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News Release

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Acoramidis receives positive CHMP opinion for treatment of transthyretin amyloid cardiomyopathy

- The Committee for Medicinal Products for Human Use (CHMP) has recommended the approval of acoramidis in the EU based on positive results from the Phase III ATTRibute-CM study¹
 - Final decision from the European Commission is anticipated within the coming months
 - Acoramidis, is an orally administered near-complete ($\geq 90\%$) stabilizer of transthyretin (TTR),² for the treatment of adult patients with transthyretin amyloid cardiomyopathy (ATTR-CM)
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Berlin, December 13, 2024 – The Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) has recommended acoramidis, a selective small molecule, orally administered transthyretin (TTR) stabilizer, for marketing authorization in the European Union (EU). The compound is recommended in adult patients with transthyretin amyloid cardiomyopathy (ATTR-CM), a progressive fatal disease that presents as an infiltrative, restrictive cardiomyopathy resulting in heart failure. In vitro, acoramidis demonstrated near-complete TTR stabilization. In the Phase III study ATTRibute-CM, acoramidis has been shown clear benefits on cardiovascular endpoints.

The positive CHMP opinion for acoramidis is based on the pivotal ATTRibute-CM study results. The study investigated the efficacy and safety of acoramidis given twice daily compared to placebo, in patients with ATTR-CM.¹ In the ATTRibute-CM Phase III study, acoramidis was superior to placebo in reducing the composite of all-cause mortality (ACM) and cardiovascular-related hospitalizations (CVH).¹

“ATTR-CM is often delayed in its recognition or is misdiagnosed, which can have a negative impact on patients’ outcomes. It is a condition that invariably progresses in the absence of treatment, and is ultimately fatal,” said Julian Gillmore, Professor of Medicine, University College London (UCL) Centre for Amyloidosis, UK. “The CHMP's positive opinion offers hope to individuals living with ATTR-CM. We are encouraged by the prospect of additional treatment, which provides near-complete stabilization of TTR, to slow the progression of symptoms and improve outcomes in patients with ATTR-CM.”

“Patients living with ATTR-CM often see a decline in their quality of life due to the physical and functional challenges posed by their condition. There is a clear need for continued innovation and further options for patients affected,” said Christine Roth, Executive Vice President, Global Product Strategy and Commercialization and Member of the Pharmaceuticals Leadership Team at Bayer. “This positive recommendation from the CHMP represents a significant milestone in the fight against this life-threatening heart disease. We eagerly anticipate the European Medicines Agency's approval for acoramidis, expected in early 2025 and are striving to make this new treatment available to patients as soon as possible.”

The final decision from the European Commission on the marketing authorization is expected in the coming months. Acoramidis, developed by BridgeBio Pharma, Inc. (Nasdaq: BBIO), was recently approved by the Food and Drug Administration (FDA) for the U.S. with a label specifying near-complete stabilization of TTR.³ BridgeBio holds the marketing rights for acoramidis in the U.S., while Bayer holds the exclusive marketing rights for the product in Europe. Pending approval, Bayer plans to launch acoramidis in Europe in the first half of 2025.

Since March 2024, Bayer and BridgeBio and affiliates, have pursued a collaboration for acoramidis. This partnership leverages Bayer’s long legacy of expertise in cardiovascular disease and its established European cardiovascular infrastructure paired with BridgeBio’s leadership in the emerging field of ATTR-CM.

About ATTR-CM

Transthyretin amyloid cardiomyopathy (ATTR-CM) is a progressive and fatal disease in elderly adults, characterized by the deposition of abnormal protein in the heart. It occurs when a tetrameric protein called transthyretin (TTR) becomes unstable, caused by an inherited mutation in the TTR gene or due to aging, and dissociates into monomers.

These monomers misfold, aggregate, and form amyloid fibrils that deposit in the heart muscle, leading eventually to heart failure. The disease is often diagnosed late, when the accumulation of amyloid has already occurred, and patients are symptomatic. Once diagnosed, ATTR-CM patients have a median survival of 3-5 years if left untreated.⁴

About Acoramidis and the Phase III study results

Acoramidis is an investigational, orally administered, highly potent selective small molecule stabilizer of transthyretin (TTR). Acoramidis was designed to mimic a naturally occurring "rescue mutation" of the TTR gene (T119M) that targets the root cause of ATTR-CM, destabilization of the native TTR tetramer. In vitro, acoramidis, demonstrated near-complete ($\geq 90\%$)² TTR stabilization. In the Phase III study ATTRibute-CM, acoramidis has been shown clear benefits on cardiovascular endpoints.

In the ATTRibute-CM Phase III study, acoramidis met the primary endpoint. The primary objective of the ATTRibute-CM Phase III study was to establish superiority of acoramidis versus placebo on a hierarchical endpoint that included all-cause mortality (ACM) and cumulative frequency of cardiovascular-related hospitalizations (CVH).

When compared to placebo, acoramidis twice daily demonstrated a rapid and sustained clinical benefit on the composite endpoint of all-cause mortality and cardiovascular hospitalization in ATTR-CM patients through month 30⁵:

- Significant benefit in the primary endpoint
- 36% reduction in the combined endpoint of all-cause mortality or first cardiovascular hospitalization, showing benefits already after 3 months of treatment
- 50% reduction in cardiovascular hospitalizations
- 45% of individuals, relative to placebo with 9% experienced a reduction (improvement from baseline) in NT-proBNP⁵
- 40% of individuals, relative to placebo with 22% experienced an increase (improvement from baseline) in 6-minute walk distance⁵

Acoramidis was well-tolerated.⁶

About Bayer's Commitment in Cardiovascular Diseases

Bayer is a leader in cardiology and is advancing a portfolio of innovative treatments in cardiovascular (CV) diseases of high unmet medical need. The strategy is to unlock the strong potential of the future CV market by transforming Bayer's portfolio into precision cardiology, addressing the high CV disease burden, and driving the long-term growth.

Bayer's portfolio already includes several innovative products and compounds in various stages of preclinical and clinical development.

About Bayer

Bayer is a global enterprise with core competencies in the life science fields of health care and nutrition. In line with its mission, "Health for all, Hunger for none," the company's products and services are designed to help people and the planet thrive by supporting efforts to master the major challenges presented by a growing and aging global population. Bayer is committed to driving sustainable development and generating a positive impact with its businesses. At the same time, the Group aims to increase its earning power and create value through innovation and growth. The Bayer brand stands for trust, reliability and quality throughout the world. In fiscal 2023, the Group employed around 100,000 people and had sales of 47.6 billion euros. R&D expenses before special items amounted to 5.8 billion euros. For more information, go to www.bayer.com.

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Forward-Looking Statements

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