



Alliance for Rare Cancers

## **An unprecedented alliance of rare cancer organizations**

### **Our Mission:**

To unite the entire rare cancer community at an unprecedented scale to accelerate cures for all rare cancers – the hidden unmet need of our modern era.

### **The Vision:**

**Every rare cancer patient deserves access to the latest advances in cancer.**

We envision a world where every patient diagnosed with any cancer will quickly have access to a data-driven, clinically proven approach to treatment. The promise of data-driven cancer therapies has engendered hope that, at the time of diagnosis, the molecular profile of a patient's tumor will reliably reveal a tumor's vulnerabilities and guide a patient's options for treatment. Data-driven therapy can and should be available for every rare cancer patient, no matter where they live and no matter how rare their specific cancer diagnosis.

We must transform the fractured landscape of rare cancer research into a coordinated, galvanized effort to directly improve outcomes and treatment options. We will do this by creating a strategic and coordinated alliance of rare cancer stakeholders capable of solving the key challenges that impede innovation in rare cancer treatments. Every patient deserves the right to understand and make data-informed choices in partnership with a medical and scientific community that encourages patient voices and active participation. Technological innovations have made data-driven approaches for rare cancer possible. By centering equity and inclusion of rare cancer patients, we can usher in a new era of hope for rare cancer patients everywhere.

### **The Challenge:**

**Rare cancer patients have been left behind in the fight against cancer.**

While individually rare, rare cancers represent **1 in 4 cancer diagnoses** in the US and include **every type of pediatric cancer**. Yet, rare cancers commonly have no standard of care or suggested set of treatment options. While there is some debate over which cancer types qualify as rare cancers, it is certain that the combined group of patients facing any rare cancer diagnosis represent one of today's greatest unmet medical needs.

Extensive research investments over the past 25 years have led to considerable progress in overall outcomes for "common cancers," the other three quarters of annual cancer diagnoses, and in some select cases, durable remissions. But rare cancer patients have not meaningfully shared in these improvements, with 5-year survival rates for people diagnosed with rare cancers stagnant with an average around 55-60%. In the race for better cancer treatments, rare cancer patients have been left behind despite comprising 25% of cancer patients.

But why does this disparity exist? The good news is that, scientifically, there is no reason to believe that rare tumor types are inherently untreatable. The problem is in fact a human systems issue: the current landscape for basic research, therapeutic development and patient care in the U.S. presents fundamental, compounding challenges that negatively impact progress in treatments for rare cancers.

**These formidable challenges can be reduced to key, intersecting themes** that impact each individual rare cancer type, and if overcome, would clear the way for rapid progress across all rare cancers:

**Geographical dispersion and relative scarcity of patients, resources, and researchers.** Low numbers of patients in any one geographical location makes it difficult to conduct traditional clinical trials and exacerbates challenges to accessing care for these patients. Further, no single study or institution can collect the amount of patient samples and data needed to mount a data-driven treatment program at scale. Without concentrated resources, there is no coordinated cohort of researchers committed to rare cancer.

**Lack of knowledge needed to identify and deliver new therapies.** Without a critical mass of patients, many rare cancers have been woefully understudied. As a result, the information and biospecimens necessary to develop data-driven therapies are not available. Further, clinicians with rare cancer expertise are limited to major academic medical centers, with some rare cancers having no clinical champions. Where investments have been made for rare cancer research, institutional resistance to “real time” collaboration (sharing biospecimens, disease models, and data) has impeded the scientific progress that patients deserve and expect when they participate.

**Mismatch between the level of investment and the scale of the challenge.** Government funding focused on rare cancers has historically not been scaled or sustained to overcome rare cancer-specific obstacles. While structural challenges remain unaddressed, private pharmaceutical companies have not been incentivized to invest in rare cancer patients, who are seen as outside of a serviceable addressable market for innovative oncology products.

### **The Opportunity:**

#### **A chance to collectively propel rare cancers to the forefront of therapeutic advances.**

At present, new transformative science is poised to deliver life-saving treatment and even cures for some cancer patients. Key technologies such as organoid models, single-cell genomics, CRISPR tools, and machine-learning are now fully ripe for fundamentally changing our understanding of what drives cancers and transforming our strategies for treatment development. The organizations that fully seize this opportunity, at the needed scale, will become world leaders for ushering in the full promise of data-driven therapy for all cancer patients.

Together, the rare cancer community is now able to seize such a moment. The ability to perform distributed research and clinical science to overcome challenges of geographic dispersion are finally addressable thanks to the widespread adoption of telecommunication platforms and the digital transformation of many sectors. Further, the precedent for fast-paced

nationally and internationally coordinated team science set in the response to the COVID-19 pandemic has whetted the appetite of researchers for overcoming logistical hurdles to real-time, multi-institutional collaboration in order to make a tangible impact.

Further, the rare cancer community is uniquely positioned to lead the transformation to data-driven cancer treatments and rapidly bring them into existence. Rare cancer patients are highly engaged and responsive to calls for partnership in co-creating solutions for the challenges they face. Rare cancer foundations and advocacy groups are increasingly collaborative in coordinating their efforts.

Institutions across the discovery, development, and care delivery ecosystem currently lack infrastructure for rare cancers, presenting an opportunity to rapidly build novel infrastructure for data-driven approaches in rare cancers without the friction of large-scale change management. The lack of progress for rare cancer patients to-date presents a wide range of opportunities for accelerating the clinical development and regulatory approval of novel, data-driven treatment approaches focused on breakthroughs for rare cancers.

If we act today, we can propel rare cancer patients to the forefront of scientific advancement and ensure they finally have access to the life-saving treatments they deserve. With an organized, systematic approach we can ensure these emerging technologies are focused on and optimized for rare cancers, ensuring that patients facing rare cancers are not left behind.

**The Alliance for Rare Cancers (ARC):  
An unprecedented alliance of rare cancer organizations.**

In November 2023, a group of 35 rare cancer-focused academic leaders, regulatory experts, foundations, and patient advocacy groups convened a workshop to examine the issues and opportunities for rare cancer, and to imagine a path forward towards effective treatments and cures for rare cancer patients. With a shared vision, our coalition is determined to chart that path by **integrating and scaling the best solutions for rare cancer challenges across the discovery, development, and care delivery systems.**

Our number only continues to grow. As we have begun to share our vision, additional key players in the ecosystem have joined our “coalition of the willing,” donating their time and offering to commit resources to this endeavor. Leaders from across medical institutions, major research labs, and large patient-focused research organizations have signaled they are ready to commit to radical rare cancer collaboration: to collaborate at unprecedented scale to make data-driven approaches in rare cancer a reality.

**Join Us.**

Never has this level of collaboration been undertaken in rare cancers, but the promise of data-driven therapies finally reaching our patients has galvanized this community into action. Join us for an unprecedented opportunity to forge a direct path to ending rare cancer as we know it.

Alliance for Rare Cancers | Jedi Rare Cancer Foundation  
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