BioNTech and OncoC4 Receive FDA Orphan Drug Designation for Gotistobart in Squamous Non-Small Cell Lung Cancer

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- *Gotistobart is an innovative investigational chemotherapy-free treatment approach for patients living with squamous non-small cell lung cancer ("NSCLC") designed to selectively deplete tumor-infiltrating regulatory T cells within the tumor microenvironment*
- *Advanced squamous NSCLC remains an aggressive and difficult-to-treat lung cancer with limited therapeutic options for patients and a median survival of less than a year*
- *Pivotal Phase 3 clinical trial is ongoing to evaluate gotistobart in patients with metastatic squamous NSCLC who have progressed on prior immunotherapy plus chemotherapy*

MAINZ, Germany, and ROCKVILLE, Md., January 12, 2026 -- BioNTech SE (Nasdaq: BNTX, "BioNTech") and OncoC4, Inc. ("OncoC4") today announced that the U.S. Food and Drug Administration ("FDA") has granted Orphan Drug Designation to gotistobart (also known as BNT316 or ONC-392) for the treatment of squamous NSCLC, an aggressive subtype of lung cancer with limited therapeutic options in the advanced stage. The FDA grants Orphan Drug Designation to potential new medicines for prevention, diagnosis, or treatment of patients with either a rare disease, or a specific patient population with a non-rare disease. This designation underscores the urgent medical need for new therapeutic options for patients living with this condition.

Gotistobart is a novel tumor microenvironment-selective regulatory T cell ("Treg") depletion candidate targeting CTLA-4. With its unique mode of action, gotistobart has the potential to address the high unmet medical need in patients with squamous NSCLC, which accounts for around 25% of all lung cancer cases[1] and high disease-related mortality[2]. Squamous NSCLC is a devastating disease with a 5-year relative survival rate of 15%, a median survival time of 11 months in the United States (2000-2017)[3], and with limited treatment options in the advanced stage. For advanced or metastatic squamous NSCLC patients, the treatment options for second-line therapy after first-line immunotherapy and chemotherapy are usually limited to chemotherapy or palliative therapy.[4]

The pivotal Phase 3 clinical trial PRESERVE-003 (NCT05671510; EUCT:2023-505311-20-01) is ongoing, evaluating gotistobart in patients with metastatic squamous NSCLC at 160 sites globally. In a data readout from the non-pivotal dose-confirmation stage of the trial, gotistobart demonstrated a clinically meaningful overall survival ("OS") benefit, compared to standard-of-care chemotherapy and a manageable safety profile in squamous NSCLC patients whose disease had progressed following anti-PD-(L)1 therapy and platinum-based chemotherapy. These data were previously announced and presented in an oral presentation at the IASLC ASCO 2025 North America Conference on Lung Cancer. In addition to the recently granted Orphan Drug Designation, the FDA granted Fast Track Designation to gotistobart in 2022 for the treatment of patients with metastatic NSCLC whose disease progressed on prior anti-PD-(L)1 therapy.

About gotistobart (BNT316/ONC-392)
Gotistobart (BNT316/ONC-392) is a tumor microenvironment-selective Treg depletion candidate developed jointly by BioNTech and OncoC4. As a pH-sensitive monoclonal antibody, gotistobart is designed to enable CTLA-4 protein recycling. After binding to the CTLA-4 receptor on the cell surface, the complex is internalized, and the pH change causes the antibody to unbind, allowing CTLA-4 to return to the surface to preserve the immune checkpoint function at peripheral organs and to enhance anti-tumor immunity in the tumor microenvironment[5]. Gotistobart is currently in late-stage clinical development as monotherapy and as a component of combination therapy in various cancer indications. Gotistobart received Fast Track Designation from the U.S. Food and Drug Administration ("FDA") in 2022 for the treatment of patients with metastatic NSCLC whose disease progressed on prior anti-PD-(L)1 therapy and Breakthrough Therapy Designation from China's National Medical Products Administration ("NMPA") in 2025.

About PRESERVE-003 Trial
PRESERVE-003 (NCT05671510; EUCT:2023-505311-20-01) is a two-stage, open-label Phase 3 trial evaluating the efficacy and safety of gotistobart as monotherapy compared to the standard-of-care chemotherapy (docetaxel) in sqNSCLC patients, who have progressed on PD-(L)1 inhibitors and platinum-based chemotherapy. The non-pivotal stage of the trial originally included all NSCLC patients. The ongoing pivotal stage is currently enrolling patients with squamous NSCLC. During the ongoing pivotal stage, patients are planned to be enrolled at 160 clinical sites in various countries and regions, including Australia, Belgium, Canada, China, Germany, Italy, the Netherlands, Spain, South Korea, Türkiye, the United Kingdom and the United States. The primary endpoint is overall survival. Secondary endpoints include overall response rate, progression-free survival and safety profile.

About BioNTech
Biopharmaceutical New Technologies (BioNTech) is a global next generation immunotherapy company pioneering novel investigative therapies for cancer and other serious diseases. BioNTech exploits a wide array of computational discovery and therapeutic modalities with the intent of rapid development of novel biopharmaceuticals. Its diversified portfolio of oncology product candidates aiming to address the full continuum of cancer includes mRNA cancer immunotherapies, next-generation immunomodulators and targeted therapies such as antibody-drug conjugates (ADCs) and innovative chimeric antigen receptor (CAR) T cell therapies. Based on its deep expertise in mRNA development and in-house manufacturing capabilities, BioNTech and its collaborators are researching and developing multiple mRNA vaccine candidates for a range of infectious diseases alongside its diverse oncology pipeline. BioNTech has established a broad set of relationships with multiple global and specialized pharmaceutical collaborators, including Bristol Myers Squibb, Duality Biologics, Fosun Pharma, Genentech, a member of the Roche Group, Genmab, MediLink, OncoC4, Pfizer and Regeneron.

For more information, please visit www.BioNTech.com.

BioNTech Forward-Looking Statements

This statement contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995, as amended, including, but not limited to, statements concerning: BioNTech's collaboration with OncoC4; BioNTech and OncoC4's ability to successfully co-develop and co-commercialize gotistobart (also known as BNT316 or ONC-392), if approved; the rate and degree of market acceptance of gotistobart, if approved; the initiation, timing, progress, and results of BioNTech's research and development programs, including the ongoing Phase 3 PRESERVE-003 clinical trial; expectations regarding the potential indications in which gotistobart may be approved, if at all; and discussions with regulatory agencies. In some cases, forward-looking statements can be identified by terminology such as "will," "may," "should," "expects," "intends," "plans," "aims," "anticipates," "believes," "estimates," "predicts," "potential," "continue," or the negative of these terms or other comparable terminology, although not all forward-looking statements contain these words.

The forward-looking statements in this statement are based on BioNTech's current expectations and beliefs of future events and are neither promises nor guarantees. You should not place undue reliance on these forward-looking statements because they involve known and unknown risks, uncertainties, and other factors, many of which are beyond BioNTech's control and which could cause actual results to differ materially and adversely from those expressed or implied by these forward-looking statements. You should review the risks and uncertainties described under the heading "Risk Factors" in BioNTech's Report on Form 6-K for the period ended September 30, 2025 and in subsequent filings made by BioNTech with the SEC, which are available on the SEC's website at www.sec.gov. These forward-looking statements speak only as of the date hereof. Except as required by law, BioNTech disclaims any intention or responsibility for updating or revising any forward-looking statements contained in this statement in the event of new information, future developments or otherwise.

About OncoC4
Based in Rockville, Maryland, OncoC4 is a privately held, late clinical-stage biopharmaceutical company that is actively engaged in the discovery and development of novel biologicals for the treatment of cancer and immunological diseases. OncoC4's pipeline features assets with first-in-class and best-in-class potential targeting both novel and well validated targets across oncology and immunological diseases. Among them, AI-081 is a fully owned bispecific antibody candidate targeting PD-1 and VEGF. ONC-841 is a first-in-class anti-SIGLEC10 antibody currently in a Phase 2 trial for oncology indications and being explored for neurodegenerative diseases. OncoC4 has a strategic collaboration with BioNTech to co-develop gotistobart (BNT316/ONC-392), a tumor microenvironment-selective Treg depletion candidate targeting CTLA-4, in multiple solid tumor indications, including an ongoing pivotal clinical trial in squamous non-small cell lung cancer.

More information: www.oncoc4.com

CONTACTS

BioNTech

Media Relations
Jasmina Alatovic
Media@biontech.de

Investor Relations
Douglas Maffei, PhD
Investors@biontech.de

OncoC4

Media Relations
Helen Schiltz
hschiltz@oncoc4.com

Investor Relations
Ryan Cui
ir@oncoc4.com

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