[429] Emendo Biotherapeutics - High Precision Gene Editing with Next Generation CRISPR Nucleases

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Emendo is a genome editing company developing genetic medicines for severe congenital diseases. To address the bottlenecks of the field, Emendo is employing directed evolution and advanced selection platforms to meet the highest bar of specificity – allele specific editing, as it requires differential cutting based on a single mismatch between the alleles.

Emendo's next generation CRISPR nucleases are highly specific and active, carefully selected per target sequence. By optimizing our OMNITM nucleases to be ultra-specific, Emendo is able to treat dominant, dominant negative and compound heterozygous indications. Emendo's initial clinical focus is on autologous, ex-vivo, allele specific editing of HSC's, the first program being ELANE-related Severe Congenital Neutropenia (SCN). ELANE patients are treated by knocking out the mutated allele while avoiding any damage to the healthy allele. This program is run in collaboration with Professor David C. Dale's research group at the University of Washington in Seattle.
Emendo's next clinical programs in primary immunodeficiencies and bone marrow failure syndromes will employ allele specific correction according to the genetic setting of the disease.