



A GOAL FOR LIFE

For Friends and Supporters of the Ara Parseghian Medical Research Foundation



The true gift of Christmas...new life.



The Parseghian family announces with great joy the arrival of

Lila Elizabeth Parseghian

*born October 6, 2013
to Ara & Cicely Parseghian.*

Science Update

Hope for NP-C Patients

A clinical trial is expected to begin in the summer of 2014 using a Histone Deacetylase Inhibitors (HDACi). The goal of the Phase 1 clinical trial will be to establish the safety and tolerability of an oral HDACi as a treatment for NPC1 disease. To meet this objective, researchers at the National Institutes of Health, Mayo Clinic, Washington University and Cornell will develop a Phase 1, first-in-human, open-label, multi-center, dose escalation study of a HDACi in late adolescents and adults with NPC1 disease. Results from this study will lay the foundation for future clinical trials to assess the effectiveness of an HDACi in slowing disease progression, and may lead to the first FDA-approved drug for the treatment of this devastating disorder. The trial is dependent on an approval of an Investigational New Drug Application (IND) by the Food and Drug Administration (FDA).

The secondary objectives of the trial will be to determine biochemical efficacy of the HDACi to increase expression of the NPC1 protein and normalize lipid and protein biomarkers. A team of researchers will examine data on plasma and cerebrospinal fluid markers to use as potential clinical endpoints. These outcome measures can potentially serve as surrogate outcome measures in future Phase 2 and Phase 3 HDACi trials.

Twelve NPC1 patients (18 years and older) will be recruited for the study. Eligible

patients will demonstrate at least one neurological manifestation of NPC1 (e.g., hearing loss, vertical supranuclear gaze palsy, ataxia, dementia, dystonia, seizures, dysarthria, or dysphagia). Symptoms cannot be so severe that they interfere with the patient's ability to comply with the requirements of this study. Testing will be performed on patient fibroblasts to establish in vivo responsiveness to the HDACi. The safety of HDACi in pediatric populations has not been established. Children under 18 years old are thus currently excluded due to FDA comments, in a pre-IND meeting, suggesting that safety and tolerability first be established in adults. Potential participants will be admitted to the NIH Clinical Center or Mayo Clinic for determination of eligibility and baseline clinical evaluations. Patients who have received any form of cyclodextrin or an HDACi in the past in an attempt to treat NPC1 will not qualify for the trial.

Clinical sites will be overseen by Dr. Porter at the National Institutes of Health Clinical Center and Dr. Marc Patterson at the Mayo Clinic in Rochester, MN. Dr. Ory will oversee performance biomarker analysis of clinical samples at Washington University, while Dr. Maxfield will supervise personnel in his laboratory who will perform quantification of NPC1 protein expression and histone acetylation in the clinical samples. Dr. Maxfield and his personnel will also test fibroblasts from prospective trial participants to confirm that their cells respond to HDACi treatment.

The discovery of the use of HDACi for the treatment of NP-C was first made in the labs of Paul Helquist and Olaf Wiest at the University of Notre Dame. They soon collaborated with Dr. Fred Maxfield's lab at Cornell who helped confirm their hypothesis. Recently, the Maxfield laboratory found that treatment of human NPC1 mutant cells with certain HDACi leads to clearance of excess cholesterol and other lipids from certain parts of the cells and it corrects the overall defect in cholesterol regulation. This metabolic correction was associated with increased expression of the NPC1 protein. Based on this preliminary data, the HDACi treatment could result in a benefit to NPC1 patients.

Funding for the Phase 1 trial has been provided by a grant from the National Institutes of Health, funds raised by Greg Crawford, Dean of the College of Science at the University of Notre Dame and the APMRF.

The APMRF has been instrumental in funding the labs of Helquist, Wiest, Maxfield, Ory and Dr. Steven Sturley, at Columbia University, in the investigation of HDACi, providing the ground work for this Phase 1 trial. In addition, the APMRF has been a major supporter of the Natural History Study conducted by Dr. Porter at the NIH which has focused on gathering of clinical data and establishing biomarkers for use in clinical trials.

Heartfelt Thanks...

The 6th Annual **Smith Family BReak Thru Golf Outing** was another great success! Held on August 3rd at the Lafayette Elks Club Country Club, Indiana, with the support of this caring group of participants, volunteers, sponsors and staff, all the elements were in place to once again make this event so special.

The Smith family has joined many other families in the passionate battle against Niemann Pick Type C disease and we thank them for their time, energy and dedication on behalf of their NP-C children, Braden, Keaton and Riley, and all children afflicted with NP-C.



Riley, Keaton & Chandlar Smith



Heartfelt Thanks...

(cont'd. from page 2)

The Knollwood Country Club in Granger, IN hosted the **Club Corp Charity Classic** on Friday, August 23rd with proceeds benefiting APMRF and two other charities. We wish to thank Dean Greg Crawford, the volunteers and the staff for a beautiful day of "Give, Golf, Dine".



The College of Science at the University of Notre Dame partnered with the Notre Dame Women's Soccer Team on August 31st raising money for NP-C research in a very "lofty" way. **The Flying Frisbee Contest** involved the sale of 500 frisbees with participants aiming to hit a target...the winner, of which there was only one, became the proud owner of a Coach Parseghian autographed football! Go Irish!

The Northwestern University chapter of **Uplifting Athletes** held their third annual **Lift for Life** event benefiting the Parseghian Foundation on July 24th.



Held on the campus of NU at the Ryan Field Complex, the football players gave fans a glimpse of their summer workouts while raising money for a worthy cause. Afterwards the fans were treated to a "meet and greet" picnic.

"We want to use our status as student-athletes to promote something bigger than ourselves," said Junior offensive lineman Paul Jorgensen, President of the NU Chapter of Uplifting Athletes. We had some friends and family out here tonight and had a good time all around raising money for a good cause."

Ara Parseghian served as the head football coach at Northwestern from 1956-63 and led the Wildcats to a No. 1 ranking in 1962.

Stacey Friedl, mother of 11 year old Ashton Friedl, organized a fun and creative **"Uncork Your Creativity"** gathering at Spirited Art in Richmond, VA. The group had a blast painting their very own masterpiece as evidenced by the photo. Great way to keep the fun in fundraising! Thank you Stacey and to all who attended.



Mark Your Calendar



April 5, 2014

The University of Arizona Rugby Team will travel to South Bend to take on the Irish at Notre Dame's new Stinson Rugby Field for the second annual [Ara Parseghian Cup](#) rugby match. For more information contact: soleary@nd.edu

May 15, 2014

Irish Legends takes on a new format this year. Plans are shaping up for the [Coaches Challenge](#), a limited field golf tournament based on a President's Cup format. An additional event will feature a lovely reception and dinner to be



held at the Drake Hotel in downtown Chicago. For information contact Angela Monger at (800) 628-9922, Ext. 503 or acmonger@sap-corp.com



June 5-9, 2014

The third annual "[Parseghian Classic](#)", a 3-day golf tournament, will be held at the magnificent Pebble Beach Golf Resort. Golfers will play a round of golf at The Links at Spanish Bay and another at the Pebble Beach Golf Links. The all-inclusive event includes a welcome reception and dinner, a putting tournament, and a final reception and dinner with a special guest performance. Full golf and non-golf packages are available. Visit parseghianclassic.nd.edu for additional information.

June 12-14, 2014

The annual "[Michael, Marcia & Christa Parseghian Scientific Conference](#)" for Niemann Pick Type C research will be held on the campus of the University of Notre Dame. Researchers will gather for three days to discuss the advances in NP-C research. This yearly meeting helps to form collaborations and determine the future direction of NP-C research.



PLEASE NOTE OUR NEW MAILING ADDRESS:

Ara Parseghian Medical Research Foundation

4729 E. Sunrise Drive, Suite 327

Tucson, AZ 85718-4535

Heart and Soul

“Help one another; there’s no time like the present and no present like the time.” – James Durst

Volunteers have been the heart and soul of the Foundation from the very beginning... and continue to do so!

Helping to forge and sustain the course, hundreds of volunteers have devoted unlimited hours to serving on committees, logged thousands of hours on computers, coordinated a multitude of mailings, processed mountains of acknowledgments and tirelessly helped to bring the cause to the attention of hundreds of thousands worldwide. We would not be where we are today without their helping hands and loving hearts.

Thank you to volunteers everywhere...



A very special group that are part of the Foundation’s heart and soul in Tucson, AZ

L to R: Cindy Parseghian, Peg Romano, Karen Sarikas, Pia Park, Annegret Mansuripur, Susan McDonald



The “cast and crew” from one of the Ara’s Outings

Standing L to R: Tom Mulcahy, III (Tom’s third son), Wilbur Barrett, Terry Mulcahy (Tom’s oldest son who helped run the golf outing every year) and Bill Barrett. Seated L to R: Thomas L. Mulcahy and Ara Parseghian

Remembering

Thomas L. Mulcahy

August 5, 1935- August 24, 2013

A longtime friend and enthusiastic supporter of the Parseghian family and Foundation, Tom was the inspiration and driving force behind the successful golf tournament, “Ara’s Outing”.

Along with the Notre Dame Club of Milwaukee, this event continued for 10 wonderful years, raising over \$250,000 for NP-C research.

Tom will be missed by many but his legacy of love, loyalty, integrity and commitment that he practiced in all areas of his life will live on.



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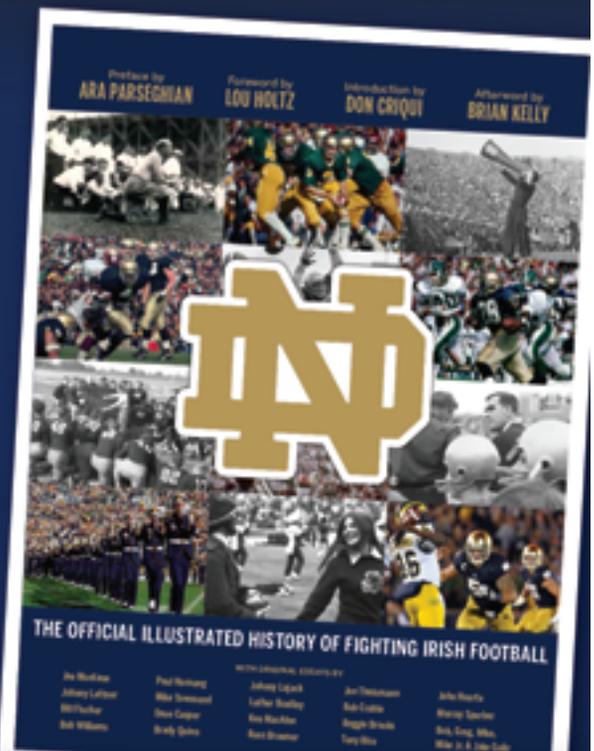
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