



The National **Fragile X** Foundation



April 2009

The National Fragile X Foundation - Conquer Fragile X Working to Advance the Understanding of Fragile X Syndrome

The merger of Conquer Fragile X Foundation and the National Fragile X Foundation in late 2007 provided an array of opportunities to advance the Fragile X research field. Thanks to the generosity of Conquer Fragile X donors, and the relentless dedication and focus of Harris Hollin, a series of important events, each destined to move fragile X research forward, are taking place in S. Florida.

Palm Beach Fundraiser

On January 18, 2009 at the Colony Hotel in Palm Beach Florida, more than 60 people attended the annual Palm Beach cocktail party fundraiser. This event, organized by Conquer Fragile X founder Harris Hollin and sponsored by four of CFXF's most generous supporters, Zelda Mason, Beverly Robbins, Rosalee Davison, and Sheila Engelstein, raised over \$100,000 for the Harris Hollin Conquer Fragile X Fund at the National Fragile X Foundation. By agreement between CFXF and NFXF, funds raised by CFXF each year will fund basic research for a cure or treatment for Fragile X. The call for proposals will go out by the end of April.

Gary Bassell, PhD, began the evening by providing a highly understandable, non-scientific explanation about the cause of fragile X syndrome. Attendees also heard Harris, along with featured speaker and genetic counselor Brenda Finucane and National Fragile X Foundation VP Michael Kelley, speak about the latest research and the hope for new treatments. Brenda also described the long history of Harris's efforts to achieve breakthroughs in our understanding of Fragile X. His engaging grandson, Matthew Hollin, added to the special nature of the event with a big "hi" from the podium.

From Mechanisms to Medicine: Florida Fragile X Research Conference, April 19-21

Thanks to the extraordinary efforts and many hours of work by Harris Hollin and Karen Fay, along with a dedicated and active scientific organizing committee of world renowned researchers, this important event, co-sponsored by Scripps Research Institute of Florida and the National Fragile X Foundation, will soon take place in West Palm Beach. The unique conference will bring together veteran Fragile X researchers with young researchers to share ideas and discuss their research. The

idea of bringing veteran researchers together with junior scientists is meant to establish personal relationships that will attract bright young scientists to Fragile X research, for whom there is much competition among more well-funded disease research. This year's event will include veteran researchers Dr. David Nelson, from Baylor University in Texas, speaking on "20 Years in FX Research", Dr. Richard Paylor, also of Baylor, speaking on "Modifying behavioral responses in Fmr1 KO mice," Dr. Gary Bassell of Emory University, speaking on "FMRP-mediated translation and Synaptic Signaling," Dr. Elizabeth Berry Kravis of Rush University Medical Center in Chicago presenting on "Translating Basic Science to Treatment: A New Era for Fragile X Syndrome," Drs. Claes Wahlestedt from Scripps, Florida and Randy Carpenter of Seaside Pharmaceuticals talking about "Translating Research Discoveries into Novel Therapeutics," and Dr. Karen Usdin, of the National Institutes of Health addressing "Repeat expansion and transcription from the FMR1 locus."

As you can see from the titles, these are complex issues requiring years of commitment, mentoring of young researchers by senior researchers, a passion for science, and an awareness to the challenges faced by those with fragile X syndrome, including a sensitivity to the urgency felt by their parents. This conference is poised to provide those ingredients and more.

Conquer Fragile X Research Update

Conquer Fragile X has been supporting important research, studying the underlying cause of fragile X syndrome, for many years. At the time of the merger of Conquer Fragile X Foundation and the National Fragile X Foundation, the following critical projects were in mid-stream:

- Claes Wahlestedt MD PhD, Scripps Research Institute, Florida USA

Imaging of the Fragile X protein and role in mRNA translation and spine-synapse regulation in hippocampal neurons

- Sharon Eden PhD, Hebrew University of Jerusalem – ISRAEL

Regulation by Rac of the Fragile-X mental retardation protein activity

- Gary Bassell PhD, Emory University School of Medicine, Georgia USA

- Menahem Segal PhD, Weizmann Institute of Science – Rehovot ISRAEL

Imaging of the Fragile X protein and role in mRNA translation and spine-synapse regulation in hippocampal neurons

Scientific updates from the lead researchers on these projects have been received and reviewed and all four report unique findings that will add considerably to the fragile X syndrome body of knowledge and, more importantly, will advance the day when new therapeutic medications will become available. Thank you for helping make this possible.

Robert M. Miller, Executive Director