

Notes from Dr. Dan Chung's presentation at the CRB1 research symposium March 23, 2013

Dan Chung of the Scheie Eye Institute described the successful gene therapy for RPE65-LCA2. Evaluating increase in activities that patients can do independently post-treatment may be a meaningful way to evaluate efficacy, he said. "It's brightness, not so much visual acuity. Patient comments are very important. We are standardizing and doing a validation of the mobility course. Patients visit CHOP three times to do the mobility course at different light levels. We're now recruiting, and *CRB1* kids can do this," he said.

ERGs don't change in the gene therapy patients. Dr. Chung mentioned the recent Jacobson paper that found no halt to photoreceptor degeneration following gene therapy, pointing out that that paper did not state whether the researchers evaluated the treated part of the RPE.
(<http://blogs.plos.org/dnascience/2013/01/31/another-bump-in-the-road-to-gene-therapy/> -- Ricki)