



NEWS RELEASE

bridgebio pharma, sentynl therapeutics and medison pharma announce approval in israel for nulibry® (fosdenopterin) for the treatment of moCD type a

2022-08-10

- NULIBRY is the first and only approved therapy in the United States (U.S.) and in Israel to treat patients with MoCD Type A, an ultra-rare, life-threatening genetic disorder that often progresses rapidly in infants with a median overall survival age of about four years
- NULIBRY received a positive opinion from the Committee for Medicinal Products for Human Use (CHMP) in July 2022 recommending approval by the European Commission (EC) in the European Union (EU)
- NULIBRY was approved by the U.S. Food and Drug Administration (FDA) in February 2021 and was BridgeBio's first approved therapy; Medison acquired commercialization rights to NULIBRY in Israel in December 2019; Sentynl acquired global rights to NULIBRY in March 2022

PALO ALTO and SOLANA BEACH, Calif. and PETACH TIKVA, Israel, Aug. 10, 2022 (GLOBE NEWSWIRE) -- BridgeBio Pharma, Inc. (Nasdaq: BBIO) (BridgeBio), a commercial-stage biopharmaceutical company that focuses on genetic diseases and cancers, and Sentynl Therapeutics, Inc. (Sentynl), a U.S.-based biopharmaceutical company focused on bringing innovative therapies to patients living with rare diseases owned by Zydus Lifesciences Ltd. (formerly known as Cadila Healthcare Ltd.), along with partner Medison Pharma, a global pharmaceutical company focused on providing access to highly innovative therapies to patients in international markets, today announced that the State of Israel Ministry of Health has approved NULIBRY® (fosdenopterin) for Injection as the first therapy in Israel to treat MoCD Type A with the indication to reduce the risk of mortality for patients with molybdenum cofactor

deficiency (MoCD) Type A. MoCD Type A is an ultra-rare and progressive condition, known to impact less than 150 patients globally with a median survival of four years.

“At BridgeBio, we believe that no disease is too rare to address. We are grateful that NULIBRY is now an approved therapy in Israel, and we are focused on ensuring that even more children around the world who suffer with MoCD Type A have access to this treatment,” said BridgeBio founder and CEO Neil Kumar, Ph.D.

NULIBRY is a cPMP substrate replacement therapy that was approved by the U.S. FDA in February 2021 to reduce the risk of mortality in patients with MoCD Type A. In July 2022 the CHMP recommended that NULIBRY be approved in the EU as a treatment for patients with MoCD Type A. Based on the CHMP recommendation, a decision by the European Commission (EC), which authorizes marketing applications in the EU, is expected on the NULIBRY application later this year.

“The approval of NULIBRY in Israel is a promising development for children with MoCD Type A. At Zydus and Sentynl we are driven by the purpose of empowering people with the freedom to live healthier and more fulfilled lives. NULIBRY will bring us closer to realizing our purpose and making a meaningful difference in the lives of children with this rare disorder,” said Dr. Sharvil Patel, Managing Director of Zydus Lifesciences, the parent company of Sentynl Therapeutics.

“We are very pleased to share the news of the Israeli approval on the heels of receiving the CHMP’s recommendation in favor of NULIBRY. We are confident this is only the beginning for patients living with MoCD Type A and we intend to continue advancing our efforts to broaden access to this therapy throughout the world for children in need, while increasing awareness of a disease that has historically been underdiagnosed,” said Matt Heck, CEO of Sentynl.

The approval by the State of Israel Ministry of Health is supported by data from three clinical trials that demonstrated efficacy of NULIBRY for the treatment of patients with MoCD Type A compared to data from a natural history study. These studies showed that NULIBRY reduced the risk of death by 82% and increased the probability of survival to 84% at three years compared to 55% in the untreated, genotype-matched, historical control group in the natural history study.

Animal studies have identified that NULIBRY has phototoxic potential. In the clinical trials, the most common adverse reactions reported in two or more NULIBRY-treated patients with MoCD Type A were catheter-related complications (89%), pyrexia (fever; 78%), viral infection (56%), pneumonia (44%), otitis media (ear infection; 44%), vomiting (44%) and cough/sneezing (44%). Adverse reactions for the rcPMP-treated patients were similar to the NULIBRY-treated patients.

“We are proud that our partnership with BridgeBio Pharma and Sentyln enables us to provide this highly innovative therapy to babies born with MoCD Type A, a severely progressive genetic disease that results in irreversible neurological damage and eventual death,” said Meir Jakobsohn, Founder and CEO of Medison Pharma. “The Israeli Ministry of Health’s decision brings us one step closer to accelerating access of this cutting-edge therapy to those in need.”

In March 2022, Sentyln acquired the global rights to NULIBRY and is responsible for the ongoing development and commercialization of NULIBRY in the U.S. and developing, manufacturing, and commercializing fosdenopterin globally. Sentyln and BridgeBio share development responsibilities through the approval of the marketing authorization application under accelerated assessment with the European Medicines Agency.

About Molybdenum Cofactor Deficiency (MoCD) Type A

MoCD Type A is an autosomal recessive, inborn error of metabolism caused by mutations in the molybdenum cofactor synthesis 1 gene and characterized by a deficiency in molybdenum cofactor production, leading to a lack of molybdenum-dependent enzyme activity.^{1,2} The lack of activity leads to decreased sulfite oxidase activity with buildup of sulfite and secondary metabolites (such as S-sulfocysteine) in the brain, which causes irreversible neurological damage.²

The most common presenting symptoms of MoCD Type A are seizures, feeding difficulties and encephalopathy. Patients with MoCD Type A who survive beyond infancy typically suffer from progressive brain damage, which presents in characteristic patterns on magnetic resonance imaging (MRI). This damage leads to severe psychomotor impairment and an inability to make coordinated movements or communicate with their environment.

About NULIBRY® (fosdenopterin) for Injection

NULIBRY®(fosdenopterin) for Injection is a substrate replacement therapy that provides an exogenous source of cPMP, which is converted to molybdopterin. Molybdopterin is then converted to molybdenum cofactor, which is needed for the activation of molybdenum-dependent enzymes, including sulfite oxidase, an enzyme that reduces levels of neurotoxic sulfites. It is the first and only FDA-approved therapy indicated to reduce the risk of mortality in patients with MoCD Type A, and clinical trials have demonstrated that patients treated with NULIBRY or rcPMP had an improvement in overall survival compared to the untreated, genotype-matched, historical control group.

References

¹ Mechler K et al. Genet Med. 2015;17(12):965-970.

² Schwarz G. Cur Op in Che Bio. 2016;31:179-187.

About BridgeBio Pharma, Inc.

BridgeBio Pharma, Inc. (BridgeBio) is a commercial-stage biopharmaceutical company founded to discover, create,

test and deliver transformative medicines to treat patients who suffer from genetic diseases and cancers with clear genetic drivers. BridgeBio's pipeline of development programs ranges from early science to advanced clinical trials. BridgeBio was founded in 2015 and its team of experienced drug discoverers, developers and innovators are committed to applying advances in genetic medicine to help patients as quickly as possible. For more information visit [bridgebio.com](https://www.bridgebio.com) and follow us on [LinkedIn](#) and [Twitter](#).

About Sentyln Therapeutics

Sentyln Therapeutics is a U.S.-based biopharmaceutical company focused on bringing innovative therapies to patients living with rare diseases. The company was acquired by the Zydus Group in 2017. Sentyln's experienced management team has previously built multiple successful pharmaceutical companies. With a focus on commercialization, Sentyln looks to source effective and highly differentiated products across a broad spectrum of therapeutic areas to address unmet needs. Sentyln is committed to the highest ethical standards and compliance with all applicable laws, regulations, and industry guidelines. For more information, visit www.sentyln.com and follow us on [LinkedIn](#).

About Zydus

The Zydus Group, with an overarching purpose of empowering people with freedom to live healthier and more fulfilled lives, is an innovative, global pharmaceutical company that discovers, develops, manufactures, and markets a broad range of healthcare therapies. The group employs over 23000 people worldwide and is driven by its mission to unlock new possibilities in life-sciences through quality healthcare solutions that impact lives. The group aspires to become a global life-sciences company transforming lives through pathbreaking discoveries. For more information, visit <https://www.zyduslife.com/zyduslife/>.

About Medison Pharma

Medison is a global pharma company focused on providing access to highly innovative therapies to patients in international markets.

Medison is the first to create an international commercialization platform for highly innovative therapies, helping to save and improve lives by making the best available novel treatments accessible to patients in international markets. Medison has a track record of multi-territorial partnerships with leading pharmaceutical and biotech companies seeking to expand their global reach.

Medison is also an active investor in disruptive healthcare technologies and provides its partners with exposure to innovation in biotech and digital health. To learn more visit www.medisonpharma.com.

BridgeBio Pharma, Inc. Forward-Looking Statements

This press release contains forward-looking statements. Statements we make in this press release may include

statements that are not historical facts and are considered forward-looking within the meaning of Section 27A of the Securities Act of 1933, as amended (the “Securities Act”), and Section 21E of the Securities Exchange Act of 1934, as amended (the “Exchange Act”), 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 Exchange Act and are making this statement for purposes of complying with those safe harbor provisions. These forward-looking statements, including statements relating to, the timing and outcome of the EC’s decision on NULIBRY, the potential ability for NULIBRY to be the first and only approved therapy in the EU to treat patients with MoCD type A, and the potential ability to provide treatment options to MoCD Type A patients in Europe, Israel and around the world, reflect our current views about our plans, intentions, expectations, strategies and prospects, and are based on the information currently available to us and on assumptions we have made and are not forecasts, promises nor guarantees. Although we believe that our plans, intentions, expectations, strategies and prospects as reflected in or suggested by these 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 number of risks, uncertainties and assumptions, including, but not limited to, the success of our ongoing collaboration with Sentyln, including our co-development responsibilities through approval of the marketing authorization application under accelerated assessment with the EMA and through approval of NULIBRY’s regulatory submission with the Israeli Ministry of Health, Sentyln’s ability to successfully develop and commercialize NULIBRY in the U.S. and to develop, manufacture, and commercialize fosdenopterin globally, Medison Pharma’s ability to successfully provide access to NULIBRY in Israel, as well as those risks set forth in the Risk Factors section of our most recent Annual Report on Form 10-K and BridgeBio Pharma’s other SEC filings. Moreover, we operate in a very competitive and rapidly changing environment in which new risks emerge from time to time. Except as required by applicable 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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