

A GOAL FOR LIFE

For Friends and Supporters of the Ara Parseghian Medical Research Foundation



It's about loving unconditionally...

Celebrating family and friends...

*Giving thanks for our blessings
all year long...*

*May the holiday season fill your home with joy,
your heart with love and your life with peace.*

*With warmest wishes,
The Parseghian Family*

Science Update

NP-C DRUG TRIAL TO START IN EARLY 2013

A clinical trial will begin in early 2013 with Cyclodextrin. The potential of this drug to be used as an NP-C therapy was first discovered by Parseghian Foundation sponsored researchers at the University of Texas-Southwestern, Mount Sinai and University of California – San Francisco. Since then, these researchers, along with other Foundation funded researchers, have done extensive investigation into these drugs efficacy for slowing the disease process. In addition, the safety of the drug has been studied. This research, along with a Foundation sponsored Natural History Study at the National Institutes of Health, has made it possible for this trial to become a reality.

The trial is being run by the National Institutes of Health Therapeutics for Rare and Neglected -Niemann-Pick Disease Type C (TRND team). This team is composed of researchers and clinicians from the National Institutes of Health, Johnson & Johnson, and APMRF funded researchers.

After two Pre-IND meetings with the Food and Drug Administration (FDA) in late 2011, the TRND team has continued to move forward in the preparation of the Phase I Study of Cyclodextrin for NPC1 Disease.

The goal is to collaboratively develop a safe, effective and FDA-approved therapy for patients with NPC1. The protocol has undergone ethics review by the National Institute of Child Health and Human Institutional Review Board and they requested only minor changes.

An Investigational New Drug application was submitted to the Food and Drug Administration mid-November 2012. To date, the major issue has been completing the juvenile dog study that the FDA has requested. If no problems are encountered with the IND, it is anticipated that the clinical trial will start in January.

This Phase 1 trial will establish the groundwork for a safe and effective dosing regimen for Cyclodextrin treatment of individuals with NPC1. To obtain useful data that will allow use Cyclodextrin as an effective therapy, the trial must be designed using a systematic, controlled, and scientifically rigorous approach.

Cyclodextrin will be given by intracerebroventricular (ICV) administration via an Ommaya reservoir. This dose escalation study will include 9 patients divided in 3 dosing cohorts. The study is designed to determine the best benefit-risk profile, determine whether dose or time of treatment is most important in humans, and to avoid giving suboptimal doses. Information of the eligibility criteria will be available once the protocol is approved.

If a safe and biochemically effective dose is determined in this Phase I study, the plan is to move forward with a Phase II, multi-center trial to evaluate for efficacy. Planning for this trial is already in progress.



The TRND Team



Heartfelt Thanks...

Tucson Orthopedic Institute

We wish to thank everyone at the Tucson Orthopedic Institute for their ongoing support that they have shown the Parseghian family and the APMRF through their annual fundraising activities. These activities take on various themes from year to year but most importantly continue to raise awareness and provide support for NP-C families everywhere.



Walk For Ashton

Last May the students of Washington Henry Elementary School in Mechanicsville, Virginia, gathered to walk for a very special classmate, Ashton Friedl. Under the caring direction of Kindergarten teacher, Melinda Johnson, the students participated enthusiastically in the fifth annual "Walk for Ashton". With the thoughtful and compassionate efforts of this caring community hope continues for the Friedl family and all families living with NP-C disease.



Ashton Friedl and friends at the "Walk For Ashton" event.

Northwestern Football Uplifting Athletes

The Northwestern chapter of Uplifting Athletes held their second annual "Lift for Life" on Friday, July 27, 2012. This enthusiastic group of athletes held a public work-out and provided the fans an opportunity to see a glimpse of their preparation for the 2012 season while raising funds for NP-C research. NU defensive lineman Quentin Williams stated, "Last year's event received a lot of praise, raising significant awareness and funding for the rare disease Niemann Pick Type C. Like all charity events, "Lift for Life" is an opportunity to give to a great cause while having a great experience." During the 2010 season, former

Northwestern coach, Ara Parseghian, met with the team and the impact of this visit is what motivated the chapter to choose APMRF. Chapter president, Brian Arnfelt states, "Lift for Life" is a really



Members of the Northwestern University football team at the "Lift for Life" fundraising event.

great way to give back to someone who gave so much to our program, especially because it is such a rare disease." Formed in October of 2010, the Northwestern chapter of Uplifting Athletes uses college football as a platform to generate awareness and funds to help cure rare diseases.

BReak Thru Fund Golf Outing

Another year...another great event! Thank you to everyone who came out and supported the Smith Family at their fifth annual golf outing. Held on August 4, 2012 at the Lafayette Elks Country Club in Lafayette, Indiana the day provided a great day of golf while raising significant funds for NP-C research. The Smith family works in collaboration with the Parseghian Foundation to decide what research should be funded with the money raised through these efforts. To date over \$270,000 has been raised in support of the Smith family's BReak Thru Fund.

The Notre Dame Women Connect, New York chapter hosted their third inaugural event in May 2012. Hosted by alumnus Tony Kahn (JD, MBA 1980) and White & Case LLP, over 100 alumna, friends and family enjoyed an inspiring and engaging evening to connect, nurture and give back to the women's community of Notre Dame through support of the Ara Parseghian Foundation for Medical Research. In attendance were Jean Collier, Senior Corporate Advisor-Corporate & Foundations Relations, University of Notre Dame and Dyan Rohol, NDNY Club President and Cindy Parseghian, who was the evening's featured speaker.



Jean Collier at Notre Dame Women Connect, NYC.

Niemann Pick Family Conference

Nashville, Tennessee was the setting of this year's 20th annual National Niemann Pick Disease Foundation Family Support and Medical Conference "Where Hope and Harmony Unite", sponsored by the National Niemann Pick Disease Foundation (NNPDF). Parents, many accompanied by their NP-C children, met with researchers and healthcare personnel. The weekend was an opportunity for families to share information and connect with other parents who also struggle with the same challenges of raising a disabled child afflicted with the ravages of a fatal disorder. Families from the U.S., Canada, Europe and several other countries attended the meetings. The NNPDF supports all Niemann Pick diseases, including A, B, C and D.

Several meeting sessions focused on the advances in NP-C research in which many APMRF researchers presented their latest research findings. The highlight of these sessions was the lecture discussing the upcoming drug trial with Cyclo-dextrin at the National Institutes of Health (See article on Page 2). Other sessions dealt with issues of disease symptom management, along with advice for caregivers. Nicole Yanjanin, a funded APMRF researcher, was also on hand to take blood samples as part of the continuing Natural History Study and development of an infantile screen for NP-C.

Niemann Pick families and researchers had the opportunity to mingle during breaks to get to know each other. The parents were able to get many of their questions answered, while the researchers were able to learn first hand the struggles facing Niemann Pick patients and their families. NP-C mother, Brenda Teller, once again coordinated day care for the Niemann Pick children and their siblings. She provided a fun and safe environment for the children.

Barb Vorpahl was honored for her 20 year commitment to the NNPDF. Barb and her husband Gary are founding members of the NNPDF and have provided direction and leadership for the organization for two decades. The Vorpahl's youngest daughter, Stacey, died in 2004, after a courageous battle with NP-C. Barb's dedication has been a blessing to all Niemann Pick families.

Thanks to the NNPDF for once again organizing this important meeting which is a lifeline for many families. Next year's conference will be held in Baltimore, Maryland in late summer and will be hosted by Mark and Darille Papier.



Barb Vorpahl is recognized for 20 years of dedication and commitment to the NNPDF.



Cindy Parseghian and Andy Wood share a moment at the Family Conference.



Having fun at the Family Conference!



The Papier Family



Mark Your Calendar

January 26, 2013

Verna Burrows and friends in Las Vegas, Nevada are hosting the eighth annual "A Touchdown for Ty" at the College of Southern Nevada. This event held in honor of Verna's grandson is a wonderful, family oriented, variety program with a silent auction and reception. For more information contact Verna at (702) 363-5443.

March 17, 2013

Athlete Bill Keen, sidelined by a foot injury, was not able to fully compete in the Ironman Competition in November as planned. However, he is determined to achieve his original goal to raise funds for the three Irish Legends coaches; Coach Parseghian, Coach Holtz and Coach Kelly and their respective foundations and has committed to competing in the [Ironman Triathlon](#) on March 17th in Los Cabos, Mexico. Please consider supporting Bill in this ambitious endeavor! For information contact Bill at (219) 381-6852 or go to [IrishLegendsIronMan.com](#).



May 4, 2013

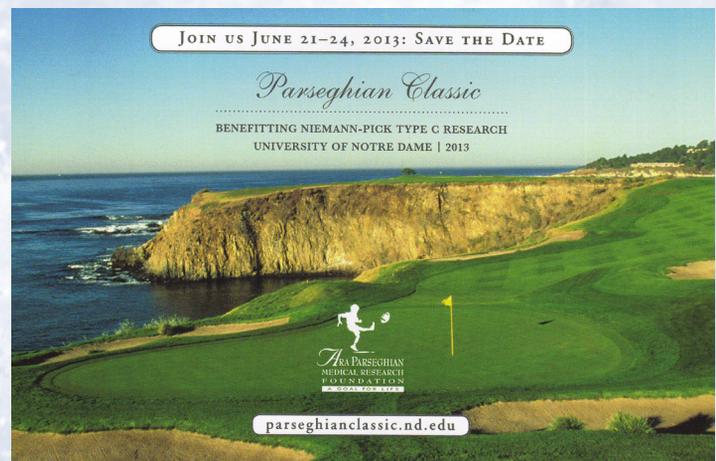
The fifth annual event held in honor of Ashton Friedl will for the second time be the "It Comes In Pink – PinkNic". Held at the Richmond International Raceway in Richmond, Virginia this event promises to be a great afternoon for the entire family! For ticket information go to: [www.itcomesinpink.org](#)

June 12, 2013

The seventh annual "Irish Legends Golf Classic" will be held at the Lost Dunes Golf Club in Bridgman, Michigan. Legends Ara Parseghian, Lou Holtz and current fighting Irish football coach Brian Kelly support this wonderful golf outing that supports their foundations. For information contact Angela Monger at (800) 628-9922, Ext 503 or [acmonger@sap-corp.com](#)

June 13-15, 2013

The annual "Michael, Marcia & Christa Parseghian Scientific Conference" for Niemann Pick Type C research will be held on the campus at the University of Notre Dame. Researchers and NP-C family members will attend 2 full days of presentations, discussions and collaborations on the latest developments in NP-C research.



June 21-24, 2013

The second 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 opens with a welcome reception and dinner and to round out this wonderful weekend, a final reception and dinner will be held with a performance by special guest, singer/songwriter, Amy Grant. Full golf and non-golf packages are available. Visit [www.parseghianclassic.nd.edu](#) for additional information.





Ara Parseghian Medical Research Foundation

3530 E. Campo Abierto
Suite 105
Tucson, AZ 85718
Phone: (520) 577-5106
Fax: (520) 577-5212
www.parseghian.org

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In this season of giving please consider a tribute to a family member, friend or client to the Parseghian Foundation. When you make a donation (whether honor or memorial), a card advising of your gift will be sent to the person(s) you indicate. Call APMRF at (520) 577-5106, make a donation online at www.parseghian.org or use the enclosed envelope. Your support is greatly appreciated.

