CDMRP
Department of Defense

Rare Cancers Research Program

Strategic Plan

INTRODUCTION

The Congressionally Directed Medical Research Programs (CDMRP) represents a unique partnership among the U.S. Congress, the military, and the public to fund innovative and impactful medical research in targeted program areas. Programs managed by the CDMRP have formalized strategic plans that identify program-specific research priorities, how to best address these urgencies, short- and long-term goals, investment strategies, and ways to identify and evaluate program successes with respect to the priorities.

The Rare Cancers Research Program (RCRP) Strategic Plan identifies the high-impact research goals are most important to its stakeholders while providing a framework that is adaptable to changes in the medical research environment to address those goals. This plan has been formulated to provide greater clarity of the program's goals over time to the public and other stakeholders. Congress appropriates funding for RCRP on an annual basis; there is no guarantee of future funding. The RCRP Programmatic Panel members will review the Strategic Plan during the program's annual Vision Setting meeting and update it as necessary.

RCRP BACKGROUND AND OVERVIEW

The American Cancer Society defines rare cancers as those affecting less than 6 persons per 100,000 per year in the United States.[1] By this definition, there are around 200 forms of rare cancer, affecting more than 400,000 Americans per year. These patients face additional challenges not shared by those affected with more common cancers. Due to their low prevalence, rare cancers are harder to diagnose and are often diagnosed at later stages, making them more difficult to treat. Treatment options for rare cancers are limited due to fewer clinical research and clinical trials. This translates to worse outcomes for patients with these cancers. A European study estimated the average 5-year survival rate for patients with rare cancers is 47%, compared to 65% for those with common cancers.[2]

The RCRP was established in fiscal year 2020 (FY20), with a Congressional appropriation of $7.5 million (M). In order to gain an understanding of the current landscape in rare cancers research, the RCRP distributed a survey to stakeholders soliciting their input on the knowledge gaps and needs of the field. Responses were received from over 40 organizations/institutes. The top two cross-cutting challenges identified were scarcity of patients and patient samples and the need for a network to facilitate collaboration and resource sharing.

A summary of the results of the RCRP FY20 Stakeholder survey is available on the CDMRP website.[3] Subsequently, a Stakeholders meeting was held to further identify what initiatives are needed to propel the rare cancers field forward, a summary of which is also available on the website.[4] It was also determined at this meeting that the RCRP would follow the American Cancer Society's definition of a rare cancer: cancers affecting less than 6 persons per 100,000 per year in the United States.

VISION AND MISSION OF THE RCRP

VISION: To greatly improve outcomes for people with rare cancers through discovery and community building, and expansion of knowledge across the cancer landscape

MISSION: Elevate rare cancers research to enable clinically impactful discoveries for the benefit of Service members, their families, Veterans, and the American public

FUNDING HISTORY

The rare cancers research topic area was first directed by the Congress under the Peer Reviewed Cancer Research Program (PRCRP) in FY19. Since its inception (FY09) the PRCRP funded research on rare cancer types/subtypes, totaling over $90M (Figure 1). Research on other rare cancers topic/subtopic areas had also been awarded by the Breast Cancer Research Program (FY92-FY19), Melanoma Research Program (FY19), and the Ovarian Cancer Program (FY99-FY19).

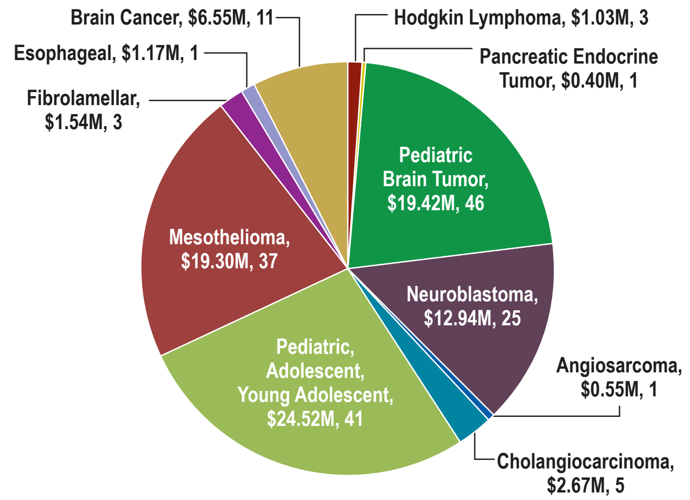

Figure 1. Rare Cancer Types/Subtypes funded by the PRCRP (FY09-FY19). Graph depicts the total dollar amount and number of awards funded.

STRATEGIC DIRECTION

The FY20 Vision Setting meeting was held to develop a roadmap of the program in order to address the unmet medical needs, knowledge gaps, and consumer concerns identified during the FY20 Stakeholders meeting. Four Focus Areas were developed at the Vision Setting meeting to provide a specific direction of the program by utilizing the available resources and scopes judiciously (Table 1). For its inaugural year, the RCRP developed three Program Announcements (Concept Award [CA], Idea Development Award [IDA], and Resources and Community Development Award [RCDA]) with defined programmatic focus of improving outcomes for people with rare cancers through discovery, development of therapeutics, community building, and expansion of knowledge across the cancer landscape.

The RCRP required all FY20 applications to address at least one of the Focus Areas (Table 1). Applicants were also required to provide a justification statement explaining the relevance of the cancer types/subtypes according to the RCRP's definition of rare cancer. During future Vision Setting meetings, the Programmatic Panel will reassess the investment strategy, research landscape, and gaps in patient care and modify offered funding opportunities accordingly.

Table 1. FY20 RCRP Focus Areas and Award Mechanisms

Focus Areas	Award Mechanisms that Apply
Biology and Etiology: Identify disease-defining molecular pathways, cell context, and microenvironment.	CA and IDA
Research Model: Develop and validate rare tumor-specific models that can support clinical trial readiness.	CA and IDA
Platform Development: Development of platforms (such as tumor tissue repository with clinical annotation; centralized databanks; patient registry with common data structure; research model, and Omics database; longitudinal studies of natural history and treatment response) for multiple rare cancers to allow sharing of data, bio-specimens, and resources.	CA and RCDA
Therapy: Identify novel therapeutic strategies, including drug repurposing.	CA and IDA

INVESTMENT STRATEGY

In addition to the Focus Areas, the investment strategy (Table 2) was developed to reflect the vision and mission of the program, as well as to answer the needs of the research and patient communities. Three FY20 Program Announcements were released to support novel research ideas for rare cancers, to foster the development of platforms for resource sharing across multiple rare cancer types, and to establish collaborative endeavors among stakeholders.

The CA supports highly innovative, untested, potentially groundbreaking novel concepts in rare cancers that have the potential to reveal entirely new avenues for investigation.

The IDA promotes new ideas that are still in the early stages of development and have the potential to yield impactful data and new avenues of investigation. In addition, the IDA supports conceptually innovative, high-risk/high-reward research that could lead to critical discoveries or major advancements that will accelerate progress toward eradicating deaths and suffering from rare cancers.

The RCDA supports the development of platforms that can share resources and knowledge pertaining to available models, molecular pathways, and therapeutic approaches to facilitate collaboration and information sharing among researchers, patients, caregivers, clinicians, and other members of the rare cancers community.

Table 2. FY20 RCRP Award Mechanism Key Elements and Funding Information

Award Mechanism	Funding
Concept Award	Maximum funding of $100k for direct costs (plus indirect costs) for up to 1 year
Idea Development Award	Maximum funding of $350k for direct costs (plus indirect costs) for up to 3 years
Resource and Community Development Award	Maximum funding of $600k for direct costs (plus indirect costs) for up to 3 years

RCRP GOALS

RCRP near- to long-term goals are listed in Table 3.

Table 3. RCRP Goals

NEAR- TO MIDDLE-TERM GOALS (2-5 YEARS)
• Develop novel studies through collaborative research endeavors
• Create partnerships between the research community and advocacy organizations
• Develop innovative cellular and pre-clinical models for rare cancer research
MEDIUM- TO LONG-TERM GOALS (5-10 YEARS)
• Invest in studies that will foster drug repurposing with older agents and/or novel combinations
• Build registries that have a common data structure and give patients control of their data
• Enable longitudinal study of the disease nature and treatment response
• Invest in studies that link the molecular data to natural history
• Generate data/reagent/model exchange programs where researchers share resources tagged with indications that are relevant to the rare cancer research

MEASURING PROGRESS

The RCRP will measure its progress based on how Focus Areas are addressed and goals are achieved. The near-term success will be determined by the following outcomes:

- Contribution to the scientific community (publications, presentations, patents, etc.) by developing pre-clinical research models or pathways
- Number of applications received and funded within each of the RCRP Focus Areas

Long-term success will be evaluated based on the following outcomes:

- Development of a number of various resources, such as patient registries, tissue repositories with clinical annotations and centralized databanks, research databases, and others
- Clinical treatment paradigms resulting from RCRP-funded research

- Investigational New Drug Application/Investigative New Device applications to the US Food and Drug Administration as a result of RCRP funded research
- Subsequent Federal or non-Federal funding of initial RCRP-funded research

In addition, the RCRP will evaluate all projects at the time of award to determine the appropriate knowledge readiness level (KRL). At the end of each award's period of performance, the project's KRL will be measured for state-of-science and impact on the RCRP field at large. This will give the RCRP an understanding of the program's impact and the relevance of the strategic goals and priorities toward the program's vision and mission.

REFERENCES

1. DeSantis CE. 2017. The burden of rare cancers in the United States. *CA Cancer J Clin,* 67:261-272.
2. Gatta G, van der Zwan JM, Casali PG, et al. 2011. Rare cancers are not so rare: The rare cancer burden in Europe. Eur J Cancer Nov; 47(17):2493-2511. doi: 10.1016/j.ejca.2011.08.008. Epub 2011 Oct 25. PMID: 22033323.
3. https://cdmrp.army.mil/rcrp/pdf/Summary%20Stakeholders%20Survey%20for%20website.pdf
4. https://cdmrp.army.mil/rcrp/pdf/Summary%20Stakeholders%20Meeting%20for%20website.pdf