

Novartis to license Akcea-Ionis cardiac disease drug

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Akcea earns \$150M license fee

Akcea Therapeutics, an affiliate of Ionis Pharmaceuticals announced that Novartis has exercised its option to license AKCEA-APO(a)-LRx, a drug to treat patients with elevated levels of lipoprotein(a), or Lp(a), and established cardiovascular disease (CVD).

AKCEA-APO(a)-LRx, referred to by Novartis as TQJ230, was discovered by Ionis and co-developed by Akcea and Ionis.

Elevated Lp(a) is an independent genetic risk factor for CVD that cannot be managed by lifestyle modifications, including diet or exercise, or with treatment using existing cholesterol-lowering therapies. It is estimated that there are more than eight million patients living with CVD and elevated levels of Lp(a).

Paula Soteropoulos, chief executive officer at Akcea said, "We are very pleased that Novartis, an established global leader in drug development and commercialization, will now shepherd this landmark therapy through late-stage clinical development and toward the market. The Phase 2 study results presented last year at AHA showed that AKCEA-APO(a)-LRx significantly reduced Lp(a) levels below the recognized threshold for cardiovascular risk in patients living with cardiovascular disease and elevated Lp(a) levels with a favorable safety and tolerability profile. AKCEA-APO(a)-LRx is the first and only medicine to do this. We believe that this therapy has the potential to be a significant advance for millions of people affected by cardiovascular disease around the world."

Novartis will assume responsibility for all future development activities for AKCEA-APO(a)-LRx, including a planned global Phase 3 cardiovascular outcomes study and, pending regulatory approval, global commercialization activities.

AKCEA-APO(a)-LRx is an antisense drug developed based on Ionis' proprietary Ligand Conjugated Antisense, or LICA, technology platform. Ionis' proprietary LICA technology platform has the potential to produce new drugs that can be used at lower doses and with less frequent administration compared to non-LICA antisense drugs. AKCEA-APO(a)-LRx is designed to inhibit production of apolipoprotein(a), or Apo(a) protein, thereby reducing systemic levels of Lp(a).

Brett Monia, chief operating officer at Ionis said, "The Ionis LICA platform is a game-changing breakthrough for our antisense technology. AKCEA-APO(a)-LRx, our most advanced LICA medicine, is a particularly exciting and important new medicine because of its potential to treat millions of patients with cardiovascular disease. This treatment represents one of many important new medicines within our LICA pipeline and illustrates our continued commitment to innovation and in bringing transformative medicines to patients in great need."

Based on the strategic collaboration agreement between Akcea and Novartis, Akcea will receive a \$150 million license fee that will be split equally with Ionis. Akcea will settle its \$75 million obligation to Ionis in Akcea common stock.