

## **Summary of the 5<sup>th</sup> International Lafora Disease Workshop**

The 5<sup>th</sup> International Lafora Disease Workshop was held in Madrid, Spain September 9<sup>th</sup> through September 11<sup>th</sup>. The Workshop, which was first held in San Diego in 2014, presents an opportunity for clinicians, researchers, pharmaceutical companies and affected families to come together to discuss the progress of research toward a cure for Lafora. This year, in addition to researchers and clinicians from all over the world, families from Spain, Germany, the Ukraine, Slovakia, the Netherlands and the United States participated in the workshop. With so many Lafora families attending, researchers get to meet and connect with the people whose lives are so terribly affected by Lafora disease.

The first day of the workshop began with introductory remarks by Dr. Jose Serratosa, Chief of Neurology at Fundacion Jimenez Diaz, Madrid. Frank Harris, President of Chelsea's Hope spoke to the group about the need to move as quickly as possible to begin clinical trials. [You can read Frank's remarks here](#). Professor Matt Gentry of the University of Kentucky discussed the progression of research over the past year.

Paul Goldberg, Vice President of Ionis Pharmaceuticals discussed the progress the company is making to develop a potential treatment. Mr. Goldberg also described the drug development process and the progression of the Natural History study. Dustin Armstrong, the Chief Scientific Officer of Valerion Therapeutics also discussed the progress it is making to develop a potential treatment. The second day of the workshop was dedicated to researchers from all over the world discussing the status of their current research related to Lafora Disease.

### **Researchers Have Started the Natural History Study**

In order to receive approval from the European Medicines Agency (EMA) and the United States Food and Drug Administration (FDA) to begin clinical trials, Ionis and Valerion have collaborated with researchers around the world to develop a study that will record and evaluate the natural progression of Lafora Disease in a robust and structured manner. This Natural History Study will evaluate Lafora patients in four study

locations: Dr. Antonio Delgado-Escueta leads the study at the University of California in Los Angeles, California; Dr. Roberto Michelucci leads the study located at the Instituto delle Scienze Neurologiche in Bologna, Italy; Dr. Berge Minassian leads the study at the University of Texas, Southwestern in Dallas, Texas; and Dr. Jose Serratosa leads the study at the Fundacion Jimenez Diaz in Madrid, Spain.

While the Los Angeles location has not yet opened, the Dallas location expects to begin evaluating patients in September. The locations in Italy and Spain began enrolling and evaluating patients early in 2019.

### **Progress Toward a Drug**

We are thrilled to report that Ionis and Valerion each reported that they have made progress in developing a candidate for a human drug.

Ionis has identified and developed a candidate for a human drug and is beginning the phase to conduct an animal safety study. Valerion has identified a candidate for a human drug and is preparing it for safety evaluations.

Chelsea's Hope is both pleased with the progress being made and impatient for a cure. The scientific collaboration that began with the first International Lafora Workshop in 2014 has grown into a widespread effort to save lives. But Chelsea's Hope has more work to do. Many study participants face significant travel expenses and look for any help possible to be able to continue to participate. Additionally, Chelsea's Hope helps to facilitate the annual workshop, bringing clinicians and researchers together to find a cure. Next year's International Workshop will be held in San Diego, with a date yet to be identified. I encourage you to contact me with any questions or ideas about how to help us reach our goal.

With Hope,

Frank Harris  
President – Chelsea's Hope Lafora Children Research Fund  
fharrischelseashope@gmail.com