

To Whom it may concern

September 16, 2026

Ohara Pharmaceutical Co., Ltd.

ANTINEOPLASTIC AGENT
MODEYSO® Capsules 125 mg

Marketing Approval in Japan for the Treatment of Diffuse Midline Glioma

Ohara Pharmaceutical Co., Ltd. (head office: Chuo-ku, Tokyo; President and CEO: Seiji Ohara; hereinafter, “Ohara”) announced today that it has received marketing approval from the Ministry of Health, Labour and Welfare (MHLW) for the antineoplastic agent MODEYSO® Capsules 125 mg (generic name: dordaviprone hydrochloride; hereinafter, “MODEYSO”) for the indication of diffuse midline glioma. In February 2026, MODEYSO was designated as an orphan drug by the MHLW and was also granted priority review status.

MODEYSO is a small-molecule compound believed to inhibit tumor growth by activating caseinolytic protease P (ClpP), a mitochondrial protease, thereby inducing apoptosis in tumor cells, among other mechanisms.^{1,2}

Diffuse midline glioma is an infiltrative glioma that develops near the midline of the central nervous system, including the thalamus, brainstem, and spinal cord, and is associated with a poor prognosis. Radiotherapy is the mainstay of treatment; however, no standard subsequent pharmacotherapy has been established, underscoring the need for effective new treatments.^{3,4}

This approval was based on results from the Japanese phase I/II study (OP-10-001 study) as well as the global data in patients with recurrent or progressive glioma, from clinical studies conducted outside Japan.

With this approval, MODEYSO is expected to provide a new treatment option for patients with diffuse midline glioma.

Ohara will work to promote the appropriate use of MODEYSO and ensure its stable supply. By providing a new treatment option for patients in Japan with diffuse midline glioma, their families, and healthcare professionals, Ohara aims to contribute to advances in treatment.

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Reference Information

ABOUT DORDAVIPRONE HYDROCHLORIDE

In the United States, Jazz Pharmaceuticals plc (NASDAQ: JAZZ) received accelerated approval in August 2025 for MODEYSO, the U.S. brand name for dordaviprone, for the treatment of patients with H3 K27M-mutant diffuse midline glioma with disease progression following prior therapy. In 2019, Ohara acquired the rights to develop and commercialize the product in Japan from Oncoceutics, Inc., which was subsequently acquired by Jazz Pharmaceuticals plc. Ohara advanced the product's development in Japan and submitted an application for marketing approval in January 2026.

ABOUT ORPHAN DRUG DESIGNATION

In Japan, an orphan drug is designated by the Minister of Health, Labour and Welfare following review confirming, among other requirements, that the number of patients in Japan for the proposed indication is fewer than 50,000 and that the drug is considered to have particularly high medical value.

ABOUT THE JAPANESE CLINICAL STUDY

The OP-10-001 study is an open-label, uncontrolled study designed to evaluate the safety, efficacy and pharmacokinetics of MODEYSO in patients with recurrent or progressive glioma. In the phase II portion of the study, the objective response rate, adverse events, and other outcomes were evaluated in 31 patients with recurrent or progressive diffuse midline glioma who had previously received radiotherapy, based on blinded independent central review according to the RANO-LGG criteria^{*1}.

^{*1} Response assessment criteria for evaluating non-enhancing gliomas on T2/FLAIR images.

ABOUT THE OVERSEAS CLINICAL STUDIES

In 50 patients with recurrent H3 K27M-mutant diffuse midline glioma who had previously received radiotherapy and were selected from patients enrolled in five overseas clinical studies,^{*2} the objective response rate was evaluated by blinded independent central review according to the RANO 2.0 criteria.^{*3} In addition, adverse events and other safety outcomes were evaluated in 376 patients with glioma.

^{*2} One phase I study(ONC014), two phase II studies (ONC006 and ONC013), an expanded-access study (ONC018), and compassionate use (ONC016).

^{*3} Response assessment criteria for evaluating enhancing or non-enhancing gliomas on contrast-enhanced T1-weighted or T2/FLAIR images.

REFERENCES

1. Ishizawa J, et al. Mitochondrial ClpP-Mediated Proteolysis Induces Selective Cancer Cell Lethality. *Cancer Cell*. 2019;35(5):721-737.e9.
2. Jackson ER, et al. ONC201 in Combination with Paxalisib for the Treatment of H3K27-Altered Diffuse Midline Glioma. *Cancer Res*. 2023;83(14):2421-2437.
3. The Japan Neurosurgical Society and Japanese Society of Pathology. *Clinical and Pathological Classification of Brain Tumors*, 5th Edition. Kanehara & Co., 2023.
4. Japan Society for Neuro-Oncology; supervised by The Japan Neurosurgical Society. *Clinical Practice Guidelines for Brain Tumors: Pediatric Brain Tumors 2022 Edition*, 3rd Edition. Kanehara & Co., 2022.

ABOUT OHARA PHARMACEUTICAL CO., LTD.

Ohara is a pharmaceutical company with three core businesses: innovative medicines focused primarily on orphan drugs, generic pharmaceuticals, and global operations, including activities in Africa.

Guided by its mission, “Everything from the Patient’s Perspective: Trusted Pharmaceuticals for the Future of Healthcare,” Ohara responds precisely to diversifying medical needs and changes in the healthcare environment while contributing to improved treatment outcomes. Beyond treatment, the company pursues innovation across a broad range of areas, including prevention, diagnosis, and aftercare.

Ohara aims to become a company capable of providing “Total Healthcare Solutions” that contribute to improving the quality of healthcare and will continue delivering trusted pharmaceuticals and services to patients around the world.