NSU Florida

APRIL 2024

RESEARCH UPDATE

NSU Rumbaugh-Goodwin Institute for Cancer Research

Royal Dames of Cancer Research, Inc.

NSU RUMBAUGH-GOODWIN INSTITUTE FOR CANCER RESEARCH

MISSION

Through innovative research, we are developing sensitive methods for detection, devising novel strategies for prevention, discovering new drugs for curing cancer, and saving human lives.

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"Mankind is not destined to die from cancer."

NOVA SOUTHEASTERN UNIVERSITY
Barry and Judy Silverman College of Pharmacy
Rumbaugh-Goodwin Institute for Cancer Research

EXECUTIVE SUMMARY

"Our exceptionally trained research scientists and students come to the lab every day collectively focused on developing novel cancer therapies that will reach patients' bedsides in the near future."

—Appu Rathinavelu, Ph.D.
Director, NSU Rumbaugh-Goodwin Institute for Cancer Research

For more than 60 years, NSU's Rumbaugh-Goodwin Institute for Cancer Research (RGI) has been conducting groundbreaking studies to advance the understanding and treatment of the most aggressive forms of cancer—one of humanity's most formidable foes. With the generous and dedicated support of the Royal Dames of Cancer Research, which totals $4,935,350 as of March 2024, RGI has focused on research to improve cancer diagnosis, treatments, and prognosis. Its breadth of research and discovery includes drug developments for brain, breast, ovarian, pediatric, lung, and prostate cancers, among others. This issue of the RGI Research Update highlights the various research activities and achievements occurring between October 2023 and March 2024, including the institute's most promising drug discovery for treating brain cancer, F16, which will be renamed RD1 in tribute to the Royal Dames' longstanding support.

GLIOBLASTOMA PROJECT

In 2007, RGI director Appu Rathinavelu, Ph.D., identified drug targets and small molecule compounds (drugs code named F16 and JFD) that inhibit cancer growth with low toxicity. The next several years were dedicated, in part, to conducting extensive preclinical testing and developing formulations for F16 and JFD.

In 2015, Rathinavelu made a breakthrough discovery: intraperitoneally injected F16 passed through the blood-brain barrier. NSU responded quickly by developing a team of Ph.D.-level technology managers and patent attorneys to design definitive and enabling experiments. Rathinavelu and his research team at RGI spent three weeks at the Mayo Clinic to study their animal models for brain cancer (glioblastoma) research. Upon returning, the RGI team reproduced the Mayo Clinic animal model at the NSU Center for Collaborative Research (CCR) and began experiments. The results from the RGI's F16 experiments were promising and used to file for worldwide patent protection and publish high-impact papers.

In 2020, the COVID-19 pandemic delayed progress in many of RGI's projects, including the F16 and JFD studies.

In 2022, RGI began its planning for first in-human phase I clinical trials. RGI and NSU leadership considered two traditional approaches to advance the F16 study.

In 2023, RGI and NSU engaged a top-tier pharmaceutical company to reformulate and externally validate the clinical use of F16 for treating glioblastoma.

In 2024, RGI and NSU are finalizing the collaborative agreement with its pharmaceutical partner.

RGI's patented F16 drug therapy will be renamed RD1 (Royal Dames 1) in tribute to the philanthropic organization whose investment made this drug discovery possible.

PRODUCT DEVELOPMENT PLAN

STEP 1

COMPLETE (SUMMER 2023)

Engaged a top-tier contract research development organization (CRDO).

STEP 2

DEFINE ROLES AND RESPONSIBILITIES

CRDO outline charts a clear course for navigating clinical trial planning and administration.

STEP 3

EXECUTE DEVELOPMENT PLAN

- Refine formulation of F16 as an intravenous (IV) injectable with therapeutic solubility. (CRDO + RGI)
- Conduct confirmation preclinical tests to freeze the formulation. (CRDO + RGI)
- Develop pre-investigational new drug (IND) documents and meet with the Food and Drug Administration (FDA) for a pre-IND meeting. (CRDO or + RGI)
- Manufacture F16 under good manufacturing practices (GMP). (CRDO)
- Conduct definitive preclinical studies under good laboratory practices (GLP) conditions. (RGI)
- Develop documents and apply for IND for phase I clinical trials. (CRDO)
- Secure granted IND. (FDA)
- Initiate phase I clinical trials. (CRDO)

2023–2025

ADDITIONAL TESTING AND RESEARCH

PEDIATRIC NEUROBLASTOMA

RGI has initiated new research under the leadership of Appu Rathinavelu, Ph.D., RGI director, to find better cures for treating pediatric neuroblastoma. With start-up funding from the National Pediatric Cancer Foundation, RGI initiated testing of four compounds for the treatment of pediatric neuroblastoma, including F16, JFD, bortezomib, and a new drug known as CM272. Of the four compounds, CM272 seems to be the most potent. Additional tests are being conducted at RGI to validate *in vitro* findings. Once the *in vitro* studies are completed, RGI will initiate preclinical evaluations to determine the clinical use of CM272, as well as some of the analogs of F16. In February 2024, Rathinavelu and his research team submitted an Invention Disclosure to NSU's Office of Research and Technology Transfer (RTT), regarding the discovery of CM272 and RG-7388 for treating neuroblastoma. This Invention Disclosure will be reviewed by the RTT internal team, and a determination will be made regarding the filing of a patent application to protect this invention.

PERIPHERAL NEUROPATHY

Chemotherapy drugs can induce a debilitating side effect called chemotherapy-induced peripheral neuropathy (CIPN). Development of CIPN causes severe pain and can affect patients' abilities to walk, write, or even button a shirt. CIPN can last for weeks, months, or even years after chemotherapy treatment is done. RGI has completed the analysis of 40 patient samples, in collaboration with Chieh-Lin Fu, M.D., of Cleveland Clinic of Florida, and Sultan Ahmed, M.D., of JAS Medical Management, and is currently engaged in analyzing nearly 400,000 data points collected from these patients. Once the analysis and the identification of the epigenetic signature is completed, and after additional validation of the findings, RGI is planning to submit an invention disclosure with NSU.

MELANOMA PROJECT

The National Cancer Institute estimates more than 900,000 people live with melanoma in the United States. Despite recent advances in melanoma drug discovery, overall survival for patients with late-stage metastatic melanoma averages less than three years.

Under the leadership of RGI's Dmitriy Minond, Ph.D., novel compounds are being tested for the treatment of melanoma. Preliminary findings suggest that some of the molecules discovered by RGI can modulate the spliceosome function in cancer cells, leading to the increase of signaling toward melanoma cells—a response which can be beneficial to patients.

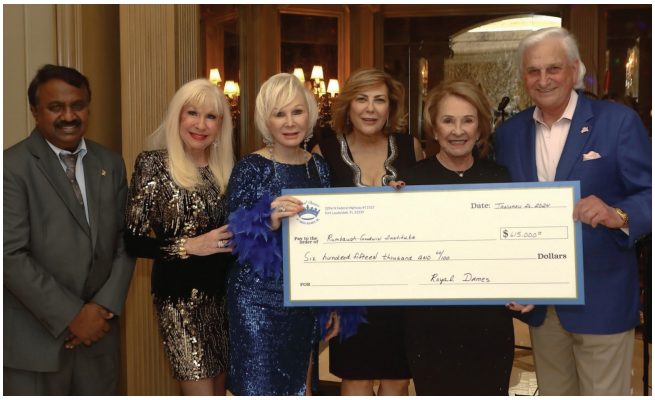
RECENT GRANT SUBMISSIONS

BREAST CANCER IMMUNOTHERAPY
Sponsor: Bankhead-Coley Cancer Research Program at the Florida Department of Health
Investigators/Collaborations: Dmitriy Minond, Ph.D., associate professor at the NSU Barry and Judy Silverman College of Pharmacy and lead researcher at NSU's RGI and the NSU AutoNation Institute for Breast Cancer Research and Care
Project: "Novel Strategy for Breast Cancer Immunotherapy"

PROSTATE CANCER THERAPY
Sponsor: National Institutes of Health (NIH)
Investigator: Dmitriy Minond, Ph.D., associate professor at the NSU Barry and Judy Silverman College of Pharmacy and lead researcher at NSU's RGI
Project: "In Vivo Probe Development of Inducers of RNA-Binding Protein 3 Expression for Taxane-Resistant Prostate Cancer Therapy"

MILESTONES

- 7 patents awarded (#1 in NSU)
- 70+ publications
- 100+ presentations
- 120+ undergrad students trained
- 11 Ph.D. students trained
- powerful collaborations with Moffitt, University of Miami, Mayo Clinic, and others
- $2 million+ in sponsored research
- $15 million+ in philanthropy



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WAYS TO SUPPORT

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At NSU, you can customize your philanthropic investment. Gifts can be made outright, pledged over time, or deferred as a gift of insurance or retirement benefits.

All gifts are tax-deductible.

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