RS-2023-00258840

Development of AAV based ocular cell/tissue targeting technology and treatment candidates for the development of gene therapy for X-chromosome-linked retinitis pigmentosa

ENCell

OPHTHALMOLOGY | Hit

Product Type	Adeno-associated virus (AAV) with active RPGR gene
Indication	X-linked retinitis pigmentosa, XLRP
Target	Photoreceptor in retina
MoA(Mechanism of Action)	• Verification of AAV Gene Expression Specificity in Photoreceptors, Tropism for Ocular Tissues, and Expression Efficiency • Validation through Co-IP of RPGR with RPGRIP1, as Protein Network Partner • Evidence of Photoreceptor Function Recovery Through Dark- and Light-Adapted ERG
Competitiveness	• currently no FDA-approved treatment • Current Status of Ongoing Clinical Trials: - Nightstar Therapeutics now biogen: Phase II/III XIRIUS study (completed) - MeiraGTx UK II Ltd // Janssen: Phase III (recruiting) - Applied Genetic Technologies Corp. : Phase I/II (recruiting)
Development Stage	Hit
Route of Administration	Subretinal injection

kddf_bd@kddf.org
KDDF Business Development Team

KDDF Korea Drug Development Fund