Strengthening Program for Pharmaceutical Startup Ecosystem

Japan Agency for Medical Research and Development

AMED

Strengthening Program for Pharmaceutical Startup Ecosystem

Program Outline

Most new drugs in recent years have been developed by pharmaceutical startup companies. For example, the first development of vaccines was succeeded by startups in the pandemic of COVID-19. The development of new drugs requires a large amount of funding. In Japan, it is more difficult to secure the necessary funds in the pharmaceutical startup ecosystem, compared with those in Europe or the United States.

To break away this situation, under the "Strategy for Strengthening the Vaccine Development and Production System" approved by the Cabinet in June 2021, this Program was established to support Pharmaceutical Startups that develop for practical application of technologies related to vaccines and therapeutics for infectious diseases. Furthermore, in October 2022, the "Priorities for Comprehensive Economic Measures for the Implementation of the GRAND DESIGN AND ACTION PLAN FOR A NEW FORM OF CAPITALISM" included the following statement regarding this Program: "In the future, we will strengthen its support by expanding the scope to include drug discovery fields other than those related to infectious diseases, where it is difficult to raise funds."

To solve the shortage of large-scale development of fundings, this Program supports pharmaceutical startup for development and commercialization, especially those engaged in non-clinical, Phase I, Phase II or exploratory clinical trials on the condition that they also receive funding from venture capital firms registered by AMED (hereinafter referred to as “Registered VC”) specializing in drug development and providing hands-on business management and commercialization support.

In particular, to achieve sufficient sales and growth of pharmaceutical startups, we will actively support plans to commercialize in overseas markets in addition to Japan.

Program Supervisor (PS)

Former chairperson of Drug Evaluation Committee
Japan Pharmaceutical Manufacturers Association (JPMA)

INAGAKI Osamu

Program Officer (PO)

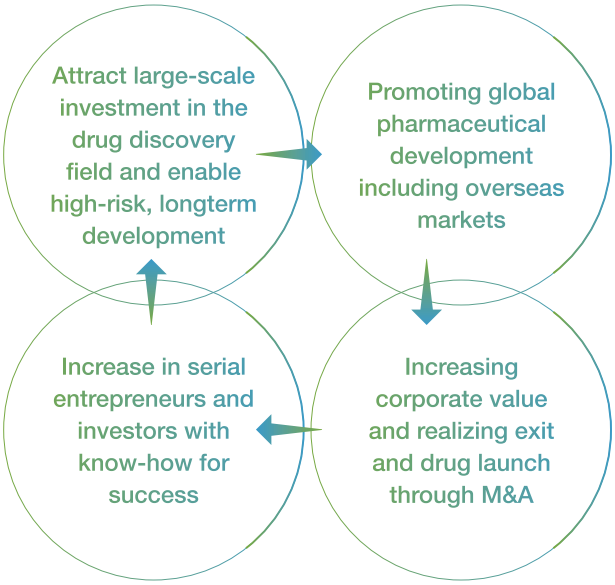
President
Gallasus, LLC

HASHIMOTO Chika

Contents

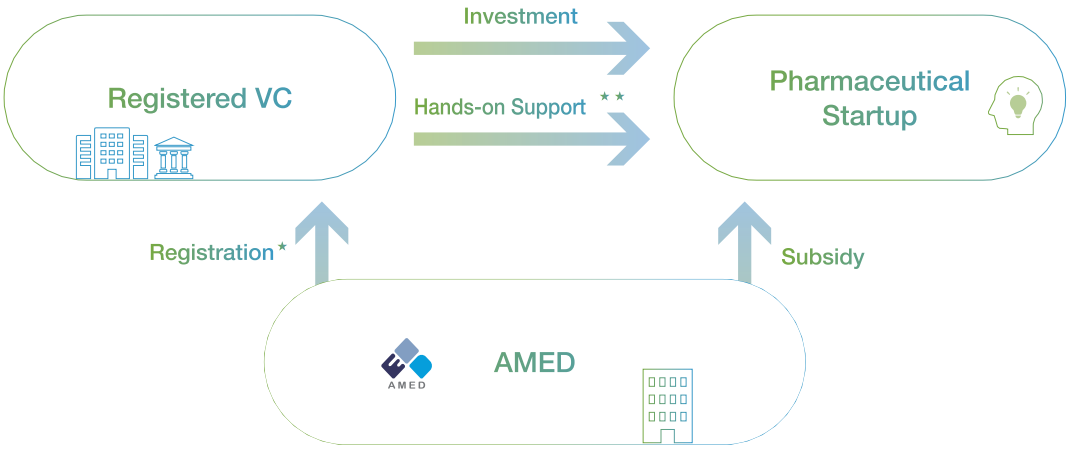
Program Objective

In order to strengthen the Pharmaceutical Startup Ecosystem in Japan, we aim to build the effective and synergistic cycle by creating as many successful examples of global standards as possible.



Program Scheme

In this Program, AMED subsidizes the practical development of pharmaceuticals conducted by Pharmaceutical Startups in which registered VCs invest more than 1/3 of total expenses covered by the Subsidy.
This Program makes two stages of calls for proposals, which are Call for Proposals for VC Registered by AMED (**(ⅰ)Call for Proposals for VC Registration**,) and Call for Proposals for the practical development of pharmaceuticals conducted by Pharmaceutical Startups invested by Registered VCs (**(ⅱ)Call for Proposals from Pharmaceutical Startups**).



* Registration ····· Registration of VCs with track records of investment and support, etc. in the drug discovery field

** Hands-on Support ····· Support according to the growth stage of Pharmaceutical Startups from the perspectives on management, development and technology, and regulatory affairs

Call for Proposals for VC Registration

Requirements for certified VCs

- As a lead investor, invest more than 1 billion yen in the startup from the initial investment to the end of the Subsidized Project Period.
- Consistently support the startup as a lead investor during the Subsidized Project Period.

* The definition of "lead" in this program is, in principle, the investor who has the largest amount of investment during the period covered by this program, and the investor who plays a leading role in fundraising and hands-on activities.

Registration Period

- 2 business years from the date of registration (Up to the end of the business year in which this program ends)
- An evaluation is conducted every 2 business years to determine whether or not registration can be renewed. No limit on number of renewals.

Evaluation Items

- Conformity to Program Objectives
- Sourcing capability
- Ability to carry out fundraising
- Hands-on capability

Mandatory Requirements

(ⅰ) Investing 1/3 or more of its total investment as a VC in the drug discovery field in the last 5 years.
(If the applicant has a fund specialized in investing in the drug discovery field, or if the applicant is evaluated as capable of providing particularly high-quality support to Pharmaceutical Startups in the evaluation items, the applicant will be considered for reviewing even if the applicant does not satisfy (ⅰ)).

(ⅱ) The applicant must have a track record of supporting clinical trials conducted by the Pharmaceutical Startup in which it has invested as a Lead VC.
(In the cases of a newly established VC or fund, the requirement (ⅱ) may be subject to review in light of the past performance of the individual[*1] to whom the VC belongs.)

(ⅲ) The applicant must have a track record of dispatching directors to the Pharmaceutical Startups in which it has invested as a Lead VC.
(In the case of a newly established VC or fund, the requirement (ⅲ) may be subject to review in light of the past performance of the individual[*1] to whom the VC belongs.)

(ⅳ) Members[*2] who make investment decisions or provide expert advice on investment decisions as hands-on members have experience in drug development at pharmaceutical companies, etc. (regulatory affairs, business development, development planning, etc.) or have important experience (review by organizations such as PMDA and FDA, etc.) in advancing drug development.

(ⅴ) Members[*2] who make investment decisions or provide expert advice on investment decisions as hands-on members have experience in global drug development (experience in conducting global clinical trials, experience in providing hands-on support for global clinical trials, etc.).

*1 Members who make investment decisions or provide expert advice on investment decisions as hands-on members.
*2 General partner, partner, etc.

Plan of call for Proposals

The Call for Proposals are scheduled to be held periodically several times a year.

*Please check the latest Application Guidelines

List of Registered VCs

Catalys Pacific, LLC

Website

Contact info“AT”catalyspacific.com

Website https://catalyspacific.com/

Catalys Pacific is an independent venture capital firm focusing on early-stage investments in life sciences. The firm’s mission is improving patient lives worldwide by driving impact and innovation in healthcare and contributing to advancements in life sciences. Founded in 2019, the firm acts as a trusted regional partner, building companies and catalyzing transpacific partnerships to fulfill its mission. The Catalys Pacific team is based in Tokyo, Japan and San Francisco, California.
Learn more at website.

CATALYS PACIFIC

Person in charge

TAKAHASHI Takeshi

DCI Partners Co., Ltd.

Website

Contact dcip_info“AT”daiwa.co.jp

Website https://www.daiwa-inv.co.jp/dcip/en/

We have over 20 years' history of investing in biotech start-ups.
We are currently managing the largest size of the life science fund in Japan and playing a key role in supplying funds to promising start-ups.
We aim at increasing the value of our portfolios by providing various hands-on supports utilizing our team's expertise of clinical development, intellectual property and business development as well as our extensive network across academics, biotech and pharmaceutical industry primarily in Japan and Taiwan.
Beyond classical venture capital firms, we act as a platform to incubate seeds and create new start-ups that have great potential.

DCI Partners

Person in charge

YOKOTA Junichi

TANAKA Shoma

NIIMI Yuka

D3LLC

Website

Contact info“AT”d3growth.com

Website https://www.d3growth.com/

D3 LLC is a Japanese venture capital firm specializing in bio-healthcare. Our investment professionals with a background in science (bio, med, pharma) and global business experience at McKinsey or US based VC/PE funds provide global standard bio-healthcare investments that have not been made in Japan before. We actively collaborate with investors and business companies in Japan and abroad, with the purpose of "contributing to global medical health from Japan". If you are an investor or a company that sees the potential of science and startups in Japan, please feel free to contact us.

D3 LLC
Discovery Development Deployment

Person in charge

NAGATA Tomoya

WATATANI Kenji

HASEBE Yasuaki

＊Replace “AT” with “@”.

Eight Roads Ventures Japan

Website

Contact admin“AT”eightroads.com

Website https://eightroads.com/en/

Global venture capital firm with deep analysis and understanding to science and technologies that can solve unmet medical needs and social problem. Stage agnostic investment from pre-foundation stage or seed stage to later growth stage. Leveraging global footprint as a fund and co-work with F-Prime Capital in US, a sister fund, Eight Roads Ventures Japan provides Japanese biotech startups with patient capital and hands-on support for global business expansion.

Person in charge

KOMOTO Shinichiro

ASHIDA Hiroki

Fast Track Initiative, Inc.

Website

Contact kkiriya“AT”fti-jp.com

Website https://us.fasttrackinitiative.com/

Established in 2004, Fast Track Initiative, Inc. (FTI) is a life science venture capital firm focusing its investments on early-stage startups with a strong focus on biotech and therapeutics. FTI has a strict mission to make a ground-breaking impact on the quality of our life and human wellness through investments across a broad range of areas in the life science community including therapeutics, diagnostics, digital health, healthcare services, and more. The company provides valuable, hands-on support to its 30+ portfolio companies through business and R&D strategy, partnering, investor syndication, and more, including access to its network of professionals in Japan.

Person in charge

KIRIYA Keita

F-Prime Capital Partners

Website

Contact admin“AT”eightroads.com

Website https://fprimecapital.com/

Global venture capital firm with 20+ years experience in providing biotech startup with patient capital and hands-on support, based in US and cross-boarder to Japan, China, India and Europe. Deep analysis and understanding to science and technologies that can solve unmet medical needs and social problem. Stage agnostic investment from pre-foundation stage or seed stage, to later growth stage. Japan is one of focusing areas by co-working with the sister fund, Eight Roads Ventures Japan.

Person in charge

KOMOTO Shinichiro

ASHIDA Hiroki

Robert Weisskoff

Brian Yordy

*Replace “AT” with “@”.

JAFCO Group Co., Ltd.

Website

Contact https://jafco.co.jp/english/contact/ | Website https://jafco.co.jp/english/

We understand and deeply empathize with the aspirations of entrepreneurs to realize their businesses. We have executed investments of several hundred million yen, mainly in the seed/early stage, and have continued to execute investments after the initial round. The investment theme of the Investment Group for Academy & Life Science is to invest in businesses that will create the mainstream of the next generation, capturing the restructuring of the industrial structure through technological innovation. The main areas of investment are drug discovery, medical devices, and healthcare, which are essential to society, have high growth potential, and are expected to expand their markets, and support the challenges of companies that aspire to innovative and creative management. In the drug discovery business, we have been actively supporting startups since the dawn of investment in Japan, and have accumulated a great deal of knowledge and network. We concentrate our investments on next-generation innovative drug discovery technologies and seeds that can be delivered to a large number of patients through technological innovation.

JAFCO

Person in charge


KOBAYASHI Taira


MIURA Kengo


ISHIMOTO Yuki

KITAZAWA Tomotake

MIYAKAWA Yukari

KOZAWA Takafumi

JIC Venture Growth Investments Co., Ltd.

Website

Contact info_lifescience“AT”j-vgi.co.jp | Website https://www.j-vgi.co.jp/en/

JIC Venture Growth Investments Co., Ltd. (JIC VGI), a venture capital arm of Japan Investment Corporation group (JIC), strategically drives Japanese innovation and global competitiveness. Focusing on diverse investment stages in life science, JIC VGI nurtures startups through a robust network encompassing venture capitals, pharmaceutical companies, and government agencies. We contribute to the strengthening of the venture ecosystem and the creation of new drugs originating in Japan.

VENTURE GROWTH INVESTMENTS

Person in charge


URUNO Yoshiharu


SUZUKI Hanae


TSUZUKU Takuma

Kyoto University Innovation Capital Co., Ltd.

Website

Contact ueno.hiroyuki“AT”kyoto-unicap.co.jp
https://www.kyoto-unicap.co.jp/contact/ | Website https://www.kyoto-unicap.co.jp/en/

Kyoto University Innovation Capital Co., Ltd is the venture capital firm established as a wholly owned subsidiary of Kyoto University. We aim to contribute creating new industries that will lead the next generation by utilizing the research results of Kyoto University and Japanese national universities via investments. We've been supporting various biotech companies that promote the practical application from innovative university research results. In the “Strengthening Program for Pharmaceutical Startup Ecosystem”, we will support the research and development and business development of biotech companies to create new vaccine and new therapeutic option from the point of view of venture capital.

KYOTO-iCAP KYOTO UNIVERSITY INNOVATION CAPITAL Co.,Ltd.

Person in charge


UENO Hiroyuki


YAGI Nobuhiro


KONO Osami

*Replace “AT” with “@”.

Mitsubishi UFJ Capital Co., Ltd.

Website

Contact ninteivc“AT”mucap.co.jp

Website https://www.mucap.co.jp/english/

Mitsubishi UFJ Capital invests in a wide range of industries as the venture capital of the Mitsubishi UFJ Financial Group. In the life science field, we have continuously established funds totaling ¥50 billion, including the Mitsubishi UFJ Life Science Fund IV (¥20 billion), since the first fund in February 2017. Our life science funds pursue the strategy of facilitating the creation of a drug discovery ecosystem in Japan. Specifically, besides follower investments in start-ups, we aim to support various processes from the nurturing of seeds for drug discovery to clinical development, by (1) drug discovery in academia: investing in start-ups that spun off from universities, (2) carveouts: investing in start-ups that were carved out from pharmaceuticals and specialize in specific technology or disease fields, and (3)open-innovation projects between academia and pharmaceuticals. Through these activities, we trust that we could contribute to advances in pharmaceutical development. We have several capitalists with pharmaceutical backgrounds, and we cover a wide range of drug discovery processes and disease areas in pharmaceuticals. In addition, we have concluded comprehensive agreements with specialized companies and organizations that can consult on intellectual property, non-clinical trials, pharmaceutical manufacturing, clinical development strategies, and medical needs, and have established a system that allows consultation before investment.

MUFG
Mitsubishi UFJ Capital

Person in charge


HASEGAWA Hiroyuki


SHIMASAKI Makoto


SHINOZAKI Mikihiko

SEKIYA Michiko

MIYOKO Nobuhiko


KAKIUCHI Akihito

IZAWA Yosuke

KUBO Yuki

MP Healthcare Venture Management, Inc. (MPH)

Website

Contact https://www.mp-healthcare.com/contact

Website https://www.mp-healthcare.com/

MP Healthcare Venture Management (MPH) is a Boston-based Lifesciences venture capital firm affiliated with Mitsubishi Tanabe Pharma Corporation (MTPC). MPH invests globally in early-stage companies developing innovative therapeutics and platform technologies. Our focus therapeutics areas are neurodegeneration, immunology, oncology, and rare diseases. Please see the detail of our current portfolio companies.

MP Healthcare
Venture Management, Inc.

Person in charge


OZAWA Shota

Newton Biocapital Partners GK

Website

Contact https://newtonbiocapital.com/en/contact

Website https://newtonbiocapital.com/en/

Newton BioCapital ("NBC") is a venture capital fund with offices in Belgium and Japan investing in pre-clinical and clinical stage early stage companies in the life sciences sector in Europe and Japan. With a focus on reducing the burden on patients and society, NBC's strategy is to leverage innovation in the treatment of chronic diseases. The team members have a wealth of experience and a wide range of knowledge in the fields of chemistry, industry, and venture capital.

NEWTON BIOCAPITAL

Person in charge


SUZUKI Sadashi

*Replace “AT” with “@”.

Outlines of Registered VCs

OSAKA University Venture Capital Co., Ltd.

Website

Contact info“AT”ouvc.co.jp

Website https://www.ouvc.co.jp/en/

Osaka University Venture Capital Co., Ltd. (OUVC) is a venture capital firm that supports ventures utilizing outstanding research outcomes not only from Osaka University but also from other national universities. Until now, we have track records of investing primarily in the medical and pharmaceutical fields, and we have provided comprehensive support from startup to exit assistance. In recent times, we are focusing on finding CxO talents. We also take advantage of being a venture capital firm 100% funded by Osaka University and place emphasis on specialized hands-on support, including assistance with regulatory authorities, in collaboration with Medical Center for Translational Research Osaka University Hospital. We are ready to accept consultations from researchers at national universities who are not yet fully prepared for entrepreneurship, particularly focusing on national university settings. For more detailed information and contact details, please refer to our website.

Person in charge

UOTANI Akira

UEHIRA Masahiro

Remiges Ventures, Inc.

Website

Contact info“AT”remigesventures.com

Website https://remigesventures.com/

Remiges is a venture capital operated by the investment team based in Japan and the US and invests in and supports drug discovery ventures. Our investment target is all diseases' area and all modalities. We invest in early-stage drug discovery ventures (Seed to Series A/B) that are developing advanced drugs. In addition, we create new ventures based on technologies from universities and other institutions. When investing, we mainly invest and support as a lead investor. Through participation in the board of directors, we are involved in a wide range of management including business strategy planning, external alliances and EXIT activities. Also, in order to increase the value after investment, we implement various measures such as strengthening the management team by hiring management personnel, strengthen intellectual property by conducting detailed examination, providing input in development strategy and clinical trial design, fundraising activities including inviting new investors, and introducing external advisors. We provide direct value-added services in drug development by assisting in accessing needed technologies, scientific expertise, and outsourced services to help ventures develop their business.

Person in charge

MATSUMOTO Kyoko

Saisei Ventures LLC

Website

Contact info“AT”saiseiventures.com

Website https://www.saiseiventures.com/

Saisei Ventures LLC is a leading venture capital firm focused on growing and harnessing the Japanese biotechnology ecosystem; a region rich in opportunities and potential. We create ventures that start from bold ideas and empower dynamic entrepreneurs by filling in technical, operational, or financial gaps. Our approach combines Western expertise and Japanese innovation to build globally competitive companies that will have the greatest impact on patient lives. We have offices in Japan and Boston, and our first fund is focused on fostering companies related to regenerative medicine, from fundamental technologies to groundbreaking cell and gene therapies originating in Japan.

Person in charge

SAITO Hikaru

Jonathan Yeh

*Replace “AT” with “@”.

The University of Tokyo Edge Capital Partners Co., Ltd.

Website

Contact utec-kanri“AT”ut-ec.co.jp

Website https://www.ut-ec.co.jp/english/

Since its inception in April 2004, UTEC, in collaboration with entrepreneurs and researchers, has been investing in numerous startups that are tackling global challenges and advancing human progress. In the field of drug discovery, UTEC's investments span a diverse range of startups focusing on assets such as peptides, nucleic acids, cellular genes, and small molecules, as well as those equipped with advanced drug discovery platform technologies. These efforts are directed towards addressing areas of disease where new treatments are eagerly anticipated. Our members of highly specialized professionals in life sciences and drug discovery leverage our expertise and networks across various domains, including research and development, clinical trials, regulatory affairs, business development, and management and administration. We contribute significantly to the business expansion of drug discovery startups, both within Japan and internationally.

UTEC

Person in charge

USAMI Atsushi

SHIOHARA Azusa

UTokyo Innovation Platform Co., Ltd.

Website

Contact https://www.utokyo-ipc.co.jp/en/contact/

Website https://www.utokyo-ipc.co.jp/en/

UTokyo Innovation Platform Co., Ltd. (UTokyo-IPC) is a wholly-owned investment company by the University of Tokyo, actively investing in venture projects that utilize the achievements of the University of Tokyo and other universities. We provide hands-on support from experienced capitalists with a rich history of pharmaceutical investments, business development, and exit strategies in the United States and Japan. We welcome contact with bio-tech ventures engaged in innovative technology development, ranging from preclinical to Phase 2 clinical trials.

UTokyoIPC
Innovation Platform for The University of Tokyo

Person in charge

OHORI Makoto

BINGO Atsuhiro

*Replace “AT” with “@”.

Call for Proposals from Pharmaceutical Startups

Target of Call for Proposals

Field		Scale of Expenses Covered by Subsidy (Including indirect costs and Registered VC investment)	Subsidized Project Period
#1	Innovative technological development for development of infectious disease vaccines and therapeutic drugs.	[amount of money] (upper limit) 10 billion yen (Accept even if the upper limit is exceeded) ＊AMED Subsidy covers up to 2/3 of the expenses.	Up to September 2031 (Set for each Adopted Project)
#2	Innovative technological development for development of pharmaceuticals etc. for diseases other than infectious diseases.		

- The applicant must have received, or be scheduled to receive in the future, investment from a Registered VC (must include the lead VC) in the amount of 1/3 or more of the expenses covered by the Subsidy.
- Pre-clinical study, Phase 1 clinical study, Phase 2 clinical study or Exploratory clinical study will be covered.
- "Pharmaceuticals, etc." includes pharmaceuticals and regenerative medicine products.
- You must have filed a domestic or foreign patent application for the final development candidate product. However, if you have not filed an application at the time of application for strategic reasons, please provide details of your strategy (development strategy, intellectual property strategy, business strategy, pharmaceutical strategy, etc.) in your proposal.
- Non-clinical studies will be conducted on the condition that there is a final development candidate to proceed to the clinic.
- If all Stage Gate Evaluations stipulated in the Subsidized Project Plan are passed, the Subsidized Project period will be up to September 2031.

Goals of this Program

- Completion of phase 2 clinical study or Exploratory clinical study (POC acquisition)
- If IPO & M&A is carried out during Subsidized Project Period, the Subsidized Project will be terminated in principle.

Evaluation Items

- Compatibility with the program's purpose
- Superiority and effectiveness of technology, etc.
- Development plans and goals
- Business plan
- Support plan by Registered VC

Plan of call for Proposals

The Call for Proposals are scheduled to be held periodically several times a year.

＊The retroactive period for investments from Registered VCs is from November 8, 2022 (Date of Cabinet decision on the FY2022 supplementary budget) until the time of application.

＊Please check the latest Application Guidelines

List of Adopted Projects

Subsidized Project	Business Operator	Registered VC	Page
Development of an innovative therapeutic agent for myotonic dystrophy type1 by a sequence-specific RNA binding protein targeting pathogenic CUG-repeat RNA	EditForce, Inc.	Newton Biocapital Partners	13
Development of RSV vaccine with novel antigen and adjuvant targeting TLR9 in pDC	Immunohelix Co., Ltd.	Remiges Ventures, Inc.	14
Development of ENDOPIN, a one-of-a-kind oral analgesic that activates the descending pain suppression pathway	BTB Drug Development Research Center Co., Ltd.	Kyoto University Innovation Capital Co., Ltd.	15
Clinical proof of concept study of OZTx-556, a human iPS cell-derived cardiomyocyte, in a global clinical trial for patients with severe heart failure	Orizuru Therapeutics, Inc.	Kyoto University Innovation Capital Co., Ltd.	16
Obtaining POC in global Phase II clinical trial of a viral vector-based gene therapy	Restore Vision Inc.	Remiges Ventures, Inc.	17

Subsidized Project

Development of an innovative therapeutic agent for myotonic dystrophy type1 by a sequence-specific RNA binding protein targeting pathogenic CUG-repeat RNA

Business Operator

EditForce, Inc.

General Manager,
CSO Board of Directors
NAKANISHI Osamu, Ph.D.

Registered VC

Newton Biocapital Partners

Advisor
Dr. WADA Michihiko

Overview

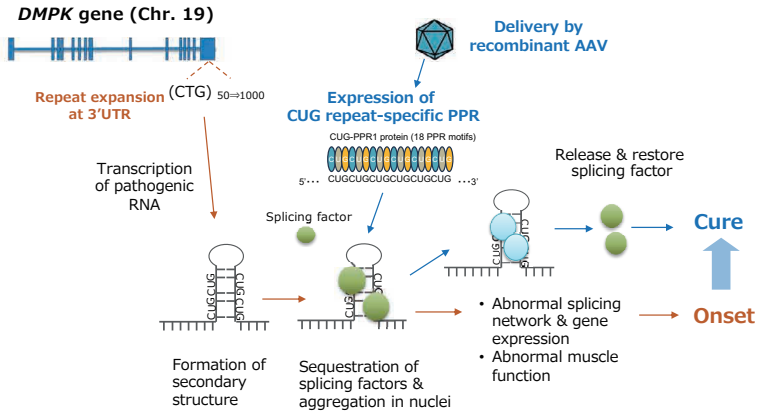
We have developed a unique technology to design artificial proteins by fusing a domain consisting of a plant-derived PPR (Pentatricopeptide Repeat) motif that can bind RNA in a sequence-specific manner with functional domains such as RNase and base editing enzymes.

The designed artificial proteins that can control RNAs that cause various diseases are under investigation for their therapeutic effects. The goal of this program is to develop a curative therapeutic agent by blocking the pathogenicity of CUG-repeat RNA that causes the intractable genetic disease myotonic dystrophy type 1 (DM1) by using our proprietary PPR technology.

Since the PPR technology can design proteins that bind to any RNA sequences, the success of this program is expected to provide a powerful platform technology in the future not only against disease-causing endogenous RNAs but also against exogenous RNAs of RNA viruses such as coronaviruses and dengue viruses.

Treatment of DM1 by PPR protein

- Myotonic dystrophy type 1 (DM1) is caused by abnormal splicing arising from sequestration of splicing factors essential for muscle function by expanded CUG repeat
- Delivery of a CUG repeat-specific PPR protein restores splicing factors and improves muscle function



Company Info

EditForce, Inc.

President and CEO
ONO Takashi, Ph.D.

We are researching and developing innovative gene-therapies, with our unique RNA editing technology ("PPR Platform Technology") utilizing PPR protein based on the study made by Kyushu University. PPR Platform Technology enables design of artificial proteins which will bind to targeted DNA/RNA sequences by applying PPR protein which is RNA combined protein in various plants with specific sequence and changing some amino acid of it. Off-target is the issue to overcome for DNA editing due to its irreversibility. With PPR Platform Technology, we can target specific genes at the RNA-level and control functions of the targeted genes. PPR Platform Technology has possibilities to take a different approach to gene controls which has not even been realized by the existing technologies. With the slogan of "New Tools Lead to a New World", our mission is to deliver safer and more reliable gene therapies to patients around the world suffering from genetic disorders as soon as possible.

Contact https://www.editforce.co.jp/en/contact/

Website https://www.editforce.co.jp/en/

Subsidized Project

Development of RSV vaccine with novel antigen and adjuvant targeting TLR9 in pDC

Business Operator

Immunohelix Co., Ltd.

Head of Research and Development

UNO Atsushi Ph.D

Registered VC

Remiges Ventures, Inc.

Principal

NONOMURA Kazuhiko

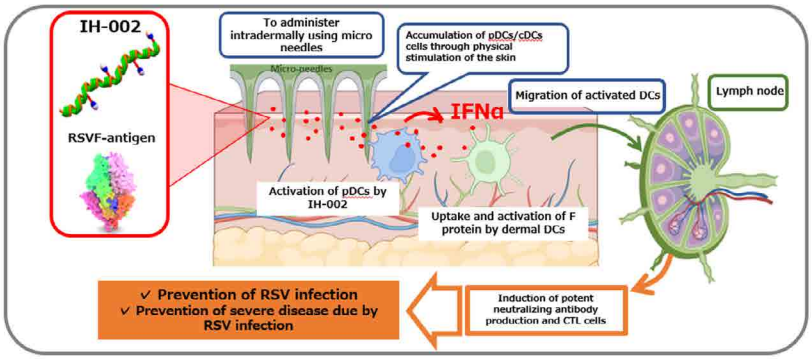
Overview

The COVID-19 pandemic reminded us of the importance of vaccine development. Having solid vaccine research and related technologies in Japan is quite important from the perspective of national security and crisis management. Immunohelix proposes to develop a novel safe and effective RSV vaccine using a novel highly active adjuvant targeting plasmacytoid dendritic cells pDC (IH-002) and a novel RSV antigen in collaboration with Profs. Ueno, Hashiguchi, and Nakajima at Kyoto University. IH-002 is a complex composed by a patent-protected novel TLR9 agonist IH-002 ODN and β-glucan SPG, which forms a triple helix structure.

This complex is captured by the β-glucan receptor Dectin-1 expressed on pDCs and stimulates TLR9 in the cells effectively. This is the very unique biological profile of IH-002, which other DDS technology cannot achieve.

In this project, the company plans to develop intradermal RSV vaccine with microneedles in a simple mixture formulation of IH-002 to directly stimulate pDCs in the dermis.

This is expected to produce high-affinity neutralizing antibodies by inducing a strong germinal center response in the lymph nodes through strong type I IFN production by pDCs. This is also expected to induce T cell responses such as cytotoxic T cells (CTL) and Th1 cells, which are important for antiviral activity, i.e., the establishment of robust acquired immunity. Through this project, the company aims to establish a manufacturing infrastructure which can produce GMP-grade IH-002. This will enable the company and Japan to supply it globally not only as an adjuvant for RSV vaccines but also as a universal adjuvant applicable to various infectious disease.



Company Info

Immunohelix Co., Ltd.

Representative derector

NAKAGAWA Atsuko

Immunohelix Co., Ltd. conducts research and development of pharmaceuticals, employing a drug delivery system technology grounded in the triple helix architecture of nucleic acids and sugar chains. The SPG (Schizophyllan) triple-helical technology allows drugs (e.g., nucleic acid drugs, medium and small molecules) to be bound to nucleic acids to form SPG complexes. The SPG complexes are selectively delivered via Dectin-1 (C-type lectin receptor), which is specifically expressed on the surface of antigen-presenting cells such as macrophages and dendritic cells among immune cells. This delivery technology has great potential for the creation of drugs in the therapeutic areas of the immune system, such as immunological diseases, cancer, organ transplantation, and infectious diseases, and our objective is to globally disseminate our delivery technology.

Contact https://www.napajen.com/en/contact/

Website https://www.napajen.com/en/

Subsidized Project

Development of ENDOPIN, a one-of-a-kind oral analgesic that activates the descending pain suppression pathway

Business Operator

BTB Drug Development Research Center Co., Ltd.

Chief Executive Officer

OGIKU Tsuyoshi Ph.D.

Registered VC

Kyoto University Innovation Capital Co., Ltd.

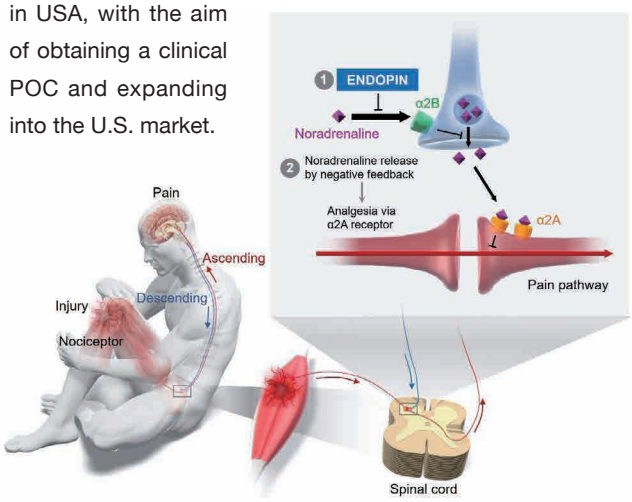
Head of Investment Department I

YAGI Nobuhiro

Overview

ENDOPIN is a candidate compound for analgesics with a completely new mechanism of action, which was discovered by Hagiwara et al. at Kyoto University Graduate School of Medicine from their original compound library using an original idea based on a discovery with a novel assay method. Animals, including humans, increase noradrenaline secretion in times of crisis and activate the adrenergic receptor α 2A-dependent descending analgesics pathway to escape crisis without feeling pain. ENDOPIN, a selective inhibitor of the adrenergic receptor α2B, increases noradrenaline secretion into the cerebrospinal fluid through negative feedback caused by α2B inhibition, activating the descending pain suppression pathway, thereby producing analgesic effects. Based on this new hypothesis of action, ENDOPIN was tested in various pain models, including postoperative pain, inflammatory pain, and cancer pain. ENDOPIN showed strong analgesic effects comparable to those of morphine, but even at more than 100 times the effective dose, ENDOPIN showed no central nervous system effects, respiratory depression, or behavioral changes, as seen with morphine or other opioid analgesics, were observed.

Therefore, ENDOPIN has the potential to be a breakthrough analgesic to solve the opioid crisis, which has been a significant medical and social problem in the U.S. and Europe. In this project, we will conduct Phase II clinical trials in USA, with the aim of obtaining a clinical POC and expanding into the U.S. market.



Company Info

BTB Drug Development Research Center Co., Ltd.

Chief Executive Officer
OGIKU Tsuyoshi Ph.D.

BTB Drug Development Research Center, Inc. is a venture company established in June 2020 to develop drug discovery seeds originating from Kyoto University. Currently, in collaboration with Kyoto University Graduate School of Medicine, we are developing drugs for pain, next-generation cancer immunotherapy, and genetic disease RNA therapeutics. We are always aiming to obtain clinical POC as fast as possible, expand into the global market, and expand the range of indications.

Contact info“AT”btb-newdrug.co.jp

＊Replace “AT” with “@”.

Website https://www.btb-newdrug.co.jp/en/

Subsidized Project

Clinical proof of concept study of OZTx-556, a human iPS cell-derived cardiomyocyte, in a global clinical trial for patients with severe heart failure

Business Operator

Orizuru Therapeutics, Inc.

Head of iCM Therapy Business Unit

NISHIMOTO Tomoyuki, Ph.D.

Registered VC

Kyoto University Innovation Capital Co., Ltd.

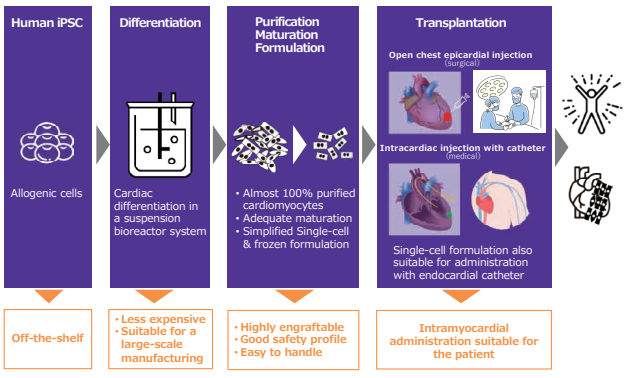
Head of Investment Department II

UENO Hiroyuki, Ph.D.

Overview

Severe chronic heart failure (CHF) is a major cause of morbidity and mortality in the world. The prevalence of severe CHF is estimated at around 10 million in the EU, US, China, and Japan in 2036. About half of these patients are refractory to pharmacotherapy. For these patients, heart transplantation will be the last resort, and the Left Ventricular Assist Device (LVAD) has been developed as a circulation assist device mechanically for the purpose of bridge-to-transplantation. Recently LVAD has been also approved for use as a destination therapy in Japan in addition to the bridging use, yet it is not radical therapy compared to the replacement by functioned healthy cells, which can be expected "cure". In these situations, pluripotent stem cell-derived regenerative therapy is expected to be an innovative therapy for treatment-resistant severe heart failure patients. So far, we have developed new technologies to effectively produce highly engraftable and highly purified cardiomyocytes from human iPS cells. We demonstrated that these cells, named OZTx-556, engrafted both in rodent and monkey myocardial infarction models, and improved their cardiac functions. We plan to initiate a clinical trial in Japan to confirm the safety of OZTx-556 from FY24.
It is expected that several hundred million cells will need to be transplanted so that the transplanted cells to become viable and function in the heart. The production of such a large number of cells at low cost is a major challenge to overcome to expand the use of iPS cell-derived cardiomyocyte therapy to many patients.

Our method of cardiomyocyte differentiation is based on a suspension culture, rather than the monolayer culture used by many competitors, and is suitable for deployment to a low-cost large culture system. The project aims to establish a large-scale manufacturing method with a view to a commercial scale. In addition, the development of a catheter, which would be a less-invasive to patients, will be advanced to select the most advantageous administration method for patients compared to the current open chest administration, and together, we will conduct a global Ph1/2 study and obtain PoC, aiming for the progress to late-stage clinical development and early approval.



Company Info

Orizuru Therapeutics, Inc.

President,
Representative Director
and CEO

NONAKA Kenji.
M.D., Ph.D.

Orizuru Therapeutics, Inc., founded in April 2021, is steadfast in its dedication to bringing hope for better health through the infinite power of science. To deliver cell therapies to patients, the company promotes the wide use of cell therapy products and innovative iPSC-related technology through the following activities:

1. Development of regenerative medical products through cell transplantation
2. Support for drug discovery research and development of regenerative medicine research infrastructure using iPSC-related technology

For details, please refer to website.

Contact https://orizuru-therapeutics.com/en/contact/

Website https://orizuru-therapeutics.com/en/

Subsidized Project

Obtaining POC in global Phase II clinical trial of a viral vector-based gene therapy

Business Operator

Restore Vision Inc.

President & CEO

KATADA Yusaku, MD, Ph.D.

Registered VC

Remiges Ventures, Inc.

Principal

NONOMURA Kazuhiko

Overview

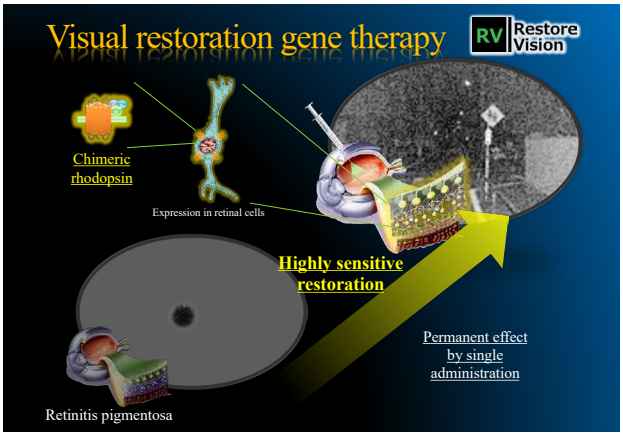
Retinitis pigmentosa (RP), a designated intractable and rare disease that is the second leading cause of blindness in Japan, along with other inherited retinal diseases and atrophic age-related macular degeneration (AMD), currently have no effective treatments and is necessitating urgent development globally. To address this unmet need, we are developing an innovative visual restoration gene therapy, or optogenetic therapy, using our proprietary light-sensor protein, Chimeric Rhodopsin (RV-001).

RP is a genetically diverse condition, with over 100 causative genes identified. This genetic heterogeneity complicates the development of mutation-specific treatments, requiring substantial resources and fragmenting potential market share. As RP eventually damages photoreceptor cells or retinal pigment epithelial cells regardless of the causative gene, gene-agnostic optogenetic therapies are in development to restore vision by expressing a light-sensor protein in cells that are not affected by RP. However, existing clinical trials utilize microbial rhodopsin, a light-sensor protein with limited sensitivity that is ineffective without goggles and still has a ways to go to visual restoration in low light conditions, which is imperative to improving the patients' QOL.

RV-001, our proposed gene therapy, utilizes adeno-associated virus vectors and is designed to have durable therapeutic effects with a single administration. Our core technology, Chimeric Rhodopsin stems from joint research between Nagoya Institute of Technology and Keio University. This high-sensitivity protein can function autonomously, surmounting the limitations of both animal and microbial rhodopsins, making it an ideal basis for visual restoration.

Patients with RP, for whom no treatment had been available, could restore their sight and their social engagement with this convenient, high-quality vision restoration treatment and potentially reduce social security expenses. Further, this treatment can be expanded to AMD patients who are also waiting for a cure.

This project will take full advantage of developing the drug in Japan, leveraging an established patient cohort network as well as a medical system and regulations favorable to launch the product as a first-in-class medication that can compete in the global market. This development plan builds upon these strengths, initially targeting specific patients in obtaining domestic P1/2a clinical trial data. Concurrently, we aim to expand our dataset to domestic P2b clinical trials that involve conditional and term-limited approvals, and international P2b clinical trials to move toward global approval.



Company Info

Restore Vision Inc.

President & CEO

KATADA Yusaku, MD, Ph.D.

We are a startup developing a visual restoration gene therapy using optogenetics technology for blindness caused by inherited retinal disorders. Founded based on promising results of joint research between Keio University School of Medicine and Nagoya Institute of Technology, Restore Vision's mission is to deliver gene therapy at light speed to patients waiting for effective treatment and, in doing so, contribute to the economy by marketing gene therapy technology rooted in academia.

Contact https://restore-vis.com/en/contact/

Website https://restore-vis.com/en/

Consultation regarding application

Now accepting interview (individual consultations) regarding applications.

*Individual consultations will not be provided during the application period.

- Persons eligible for consultation:VCs and Pharmaceutical Startups who are considering applying for this program.
- Consultation process:Please send an e-mail to the address above with your consultation matters.
- Implementation method:Online (web conference) or face-to-face interview

Contact

E-mail: v-eco“AT”amed.go.jp

Division of Technology Transfer, Department of Intellectual Property and Technology Transfer,
Japan Agency for Medical Research and Development (AMED)

*Replace “AT” with “@”.

Japan Agency for Medical Research and Development

Strengthening Program for Pharmaceutical Startup Ecosystem

Division of Technology Transfer,
Department of Intellectual Property and Technology Transfer

https://www.amed.go.jp/program/list/19/02/005.html

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October 2023