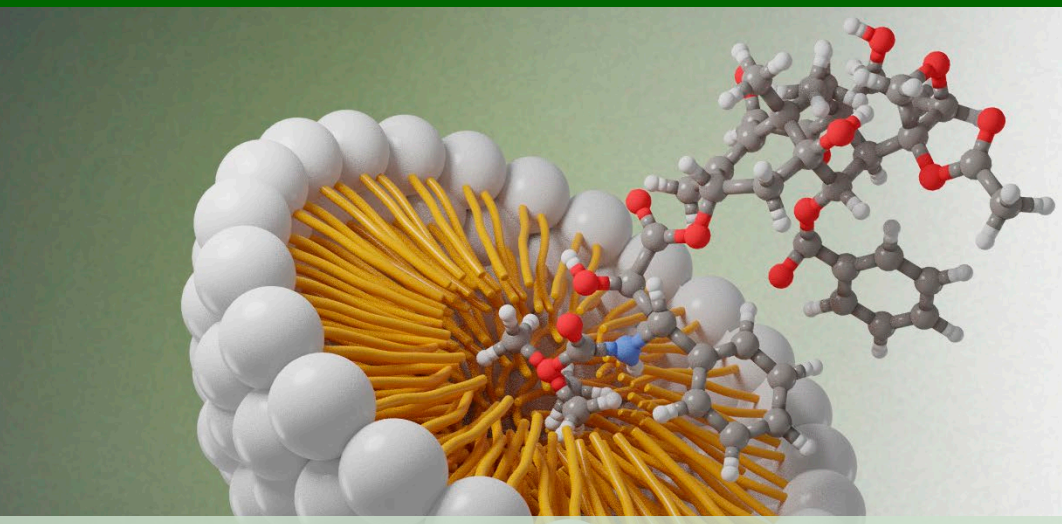
Nanotechnology Characterization Laboratory



NCL
Nanotechnology
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Laboratory

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Each quarter the NCL accepts the most promising cancer nanomedicine candidates into its Assay Cascade characterization and testing program. Nanomedicines accepted into the program will undergo a rigorous evaluation that may include sterility and endotoxin testing, physicochemical characterization, in vitro hemato- and immunotoxicity, and in vivo studies to evaluate safety, efficacy and pharmacokinetics. The studies are tailored to each individual nanomedicine and are designed to promote the clinical translation of these novel therapies. **All studies are conducted free of charge for Awardees**.

Congratulations to this Quarter's Awardees

Dr. Young Jik Kwon, University of California, Irvine &
PharmaResearch Products, Co., Ltd. (Bundang, South Korea)

The clinical translation and commercialization of cancer nanomedicines is hindered by limited capacity of drug loading, cumbersome preparation methods, and high costs associated with production. In response to these shortcomings, the international team of UC Irvine and PharmaResearch Products, Co., Ltd. has developed a novel nano-formulation that utilizes DNA fragments extracted from a natural resource to create monodispersed nanocomplexes for the effective and safe delivery of cancer chemotherapeutics. These nanocomplexes are capable of carrying the high loads of drugs necessary for therapeutic benefit with lowered side effects and can potentially be used to treat a broad range of cancers. In addition, they are easily prepared at large scales using simple and cost-effective manufacturing processes. Based on the preliminary studies which demonstrated its efficacy in treating cancer with minimal adverse effects, the new collaboration with the NCI Nanotechnology Characterization Laboratory (NCL) team will involve re-assessing the technology in-depth and validating its feasibility for clinical translation and commercialization. Particularly, this collaboration among academia, industry, and a federally-funded research agency will establish a solid foundation towards attaining the primary goal of preparing a successful IND application.

https://faculty.sites.uci.edu/biotel/

http://www.pr-products.co.kr/eng/company/sub03.php

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Congratulations to this Quarter's Awardees (continued)

Dr. Gregory Lanza, Washington University & Alembic Pharmaceuticals, Ltd.

Metastatic breast cancer often derives from the reactivation of resistant occult breast cancer (BC) micrometastases harbored within the protective bone marrow niche. Among metastatic breast cancer patients, approximately 70% (~60,000 to 90,000/yr.) have significant bone tumor that can lead to significant adverse skeletal complications, including pathological fracture, spinal cord compression, and severe bone pain. Importantly, skeletal related morbidity increases mortality risk 42% to 2.4-fold. In research funded by the NCI, Katherine Weilbaecher, M.D. and Gregory Lanza, M.D. Ph.D. demonstrated that the majority of chemo-resistant BC metastases in bone have increased expression of αvβ3-integrin (αvβ3). Using a novel prodrug-nanoparticle (20nm) technology, Weilbaecher and Lanza specifically targeted BC cells, circumvented their chemoresistance, and reduced tumor burden and skeletal damage. Based on this and additional studies, Alembic Pharmaceuticals Ltd. licensed the intellectual property estate from Washington University and has partnered with Lanza, Director of the Consortium for Translational Research in Advanced Imaging and Nanomedicine (C-TRAIN) and BGJ Pharma LLC (St. Louis) to develop and de-risk the GMP manufacturing, safety and efficacy of the BC product candidate. Nanoparticle biocompatibility is a key element for translation and a strength of the NCI Nanotechnology Characterization Laboratory (NCL). After a technology review and impact assessment, the concept was accepted into the NCL characterization program, in support of this industry-academic development collaboration.

Conflict of interest statement: Gregory M. Lanza has a financial stake in BGJ Pharma LLC.

Sitka Biopharma, Inc.

Sitka Biopharma is a preclinical biotechnology company focused on developing its breakthrough nanoparticle platform technology to increase absorption of drugs in difficult-to-penetrate tissues. Initially targeting oncology indications, Sitka is developing the lead candidate (STK-01) to address the absorption challenge of intravesical chemotherapy for bladder cancer, and later intraperitoneal delivery for ovarian cancer. The nanoparticle platform is particularly well-suited for treating relatively localized diseases where direct administration of the therapeutic is possible but is currently ineffective or unsafe. Bladder cancer is characterized by therapies that exhibit limited uptake and efficacy, or are associated with significant tolerability issues and side effects. Sitka's novel product (STK-01) is designed to overcome these issues and improve treatment outcomes by delivering a much higher concentration of the chemotherapeutic into the bladder wall with long term retention. The NCL collaboration will focus on characterizing unique properties of the nanoparticle that can be better understood for improved behavior during production and formulation.

http://www.sitkabiopharma.com/

If you are interested in learning more about the NCL's services, please visit our website, https://ncl.cancer.gov, or contact us for more information, ncl@mail.nih.gov. **The next application deadline is June 1, 2021**.

The company and nanoparticle descriptions herein were supplied by the Awardee.
No endorsement of any company or product by NCI or NCL is implied.