# Roche announces FDA approval of Gavreto (pralsetinib) for the treatment of adults with metastatic RET fusion-positive non-small cell lung cancer

- **Gavreto is a once-daily, oral precision therapy that selectively inhibits RET-altered cancers**
- **Genentech and Blueprint Medicines will co-commercialise Gavreto in the United States**
- **FDA also granted Priority Review to Gavreto for the treatment of people with advanced or metastatic RET-mutant medullary thyroid cancer and RET fusion-positive thyroid cancer**

Basel, 7 September 2020 - Roche (SIX: RO, ROG; OTCQX: RHHBY) today announced that the United States (US) Food and Drug Administration (FDA) has approved Gavreto™ (pralsetinib) for the treatment of adults with metastatic rearranged during transfection (RET) fusion-positive non-small cell lung cancer (NSCLC) as detected by an FDA approved test. This indication was approved under the FDA's Accelerated Approval programme, based on data from the phase I/II ARROW study. Continued approval for this indication may be contingent upon verification and description of clinical benefit in a confirmatory trial. Gavreto is a once-daily, oral precision therapy designed to selectively target RET alterations, including fusions and mutations. It is jointly commercialised by Genentech, a wholly owned member of the Roche Group, and Blueprint Medicines in the US and will be commercialised by Roche outside of the US, excluding Greater China*.

"The FDA approval of Gavreto for RET fusion-positive non-small cell lung cancer is an important step towards our goal of providing an effective treatment option for every person diagnosed with lung cancer, no matter how rare or hard-to-treat their type of disease," said Levi Garraway, M.D., Ph.D., Roche's Chief Medical Officer and Head of Global Product Development. "We remain committed to finding personalised treatment options for people with cancer based on specific genomic or molecular alterations, and we look forward to partnering with Blueprint Medicines to further explore the potential of Gavreto across multiple RET-altered tumour types."

RET-activating fusions and mutations are key disease drivers in many cancer types, including NSCLC and medullary thyroid cancer (MTC), and treatment options that selectively target these genetic alterations are limited. In NSCLC, RET fusions represent approximately 1-2% of patients.[1] Biomarker testing for these fusions is the most effective way to identify people who are eligible for treatment with Gavreto.

The approval is based on the results from the phase I/II ARROW study, in which Gavreto produced durable clinical responses in people with RET fusion-positive NSCLC with or without prior therapy, and regardless of RET fusion partner or central nervous system involvement.[2] Gavreto demonstrated an overall response rate (ORR) of 57% (95% CI: 46%, 68%) and complete response (CR) rate of 5.7% in the 87 people with NSCLC previously treated with platinum-based chemotherapy, and the median duration of response (DoR) was not reached (95% CI: 15.2 months, not reached).[2] In the 27 people with treatment-naïve NSCLC, the ORR was 70% (95% CI: 50%, 86%), with an 11% CR rate.[2] The most common adverse reactions (≥25%) were fatigue, constipation, musculoskeletal pain and increased blood pressure (hypertension).[2]

** Greater China encompasses Mainland China, Hong Kong, Macau and Taiwan.*

**F. Hoffmann-La Roche Ltd** 4070 Basel Switzerland
Group Communications Roche Group Media Relations
Tel. +41 61 688 88 88 www.roche.com

Gavreto is now the sixth FDA-approved medicine in Roche's portfolio of treatments for lung cancer. The FDA granted Breakthrough Therapy Designation to Gavreto for the treatment of RET fusion-positive NSCLC that has progressed following platinum-based chemotherapy and for RET mutation-positive MTC that requires systemic treatment and for which there are no acceptable alternative treatments.

The FDA has also granted Priority Review to Gavreto for the treatment of people with advanced or metastatic RET-mutant MTC and RET fusion-positive thyroid cancer, and is expected to make a decision on approval by 28 February 2021. This New Drug Application (NDA) was accepted for review under the FDA's Real-Time Oncology Review (RTOR) pilot programme, which aims to explore a more efficient review process to ensure safe and effective treatments are available to patients as early as possible.

**About the ARROW study[3]**
ARROW (NCT03037385) is a phase I/II, open-label, first-in-human study designed to evaluate the safety, tolerability and efficacy of Gavreto, administered orally in people with rearranged during transfection (RET) fusion-positive non-small cell lung cancer (NSCLC), RET-mutant medullary thyroid cancer (MTC), RET fusion-positive thyroid cancer and other RET-altered solid tumours. The trial consists of two parts: a dose escalation portion, which is complete, and an expansion portion in people treated with 400 mg of Gavreto, once-daily. ARROW is being conducted at multiple sites across the United States, European Union and Asia.

**About Gavreto**
Gavreto is a once-daily, oral precision therapy designed to selectively target RET alterations, including fusions and mutations, regardless of the tissue of origin. Preclinical data have shown that Gavreto inhibits primary RET fusions and mutations that cause cancer in subsets of patients, as well as secondary RET mutations predicted to drive resistance to treatment. Blueprint Medicines and Roche are also co-developing Gavreto for the treatment of patients with various types of RET-altered thyroid cancers and other solid tumours.

**About Roche in lung cancer**
Lung cancer is a major area of focus and investment for Roche, and we are committed to developing new approaches, medicines and tests that can help people with this deadly disease. Our goal is to provide an effective treatment option for every person diagnosed with lung cancer. We currently have six approved medicines to treat certain kinds of lung cancer and more than ten medicines being developed to target the most common genetic drivers of lung cancer or to boost the immune system to combat the disease.

## About Roche

Roche is a global pioneer in pharmaceuticals and diagnostics focused on advancing science to improve people's lives. The combined strengths of pharmaceuticals and diagnostics under one roof have made Roche the leader in personalised healthcare – a strategy that aims to fit the right treatment to each patient in the best way possible.

Roche is the world's largest biotech company, with truly differentiated medicines in oncology, immunology, infectious diseases, ophthalmology and diseases of the central nervous system. Roche is also the world leader in in vitro diagnostics and tissue-based cancer diagnostics, and a frontrunner in diabetes management.

Founded in 1896, Roche continues to search for better ways to prevent, diagnose and treat diseases and make a sustainable contribution to society. The company also aims to improve patient access to medical innovations by working with all relevant stakeholders. More than thirty medicines developed by Roche are included in the World Health Organization Model Lists of Essential Medicines, among them life-saving antibiotics, antimalarials and cancer medicines. Moreover, for the eleventh consecutive year, Roche has been recognised as one of the most sustainable companies in the Pharmaceuticals Industry by the Dow Jones Sustainability Indices (DJSI).

The Roche Group, headquartered in Basel, Switzerland, is active in over 100 countries and in 2019 employed about 98,000 people worldwide. In 2019, Roche invested CHF 11.7 billion in R&D and posted sales of CHF 61.5 billion. Genentech, in the United States, is a wholly owned member of the Roche Group. Roche is the majority shareholder in Chugai Pharmaceutical, Japan. For more information, please visit www.roche.com.



## References

[1] Drilon et al. Targeting RET-driven cancers: lessons from evolving preclinical and clinical landscapes. *Nat Rev Clin Oncol.* 2018;15:151–67.
[2] Blueprint Medicines. Data on file.
[3] ClinicalTrials.gov. Phase 1/2 Study of the Highly-selective RET Inhibitor, Pralsetinib (BLU-667), in Patients With Thyroid Cancer, Non-Small Cell Lung Cancer, and Other Advanced Solid Tumors (ARROW) [Internet; cited August 2020]. Available from: https://clinicaltrials.gov/ct2/show/NCT03037385.

**F. Hoffmann-La Roche Ltd** 4070 Basel Switzerland
Group Communications Roche Group Media Relations
Tel. +41 61 688 88 88 www.roche.com

**Roche Group Media Relations**
Phone: +41 61 688 8888 / e-mail: media.relations@roche.com

Dr. Nicolas Dunant
Phone: +41 61 687 05 17

Patrick Barth
Phone: +41 61 688 44 86

Daniel Grotzky
Phone: +41 61 688 31 10

Karsten Kleine
Phone: +41 61 682 28 31

Nina Mählitz
Phone: +41 79 327 54 74

Nathalie Meetz
Phone: +41 61 687 43 05

Barbara von Schnurbein
Phone: +41 61 687 89 67

**Roche Investor Relations**

Dr. Karl Mahler
Phone: +41 61 68-78503
e-mail: karl.mahler@roche.com

Jon Kaspar Bayard
Phone: +41 61 68-83894
e-mail: jon_kaspar.bayard@roche.com

Dr. Sabine Borngräber
Phone: +41 61 68-88027
e-mail: sabine.borngraeber@roche.com

Dr. Bruno Eschli
Phone: +41 61 68-75284
e-mail: bruno.eschli@roche.com

Dr. Birgit Masjost
Phone: +41 61 68-84814
e-mail: birgit.masjost@roche.com

Dr. Gerard Tobin
Phone: +41 61 68-72942
e-mail: gerard.tobin@roche.com

**Investor Relations North America**

Loren Kalm
Phone: +1 650 225 3217
e-mail: kalm.loren@gene.com

Dr. Lisa Tuomi
Phone: +1 650 467 8737
e-mail: tuomi.lisa@gene.com