

May 2019

Dear Association for Creatine Deficiencies,

My first Lumos Update Letter to your budding organization, dated April 2015, was the beginning of our walk together on the CTD clinical road with the announcement of our Vigilant Study (Observational Study of Creatine Transporter Deficiency in Males) and an update on our LUM-001 (cyclocreatine) program. And now with this letter, although filled with much sadness but also filled with hope for your community, I am letting you know that we will no longer be able to continue our walk together on the clinical development road of finding a therapeutic for Creatine Transporter Deficiency.

In the past few weeks, after convening meetings with our Lumos internal team and external research experts to review the most recent data from our LUM-001 program, we have unfortunately concluded that there is not a viable clinical path forward for Lumos to develop cyclocreatine for CTD. Hence, we are in the process of discontinuing our CTD program from our company's pipeline.

All the employees and consultants of Lumos are crushed that our work will not lead to an ultimate therapeutic for this incredibly important unmet-medical need but we find hope and satisfaction that the body of scientific information that has been created, and would not have been possible without the selfless participation of the CTD families involved, will be of benefit and good to the CCDS community. At the end of this letter, I have listed some of the important publications and communications that have resulted from this collaboration.

Working with our esteemed multi-disciplinary clinicians and their teams, we have learned so much from the Vigilant Study and are diligently working to find another sponsor who can take over the sponsor role and continue the advancement of Vigilant. We have spoken with the Vigilant sites to let them know of our winding down efforts and they are supportive of continuing their work with a potential new partner. It is our intention to give you an update soon with the future status of the Vigilant Study.

In keeping with our Lumos mission, we will do our best efforts to publish the data that we have recently reviewed by working closely with the current researchers and clinicians.

I am incredibly proud and humbled to have witnessed, through the course of these four years since my first Lumos Pharma Update letter, the phenomenal growth and progress the ACD has demonstrated. From one of the ACD's first clinician interaction experiences at the 2015 Child Neurology Society conference (Columbus, OH) to the hugely impressive 2018 ACD Scientific and Family Symposium this past summer in Austin, my Lumos Pharma team and I are confident that more and more researchers and industry partners will be interacting with your fine organization to develop potential therapeutics for your children and loved ones.

Always in hope and admiration,

A handwritten signature in blue ink, appearing to read "Richard Hawkins".

Richard Hawkins
CEO, Lumos Pharma



CTD families contributing to the Vigilant Study have made this list possible!
Researchers have been able to share their works with their community
and spawn further scientific discussion and ideas.
Lumos Pharma thanks YOU.

Year	Title	Authors	Venue
9/2016	Red Flags for Creatine Transporter Deficiency, and Potential Outcome Variables for the Severely Impaired	Judith Miller; K Mulray, D Fichter, K Cunningham, R Anderson, Rebecca P Thomas, Robert J Davis, A Bruchey, Audrey Thurm	Society For Developmental Behavioral Pediatricians (SDBP)
3/2017	Creatine Transporter Deficiency / X-Linked Creatine Transporter Deficiency	Simona Bianconi	Rare Neurological Disease Special Report - Neurology Reviews
3/2017	Early Warning Signs And Clinical Endpoints For Creatine Transporter Deficiency	Judith Miller, Rebecca P Thomas, K Mulray, D Fichter, K Cunningham, R Anderson, Can Ficicioglu, Robert J Davis, Aleksandra Bruchey, Audrey Thurm	American College Of Medical Genetics (ACMG)
4/2017	Creatine Transporter Deficiency And Potential Outcome Variables For The Severely Impaired	Judith Miller, Rebecca P Thomas, K Mulray, D Fichter, K Cunningham, R Anderson, Can Ficicioglu, Robert J Davis, Aleksandra Bruchey, M Calkins	Gatlinburg Conference On Research And Theory In Intellectual And Developmental Disabilities
5/2017	Creatine Transporter Deficiency: A Rare Disorder With ASD Symptomatology	Judith Miller, Rebecca P. Thomas, Aleksandra Bruchey, Robert. J. Davis Audrey Thurm	International Meeting For Autism Research (IMFAR)
7/2017	Characterization Of Slc6a8 Mutations In Creatine Transporter Deficiency	Courtney Alexander, Aleksandra Bruchey	Southeastern Regional Genetics Group (SERGG)
4/2018	The Creatine Transporter Deficiency Developmental History Questionnaire	Rebecca P Thomas, Amanda Bennett-Palladino, Can Ficicioglu, Whitney Guthrie, Robert J Davis, Aleksandra Bruchey, Simona Bianconi, Audrey Thurm, Judith S Miller	Gatlinburg Conference On Research And Theory In Intellectual And Developmental Disabilities

Year	Title	Authors	Venue
4/2018	Prolonged QTc Interval In Creatine Transport Deficiency	Simona Bianconi, An Dang Do, Mark Levin, John Perreault, Dylan Hammond, Forbes D Porter	American College of Medical Genetics (ACMG)
6/2018	Phosphorous And Proton MR Spectroscopy Study Of Creatine Transporter Deficiency	Shizhe Steve Li , Simona Bianconi , Jan Willem van der Veen , JoEllyn Stolinski , An Dang Do , Kim Cecil, Forbes Porter , and Jun Shen	The International Society For Magnetic Resonance In Medicine (ISMRM)
5/2018	Significant Increase In Power In Clinical Trials With Use Of The Newly Available Vineland-3 Growth Scale Values	Cristian Farmer, Audrey Thurm, Judith S. Miller, and the Vigilant CTD Observational Study Team	International Meeting For Autism Research (IMFAR)
5/2018	Measurement Of Challenging Behaviors And Child Engagement In Youth With Creatine Transporter Deficiency	Manisha Udhani, Judith S. Miller, Audrey Thurm, and the Vigilant CTD Observational Study Team	International Meeting For Autism Research (IMFAR)
3/2019	Early Indicators Of Creatine Transporter Deficiency	Judith S. Miller, Rebecca P. Thomas, Amanda Bennett, Simona Bianconi, Aleksandra Bruchey, Robert J. Davis, Can Ficicioglu, Whitney Guthrie, Forbes D. Porter, MD, and Audrey Thurm	Journal of Pediatrics
5/2019	Potential For Significant Increase In Power Of Clinical Trials With Use Of The Newly Available Vineland-3 Growth Scale Values	Cristian Farmer, Audrey Thurm, Judith S. Miller, and the Vigilant CTD Observational Study Team	International Meeting For Autism Research (IMFAR)

And to the Vigilant CTD Observational Study Team, under the leadership of Dr Judith Miller, Dr F. Porter and the Vigilant Joint Steering Committee, your support and contributions have made and will continue to make a huge impact in understanding the complex disorder of Creatine Transporter Deficiency.

Lumos Pharma thanks all of YOU, the hardworking teams at:

Childrens Hospital of Philadelphia, National Institutes of Health- NICHD, Cincinnati Children's Hospital Medical Center, Duke University, University of Utah, Texas Children's Hospital, University of Toronto Hospital for Sick Children, University of California San Diego, Rush University Medical Center, Boston Children's Hospital