

Info Sheet for Technical description

No. 0014

Organization

* Mandatoty fields

Name of Organization*	C4U Corporation		
Address, City, States, Zip, Country*	Yamadaoka 2-8, Suita, Osaka, 565-0871, JAPAN		
URL	https://www.crispr4u.jp/en/		
Brief Descriptions of Organization* (Approx. 100 words)	C4U is a biotechnology venture company from Osaka University, engaging in research and development of novel genome editing technology, CRISPR-Cas3. Utilizing newly invented cutting-edge genome editing technology, C4U promotes the development of new treatments for various diseases, including genetic diseases. C4U also collaborates with other companies and is working to develop platforms for applications in other industries.		
Contact address	Name*	Kohei Kishi	
	Department* / Position	Business Development / General Manager	
	E-mail* / TEL	k.kishi@crispr4u.com / +81-80-4384-9466	

What kind of technology do you want to offer? *

- ☒ **A.** Clinical Development Pipelines
- ☐ **B.** Regenerative Medicine-related Consumables / Instruments / Materials / CDMO Services etc.
- ☒ **C.** Platform Technologies(*) that are not included in the above (Group B)
- * Peripheral technologies that contribute to a significant improvement in productivity throughout the value chain of pharmaceuticals, from research and development to manufacturing and ultimately market launch.

→ Please see **Sheet [A]**→ Please see **Sheet [B]**→ Please see **Sheet [C]**

If you agree to the following, please check "Yes" below. *

The technologies introduced in this 'Info Sheet' are in the public domain, as they have been published in research papers or have related patent applications.

- ☒ Yes

Do you have any collaborations/partnerships with pharmaceutical companies?

- ☒ Yes
- ☐ No

If you have already received funding from VCs or other sources, up to which stage has the investment round progressed?

- ☐ Angel / Seed (including AMED/JST grants)
- ☐ Series A
- ☒ Series B
- ☐ Series C
- ☐ Series D or further advanced stages

Do you agree to leave your presentation materials at FIRM hands and entrust us to make use of them for the purpose of promoting your partnering opportunities? *

Options*	Comments
☒ Yes	
☐ No	

Filled in by*

Date*

Kohei Kishi
2025/9/3

Sheet [A] Clinical Development Pipelines**Info Sheet for Technical overview**

No. A-0014-1

* Mandatoty fields

Title***DMD (Duchenne Muscular Dystrophy)****Development Phase***

- | | | |
|--|--|---|
| ☒ Basic Research | ☒ Drug Discovery | ☐ Pre-Clinical |
| ☐ Clinical Trial (Phase I) | ☐ Clinical Trial (Phase II) | ☐ Clinical Trial (Phase III) |
| ☐ Review | ☐ Others | |

Diesease Area*

- | | | |
|---|---|---|
| ☐ Cancer | ☐ Central nervous system | ☐ Ophthalmology |
| ☒ Musculoskeletal | ☐ Endocrine / Metabolism | ☐ Cardiovascular |
| ☐ Urogenital | ☐ Digestive organ | ☐ Blood |
| ☐ Infection | ☐ Dermatology | ☐ Immunity |
| ☐ Otolar yngology | ☐ Respiratory | ☐ Others |

Description*

CRISPR-Cas3 is developed for treating DMD by exon 45 skipping at in vivo setting.

Filled in by*

Kohei Kishi

Date*

2025/9/2