STARFiSH Study
(Study of Testosterone and rHGH in FSHD)

By

Chad Heatwole, MD, MS-CI

Therapeutics Pipeline Panel

FSHD Connect

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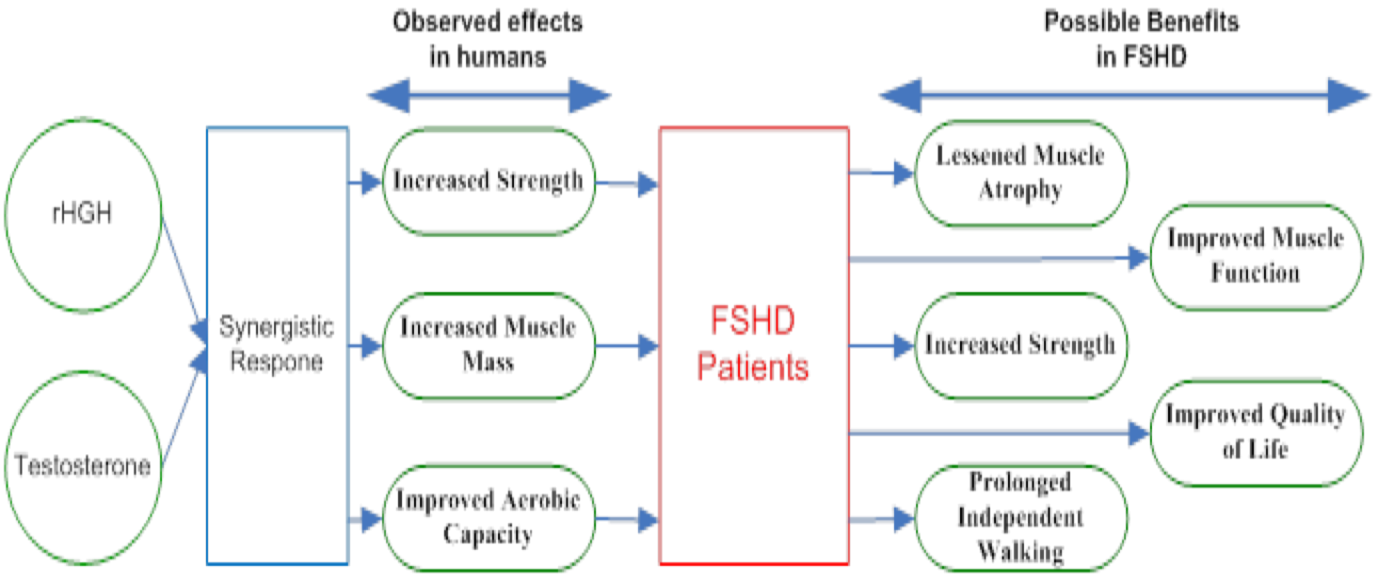
Promising Results in Healthy Elderly Men

- Increased lean (muscle) body mass
- Increased strength (up to 29%)
- Increased aerobic activity (cycle and treadmill testing)
- Reasonable safety profile

- 1. Blackman MR, Sorkin JD, Munzer T, et al. Growth hormone and sex steroid administration in healthy aged women and men: a randomized controlled trial. JAMA 2002;288:2282-92.
- 2. Giannoulis MG, Sonksen PH, Umpleby M, et al. The effects of growth hormone and/or testosterone in healthy elderly men: a randomized controlled trial. J Clin Endocrinol Metab 2006;91:477-84.
- 3. Sattler FR, Castaneda-Sceppa C, Binder EF, et al. Testosterone and growth hormone improve body composition and muscle performance in older men. J Clin Endocrinol Metab 2009;94:1991-2001.

The utility and safety of combination therapy has never been tested in a muscular dystrophy population

Potential Benefits of Combination Therapy in FSHD



Aims

- **Specific Aim 1:** To examine the safety and tolerability of testosterone enanthate paired with recombinant human growth hormone (rHGH) over a 24 week treatment period and a 12 week washout period in 20 adult ambulatory male participants with FSHD.

- **Specific Aim 2:** To measure the effect of combination therapy on biomarkers including lean body mass systematically measured using Dual Energy X-Ray Absorptiometry and serum markers including total testosterone, free testosterone, and total IGF-1.

- **Specific Aim 3:** To examine the exploratory effect of combination therapy on measures of clinical function including those involving ambulation, strength, pulmonary function, and patient reported disease-burden in FSHD participants.

Inclusion Criteria

- Men age 18 to 65
- A genetically confirmed diagnosis of FSHD (or clinical symptoms suggestive of FSHD with a first degree relative with genetically confirmed FSHD)
- Hematocrit of ≤ 50%
- Prostate-specific antigen ≤ 4.0 ng/ml (or ≤ 3.0 ng/ml if the participant has a first-degree relative with prostate cancer)
- Fasting blood glucose <126 mg/dl
- Able to walk continuously for six minutes (cane, walker, orthoses allowed)
- Able to independently administer intramuscular and subcutaneous injections (or have a family member who is capable and willing to administer these injections)
- Exclusion criteria listed on clinicaltrials.gov

Table 2: Schedule of Study Activities and Testing									
	Screening	Baseline	4w	8w	12w	16w	20w	24w	36w
Activity									
On Combination Therapy									
Washout Period									
Informed consent	X								
Documentation of Inclusion/Exclusion criteria	X								
Clinic Visits	X	X		X		X		X	X
Laboratory Testing *	X	X		X		X		X	X
Study Medication Dispensed or Mailed		X	X	X	X	X	X		
Dual Energy X-Ray Absorptiometry (DEXA)		X				X		X	X
Strength, Ambulation, and Functional Testing		X		X		X		X	X
Exercise Log Review				X		X		X	X
Patient-Reported Outcome Measures	X	X		X		X		X	X
Safety Monitoring									
Vitals **	X	X		X		X		X	X
Physical Exam	X			X		X		X	X
Fundoscopic Exam		X		X		X		X	X
Digital Rectal Exam	X			X		X		X	X
International Prostate Symptom Score	X			X		X		X	X
Quantitative Insulin Sensitivity Check Index		X		X		X		X	X
Side effect questionnaire		X		X		X		X	X
EKG	X			X		X		X	X
Weekly Phone Calls									
* Free and total T levels, IGF-1 (Free/ total), PSA, fasting glucose, chem 14, CK									
lipid profile, luteinizing hormone, Insulin levels, HGA1c, FSH, CBC, CRP, TSH, and Free T4 levels									
** Height, weight, blood pressure, body mass index, pulse, respiratory rate									

Progress to Date

- 6 participants enrolled
- 3 additional participants scheduled for first visit
- 10 study visits completed
- No participant drop outs
- 27 patients identified for the last 11 spots
- Full results expected by 2022

Thank you