IntraBio Receives Niemann-Pick Disease Orphan Drug Designation from the FDA for IB1000 Series

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IntraBio Inc., a late-stage biopharmaceutical company developing novel therapies for rare ("orphan") and common neurodegenerative diseases, announced that the US Food and Drug Administration (FDA) has granted Orphan Drug Designation to its lead compound series (IB1000s) for the treatment of Niemann-Pick disease Type C (NPC), a rare, devastating, neurovisceral autosomal-recessive inherited metabolic, lysosomal storage disorder that predominately affects pediatric patients.

IntraBio was previously granted Orphan Medicinal Drug Designation from the European Commission for IB1000s for the treatment of NPC.

Niemann-Pick disease Type C affects 1:100,000 live births and is most commonly caused by dysfunction of the NPC1 protein leading to the accumulation of lipids in lysosomes, resulting in impaired cell function and cell death in various organs, leading to a spectrum of symptoms in NPC patients. The disease typically begins in early childhood and is chronic and progressive in nature; motor and cognitive symptoms become more disabling over the course of the disease, negatively impacting the quality of life and leading to an increase in the utilization of health resources. Currently, the average age of death for NPC patients is approximately 10 years, with half of the patients dying before the age of 12.5 years.

"We are excited to have another therapeutic option being evaluated in the NPC community and look forward to working with IntraBio through the drug approval process" added Dr. Justin Hopkin, Board Chair of the National Niemann-Pick Disease Foundation (NNPDF) in the United States. "We are encouraged that this compound has received Orphan Drug Designation by the FDA, displaying the urgency of bringing treatments to the Niemann-Pick disease community, a rare disease community fighting for the lives of its loved ones."

This orphan designation provides a number of regulatory benefits to IB1000s, such as a 25% tax credit for the costs of clinical development, a waiver for all prescription drug user fees at the time of marketing approval (approximately $2.5 Million dollars per indication), and 7 years' exclusivity in the US from the date of marketing authorization.
The company is currently in the process of applying for multi-national, multi-center clinical trials with its lead asset (IB1001) for the treatment of Niemann-Pick disease Type C (NPC), GM2 Gangliosidosis (Tay-Sachs and Sandhoff disease) and inherited Cerebellar Ataxias (CA).

IntraBio, with its collaborators, has evaluated the effect of IB1000s in compassionate use studies in over 175 patients, forming the scientific basis for IB1000s to be further investigated for the treatment of 18 indications, including neurodegenerative diseases and lysosomal storage disorders. Future opportunities to further develop the IB1000s series in additional indications include Lewy Body Dementia, Restless Leg Syndrome, ALS, and Multiple Sclerosis, all of which of have high unmet medical needs.

About IntraBio
IntraBio Inc. is a biopharmaceutical company with a late-stage drug pipeline including novel treatments for common and rare neurodegenerative diseases. IntraBio's platform results from decades of research and investment at premier universities and institutions worldwide. IntraBio's clinical programs leverage the expertise in lysosomal function and intracellular calcium signaling of its scientific founders from the University of Oxford and the University of Munich.

IntraBio's management team and consultants have vast commercial experience and a successful track record of drug development in the USA and Europe. Together, IntraBio's team translates innovative scientific research in the fields of lysosomal biology, autophagy, and neurology into novel drugs for a broad spectrum of neurodegenerative and genetic diseases to significantly improve the lives of patients and their families.

IntraBio Inc. is a U.S. corporation with its principal laboratory and offices in Oxford, United Kingdom.

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