The Phase 3 PolarisDMD Trial of Edasalonexent Enrolling Young Boys with Duchenne and GalaxyDMD, an Open-Label Extension Trial

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Duchenne Qualitative Research Findings

Learning from Qualitative Research with Physicians and Members of the Duchenne Community Regarding Their Expectations for Future Treatments

Excitement
The large number of treatments in development could mean **all boys** will benefit from a tailored treatment, not just a select few with specific mutations

"I think the future is definitively positive because having more options and different actions means we can better treat more boys." **(Physician treating Duchenne patients)**

"In the near future … it could be even I would get away from steroids." **(Physician treating Duchenne patients)**

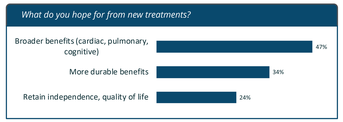
Expectation
Combination therapy will be the norm – for example combining genetic and non-genetic therapies – bringing **broader benefits**
Physicians predict that **6 in 10 boys will receive combination therapy** within the next few years

"I would have my patients on every medicine that is available and that they are eligible for… I would have patients on medicines from different therapeutic categories." (Key opinion leader)

29 members of the DMD community, including key opinion leaders, physicians that treat Duchenne patients, parents and representatives from patient advocacy groups contributed to a blinded qualitative research project.

Physicians and Members of the Duchenne Community Hope that New Treatments Will Broaden the Benefits of Therapy

Hope
New treatments will go beyond benefiting skeletal muscle to positively impacting **additional important aspects of DMD**:

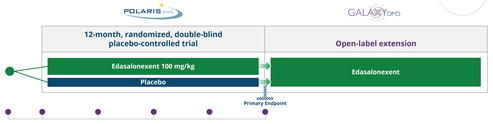


"A treatment that addresses multiple aspects of the disease would be ideal." **(Physician treating Duchenne patients)**

"To continue to climb stairs, to continue to walk or play, it's those things that you take for granted when you have an able body child." **(Parent)**

Background & Study Design

Phase 3 PolarisDMD Trial Design and Schedule



- Enrolling ~125 boys ages 4 to 7 (up to 8th birthday)
  - Not on corticosteroids for at least 6 months
- 2:1 randomization, 67% of boys receive drug initially, all boys may continue to receive drug after completing PolarisDMD through GalaxyDMD
- Clinical trial site visits and key assessments every 3 months
- Safety measures including labs every 3 months
- Trial overseen by Data Safety Monitoring Board

Edasalonexent: Potential to Slow Disease Progression for All Those Affected by Duchenne

- Being developed as a new oral foundational therapy for all affected by Duchenne, regardless of mutation type, from time of diagnosis throughout their lifetime
- Being developed for treatment alone and potential to be combined with dystrophin-targeted therapies
- In Phase 2 MoveDMD trial and open-label extension, edasalonexent substantially slowed disease progression compared to off-treatment control period
- Edasalonexent is an investigational agent not currently approved in any territory

PolarisDMD Trial Designed with Input from the Duchenne Community

- **Designed the trial with input from advocacy organizations, families, physicians**
  - Understand the burden of clinical trial participation
- **NSAA and additional endpoints are measures that reflect everyday life**
  - Standing up from the ground, walking, climbing stairs

Additional Assessments Include Growth, Cardiac and Bone Health Measures

Growth
- Monitoring height and weight to assess how boys are growing relative to their expected growth curves

Heart
- Monitoring with an easy to wear at-home small adhesive device at baseline, 6 and 12 months
- Will be analyzed for changes in heart rate as well as heart rate variability

Bone
- X-rays of the spine at baseline and after one year of treatment
- Bone mineral density by DXA at baseline and after one year of treatment

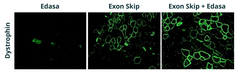
We Plan to Investigate the Potential for Co-Administration of Edasalonexent with Dystrophin-Targeted Therapies

Edasalonexent in combination with exon-skipping increased dystrophin expression in *mdx* mice

- Activated NF-κB increases the expression of several microRNAs that suppress dystrophin production
- Inhibiting NF-κB may enhance dystrophin expression in combination with dystrophin-targeted therapies in DMD

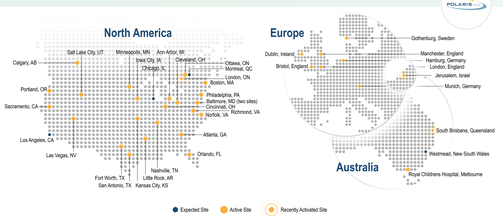
In eligible boys in the MoveDMD open-label extension, treatment of edasalonexent with EXONDYS 51® (exon skipping) was well tolerated

Boys on EXONDYS 51 are eligible for Phase 3 PolarisDMD and GalaxyDMD trials



Presented at the 22nd International Annual Congress of the World Muscle Society, 2017

Many Clinical Trial Sites to Improve Patient Access



Sites active and enrolling patients globally

GalaxyDMD Open-Label Extension

Launching New GalaxyDMD Trial for Boys Receiving Open-Label Edasalonexent

- **GalaxyDMD is enrolling boys from MoveDMD® open-label extension and provides an opportunity for open-label edasalonexent after completing 1-year PolarisDMD trial**
- **Ongoing monitoring with patient visits every 6 months**
  - Assessments of muscle function:
    - North Star Ambulatory Assessment
    - Timed Function Tests
  - Long term safety including growth and bone health

GalaxyDMD Focuses on Long-Term Safety and Allows Sibling Participation

- **Primary focus is evaluation of edasalonexent long-term safety with site visits every 6 months**
- **Participants from MoveDMD trial transitioning to GalaxyDMD**
  - Boys have received edasalonexent for 2+ years, average age ~9
- **Once boys from MoveDMD and PolarisDMD enter GalaxyDMD, there will also be an opportunity for their eligible brothers to join**
  - May also be eligible if receiving approved exon-skipping therapies

GalaxyDMD Inclusion and Exclusion Criteria

- **Inclusion: completion of the MoveDMD or PolarisDMD study**
- **For siblings of boys who completed MoveDMD or PolarisDMD study:**
  - Inclusion: Genetic diagnosis of Duchenne, age 4-10 (up to 11th birthday)
  - Exclusion: Use of investigational drug or growth hormone, on corticosteroids during previous 24 weeks

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