

This press release is an English translation of a Japanese-language press release. The official language of this press release is Japanese, and the Japanese version takes precedence over the English version in terms of content and interpretation.

<Press Release>
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Announcement of Dose-Escalation Cohort Data for Rogocekib
Presented at the EHA Congress 2026

Chordia Therapeutics Inc (Head Office: Fujisawa City, Kanagawa Prefecture; CEO: Hiroshi Miyake, “Chordia”) announced data from a Phase 1/2 study (CTX-712-CL-02) of the CLK inhibitor rogocekib (CTX-712) in patients with relapsed or refractory acute myeloid leukemia (AML) and higher-risk myelodysplastic syndrome (MDS). Rogocekib is a first-in-class, orally administered small-molecule inhibitor that selectively targets CLK, a key regulator of RNA splicing, and is expected to provide a novel therapeutic option for myeloid malignancies with no approved targeted therapies. In this study, rogocekib demonstrated a manageable safety profile consistent with the Phase 1 study conducted in Japan, and preliminary anti-tumor activity was observed as monotherapy even in a more heavily pretreated and difficult-to-treat patient population compared to the Japanese study.

Key Highlights

- In a Phase 1/2 study in patients with relapsed or refractory AML and higher-risk MDS, rogocekib demonstrated a manageable safety profile.
- In this study, 34 evaluable patients, including those in lower dose cohorts, had baseline characteristics that reflected an elderly, heavily pretreated, and difficult-to-treat population. Despite these challenging characteristics, rogocekib demonstrated objective responses in 4 patients as monotherapy (3 CRi in AML and 1 mCR in MDS).
 - The overall response rate (ORR) was 11.8% (4/34), with a total of 4 objective responses observed.
 - In subgroup analyses, CRi rates of 50% (2/4) were observed in AML patients in the 100 mg once-weekly cohort, and 20% (1/5) in AML patients in the 80 mg twice-weekly cohort. In MDS, an mCR rate of 25% (1/4) was observed in the 80 mg once-weekly cohort.
 - Among 14 patients with splicing factor mutations, 5 achieved a reduction of greater than 50% in bone marrow blasts compared to baseline.

Overview of the CTX-712-CL-02 Study

This study (NCT05732103) is an ongoing multicenter Phase 1/2 study being conducted in the United States in patients with relapsed or refractory AML and higher-risk MDS. In the dose-escalation cohort, rogocekib tablets were administered at doses ranging from 20 mg to 140 mg once weekly or from 60 mg to 100 mg twice weekly, and were evaluated for safety, pharmacokinetics/pharmacodynamics (PK/PD), and preliminary efficacy.

Details of the Dose-Escalation Cohort Data

Among the 42 patients enrolled, 23 (54.8%) had AML, 16 (38.1%) had MDS, and 3 (7.1%) had chronic myelomonocytic leukemia (CMML). A total of 78.6% of patients were aged 65 years or older, and 57.1% had received three or more prior lines of therapy, indicating that more than half of the study population consisted of elderly and difficult-to-treat patients.

In this study, a new tablet formulation of rogocekib was used. Pharmacokinetic analyses showed results consistent with those observed in the Japanese study.

In terms of safety, adverse events were observed in all patients; however, an overall manageable safety profile was confirmed. The most common adverse events were hematologic toxicities and gastrointestinal events, and toxicity profiles were observed to be dependent on dose and dosing schedule. These findings were generally consistent with those observed in the Japanese study.

With respect to efficacy, among 34 evaluable patients in the dose-escalation cohort, the ORR was 11.8% (4/34), including a CRi rate of 16.7% (3/18) in AML patients and one mCR observed in MDS patients. In subgroup analyses, CRi rates of 50% (2/4) were observed in AML patients in the 100 mg once-weekly cohort, which Chordia considers a potential recommended dose, and 20% (1/5) in AML patients in the 80 mg twice-weekly cohort, demonstrating efficacy signals consistent with those observed in the Japanese study. In MDS, an mCR rate of 25% (1/4) was observed in the 80 mg once-weekly cohort. While anti-tumor activity of rogocekib as a monotherapy was confirmed in the dose-escalation cohort, no complete remissions (CR) were observed; Chordia believes that this may be attributable to the availability of multiple treatment options in the United States leading to prolonged treatment histories, as well as potential cumulative bone marrow exhaustion due to prior therapies. This point was also discussed at an investigators' meeting held in December 2025, and based on these insights, the study protocol has been amended to appropriately enroll patient populations in whom greater efficacy is expected, and expansion cohorts are currently underway.

Based on these results, rogocekib demonstrated a manageable safety profile and anti-tumor activity as a monotherapy in high-risk patients with relapsed or refractory hematologic malignancies. In particular, promising response signals were observed in AML, and to the best of Chordia's knowledge, rogocekib is the only CLK inhibitor to demonstrate this level of efficacy as monotherapy. The Company believes that these results support the clinical utility and future development potential of rogocekib as a novel therapeutic approach targeting RNA splicing regulation.

Chair ad interim for the Department of Leukemia at the University of Texas MD Anderson Cancer Center and principal investigator for the CTX-712-C1-02 trial, Dr.

Guillermo Garcia-Manero, commented, “CLK is a major vulnerability in leukemia. In this clinical trial of patients with advanced MDS and AML, rogocekib has been shown to be safe and demonstrated a signal of clinical activity in patients with very advanced disease. Further studies are warranted.”

Future Development

Based on these results, Chordia will continue to evaluate the potential recommended dose and dosing schedule of rogocekib in expansion cohorts, while advancing further clinical development in relapsed or refractory AML and higher-risk MDS by incorporating protocol amendments designed to appropriately assess patient populations in whom greater efficacy is expected. Through these efforts, Chordia aims to maximize the value of rogocekib and establish it as a new therapeutic option addressing significant unmet medical needs.

Hiroshi Miyake, CEO of Chordia, commented, “we are greatly encouraged by the confirmation of anti-tumor activity of rogocekib as a monotherapy in this ongoing study in the United States, particularly in an elderly and heavily pretreated, difficult-to-treat patient population. Based on these results, we will continue to evaluate the optimal dose and dosing schedule of rogocekib in the expansion cohorts and strive to deliver a new treatment option to patients with limited available therapies.”

Announcement Information

Abstract No	EHA-3974
Title	SAFETY, TOLERABILITY, AND PRELIMINARY ACTIVITY OF ROGOCEKIB IN PATIENTS WITH RELAPSED/REFRACTORY MYELOID MALIGNANCIES: RESULTS FROM A PHASE 1/2 STUDY (CTX-712-CL-02)
Session No	Poster
Time	June 13, 2026, 6:45 PM - 7:45 PM(CEST)
Abstract	SAFETY, TOLERABILITY, AND PRELIMINARY ACTIVITY OF ROGOCEKIB IN... - Garcia-Manero G - EHA-3974 - Jun 11 2026

Glossary of Terms

Term	Explanation
AML	Abbreviation for <u>A</u> cute <u>M</u> yeloid <u>L</u> eukemia, a hematologic malignancy characterized by the clonal proliferation of myeloid precursor cells in the bone marrow.
CMML	Abbreviation for <u>C</u> hronic <u>M</u> yelom <u>o</u> nocytic <u>L</u> eukemia, a disorder characterized by abnormal proliferation of monocytic lineage cells in the bone marrow and is classified as a hematologic malignancy with features of both myelodysplastic syndromes (MDS) and myeloproliferative neoplasms.
CLK	Abbreviation for <u>C</u> DC2- <u>L</u> ike <u>K</u> inase, an enzyme that catalyzes the transfer of phosphate groups to target proteins and plays an important role in splicing.
CR	Abbreviation for <u>C</u> omplete <u>R</u> emission, a measure used to evaluate the anti-tumor activity of a drug; in hematologic

	malignancies, it refers to a state in which the proportion of cancer cells in the bone marrow is less than 5% and normal blood cell counts, including neutrophils and platelets in peripheral blood, have fully recovered.
CRi	Abbreviation for <u>CR</u> with <u>incomplete</u> hematologic recovery, a state in which the proportion of cancer cells in the bone marrow is less than 5%, but recovery of normal blood cell counts, including neutrophils and platelets in peripheral blood, is <u>incomplete</u> .
Dose-escalation cohort	A group of patients in a Phase 1 clinical trial in which doses gradually increased from a low starting level to evaluate safety and tolerability.
Hematologic toxicity	Hematologic toxicity refers to a group of adverse events characterized by reductions in blood cells, including white blood cells, neutrophils, platelets, and red blood cells, due to drug administration; it is commonly observed in anticancer treatments.
mCR	Abbreviation for <u>Marrow</u> <u>Complete</u> <u>Remission</u> , a state in which the proportion of cancer cells in the bone marrow is reduced to less than 5%; unlike CR, recovery of peripheral blood cell counts is not required, and it is primarily used to assess treatment response in MDS.
MDS	Abbreviation for <u>Myelodysplastic</u> <u>Syndrome</u> , a hematologic malignancy characterized by dysfunctional hematopoietic stem cells in the bone marrow, resulting in ineffective hematopoiesis and a deficiency of normal blood cells. HR-MDS refers to subtypes of MDS that are associated with a higher risk of progression to AML and a poor prognosis.
ORR	Abbreviation for <u>Overall</u> <u>Response</u> <u>Rate</u> , a measure of overall response; in AML, it includes CR, CRi, and MLFS, while in MDS it includes CR and mCR,
Pharmacodynamic (PD)	A measure of the biological effects of a drug in the body and the extent to which it engages its target molecules. In this study, target engagement was confirmed through changes in biomarkers related to splicing.
Pharmacokinetics (PK)	An assessment of how a drug is absorbed, distributed, metabolized, and excreted in the body over time.

About Rogocekib (CTX-712)

Rogocekib is a first-in-class, selective, orally available small-molecule inhibitor of CDC2-like kinase (CLK), a key regulator of RNA splicing. In myeloid malignancies, mutations in splicing factor genes are frequently observed and are associated with poor prognosis; however, there are currently no approved therapies targeting this pathway. Rogocekib is being developed in Japan and the United States as a novel therapeutic approach to address this unmet medical need.

The results of the Phase 1 study conducted in Japan (CTX-712-CL-01) have been published for the hematologic malignancies cohort in Yokoyama H et al., Blood Advances 2026; 10(1): 262-272, and for the solid tumor cohort in Sato J et al., Clinical Cancer Research 2026; doi: 10.1158/1078-0432.CCR-25-4896.



About Chordia Therapeutics

Chordia Therapeutics is a research and development-stage biopharmaceutical company specializing in oncology with clinical-stage assets, headquartered in Fujisawa City, Kanagawa Prefecture. Chordia's lead asset, rogocekib (CLK inhibitor CTX-712), is under Phase 1/2 clinical study in the US. Rogocekib potentially targets the vulnerability of cancer and is expected to deliver benefits to patients of various types of cancer. In addition to rogocekib, Chordia is engaged in the research and development of several assets, including ocipumaltib (MALT1 inhibitor CTX-177), CTX-439, a CDK12 inhibitor, and GCN2 inhibitors. For more information, please visit our website <https://www.chorditherapeutics.com/en/>.