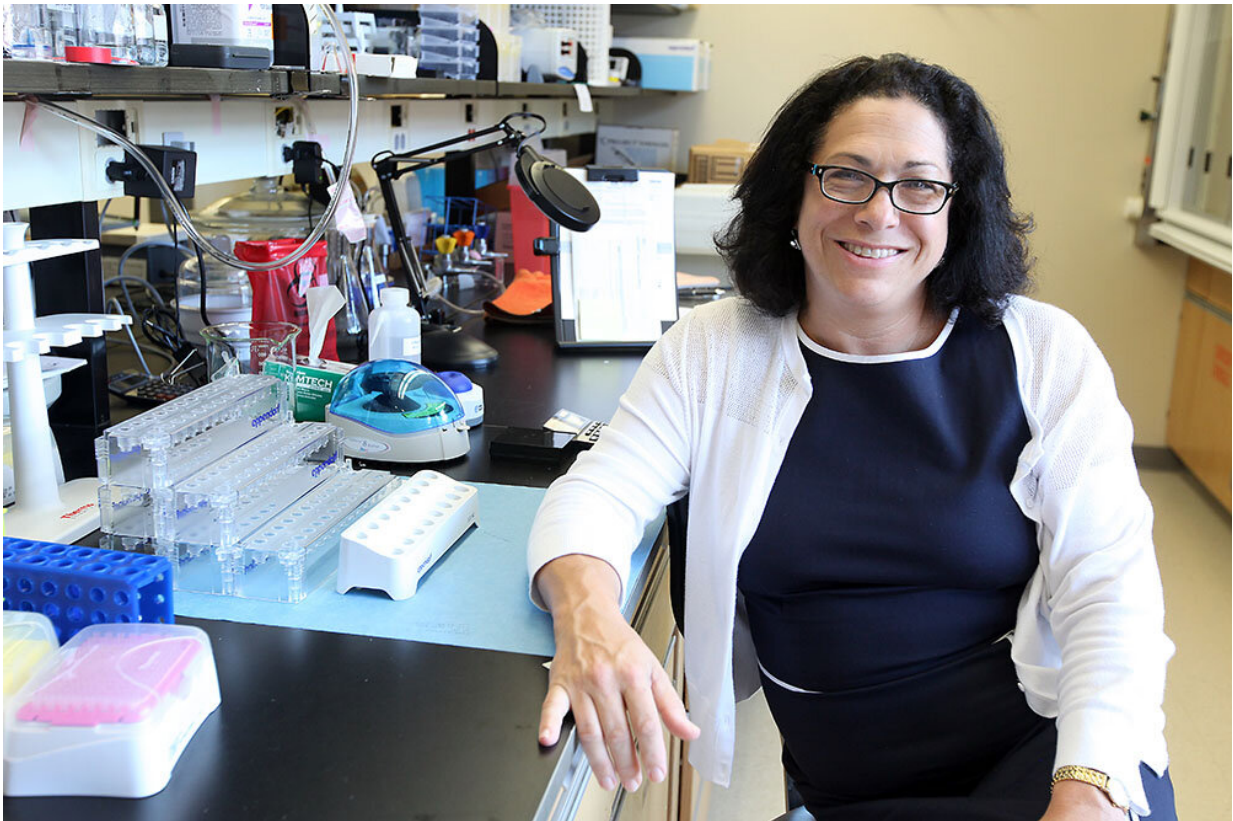


# New osteosarcoma treatment receives rare pediatric disease designation from the FDA

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Dr. Nancy Klauber-DeMore's innovative research laid the foundation for the development of the first potential new treatment for osteosarcoma in over 30 years. Photo by Sarah Pack. Credit: Medical University of South Carolina

Innova Therapeutics Inc., a biotechnology company dedicated to

developing innovative cancer therapies for patients who have inadequate treatment options, recently announced that the U.S. Food and Drug Administration (FDA) has granted rare pediatric disease designation for IVT-8086 for the treatment of osteosarcoma.

MUSC Hollings Cancer Center researcher Nancy Klauber-DeMore, M.D., who is the co-founder and chairman of the Scientific Advisory Board for Innova Therapeutics, based in Charleston, South Carolina, was instrumental in the development of the research that led to the therapy. IVT-8086 is a humanized monoclonal antibody (mAb) with a high affinity receptor for secreted frizzled-related protein 2 (SFRP2) and is believed to be the only SFRP2 antagonist in development.

Under the Creating Hope Act passed into federal law in 2012, the FDA grants rare pediatric disease designation for serious and life-threatening diseases that primarily affect children ages 18 years or younger and includes fewer than 200,000 people in the U.S.

Robert Ryan, Ph.D., president and chief executive officer of Innova Therapeutics, said the FDA's rare pediatric disease designation for IVT-8086 for the treatment of osteosarcoma highlights the significant unmet medical needs of patients with this devastating and life-threatening disease.

"Receiving rare pediatric disease designation from the FDA is a significant milestone for this program and underscores the critical value of our work," Ryan said. "The entire Innova team is encouraged by this designation and will continue to work diligently toward determining the efficacy and registration for IVT-8086 for the treatment of osteosarcoma."

Klauber-DeMore, who holds the BMW Endowed Chair in Cancer Research at MUSC, said the FDA designation of their drug is a huge

breakthrough for osteosarcoma research. "This designation is an enormous validation of the work that we've been doing. It's really exciting because there have been no breakthroughs in osteosarcoma treatments in decades," she said. "IVT-8086 has the potential to become the first FDA-approved therapy for individuals with osteosarcoma in over 30 years."

Hollings Cancer Center director Raymond N. DuBois, M.D., Ph.D., helped form the collaboration, capitalizing on the innovative research of Klauber-DeMore and the expertise of Ryan, who has extensive experience in rare diseases. "This is very exciting for the cancer center. This is a new designation approved by the FDA for a terrible disease in children—osteosarcoma," DuBois said. "Hollings values these kinds of partnerships that help to advance the great science happening here in our laboratories. Also, translating our [basic science](#) into the clinic is a major goal of Hollings, especially when there is such a great unmet need."

Klauber-DeMore and her team at Hollings Cancer Center have been studying IVT-8086 for the treatment of osteosarcoma, and preclinical results are encouraging. Her laboratory is focused on the role of SFRP2 in metastatic cancers. Her research shows that blocking SFRP2 with IVT-8086 reduces tumor growth in preclinical models. SFRP2 is also a potential marker of prognosis in osteosarcoma, as patients with high expression levels of SFRP2 have been shown to have poor survival. The results of her studies on osteosarcoma led to the rare pediatric disease designation for IVT-8086.

IVT-8086 currently is in late-stage pre-investigational new drug (pre-IND) development. The next step is to move the development of the therapy into the clinic and confirm the safety and efficacy of the drug in osteosarcoma patients. Klauber-DeMore, Ryan and colleagues are dedicated to developing IVT-8086 as an [osteosarcoma](#) therapy.

Osteosarcoma is a primary malignant bone tumor that generally arises in the long bones and, more rarely, in soft tissues. Osteosarcoma affects approximately 40,000 Americans, predominantly children, teenagers and young adults. It is a highly metastatic [cancer](#) wherein up to 20% of patients have detectable lung metastasis upon initial diagnosis, with a greater incidence in pediatric patients compared to adult patients, and 40% of patients develop lung metastasis in later stages. In the last 20 years, there has been little improvement in prognosis, even with available treatments, so new therapies are needed to improve the outcomes of patients with this serious and debilitating [disease](#).

"We're passionate about trying to move the field forward and hopeful that we can find something to improve survival for these patients," Klauber-DeMore said.

Provided by Medical University of South Carolina

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