



Longboard Pharmaceuticals Announces Positive Topline Data from the PACIFIC Study, a Phase 1b/2a Clinical Trial, for Bexicaserin (LP352) in Participants with Developmental and Epileptic Encephalopathies (DEEs)

January 2, 2024

- *Bexicaserin achieved a median seizure reduction of 53.3% in countable motor seizures compared to 20.8% in the placebo group across the DEE study population*
- *A median seizure reduction of 72.1% in Dravet Syndrome (DS), 48.1% in Lennox-Gastaut Syndrome (LGS) and 61.2% in DEE Other was achieved*
- *Favorable safety and tolerability results*
- *Longboard is rapidly moving forward with preparations for its global Phase 3 program*
- *Conference call and webcast to be held today at 8:30am ET*

LA JOLLA, Calif.--(BUSINESS WIRE)--Jan. 2, 2024-- [Longboard Pharmaceuticals, Inc.](#) (Nasdaq: LBPH), a clinical-stage biopharmaceutical company focused on developing novel, transformative medicines for neurological diseases, today announced positive topline data from the PACIFIC Study evaluating bexicaserin (LP352), a potentially best-in-class and highly selective, oral, novel 5-HT_{2C} receptor superagonist for seizures associated with a broad range of Developmental and Epileptic Encephalopathies (DEEs).

The PACIFIC Study Topline Results:

In the innovative PACIFIC Study, 52 participants ages 12-65 years old with a DEE diagnosis were enrolled at 34 study sites across the United States and Australia to evaluate the safety, tolerability, efficacy and pharmacokinetics of oral bexicaserin (6 mg, 9 mg and 12 mg) three times daily (TID) versus placebo. Participant DEE diagnoses included DS, LGS, and other qualifying DEEs (DEE Other). Following a 5-week screening period and baseline evaluations, study participants initiated a dose titration over a 15-day period and subsequently continued on the highest tolerated dose throughout the maintenance period of 60 days. Of the 52 participants enrolled in the study, 43 participants were randomized to bexicaserin (DS=4, LGS=24, DEE Other=15) and 9 to placebo (DS=0, LGS=5, DEE Other=4). The median number of countable motor seizures per 28-day period at baseline was 38.8 in the bexicaserin group compared to 20.8 in the placebo group. Participants were able to remain on a contemporary, stable polytherapy regimen of up to 4 anti-seizure medications (ASMs) throughout the study, with the most common ASMs being clobazam, cannabidiol, lamotrigine and levetiracetam.

Summary of Efficacy Data:

The median change in countable motor seizure frequency (primary efficacy endpoint) from baseline for the evaluable participants treated with bexicaserin (n=35) was a decrease of 53.3%, compared to a 20.8% decrease for those receiving placebo (n=9). Overall, this represents a placebo-adjusted reduction in seizure frequency of 32.5%. The median change in countable motor seizure frequency from baseline in the DS, LGS and DEE Other cohorts was a decrease of 72.1%, 48.1% and 61.2%, respectively. This represents a placebo-adjusted reduction in seizure frequency of 27.3% and 28.6% in LGS and DEE Other, respectively.

Summary of Safety Data:

Bexicaserin exhibited favorable safety and tolerability results. Most participants (85.7%) in the bexicaserin treated group (n=35) that started the maintenance period tolerated the highest dose (12 mg). The most common adverse events (AEs) observed were somnolence, decreased appetite, constipation, diarrhea and lethargy. There were 3 participants that reported a serious adverse event (SAE) (ankle fracture, constipation, increased seizures) and no deaths were reported in the study. Overall, 9 participants in the bexicaserin group discontinued due to an AE. Of note, 2 of these participants discontinued during the maintenance period (7 participants discontinued during the titration period). No participants in the placebo group discontinued or experienced an SAE.

100% of the participants who completed the PACIFIC Study elected to enroll in the ongoing 52-week open-label extension study.

Additional data from the PACIFIC Study are intended to be presented at future medical meetings.

"These exciting PACIFIC Study results underscore our belief that bexicaserin's differentiated profile will translate into a clinically and commercially best-in-class product and has the potential to redefine the standard of care in DEEs. We are pleased to see such strong seizure reduction across a wide range of DEE syndromes with varying etiologies coupled with favorable safety and tolerability results," stated Dr. Randall Kaye, Longboard's Chief Medical Officer. "We would like to thank the entire DEE community, including study participants, their caregivers and advocacy groups, as well as the investigators, sites and coordinators for their participation and continued partnership as we advance into a Phase 3 program. This tremendous milestone brings us one step closer to improving the lives of those living with these devastating diseases and their families."

"The remarkable results from the PACIFIC Study give hope to patients and their loved ones who are in dire need of research and novel therapies in these severe syndromes. A tremendous unmet need remains not only for those living with LGS, but for the many other DEE patients who have not received the attention they deserve, and I applaud this innovative and inclusive approach that is designed to get therapies quickly and safely to even more people," said Tracy Dixon-Salazar, PhD, Executive Director of the LGS Foundation.

"As the principal investigator, I am delighted to see these highly anticipated and, more importantly, clinically meaningful results from the PACIFIC Study. Physicians are looking for options with fewer side effects and less burden, and that are easy to add onto existing medications in these patients

with highly refractory, treatment resistant seizures. This is an innovative and unique approach to clinical development in broadening research across the DEE population. I am looking forward to participating in the future development of this compound," stated Dennis Dlugos, MD, MSCE, pediatric neurologist at Children's Hospital of Philadelphia, Vice President & Officer of the Epilepsy Study Consortium, and Principal Investigator of the PACIFIC Study.

"Given the groundbreaking design of the PACIFIC Study and the broad efficacy of bexicaserin observed across DEEs in this study, we believe that bexicaserin provides us with the cornerstone to build a world-class epilepsy franchise and to explore development paths forward that may offer novel options to DEE patients that are vastly underserved. We are continuing our Phase 3 preparations as we evaluate the broader dataset," stated Kevin R. Lind, Longboard's President and Chief Executive Officer.

About the PACIFIC Study

The PACIFIC Study is a Phase 1b/2a double-blind, placebo-controlled clinical trial to assess the safety, tolerability, efficacy and pharmacokinetics of bexicaserin (LP352) in 52 participants between the ages of 12 and 65 years old at 34 sites across the United States and Australia. Following a 5-week screening period and baseline evaluations, study participants initiated a dose titration over a 15-day period and subsequently continued on the highest tolerated dose throughout the maintenance period of 60 days. Following the maintenance period, participants were then titrated down, and eligible participants were given the opportunity to enroll in a 52-week open-label extension program. The primary efficacy measure was median percent change from baseline in countable motor seizure frequency over the 75-day treatment period.

Conference Call and Webcast Details

Longboard will host a conference call today at 8:30am ET. Stockholders and other interested parties may participate in the call by following the instructions below. The live webcast can be accessed on the [Events & Presentations](#) portion of the investor page of Longboard's website at <https://ir.longboardpharma.com>. A replay will be available on Longboard's website shortly after completion of the event and will be archived for up to 30 days.

Participant Webcast Link: <https://edge.media-server.com/mmc/p/sqq9yxpf>

Participant Call Link: <https://register.vevent.com/register/B1b92b4a3dd66f44fdb3cd202fca9caf>

About Longboard Pharmaceuticals

[Longboard Pharmaceuticals, Inc.](#) is a clinical-stage biopharmaceutical company focused on developing novel, transformative medicines for neurological diseases. Longboard is working to advance a portfolio of centrally acting [product candidates](#) designed to be highly selective for specific G protein-coupled receptors (GPCRs). Longboard's small molecule product candidates are based on more than 20 years of GPCR research. Longboard plans to advance bexicaserin (LP352), an oral, centrally acting 5-hydroxytryptamine 2C (5-HT_{2C}) receptor superagonist, with no observed impact on 5-HT_{2B} and 5-HT_{2A} receptor subtypes, into a global Phase 3 program. Longboard reported topline data from a Phase 1b/2a clinical trial for bexicaserin, the PACIFIC Study, evaluating participants ages 12 to 65 years old with a broad range of Developmental and Epileptic Encephalopathies (DEEs), including Lennox-Gastaut syndrome, Dravet syndrome and other DEEs. Longboard is also evaluating LP659, an oral, centrally acting, sphingosine-1-phosphate (S1P) receptor subtypes 1 and 5 modulator, which is in development for the potential treatment of rare neuroinflammatory conditions. Longboard has initiated a Phase 1 single-ascending dose (SAD) clinical trial for LP659 in healthy volunteers, with topline data expected in the first half of 2024.

Forward-Looking Statements

Certain statements in this press release are forward-looking statements that involve a number of risks and uncertainties. In some cases, you can identify forward-looking statements by words such as "moving forward", "to be held", "focused on", "potential", "intended", "belief", "will", "would", "advance into", "closer to", "hope", "designed to", looking forward to", "future", "opportunity", "may", "working to", "plans", "expect" or the negative, plural or other tenses of these words or other comparable language, and they may include, without limitation, statements about the following: the potential of bexicaserin (including to be best-in-class, to change the DEE landscape and to serve as the cornerstone of a world-class epilepsy franchise); Longboard's planned global Phase 3 program for bexicaserin; Longboard's clinical and preclinical product candidates and programs, including their advancement, timing of initiating dosing in clinical trials, timing of topline data from clinical trials, characteristics of clinical trial participants, their potential (including to be highly selective and the numbers and types of conditions they may address), and their design and characteristics; Longboard's ability to develop product candidates and deliver medicines; Longboard's focus and work; and Longboard's plans to present additional data from the PACIFIC Study at future medical meetings. For such statements, Longboard claims the protection of the Private Securities Litigation Reform Act of 1995. Actual events or results may differ materially from Longboard's expectations. Factors that could cause actual results to differ materially from those stated or implied by Longboard's forward-looking statements include, but are not limited to, the following: topline data may not accurately reflect the complete results of a particular study or trial and remain subject to audit, and final data may differ materially from topline data; PACIFIC Study participants' diagnoses are as of time of screening and are subject to change; risks related to Longboard's limited operating history, financial position and need for additional capital; Longboard will need additional managerial and financial resources to advance all of its programs, and you and others may not agree with the manner Longboard allocates its resources; risks related to the development and commercialization of Longboard's product candidates; Longboard's product candidates are in the early to middle phases of a lengthy research and development process, the timing, manner and outcome of research, development and regulatory review is uncertain, and Longboard's product candidates may not advance in research or development or be approved for marketing; enrolling participants in Longboard's ongoing and intended clinical trials is competitive and challenging; nonclinical and clinical data is voluminous and detailed, and regulatory agencies may interpret or weigh the importance of data differently and reach different conclusions than Longboard or others, request additional information, have additional recommendations or change their guidance or requirements before or after approval; results of clinical trials and other studies are subject to different interpretations and may not be predictive of future results; macroeconomic events stemming from the COVID-19 pandemic or evolving geopolitical developments such as the conflicts in Ukraine and the Middle East, including but not limited to the impact on Longboard's clinical trials and operations, the operations of Longboard's suppliers, partners, collaborators, and licensees, and capital markets, which in each case remains uncertain; risks related to unexpected or unfavorable new data; risks related to principal stockholders or management selling some or all of their stock; risks related to relying on licenses or collaborative arrangements; other risks related to Longboard's dependence on third parties; competition; product liability or other litigation or disagreements with others; government and third-party payor actions, including relating to reimbursement and pricing; risks related to regulatory compliance; and risks related to Longboard's and third parties' intellectual property rights. Additional factors that could cause actual results to differ materially from those

stated or implied by Longboard's forward-looking statements are disclosed in Longboard's filings with the Securities and Exchange Commission (SEC). These forward-looking statements represent Longboard's judgment as of the time of this release. Longboard disclaims any intent or obligation to update these forward-looking statements, other than as may be required under applicable law.

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