RARE CANCERS RESEARCH PROGRAM

CDMRP
DEPARTMENT OF DEFENSE
CONGRESSIONALLY DIRECTED MEDICAL RESEARCH PROGRAMS

MISSION: Elevate rare cancers research to catalyze knowledge building and enable clinically impactful discoveries for the benefit of Service Members, their Families, Veterans and the American public

Congressional Appropriations

FY20-FY25:

$95M total

"It is important to me to be a voice for all rare cancer patients. Rare cancers are in desperate need to be studied and understood. While this is a daunting task, because there are less patients to be studied, the research is crucial in finding treatments and cures for those without a successful treatment path. As they say, the squeaky wheel gets the oil, so I will continue to squeak for rare cancers for as long as I can. It has truly been a privilege to serve on the RCRP. As a consumer reviewer, with no medical or scientific background, it is humbling and often brings a tear to my eye to be in the presence of the brilliant minds serving as scientific reviewers and yet be a voice that is heard and valued."

Leah Heinrich, The EHE Foundation, FY20-FY22 Consumer Peer Reviewer

SCOPE OF THE PROBLEM

Rare Cancers: Incidence of fewer than 6 cases per 100,000 people per year in the U.S.

Rare cancers account for **20%-25%** of all cancer diagnoses and **~25%** of all cancer deaths each year.[1]

An estimated **200 cancers** are considered rare.[2]

The Gene Expression Omnibus repository lacks publicly available datasets for **more than 100** rare cancers.[3]

RELEVANCE TO MILITARY HEALTH

Over **60** forms of cancer, of which most are rare, disproportionately impact Service Members and Veterans compared to the general public[4]

Of the cancers treated by the Veterans Health Administration, **16%** are rare[5]

PROGRAM PRIORITIES

- **Biology:** Identify disease-defining molecular pathways, cell context and microenvironment
- **Research Models:** Develop and validate rare tumor-specific models that can support clinical trial readiness
- **Therapy:** Identify novel therapeutic strategies, including drug repurposing
- **Research Platform Development:** Development of research platforms for multiple rare cancers to allow sharing of data, biospecimens and resources

1 DeSantis CE. *CA Cancer J Clin* 67, 2017:261-272.
2 Vivekanandhan S, et al. *Mol Cancer* 22, 2023:23.
3 Rare Cancer's "Valley of Death." American Association of Cancer Researchers 2019: Abstract 4194.
4 https://www.congress.gov/congressional-report/117th-congress/house-report/118/1.
5 https://news.va.gov/101361/vas-progress-on-treating-cancer-since-2016/

PROGRAM IMPACT AND OUTCOMES

Developing Platforms for Resource Sharing

RCRP addresses gaps in data communication and dissemination strategies through knowledge-sharing and resource-sharing platforms.

Acral and mucosal melanoma registry

Rare ovarian cancer registries and biobanks

SCCOHT-SMARCA4

SCST

Rare pediatric eye cancers biobank

RCRP supports collaborations across nearly **40** countries all over the world.

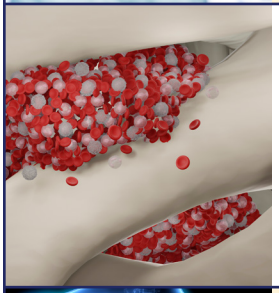
RCRP-supported research addresses over **85** types/subtypes of cancers.

30%-35% of RCRP-funded research addresses rare pediatric cancers.

RESEARCH BREAKTHROUGHS — MAKING A DIFFERENCE

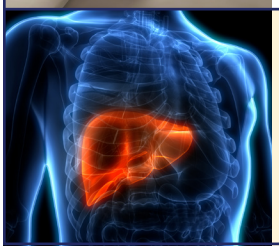
Soft Tissue Sarcoma Functional Genomics Platform

- **Direct-to-patient** collection vehicle for living tissue donation in rare soft tissue sarcomas, including desmoid tumors, clear cell sarcomas and leiomyosarcoma
- Pipeline for patient-directed tissue donation through to patient-specific drug compound prioritization for **personalized treatment** recommendations
- **Computational model** to predict genetic dependencies, which to date identified eight high-priority targets in two desmoid tumor models



Amyloid Clearance Immunotherapy for Light Chain Amyloidosis

- Light chain amyloidosis is irreversible damage to tissues and organs when bone marrow plasma cells produce insoluble fiber deposits
- Engineered macrophages, a type of immune cell, demonstrate the ability to clear amyloids, or the insoluble fiber deposits
- Suggests a potential novel **immunotherapy** for treating amyloidosis to significantly improve patient outcomes



Identifying Targeted Combination Therapies in a Novel In Vivo Model of Fibrolamellar Carcinoma

- Development of a **model** for fibrolamellar carcinoma, a rare liver cancer that primarily affects teens and young adults
- **Combination screening** identified two synergistic drug combinations for future treatment development
- Addresses lack of pre-clinical models needed to **develop treatments**

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