Media Update

Ipsen welcomes the European Union's publication of the Joint Clinical Assessment for Ojemda® (tovorafenib)

- Ojemda® (tovorafenib) becomes the first medicine to complete the European Union's Joint Clinical Assessment process
- Assessment confirms the challenges of comparative evidence in relapsed or refractory BRAF-altered pediatric low-grade glioma where often no standard of care exists, illustrating the high unmet need for this rare, chronic childhood tumor
- Ojemda is approved by the European Commission as the first targeted therapy for patients with relapsed or refractory pediatric low-grade glioma regardless of BRAF alteration

PARIS, FRANCE, 9 JUNE 2026 – Ipsen (Euronext: IPN; ADR: IPSEY) today welcomes the publication of the European Union's (EU) Joint Clinical Assessment (JCA) report for Ojemda® (tovorafenib) for the treatment of patients with relapsed or refractory BRAF-altered pediatric low-grade glioma (pLGG).[i] Ojemda is the first medicine to complete assessment under the EU's new Health Technology Assessment framework.

"Ojemda represents a long-awaited advancement for a rare pediatric disease – a targeted therapy developed specifically for children with relapsed or refractory BRAF-altered pLGG," said Josep Catlla, Executive Vice President, Chief Corporate Affairs Officer, Ipsen. "In a field with limited options, and few medicines designed for children, this innovation reflects both the scientific complexity of the disease and the significant need for new therapies."

The JCA report highlights the absence of a clearly established clinical comparator for much of the pLGG patient population, underscoring the significant unmet need in a setting with very limited approved treatments, as well as the challenges of generating comparative evidence in small, heterogeneous patient cohorts. These findings echo clinical practice realities and reinforce the ongoing need for new treatment options for children and families affected by this disease.

"In pediatric rare diseases, like relapsed or refractory pLGG, which are characterized by very small and biologically heterogeneous patient populations, single-arm trials are often the most appropriate and ethical approach," said Professor Olaf Witt, Director, Translational Pediatric Oncology, Hopp Children's Cancer Center Heidelberg, German Cancer Research Center and University Hospital Heidelberg. "Randomized comparative studies in r/r pLGG are often unfeasible due to limited patient availability, while withholding potentially beneficial therapy raises ethical concerns. In this context, ensuring timely access to innovation without compromising scientific rigor is critical."

Data from the FIREFLY-1 study, which supported the regulatory approval of tovorafenib in the EU, showed clinically meaningful and durable tumor responses with a manageable safety profile in pretreated pLGG[ii,iii]. For these children, treatment value extends beyond traditional clinical endpoints to include symptom control, long-term physical and neurological development, quality of life, and reduced burden on families and caregivers. Time to next treatment, a pre-specified endpoint in FIREFLY-1, is particularly relevant in this setting. After more than three years of follow-up, the median time before patients required subsequent treatment was 42.6 months (95% CI: 36.7–not estimable). These data provide insight into the duration of disease control following therapy and highlight the potential for extended treatment-free intervals—an important consideration in the long-term management of this chronic pediatric condition.

Ipsen supports the EU's JCA ambition to strengthen collaboration and consistency across Member States. We remain committed to working with European and national stakeholders to help ensure that access frameworks evolve alongside scientific advances and reflect the realities of rare pediatric oncology. We look forward to engaging with Member States and the pLGG community to facilitate timely reimbursement and access to this innovation for eligible patients.

About tovorafenib

Tovorafenib (Ojemda®) is a Type II RAF kinase inhibitor of mutant BRAF V600, wild-type BRAF, and wild-type CRAF kinases. It targets the signaling pathways regulating cell growth and division, which can slow, stop, or shrink tumors.

In April 2026, Ipsen received conditional EU marketing authorization for tovorafenib as monotherapy for the treatment of patients 6 months of age and older with pediatric low-grade glioma harboring a BRAF fusion or rearrangement, or BRAF V600 mutation, who have progressed after one or more prior systemic therapies.[iv]

On April 23, 2024, the Food and Drug Administration granted Day One Biopharmaceuticals accelerated approval of tovorafenib for patients 6 months of age and older with relapsed or refractory pediatric low-grade glioma (LGG) harboring a BRAF fusion or rearrangement, or BRAF V600 mutation.[v] This represents the first FDA approval of a systemic therapy for the treatment of patients with pediatric LGG with BRAF rearrangements, including fusions. Continued approval for this indication may be contingent upon verification and description of clinical benefit in a confirmatory trial.

These approvals were based, in part, on response rate and duration of response according to multiple response assessment criteria: Response Assessment in Neuro-Oncology High-Grade Glioma (RANO-HGG) criteria, Response Assessment in Pediatric Neuro-Oncology Low-Grade Glioma (RAPNO-LGG) criteria, and Response Assessment for Neuro-Oncology Low-Grade Glioma (RANO-LGG) criteria.

Tovorafenib is under evaluation as a therapy for patients aged less than 25 years with pLGG harboring BRAF fusion or rearrangement, or BRAF V600 mutation requiring front-line treatment (Phase III FIREFLY-2/LOGGIC). Additional information about FIREFLY-2 may be found at ClinicalTrials.gov, using Identifier NCT05566795 and at CTIS under EUCT number 2024-510742-13-00.

Tovorafenib has been granted Orphan Drug Designation in Russia, Taiwan, Japan, South Korea and Australia.

Ipsen licensed the ex-U.S. rights to tovorafenib from Day One Biopharmaceuticals Inc. in 2024.

About pediatric low-grade glioma

Pediatric low-grade glioma (pLGG) is a rare childhood brain tumor. More than 800 new cases of BRAF-altered pLGG are identified in the European Union each year.[vi] BRAF is the gene most commonly altered in pLGG, which includes two primary types of BRAF alterations – a BRAF gene fusion and BRAF V600E mutation.[vii] These BRAF alterations account for more than 50% of pLGG cases worldwide and until recently there were no approved treatments for patients with pLGG driven by BRAF fusions.[viii]

pLGG can be chronic and relentless, with patients suffering profound side effects from both the tumor and the treatment, which may include chemotherapy and radiation.[ix] These side effects can impact their life over the long term, and may include muscle weakness, loss of vision, and difficulty speaking. This type of tumor has a high risk of progression, and many children with pLGG require long-term treatment.[x] While most children with pLGG survive their cancer, children who do not achieve a complete resection following surgery may face years of treatment.

About Ipsen

We are a global biopharmaceutical company with a focus on bringing transformative medicines to patients in three therapeutic areas: Oncology, Rare Disease and Neuroscience. Our pipeline is fueled by internal and external innovation and supported by nearly 100 years of development experience and global hubs in the U.S., France and the U.K. Our teams in more than 40 countries and our partnerships around the world enable us to bring medicines to patients in more than 100 countries.

Ipsen is listed in Paris (Euronext: IPN) and in the U.S. through a Sponsored Level I American Depositary Receipt program (ADR: IPSEY). For more information, visit ipsen.com.

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[i] Joint Clinical Assessment (JCA) report for Ojemda® (tovorafenib) for the treatment of patients with relapsed or refractory BRAF-altered pediatric low-grade glioma (pLGG).

[ii] Kilburn LB, et al. The type II RAF inhibitor tovorafenib in relapsed/refractory pediatric low-grade glioma: the phase 2 FIREFLY-1 trial. Nat Med. 2024;30(1):207–217.

[iii] Kline C, et al. Neuro-Oncology Pediatrics. April 2026

[iv] European Medicines Agency (EMA) Ojemda® (tovorafenib) Summary of Product Characteristics (SmPC).

[v] OJEMDA (tovorafenib) US prescribing information.

[vi] Estimates of annual incidence and prevalence for addressable patient population in E.U. 4 + U.K. are based on Ipsen calculations from publicly available data (Eurostat, <25yo population; Global Burden of Disease 2019; Desandes et al. Incidence and survival of children with central nervous system primitive tumors in the French National Registry of Childhood Solid Tumors. Neuro Oncol. 2014 Jul;16(7):975-83. doi: 10.1093/neuonc/not309; Qaddoumi et al. Outcome and prognostic features in pediatric gliomas: a review of 6212 cases from the Surveillance, Epidemiology, and End Results database. Cancer. 2009 Dec 15;115(24):5761-70. doi: 10.1002/cncr.24663).

[vii] Ryall S, et al. *Acta Neuropathol Commun*. 2020;8(1):30.

[viii] Ryall S, et al. Integrated molecular and clinical analysis of 1,000 pediatric low-grade gliomas. Cancer Cell. 2020;37(4):569-583.e5.

[ix] Dana-Farber Cancer Institute. Childhood Low-Grade Gliomas. Available at: https://www.dana-farber.org/cancer-care/types/childhood-low-grade-gliomas. Accessed June 2026.

[x] Pediatric Brain Tumor Foundation. Voice of the Patient Report. August 5, 2024. Accessed June 2026.