



NEWS RELEASE

bridgebio receives fda's regenerative medicine advanced therapy (rmat) designation for bbp-812 canavan disease gene therapy program

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- Receipt of RMAT Designation is based on preliminary clinical evidence from the CANaspire Phase 1/2 clinical trial, which showed functional improvements in all dosed patients indicating that BBP-812 has potential to address the unmet needs of individuals with Canavan disease
- BridgeBio will leverage the benefits of RMAT designation, including early and more frequent interactions with the FDA, to establish an Accelerated Approval pathway for BBP-812
- If approved, BridgeBio's gene therapy for Canavan disease could be the first therapeutic option for children born with this devastating and fatal neurodevelopmental disorder

PALO ALTO, Calif., Sept. 10, 2024 (GLOBE NEWSWIRE) -- BridgeBio Pharma, Inc. (Nasdaq: BBIO) (BridgeBio), a commercial-stage biopharmaceutical company focused on genetic diseases, today announced that the United States Food and Drug Administration (FDA) has granted Regenerative Medicine Advanced Therapy (RMAT) designation to BBP-812, an investigational intravenous (IV) adeno-associated virus serotype 9 (AAV9) gene therapy for the treatment of Canavan disease. RMAT designation was granted following the FDA's review of clinical data from the CANaspire Phase 1/2 clinical trial investigating BBP-812 as a potential therapy to address the unmet medical needs of individuals with Canavan disease.

RMAT is an expedited FDA program available to sponsors of regenerative medicine therapies intended to treat, modify, reverse, or cure serious conditions. Benefits of the RMAT designation include all the advantages of the Fast

Track and Breakthrough Therapy Designation programs, including faster and more frequent interactions with the FDA to achieve early alignment on critical aspects of the program. FDA granted RMAT designation based on its review of 12 months of safety and efficacy data from the first eight patients with Canavan disease dosed with BBP-812 in the CANaspire Phase 1/2 clinical trial.

“We are honored to be granted RMAT designation for BBP-812 and are eager to work closely with the FDA and the Canavan community with the goal of bringing our therapy to families living with Canavan disease as fast as possible,” said Eric David, M.D., J.D., CEO at BridgeBio Gene Therapy. “We are beyond grateful to the children and their families who are participating in CANaspire, as well as to the study investigators. RMAT will allow us to work more closely with FDA to ensure we are responding to the urgency that families feel.”

To date, results from CANaspire show that all patients dosed with at least one follow-up assessment have demonstrated improvements in functional outcomes in key areas important to caregivers such as head control, sitting upright, reaching for and grasping objects, and visual tracking. All patients dosed with BBP-812 with at least one follow-up assessment have shown reductions in N-acetylaspartate (NAA), both in urine and in the central nervous system, to levels associated with mild disease. BBP-812 has been well-tolerated, with a safety profile generally consistent with that of other AAV9 gene therapy programs.

“Canavan disease is an extremely rare and rapidly progressive neurodegenerative disease that prevents most children from meeting basic developmental milestones, such as crawling, walking, speaking, and even holding their heads up. It is a terminal diagnosis with no approved treatment to date. The news of the RMAT designation, coupled with the preliminary results seen in the clinical trial, provides hope to children worldwide living with Canavan disease and their families,” said Kathleen Flynn, CEO of **National Tay-Sachs & Allied Diseases Association**, an advocacy organization dedicated to driving research, forging collaboration, and supporting families within the Tay-Sachs, Canavan, GM1, and Sandhoff disease communities.

In addition to RMAT designation, BBP-812 has been granted Orphan Drug, Rare Pediatric Disease (RPDD), and Fast Track Designations from the FDA, as well as Orphan Drug Designation from the European Medicines Agency. With RPDD, if approved, BridgeBio may qualify for a Priority Review Voucher.

About CANaspire

CANaspire is a Phase 1/2 open-label study designed to evaluate the safety, tolerability, and pharmacodynamic activity of BridgeBio’s AAV9 gene therapy candidate, BBP-812, in pediatric patients with Canavan disease. Each eligible patient will receive a single IV infusion of BBP-812. The primary outcomes of the study are safety, as well as change from baseline of urine and central nervous system NAA levels. Motor function and development will also be assessed.

For more information about the CANaspire trial, visit TreatCanavan.com or ClinicalTrials.gov ([NCT04998396](https://clinicaltrials.gov/ct2/show/study/NCT04998396)).

About Canavan Disease

Affecting approximately 1,000 children in the U.S. and European Union, Canavan disease is an ultra-rare, disabling and fatal disease with no approved therapy. Most children are not able to meet developmental milestones, are unable to crawl, walk, sit or talk, and die at a young age. The disease is caused by an inherited mutation of the ASPA gene that codes for aspartoacylase, a protein that breaks down a compound called NAA. Deficiency of aspartoacylase activity results in accumulation of NAA, and ultimately results in toxicity to myelin in ways that are not currently well understood. Myelin insulates neuronal axons, and without it, neurons are unable to send and receive messages as they should. The current standard of care for Canavan disease is limited to supportive therapy.

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. 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 and follow us on [LinkedIn](#), [Twitter](#) and [Facebook](#).

BridgeBio Pharma, Inc. Forward-Looking Statements

This press release contains forward-looking statements. Statements BridgeBio makes 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," "continues," "estimates," "expects," "hopes," "intends," "may," "plans," "projects," "remains," "seeks," "should," "will," and variations of such words or similar expressions. BridgeBio intends 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. These forward-looking statements, including statements relating to the timing and success of BridgeBio's Phase 1/2 clinical trial of BBP-812 for the treatment of Canavan disease, expectations, plans and prospects regarding BridgeBio's regulatory approval process for BBP-812, the ability of BBP-812 to be the first therapeutic treatment option for children born with Canavan disease, reflect BridgeBio's current views about its plans, intentions, expectations, strategies and prospects, which are based on the information currently available to BridgeBio and on assumptions BridgeBio has made. Although BridgeBio believes that its plans, intentions, expectations, strategies and prospects as reflected in or suggested by those forward-looking statements are reasonable, BridgeBio 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, BridgeBio's ability to continue and complete its Phase 1/2 clinical trial of BBP-812 for the treatment of Canavan disease, BridgeBio's ability to advance BBP-812 in clinical development according to its plans, the ability of BBP-812 to treat Canavan disease, the ability of BBP-812 to retain Fast Track Designation, Rare Pediatric Drug Designation, Regenerative Medicine Advanced Therapy Designation and Orphan Drug Designation from the U.S. Food and Drug Administration and Orphan Drug Designation from the European Medicines Agency, and potential adverse impacts due to global health emergencies, including delays in regulatory review, manufacturing and supply chain interruptions, adverse effects on healthcare systems and disruption of the global economy, the impacts of current macroeconomic and geopolitical events, including changing conditions from hostilities in Ukraine and in Israel and the Gaza Strip, increasing rates of inflation and rising interest rates, on our business operations and expectations as well as those risks set forth in the Risk Factors section of BridgeBio's most recent Annual Report on Form 10-K, and BridgeBio's other filings with the U.S. Securities and Exchange Commission. Moreover, BridgeBio operates in a very competitive and rapidly changing environment in which new risks emerge from time to time. These forward-looking statements are based upon the current expectations and beliefs of BridgeBio's management as of the date of this press release and are subject to certain risks and uncertainties that could cause actual results to differ materially from those described in the forward-looking statements. 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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