

Pooled Safety Analysis from Phase 1 to Phase 3 Clinical Trials of Delandistrogene Moxeparvovec in Duchenne Muscular Dystrophy

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Supplementary information

Supplementary Table 1 Laboratory abnormalities of patients meeting criteria for acute liver injury

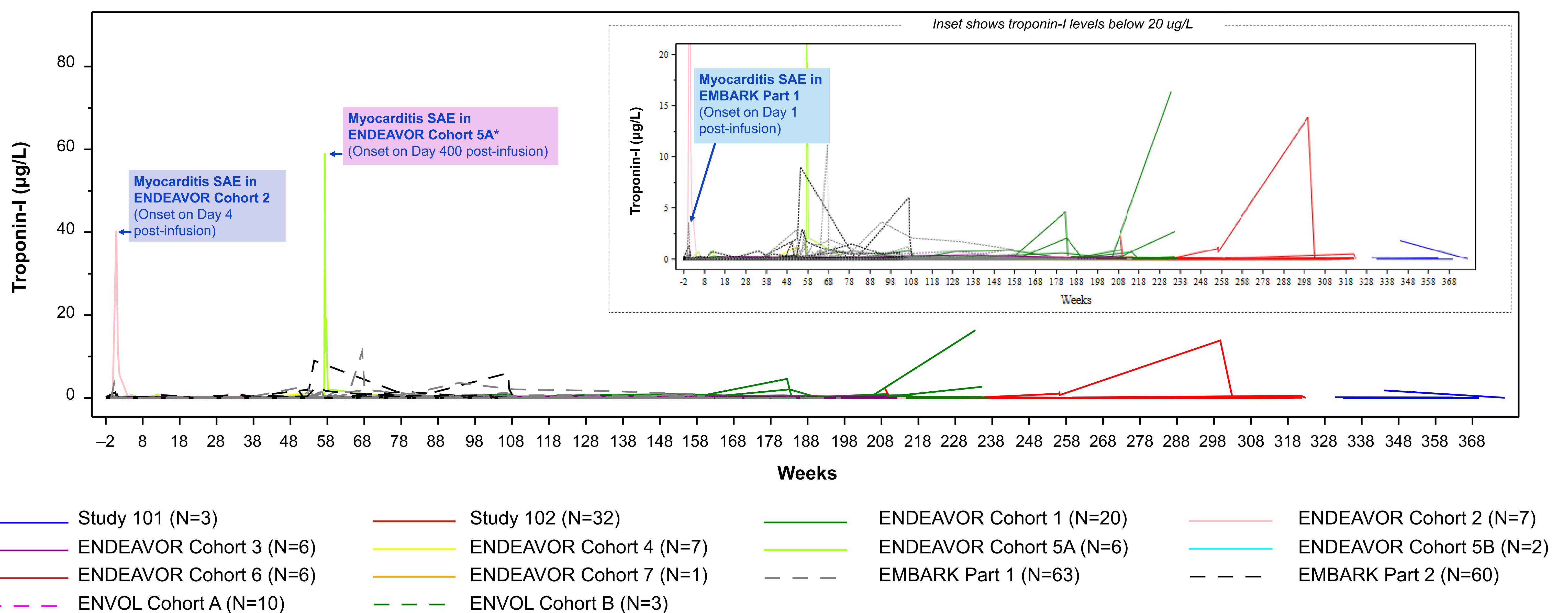
n (%)	Total patients (N=236)*
Patients meeting the definition of acute liver injury	110 (46.6)
GGT >3.0×ULN	38 (16.1)
GLDH >2.5×ULN*	100 (42.4)
ALT >3.0×BL†	27 (11.4)
ALP >2.0×ULN	1 (0.4)
Total bilirubin >2×ULN	7 (3.0)

*GLDH was not collected in Study 102 Part 1. †Excluding elevations from muscle.

Liver abnormalities of interest

- Liver function tests increased within 8 weeks after delandistrogene moxeparvovec infusion, with no clinically significant cases starting after 90 days
- Most hepatic laboratory parameters classified as AEs resolved either spontaneously or with corticosteroid treatment
- There were no instances of acute liver failure or confirmed elevations in the international normalized ratio in this pooled analysis
 - To date, there have been two treatment-related deaths due to acute liver failure approximately 3 months post-infusion in non-ambulatory patients (one in the ENVISION [Study 303] trial and one in the commercial setting) at 15 and 16 years of age¹

Supplementary Figure 1 Troponin-I levels over time



Troponin-I was not measured in Study 101 and Study 102; patients included from those studies rolled over into EXPEDITION. ULNs ranged from 0.06 µg/L for Study 101, 0.06–0.1 µg/L for Study 102, 0.06–0.4 µg/L for ENDEAVOR, 0.06–36 µg/L for EMBARK, and 0.06 µg/L for ENVOL. *One patient had two events of myocarditis with the second event associated with recurrent IMM.

Troponin-I levels

- Troponin-I levels were monitored regularly in the ENDEAVOR, EMBARK, ENVOL, and EXPEDITION studies only
- Troponin-I elevation was associated with definite, probable, or possible myocarditis SAEs that were deemed related to treatment that were observed in three patients (ENDEAVOR, n=2, 4 and 400 days post-infusion; EMBARK, n=1, 1-day post-infusion)
 - One patient experienced myocarditis following weaning of immunosuppression in the context of IMM, 400 days post-infusion²
- Fluctuations in troponin-I levels were consistent with the natural history of DMD³