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BIAL Announces First Patient Out in its Phase 2 Clinical Trial of BIA 28-6156 - a Novel Therapy for GBA1 Parkinson's Disease

Porto, Portugal (ots/PRNewswire) -

BIAL, a hundred-year-old innovation-driven biopharmaceutical company focused on neurosciences and rare diseases, announced that the first patient has completed the full dose regimen in the ACTIVATE Phase 2 study.

BIA 28-6156 is a first-in-class, small molecule for once-daily oral administration, allosteric activator of beta-glucocerebrosidase (GCase), in development for the treatment of patients with Parkinson's disease (PD) who have a mutation in the glucocerebrosidase 1 (GBA1) gene (GBA-PD). By increasing the activity of GCase, BIA 28-6156 may be the first drug to directly modify the underlying cause of the disease by re-establishing the sphingolipid recycling(1,2).

Joerg Holenz, BIAL´s Chief Scientific Officer, comments: "The first patient out in the ACTIVATE study marks a pivotal milestone in the development of BIA 28-6156, as well as for our ambition to create transformative value for people living with neurodegenerative diseases. We are confident that our medicine has the potential to become a groundbreaking, novel treatment for patients with a confirmed diagnosis of GBA-PD. BIA 28-6156 offers a specific, potentially disease-modifying mechanism of action, with the potential to delay clinical motor progression."

The ACTIVATE study (clinicaltrials.gov: NCT05819359) is a Phase 2, multicenter, randomised, double-blind, placebo-controlled study evaluating the efficacy, safety, tolerability, pharmacodynamics, and pharmacokinetics of two fixed-dose levels (10mg/day and 60mg/day) of BIA 28-6156. Topline data from this Phase 2 study is expected to be released in mid-2026.

Joaquim Ferreira, Professor of Neurology and Clinical Pharmacology at Lisbon School of Medicine, ACTIVATE study investigator, and member of the ACTIVATE Steering Committee, comments: "The completion by the first patient in the ACTIVATE study marks a significant milestone in this journey. It reflects another step forward in this exceptional effort to advance treatment options for PD, particularly for patients with GBA1 mutations. This step follows the remarkable success of the recruitment phase, which has enrolled over 230 genetically confirmed GBA-PD patients across 85 sites in Europe and North America. There is immense anticipation surrounding the potential of BIA 28-6156, not only for the GBA-PD patient community but also for the broader Parkinson's community."

PD is the second most common neurodegenerative disorder affecting more than 10 million people globally(3), and between 5-15% of PD patients have mutations in the GBA gene, making it numerically the most important genetic risk factor for PD.(4)

GBA-PD patients tend to have, on average, an earlier onset of symptoms compared to those with idiopathic PD(5). They also have more severe clinical symptoms, that progress significantly faster, leading to a worse overall prognosis(4), thus emphasizing the importance of developing new solutions that can impact the progression of the disease.

About Parkinson´s Disease and GBA mutations Parkinson's disease is the second most common neurodegenerative disorder. Mutations in GBA1 gene, the gene encoding the lysosomal enzyme glucocerebrosidase, are one of the most known genetic risk factors for the development of PD and related synucleinopathies. Mutations in the GBA1 gene may lead to degradation of the protein, disruptions in lysosomal targeting, and diminished performance of the enzyme in the lysosome.(4)

About BIA 28-6156

BIA 28-6156 (formerly called LTI-291 or LTI-00291) is a novel GCase allosteric activator that increases GCase activity, re-establishing sphingolipid recycling. It is being developed by BIAL R&D for the proposed indication of treatment of PD in patients with a pathogenic mutation variant in the glucocerebrosidase 1 (GBA1) gene (GBA-PD).

BIA 28-6156 is a small molecule, for oral administration, once-daily, with a low toxicity profile and the ability to cross the blood-brain barrier, features that were validated in vitro and in vivo experiences and extensive toxicology studies.(1,2)

About BIAL R&D

BIAL - R&D Investments S.A. (BIAL R&D), a Portuguese Company, is a subsidiary of BIAL Holding, S.A. and part of the BIAL Group, dedicated to the execution and management of research projects to discover new drugs for human use.

BIAL - R&D is also the owner of intellectual property relating to proprietary pharmaceutical compounds for neurodegenerative diseases, namely PD, at various stages of clinical development, such as BIA 28-6156.

BIAL - R&D, as part of BIAL Group, benefits from its expertise, knowledge, and long experience, namely, in discovery, development, and clinical activities.

About BIAL

BIAL is a hundred-year-old innovation-driven biopharmaceutical company aiming to improve people's lives worldwide.

Being a fully integrated company strongly committed to therapeutic innovation, BIAL has established an ambitious R&D program, consistently investing more than 20% of its annual revenue in this field. Key focus areas for the company are the neurosciences and rare diseases.

In Europe, BIAL has a production site and a R&D unit in Portugal (headquarters) and affiliates in several European countries, such as Spain, Germany, United Kingdom, Italy, and Switzerland, as well as in the US.

The company's internationalisation strategy also sought the establishment of partnerships and license agreements, thus collaborating with well-established partners to bring its health solutions to everyone in need. BIAL products are present in more than fifty countries, fulfilling its purpose of making a real difference in the lives of people living with severe diseases across the world.

For more information about BIA28-6156, please visit: https://www.bial.com/com/our-research/pipeline/bia-28-6156

For more information about the trial design, please visit: http://www.clinicaltrials.gov (identifier: NCT05819359)

For more information about BIAL, please visit: www.bial.com

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