Safety Analysis Following Real-World Use of Delandistrogene Moxeparvovec for Duchenne Muscular Dystrophy (DMD): Interim Analysis of the Phase 4 ENDURE Study

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Background

- The phase 4, prospective observational ENDURE study (NCT06270719) measures the long-term effectiveness and safety of delandistrogene moxeparvovec compared with standard of care (SOC), for boys with Duchenne muscular dystrophy (DMD) in the United States (US)
- DMD is a rare, progressive neuromuscular disease caused by pathogenic variants in the *DMD* gene, leading to the absence of functional dystrophin protein.[1,2]
- Delandistrogene moxeparvovec is a recombinant adeno-associated virus (AAV) vector-based gene transfer therapy that delivers a transgene encoding delandistrogene moxeparvovec micro-dystrophin.[3-5] It is approved in the US and in other select countries for the treatment of ambulatory patients with DMD aged ≥4 years who have a confirmed *DMD* gene mutation
- Acute liver injury (ALI) is a recognized adverse event (AE) requiring close safety monitoring for patients treated with AAV vector-based gene therapies[6-9]
- Several real-world management strategies may mitigate liver risk, including adjunctive prophylactic immunosuppression use in addition to prescribed steroids prior to ALI development[8,10]

Objective

- This planned annual interim analysis of ENDURE describes participant characteristics and safety outcomes along with the impact of adjunctive prophylactic immunosuppression on ALI among participants receiving delandistrogene moxeparvovec

Methods

Study Design

- ENDURE is designed to prospectively collect effectiveness and safety data in boys receiving delandistrogene moxeparvovec versus SOC (chronic glucocorticoid treatment) in routine clinical care
  - Medical history, medications, and AEs are recorded in an electronic case report form. AEs are reported verbatim and queried per usual protocols for clarification and until resolution

Participant Population

- Boys with DMD who were commercially prescribed delandistrogene moxeparvovec prior to entry into the study or were receiving SOC therapy exclusive of delandistrogene moxeparvovec or other DMD gene therapies were eligible
- The ENDURE eligibility criteria align with the delandistrogene moxeparvovec USPI at the time of enrollment and subsequent updates[4,11-13]
  - Following two fatalities due to acute liver failure (ALF) in spring of 2025,[4] non-ambulatory patients with DMD are currently ineligible for delandistrogene moxeparvovec[4] and ineligible for new enrollment into ENDURE. Non-ambulatory patients enrolled when the non-ambulatory indication was in the label continue to be followed for the duration of the study

Outcomes/Analysis

- Participant demographics, baseline characteristics, treatment-emergent AEs (TEAEs), and the rate of adjudicated treatment-related ALI stratified by adjunctive prophylactic immunosuppressant use were analyzed descriptively
- Adjunctive immunosuppression included both prophylactic (defined as use of immunosuppression at any time prior to delandistrogene moxeparvovec infusion and within 14 days following infusion) and reactive (defined as use of immunosuppression for the treatment of liver injury following delandistrogene moxeparvovec infusion) immunosuppression

Results

Participant Demographics and Baseline Characteristics

- As of February 2026, 80 participants from 19 sites were enrolled, including 56 who received delandistrogene moxeparvovec (45 ambulatory and 11 non-ambulatory at baseline) and 24 who received SOC only (all ambulatory)
- The mean age at baseline was 9.4 years for ambulatory participants and 16.0 years for non-ambulatory participants treated with delandistrogene moxeparvovec, and 10.0 years for SOC participants (**Table 1**)
- Glucocorticoids were used prior to baseline in 88.9% of ambulatory participants and 100% of non-ambulatory participants treated with delandistrogene moxeparvovec, and in 91.7% of SOC participants (**Table 1**)
- In this interim analysis, sirolimus was the only adjunctive immunosuppressant (prophylactic and reactive) reported (hereafter referred to as "prophylactic or reactive sirolimus")
- Sirolimus was used prophylactically at 3 sites, in 9 (20.0%) ambulatory participants and 2 (18.2%) non-ambulatory participants treated with delandistrogene moxeparvovec (**Table 1**)
- Sirolimus was used reactively to manage a liver injury at 3 sites in 3 (6.7%) participants (all ambulatory) treated with delandistrogene moxeparvovec (**Table 1**)

Table 1 *Demographics and Baseline Characteristics*

Characteristic	Delandistrogene moxeparvovec				Standard of care
	Baseline ambulatory N=45		Baseline non-ambulatory N=11		Baseline ambulatory N=24
	Prophylactic sirolimus N=9	No prophylactic sirolimus N=36	Prophylactic sirolimus N=2	No prophylactic sirolimus N=9	
Age at baseline, years[a]					
Mean (SD)	10.1 (2.9)	9.3 (3.2)	17.5 (2.3)	15.7 (4.5)	10.0 (3.6)
Median (range)	10.0 (6.3-15.8)	9.8 (4.2-15.8)	17.5 (15.9-19.1)	15.3 (10.6-23.2)	9.6 (4.3-16.2)
Ethnicity, n (%)					
Hispanic or Latino	1 (11.1)	14 (38.9)	0 (0)	0 (0)	8 (33.3)
Not Hispanic or Latino	7 (77.8)	21 (58.3)	2 (100)	8 (88.9)	16 (66.7)
Unknown	1 (11.1)	1 (2.8)	0 (0)	1 (11.1)	0 (0)
Weight, kg					
Mean (SD)	42.5 (12.6)	30.5 (12.4)	78.9 (19.9)	52.7 (14.7)	32.4 (11.2)
Missing, n (%)	1 (11.1)	2 (5.6)	0 (0)	1 (11.1)	0 (0)
BMI, kg/m^2					
Mean (SD)	23.8 (4.3)	19.5 (4.1)	-	27.4 (4.5)	20.5 (4.9)
Missing, n (%)	1 (11.1)	3 (8.3)	2 (100)	7 (77.8)	1 (4.2)
Glucocorticoid use prior to baseline, n (%)	8 (88.9)	29 (80.6)	2 (100)	9 (100)	22 (91.7)
Prednisone	8 (88.9)	19 (52.8)	1 (50.0)	5 (55.6)	13 (54.2)
Deflazacort	1 (11.1)	15 (41.7)	2 (100)	7 (77.8)	17 (70.8)
Vamorolone	1 (11.1)	4 (11.1)	1 (50.0)	0 (0)	3 (12.5)
Sirolimus use, n (%)	9 (100)	3 (8.3)	2 (100)	0 (0)	0 (0)
Prophylactic	9 (100)	0 (0)	2 (100)	0 (0)	0 (0)
Reactive	0 (0)	3 (8.3)	0 (0)	0 (0)	0 (0)
Select medical history, n (%)					
ADHD	0 (0)	5 (13.9)	1 (50.0)	2 (22.2)	6 (25.0)
Autism spectrum	1 (11.1)	5 (13.9)	0 (0)	2 (22.2)	4 (16.7)
Cardiomyopathy	2 (22.2)	4 (11.1)	1 (50.0)	2 (22.2)	5 (20.8)
Restrictive pulmonary disease	0 (0)	3 (8.3)	1 (50.0)	6 (66.7)	6 (25.0)

[a]Baseline was defined as the time of infusion for the delandistrogene moxeparvovec cohorts and the last baseline assessment date within 60 days of enrollment for the standard of care cohort. ADHD, attention-deficit/hyperactivity disorder; BMI, body mass index; SD, standard deviation.
Note: A subset of participants had laboratory assessments collected outside of the ENDURE protocol, and these data are reported elsewhere.[14]

Table 2 *Treatment-Related TEAEs of Liver Injury With Delandistrogene Moxeparvovec*

TEAEs, n (%)	Baseline ambulatory N=45				Baseline non-ambulatory N=11			
	Prophylactic sirolimus N=9		No prophylactic sirolimus N=36		Prophylactic sirolimus N=2		No prophylactic sirolimus N=9	
	Any	Serious	Any	Serious	Any	Serious	Any	Serious
Any treatment-related TEAE of liver injury (adjudicated)	**0 (0)**	**0 (0)**	**6 (16.7)**	**5 (13.9)**	**0 (0)**	**0 (0)**	**3 (33.3)**	**2 (22.2)**
Hospitalization due to ALI (adjudicated)[a]	**0 (0)**	**0 (0)**	**4 (11.1)**	**4 (11.1)**	**0 (0)**	**0 (0)**	**1 (11.1)**	**1 (11.1)**
Investigations	**0 (0)**	**0 (0)**	**4 (11.1)**	**3 (8.3)**	**0 (0)**	**0 (0)**	**1 (11.1)**	**0 (0)**
Increased GGT	0 (0)	0 (0)	3 (8.3)	2 (5.6)	0 (0)	0 (0)	0 (0)	0 (0)
Increased ALT	0 (0)	0 (0)	2 (5.6)	1 (2.8)	0 (0)	0 (0)	0 (0)	0 (0)
Increased AST	0 (0)	0 (0)	2 (5.6)	1 (2.8)	0 (0)	0 (0)	0 (0)	0 (0)
Increased transaminase	0 (0)	0 (0)	2 (5.6)	1 (2.8)	0 (0)	0 (0)	0 (0)	0 (0)
Increased bilirubin	0 (0)	0 (0)	1 (2.8)	1 (2.8)	0 (0)	0 (0)	0 (0)	0 (0)
Increased hepatic enzyme	0 (0)	0 (0)	0 (0)	0 (0)	0 (0)	0 (0)	1 (11.1)	0 (0)
Hepatobiliary disorders	**0 (0)**	**0 (0)**	**3 (8.3)**	**2 (5.6)**	**0 (0)**	**0 (0)**	**2 (22.2)**	**2 (22.2)**
Liver injury	0 (0)	0 (0)	1 (2.8)	1 (2.8)	0 (0)	0 (0)	1 (11.1)	1 (11.1)
Acute hepatic failure	0 (0)	0 (0)	0 (0.0)	0 (0)	0 (0)	0 (0)	1 (11.1)	1 (11.1)
Hepatic function abnormality	0 (0)	0 (0)	1 (2.8)	1 (2.8)	0 (0)	0 (0)	0 (0)	0 (0)
Hepatotoxicity	0 (0)	0 (0)	0 (0)	0 (0)	0 (0)	0 (0)	1 (11.1)	1 (11.1)
Ocular icterus	0 (0)	0 (0)	1 (2.8)	0 (0)	0 (0)	0 (0)	0 (0)	0 (0)

[a]Acute hepatic failure is included with the ALI events leading to hospitalization.
Note: Bolded numbers for each TEAE system/organ class represent unique patients. Patients may have had more than 1 event reported for each system/organ class. Serious events are shown as a subset of any event. All terms are reported verbatim as recorded in the electronic case report form.
ALI, acute liver injury; ALT, alanine aminotransferase; AST, aspartate aminotransferase; GGT, gamma-glutamyl transferase; TEAE, treatment-emergent adverse event.

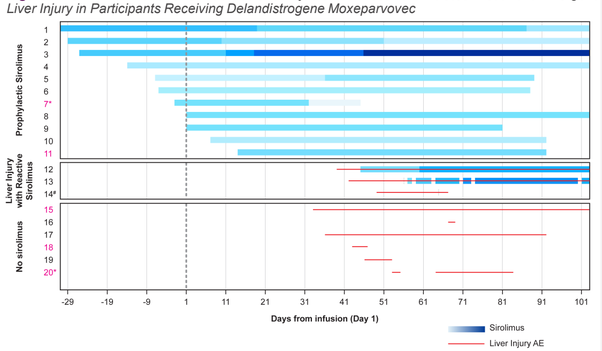
TEAEs

- Thiry-five (62.5%) participants treated with delandistrogene moxeparvovec had at least one treatment-related TEAE, the most common of which were vomiting (30.4%; 17/56) and nausea (25.0%; 14/56) (**Table S1**)
- Nine (16.1%) participants had at least one serious treatment-related TEAE (**Table S1**), seven (12.5%) of which were liver related (**Table 2**)
- Two deaths occurred in participants treated with delandistrogene moxeparvovec, one from treatment-related ALF (previously published[8]) and one from empyema/sepsis 8 months after treatment (deemed unrelated to treatment)

Prophylactic Sirolimus Use in Patients Receiving Delandistrogene Moxeparvovec

- All 9 ambulatory participants who received prophylactic sirolimus received concomitant sulfamethoxazole-trimethoprim prophylaxis
- Prophylactic sirolimus initiation ranged from 43 days before delandistrogene moxeparvovec infusion to 14 days after infusion
- Three (6.7%) baseline ambulatory participants received reactive sirolimus to manage a treatment-emergent liver injury

Figure 1 *Swimmer Plot of Sirolimus Use and Adjudicated Treatment-Related Treatment-Emergent Liver Injury in Participants Receiving Delandistrogene Moxeparvovec*



Day 1 is the day of infusion. Participants in pink were non-ambulatory at baseline. Darker blue shading represents increased sirolimus dosing to achieve appropriate trough levels.
*Participant 7 died on Day 240 after infusion due to empyema and sepsis (unrelated to study treatment). Participant 20 died on Day 84 after infusion due to ALF (related to study treatment). Both participants were non-ambulatory at baseline. #Participant 14 received one day of sirolimus.
AE, adverse event; ALF, acute liver failure; IS, immunosuppression; TEAE, treatment-emergent adverse event.

- The duration of prophylactic sirolimus use ranged from 47 to 149 days, with a median duration of 94 days in ambulatory participants and 63 days in non-ambulatory participants

Impact of Prophylactic Sirolimus on ALI

- Approximately two-thirds (64.3%; 36/56) of participants neither received prophylactic sirolimus or had a treatment-related liver injury
- Overall, treatment-related ALI occurred in 9/56 (16.1%) participants treated with delandistrogene moxeparvovec, 7 (12.5%) of which were considered serious (5 in ambulatory participants, 2 in non-ambulatory participants) (**Table 2**)
  - The onset of treatment-related ALI ranged from 33 to 53 days after infusion (**Figure 1**)
- Hospitalization occurred in 5/7 participants with serious ALI (4 ambulatory, 1 non-ambulatory) (**Table 2**)
- No ALI cases were observed in participants who received prophylactic sirolimus (**Table 2**)
- Select non-liver TEAEs potentially attributable to adjunctive immunosuppressant use, such as infections, metabolism, and hematologic disorders, only occurred in patients without prophylactic sirolimus (**Table 3**)

Table 3 *Select TEAEs Potentially Attributable to Adjunctive Immunosuppressant Use[a] (≤6 Months Post Delandistrogene Moxeparvovec)*

TEAEs, n (%)	Baseline ambulatory N=45		Baseline non-ambulatory N=11	
	Prophylactic sirolimus N=9	No prophylactic sirolimus N=36	Prophylactic sirolimus N=2	No prophylactic sirolimus N=9
Any TEAE potentially attributable to immunosuppressant use	**0 (0)**	**4 (11.1)**	**0 (0)**	**1 (11.1)**
Serious infections and infestations	**0 (0)**	**1 (2.8)**	**0 (0)**	**1 (11.1)**
COVID-19	0 (0)	1 (2.8)	0 (0)	0 (0)
Cytomegalovirus hepatitis	0 (0)	0 (0)	0 (0)	1 (11.1)
Metabolism disorders	**0 (0)**	**1 (2.8)**	**0 (0)**	**0 (0)**
Hypertriglyceridemia	0 (0)	1 (2.8)	0 (0)	0 (0)
Blood/investigations	**0 (0)**	**3 (8.3)**	**0 (0)**	**0 (0)**
Thrombocytopenia	0 (0)	1 (2.8)	0 (0)	0 (0)
Platelet count decreased	0 (0)	1 (2.8)	0 (0)	0 (0)
Neutrophil count decreased	0 (0)	1 (2.8)	0 (0)	0 (0)

[a]TEAEs were selected based on known AEs of sirolimus listed in the US prescribing information.[15]
AE, adverse event; TEAE, treatment-emergent adverse event.

Limitations

- Limitations of these interim data include the lack of recorded laboratory parameters, limited number of sites, variable sirolimus dosing, timing, and duration relative to delandistrogene moxeparvovec infusion, along with the majority of participants being enrolled from one site

Conclusions

- In this interim analysis of ENDURE, overall rates of treatment-related ALI were consistent with US post-marketing reports[16]
- Despite variable timing of sirolimus initiation relative to delandistrogene moxeparvovec infusion, prophylactic immunosuppression with sirolimus appears to mitigate risk of liver injury, with no ALI events observed among the 11 participants who received prophylaxis
- Reactive sirolimus was used to manage ALI after delandistrogene moxeparvovec infusion in only 3 patients (all ambulatory), limiting the ability to characterize its reactive use
- Real-world data from this interim analysis of the first 2 years of ENDURE show no new safety signals, and ALI frequency is consistent with prior disclosures. Preliminary findings of prophylactic sirolimus use in a limited number of patients suggest protection against ALI. Evaluation including the reporting of laboratory testing is ongoing in ENDURE

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Supplementary material

Table S1 *Treatment-Related TEAEs With Delandistrogene Moxeparvovec (Reported in ≥10% of Participants)*

TEAEs, n (%)	Delandistrogene moxeparvovec			
	Baseline ambulatory N=45		Baseline non-ambulatory N=11	
	Any	Serious	Any	Serious
Any treatment-related TEAE	**27 (60.0)**	**7 (15.6)**	**8 (72.7)**	**2 (18.2)**
Gastrointestinal disease	**18 (40.0)**	**1 (2.2)**	**6 (54.5)**	**1 (9.1)**
Vomiting	13 (28.9)	0 (0)	4 (36.4)	0 (0)
Nausea	11 (24.4)	0 (0)	3 (27.3)	0 (0)
Abdominal pain	2 (4.4)	0 (0)	0 (0)	0 (0)
Diarrhea	2 (4.4)	1 (2.2)	0 (0)	0 (0)
Ascites[a]	0 (0)	0 (0)	1 (9.1)	1 (9.1)
Investigations	**6 (13.3)**	**4 (8.9)**	**1 (9.1)**	**0 (0)**
Increased GGT	3 (6.7)	2 (4.4)	0 (0)	0 (0)
Increased ALT	2 (4.4)	1 (2.2)	0 (0)	0 (0)
Increased AST	2 (4.4)	1 (2.2)	0 (0)	0 (0)
Increased transaminase	2 (4.4)	1 (2.2)	0 (0)	0 (0)
Increased bilirubin	1 (2.2)	1 (2.2)	0 (0)	0 (0)
Increased CK	1 (2.2)	1 (2.2)	0 (0)	0 (0)
Increased hematocrit	1 (2.2)	0 (0)	0 (0)	0 (0)
Increased hemoglobin	1 (2.2)	0 (0)	0 (0)	0 (0)
Increased hepatic enzyme	0 (0)	0 (0)	1 (9.1)	0 (0)
Increased neutrophil count	1 (2.2)	0 (0)	0 (0)	0 (0)
Increased troponin	1 (2.2)	0 (0)	0 (0)	0 (0)
Metabolism/nutrition disorders	**8 (17.8)**	**1 (2.2)**	**2 (18.2)**	**1 (9.1)**
Decreased appetite	7 (15.6)	0 (0)	1 (9.1)	0 (0)
Dehydration	1 (2.2)	1 (2.2)	0 (0)	0 (0)
Hypoalbuminemia	0 (0)	0 (0)	1 (9.1)	1 (9.1)

Note: Bolded numbers for each TEAE system/organ class represent unique patients. Patients may have had more than 1 event reported for each system/organ class. Serious events are shown as a subset of any event. All terms are reported verbatim as recorded in the electronic case report form. [a]Refers to a TEAE resulting in the death of one participant. There were two total deaths (one related to treatment, one unrelated to treatment), each attributable to multiple TEAEs. ALT, alanine aminotransferase; AST, aspartate aminotransferase; CK, creatinine phosphokinase; DNA, deoxyribonucleic acid; GGT, gamma-glutamyl transferase; TEAE, treatment-emergent adverse event.

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