

Nanomachines that carry nucleic acid drug for the fundamental treatment of hereditary intractable disease.

Launch crowdfunding for R&D and disease enlightenment

1. Begin Research and Development of nucleic acid drug that leads to the fundamental treatment of designated intractable disease "cystic fibrosis".
2. Start the recruitment of crowdfunding in partnership with Academia Mist.
3. Connect to external constituencies, not only for raising research funding but also to social enlightenment of "cystic fibrosis".

To the Press and whom it may concern,

Kawasaki Institute of Industrial Promotion Foundation Innovation Center of NanoMedicine (Director-General: Kazunori Kataoka, Location: Kawasaki-ku, Kawasaki City, Abbreviation: iCONM) is planning to conduct research to suppress the expression of genes that cause the onset of cystic fibrosis using nano-sized micelle (nanomachines) loaded with a nucleic acid drug to make it cystic.

Cystic fibrosis is a rare disease that occurs at a rate of 1 in 600,000 people in Japan, and highly viscous secretions are excessively secreted in major organs throughout the body, such as the lungs and the pancreas. It is a disease that causes various pains such as dyspnea and digestive disorders. Currently, there is no effective drug for its fundamental treatment, and it is classified as a designated intractable disease by the Ministry of Health, Labor and Welfare.

It is identified that the number of recognized patients in Japan is 40, but there are many patients who are mild, and / or not aware of the disease, thus we recognize that social awareness and understanding of this disease is important. Please refer to the following site for details. (<http://www.nanbyou.or.jp/entry/4531>)



Y a n g   W e n q i a n

Cystic fibrosis is known to be a disease which is based on the mutation of CFTR gene, therefore, it is hypothesized that nucleic acid drugs that suppress the expression of the mutant gene are effective for radical treatment. However, nucleic acid drugs are so unstable that they are not able to reach the target gene sufficiently. At our center, the results of basic research showing therapeutic effects on brain tumors by encapsulating nucleic acid drugs in nanomachines designed to pass through the blood-brain barrier were published in the UK magazine Nature Communications in April this year.

(<https://www.nature.com/articles/s41467-019-09856-w>).

Similarly, a doctoral student Yang Wenqian from iCONM / Kataoka-Kinoh Lab is planning research to design a nanomachine that acts on the CFTR gene and so demonstrate the above hypothesis.

Serving as awareness building for cystic fibrosis amongst the wider population, we started cloud funding today in collaboration with Academist Inc. (CEO: Ryosuke Shibato, Location: Chiyoda-ku, Tokyo).

Please go to <https://academist-cf.com/projects/129> for more details.

In the Integrated Innovation Strategy Promotion Conference 2019, the establishment of an innovation ecosystem similar to Boston and Singapore in Japan is regarded as critical. However, to activate the system positively, it is a necessary that the funding initiative is successful. Moreover, since the crowdfunding plays an important role in disease awareness, we hope that the significance of crowdfunding will be further spread in Japan.

#### Public Interest Incorporated Foundation Kawasaki Institute of Industrial Promotion

It was established in 1988 funded 100% by Kawasaki City in the purpose of dealing with the hollowing out of traditional industry and the change of demand structure. As such, it contributes the local economy

In order to realize market development, transformation into R&D-type companies, training of technological skills to support it, training of human resources, understanding of market needs, etc., utilizing the functions of Kawasaki City Industrial Promotion Hall, promoting exchange information of regional industries, advancement of technology by establishment of R&D organization and business exchange, training of creative human resources by training sessions, promotion of business such as expansion of sales channels by exhibition business, contributing to the revitalization of regional economy.

<https://www.kawasaki-net.ne.jp/>

#### Innovation Center of NanoMedicine

As a leading facility at the core of the Life Sciences field at King SkyFront, the Kawasaki Institute of Industrial Promotion which utilizes the measures and policies of the country started operation of the Innovation Center of NanoMedicine (iCONM) at the request of Kawasaki City in April 2015. It is equipped with state-of-the-art facilities and experiment apparatus that enables R&D related to organic synthesis, fine processing and preclinical studies. It is a unique research center that aims to promote open innovation through industry-university-government and medical-engineering collaboration.

<https://iconm.kawasaki-net.ne.jp/>

#### KING SKYFRONT: Tonomachi International Strategic Hub

Focusing on Life Sciences and environmental research fields where global growth is expected, the King SkyFront is an open innovation hub that creates new industries from world-class research and development.

The “KING” is an acronym of “Kawasaki Innovation Gateway” and “Tonomachi” (Tono” referring to the Japanese word “king”) and “SKYFRONT” indicates the location in front of Haneda Airport and that this area is connected to the world. A bridge will be built over the Tamagawa River in 2020, which will make this name more realistic.

<https://www.king-skyfront.jp/>

## **academist Inc.**

The academist offers academic crowdfunding service and manages academic media “academist Journal” with mission to contribute to academic development by realizing an open academic industry and maximizing the value of researchers. The academist is the first crowdfunding service dedicated to specialize research funds in Japan. About 100 researchers have challenged crowdfunding projects and succeeded to obtain a total of one hundred million yen. A monthly bases crowdfunding “academist Fanclub” for graduate students’ research and daily expenses has started in January 2019.

In addition, the academist Journal publishes a number of columns written by researchers themselves and interview articles of researchers who are active in the forefront, as popular academic media in which the researchers play a leading role.

About 400 researchers have cooperated so far, from young researchers to top runner researchers.

- Academic crowdfunding sight 「academist」 <https://academist-cf.com/>
- Academic media 「academist Journal」 <https://academist-cf.com/journal/>