January 25, 2025

Re: CLIN2-17081

Dear CIRM,

I am writing in support of application CLIN2-17081: REGEN4HD. My husband has Huntington's disease (HD), and our 3 children are at risk. Two of them have already tested positive for the HD gene, so time is of the essence in finding a treatment or cure.

My husband was very intelligent - he worked as an aerospace engineer. He was a caring husband and father. I loved discussing any topic with him, as he had such broad knowledge. However, since he was diagnosed with HD, I have had to basically just watch as the disease has gradually taken its toll. He had to retire at the height of his career, his mental changes have affected his relationships, he can no longer drive, he cannot walk without assistance, and he needs full-time care. He can no longer carry on a real conversation. He is losing the ability to communicate at all and it is devastating. I worked as an attorney and as a coach prior to his diagnosis but now I am his caregiver.

I started researching clinical trials as soon as my husband was diagnosed. We have participated in some, and have experienced significant disappointment as no treatment has yet been discovered. I am hopeful that the Uniqure study and other research will prove helpful, but I have followed the progress of this HD cell-based therapy for years and have been involved in the Community Engagement Studio, which resulted in my being very optimistic and excited for the study to move forward. I have also read the research associated with the study, so I was distraught to find that the review seemed so flawed.

My daughter also participated in the Community Engagement Studio for the study. She is asymptomatic, but tested positive a few years ago. She is so excited about this study because of the ground-breaking nature of the research, and because of the need for different types of treatment. None of the other studies we are aware of are even attempting to rebuild the striatum or remedy to any extent what has already deteriorated. It is of course too early to say that will work in this case, but it is an end goal and something no one else is even close to bringing to the clinic, so it seems particularly in line with CIRM's mission to fund research that is regenerative!

So much effort and CIRM support has gone into getting the study to this point – the study medication has FDA IND status, and is ready to go forward. Please do not stop or delay the study at this point. Every year lost is a blow to families with HD.

Please reconsider the decision for CLIN2-17081 and help move this critical research forward.

Thank you!

Melody Bandley