CARNA BIOSCIENCES

News Release

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Carna Biosciences, Inc.

Carna Announces FDA Orphan Drug Designation Granted to monzosertib (AS-0141) for Acute Myeloid Leukemia

Carna Biosciences announces that the U.S. Food and Drug Administration (FDA) has granted Orphan Drug Designation (ODD) to monzosertib (AS-0141), a CDC7 inhibitor, for the treatment of acute myeloid leukemia (AML).

AML is a hematologic malignancy characterized by the proliferation of abnormal leukemic cells in the bone marrow and is designated as a rare disease in the United States. Although treatment outcomes have improved in recent years, there remains a significant unmet medical need among patients with relapsed or refractory disease and those who are not eligible for currently available molecularly targeted therapies.

Monzosertib is a highly selective CDC7 inhibitor discovered by Carna Biosciences and is being developed as a novel therapeutic approach that does not depend on specific genetic mutations or signaling pathways. The Company is currently collaborating with The University of Texas MD Anderson Cancer Center to advance the development of a triplet combination therapy consisting of monzosertib, azacitidine (AZA), and venetoclax (VEN) for patients with relapsed or refractory AML.

The FDA's Orphan Drug Designation is granted to investigational drug candidates being developed for rare diseases affecting fewer than 200,000 patients in the United States. Receipt of this designation provides various incentives, including enhanced opportunities for consultation with the FDA during development, waiver or reduction of regulatory application fees, and market exclusivity following approval.

Kohichiro Yoshino, Ph.D., President and Chief Executive Officer at Carna Biosciences commented, “We are very pleased that monzosertib has received Orphan Drug Designation from the FDA. We believe that this designation represents an important milestone in the development of monzosertib, given the significant unmet medical need in AML. Monzosertib is an investigational drug with a novel mechanism of action targeting CDC7, and we are currently advancing its clinical development for AML in collaboration with MD Anderson Cancer Center. With this designation, we will continue to steadily advance the clinical development of monzosertib and strive to contribute to addressing the unmet medical needs of patients with AML.”

About monzosertib (AS-0141), a CDC7 Inhibitor

Monzosertib (AS-0141) is an orally available drug candidate discovered by Carna Biosciences that selectively and potently inhibits CDC7 kinase.
CDC7 (cell division cycle 7) kinase is a key enzyme that regulates the initiation of DNA replication. In cancer cells with dysregulated cell cycle control, inhibition of CDC7 activity is known to induce replication stress, leading to cell death. In contrast, normal cells maintain functional cell cycle checkpoint mechanisms and

are therefore considered less susceptible to the effects of CDC7 inhibition, raising expectations that CDC7 inhibition may provide a novel therapeutic option with favorable tolerability.
CDC7 has been reported to be overexpressed in a variety of cancers, and CDC7 inhibition has attracted attention as a novel therapeutic approach that induces DNA replication stress, resulting in suppression of cancer cell proliferation and induction of cancer cell death. Furthermore, in preclinical studies, monzosertib has demonstrated potent antitumor activity in combination with DNA methyltransferase (DNMT) inhibitors and BCL-2 inhibitors, suggesting the potential to overcome resistance to existing therapies and improve treatment efficacy.
Carna Biosciences has successfully discovered monzosertib, a highly selective CDC7 inhibitor, and is advancing the development of innovative therapies for acute myeloid leukemia (AML) and various other cancer indications.

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