CHILD NEUROLOGY SOCIETY

E-CONNECTIONS

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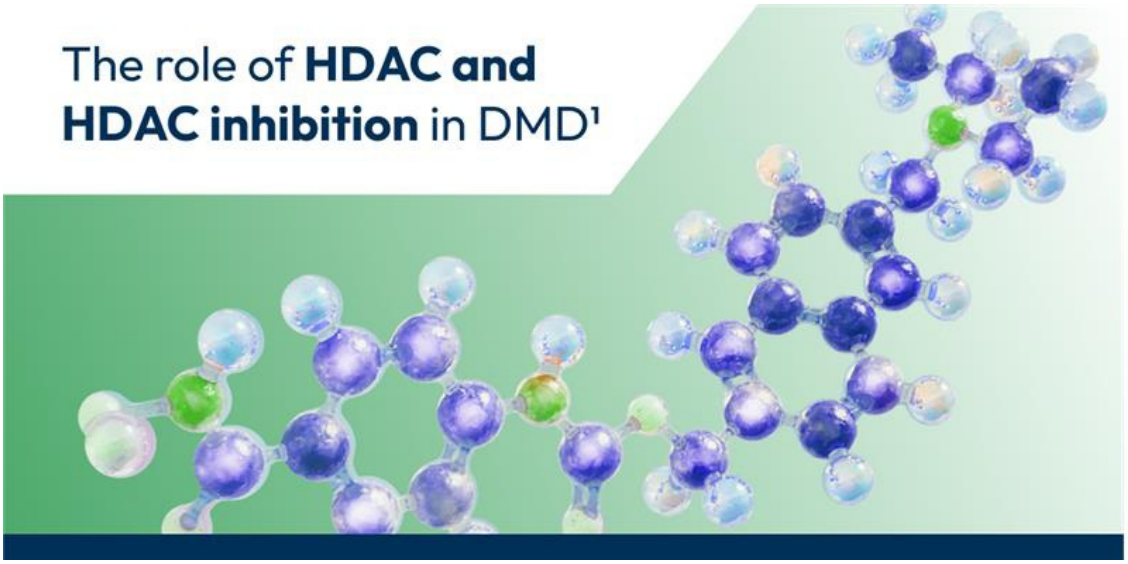

Greetings!

As you may know, histone deacetylase (HDAC) overactivity is thought to be a key pathologic process in Duchenne muscular dystrophy (DMD).[1-5]

In healthy tissue, HDACs play important roles in maintaining muscle homeostasis.[1,2] They are located in the nucleus and cytoplasm of cells.[1,2]

Through nuclear and cytoplasmic activity, HDACs modify both histone and nonhistone proteins, which affects many cellular pathways.[1,2]

In DMD, HDAC is overactive. In combination with the structural instability of dystrophin-deficient muscle cells, overactive HDAC disrupts the process of normal muscle repair and accelerates the deterioration of tissue, resulting in[2,4,5]:

- Activation of chronic inflammatory pathways
- Impairment of muscle repair mechanisms
- Fibrogenesis
- Adipogenesis
- Muscular atrophy

Want to learn more about an HDAC inhibitor for DMD?

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References: 1. Yang C, Croteau S, Hardy P. Histone deacetylase (HDAC) 9: versatile biological functions and emerging roles in human cancer. *Cell Oncol (Dordr*). 2021;44(5):997-1017. **2.** Sandonà M, Cavioli G, Renzini A, et al. Histone deacetylases: molecular mechanisms and therapeutic implications for muscular dystrophies. *Int J Mol Sci.* 2023;24(5):4306. **3.** Seto E, Yoshida M. Erasers of histone acetylation: the histone deacetylase enzymes. *Cold Spring Harb Prospect Biol.* 2014;6(4):a018713. **4.** Mercuri E, Vilchez JJ, Boespflug-Tanguy O, et al; EPIDYS Study Group. Safety and efficacy of givinostat in boys with Duchenne muscular dystrophy (EPIDYS): a multicentre, randomised, double-blind, placebo-controlled, phase 3 trial *Lancet Neurol*. 2024;23(4):393-403. **5.** Consalvi S, Saccone V, Giordani L, Minetti G, Mozzetta C, Puri PL. Histone deacetylase inhibitors in the treatment of muscular dystrophies: epigenetic drugs for genetic diseases. *Mol Med.* 2011;17(5-6):457-465.

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