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

The utility and safety of combination therapy has never been tested in a muscular dystrophy population
Potential Benefits of Combination Therapy in FSHD

- **rHGH**
  - Synergistic Response
  - Increased Strength
  - Increased Muscle Mass
  - Improved Aerobic Capacity

- **Testosterone**

**Observed effects in humans**

**Possible Benefits in FSHD**

- Lessened Muscle Atrophy
- Improved Muscle Function
- Increased Strength
- Improved Quality of Life
- Prolonged Independent Walking
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 $\leq 50\%$
- Prostate-specific antigen $\leq 4.0$ ng/ml (or $\leq 3.0$ 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

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<thead>
<tr>
<th>Activity</th>
<th>Screening</th>
<th>Baseline</th>
<th>4w</th>
<th>8w</th>
<th>12w</th>
<th>16w</th>
<th>20w</th>
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### Safety Monitoring

| Vitals **                                    | X         | X         | X   | X   | X   | X   | X   |
| Physical Exam                                |           |           | X   | X   | X   |     |     |
| Fundoscopic Exam                             |           |           | X   | X   | X   |     |     |
| Digital Rectal Exam                          |           |           | X   | X   | X   |     |     |
| International Prostate Symptom Score         |           |           | X   | X   | X   |     |     |
| Quantitative Insulin Sensitivity Check Index |           |           | X   | X   | X   |     |     |
| Side effect questionnaire                    |           |           | X   | X   | X   |     |     |
| EKG                                          |           |           | 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, HbA1c, 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