



TO: Sandra J. Horning, MD, Senior Vice President, Global Head, Clinical Development, Hematology/Oncology, Roche-Genentech, Inc.  
1 DNA Way  
South San Francisco, CA 94080-4990

FROM: The GIST Community

**REGARDING: Urgent need for R1507 trial for pediatric and wildtype GIST**

Dear Dr. Horning,

We represent the worldwide GIST community: patients with gastrointestinal stromal tumor and their friends and families from around the globe.

We have been waiting for about a year for a clinical trial of R1507 to begin, both at the National Institutes of Health Pediatric GIST clinic and at a number of other sites sponsored by SARC. We were recently very disappointed to learn that this trial may not take place. The unintended consequence of this decision is that it inevitably introduces a delay before a very promising useful treatment can be offered to young people who currently have few options and are in desperate need.

As shown in a recent poster at CTOS, fewer than 20% of the patients who have participated in the NIH Clinics for Pediatric & Wildtype GIST have derived any benefit from tyrosine kinase inhibitors. The IGF pathway is the most promising drug target in prospect to arrest these patients' tumors.

Indeed, anecdotal experience from one young patient has shown that inhibiting the IGF pathway has had a remarkable and continuing response.

We have been informed by SARC that there have been alternative suggestions made which would allow the planned SARC trial of R1507 for Pediatric & Wildtype GIST to proceed. We hope you can find a way to proceed with this trial as soon as possible. Current patients need new therapies to be in development now if they are to benefit from them, and almost certainly as a spin off there will be benefits to other patients suffering from mainstream GIST or other diseases, malignant or otherwise.

Therefore we, the undersigned representatives of many of the GIST patient communities across the world, would request that you pursue all options to allow R1507 to be used in the SARC trial for Pediatric & Wildtype GIST as these patients are in urgent need of treatment options which currently they lack.

**Thank you for saving the lives of our children,**

Michael Josephy, Life Raft Group Science Team, Universidad de Costa Rica, Costa Rica  
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cc William Burns, CEO, Roche