

Lithea Receives U.S. FDA Orphan Drug Designation for Lead Osteosarcoma Therapy, LIT1001

Lithea today announced that the U.S. Food and Drug Administration (FDA) has granted Orphan Drug Designation (ODD) for its lead investigational therapy, LIT1001, intended for the treatment of osteosarcoma.

This designation marks a key regulatory milestone and reinforces the potential of LIT1001 to address a critical unmet medical need in oncology. The ODD status provides significant development incentives, including seven years of market exclusivity upon approval, tax credits for clinical trial costs, waived FDA fees, and protocol assistance from the FDA.

*"We are proud that the FDA acknowledges the potential of LIT1001 to outperform current standard-of-care treatments," said **Ludvig Sjöberg, CEO of Lithea**. "Our preclinical data demonstrate that LIT1001's targeted delivery of doxorubicin significantly improves tumor control while reducing systemic toxicity – a promising advancement for young patients with osteosarcoma."*



Ludvig Sjöberg, CEO of Lithea.

LIT1001: An innovative localized chemotherapy approach

LIT1001 is a next-generation chemotherapeutic formulation designed to deliver doxorubicin directly into the tumor environment, bypassing systemic exposure and enhancing local efficacy. Built on Lithea's proprietary bone mineral platform – a biocompatible matrix of hydroxyapatite and calcium sulfate – LIT1001 enables sustained, controlled release of doxorubicin at the tumor site, with the goal of minimizing toxic side effects while maximizing therapeutic impact.

*"Osteosarcoma is highly aggressive and unforgiving. In preclinical studies, LIT1001 has shown the ability to suppress local tumor growth while preserving healthy tissue," said **Dr. Mathias Lidgren, Chief Medical Officer at Lithea**. "By delivering chemotherapy directly to the tumor, we are pioneering a new paradigm for localized cancer treatment."*



Mathias Lidgren, MD, PhD, CMO of Lithea.

Osteosarcoma – a life-threatening diagnosis with no new treatments in over 40 years

Osteosarcoma is the most common primary bone cancer in children and young adults. Despite aggressive multimodal treatment, including surgery and systemic chemotherapy, survival rates



have remained stagnant for decades. While localized disease has a 5-year survival rate of around 60–70%, metastatic or recurrent osteosarcoma has a 5-year survival rate below 30%. Critically, no new drugs have been approved for this indication in over 40 years.

A platform for future oncology therapies

Lithea's proprietary platform is based on over 40 years of academic research, initially developed at Lund University and now validated across multiple preclinical oncology models. The underlying bone mineral matrix can be loaded with various anticancer agents and is designed to address a broad range of solid tumor indications. In addition, its unique ability to be “recharged” by systemically administered drugs offers further treatment flexibility and opens the door to combination therapies.

Multiple pipeline programs based on this platform are in development, targeting other hard-to-treat solid tumors with tailored, localized drug delivery.

For more information, please contact:

Ludvig Sjöberg, CEO

ludvig.sjoberg@lithea-pharma.com

About Lithea

Lithea is a pharmatech company based in Lund, Sweden, focused on the development of advanced therapies for solid tumor, and with the mission to prolong the lives of cancer patients.

LIT1001 is the company's chemotherapeutic formulation, designed to leverage the biocompatible matrix to deliver doxorubicin directly into the tumor environment, addressing the limitations of systemic chemotherapy in bone cancer, bypassing systemic exposure and enhancing local efficacy.

Many more products and indications based on the bone mineral platform are in the development pipeline, addressing a plethora of solid tumors with a wide range of different anticancer agents that can be loaded onto the platform. For more information, please visit www.lithea-pharma.com.

Lithea AB

Kung Oskars väg 11C, 222 35, Lund, Sweden
www.lithea-pharma.com