Taiho Oncology and Taiho Pharmaceutical to Present Data from AZTOUND Trial Evaluating Oral Azacitidine and Cedazuridine in Myelodysplastic Syndromes or Chronic Myelomonocytic Leukemia at the 68th American Society of Hematology Annual Meeting and Exposition

PRINCETON, New Jersey, TOKYO, Japan, November 4, 2026 — Taiho Oncology, Inc., and Taiho Pharmaceutical Co., Ltd., today announced that data from the AZTOUND trial evaluating oral azacitidine and cedazuridine (ASTX030) in patients with myelodysplastic syndromes (MDS) or chronic myelomonocytic leukemia (CMML) will be shared in an oral presentation at the 68th American Society of Hematology (ASH) Annual Meeting and Exposition, to be held from December 12–15 in New Orleans.

As previously announced, the Phase 3 portion of the study met its primary endpoint of total cycle area under the curve (AUC) from 0 to 24 hours (AUC0-24) exposures, measured as the ratio of azacitidine total cycle AUC0-24 exposures following oral azacitidine and cedazuridine compared with subcutaneous azacitidine. The safety profile was consistent with parenteral azacitidine.

Based on results from the AZTOUND study, Taiho Oncology intends to submit a New Drug Application to the U.S. Food and Drug Administration (FDA) seeking approval of azacitidine and cedazuridine as an oral treatment regimen for adults with MDS or CMML.

“We look forward to sharing our latest findings on the PK exposure of orally available azacitidine and cedazuridine and continuing our interactions to advance ASTX030 toward regulatory approval to potentially bring this treatment option to patients with MDS or CMML,” said Harold Keer, MD, PhD, Chief Medical Officer, Taiho Oncology. “We are committed to developing therapies that address the needs of patients with blood cancers, including oral treatment options that may help reduce treatment burden.”

Summary of results:

AZTOUND Phases 1 and 2: A pooled efficacy analysis included patients with MDS from Phases 1 and 2 (n=45) treated with ASTX030 at approximate recommended phase 2 dose (RP2D) levels in phase 1 (azacitidine/cedazuridine: 136 mg/20 mg, 140 mg/20 mg, and 144 mg/20 mg) and at the RP2D in phase 2 (azacitidine/cedazuridine: 140 mg/20 mg).

The median age was 73 years. Patients were treated at or near RP2D for a median of 6

treatment cycles. The median duration of treatment was 5.7 months, and the median duration of follow-up (range) was 25.6 months (19–33). Overall response was achieved in 44.4% of patients (95% CI 29.6, 60.0), and 9 patients (20.0%) had a complete response according to the International Working Group 2006 response criteria. Overall response rate refers to the proportion of patients who achieved complete response (CR), partial response (PR), marrow complete response (mCR), or hematologic improvement (HI). Median overall survival (95% CI) was not reached (21.6, not evaluable).

AZTOUND Phase 3: The ongoing randomized, crossover Phase 3 part of the trial is evaluating azacitidine exposure following oral ASTX030 and subcutaneous azacitidine in patients with MDS or CMML. ASTX030 is dosed based on body surface area (BSA) to provide optimal azacitidine exposure (105 mg/20 mg, 140 mg/20 mg, and 175 mg/20 mg, respectively, for low-, intermediate-, and high-BSA groups).

In the AUC equivalence analysis (n=83), the geometric mean ratio for the primary PK endpoint was 95.2% (90% CI 86.5–104.7), indicating that the trial met its primary endpoint and confirming AUC equivalence of oral ASTX030 with subcutaneous azacitidine.

Oral ASTX030 and subcutaneous azacitidine had similar demethylation profiles based on percent long interspersed nuclear elements-1 (LINE-1) demethylation from baseline.

In a safety analysis (n=71), the median duration of treatment was 1.6 months. Findings were consistent with azacitidine's known safety profile. Treatment-emergent adverse events (TEAEs) were reported in 92% of patients, with 68% experiencing grade ≥3 TEAEs. The most common grade ≥3 TEAEs were anemia (24%) and neutropenia (20%). Serious AEs occurred in 34% of patients, most commonly febrile neutropenia (9%) and pneumonia (6%). No new safety signals were identified.

Other Phase 3 trial objectives, including clinical activity, will be assessed as the study continues.

Session information for the data presentation:

Title: Pooled Phase 1/2 Efficacy and Phase 3 Pharmacokinetic (PK), Pharmacodynamic (PD), and Safety Analyses of ASTX030 (Oral Azacitidine-Cedazuridine)

Presenting Author: Guillermo Garcia-Manero, MD, Chair Ad Interim, Department of Leukemia, Division of Cancer Medicine, The University of Texas MD Anderson Cancer Center, Houston, Texas

Session Name: 637. Myelodysplastic Syndromes: Clinical and Epidemiological

Session Type: Oral Presentation

Session Date: December 14, 2026

Session Time: 10:30 a.m. – 12 p.m.

Presentation Time: 11:45 a.m.

Room: MCCNO - Great Hall AD

Additional Taiho Oncology presentations at the ASH Annual Meeting and Exposition:

Title: Phase 1 ABLATE-101 study to evaluate safety, tolerability, pharmacokinetics (PK), and clinical activity of the antibody-drug conjugate (ADC) ARC-02 as monotherapy and in combination with rituximab in adults with B-cell non-Hodgkin lymphoma (NHL)

Presenting Author: Jean-Marie Michot, MD, a medical doctor in the Department of Hematology and Innovative Drugs at the Institut de Cancérologie Gustave Roussy in Villejuif, France

Session Name: 627. Aggressive Lymphomas: Targeted and Pharmacologic Therapies: Poster II

Session Type: Poster

Session Date: December 13, 2026

Session Time: 6 – 8 p.m.

Room: MCCNO - Hall F

Title: Clinical Outcomes with Oral Decitabine-Cedazuridine and Parenteral Hypomethylating Agents in Higher-Risk MDS, CMML, and Low-Blast AML: A Systematic Review and Meta-Analysis

Presenting Author: Amer Zeidan, MD, MBBS, Professor of Internal Medicine (Hematology) at Yale School of Medicine

Session Name: 637. Myelodysplastic Syndromes: Clinical and Epidemiological: Poster II

Session Type: Poster

Session Date: December 13, 2026

Session Time: 6 – 8 p.m.

Room: MCCNO - Hall F

Title: Patient preferences for benefits, risks, and administration route of hypomethylating agents in acute myeloid leukemia

Presenting Author: Amer Zeidan, MD, MBBS, Professor of Internal Medicine (Hematology) at Yale School of Medicine

Session Name: 903. Health Services and Quality Improvement: Myeloid Malignancies: Poster II

Session Type: Poster

Session Date: December 13, 2026

Session Time: 6 – 8 p.m.

Room: MCCNO - Hall F

About the AZTOUND Trial

Phase 3 of the AZTOUND trial (A Multi-phase, Pharmacokinetics, Safety, and Efficacy Study of ASTX030 (Azacitidine and Cedazuridine) as Monotherapy in Subjects With Myeloid Neoplasm or in Combination With Venetoclax in Subjects With AML) is a randomized open-label crossover study that enrolled 88 adults with MDS or CMML. The primary objective was to assess total cycle AUC0-24 exposures.

About Oral Azacitidine and Cedazuridine

Azacitidine and cedazuridine (ASTX030) is an oral combination of azacitidine, a DNA methyltransferase inhibitor, and cedazuridine, a cytidine deaminase inhibitor added to help azacitidine stay active in the body without being degraded. Azacitidine and cedazuridine (ASTX030) is investigational and has not been approved by any health authority. Azacitidine and cedazuridine is being developed by Taiho Oncology, Inc. and its parent company, Taiho Pharmaceutical Co., Ltd.

About Myelodysplastic Syndromes (MDS) and Chronic Myelomonocytic Leukemia (CMML)

Myelodysplastic syndromes are a heterogeneous group of hematopoietic stem cell disorders. Each year, there are approximately 10,000 to 15,000 new cases in the U.S., though the disease is believed to be underdiagnosed.[1,2,3] Approximately one-third of these cases may progress to acute myeloid leukemia.[4,5] Chronic myelomonocytic leukemia is a clonal hematopoietic malignancy characterized by accumulation of abnormal monocytes in the bone marrow and in blood. Approximately 2,000 new cases of CMML are diagnosed annually in the U.S.[6,7] Between 15% and 30% of CMML cases may progress to acute myeloid leukemia.[8]

About Taiho Oncology, Inc.

The mission of Taiho Oncology, Inc. is to improve the lives of patients with cancer, their families and their caregivers. The company specializes in the development and commercialization of orally administered anti-cancer agents for various tumor types. Taiho Oncology has a robust pipeline of small-molecule clinical candidates targeting solid-tumor and hematological malignancies, with additional candidates in pre-clinical development. Taiho Oncology is a subsidiary of Taiho Pharmaceutical Co., Ltd. which is part of Otsuka Holdings Co., Ltd. Taiho Oncology is headquartered in Princeton, New Jersey and oversees its parent company's European and Canadian operations, which are located in Baar, Switzerland and Oakville, Ontario, Canada.

For more information, visit https://www.taihooncology.com/, and follow us on LinkedIn and X.

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About Taiho Pharmaceutical Co., Ltd. (Japan)

Taiho Pharmaceutical, a subsidiary of Otsuka Holdings Co., Ltd. (https://www.otsuka.com/en/), is an R&D-driven specialty pharma focusing on the fields of oncology and immune-related diseases. Its corporate philosophy takes the form of a pledge: "We strive to improve human health and contribute to a society enriched by smiles." In the field of oncology, in particular, Taiho Pharmaceutical is known as a leading company in Japan for developing innovative medicines for the treatment of cancer, a reputation that is rapidly expanding through their extensive global R&D efforts. In areas other than oncology, as well, the company creates and markets quality products that effectively treat medical conditions and can help improve people's quality of life. Always putting customers first, Taiho Pharmaceutical also aims to offer consumer healthcare products that support people's efforts to lead fulfilling and rewarding lives. For more information about Taiho Pharmaceutical, please visit https://www.taiho.co.jp/en.

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