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Ionis, Roche unite to treat Complement-Mediated Diseases

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Ionis Pharmaceuticals announced a new collaboration with Roche to develop IONIS-FB-LRx for the treatment of complement-mediated diseases.

This collaboration will leverage Ionis' leadership in RNA-targeted therapeutics to develop IONIS-FB-LRx targeting Factor B (FB) for a broad range of diseases.

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"Ionis is committed to bringing new therapies to patients living with unmet medical needs. The collaboration is designed to maximize both the potential benefit to patients and the likelihood of success, while optimizing our commercial participation in IONIS-FB-LRx. This new agreement builds upon our productive relationship with Roche on IONIS-HTTRx (RG6042), an antisense drug for the treatment of people with Huntington's disease," said Brett P. Monia, chief operating officer at Ionis.

IONIS-FB-LRx, an antisense drug using Ionis' advanced LIgand Conjugated Antisense (LICA) technology, reduces the production of FB, a key protein in the complement innate immune system. FB is predominately produced in the liver and circulates throughout the vascular system, including vessels in the eye and kidney.

This complement protein plays a pivotal role in an innate immunogenic cascade that, when overactivated, has been associated with the development of several complement-mediated diseases, including dry AMD.

In a Phase 1 study in 54 healthy volunteers, IONIS-FB-LRx reduced plasma FB and was safe and well tolerated.

Under this new collaboration with Roche, Ionis will receive a $75 million upfront payment. In addition, Ionis is eligible to receive up to $684 million in development, regulatory and sales milestone payments and license fees.

Ionis also has the potential to receive tiered royalties that range from the high teens to twenty percent on sales from the product when commercialized. Ionis is responsible for conducting a Phase 2 study in patients with dry AMD and exploring the drug in a rare severe renal indication.

Roche has the option to license IONIS-FB-LRx at the completion of the studies. Upon licensing, Roche will be responsible for all global development and commercialization activities.