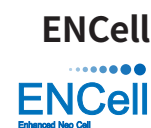


## Development of AAV based ocular cell/tissue targeting technology and treatment candidates for the development of gene therapy for X-chromosome-linked retinitis pigmentosa



| 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) | <ul style="list-style-type: none"> <li>• Verification of AAV Gene Expression Specificity in Photoreceptors, Tropism for Ocular Tissues, and Expression Efficiency</li> <li>• Validation through Co-IP of RPGR with RPGRIP1, as Protein Network Partner</li> <li>• Evidence of Photoreceptor Function Recovery Through Dark- and Light-Adapted ERG</li> </ul>   |
| Competitiveness          | <ul style="list-style-type: none"> <li>• currently no FDA-approved treatment</li> <li>• Current Status of Ongoing Clinical Trials:               <ul style="list-style-type: none"> <li>- Nightstar Therapeutics now biogen: Phase II/III XIRIUS study (completed)</li> <li>- MeiraGTx UK II Ltd // Janssen: Phase III (recruiting)</li> <li>- Applied Genetic Technologies Corp. : Phase I/II (recruiting)</li> </ul> </li> </ul> |
| Development Stage        | Hit  |
| Route of Administration  | Subretinal injection   |