

Kymriah Treatment Available at Children's National Health System

New therapy shows 81 percent success rate at achieving remission in childhood leukemia

WASHINGTON— (April 16, 2019) – Children's National Health System announced today that <u>Kymriah</u>, the first chimeric antigen receptor T cell (CAR-T) therapy approved by the U.S. Food and Drug Administration (FDA), is now available to patients at Children's National.

The decision to approve the Kymriah treatment followed a unanimous recommendation for approval by the FDA's oncologic drugs advisory committee (ODAC) that included <u>Catherine Bollard, M.D., MB.Ch.B,</u> director of the Center for Cancer and Immunology Research at the Children's Research Institute and director of the Program for Cell Enhancement and Technologies for Immunotherapy.

"I'm really excited because it's the first gene therapy approved by the FDA and the fact that it was approved for a pediatric indication is fantastic," says Bollard. "It's an honor that Children's National was chosen as one of the hospitals to deliver this treatment."

Kymriah, also known as tisagenlecleucel, is comprised of genetically modified T cells that target CD19, expressed by a type of cancer called B-cell acute lymphoblastic leukemia (ALL). In clinical trials, Kymriah has shown unprecedented effectiveness in treating children and young adults with relapsed/refractory ALL. Out of the 75 patients evaluable for response, 61 (81 percent) achieved remission.

Kymriah marks a new era of cancer treatment and the pediatric indication for it paves the way for the use of similar therapies with promise to treat other deadly cancers affecting children.

"CAR-T therapy represents a significant breakthrough in the treatment of leukemia that has not responded to traditional therapies," says <u>Jeffrey Dome, M.D., Ph.D.</u>, vice president, Center for Cancer and Blood Disorders. "We're excited to be able to offer the Kymriah treatment to patients at Children's National and add a new component to our arsenal to fight childhood cancer."

Families interested in Kymriah should contact Children's National, at <u>CART@childrensnational.org</u> or call 202-476-1735.

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Children's National Health System, based in Washington, D.C., has served the nation's children since 1870. Children's National is one of the nation's Top 5 pediatric hospitals and, for a second straight year, is ranked No. 1 in newborn care, as well as ranked in all specialties evaluated by U.S. News & World Report. It has been designated two times as a Magnet[®] hospital, a designation given to hospitals that demonstrate the highest standards of nursing and patient care delivery. This pediatric academic health



system offers expert care through a convenient, community-based primary care network and specialty outpatient centers in the D.C. Metropolitan area, including the Maryland suburbs and Northern Virginia. Home to the Children's Research Institute and the Sheikh Zayed Institute for Pediatric Surgical Innovation, Children's National is the seventh-highest NIH-funded pediatric institution in the nation. Children's National is recognized for its expertise and innovation in pediatric care and as a strong voice for children through advocacy at the local, regional and national levels. For more information, follow us on Facebook and Twitter.