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AVROBIO announces clinical and regulatory progress for Gaucher Disease Program

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US based AVROBIO, a leading clinical-stage gene therapy company with a mission to free people from a lifetime of genetic disease, has received notice of clearance from the U.S. Food and Drug Administration (FDA) regarding an Investigational New Drug (IND) application for AVR-RD-02, its investigational gene therapy for the treatment of Gaucher disease. This follows receipt of FDA orphan drug designation status for AVR-RD-02, and now clears AVROBIO to expand its ongoing Phase 1/2 clinical trial in Gaucher disease to the United States, supported by the Company's proprietary plato™ gene therapy platform.

"Many people with Gaucher disease type 1 experience life-limiting symptoms even while on chronic enzyme replacement therapy. Our investigational therapy is designed to address these unmet needs with a single dose," said Geoff MacKay, AVROBIO's President and Chief Executive Officer. "We are eager to bring patients a fundamentally new approach with the potential to halt the progression of their disease and alleviate or even reverse symptoms not addressed by the standard of care. As we work toward that goal, we are pleased to announce important milestones in our Gaucher program, including IND clearance in the U.S. and consent from the first patient in our global GAU-201 trial."

The standard of care for Gaucher disease type 1 is enzyme replacement therapy (ERT), which often does not halt disease progression or adequately address common life-limiting symptoms reported by Gaucher disease type 1 patients on ERT, such as fatigue and bone pain. Unlike ERT, AVR-RD-02 aims to provide a therapeutic benefit both systemically and throughout the central nervous system (CNS). AVROBIO believes AVR-RD-02 could slow, halt or potentially reverse symptoms throughout the entire body and brain, such as GBA-related Parkinson's disease which occurs more frequently in people with Gaucher disease type 1.

AVROBIO has received consent from the first patient to enroll in GAU-201, a global Phase 1/2 trial of AVR-RD-02. The trial is actively recruiting in Australia and Canada, with additional sites planned in the United States. It is intended to recruit 8 to 16 patients between the ages of 16 and 35 with Gaucher disease type 1, including both those who are treatment-naïve and those who are stable on enzyme replacement therapy. AVROBIO's gene therapy platform, plato, has now been cleared by regulators in Canada, Australia and the United States for use in the AVR-RD-02 clinical program.