NEWS RELEASE

bridgebio pharma's origin biosciences initiates rolling submission of new drug application with the u.s. fda for bbp-870 for the treatment of mocd type a

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BOSTON, Dec. 03, 2019 (GLOBE NEWSWIRE) -- BridgeBio Pharma, Inc. (Nasdaq: BBIO) subsidiary Origin Biosciences has initiated a rolling submission of a New Drug Application (NDA) with the United States Food and Drug Administration (FDA) for fosdenopterin (BBP-870/ORGN001) for the treatment of patients with molybdenum cofactor deficiency (MoCD) Type A.

Currently, there are no approved therapies that alter the course of MoCD Type A, which results in severe and irreversible neurological injury. Fosdenopterin, an investigative cPMP substrate replacement therapy, aims to reduce buildup of toxic sulfites and alleviate central nervous system symptoms in infants and children with MoCD Type A.

"Initiation of our rolling NDA submission is a significant milestone for our company and an important step in providing a therapy to patients and their families living with MoCD Type A," said Origin Biosciences CEO Neil Kirby, Ph.D. "We look forward to making fosdenopterin available to infants and children with this devastating disease as soon as possible as they currently have no treatment options that target the disease at its source."

"BridgeBio was founded to develop breakthrough medicines for patients with genetic diseases. As we begin our first rolling new drug application with the FDA, we are taking a significant step forward for patients by targeting MoCD Type A at its source through the provision of the monophosphate cPMP. We want to thank patients and their families, the scientists, and all others involved who helped us reach this critical moment," BridgeBio CEO Neil

Kumar, Ph.D., said. “Our growing company has more than 15 drug discovery and development programs. We hope Origin’s NDA will be the first of many we submit to the FDA in our pursuit of life-changing therapies for patients, including an anticipated submission in 2020 from BridgeBio subsidiary QED Therapeutics for infigratinib for second line treatment of cholangiocarcinoma.”

The regulatory submission will primarily be supported by data from two ongoing trials, a global Phase 2 clinical trial (**NCT02629393**) and a global Phase 2/3 clinical trial (**NCT02047461**). Under its rolling review process, the FDA can review components of a marketing application as they are submitted rather than requiring all components to be received prior to initiating review, potentially allowing for faster review of the application.

BBP-870 has received Orphan Drug Designation in the US and Europe, and Rare Pediatric Disease Designation and Breakthrough Therapy Designation in the US. Since the NDA for fosdenopterin is seeking approval of treatment for patients with a serious and life-threatening disease with no other treatment options (MoCD Type A), it is also eligible for Priority Review Designation, which, if granted, may further expedite the NDA review time for the NDA approval of this new medicine.

About Molybdenum Cofactor Deficiency (MoCD) Type A

MoCD Type A is an ultra-rare, autosomal recessive, inborn error of metabolism caused by disruption in molybdenum cofactor (MoCo) synthesis that is vital for sulfite oxidase (SOX) activity. Patients are often infants with severe encephalopathy and intractable seizures. Disease progression is rapid with a high infant mortality rate.[1,2] Those who survive beyond the first few months experience profuse developmental delays and suffer the effects of irreversible neurological damage, including brain atrophy with white matter necrosis, dysmorphic facial features, and spastic paraplegia.[1,4] Clinical presentation that can be similar to hypoxic-ischemic encephalopathy (HIE) or other neonatal seizure disorders may lead to misdiagnosis and underdiagnosis.[2,3] Immediate testing for elevated sulfite levels and S-sulfocysteine in the urine and very low serum uric acid may help with suspicion of MoCD Type A.[2,4]

About Origin Biosciences

Origin Biosciences, a subsidiary of BridgeBio Pharma, is a biotechnology company focused on developing and commercializing a treatment for MoCD Type A. 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 Type A to market as quickly as possible. For more information on Origin Biosciences, please visit the company's website at **www.originbx.com**.

About BridgeBio Pharma

BridgeBio is a team of experienced drug discoverers, developers and innovators working to create life-altering medicines that target well-characterized genetic diseases at their source. BridgeBio was founded in 2015 to identify and advance transformative medicines to treat patients who suffer from Mendelian diseases, which are diseases that arise from defects in a single gene, and cancers with clear genetic drivers. BridgeBio's pipeline of over 15 development programs includes product candidates ranging from early discovery to late-stage development.

BridgeBio Pharma Forward Looking Statements

This press release contains forward-looking statements. Statements we make in this press release may include statements which are not historical facts and are considered forward-looking within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended, which are usually identified by the use of words such as "anticipates," "believes," "estimates," "expects," "intends," "may," "plans," "projects," "seeks," "should," "will," and variations of such words or similar expressions. We intend these forward-looking statements to be covered by the safe harbor provisions for forward-looking statements contained in Section 27A of the Securities Act and Section 21E of the Securities Exchange Act and are making this statement for purposes of complying with those safe harbor provisions. These forward-looking statements, including statements relating to Origin Biosciences' clinical development plans, including its plans to initiate a rolling NDA submission for BBP-870 (ORGN001), clinical trial results, timing and completion of clinical trials and regulatory submissions, competitive environment and clinical and therapeutic potential of BBP-870, reflect our current views about our plans, intentions, expectations, strategies and prospects, which are based on the information currently available to us and on assumptions we have made. Although we believe that our plans, intentions, expectations, strategies and prospects as reflected in or suggested by those forward-looking statements are reasonable, we can give no assurance that the plans, intentions, expectations or strategies will be attained or achieved. Furthermore, actual results may differ materially from those described in the forward-looking statements and will be affected by a variety of risks and factors that are beyond our control including, without limitation, Origin Biosciences' ability to continue its planned clinical development and regulatory submissions for BBP-870 and the timing and success of any such continued clinical development and planned regulatory submissions, as well as those set forth in the Risk Factors section of BridgeBio Pharma Inc.'s most recent Quarterly Report on Form 10-Q and our other SEC filings. Except as required by law, we assume no obligation to update publicly any forward-looking statements, whether as a result of new information, future events or otherwise.

[1]Spiegel R, Schwahn B, Scribner C L, Confer N. A natural history study of molybdenum cofactor (MoCo) and isolated sulfite oxidase deficiencies (ISOD). **https://origintx.com/posters/**

[2]Mechler K, Mountford WK, Hoffmann GF, Ries M. Ultra-orphan diseases: a quantitative analysis of the natural history of molybdenum cofactor deficiency. Genet Med. 2015;17(12):965-970.

[3]Durmaz MS, Özbakır B. Molybdenum cofactor deficiency: neuroimaging findings. Radiol Case Rep. 2018;13(3):592-595.

[4]Schwahn BC, Van Spronsen FJ, Belaidi AA, et al. Efficacy and safety of cyclic pyranopterin monophosphate substitution in severe molybdenum cofactor deficiency type A: a prospective cohort study. Lancet. 2015;386(10007):1955-1963.

Contact:
Grace Rauh
grace.rauh@bridgebio.com
(917) 232-5478