Figure 1.

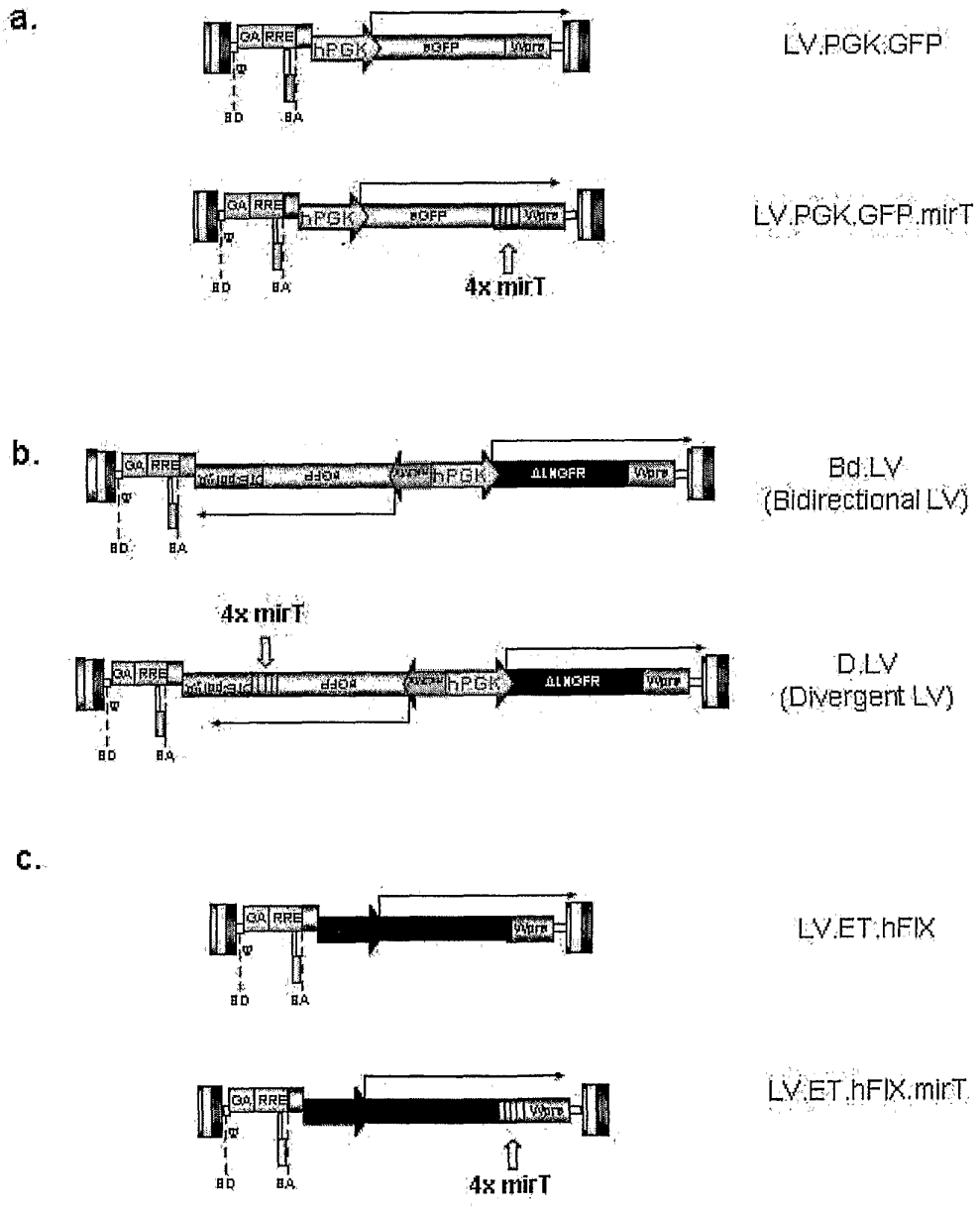
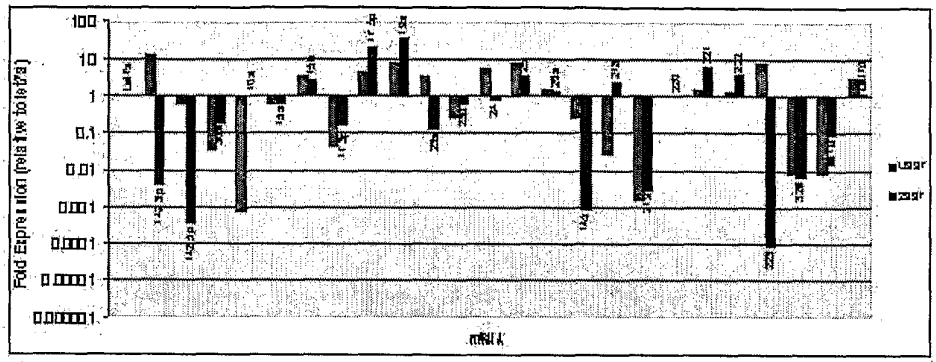
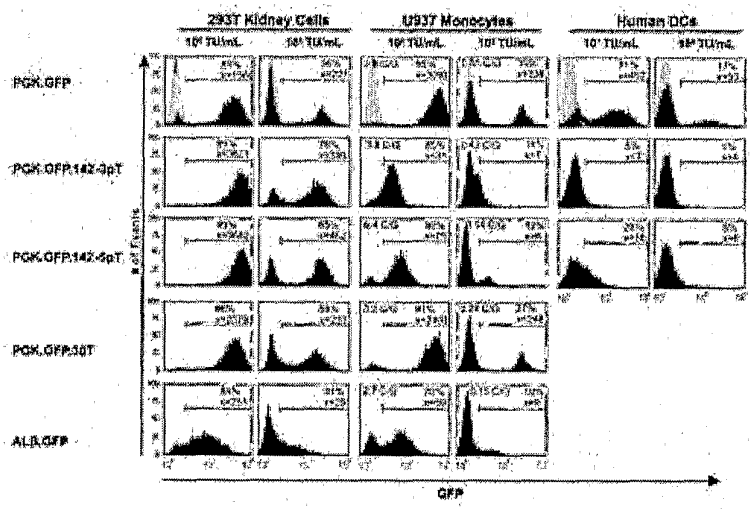

a.
GA
RRE
hPGK
ΔLNGFR
WPRE
ΨI
BD
BA
LV.PGK.GFP
LV.PGK.GFP.mirT
4x mirT
b.
Bd.LV
(Bidirectional LV)
D.LV
(Divergent LV)
c.
LV.ET.hFIX
LV.ET.hFIX.mirT

Figure 2.

a.


100
10
1
0.1
0.01
0.001
0.0001
0.00001
0.000001
Fold Expression (relative to Ref2)
miRNA

b.


293T Kidney Cells
U937 Monocytes
Human DCs
PGK.GFP
PGK.GFP.142-3pT
PGK.GFP.142-5pT
PGK.GFP.3pT
ALB.GFP
# of Events
GFP

c.

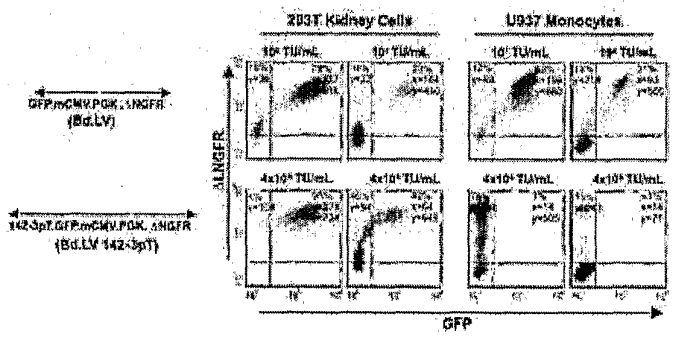

293T Kidney Cells
U937 Monocytes
GFP.mCMV.PGK.ΔNGFR
(Bd.LV)
142-3pT.GFP.mCMV.PGK.ΔNGFR
(Bd.LV 142-3pT)
ΔNGFR
GFP

Figure 2.

d.

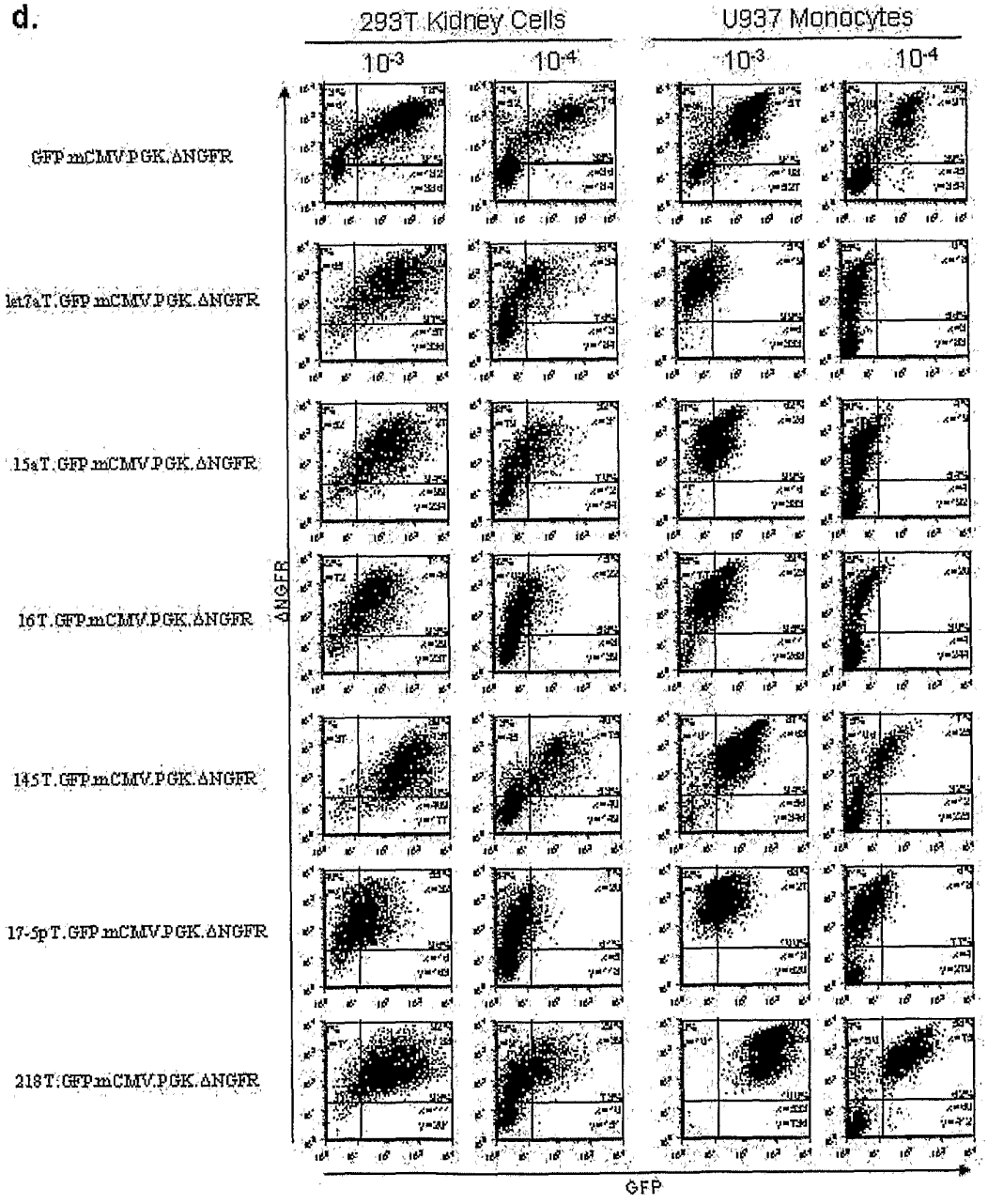

293T Kidney Cells
U937 Monocytes
10-3
10-4
10-3
10-4
GFP.mCMV.PGK.ΔNGFR
let7aT.GFP.mCMV.PGK.ΔNGFR
15aT.GFP.mCMV.PGK.ΔNGFR
16T.GFP.mCMV.PGK.ΔNGFR
145T.GFP.mCMV.PGK.ΔNGFR
17-5pT.GFP.mCMV.PGK.ΔNGFR
218T.GFP.mCMV.PGK.ΔNGFR
ΔNGFR
GFP

Figure 3.

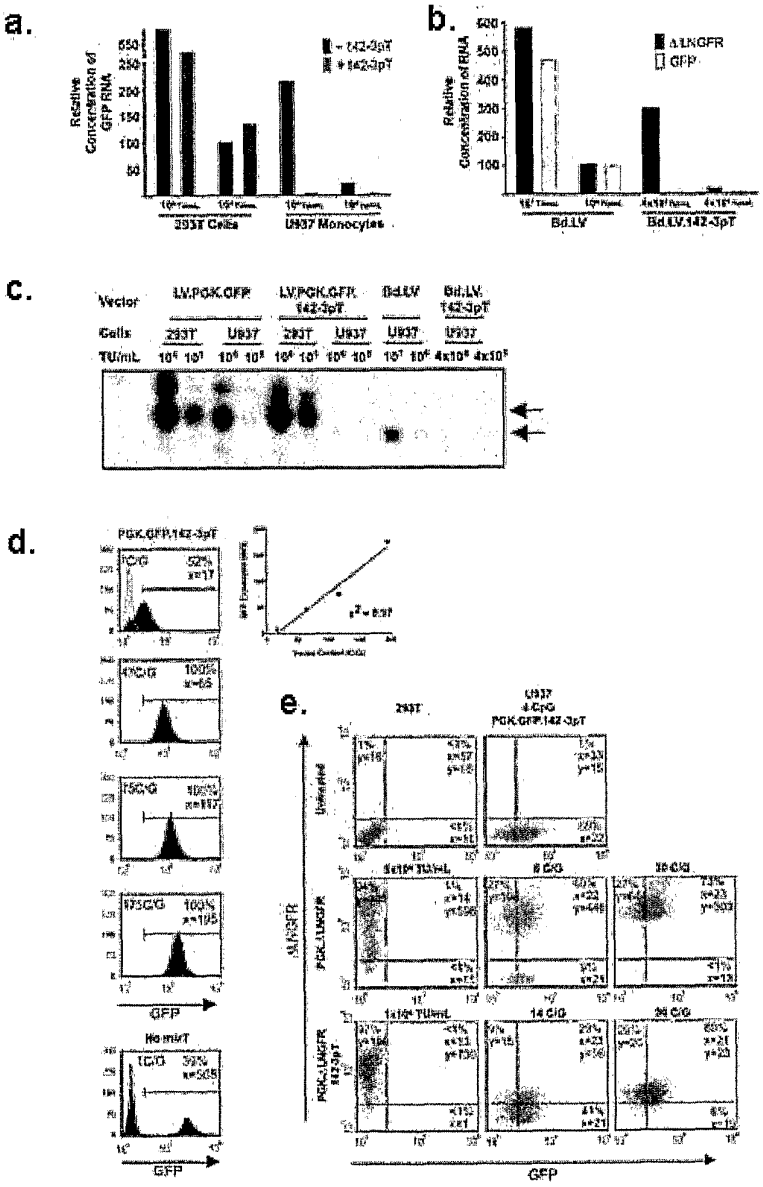

a.
b.
c.
d.
e.
Vector
LV.PGK.GFP
LV.PGK.GFP 142-3pT
Bd.LV
Bd.LV 142-3pT
Cells
293T
U937
TU/mL
GFP
293T Cells
U937 Monocytes
Bd.LV
Bd.LV.142-3pT

Figure 4.

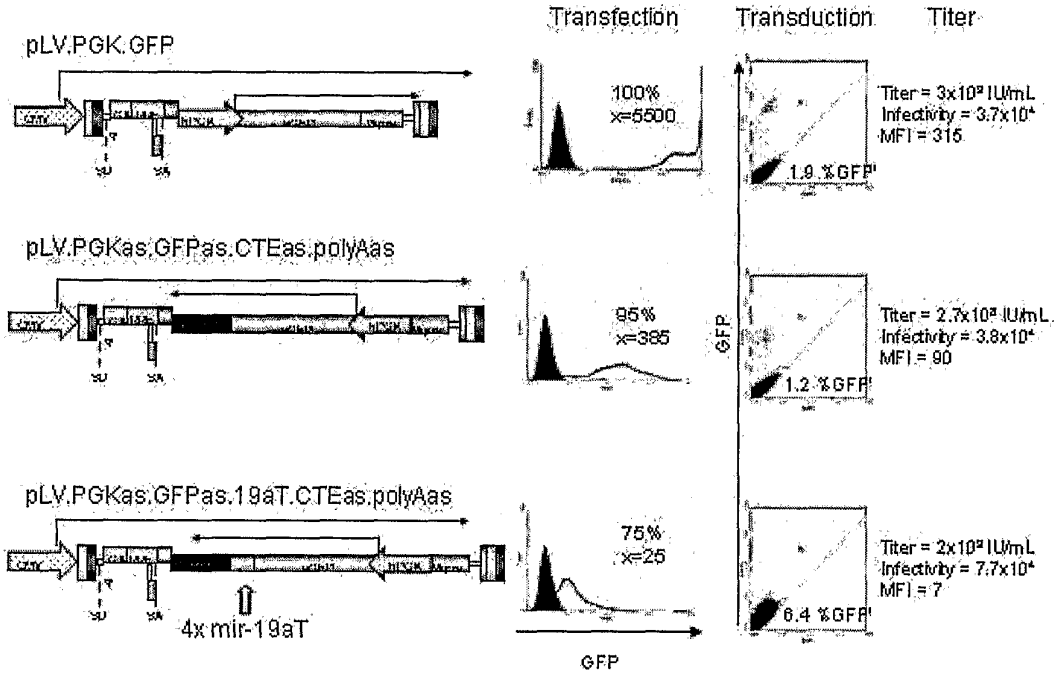

Transfection
Transduction
Titer
pLV.PGK.GFP
100%
x=5500
1.9 %GFP
Titer = 3x10⁸ IU/mL
Infectivity = 3.7x10⁴
MFI = 315
pLV.PGKas.GFPas.CTEas.polyAas
95%
x=385
1.2 %GFP
Titer = 2.7x10⁸ IU/mL
Infectivity = 3.8x10⁴
MFI = 90
pLV.PGKas.GFPas.19aT.CTEas.polyAas
4x mir-19aT
75%
x=25
6.4 %GFP
Titer = 2x10⁸ IU/mL
Infectivity = 7.7x10⁴
MFI = 7
GFP
GFP

Figure 5.

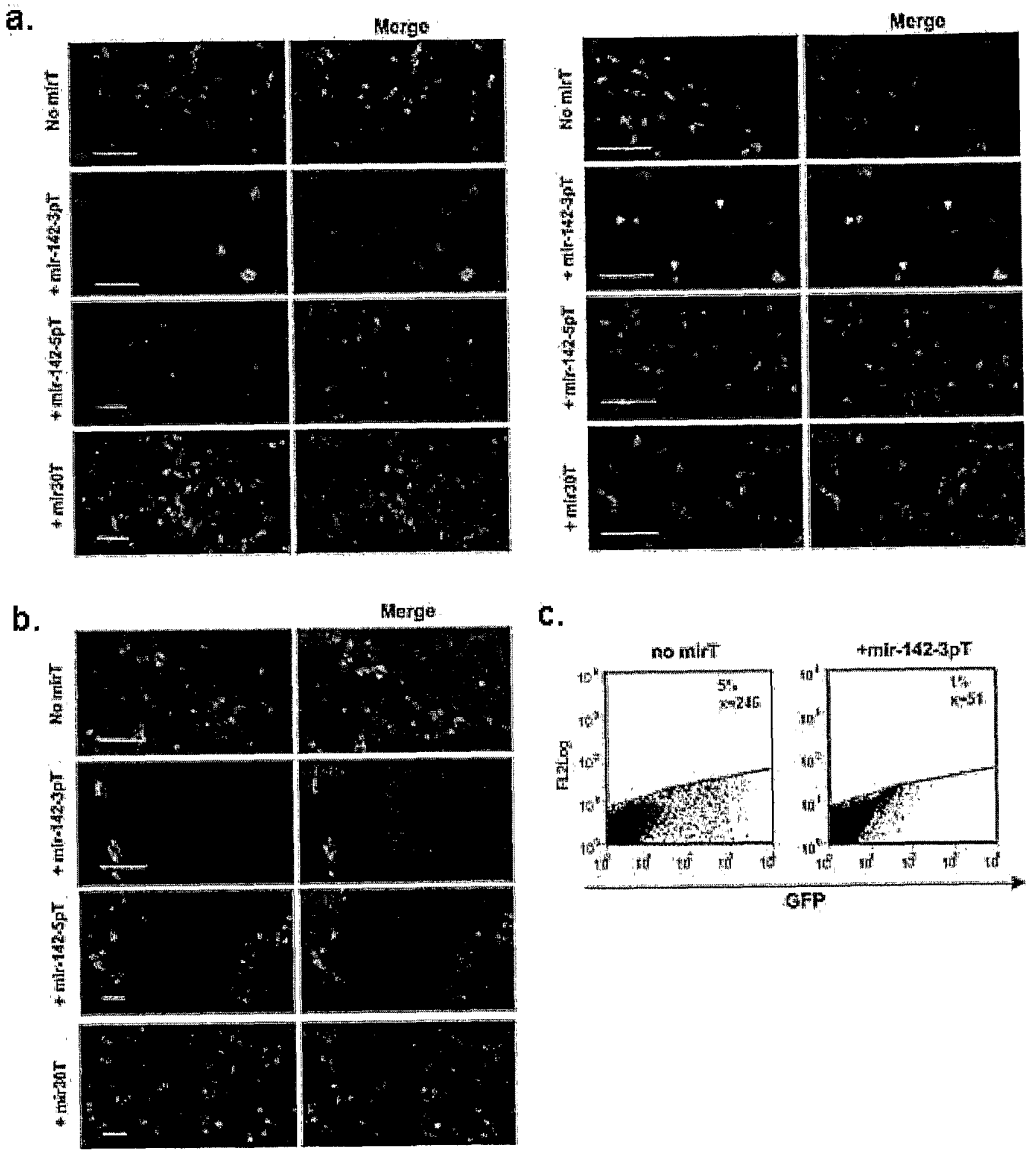
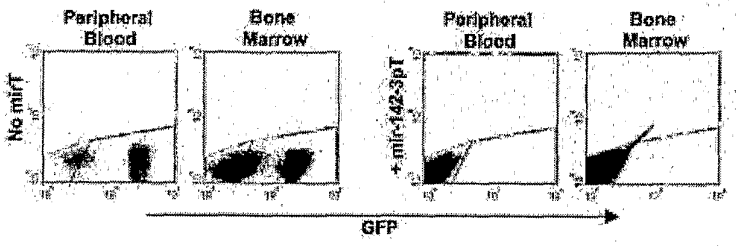

a.
Merge
No mirT
+ mir-142-3pT
+ mir-142-5pT
+ mir30T
Merge
No mirT
+ mir-142-3pT
+ mir-142-5pT
+ mir30T
b.
Merge
No mirT
+ mir-142-3pT
+ mir-142-5pT
+ mir30T
c.
no mirT
+mir-142-3pT
5%
1%
FL2Log
GFP

Figure 6

a.


Peripheral Blood
Bone Marrow
Peripheral Blood
Bone Marrow
No mirT
+ mir-142-3pT
GFP

b.

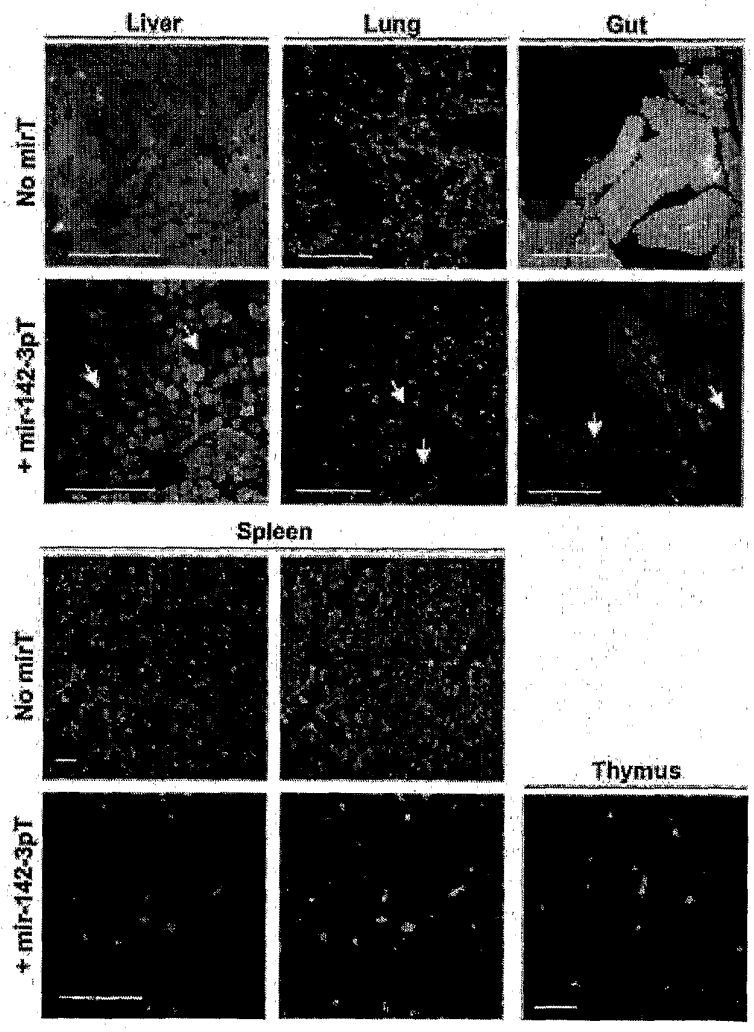
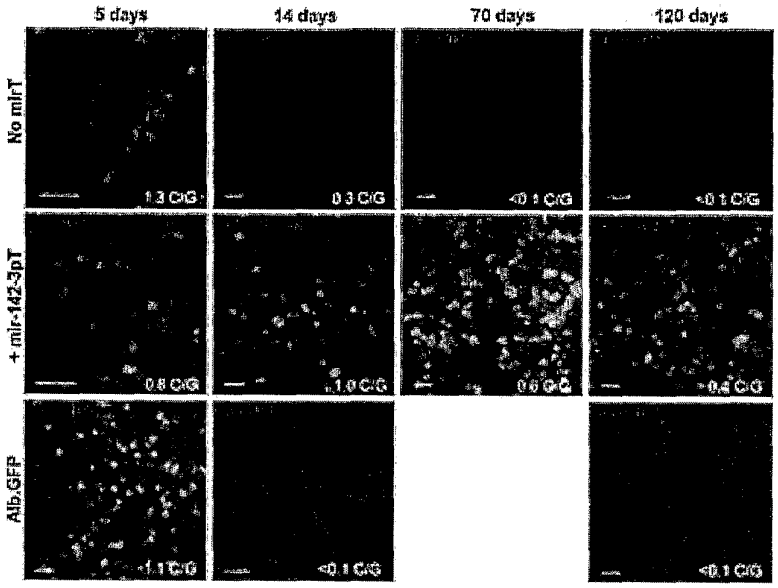
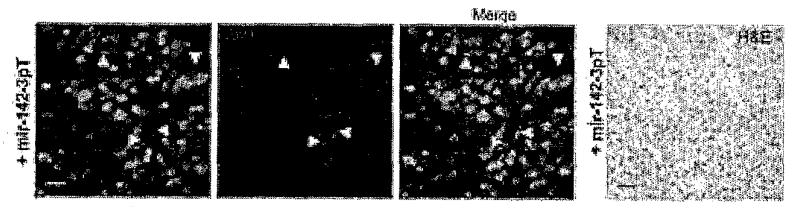

Liver
Lung
Gut
No mirT
+ mir-142-3pT
Spleen
Thymus
No mirT
+ mir-142-3pT

Figure 7.

a.


5 days
14 days
70 days
120 days
No mirT
+ mir-142-3pT
Alb.GFP
1.3 C/G
0.3 C/G
<0.1 C/G
<0.1 C/G
0.8 C/G
1.0 C/G
0.6 C/G
<0.1 C/G
<1.1 C/G
<0.1 C/G
<0.1 C/G

b.


Merge
H&E
+ mir-142-3pT
+ mir-142-3pT

d.

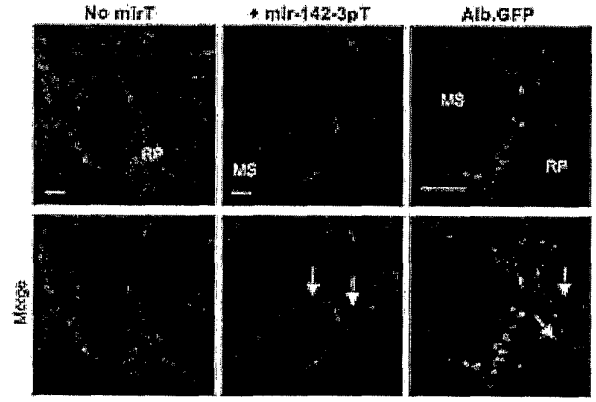

No mirT
+ mir-142-3pT
Alb.GFP
RP
MS
MS
RP
Merge

Figure 8.

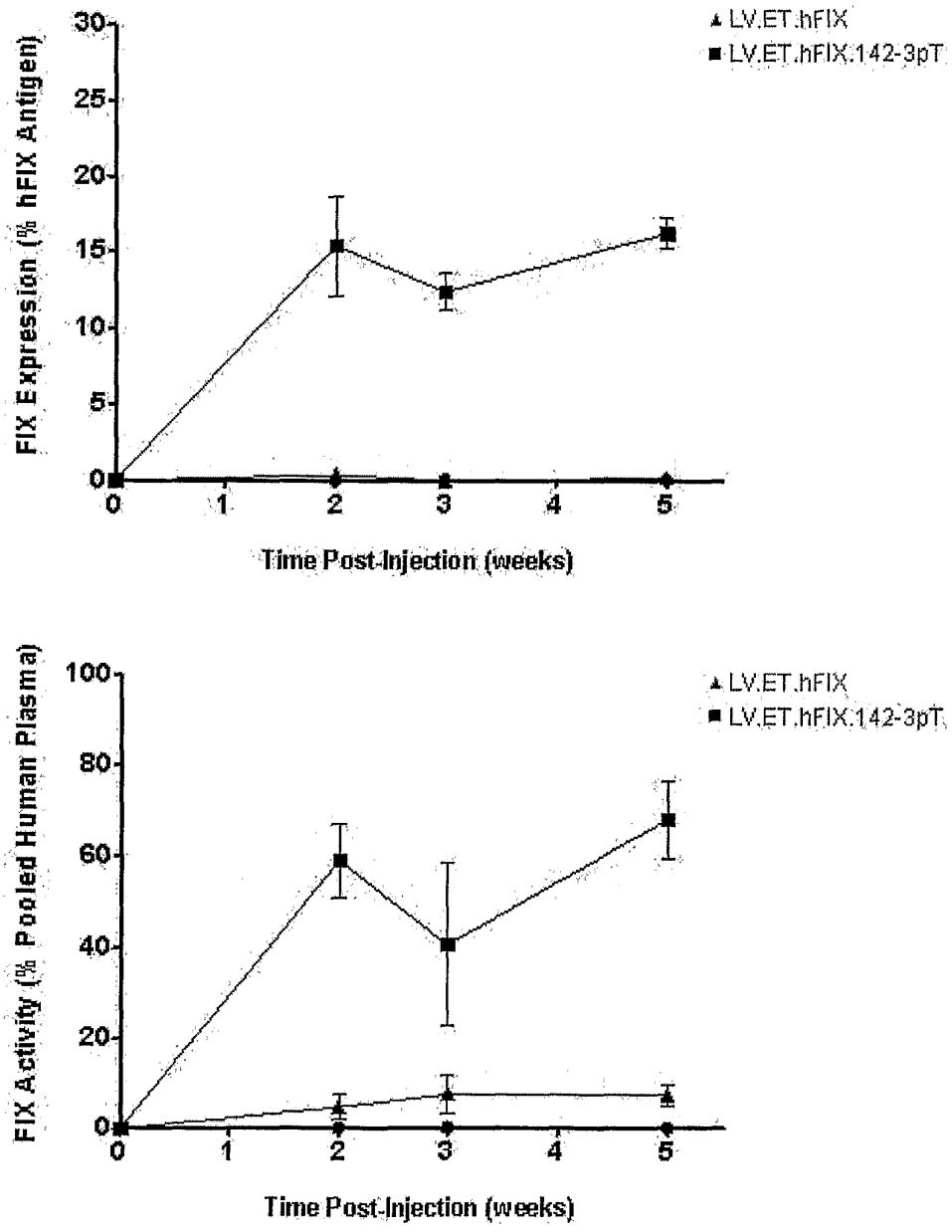
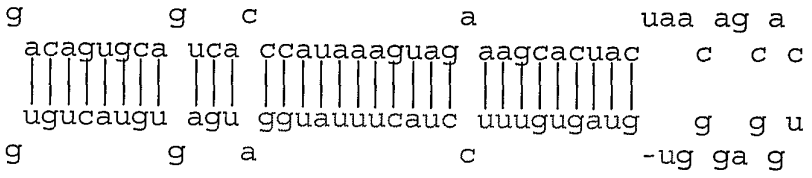

LV.ET.hFIX
LV.ET.hFIX.142-3pT
FIX Expression (% hFIX Antigen)
30
25
20
15
10
5
0
0
1
2
3
4
5
Time Post-Injection (weeks)
LV.ET.hFIX
LV.ET.hFIX.142-3pT
FIX Activity (% Pooled Human Plasma)
100
80
60
40
20
0
0
1
2
3
4
5
Time Post-Injection (weeks)

Figure 9

A. Mature hsa-mir-142 stem loop sequence.



B. 1x.mir-142as.Target
TCCATAAAGTAGGAAACACTACA