Supplementary Figure 1

Trial Procedures

BASELINE SCREENING
▪ Day -14 to -1 ▪

Subject consented
Subject ID Number assigned
Patient History
Physical Exam

Tests & Assessments

- ECHO
- EKG
- Chest X-Ray
- Hepatitis B&C Screen
- GGT
- Total Bilirubin
- Glucose
- Creatine Kinase
- Creatinine/BUN
- Urinalysis
- PT/PTT
- CBC/Diff/Platelet with smear
- Neutralizing Antibody to AAV
- Antibody to Dystrophin
- Forced vital capacity
- MMT
- MVICT

VECTOR INFUSION & INPATIENT FOLLOW-UP
▪ Day 0 to Day 2 ▪

Day 0

Subject admitted to the CCH

Peri-injection immunosuppressant:
IV Methylprednisolone 2.0 mg/kg
(4 hours before gene transfer)

Study Procedures:
Marker injection site
Photograph of injection site/surrounding area
Ultrasound (injection needle guide)
AAV2.5/Control infusion
Toxicity monitoring post-gene transfer
Vital Signs hourly x 4hours
Vital Signs every 4 hours until patient released

Days 1 & 2

Peri-injection immunosuppression:
IV Methylprednisolone 2.0 mg/kg
(2 consecutive mornings)

Toxicity monitoring post-gene transfer:
Vital signs every 4 hours
Physical Exam
GGT/Total bilirubin
Glucose
Creatine kinase
PT/PTT
CBC/Diff/Platelet with smear
Urinalysis
Photograph of injection site/surrounding area
Patient released (Day 2)

OUTPATIENT FOLLOW-UP
▪ Day 8 to Year 2 ▪

Days: 8, 15, 30, 43, 53, 60, 90, 120
Months: 6, 12, 18, 24

Neutralizing antibody to AAV

Antibody to Dystrophin

ELISPOT assay will be performed on positive samples for antibodies to Dystrophin or AAV.

Semen test, Day 90* (vector identification by DNA-PCR in semen and motile sperm fraction of patients in reproductive age and sexually active if they are able to provide it; testing will continue every 7 days, until 2 consecutive negative samples are obtained)

Day 15	Day 30	Day 43	Day 90
GGT CBC/Diff Creatinine Alkaline phosphatase Urinalysis Photograph of injection site/surrounding area	Bilirubin Platelets Electrolytes	Amylase PT/PTT Total Protein Glucose Muscle Biopsy – First two subjects/each cohort	Muscle Biopsy – Third subject/each cohort

LONG-TERM FOLLOW-UP
▪ Year 1 to Year 15 ▪

We will evaluate short-term safety over a two-year period that incorporates the active phase of the protocol. If newly identified risks are associated with our product, or if the patients suffer any adverse events during this period, we will initiate a long-term follow-up.