

Dear Congressperson:

Imagine for a moment that a child you love, perhaps your son, grandson, or your nephew was unexpectedly diagnosed with one of those “parent’s worst nightmare diseases.” In this case, a ravaging neuro-degenerative disease that would lead to death, typically just thirty months after onset. More than 1 in every 17,000 children of our great nation are diagnosed each year with Adrenoleukodystrophy (ALD). Because there is little to no funding dedicated to clinical or therapeutic ALD and ALD-related research, I am asking you to help reverse this dilemma.

ALD is more widespread than ALS (Lou Gehrig’s disease). It is a genetic disorder characterized by progressive brain and other central nervous system deterioration. ALD affects boys between the ages of 5 and 12 years. Symptoms include the loss of vision, hearing, and speech, often just six months into onset. These symptoms lead to a vegetative state usually within the first year.

Adrenomyeloneuropathy (AMN), is the adult form of ALD. AMN affects mainly the spinal cord and leads to Multiple Sclerosis type symptoms, including a diminished gait, bladder, and bowel difficulties which worsen over a period of decades. AMN affects otherwise healthy adult males between the ages of 20 and 40. While not life shortening for 65% of those men with this disease, the other 35% develop cerebral symptoms similar to ALD that leads to death in just a few short years.

While there is currently research being conducted to remedy these disorders, today there are no guaranteed treatment options available for either disease. Traditional bone-marrow transplants (BMT’s) have been attempted in both diseases, however, in the case of AMN, 70% of the men who have gone through this process die from complications related to this procedure. Additionally, there is a mean cost of BMT’s of almost \$75,000.00. Currently, there is hope for the development of therapies that will better address the needs of those affected; however, in order for such treatments to be developed, congressional assistance is required.

Both diseases carry with them an additional burden in terms of life management. A man who develops AMN during early mid-life, who can no longer provide for himself or his family, will certainly incur financial hardships, as well as the emotional strain one experiences going through such a major life change. The transition from healthy, vital adult to a physically and often emotionally unsure human being is certainly challenging. Having your independence stripped away so suddenly, with uncertainty as your only guarantee, is not easy. The family of a boy affected by ALD also encounters problems along the roads they travel. Finding assistance, whether financially, or with medical treatment from home health agencies or a hospice service, is quite burdensome. Most families exploring this type of assistance often find that by the time an agency is located, and the red tape is sifted through, the life their child now endures has been diminished to the point of no return.

Again, there is no funding dedicated for ALD/AMN research, and I am asking you to help change that. By providing the necessary resources for novel approaches to be attempted, you will not only be assisting those people who are afflicted by these diseases, but also the family members and friends who stand by each day and watch, without hope, as their love one’s life slips away. Please help all people affected by these diseases. Your participation will make a difference.

For more information on these disorders, you may visit the following web-sites:

www.ALDFoundation.org
www.StopALD.org

Thank you for your willingness to review the concerns that I have expressed to you today. I look forward to hearing back from you regarding both ALD and AMN. Again, I know that your involvement will make a difference.

Very Truly Yours,