

Inclusion of the first patient in the TEMOkids pediatric clinical trial

ORPHELIA Pharma announces the inclusion of the first patient in the TEMOkids pediatric clinical trial

Paris and Lyon, on May 4th, 2021 – ORPHELIA Pharma, a French biopharmaceutical company dedicated to the development and marketing of pediatric and orphan drugs, announces the inclusion of a first patient in the TEMOkids pediatric clinical study. The TEMOkids study aims to determine the pharmacokinetic parameters, tolerance, acceptability and response to treatment of Kimozo[®], the first oral suspension of temozolomide, in the pediatric population (www.temokids.eu).

Temozolomide is an anti-cancer drug used in treatment regimens for refractory or relapsed neuroblastoma that mainly affect very young children. It is also used to treat rhabdomyosarcoma and certain brain tumors. The only oral forms of temozolomide currently available are capsules which are not suited to children. In fact, to be administered, the capsules must be opened and their content added to food. In young children, the formulation is essential to ensure correct medication intake, safety, accuracy of the delivered dose and adherence to treatment. To meet the requirements of onco-pediatricians and the needs of children, ORPHELIA Pharma, working in close collaboration with Gustave Roussy hospital teams, has developed Kimozo[®], the first ready-to-use drinkable suspension of temozolomide.

“The TEMOkids trial, during which our oral suspension of temozolomide will be administered to children for the first time, is a key stage in the clinical development of Kimozo[®],” says Caroline Lemarchand, Chief Pharmaceutical Development Officer for ORPHELIA Pharma, *“This study will evaluate the pharmacokinetics of temozolomide, alone or in combination with other treatments, in children from 1 year of age. It will also define the tolerance and acceptability of Kimozo[®] within the study population, and collect data on tumor response to treatment. The first results of this clinical trial are expected mid-2022.”*

“We are very pleased to be involved in the clinical development of this long-awaited new pediatric formulation of temozolomide for the treatment of cancer in children,” says Dr Samuel Abbou, pediatric oncologist at the Gustave Roussy hospital, and coordinating investigator of the TEMOkids study. *“The study plans to recruit 40 children in 5 countries; that is France, Germany, Spain, the UK and the Netherlands, thanks to the support of a structured European-scale pediatric oncology network.”*

“We have received an enthusiastic response from the members of the European pediatric oncology community who have chosen to participate in this clinical trial,” says Hugues Bienaymé, General Manager and Chief Scientific Officer for ORPHELIA Pharma, before adding by way of a conclusion that *“TEMOkids complements our ongoing bioequivalence study in adult patients, and should support the filing of the Kimozo[®] registration dossier at European level in 2023.”*

About the TEMOkids study (NCT04610736)

The clinical trial entitled “TEMOkids Study: A Phase I Pediatric Study for KIMOZO, Oral Suspension of temozolomide” is an international, open-label, non-randomized, phase 1 study involving 40 pediatric patients aged 1 year and over. The main objective of this study is to determine the pharmacokinetic parameters of Kimozo® in the population concerned by the study. The secondary objectives are to evaluate its tolerance and acceptability among children, and their response to treatment.

Ten clinical centers are involved in the TEMOkids study: Gustave Roussy in Villejuif (the coordinating center); Institut Curie in Paris, France; La Timone Children’s Hospital in Marseille, France; The Institute of Pediatric Hematology and Oncology in Lyon, France; The Oscar Lambret Center in Lille, France; The Charité University Medical Hospital in Berlin, Germany; The *Hopp Children’s Cancer Center in Heidelberg*, Germany; The Princess Máxima Center for Pediatric Oncology in Utrecht, the Netherlands; The Vall d’Hebron Research Institute in Barcelona, Spain; and Southampton General Hospital in the United Kingdom.

For further information, please go to www.temokids.eu.

About Kimozo®

Kimozo® (also known as ORP-005 or Ped-TMZ) is a ready-to-use and taste-masked oral suspension of temozolomide that is currently under development to address the needs of children. Kimozo® is an investigational medicinal product not yet approved for marketing.