

Children's Hospital Los Angeles

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Center for Innovation at Children's Hospital Los Angeles

CHLA is engaging with internal and external partners to develop healthcare solutions that deliver lifelong benefits for conditions that originate in childhood.

Options for pediatric care are increasing, and that is excellent news for the health of our children. The global market for pediatric medicines is predicted to reach \$100.7 billion by 2019, up from \$80.7 billion in 2013. This significant increase can be attributed to the commercial viability of therapeutics addressing high unmet clinical needs, such as diseases designated under the Orphan Drug Act. However, the market is also affected by the growing incidence of chronic disease conditions and greater recognition of behavioral health conditions in children.

To guide industry leaders in navigating this burgeoning pediatric market, Children's Hospital Los Angeles (CHLA) recently established its own Center for Innovation (CFI). "Our Center's goal is to facilitate access to the unique assets and strengths of Children's Hospital Los Angeles, delivering value to our industry partners for the benefit of patients," said Jessica Rousset, director of the CFI (Fig. 1).

CHLA is the premier pediatric hospital on the West Coast, treating one of the largest and most diverse pediatric populations in North America. In addition to its affiliation with the Keck School of Medicine of the University of Southern California, CHLA houses The Saban Research Institute, which is one of the few freestanding research centers in the country devoted exclusively to children, where scientific inquiry is combined with clinical care. CHLA's goal is to deliver better models of diagnosis, prevention and treatment to improve the lives of children and the adults they will become.

A wealth of data

When working with the CFI, industry partners have access to an abundance of expertise in pediatric medicine as well as targeted patient information through CHLA's Center for Personalized Medicine (CPM). In early 2015, CHLA announced a \$50 million investment in its CPM to expand the use of genomic data to develop new biomarkers and innovative, targeted therapies. "In the near future, a newborn's genome will be sequenced at or before birth, allowing clinicians to plan a lifetime of personalized health care that focuses on preventing, rather than simply reacting to, illness," explained Alexander Judkins, executive director of the CPM and head of the hospital's Department of Pathology and

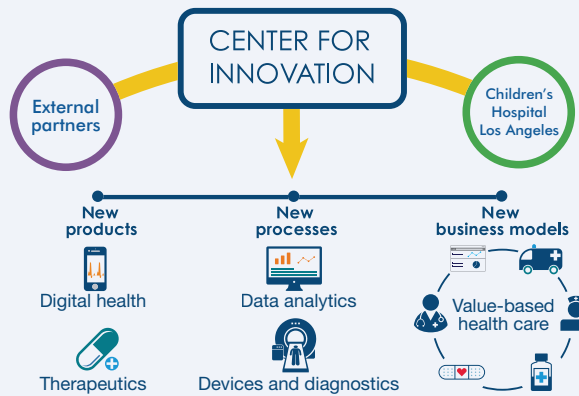


Figure 1: Children's Hospital Los Angeles innovation model.

Laboratory Medicine. The CPM team includes physicians, scientists, genetic counselors and staff who are recognized for their expertise in genomics, clinical genetics, bioinformatics and molecular diagnostics.

For industry partners, the CPM offers a wealth of data, with particular focus on pediatric oncology, inherited disease and infectious disease. "Comprehensive genomic profiling will reveal patient populations uniquely responsive to certain drugs," said Rousset. "We see great opportunity and potential benefit for our industry partners and patients from the repurposing of drugs and potential expansion of product labels, enabled through genomics."

This opportunity may expand with the introduction of new bipartisan legislation, the Orphan Product Extensions Now Accelerating Cures and Treatments Act, modeled on the incentive programs in the Best Pharmaceuticals for Children Act. Passage of this act would provide an additional six months of exclusivity to the patent life of a major market drug being repurposed for the treatment of a rare disease.

Collaborative consortia

CHLA has a long history of collaboration, engaging a vast network of experts in pediatric medicine. The New Approaches to Neuroblastoma Therapy (NANT) consortium links laboratory and clinical investigators to develop new cancer therapies in collaboration with 15 North American neuroblastoma centers.

To tackle childhood leukemia, CHLA established the Therapeutic Advances in Childhood Leukemia (TACL) consortium for carrying out early studies of new drugs. The TACL consortium is an international group of 33 institutions

working together to rapidly develop and carry out phase 1 and 2 clinical trials of new therapeutic approaches to treat recurrent childhood leukemia.

Through the CFI, CHLA oversees the Consortium for Technology and Innovation in Pediatrics (CTIP). This initiative, started with a \$1.5 million grant from the US Food and Drug Administration's Office of Orphan Products Development, aims to commercialize pediatric devices. "Through this grant, we provide expert consultation to pediatric innovators seeking to commercialize promising devices and diagnostics that address pediatric needs," explained Rousset. "Our job is to help articulate the

product's value proposition, health economic benefit and potential to generate revenue while minimizing the development risks."

Branching out

In addition to promoting innovation at the hospital, the CFI supports a variety of early-stage companies, such as the clinical-stage oncology company CerRx, which was founded in 2008 to commercialize cancer therapies that target the body's mechanism for making cell membranes.

Other CHLA-related early-stage companies include NanoValent Pharmaceuticals, a preclinical-stage oncology company developing a targeted nanoparticle drug delivery system; Avexegen, a preclinical-stage company developing neuregulin-4 to treat inflammatory bowel disease and the neonatal orphan condition necrotizing enterocolitis; and Oak Therapeutics, a preclinical-stage company developing an oral, dissolvable drug-delivery strip with taste-masking properties for improved compliance.

CHLA is a leader in pediatric innovation, and the CFI will catalyze new models of engagement and value creation for our stakeholders. With new government initiatives in place to help drive the development of pediatric therapies and technologies, the future is bright for the generations to come.

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