

1/22



FIG. 1A

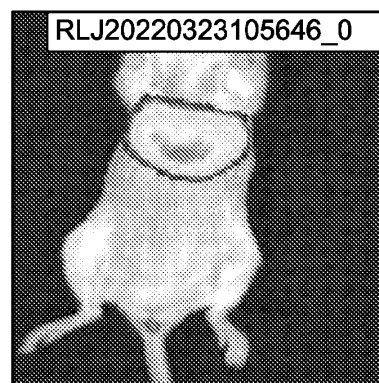
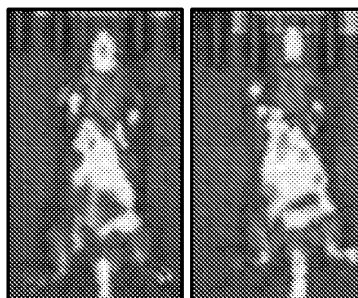


FIG. 1B

CMW:flUC



LSP:flUC

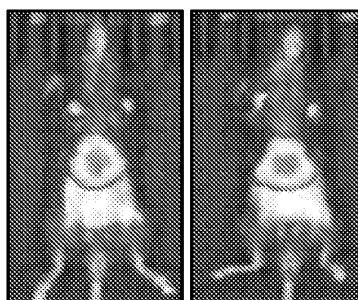


FIG. 1C

2/22

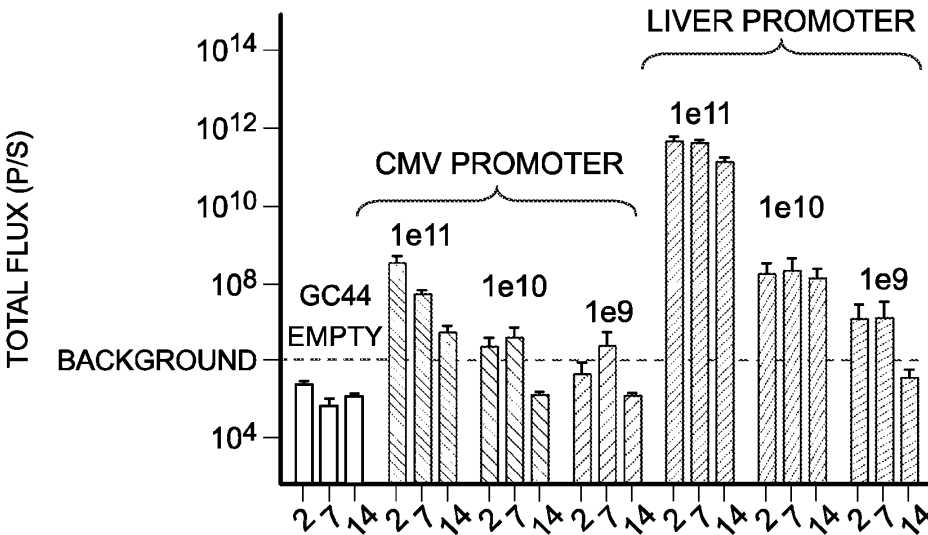


FIG. 2A

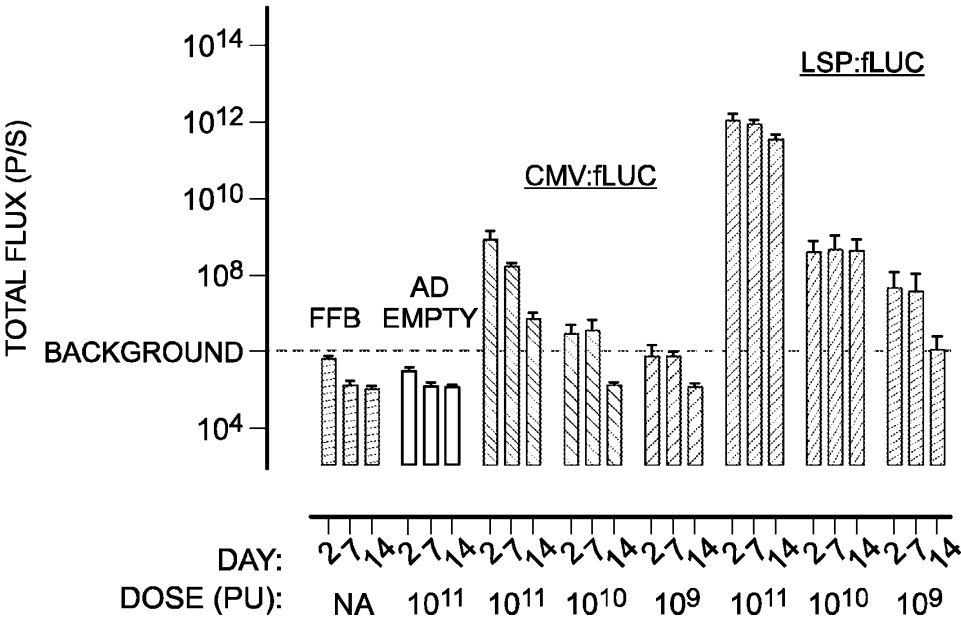


FIG. 2B

3/22

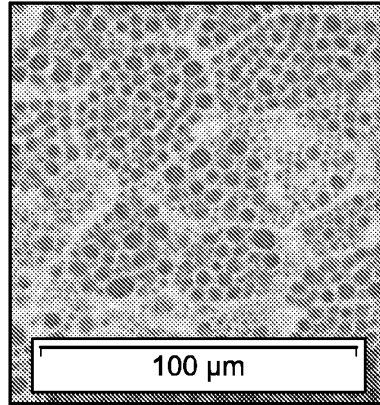


FIG. 3A

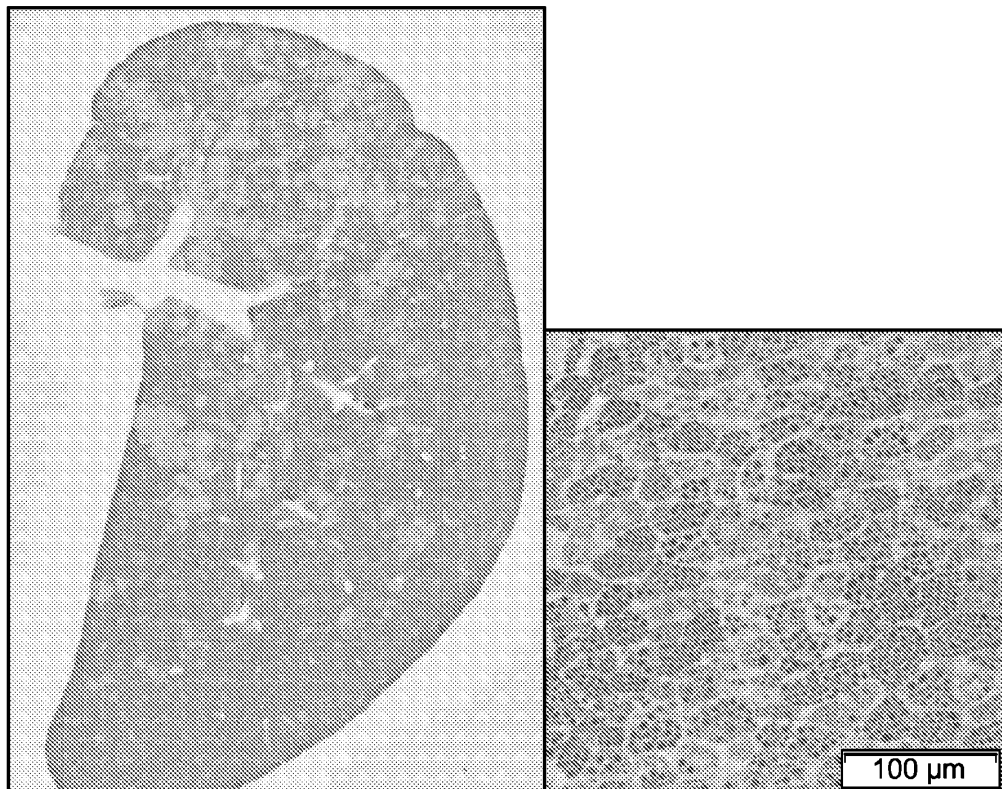


FIG. 3B

4/22

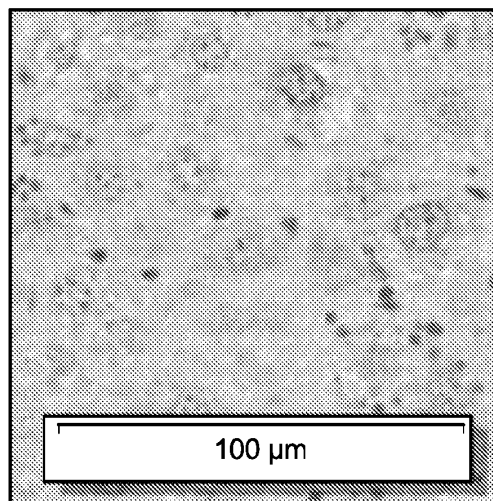


FIG. 4A

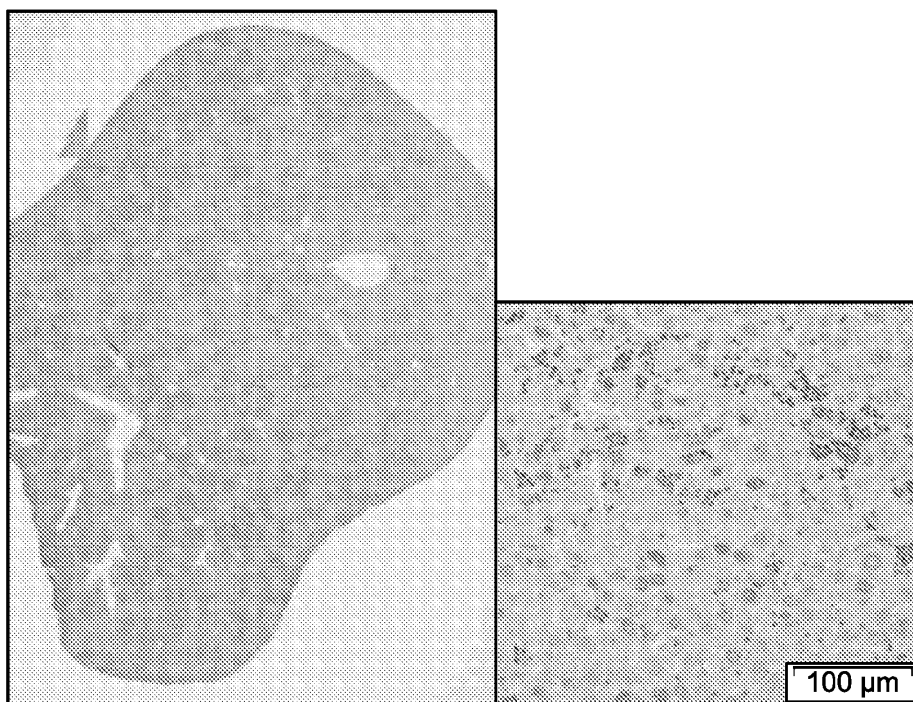
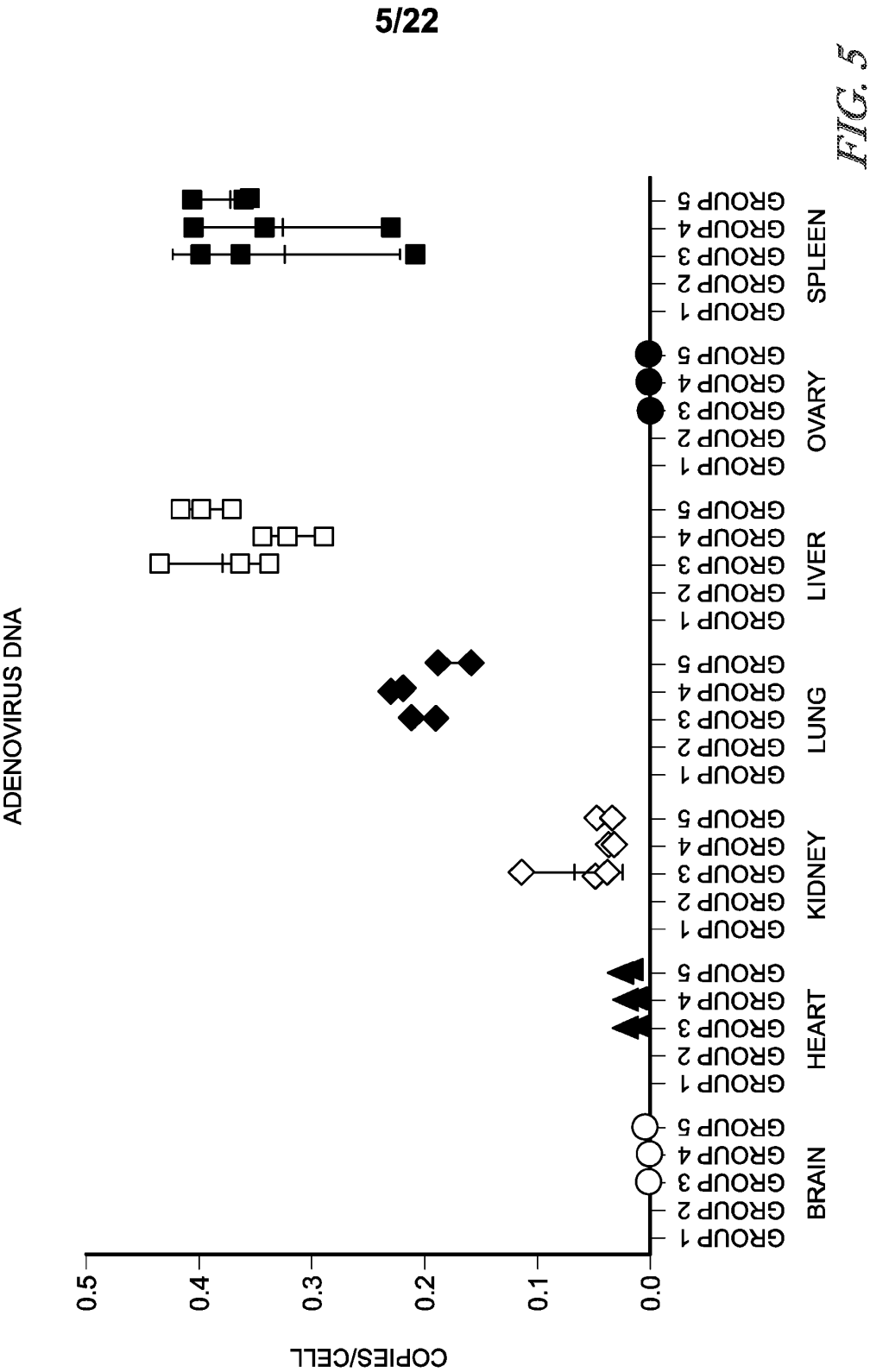


FIG. 4B



6/22

			AAT TRANSGENE TRANSCRIPT COPIES NORMALIZED BY ACTB						
STRAIN	GROUP	MOUSE ID	BRAIN	HEART	KIDNEY	LUNG	LIVER	OVARY	SPLEEN
NSG	1	31	X	X	X	X	X	X	X
		32	X	X	X	X	X	X	X
		33	X	X	X	X	X	X	X
NSG-PiZ	2	34	X	X	X	X	X	X	X
		35	X	X	X	X	X	X	X
		36	X	X	X	X	X	X	X
	3	37	X	X	278	17	421,070	3	X
		38	X	X	X	11	927,203	2	X
		39	X	X	X	8	342,214	X	30
	4	40	X	X	X	38	193,031	X	X
		41	X	65	X	7	385,215	2	X
		42	X	X	X	6	576,694	X	X
	5	43	X	X	44	8	1,133,911	3	X
		44	X	X	X	1,077	1,258,601	X	X
		45	X	146	X	6	1,130,359	X	X

FIG. 6

7/22

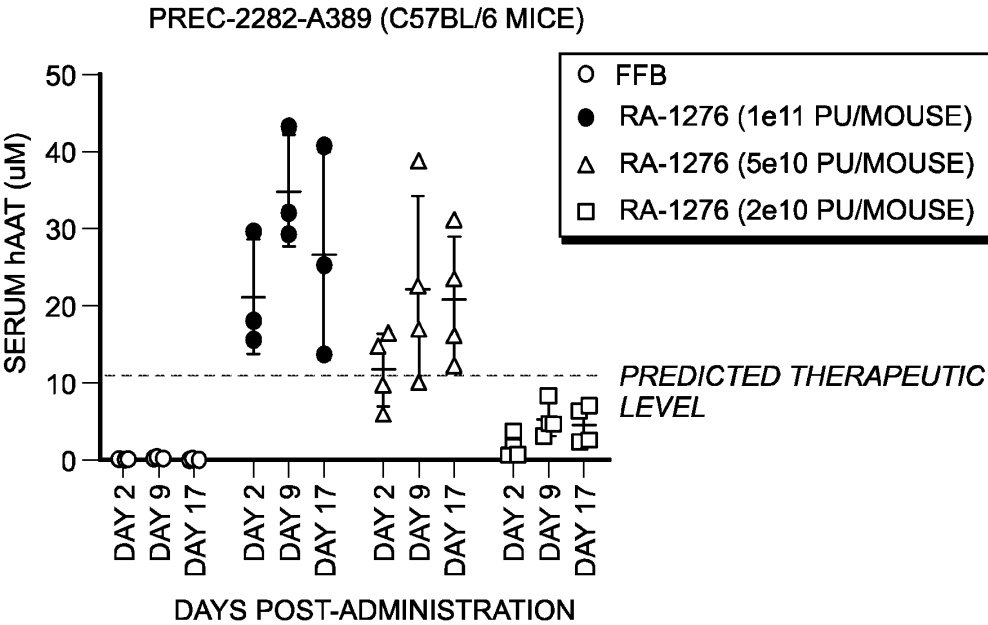


FIG. 7A

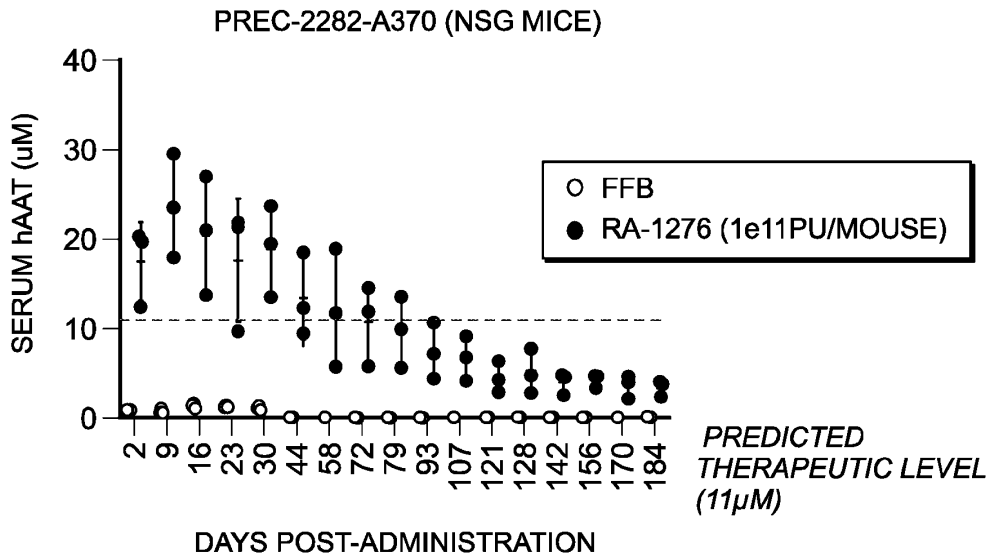


FIG. 7B

8/22

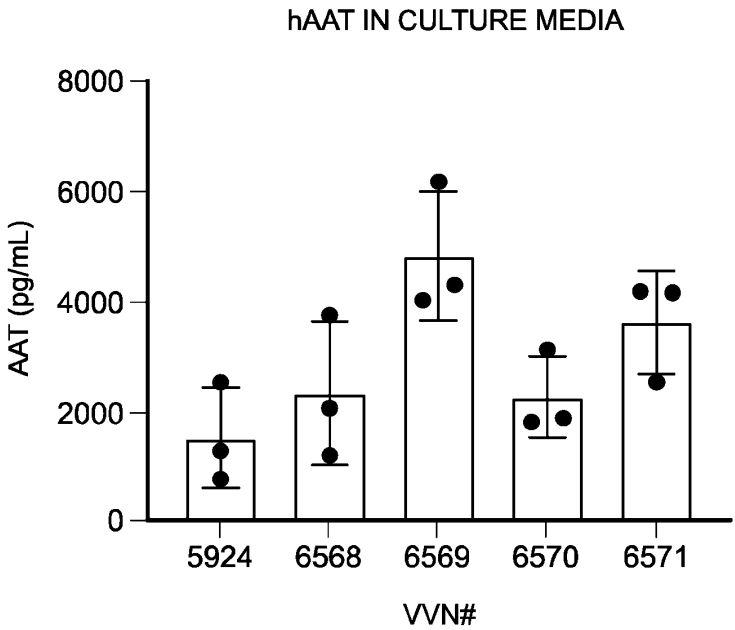


FIG. 8A

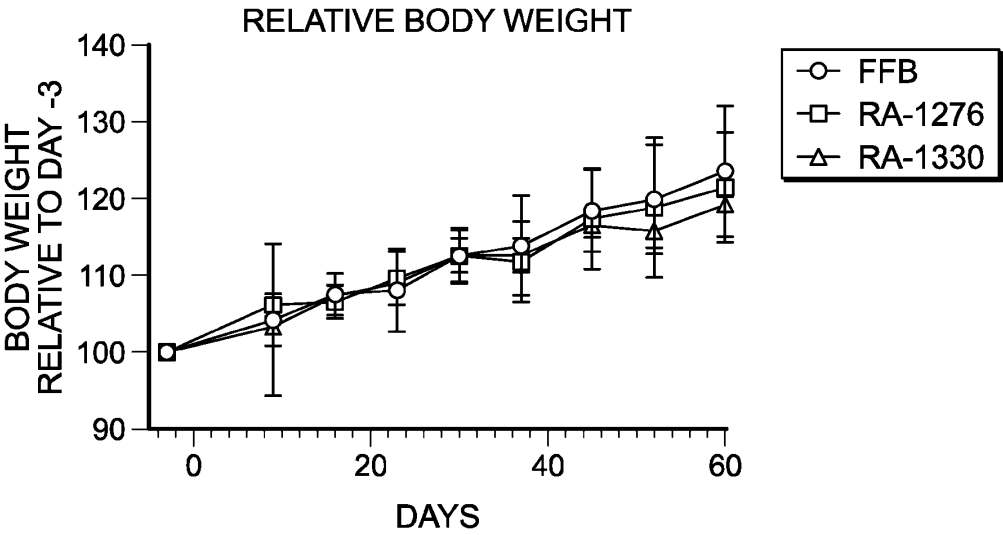


FIG. 8B

9/22

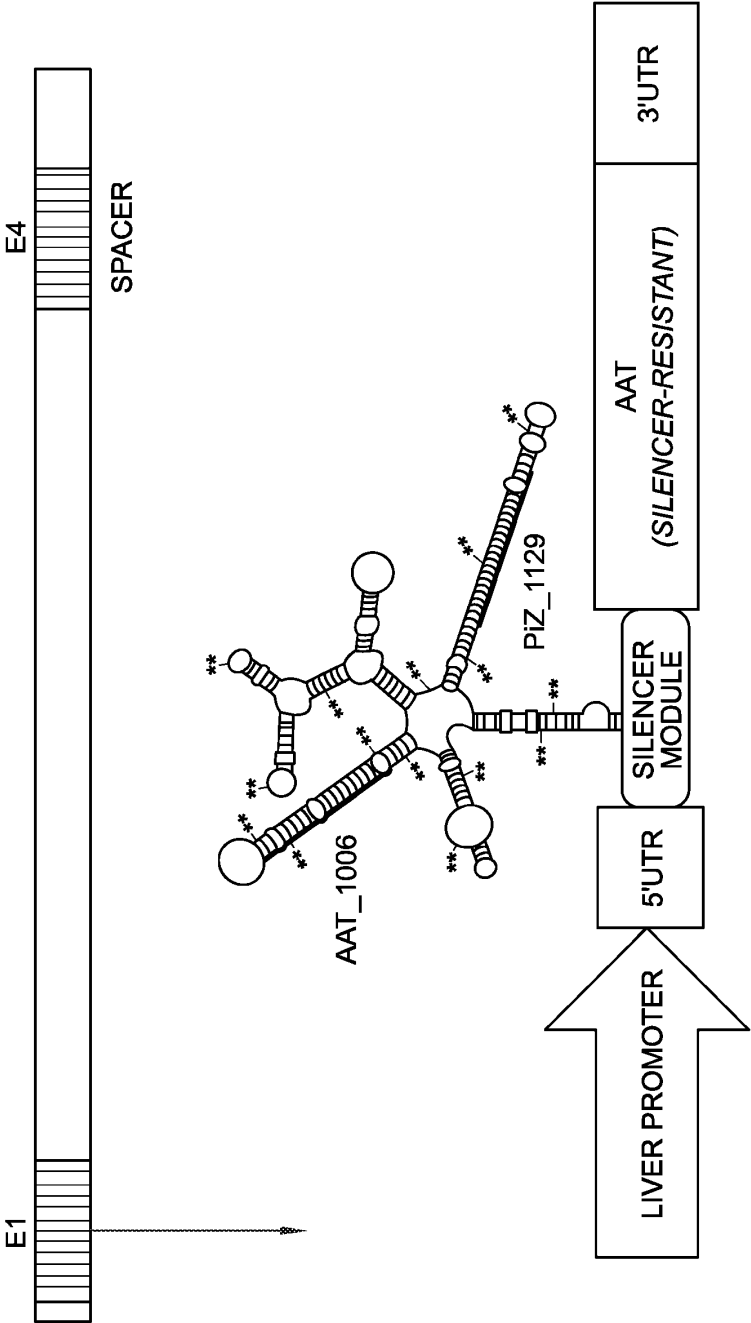


FIG. 8C

10/22

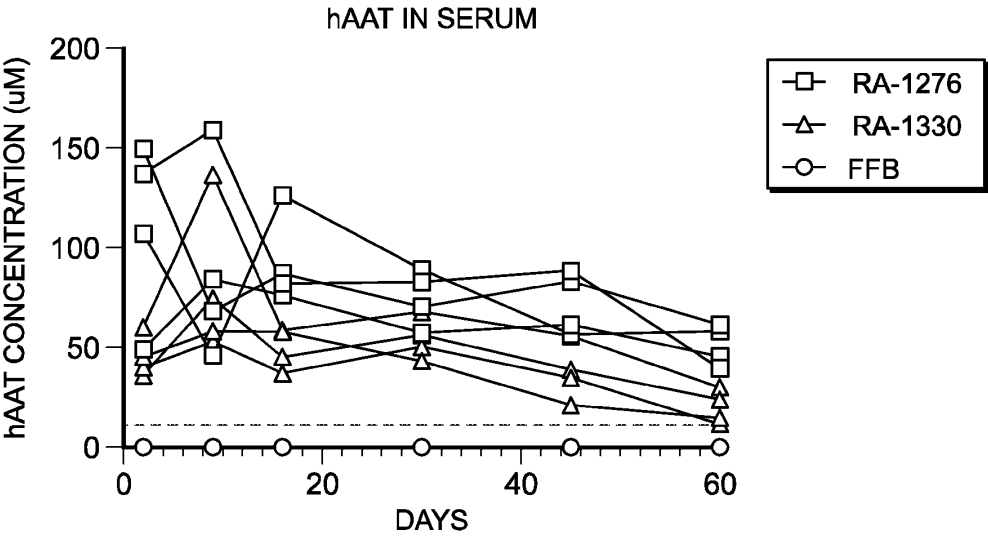


FIG. 9

11/22

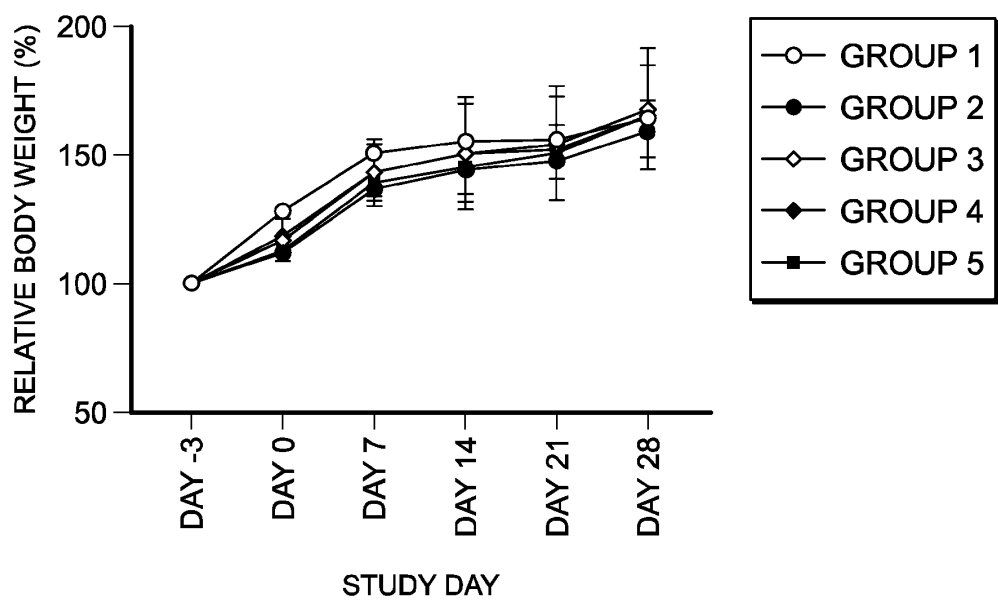


FIG. 10

12/22

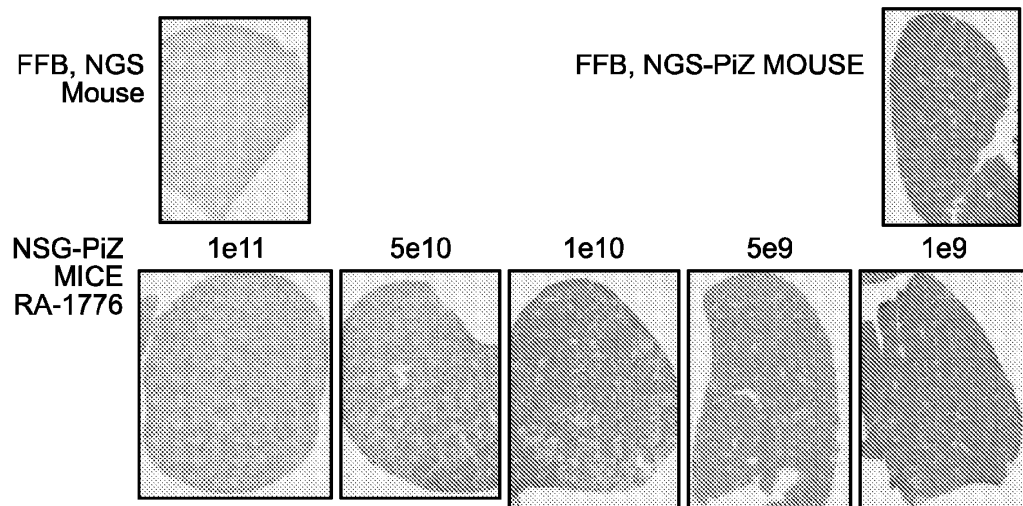
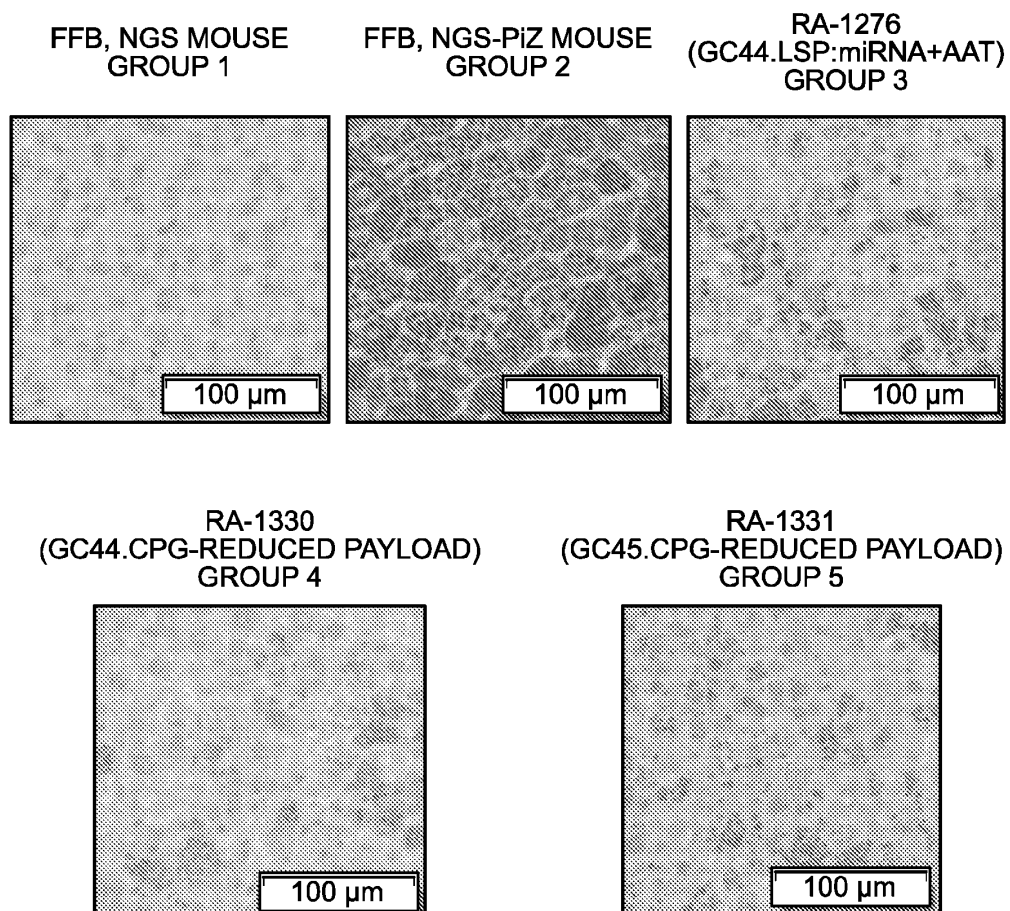


FIG. 11

13/22

*FIG. 12A*

14/22

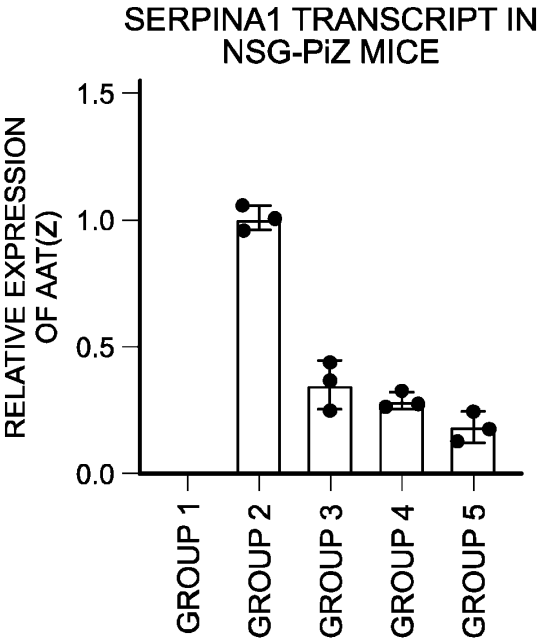


FIG. 12B

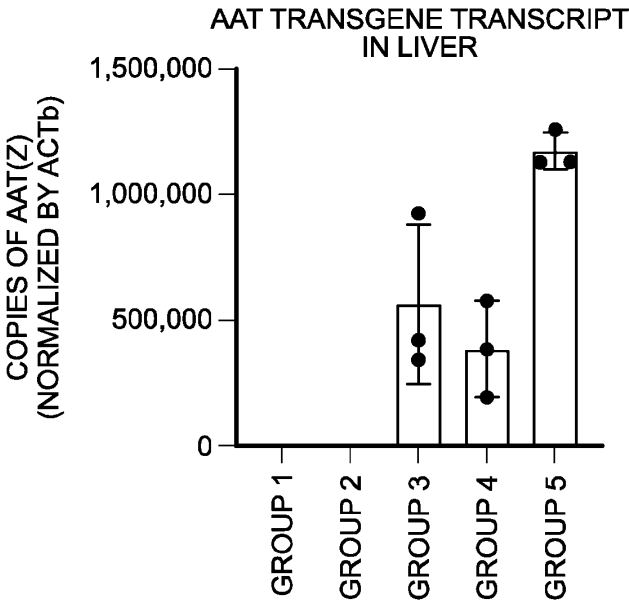


FIG. 12C

15/22

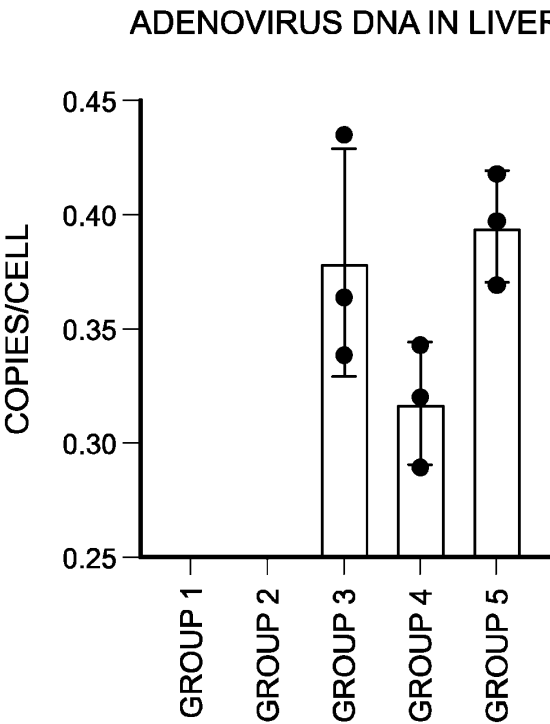


FIG. 12D

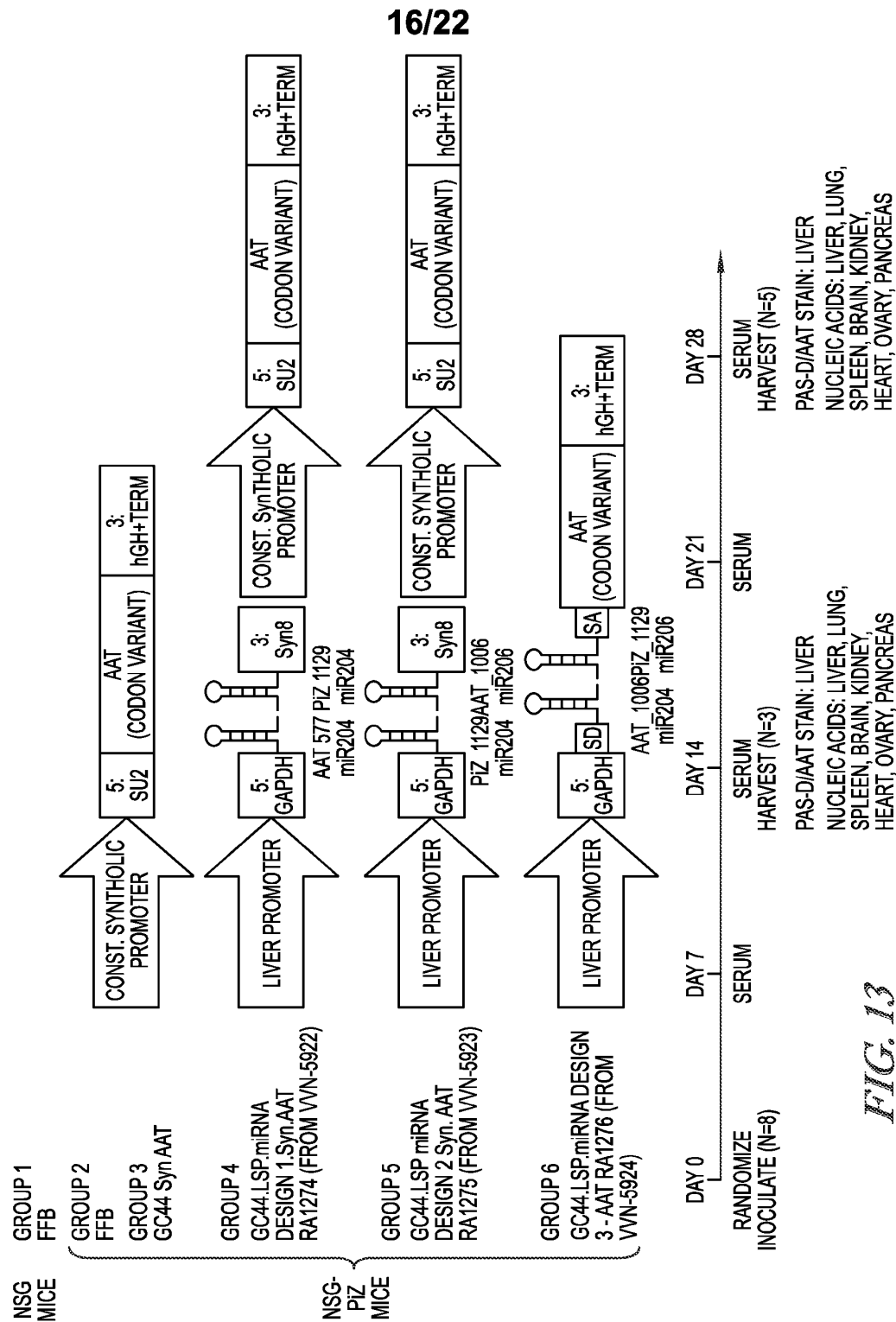


FIG. 13

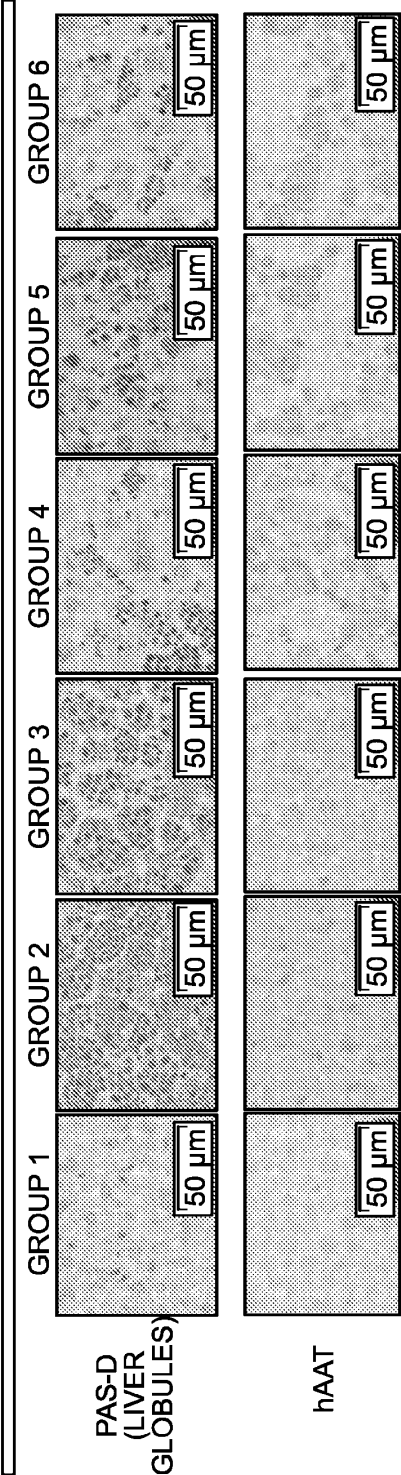


FIG. 14A

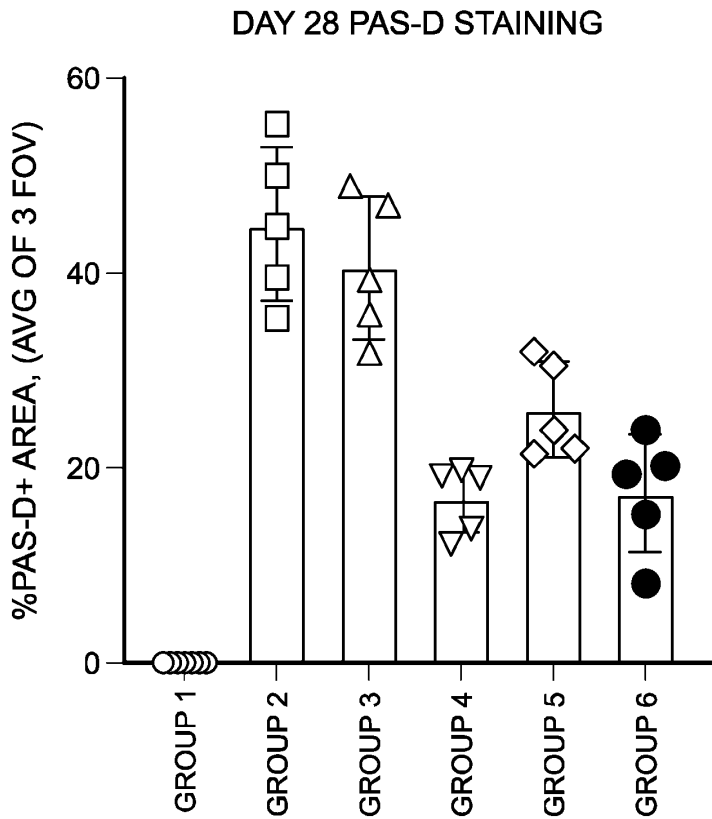


FIG. 14B

TISSUE	COLLECTION DAY	GROUP			
		SynP:AAT ₃	LiverP:miR#1 SynP:AAT ₄	LiverP:miR#2 SynP:AAT ₅	LiverP:miR#3 +AAT ₆
LIVER	14	596.1	476.9	544.5	876.8
	28	468.2	414.7	509.7	605.2
LUNG	14	92.8	77.5	84.0	114.6
	28	108.1	97.2	114.1	153.1
SPLEEN	14	96.5	112.5	102.6	149.3
	28	108.0	126.4	196.6	199.6
KIDNEY	14	19.3	18.1	21.6	18.5
	28	25.1	23.2	31.7	31.4
HEART	14	10.7	7.9	11.2	13.2
	28	11.5	9.6	13.1	13.5
PANCREAS	14	84.4	3.1	8.8	10.1
	28	7.8	3.8	4.2	11.8
OVARY	14	2.4	4.2	3.6	3.2
	28	5.1	5.5	8.9	4.9
BRAIN	14	1.0	1.1	1.6	1.4
	28	2.3	2.5	2.6	3.0

FIG. 15

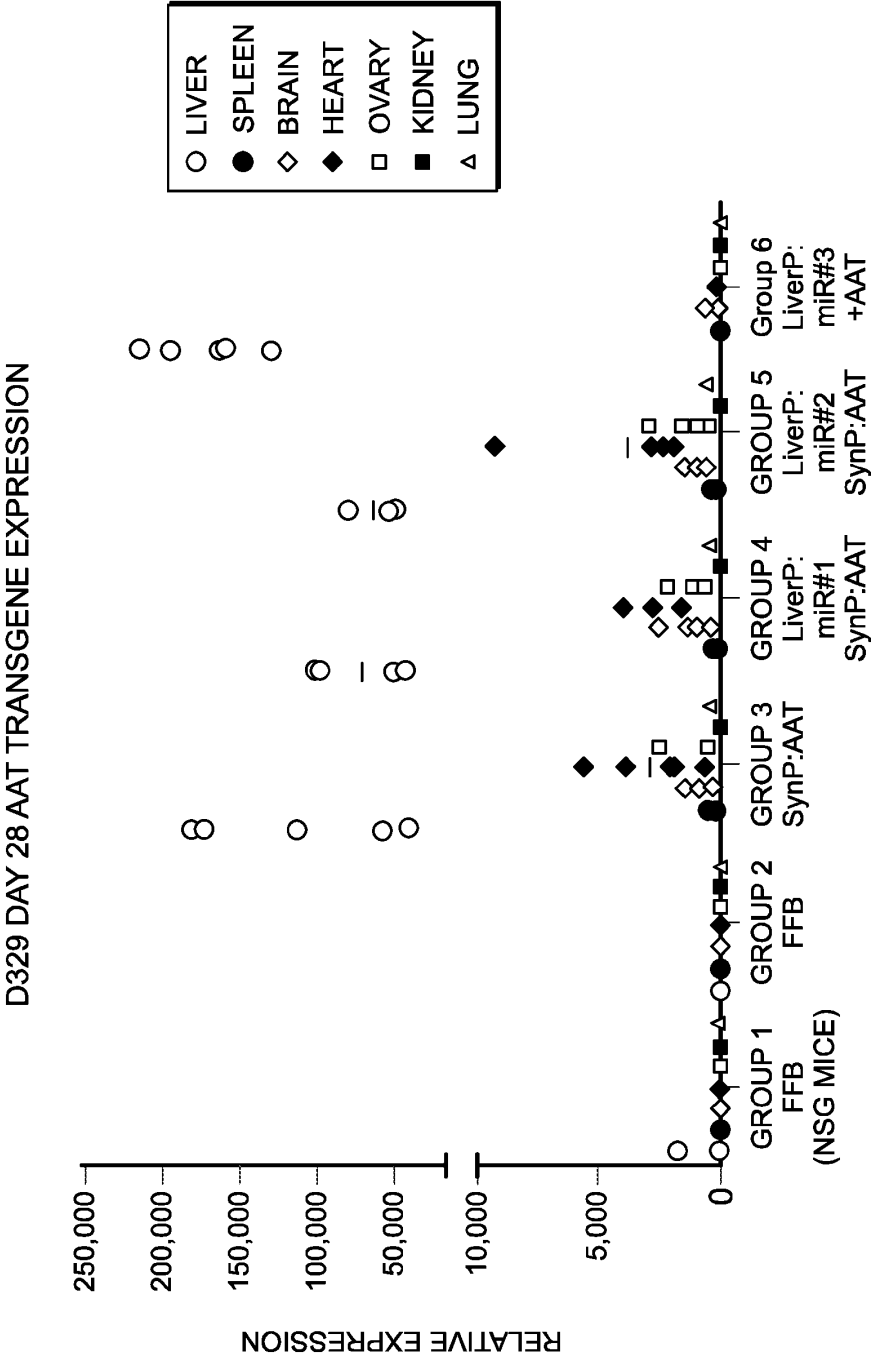


FIG. 16A

21/22

	TISSUE						
GROUP #	LIVER	BRAIN	HEART	OVARY	SPLEEN	KIDNEY	LUNG
GROUP 1	1.0	1.0	1.0	1.0	1.0	1.0	1.0
	66.9	0.8	7.2	2.8	0.8	1.2	0.5
	4.2	0.9	2.1	3.4	0.7	0.6	1.0
	18.2	0.5	2.9	0.4	1.2	1.0	0.7
	6.8	0.9	21.8	0.6	0.9	1.9	0.7
GROUP 2	3.5	1.0	2.4		6.9	0.6	0.8
	22.6	1.2	1.3	1.5	5.1	1.1	0.7
	8.1	0.7	28.7	1.5	1.6	0.8	0.7
	2.8	0.7	2.0	1.0	0.9	1.6	3.8
	5.5	0.6	20.0	0.9	6.3	0.6	1.4
GROUP 3	112250.5	3400.3	3794.7	547.5	384.8	30.1	263.4
	179053.5	893.4	5454.0	596.0	298.1	34.9	271.6
	39879.1	244.9	474.1	2533.4	97.9	8.2	220.8
	56188.8	531.0	1908.6	644.8	141.7	13.9	223.1
	172775.1	1415.2	2116.3	575.4	157.9	11.9	210.7
GROUP 4	46767.8	815.1	2740.2	1113.3	142.1	16.9	354.5
	98510.0	2437.1	4076.9	573.1	179.5	22.5	379.6
	99070.4	1449.3	3898.2	694.7	129.8	17.5	229.7
	49285.8	975.4	1554.0	873.6	287.2	9.0	184.3
	42396.7	401.9	1448.8	2051.0	160.8	12.3	305.1
GROUP 5	79493.6	552.9	2211.2	1443.1	232.6	34.7	521.7
	46638.0	3468.3	9271.9	1801.7	335.7	31.5	
	52851.0	1134.9	2821.2	2910.2	370.5	31.0	264.7
	79426.4	1157.3	2818.9	782.5	243.0	29.2	418.2
	51501.6	1185.0	1876.7	391.5	116.2	29.6	672.1
GROUP 6	142186.8	137.7	22.8	0.5	5.8	1.7	2.3
	188549.2	698.5	92.3	8.3	1.2	1.1	2.7
	159193.7	115.8		25.3	1.6	1.4	30.7
	213047.5	241.2	65.4	8.3	1.5	1.3	2.2
	174984.8	648.7	176.6	1.6	3.9	1.4	3.7

FIG. 16B

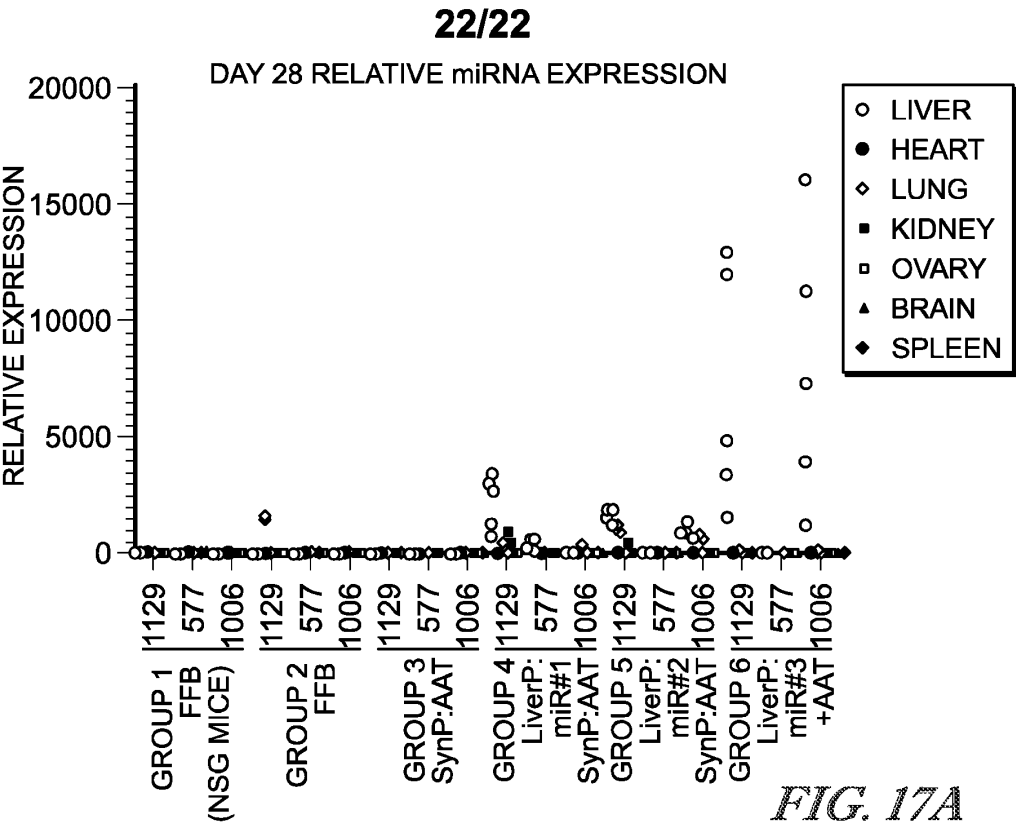


FIG. 17A

AVERAGE EXPRESSION

MIRNA	GROUP #	TISSUE						
		LIVER	HEART	LUNG	KIDNEY	OVARY	BRAIN	SPLEEN
PiZ_1129	1	3	1	0	2	0	0	0
	2	1	7	591	1	15	21	21
	3	1	1	0	1	5	52	52
	4	2,198	6	121	413	1	5	5
	5	1,644	2	429	207	1	3	3
	6	6,972	2	135	131	1	1	1
AAT_577	1	2	1	0	1	0	1	0
	2	1	1	36	1	51	4	9
	3	1	1	1	1	5	1	15
	4	375	0	0	26	0	0	0
	5	1	1	1	1	0	1	0
	6	1	1	0	1	0	0	0
AAT_1006	1	2	1	1	2	2	1	1
	2	1	1	1	1	6	1	1
	3	1	2	1	1	2	2	44
	4	1	6	83	1	2	4	55
	5	948	7	254	17	2	4	34
	6	7,979	5	93	6	4	4	14

FIG. 17B