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Summit Overview

Publicly traded company located in the UK (Abingdon) and US (Cambridge, MA)

Technology pioneered by Prof. Kay Davies, University of Oxford, who identified utrophin and utrophin modulation as a universal treatment approach for DMD

Utrophin modulation:

> Potential to slow or stabilize disease progression in all patients with DMD
> Independent of dystrophin gene mutation
> Could be complementary to other approaches to DMD

> Exclusive license and collaboration agreement granting Sarepta Therapeutics Inc. European rights to Summit’s utrophin modulator pipeline
Dystrophin and Utrophin Look and Act Similarly in Muscles

Dystrophin

- Actin Binding
- H1
- H2
- Rod domain
- H3
- H4
- CRD
- CTD

Utrophin

- Actin Binding
- H1
- H2
- Rod domain
- H3
- H4
- CRD
- CTD

Muscle cell membrane

Dystrophin or utrophin protein
But They do so at Different Times in Muscle Development

- Utrophin is present in early developing fibers and repairing muscle fibers; dystrophin is present in mature muscle fibers
Muscles Affected by Duchenne Naturally Make Utrophin in Early Development and Fiber Repair

> Without dystrophin, muscle fibers are easily damaged and when the muscle fiber begins the natural repair process, utrophin is turned on again.
Utrophin Modulation Aims to Keep Natural Production of Utrophin Turned on in All Muscle Fibers

- Modulation of utrophin protein has potential to compensate for lack of dystrophin
Ezutromid:
Oral Utrophin Modulator in a Phase 2 Clinical Trial
Ezutromid Clinical Program Aimed to Answer Key Questions

Phase 1
• Is ezutromid well-tolerated and suitable for future testing? (Well tolerated in ~100 healthy volunteers and 22 individual patients with DMD)

Phase 2 (PhaseOut DMD)
• Can ezutromid modulate utrophin in patients with DMD?
• Can ezutromid have a positive effect on biomarkers of muscle structure and health?

Future trials
• Over the long term, does ezutromid have positive effects on muscle function?
• Does ezutromid have positive effects on Quality of Life measures?
PhaseOut DMD:
A Phase 2 Proof of Concept Trial of Ezutromid

<table>
<thead>
<tr>
<th>Design:</th>
<th>Open label, 48-week trial with extension phase</th>
</tr>
</thead>
<tbody>
<tr>
<td>Subjects:</td>
<td>40 ambulatory DMD boys, fully enrolled</td>
</tr>
<tr>
<td></td>
<td>5-10 years old</td>
</tr>
<tr>
<td>Trial Sites:</td>
<td>9 US</td>
</tr>
<tr>
<td></td>
<td>7 UK</td>
</tr>
<tr>
<td>Endpoints:</td>
<td>Primary: leg magnetic resonance spectroscopy parameters</td>
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<tr>
<td></td>
<td>Secondary: biopsy parameters</td>
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<tr>
<td></td>
<td>Exploratory: range of functional endpoints</td>
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<tr>
<td></td>
<td>Other: safety and pharmacokinetics</td>
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<tr>
<td>Data:</td>
<td>24-week data reported Q1 2018</td>
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<td></td>
<td>48-week data expected Q3 2018</td>
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</tbody>
</table>
Endpoints in PhaseOut DMD
Track DMD Disease Progression Over Time

Inflammation → MRS-T2 → Damage → Repair → Developmental myosin

Replacement of muscle with fat → Loss of function

Immediate disease impact → Longer-term impact of disease

MRS Fat Fraction → 6 Minute walk / North Star
PhaseOut DMD Interim Data: Key Findings after 24-Weeks of Ezutromid Treatment

1. Stabilization of muscle membranes
   - Background utrophin levels high in DMD patients due to muscle damage
   - Ezutromid maintained utrophin expression with a mean increase of 7% observed in muscle biopsies

2. Significant decrease in muscle damage
   - Developmental myosin is a muscle damage biomarker with higher levels shown to correlate with disease severity
   - Ezutromid statistically, meaningfully decreased developmental myosin (23%)

3. Significant decrease in muscle inflammation
   - MRS-T2 increases in DMD patients over time; natural history shows increasing MRS-T2 correlates with loss of functional ability
   - Ezutromid significantly decreased inflammation as measured by MRS-T2
Significant Reduction in Muscle Damage after 24 Weeks of Ezutromid Treatment

Example biopsies taken from a single patient and evaluated for amount of damage/repair via developmental myosin at baseline and week 24

> Significant reduction in muscle damage observed
Reduction in Muscle Damage in PhaseOut DMD is Meaningful; Supported by Validation Studies

A reduction in the percentage of developmental myosin positive fibers correlates with a reduction in disease severity as determined by validation work assessing DMD, BMD and control biobank muscle biopsy samples.

Data from Summit’s validation work using biobank muscle biopsy samples

From Summit’s validation work using biobank samples
Significant Reduction in Muscle Inflammation After 24 Weeks of Ezutromid Treatment

Mean Absolute Decrease from Baseline in MRS-T2

* (95% CI, -1.440, -0.281)

Evidence of early impact of ezutromid on downstream muscle health
PhaseOut DMD: Additional Interim 24-Week Measures

> All patients retained ambulation after 24 weeks of treatment
> Ezutromid has been well tolerated to date
> Regardless of formulation, all patients achieved plasma levels of ezutromid sufficient to modulate utrophin with no apparent difference in safety or muscle parameters between the formulations
Positive Interim 24-Week Data Show Ezutromid Activity in PhaseOut DMD; Other Changes Expected Over Time

Inflammation → Damage → Repair → Developmental Myosin

MRS-T2

Replacement of muscle with fat → Loss of function

MRS Fat Fraction

Immediate disease impact → Longer-term impact of disease

6 Minute walk / North Star
What’s Next?

**PhaseOut DMD**
- Ongoing trial
- Positive interim 24-week data reported
- Top-line 48-week data expected to be reported Q3 2018
- Extension phase could provide valuable long term safety and efficacy data

**Pivotal Trial**
- Data could support an accelerated or conditional approval pathway
- Design will be influenced by PhaseOut DMD
Keep in Touch

> Sign up for utrophin modulator clinical trial news at [www.utrophintrials.com](http://www.utrophintrials.com)

Utrophin Modulation in DMD

Utrophin modulation is being evaluated for its potential to slow or stop the disease progression in all boys and men with Duchenne muscular dystrophy (DMD).

Summit Therapeutics is currently conducting clinical trials in patients using this approach. This site is intended for patients and families to find out more information about utrophin modulation and associated clinical trials.

[View our clinical trials](#)
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