Blueshift RESEARCH

November 15, 2023

BridgeBio Pharma Idea Proposal

What will the market split look like if BridgeBio Pharma's Acoramidis is approved in 2024 as a challenger to Pfizer's tafamidis?

Report Available: December 14, 2023

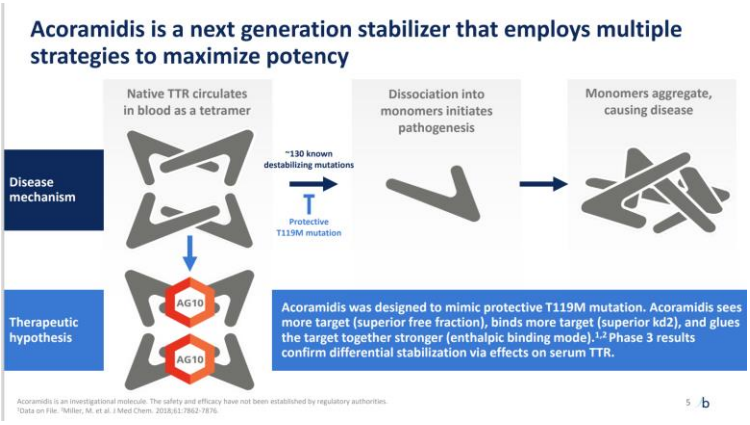
Blueshift Research will examine how the standard of care for treating transthyretin amyloid cardiomyopathy (ATTR-CM) will be affected if the U.S. Food and Drug Administration (FDA) approves BridgeBio Pharma Inc's (BBIO) Phase 3 drug, Acoramidis, in 2024. Is Acoramidis a differentiated drug from Pfizer's tafamidis (sold under the brand names Vyndaqel and Vyndamax)? Is it a better TTR stabilizer? Will physicians and payors view Acoramidis as a better first-line treatment than tafamidis? How will market share look after Acoramidis is approved? When will a generic TTR stabilizer become available? How will the market be affected by a generic option? Are ATTR-CM drugs called silencers that are currently being studied potentially more effective than stabilizers like Acoramidis? Once a silencer is in the treatment space, what will happen to the stabilizer market? How will the price of Acoramidis affect prescribing and reimbursement? How will the Inflation Reduction Act impact the adoption of Acoramidis and the TTR stabilizer market? To answer these and other questions, Blueshift will interview cardiologists and their teams, cardiac drug sales professionals, third-party payors, and other industry specialists.

Observations

1. ATTR-CM is a rare and underdiagnosed heart disease that is potentially fatal. It occurs when the liver produces faulty transthyretin (TTR) proteins. Clumps of these abnormal proteins (called fibrils) build up in the heart's main pumping chamber and the left ventricle becomes stiff and weak. This cardiomyopathy makes it harder for the heart to pump blood and can lead to heart failure.

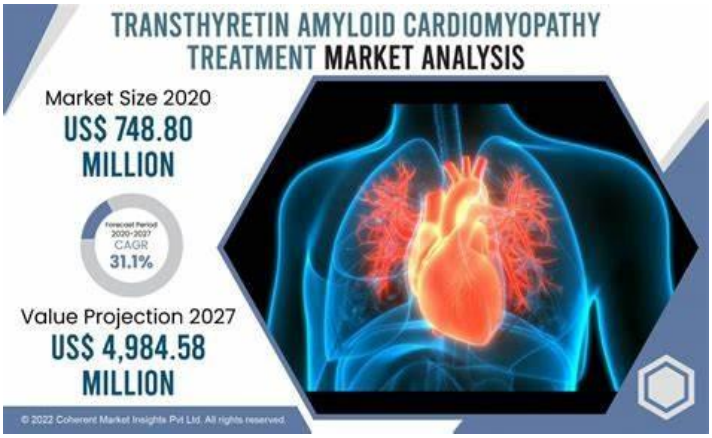
2. Although there is no cure for ATTR-CM, BridgeBio's Phase 3 drug, Acoramidis, was designed to achieve maximal stabilization and preserve native TTR. The company's placebo-based clinical trial, ATTRibute-CM, achieved excellent results, including prolonging life, reducing hospitalization,s and improving heart function in ATTR-CM patients. BridgeBio plans to submit a new drug application (NDA) for Acoramidis to the FDA later in 2023 and to other markets in 2024.

If you are interested in seeing this Blueshift Research report, please contact Bill Jenks at (617) 244-4960 or billjenks@blueshiftideas.com. You can find more Idea Proposals on our website: www.blueshiftideas.com.



3. If approved, commercialization of Acoramidis is expected in 2024. The new drug will face formidable competition from Pfizer Inc.'s (PFE) TTR stabilizer Vyndamax and Vyndaqel. Those drugs were approved in 2019 and have become the standard of care for treating ATTR-CM, with market penetration as high as 60%. There are also other treatments available and other new drugs in clinical trials from Alnylam Pharmaceuticals Inc. (ALNY) and Ionis Pharmaceuticals Inc. (IONS) that could further challenge the use and adoption of Acoramidis.

4. The prevalence of ATTR-CM and the total available market for treatments is unclear due to the lack of data. Several organizations have attempted to quantify the number of people ATTR-CM affects, but there is no definitive number. A 2018 population study estimated that ATTR-CM affects 5 in 100,000 people. In the United States, the prevalence of ATTR-CM is estimated to be around 13% to 17% among people with diastolic heart failure, which is the most common form of heart failure, translating to approximately 425,000 individuals. A report published last year suggested the global ATTR-CM treatment market could grow 31.1% a year to rise from about $750 million in 2020 to almost $5 billion by 2028.



5. Another challenge facing BridgeBio and its new drug is establishing a price and securing third-party reimbursement. Pfizer's Vyndamax and Vyndaqel retail at $250,000 per year—the most expensive cardiovascular drugs on the market—leading to out-of-pocket costs for patients with insurance coverage of $1,000 to $2,000 per month. Considerable outrage at their pricing was expressed shortly after their approval and accusations of price gouging have been raised by the medical community. The Inflation Reduction Act has listed Vyndamax and Vyndaqel as among the 60 medications that the government will be negotiating lower prices on. BridgeBio has yet to set a price for Acroamidis, but the current environment suggests its pricing could have broad implications for its adoption as an alternative to Pfizer's medications.

Companies: BridgeBio Pharma Inc. (BBIO), Alnylam Pharmaceuticals Inc. (ALNY), Ionis Pharmaceuticals Inc. (IONS), Pfizer Inc. (PFE)

Research Begins: November 27, 2023