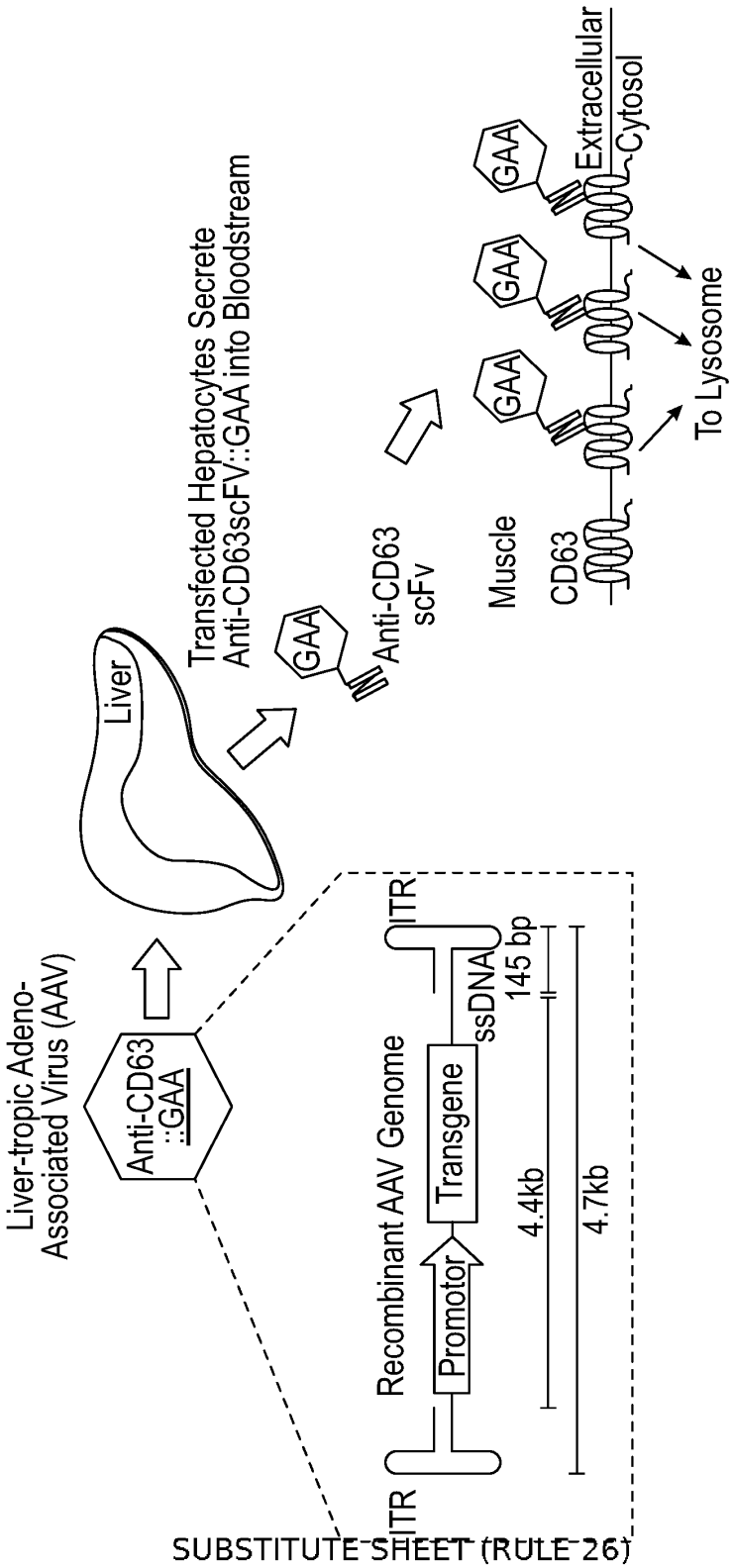

Liver-tropic Adeno-Associated Virus (AAV)
Anti-CD63 ::GAA
Liver
Transfected Hepatocytes Secrete Anti-CD63scFV::GAA into Bloodstream
GAA
Anti-CD63 scFv
Muscle
CD63
GAA
GAA
GAA
Extracellular
Cytosol
To Lysosome
Recombinant AAV Genome
ITR
Promotor
Transgene
ssDNA
ITR
145 bp
4.4kb
4.7kb

FIG. 1

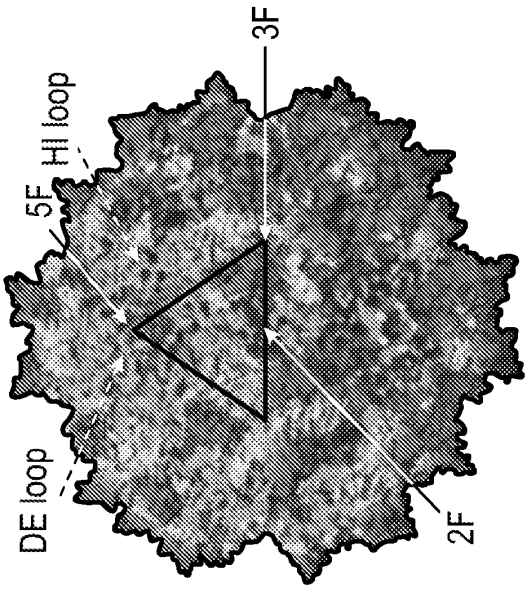

3F
HI loop
5F
DE loop
2F

FIG. 2A

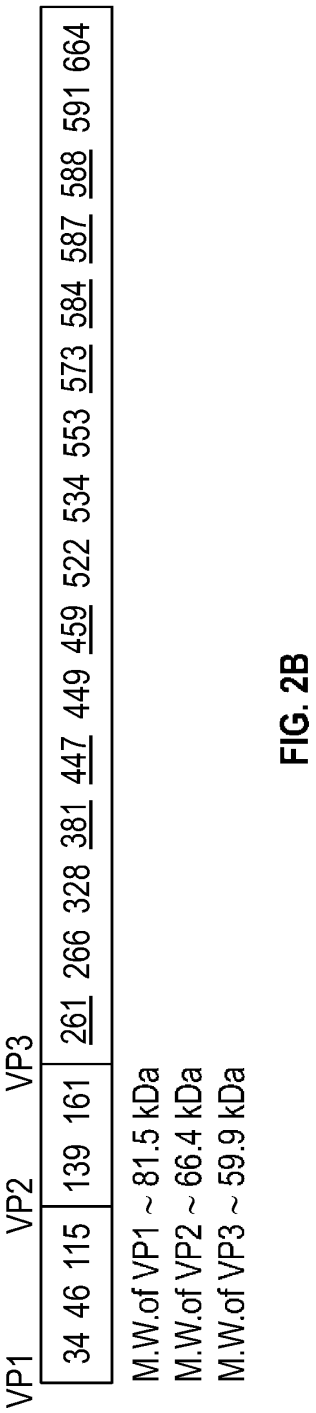
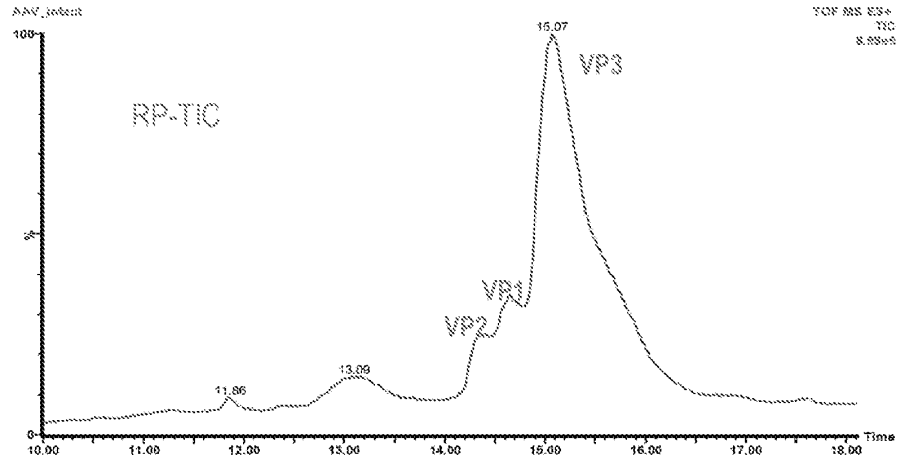

VP1
VP2
VP3
34 46 115 139 161 261 266 328 381 447 449 459 522 534 553 573 584 587 588 591 664
M.W.of VP1 ~ 81.5 kDa
M.W.of VP2 ~ 66.4 kDa
M.W.of VP3 ~ 59.9 kDa

FIG. 2B

FIG. 3A


RP-TIC
VP3
VP1
VP2
15.07
13.09
11.86
100
%
0
Time
10.00
11.00
12.00
13.00
14.00
15.00
16.00
17.00
18.00

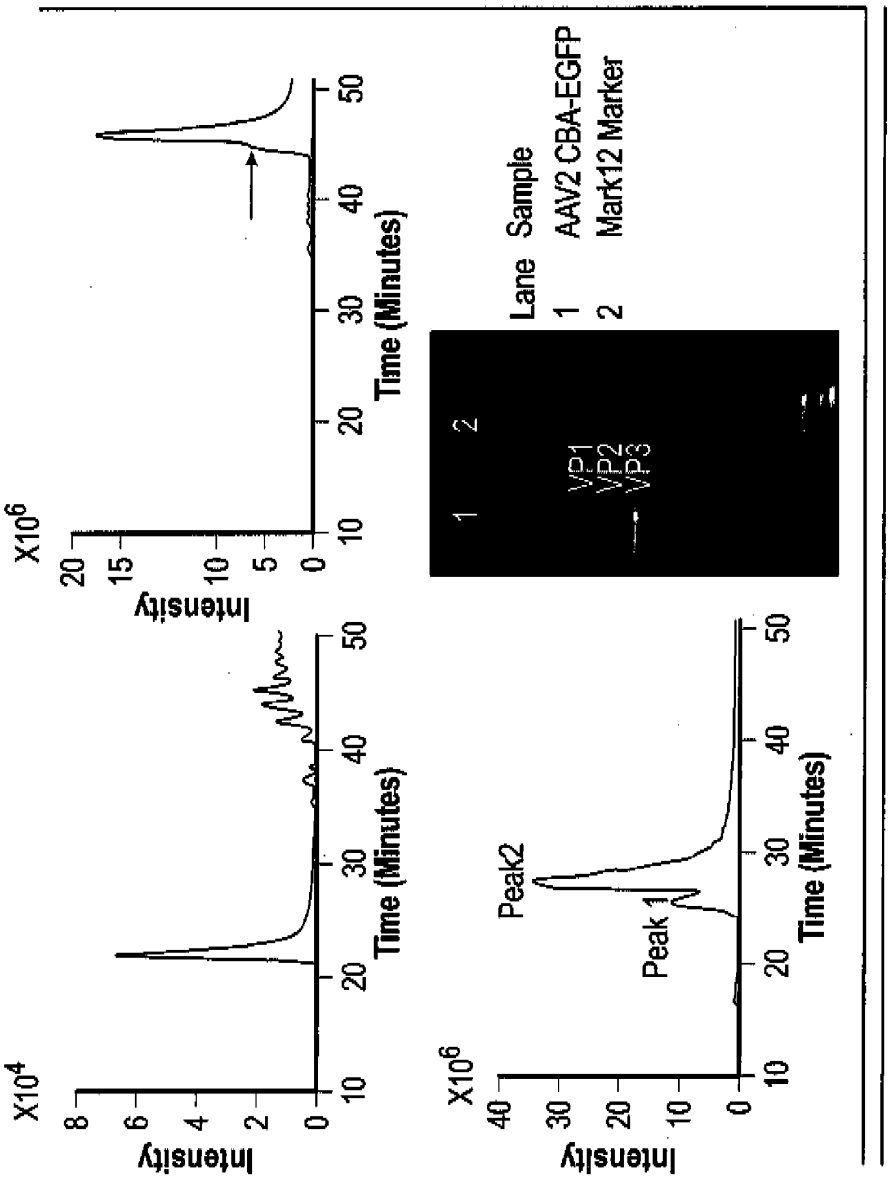

X10^6
Intensity
Time (Minutes)
X10^4
Intensity
Time (Minutes)
Lane Sample
1 AAV2 CBA-EGFP
2 Mark12 Marker
1
2
VP1
VP2
VP3
X10^6
Intensity
Peak2
Peak 1
Time (Minutes)

FIG. 3B

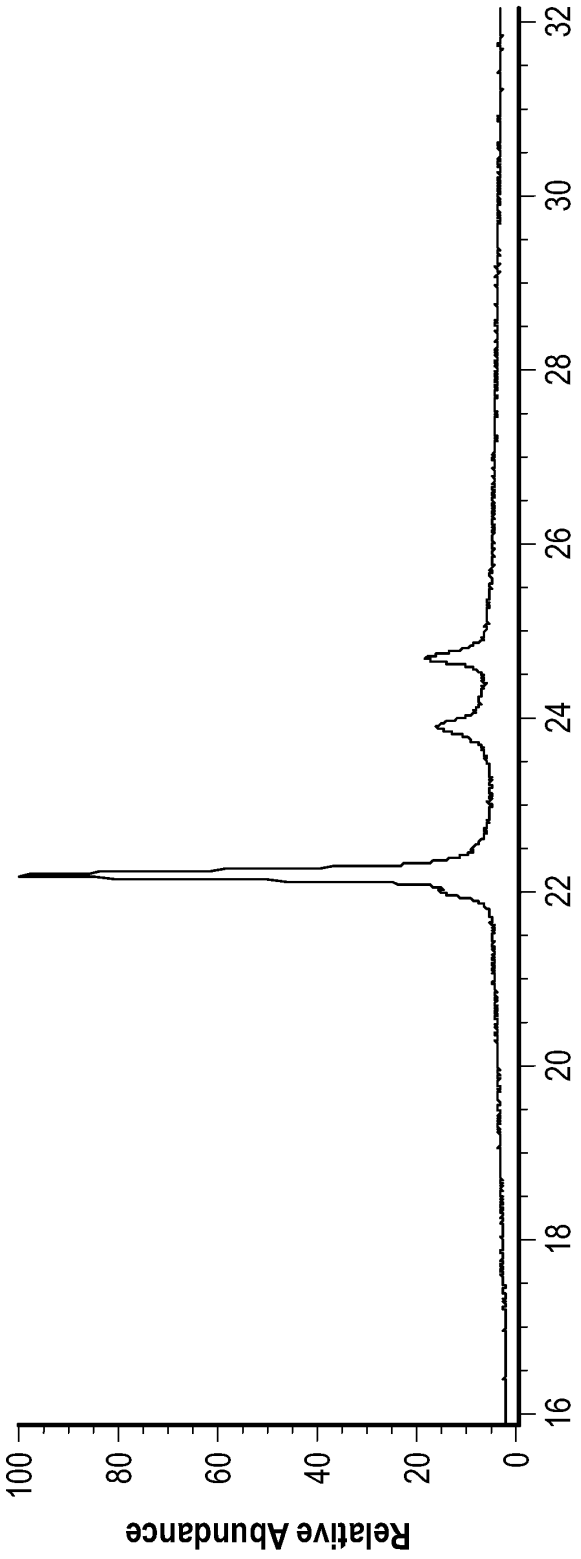

Relative Abundance
100
80
60
40
20
0
16
18
20
22
24
26
28
30
32
Time (min)

FIG. 4

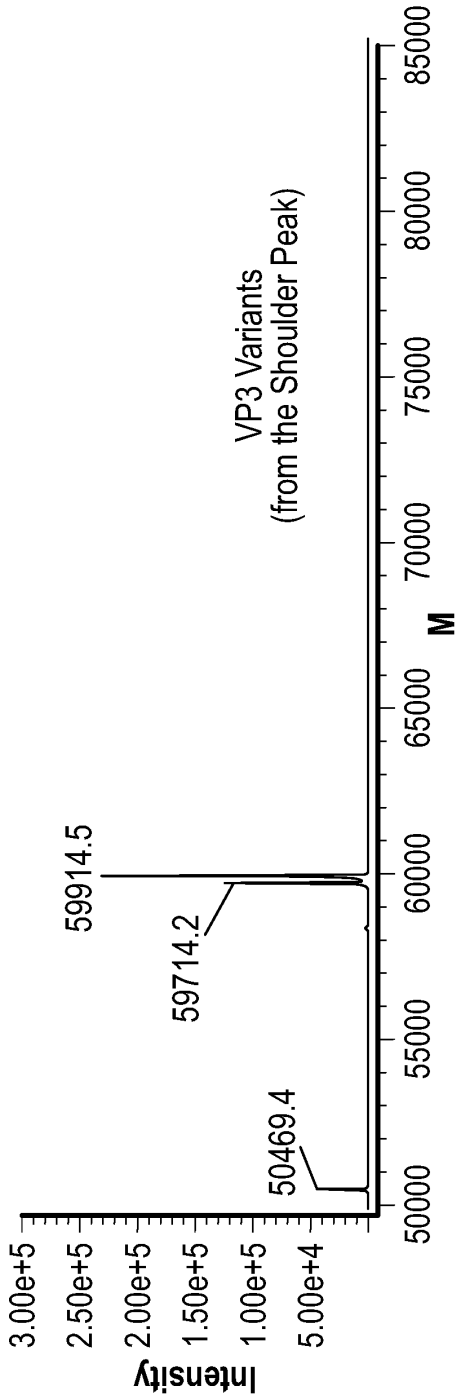

VP3 Variants
(from the Shoulder Peak)
59914.5
59714.2
50469.4
Intensity
3.00e+5
2.50e+5
2.00e+5
1.50e+5
1.00e+5
5.00e+4
50000
55000
60000
65000
70000
75000
80000
85000
M

FIG. 5A

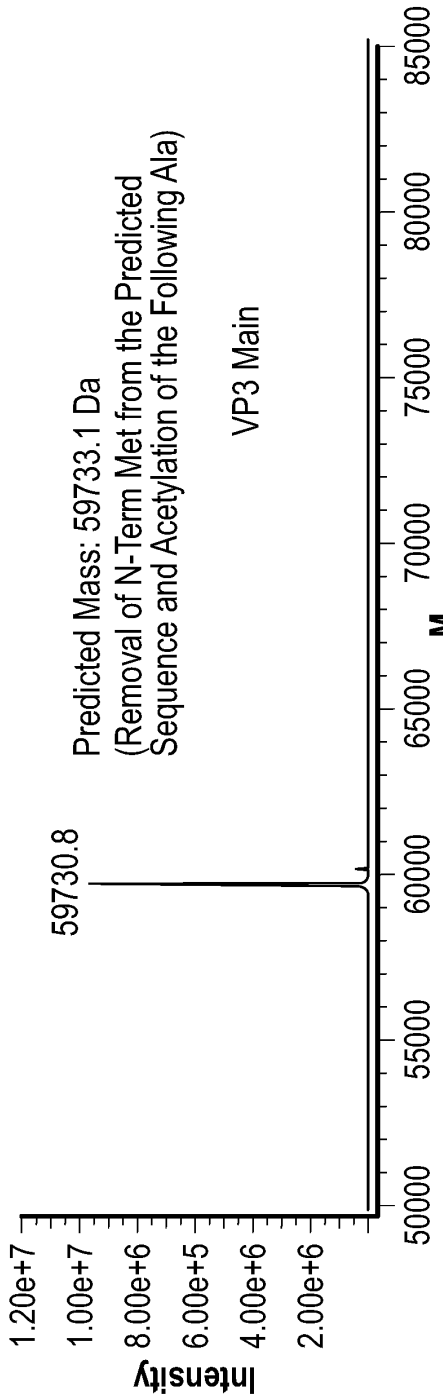

Predicted Mass: 59733.1 Da
(Removal of N-Term Met from the Predicted
Sequence and Acetylation of the Following Ala)
VP3 Main
59730.8
Intensity
1.20e+7
1.00e+7
8.00e+6
6.00e+5
4.00e+6
2.00e+6
50000
55000
60000
65000
70000
75000
80000
85000
M

FIG. 5B

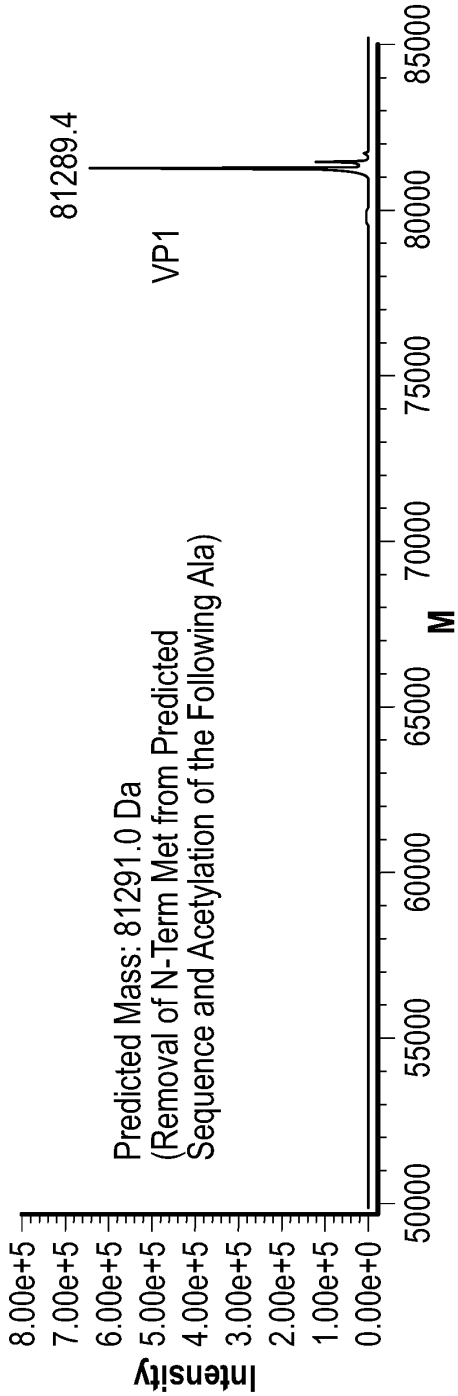

81289.4
VP1
Predicted Mass: 81291.0 Da
(Removal of N-Term Met from Predicted
Sequence and Acetylation of the Following Ala)
Intensity
8.00e+5
7.00e+5
6.00e+5
5.00e+5
4.00e+5
3.00e+5
2.00e+5
1.00e+5
0.00e+0
50000
55000
60000
65000
70000
75000
80000
85000
M

FIG. 5C

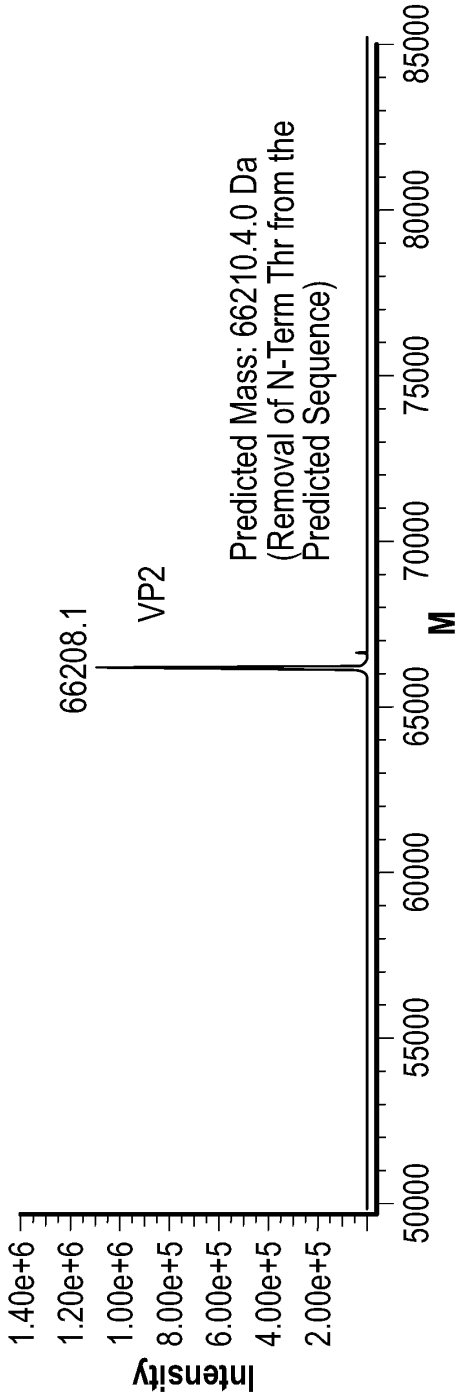

66208.1
VP2
Predicted Mass: 66210.4.0 Da
(Removal of N-Term Thr from the
Predicted Sequence)
Intensity
1.40e+6
1.20e+6
1.00e+6
8.00e+5
6.00e+5
4.00e+5
2.00e+5
50000
55000
60000
65000
70000
75000
80000
85000
M

FIG. 5D

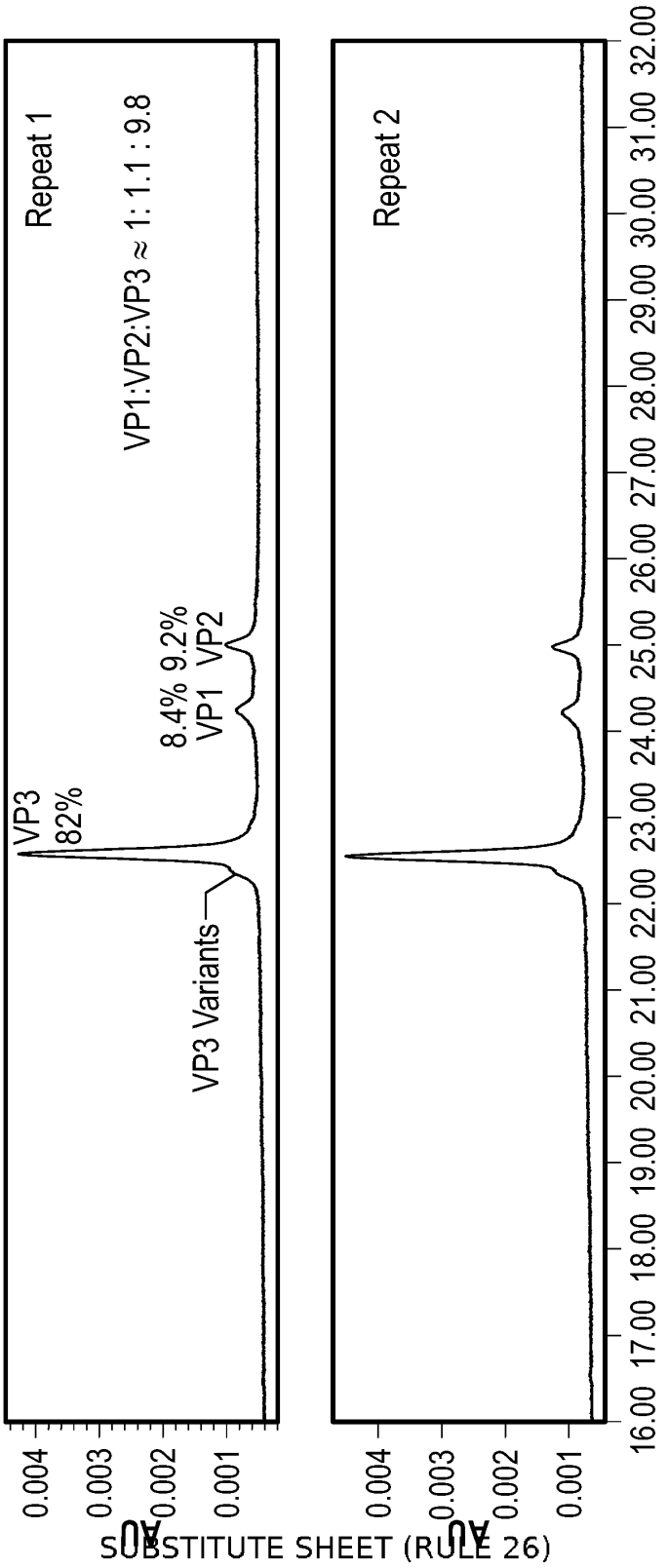

Repeat 1
VP1:VP2:VP3 ≈ 1: 1.1 : 9.8
VP3
82%
8.4% 9.2%
VP1 VP2
VP3 Variants
Repeat 2
0.004
0.003
0.002
0.001
AU
16.00 17.00 18.00 19.00 20.00 21.00 22.00 23.00 24.00 25.00 26.00 27.00 28.00 29.00 30.00 31.00 32.00
Minutes

FIG. 6