

Orphelia Pharma secures US patent covering KIZFIZO[®], first drinkable formulation of temozolomide

Company benefits from protection period until 2038

Paris and Lyon, France, November 29, 2023 – Orphelia Pharma, a pharmaceutical company dedicated to the development and marketing of pediatric and orphan medicines, today announces that the US Patent and Trademark Office (USPTO) has issued patent US11/730,732 for KIZFIZO[®], the first pediatric and ready-to-use drinkable formulation of temozolomide.

KIZFIZO (temozolomide oral suspension, 40 mg/ml), known as Ped-TMZ or KIMOZO during its clinical development and ongoing early access programs, is specifically designed for use in the treatment of children with relapsed or refractory high-risk neuroblastoma, oncology indications with a very poor prognosis. This taste-masked oral suspension was developed for children: it allows a precise dose to be administered orally or via a nasogastric tube in a small volume. Orphelia Pharma has been developing KIZFIZO in collaboration with Gustave Roussy, the leading European cancer center, for the last six years.

“Following the issuance of the European patent in 2021, this grant of the US patent once again highlights the innovation in the formulation of KIZFIZO, which lies in the discovery of an essential excipient improving the stability and the rheological properties of the suspension,” said Jeremy Bastid, chief development officer at Orphelia Pharma. “This US patent complements the European one already issued and the Orphan Drug Designation (ODD) obtained in the US and Europe for temozolomide in the treatment of neuroblastoma. Therefore, we benefit from a double exclusivity protection for KIZFIZO in these territories.”

“There is a major hospital need for an oral formulation of temozolomide, but attempts to generate such liquid formulations have been unsatisfactory, with low concentrations leading to large administration volumes, short shelf-lives and erratic recrystallization,” said Maxime Annereau, pharmacist at Gustave Roussy and co-inventor of the patent. “Our work, carried out jointly with the Orphelia Pharma team, led to the identification of a formulation with a high concentration of temozolomide while controlling its polymorphic transition process.”

Jeremy Bastid added: “Temozolomide is an essential component in the treatment armamentarium of refractory or relapsed high-risk neuroblastoma, a disease that affects very young children. KIZFIZO addresses a major unmet medical need. We will now focus our efforts on making KIZFIZO, which is already accessible under the ongoing early access program, available quickly to all patients; we have recently submitted an application for marketing authorization in Europe.”

The granted US (US11/730,732) and European (EP3613436) patents ‘oral suspension of temozolomide’ are co-owned by Orphelia Pharma and Gustave Roussy. Based on the filing date of the priority application, the patent protection is expected to last until 2038. This protection complements the ODDs already obtained in these territories.

About KIZFIZO[®] 40 mg/ml

KIZFIZO (temozolomide oral suspension, 40 mg/ml) is a ready-to-use oral liquid pediatric formulation of temozolomide developed for use in the treatment of relapsed or refractory high-risk neuroblastoma, which carry a very poor prognosis. This age-adapted and taste-masked formulation delivers an accurate dose in a small volume, while avoiding drug handling and caregiver exposure to temozolomide. It is the result of a collaboration between the pharmacists and clinicians at Gustave Roussy hospital and the development team at Orphelia Pharma.



In March 2022, KIZFIZO was granted Early Access Authorization (*Autorisation d'Accès Précoce*) by the French authorities, for the treatment of refractory and relapsed high-risk neuroblastoma as monotherapy or in combination with irinotecan or topotecan.

KIZFIZO has received an ODD from the EMA and the FDA and the formulation is covered by granted patents in Europe and the US. The company filed an MAA with the EMA in the summer of 2023.

About Orphelia Pharma

Orphelia Pharma is a pharmaceutical company based in Paris and Lyon that develops and markets medicines for the treatment of pediatric and orphan diseases. The company's mission is to provide patients with essential products in the fields of neurology and oncology, in formulations adapted to the pediatric population. Orphelia Pharma conducts research projects through academic and industrial partnerships. It has recently established regional agreements in European territories and is expanding its footprint across the world.

For further information: www.orphelia-pharma.eu, [LinkedIn](#)

About Gustave Roussy

Ranked as the leading European Cancer Center and third in the world, Gustave Roussy is a center with comprehensive expertise and is devoted entirely to patients suffering with cancer. The Institute is a founding member of the Paris Saclay Cancer Cluster. It is a source of diagnostic and therapeutic advances. Per year, it caters for almost 50,000 patients, including 3,500 pediatric patients, and its approach is one that integrates research, patient care and teaching. It is specialized in the treatment of rare cancers and complex tumors and it treats all cancers in patients of any age. Its care is personalized and combines the most advanced medical methods with an appreciation of the patient's human requirements. In addition to the quality of treatment offered, the physical, psychological and social aspects of the patient's life are respected. 4,100 professionals work on its two campuses: Villejuif and Chevilly-Larue. Gustave Roussy brings together the skills which are essential for the highest quality research in oncology: 40% of patients treated are included in clinical studies.

For further information: www.gustaveroussy.fr/en, [Twitter](#), [Facebook](#), [LinkedIn](#), [Instagram](#)

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