FIGURE 1

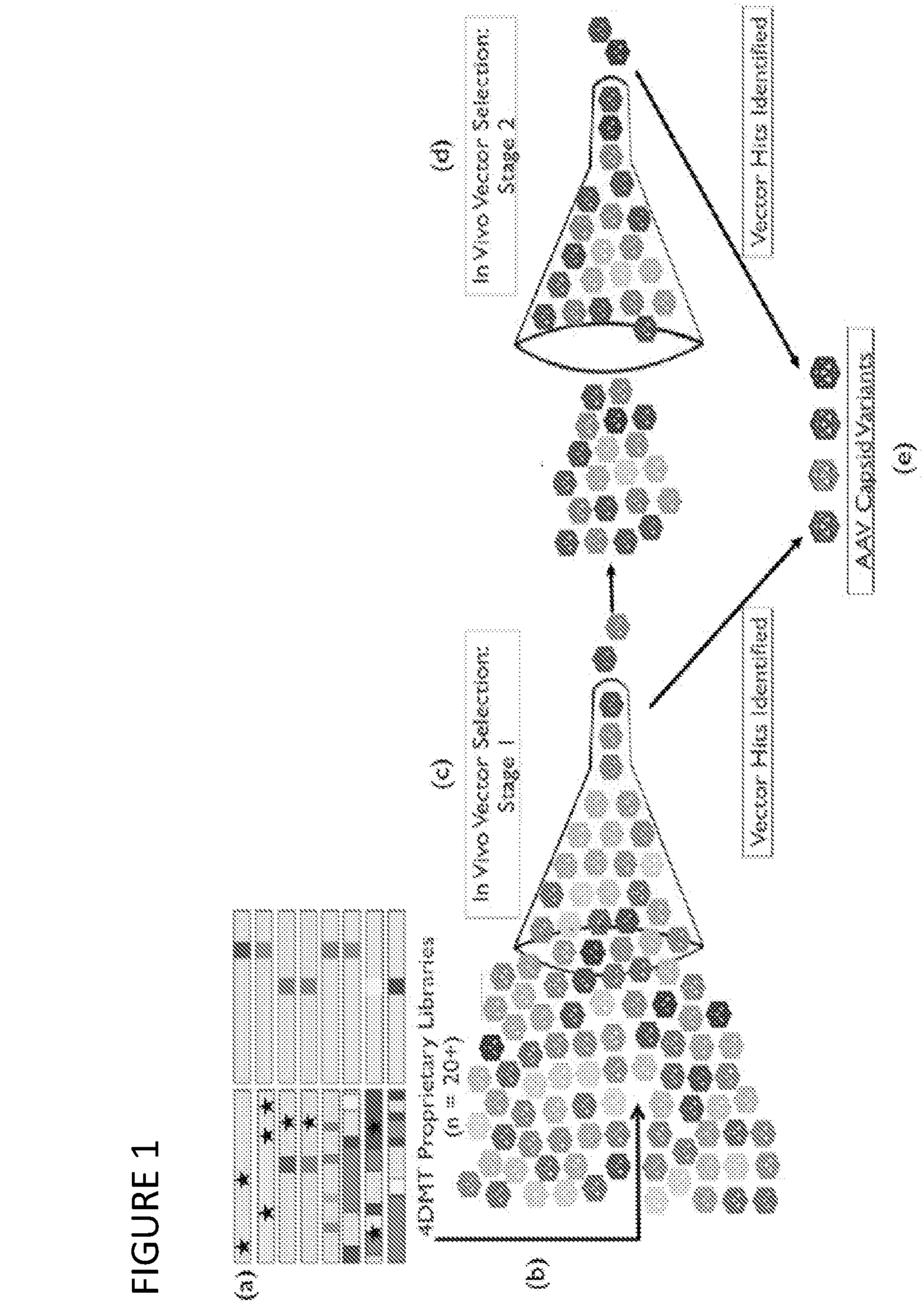

(a)
(b)
4DMT Proprietary Libraries
(n = 20+)
(c)
In Vivo Vector Selection:
Stage 1
Vector Hits Identified
(d)
In Vivo Vector Selection:
Stage 2
Vector Hits Identified
(e)
AAV Capsid Variants

FIGURE 2

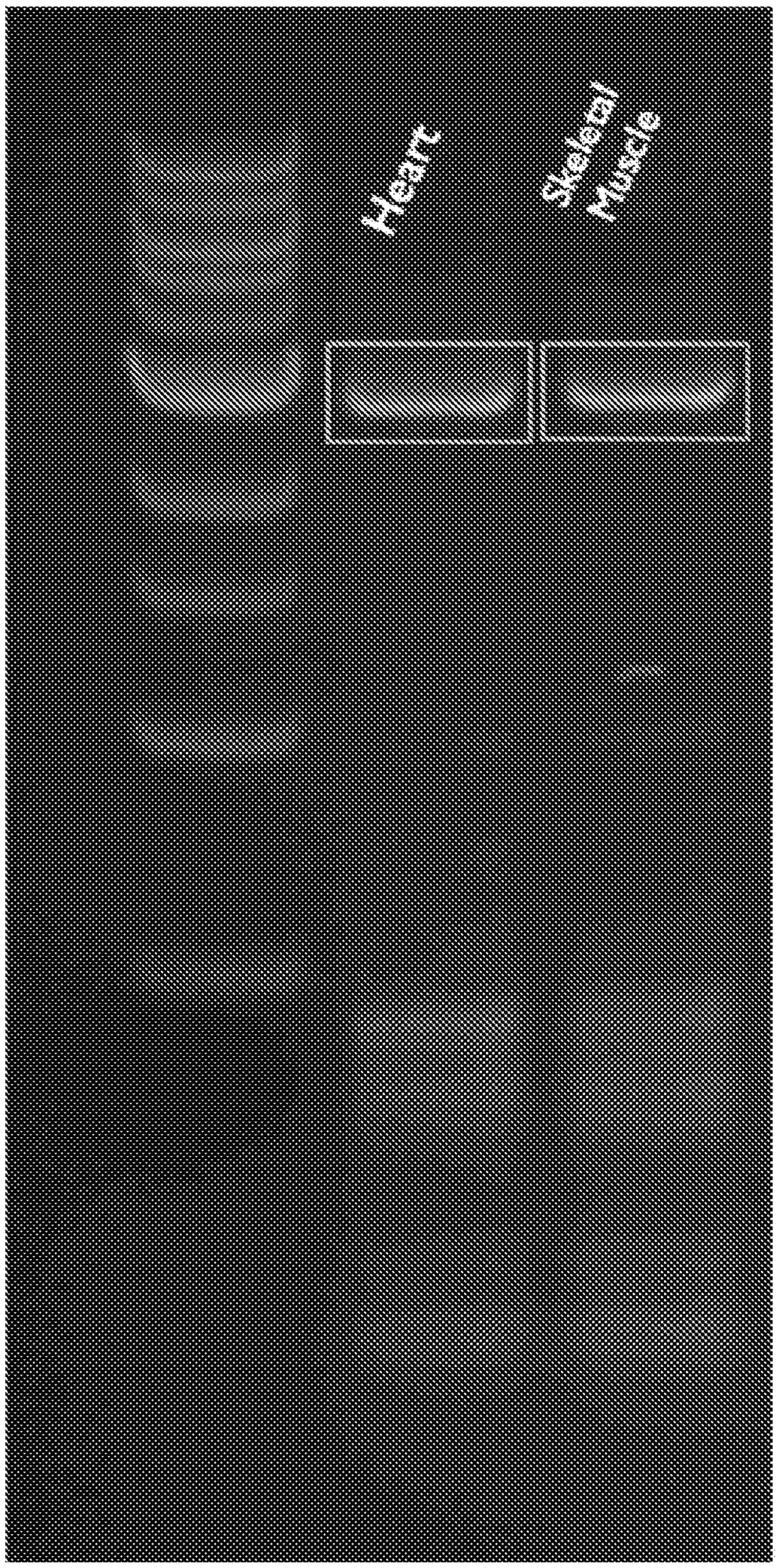


FIGURE 3A

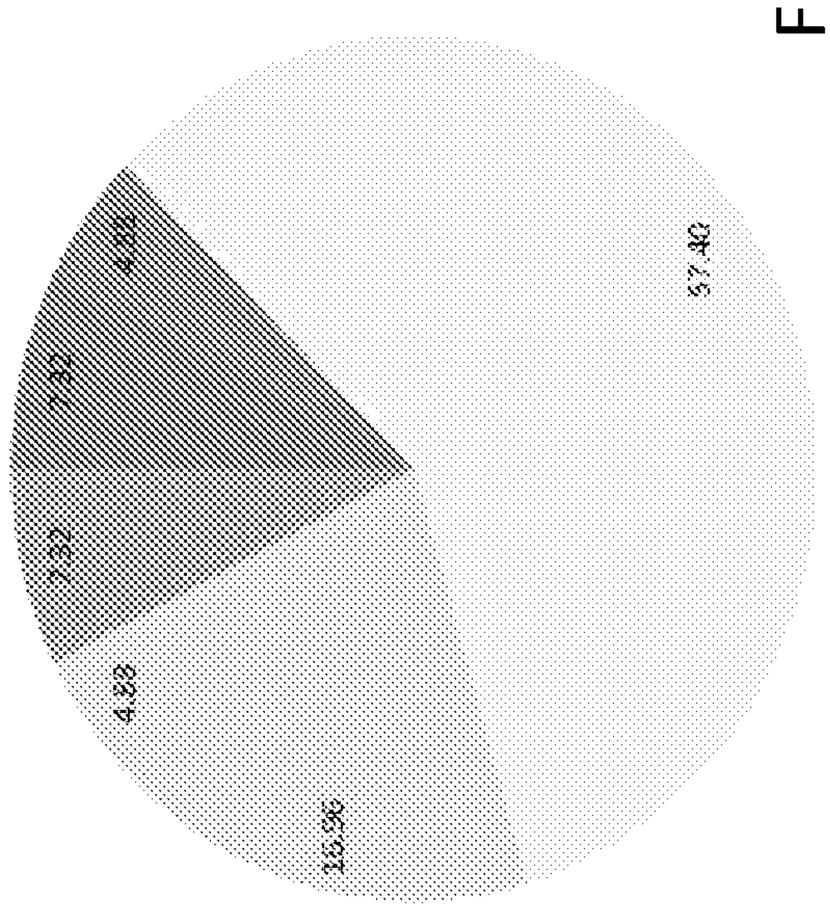


FIGURE 3B

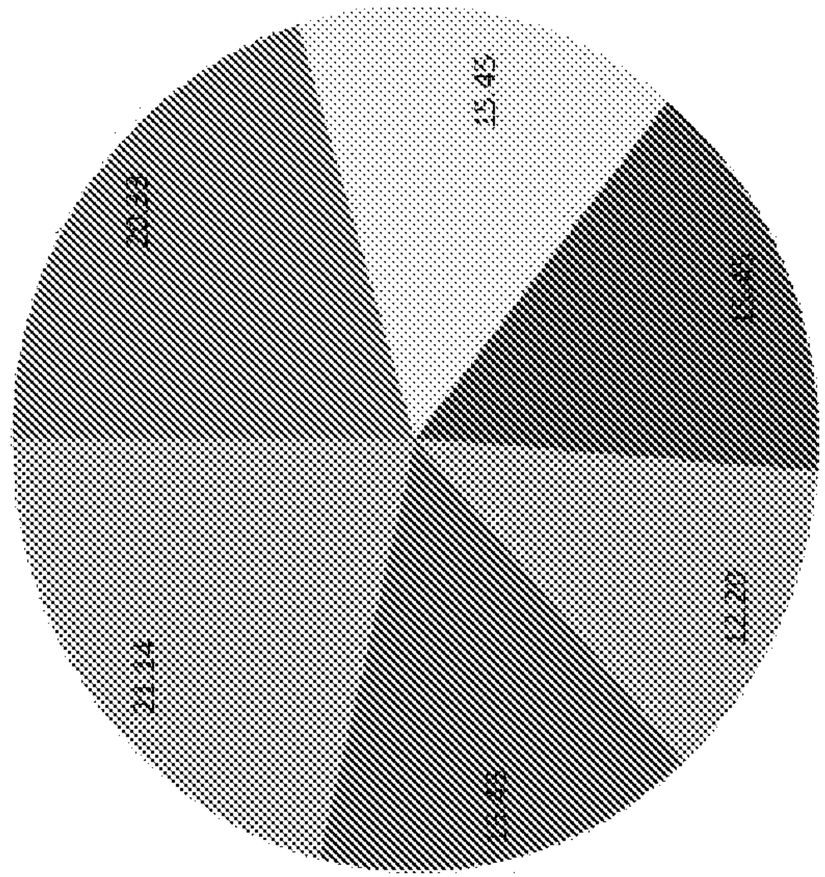
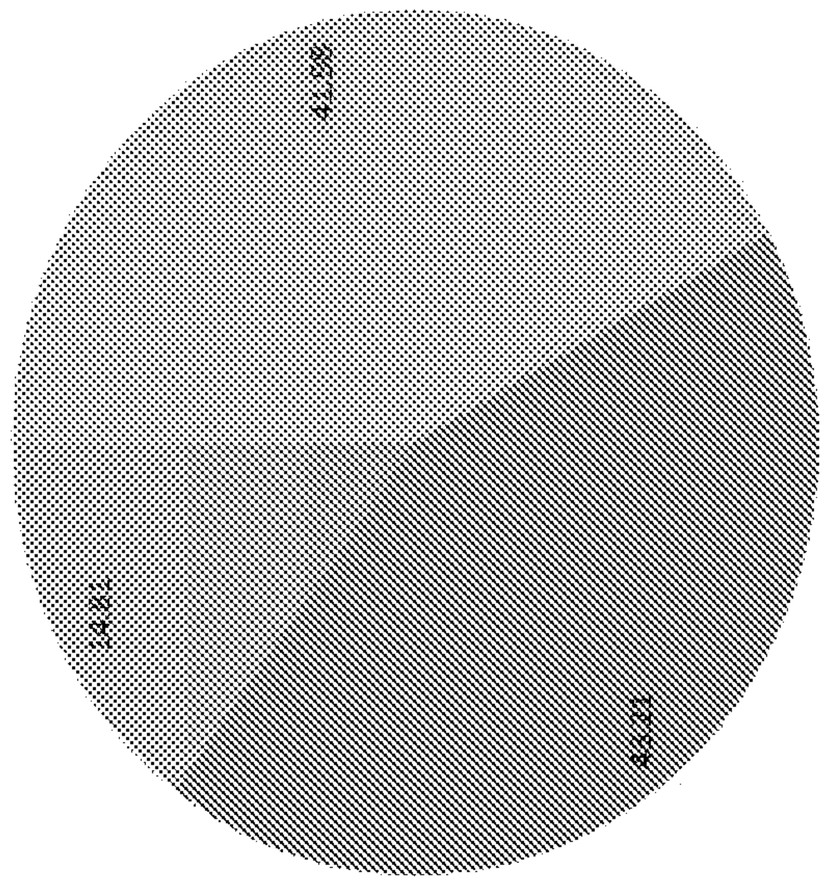


FIGURE 3C



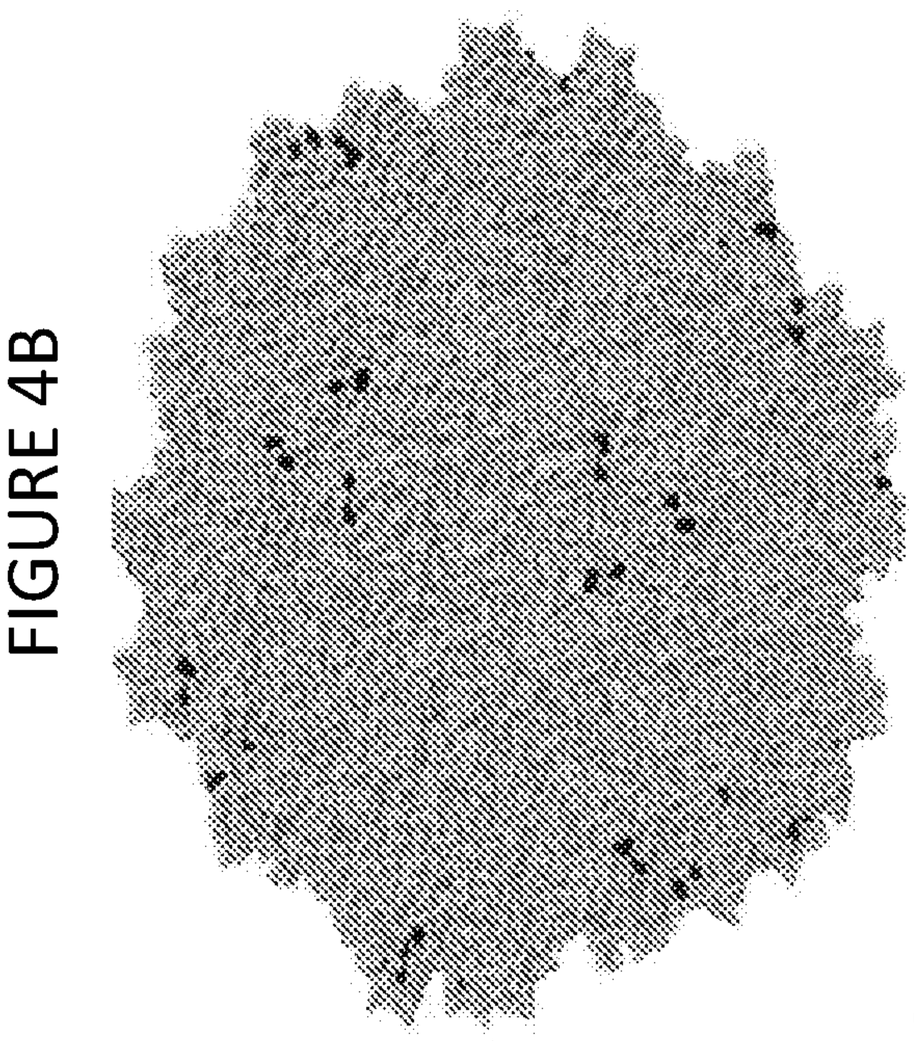


FIGURE 4B

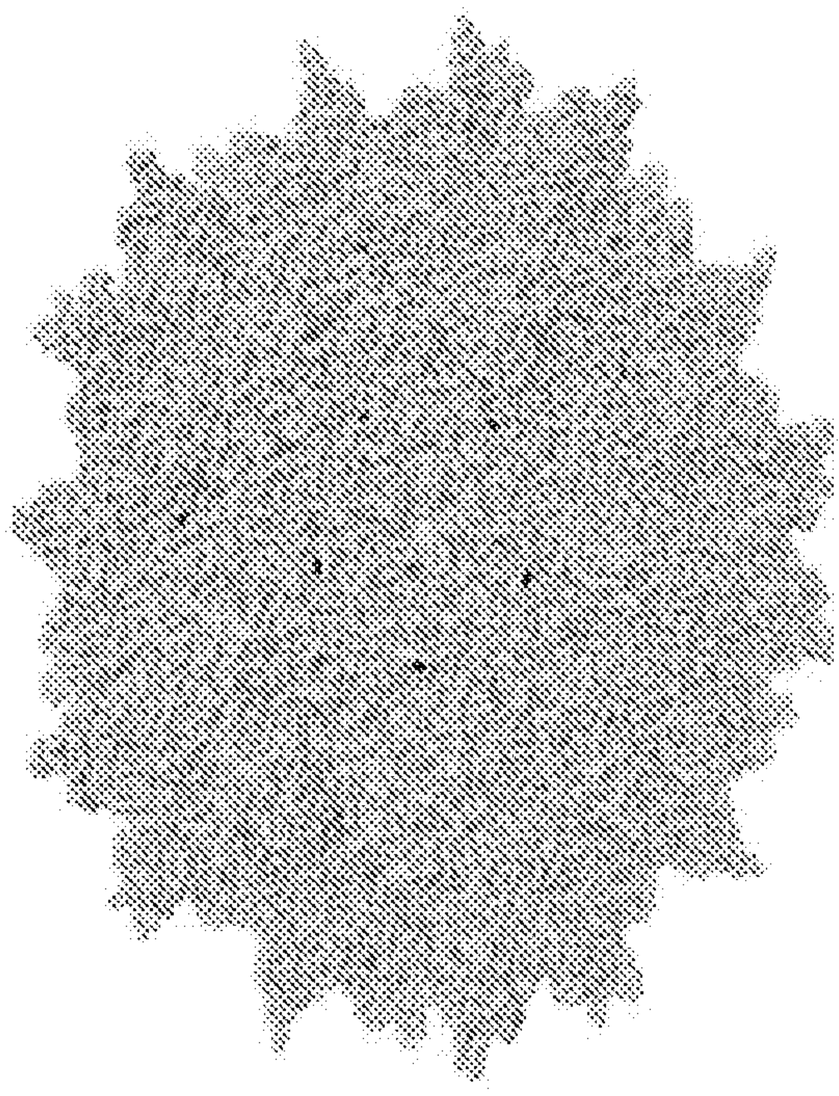


FIGURE 4C

FIGURE 4A

FIGURE 5

```
                1                  10                  20                  30                  40
AAV1    MAADGYLPDWLEDNLSEGIREWWDLKPGAPKPKANQQKQDDG
AAV2    MAADGYLPDWLEDTLSEGIRQWWKLKPGPPPPKPAERHKDDS
AAV3A   MAADGYLPDWLEDNLSEGIREWWALKPGVPQPKANQQHQDNR
AAV3B   MAADGYLPDWLEDNLSEGIREWWALKPGVPQPKANQQHQDNR
AAV4    M-TDGYLPDWLEDNLSEGVREWWALQPGAPKPKANQQHQDNA
AAV5    MSFVDHPPDWLEE-VGEGLREFLGLEAGPPKPKPNQQHQDQA
AAV6    MAADGYLPDWLEDNLSEGIREWWDLKPGAPKPKANQQKQDDG
AAV7    MAADGYLPDWLEDNLSEGIREWWDLKPGAPKPKANQQKQDNG
AAV8    MAADGYLPDWLEDNLSEGIREWWALKPGAPKPKANQQKQDDG
AAV9    MAADGYLPDWLEDNLSEGIREWWALKPGAPQPKANQQHQDNA
AAV10   MAADGYLPDWLEDNLSEGIREWWDLKPGAPKPKANQQKQDDG

                50                  60                  70                  80
AAV1    RGLVLPGYKYLGPFNGLDKGEPVNAADAAALEHDKAYDQQLK
AAV2    RGLVLPGYKYLGPFNGLDKGEPVNEADAAALEHDKAYDRQLD
AAV3A   RGLVLPGYKYLGPGNGLDKGEPVNEADAAALEHDKAYDQQLK
AAV3B   RGLVLPGYKYLGPGNGLDKGEPVNEADAAALEHDKAYDQQLK
AAV4    RGLVLPGYKYLGPGNGLDKGEPVNAADAAALEHDKAYDQQLK
AAV5    RGLVLPGYNYLGPGNGLDRGEPVNRADEVAREHDISYNEQLE
AAV6    RGLVLPGYKYLGPFNGLDKGEPVNAADAAALEHDKAYDQQLK
AAV7    RGLVLPGYKYLGPFNGLDKGEPVNAADAAALEHDKAYDQQLK
AAV8    RGLVLPGYKYLGPFNGLDKGEPVNAADAAALEHDKAYDQQLQ
AAV9    RGLVLPGYKYLGPGNGLDKGEPVNAADAAALEHDKAYDQQLK
AAV10   RGLVLPGYKYLGPFNGLDKGEPVNAADAAALEHDKAYDQQLK

                90                 100                 110                 120
AAV1    AGDNPYLRYNHADAEFQERLQEDTSFGGNLGRAVFQAKKRVL
AAV2    SGDNPYLKYNHADAEFQERLKEDTSFGGNLGRAVFQAKKRVL
AAV3A   AGDNPYLKYNHADAEFQERLQEDTSFGGNLGRAVFQAKKRIL
AAV3B   AGDNPYLKYNHADAEFQERLQEDTSFGGNLGRAVFQAKKRIL
AAV4    AGDNPYLKYNHADAEFQQRLQGDTSFGGNLGRAVFQAKKRVL
AAV5    AGDNPYLKYNHADAEFQEKLADDTSFGGNLGKAVFQAKKRVL
AAV6    AGDNPYLRYNHADAEFQERLQEDTSFGGNLGRAVFQAKKRVL
AAV7    AGDNPYLRYNHADAEFQERLQEDTSFGGNLGRAVFQAKKRVL
AAV8    AGDNPYLRYNHADAEFQERLQEDTSFGGNLGRAVFQAKKRVL
AAV9    AGDNPYLKYNHADAEFQERLKEDTSFGGNLGRAVFQAKKRLL
AAV10   AGDNPYLRYNHADAEFQERLQEDTSFGGNLGRAVFQAKKRVL

               130                 140                 150                 160
AAV1    EPLGLVEEGAKTAPGKKRPVEQSPQ-EPDSSSGIGKTGQQPA
AAV2    EPLGLVEEPVKTAPGKKRPVEHSPV-EPDSSSGTGKAGQQPA
AAV3A   EPLGLVEEAAKTAPGKKGAVDQSPQ-EPDSSSGVGKSGKQPA
AAV3B   EPLGLVEEAAKTAPGKKRPVDQSPQ-EPDSSSGVGKSGKQPA
AAV4    EPLGLVEQAGETAPGKKRPLIESPQ-QPDSSTGIGKKGKQPA
AAV5    EPFGLVEEGAKTAPTGKRIDDHFPKRKKART----EEDSKPS
AAV6    EPFGLVEEGAKTAPGKKRPVEQSPQ-EPDSSSGIGKTGQQPA
AAV7    EPLGLVEEGAKTAPAKKRPVEPSPQRSPDSSTGIGKKGQQPA
AAV8    EPLGLVEEGAKTAPGKKRPVEPSPQRSPDSSTGIGKKGQQPA
AAV9    EPLGLVEEAAKTAPGKKRPVEQSPQ-EPDSSAGIGKSGAQPA
AAV10   EPLGLVEEAAKTAPGKKRPVEPSPQRSPDSSTGIGKKGQQPA
```

FIGURE 5 (continued)

```
          170                 180                 190                 200                 210
AAV1    KKRLNFGQTGDSESVPDPQPLGEPPATP-AAVGPTTMASGGG
AAV2    RKRLNFGQTGDADSVPDPQPLGQPPAAP-SGLGTNTMATGSG
AAV3A   RKRLNFGQTGDSESVPDPQPLGEPPAAP-TSLGSNTMASGGG
AAV3B   RKRLNFGQTGDSESVPDPQPLGEPPAAP-TSLGSNTMASGGG
AAV4    KKKLVFE---DETGAGDGPPEGSTSGA--MSDDSEMRAAAGG
AAV5    T--------SSDAEAGPSGSQQLQIPAQPASSLGADTMSAGGG
AAV6    KKRLNFGQTGDSESVPDPQPLGEPPATP-AAVGPTTMASGGG
AAV7    RKRLNFGQTGDSESVPDPQPLGEPPAAP-SSVGSGTVAAGGG
AAV8    RKRLNFGQTGDSESVPDPQPLGEPPAAP-SGVGPNTMAAGGG
AAV9    KKRLNFGQTGDTESVPDPQPIGEPPAAP-SGVGSLTMASGGG
AAV10   KKRLNFGQTGESESVPDPQPIGEPPAGP-SGLGSGTMAAGGG
          220                 230                 240                 250
AAV1    APMADNNEGADGVGNASGNWHCDSTWLGDRVITTSTRTWALP
AAV2    APMADNNEGADGVGNSSGNWHCDSTWMGDRVITTSTRTWALP
AAV3A   APMADNNEGADGVGNSSGNWHCDSQWLGDRVITTSTRTWALP
AAV3B   APMADNNEGADGVGNSSGNWHCDSQWLGDRVITTSTRTWALP
AAV4    AAV-EGGQGADGVGNASGDWHCDSTWSEGHVTTTSTRTWVLP
AAV5    GPLGDNNQGADGVGNASGDWHCDSTWMGDRVVTKSTRTWVLP
AAV6    APMADNNEGADGVGNASGNWHCDSTWLGDRVITTSTRTWALP
AAV7    APMADNNEGADGVGNASGNWHCDSTWLGDRVITTSTRTWALP
AAV8    APMADNNEGADGVGSSSGNWHCDSTWLGDRVITTSTRTWALP
AAV9    APVADNNEGADGVGSSSGNWHCDSQWLGDRVITTSTRTWALP
AAV10   APMADNNEGADGVGSSSGNWHCDSTWLGDRVITTSTRTWALP
          280                 270                 280                 290
AAV1    TYNNHLYKQISSA-STGASNDNHYFGYSTPWGYFDFNRFHCH
AAV2    TYNNHLYKQISSQ--SGASNDNHYFGYSTPWGYFDFNRFHCH
AAV3A   TYNNHLYKQISSQ--SGASNDNHYFGYSTPWGYFDFNRFHCH
AAV3B   TYNNHLYKQISSQ--SGASNDNHYFGYSTPWGYFDFNRFHCH
AAV4    TYNNHLYKRLGE-----SLQSNTYNGFSTPWGYFDFNRFHCH
AAV5    SYNNHQYREIKSGSVDG-SNANAYFGYSTPWGYFDFNRFHSH
AAV6    TYNNHLYKQISSA-STGASNDNHYFGYSTPWGYFDFNRFHCH
AAV7    TYNNHLYKQISSE-TAGSTNDNTYFGYSTPWGYFDFNRFHCH
AAV8    TYNNHLYKQISNGTSGGATNDNTYFGYSTPWGYFDFNRFHCH
AAV9    TYNNHLYKQISNSTSGGSSNDNAYFGYSTPWGYFDFNRFHCH
AAV10   TYNNHLYKQISNGTSGGSTNDNTYFGYSTPWGYFDFNRFHCH
          300                 310                 320                 330
AAV1    FSPRDWQRLINNNWGFRPKRLNFKLFNIQVKEVTTNDGVTTI
AAV2    FSPRDWQRLINNNWGFRPKRLNFKLFNIQVKEVTQNDGTTTI
AAV3A   FSPRDWQRLINNNWGFRPKKLSFKLFNIQVRGVTQNDGTTTI
AAV3B   FSPRDWQRLINNNWGFRPKKLSFKLFNIQVKEVTQNDGTTTI
AAV4    FSPRDWQRLINNNWGMRPKAMRVKIFNIQVKEVTTSNGETTV
AAV5    WSPRDWQRLINNYWGFRPRSLRVKIFNIQVKEVTVQDSTTTI
AAV6    FSPRDWQRLINNNWGFRPKRLNFKLFNIQVKEVTTNDGVTTI
AAV7    FSPRDWQRLINNNWGFRPKKLRFKLFNIQVKEVTTNDGVTTI
AAV8    FSPRDWQRLINNNWGFRPKRLSFKLFNIQVKEVTQNEGTKTI
AAV9    FSPRDWQRLINNNWGFRPKRLNFKLFNIQVKEVTDNNGVKTI
AAV10   FSPRDWQRLINNNWGFRPKRLSFKLFNIQVKEVTQNEGTKTI
```

FIGURE 5 (continued)

```
                340       350       360       370
AAV1    ANNLTSTVQVFSDSEYQLPYVLGSAHQGCLPPFPADVFMIPQ
AAV2    ANNLTSTVQVFTDSEYQLPYVLGSAHQGCLPPFPADVFMVPQ
AAV3A   ANNLTSTVQVFTDSEYQLPYVLGSAHQGCLPPFPADVFMVPQ
AAV3B   ANNLTSTVQVFTDSEYQLPYVLGSAHQGCLPPFPADVFMVPQ
AAV4    ANNLTSTVQIFADSSYELPYVMDAGQEGSLPPFPNDVFMVPQ
AAV5    ANNLTSTVQVFTDDDYQLPYVVGNGTEGCLPAFPPQVFTLPQ
AAV6    ANNLTSTVQVFSDSEYQLPYVLGSAHQGCLPPFPADVFMIPQ
AAV7    ANNLTSTIQVFSDSEYQLPYVLGSAHQGCLPPFPADVFMIPQ
AAV8    ANNLTSTIQVFTDSEYQLPYVLGSAHQGCLPPFPADVFMIPQ
AAV9    ANNLTSTVQVFTDSDYQLPYVLGSAHEGCLPPFPADVFMIPQ
AAV10   ANNLTSTIQVFTDSEYQLPYVLGSAHQGCLPPFPADVFMIPQ

                380       390       400       410       420
AAV1    YGYLTLN--NGS-QAVGRSSFYCLEYFPSQMLRTGNNFTFSY
AAV2    YGYLTLN--NGS-QAVGRSSFYCLEYFPSQMLRTGNNFTFSY
AAV3A   YGYLTLN--NGS-QAVGRSSFYCLEYFPSQMLRTGNNFQFSY
AAV3B   YGYLTLN--NGS-QAVGRSSFYCLEYFPSQMLRTGNNFQFSY
AAV4    YGYCGLVTGNTSQQQTDRNAFYCLEYFPSQMLRTGNNFEITY
AAV5    YGYATLNRDNTE-NPTERSSFFCLEYFPSKMLRTGNNFEFTY
AAV6    YGYLTLN--NGS-QAVGRSSFYCLEYFPSQMLRTGNNFTFSY
AAV7    YGYLTLN--NGS-QSVGRSSFYCLEYFPSQMLRTGNNFEFSY
AAV8    YGYLTLN--NGS-QAVGRSSFYCLEYFPSQMLRTGNNFQFTY
AAV9    YGYLTLN--DGS-QAVGRSSFYCLEYFPSQMLRTGNNFQFSY
AAV10   YGYLTLN--NGS-QAVGRSSFYCLEYFPSQMLRTGNNFEFSY

                430       440       450       460
AAV1    TFEEVPFHSSYAHSQSLDRLMNPLIDQYLYYLNRTQNQ-SGS
AAV2    TFEDVPFHSSYAHSQSLDRLMNPLIDQYLYYLSRT-NTPSGT
AAV3A   TFEDVPFHSSYAHSQSLDRLMNPLIDQYLYYLNRTQGTTSGT
AAV3B   TFEDVPFHSSYAHSQSLDRLMNPLIDQYLYYLNRTQGTTSGT
AAV4    SFEKVPFHSMYAHSQSLDRLMNPLIDQYLWGLQSTTTGTTLN
AAV5    NFEEVPFHSSFAPSQNLFKLANPLVDQYLYRFVSTNNTGG--
AAV6    TFEDVPFHSSYAHSQSLDRLMNPLIDQYLYYLNRTQNQ-SGS
AAV7    SFEDVPFHSSYAHSQSLDRLMNPLIDQYLYYLARTQSNPGGT
AAV8    TFEDVPFHSSYAHSQSLDRLMNPLIDQYLYYLSRTQTT-GGT
AAV9    EFENVPFHSSYAHSQSLDRLMNPLIDQYLYYLSKTIN--GSG
AAV10   TFEDVPFHSSYAHSQSLDRLMNPLIDQYLYYLSRTQST-GGT

                470       480       490       500
AAV1    AQNKDLLFSRGSPAGMSVQPKNWLPGPCYRQQRVSKTKTDN-
AAV2    TTQSRLQFSQAGASDIRDQSRNWLPGPCYRQQRVSKTSADN-
AAV3A   TNQSRLLFSQAGPQSMSLQARNWLPGPCYRQQRLSKTANDN-
AAV3B   TNQSRLLFSQAGPQSMSLQARNWLPGPCYRQQRLSKTANDN-
AAV4    AGTATTNFTKLRPTNFSNFKKNWLPGPSIKQQGFSKTANQNY
AAV5    -----VQFNKNLAGRYANTYKNWFPGPMGRTQGWNLGSGVNR
AAV6    AQNKDLLFSRGSPAGMSVQPKNWLPGPCYRQQRVSKTKTDN-
AAV7    AGNRELQFYQGGPSTMAEQAKNWLPGPCFRQQRVSKTLDQN-
AAV8    ANTQTLGFSQGGPNTMANQAKNWLPGPCYRQQRVSTTTGQN-
AAV9    QNQQTLKFSVAGPSNMAVQGRNYIPGPSYRQQRVSTTVTQN-
AAV10   QGTQQLLFSQAGPANMSAQAKNWLPGPCYRQQRVSTTLSQN-
```

FIGURE 5 (continued)

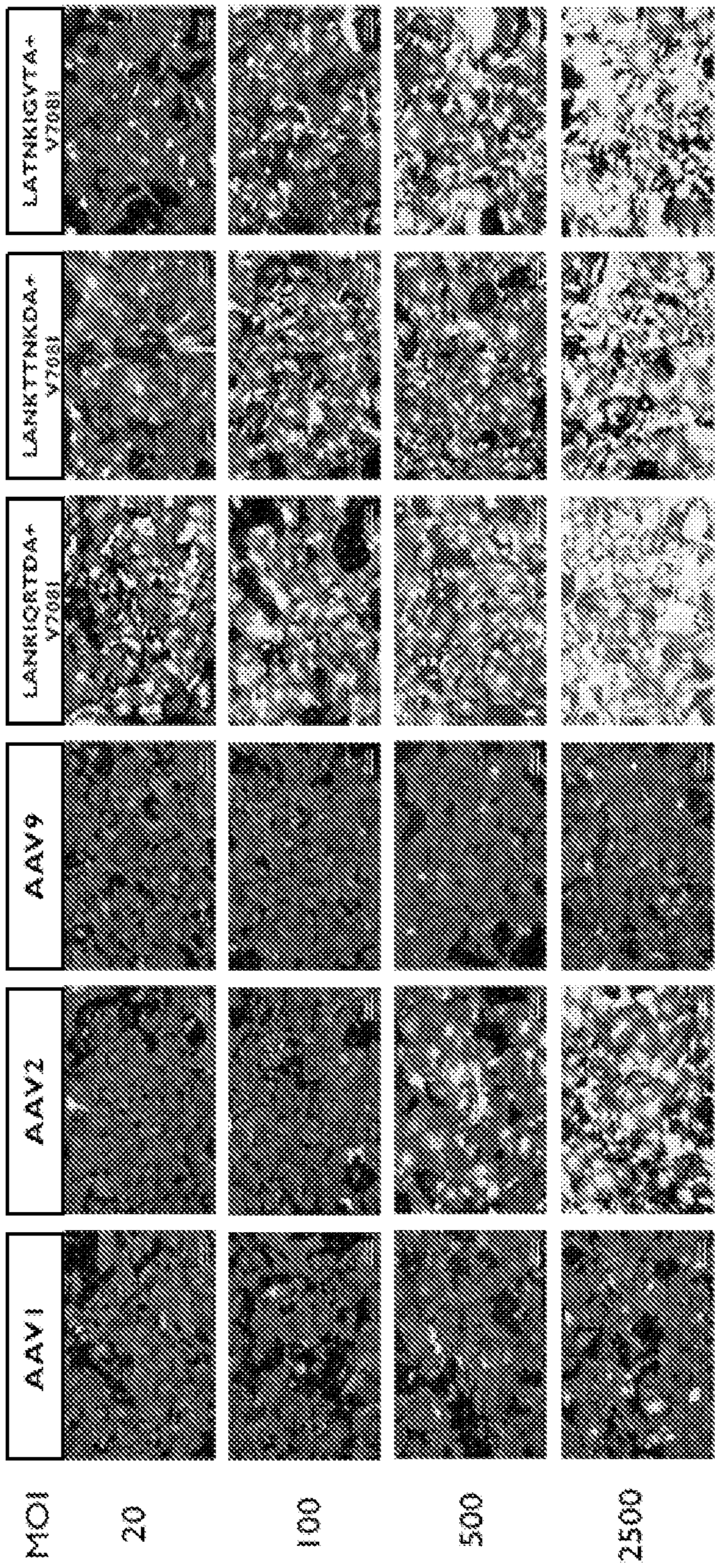
```
                    510        520        530        540
AAV1    ----NNSNFTWTGASKYNLNGRESIINPGTAMASHKDDEDKF
AAV2    ----NNSEYSWTGATKYHLNGRDSLVNPGPAMASHKDDEEKF
AAV3A   ----NNSNFPWTAASKYHLNGRDSLVNPGPAMASHKDDEEKF
AAV3B   ----NNSNFPWTAASKYHLNGRDSLVNPGPAMASHKDDEEKF
AAV4    KIPATGSDSLIKYETHSTLDGRWSALTPGPPMATAGPADSKF
AAV5    ------ASVSAFATTNRMELEGASYQVPPQPNGMTNNLQGSNT
AAV6    ----NNSNFTWTGASKYNLNGRESIINPGTAMASHKDDKDKF
AAV7    ----NNSNFAWTGATKYHLNGRNSLVNPGVAMATHKDDEDRF
AAV8    ----NNSNFAWTAGTKYHLNGRNSLANPGIAMATHKDDEERF
AAV9    ----NNSEFAWPGASSWALNGRNSLMNPGPAMASHKEGEDRF
AAV10   ----NNSNFAWTGATKYHLNGRDSLVNPGVAMATHKDDEERF
                    550        560        570        580
AAV1    FPMSGVMIF-GKESAGASNTAL--DNVMITDEEEIKATNPVA
AAV2    FPQSGVLIF-GKQGSEKTNVDI--EKVMITDEEEIRTTNPVA
AAV3A   FPMHGNLIF-GKEGTTASNAEL--DNVMITDEEEIRTTNPVA
AAV3B   FPMHGNLIF-GKEGTTASNAEL--DNVMITDEEEIRTTNPVA
AAV4    --SNSQLIFAGPKQNGNTATVP--GTLIFTSEEELAATNATD
AAV5    YALENTMIFNSQPANPGTTATYLEGNMLITSESETQPVNRVA
AAV6    FPMSGVMIF-GKESAGASNTAL--DNVMITDEEEIKATNPVA
AAV7    FPSSGVLIF-GKTGATNKTTL---ENVLMTNEEEIRPTNPVA
AAV8    FPSNGILIF-GKQNAARDNADY--SDVMLTSEEEIKTTNPVA
AAV9    FPLSGSLIF-GKQGTGRDNVDA--DKVMITNEEEIKTTNPVA
AAV10   FPSSGVLMF-GKQGAGRDNVDY--SSVMLTSEEEIKTTNPVA
                    590        600        610        620        630
AAV1    TERFGTVAVNFQSSSTDPATGDVHAMGALPGMVWQDRDVYLQ
AAV2    TEQYGSVSTNLQRGNRQAATADVNTQGVLPGMVWQDRDVYLQ
AAV3A   TEQYGTVANNLQSSNTAPTTGTVNHQGALPGMVWQDRDVYLQ
AAV3B   TEQYGTVANNLQSSNTAPTTRTVNDQGALPGMVWQDRDVYLQ
AAV4    TDMWGNLPGGDQSNSNLPTVDRLTALGAVPGMVWQNRDIYYQ
AAV5    YNVGGQMATNNQSSTTAPATGTYNLQEIVPGSVWMERDVYLQ
AAV6    TERFGTVAVNLQSSSTDPATGDVHVMGALPGMVWQDRDVYLQ
AAV7    TEEYGIVSSNLQAANTAAQTQVVNNQGALPGMVWQNRDVYLQ
AAV8    TEEYGIVADNLQQQNTAPQIGTVNSQGALPGMVWQNRDVYLQ
AAV9    TESYGQVATNHQSAQAQAQTGWVQNQGILPGMVWQDRDVYLQ
AAV10   TEQYGVVADNLQQANTGPIVGNVNSQGALPGMVWQNRDVYLQ
                    640        650        660        670
AAV1    GPIWAKIPHTDGHFHPSPLMGGFGLKNPPPQILIKNTPVPAN
AAV2    GPIWAKIPHTDGHFHPSPLMGGFGLKHPPPQILIKNTPVPAN
AAV3A   GPIWAKIPHTDGHFHPSPLMGGFGLKHPPPQIMIKNTPVPAN
AAV3B   GPIWAKIPHTDGHFHPSPLMGGFGLKHPPPQIMIKNTPVPAN
AAV4    GPIWAKIPHTDGHFHPSPLIGGFGLKHPPPQIFIKNTPVPAN
AAV5    GPIWAKIPETGAHFHPSPAMGGFGLKHPPPMMLIKNTPVPGN
AAV6    GPIWAKIPHTDGHFHPSPLMGGFGLKHPPPQILIKNTPVPAN
AAV7    GPIWAKIPHTDGNFHPSPLMGGFGLKHPPPQILIKNTPVPAN
AAV8    GPIWAKIPHTDGNFHPSPLMGGFGLKHPPPQILIKNTPVPAD
AAV9    GPIWAKIPHTDGNFHPSPLMGGFGMKHPPPQILIKNTPVPAD
AAV10   GPIWAKIPHTDGNFHPSPLMGGFGLKHPPPQILIKNTPVPAD
```

FIGURE 5 (concluded)

```
                                  680        690        700        710
AAV1    PPAEPSATKFASFITQYSTGQVSVEIEWELQKENSKRWNPEV
AAV2    PSTTFSAAKFASFITQYSTGQVSVEIEWELQKENSKRWNPEI
AAV3A   PPTTFSPAKFASFITQYSTGQVSVEIEWELQKENSKRWNPEI
AAV3B   PPTTFSPAKFASFITQYSTGQVSVEIEWELQKENSKRWNPEI
AAV4    PATTFSSTPVNSFITQYSTGQVSVQIDWEIQKERSKRWNPEV
AAV5    -ITSFSDVPVSSFITQYSTGQVTVEMEWELKKENSKRWNPEI
AAV6    PPAEFSATKFASFITQYSTGQVSVEIEWELQKENSKRWNPEV
AAV7    PPEVFTPAKFASFITQYSTGQVSVEIEWELQKENSKRWNPEI
AAV8    PPTTFNQSKLNSFITQYSTGQVSVEIEWELQKENSKRWNPEI
AAV9    PPTAFNKDKLNSFITQYSTGQVSVEIEWELQKENSKRWNPEI
AAV10   PPTTFSQAKLASFITQYSTGQVSVEIEWELQKENSKRWNPEI

                  720        730        740        750 752
AAV1    QYTSNYAKSANVDFTVDNNGLYTEPRPIGTRYLTRPL*
AAV2    QYTSNYNKSVNVDFTVDTNGVYSEPRPIGTRYLTRNL*
AAV3A   QYTSNYNKSVNVDFTVDTNGVYSEPRPIGTRYLTRNL*
AAV3B   QYTSNYNKSVNVDFTVDTNGVYSEPRPIGTRYLTRNL*
AAV4    QFTSNYGQQNSLLWAPDAAGKYTEPRAIGTRYLTHHL*
AAV5    QYTNNYNDPQFVDFAPDSTGEYRTTRPIGTRYLTRPL*
AAV6    QYTSNYAKSANVDFTVDNNGLYTEPRPIGTRYLTRPL*
AAV7    QYTSNFEKQTGVDFAVDSQGVYSEPRPIGTRYLTRNL*
AAV8    QYTSNYYKSTSVDFAVNTEGVYSEPRPIGTRYLTRNL*
AAV9    QYTSNYYKSNNVEFAVNTEGVYSEPRPIGTRYLTRNL*
AAV10   QYTSNYYKSTNVDFAVNTEGTYSEPRPIGTRYLTRNL*
```

FIGURE 6A


eTnTIeGFP/DAPI
AAV1
AAV2
AAV9
LANKIQRTDA+ V7081
LANKTTNKDA+ V7081
LATNKIGVTA+ V7081
MOI
20
100
500
2500
Pi Time: 6 days

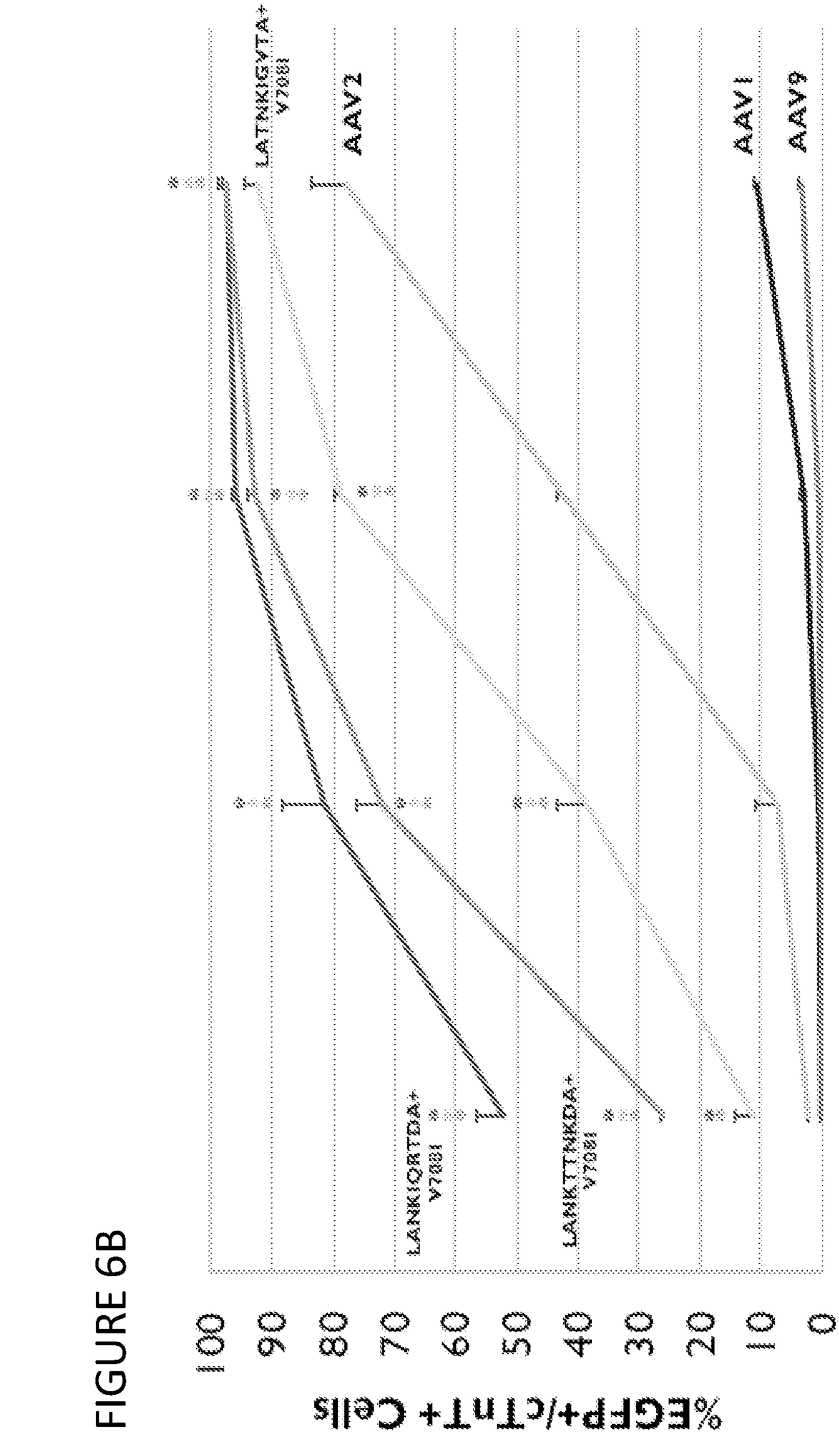

AAV2
AAV1
AAV9
LANKTTNKDA+ V708I
LANKIQRTDA+ V708I
MOI
20
100
500
2500
%EGFP+/cTnT+ Cells
100
90
80
70
60
50
40
30
20
10
0
n=3
Mean ± SD
Two-tailed T-tests
* p< 0.05

FIGURE 6B

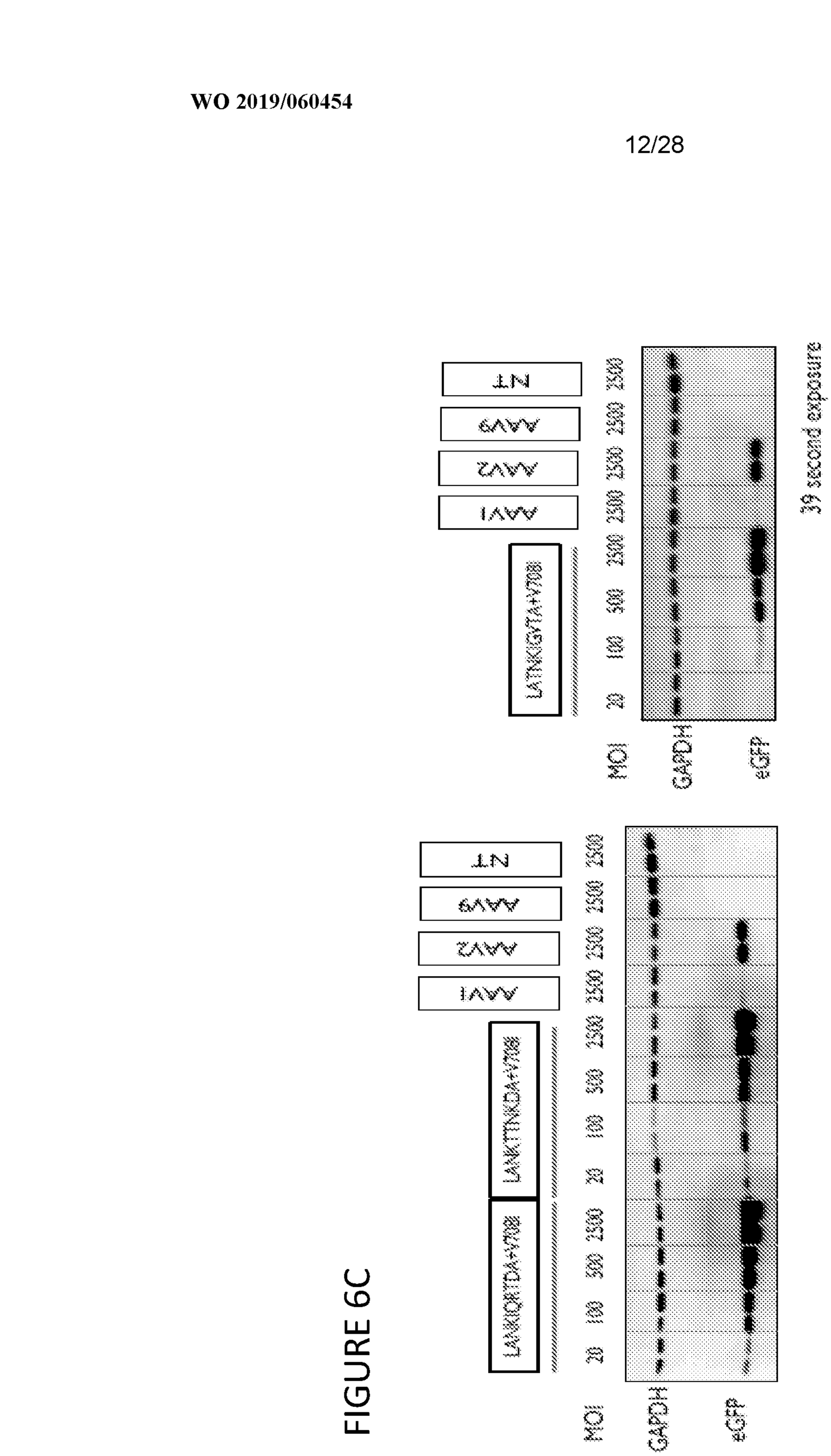

MOI
20
100
500
2500
2500
2500
2500
2500
GAPDH
eGFP
LATNKIGVTA+V708
AAV1
AAV2
AAV6
AAV9
NT
LANKTTNKDA+V708
LANKIQRTDA+V708
39 second exposure

FIGURE 6C

FIGURE 6D

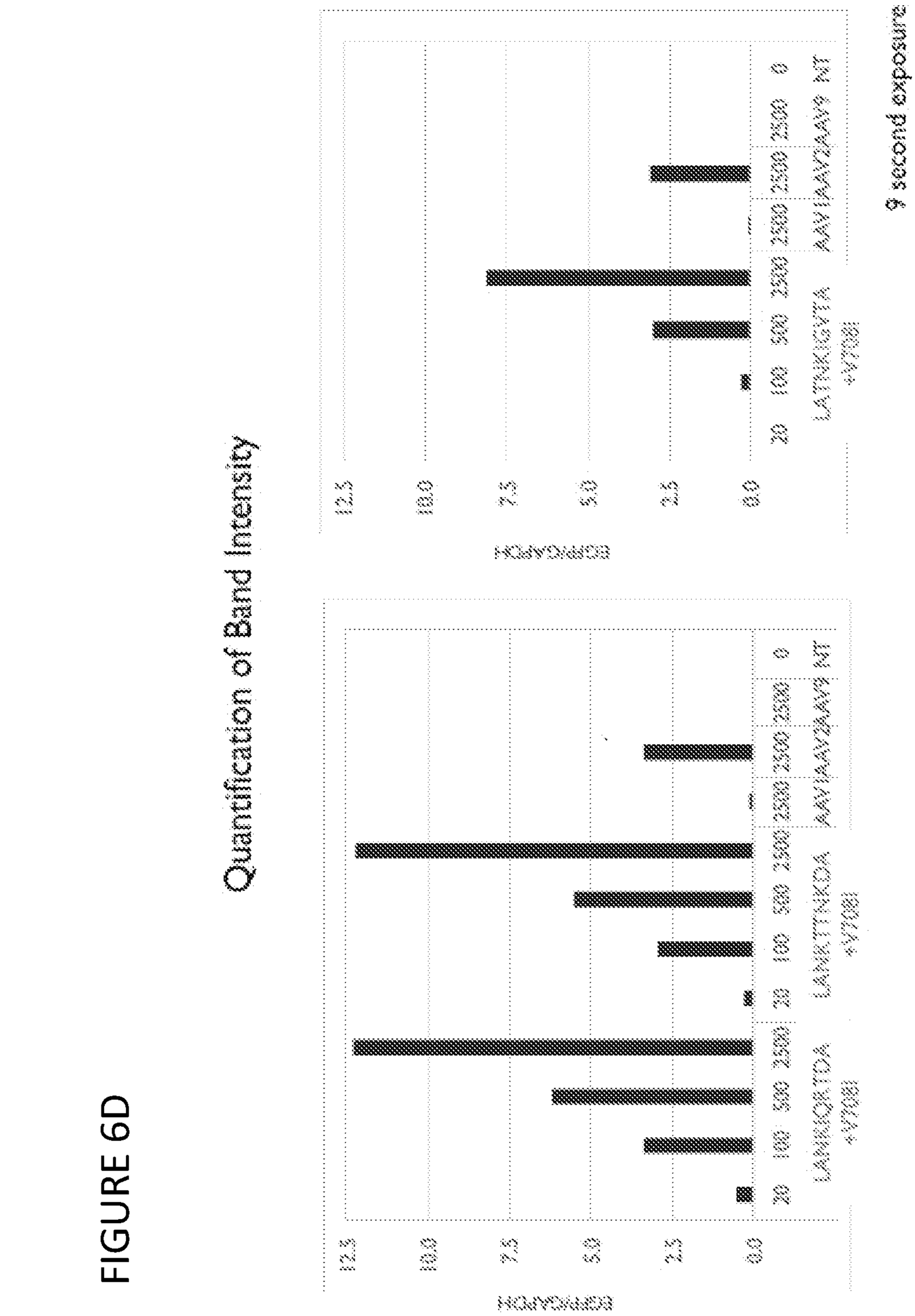

Quantification of Band Intensity
INTENSITY
20
100
500
2500
LAMBDA-TUBULIN
+/-TMBIM
20
100
500
2500
ANNEXIN-A4
+/-TMBIM
20
100
500
2500
LAMBDA-TUBULIN
+/-TMBIM
20
100
500
2500
ANNEXIN-A4
+/-TMBIM
8.0
2.5
5.0
7.3
18.0
12.3
EXPOSURE
9 second exposure

FIGURE 6E

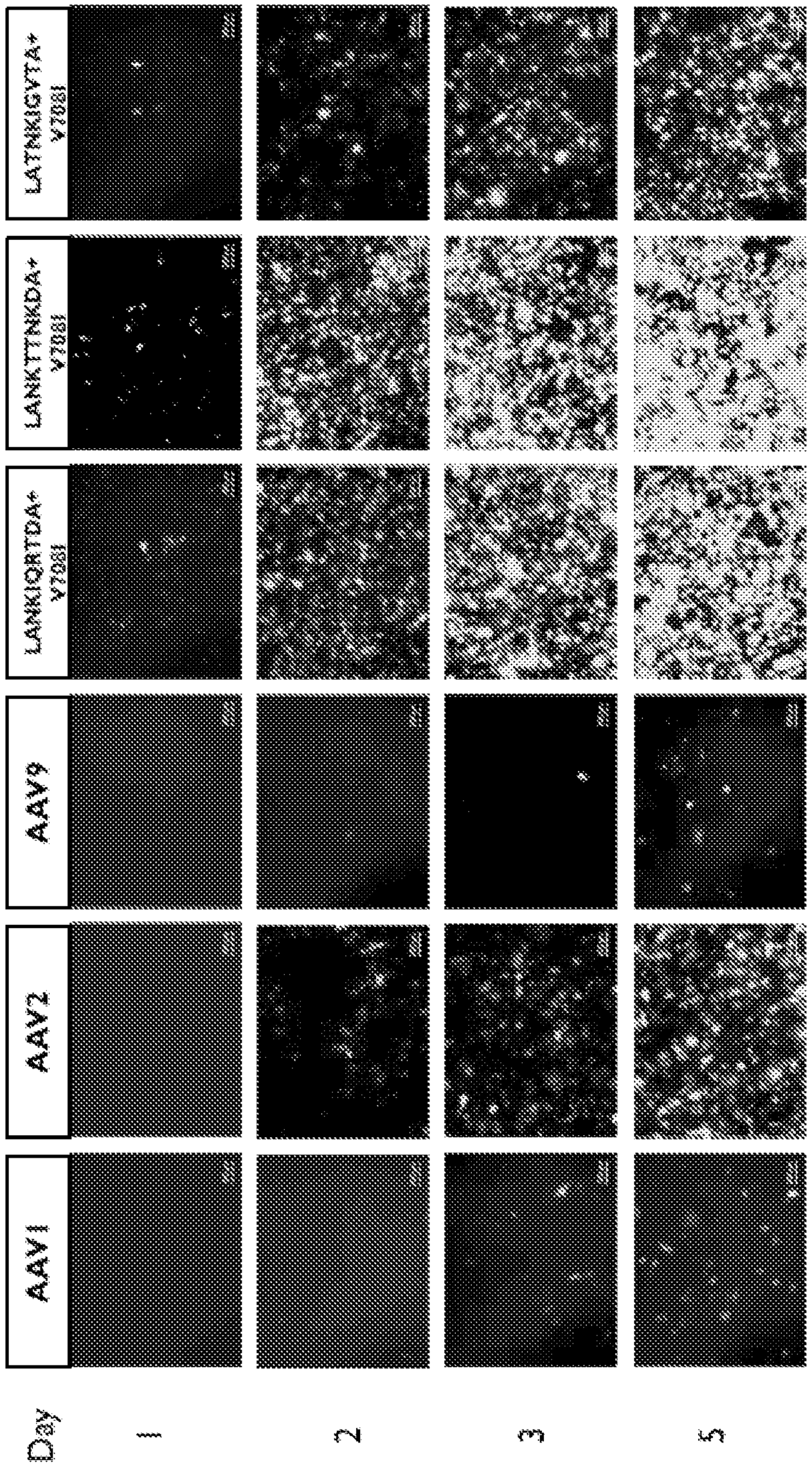
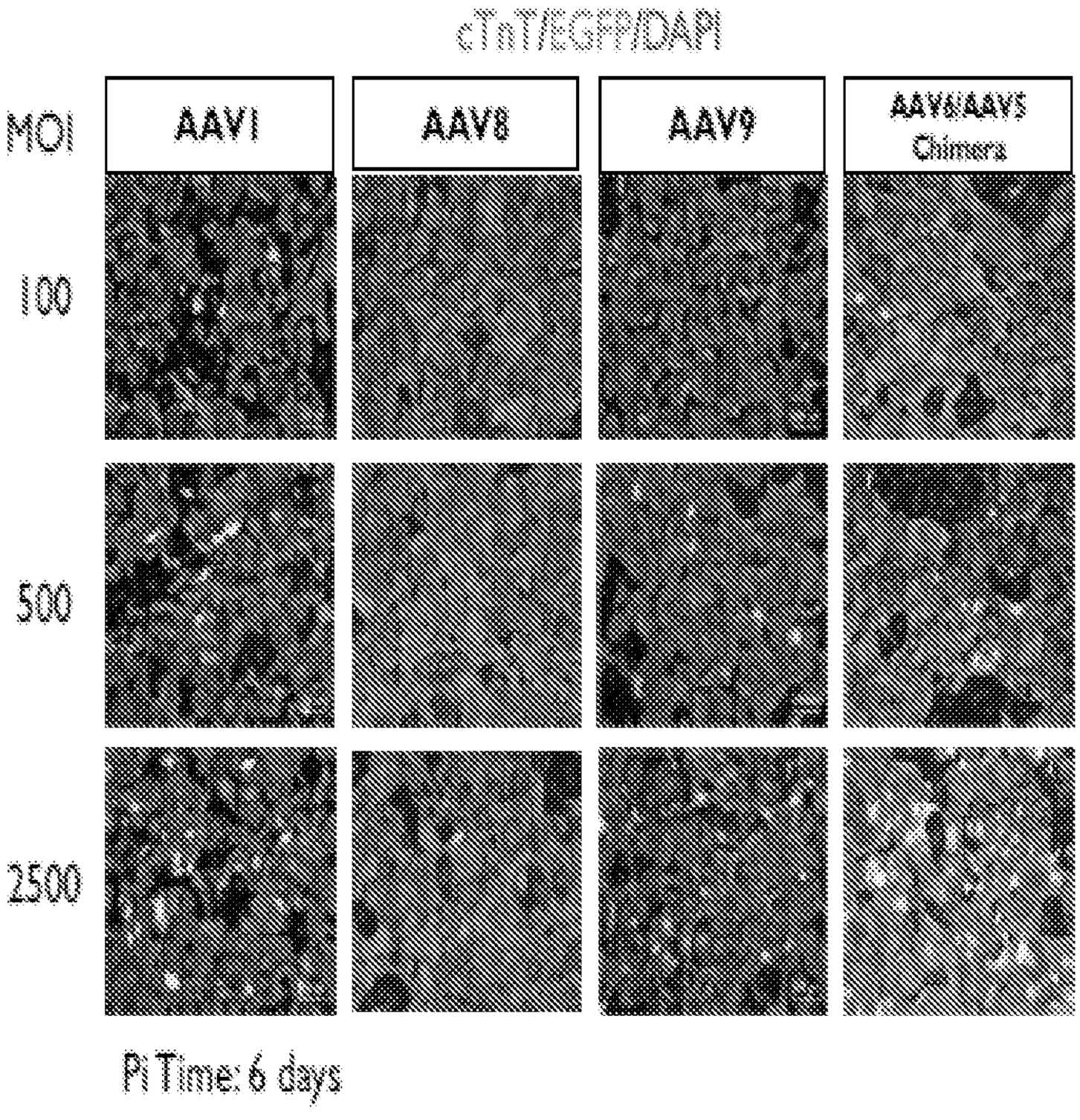

AAV1
AAV2
AAV9
LANKIQRTDA+ Y708I
LANKTTNKDA+ Y708I
LATNKIGVTA+ Y708I
MOI: 500
Day 1
2
3
5

FIGURE 7A


cTnT/EGFP/DAPI
MOI
AAV1
AAV8
AAV9
AAV8/AAV3 Chimera
100
500
2500
Pi Time: 6 days

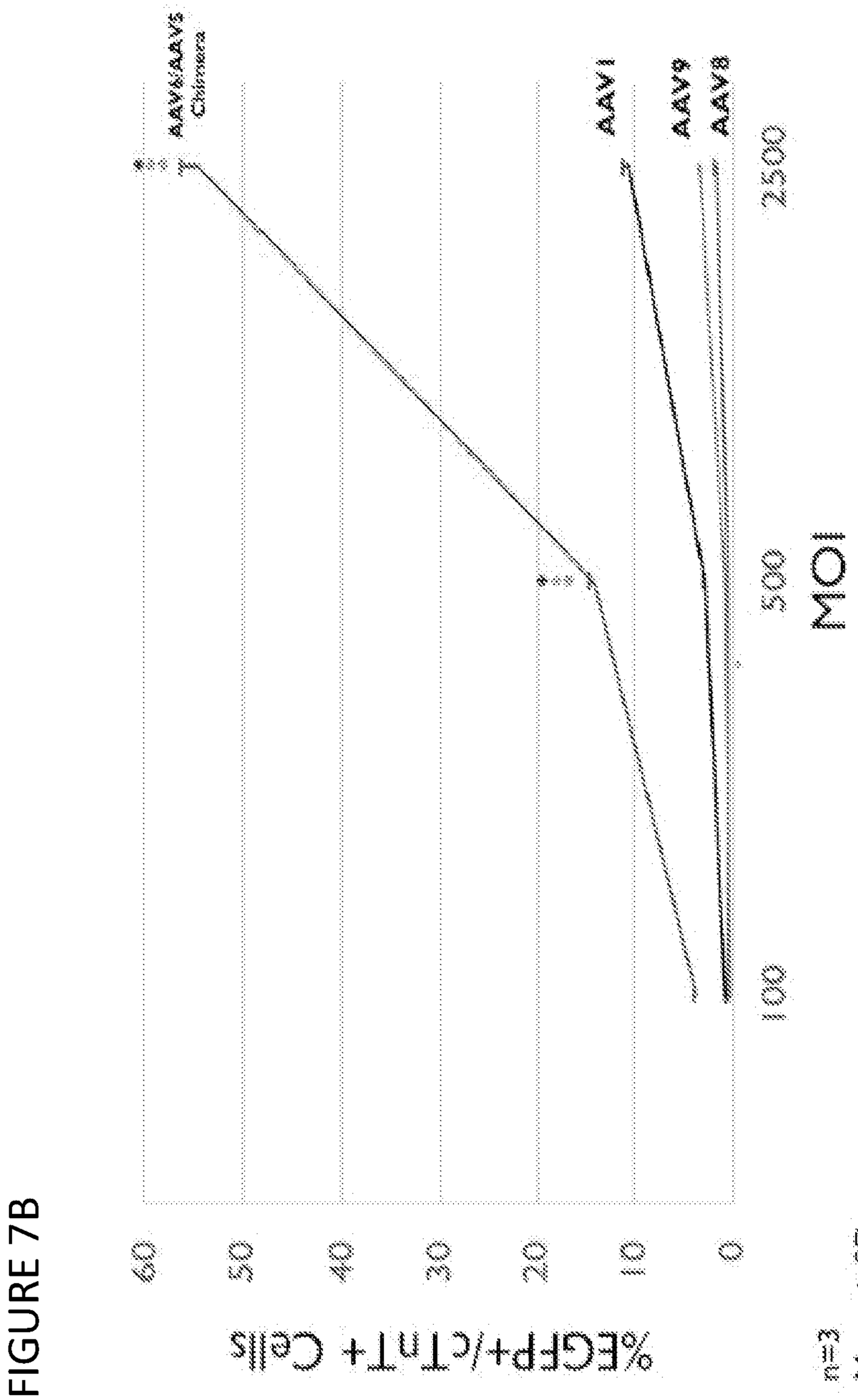

%EGFP+/cTnT+ Cells
60
50
40
30
20
10
0
n=3
Mean ± SD
Two-tailed T-test
* p< 0.05
AAV6/AAV3 Chimera
AAV1
AAV9
AAV8
250
500
1000

FIGURE 7B

FIGURE 7C

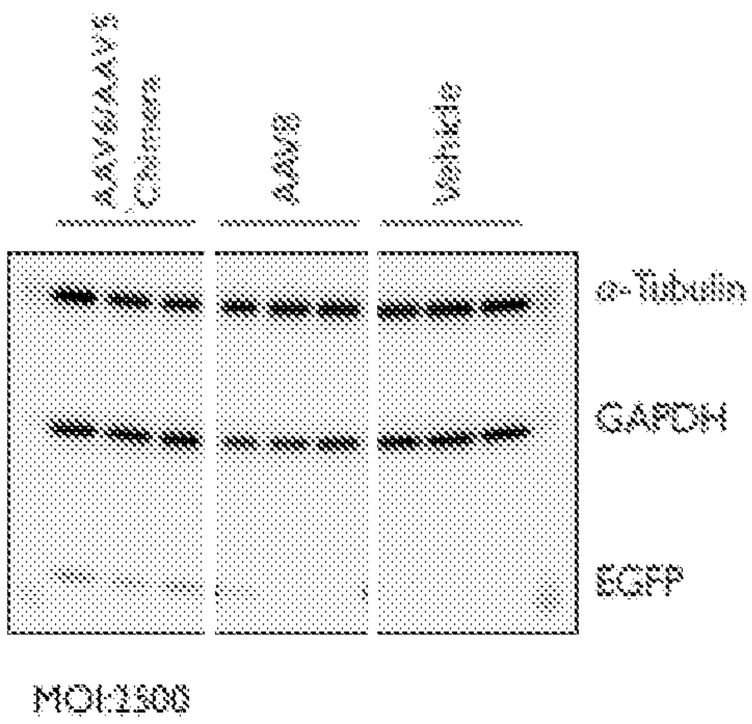


FIGURE 7D

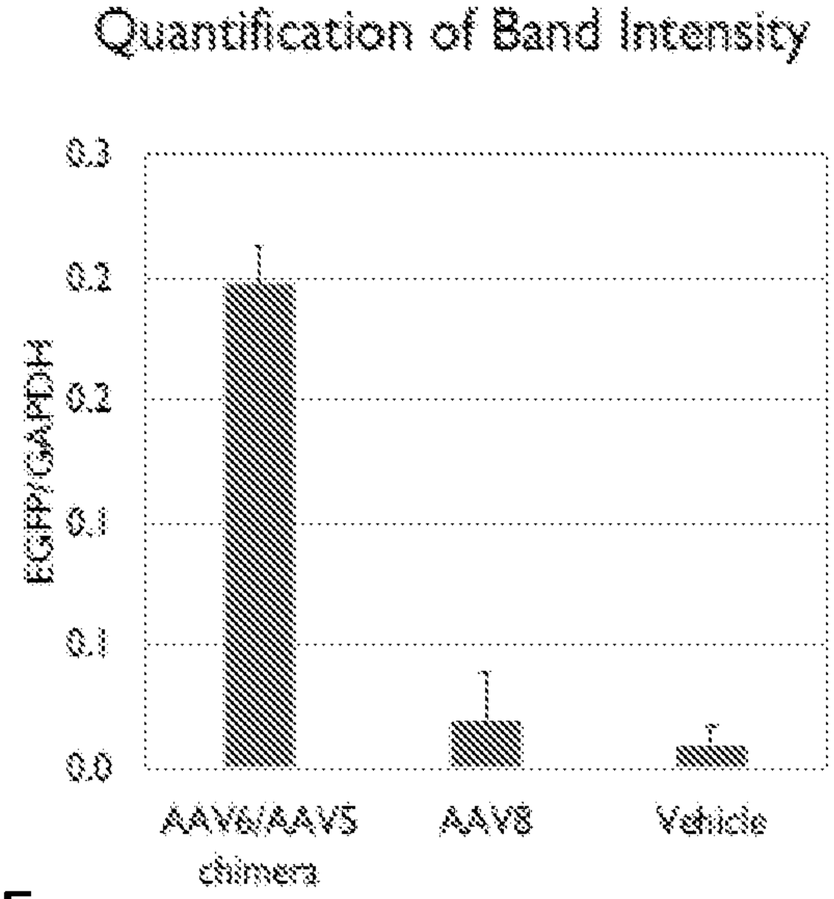
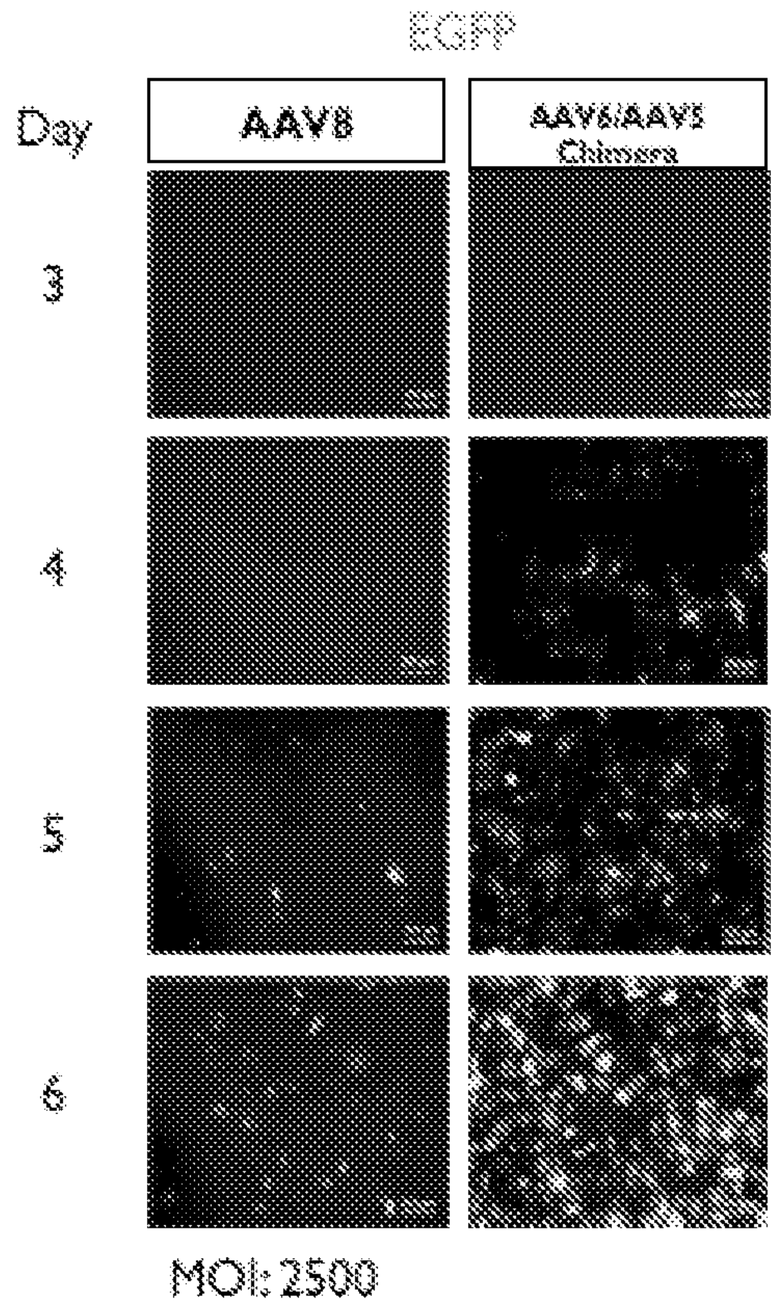


FIGURE 7E



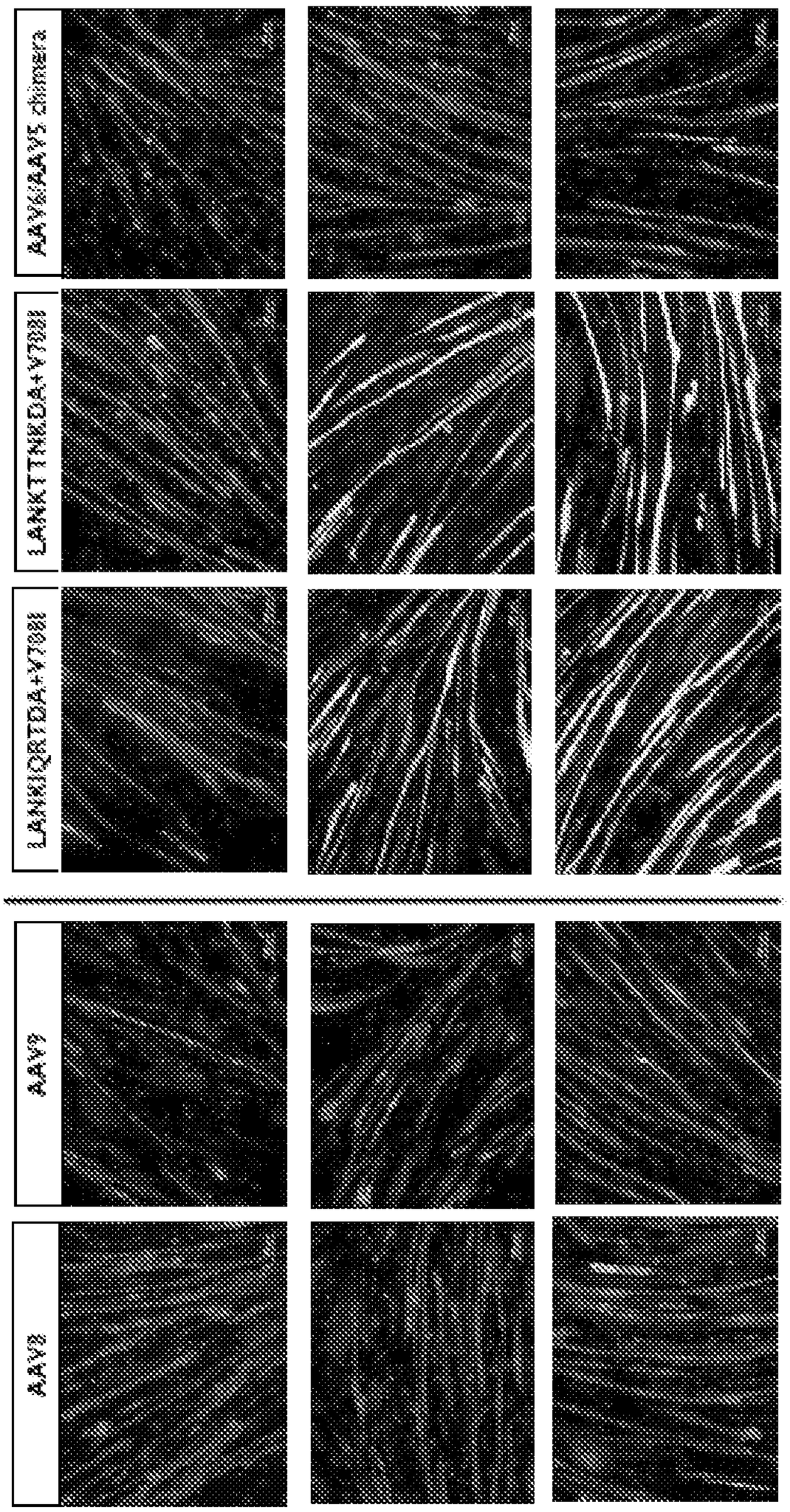

AAV6/AAV5 chimera
LAMKTTNEDA+Y730F
LAMKIQRTDA+Y730F
AAV8
AAV9
Myosin Heavy Chain (MHC)/AAV-ECFP/DAPI
8 Times 7 days
MOI
100
500
2500

FIGURE 8A

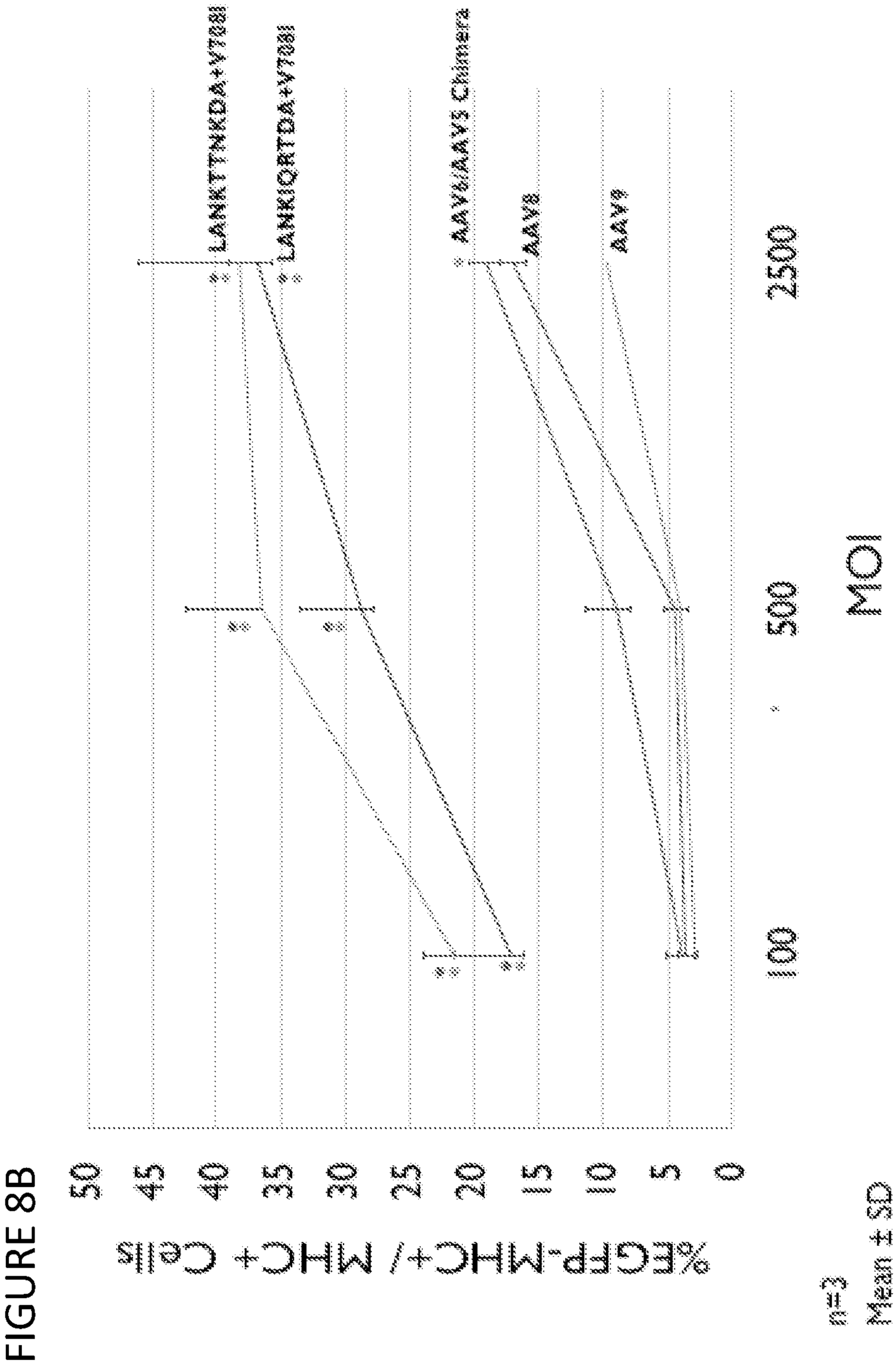

LANKTTNKDA+V708
LANKIQRTDA+V708
AAV8/AAV3 Chimera
AAV8
AAV3
%EGFP-MHC+/ MHC+ Cells
50
45
40
35
30
25
20
15
10
5
0
500
2500
MOI
100
n=3
Mean ± SD
Two-tailed T-tests
* p< 0.05

FIGURE 8B

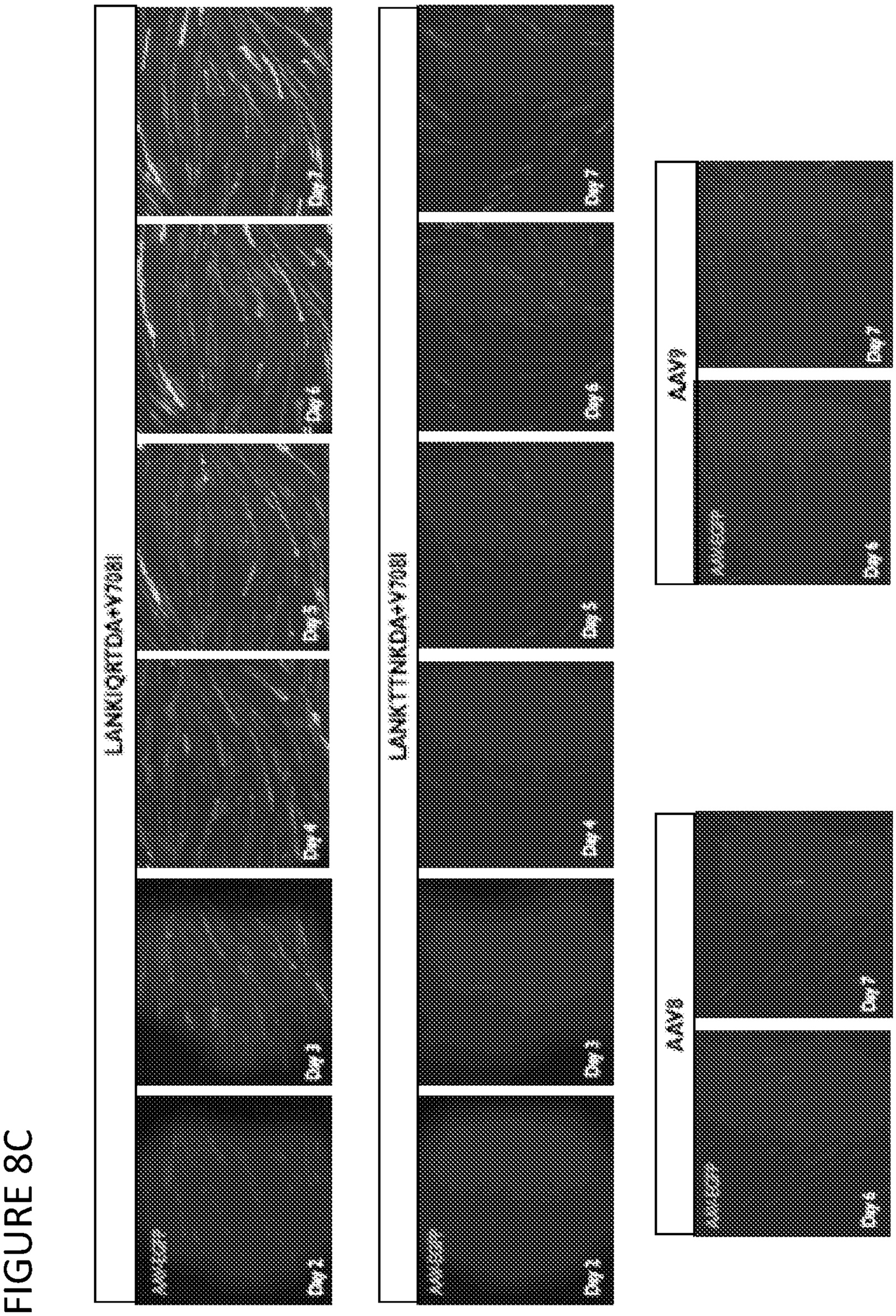

LANKQRTDA+Y708I
Ctrl 1
Ctrl 2
Ctrl 3
Ctrl 4
Ctrl 5
Ctrl 6
Ctrl 7
AAV8

FIGURE 8C

FIGURE 9A

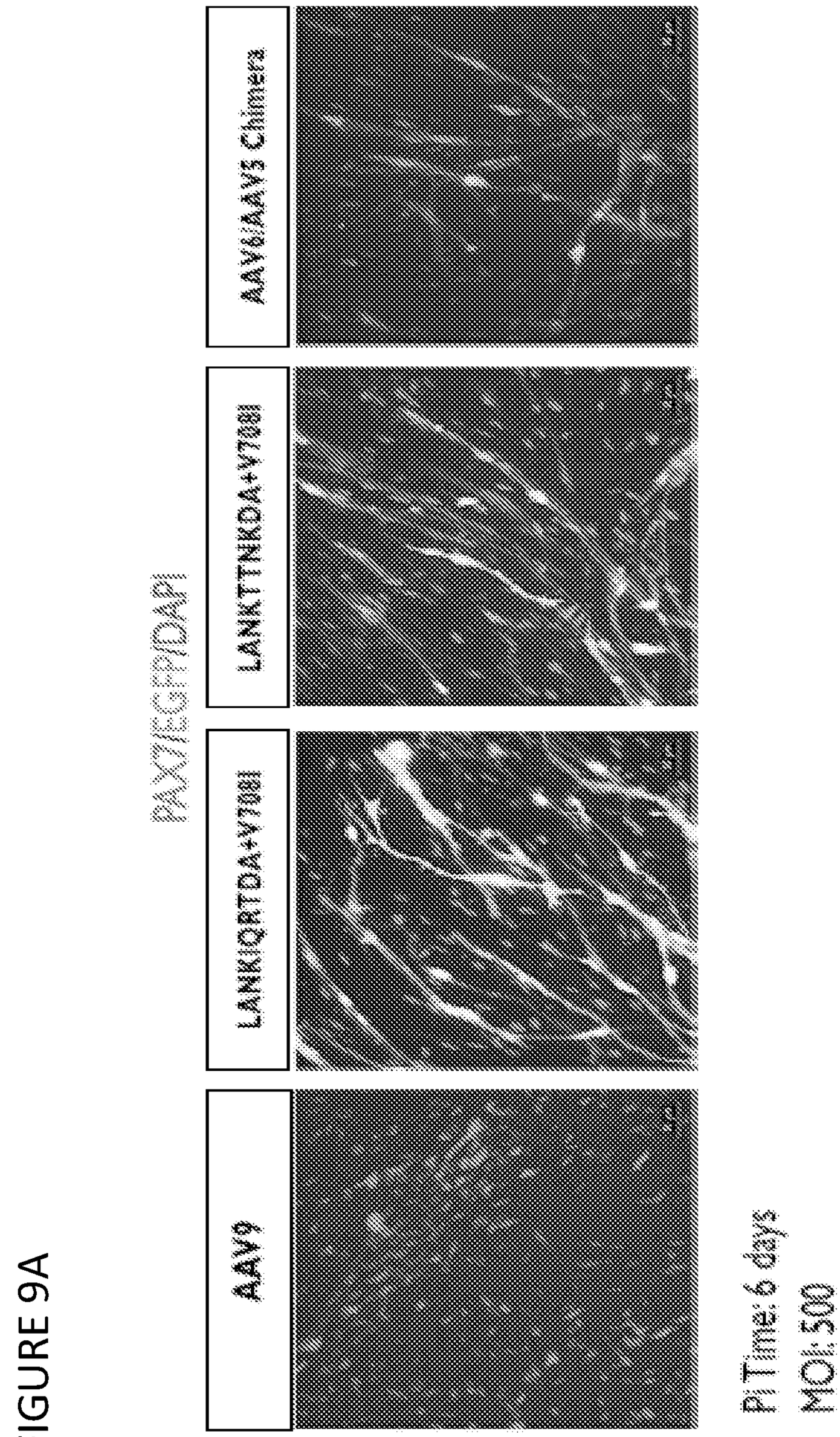
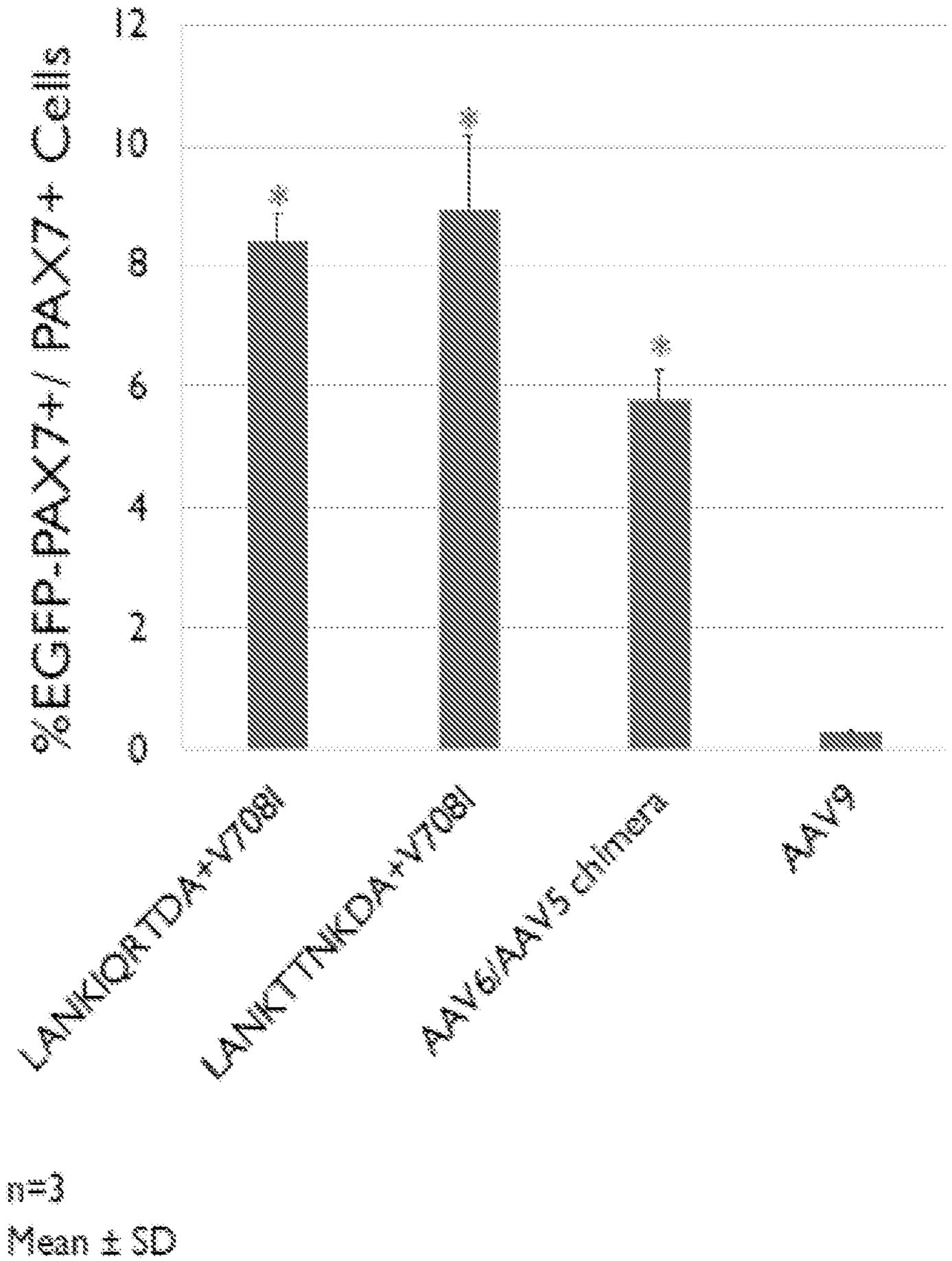

AAV9
LANKTTNKDA+Y788I
LANKIQRTDA+Y788I
AAV6(AAV3 Chimera
LANKTTNKDA+Y788I
PAX7IE
Pi Time: 6 days
MOI: 500

FIGURE 9B



n=3
Mean ± SD
Two-tailed T-tests
※ p< 0.05

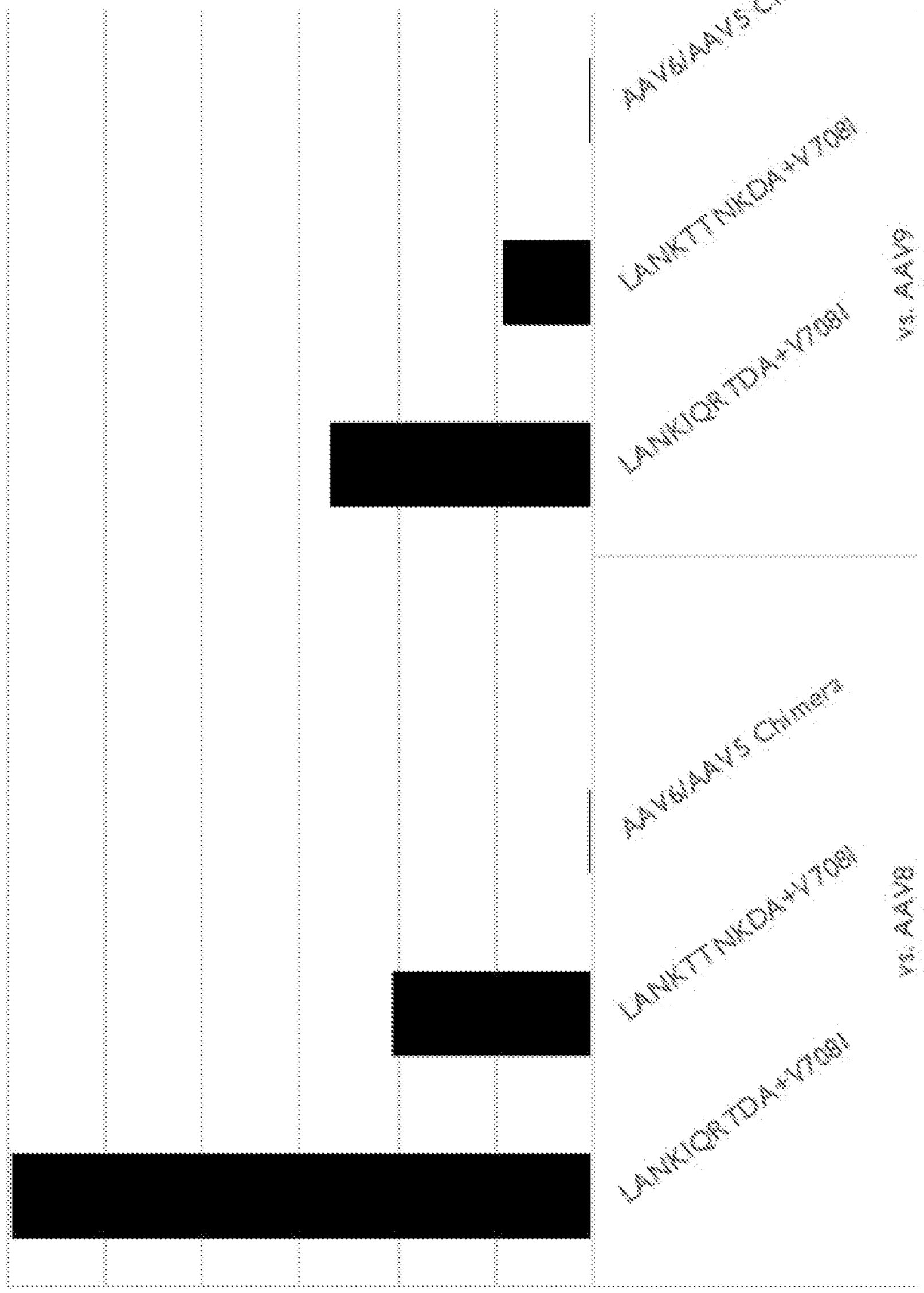

AAV6/AAV5 Chimera
LANKTTNIKDA+V708I
LANKIQRTDA+V708I
AAV6/AAV5 Chimera
LANKTTNIKDA+V708I
LANKIQRTDA+V708I
0
1000
2000
3000
4000
5000
6000
Fold Increase vs. Natural Serotype

FIGURE 10A

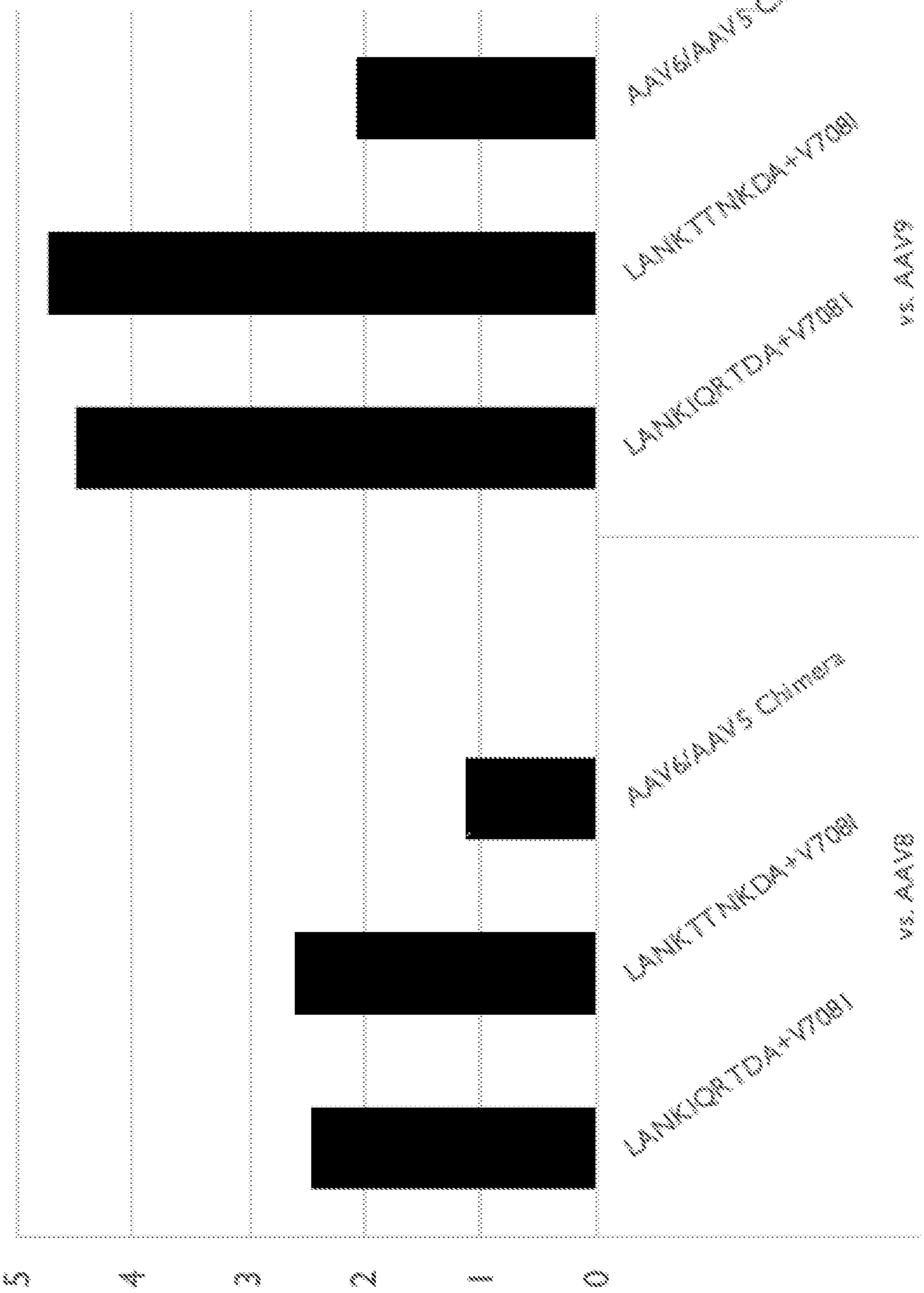
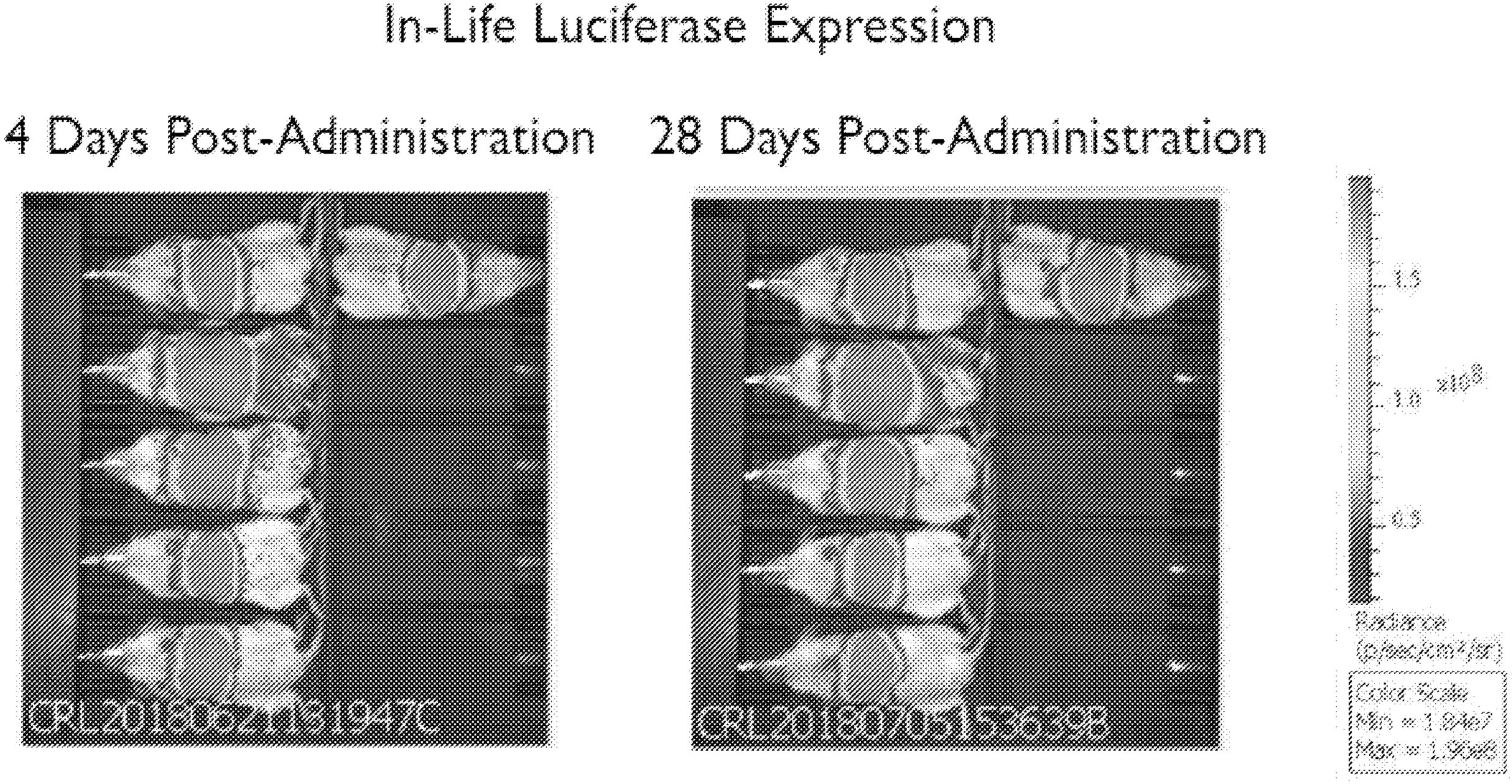

AAV6/AAV5-Chimera
LANKTTNKDA+V708I
LANKIQRTDA+V708I
AAV6/AAV5-Chimera
LANKTTNKDA+V708I
LANKIQRTDA+V708I
0
1
2
3
4
5
Fold Increase vs. Natural Serotype

FIGURE 10B

FIGURE 11A


In-Life Luciferase Expression
14 Days Post-Administration
28 Days Post-Administration
ORL20180621319147C
ORL20180705153639B
1.5
1.0
0.5
x10^8
Radiance (p/sec/cm²/sr)
Color Scale
Min = 1.04e7
Max = 1.90e8

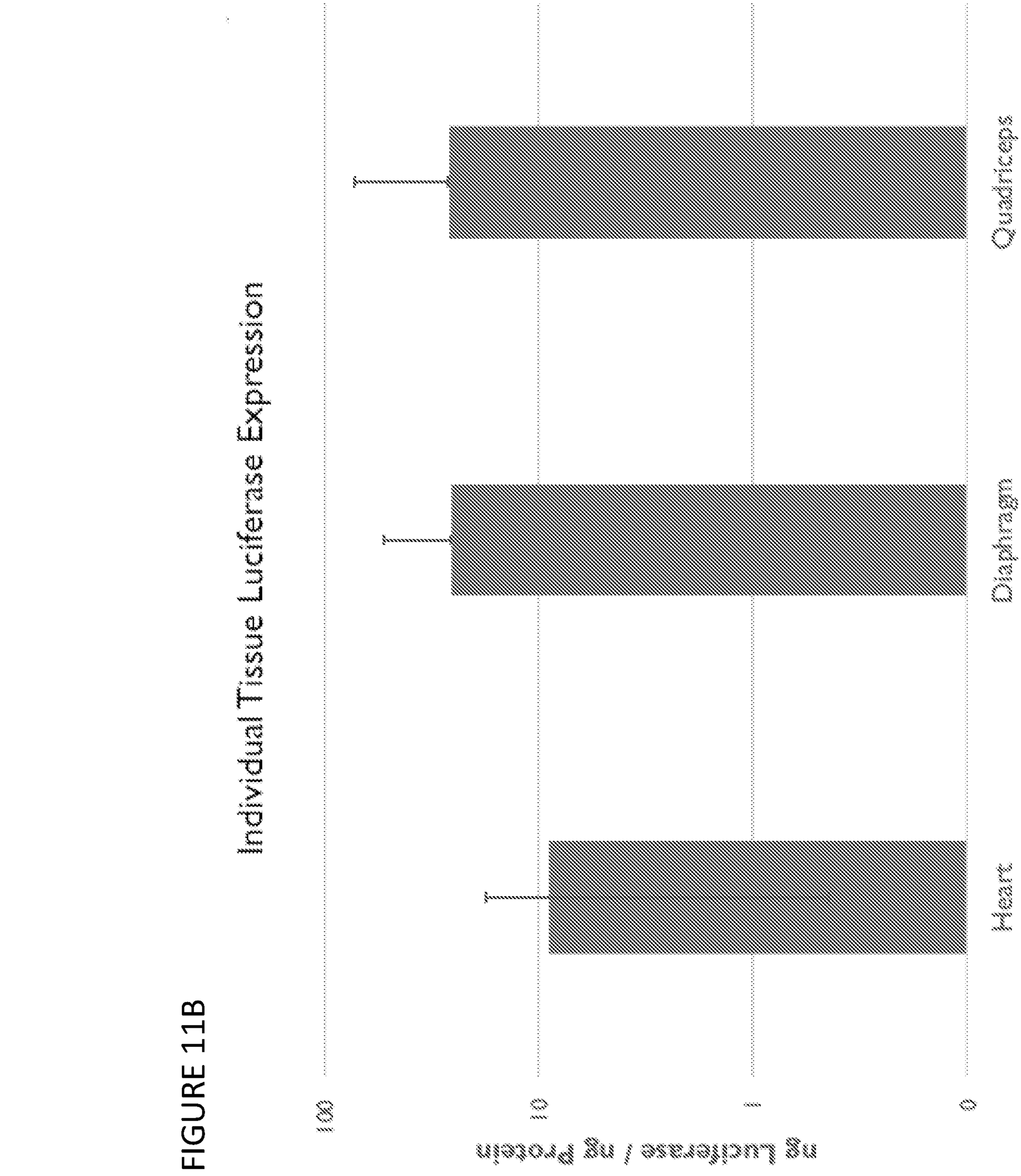

Individual Tissue Luciferase Expression
ng Luciferase / mg Protein
0
2
4
6
Heart
Diaphragm
Quadriceps

FIGURE 1B

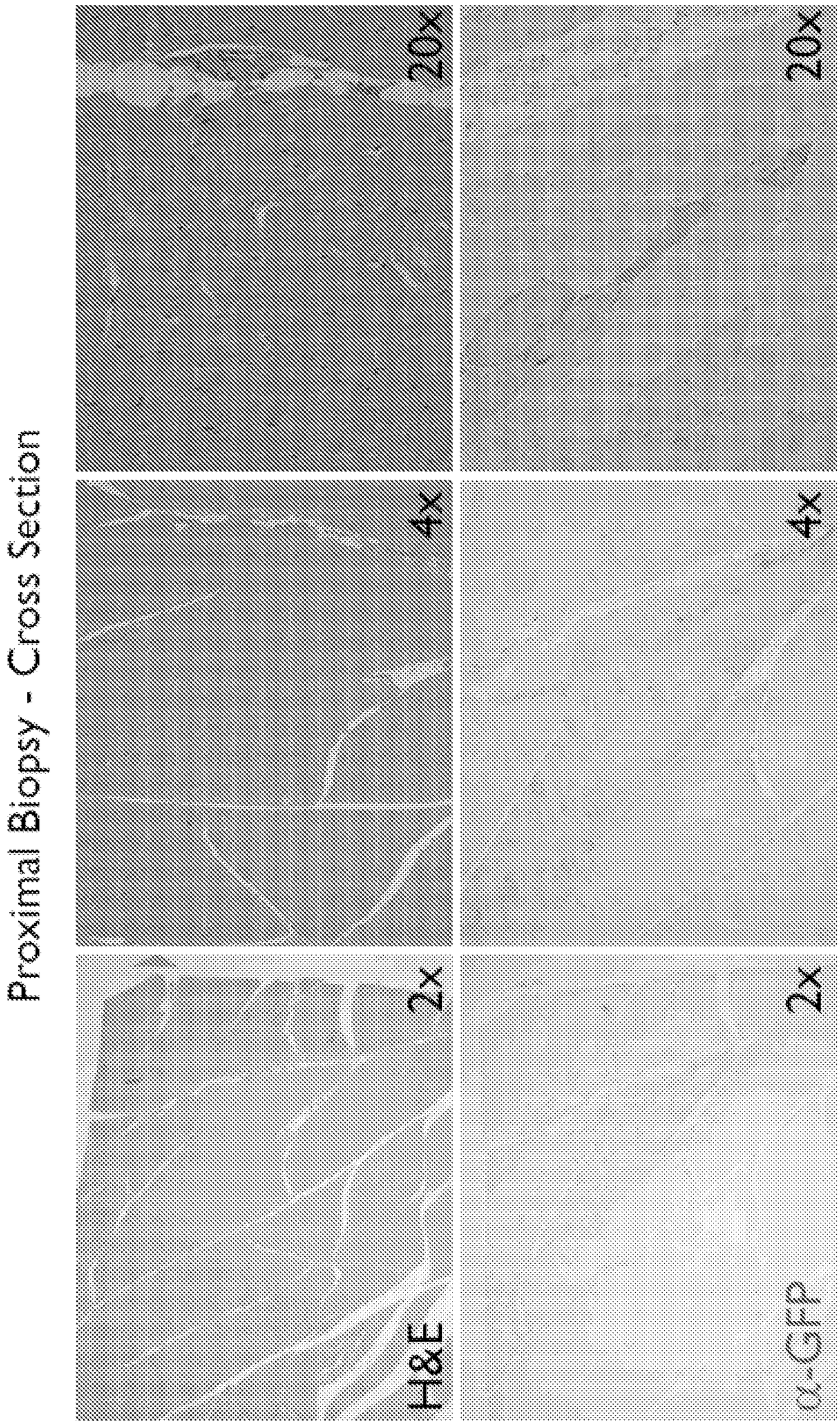


FIGURE 12A

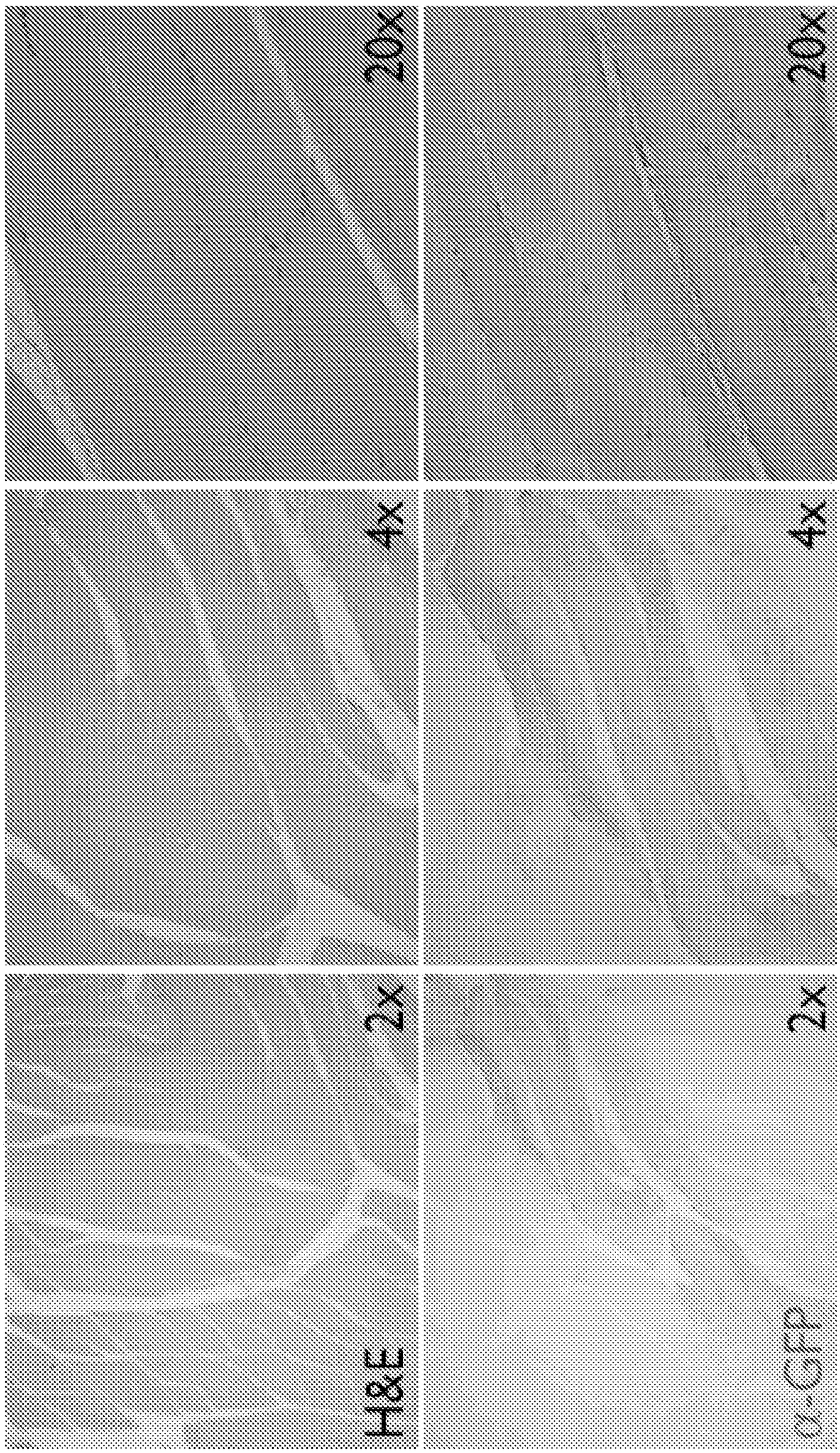


FIGURE 12B