Attruby™ (acoramidis) – New orphan drug approval

- On November 22, 2024, BridgeBio Pharma announced the FDA approval of Attruby (acoramidis), for the treatment of the cardiomyopathy of wild-type or variant transthyretin-mediated amyloidosis (ATTR-CM) in adults to reduce cardiovascular death and cardiovascular-related hospitalization.

- Transthyretin is a protein that normally transports thyroid hormone and vitamin A. In ATTR-CM, transthyretin proteins misfold and deposit in different organs of the body, including the heart. When amyloid deposits build up in the heart, it can cause dysfunction of the heart muscles (cardiomyopathy) and ultimately lead to symptoms of heart failure.

    - ATTR-CM affects over 120,000 people in the U.S.

- Attruby is a transthyretin stabilizer and the second drug approved for ATTR-CM. Pfizer's transthyretin stabilizer, tafamidis (Vyndaqel®, Vyndamax™), was approved for a similar indication in May 2019.

- The efficacy of Attruby was established in a randomized, double-blind, placebo-controlled study in 611 adult patients with ATTR-CM. Patients were randomized to receive Attruby or placebo for 30 months. The primary composite endpoint included all-cause mortality and cumulative frequency of cardiovascular-related hospitalizations (CVH) over 30 months.

    - There was a statistically significant reduction (p = 0.018) in all-cause mortality and cumulative frequency of CVH in the Attruby arm vs. the placebo arm.
    - All-cause mortality was reported in 19% and 26% of participants in the Attruby and placebo groups, respectively. The majority (79%) of the deaths were cardiovascular.
    - CVH was reported in 27% and 43% of participants in the Attruby and placebo groups, respectively. The mean number of CVH events was 0.3 vs 0.6 per year. The majority (59%) of CVH were heart failure hospitalizations reported in 13% and 26% of the participants in the Attruby and placebo groups, respectively.

- The recommended dose of Attruby is 712 mg orally twice daily.

- The list price of Attruby will be approximately $245,000 annually.

- BridgeBio Pharma's launch plans for Attruby are pending. Attruby will be available as a 356 mg tablet.