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Dr. Veenstra-VanderWeele is a child and adolescent psychiatrist who uses molecular and translational neuroscience research tools in the pursuit of new treatments for autism spectrum disorder (ASD) and pediatric obsessive-compulsive disorder (OCD). As a pre-doctoral fellow, medical student, and resident, he trained in human molecular genetics in the laboratory of Edwin H. Cook at the University of Chicago. He expanded his research experience with a post-doctoral research fellowship in molecular neuroscience with Randy Blakely and Jim Sutcliffe at Vanderbilt University, with the goal to develop mouse models of social dysfunction and repetitive behavior. Currently, his molecular lab focuses on the serotonin, oxytocin and glutamate systems in genetic mouse models related to ASD and OCD. While developing a molecular neuroscience research program, he also built a clinical/translational research program to study new treatments for ASD and Fragile X Syndrome. He moved both arms of his research program to Columbia University in 2014 to continue to pursue novel treatments for children with these challenging disorders.

Pathways to new treatments for Autism Spectrum Disorder

Two main approaches are being pursued to identify new medication treatments that may benefit children with Autism Spectrum Disorder. The first and most common approach is to evaluate a treatment in the total group of people affected by ASD, usually with a small number excluded due to the presence of a known genetic syndrome. This strategy is challenged by the lack of support for common genetic or environmental risk factors that contribute substantially to risk in the entire group of children with ASD. Therefore, treatment studies in the overall group of children with ASD are largely tied to brain systems and pathways that may modulate social function or repetitive behavior but that are not necessarily implicated in autism risk. The second approach is almost the exact opposite, to study a medication for ASD-related symptoms in a defined genetic syndrome that confers substantial risk of ASD but comprises <2% of individuals with ASD. Since animal models are enabling an understanding of the underlying neurobiology that leads to autism-related symptoms in these populations, treatments targeted the root cause of these syndromes is possible. Transformative treatments, though possibly not "cures," seem most likely to emerge from the second approach, but in a small group of children. In contrast, if the first approach is successful, we can expect a treatment that benefits a larger group of children, but likely benefits them less. With emerging knowledge of brain systems and intersections in emerging genetic data, we can hope for a third approach that is somewhere in the middle, with a treatment being studied in a larger subgroup of individuals with ASD that share a common biomarker. This could result from extension outward from treatments studies in rare genetic syndromes, or it could result from identification of subgroups that benefit from treatments studied in ASD as a whole. I will discuss current challenges and opportunities as we seek new treatments in autism, including specific examples of each approach.