Who Is PTC And What Is RG7916

Some of the information below was copied verbatim from www.ptcbio.com/en

PTC Therapeutics is headquartered in South Plainfield New Jersey. The mission at PTC Therapeutics has always been to build an integrated biopharmaceutical company based on their expertise in RNA biology. They are focused on discovering and developing novel oral treatments for patients with serious and life-limiting disorders. By targeting the processes that modulate RNA biology and affect protein production, PTC brings an innovative approach to drug discovery. They are focused particularly on the development and commercialization of treatments for rare and neglected disorders, with a commitment to finding treatment options for patients living with life-threatening diseases.

PTC currently have ongoing collaborations with Roche and the SMA Foundation, for the development and commercialization of compounds for the treatment of spinal muscular atrophy. They believe RG7916 may have the potential to target the underlying cause of the disorder, by increasing SMN protein levels in the nervous system, muscles, and other tissues, by modifying the splicing of the SMN2 gene to generate more full-length SMN mRNA in SMA patients. RG7916 is an investigational oral therapeutic which is currently in two clinical studies.

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The SUNFISH clinical study is a Phase 2 study in adult and pediatric patients with Type II and Type III SMA. It is a placebo-controlled, randomized, ascending dose study and will enroll approximately 36 patients for a minimum of 12 weeks to investigate the safety and tolerability of RG7916, and determine the dose for the second part of the study. The second part of SUNFISH will be a double-blinded, placebo-controlled, randomized, confirmatory study in approximately 150 Type II and Type III SMA patients for up to 24 months, followed by an open-label extension. The primary objective of the pivotal second part of this study is to evaluate the efficacy of RG7916 compared to placebo.

The FIREFISH clinical study is a study in infant patients with Type I SMA. It is an open-label, ascending dose study and will enroll approximately 8 patients for a minimum of four weeks to assess the safety profile of RG7916 in infants, and determine the dose for the second part of the study. The second part of FIREFISH will be to be an open-label, single-arm study in approximately 40 infants with Type I SMA for 24 months, followed by an open-label extension. The primary objective of the second part of this study will be to assess the efficacy of RG7916 at the selected dose after 12 months of treatment.

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SUNFISH Preliminary results from an early analysis from the ongoing Part 1 of the RG7916 study, demonstrated a dose-dependent increase in SMN2 full length mRNA ratio of ~ 400% versus baseline, as measured in whole blood. These results provided proof of mechanism for oral small molecule SMN2 splicing modifiers. No drug-related adverse events leading to withdrawal have been observed to date for RG7916. These data were presented at the Cure SMA Meeting in July 2017.

In the FIREFISH clinical trial, early interim data from Part 1, the dose-finding portion of the study, show RG7916 is safe and well tolerated at all doses and there were no drug-related safety findings leading to withdrawal. In addition, data on the ability to swallow and requirements for tracheostomy or permanent ventilation, together with overall survival were also presented. Previously published natural history data indicate that in a comparable historic cohort the median age of event-free survival for SMA Type 1 infants to be between 8 and 10.5 months. These data were presented at the International Scientific Congress on spinal muscular atrophy in January 2018.

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The SMA program, was initially developed by PTC Therapeutics in partnership with the SMA Foundation, (SMAF) to utilize PTC's Alternative Splicing technology platform to identify, and develop new small-molecule therapeutics for use in the treatment or prevention of SMA. In November 2011, Roche gained an exclusive worldwide license to the PTC/ SMAF SMA program. Clinical development of RG7916 is being led by Roche and overseen by a joint steering committee with members from Roche, PTC and the SMA Foundation.

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