Aminex Therapeutics Announces FDA Orphan Drug Designation Granted to AMXT 1501 in Combination with DFMO for the Treatment of Neuroblastoma

SEATTLE — October 2, 2025 — Aminex Therapeutics, Inc., a clinical-stage biotechnology company focused on developing novel therapies for rare and difficult-to-treat cancers, today announced that the U.S. Food and Drug Administration (FDA) has granted Orphan Drug Designation (ODD) to AMXT 1501 in combination with difluoromethylornithine (DFMO) for the treatment of patients with neuroblastoma.

"Receiving Orphan Drug Designation for AMXT 1501 in combination with DFMO represents an important milestone in our mission to develop innovative therapies for children with life-threatening cancers," said Mark Burns, PhD, Chief Scientific Officer and President of Aminex Therapeutics, Inc. "We are committed to working closely with regulators, investigators and patient advocacy groups, to accelerate the clinical development efforts for AMXT 1501 in combination with DFMO for the treatment of neuroblastoma and other childhood and adult tumor types in collaboration with the Beat Childhood Cancer Research Consortium."

The FDA grants ODD to drugs and biologics that are intended for safe and effective treatment, diagnosis or prevention of rare diseases or disorders. ODD provides certain incentives, such as tax credits toward the cost of clinical trials upon approval and prescription drug user fee waivers. If a product receives Orphan Drug Status from the FDA, that product is entitled to a unique seven years of market exclusivity for the disease in which it has ODD, which is an added value in that it is separate from intellectual property protection.

"Neuroblastoma is a rare childhood cancer that unfortunately, accounts for 12-15% of all pediatric cancer deaths in the United States," said Dr. Giselle Sholler, chief of the division of Pediatric Hematology and Oncology at Penn State Health Children's Hospital, director of Pediatric Oncology Research and professor of Pediatrics and Neuroscience at Penn State College of Medicine and founder and chair of the Beat Childhood Cancer Research Consortium. "We believe this combination has the potential to build upon the FDA approval of DFMO to further improve clinical outcomes for children with neuroblastoma and other rare childhood cancers."

The Beat Childhood Cancer Research Consortium at Penn State College of Medicine is currently initiating a Phase 1/2 clinical trial in pediatric patients who will be administered AMXT 1501 in combination with DFMO entitled “A Dose Escalation Study Using Eflornithine (DFMO) and AMXT 1501 Followed by a Randomized Controlled Trial of DFMO with or without AMXT 1501 for Neuroblastoma, CNS Tumors, and Sarcomas” (NCT06465199).

Aminex Therapeutics, Inc. is currently preparing to initiate clinical trials to further evaluate the safety and efficacy of AMXT 1501 in combination with DFMO in metastatic melanoma and in breast cancer.

About Neuroblastoma

Neuroblastoma is an aggressive pediatric cancer of the nervous system and the most common extracranial solid tumor in children. Children with high-risk neuroblastoma face survival rates of less than 50% despite intensive multimodal therapy. Furthermore, patients who relapse following conventional standard of care therapies have a long-term survival rate of <10%, underscoring the urgent need for novel treatment approaches.

About AMXT 1501 and DFMO

AMXT 1501 is a novel polyamine transport inhibitor designed to block the uptake of polyamines, which are essential for tumor growth and survival. In combination with DFMO, an irreversible inhibitor of ornithine decarboxylase, AMXT 1501 is intended to comprehensively suppress polyamine metabolism, a pathway shown to be critical in the development, metastasis and resistance to treatment of neuroblastoma and other cancers. DFMO is an established inhibitor of polyamine biosynthesis. Together, the combination aims to comprehensively inhibit polyamine metabolism and tumor growth.

About Aminex Therapeutics, Inc.

Aminex Therapeutics, Inc. is a clinical-stage biotechnology company focused on the development of a novel small molecule combination immunotherapy for the treatment of cancer. Aminex has advanced AMXT 1501 through target discovery, patenting, preclinical research and into clinical development. For more information, please visit www.aminextx.com.

About Beat Childhood Cancer Research Consortium

The Beat Childhood Cancer Research Consortium is an international academic group of 50+ universities and children's hospitals that offer a worldwide network of childhood cancer clinical trials coordinated through Penn State College of Medicine. They have opened over 27 clinical trials based on the research from collaborating investigators who are linked with laboratory

programs developing novel therapies for high-risk pediatric cancers. Their mission is to improve outcomes for children with cancer.

About Penn State College of Medicine

Located on the campus of Penn State Health Milton S. Hershey Medical Center in Hershey, Pa., Penn State College of Medicine boasts a portfolio of more than $150 million in funded research. Projects range from the development of artificial organs and advanced diagnostics to groundbreaking cancer treatments and understanding the fundamental causes of disease. Enrolling its first students in 1967, the College of Medicine has more than 1,700 students and trainees in medicine, nursing, the health professions and biomedical research on its two campuses.

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