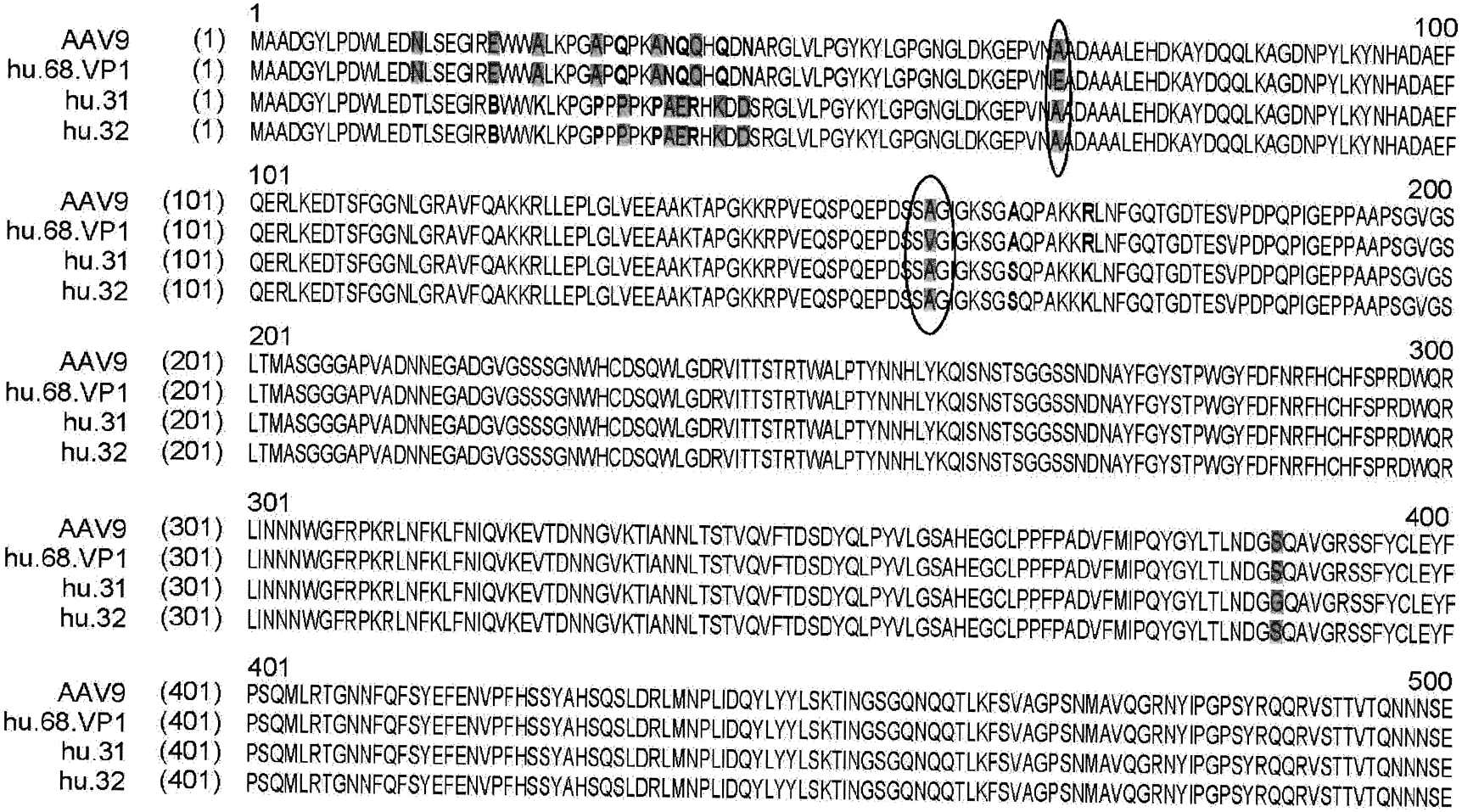

1
100
AAV9 (1) MAADGYLPDWLEDNLSEGIREWWALKPGAPQPKANQQHQDNARGLVLPGYKYLGPGNGLDKGEPVNAADAAALEHDKAYDQQLKAGDNPYLKYNHADAEF
hu.68.VP1 (1) MAADGYLPDWLEDNLSEGIREWWALKPGAPQPKANQQHQDNARGLVLPGYKYLGPGNGLDKGEPVNEADAAALEHDKAYDQQLKAGDNPYLKYNHADAEF
hu.31 (1) MAADGYLPDWLEDTLSEGIRBWWKLKPGPPPPKPAERHKDDSRGLVLPGYKYLGPGNGLDKGEPVNAADAAALEHDKAYDQQLKAGDNPYLKYNHADAEF
hu.32 (1) MAADGYLPDWLEDTLSEGIRBWWKLKPGPPPPKPAERHKDDSRGLVLPGYKYLGPGNGLDKGEPVNAADAAALEHDKAYDQQLKAGDNPYLKYNHADAEF
101
200
AAV9 (101) QERLKEDTSFGGNLGRAVFQAKKRLLEPLGLVEEAAKTAPGKKRPVEQSPQEPDSSAGIGKSGAQPAKKRLNFGQTGDTESVPDPQPIGEPPAAPSGVGS
hu.68.VP1 (101) QERLKEDTSFGGNLGRAVFQAKKRLLEPLGLVEEAAKTAPGKKRPVEQSPQEPDSSVGIGKSGAQPAKKRLNFGQTGDTESVPDPQPIGEPPAAPSGVGS
hu.31 (101) QERLKEDTSFGGNLGRAVFQAKKRLLEPLGLVEEAAKTAPGKKRPVEQSPQEPDSSAGIGKSGSQPAKKKLNFGQTGDTESVPDPQPIGEPPAAPSGVGS
hu.32 (101) QERLKEDTSFGGNLGRAVFQAKKRLLEPLGLVEEAAKTAPGKKRPVEQSPQEPDSSAGIGKSGSQPAKKKLNFGQTGDTESVPDPQPIGEPPAAPSGVGS
201
300
AAV9 (201) LTMASGGGAPVADNNEGADGVGSSSGNWHCDSQWLGDRVITTSTRTWALPTYNNHLYKQISNSTSGGSSNDNAYFGYSTPWGYFDFNRFHCHFSPRDWQR
hu.68.VP1 (201) LTMASGGGAPVADNNEGADGVGSSSGNWHCDSQWLGDRVITTSTRTWALPTYNNHLYKQISNSTSGGSSNDNAYFGYSTPWGYFDFNRFHCHFSPRDWQR
hu.31 (201) LTMASGGGAPVADNNEGADGVGSSSGNWHCDSQWLGDRVITTSTRTWALPTYNNHLYKQISNSTSGGSSNDNAYFGYSTPWGYFDFNRFHCHFSPRDWQR
hu.32 (201) LTMASGGGAPVADNNEGADGVGSSSGNWHCDSQWLGDRVITTSTRTWALPTYNNHLYKQISNSTSGGSSNDNAYFGYSTPWGYFDFNRFHCHFSPRDWQR
301
400
AAV9 (301) LINNNWGFRPKRLNFKLFNIQVKEVTDNNGVKTIANNLTSTVQVFTDSDYQLPYVLGSAHEGCLPPFPADVFMIPQYGYLTLNDGSQAVGRSSFYCLEYF
hu.68.VP1 (301) LINNNWGFRPKRLNFKLFNIQVKEVTDNNGVKTIANNLTSTVQVFTDSDYQLPYVLGSAHEGCLPPFPADVFMIPQYGYLTLNDGSQAVGRSSFYCLEYF
hu.31 (301) LINNNWGFRPKRLNFKLFNIQVKEVTDNNGVKTIANNLTSTVQVFTDSDYQLPYVLGSAHEGCLPPFPADVFMIPQYGYLTLNDGCQAVGRSSFYCLEYF
hu.32 (301) LINNNWGFRPKRLNFKLFNIQVKEVTDNNGVKTIANNLTSTVQVFTDSDYQLPYVLGSAHEGCLPPFPADVFMIPQYGYLTLNDGSQAVGRSSFYCLEYF
401
500
AAV9 (401) PSQMLRTGNNFQFSYEFENVPFHSSYAHSQSLDRLMNPLIDQYLYYLSKTINGSGQNQQTLKFSVAGPSNMAVQGRNYIPGPSYRQQRVSTTVTQNNNSE
hu.68.VP1 (401) PSQMLRTGNNFQFSYEFENVPFHSSYAHSQSLDRLMNPLIDQYLYYLSKTINGSGQNQQTLKFSVAGPSNMAVQGRNYIPGPSYRQQRVSTTVTQNNNSE
hu.31 (401) PSQMLRTGNNFQFSYEFENVPFHSSYAHSQSLDRLMNPLIDQYLYYLSKTINGSGQNQQTLKFSVAGPSNMAVQGRNYIPGPSYRQQRVSTTVTQNNNSE
hu.32 (401) PSQMLRTGNNFQFSYEFENVPFHSSYAHSQSLDRLMNPLIDQYLYYLSKTINGSGQNQQTLKFSVAGPSNMAVQGRNYIPGPSYRQQRVSTTVTQNNNSE

FIG 1-1

```
AAV9        (501)  FAWPGASSWALNGRNSLMNPGPAMASHKEGEDRFFPLSGSLIFGKQGTGRDNVDADKVMITNEEEIKTTNPVATESYGQVATNHQSAQAQAQTGWVQNQG
hu.68.VP1   (501)  FAWPGASSWALNGRNSLMNPGPAMASHKEGEDRFFPLSGSLIFGKQGTGRDNVDADKVMITNEEEIKTTNPVATESYGQVATNHQSAQAQAQTGWVQNQG
hu.31       (501)  FAWPGASSWALNGRNSLMNPGPAMASHKEGEDRFFPLSGSLIFGKQGTGRDNVDADKVMITNEEEIKTTNPVATESYGQVATNHQSAQAQAQTGWVQNQG
hu.32       (501)  FAWPGASSWALNGRNSLMNPGPAMASHKEGEDRFFPLSGSLIFGKQGTGRDNVDADKVMITNEEEIKTTNPVATESYGQVATNHQSAQAQAQTGWVQNQG

                   601                                                                                              700
AAV9        (601)  ILPGMVWQDRDVYLQGPIWAKIPHTDGNFHPSPLMGGFMKHPPPQILIKNTPVPADPPTAFNKDKLNSFITQYSTGQVSVEIEWELQKENSKRWNPEIQ
hu.68.VP1   (601)  ILPGMVWQDRDVYLQGPIWAKIPHTDGNFHPSPLMGGFMKHPPPQILIKNTPVPADPPTAFNKDKLNSFITQYSTGQVSVEIEWELQKENSKRWNPEIQ
hu.31       (601)  ILPGMVWQDRDVYLQGPIWAKIPHTDGNFHPSPLMGGFMKHPPPQILIKNTPVPADPPTAFNKDKLNSFITQYSTGQVSVEIEWELQKENSKRWNPEIQ
hu.32       (601)  ILPGMVWQDRDVYLQGPIWAKIPHTDGNFHPSPLMGGFMKHPPPQILIKNTPVPADPPTAFNKDKLNSFITQYSTGQVSVEIEWELQKENSKRWNPEIQ

                   701                                  736
AAV9        (701)  YTSNYYKSNNVEFAVNTEGVYSEPRPIGTRYLTRNL
hu.68.VP1   (701)  YTSNYYKSNNVEFAVNTEGVYSEPRPIGTRYLTRNL
hu.31       (701)  YTSNYYKSNNVEFAVSTEGVYSEPRPIGTRYLTRNL
hu.32       (701)  YTSNYYKSNNVEFAVNTEGVYSEPRPIGTRYLTRNL
```

FIG 1-2


1 100
AAV9 (1) ATGGCTGCCGATGGTTATCTTCCAGATTGGCTCGAGGACAACCTTAGTGAAGGAATTCGCGAGTGGTGGGCTTTGAAACCTGGAGCCCCTCAACCCAAGG
hu.68.VP1 (1) ATGGCTGCCGATGGTTATCTTCCAGATTGGCTCGAGGACAACCTCAGTGAAGGCATTCGCGAGTGGTGGGCTTTGAAACCTGGAGCCCCTCAACCCAAGG
hu.31 (1) ATGGCTGCCGATGGTTATCTTCCAGATTGGCTCGAGGACACTCTCTCTGAAGGAATAAGACAGTGGTGGAAGCTCAAACCTGGCCCCCCACCACCAAAGC
hu.32 (1) ATGGCTGCCGATGGTTATCTTCCAGATTGGCTCGAGGACACTCTCTCTGAAGGAATAAGACAGTGGTGGAAGCTCAAACCTGGCCCACCACCACCAAAGC
101 200
AAV9 (101) CAAATCAACAACATCAAGACAACGCTCGAGGTCTTGTGCTTCCGGGTTACAAATACCTTGGACCCGGCAACGGACTCGACAAGGGGGAGCCGGTCAACGC
hu.68.VP1 (101) CAAATCAACAACATCAAGACAACGCTCGGGGTCTTGTGCTTCCGGGTTACAAATACCTTGGACCCGGCAACGGACTCGACAAGGGGGAGCCGGTCAACGA
hu.31 (101) CCGCAGAGCGGCATAAGGACGACAGCAGGGGTCTTGTGCTTCCTGGGTACAAGTACCTCGGACCCGGCAACGGACTCGACAAGGGGGAGCCGGTCAACGC
hu.32 (101) CCGCAGAGCGGCATAAGGACGACAGCAGGGGTCTTGTGCTTCCTGGGTACAAGTACCTCGGACCCGGCAACGGACTCGACAAGGGGGAGCCGGTCAACGC
201 300
AAV9 (201) AGCAGACGCGGCGGCCCTCGAGCACGACAAGGCCTACGACCAGCAGCTCAAGGCCGGAGACAACCCGTACCTCAAGTACAACCACGCCGACGCCGAGTTC
hu.68.VP1 (201) AGCAGACGCGGCGGCCCTCGAGCACGACAAGGCCTACGACCAGCAGCTCAAGGCCGGAGACAACCCGTACCTCAAGTACAACCACGCCGACGCCGAGTTC
hu.31 (201) AGCAGACGCGGCGGCCCTCGAGCACGACAAGGCCTACGACCAGCAGCTCAAGGCCGGAGACAACCCGTACCTCAAGTACAACCACGCCGACGCCGAGTTC
hu.32 (201) AGCAGACGCGGCGGCCCTCGAGCACGACAAGGCCTACGACCAGCAGCTCAAGGCCGGAGACAACCCGTACCTCAAGTACAACCACGCCGACGCCGAGTTC
301 400
AAV9 (301) CAGGAGCGGCTCAAAGAAGATACGTCTTTTGGGGGCAACCTCGGGCGAGCAGTCTTCCAGGCCAAAAAGAGGCTTCTTGAACCTCTTGGTCTGGTTGAGG
hu.68.VP1 (301) CAGGAGCGGCTCAAAGAAGATACGTCTTTTGGGGGCAACCTCGGGCGAGCAGTCTTCCAGGCCAAAAAGAGGCTTCTTGAACCTCTTGGTCTGGTTGAGG
hu.31 (301) CAGGAGCGGCTCAAAGAAGATACGTCTTTTGGGGGCAACCTCGGGCGAGCAGTCTTCCAGGCCAAAAAGAGGCTTCTTGAACCTCTTGGTCTGGTTGAGG
hu.32 (301) CAGGAGCGGCTCAAAGAAGATACGTCTTTTGGGGGCAACCTCGGGCGAGCAGTCTTCCAGGCCAAAAAGAGGCTTCTTGAACCTCTTGGTCTGGTTGAGG
401 500
AAV9 (401) AAGCGGCTAAGACGGCTCCTGGAAAGAAGAGGCTGTAGAGCAGTCTCCTCAGGAACCGGACTCCTCCGCGGGTATTGGCAAATCGGGTGCACAGCCCGC
hu.68.VP1 (401) AAGCGGCTAAGACGGCTCCTGGAAAGAAGAGGCTGTAGAGCAGTCTCCTCAGGAACCGGACTCCTCCGTGGGTATTGGCAAATCGGGTGCACAGCCCGC
hu.31 (401) AAGCGGCTAAGACGGCTCCTGGAAAGAAGAGGCTGTAGAGCAGTCTCCTCAGGAACCGGACTCCTCCGCGGGTATTGGCAAATCGGGTTCACAGCCCGC
hu.32 (401) AAGCGGCTAAGACGGCTCCTGGAAAGAAGAGGCTGTAGAGCAGTCTCCTCAGGAACCGGACTCCTCCGCGGGTATTGGCAAATCGGGTTCACAGCCCGC

FIG 2A-1


501
600
AAV9 (501) TAAAAAGAGACTCAATTTCGGTCAGACTGGCGACACAGAGTCAGTCCCAGACCCTCAACCAATCGGAGAACCTCCCGCAGCCCCCTCAGGTGTGGGATCT
hu.68.VP1 (501) TAAAAAGAGACTCAATTTCGGTCAGACTGGCGACACAGAGTCAGTCCCCGACCCTCAACCAATCGGAGAACCTCCCGCAGCCCCCTCAGGTGTGGGATCT
hu.31 (501) TAAAAAGAAACTCAATTTCGGTCAGACTGGCGACACAGAGTCAGTCCCCGACCCTCAACCAATCGGAGAACCTCCCGCAGCCCCCTCAGGTGTGGGATCT
hu.32 (501) TAAAAAGAAACTCAATTTCGGTCAGACTGGCGACACAGAGTCAGTCCCCGACCCTCAACCAATCGGAGAACCTCCCGCAGCCCCCTCAGGTGTGGGATCT
601
700
AAV9 (601) CTTACAATGGCTTCAGGTGGTGGCGCACCAGTGGCAGACAATAACGAAGGTGCCGATGGAGTGGGTAGTTCCTCGGGAAATTGGCATTGCGATTCCCAAT
hu.68.VP1 (601) CTTACAATGGCTTCAGGTGGTGGCGCACCAGTGGCAGACAATAACGAAGGTGCCGATGGAGTGGGTAGTTCCTCGGGAAATTGGCATTGCGATTCCCAAT
hu.31 (601) CTTACAATGGCTTCAGGTGGTGGCGCACCAGTGGCAGACAATAACGAAGGTGCCGATGGAGTGGGTAGTTCCTCGGGAAATTGGCATTGCGATTCCCAAT
hu.32 (601) CTTACAATGGCTTCAGGTGGTGGCGCACCAGTGGCAGACAATAACGAAGGTGCCGATGGAGTGGGTAGTTCCTCGGGAAATTGGCATTGCGATTCCCAAT
701
800
AAV9 (701) GGCTGGGGGACAGAGTCATCACCACCAGCACCCGAACCTGGGCCCTGCCCACCTACAACAATCACCTCTACAAGCAAATCTCCAACAGCACATCTGGAGG
hu.68.VP1 (701) GGCTGGGGGACAGAGTCATCACCACCAGCACCCGAACCTGGGCCCTGCCCACCTACAACAATCACCTCTACAAGCAAATCTCCAACAGCACATCTGGAGG
hu.31 (701) GGCTGGGGGACAGAGTCATCACCACCAGCACCCGAACCTGGGCCCTGCCCACCTACAACAATCACCTCTACAAGCAAATCTCCAACAGCACATCTGGAGG
hu.32 (701) GGCTGGGGGACAGAGTCATCACCACCAGCACCCGAACCTGGGCCCTGCCCACCTACAACAATCACCTCTACAAGCAAATCTCCAACAGCACATCTGGAGG
801
900
AAV9 (801) ATCTTCAAATGACAACGCCTACTTCGGCTACAGCACCCCCTGGGGGTATTTTGACTTCAACAGATTCCACTGCCACTTCTCACCACGTGACTGGCAGCGA
hu.68.VP1 (801) ATCTTCAAATGACAACGCCTACTTCGGCTACAGCACCCCCTGGGGGTATTTTGACTTCAACAGATTCCACTGCCACTTCTCACCACGTGACTGGCAAAGA
hu.31 (801) ATCTTCAAATGACAACGCCTACTTCGGCTACAGCACCCCCTGGGGGTATTTTGACTTCAACAGATTCCACTGCCACTTCTCACCACGTGACTGGCAGCGA
hu.32 (801) ATCTTCAAATGACAACGCCTACTTCGGCTACAGCACCCCCTGGGGGTATTTTGACTTCAACAGATTCCACTGCCACTTCTCACCACGTGACTGGCAGCGA
901
1000
AAV9 (901) CTCATCAACAACAACTGGGGATTCCGGCCTAAGCGACTCAACTTCAAGCTCTTCAACATTCAGGTCAAAGAGGTTACGGACAACAATGGAGTCAAGACCA
hu.68.VP1 (901) CTCATCAACAACAACTGGGGATTCCGGCCTAAGCGACTCAACTTCAAGCTCTTCAACATTCAGGTCAAAGAGGTTACGGACAACAATGGAGTCAAGACCA
hu.31 (901) CTCATCAACAACAACTGGGGATTCCGGCCTAAGCGACTCAACTTCAAGCTCTTCAACATTCAGGTCAAAGAGGTTACGGACAACAATGGAGTCAAGACCA
hu.32 (901) CTCATCAACAACAACTGGGGATTCCGGCCTAAGCGACTCAACTTCAAGCTCTTCAACATTCAGGTCAAAGAGGTTACGGACAACAATGGAGTCAAGACCA

FIG 2A-2


901
1000
AAV9 (901) CTCATCAACAACAACTGGGGATTCCGGCCTAAGCGACTCAACTTCAAGCTCTTCAACATTCAGGTCAAAGAGGTTACGGACAACAATGGAGTCAAGACCA
hu.68.VP1 (901) CTCATCAACAACAACTGGGGATTCCGGCCTAAGCGACTCAACTTCAAGCTCTTCAACATTCAGGTCAAAGAGGTTACGGACAACAATGGAGTCAAGACCA
hu.31 (901) CTCATCAACAACAACTGGGGATTCCGGCCTAAGCGACTCAACTTCAAGCTCTTCAACATTCAGGTCAAAGAGGTTACGGACAACAATGGAGTCAAGACCA
hu.32 (901) CTCATCAACAACAACTGGGGATTCCGGCCTAAGCGACTCAACTTCAAGCTCTTCAACATTCAGGTCAAAGAGGTTACGGACAACAATGGAGTCAAGACCA
1001
1100
AAV9 (1001) TCGCCAATAACCTTACCAGCACGGTCCAGGTCTTCACGGACTCAGACTATCAGCTCCCGTACGTGCTCGGGTCGGCTCACGAGGGCTGCCTCCCGCCGTT
hu.68.VP1 (1001) TCGCTAATAACCTTACCAGCACGGTCCAGGTCTTCACGGACTCAGACTATCAGCTCCCGTACGTGCTCGGGTCGGCTCACGAGGGCTGCCTCCCGCCGTT
hu.31 (1001) TCGCCAATAACCTTACCAGCACGGTCCAGGTCTTCACGGACTCAGACTATCAGCTCCCGTACGTGCTCGGGTCGGCTCACGAGGGCTGCCTCCCGCCGTT
hu.32 (1001) TCGCCAATAACCTTACCAGCACGGTCCAGGTCTTCACGGACTCAGACTATCAGCTCCCGTACGTGCTCGGGTCGGCTCACGAGGGCTGCCTCCCGCCGTT
1101
1200
AAV9 (1101) CCCAGCGGACGTTTTCATGATTCCTCAGTACGGGTATCTGACGCTTAATGATGGAAGCCAGGCCGTGGGTCGTTCGTCCTTTTACTGCCTGGAATATTTC
hu.68.VP1 (1101) CCCAGCGGACGTTTTCATGATTCCTCAGTACGGGTATCTAACGCTTAATGATGGAAGCCAAGCCGTGGGTCGTTCGTCCTTTTACTGCCTGGAATATTTC
hu.31 (1101) CCCAGCGGACGTTTTCATGATTCCTCAGTACGGGTATCTGACGCTTAATGATGGAGGCCAGGCCGTGGGTCGTTCGTCCTTTTACTGCCTGGAATATTTC
hu.32 (1101) CCCAGCGGACGTTTTCATGATTCCTCAGTACGGGTATCTGACGCTTAATGATGGGAGCCAGGCCGTGGGTCGTTCGTCCTTTTACTGCCTGGAATATTTC
1201
1300
AAV9 (1201) CCGTCGCAAATGCTAAGAACGGGTAACAACTTCCAGTTCAGCTACGAGTTTGAGAACGTACCTTTCCATAGCAGCTACGCTCACAGCCAAAGCCTGGACC
hu.68.VP1 (1201) CCGTCGCAAATGCTAAGAACGGGTAACAACTTCCAGTTCAGCTACGAGTTTGAGAACGTACCTTTCCATAGCAGCTATGCTCACAGCCAAAGCCTGGACC
hu.31 (1201) CCGTCGCAAATGCTAAGAACGGGTAACAACTTCCAGTTCAGCTACGAGTTTGAGAACGTACCTTTCCATAGCAGCTACGCTCACAGCCAAAGCCTGGACC
hu.32 (1201) CCGTCGCAAATGCTAAGAACGGGTAACAACTTCCAGTTCAGCTACGAGTTTGAGAACGTACCTTTCCATAGCAGCTACGCTCACAGCCAAAGCCTGGACC
1301
1400
AAV9 (1301) GACTAATGAATCCACTCATCGACCAATACTTGTACTATCTCTCAAAGACTATTAACGGTTCTGGACAGAATCAACAAACGCTAAAATTCAGTGTGGCCGG
hu.68.VP1 (1301) GACTCATGAATCCACTCATCGACCAATACTTGTACTATCTCTCAAAGACTATTAACGGTTCTGGACAGAATCAACAAACGCTAAAATTCAGTGTGGCCGG
hu.31 (1301) GACTAATGAATCCACTCATCGACCAATACTTGTACTATCTCTCAAAGACTATTAACGGTTCTGGACAGAATCAACAAACGCTAAAATTCAGTGTGGCCGG
hu.32 (1301) GACTAATGAATCCACTCATCGACCAATACTTGTACTATCTCTCAAAGACTATTAACGGTTCTGGACAGAATCAACAAACGCTAAAATTCAGCGTGGCCGG

FIG 2B-1


1401
1500
AAV9 (1401) ACCCAGCAACATGGCTGTCCAGGGAAGAAACTACATACCTGGACCCAGCTACCGACAACAACGTGTCTCAACCACTGTGACTCAAAACAACAACAGCGAA
hu.68.VP1 (1401) ACCCAGCAACATGGCTGTCCAGGGAAGAAACTACATACCTGGACCCAGCTACCGACAACAACGTGTCTCAACCACTGTGACTCAAAACAACAACAGCGAA
hu.31 (1401) ACCCAGCAACATGGCTGTCCAGGGAAGAAACTACATACCTGGACCCAGCTACCGACAACAACGTGTCTCAACCACTGTGACTCAAAACAACAACAGCGAA
hu.32 (1401) ACCCAGCAACATGGCTGTCCAGGGAAGAAACTACATACCTGGACCCAGCTACCGACAACAACGTGTCTCAACCACTGTGACTCAAAACAACAACAGCGAA
1501
1600
AAV9 (1501) TTTGCTTGGCCTGGAGCTTCTTCTTGGGCTCTCAATGGACGTAATAGCTTGATGAATCCTGGACCTGCTATGGCCAGCCACAAAGAAGGAGAGGACCGTT
hu.68.VP1 (1501) TTTGCTTGGCCTGGAGCTTCTTCTTGGGCTCTCAATGGACGTAATAGCTTGATGAATCCTGGACCTGCTATGGCCAGCCACAAAGAAGGAGAGGACCGTT
hu.31 (1501) TTTGCTTGGCCTGGAGCTTCTTCTTGGGCTCTCAATGGACGTAATAGCTTGATGAATCCTGGACCTGCTATGGCCAGCCACAAAGAAGGAGAGGACCGTT
hu.32 (1501) TTTGCTTGGCCTGGAGCTTCTTCTTGGGCTCTCAATGGACGTAATAGCTTGATGAATCCTGGACCTGCTATGGCCAGCCACAAAGAAGGAGAGGACCGTT
1601
1700
AAV9 (1601) TCTTTCCTTTGTCTGGATCTTTAATTTTTGGCAAACAAGGAACTGGAAGAGACAACGTGGATGCGGACAAAGTCATGATAACCAACGAAGAAGAAATTAA
hu.68.VP1 (1601) TCTTTCCTTTGTCTGGATCTTTAATTTTTGGCAAACAAGGAACTGGAAGAGACAACGTGGATGCGGACAAAGTCATGATAACCAACGAAGAAGAAATTAA
hu.31 (1601) TCTTTCCTTTGTCCGGATCTTTAATTTTTGGCAAACAAGGAACTGGAAGAGACAACGTGGATGCGGACAAAGTCATGATAACCAACGAAGAAGAAATTAA
hu.32 (1601) TCTTTCCTTTGTCTGGATCTTTAATTTTTGGCAAACAAGGAACTGGAAGAGACAACGTGGATGCGGACAAAGTCATGATAACCAACGAAGAAGAAATTAA
1701
1800
AAV9 (1701) AACTACTAACCCGGTAGCAACGGAGTCCTATGGACAAGTGGCCACAAACCACCAGAGTGCCCAAGCACAGGCGCAGACCGGCTGGGTTCAAAACCAAGGA
hu.68.VP1 (1701) AACTACCAACCCAGTAGCAACGGAGTCCTATGGACAAGTGGCCACAAACCACCAGAGTGCCCAAGCACAGGCGCAGACCGGCTGGGTTCAAAACCAAGGA
hu.31 (1701) AACTACTAACCCGGTAGCAACGGAGTCCTATGGACAAGTGGCCACAAACCACCAGAGTGCCCAAGCACAGGCGCAGACCGGCTGGGTTCAAAACCAAGGA
hu.32 (1701) AACTACTAACCCGGTAGCAACGGAGTCCTATGGACAAGTGGCCACAAACCACCAGAGTGCCCAAGCACAGGCGCAGACCGGCTGGGTTCAAAACCAAGGA

FIG 2B-2

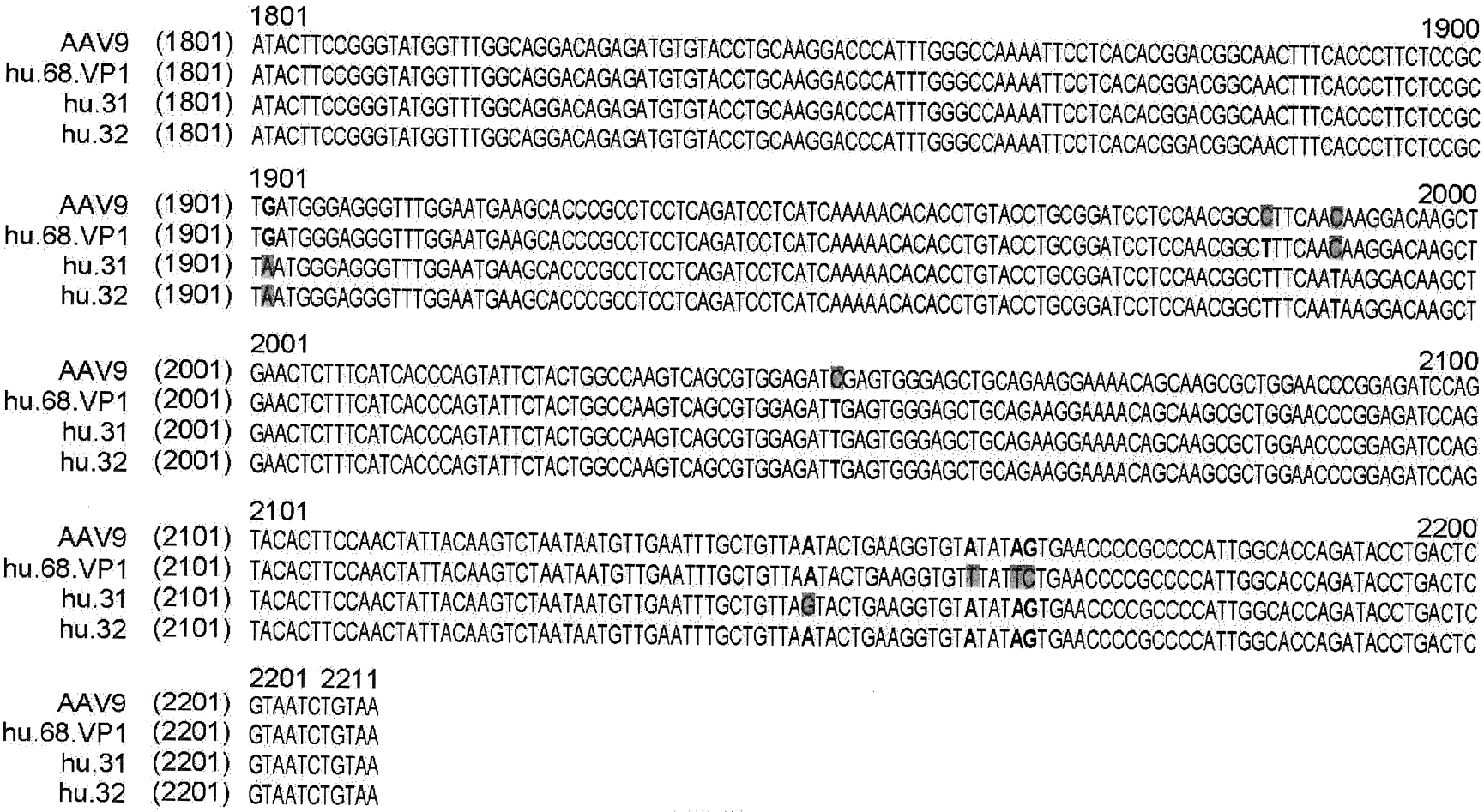

1801
1900
AAV9 (1801) ATACTTCCGGGTATGGTTTGGCAGGACAGAGATGTGTACCTGCAAGGACCCATTTGGGCCAAAATTCCTCACACGGACGGCAACTTTCACCCTTCTCCGC
hu.68.VP1 (1801) ATACTTCCGGGTATGGTTTGGCAGGACAGAGATGTGTACCTGCAAGGACCCATTTGGGCCAAAATTCCTCACACGGACGGCAACTTTCACCCTTCTCCGC
hu.31 (1801) ATACTTCCGGGTATGGTTTGGCAGGACAGAGATGTGTACCTGCAAGGACCCATTTGGGCCAAAATTCCTCACACGGACGGCAACTTTCACCCTTCTCCGC
hu.32 (1801) ATACTTCCGGGTATGGTTTGGCAGGACAGAGATGTGTACCTGCAAGGACCCATTTGGGCCAAAATTCCTCACACGGACGGCAACTTTCACCCTTCTCCGC
1901
2000
AAV9 (1901) TGATGGGAGGGTTTGGAATGAAGCACCCGCCTCCTCAGATCCTCATCAAAAACACACCTGTACCTGCGGATCCTCCAACGGCCTTCAACAAGGACAAGCT
hu.68.VP1 (1901) TGATGGGAGGGTTTGGAATGAAGCACCCGCCTCCTCAGATCCTCATCAAAAACACACCTGTACCTGCGGATCCTCCAACGGCTTTCAACAAGGACAAGCT
hu.31 (1901) TAATGGGAGGGTTTGGAATGAAGCACCCGCCTCCTCAGATCCTCATCAAAAACACACCTGTACCTGCGGATCCTCCAACGGCTTTCAATAAGGACAAGCT
hu.32 (1901) TAATGGGAGGGTTTGGAATGAAGCACCCGCCTCCTCAGATCCTCATCAAAAACACACCTGTACCTGCGGATCCTCCAACGGCTTTCAATAAGGACAAGCT
2001
2100
AAV9 (2001) GAACTCTTTCATCACCCAGTATTCTACTGGCCAAGTCAGCGTGGAGATCGAGTGGGAGCTGCAGAAGGAAAACAGCAAGCGCTGGAACCCGGAGATCCAG
hu.68.VP1 (2001) GAACTCTTTCATCACCCAGTATTCTACTGGCCAAGTCAGCGTGGAGATTGAGTGGGAGCTGCAGAAGGAAAACAGCAAGCGCTGGAACCCGGAGATCCAG
hu.31 (2001) GAACTCTTTCATCACCCAGTATTCTACTGGCCAAGTCAGCGTGGAGATTGAGTGGGAGCTGCAGAAGGAAAACAGCAAGCGCTGGAACCCGGAGATCCAG
hu.32 (2001) GAACTCTTTCATCACCCAGTATTCTACTGGCCAAGTCAGCGTGGAGATTGAGTGGGAGCTGCAGAAGGAAAACAGCAAGCGCTGGAACCCGGAGATCCAG
2101
2200
AAV9 (2101) TACACTTCCAACTATTACAAGTCTAATAATGTTGAATTTGCTGTTAATACTGAAGGTGTATATAGTGAACCCCGCCCCATTGGCACCAGATACCTGACTC
hu.68.VP1 (2101) TACACTTCCAACTATTACAAGTCTAATAATGTTGAATTTGCTGTTAATACTGAAGGTGTTTATTCTGAACCCCGCCCCATTGGCACCAGATACCTGACTC
hu.31 (2101) TACACTTCCAACTATTACAAGTCTAATAATGTTGAATTTGCTGTTAGTACTGAAGGTGTATATAGTGAACCCCGCCCCATTGGCACCAGATACCTGACTC
hu.32 (2101) TACACTTCCAACTATTACAAGTCTAATAATGTTGAATTTGCTGTTAATACTGAAGGTGTATATAGTGAACCCCGCCCCATTGGCACCAGATACCTGACTC
2201 2211
AAV9 (2201) GTAATCTGTAA
hu.68.VP1 (2201) GTAATCTGTAA
hu.31 (2201) GTAATCTGTAA
hu.32 (2201) GTAATCTGTAA

FIG 2C

FIG. 3A

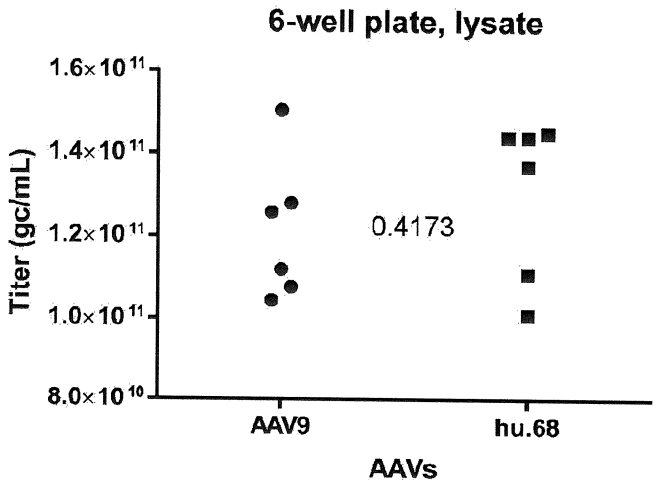

6-well plate, lysate
1.6×10^11
1.4×10^11
1.2×10^11
1.0×10^11
8.0×10^10
Titer (gc/mL)
0.4173
AAV9
hu.68
AAVs

FIG. 3B

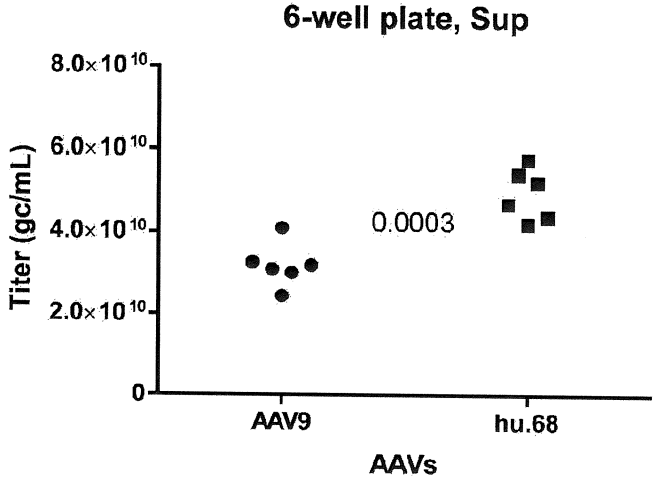
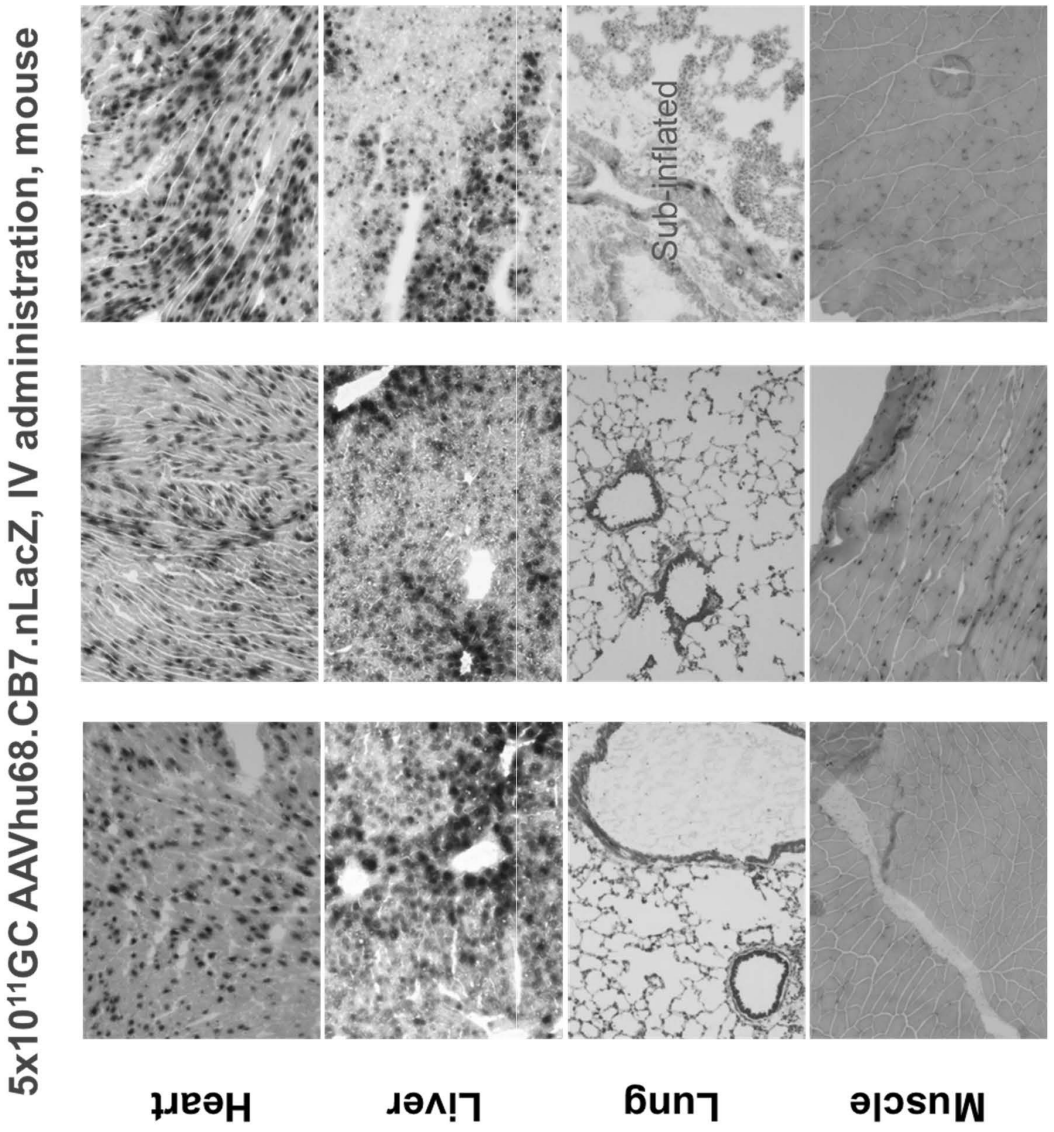

6-well plate, Sup
8.0×10^10
6.0×10^10
4.0×10^10
2.0×10^10
0
Titer (gc/mL)
0.0003
AAV9
hu.68
AAVs

FIG. 4A


5x10^11GC AAVhu68.CB7.nLacZ, IV administration, mouse
Heart
Liver
Lung
Muscle
Sub-inflated

6/20

FIG. 4B

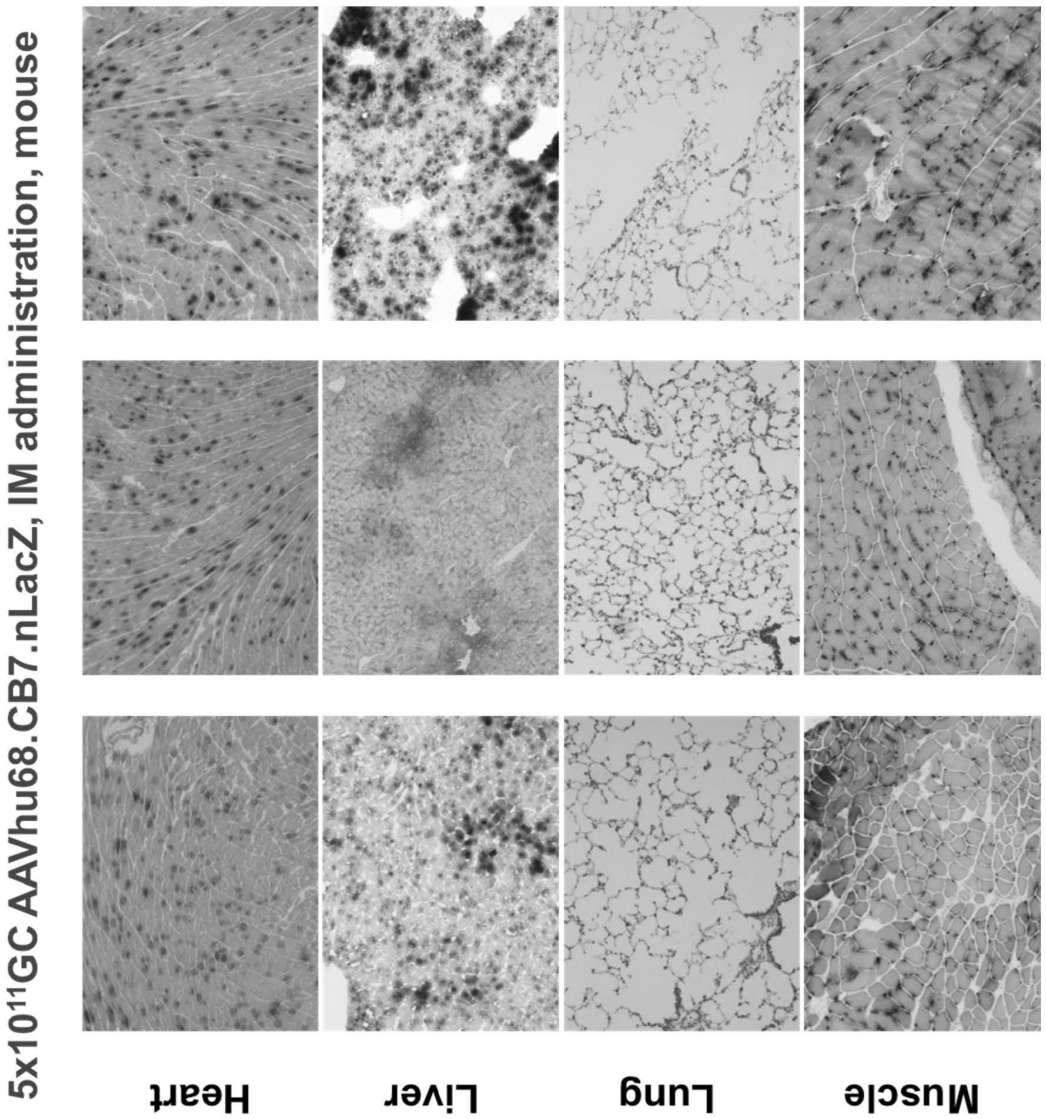

5x10^11GC AAVhu68.CB7.nLacZ, IM administration, mouse
Heart
Liver
Lung
Muscle

FIG. 4C

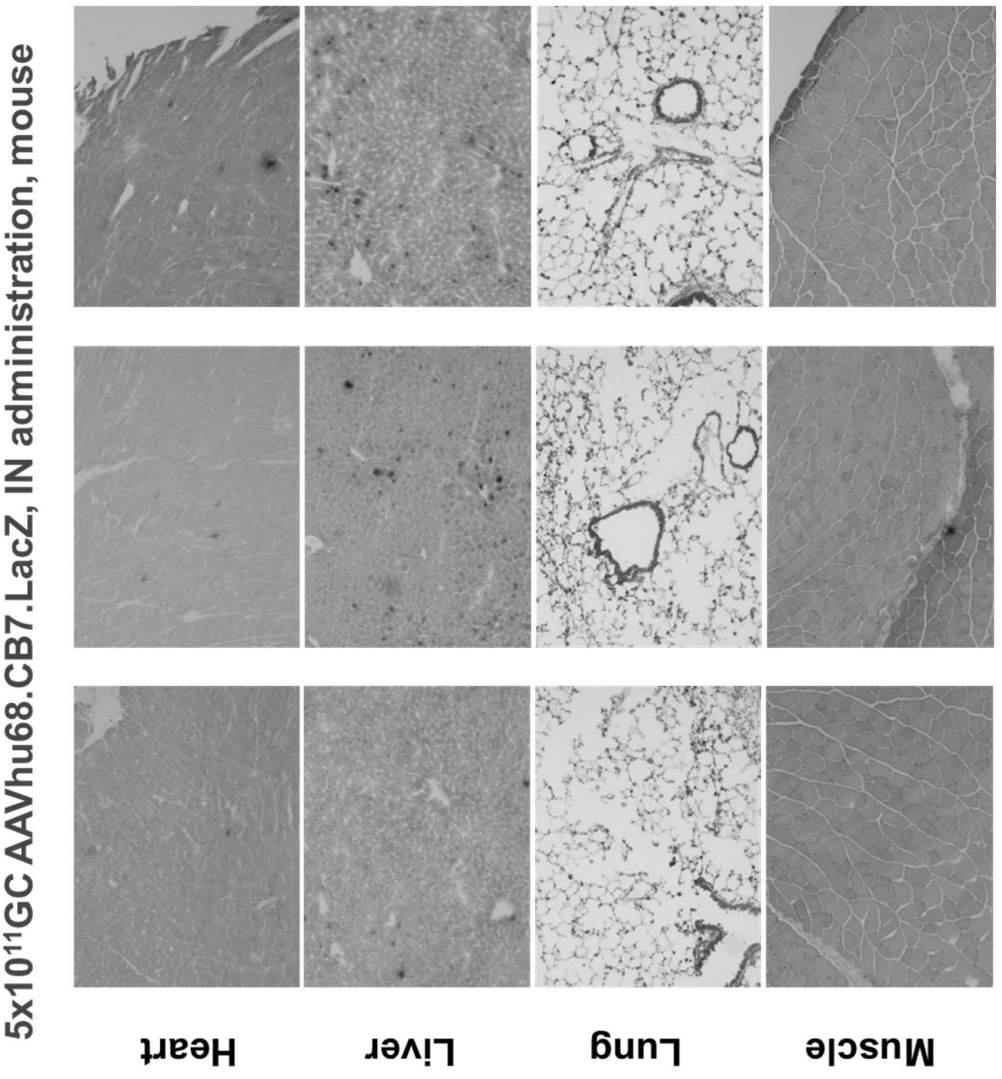
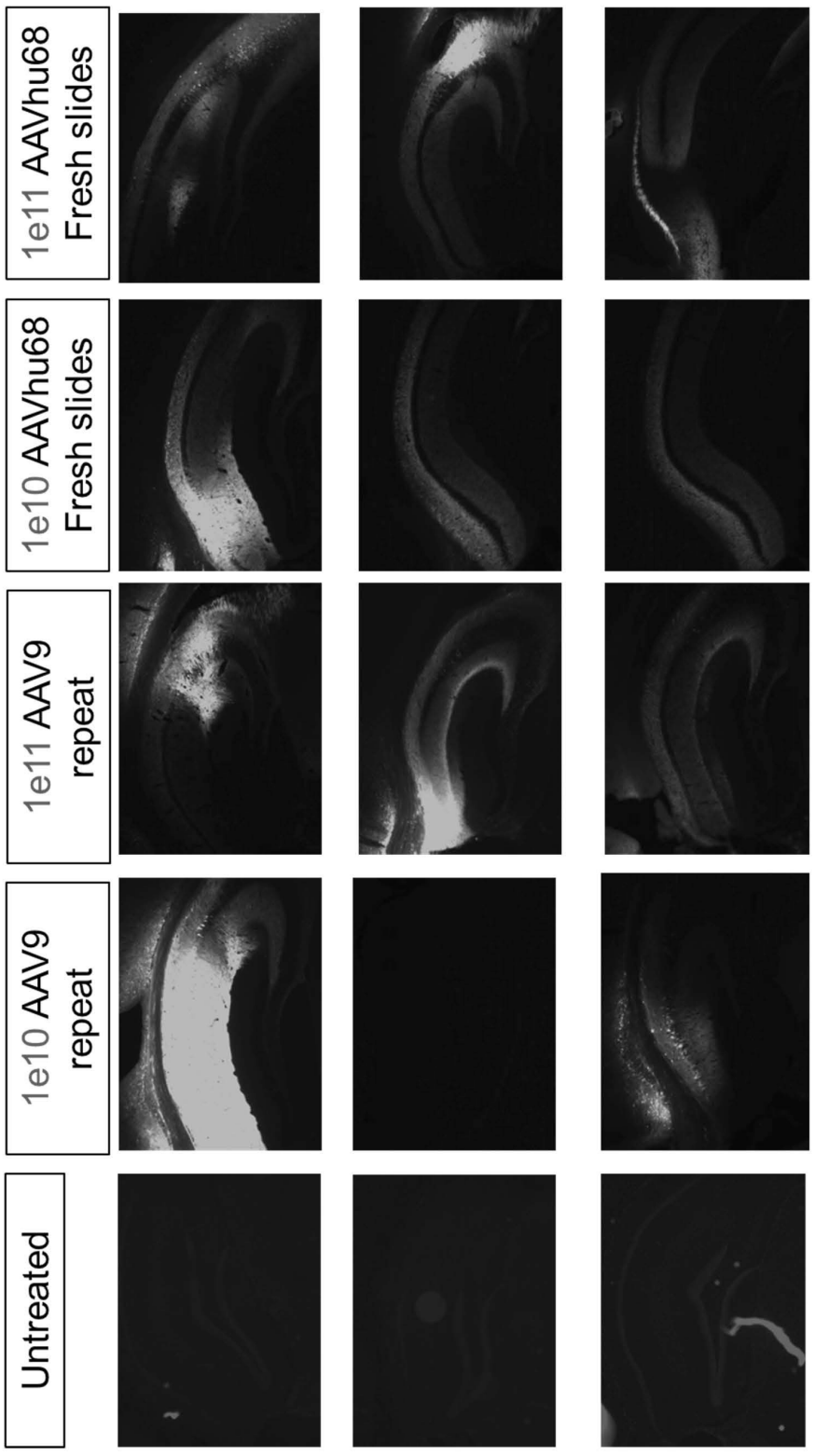

5x10^11GC AAVhu68.CB7.LacZ, IN administration, mouse
Heart
Liver
Lung
Muscle

FIG. 5A


Untreated
1e10 AAV9 repeat
1e11 AAV9 repeat
1e10 AAVhu68 Fresh slides
1e11 AAVhu68 Fresh slides

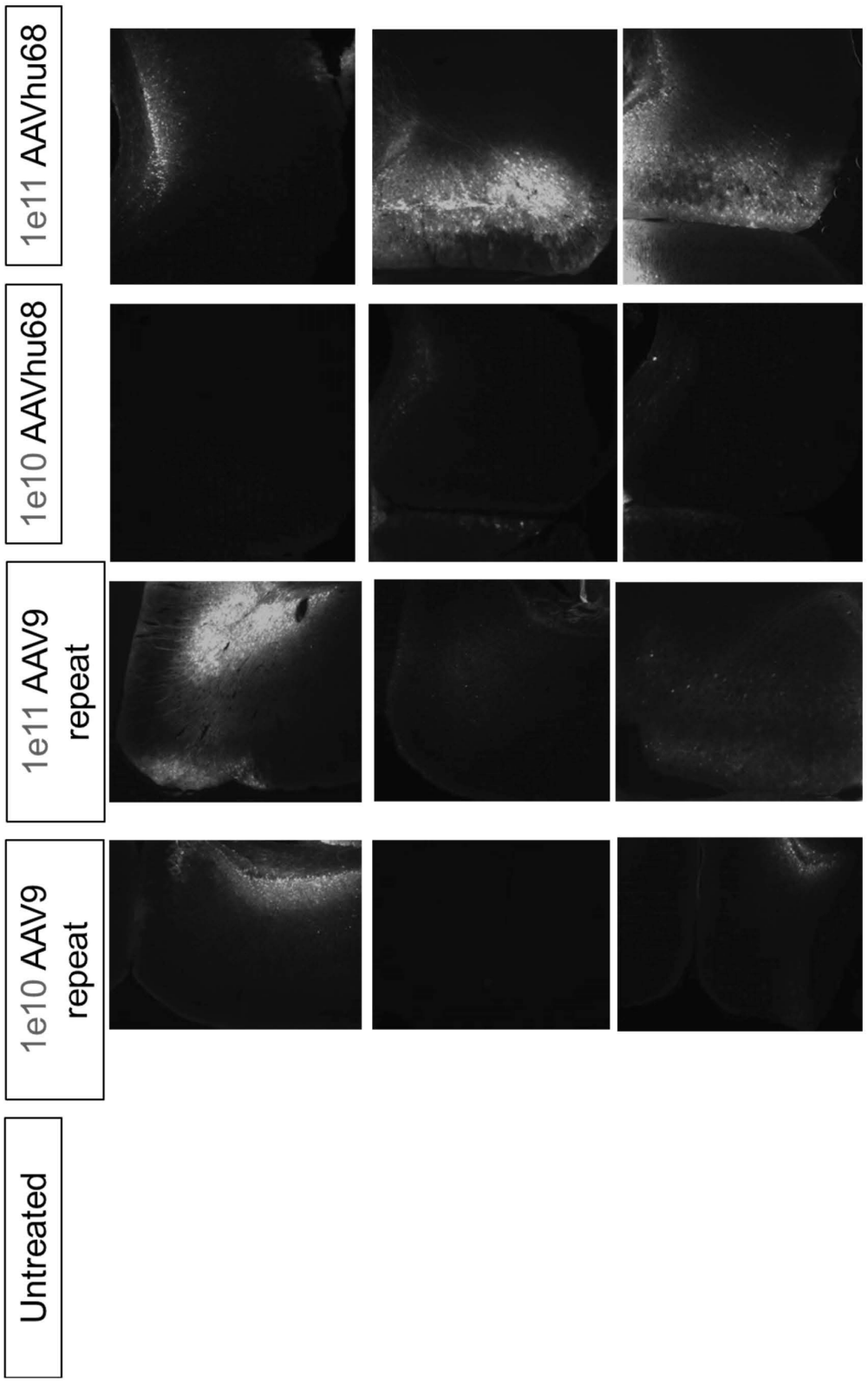

Untreated
1e10 AAV9 repeat
1e11 AAV9 repeat
1e10 AAVhu68
1e11 AAVhu68

FIG. 5B

FIG. 5C

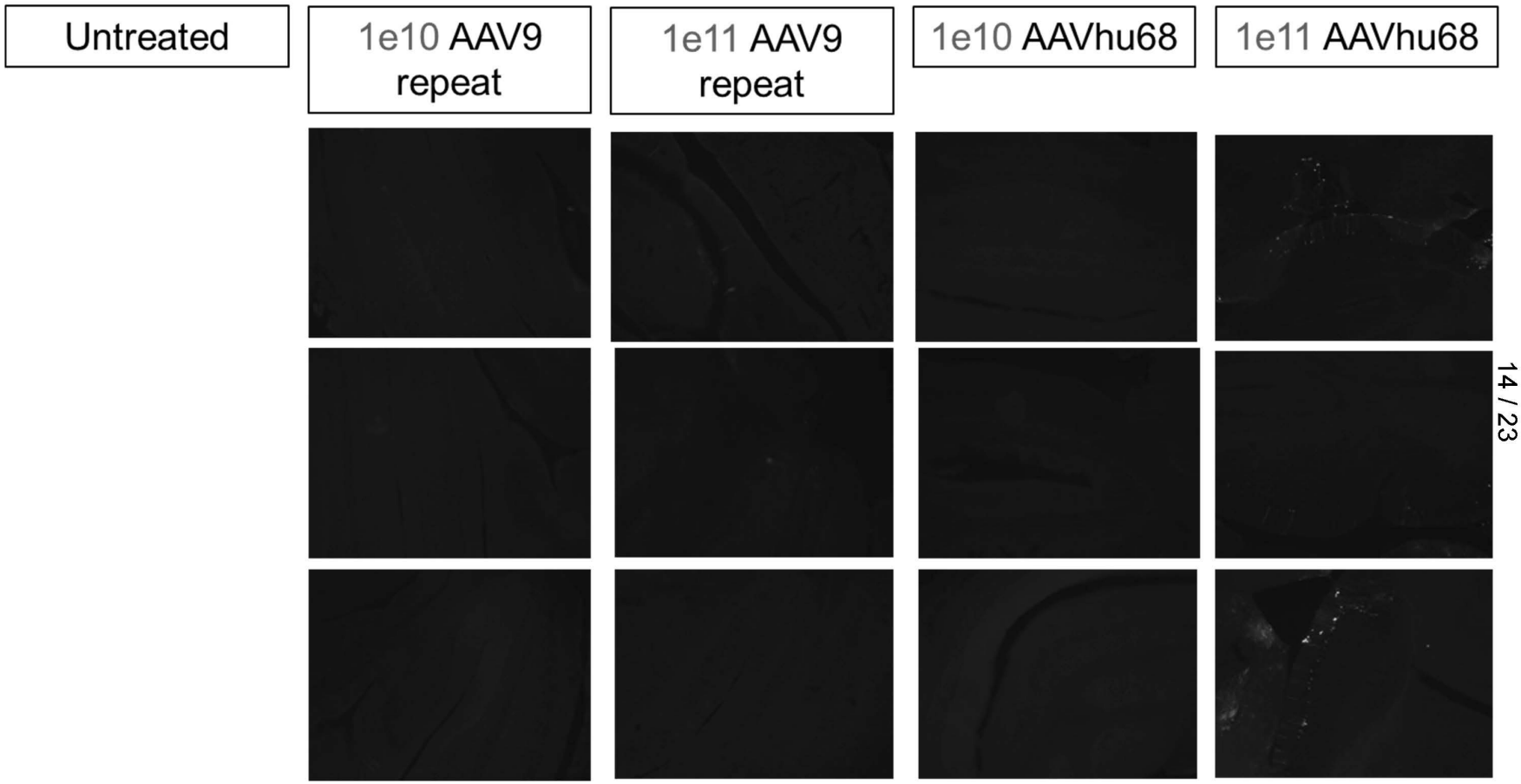
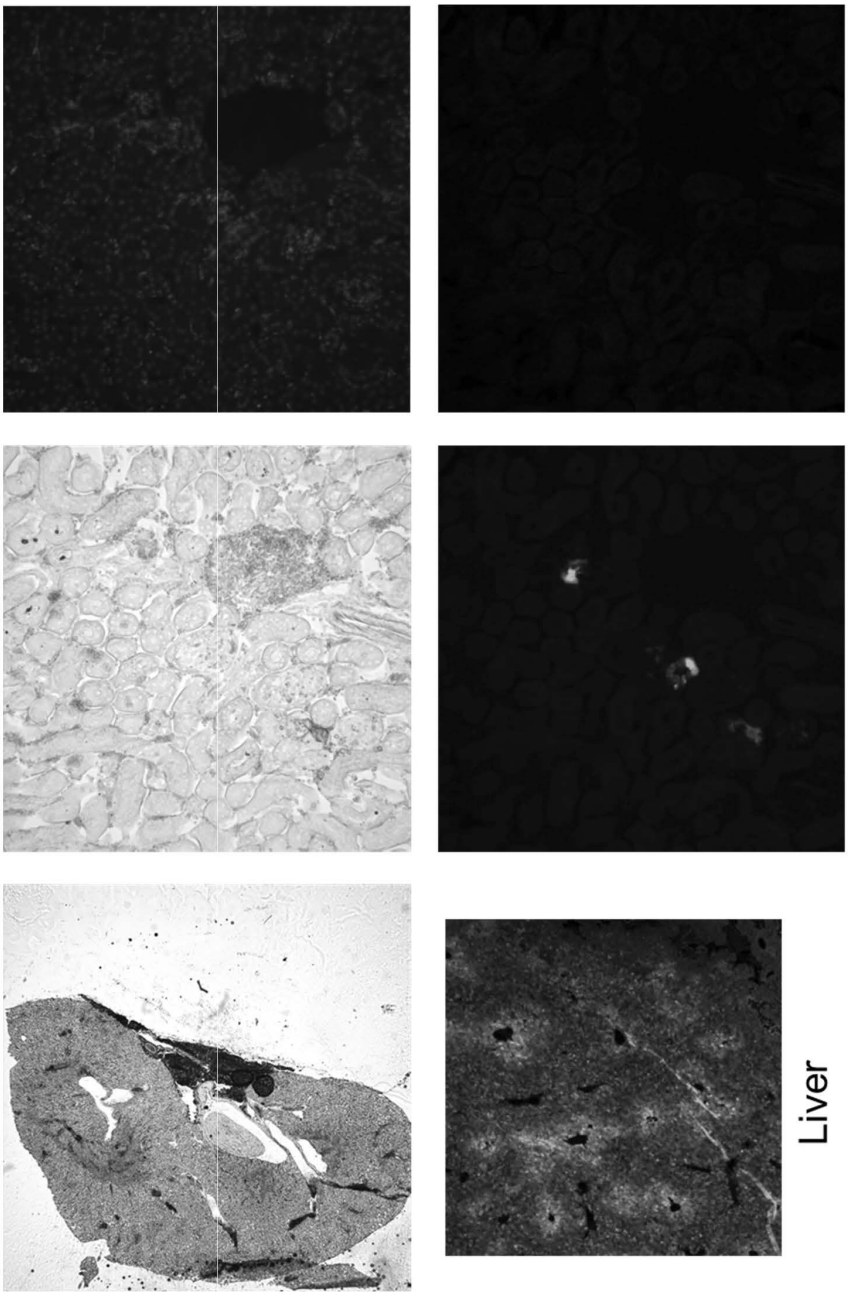

Untreated
1e10 AAV9 repeat
1e11 AAV9 repeat
1e10 AAVhu68
1e11 AAVhu68

FIG. 6A



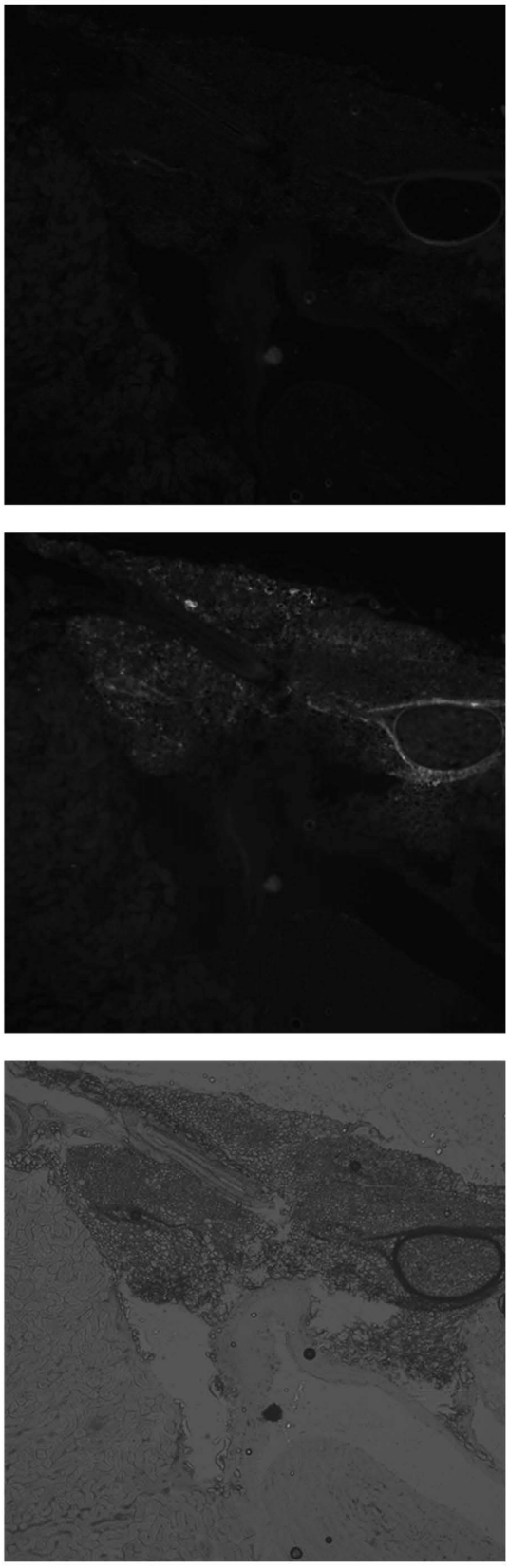


FIG. 6B

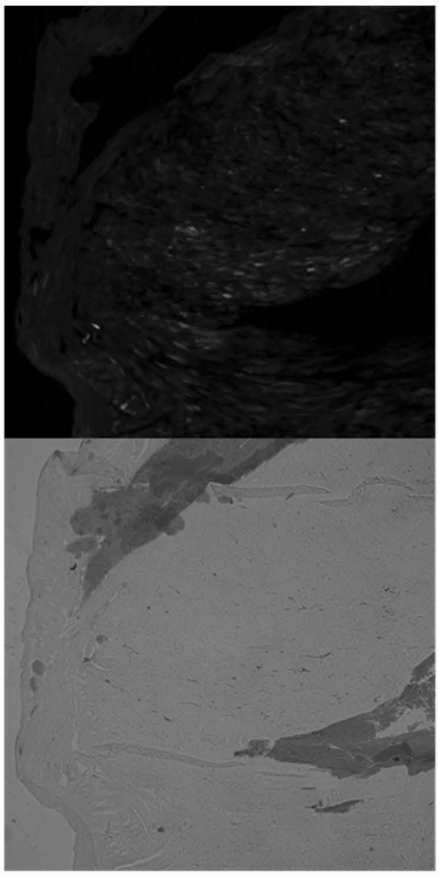


FIG. 6C

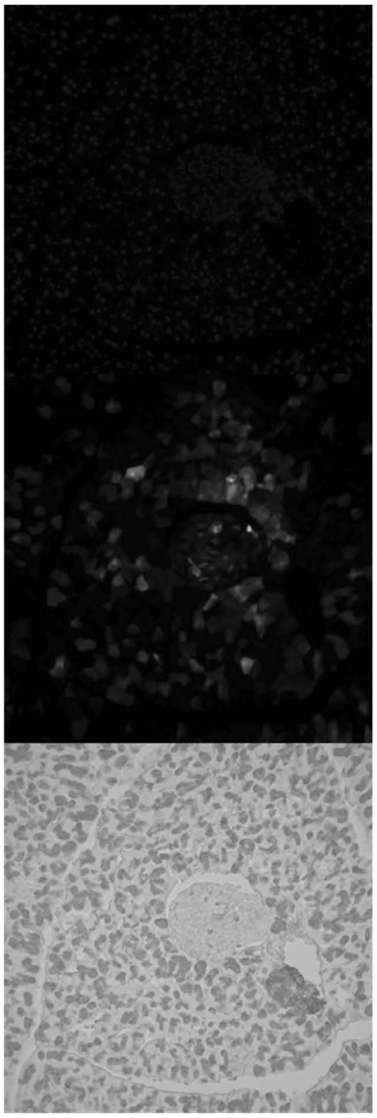


FIG. 6D

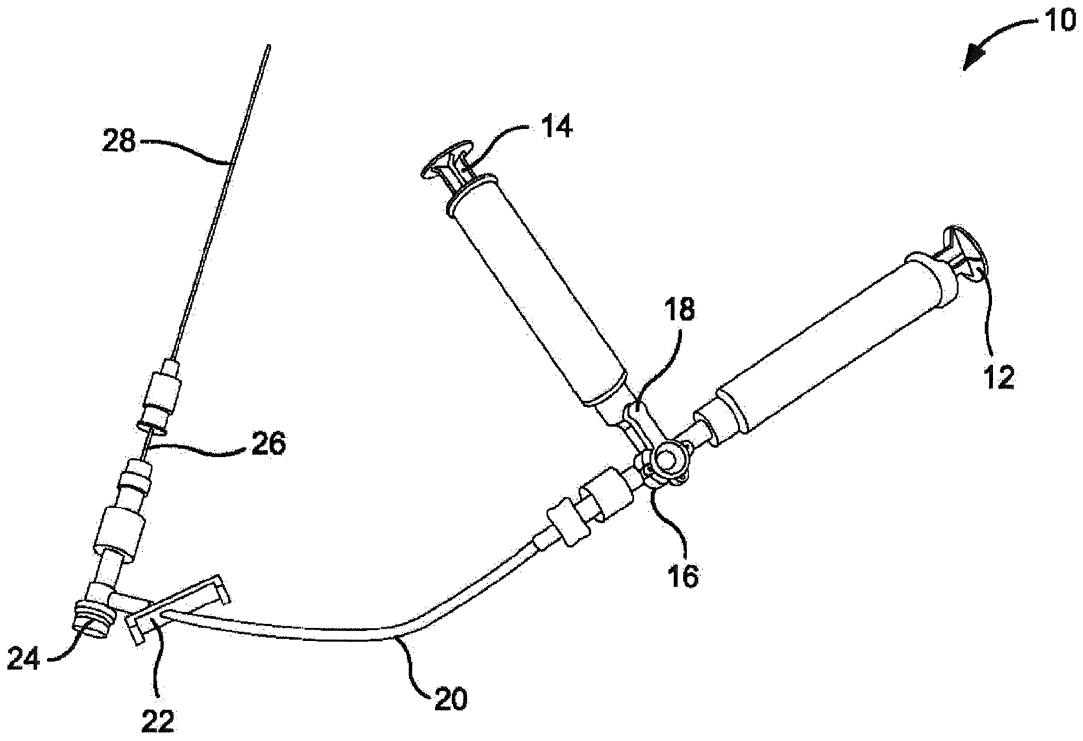


FIG 7

FIG. 8A

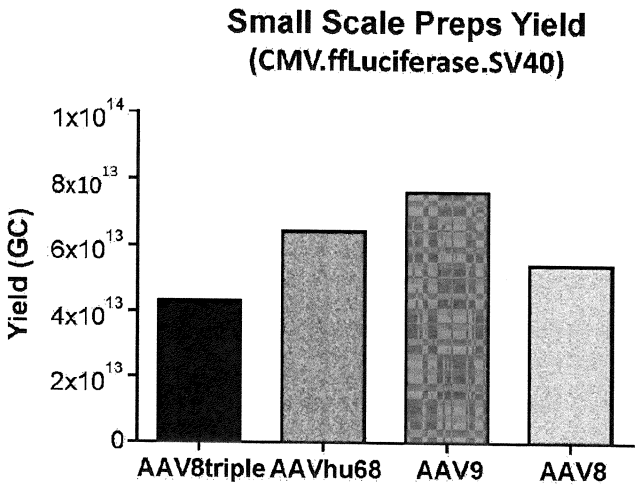

Small Scale Preps Yield
(CMV.ffLuciferase.SV40)
Yield (GC)
1x10^14
8x10^13
6x10^13
4x10^13
2x10^13
0
AAV8triple
AAVhu68
AAV9
AAV8

FIG. 8B

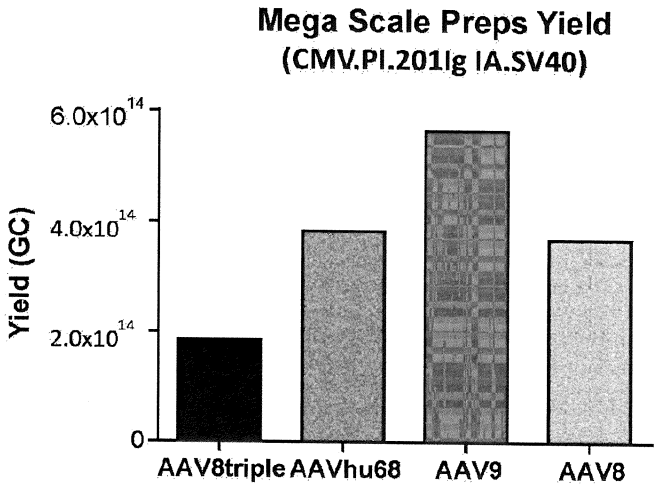

Mega Scale Preps Yield
(CMV.PI.201lg IA.SV40)
Yield (GC)
6.0x10^14
4.0x10^14
2.0x10^14
0
AAV8triple
AAVhu68
AAV9
AAV8

FIG. 9

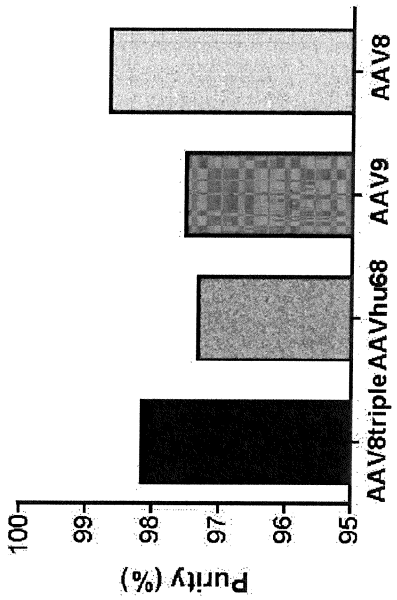

Mega Scale Preps Purity
Purity (%)
100
99
98
97
96
95
AAV8triple
AAVhu68
AAV9
AAV8

FIG. 10A

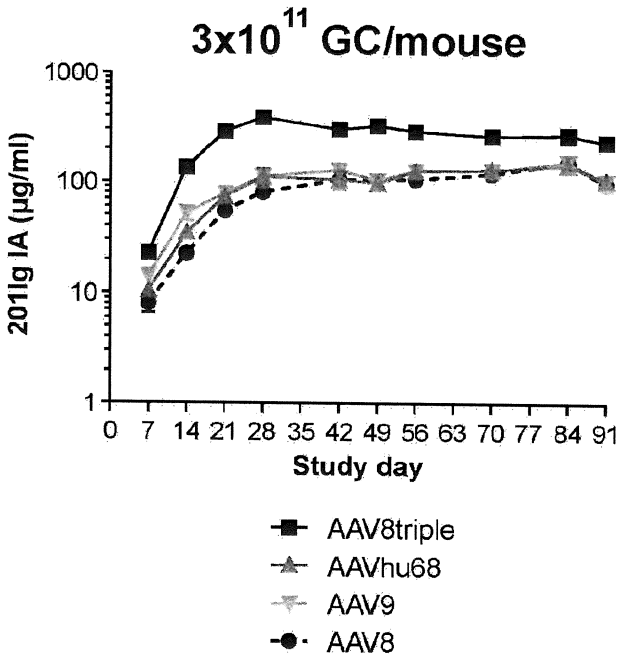


FIG. 10B

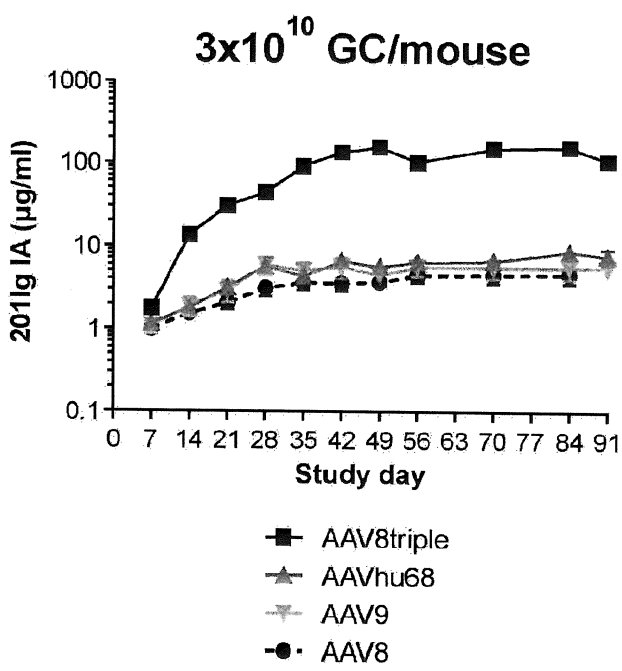


FIG. 11A

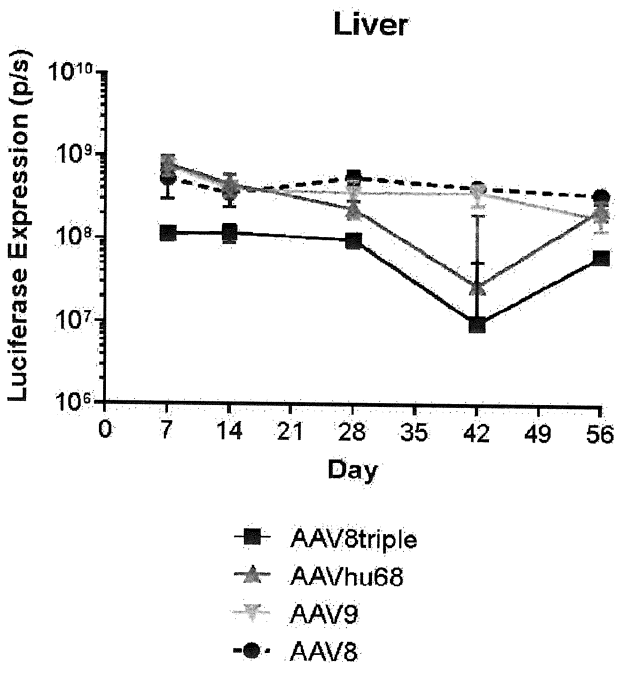


FIG. 11B

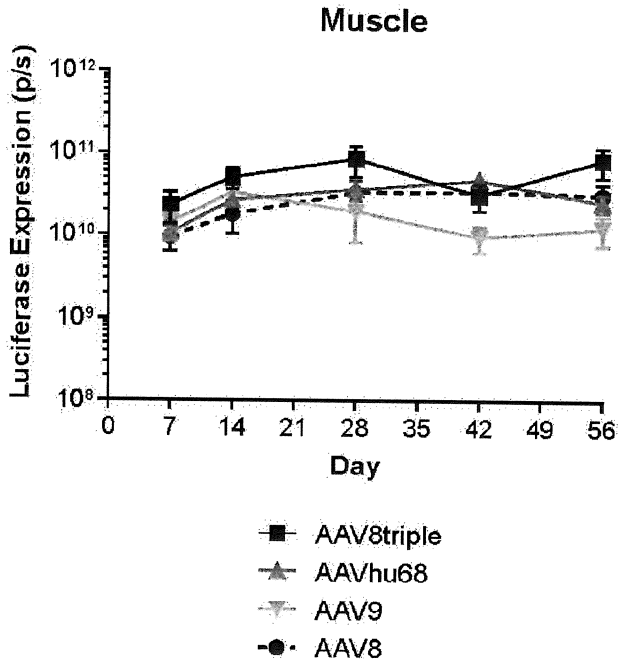
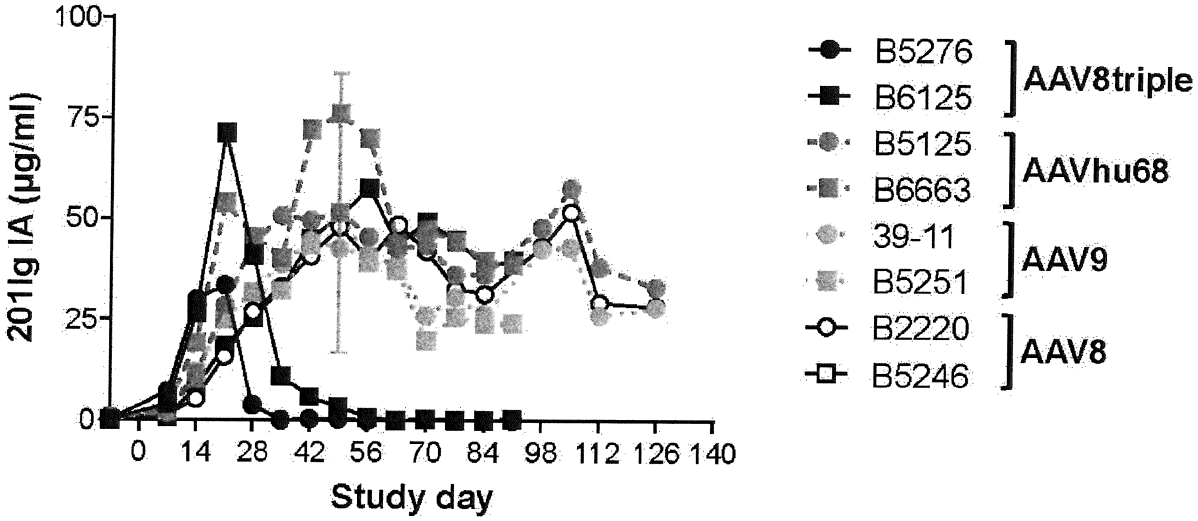


FIG 12


100
75
50
25
0
201lg IA (µg/ml)
0 14 28 42 56 70 84 98 112 126 140
Study day
B5276
B6125
AAV8triple
B5125
B6663
AAVhu68
39-11
B5251
AAV9
B2220
B5246
AAV8