



Decibel Therapeutics Announces Submission of Investigational New Drug (IND) Application for Lead Gene Therapy Candidate DB-OTO

September 20, 2022

BOSTON, Sept. 20, 2022 (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 the submission of an Investigational New Drug (IND) application to the U.S. Food and Drug Administration (FDA) for a Phase 1/2 clinical trial in pediatric patients of DB-OTO, a gene therapy product candidate designed to provide durable restoration of hearing in individuals with profound congenital hearing loss due to an otoferlin deficiency.

DB-OTO is being developed in collaboration with Regeneron Pharmaceuticals and is an adeno-associated virus (AAV)-based dual-vector gene therapy designed to provide durable hearing to individuals with profound congenital hearing loss caused by mutations of the otoferlin gene. The product candidate uses a proprietary, cell-selective promoter to express the otoferlin transgene in hair cells, with the goal of enabling the ear to transmit sound to the brain and provide hearing. In preclinical studies, Decibel observed that local delivery of DB-OTO to the ear resulted in production of otoferlin protein and reinstatement of auditory brainstem responses to sound in a congenitally deaf rodent disease model. DB-OTO received Orphan Drug and Rare Pediatric Disease designations from the FDA in 2021.

"This submission is an important milestone for the Decibel team as we continue advancing our gene therapy pipeline to address areas of unmet medical need. DB-OTO may potentially provide a new treatment option for children born with otoferlin deficiency, a leading cause of infant hearing loss, for which there are no approved pharmaceutical remedies," said Laurence Reid, Ph.D., Chief Executive Officer at Decibel. "We look forward to initiating a Phase 1/2 clinical trial of DB-OTO in pediatric patients in the first half of 2023, pending regulatory clearance."

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](https://twitter.com/decibeltx).

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 cell-selective expression and the expected timeline for initiating a Phase 1/2 clinical trial of DB-OTO 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 Quarterly Report on Form 10-Q for the quarterly period ended June 30, 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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