Results of Stakeholder Surveys

In FY20 the Rare Cancer Research Program (RCRP) was included in the United States FY20 omnibus for the first time with an appropriation of $7.5M. The RCRP will be established as a CDMRP program under the Defense Health Agency (DHA) J9, Research and Development Directorate, of the U.S. Army Medical Research Acquisition Activity.

In an effort to receive immediate feedback from across the rare cancers community, the RCRP distributed a survey aimed at helping to identify the needs of the field and shaping the research focus of this new program. A total of 51 people responded to the survey, representing more than 40 organizations/institutes.

Respondents identified the **top common and cross-cutting challenge** across all rare cancer research as the **lack of patients and patient samples** (Figure 1). Two other major challenges identified included **lack of funding** and **limited data sharing and collaboration** including the **lack of a network** to facilitate working together.

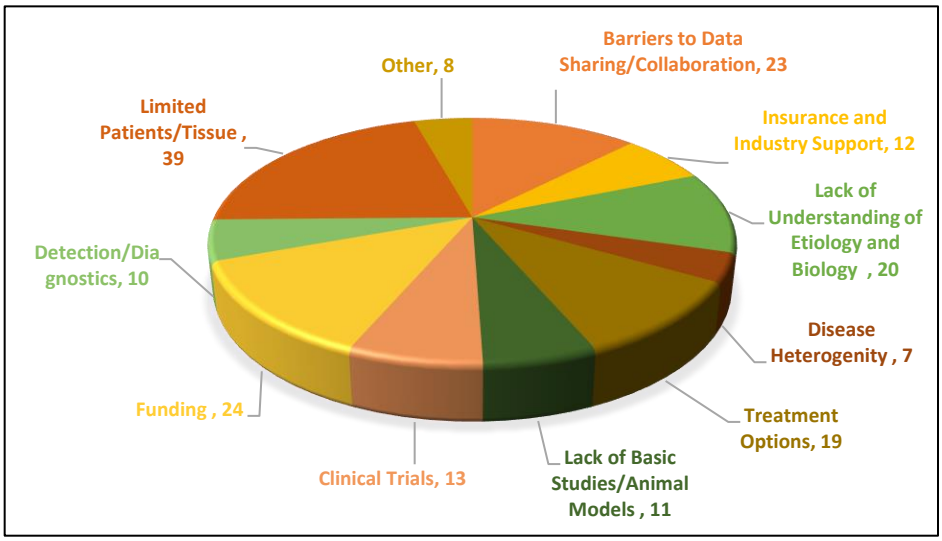

Figure 1: Identified Challenges Across All Rare Cancers

The need for **more basic research, preclinical data, and animal and cell-based models** were identified as areas of rare cancer research that are currently underfunded or understudied with the potential to have a significant impact on the state of the science across all rare cancers, if well-resourced (Figure 2). Research into **genomics** and the **identification of biomarkers** was also identified as an understudied area, which included both whole genome or exome sequencing and bioinformatics studies to single cell sequencing.

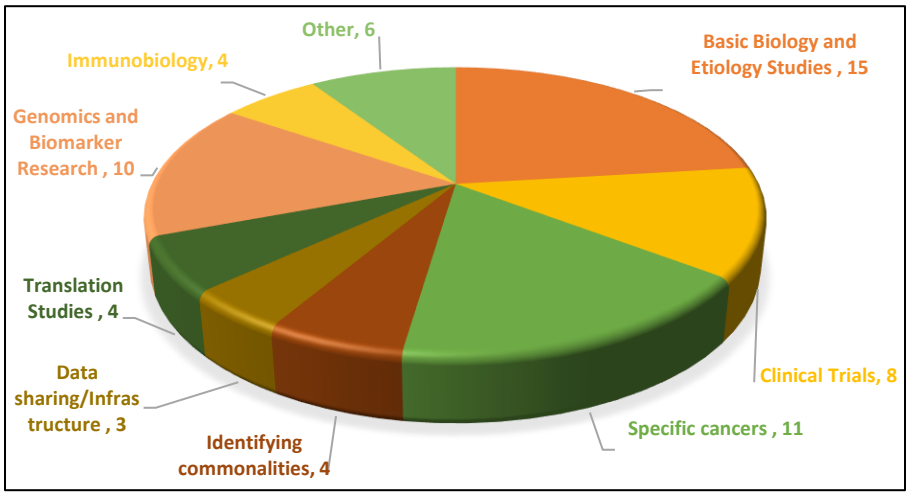

Figure 2: Identified Areas of Rare Cancer Research That Are Underfunded or Understudied

When asked how the program can best address the **current funding gaps** to facilitate progress for patient care (Figure 3), the largest response received addressed the need for the program to **support collaboration and infrastructure** to facilitate **data and resource sharing** between investigators and institutes. Responses mentioned the need to bring clinicians and researchers together with patient advocacy and patients. It was suggested that collaborations should span both multiple sites and multiple disciplines to foster research that may be cross-cutting across rare cancer types. The second most common response was the recommendation to fund **clinical trials and clinical trial consortiums, particular early-phase trials**.

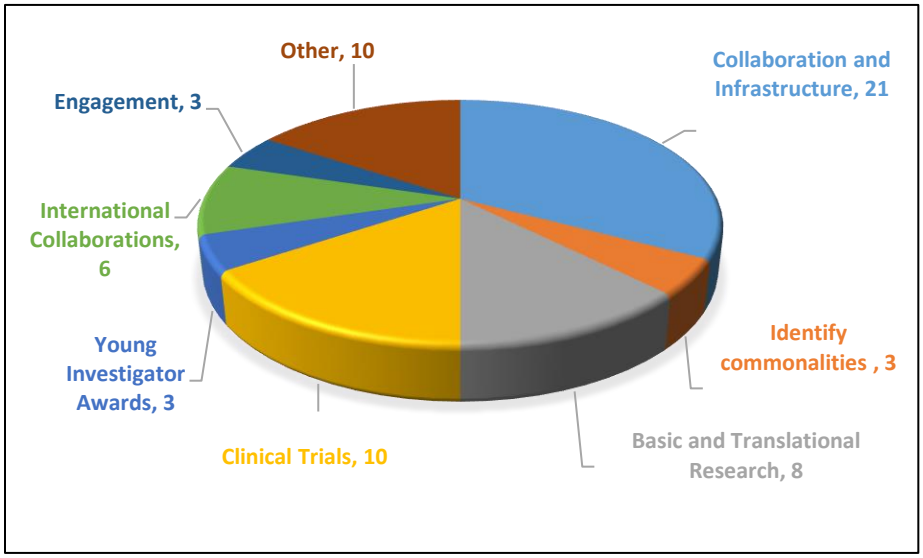

Figure 3: Recommendations for How the RCRP Can Address Current Funding Gaps and Facilitate Progress for Patient Care

Key Alerts/Gaps/Recommendations

The following were generated from the results of the Stakeholders Surveys.

Key Alerts:
• Small numbers of patients with any given rare cancer
• Poor collaboration amongst researchers and consortia
• Limited data sharing and hard to access the data: Poor infrastructure for data collection: clinical, biological, imaging
• Smaller critical mass of researchers per cancer
• Inadequate overall cross cutting strategy among Government agencies, patient advocacy, and industry
• Limited drug development specific for rare cancers
Identified Gaps
• Understanding of the early stages and natural history of the disease
• Tumor associated genetic profiles of rare cancer types (including single-cell and multi-omics strategies)
• Biomarkers for diagnosis, prognosis, and drug effectiveness
• Clinical trial strategies (including novel statistical methods for small sample sizes)
• Insufficient focus on study what is common across many rare cancers to improve understanding and accelerate development of treatments
• Lack of understanding of the natural history of rare cancers
• Better understanding of the molecular and cellular mechanisms regulating tumorigenesis
• Understanding the failure of current therapies
• Drug development specific for rare cancers
• Preclinical data on repurposed and new drugs; safety trials for repurposing drugs that could help patients
• Immunoprofiling of rare tumor specimens
• Development of early diagnostic assays
• Lack of basic research tools (cell lines, PDX, organoid models)
• Lack of transgenic models are rare cancers
• Lack of preclinical models to show mechanism of action often required by pharma to extend drug support for rare tumor clinical protocols
• Lack of support for platform innovations that can work across multiple rare cancers
Key Recommendations
• Support for collaborative research, including international studies
• Clinical trial consortiums, especially early Phase I trials
• Support for basic and translational research
• Use of platform trials or trials that search for commonalities across rare cancers that could exert a broad impact