

Clinical Update: Micro-dystrophin Study-101

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Micro-dystrophin Study-101 Clinical Trial Design



Open-Label Trial Design

- **Cohort B**
 - 4 Patients
 - 4-7 years of age
- **Inclusion criteria**
 - Confirmed *DMD* mutation
 - Negative for AAVrh74 antibodies

ClinicalTrials.gov Identifier: NCT03375164.

Sarepta Therapeutics Data on File. AAVrh74.MHCK7.Micro-dystrophin is investigational and not approved in Argentina.

Cohort B (4-7 Years of Age) Endpoints

- **Primary endpoint**
 - Safety
- **Secondary endpoints**
 - Change in micro-dystrophin expression pre- vs post-treatment
 - Decrease in CK
 - 100-meter timed test (100 m)
 - North Star Ambulatory Assessment (NSAA; 10-meter timed test included)
 - Timed up and go (TUG)
 - Ascend and descend 4 steps
 - Hand-held dynamometry (HHD)
 - Cardiac magnetic resonance imaging (at 1 year)

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Subject Demographics at Baseline

| Subject | Age (years) | CK Levels at Baseline (U/L) |
|---------|-------------|-----------------------------|
| 1 | 5 | 20,691 |
| 2 | 4 | 23,414 |
| 3 | 6 | 34,942 |
| 4 | 4 | 29,210 |

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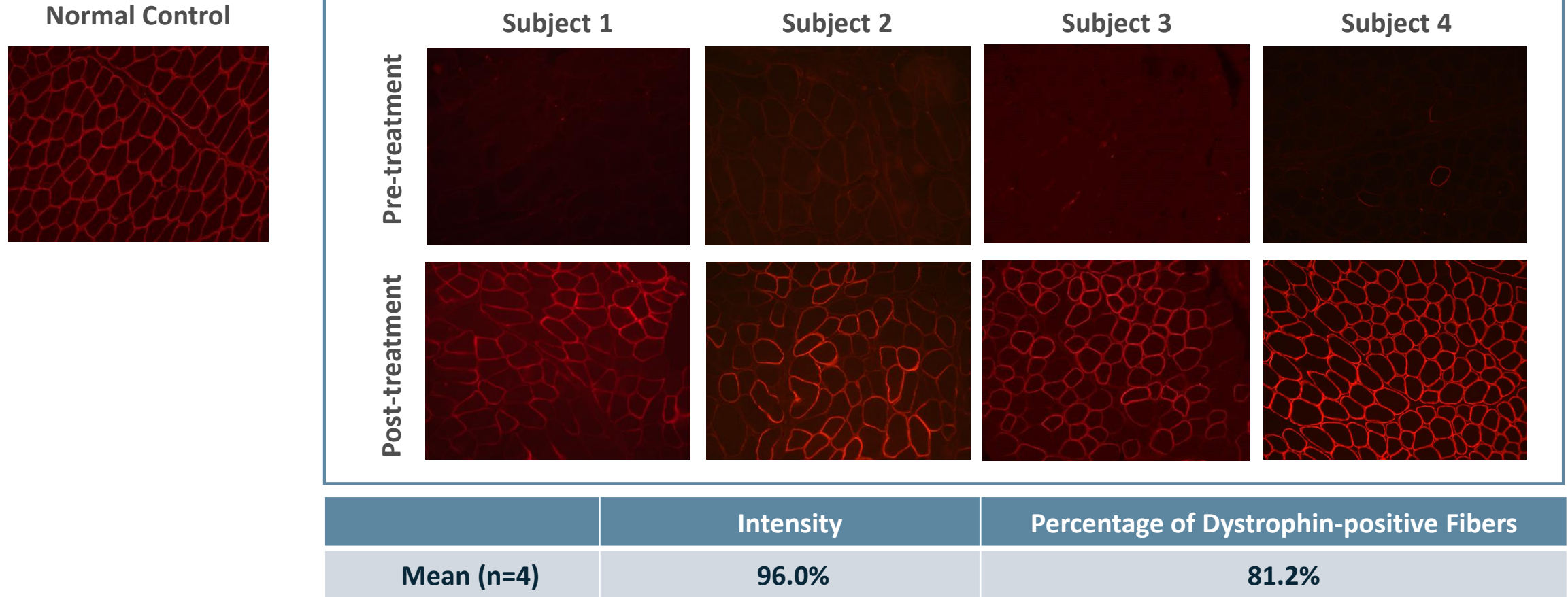
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Micro-dystrophin Study-101
Summary:
All Patients (n=4)

Robust Micro-dystrophin Expression in Muscle Fibers from the Gastrocnemius in All 4 Patients at Day 90

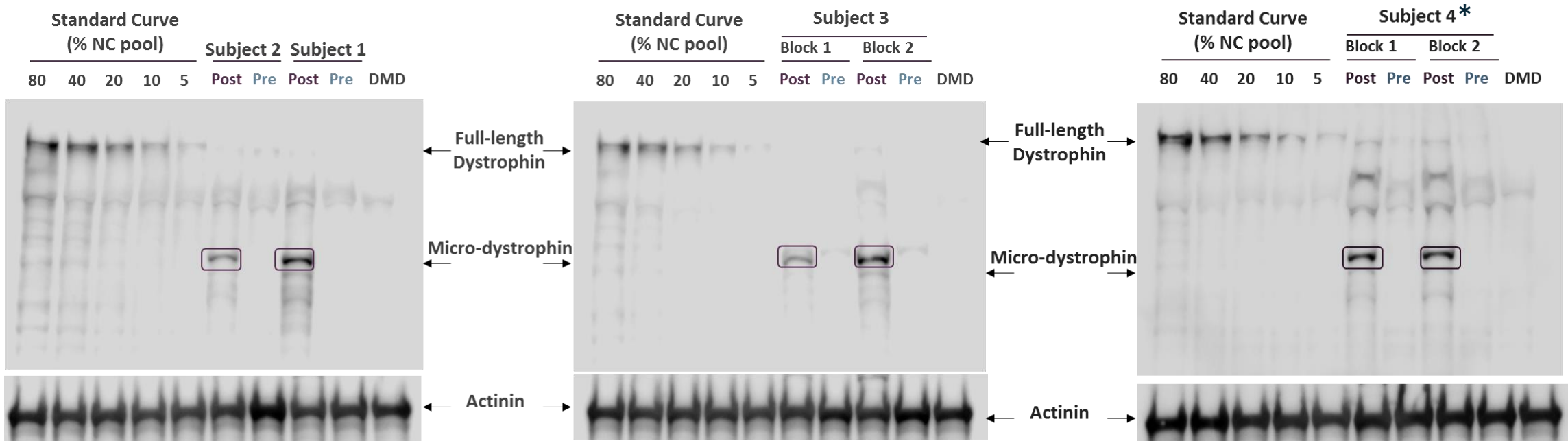
Micro-dystrophin Expression (IHC)



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Detection of Micro-dystrophin Expression by Western Blot Post-treatment in All 4 Patients at Day 90



| Western Quantitation Method | Mean Micro-dystrophin Expression (N=4) vs Normal |
|-----------------------------|--|
| Sarepta | 74.3% (not adjusted for fat and fibrotic tissue) |
| Nationwide | 95.8% (adjusted for fat and fibrotic tissue) |

p-Tyr phosphotyrosine; ULOQ, upper limit of quantification.

*Samples diluted 1:4 due to sample above ULOQ.

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Summary of Expression Data for all 4 Patients

Micro-dystrophin Expression (IHC)

| | Intensity | Percentage of Dystrophin-positive Fibers |
|------------|-----------|--|
| Mean (n=4) | 96.0% | 81.2% |

Micro-dystrophin Expression (Western Blot)

| | Sarepta (not adjusted for fat/fibrosis) | Nationwide (adjusted for fat/fibrosis) |
|------------|--|---|
| Mean (n=4) | 74.3% | 95.8% |

Vector Genome Number

| | Vector Copies/ μ g DNA | Copies per Nucleus |
|------------|----------------------------|--------------------|
| Mean (n=4) | $>10^5$ | 3.3 |

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Clinical Data Summary for Study-101 (n=4)

Summary of NSAA Data for All 4 Patients

NSAA Change from Baseline to Day 270

| Patient | Baseline | Day 30 | Day 60 | Day 90 | Day 180 | Day 270 | Change from Baseline |
|-------------------------|----------|--------|--------|--------|---------|---------|----------------------|
| 1 | 18 | 22 | 24 | 23 | 25 | 26 | 8 |
| 2 | 19 | 21 | 23 | 25 | 27 | 27 | 8 |
| 3 | 26 | 28 | 28 | 30 | 30 | 28 | 2 |
| 4 | 19 | 20 | 20 | 25 | 25 | 27 | 8 |
| Mean Improvement | 20.5 | 22.75 | 23.75 | 25.75 | 26.75 | 27 | 6.5 |

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Summary of Clinical Data: Consistent Durable Improvement at 9 Months

Change from Baseline to Day 270

| Subject | Assessment | NSAA (Δ) | Time to Rise (sec) | 4 Stairs Up (sec) | 100 m (sec) |
|----------------|----------------------|-----------------------|-----------------------|------------------------|-------------------------|
| 1 | Baseline | 18 | 3.7 | 3.4 | 49.3 |
| | Day 270* | 26 | 3.0 | 2.3 | 43.2 |
| 2 | Baseline | 19 | 3.0 | 3.8 | 49.9 |
| | Day 180* | 27 | 3.7 | 2.6 | 48.6 |
| | Day 270 | 27 | 3.3 | 2.7 | 50.3 |
| 3 | Baseline | 26 | 3.9 | 1.9 | 59.3 |
| | Day 180* | 30 | 3.4 | 1.8 | 48.4 |
| | Day 270 | 28 | 2.8 | 1.9 | 50.7 |
| 4 | Baseline | 19 | 4.1 | 4.8 | 67.2 |
| | Day 90* | 25 | 2.3 | 2.2 | 50.7 |
| | Day 270 | 27 | 2.4 | 2.2 | 49.7 |
| Average | Change From Baseline | 6.5 point Improvement | .8 second Improvement | 1.2 second Improvement | 7.95 second Improvement |

* Last timepoint disclosed at World Muscle Society

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Summary of CK Data for All 4 Patients

CK Change from Baseline to Day 270

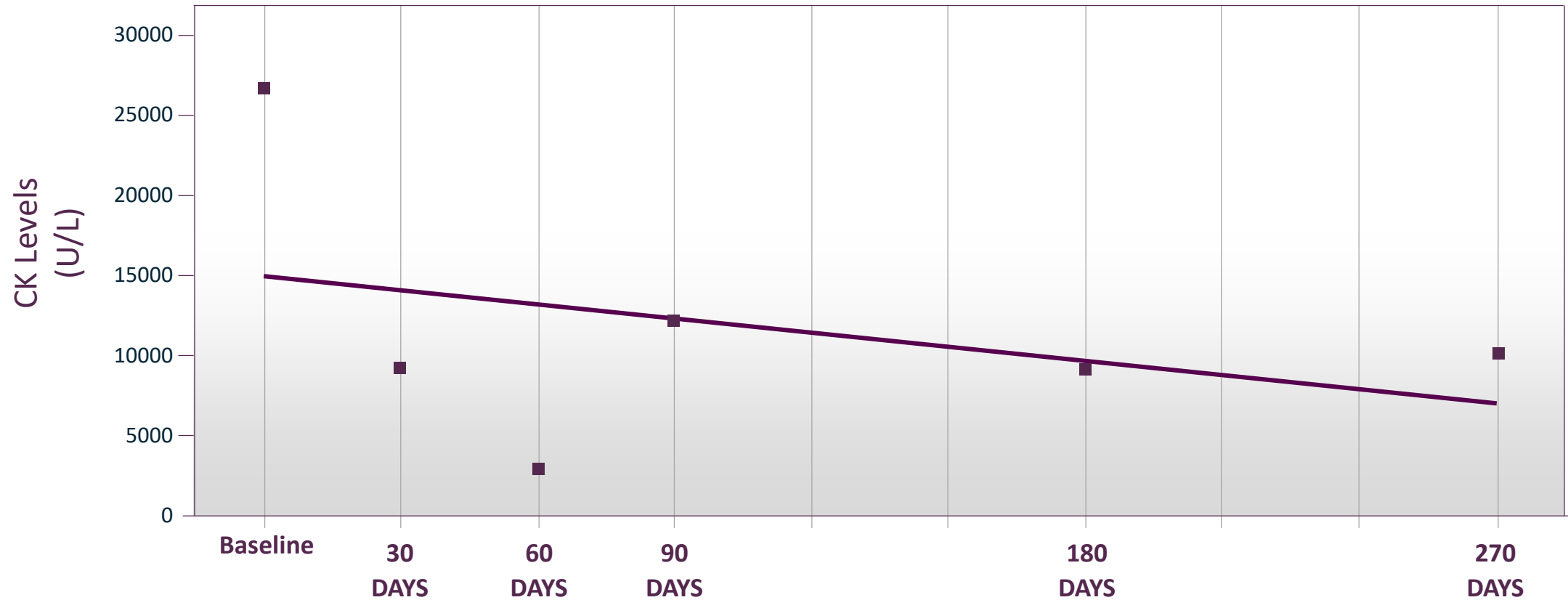
| Patient | Baseline | Day 30 | Day 60 | Day 90 | Day 180 | Day 270 |
|---------|----------|--------|--------|--------|---------|---------|
| 1 | 20691 | - | 2984 | 2444 | 18476 | 6317 |
| 2 | 23414 | 10427 | 4283 | 41920 | 6209 | 10494 |
| 3 | 34942 | 10430 | 2966 | 2546 | 9650 | 18855 |
| 4 | 29210 | 7215 | 908 | 1382 | 2580 | 4262 |

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CK Significantly Decreases Over Time

Mean CK Change from Baseline to Day 270

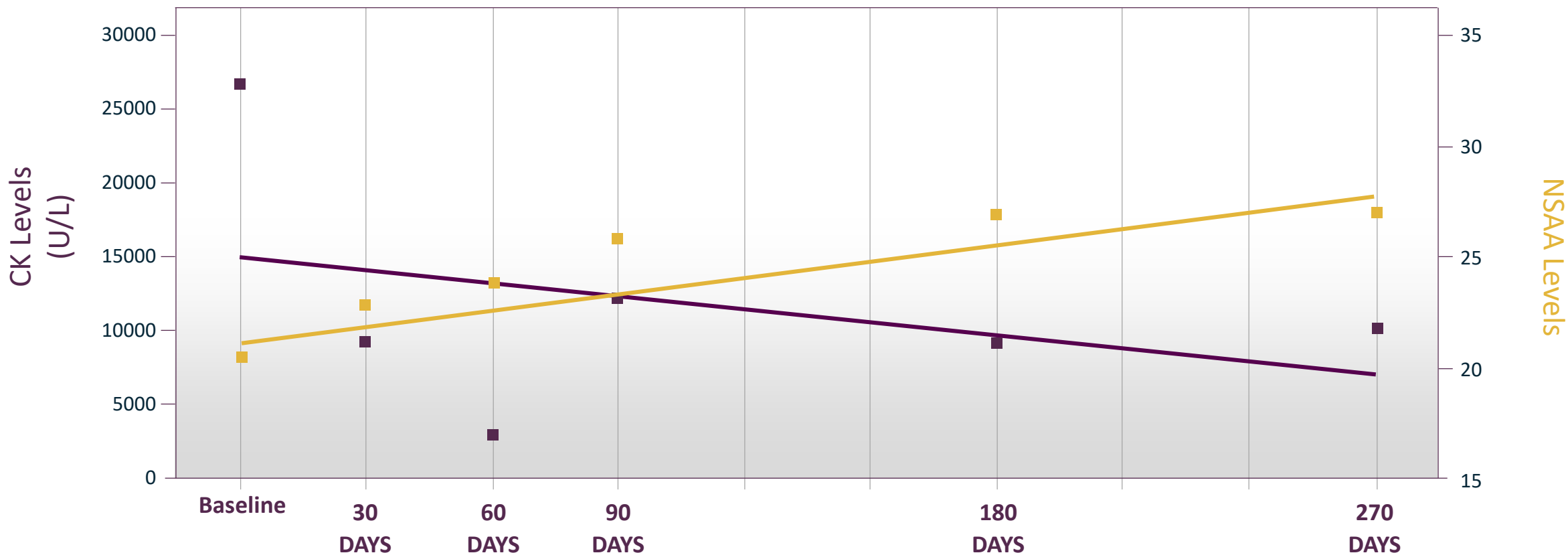


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NSAA Significantly Increases Over Time

Mean NSAA Change from Baseline to Day 270



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Safety (n=4)

- No serious adverse events in this study
- 3 Patients had elevated γ -glutamyl transpeptidase, which resolved with steroid treatment within a week
- No other clinically significant laboratory findings
- Subjects had transient nausea generally within the first week coincident with increased steroid dosing
 - Did not correlate with liver enzyme elevations or any other abnormality

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Summary

- All 4 treated Patients are doing well
 - Biomarkers show large magnitude of effect within 3 months (CK and dystrophin)
 - Initial functional data shows consistent and persistent improvement from Baseline to Day 270
 - Early results show treated patients performing better than DMD natural history would predict
 - Favorable safety profile to date with 9 months of follow-up
 - Currently enrolling Study-102 (24 patient placebo controlled study)

Question and Answer



Thank You

