

NBIA center of
excellence 
Neurodegeneration with Brain Iron Accumulation
International Center of Excellence



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Dear families touched by PLAN and your providers,

A recent scientific paper (see attached) reports that PLAN is associated with ceramide accumulation in a fruit fly model of this disorder. Ceramide is a type of fat (lipid) used widely in the body, especially in the membranes of cells. The authors report that accumulation of ceramide in the nervous system puts stress on an organelle in the cell called the lysosome, which has the job of breaking down waste products. An FDA-approved drug called desipramine, a tricyclic antidepressant, can block ceramide accumulation and its downstream consequences in the fly model.

Individuals with PLAN or their family members / care providers may wish to provide the attached paper to their neurologists or primary care physicians. Since this is an FDA-approved drug available for other medical indications, feasibility of using it on an "off label" basis in PLAN could be considered. We have no experience yet with this drug in PLAN, so monitoring for safety, including of cardiac rhythm functions as well as other safety parameters, would be important for anyone prescribing it. While our NBIAcure team has a significant interest in learning whether there is a therapeutic role for desipramine, we currently do not have the resources to start a new trial at OHSU. We therefore hope to help make the PLAN community aware of this new data in an informed and thoughtful way so that it can be discussed further with individual providers. For those with PLAN who are currently enrolled in our online natural history study, PLANready, it will be key to continue with participation so that we can collect information over time about any impact from the addition of desipramine treatment. We would encourage those *not* enrolled in PLANready to do so before starting desipramine and then continue in the study for the same reason. Any questions related to this information can be directed to our general box at info@NBIAcure.org.

Sincerely,



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