**Additional File 5. Study assessment schedule**

| Assessment | Screening visit | Baseline + Start treatment | 2 weeks | 4 weeks | 6 weeks | 12 weeks | 24 weeks (End Treatment) | 36 weeks (Follow up) |
|---|---|---|---|---|---|---|---|---|
| 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 | X | | | | | | | |
| Inclusion/Exclusion Criteria | X | | | | | | | |

| Assessment | Screening visit | Baseline + Start treatment | 2 weeks | 4 weeks | 6 weeks | 12 weeks | 24 weeks (End Treatment) | 36 weeks (Follow up) |
|---|---|---|---|---|---|---|---|---|
| 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/ Current Medical Conditions/ TSC check | X | | | | | | | |
| Hepatitis screen and HIV history | X | | | | | | | |
| Serum pregnancy test for female patients / ask males if partner pregnant | X | X | X | X | X | X | X | X |
| Prior/Current concomitant medications | X | X | X | X | X | X | X | |
| Randomisation | | X | | | | | | |

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|---|---|---|---|---|---|---|---|---|
| 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 | X | X | X | X | X | X | |
| Physical exam (including Neurological Exam) | X | X | | X | | X | X | |
| Neuropsychological outcome measures | X | X | | (X) | | X | X | X |
| Spot urine protein:creatinine ratio | X | X | X | X | X | X | X | X |
| Haematology[b] | X | X | X | X | X | X | X | |

| Assessment | Screening visit | Baseline + Start treatment | 2 weeks | 4 weeks | 6 weeks | 12 weeks | 24 weeks (End Treatment) | 36 weeks (Follow up) |
|---|---|---|---|---|---|---|---|---|
| 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 (PTT/INR) | X | | | | | X | X | |
| Serum Chemistry[c] | X | X | X | X | X | X | X | |
| Serum Lipid Profile[d] | X | X | | | | X | X | |
| Dipstick Urinalysis[e] | X | X | X | X | X | X | X | X |
| Spirometry[f] | X | X | X | X | X | X | X | X |
| PK Assessments | | | X | X | X | X | X | |
| Adverse Events | X | X | X | X | X | X | X | X |
| Seizure diary | X | X | X | X | X | X | X | X |
| Brain DTI MR Scan | | X | | | | | X | |

[a] Vital signs: pulse, respiration rate, blood pressure, temperature, height, weight.

[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.