



NEWS RELEASE

# bridgebio pharma and affiliate origin biosciences announces fda acceptance of its new drug application for fosdenopterin for the treatment of moCD type a

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Application accepted under Priority Review designation with Breakthrough Therapy Designation and Rare Pediatric Disease Designation previously granted

There are currently no approved therapies for the treatment of MoCD Type A, which results in severe and irreversible neurological injury for infants and children.

This is BridgeBio's first NDA acceptance

SAN FRANCISCO, Sept. 29, 2020 (GLOBE NEWSWIRE) -- BridgeBio Pharma, Inc. (Nasdaq: BBIO) and affiliate Origin Biosciences today announced the US Food and Drug Administration (FDA) has accepted its New Drug Application (NDA) for fosdenopterin (previously BBP-870/ORGN001), a cyclic pyranopterin monophosphate (cPMP) substrate replacement therapy, for the treatment of patients with molybdenum cofactor deficiency (MoCD) Type A.

The NDA has been granted Priority Review designation. Fosdenopterin has previously been granted Breakthrough Therapy Designation and Rare Pediatric Disease Designation in the US and may be eligible for a priority review voucher if approved. It received Orphan Drug Designation in the US and Europe. This is BridgeBio's first NDA acceptance.

"We want to thank the patients, families, scientists, physicians and all others involved who helped us reach this critical milestone," said BridgeBio CEO and founder Neil Kumar, Ph.D. "MoCD Type A is a devastating disease with a

median survival of less than four years and we are eager for our investigational therapy to be available to patients, who currently have no approved treatment options. BridgeBio exists to help as many patients as possible afflicted with genetic diseases, no matter how rare. We are grateful that the FDA has accepted our first NDA for priority review and we look forward to submitting our second NDA later this year for infigratinib for second line treatment of cholangiocarcinoma.”

#### About Fosdenopterin

Fosdenopterin is being developed for the treatment of patients with MoCD Type A. Currently, there are no approved therapies for the treatment of MoCD Type A, which results in severe and irreversible neurological injury with a median survival between 3 to 4 years. Fosdenopterin is a first-in-class cPMP hydrobromide dihydrate and is designed to treat MoCD Type A by replacing cPMP and permitting the two remaining MoCo synthesis steps to proceed, with activation of MoCo-dependent enzymes and elimination of sulfites.

#### 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 which is vital to prevent buildup of s-sulfocysteine, a neurotoxic metabolite of sulfite. Patients are often infants with severe encephalopathy and intractable seizures. Disease progression is rapid with a high infant mortality rate. Those who survive beyond the first few month's 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. Clinical presentation that can be similar to hypoxic-ischemic encephalopathy (HIE) or other neonatal seizure disorders may lead to misdiagnosis and underdiagnosis. Immediate testing for elevated sulfite levels and S-sulfocysteine in the urine and very low serum uric acid may help with suspicion of MoCD.

#### About Origin Biosciences

Origin Biosciences, an affiliate of BridgeBio Pharma, is a biotechnology company focused on developing and commercializing a treatment for Molybdenum Cofactor Deficiency (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.origintx.com](http://www.origintx.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 20

development programs includes product candidates ranging from early discovery to late-stage development. For more information visit [bridgebio.com](http://bridgebio.com).

#### 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, 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.

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