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**Backgrounder**

**Need:** Thousands of children are diagnosed with cancer and other serious conditions each

year, yet new treatment options for kids have fallen short. In fact, there are few

pediatric-specific drugs on the market; instead, children receive “hand-me-down”

medications that were developed for adults.

This creates a serious problem. In addition to obvious physiological and developmental disparities between children and adults, children metabolize drugs and compounds differently than adults, and the diseases they face are often biologically different than adult diseases.

The need for new drugs and treatments is particularly acute in pediatric oncology. Despite the fact that cancer is the leading cause of death from disease for children, with 13,500 new diagnoses each year, no new therapies have been introduced in the past two decades. Treatment options are limited. In fact, many childhood cancers are terminal upon diagnosis. And the outlook for adolescent and young adult cancers is even worse. More than 70,000 patients between the ages of 15 to 39 are diagnosed with cancer each year – nearly five times as many as childhood cancer – with few treatment options and even poorer outcomes. Like childhood cancer, no new therapies for adolescent and young adult cancers have been developed in decades.

**Opportunity:** Phoenix Children’s Hospital, in collaboration with the University of Arizona College of Medicine – Phoenix and the Translational Genomics Research Institute (TGen), have joined forces to bring new hope to pediatric patients with life-threatening diseases. The Ronald A. Matricaria Institute of Molecular Medicine will offer innovative and effective therapies for pediatric, adolescent and young adult populations determined by sophisticated genomic analysis and real-time access to drugs.

Unlike other children’s hospitals, the Institute will diagnose and treat children with serious conditions based on genetic information rather than only clinical symptoms. With an ultimate goal of unlocking genetic codes, the Institute is poised to alter the course of the diseases, define a new paradigm for treatments and bring new hope to patients.

**Approach:** Initially, the Institute will focus on cancer, with the addition of other disease groups over time. The goal is to begin phase one clinical trials in 2013 with child, adolescent and young adult patients with no other treatment options. Longer term, the Institute will establish a comprehensive molecular medicine program that extends across a broad range of medical disciplines and opens the door to innovative therapies determined by patient-specific genomic information.

Clinical trials will be based on underlying genetic and molecular functions of pediatric cancer as opposed to tumor type – an approach never before used in pediatric patient populations.

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In addition, physicians at the Institute will devise treatment plans on a patient-by-patient basis, using drug therapies they believe will attack and correct malfunctioning genes. And the Institute will support a dynamic relationship among the clinic, laboratory and pharmaceutical companies focusing on real-time translation of scientific knowledge and patient data.

Results from the clinic will be shared continuously with researchers, who will incorporate this information into their database of knowledge. In turn, researchers will feed back knowledge to the clinic and provide more informed and enhanced design of clinical trials, which ultimately will result in improved clinical care.

**Infrastructure:** The Ronald A. Matricaria Institute of Molecular Medicine at Phoenix Children's Hospital will be built around a medical and scientific, genomic and clinical infastructure.

*Medical and scientific infrastructure:* Basic biomedical research will be the backboneof the Institute – a discovery engine that will inform the clinical trial design process. Here, researchers will work to understand the genetic and cellular mechanisms of disease, identifying potential signaling and other pathways that may be targets for drugs or biomarkers for indication of disease or disease progression. The Institute team will develop clinical treatment protocols that integrate genomic research.

*Genomic infrastructure:* Developing genomic capabilities also will be essential to establishing the Institute. Meticulous biospecimen collection and storage will be the first step to extracting high-quality DNA necessary for sequencing and analysis. The Institute team will create a specimen repository and a CLIA lab for genomic profiling. In addition, the team will work closely with TGen’s bioinformatics resources and develop additional analytic capabilities to integrate genomic data with clinical and biological data.

*Clinical infrastructure:* Much of the overall success of the program will depend on developing a highly functional clinical infrastructure support system. This will involve maintaining a relationship with pharmaceutical companies for access to drugs and compounds, determining how to admit patients into trials, and understanding the logistics of conducting and funding the trials.

**Collaborators:** A venture as ambitious as this requires the expertise of a highly regarded children’s hospital, access to progressive biomedical research institutions, and the resources of expert physician scientists.

As one of the nation’s largest children’s hospitals with a burgeoning population of patients, Phoenix Children’s Hospital is ideally suited to spearhead the Institute of Molecular Medicine. The Hospital is laser-focused on pediatric care in a broad range of specialties – from cardiology and neurology to cancer and blood disorders. In fact, Phoenix Children’s Center for Cancer and Blood Disorders is among the busiest in the Southwest.

Phoenix Children’s also is part of a bioscience community that is growing in both size and stature. The University of Arizona College of Medicine – Phoenix provides vital access to academia, research, and drugs and compounds. Similarly, Translational Genomics Research Institute brings deep experience in genomics and bioinformatics, along with strong relationships with biopharmaceutical companies and expertise in clinical trial design and management.

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Finally, the Institute has recruited a team of world-renowned physician scientists who will push the bounds of genomic profiling. Dr. Timothy Triche, a pediatric pathologist and former director of the Center for Personalized Medicine at Children’s Hospital Los Angeles, along with Dr. Robert Arceci, a pediatric oncologist from Johns Hopkins University, will serve as the Institute’s co-directors. Triche’s expertise lies in DNA sequencing, while Arceci is known as an international authority in clinical pediatric oncology.

They will be joined by Dr. Daniel D. Von Hoff, a medical oncologist at TGen with unprecedented experience in oncology drug development in adults. Von Hoff will use his learnings in adult clinical trials to inform therapy development for children.

Leveraging the assets of partnering institutions and expert physician scientists will not only save time and money, but also will avoid duplicating costly scientific infrastructure.

Additionally, Phoenix Children’s will foster scientific and academic partnerships that will strengthen the medical and collegial environment that has been growing in Phoenix during the past decade. The Hospital brings to its collaborators a world-class pediatric facility and the ability to conduct research seldom done in adults and never done in children. This will elevate the Hospital as a model for innovative pediatric care.

**Funding:** The inspiration and founding giftfor the Institute of Molecular Medicine has been graciously led by Ronald A. Matricaria, whose career in health care spans 40 years. Matricaria is the former chairman/CEO of St. Jude Medical Inc., and held leadership positions at Eli Lilly and Company. He has served on numerous public and private company boards of directors. He is currently a member of LIFE Technology, Inc., where he serves as presiding director; Volcano Inc., where he serves as chairman; and Phoenix Children’s Hospital as a board member. He also is an adjunct lecturer at Northwestern University’s Farley Center for Entrepreneurship and Innovation.

Additional funding for the Institute will come primarily from philanthropic contributions and grants. The goal is to augment the clinical research infrastruture enhance metropolitan Phoenix’s position as a biomedical hub.

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