NSU Florida

SEPTEMBER 2024

RESEARCH UPDATE

Royal Dames of Cancer Research, Inc.

NSU Rumbaugh-Goodwin Institute for Cancer Research

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.

IN THIS REPORT

"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 September 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 March and September 2024, including the institute's most promising drug discovery for treating brain cancer, F16, which has been renamed RD1 in tribute to the Royal Dames' long-standing support.

GLIOBLASTOMA PROJECT

In 2007, RGI director Appu Rathinavelu, Ph.D., identified drug targets and small molecule compounds (drugs code named RD1 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 RD1 and JFD.

In 2015, Rathinavelu made a breakthrough discovery: intraperitoneally injected RD1 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 RD1 experiments were promising and used to file for worldwide patent protection and publish high-impact papers.

In 2022, RGI began its planning for first in-human phase I clinical trials.

In 2024, NSU initiated discussions with University of Iowa Pharmaceuticals (UI Pharmaceuticals), a clinical research development organization (CRDO), and Charles River Laboratories of Boston, Massachusetts, a contract research organization (CRO), to reformulate and externally validate the clinical use of RD1 for treating glioblastoma.

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

PRODUCT DEVELOPMENT PLAN

STEP 1

COMPLETE (FALL 2024)

Engage UI Pharmaceuticals (CRDO) and Charles River Laboratories (CRO) of Boston, Massachusetts, to develop human formulation for RD1 and conduct safety studies.

STEP 2

DEFINE ROLES AND RESPONSIBILITIES

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

STEP 3

EXECUTE DEVELOPMENT PLAN

- Refine formulation of RD1 as an intravenous (IV) injectable with therapeutic solubility. (CRDO + RGI)
- Conduct confirmation preclinical testing 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 RD1 under good manufacturing practices (GMP). (CRDO)
- Conduct definitive preclinical studies under good laboratory practices (GLP) conditions. (RGI + CRDO)
- 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 RD1, 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 RD1. 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. In October 2024, Rathinavelu and his team are submitting a proposal to the Live Like Bella Pediatric Cancer program of the Florida Department of Health and will be requesting $250,000 in funding for conducting this research.

OVARIAN CANCER RESEARCH

RGI scientists and graduate students are exploring the feasibility of combining anticancer drugs with nuclear export signal (NES) inhibitors for treating chemo-resistant ovarian cancer. The researchers are conducting experiments with drug-resistant ovarian cancer cells obtained from England.

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.

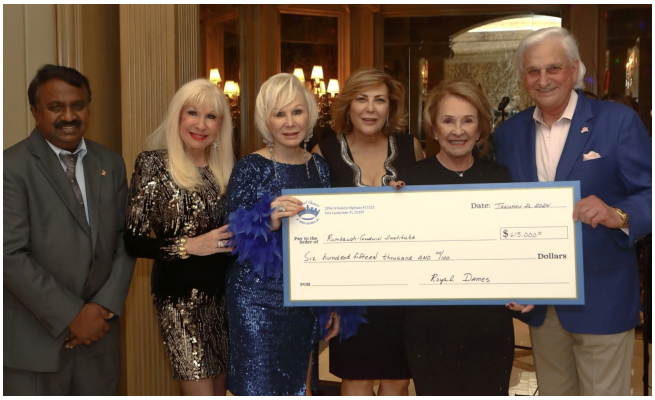
RECENT GRANT SUBMISSIONS

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"

NEUROFIBROMA TREATMENT
Sponsor: Department of Defense (DoD)
Investigators: Appu Rathinavelu, Ph.D., Umamaheswari Natarajan, Ph.D., and Young Kwon, Ph.D.
Project: "Development of Topical Gel by Including F16 and Sutent for Treating Cutaneous Neuro Fibroma (cNFS)"

MILESTONES

- 7 patents awarded (#1 in NSU)
- 70+ publications
- 120+ presentations
- 150+ 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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royaldames.org

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NSU RESEARCH AT A GLANCE

- Alternative Therapies for the Treatment of Aging and CNS Neurological Conditions
- Cancer/Anticancer Therapies
- Cancer Biology
- Cervical Cancer and HIV Prevention
- Degenerative and Cognitive Brain Research
- Dental/Oral Cancer
- Developing Targeted Therapies for Sarcomas
- Neuroscience and ALS
- Regenerative Medicine

TECHNOLOGY TRANSFER

71	38	22
Patent Applications	Worldwide Patents	High-Potential Technologies

CLINICAL RESEARCH

31 Active Clinical Trials

$14M Clinical Research Awards

SPONSORED PROGRAMS

257 Active Awards

$165M Active Funding Across 14 Colleges

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