Decibel Therapeutics Receives European Orphan Drug Designation for Lead Gene Therapy Candidate DB-OTO

March 30, 2023

- Decibel intends to initiate a Phase 1/2 clinical trial of DB-OTO in patients with congenital hearing loss caused by mutations of the otoferlin gene in the first half of 2023 -

- There are currently no approved pharmacologic treatment options for individuals with otoferlin-related hearing loss -

Corrected

BOSTON, March 30, 2023 (GLOBE NEWSWIRE) -- Decibel Therapeutics (Nasdaq: DBTX), a clinical-stage biotechnology company dedicated to discovering and developing transformative treatments to restore and improve hearing and balance, today announced that the European Medicines Agency (EMA) Committee on Orphan Medicinal Products (COMP) has issued a positive opinion on orphan drug designation for DB-OTO, Decibel's lead gene therapy product candidate. DB-OTO is being developed in collaboration with Regeneron Pharmaceuticals, Inc and is designed to provide durable, high quality, physiological hearing to individuals with profound, congenital hearing loss caused by mutations of the otoferlin gene. This opinion was adopted by the European Commission (EC).

"We are pleased to receive this important designation from the EC, which supports our conviction that innovative treatments for congenital hearing loss are urgently needed," said Laurence Reid, Ph.D., Chief Executive Officer at Decibel. "Decibel has generated compelling preclinical data showing DB-OTO's potential, and we are on track to initiate CHORD™, our global Phase 1/2 clinical trial of DB-OTO, in the first half of this year."

Orphan drug designation is granted by the EC for medicines in development to treat rare conditions affecting no more than five in 10,000 people in the European Union, provided there is no other satisfactory treatment option or the medicine can be of significant benefit to those affected by a specific condition. Medicines that are granted orphan drug designation by the EC qualify for financial and regulatory incentives including protocol assistance, possible exemptions or reductions in certain regulatory fees, and, if approved for marketing, ten years of market exclusivity in the European Union.

DB-OTO received Orphan Drug and Rare Pediatric Disease designations from the U.S. Food and Drug Administration in 2021. Currently, there are no approved pharmacologic treatment options for people with hearing loss caused by genetic mutations of otoferlin.

About Decibel Therapeutics
Decibel Therapeutics is a clinical-stage biotechnology company dedicated to discovering and developing transformative treatments to restore and improve hearing and balance, one of the largest areas of unmet need in medicine. Decibel has built a proprietary platform that integrates single-cell genomics and bioinformatic analyses, precision gene therapy technologies and expertise in inner ear biology. Decibel is leveraging its platform to advance gene therapies designed to selectively replace genes for the treatment of congenital, monogenic hearing loss and to regenerate inner ear hair cells for the treatment of acquired hearing and balance disorders. Decibel's pipeline, including its lead gene therapy product candidate, DB-OTO, to treat congenital, monogenic hearing loss, is designed to deliver on our vision of creating a world of connection for people with hearing and balance disorders. For more information about Decibel Therapeutics, please visit www.decibeltx.com or follow us on Twitter.

Forward-Looking Statements
This press release contains forward-looking statements that involve substantial risks and uncertainties. All statements, other than statements of historical facts, contained in this press release, including statements regarding Decibel's strategy, future operations, prospects, plans, objectives of management, the therapeutic potential for Decibel's product candidates and preclinical programs, the potential benefits of orphan drug designation in the European Union, the expected timeline for initiating clinical trials and expectations regarding the translation of preclinical findings to human disease constitute forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. The words "anticipate," "believe," "continue," "could," "estimate," "expect," "intend," "may," "might," "objective," "ongoing," "plan," "predict," "project," "potential," "should," or "would," or the negative of these terms, or other comparable terminology are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Decibel may not actually achieve the plans, intentions or expectations disclosed in these forward-looking statements, and you should not place undue reliance on these forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in these forward-looking statements as a result of various important factors, including: uncertainties inherent in the identification and development of product candidates, including the timing of and Decibel's ability to obtain approval to initiate clinical development of its program candidates, whether results from preclinical studies will be predictive of the results of later preclinical studies and clinical trials, whether Decibel's cash resources are sufficient to fund its foreseeable and unforeseeable operating expenses and capital expenditure requirements, uncertainties related to the impact of the COVID-19 pandemic on Decibel's business and operations, as well as the risks and uncertainties identified in Decibel's filings with the Securities and Exchange Commission (SEC), including those risks detailed under the caption "Risk Factors" in Decibel's Annual Report on Form 10-K for the fiscal year ended December 31, 2022 and in other filings Decibel may make with the SEC. In addition, the forward-looking statements included in this press release represent Decibel's views as of the date of this press release. Decibel anticipates that subsequent events and developments will cause its views to change. However, while Decibel may elect to update these forward-looking statements at some point in the future, it specifically disclaims any obligation to do so. These forward-looking statements should not be relied upon as representing Decibel's views as of any date subsequent to the date of this press release.

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