



## Press Release

# **RIKEN and Astellas Enter into Worldwide Exclusive Licensing Agreement for Artificial Adjuvant Vector Cell Technology in Oncology**

**Saitama and Tokyo, September 2, 2019** - RIKEN (President: Hiroshi Matsumoto, Ph.D.) and Astellas Pharma Inc. (TSE: 4503, President and CEO: Kenji Yasukawa, Ph.D., “Astellas”) announced today that they have entered into a worldwide licensing agreement for the research, development and commercialization of cell therapy formulations applying RIKEN’s artificial adjuvant vector cell (“aAVC”) technology in oncology.

Based on the agreement, Astellas has acquired the rights for the research, development and commercialization of cell therapy formulations applying RIKEN’s aAVC technology that targets selected cancer antigens. RIKEN retains the rights for the use of aAVC technology in the research, development and commercialization of cell therapy formulations etc. for antigens not covered by the agreement and the non-profit academic research. Astellas will make an upfront payment of 1 billion yen to RIKEN. Astellas may also make milestone payments depending on achievement of development and commercialization milestones, as well as royalty payments on any future product sales.

Both parties have been conducting the collaboration research for cell therapy formulations applying the aAVC technology. Astellas is currently proceeding with several programs gained through the collaboration research. Among them, the most progress has been made with ASP7517, an aAVC formulation loaded with WT1, an antigen highly expressed in patients with acute myeloid leukemia (AML) and other cancers. It is currently in the phase I/II clinical development stage for AML and myelodysplastic syndrome.

Cancer immunotherapy attacks cancer cells by activating immune systems, which are the defense mechanisms of the body. There are two types of immune systems: “innate immunity” that attacks cancer cells non-specifically in the early stage, and “adaptive immunity” that attacks cancer cells in an antigen-specific manner. Many conventional cancer immunotherapy agents exert their effects through activation of either innate or adaptive immunity. Among them, cancer peptide vaccines attack cancer cells by activating adaptive immunity. aAVC formulations contain modified human cells to

which glycolipids and cancer antigens are loaded. The glycolipids activate innate immunity via natural killer T cells and the cancer antigens induce antigen-specific T cells to activate adaptive immunity, thus it is expected that aAVC could effectively attack cancer cells by activating both innate immunity and adaptive immunity. In addition, long-lasting anti-tumor effects can be expected by inducing antigen-specific memory T cells. These multiple immune activations depend on the full activation of dendritic cells (DCs) in the body.

In addition, the action of cancer peptide vaccines varies depending on patient's human leukocyte antigen (HLA) types. The HLA type is a biological system to distinguish between self and non-self. Cancer peptide vaccines are applicable for patients with specific HLA types. On the other hand, aAVC loaded with full-length cancer antigens are applicable for many patients regardless of their HLA types.

The aAVC technology was developed by Shinichiro Fujii, M.D., Ph.D., Deputy Program Director, RIKEN Program for Drug Discovery and Medical Technology Platforms, RIKEN Cluster for Science, Technology and Innovation Hub, and Team Leader, Laboratory for Immunology, RIKEN Center for Integrative Medical Sciences. Regarding the collaboration, he said, "aAVC is a new type of unique cell therapy formulation with a different mechanism of action from previous immunotherapy agents. This press release states that we have transferred our aAVC technology to Astellas and advanced the technology to the clinical development stage. As a medical researcher, I have been aiming at delivering basic science to clinical research. I think this project is another bridge from academia to industry. We can also say that it has opened the new door in the development of immune cell-based agents in industry-academia collaboration originating in Japan."

"aAVC is a cell therapy technology that treats cancers based on activation of the immune system and has the potential as a new cancer immunotherapy platform. Astellas is committed to exploring all types of partnership opportunities to turn cutting-edge science and technological advances into VALUE for patients," said Naoki Okamura, Representative Director, Corporate Executive Vice President and Chief Strategy Officer, Astellas. "Going forward, we will press ahead with research and development in aAVC programs that may produce potential new therapies meeting the unmet medical needs of cancer patients around the world."

The impact of this agreement on Astellas' financial results in the fiscal year ending March 31, 2020 will be limited.

### **About RIKEN**

RIKEN is Japan's largest research institute for basic and applied research. Over 2500 papers by RIKEN researchers are published every year in leading scientific and technology journals covering a broad spectrum of disciplines including physics, chemistry, biology, engineering, and medical science. RIKEN's research environment and strong emphasis on interdisciplinary collaboration and globalization has earned a worldwide reputation for scientific excellence. Website: [www.riken.jp/en/](http://www.riken.jp/en/)

### **RIKEN Program for Drug Discovery and Medical Technology Platforms (DMP)**

The aim of DMP is to contribute to the identification of new treatments for cancer and other diseases by promoting collaboration within RIKEN for the development of innovative new pharmaceuticals and medical technologies. The program is involved in all phases of development, from the discovery of promising targets to the identification of potential leads such as small molecules, antibodies and cell-based drugs, and the acquisition of intellectual property rights to drugs and technologies that can then be brought to the development phase. The program also provides support for translational research and the transfer of potential drug candidates to pre-clinical and clinical phases of drug development. DMP adopted the aAVC project in April 2012 and since then has been providing development support for it.

### **About Astellas**

Astellas Pharma Inc., based in Tokyo, Japan, is a company dedicated to improving the health of people around the world through the provision of innovative and reliable pharmaceutical products. For more information, please visit our website at <https://www.astellas.com/en>

### **Cautionary Notes**

In this press release, statements made with respect to current plans, estimates, strategies and beliefs and other statements that are not historical facts are forward-looking statements about the future performance of Astellas. These statements are based on management's current assumptions and beliefs in light of the information currently available to it and involve known and unknown risks and uncertainties. A number of factors could cause actual results to differ materially from those discussed in the forward-looking statements. Such factors include, but are not limited to: (i) changes in general economic conditions and in laws and regulations, relating to pharmaceutical markets, (ii) currency exchange rate fluctuations, (iii) delays in new product launches, (iv) the inability of Astellas to market existing and new products effectively, (v) the inability of Astellas to continue to effectively research and develop products accepted by customers in highly competitive markets, and (vi) infringements of Astellas' intellectual property rights by third parties. Information about pharmaceutical products (including products currently in development) which is included in this press release is not intended to constitute an advertisement or medical advice.

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