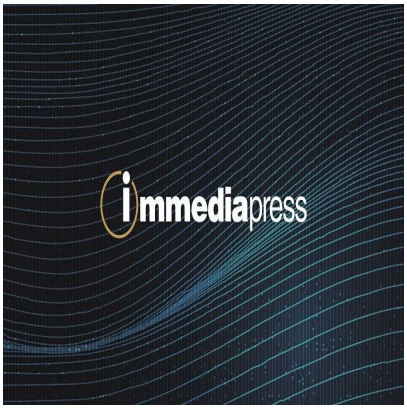

High-Precision Base-Editing Therapy Demonstrates Durable VOC-Free Efficacy and Favorable Safety in Sickle Cell Disease

Descrizione

COMUNICATO STAMPA â?? CONTENUTO PROMOZIONALE

CorrectSequence Therapeutics Reports Positive 15-Month Follow-Up Data for CS-206

SHANGHAI, June 2, 2026 /PRNewswire/ â?? CorrectSequence Therapeutics Co., Ltd. (Correctseq), a clinical-stage biotechnology company pioneering transformer Base Editing (tBE) technology for severe diseases, announced that the first sickle cell disease (SCD) patient being treated in China with its high-precision base-editing therapy CS-206 has remained free of vaso-occlusive crises (VOCs) for more than 15 months following engraftment. Starting from 60 Days after the last red-cell transfusion, the patient has remained free from VOCs and anemia for 13 consecutive months, achieving the primary efficacy endpoint. The follow-up data demonstrated favorable safety and efficacy of CS-206.

The patient, a 21-year-old woman from Nigeria, had experienced recurrent severe VOCs prior to treatment with CS-206. After receiving CS-206 treatment in February 2025, the patient achieved rapid and efficient hematopoietic reconstitution, with neutrophil engraftment observed on Day 13 and platelet counts exceeding 50Ã?10^9/L on Day 21 post-treatment. Within one month after treatment, fetal hemoglobin (HbF) levels increased significantly and continuously, while sickle hemoglobin (HbS) levels declined substantially and persistently. Beginning at Month 3 after treatment, the HbF-to-HbS ratio stabilized at approximately 6:4 and has remained stable thereafter. No product-related adverse events have been observed to date.

CS-206 is a proprietary base-editing therapy independently developed by Correctseq for the treatment of SCD. The therapy utilizes the highly precise transformer Base Editor (tBE), an original gene-editing technology developed by Correctseqâ??s scientific co-founders (Wang et al., Nature Cell Biology, 2021), to precisely edit the HBG1/2 promoter region in autologous hematopoietic stem cells collected from patients. The editing mimics naturally occurring beneficial mutations found in healthy individuals, thereby reactivating Î³-globin expression and rapidly increasing fetal hemoglobin levels.

This therapy effectively suppresses red blood cell sickling and significantly reduces vaso-occlusive crises and hemolysis. Compared with CRISPR-based gene-editing therapies for SCD, CS-206 offers the technological advantage of precise single-base correction without introducing DNA double-strand breaks, thereby avoiding risks such as large genomic deletions, chromosomal abnormalities, and off-target mutations. It is a safer and more efficient therapeutic approach. In addition, the therapy enables rapid hematopoietic recovery and substantial elevation of fetal hemoglobin levels, reducing the proportion of sickle hemoglobin and more effectively and durably preventing red blood cell sickling.

SCD is a hereditary hemoglobin disorder caused by mutations in the Î²-globin gene. Patients may experience chronic anemia, recurrent pain crises, infections, and progressive organ damage, with severe cases becoming life-threatening. Approximately 3.5% of global population carries the sickle cell mutation gene, and around 300,000 babies are born with the disease each year, with particularly high prevalence in Africa, the Mediterranean, the Middle East, and South Asia. SCD and Î²-thalassemia are both classified as hemoglobinopathies, among the worldâ??s most common monogenic inherited diseases. Approximately 7% of global population carries abnormal hemoglobin genes, with an estimated 400,000 affected births annually worldwide.

Based on the same tBE technology platform and therapeutic pathway, CS-101 developed by Correctseq for Î²-thalassemia has successfully cured more than ten Î²-thalassemia patients from China (Lai et al., Nature, 2026), Laos, Malaysia, Pakistan in clinical trials. As of the end of May 2026, all patients treated with CS-101 have remained transfusion-independent for more than 15 months, with the longest duration exceeding 30 months.

Global recruitment for the investigator-initiated trial (IIT) of CS-206 for SCD is currently ongoing. Correctseq is accelerating the clinical development and commercialization of both CS-101 and CS-206, with the goal of providing hemoglobinopathy patients worldwide with safer, more effective, and more affordable treatment options.

About CorrectSequence TherapeuticsCorrectSequence Therapeutics (Correctseq), is a clinical-stage biotech company employing its proprietary transformer Base Editor (tBE) to pioneer next-generation gene editing therapies. The company has developed multiple state-of-the-art base-editing systems that offer exceptional precision, minimize off-target effects, and enhance ex vivo and in vivo editing efficiency. Its robust pipeline spans genetic disorders, metabolic diseases, and cardiovascular conditions, with several programs already advancing toward clinical development.

For more information, visit www.correctsequence.com.

Acknowledgments: The First Affiliated Hospital of Guangxi Medical University, ShanghaiTech University, Shanghai Clinical Research and Trial Center.

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Categoria

1. Comunicati

Tag

1. ImmediaPress

Data di creazione
Giugno 2, 2026
Autore
redazione