

# News from the Hereditary Disease Foundation

### **Excitement Is Building!**

We're looking forward to a wonderful night Celebrating Discoveries in Neuroscience at our Symposium and Gala on Monday, October 28, at the Harvard Club in New York. We hope you will join us! We're thrilled that Lesley Stahl, 60 Minutes, CBS News Correspondent, will host the evening and that Cori Bargmann of the Chan Zuckerberg Initiative will present the keynote. To top it off, Emmy award winning journalist Charles Sabine, and brilliant researcher and HDF Scientific Advisory Board Member Scott Zeitlin will be featured. It will be night of important science, special surprises and great fun. You don't want to miss it! For ticket info, call our offices at 212-928-2121, email cures@hdfoundation.org or visit the <u>Hereditary Disease Foundation</u> website.

## **Funding Great Science**

Supporting collaborative, game-changing research is what we do. We celebrate discoveries, and we celebrate the amazing scientists in laboratories around the world who are making these discoveries happen. Each year our dedicated Scientific Advisory Board, under the leadership of Anne Young and Leslie Thompson, reviews grant applications from around the world and selects the most innovative and promising research projects to fund. We're delighted to announce new grant and postdoctoral fellowship awards of almost \$1.5 million for 2019. Thanks to our wonderful donors who understand that philanthropy is the fuel that drives research discoveries!

Here's an overview of the recipients and their projects. You'll be hearing more about their research in the months ahead.

### **2019 Funded Grants and Postdoctoral Fellowships**

#### Osama Al Dalahmah, Columbia University Medical Center

The regional heterogeneity of Huntington's disease pathology: Clues from diverse astrocytic responses



# Lauren Byrne, University College London Mentor: Ed Wild

Influx-HD: Advancing biofluid biomarkers for disease-modifying trials in Huntington's disease



**Rivka Dikstein, The Weizmann Institute of Science, Israel** Unraveling the role of Spt4/Spt5 in inherited neurodegenerative diseases using newly discovered pharmacological tools



Steven Finkbeiner, Gladstone Institute, University of California San Francisco and Leslie Thompson, University of California, Irvine Assessment of WGS-derived genetic modifiers in differentiated HDderived iPSCs



## Brent Fitzwalter, Broad Institute of MIT and Harvard

Mentor: Myriam Heiman Cell type-specific mechanisms of Foxo3-dependent neuroprotection in Huntington's Disease



### Richard Hickman, Columbia University Medical Center

Generation of striatal neurons from HD patient-derived fibroblasts: a feasibility study with direct correlation to human neuropathology



# Ali Khoshnan, California Institute of Technology

Editing of gut microbiota to reduce brain pathology in Huntington's disease



**Katerina Oikonomou, University of California, Los Angeles** Mentor: Michael S. Levine Targeting Perturbed Calcium Signaling for the Treatment of Huntington's Disease Using Miniscopes and Ca2+ Sensors



#### Anna Pluciennik, Thomas Jefferson University

Crosstalk between DNA repair pathways in Huntington's disease



### Paul Ranum, The Children's Hospital of Philadelphia

Mentor: Beverly Davidson High throughput quantification of gene expression and mRNA structure from single-cells in the HD brain



#### **Charlene Smith-Geater, University of California, Irvine** Mentor: Leslie Thompson PIAS1 intersecting with DNA repair, synaptic biology, bioenergetics and protein homeostasis in HD 2D and 3D human neurons



Nicholas Todd, Brigham and Women's Hospital, Harvard University Targeted Delivery of Huntington's Disease Gene Therapeutics



#### Ray Truant, McMaster University

DNA Damage Repair Links to Energy Metabolism Defects in Huntington's disease: Defining New Therapeutic Targets



#### **Gong-Her Wu, Stanford University**

Mentor: Wah Chiu Deciphering mutated huntingtin aggregates and cellular architecture in Huntington disease neuron by cryogenic electron microscopy



#### X. William Yang, University of California, Los Angeles

Novel Fan1 Knock-in Mice to Study the Role of Fan1 in Normal Brain Function and Huntington's Disease Pathogenesis



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