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CURE MECP2 DUPLICATION SYNDROME REAFFIRMS ITS MISSION AND LAUNCHES WEBSITE

CURE MECP2 DUPLICATION SYNDROME (CURE MDS) is excited to announce that it has redoubled its commitment to its mission and has officially launched a new website at www.curemds.org. This is the result of the collaborate efforts of many committed families that have a loved one who struggles with MECP2 Duplication Syndrome (MDS), a severe neurological condition resulting from the erroneous duplication of the MECP2 gene. The mission of CURE MDS is to bring together affected families from around the world to raise and contribute the funds needed to support curative strategic research efforts for MDS.

The history behind the founding of CURE MDS is important as it relates to the promise and urgency of the research on this disorder. In 2008 the Rett Syndrome Research Trust (RSRT) launched with a laser focus on curing Rett Syndrome. Rett Syndrome and MDS are interrelated because both disorders are linked to a gene called MECP2. Rett Syndrome results from random mutations or deletions in that gene, whereas MDS symptoms arise when the same gene is erroneously duplicated. The symptoms of both disorders have some overlap, and the target culprit gene that researchers need to focus on is the same.

In 2010, RSRT agreed to a request made by parents of MDS children to also drive research efforts for a cure for MDS, leveraging RSRT's infrastructure, deep knowledge base and global scientific networks. Following its rigorous scientific peer review process, RSRT awarded funding to its first MDS-focused project: Is MECP2 Duplication Syndrome Reversible? from the lab of Dr Huda Zoghbi, Baylor College of Medicine, Houston Texas.

Remarkably, not only did Dr. Zoghbi show that indeed MDS is reversible but she offered up a strategy to accomplish this in the clinic, Antisense Oligonucleotide Therapy. The results were published in "Nature" November 2015.

During the ensuing decade the close collaboration between RSRT and Cure MDS (formerly 401 Project) has resulted in the funding of seven research projects, numerous scientific breakthroughs, pre-clinical trials, and most importantly, three potentially curative therapeutic approaches.

In response to the excitement and sense of urgency, CURE MDS has further developed its structure, retaining foundational members Peter Anderson, Collene Wright, Jenny Howe, Kim Spangler and Beatrice Palma and adding new appointments, Oliver Freeman, Fiona Walton, Amy Baker, Daleth Scaramuzzi, and Susan Centrone. Further opportunities for participation are welcome especially with the development of a parent advisory committee.

While the CURE MDS team is focused exclusively on raising funds to support research, the entire RSRT team works on behalf of both MDS and Rett. The RSRT research team encompasses Monica Coenraads, Randy Carpenter, MD, and Jana von Hehn, PhD, who collectively have decades of disease advocacy, drug development and clinical trial experience. Tim Freeman and Ricki Davis are the fundraising and adminis

Importantly, RSRT's engagement comes at no cost to CURE MDS. 100% of every dollar contributed is invested in research – not a single penny goes to overhead. Furthermore, RSRT is the gold standard with its rigor, governance, transparency, accountability and responsible donation handling. Donors and fundraisers can have complete confidence that their investment is in responsible hands.

The leadership of Cure MDS said in a joint statement, “A cure for MDS is our mission. Engaging and working with the global leaders and pioneers in potential curative approaches is essential to achieve our goal. The closer we get to a cure the more important it is to sharpen our focus, increase our efforts, push harder and doggedly challenge the status quo to achieve the outcome we desire. We are incredibly thankful for RSRT's ongoing and invaluable support, ensuring we couldn't be better placed to CURE MDS.”