



FOUNDATION
Mucopolipidosis IV Foundation

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Dear Friends,

With the close of 2003 approaching, the ML4 Foundation would like to update you on the past year's accomplishments. We also want to thank you for your support and generosity that make it possible for the ML4 Foundation to continue its work.

The ML4 Foundation is thrilled to announce that the carrier test for ML4 now is available to the general population through a simple blood test. Thanks to the efforts of concerned parents and professionals, the American College of Obstetricians and Gynecologists has been encouraged to recommend ML4 carrier screening to all Ashkenazi Jewish patients.

We continue to educate the medical population as well as the general public about the diagnostic criteria for ML4. We are concerned that affected children remain undiagnosed or are misdiagnosed until the age of two or even older.

The ML4 Foundation is pleased to fund the following researchers at major medical institutions:

1. Craig Montell, Ph.D., Professor, Biological Chemistry, Johns Hopkins University School of Medicine, Baltimore, MD, for the molecular and biophysical characterization of the mucopolip protein that is defective in individuals with ML4;
2. Andrew Scharenberg, MD, Assistant Professor of Pediatrics and Adjunct Assistant Professor of Immunology, University of Washington School of Medicine, Seattle, WA, for the construction of mucopolipin-deficient cell lines using somatic cell genetics; and,
3. Janice LaPlante, Ph.D., Brigham & Women's Hospital, Boston, MA, for screening for compounds that promote lysosomal clearance from cells of individuals with ML4.

The National Institutes of Health has renewed a generous grant to Susan Slaughaupt, Ph.D., Assistant Geneticist, Molecular Neurogenetics Unit of the Harvard Institute of Human Genetics/Massachusetts General Hospital, Boston, MA, to continue to perform ML4 research.

Raffi Schiffmann, MD, and Ehud Goldin, Ph.D., National Institute of Neurological Disorders and Stroke, Bethesda, MD, continue their ML4 clinical and basic science research. They are working to develop a mouse model for ML4 that will enable researchers to study the function of the ML4 gene and possible treatments.

We have been very active in pursuing our dream for the treatment and cure of ML4. Without your contributions, the research we describe here would not have been possible. Without your continuing support, our dream cannot become a reality.

Thank you in advance for your continuing support of the ML4 Foundation. Your contributions will affect the future quality of life of the ML4 children.

Sincerely,

Randy Yudenfriend Glaser
President

Save the Date: March 20, 2004
The ML4 Foundation's Benefit Performance of
The Chosen
by Aaron Posner and Chaim Potok,
starring Theodore Bikel.