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# [Review] The Human Avatar

Disease Modeling, Gene Editing / By Yoanne Clovis, Ph.D.

# How worms with muscular dystrophy are helping cure sick children

A clinically relevant strain of *C. elegans* mirrors both muscular dystrophy pathology and response to drug*s* 



# **Summary**

Knowledge acquired in animal models can be demonstrably extrapolated to human conditions when certain criteria are set and adhered to at the earliest stage - specifically, during model selection or building. *C. elegans'* simplicity, as well as its high tractability both on a genomic and phenotypic level, make this tiny nematode a very powerful model for early stage translational research. Here we discuss the *C. elegans* mutant dys-1(eg33), which recapitulates

many salient phenotypes of Duchenne Muscular Dystrophy (DMD), including loss of mobility, muscle necrosis, early pathogenesis, increased lethality, and the improvement of several muscular parameters with increased physical activity. The high degree of genetic conservation and phenotypic similarities between dystrophic worms and DMD patients provides a unique opportunity to gain insight into the etiology of the disease, and perform initial assessments of potential treatment strategies. We discuss multiple criteria that make *C. elegans* a clinically relevant model for DMD.

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NemaMetrix is the market leader for early preclinical in-vivo testing using small animal models such as *C. elegans* and zebrafish to gain a better understanding of the efficacy, mode of action, toxicity and potential targets for novel compounds.

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