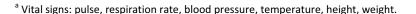
Additional File 5. Study assessment schedule

	Screening visit	Baseline +	2 weeks	4 weeks	6 weeks	12 weeks	24 weeks (End	36 weeks
Assessment		Start					Treatment)	(Follow up)
		treatment						
Time point (weeks)	-4 to -1	0	2	4	6	12	24	36
Visit no.	1	2	3	4	5	6	7	8
Written Informed Consent	Х							
Inclusion/Exclusion Criteria	Х							

	Screening visit	Baseline +	2 weeks	4 weeks	6 weeks	12 weeks	24 weeks (End	36 weeks
Assessment		Start					Treatment)	(Follow up)
		treatment						
Time point (weeks)	-4 to -1	0	2	4	6	12	24	36
Visit no.	1	2	3	4	5	6	7	8
Medical History/	Х							
Current Medical Conditions/								
TSC check								
Hepatitis screen and HIV	Х							
history								
Serum pregnancy test for	X	X	Х	Х	Х	Х	X	Х
female patients / ask males								
if partner pregnant								
Prior/Current concomitant	X	X	Х	Х	Х	Х	X	
medications								
Randomisation		X						

	Screening visit	Baseline +	2 weeks	4 weeks	6 weeks	12 weeks	24 weeks (End	36 weeks
Assessment		Start					Treatment)	(Follow up)
		treatment						
Time point (weeks)	-4 to -1	0	2	4	6	12	24	36
Visit no.	1	2	3	4	5	6	7	8
Drug administration		X*	X*	X*	X*	X*	X*	
Vital signs ^a	X	Х	X	Х	Х	Х	X	
Physical exam (including	X	X		X		Х	Х	
Neurological Exam)								
Neuropsychological outcome	Х	Х		(X)		Х	Х	Х
measures								
Spot urine protein:creatinine	X	Х	Х	Х	Х	Х	Х	Х
ratio								
Haematology ^b	Х	Х	Х	X	X	X	X	

	Screening visit	Baseline +	2 weeks	4 weeks	6 weeks	12 weeks	24 weeks (End	36 weeks
Assessment		Start					Treatment)	(Follow up)
		treatment						
Time point (weeks)	-4 to -1	0	2	4	6	12	24	36
Visit no.	1	2	3	4	5	6	7	8
Coagula - tion Studies	Х					Х	Х	
(PTT/INR)								
Serum Chemistry ^c	Х	Х	Х	Х	х	х	Х	
Serum Lipid Profile ^d	Х	Х				х	Х	
Dipstick Urinalysis ^e	Х	Х	Х	Х	Х	Х	Х	Х
Spirometry ^f	Х	Х	Х	Х	Х	Х	Х	Х
PK Assessments			Х	Х	Х	Х	X	
Adverse Events	Х	Х	Х	Х	Х	Х	Х	Х
Seizure diary	Х	Х	Х	Х	Х	х	Х	Х
Brain DTI MR Scan		Х					Х	



- ^b Haematology: hemoglobin, hematocrit, platelets, red blood cell count, white blood cell count, absolute and differential including neutrophils, lymphocytes, monocytes, eosinophils, basophils.
- ^c Serum chemistry: total LDH, fasting glucose, sodium, magnesium, phosphate, potassium, creatinine, blood urea, albumin, total protein, SGOT (AST), SGPT (ALT), total bilirubin, alkaline phosphatase, uric acid, calcium, CK.
- ^d Serum lipid profile: total cholesterol, triglycerides, LDL, HDL.
- ^e Urinalysis: pH, protein, glucose, blood, ketones, leucocytes.
- f FEV1, FVC
- * Patients are not to take study drug (or placebo) on day on which blood levels are measured until after blood samples have been taken.