Public Comment Submitted to the FDA Cellular, Tissue, and Gene Therapies Advisory Committee
Re: Deramiocel (CAP-1002) for the Treatment of Duchenne Muscular Dystrophy
Submitted by Parent Project Muscular Dystrophy (PPMD)
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Parent Project Muscular Dystrophy (PPMD) appreciates the opportunity to provide comments in support of Deramiocel for the treatment of Duchenne muscular dystrophy (Duchenne). PPMD is the leading nonprofit organization in the United States focused on Duchenne and Becker, representing a nationwide community of patients, families, clinicians, researchers, and industry partners committed to advancing treatments and improving outcomes.

Duchenne is a rare, progressive, and ultimately fatal neuromuscular disease characterized by relentless muscle degeneration affecting both skeletal and cardiac muscle. Disease progression is predictable and does not stabilize without intervention. As muscle function declines, individuals lose independence, experience increasing medical complications, and face premature mortality - most often due to cardiac or respiratory failure.

Despite meaningful clinical progress, there remains a significant unmet need for therapies that address the full scope of Duchenne, particularly for individuals who have lost the ability to walk and experience continued functional and cardiac decline. The Duchenne community urgently needs additional therapeutic options that can slow progression, preserve function, and meaningfully impact both skeletal and cardiac muscle integrity to improve patient outcomes.

Deramiocel represents a novel, cell-based therapeutic approach with a mechanism of action that is anti-inflammatory and anti-fibrotic. This multi-modal mechanism is highly relevant in Duchenne, where chronic inflammation and fibrosis drive irreversible muscle damage. Importantly, Deramiocel is designed to target both skeletal and cardiac muscle, reflecting the multi-system nature of the disease and the realities faced by patients and families.

Clinical data from the HOPE-2 and HOPE-3 programs demonstrate evidence of benefit in outcomes that matter most to patients and families. Preservation of upper limb function, as measured by Performance of the Upper Limb (PUL), is critically important for maintaining independence in non-ambulatory individuals. Retaining the ability to use one's arms and hands allows individuals to perform essential activities of daily living (ADL) such as self feeding , operating assistive devices, and engaging with their environment. Preservation of function is preservation of independence, dignity, and quality of life.

Equally important are the observed effects on cardiac function, including stabilization of left ventricular ejection fraction (LVEF). Cardiac decline is a leading cause of death in Duchenne, and interventions that stabilize cardiac function may have meaningful implications for survival. In a disease where deterioration is inevitable, stabilization represents a clinically meaningful and highly relevant outcome.

The Duchenne community's preference study data consistently emphasizes that incremental slowing of disease progression - has a profound impact on daily life. Maintaining function for longer extends independence, reduces caregiver burden, and allows individuals to remain active participants in their own lives. These outcomes align directly with the FDA's framework of assessing whether a therapy improves how patients feel, function, or survive.

All therapies must demonstrate benefits which exceed risks.Duchenne is a severe, life-limiting disease defined by continuous and irreversible decline. In this reality, expanding access to therapies that can meaningfully alter disease progression is a benefit which is not optional, it is essential.

PPMD also underscores the urgency of timely access to therapy. Duchenne progression does not pause, and lost muscle cannot be regained. Every delay represents irreversible loss of function - both skeletal and cardiac. As our community often states, "time is muscle," and in Duchenne, time is also independence and survival.

For the patients and families we represent, the question is not whether to act, but how quickly meaningful therapies can be made available. Deramiocel represents an important potential addition to the limited therapeutic landscape for Duchenne - particularly for those who continue to face ongoing decline with few options.

PPMD respectfully urges the Advisory Committee and the FDA to consider the totality of evidence, the biological rationale, and especially the lived experience and expectations of patients and families. We ask that you act with the urgency this disease demands and support the advancement of Deramiocel as a meaningful therapeutic option for individuals living with Duchenne.

Thank you for your consideration and for your continued commitment to the Duchenne community.