FOR IMMEDIATE RELEASE
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Vanguard Clinical Rare Disease Foundation Launches Development of VCRDF-CLN3, a Precision ASO Therapy for CLN3 Batten Disease

Initiative endorsed by the ForeBatten Foundation and launched in collaboration with Dr. Michelle Hastings at the University of Michigan and Dr. Yael Shiloh-Malawsky at the University of North Carolina Chapel Hill; Type B Meeting with FDA indicates alignment with initial development plan

San Diego, CA — The **Vanguard Clinical Rare Disease Foundation (VCRDF)** is proud to announce the initiation of development of **VCRDF-CLN3**, an investigational **antisense oligonucleotide (ASO)** therapy designed to treat **CLN3 Batten disease**, a fatal pediatric neurodegenerative disorder. This work is endorsed **by the ForeBatten Foundation** and is being developed in close **collaboration with Dr. Michelle Hastings** at the **University of Michigan**.

The **VCRDF-CLN3 program was made possible by the foundational work and early investment of the ForeBatten Foundation**, which helped fund research spearheaded by Dr. Michelle Hastings. This early-stage development laid the scientific groundwork for what is now becoming a formalized clinical path.

The new ASO will specifically target the ~**1-kilobase deletion**, the most common disease-causing variant in CLN3 Batten disease. This approach builds on the model used in the Zebronkysen program, an individualized ASO developed specifically for twin girls with an ultra-rare mutation of CLN3 Batten Disease —**developed by the ForeBatten Foundation**—which demonstrated the feasibility, safety, and early evidence of efficacy of individualized ASO development for rare pediatric conditions.

VCRDF recently received written feedback from the **U.S. Food and Drug Administration (FDA)** in response to its Pre-IND meeting request. The **FDA indicated that the plan to develop VCRDF-CLN3 under the Agency's Individualized ASO (n-of-1) Guidance and to begin by treating two siblings appears scientifically reasonable**, **with general alignment on the proposed toxicology studies and clinical development plan**.

"This is such a meaningful next step—not just for our foundation, but for the entire CLN3 community," said **Tiffany Sepp**, Founder and Director of VCRDF and CEO of Vanguard Clinical, Inc. "We are **honored and humbled** to carry the baton forward from the ForeBatten Foundation, and we owe deep gratitude to **Karen and David Kahn**, and **Carol Schwimmer**, whose early leadership, relentless advocacy, and personal courage laid the groundwork for this program. Their work has given hope to others living with this disease. We are taking everything we've learned—from Zebronkysen and beyond—and applying it with urgency, precision, and

heart. This is what it looks like when community and science come together to rewrite what's possible."

The **first two patients planned for treatment with VCRDF-CLN3 are brothers living in North Carolina**, both diagnosed with this form of Batten disease. VCRDF intends to partner again with **Dr. Yael Shiloh-Malawsky at UNC Chapel Hill**, where Zebronkysen was successfully administered, to deliver this next therapy in a familiar and successful clinical setting.

VCRDF intends to expand the clinical program to include additional CLN3 patients with the same common deletion, pending demonstration of safety in the initial participants and continued alignment with the FDA on the development approach.

The parents of the siblings who will be treated first shared:

"When we learned there might be a chance to help our boys—not years from now, but soon—we felt hope again. Knowing that this team is experienced, thoughtful, and driven by love gives us confidence to keep moving forward. We're honored our sons will be among the first to receive this treatment."

The scientific collaboration is led by **Dr. Michelle Hastings** at the University of Michigan, a pioneer in targeted therapies development for rare neurological diseases.

"The approach we are taking with VCRDF-CLN3 is grounded in the latest science and powered by a remarkable collaborative model," said Hastings. "We are designing this therapy for a specific patient population, beginning with treatment in two children with the intent to quickly expand to include all those with this particular CLN3 deletion."

VCRDF's approach mirrors that of the **Zebronkysen program**, which recently achieved a major milestone.

"Earlier this month, my daughters Amelia and Makenzie received their **fifth and highest planned dose escalation of Zebronkysen**," said **Karen Kahn**, mother of the Kahn twins and a key advocate in the rare disease community. "While we initially hoped only to establish safety or slow the disease, both girls are now demonstrating **positive clinical outcomes**. We fully support the VCRDF-CLN3 effort and the incredible team behind it."

The **University of Michigan has submitted an NIH URGent grant application** to support early-stage development of VCRDF-CLN3, with VCRDF leading the translational and clinical execution. Meanwhile, VCRDF is **immediately launching a fundraising campaign** to ensure timelines can be expedited without delay. Private philanthropic support will be essential to bridging critical funding gaps and keeping the program on track to reach the first patients as swiftly and safely as possible.

Support from the broader scientific community continues to strengthen the initiative.

“The Zebronkysen program paved the way for this next step,” said **Dr. Jill Weimer**, Senior Director of Therapeutic Development at Sanford Research. “This type of targeted, patient-first therapeutic development is exactly what our community needs. I’m inspired by the dedication of this team and excited to see VCRDF-CLN3 move forward.”

About Vanguard Clinical Rare Disease Foundation

The **Vanguard Clinical Rare Disease Foundation (VCRDF)** is a nonprofit organization working to transform the way treatments for ultra-rare pediatric diseases are developed. With a focus on speed, collaboration, and family-centered innovation, VCRDF aims to collapse traditional timelines and bring hope to children and families who need it most.

About the ForeBatten Foundation

The **ForeBatten Foundation** funds cutting-edge research and therapeutic development for CLN3 Batten disease in honor of their daughters, Amelia and Makenzie. Their strategic, compassionate leadership continues to drive progress across the CLN3 research and patient community.

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