UNIVERSITY OF MINNESOTA
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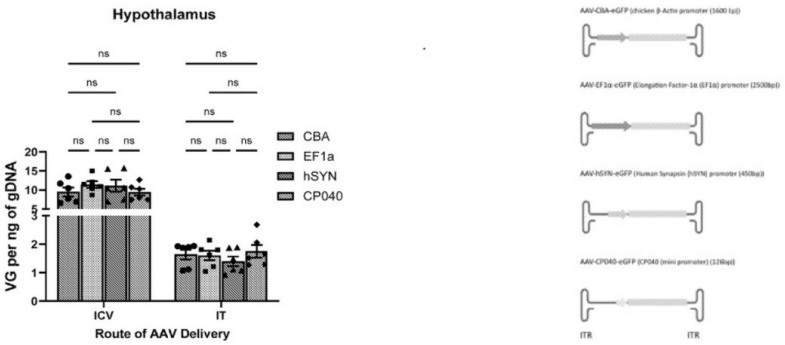
A minipromoter for gene therapies

A mini-promoter to accommodate larger therapeutic genes of interest in gene therapy programs



IP Status: PCT Pending; Application No. PCT/US2024/037893

Applications

- Gene Therapy
- DNA Repair Disorder Treatments
- Genome Editing

Key Benefits & Differentiators

- **Enables larger therapeutic genes of interest:** The reduced size of the promoter consumes less of the limited space available in gene delivery vectors.
- **Improves expression levels:** Studies show comparable performance to larger, standard promoters in vivo.

Technology Overview

DNA repair disorders such as Cockayne syndrome and Xeroderma Pigmentosum cause premature aging features with shorter life spans and photosensitivity. Gene therapies are a promising approach to treating these disorders with the delivery of the healthy gene sequence. However, for some diseases, the combined size of the gene with the required promotor to enhance uptake and expression does not fit the approximately 4.9 kilobase packaging constraint of adeno-associated viruses. This problem is similarly present more broadly in the development of other gene therapies, requiring less effective, alternative delivery methods or smaller and limiting promoters.

Researchers at the University of Minnesota have developed a minipromoter to accommodate larger therapeutic genes of interest for gene therapies (CP040 in the figure above). The new promoter is only 126 bp, enabling larger genes than even the smallest available alternatives. In vitro and in vivo studies have demonstrated that the minipromoter drives gene expression at comparable levels to its larger counterparts. Additionally, the promoter exhibits limited off-

Technology ID

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target expression. The minipromoter successfully drives transgene expression in neurological tissues that would otherwise not be possible.

Phase of Development

TRL: 3
In vivo proof-of-concept demonstrated

Desired Partnerships

This technology is now available for:

- License
- Sponsored research
- Co-development

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Researchers

- **Christina Pacak, Ph.D.** Professor, Department of Neurology