FOUNDATION FOR
ANGELMAN SYNDROME
THERAPEUTICS

t: (630) 852-FAST
f: (630) 852-3270

Dear CIRM committee,

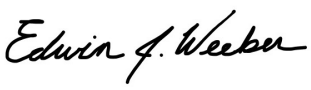
I am writing to express my enthusiastic support for Dr. Roger Hollis and his pioneering work in the field of gene therapy (GT) for Angelman Syndrome (AS). I make this statement from the perspective of a scientist who has worked and published in the field of AS for the past 25 years. My laboratory was the first to publish on the use of gene therapy to rescue the phenotypes in an AS mouse model in 2011 and I continued to work on an AS-GT for 5 years at PTC Therapeutics. Despite the potential for a traditional direct injection of an AAV-GT there are undeniable challenges in obtaining a CNS-wide distribution of AAV particles while being mindful of a resulting neuronal over-expression of protein that can be equally detrimental to the patient.

Dr. Hollis has solved this dilemma with an incredibly innovative ex-vivo hematopoietic gene therapy approach. It cannot be overstated that a hematopoietic-focused gene therapy represents a groundbreaking change in the treatment landscape of rare disorders in general and Angelman Syndrome specifically. This cutting-edge approach involves the use of the patient's hematopoietic stem cells essentially bypassing concerns of off-target AAV effects, a high AAV titer-dependent immune response, and the need for a highly invasive CNS injection into a fragile patient population.

I believe Dr. Hollis is a visionary researcher who possesses a deep understanding of both the scientific difficulties associated with therapeutic AAV design and the human implications of treating such a complex and highly heterogeneous disorder like AS. With support, this approach has the potential to make real and profound changes in the lives of countless individuals and families affected by this devastating condition with no currently available treatment.

Please do not hesitate to reach out if you require any further information or clarification of my support for Dr. Hollis or his research.

Best regards,



Edwin J. Weeber, Ph.D.
Head of Research and Discovery
Foundation for Angelman Syndrome Therapeutics
Ph: (615)-473-2138