BRIDGEBIO

NEWS RELEASE

Welgemda™ (BBP-418, Ribitol) Receives Traditional Approval as the First and Only Treatment for Individuals Living with LGMD2I/R9

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- Welgemda is the first and only FDA-approved therapy for individuals living with LGMD2I/R9 (FKRP-related), and represents the first approved treatment for any form of limb-girdle muscular dystrophy

- FDA granted traditional approval to Welgemda based on positive Phase 3 FORTIFY results: a 0.27 m/s benefit versus placebo on 100MTT velocity and 4.89% benefit versus placebo on FVC percent predicted at 12 months; and a nominally significant improvement on NSAD, a measure of motor performance in ambulatory and non-ambulatory patients, versus a decline with placebo

- BridgeBio anticipates an immediate U.S. launch and is committed to helping eligible individuals access Welgemda through its patient support program, ForgingBridges®, which includes insurance resources, financial assistance options, and a dedicated support team to assist at every stage of the diagnosis and treatment journey

- With this approval, the FDA also issued a Rare Pediatric Disease Priority Review Voucher

- Management remarks video on FDA approval available

PALO ALTO, Calif., Nov. 23, 2026 (GLOBE NEWSWIRE) -- BridgeBio Pharma, Inc. (Nasdaq: BBIO) (“BridgeBio” or the “Company”), a commercial-stage, multi-product biopharmaceutical company focused on developing medicines for genetic conditions, today announced that the FDA granted traditional (full) approval to Welgemda™ (BBP-418, ribitol), an oral small molecule, for the treatment of adult and pediatric individuals living with limb-girdle muscular dystrophy type 2I/R9 (LGMD2I/R9) (FKRP-related) weighing at least 30 kg. The approval is based on positive clinical data from FORTIFY, the Phase 3 clinical trial of Welgemda in individuals with LGMD2I/R9. With this approval, the

FDA also issued a Rare Pediatric Disease Priority Review Voucher.

"This is a transformative moment for the LGMD2I/R9 community, with the first and only treatment now approved for this chronic, progressive disease," said Christine Siu, Chief Executive Officer of BridgeBio Neuromuscular. "We are grateful to the community, the trial participants and their families, our investigators, and patient advocates, and the FDA review team, who have all worked tirelessly with our team to accomplish this milestone. Welgemda has demonstrated the potential to be a disease-modifying treatment with clinically meaningful and statistically significant benefit observed across all key endpoints, and exploratory analyses showed a consistent benefit across all prespecified subgroups. We are committed to making Welgemda available to as many patients as possible as quickly as possible."

In the Phase 3 FORTIFY trial, a randomized, double-blind, placebo-controlled study, Welgemda met its primary and key secondary endpoints at the 12-month interim analysis. On the 100-meter timed test (100MTT), a measure of ambulatory ability, individuals treated with Welgemda showed a 0.27 m/s benefit in velocity relative to placebo at 12 months ($p<0.0001$). Forced vital capacity (FVC), a measure of pulmonary function, improved approximately 3% from baseline in individuals treated with Welgemda and declined approximately 2% in the placebo group, a treatment benefit of roughly 5% at 12 months ($p=0.0071$). Welgemda also demonstrated a nominally significant improvement from baseline, compared to a decline in the placebo arm, on the North Star Assessment for Limb Girdle Muscular Dystrophies (NSAD), a composite scale measuring motor performance in both ambulatory and non-ambulatory individuals. Consistent with its proposed mechanism of action, individuals treated with Welgemda also showed a rapid and sustained increase in alpha-dystroglycan glycosylation observed at 3 months ($p<0.0001$) and sustained at 12 months ($p<0.0001$) and a substantial reduction in serum creatine kinase (CK) at 12 months ($p<0.0001$). Welgemda was generally well tolerated. The most common adverse reaction was diarrhea, with no serious cases reported, and there were no discontinuations due to adverse reactions associated with Welgemda.

"When I was 5 years old, I learned that I had LGMD2I/R9. After my diagnosis, my mom founded the CureLGMD2i Foundation, and for 15 years, my family along with others across the community have devoted tireless effort, made countless sacrifices, and persevered to bring a treatment forward, so this approval is incredibly meaningful," said Sammy Brazzo, an individual living with LGMD2I/R9 and intern at CureLGMD2i Foundation. "Most importantly, it also means hope for people living with LGMD2I/R9 and for families facing this diagnosis today and in the future. Our community has worked toward a moment like this, driven by the belief that a better future was possible. Knowing that people may now have an option that did not exist before makes every late night, every fundraiser, every meeting, and every setback worth it."

With FDA approval, BridgeBio will transition all active U.S. FORTIFY participants, including those randomized to placebo during the double-blind period, into the ongoing FORTIFY open-label extension (OLE), through which they

will receive Welgemda. Trial sites outside the U.S. will continue the study as planned until each participant completes 36 months of treatment. Welgemda is not yet approved or available in any market outside of the U.S. and continued participation in the study will support generation of the data needed to support regulatory approval and access in territories outside of the U.S.

"Families living with LGMD2I/R9 have never had disease specific treatment and only had supportive care to manage progression," said Matthew Wicklund, M.D., FAAN, Professor of Neurology and Vice Chair for Research, Department of Neurology at the University of Texas (UT) Health Science Center San Antonio. "What we saw in FORTIFY was different: individuals became measurably better at the things that matter most day to day, including breathing easier, walking faster, walking farther, and less fatigue. This is the first time any therapy has shown this kind of functional improvement in LGMD2I/R9, and certainly a first in the limb girdle muscular dystrophies. I am thrilled by what this approval means for the community. For a relentlessly progressive disease, the ability to regain function, rather than simply lose it more slowly, is what will let people hold onto their mobility, their independence, and their quality of life for longer."

BridgeBio anticipates an immediate U.S. launch of Welgemda. As part of ensuring individuals with LGMD2I/R9 can access Welgemda as quickly as possible, the Company offers a patient support services program, ForgingBridges®, for individuals in the U.S. prescribed Welgemda and their families, including insurance resources, financial assistance options, and a dedicated support team to assist at every stage of the diagnosis and treatment journey.

BridgeBio intends to submit a Marketing Authorization Application (MAA) to the European Medicines Agency (EMA) in the first half of 2028. The MAA will be based on the final analysis of the Phase 3 FORTIFY study at Month 36, with topline results expected late 2027/early 2028. The Company intends to initiate clinical studies of BBP-418 in LGMD2I/R9 for individuals who are less than 12 years of age weighing less than 30 kg in the first half of 2027, as well as in LGMD2M/R13 and in LGMD2U/R20 in the near future.

INDICATION AND IMPORTANT SAFETY INFORMATION
About Welgemda™ (ribitol)
Welgemda is indicated for the treatment of limb-girdle muscular dystrophy type 2I/R9 (LGMD2I/R9) in adult and pediatric patients weighing at least 30 kg.

IMPORTANT SAFETY INFORMATION
What are the possible side effects of Welgemda?
The most common side effects of Welgemda include: diarrhea, stomach pain, nausea, joint pain, indigestion or heartburn, flu, and passing gas. There were no severe or serious cases of diarrhea reported. If you experience persistent or worsening diarrhea, tell your healthcare provider.

These are not all of the possible side effects of Welgemda.

In the clinical study, no one stopped taking Welgemda because of side effects.

Management Remarks Video

A video featuring remarks from BridgeBio's management team about today's announcement is available on the Investors section of the Company's website **here**.

About Limb-Girdle Muscular Dystrophy Type 2I/R9 (LGMD2I/R9)

LGMD2I/R9 (FKRP-related) is a monogenic autosomal recessive disease caused by partial loss of function mutations in the fukutin-related protein (FKRP) gene, and FKRP mutations impair glycosylation of alpha-dystroglycan (αDG), a protein associated with stabilizing muscle cells. Clinical manifestations typically present as a skeletal myopathy affecting the lower and then upper limbs, which is commonly later accompanied by pulmonary muscle and cardiac muscle involvement. Individuals who harbor a homozygous L276I genotype typically develop disease manifestations during late childhood with progression to loss of independent ambulation (25%), assisted ventilation (10%), and cardiomyopathy (30%) in adulthood. Cardiomyopathy is progressive, with an annual loss of 0.4% of left ventricular ejection fraction (LVEF). Individuals with other FKRP genotypes typically have an earlier childhood onset with a more severe clinical course, rapid loss of mobility by 20 years of age, more frequent cardiac involvement (60%), and eventual pulmonary decline by 30 years of age in nearly all cases. Approximately 7,000 individuals currently live with LGMD2I/R9 and other addressable α-dystroglycanopathies in the U.S. and Europe.

About BridgeBio

BridgeBio exists to develop transformative medicines for genetic conditions. Millions of people worldwide living with genetic conditions lack treatment options, often because drug development for small patient populations can be commercially challenging. We aim to bridge the gap between advancements in genetic science and meaningful medicines for underserved patient populations. Our decentralized, hub-and-spoke model is designed for speed, precision, and scalability. Autonomous and empowered teams focus on individual conditions, while a central hub provides the clinical, regulatory, and commercial capabilities needed to bring innovation to market. For more information, visit **bridgebio.com** and follow us on **LinkedIn**, **X**, **Facebook**, **Instagram**, **YouTube**, and **TikTok**.

BridgeBio Forward-Looking Statements

This press release contains forward-looking statements. Statements 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 include statements regarding the potential clinical significance and therapeutic implications of data regarding Welgemda (BBP-418), including the potential for Welgemda to act as a disease-modifying therapy for LGMD2I/R9 and provide sustained functional benefits, including with respect to mobility, independence and quality of life; BridgeBio's anticipated immediate U.S. launch of Welgemda and plans to support access to Welgemda through its patient support program, ForgingBridges; the continued conduct of the FORTIFY study, including the transition of active U.S. participants into the ongoing open-label extension, continued follow-up of participants outside the U.S. through 36 months of treatment, and the potential for continued follow-up to generate data to support regulatory approval and access outside the U.S.; BridgeBio's plans to submit a Marketing Authorization Application for Welgemda to the European Medicines Agency in the first half of 2028 based on the final analysis of the Phase 3 FORTIFY study at Month 36, with topline results expected in late 2027 or early 2028; and BridgeBio's intention to initiate clinical studies of BBP-418 in LGMD2I/R9 in individuals less than 12 years of age weighing less than 30 kg in the first half of 2027, and in LGMD2M/R13 and LGMD2U/R20 in the near future.. Although the Company believes that its plans, intentions, expectations and strategies as reflected in or suggested by those forward-looking statements are reasonable, the Company 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, initial and ongoing data from the Company's clinical trials not being indicative of final data; that longer-term data from FORTIFY may not confirm or demonstrate the durability of the observed functional benefits or the disease-modifying potential of Welgemda; that observed improvements in functional measures may not be sustained over longer periods or translate into longer-term benefits in mobility, independence or quality of life; that the ongoing FORTIFY open-label extension and continued follow-up of participants outside the U.S. may not proceed according to the anticipated design or timeline or may not generate data sufficient to support regulatory approval or access outside the U.S.; that the final Month 36 analysis of FORTIFY or topline results may be delayed, may differ from earlier results or may not support the anticipated regulatory or commercial profile of Welgemda; that BridgeBio may not launch Welgemda in the U.S. immediately or as anticipated, and that patient access, reimbursement, commercial uptake or the effectiveness of BridgeBio's patient support efforts may differ from current expectations; that BridgeBio's planned Marketing Authorization Application submission may be delayed, modified or may not occur as expected, or that the EMA or other regulatory authorities outside the U.S. may not approve Welgemda on the anticipated timeline or at all, may require additional data, studies or other information, or may not grant the anticipated label or access; the potential size of the target patient populations Welgemda is designed to treat not being as large as anticipated; the impacts of current macroeconomic and geopolitical events, including changing conditions from hostilities in Ukraine and the Middle East, increasing rates of inflation and changing interest rates, on business operations and expectations, as well as those risks set forth in the Risk Factors section of the Company's most recent Quarterly Report on Form 10-Q and Annual Report on Form 10-K and the Company's other filings with the U.S. Securities and Exchange Commission.

Moreover, the Company 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 the Company'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, BridgeBio assumes no obligation to update publicly any forward-looking statements, whether as a result of new information, future events or otherwise.

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