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Press Release

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FOR IMMEDIATE RELEASE
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CD Access to Provide Special Access Program for Duchenne Muscular Dystrophy Patients in Canada

NEWPORT BEACH, Calif., May 31, 2017 – CD Access is a new nonprofit that provides patients access to drugs and therapies that are currently unavailable through clinical trial or prescription. CD Access' first program allows Duchenne muscular dystrophy patients in Canada that previously participated in a clinical trial for drisapersen to begin redosing through a Health Canada Special Access Programme.

BioMarin transferred their supply of exon 51 skipping drug, drisapersen, to CD Access for distribution to boys who were participants in previous clinical trials in Canada. Former trial participants in Canada will need to contact their physician to confirm eligibility. The drug will be administered at Children's Hospital of Western Ontario, Children's Hospital of Eastern Ontario, Children's & Women's Health Centre of BC and potentially one additional site in Canada.

"We are delighted to provide access to eligible patients who benefited from drisapersen," said Debra Miller, CEO and founder of CureDuchenne and the newly formed CD Access. "We believe it is important for Duchenne patients who participated in the clinical trial to have access to investigational drugs when and where there are no approved options for treatments for this rare disease."

"When BioMarin discontinued development of drisapersen we received multiple calls from parents who wanted their sons to continue treatment with drisapersen. We have been working with BioMarin diligently ever since to establish a structure to accommodate these patients who participated in the clinical trial," said Miller. "BioMarin's executive and clinical team have demonstrated tremendous compassion for the Duchenne community in their willingness to work with us on a model that would allow former drisapersen trial participants in Canada to have access to the drug in a market where there is no approved treatment for Duchenne."

"We are grateful for CureDuchenne for going to bat for us because otherwise the boys in Canada would not have any other options," said Debra Chiabai, whose son Alex lives with Duchenne and is the first patient to be re-dosed with drisapersen through CD Access. "We are very appreciative to CureDuchenne, the doctors and Health Canada for supporting this. It is a real blessing. Having access provides a source of hope and allows us to be proactive."

"We are pleased to re-dose the first patient with drisapersen. We acknowledge CD Access and their dedication to helping these boys in Canada," said Dr. Hugh McMillan, Pediatric Neurologist at the Children's Hospital of Eastern Ontario in Ottawa, Canada. "This boy participated in the clinical trial and has benefited

from drisapersen treatment. He and his family are grateful to have once again have access to this medication."

About CD Access

CD Access is a new nonprofit that provides patients access to drugs and therapies that are currently unavailable through clinical trial or prescription. CD Access' first program allows Duchenne muscular dystrophy patients in Canada that previously participated in a clinical trial for drisapersen to begin redosing through a Health Canada Special Access Programme. For more information, please visit www.CDAccess.org.

About CureDuchenne

CureDuchenne was founded in 2003 with a focus on saving the lives of those with Duchenne muscular dystrophy, a disease that affects more than 300,000 boys worldwide. With support from CureDuchenne, nine research projects have advanced to human clinical trials. CureDuchenne also is the innovator bringing physical therapy and standard of care to local communities around the country through CureDuchenne Cares. For more information, please visit CureDuchenne.org and follow us on Facebook, Twitter, Instagram and YouTube.

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