



Longboard Pharmaceuticals Receives Breakthrough Therapy Designation for Bexicaserin (LP352)

July 1, 2024

- *U.S. Food and Drug Administration (FDA) has granted Breakthrough Therapy designation for bexicaserin for the treatment of seizures associated with Developmental and Epileptic Encephalopathies (DEEs)*

LA JOLLA, Calif.--(BUSINESS WIRE)--Jul. 1, 2024-- [Longboard Pharmaceuticals, Inc.](#) (Nasdaq: LBPH), a clinical-stage biopharmaceutical company focused on developing novel, transformative medicines for neurological diseases, today announced that the FDA has granted Breakthrough Therapy designation for its investigational drug bexicaserin for the treatment of seizures associated with Developmental and Epileptic Encephalopathies (DEEs) for patients two years of age or older.

"We are thrilled to receive Breakthrough Therapy designation for bexicaserin and believe this important milestone underscores our innovative approach to potentially treating a broad range of DEE patients," stated Dr. Randall Kaye, Longboard's Chief Medical Officer. "The FDA will work closely with us to provide guidance on subsequent development of bexicaserin to help us design and conduct a development program as efficiently as possible. We are looking forward to initiating our global Phase 3 program later this year."

"I am excited about what this designation means for the DEE community. Most of those living with DEEs do not have access to novel medications, nor have they had the opportunity to participate in trials designed to collect data specific to their condition. I am thrilled that we are making strides towards advancing DEE research for the broader population and pleased that there is a move towards increased equity and access for underserved patients and families to clinical trials and potential novel treatments," stated Gabrielle Conecker, MPH, Executive Director & Co-Founder of Decoding Developmental Epilepsies, home of the International SCN8A Alliance, DEE-P Connections, and The Inchstone Project.

Breakthrough Therapy designation is a process designed to expedite the development and regulatory review of drugs that are intended to treat serious or life-threatening conditions and preliminary clinical evidence indicates that the drug may demonstrate a substantial improvement over available therapy on at least one clinically significant endpoint. A drug that receives Breakthrough Therapy designation is eligible for more intensive guidance on an efficient drug development program and organizational commitment involving senior managers from the FDA.

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. Earlier this year, Longboard reported positive topline data from a Phase 1b/2a clinical trial (the PACIFIC Study) evaluating bexicaserin in participants ages 12 to 65 years old with 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 recently completed a Phase 1 single-ascending dose (SAD) clinical trial for LP659 in healthy volunteers.

Bexicaserin and LP659 are investigational compounds that are not approved for marketing by the FDA or any other regulatory authority.

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 "focus", "believe", "potential", "will", "optimistic", "designed to", "intended to", "eligible", "may", "working to", "plans", or the negative, plural or other tenses of these words, references to future dates or time periods, or other comparable language, and they may include, without limitation, statements about the following: bexicaserin, including its potential to treat a broad range of DEEs, its development and advancement into a global Phase 3 program, the regulatory process and Breakthrough Therapy designation, and its selectivity and novel chemistry; the DEE community; Longboard's ability to develop product candidates and deliver medicines; Longboard's focus and work; and LP659, including its potential and Longboard's Phase 1 SAD clinical trial for LP659. 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: the standard for Breakthrough Therapy designation is not the same as the standard for drug approval, the clinical evidence supporting Breakthrough Therapy designation is preliminary, and not all drugs designated as Breakthrough Therapies ultimately will be shown to have substantial improvement over available therapies; the FDA may later decide to rescind a Breakthrough Therapy designation if it determines the designation is no longer supported by subsequent data; Longboard's product candidates are in a lengthy research and development process, the timing, manner and outcome of research, development and regulatory review is uncertain, and Longboard's product candidates, including bexicaserin and LP659, may not advance in research or development or be approved for marketing; results of clinical trials and other studies are subject to different interpretations and may not be predictive of future results; topline or interim 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 or interim data; enrolling participants in clinical trials is competitive and challenging; risks related to unexpected or unfavorable new data; 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; 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; 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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