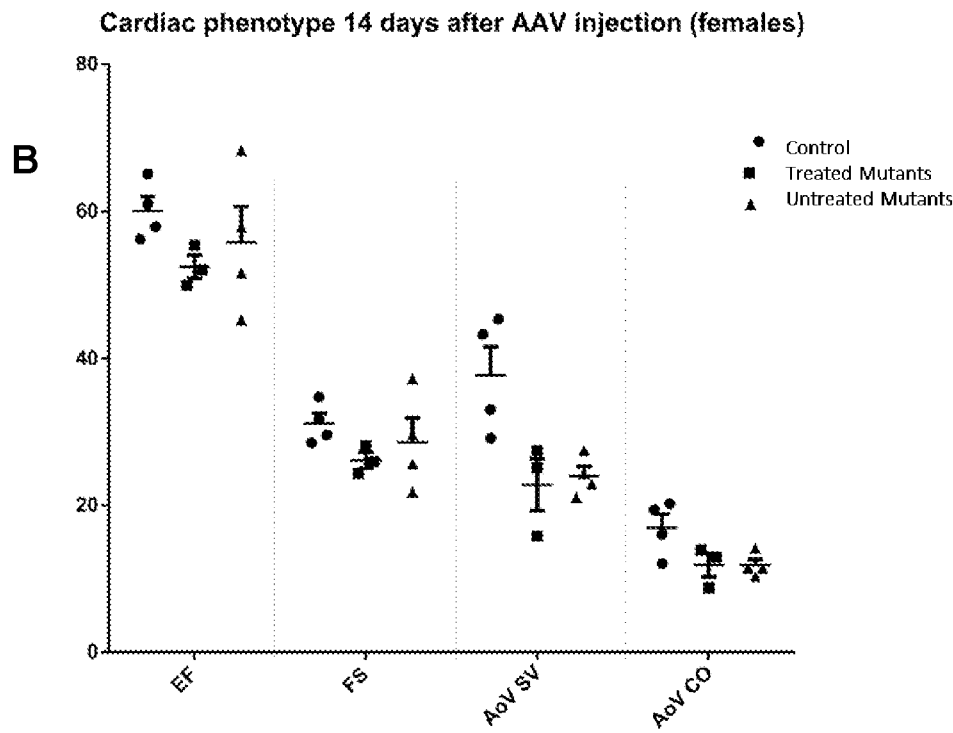
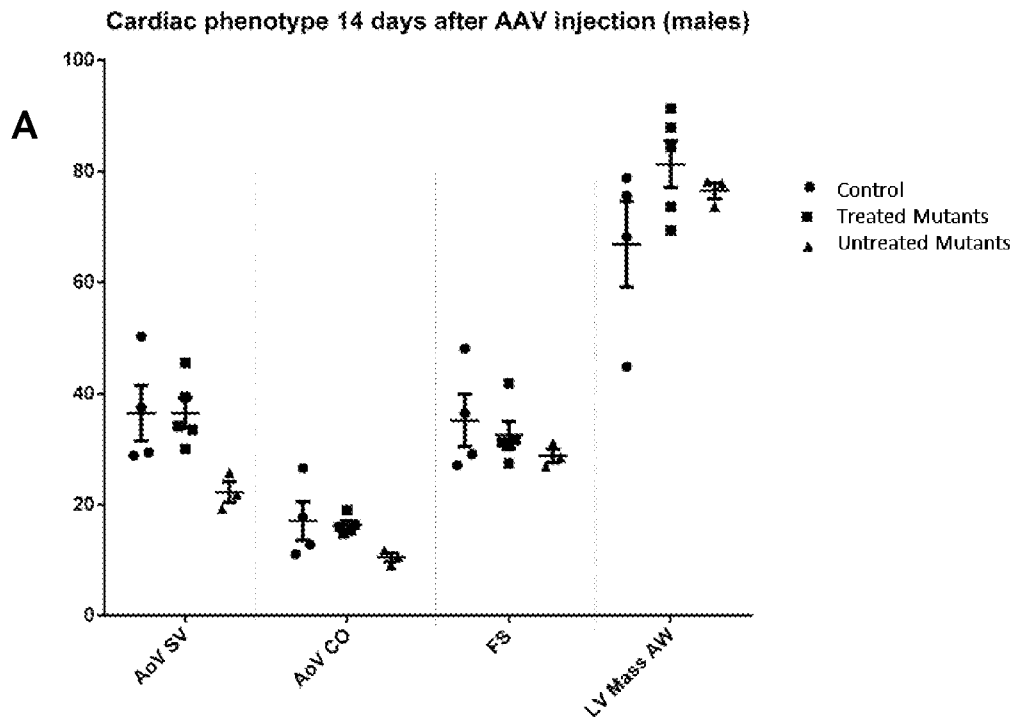


FIGURE 3



FIGURES 4A-4B



FIGURES 5A-5B

FIGURES 6A-6C

● Control (M+F)
● Treated Mutants (M+F)
● Untreated Mutants (M+F)

