

# THE CHALLENGES POSED BY RARE CANCERS

The National Cancer Institute (NCI) considers a type of cancer rare if it occurs in fewer than 15 out of 100,000 people each year. All childhood cancers are considered rare cancers. Rare cancers pose significant challenges to many stakeholders in the cancer community, including patients, physicians, and researchers. These include:



## Challenges for Patients

- The long time it takes from when they first notice a symptom to the time when doctors know that the symptom is caused by a rare cancer and what type of cancer it is.
- Finding a physician who is knowledgeable about the rare cancer with which they have been diagnosed and how to treat it.
- Often the necessity to travel far from location of primary residence to get treatment for a rare cancer.



## Challenges for Physicians

- Lack of adequate training to treat a rare cancer with which their patient has been diagnosed.
- Lack of knowledge to discuss in-depth with the patient the treatment and management options for the rare cancer.
- Lack of subject matter experts who can answer questions about the rare cancer with which their patient has been diagnosed or identify someone to whom they can refer the patient.



## Challenges for Researchers

- Not enough information about the rare cancer to develop hypotheses and address key questions.
- Not enough animal or cell models of the rare cancer to test their hypotheses.
- Not enough tumor samples from patients with the rare cancer for their research.
- Not enough patients with a given rare cancer to conduct a clinical trial testing a potential new treatment.

In recent years, many initiatives have been launched with the goal of accelerating the pace of basic, translational, and clinical research in rare cancers, including the following involving the National Institutes of Health (NIH) and NCI:

### The International Rare Cancer Initiative (IRCI)

Established in 2011 by NCI, the UK National Institute for Health Research, Cancer Research UK, and the European Organisation for Research and Treatment of Cancer, the goal of the IRCI is to conduct practice-changing clinical trials for patients with rare cancers. The founding members were subsequently joined by the French National Cancer Institute, the Canadian Clinical Trials Group, the Japan Clinical Oncology Group, and the Clinical Oncology Society of Australia. The initiative to date has convened working groups looking at 12 rare cancer types, opened seven trials, and completed trials in high-grade uterine sarcoma and metastatic anal cancer. Many other clinical trials are underway or planned.

### The NCI Rare Tumor Initiative

Launched in 2013, the goal of the NCI Rare Tumor Initiative is to foster closer collaborations between basic and clinical scientists, patient advocacy groups, and industry partners in the field of rare tumors to facilitate the development of new approaches to treating patients with rare cancers.

### Rare Tumor Patient Engagement Network

As part of the Cancer Moonshot, the NIH Center for Cancer Research is building the rare tumor engagement network to study selected rare pediatric and adult tumors and develop a network of clinical trials. Finding treatments for childhood, teen, and young adult rare solid tumors is the focus of the My Pediatric and Adult Rare Tumor (MyPART) network, while the NCI Comprehensive Oncology Network Evaluating Rare CNS Tumors (NCI-CONNECT) is studying 12 rare central nervous system cancers in adults.