FRIENDS of CANCER RESEARCH

PARTNERSHIPS **SCIENCE SOLUTIONS**

Friends of Cancer Research drives collaboration among partners from every healthcare sector to power advances in science, policy, and regulation that speed life-saving treatments to patients.

THE POWER OF **COLLABORATION**

During the past 20-plus years, Friends of Cancer Research (*Friends)* has been instrumental in the creation and implementation of policies ensuring patients receive the best treatments in the fastest and safest way possible. We've been successful due to our ability to convene the right people at the right time and put forth revolutionary, yet realistic ideas. We are energized now more than ever to continue this critical work with our trusted partners, creating innovative solutions to overcome barriers standing in the way of conquering cancer. Below are highlights of our collaborations and active initiatives.

BREAKTHROUGH THERAPY: *A PATHWAY THAT REWARDS INNOVATION*

When new drugs aimed to treat serious and life-threatening conditions show unprecedented effect in early clinical testing, patients shouldn't have to wait to receive treatment. To address this complex problem, *Friends* worked with partners in advocacy, regulation, drug development, and bipartisan Congressional champions to take the Breakthrough Therapy Designation from an innovative concept, to scientific whitepaper, to federal law in just 13 months. This resulted in the passage of an expedited FDA development program that ensures patient access to revolutionary drugs as quickly and effectively as possible.

- The concept was initiated at our Annual Meeting with an expert working group that proposed strategies to expedite FDA approval of exceptional drugs intended to treat a serious or life-threatening disease and preliminary clinical evidence suggests it provides a substantial improvement over existing therapies, without sacrificing safety and efficacy standards.
- The program has seen upwards of 450 requests, over 150 designations with more than 50 of those drugs now approved.

LUNG-MAP: *A REVOLUTIONARY PRECISION MEDICINE CLINICAL TRIAL DESIGN*

Taking a new drug from the initial discovery stage through clinical testing and regulatory review is complicated, expensive, and often inefficient. This is compounded by the fact that trials for new drugs are almost always conducted separately, even when multiple drugs are being developed to treat the same condition. To address these hurdles, *Friends* developed a first-of-its kind collaborative clinical trial.

- The approach, first discussed at our Annual Meeting, is a multi-stakeholder partnership with leadership from the FDA, NCI, Foundation for the NIH, research institutions, patient advocacy groups, and industry collaborating to develop a new and more efficient protocol for how future clinical trials could be conducted.
- The trial, a biomarker driven multi-drug study in squamous cell non-small cell lung cancer launched in June 2014, now enrolls over 1,000 patients and is open at more than 700 sites across the U.S. with 5 pharmaceutical companies collaborating on a single trial.

CREATING A BLUEPRINT *FOR DRUG/DIAGNOSTIC CO-DEVELOPMENT*

While cutting-edge drugs have access to special FDA pathways and approval mechanisms, the addition of companion diagnostics that enable their use can complicate the regulatory process. Through our annual "Blueprint" forum, we develop innovative solutions and approaches to address the challenges of drug/diagnostic co-development. Major outcomes of this forum include:

- Identify ways to overcome the challenges associated with biomarker development;
- Facilitate optimal development of diagnostics with breakthrough therapies;
- Develop a regulatory framework for next-generation sequencing as a companion diagnostic; and
- Develop a standardized approach to increase utilization and sharing of large-scale genetic databases.

THE FUTURE OF TREATING CANCER: *IMMUNOTHERAPIES*

Friends is working to further the field of immuno-oncology through the development of a Policy Advisory Group consisting of a small, but representative group of scientific, clinical, patient, policy, and industry thought leaders. These thought leaders will shape a multi-stakeholder process to pave the way for this exciting new science. In April of 2016, *Friends* became a launch partner of The Parker Institute for Cancer Immunotherapy, with *Friends'* Chair, Ellen Sigal, serving on the institute's advisory committee.

ProgressForPatients.org: *Advocacy Alliance & Education*

Advocacy Alliance: *Friends'* new Advocacy Alliance will serve as a portal to stay informed on the most pressing policy issues, current proposals, and national priorities in regulatory science. The Alliance will provide resources and a platform for organizations to join *Friends* as we engage Congress and federal health agencies on the latest issues.

Advocacy Education: Patient advocates devote their time, energy, and resources to help patients, and to make the greatest impact, they need the knowledge and understanding of the laws and regulations affecting the process of new treatments for patients, as well as the institutions that are involved in that process. That is why *Friends* developed our Advocacy Education program. Through this training program, advocates will acquire the necessary tools to effectively communicate with drug researchers, developers, and regulators enabling them to make the connections necessary to engage with all sectors who need to be better guided by patient input.

POLICY *PRIORITIES*

21ST CENTURY POLICIES *FOR 21ST CENTURY SCIENCE & INNOVATION*

- *Friends* was a primary driver of one of the most significant health-related legislative actions of Congress, the 21st Century Cures Act. The Act passed the House on November 30th by a vote of 392-26 and the Senate by a vote of 94-5, shortly before President Obama signed it into law on December 13, 2016.

- *Friends* developed key sections of the bill that represent substantive changes that will improve outcomes for patients. These sections focus on: creating a framework for patient-focused drug development, improving tools to evaluate and advance precision medicine, expanding FDA flexibility, and enhancing the ability for the agency to attract the best and brightest talent.

CENTERS OF EXCELLENCE: *CROSS-CENTER COORDINATION AT THE FDA TO REFLECT CURRENT PATIENT CARE*

Congress has not modernized FDA's organizational structure for medical products since the 1970s. The existing regulatory framework has been defined by a "divide and conquer" approach to oversight; separate centers within FDA regulate three major categories of medical products: drugs, devices, and biologics. In order to take advantage of today's advancements in science, drug development, and patient treatment, *Friends* proposed a reorganization of the FDA's structure to focus its resources and ensure the best outcomes for patients.

- Centers of Excellence will build on previous efforts to develop a more disease-oriented approach to product regulation that have demonstrated the positive effect of this type of organizational structure. They will also allow the agency to develop regular cross-center processes to align with and support employee motivation for regulating and delivering safe and effective medical products to treat major diseases.

- Our proposal was adopted by the White House as part of Vice President Biden's National Cancer Moonshot Initiative. This proposal was included in the 21st Century Cures Act, formally establishing an Oncology Center of Excellence at FDA.