

Update on the Wilson Disease Registry Study

We would like to take this opportunity to introduce Dr. Ayse Coskun, our new study lead coordinator at Yale, to the Wilson Disease Association membership. Ayse joined us in May 2019 and is working with each of our study sites to help initiate their active patient recruitment. We are pleased that recruitment has already started at Baylor College of Medicine in Houston, Texas where Dr. Sanjiv Harpavat has continued the work initiated by Dr. Tamir Miloh. Already three patients were recruited into the study at Baylor, and more will follow. Soon to begin enrollment this Summer are Florida Hospital, led by Dr. Regy Gonzalez, Seattle Childrens under the direction of Dr. Sihoun Hahn, and the Royal Surrey and Kings College Hospitals in the UK where Drs. Aftab Ala and Michelle Camarata are lead investigators.

Overall, 67 patients were enrolled in the registry, thirteen of which are pediatric patients. Recently a summary of the data from the registry was presented at the national meeting of the Wilson Disease Association in May 2019 in New York City and at an international meeting on Wilson Disease held in Aarhus, Denmark later that month. Other research derived from registry data is being presented at a major international psychiatric meeting by Dr. Paula Zimbren of Yale in July 2019 and new work was submitted for the upcoming national liver meetings in the US in the Fall. Work is continuing towards the development of new methodology for the analysis of copper in the circulation at the Royal Surrey Hospital under the direction of Dr. Chris Harrington, and other exciting work by Dr. Sihoun Hahn on ATP7B protein analysis and molecular sequencing of the Wilson disease gene at Seattle Children's Hospital is continuing.

We look forward to another productive year of patient enrollment and hope to welcome patients to our newly enrolling registry sites. We thank all our participating patients and families that have so generously donated their time and efforts to make this project a success.

Michael L. Schilsky MD FAASLD, Lead Investigator (Yale)

Ayse Coskun MD, Lead Coordinator (Yale)