

## Imfinzi granted US Orphan Drug Designation for

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### Imfinzi granted US Orphan Drug Designation for small cell lung cancer

AstraZeneca today announced that the US Food and Drug Administration (FDA) has granted Orphan Drug Designation (ODD) to Imfinzi (durvalumab) for the treatment of small cell lung cancer (SCLC).

SCLC constitutes about 15% of all lung cancer diagnoses. It is the most aggressive type of lung cancer with only 6% of patients alive after five years. The FDA grants ODD status to medicines and potential new medicines intended for the treatment, diagnosis or prevention of rare diseases or disorders that affect fewer than 200,000 people in the US.

In June 2019, the [Phase III CASPIAN trial](#) met its primary endpoint with Imfinzi by showing a statistically-significant and clinically-meaningful improvement in overall survival for patients with extensive-stage SCLC. These patients were treated with Imfinzi in combination with standard-of-care etoposide and platinum-based chemotherapy vs. chemotherapy alone. Results will be shared at a forthcoming medical meeting. Imfinzi is also being tested following concurrent chemoradiation therapy in limited-stage SCLC in the Phase III ADRIATIC trial.

José Baselga, Executive Vice President, R&D Oncology said: "This Orphan Drug Designation comes on the heels of positive results from the Phase III CASPIAN trial, which is the first trial to offer the flexibility of combining immunotherapy with different platinum-based regimens in small cell lung cancer. We are eager to expand treatment options for patients facing such a devastating diagnosis and look forward to working with regulatory authorities to bring forward new options as soon as possible."

Imfinzi is currently approved for unresectable, Stage III non-small cell lung cancer (NSCLC) after chemotherapy and radiation therapy in more than 45 countries including the US, EU, and Japan based on the Phase III PACIFIC trial.

#### About small cell lung cancer

Lung cancer is the leading cause of cancer death among both men and women and accounts for about one-fifth of all cancer deaths.<sup>1</sup> Lung cancer is broadly split into NSCLC and SCLC, with about 15% classified as SCLC.<sup>2</sup> About two-thirds of SCLC patients are diagnosed with extensive-stage disease, in which the cancer has spread widely through the lung or to other parts of the body.<sup>3</sup> SCLC is an aggressive, fast-growing cancer that recurs and progresses rapidly despite initial response to platinum-based chemotherapy.<sup>4</sup> Prognosis is particularly poor, as only 6% of all SCLC patients will be alive five years after diagnosis.<sup>3</sup>

#### About Imfinzi

Imfinzi (durvalumab) is a human monoclonal antibody that binds to PD-L1 and blocks the interaction of PD-L1 with PD-1 and CD80, countering the tumour's immune-evading tactics and releasing the inhibition of immune responses.

Imfinzi is also approved for previously-treated patients with advanced bladder cancer in the US, Canada, Brazil, Australia, Israel, India, United Arab Emirates, Qatar, Macau and Hong Kong.

As part of a broad development programme, Imfinzi is also being tested as a monotherapy and in combination with tremelimumab, an anti-CTLA4 monoclonal antibody and potential new medicine, as a treatment for patients with NSCLC, small-cell lung cancer (SCLC), bladder cancer, head and neck cancer, liver cancer, cervical cancer, biliary tract cancer and other solid tumours.

#### About AstraZeneca in lung cancer

AstraZeneca has a comprehensive portfolio of approved and potential new medicines in late-stage clinical development for the treatment of different forms of lung cancer spanning several stages of disease and lines of therapy. We aim to address the unmet needs of patients with EGFR-mutated tumours as a genetic driver of disease, which occur in 10-15% of NSCLC patients in the US and EU and 30-40% of NSCLC patients in Asia, with our approved medicines Iressa (gefitinib) and Tagrisso (osimertinib) and ongoing Phase III trials FLAURA, ADAURA and LAURA as well as the Phase II exploratory combination trials SAVANNAH and ORCHARD.<sup>5-7</sup>

Our extensive late-stage Immuno-Oncology programme focuses on lung cancer patients without a known genetic mutation which represents up to 50% of all patients with lung cancer. Imfinzi (durvalumab), an anti-PDL1 antibody is in development as monotherapy (Phase III trials ADJUVANT BR.31, PACIFIC-4, PACIFIC-5, and PEARL) and in combination with tremelimumab and/or chemotherapy (AEGEAN, PACIFIC-2, NEPTUNE, POSEIDON, ADRIATIC and CASPIAN Phase III trials).

#### About AstraZeneca's approach to Immuno-Oncology (IO)

IO is a therapeutic approach designed to stimulate the body's immune system to attack tumours. Our IO portfolio is anchored by immunotherapies that have been designed to overcome anti-tumour immune suppression. We believe that IO-based therapies offer the potential for life-changing cancer treatments for the clear majority of patients.

We are pursuing a comprehensive clinical-trial programme that includes Imfinzi (anti-PDL1) as monotherapy and in combination with tremelimumab (anti-CTLA4) in multiple tumour types, stages of disease, and lines of therapy, using the PD-L1 biomarker as a decision-making tool to define the best potential treatment path for a patient. In addition, the ability to combine our IO portfolio with small, targeted molecules from across our Oncology pipeline, and from our research partners, may provide new treatment options across a broad range of tumours.

#### About AstraZeneca in Oncology

AstraZeneca has a deep-rooted heritage in Oncology and offers a quickly-growing portfolio of new medicines that has the potential to transform patients' lives and the Company's future. With at least six new medicines to be launched between 2014 and 2020, and a broad pipeline of small molecules and biologics in development, we are committed to advance Oncology as a key growth driver for AstraZeneca focused on lung, ovarian, breast and blood cancers. In addition to our core capabilities, we actively pursue innovative partnerships and

investments that accelerate the delivery of our strategy as illustrated by our investment in Acerta Pharma in haematology.

By harnessing the power of four scientific platforms - Immuno-Oncology, Tumour Drivers and Resistance, DNA Damage Response and Antibody Drug Conjugates - and by championing the development of personalised combinations, AstraZeneca has the vision to redefine cancer treatment and one day eliminate cancer as a cause of death.

## About AstraZeneca

AstraZeneca is a global, science-led biopharmaceutical company that focuses on the discovery, development and commercialisation of prescription medicines, primarily for the treatment of diseases in three therapy areas - Oncology, Cardiovascular, Renal & Metabolism and Respiratory. AstraZeneca operates in over 100 countries and its innovative medicines are used by millions of patients worldwide. For more information, please visit [astrazeneca.com](http://astrazeneca.com) and follow us on Twitter @AstraZeneca.

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