



## DEPARTMENT OF HEALTH AND HUMAN SERVICES

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Secretary's Advisory Committee on Heritable  
Disorders in Newborns and Children  
5600 Fishers Lane, Room 18A19  
Rockville, Maryland 20857  
(301) 443-1080 – Phone  
[www.hrsa.gov/advisorycommittees](http://www.hrsa.gov/advisorycommittees)

October 1, 2012

Charles Peters, M.D.  
48055 252<sup>nd</sup> Street  
Garretson, SD 57030

Amber Salzman, Ph.D.  
The Stop ALD Foundation  
500 Jefferson Street, Suite 2000  
Houston, Texas 77002-7371

Dear Drs. Peters and Salzman:

The Secretary's Advisory Committee on Heritable Disorders in Newborns and Children (Committee) appreciates your nomination of X-linked Adrenoleukodystrophy (ALD) for inclusion in the Recommended Uniform Screening Panel (RUSP) for state newborn screening programs. As part of the formal review process, the Nomination and Prioritization Committee conducted a preliminary review of the nomination package, and results were presented for discussion and a decision during the September 2012 Committee meeting. A copy of the presentation is enclosed.

The Committee recognizes ALD as a medically important disorder that deserves serious consideration, possessing a well-established case definition as well as screening, diagnostic, and treatment protocols. However, at this time, the Committee has decided to not send the nomination forward to the external condition review group.

The Committee's decision is based primarily on the determination that sufficient prospective data is not yet available from the large pilot study presently underway at the Mayo Biochemical Genetics Laboratory (MBGL).

After additional data from the MBGL study is made available to the Committee for evaluation, we encourage you to contact us to facilitate an expedited review. The Committee will then determine whether the new data provides sufficient support for the Committee to request a formal review of the scientific evidence by the external condition review group.

Please contact Lisa Vasquez ([lvasquez@hrsa.gov](mailto:lvasquez@hrsa.gov)) when you are prepared to submit any additional data or if you have any questions or concerns.

Thank you for your nomination of ALD for inclusion in the RUSP for state newborn screening programs.

Sincerely yours,

/s/

Joseph A. Bocchini Jr., M.D.  
Chairperson

Enclosure:  
SACHDNC Nomination and Prioritization Workgroup Presentation: ALD