

# Results for Full Year Ended 31 January 2018 and Business Update

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April 2018



summit



The background of the slide features a photograph of four children running barefoot on a sandy beach. The scene is captured during sunset, with the sun low on the horizon, creating a warm, golden glow and long shadows. One child in the foreground is holding a white toy airplane aloft. The children are dressed in casual summer clothing like t-shirts and shorts. The overall mood is joyful and carefree.

# Forward-Looking Statements

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# PhaseOut DMD: A Phase 2 Proof of Concept Trial of Ezutromid

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<b>Design:</b>	Open label, 48-week trial with extension phase
<b>Subjects:</b>	40 ambulatory DMD boys, fully enrolled 5-10 years old
<b>Trial Sites:</b>	9 US 7 UK
<b>Endpoints:</b>	<i>Primary:</i> - Magnetic resonance parameters (leg) - Safety <i>Secondary:</i> - Biopsy parameters <i>Exploratory:</i> - Range of functional endpoints
<b>Data:</b>	24-week data reported Q1 2018 Top-line data from full trial expected Q3 2018

# Overview

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- The 24-week interim PhaseOut DMD data are highly encouraging
- We look forward to the top-line data from the full trial in Q3 2018 with much excitement
- Given the promising findings after 24 weeks of treatment, we are advancing preparatory activities for a placebo controlled clinical trial of ezutromid, and for a potential regulatory filing based on the full trial results

# PhaseOut DMD Interim Data: Key Findings after 24-Weeks of Ezutromid Treatment

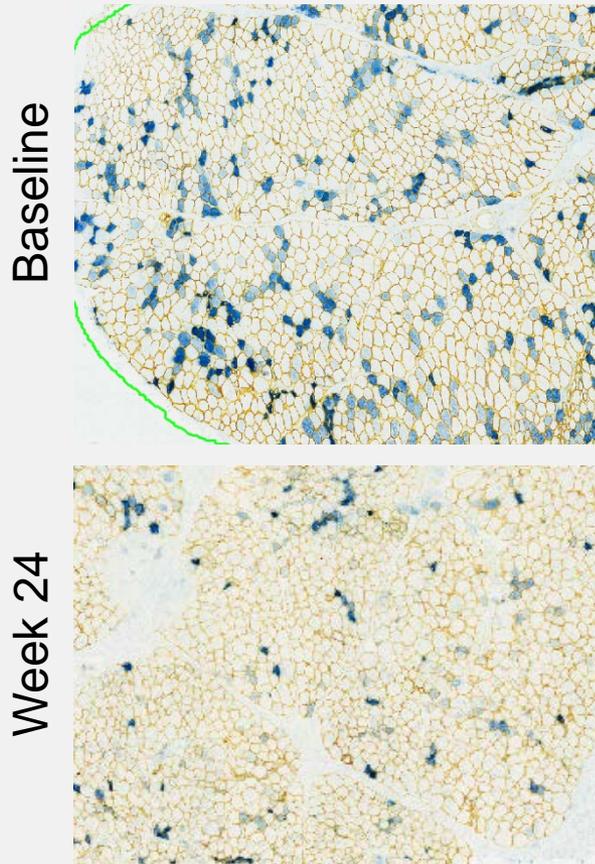
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1. Stabilization of muscle membranes

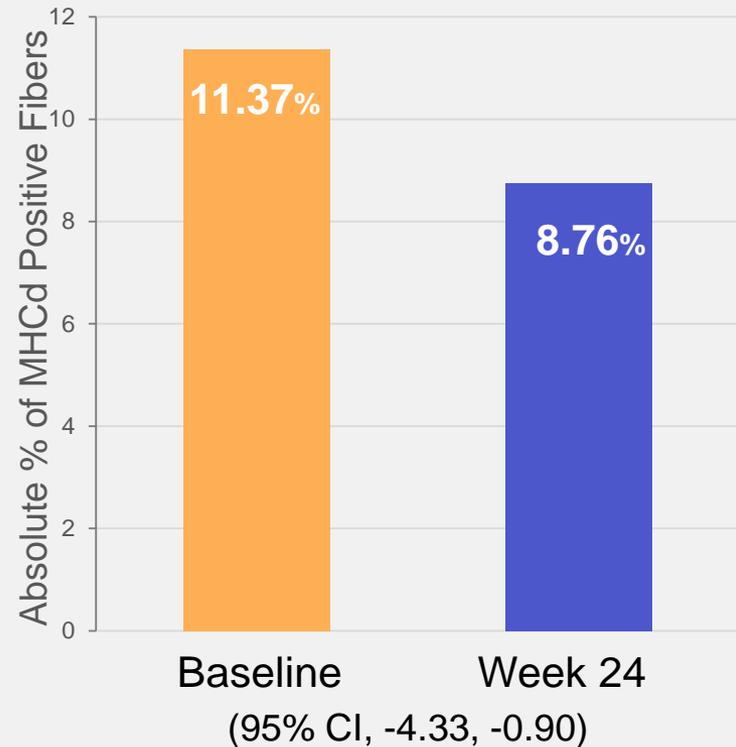
2. Significant and meaningful decrease in muscle damage

3. Significant decrease in muscle inflammation

# Significant and Meaningful Decrease in Muscle Damage as Measured by Developmental Myosin

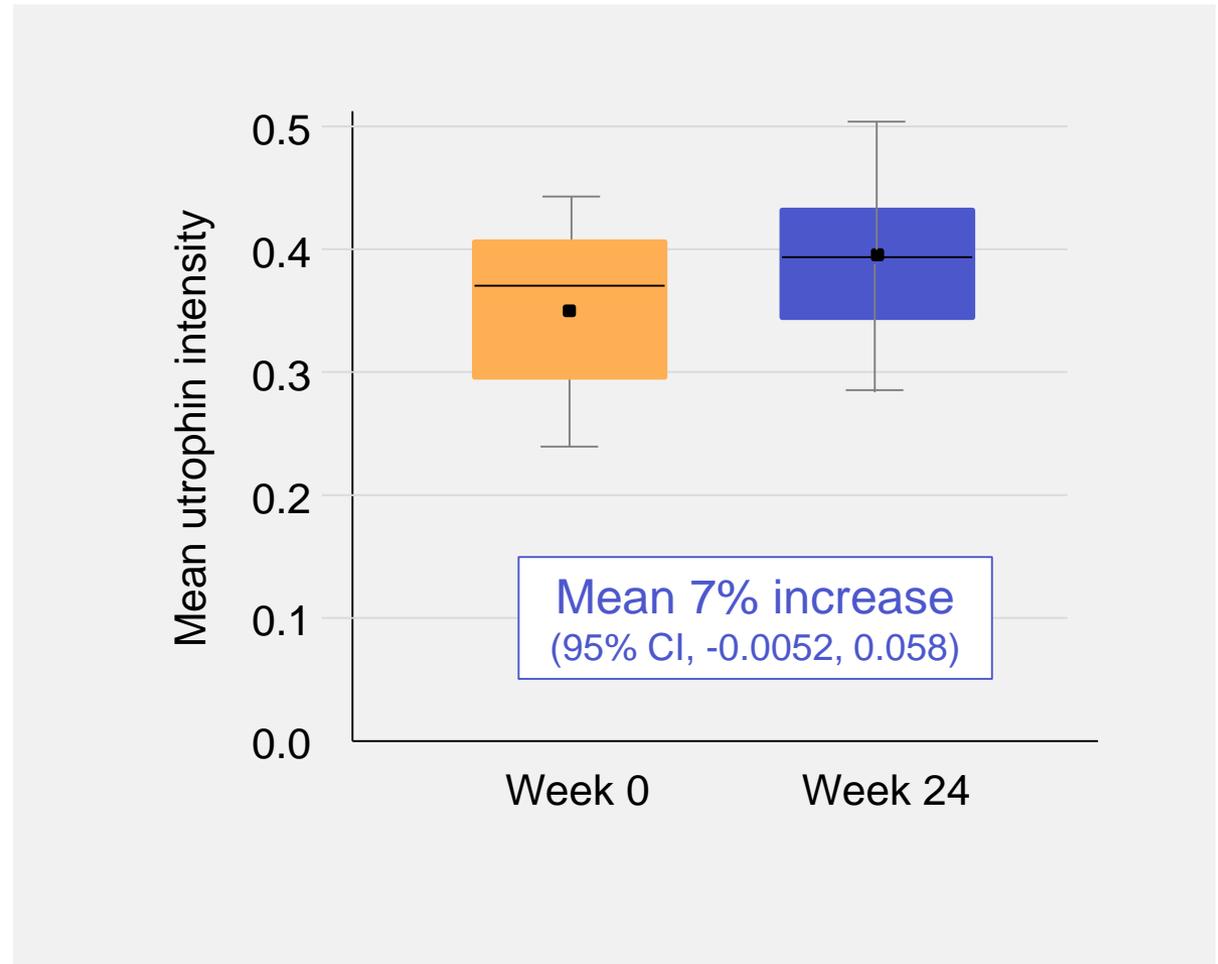


23% decrease in mean developmental myosin (MHCd) after 24 weeks



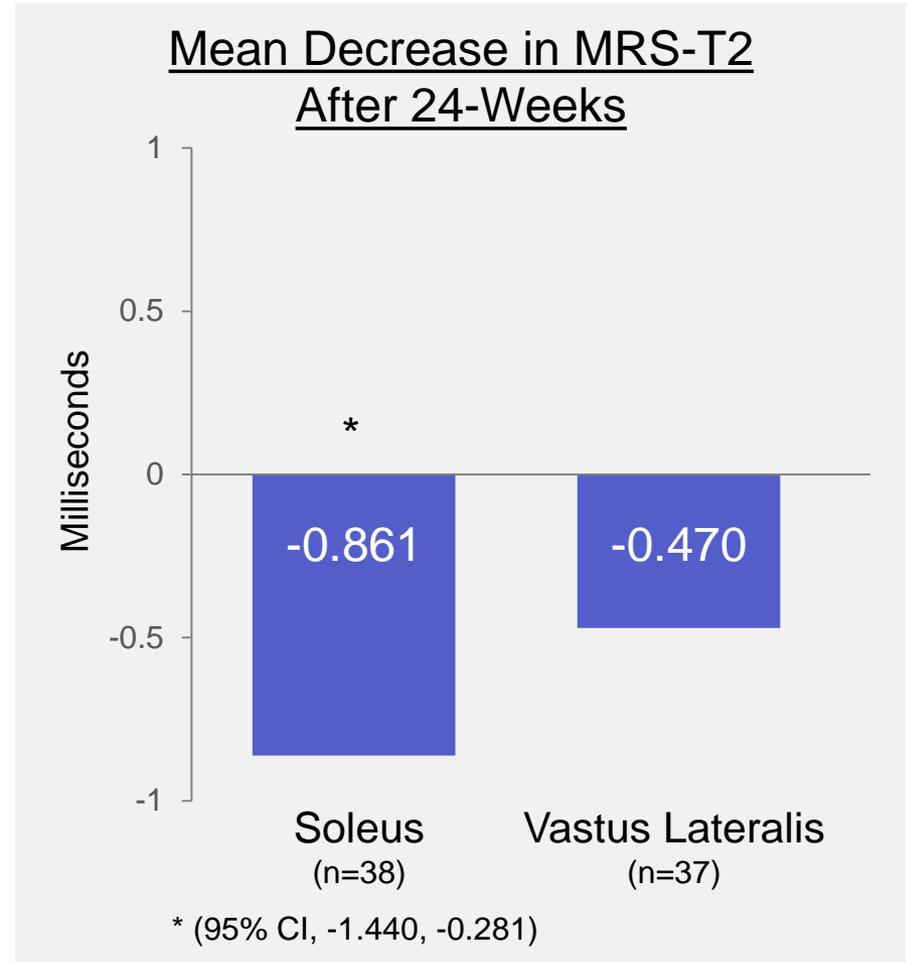
# Stabilization of Muscle Membranes as Measured by Maintenance of Utrophin

- > DMD patients have high levels of utrophin due to cycle of damage and repair
- > Mean increase in utrophin intensity after 24-weeks of ezutromid treatment



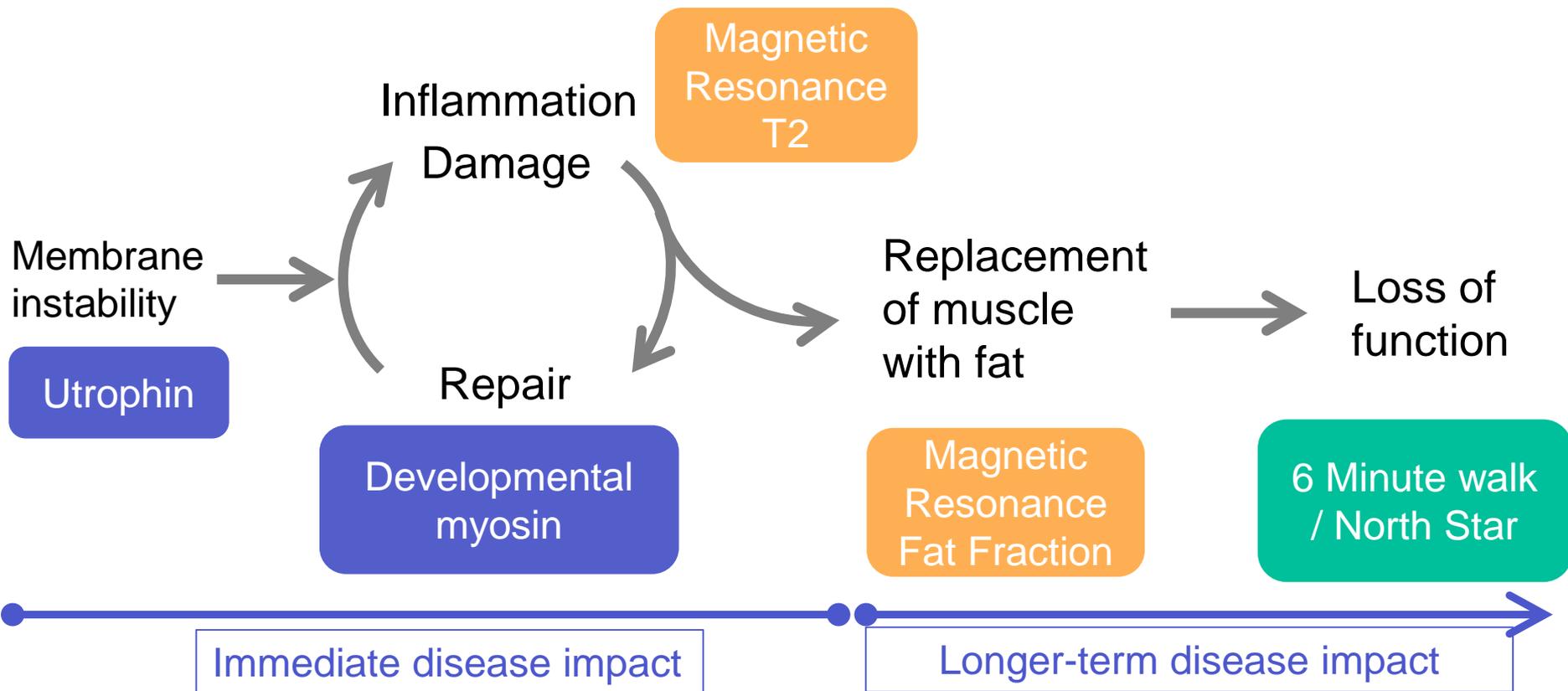
# Significant Decrease in Muscle Inflammation as Measured by MRS-T2

- MRS-T2 is a precise technique for measuring muscle damage and inflammation
- Statistically significant reduction in inflammation in the soleus (calf)
- Achieved in patients on stable steroids



Evidence of early impact of ezutromid on downstream muscle health

# Endpoints in PhaseOut DMD Track Impact of Ezutromid Over Time



# Expectations for Top-Line Results from Full PhaseOut DMD Clinical Trial

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Primary  
Endpoint

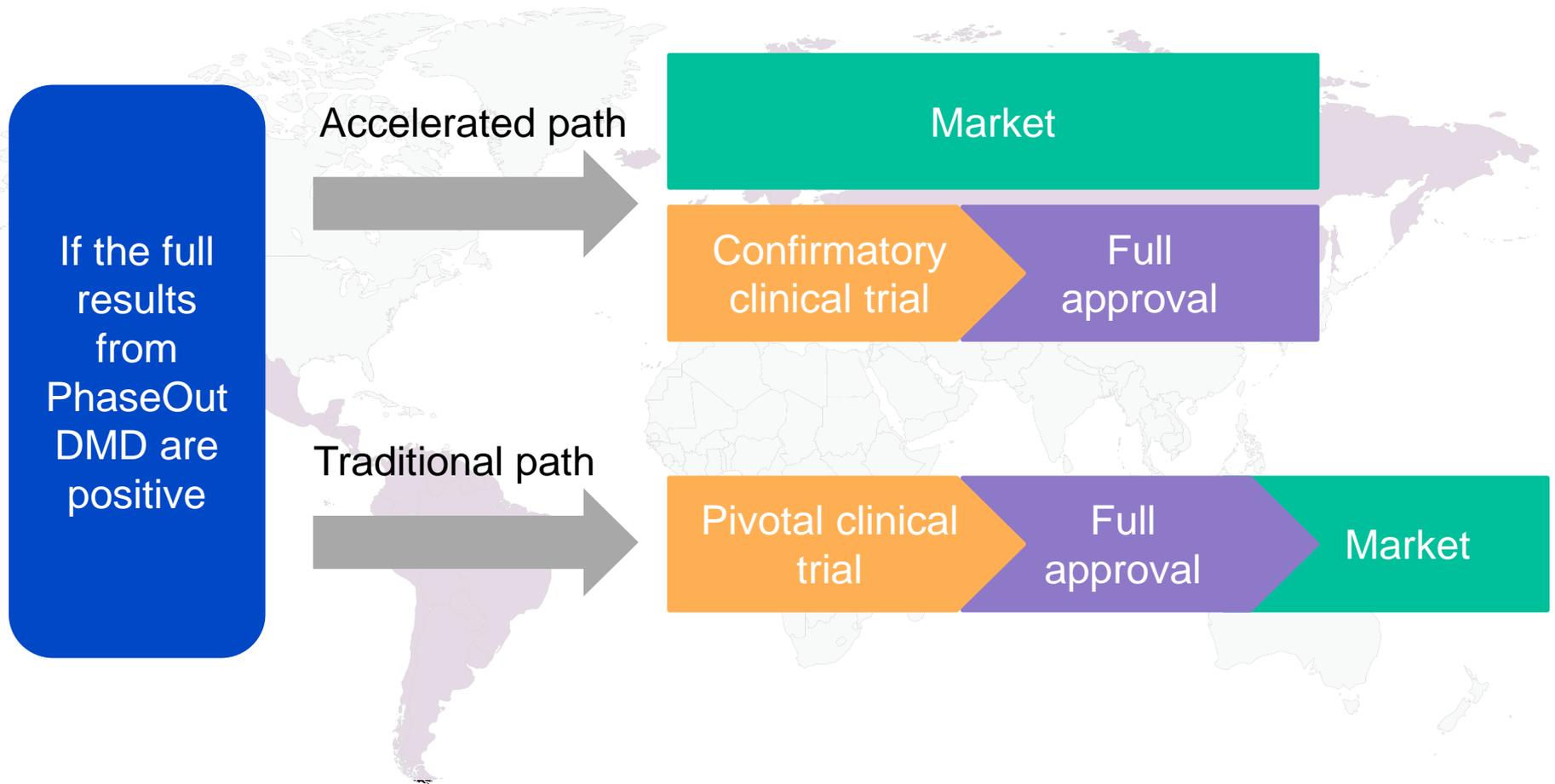
## Magnetic resonance parameters:

- **T2** – sustained decrease relative to baseline  
*-or-*
- **Fat Fraction** – slowing of disease trajectory

Secondary  
Endpoints

**Developmental myosin** – sustained reduction  
**Utrophin** – maintenance of utrophin

# Potential Scenarios to Reach Market



# Summary

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- The 24-week interim PhaseOut DMD data highly encouraging
  - We believe the data provide evidence of utrophin modulation which in turn reduced muscle damage, which in turn reduced muscle inflammation
- We look forward to the top-line data from the full trial in Q3 2018 with much excitement
  - In these data, we expect to see continued utrophin modulation and sustained changes in magnetic resonance parameters
- Given the promising findings after 24 weeks of treatment, we are advancing preparatory activities for a placebo controlled clinical trial of ezutromid, and for a potential regulatory filing based on the full trial results