Dr. Lawrence Wrabetz,
Professor of Neurology
Director, HJKRI

Lawrence Wrabetz M.D. is the Director of the Hunter James Kelly Research Institute and Professor in Neurology and Biochemistry in the School of Medicine and Biomedical Sciences, University at Buffalo. He graduated in Chemistry from Marquette University, 1980, and in Medicine from Pritzker School of Medicine, University of Chicago 1984. He is specialized and board certified in Neurology (University of Pennsylvania 1984-88), and won a Charles A. Dana Fellowship in Neuroscience (1989) and Javits Fellowship (1991) and Clinical Investigator Development Award (1993) from the NIH to study the regulation of myelin gene expression. He moved to San Raffaele Scientific Institute, DIBIT in Milan in 1993 where he was Head of the Biology of Myelin Unit from 1995-2011.

The Wrabetz Laboratory is interested in the molecular genetics of myelination, studied primarily in transgenic mice. They have focused on the pathogenesis of inherited demyelinating neuropathies, and together with the laboratory of Laura Feltri, they have developed and characterized multiple mouse models of Charcot-Marie-Tooth (CMT) neuropathies. By introducing authentic CMT mutations from patients into these mice, they are revealing the mechanisms of disease, potential avenues of therapy and the opportunity to test resulting medicines in preclinical trials for CMT. Dr. Wrabetz's laboratory is now translating this experience into the generation of new mouse models of Krabbe disease that carry authentic GalC mutations found in patients with diverse clinical phenotypes. These animal models will provide insight into the effect of different mutations on the function of the galactosylceramidase protein and on the pathogenesis of the disease. Dr. Wrabetz's work has been supported by the National Institutes of Health; Telethon, Italy; the European Community, the Italian and Great Britain Multiple Sclerosis Societies, and the Mariani and Cariplo Foundations.

Dr. Wrabetz has also served as a Neurology consultant for the Telethon Institute of Gene Therapy in Milan, where he followed a cohort of children with metachromatic leukodystrophy in order to define appropriate endpoints for clinical trials. Finally, he serves on the scientific advisory committees for the Charcot-Marie-Tooth Association, the Italian Multiple Sclerosis Foundation and the Mariani Foundation for Pediatric Neurology.