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Juvenile Onset Huntington's Disease

Guest Editor:

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Message from the Guest Editor

The single-gene disorder, Huntington's disease (HD), is experiencing tremendous advances with current phase III clinical trials for gene knock-down therapy (antisense oligonucleotide) and several more in the pipeline. Juvenile Huntington's disease (JHD) is defined as onset of the disease prior to the age of 21 years. Pediatric HD (PHD) is the term used to describe young people affected by HD who are currently <18 years of age. These subjects, due to their age alone, are also excluded from clinical trials. Even if PHD and JHD were eligible for trials, though, there are serious gaps in our understanding of early onset HD. In particular, the motor phenotype is strikingly different from typical adult onset HD. Yet basic questions of pathophysiology remain unanswered—how is early onset HD similar to typical adult onset HD, and how is it different? Are there symptoms unique to early onset HD? What are the appropriate biomarkers for disease progression in early onset HD? This Special Issue aims at providing an overview of topics—both clinical and research —on this rare and unique patient sample.













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Message from the Editor-in-Chief

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