Delandistrogene moxeparvovec in Duchenne Muscular Dystrophy: experience from a single center

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BACKGROUND

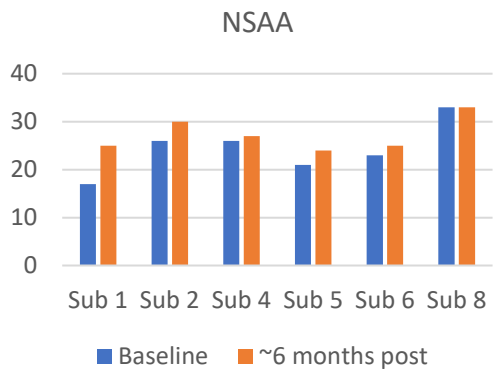
Delandistrogene moxeparvovec is the first FDA-approved microdystrophin gene therapy for Duchenne muscular dystrophy (DMD) patients above 4 years old. As this novel therapy is now commercially available, ongoing evaluation of its safety profile and treatment outcomes are essential. Here we describe our experience in a clinical cohort of DMD patients treated with delandistrogene moxeparvovec.

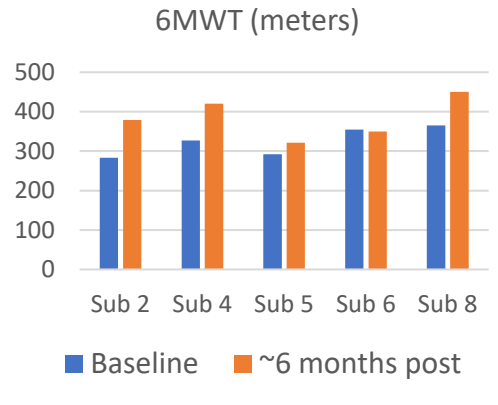
METHODS AND RESULTS

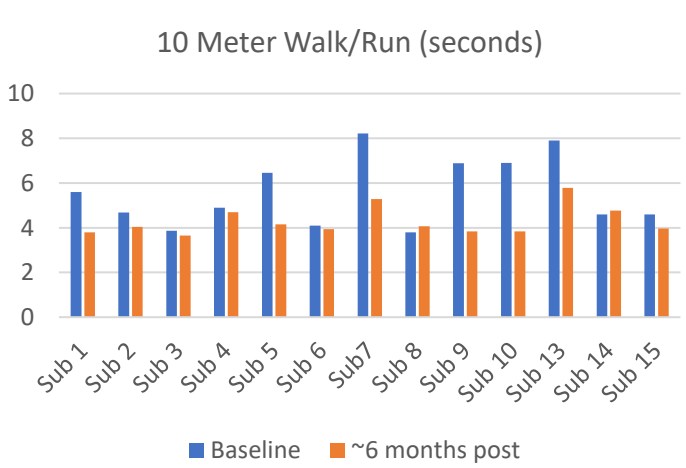
We conducted a chart review of the 21 ambulatory DMD patients who received gene therapy between 9/2023 and 8/2025. These patients had no preexisting cardiomyopathy. The data extracted included age, side effects to treatment, steroids administered, CK measurement at baseline, and motor assessments as measured by NSAA, 6MWT and 10 meter walk/run. Patients were continued on 8 weeks of 1mg/kg of prednisone/prednisolone above their baseline steroids prior to weaning. No patients experienced serious adverse events (SAE) such as myocarditis, immune mediated myositis, complement activation syndrome, symptomatic thrombocytopenia or acute liver failure. There were some patients who had a low C4 level. Maximum bilirubin was 1.0 (in 2 patients). The most common side effects included decreased appetite, nausea/vomiting, and fatigue, occasional abdominal pain, irritability, or hyperactivity. One patient experienced anaphylaxis during the infusion for which infusion had to be discontinued. No patients required additional immunomodulation beyond oral prednisone/prednisolone 2 mg/kg/day above baseline steroid dose.

#	Age at Rx (yr)	DMD Genetic variant	Steroid	CK pre treatment	Baseline 10 Meter Walk/Run (seconds)	NSAA	6MWT (meters)
1	4	del 48-50	D	41840	5.6	17	NP
2**	5	p.Lys473X* (exon 12)	P	15341	4.68	26	283
3	5	p.Arg3391Ter (exon 70)	P	27540	3.87	23	NP
4	4	del 51-54	P	19392	4.9	26	327
5	4	del 51	P	15058	6.46	21	292
6	4	del 50	P	25500	4.10	23	355
7	7	p.Trp3284Ter (exon 68)	P	17009	8.22	11	281
8	6	del 45-50	P	17290	3.8	33	365
9	4	dup 3-4 (plus muscle biopsy	P	12848	6.88	20	343
10	4	p.Arg889Ter (exon 21)	P	19554	6.9	21	386
11	8	dup 45-52	P	7893	4.1	29	354
12**	4	p.Lys473X* (exon 12)	P	29,007	6.5	15	298
13	4	del 46-50	P	14335	7.9	17	295
14	5	del 3-7	P	4956	4.59	23	338
15	6	del 46-51	P	13264	4.6	25	403
16	7	del 45	P	12928	3.84	34	400
17**	7	del 53-55	D	25460	6.62	28	305
18**	16	del 53-55	D	3954	5.27	25	338
19	13	dup 51-62	P	3866	8.46	22	368
20	6	p.Ser738* exon 18	D	31950	7.72	15	NP
21	16	del 45-50	P	3379	14.5	11	200

D = deflazacort P = prednisone/prednisolone ** = Sibling pair NP= not performed







CONCLUSIONS:

Most patients tolerated delandistrogene moxeparvovec without SAE, except anaphylaxis in one patient resulting in discontinuation of the infusion. Due to limited follow up we could not draw definite conclusions about the efficacy. The side effect profile and efficacy of delandistrogene moxeparvovec need to be monitored through real-world experience to guide its use.