

## **A new hope for epidermolysis bullosa (“butterfly children”) thanks to novel CRISPR-Cas3 platform**

**Osaka, Japan, and Lugano, Switzerland, October 30th, 2023** – [C4U](#) Corporation (C4U) and [Healiva](#) SA (Healiva) are pleased to announce that they have entered into a strategic alliance agreement regarding the development of CRISPR-Cas3 derived therapeutic solutions for Epidermolysis Bullosa ([EB](#)), also known as “butterfly children”.

Epidermolysis bullosa is a rare genetic skin disorder characterized by highly sensitive skin that blisters and erodes with minimal friction or trauma, such as rubbing or scratching. This condition predominantly affects young children, causing them to experience severe pain and a substantial reduction in their quality of life due to the constant formation of blisters in response to minor injuries. EB affects approximately 20 out of every 1 million newborns in the United States where 25,000 to 50,000 young children are living with this debilitating condition.

“The collaboration aims to provide answers and hope to the individuals affected by EB, a devastating skin condition that renders the skin of the affected children as fragile as butterfly wings,” said Valerio M. Ferrari, Bioseutica Group Chairman & Co-Founder of Healiva.

The collaborative endeavor between Healiva and C4U will focus on the development of CRISPR-Cas3 based gene therapy for EB. In this collaboration the parties will work together to develop a gene therapy for EB with a staged development process to maximize speed and approval to perform clinical studies. By combining their complementary expertise, resources, and shared vision, both entities will strategically collaborate to enhance the quality of life for young children globally affected worldwide by this rare skin condition.

“By utilizing its proprietary CRISPR-Cas3 technology, a new generation of gene editing technology, C4U’s focus is to develop alternative and complete cures to genetic diseases. Skin diseases, especially EB, are relevant targets for the CRISPR-Cas3 platform, and we believe that we will bring a bright future to EB patients throughout the world,” said Akimitsu Hirai, President & CEO of C4U.

"This collaboration integrates gene therapy prospects with our comprehensive cell therapy portfolio, leveraging our expertise in autologous ([Epidex](#)<sup>®</sup>) and allogenic therapy technologies and the capabilities of our manufacturing infrastructure," articulated Dr. Priyanka Dutta Passecker, Co-Founder & CEO of Healiva.

### **About Healiva SA**

Healiva SA ([www.healiva.com](http://www.healiva.com)) is a Lugano, Switzerland-based biotechnology company founded by Bioseutica and Dr. Priyanka Dutta Passecker in 2020.

Healiva’s Wound Care & Skin Diseases proprietary technology platform, including all related manufacturing technologies and automation processes, is focused on therapies and cures for acute and chronic wounds. The affordable end-to-end approach by combining cell therapies, autologous (EpiDex<sup>®</sup>: an autologous epidermis derived from the patient’s own hair cells) and allogenic cell therapy (an optimal mixture of skin fibroblasts and keratinocytes produce and release active growth factors into the wound,

stimulating the patient's own cells to repair and close the wound) and medical devices to deliver personalized and precision medicine in wound care.

### **About C4U Corporation**

C4U Corporation ([www.crispr4u.jp/en/](http://www.crispr4u.jp/en/)) is a privately held biotech company based in Osaka, Japan, and focused on the development of safe and efficient gene therapies for rare diseases, including pediatric, based on its proprietary next generation CRISPR-Cas3 gene editing platform. The CRISPR-Cas3 technology platform is similar to CRISPR-Cas9 in terms of its engineering, efficiency, and delivery methods and has already been validated both *in vitro* and *in vivo*. It presents the distinct benefits of: (1) no off-target deletions (improved safety); (2) efficient knockouts of large, programmable gene sequences; and (3) entirely independent patent portfolio, which has been exclusively licensed worldwide to C4U by Osaka University for use in eukaryotic cells, thus simplifying sublicensing transactions in sharp contrast with the complex and heavily litigated CRISPR-Cas9 patent landscape.

### **Media Contacts:**

C4U Corporation: Katsuyoshi Akiyama, PhD, Director of Business Development ([info@crispr4u.com](mailto:info@crispr4u.com))

Healiva SA: Priyanka Dutta Passecker, PhD, CEO ([contact@healiva.com](mailto:contact@healiva.com))