NEWS RELEASE

bridgebio pharma enters into agreement to acquire late stage therapy for ultra-rare disorder from alexion; launches origin biosciences to develop and commercialize therapy

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PALO ALTO, Calif., June 11, 2018 /PRNewswire/ — **BridgeBio Pharma** today announced that it has entered into an agreement with Alexion Pharmaceuticals, Inc. to acquire cyclic pyranopterin monophosphate (cPMP; ALXN1101), a synthetic enzyme co-factor therapy for patients with the ultra-rare disease caused by molybdenum cofactor deficiency (MoCD) Type A. In addition, BridgeBio announced that it was launching a new subsidiary, **Origin Biosciences**, with sufficient capital to support clinical development of ALXN1101 through potential regulatory approval and commercialization.

MoCD is an ultra-rare autosomal recessive inborn error of metabolism. The disease is caused by a mutation in the MOCS1 gene and leads to defective production of cPMP. Clinical signs of MoCD present shortly after birth and progress rapidly. Newborns with MoCD experience difficulty feeding and intractable seizures yet have no approved available therapies. Patients have a median survival of three years, and those who survive often have severe and irreversible injury to their central nervous system.

“Historically, replacing missing or defective proteins has proven highly efficacious for treating loss of function monogenic conditions – in the case of MoCD type A, we are replacing the missing or defective cPMP, providing children with much needed MoCD activity,” said Michael Henderson, M.D., senior vice president of asset acquisition at BridgeBio. “BridgeBio’s team is committed to continuing the development of ALXN1101 for infants born with MoCD Type A deficiency, their families and caregivers.”

ALXN1101 is a synthetic version of cPMP, the missing cofactor causing MoCD Type A. In previous work with a recombinant form of cPMP, 11 patients with MoCD Type A had normalization of biomarkers within two days, eight patients showed some suppression of seizures, and three patients had near-normal development. ALXN1101 has received Breakthrough Therapy designation from the US FDA.

“Patients born with MoCD face a bleak future, and we will do all we can to pursue the development of this exciting compound, which has the potential to replace the missing enzyme,” said Neil Kumar, Ph.D., CEO of BridgeBio. “BridgeBio aims to sustainably pursue even the rarest of diseases, such as MoCD, especially where we can support drug programs that target well described genetic diseases at their source.”

While specific terms of the deal have not been disclosed, BridgeBio has committed sufficient resources to Origin Biosciences to enable clinical development, regulatory approval and to support commercialization of ALXN1101. Alexion will receive additional payments upon the realization of development and sales milestones.

About Origin Biosciences

Origin Biosciences, a subsidiary of BridgeBio Pharma, is a biotechnology company focused on cPMP replacement therapy for patients with molybdenum cofactor deficiency (MoCD) Type A. Origin’s lead candidate will be a synthetic small-molecule replacement of cPMP.

Origin is led by a team of veteran biotechnology executives. Together with patients and physicians, the company aims to bring a safe, effective treatment for MoCD to market as quickly as possible.

About BridgeBio Pharma

BridgeBio is a clinical-stage biotech company developing novel, genetically targeted therapies to improve the lives of patients. The BridgeBio approach combines a traditional focus on drug development with a unique corporate model, allowing rapid translation of early stage science into medicines that treat disease at its source. Founded in 2015 by a team of industry veterans, the company has built a robust portfolio of nineteen transformative assets, each housed in its own subsidiary, ranging from pre-clinical to late stage development in multiple therapeutic areas including oncology, cardiology, dermatology and endocrinology. The company’s focus on scientific excellence and rapid execution aims to translate today’s discoveries into tomorrow’s medicines.