

## **Clinical Trial for Newly Diagnosed Krabbe Patients**

A new clinical trial for babies and kids with newly diagnosed Krabbe Disease just opened at Duke. The trial is testing a new cell called DUOC-01, which is grown from umbilical cord blood in 21 days. The DUOC cell is most like a microglial cell, which is the cell that grows in the brain after a cord blood transplant. Typically it takes months for the microglial cell from a regular cord blood transplant to reach the brain. The DUOC cell can be given into the child's spinal fluid through a spinal tap 4 weeks after a cord blood transplant reaching the brain within a few days after injection. The hope is that this will be safe and will be a way to help the brain months earlier than the transplant does. This should slow or stop disease progression earlier and preserve more function in the child with Krabbe disease after transplant.

The study is being run under an IND from the FDA. A full description can be found on <[clintrials.gov](http://clintrials.gov)>. Search for Dr. Kurtzberg's name as she is the PI. Up to 18 patients who are being treated with a cord blood transplant for Krabbe Disease or other leukodystrophies can be enrolled. The first 3 patients have to be enrolled over 6 months. If they do well, subsequent patients can be enrolled more quickly.

The work to develop the cells and the ways to produce them was funded by the Robertson Foundation. The study is funded by the Marcus Foundation. The costs of the cell production, the spinal tap, the cell administration and extra testing to show the cells are safe, and are all covered by the study. The costs of the regular transplant have to be covered by the child's medical insurance. Interested families should contact Dr. Joanne Kurtzberg at Duke by email [kurtz001@mc.duke.edu](mailto:kurtz001@mc.duke.edu) or phone (919-668-1102).