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FDA granted orphan drug designation for the treatment of pediatric patients with Tourette Syndrome

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INGREZZA is thought to work by reducing the amount of dopamine released in a region of the brain that controls movement and motor function, helping to regulate nerve signaling in adults with tardive dyskinesia.

Singapore - Neurocrine Biosciences announced that valbenazine, a novel selective vesicular monoamine transporter 2 (VMAT2), has been granted Orphan Drug Designation by the U.S. Food and Drug Administration (FDA) for the treatment of pediatric patients with Tourette syndrome. Tourette syndrome is a neurological disorder that becomes evident in early childhood or adolescence and is characterized by motor and vocal tics. Valbenazine is marketed in the United States as INGREZZA(valbenazine) capsules for the treatment of adults with tardive dyskinesia.

"We are pleased that the FDA has granted valbenazine orphan drug designation to treat pediatric patients with Tourette syndrome as it represents another significant regulatory milestone for our company," said Malcolm Lloyd-Smith, Chief Regulatory Officer at Neurocrine Biosciences. "We will continue our clinical development program for Tourette's focused on pediatric patients as the first symptoms of Tourette syndrome typically present early in childhood and represent a significant challenge for these young patients."

INGREZZA is thought to work by reducing the amount of dopamine released in a region of the brain that controls movement and motor function, helping to regulate nerve signaling in adults with tardive dyskinesia. VMAT2 is a protein in the brain that packages neurotransmitters, such as dopamine, for transport and release in presynaptic neurons. INGREZZA, developed in Neurocrine's laboratories, is novel in that it selectively inhibits VMAT2 with no appreciable binding affinity for VMAT1, dopaminergic (including D2), serotonergic, adrenergic, histaminergic, or muscarinic receptors. Additionally, INGREZZA can be taken as one capsule, once-daily, together with psychiatric medications such as antipsychotics or antidepressants. INGREZZA is currently in clinical development for the treatment of Tourette syndrome.