June 3, 2025

Press Release

C4U Corporation

C4U Secures up to $14M non-dilutive Grant
from the Japanese Agency for Medical Research and Development

C4U Corporation ('C4U') is pleased to announce that our ongoing research and development of a hematopoietic stem cell transplantation therapy for primary immunodeficiency utilizing CRISPR-Cas3 genome editing technology, has been selected for approximately 14 million USD (2 billion JPY) in non-dilutive grant funding by the Japanese Agency for Medical Research and Development (AMED) as part of its 'Strengthening Program for Pharmaceutical Startup Ecosystem'

C4U will utilize a total up to approximately 20 million USD (3 billion JPY), including up to 14 million USD (2 billion JPY) from AMED grant mentioned above, to demonstrate the safety and efficacy of this therapy in clinical trials for primary immunodeficiency diseases.
Through the promotion of this research and development, it is expected that autologous hematopoietic stem cell genome editing therapies will be established as a platform technology, thereby expanding the possibility of applying *ex vivo* genome editing therapy to various diseases. Additionally, systematizing safety evaluations, including off-target evaluations, will help ensure the safety of CRISPR-Cas3 technology-based therapies, including *in vivo* therapies.

About C4U Corporation

C4U is a privately held biotech company based in Osaka, Japan, and is focused on the development of safe and efficient gene therapies utilizing its proprietary next generation CRISPR-Cas3 gene editing platform. In comparison to the CRISPR-Cas9 platform, CRISPR-Cas3 presents the distinct benefits of: 1) no off-target by the higher selectivity of deletion site (improved safety); 2) efficient knockouts by the larger deletion of gene sequences; and 3) an entirely independent patent portfolio. C4U has been granted a worldwide exclusive license to CRISPR-Cas3 by Osaka University for use in eukaryotic cells thus simplifying sublicensing transactions which is in sharp contrast to the complex and heavily litigated CRISPR-Cas9 patent landscape.
https://www.crispr4u.jp/en/

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