Public Statement August 26, 2022

As advocates of the Vanishing White Matter (VWM) community, we are encouraged by the therapeutic developments regarding VWM and are delighted by the prospect of potentially multiple therapeutic trials.

VWM is an ultra-rare leukodystrophy with devastating neurological consequences. The disease is extremely heterogeneous in age of onset, ranging from before birth until adulthood, and in rate of progression, ranging from rapid decline to survival for decades.

We understand that both the ultra-rare nature of the disease and the heterogeneous presentation challenge clinical trial development. We understand it is in the best interest of patients that all promising therapies are tested regarding safety and efficacy and that trials are organized in such a way that they are adequately powered and executed. We are working closely with our membership and affiliate communities to educate our patients about fundamentals of trial design and participation in trials that will meaningfully move our community towards our shared goals of accessible therapies.

For this reason, we are working closely with the VWM Expert Consortium, led by Dr. Marjo van der Knaap, to optimize future trial design and execution for our patient community. As advocates, we will review closely any clinical trial design proposed for our community with the VWM Expert Consortium. We will share this work broadly within our patient community and we expect to refer our VWM community to the trials that follow the approach set out by the VWM expert consortium. We hope, in such a way, to continue to advocate for our patients and look forward to working in close partnership with industry stakeholders to support much anticipated clinical trials in VWM.

Sincerely,

VWM Families Foundation
United Leukodystrophy Foundation
European Leukodystrophy Association
VKS, the Dutch Foundation representing leukodystrophies