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ALLEN INSTITUTE AND BIOMARIN TEAM UP TO DEVELOP GENE THERAPIES FOR RARE BRAIN DISEASES

The collaboration leverages technologies intended to target gene expression to specific cells within the central nervous system

SEATTLE, WA AND SAN RAFAEL, CA — April 28, 2021 — The Allen Institute and BioMarin Pharmaceutical Inc. announced a collaboration today that will use technologies developed at the Allen Institute to create new gene therapies aimed at rare genetic diseases of the central nervous system. BioMarin will receive an exclusive license to each program for research, development and commercialization. The organizations did not disclose financial terms.

It is increasingly understood in the scientific research community that many central nervous system disorders affect individual brain circuits or cell types rather than the entire brain. Gene therapies are emerging as a promising approach to improving outcomes for patients with these conditions. The Allen Institute for Brain Science, a division of the Allen Institute, has developed modified adeno-associated viruses, or AAVs, that have been engineered to impact specific classes of cells in the brain.

A key component of each engineered AAV is a unique molecular enhancer, which ensures that viral gene expression is restricted to only a precise cell type relevant in a particular disease. The cell type specific enhancers enable a similar control over gene expression in central nervous system tissues from several species, strongly suggesting their potential for contributing to a new class of precision gene therapies in patients. Scientists from BioMarin and the Allen Institute will collaborate to establish whether these novel AAVs can enable the creation of a new class of gene therapies displaying a higher level of precision to treat diseases of the central nervous system.

“The technology behind these viral tools could have incredible impact for both basic neuroscience and clinical research,” said [Allan Jones](#), Ph.D., President and CEO of the Allen Institute. “On the basic research side, the biggest impact will come from the Allen Institute’s traditional model of releasing data, tools and knowledge to the public for broader scientific use, as we have with versions of these tools used in basic research. BioMarin’s industry leading expertise in gene therapy and drug development will allow for the transformation of the basic research to clinical therapy candidates.”

“BioMarin is committed to bringing transformative therapies to patients with rare genetic diseases. This collaboration offers us great opportunities to partner with scientific leaders to develop therapies in the CNS therapeutic area,” said [Lon Cardon](#), Ph.D., Senior Vice President,

Chief Scientific Strategy Officer. “Combining the Allen Institute’s leadership in large-scale genomic science in the central nervous system with BioMarin’s proven experience in developing transformational therapies for rare genetic diseases, lays the foundation to potentially deliver multiple investigational gene therapies to the clinic.”

Background on the Technology

The licensed technology was [originally developed](#) by researchers at the Allen Institute for Brain Science toward the goal of studying and classifying individual brain cell types. The tools are built by engineering AAVs to carry genes that switch on in specific types of neurons or other cells in the brain. A key component of these engineered AAVs is a molecular “zip code” or enhancer, which ensures that gene expression is restricted only to the correct cellular address in the brain.

Neuroscientists at the Allen Institute have been using these tools to ferry fluorescent labels to the brain, lighting up single brain cell types or subclasses of brain cells under the microscope to enable better studies of individual cell types. The researchers have also shown that the same tools can often selectively label comparable cell types. The viral tools for labeling brain cell types were described in two recent publications led by research teams at the Allen Institute for Brain Science, which were published March 30, 2021 in the journals [Neuron](#) and [Cell Reports](#).

About the Allen Institute

The Allen Institute is an independent, 501(c)(3) nonprofit research organization founded by philanthropist and visionary, the late Paul G. Allen. The Allen Institute is dedicated to answering some of the biggest questions in bioscience and accelerating research worldwide. The Institute is a recognized leader in large-scale research with a commitment to an open science model. Its research institutes and programs include the Allen Institute for Brain Science, launched in 2003, the Allen Institute for Cell Science, launched in 2014, the Allen Institute for Immunology, launched in 2018, and the MindScope Program, launched in 2020. In 2016, the Allen Institute expanded its reach with the launch of The Paul G. Allen Frontiers Group, which identifies pioneers with new ideas to expand the boundaries of knowledge and make the world better. For more information, visit alleninstitute.org.

About BioMarin

BioMarin is a global biotechnology company that develops and commercializes innovative therapies for serious and life-threatening rare genetic diseases. The Company's portfolio consists of six commercialized products and multiple clinical and pre-clinical product candidates. For additional information, please visit www.biomarin.com. Information on BioMarin's website is not incorporated by reference into this press release.

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