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**FOR IMMEDIATE RELEASE**

**Association for Creatine Deficiencies (ACD) Awards Dr. Laura Baroncelli, Ph.D., the First Gene Therapy Advancement Award (GTA)**

Carlsbad, CA (July 28, 2020)—The [Association for Creatine Deficiencies](#) (ACD) has awarded Dr. Laura Baroncelli, Ph.D., the first Gene Therapy Advancement Award (GTA) of \$10,000.

The ACD was established in 2012, by parents with children diagnosed with a Cerebral Creatine Deficiency Syndrome (CCDS). Our mission is to drive and facilitate research for treatments and cures for creatine deficiencies as well as to provide patient, family, and public education, and to advocate for early intervention through newborn screening. To support research, the ACD can help with research grants, biosamples, survey data, and other collaboration opportunities. We are a proud member and grantee of the [Rare as One](#) network supported by the Chan Zuckerberg Initiative.

In the winter of 2019, our parent community raised an initial fund of \$50,000 to be used towards gene therapy research efforts. With this support in mind, the ACD established the [CCDS Gene Therapy Consortium](#) in 2020 to foster concentrated efforts into gene therapy for creatine deficiencies. The mission of the consortium is to facilitate the timely sharing of information and development tools among labs that are pursuing gene therapies for creatine deficiencies. We believe that by building a collaborative environment and supporting shareable tools through grants we can shorten the timeline and effort required to find gene therapy solutions for creatine deficiencies. The consortium meets on a quarterly basis as a group to discuss the latest research and provide peer expertise.

In June, ACD awarded the first GTA grant to Baroncelli to facilitate capacity building efforts for her lab, specifically focused on the incorporation of intrathecal viral delivery techniques in mice. Intrathecal administration is a route of administration for drugs via an injection into the spinal canal so that it reaches the cerebrospinal fluid (CSF) and is useful for drugs that need to reach the brain. With this award, Baroncelli's lab will be able to test new viral vectors for expression and subsequent correction of defects in the brain. It's worth noting that learning this technique will require skills from another consortium member, Dr. Steven Grey, who generously volunteered to share his expertise.

Baroncelli graduated in Biology from the University of Pisa in 2005 and received her doctorate degree in Neurobiology from the Scuola Normale Superiore in 2009. Following a fellowship at Scuola Normale Superiore, in 2010, she was awarded a two-year post-doctoral fellowship at the Accademia Nazionale dei Lincei, in Italy. Since 2011, she has been a tenured Researcher at the Neuroscience Institute (IN) of CNR in Pisa. She has published over 38 original research papers in international peer-reviewed journals (H index: 22). She was awarded funding by Fondazione Roma, LUMOS Pharma, Italian Ministry of Health, Lejeune



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Foundation, and Telethon for the study of creatine-related disorders. She is also an Academic Editor of Neural Plasticity and Scientific Report, and reviewer for various international journals and national agencies.

**“Our goal at the ACD is to promote collaborative open science to advance research that gets us closer to gene therapy for creatine deficiencies. The GTA grant is awarded in support of Dr. Baroncelli’s work towards advancement of CCDS gene therapy while also recognizing her efforts in sharing these insights with colleague”. Commented ACD Director of Research, Laura Trutoiu.**

To learn more about creatine deficiencies please see our [educational video](#) or visit <http://www.creatineinfo.org>.