

## THE CHALLENGES POSED BY RARE CANCERS

The National Cancer Institute (NCI) defines a type of cancer as rare if fewer than 40,000 people are diagnosed with the disease 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. According to the NCI, these challenges include:

### Patients may find that it:

- takes a long time 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.
- is hard to find a physician who knows a lot about the rare cancer with which they have been diagnosed and how to treat it.
- is necessary to travel far to get treatment for a rare cancer.

### Physicians may find that they:

- have not been trained to treat a rare cancer with which their patient has been diagnosed.
- do not know what to tell the patient about what to expect with the rare cancer.
- are unable to find an expert who can answer their questions about the rare cancer with which their patient has been diagnosed or identify someone to whom they can refer the patient.

### Researchers may find that:

- there is no information about the rare cancer they are investigating to give ideas on how to go about tackling the disease.
- there are no animal or cell models of the rare cancer they are investigating in which to test their ideas.
- there are not enough tumor samples from patients with the rare cancer they are investigating for their research.
- it is hard to find 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 rare cancer research, including the following involving the National Institutes of Health (NIH) and NCI:

### The International Rare Cancer Initiative (IRCI)

Established in 2011 by the 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 make it possible 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. To date, the IRCI has convened 12 expert groups and has 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's 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 network (MyPART), while the NCI Comprehensive Oncology Network Evaluating Rare CNS Tumors (NCI-CONNECT) is studying 12 rare central nervous system cancers in adults.