Therapeutic Approach for Huntington’s Disease (HD)

Dr. Hiroko Yano and Dr. Albert Kim, and their research teams at the Washington University School of Medicine in St. Louis developed and extensively evaluated a novel therapeutic approach using DNMT inhibitors to treat Huntington’s Disease (HD) and other neurodegenerative disorders. Currently DNMT inhibitors have been a successful treatment option in oncology, as hyper methylation has been identified as a key contributor for carcinogenesis. Recently, the role of epigenetic modifications has garnered interest in the neurological field, as DNA methylation-mediated transcription may be involved in brain function and disease. Interestingly, Dr. Yano and Dr. Kim have shown that DNMT manipulation may also be a key factor in the development of HD. Through an unbiased epigenetic drug library screen using primary neurons, they demonstrated that inhibitors of DNMT effectively protect neurons from mutant Huntingtin protein (Htt)-induced toxicity and restore critical genes known to be downregulated in HD, (Pan, 2016).

Key Features:
- **Novelty:** The use of DNMT inhibitors as an HD therapeutic is a novel approach.
- **Unmet Need:** Currently, there are no effective treatments for HD.
- **Versatile:** DNMT inhibitors have the potential for treating neurodegenerative and neuropsychiatric disorders, in addition to HD.
- **Validated:** The efficacy and safety of DNMT has been evaluated in primary neurons and in a mouse model of HD.

Publications:

**Patent:** PCT patent application filed

**Lead Inventor:**
Hiroko Yano, PhD, Assistant Professor of Neurological Surgery, Neurology and Genetics at Washington University in St. Louis.
Dr. Yano’s research is directed towards the molecular pathogenesis of neurodegenerative disorders with a particular focus on Huntington’s disease.

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<th>Licensing Contact</th>
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<td>Jennifer Richards, Ph.D, <a href="mailto:richards.j@wustl.edu">richards.j@wustl.edu</a>, 314-747-0928</td>
<td>Huntington’s Disease Neurodegenerative Disorders</td>
<td>016079</td>
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