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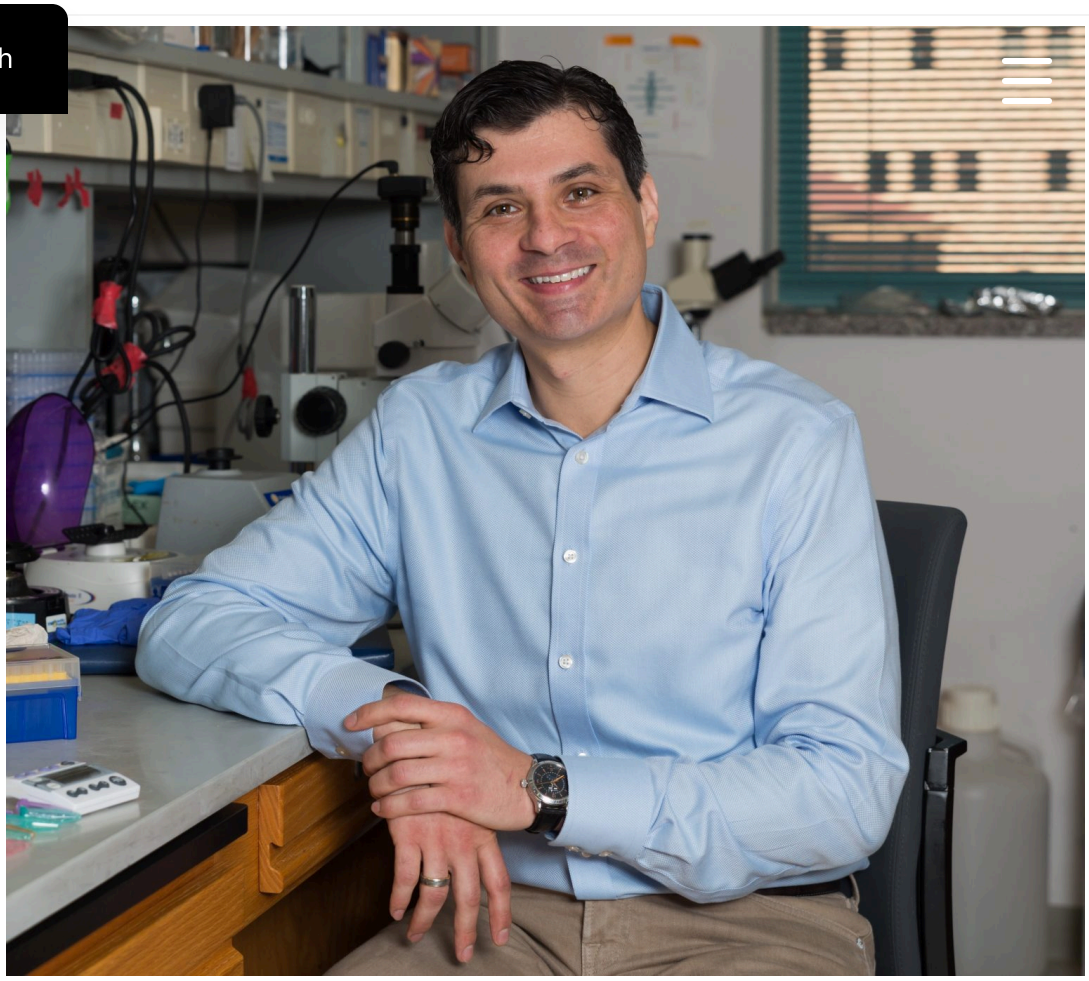
Emily's Entourage Awards New Funding to University of Texas at Austin to Advance Breakthrough Gene Editing Therapy for Hard-to-Treat Cystic Fibrosis Mutations

With support from Emily's Entourage, new retron editor technology aims to restore lung function for the 10% of people with cystic fibrosis unable to benefit from current mutation-targeted therapies.

Lower Merion, PA—July 29, 2025—Emily's Entourage (EE), an innovative 501(c)(3) organization accelerating research for individuals with cystic fibrosis (CF) who do not benefit from existing CFTR modulator therapies, announced new funding to support research at the University of Texas at Austin aimed at developing a promising gene editing approach. The project, led by Ilya Finkelstein, PhD, associate professor in the department of molecular biosciences, seeks to advance a novel technology known as retron editors, which has the potential to correct specific mutations in the CFTR gene that cause CF.

Cystic fibrosis is a hereditary disease caused by mutations in the CFTR gene, which impair the transport of salt and fluids across cell membranes. This disruption leads to thickened mucus in the lungs, causing chronic infections and progressive lung damage. While the development of CFTR modulators drastically have improved outcomes for many people with CF, approximately ten percent of individuals with CF are unable to benefit from these therapies due to ineligible genetic mutations.



Led by Ilya Finkelstein, PhD, the newly funded project seeks to advance a novel technology known as retron editors, which has the potential to correct specific mutations in the CFTR gene that cause CF.

Dr. Finke
research
on developing
retron editors, a
gene editing
platform that
combines a
molecular tool
called a nickase
Cas9 enzyme
with bacterial
reverse
transcriptases.
Unlike
conventional
CRISPR-based
gene editing
methods, which
rely on creating
double-strand
DNA breaks and
can carry
associated safety
risks, retron
editors produce single-stranded DNA repair templates directly within cells. This unique mechanism enables replacement of entire segments of the CFTR gene—specifically exons 23 and 24—with a "superexon," potentially correcting multiple mutations simultaneously with improved precision and safety.

"The potential to replace entire exons in the CFTR gene represents a significant leap forward in gene editing technology," said Finkelstein. "By avoiding double-stranded DNA breaks, our retron editor approach aims to offer a safer and more effective treatment option for people with CF who have been left behind by current mutation-targeted therapies."

The research aims to optimize the retron editor system initially in laboratory cell lines before progressing to airway cells derived from CF patients carrying the W1282X and N1303K mutations. A key objective is to restore CFTR function to therapeutic levels—estimated at greater than 15% of normal activity—which is considered sufficient to improve lung function and patient outcomes. The team will also rigorously assess editing precision and minimize off-target effects.

Emily's E[English] entific Officer, Chandra Ghose, PhD, emphasized the importance of supporting innovati[illegible]ene therapy for CF. "This funding enables critical advancement in gene editing strategies that could transform treatment for a subset of the CF community who have had very limited options. The retron editor technology holds hope, not only for cystic fibrosis, but also as a platform for other genetic diseases requiring precise DNA replacement."

In addition to developing the editing technology itself, the project explores delivery methods using RNA molecules packaged in lipid nanoparticles, designed to safely deliver the gene editors into lung cells.

About Emily's Entourage's (EE's) Grant Program

Emily's Entourage's (EE's) Grant Program provides grant funding to accelerate research and therapeutic development for people with cystic fibrosis (CF) who do not benefit from existing mutation-targeted therapies. EE provides funding through several grant funding mechanisms, including translational grants, collaborative grants, and preclinical exploratory grants, as well as through venture philanthropy investments.

To date, EE has awarded millions of dollars to multi-disciplinary teams around the world and helped to secure millions of dollars in follow-on funding. To view awarded grants, visit https://www.emilysentourage.org/awarded-grants/. To learn more about EE's funding opportunities, visit https://www.emilysentourage.org/funding-opportunities/.

About Emily's Entourage

Emily's Entourage is an innovative 501(c)3 that accelerates research for new treatments and a cure for individuals in the final 10% of the cystic fibrosis (CF) population that do not benefit from currently available mutation-targeted therapies. Since 2011, Emily's Entourage has awarded millions of dollars in research grants, launched a now clinical-stage CF gene therapy company, developed the Clinical Trial Connect (CTC) patient database and clinical trial matchmaking program to accelerate clinical trial recruitment, and led worldwide efforts to drive high-impact research and drug development. The organization has been featured in national media, including the New York Times, STAT, CNN, People, and more. Learn more at emilysentourage.org.

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