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CONTACT: Ronni Berke, Executive Director
Band of Parents
ronni@bandofparents.org
(917) 716-3158

**INTERNATIONAL CLINICAL TRIAL PROVIDES TARGETED THERAPY TO
CHILDREN WITH NEUROBLASTOMA**

Investigational 3rd generation targeted drug to be studied as a treatment for pediatric patients with relapsed or refractory ALK-driven neuroblastoma

March 30, 2017 – NEW YORK, NY: Lorlatinib, an investigational drug candidate currently in late stage clinical development for the treatment of lung cancer may also prove effective for the treatment of the pediatric cancer neuroblastoma. Solving Kids' Cancer is leading an international effort of like-minded nonprofits to provide nearly \$400,000 of collaborative funding for an innovative clinical trial to bring a potentially life-saving therapy to kids.

The phase I trial will be co-led by Dr. Yael Mossé at Children's Hospital of Philadelphia (CHOP) and Dr. Araz Marachelian at Children's Hospital of Los Angeles (CHLA), and will be conducted in 14 hospitals in the United States and Canada through the New Approaches to Neuroblastoma Therapy (NANT) consortium, as well as two European sites including Royal Marsden in London and Institut Curie in Paris. The study focuses on a subset of neuroblastoma patients with mutations in the anaplastic lymphoma kinase (ALK) gene. Abnormal ALK genes are found in 15 percent of high-risk neuroblastoma patients, and portend a very poor prognosis.

The primary objective of this study is to identify a recommended Phase II dose and assess the anti-tumor activity as a result of administering this investigational drug candidate in pediatric patients with ALK-driven neuroblastoma. Lorlatinib is a next-generation ALK/ROS1 tyrosine kinase inhibitor that has been recently reported to show very compelling responses in lung cancer patients with ALK aberrations. The drug was specifically designed to inhibit tumor mutations that drive resistance. One important feature of this drug is that it penetrates the blood brain barrier and may be effective in patients with relapsed neuroblastoma in the central nervous system.

"Lorlatinib has shown dramatic results in the lab against ALK+ neuroblastoma tumors in mice," said Dr. Yael Mosse, the physician-scientist who discovered ALK mutations in neuroblastoma and led the preclinical investigations at the Children's Hospital of Philadelphia. "If this trial proves successful, we expect the drug to quickly become part of frontline therapy in children with ALK-driven neuroblastoma – which is a tremendous accomplishment in cancer research, overall."

The hope is that this trial will provide a foundation for future work, which could lead to approval by health authorities for this use. Only three drugs have ever been approved for pediatric cancers, and this would set a remarkable precedent for rapid introduction of a new drug.

"This clinical trial could be revolutionary, and we are thrilled to know that it will be expanded to continental Europe so we can reach more families," said Jill Ostrager-Cohen, Band of Parents President. "Through collaboration, we're committed to helping neuroblastoma families wherever they are."

"We are excited to see such an innovative clinical trial come to fruition, and we are grateful to our partners who helped fund the gap so that we can continue in our joint mission to improve the overall survival rates for children diagnosed with neuroblastoma," added Scott Kennedy, Executive Director, Solving Kids' Cancer US. "This study represents an unprecedented collaboration between academic researchers, industry and charities to rapidly advance an exciting new agent, and provide access to the children who need it."

Childhood cancer is the leading cause of death by disease in the United States, and neuroblastoma is a pediatric cancer that strikes mostly infants and young children. The aggressive cancer originates in the sympathetic nervous system and manifests as tumors in the neck, abdomen and pelvis.

About Band of Parents:

The Band of Parents is a member-based organization with the goal of funding new therapies for neuroblastoma, a rare and deadly cancer that only strikes children. Our mission is to seek projects that have already been examined in the lab and are in need of assistance to rapidly move into a patient population. To date, the Band has granted more than \$6 million to pediatric cancer research. Learn more at www.bandofparents.org.

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COLLABORATIVE PARTNERS SUPPORTING THIS STUDY:

Solving Kids' Cancer US/EUR

The Band of Parents

The Ronan Thompson Foundation

The Children's Neuroblastoma Cancer Foundation

Wade's Army

The Catherine Elizabeth Blair Memorial Foundation

The Evan's Victory Against Neuroblastoma Foundation

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