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Screening New Drugs for Treatng Retinitis Pigmentosa

Repurposed Carvedilol boosts rod photoreceptor regeneration, showing promise for retinitis pigmentosa therapy.

Researchers at Purdue University have discovered drug candidates for potentially treating retinitis pigmentosa. Retinitis pigmentosa (RP) is a common group of genetically inherited retinal degeneration diseases that may lead to blindness. There is currently only a gene therapy available for patients suffering from this disease, and it targets a rare form of RP known as Leber congenital amaurosis. However, there is no approved pharmacological intervention for any form of RP. One of the identified drugs, Carvedilol a beta blocker approved by FDA for treatment of mild-to-severe chronic heart failure, increased rod photoreceptor generation. In an experiment with zebrafish larvae, treatment with Carvedilol outperformed the positive control (Retinoic acid) in promoting rod cell generation.

Related Publication: Sci Rep. 2021 Jun 1;11(1):11432. doi: 10.1038/s41598-021-89482-z.

Technology Validation: A hallmark of RP is loss of night vision—unable to see at night or slow to adjust in dark. The visual motor responses of WT and RP mutant zebrafish larvae adapted to darkness were compared in presence and absence of the screened drugs under dim light, to which only rod photoreceptor can respond. Treatment of RP mutant larvae with Carvedilol showed significantly enhanced response to dim-light change compared to DMSO-treated mutant larvae.

Advantages:

- Addresses a major unmet need—there is only one approved drug for treating retinitis pigmentosa

- Carvedilol could be used as a repurposed drug; hence, regulatory timelines are expected to be short.

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Category
Pharmaceuticals/Drug Discovery & Development
Biotechnology & Life Sciences/Analytical & Diagnostic Instrumentation

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Applications:

- Treatment for retinitis pigmentosa, other rod diseases

TRL: Pharmaceuticals

Intellectual Property:

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