

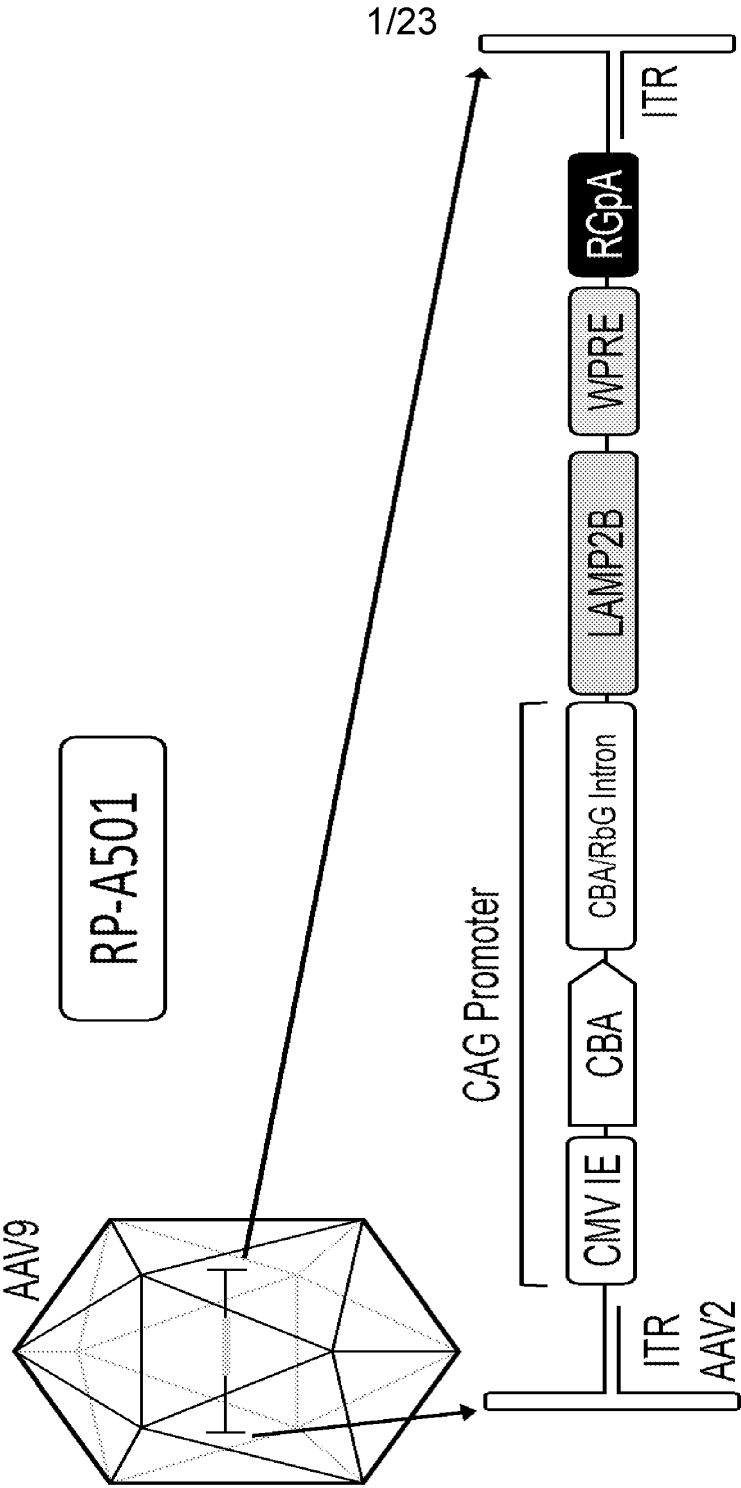


FIG. 1

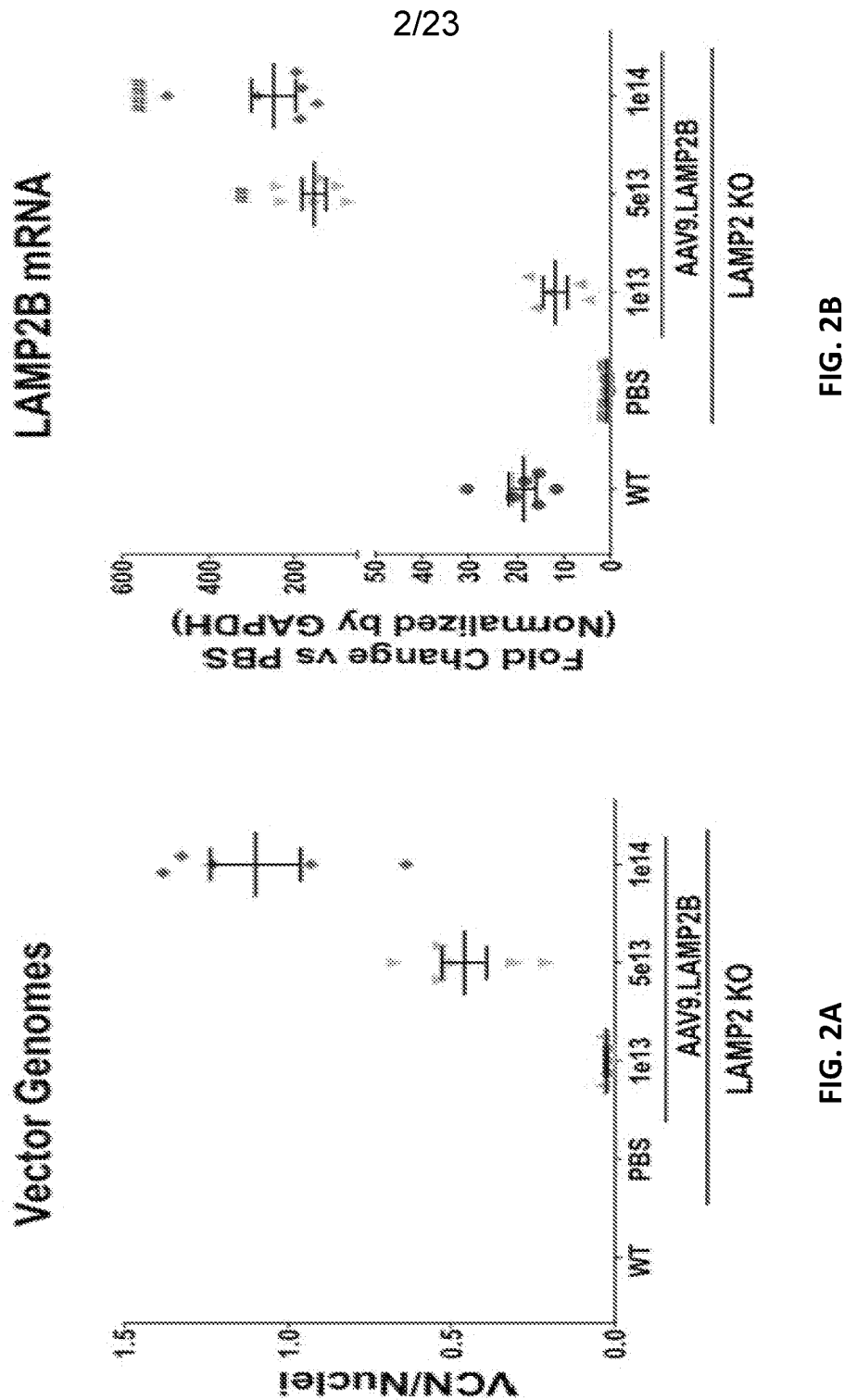


FIG. 2B

FIG. 2A

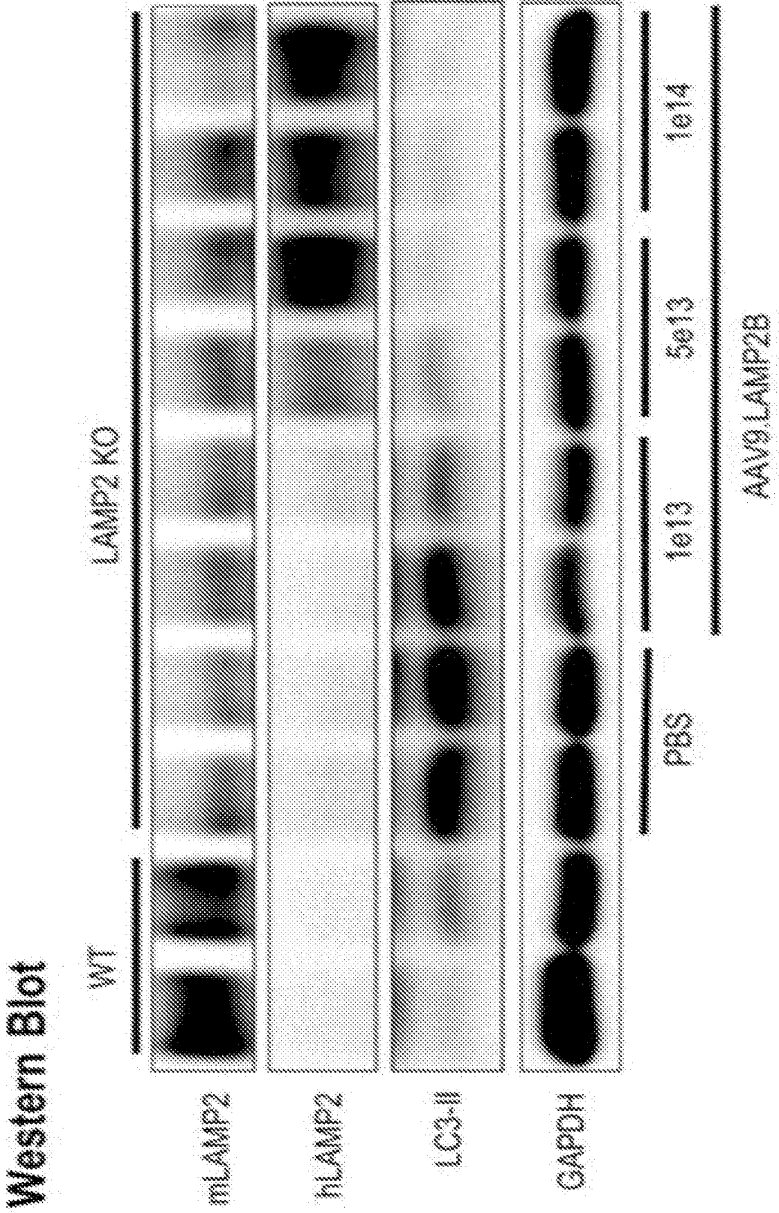


FIG. 3A

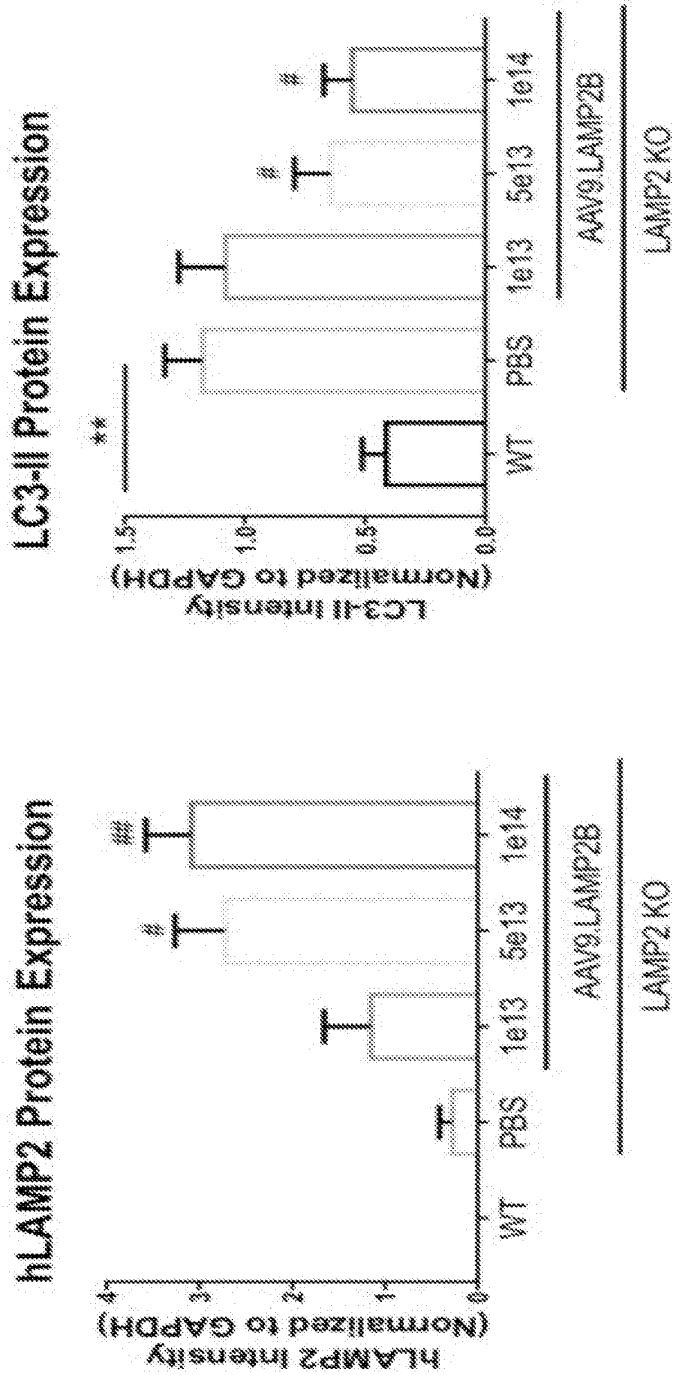


FIG. 3B

FIG. 3C

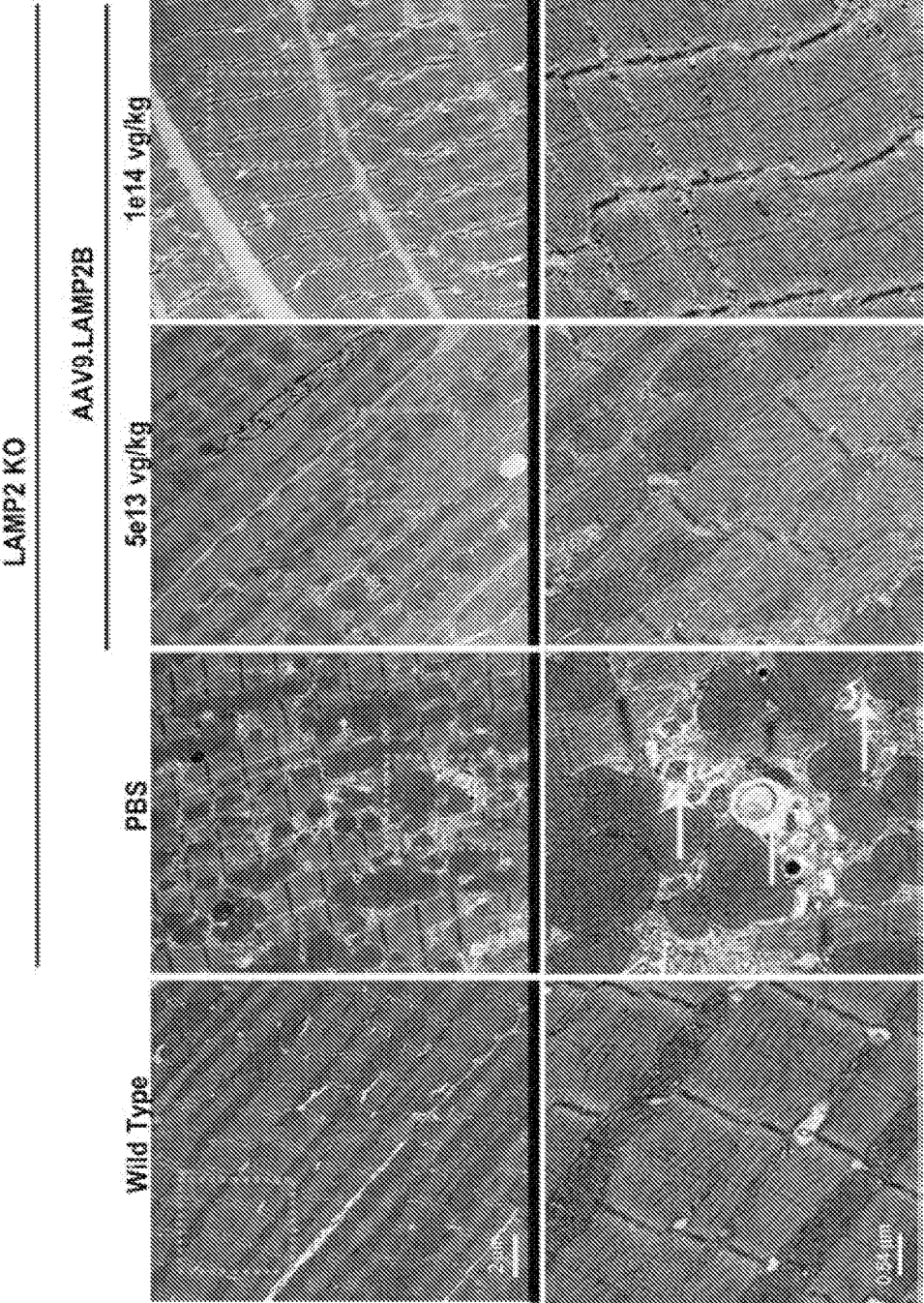
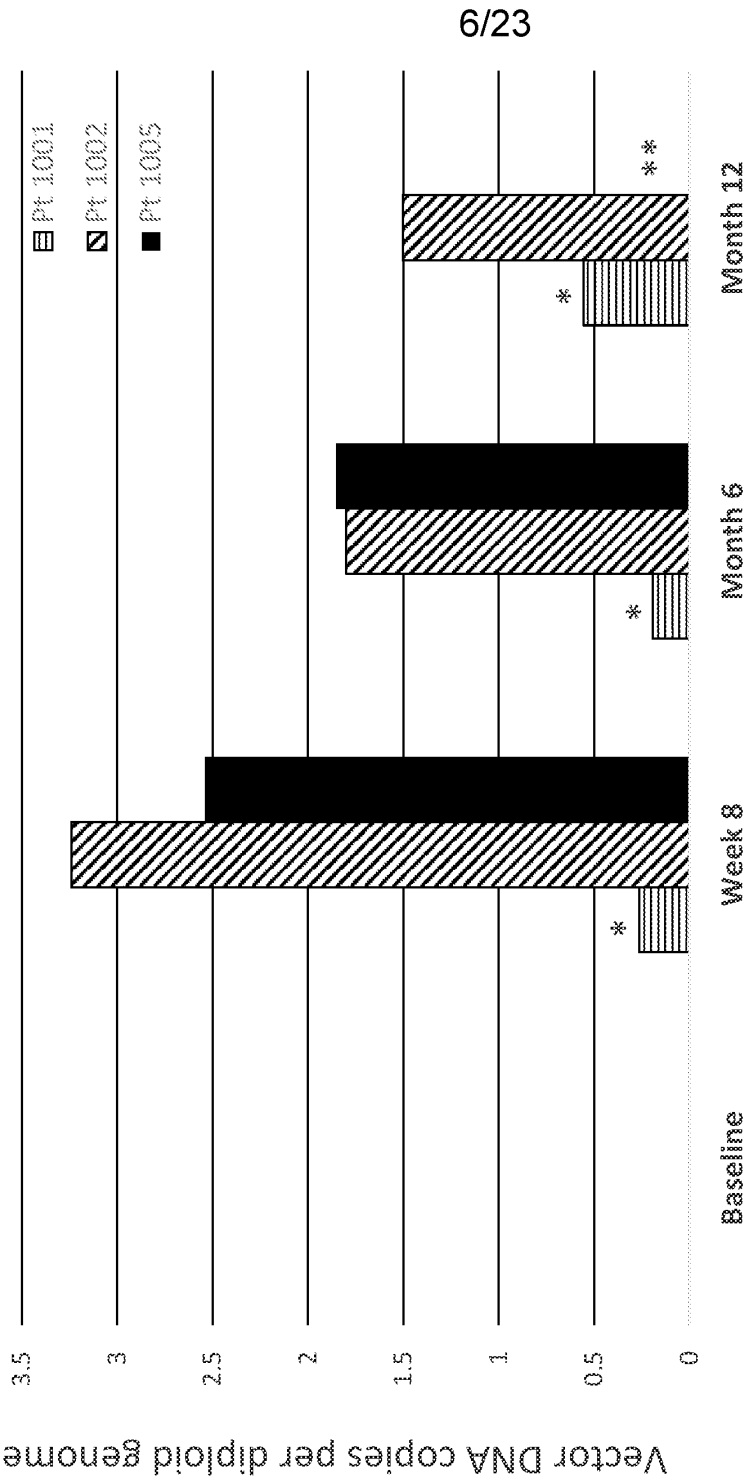


FIG. 4



* Limited compliance with steroid regimen
** January 2021

FIG. 5

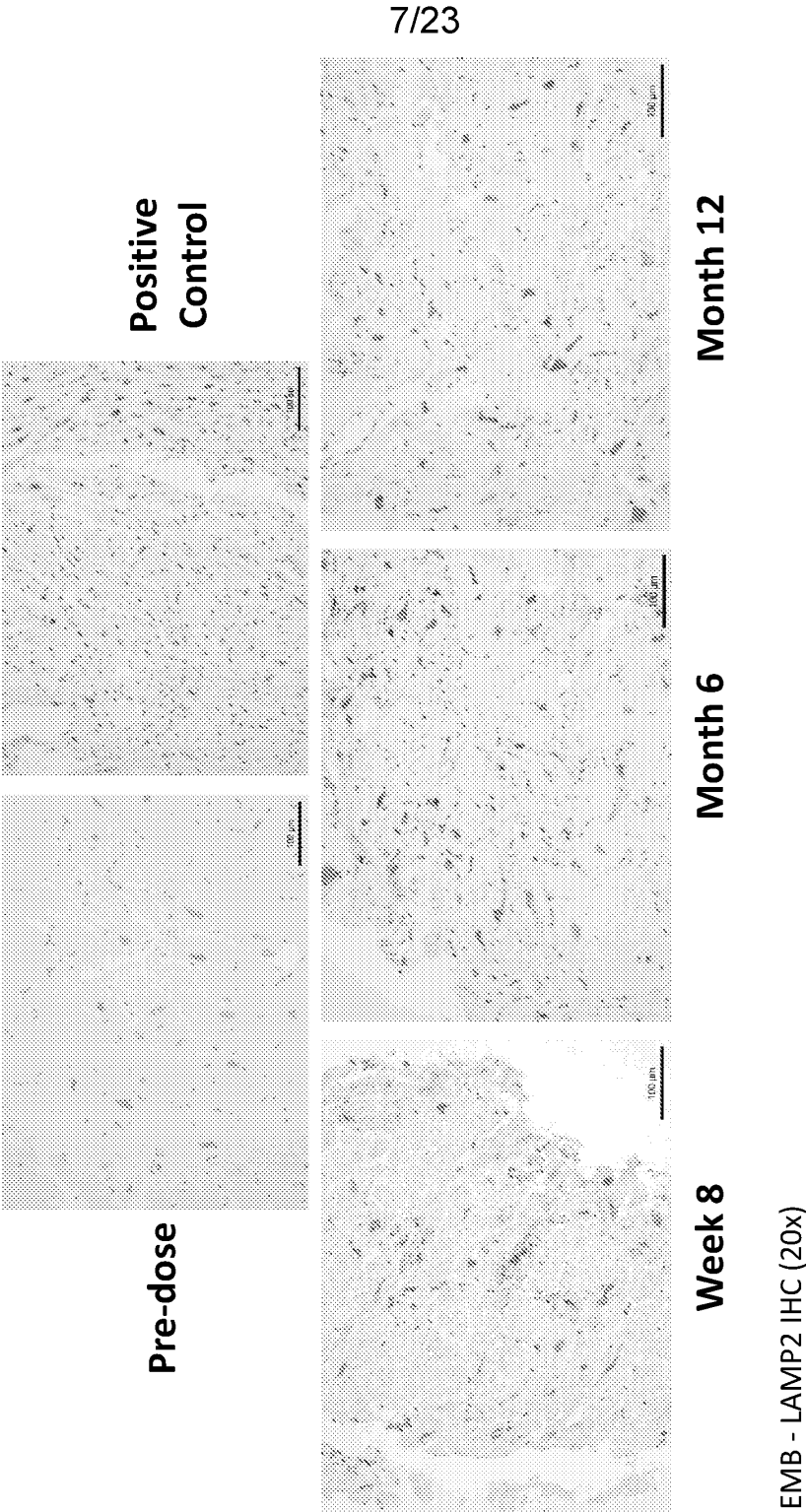


FIG. 6

8/23

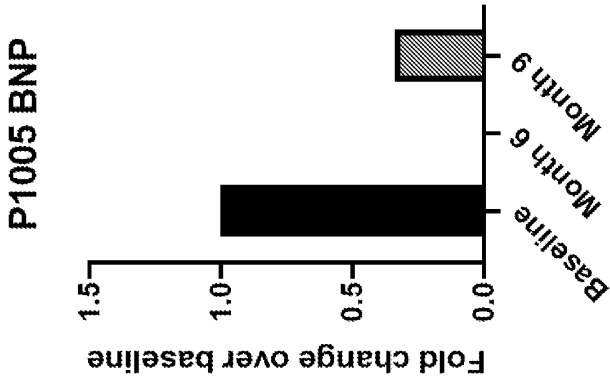


FIG. 7C

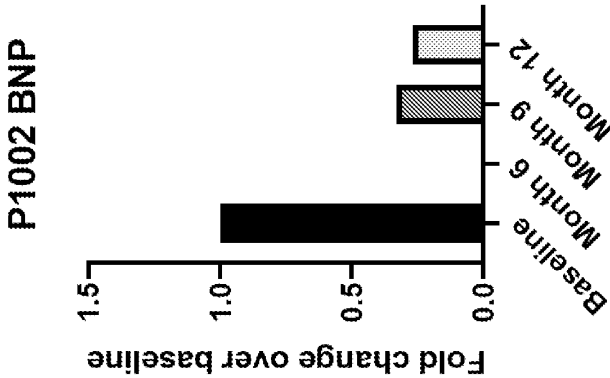


FIG. 7B

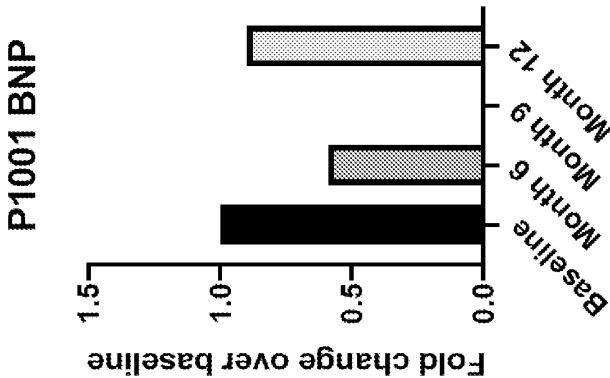
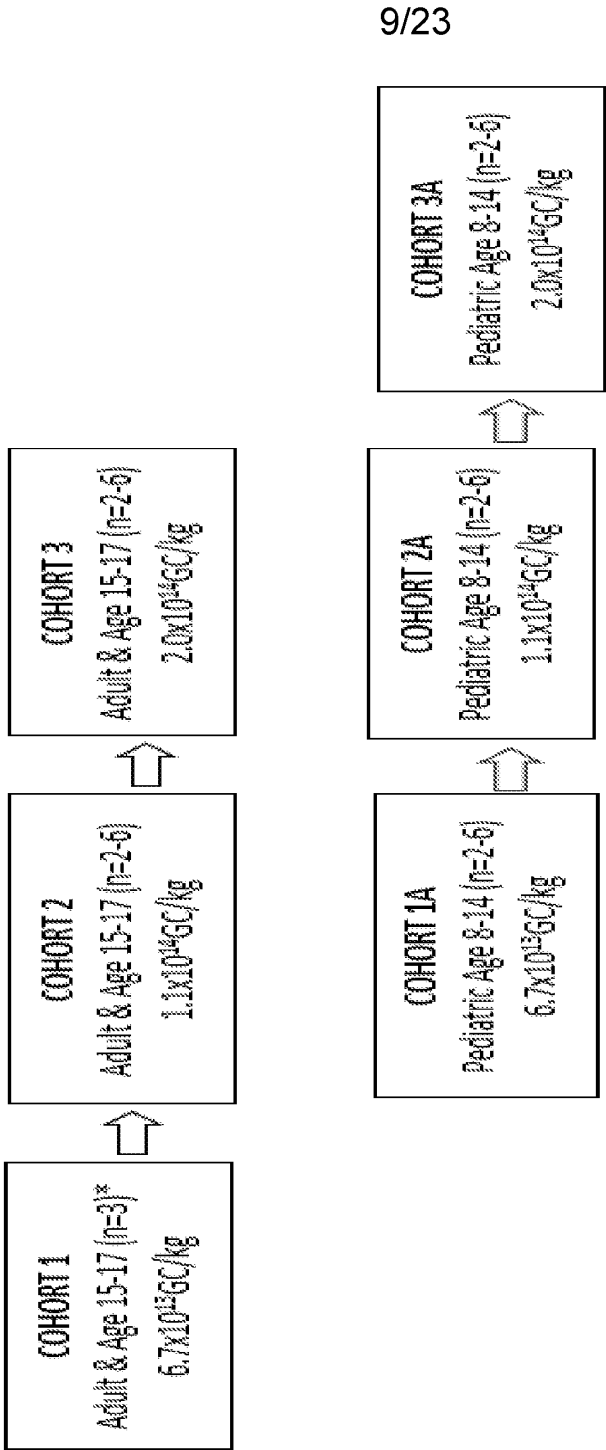


FIG. 7A



* Cohort 1 enrolled 3 patients per previous protocol version

FIG. 8

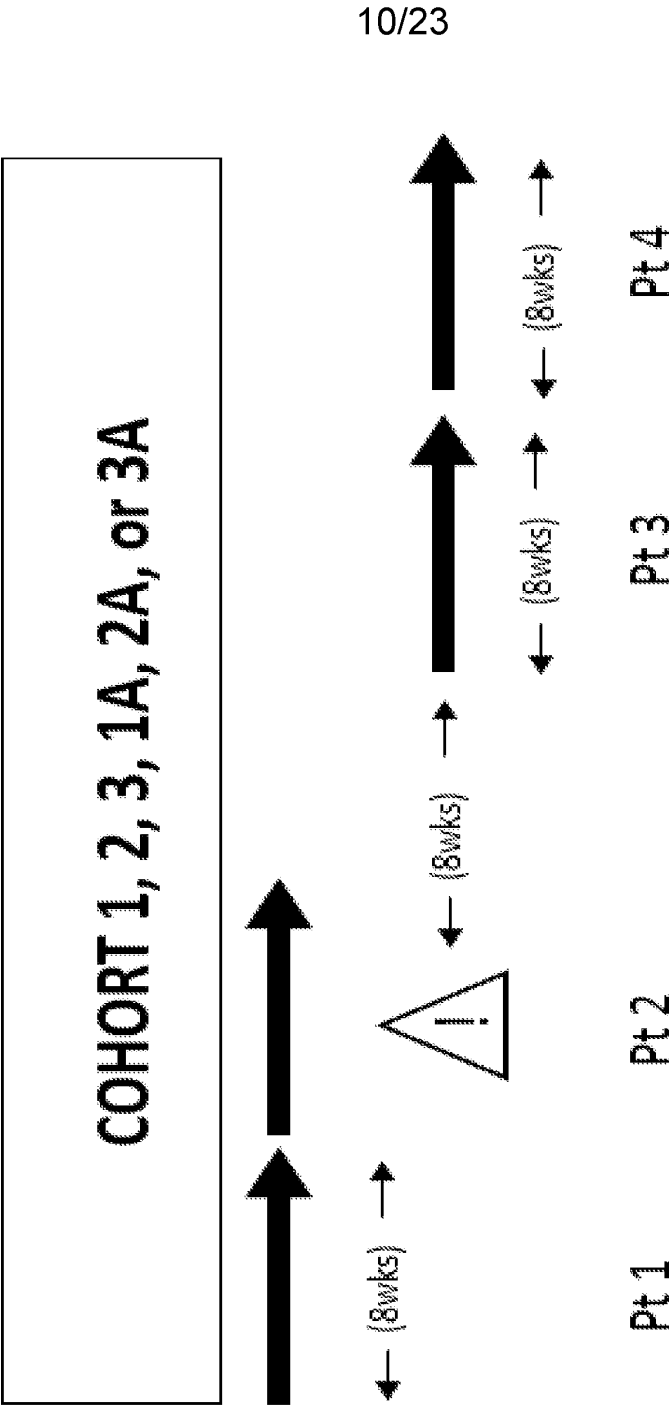


FIG. 9

Pre-IP Treatment Period				Post-IP Treatment Period														
	Baseline (D-14 to D-7)	D-14 & D-7	D-1	D0	D1 to D7*	W2	W3	W4	W6	W8	M3	M6	M9	M12	M18	M24	M30	M36
Visit windows					*D7 ±1D	±3D	±3D	±5D	±5D	±5D	±2W	±3W	±4W	±4W	±5W	±8W	±8W	±8W
General Assessments:																		
Medical history ¹	X																	
Concomitant medications & procedures ²	X	X	X	X	X	X	X	X	X	X	X	X	X	X	X	X	X	X
Adverse event assessment	X	X	X	X	X	X	X	X	X	X	X	X	X	X	X	X	X	X
Informed consent & Demographics	X																	
Inclusion & Exclusion criteria	X		X															
Vital signs	X	X	X	X	X	X	X	X	X	X	X	X	X	X	X	X	X	X
Height & weight	X ³		X		X	X	X	X	X	X	X	X	X	X ³	X	X ³	X	X
Physical examination	X	X	X	X	X	X	X	X	X	X	X	X	X	X	X	X	X	X
QOL/PRO questionnaires ⁴		X									X	X	X	X	X	X	X	X
Blood-based Assessments:																		
CBC with differential	X		X		X ⁵	X ⁵	X ⁵	X ⁵	X ⁵	X ⁵	X	X	X	X	X	X	X	X
Serum chemistry including electrolytes, LFT	X		X		X ⁵	X ⁵	X ⁵	X ⁵	X ⁵	X ⁵	X ⁵	X ⁵	X	X	X	X	X	X
Visit windows					*D7 ±1D	±3D	±3D	±5D	±5D	±5D	±2W	±3W	±4W	±4W	±5W	±8W	±8W	±8W

FIG. 10A

	Pre-IP Treatment Period				Post-IP Treatment Period													
	Baseline	D-14 & D-7	D-1	D0	D1 to D7*	W2	W3	W4	W6	W8	M2	M6	M9	M12	M18	M24	M30	M36
IgG, IgM antibody	X		X		D2, D4, D7			X		X	X	X						
Coagulation: PT, aPTT/ Fibrinogen/D-dimer/TAT complex	X		X		D2, D4, D7	X	X	X	X	X	X	X	X			X		X
Urinalysis	X				D7			X		X				X				X
Complement: C3 & C4	X		X		D1, D3, D5, D7	X*	X*	X*	X*	X*	X	X	X	X	X	X	X	X
Complement: sC5b-9 [†]	X		X		X*	X*	X	X	X	X	X	X						
Viral serology (HBV, HCV, HIV) ^{†‡}	X																	
Cardiac serology: Troponin-I [†]	X		X		D2, D4, D7	X	X	X	X	X	X	X	X	X	X	X	X	X
Cardiac serology: BNP [†]	X		X			X		X	X	X	X	X	X	X	X	X	X	X
Cardiac serology: CK-MB ^{†‡}	X		X		D7	X	X	X	X	X	X	X	X	X	X	X	X	X
Neutralizing Anti-AAV9 antibody titer (serum)	X		X			X		X		X	X	X		X		X		X
Immunogenicity: ADA (serum) ^{§,}	X				D7	X		X		X	X	X		X		X		X
Immunogenicity: ELISpot (PBMC) ^{,¶}	X							X		X	X	X		X		X		X
Visit windows					*D7 ±1D ±3D	±3D	±3D	±3D	±3D	±5D	±2W	±3W	±4W	±4W	±3W	±3W	±3W	±3W

FIG. 10B

	Pre-IP Treatment Period		Post-IP Treatment Period															
	Screening Visit	D-14 & D-7	D-1	D0	D1 to D7*	W2	W3	W4	W6	W8	M3	M6	M9	M12	M18	M24	M30	M36
Exploratory assays (serum, plasma & blood cells) ¹⁰	X							X		X		X		X		X		X
Plasma, saliva, urine & feces for vector shedding ¹⁴	X				D5	X		X		X	X	X	X					
Gene sequencing ¹⁵	X																	
Cardiac & Disease-Focused Evaluations:																		
ECG	X		X		D1	X	X	X	X	X	X	X	X	X	X	X	X	X
Echocardiogram	X						X	X	X	X	X	X	X	X	X	X	X	X
NYHA evaluation	X	X	X	X	X	X	X	X	X	X	X	X	X	X	X	X	X	X
Cardiac MRI with gadolinium ¹⁶	X									X		X		X	X	X	X	X
Hepatic Ultrasound	X																	
6-Minute Walk Test ¹⁷	X									X	X	X		X	X	X	X	X
Cardiopulmonary Exercise Test with VO ₂	X									X ¹⁸	X ¹⁹	X ²⁰		X ²¹	X ²²	X ²³	X ²⁴	X ²⁵
Pulmonary Function Tests		X												X		X		X
Right Heart Catheterization & Endomyocardial biopsy		X								X		X		X				X
Skeletal muscle biopsy		X								X		X		X				X
Neurocognitive evaluation		X												X		X		X
Visit windows					*D7 ±1D	±3D	±3D	±3D	±3D	±3D	±2W	±3W	±4W	±4W	±8W	±8W	±8W	±8W
Neuromuscular evaluation	X									X		X		X		X		X

FIG. 10C

	Pre-IP Treatment Period			Post-IP Treatment Period														
	Screening Baseline	D-14 & D-7	D-1	D0	D1 to D7*	W2	W3	W4	W6	W8	M3	M6	M9	M12	M18	M24	M30	M36
Ophthalmologic evaluation		X												X		X		X
Investigational Product & Concomitant Medications:																		
Rituximab			X															
Rituximab pre-medications**			X															
Investigational product administration (IV)				X														
Corticosteroid prophylaxis**				X ¹⁰ (Taper begin on W8 through M3; sooner when feasible)										d/c				
Tacrolimus			D-7 only	X	X	X	X	X	X	X	X							

FIG. 10D

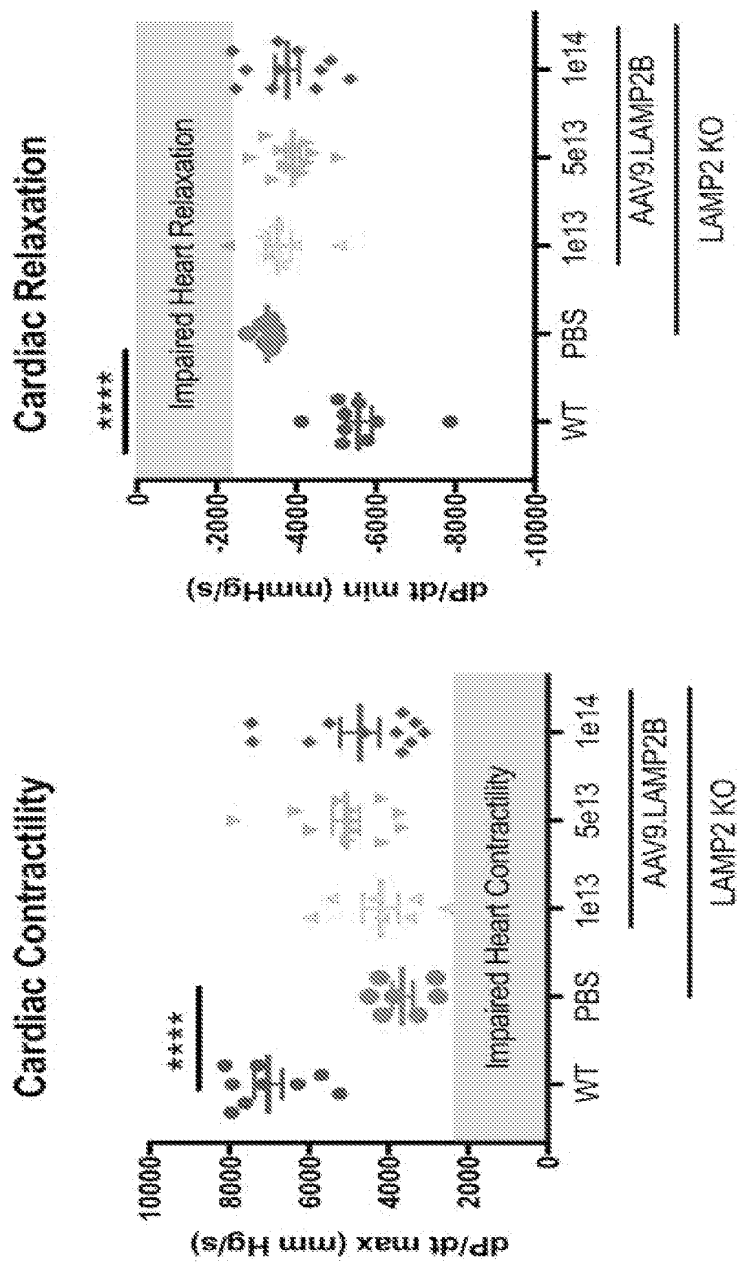
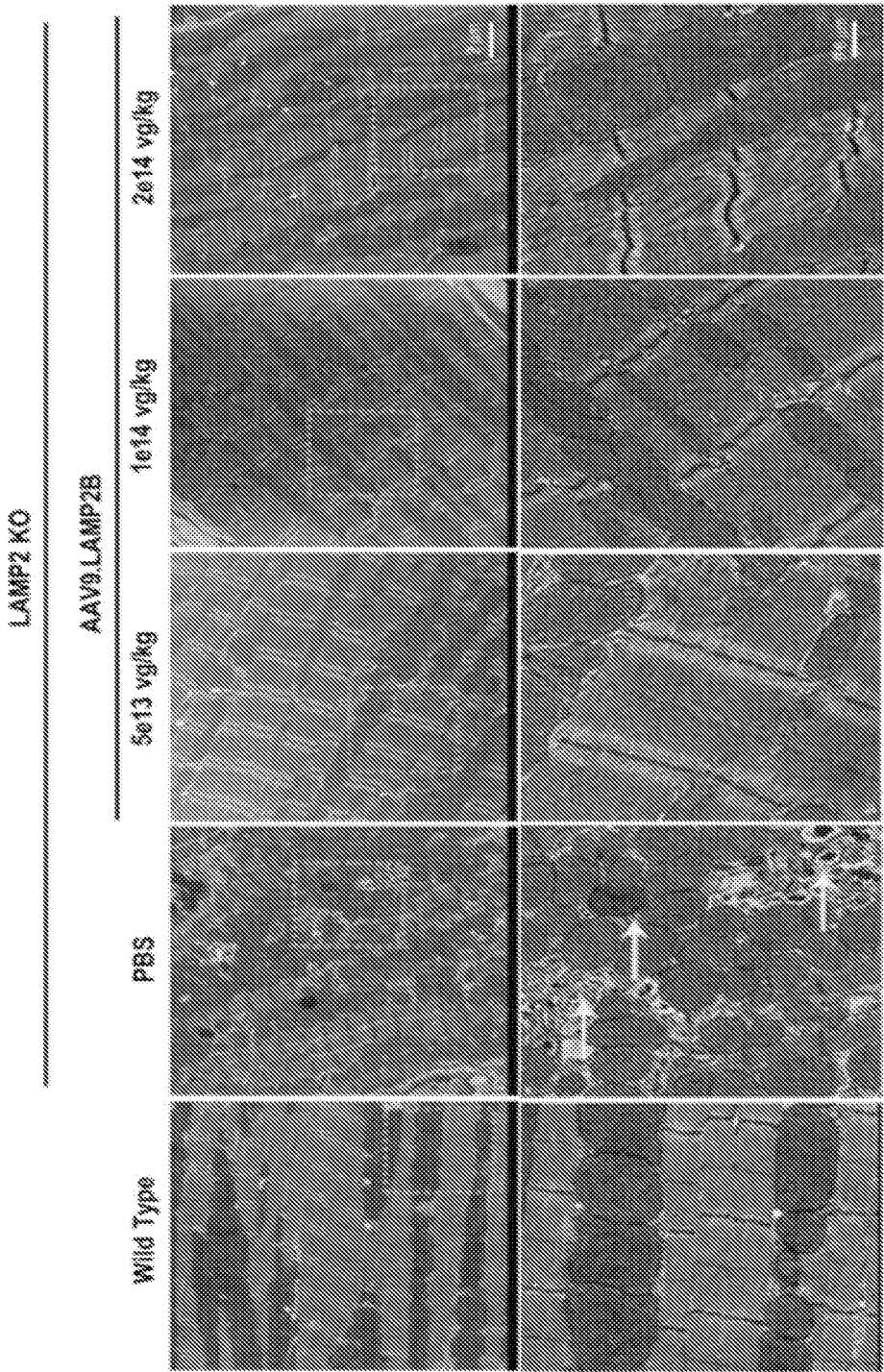


FIG. 11A

FIG. 11B



Cardiac Relaxation

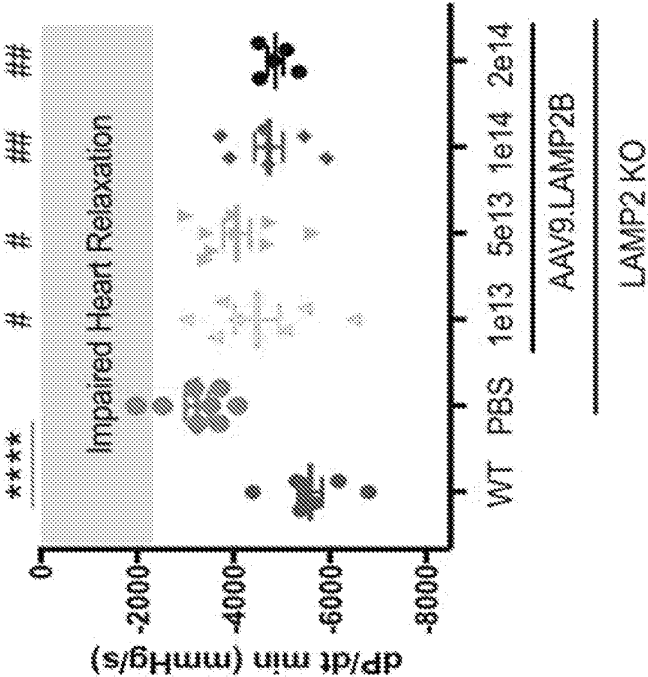


FIG. 13B

Cardiac Contractility

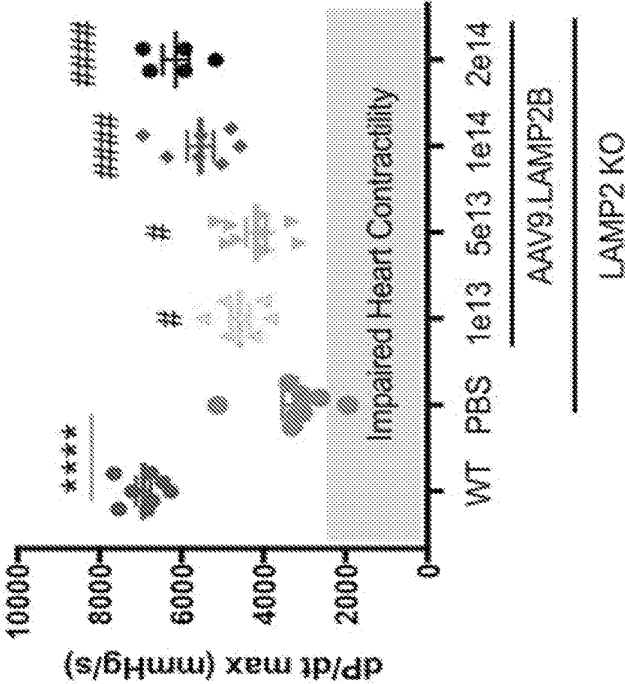


FIG. 13A

18/23

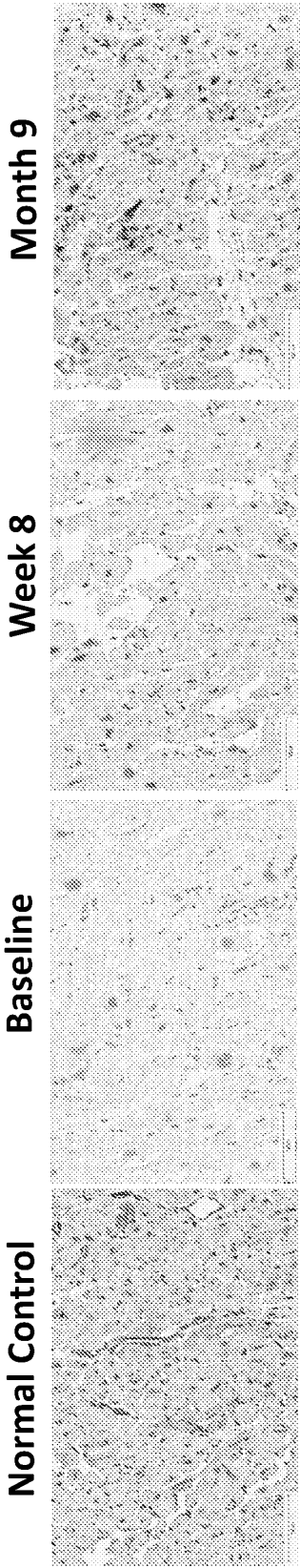


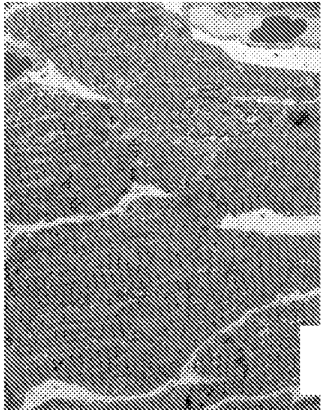
FIG. 14A

19/23

Month 9



Week 8



Baseline

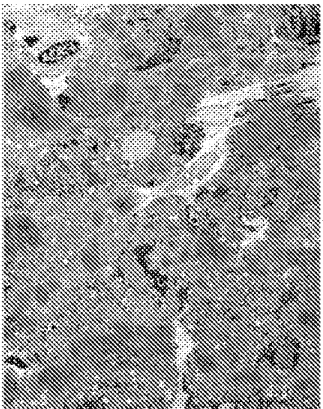


FIG. 14B

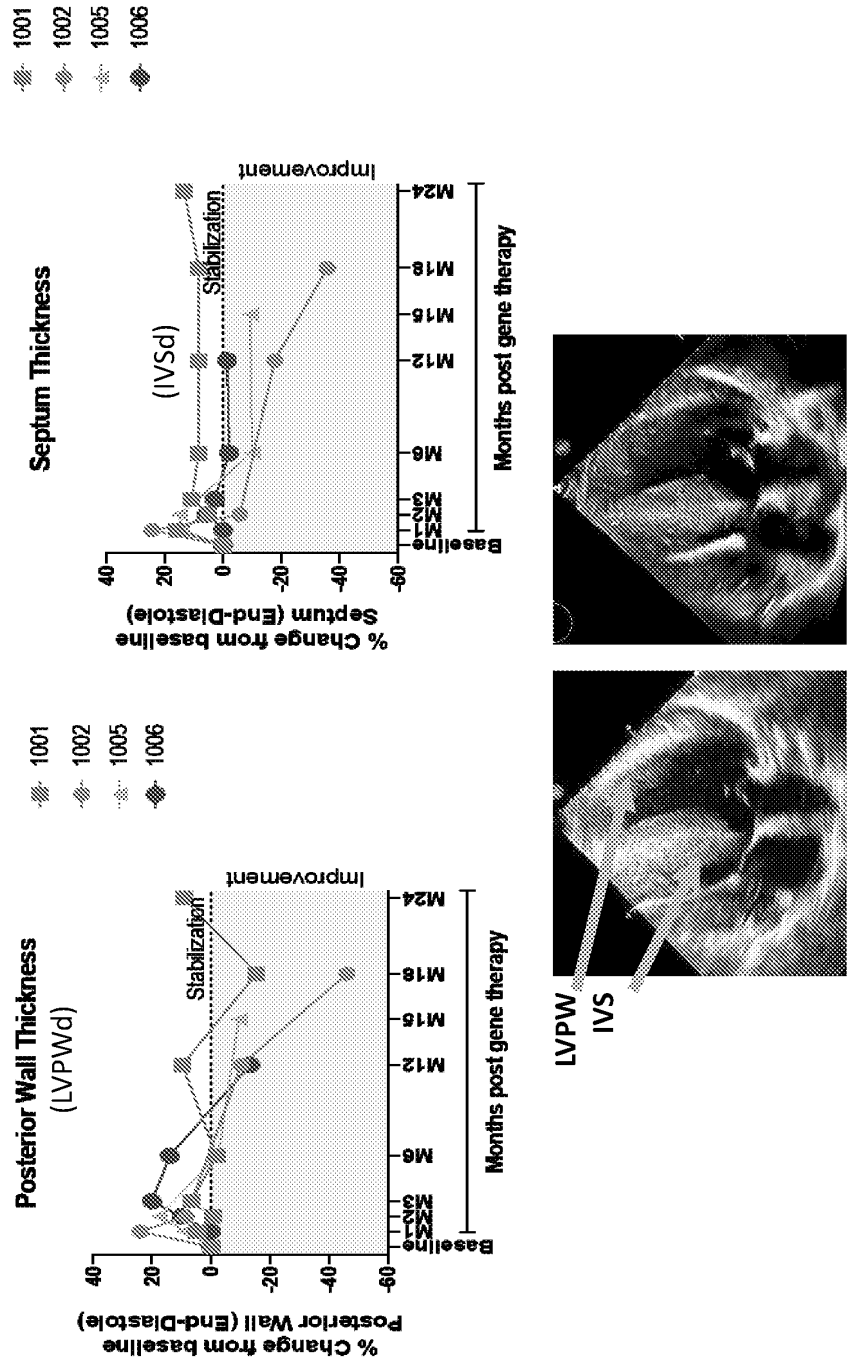


FIG. 15A

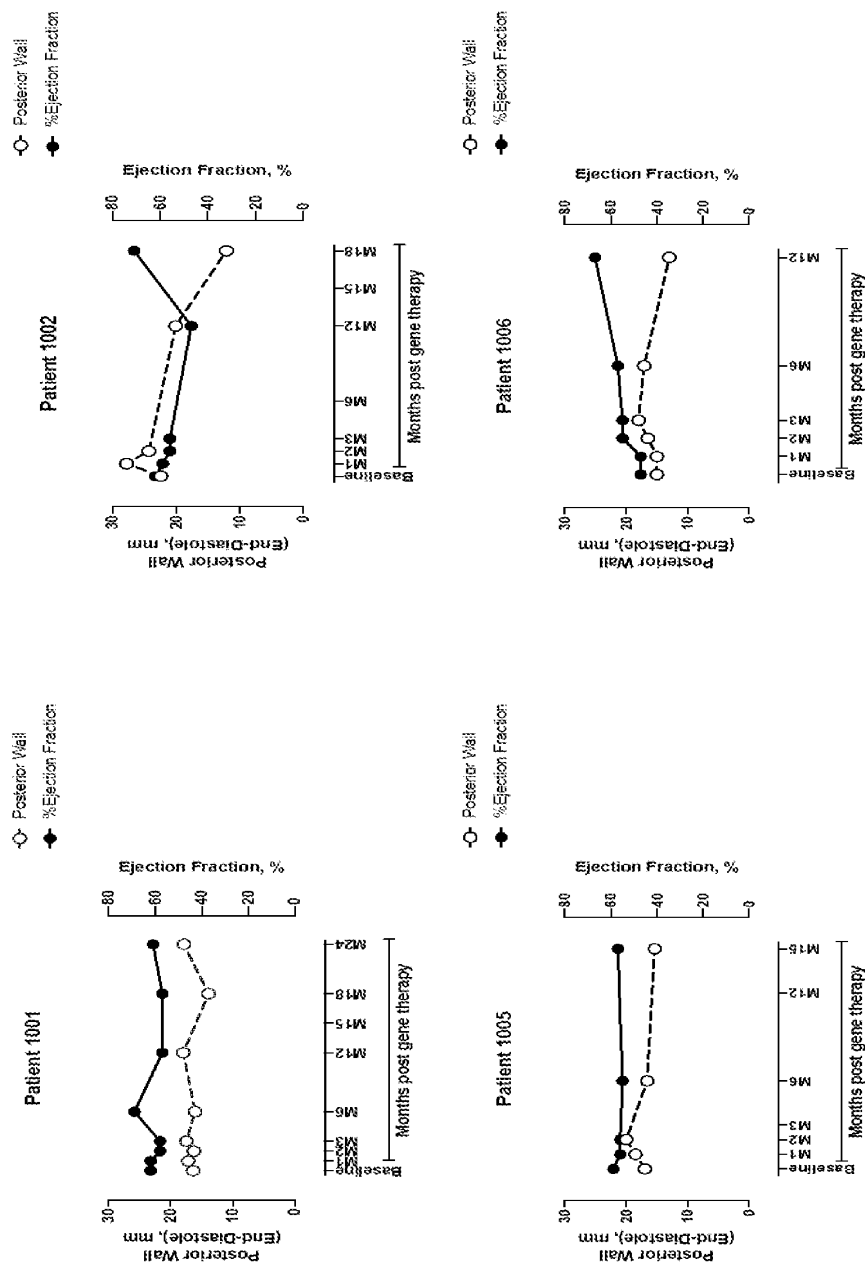


FIG. 15B

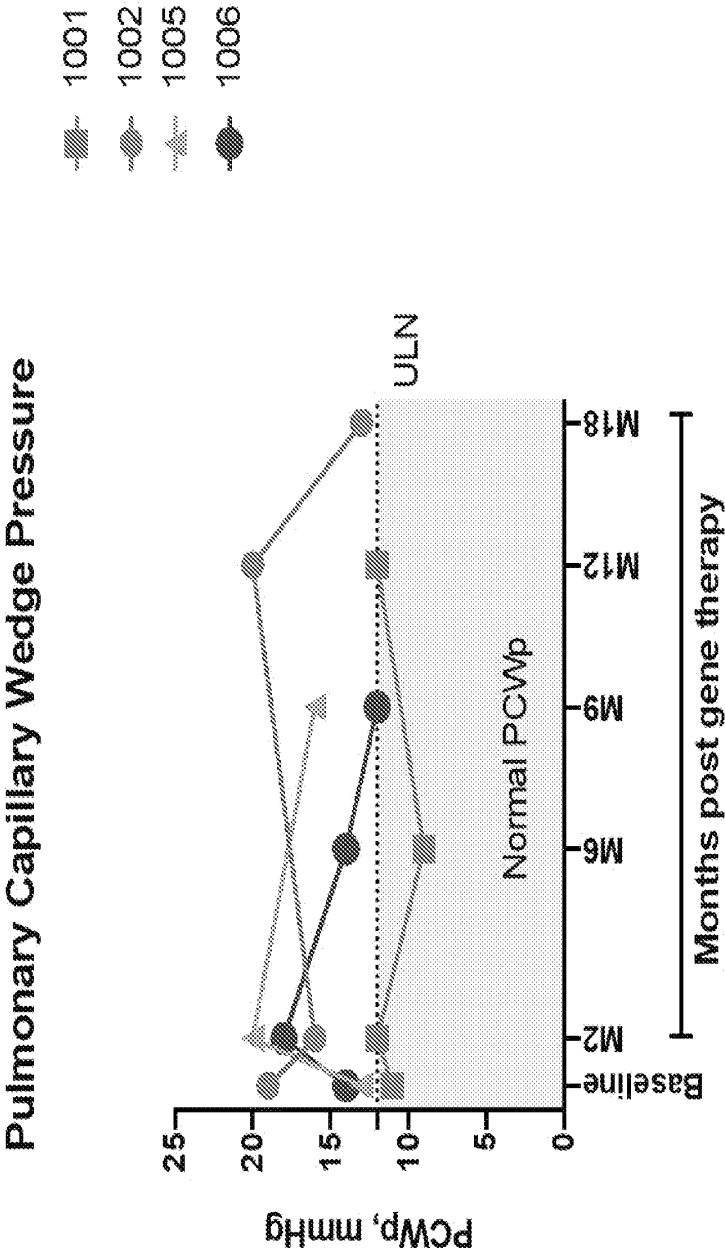
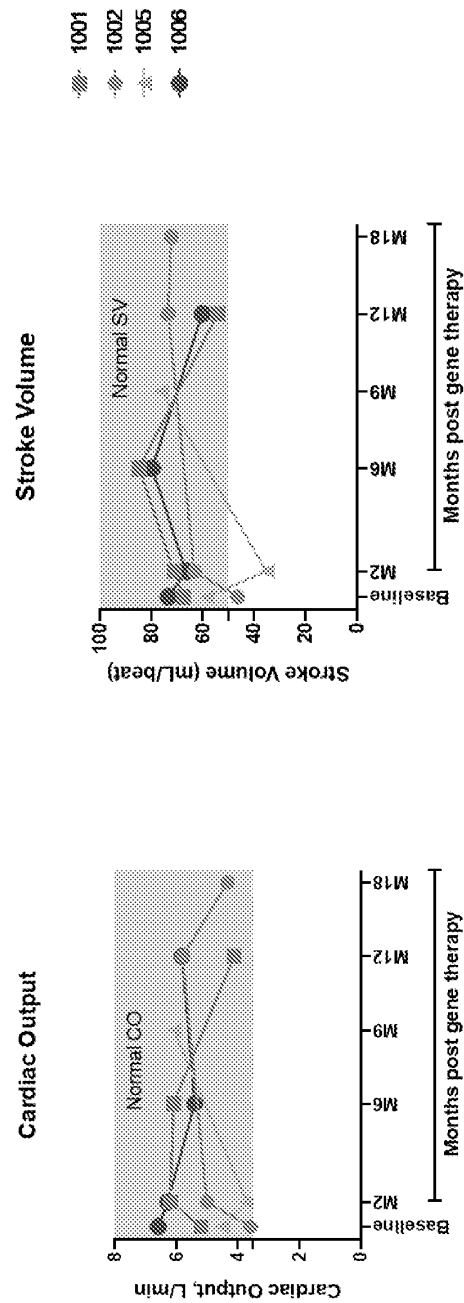


FIG. 15C



Cardiac Output = Stroke Volume x Heart Rate

FIG. 15D