

434P SAT-3247: an oral small molecule inhibitor targeting AAK1, a critical effector of skeletal muscle regeneration

~Results from an open label pilot study in a canine model of Duchenne~



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About Satellos

Satellos is developing the world's first drug that has been specifically designed to target the innate process of muscle regeneration, through regulation of satellite stem cell polarity. SAT-3247 is a potent, orally available, muscle penetrant, small molecule inhibitor of Adapter associated kinase 1 (Aak1) that when administered, enhances ongoing skeletal muscle regeneration and repair. Satellos is planning to evaluate SAT-3247 in Duchenne muscular dystrophy, in an effort to rescue the known deficit and inefficiency of Duchenne skeletal muscle to repair itself, resulting in progressive muscle loss over time. Satellos believes that this therapeutic approach will be the first of its kind that offers to not only stabilize muscle loss, but potentially bring muscle back by augmenting its natural regenerative capacity.

SAT-3247 Highlights



- Orally available tablet formulation
- Biologically active in the enhancement of skeletal muscle regeneration in models of Duchenne, FSHD, and healthy muscle injury
- First in man studies Q3 2024

Study Design & Data Legend

Duchenne Canine
Mixed genetic background, carrying the Labrador mutation (LINE element insertion in intron 19) on the corresponding X chromosome(s)

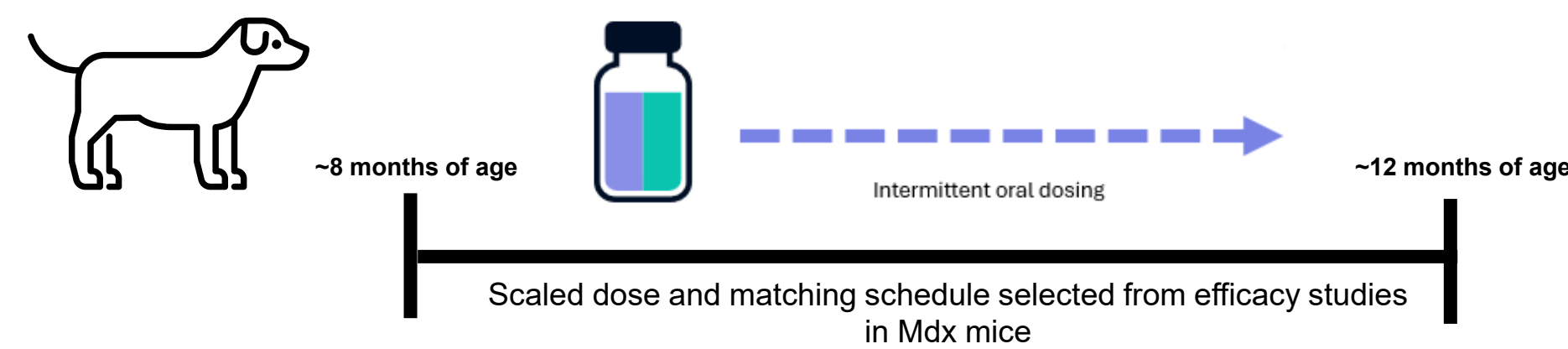
Multi-parameter pilot study using 2 affected dogs

Weekly 9 datapoints	Monthly 5 datapoints	Every 2 Months 3 datapoints	Study End 1 datapoint
Body weight	Gait analysis	Force evaluation	ECG/Echo
Clin Chem and Hematology	Overnight activity	Muscle biopsy	ECU function
	Cytokine panel		Necropsy

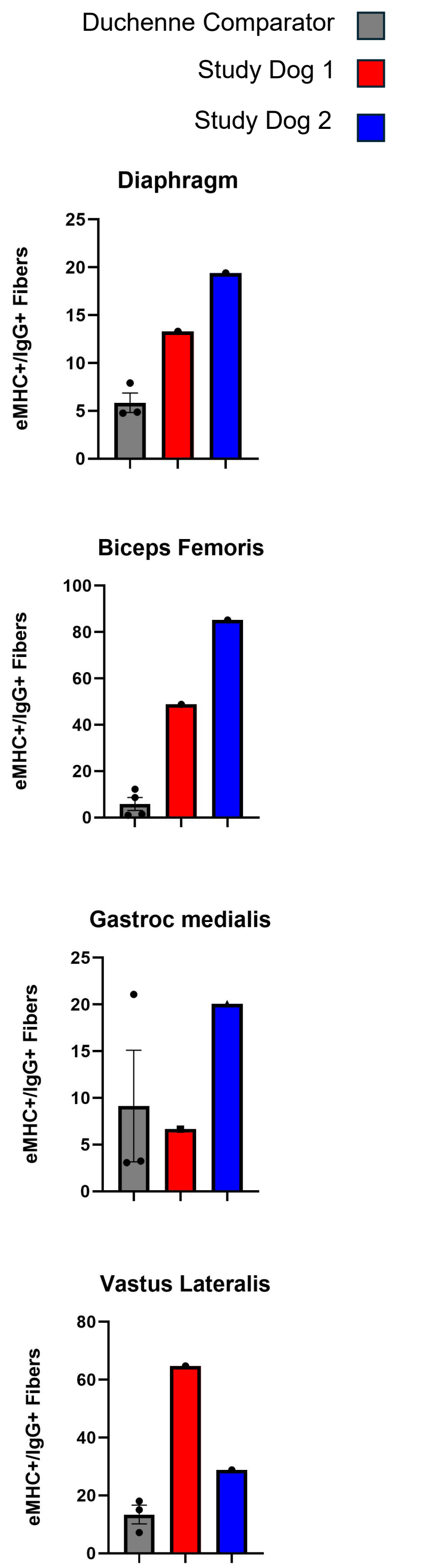
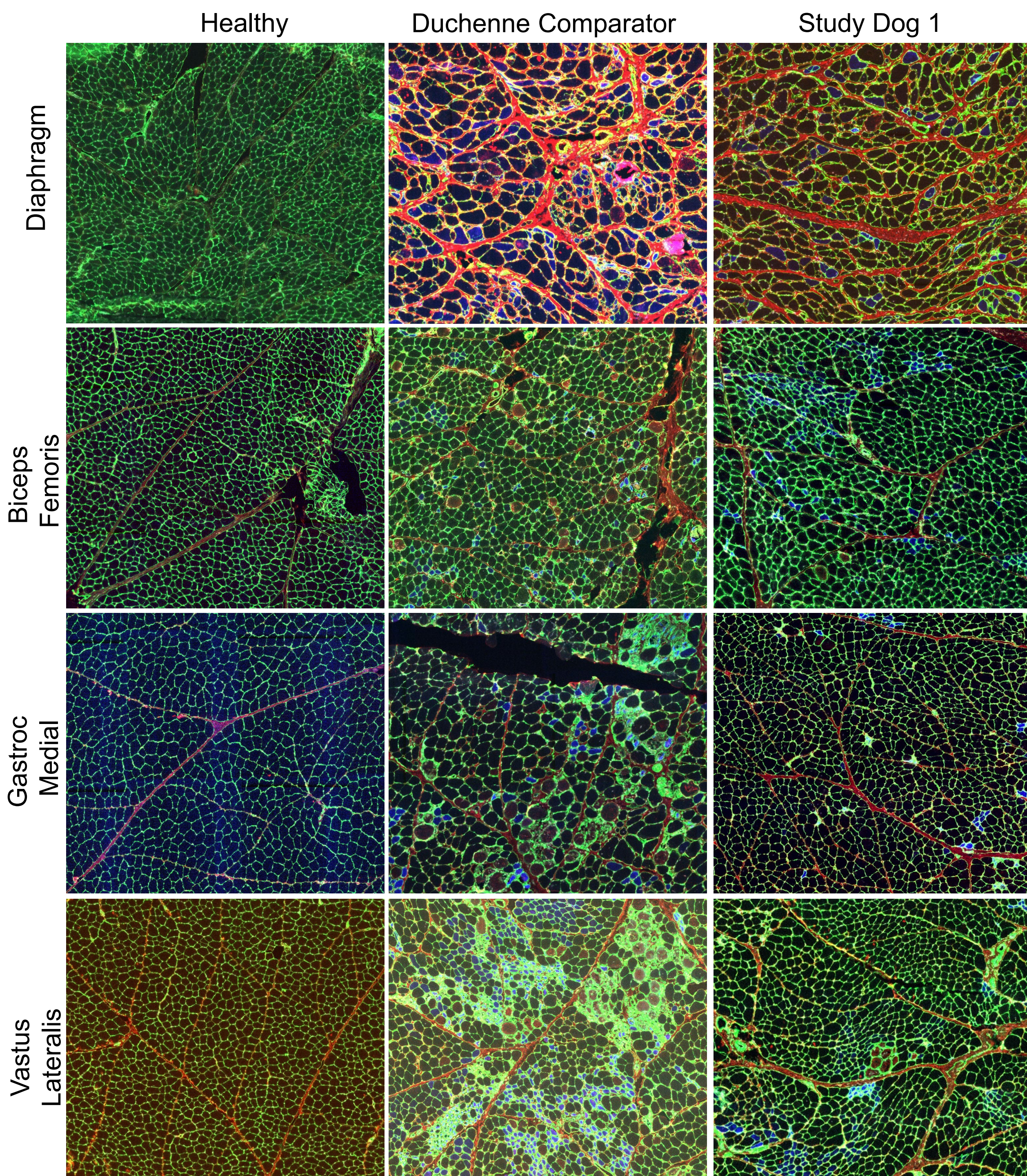
Data is presented for two animals with a consistent colour theme:

- Study Dog 1 (Red)
- Study Dog 2 (Blue)

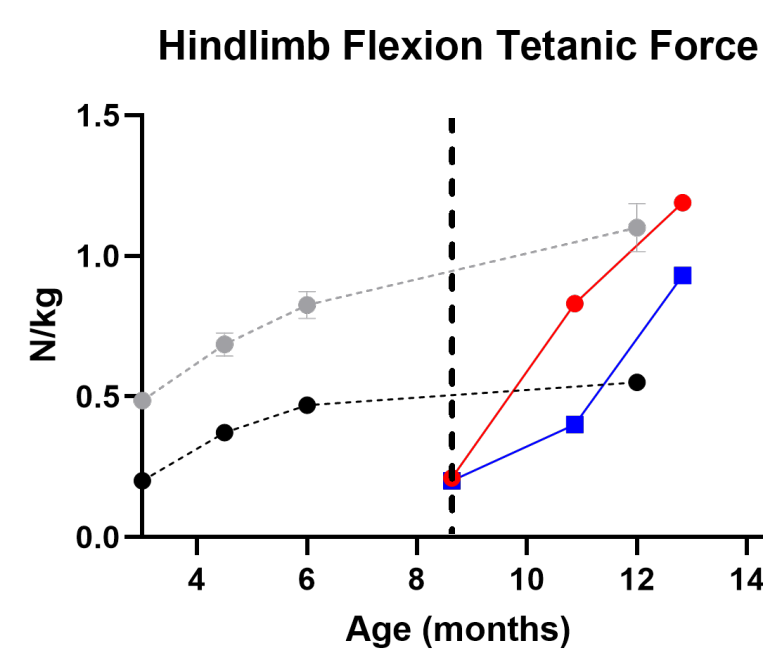
Vertical hashed line indicates start of dosing
Grey boxes indicate healthy reference value



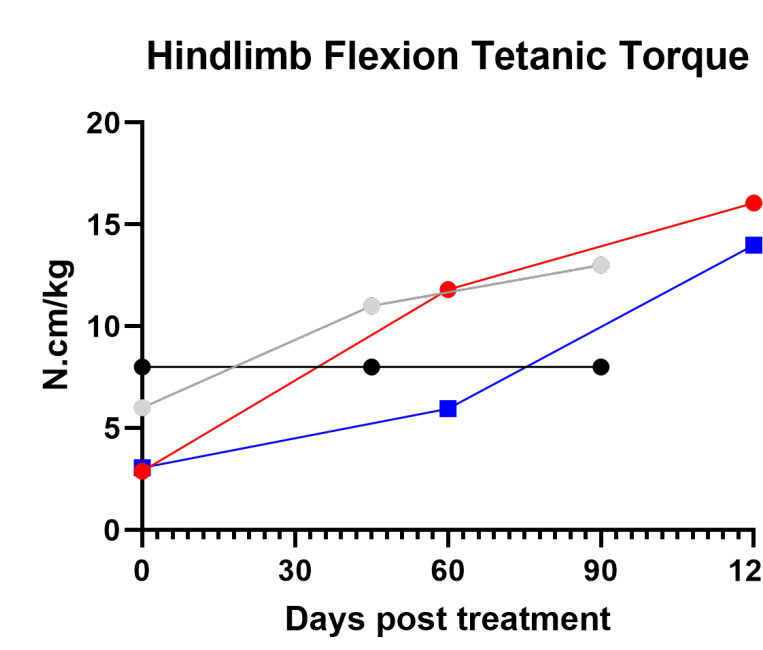
Regeneration Indexing (Laminin, IgG, eMHC)



Whole Limb Functional Testing (body weight normalized)



Benchmark Case 1
• Data adapted from Konegay et al. 1999
• GRMD (black line) and age matched healthy dogs (grey line) tracked from 3m to 12m of age
• SAT-3247 treated dogs treated from ~9m to ~13m plotted as an overlay



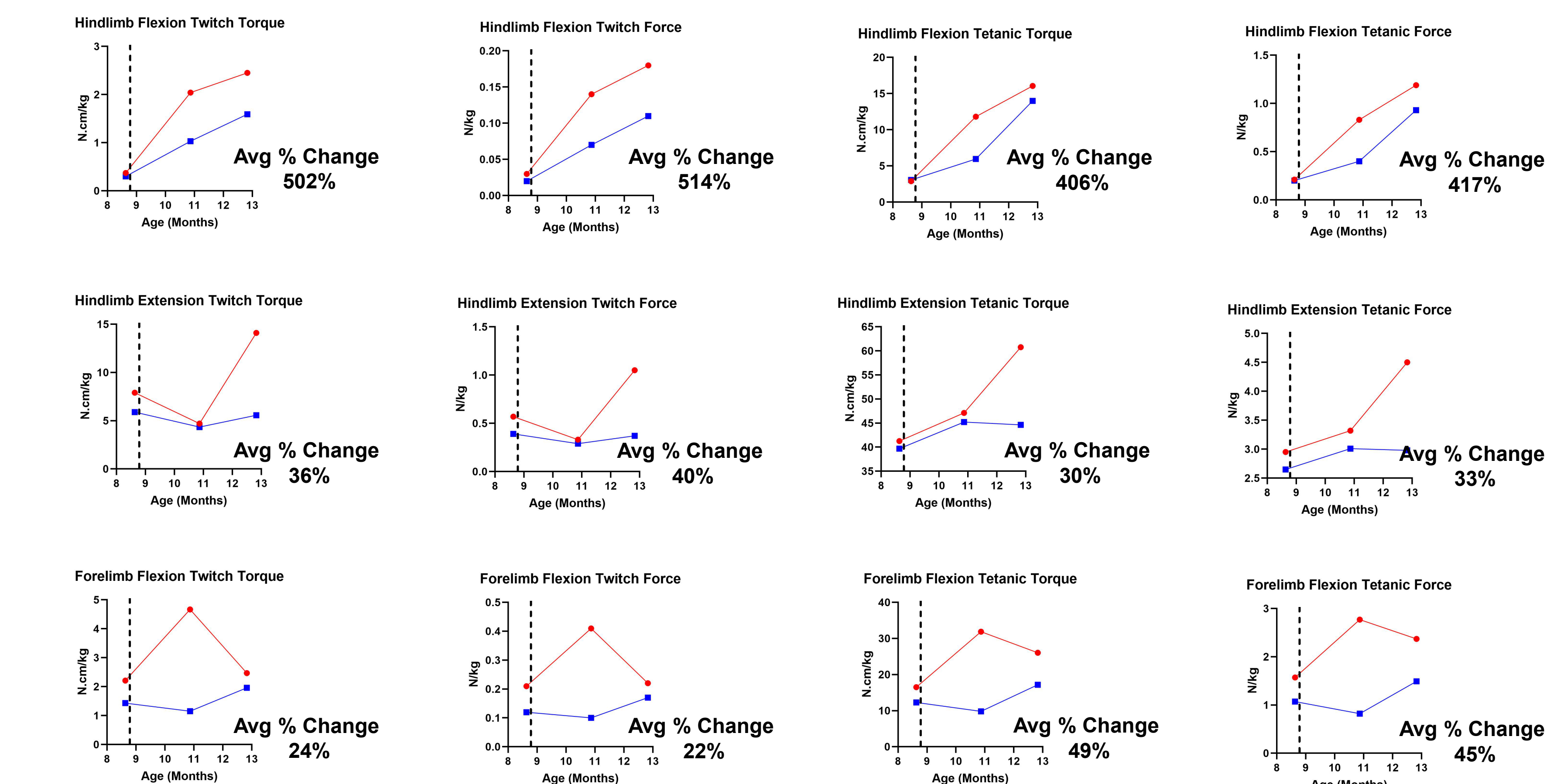
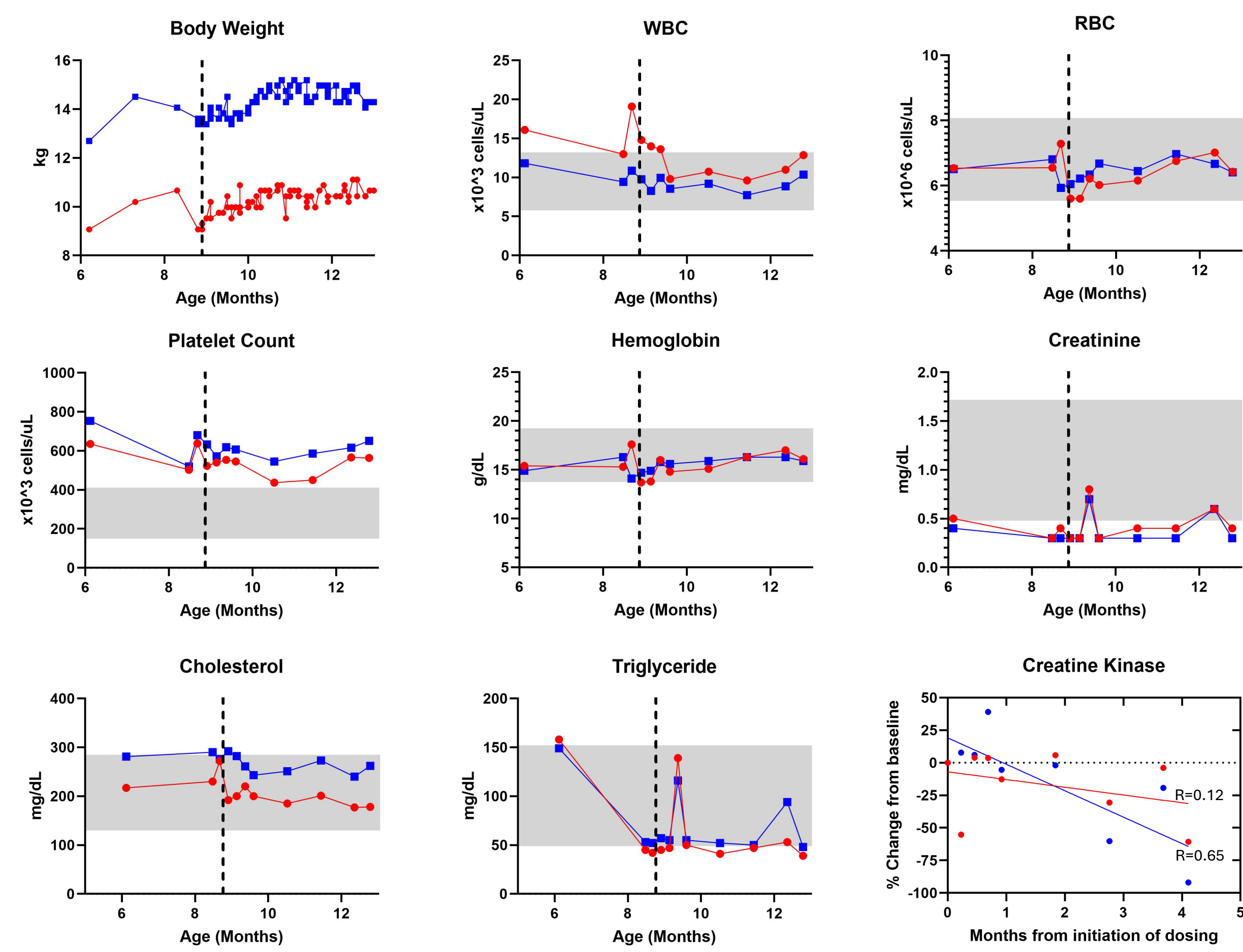
Benchmark Case 2
• Data adapted from Birch et al. 2023
• Untreated GRMD (black line) and high dose uDys treated GRMD - treated at 3m of age
• SAT-3247 treated dogs plotted as an overlay - treated at ~9m of age

$$\text{Regenerative Index (RI)} = \frac{\text{New/newly forming fibers (eMHC)}}{\text{Dead/dying fibers (IgG)}}$$

Increasing RI suggests improved regeneration

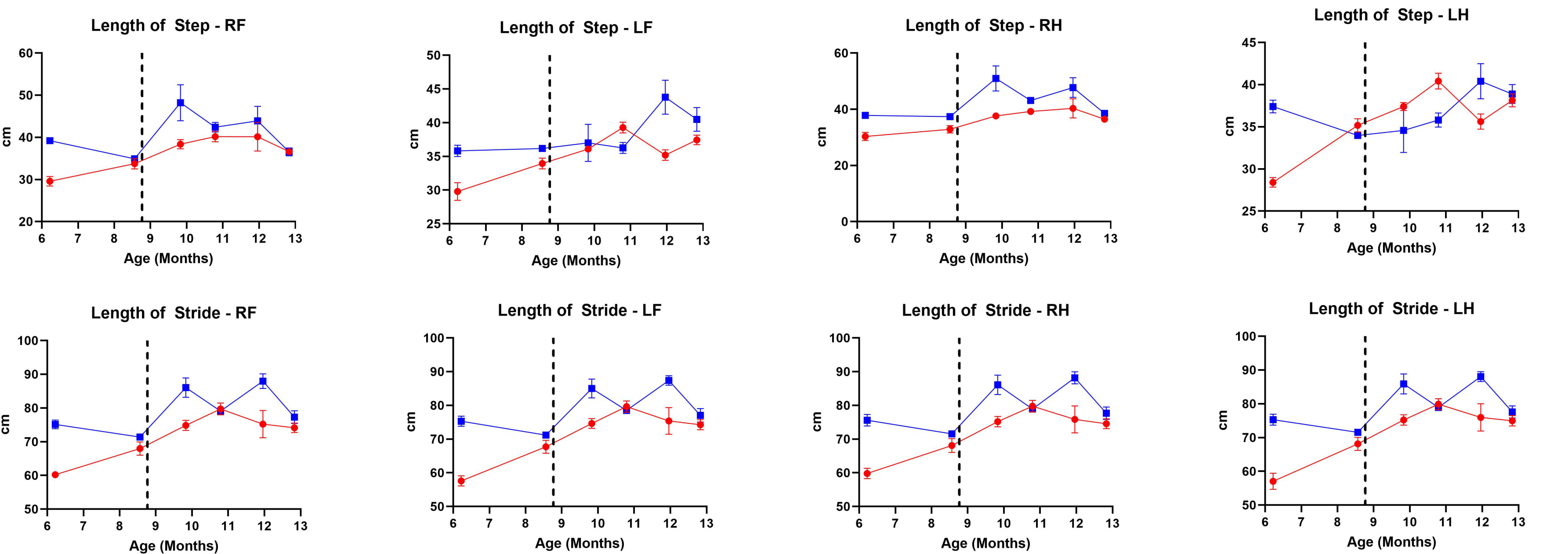
Results

Body weight, Clinical Chemistry & Hematology

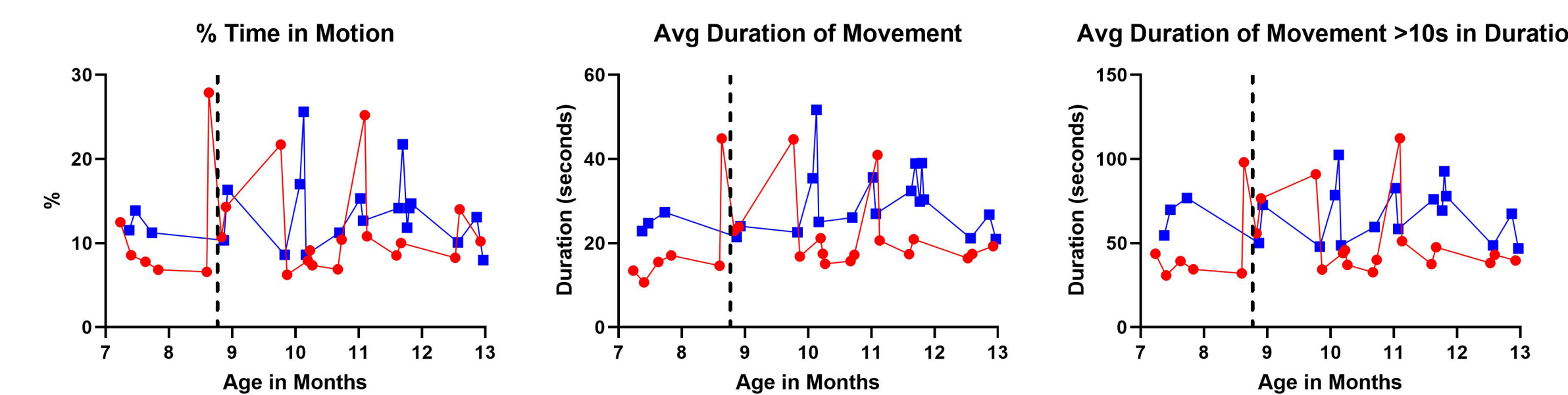


Gait Analysis

Parameter	Definition
Distance Traveled	measured on the horizontal axis from the paw center of the first paw strike to the center of the last paw strike.
Velocity	Distance/travel time
LF RF	Left Right Forelimb
LH RH	Left Right Hindlimb
Length of Step	heel center of the current paw to the previous paw print on the opposite foot (e.g. left front to right front, right hind to left hind)
Length of Stride	heel points of two consecutive paw prints of the same paw (i.e. left front to left front, right hind to right hind).



Overnight Activity



Next Steps

First in man studies with SAT-3247 are scheduled for Q3 2024. A Phase 2 PoC study in Duchene is being planned for 2025. Parties interested in learning more about SAT-3247 or Satellos Bioscience are encouraged to visit www.Satellos.com. For more information or questions regarding this poster presentation please contact Ryan Mitchell (contact details in top left)

Disclaimers & Acknowledgements:

Ryan Mitchell is a paid employee of Satellos Bioscience. Satellos is a publicly traded company. The information contained in this poster is not a solicitation to buy or sell stock. This poster may contain forward looking statements. We would like to acknowledge the Duan Lab at the University of Missouri and the Rudnicki Lab at the Ottaway Hospital Research Institute for their contributions to this work. All work was ethically conducted according to the standards set forth by each institute, and that of the NIH guidelines.