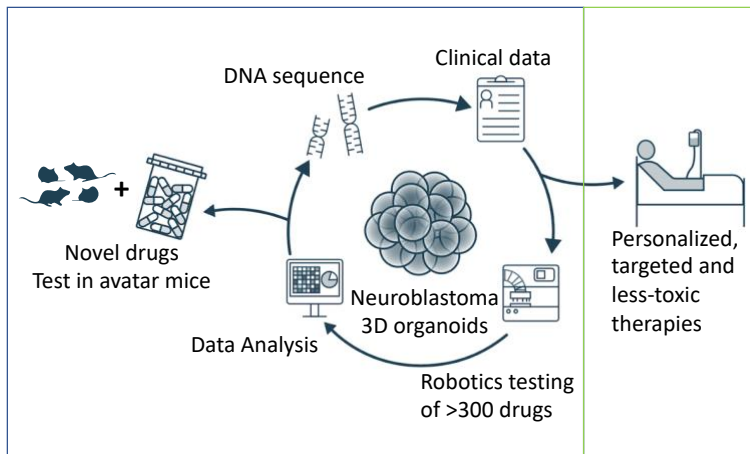


## Advancing Targeted Drugs for Children with Neuroblastoma, March 3, 2019

Dear Supporters of Cure First Neuroblastoma Project,



During the past 3 months we have compiled results, created graphs and continued the interpretation of our research to personalize and advance novel cancer treatments for neuroblastoma, together with our collaborator Dr. John Maris at Children's Hospital of Philadelphia (CHOP). The initial testing of over 300 drugs in nine neuroblastoma samples

pinpointed several drugs that were effective across all samples as well as drugs that had very personalized responses. The drug sensitivity information for each sample was then integrated with the genetic alterations of each cancer to enable the translation of genetic information to potential drug treatments for each of the nine neuroblastoma cases. This analysis revealed that certain drugs already in use for adults could be re-purposed for use in children with neuroblastoma. It also identified novel experimental drugs for further research.



### **Progress to confirm drugs in avatar mice and what's next.**

During the past year, Dr. John Maris's laboratory at CHOP has carried out an *in vivo* mouse study for one of the top drug candidates, a drug already approved for ovarian and breast cancer. The results indicated that Niraparib, an oral pill, slowed neuroblastoma tumor growth when tested in avatar mice, but it did not completely eliminate the cancer. For this reason, we proceeded to carry out additional drug testing (>140 oncology targeted drugs), to identify drugs to be used in combination with Niraparib. I am happy to report that we identified a novel drug combination and am now carrying out experiments to confirm this result. We are also continuing the analysis of all nine cases and will strategize on next steps with a face to face meeting with Dr. Maris and his team in Philadelphia on April 4<sup>th</sup>.

**How your donations were crucial.** The funding from Global Giving enabled the purchase of the drug to carry out the avatar mice experiments and also covered the cost of additional drug testing to identify novel combinations. These experiments will be essential to identify drug combinations that promise to induce complete and durable response in avatar mice, a critical step to advance this novel approach for children with neuroblastoma.

Thank you for your priceless support of our research aiming at improving quality of life and survival for children with high risk neuroblastoma.

*Rachele Rosati, lead scientist at Cure First*