Ilyce's presentation before the NIH

As a parent it is my job to protect my child and advocate on his behalf; due to Max's special needs advocacy has become even more important, it is an integral part of everyday life. I am the voice of my child, speaking on his behalf with doctors, insurance companies, teachers, therapists, and sometimes legislators.

As the mothers of a child afflicted with Canavan disease I have created a foundation to support medical research aimed at improving the lives of all children diagnosed with Canavan disease. I have vowed to make certain that Canavan disease is well known to legislators and policymakers in Washington. The old adage "the squeaky wheel gets the oil" holds true in our nation's capitol. If our children are to have equal access to medical treatment it is essential that Federal funding for rare disease research is continually increased. In order to accomplish this goal it is absolutely critical that families affected by extremely rare diseases work together to increase awareness and educate people in our communities and Government. Rare diseases may only affect a relatively small percentage of the population, but they can offer invaluable insights to aid in the cure and treatment of more common illnesses.

Unfortunately sometimes even after Congress approves a budget increase for rare disease research, the money is not allocated as promised. As a result fewer grant applications are awarded funds. Usually it is the extremely rare diseases that end up without research grants due to the lack of available funds. This factor makes it extremely important that our voices be heard.

I recently traveled to Washington, DC on a two-fold mission. First, to educate and create awareness about extremely rare diseases, specifically Canavan. And second, to explain why it is necessary, and in the public interest to increase Governmental budgets for rare disease research. I also called attention to the fact that when budgeted funds are not allocated it is our children who suffer first.

As a mother who is also the Directors of National non-profit foundation, and an experienced patient advocate my goals were to reinforce alliances with legislators already familiar with my mission, and usher additional support at several first time meetings. I explained that when not in Washington I am at home with my children, running my foundation, and continuing to do my part by aggressively raising private funds for research. In making this trip my hope was to gain increased support as I move forward with my mission of curing Canavan disease.

I met with over 20 different people in two days: members of the House and Senate, appropriation and oversight committees, the U S Department of HHS, NICHHD, NIH, ORD, and NINDS. After a dozen appointments I was physically and emotionally exhausted...but it was worth it. I reinforced existing relationships, created new, helpful contacts, and made absolutely sure that Canavan disease remains on the radar in Washington. By all accounts the "squeaky wheel" trip was a success. But I am far from finished, this was not my first visit and it will not be my last. I will work to increase our fund-raising efforts at home, advocate for my child, and continue to raise awareness about Canavan disease by joining forces with other affected families. Having a successful visit to Washington, raising more than expected at a fund-raiser, or winning a battle with school or insurance does not mean that I am finished with political activism , raising money, or advocating for Max, and all children afflicted with Canavan disease..

Successes is motivating, I work harder after seeing the results of my efforts. Looking at the impressive scientific gains made possible by parent funded research, and the addition of a grant from NINDS, brings to light the realization that with enough funding we can cure Canavan, and other genetic diseases. After gene therapy successfully halted disease progression in Canavan patients, the same technology was used in a Parkinson's trial. This medical advancement would not have been possible without private funding

coupled with a 2.3 million dollar grant from NINDS. Imagine what could be accomplished if large scale Government funding of rare disease research were increased enough to adequately fund projects aimed at curing rare diseases. Knowledge gained from the study and treatment of these diseases would ultimately benefit millions of people suffering from more common illnesses, and our children would have hope for a cure.

By: Ilyce Randell, Canavan Research Illinois

"Max's mom"