PACKARD CENTER
THE ROBERT PACKARD CENTER
FOR ALS RESEARCH AT JOHNS HOPKINS

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Mr. and Mrs. James Hall
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Dear Jim and Nancy,

On behalf of our scientists, clinicians, patients, families and staff, please accept my heartfelt thanks for your gift to the Robert Packard Center for ALS Research at Johns Hopkins given through Brian's Wish Fund. As a team, we are united by one vision: living in a world without ALS. You are a partner with us in that vision and we are deeply grateful.

Progress in ALS research - as in all academic medical research - is measured in very small increments. For example, the recent discovery of a new gene, TDP-43, mutated by "mis-folded" proteins that "clump up" (aggregate) in central nervous system cells, helps scientists understand that these aggregates are key to the early ALS disease process. The discovery of TDP-43 is important for two reasons: (1) the role of another gene with protein "clumping" suggests that flawed RNA metabolism is one of the biological pathways that goes wrong in ALS; and (2) TDP-43 is present in both familial and sporadic forms of ALS - the first such mutation to be found in both patient populations.

Basic science discoveries like the one described above, even if they seem mysterious or meaningless to the general population, are critical for Packard Center scientists to make because these findings provide clues from which to derive treatments for ALS. With this information, other scientists could develop drugs that work on "un-clumping" proteins, help to fix the flawed RNA metabolism, or keep the TDP-43 gene from mutating in the first place. This is crucial information: without knowing what the target is and how it works, we cannot develop drugs that can hit that target and correct it!

This is the kind of complex work Packard Center scientists are involved in, every single day. Each discovery yields more information about ALS and where to go next. It is imperative that we continue this journey so that, together, we will conquer ALS.

Best regards,

Liz McFarlane

Happy New Year!