

# Couragene Co-founders Developing Gene Editing Technology for Genetic Diseases Awarded Major Federal Grant

10/10/2023

NORTH BRUNSWICK, N.J.--(BUSINESS WIRE)-- Couragene, a genetic medicines company using novel delivery platforms, announces today that its co-founders Yong-Hui Jiang and Jiangbing Zhou of Yale University, together with Elizabeth Berry-Kravis of RUSH University have been awarded a competitive **UG3/UH3 grant** from the NIH's Somatic Cell Genome Editing (SCGE) program. This monumental grant, totaling roughly \$40 million contingent on milestones, marks a momentous step forward in leveraging genome editing to combat neurogenetic diseases.

Professor Jiang is a distinguished physician scientist, who made significant contributions to Angelman syndrome (AS) research including the discovery of the UBE3A gene and the creation of the first two AS mouse models. Professor Jiang is also a leading researcher of H1-4 syndrome.

Professor Zhou is a leading expert in delivery systems for genetic medicine. His team developed the platform technology STEP (Stimuli-responsive Traceless Engineering Platform), a first-in-class non-viral delivery technology that accomplishes the efficient intracellular delivery of gene editors throughout the central nervous system, which has been a limiting factor for gene editing applications in patients.

Leveraging the STEP technology, a team led by Professors Jiang and Zhou devised a precise genome editing approach directly addressing the disease-causing genetic defects. Couragene exclusively licensed these innovative technologies and is poised to make a profound and lasting impact on the future treatment of genetic disorders. The SCGE grant supports the clinical translation of STEP-based genome editing for AS and H1-4 syndrome.

"Current approaches for delivery of biologic payloads have many limitations. This grant enables us to further

establish the STEP technology, a novel delivery approach with promise to overcome these challenges,” said Professor Zhou.

“As a physician I specialize in the treatment of patients with genetic disorders. Today there are no effective treatments for AS or H1-4 syndrome, leaving affected families with few options,” said Professor Jiang. “This NIH-funded research is critical to advancing technologies that could one day lead to more effective therapeutic options, and quite possibly cures.”

Couragene’s leadership congratulates its co-founders on the achievement. The development of effective treatments for AS and H1-4 syndrome using STEP will revolutionize the delivery of genetic medicines, giving hope to patients awaiting an effective therapy.

“STEP is a versatile platform technology with the capacity to deliver various payloads. I anticipate groundbreaking therapies beyond the diseases mentioned, and I take great pride in Couragene's role in their development,” said Dr. Amy Liao, CEO of Couragene.

#### About Couragene

Couragene is a biotechnology company committed to building first-in-class in vivo genetic therapies using its proprietary delivery platforms. Couragene was co-founded in 2022 by biotech veteran and CEO Dr. Amy Liao, who previously co-founded and led GENEWIZ for over 20 years through its acquisition by Azenta Life Sciences.

To learn more visit [www.couragene.com](http://www.couragene.com).

Amy Liao, PhD

**[Info@couragene.com](mailto:Info@couragene.com)**

Source: Couragene