

2020 Forbes Norris ALS Center NEWS



December 2020

Dear Norris Center family,

The COVID pandemic of 2020 has brought home to all of us what it's like to be thrown back on our own resources, and how big a role luck plays in our health and wellbeing. In this way, we have had a small taste of the experience of ALS patients and their families, who know how cruel luck can be, and who often feel isolated by this disease with no cure.

Yet, as our patient profile of Sandy Morris explains, 2020 has also brought promising new developments in the design of clinical trials for ALS patients, bringing us hope that – with your support – integrated ALS centers like ours will one day soon have new effective treatments to offer to our patients.

I know I first announced my retirement in this newsletter in 2016. I'm still trying. I'm counting on you to sustain our extraordinary and essential ALS patient care program long into the future.

Warm regards from our family to yours,

Dee Holden Norris, R.N.

Sandy Morris, advocate for patient-centered clinical trial design

“This disease is uniquely cruel,” says Sandy Morris, “because every month, or week, or day, you lose more functionality, and with the decreasing mobility and ability to communicate, you can feel increasingly isolated.” Determined that “no other 51-year-old mother of three should go through what I did when I was diagnosed,” Morris has been working since her diagnosis with the organization **I AM ALS** to ensure that clinical trials for potential new ALS treatments are designed “with humans in mind, not zebra fish.”



Sandy Morris (center) with her husband (right) at the office of U.S. representative from Ohio Anthony Gonzalez.

“We’ve taken best practices from research in oncology and HIV and created the patient-centric trial design ratings (PaCTD) so that ALS patients can choose to participate only in trials that are designed with efficiency and humanity.” That includes having expanded-access and open-label extension programs alongside the trial, so that those who are no longer eligible for the trial, or those who received the placebo during the trial, have an opportunity to continue to receive a promising treatment once the trial is concluded. PaCTD also suggests limiting those getting the placebo to 33% or less of clinical trial participants, as “50% placebo for invasive procedures such as bone marrow aspirations and spinal injections is not humane.”

Morris also wants to prioritize trial design that “works on the ALS clock.” Because ALS patients generally survive for two to five years after diagnosis, “we need to advance ALS science faster, and enroll clinical trial participants like wildfire.” Morris and I AM ALS (<https://iamals.org/>) support two bills before Congress, H.R. 7071, ACT for ALS, and S. 3872, Promising Pathway Act.

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It is not that difficult to design a trial that meets the FDA’s scientific parameters and still treats ALS patients humanely. ALS patients are very willing to try anything that might let us stay here with our families a little longer, but that ‘anything’ needs to be checked for safety and for signaling for efficacy, so that it’s not snake oil.”

Morris says the integrated care she’s receiving at the Forbes Norris Center is “amazing,” because everyone at the Center is willing to stay with patients for the whole course of the disease. “Anyone who chooses to work with this horrific disease when they don’t have to is some kind of special human.” Her goal for her clinical trial work matches the goal of the Center itself, “to leave this disease better than she found it.”

For information about clinical trials at CPMC and Sutter Health, see sutterhealth.org/cpmc/research/clinical-trials



Sandy Morris (center right) with her husband (top right) and her three children.

Our philanthropic partners supported many of the activities of the Forbes Norris MDA/ALS Research and Treatment Center.

- A **Phase 3 clinical trial** involved a large number of patients **to test the efficacy of Brainstorm’s NurOwn technique for harvesting stem cells** from the patient’s own bone marrow and engineering them to secrete specific growth factors. The BrainStorm clinical trial was the subject of a presentation at the 10th Annual California ALS Research Summit.
- The Forbes Norris Center also **participated in an early clinical trial of agents designed to block the effects of genetic mutations** for the 10 to 20% of patients whose ALS is hereditary.
- The Forbes Norris Center **participated in the Centaur AMX0035 trial** that demonstrated encouraging results in slowing the rate of function loss in treated patients compared with placebo. The trial combines two drugs used to treat other disorders. Drs. Katz and Jenkins were authors on **papers published in the *New England Journal of Medicine and Muscle & Nerve* documenting the trial results.**
- Ongoing research collaborations **developed a new, common understanding that ALS is not one illness**, but instead develops because of multiple changes in neurologic pathways.
- CPMC **hosted over 60 physicians, nurses, therapists and research coordinators** from UCSF, UC Davis, Sutter Health, Kaiser, and Stanford at the Northern California Neuromuscular biannual meeting.
- Liberty Jenkins, M.D., and co-author Martin Turner, Ph.D., **published *Fast Facts: Diagnosing Amyotrophic Lateral Sclerosis*** with Karger, a major global scientific publisher.
- Forbes Norris staff **supported patients and families** through phone consultations, home visits, education, resource recommendations, and an equipment loan program. New staff **provide expertise in palliative medicine.**
- Our Augmentative Communication Center **provided assistive technology** to help patients communicate.
- The Forbes Norris Center is **the only Northern California site for a platform clinical trial**, with three drugs (soon to be four) being tested simultaneously, potentially leading to accelerated drug development.

Please send your charitable gift before the end of the year, so that the Forbes Norris ALS Research Center can continue to improve the quality of life for ALS patients. Use the enclosed envelope, or visit cpmc.org/giving/als. To explore other types of gift options contact Amanda Martin, Director of Gift Planning at CPMC Foundation: MartinAK@sutterhealth.org, or 415-600-4406.