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**Blazeman Foundation for ALS Research**
**Research Progress Report Summary 5/1/2016 – 4/30/2017**
**Brandeis University**

This year, the Blazeman Foundation for ALS Research supported work in our lab that led to the following achievements:

• We published an article entitled "Role of BMP receptor traffic in synaptic growth defects in an ALS model" in the October issue of the journal *Molecular Biology of the Cell*. In this article, we showed that in fruit fly models of ALS, the transport of growth factors in neurons was altered. We were able to partially restore neuronal function by diverting transport back to a healthy route, suggesting that cellular transport machinery might be an effective therapeutic target for ALS. The article was chosen for 'Highlights from MBoC', a selection of papers in the journal that are deemed important for the field. A Brandeis article describing the work was picked up and further disseminated by several ALS research sites.

• Blazeman postdoctoral fellow Dr. Mugdha Deshpande co-authored a review article in the Journal of Cell Biology, thanking the Blazeman Foundation for its support.

• We found that several neuronal transport defects in fruit fly models of ALS are related to defects in neuronal calcium channels. We are currently pursuing these studies in mammalian models of ALS.

• We developed a primary mammalian neuron model of TDP-43-linked ALS, in which we observe neuronal growth defects that parallel our fruit fly models.

• The important next question to address is how relevant these pathways are for progression of pathology in human patients, and whether they are perturbed similarly and result in equivalent cellular defects. A powerful cutting-edge technique to answer this question is reprogramming stem cells derived from ALS patients (harboring mutations in TDP-43) to generate neuronal cell lines. Thanks to our preliminary data and support from the Blazeman Foundation for ALS, Dr. Deshpande has obtained a grant from the Brandeis Provost's Innovation Fund to test our hypotheses in neurons differentiated from ALS patient cells.