



EmendoBio Scientists Participate in National Neutropenia Network Patient Retreat

Scientists attend national patient retreat and share scientific updates on investigational EMD-101 for severe congenital neutropenia treatment

NEW YORK (August 1, 2023) – Reinvigorated with purpose, [Emendo Biotherapeutics](#) (EmendoBio), a leader in next-generation novel nuclease discovery and gene editing technologies, returns to the lab after a heartfelt weekend meeting patients and their families at the [National Neutropenia Network](#) (NNN) Patient Retreat.

On July 14th, over 100 patients with the rare blood disorder came together from around the country to network, share resources, and learn more about current scientific developments in neutropenia. Held in Cincinnati, Ohio, EmendoBio’s top scientists were invited to attend the retreat as keynote speakers and given the opportunity to meet the very patients that are at the center of the Company’s research efforts.

Chief Operating Officer [Asael Herman, Ph.D.](#), and Executive Vice President of Research and Development, [Rafi Emmanuel, Ph.D.](#) presented findings from ongoing studies and answered patient questions about EmendoBio’s lead investigational product, EMD-101, which targets the most severe form of the condition, Severe Congenital Neutropenia (SCN).

“It’s a privilege to speak with families that have been searching for answers for so long,” explained Asael Herman, whose teams have been undertaking preclinical development of EMD-101 for 5 years. “Our allele-specific editing strategies allow our scientists to utilize our extremely specific and active gene editors to target the rarest of diseases, as well as more prevalent monogenic and even non-monogenic diseases, with the goal of opening the door to future therapeutics that were previously unimaginable. We have only just scratched the surface when it comes to realizing the full potential of gene editing, and EmendoBio is just getting started.” EMD-101 nearing clinical trials marks a significant milestone for the Company.

“Our annual retreat is meant to educate, raise awareness, and build community,” shared NNN’s executive director, Stephanie Long, whose youngest daughter has SCN. “Just as neutropenia patients fight for their lives, their loved ones are fighting for answers. Being able to share scientific updates made this year’s retreat extra special for so many of our members.”

EmendoBio continues to actively advance [its pipeline](#) toward the clinic.

About Severe Congenital Neutropenia (SCN)

SCN is a rare genetic disorder that affects nearly one in every 200,000 people and is characterized by low levels of white blood cells called neutrophils. Because white blood cells are critical for fighting infections in the body, patients suffering from SCN run the risk of increased infection and developing blood cancers, along with several other debilitating symptoms.

A primary cause of SCN is an autosomal dominant genetic mutation in the *ELANE* gene, accounting for around 50% of cases. Current treatments are limited to preventing and managing infections, creating an unmet medical need for therapies aimed at addressing the underlying genetic cause of the disease.

About Emendo Biotherapeutics

EmendoBio is a next-generation CRISPR gene editing company leveraging triple proprietary technology platforms to enable high precision gene editing throughout the genome. EmendoBio's novel nuclease discovery platform broadens the targetable range of the genome while its target-specific optimization platform enables highly precise editing, including allele-specific editing, while maintaining high efficiencies. The capabilities of the OMNI technology platforms, along with deep expertise in genomic medicine, protein engineering and therapeutic development, provide EmendoBio with a unique advantage when addressing indications within hematology, oncology, ophthalmology, and other disease areas. For more information, please visit us at www.emendobio.com.