



Phospholipid Ether Analogs for Treating and Imaging Pediatric Solid Tumors

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WARF: P160325US02

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The Wisconsin Alumni Research Foundation (WARF) is seeking commercial partners interested in developing alkylphosphocholine analogs shown to target malignant pediatric tumors with high selectivity. A radioiodinated alkylphosphocholine analog labeled with iodine-131 is currently being tested in clinical trials for certain adult hematologic malignancies, and in a phase I study in pediatric solid cancers and brain cancers.

The researchers have shown that fluorescent and radiolabeled alkylphosphocholine analogs are selectively sequestered and retained in a great variety of pediatric solid cancer cell lines, including brain cancers, such as neuroblastoma, rhabdomyosarcoma, Ewing sarcoma, osteogenic sarcoma, medulloblastoma, high-grade gliomas and glioblastomas, both *in vitro* and *in vivo* (xenograft models), suitable for diagnostic imaging or molecular targeted radiotherapy. Furthermore, in mice xenograft models of four different pediatric solid tumor types, a single-dose treatment with an analog labeled with iodine-131 led to significant tumor growth rate reduction and survival benefit.

Overview

Currently there is only one well-studied and established tumor-targeting radioactive agent in pediatric oncology (^{131}I -MIBG), which only targets neuroblastoma and other rare neuroendocrine tumors. It is used in the palliative care setting, and in an ongoing clinical trial of the Children's Oncology Group, in induction cycles of upfront therapy. In about 10 percent of cases, ^{131}I -MIBG is not taken up by neuroblastoma tumors at all. For other challenging pediatric cancers such as rhabdomyosarcoma, osteosarcoma, Ewing's sarcoma or most brain cancers, effective targeted radiotherapy is currently not available.

The Invention

UW-Madison researchers have developed radiolabeled, tumor-targeted alkylphosphocholine analogs shown to diagnose and treat malignant pediatric solid cancers, including pediatric brain cancers across a wide range of histological subtypes.

Disease indication – Malignant pediatric solid tumors including (but not limited to): neuroblastoma; Ewing sarcoma; rhabdomyosarcoma; osteosarcoma; retinoblastoma; Wilms tumor; and pediatric brain tumors such as medulloblastoma, high-grade glioma, glioblastoma.

Drug format – The analogs can be designed as targeted agents for local delivery of radiation therapy by incorporating a radioactive iodine isotope, or for targeted delivery of detection/imaging moieties by incorporating a fluorophore or radioactive iodine isotope.

Drug class – Potential to become the first targeted radiotherapeutic agent for multiple pediatric solid tumors, including malignant brain tumors.

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Research stage and preliminary data – Demonstrated selective *in vivo* uptake in 10 different malignant pediatric solid tumor cell lines and in various pediatric brain tumor cell lines, while exhibiting negligible uptake in various normal human primary cells. Demonstrated



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anti-tumor activity *in vivo* against five different pediatric solid tumor types in murine xenograft models. A pediatric phase I trial is in progress.

Applications

- Targeted radiotherapy, detection/imaging of pediatric solid tumors

Key Benefits

- Urgent need
- Promising preclinical data

Additional Information

For More Information About the Inventors

- [Mario Otto](#)
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Related Technologies

- [WARF reference number P160146US02 describes labeled alkylphosphocholine analogs for tumor imaging and therapy.](#)

Tech Fields

- [Therapeutics & Vaccines : Oncology.](#)

For current licensing status, please contact Rafael Diaz at rdiaz@warf.org or 608-960-9847

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