March 28, 2017

The Honorable Ed Hernandez
Chair, Senate Health Committee
California State Senate
State Capitol, Room 2191
Sacramento, CA 95814

Re: NORD Support for SB 643 (Pan)

Dear Chairman Hernandez and Members of the Committee:

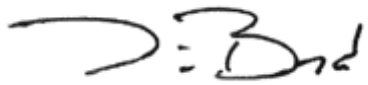
On behalf of the 1-in-10 people in California living with a rare disease, the National Organization for Rare Disorders (NORD) urges you to support SB 643, a bill to add Duchenne muscular dystrophy to the list of medical conditions eligible for the Genetically Handicapped Persons Program. NORD is the leading voice of the rare disease community dedicated to helping people with rare "orphan" diseases and assisting the organizations that serve them. Any disease affecting fewer than 200,000 Americans is considered rare. We believe strongly that every patient deserves the medical care that is best suited for their medical situation and that is most likely to give them the best results.

SB 643 would add Duchenne to the Genetically Handicapped Persons Program's (GHPP) list of eligible medical conditions. Duchenne is a rare neuromuscular disorder characterized by progressive symmetric muscle weakness and degeneration stemming from the progressive loss of contractile function. Onset of symptoms occur early in the preschool years with definitive diagnosis around 5 or 6 years of age. Patients with Duchenne currently receive specialized coordinated care through the California Children Services program (CCS), a state program that serves children with rare, complex health care needs. When they age out of the CCS system at 21 years of age, their families are left to navigate a healthcare system that provides very little specialized care and support.

The GHPP is a state health care program for adults with certain genetic diseases. The GHPP provides complete coordinated services to its clients by working closely with doctors, nurses, pharmacists, and other members of the health care team. Historically, most children diagnosed with Duchenne did not survive beyond their mid-20s and those that did often lost their ability to walk by the age of 12. When the GHPP was created, the mean age at death for patients with Duchenne was around 19 years. Fortunately, various interventions have led to improvements in quality of life and longevity so that children who are diagnosed today have the possibility of living into their 40s. Duchenne is a multisystem disease that impacts a patient's biological (neurology, cardiology, orthopedics, pulmonary, etc.) and mental health, so one aspect of care cannot be taken in isolation. Therefore, access to wraparound services like those that are offered through the GHPP are key features of treatment.

Adding Duchenne to the GHPP list of eligible conditions will improve continuity of care for this small, medically fragile population. Without the specialized care of knowledgeable physicians, the medical burden will revert to historically negative outcomes and negate the positive impact these patients have received while under superior care. For this reason, we are a proud supporter of SB 643, and respectfully request your aye vote on this measure.

Sincerely,



Tim Boyd, Director of State Policy
National Organizations for Rare Disorders