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Potential first-in-class therapy designed to treat the underlying cause of liver disease associated with AATD



Japan-based Takeda Pharmaceutical Company Limited (Takeda) and Arrowhead Pharmaceuticals Inc. in the US have announced a collaboration and licensing agreement to develop ARO-AAT, a Phase 2 investigational RNA interference (RNAi) therapy in development to treat alpha-1 antitrypsin-associated liver disease (AATLD).

ARO-AAT is a potential first-in-class therapy designed to reduce the production of mutant alpha-1 antitrypsin protein, the cause of AATLD progression.

Under the agreement, Takeda and Arrowhead will co-develop ARO-AAT which, if approved, will be co-commercialized in the United States under a 50/50 profit-sharing structure. Outside the US, Takeda will lead the global commercialization strategy and receive an exclusive license to commercialize ARO-AAT with Arrowhead eligible to receive tiered royalties of 20-25% on net sales.

Arrowhead will receive an upfront payment of \$300 million and is eligible to receive potential development, regulatory and commercial milestones up to \$740 million. Closing of the transaction is contingent on completion of review under antitrust laws, including the Hart-Scott-Rodino (HSR) Antitrust Improvements Act of 1976 in the US.

Alpha-1 Antitrypsin-Associated Deficiency (AATD) is a rare genetic disorder associated with liver disease in children and adults and pulmonary disease in adults. AATD is estimated to affect 1 per 3,000-5,000 people in the United States and 1 per 2,500 in Europe. The protein AAT is primarily synthesized and secreted by liver hepatocytes. Its function is to inhibit enzymes that can break down normal connective tissue.