

# A GOAL FOR LIFE

For Friends and Supporters of the Ara Parseghian Medical Research Foundation



Dear Friends,

In many ways, life is like a football game. Both are made up of a series of events, or plays. Some plays are perfectly planned and executed, while others don't work as well. Maybe the players are having an off day, and the moves aren't falling into place. Or maybe you face an opponent so tough that the odds seem insurmountable.

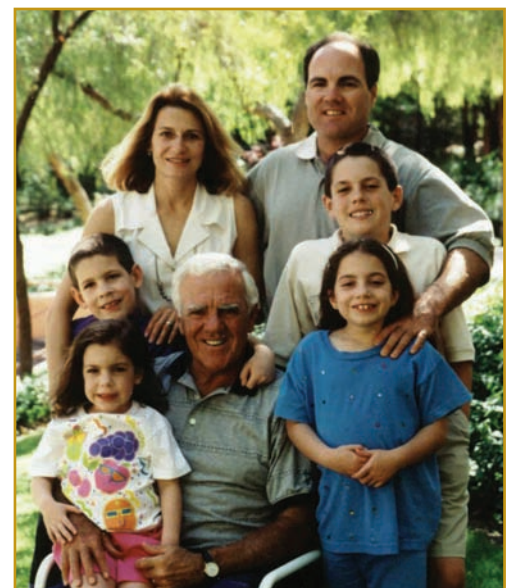
Throughout my career and my life, I have learned that the most important games are won both with skill and with attitude. And when talent is accompanied with heart, it's a winning combination. In formulating our game plan against Niemann-Pick Type C, we have built a foundation blessed by both.

A world-class scientific advisory board oversees the efforts of committed researchers made possible through the support of fantastic friends and volunteers. Pharmaceuticals trials and other NP-C treatment-oriented research remain our primary focus: a goal clearly within reach of our extraordinary offense. Because of the hard work and dedication of APMRF funded researchers, a Phase I clinical trial evaluating the drug cyclodextrin as a possible treatment for NP-C disease is underway. In addition, as you'll read in this newsletter, another Phase I clinical trial with a Histone Deacetylase Inhibitor is scheduled to begin this summer to evaluate the safety and efficacy of this class of drugs as a potential therapy.

I ask you to continue as a crucial part of our very special team. Your generous support and prayers have already helped to achieve a breakthrough which is significant not only to those affected by NP-C, but to millions who suffer from cholesterol-related disorders world-wide. Discovery of a treatment and cure could have equally vast implications.

It's third and goal, and victory against NP-C will be the triumph of a lifetime, a testament to the strength of human spirit. It's a legacy in which we can all take pride, and I look forward to celebrating together.

*Ara Parseghian*



The Parseghian Family in 1995  
with Grandpa Ara.

# Science Update

## HDACi Drug Trial Expected To Start In 2014

Tremendous progress has been made to initiate a new, two-center drug trial later this year at the Mayo Clinic and NIH Clinical Center. To reach this goal a major collaborative effort is underway to determine if a histone deacetylase inhibitor (HDACi) can be used to safely treat patients with Niemann-Pick Disease, type C1 (NPC).

This collaboration includes investigators Drs. Paul Helquist and Olaf Wiest, (University of Notre Dame), Dr. Fred Maxfield (Weill Cornell Medical College), Dr. Dan Ory (Washington University School of Medicine), Dr. Forbes Porter (National Institutes of Health). Dr. Ed Holson (Broad Institute) and Dr. Marc Patterson (Mayo Clinic). This effort has been assisted and coordinated by the Ara Parseghian Medical Research Foundation (APMRF), the University of Notre Dame College of Science, and Translational Drug Development, Inc.

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 and they soon collaborated with Dr. Maxfield 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.

The initial trial will use the HDACi drug vorinostat which has been approved by the FDA to treat a type of cancer known as cutaneous T-cell lymphoma. Vorinostat was shown by the Maxfield laboratory to be effective in cells from NPC1 patients collected as part of the NPC Natural History trial at the NIH. Drs. Ory, Maxfield and Porter have received a U01 grant from the NIH to support the clinical trial work to be performed at the NIH Clinical Center (<http://www.nih.gov/news/health/mar2014/nichd-13.htm>).

The FDA has granted an Investigational New Drug exemption to the APMRF that will allow us to study the safety and potential biochemical efficacy in adult patients with NPC1. The study will enroll 12 NPC1 patients. Although the IND exemption does not allow us to test vorinostat in children with NPC1, this exemption will save significant time and expense in obtaining proof of concept data. Working together, this collaborative group hopes to advance our understanding of the potential of an HDACi to treat individuals with NPC.

Greg Crawford, Dean of the Notre Dame College of Science, in conjunction with APMRF have raised \$500,000 to facilitate this work and to allow for a second clinical trial site at Mayo Clinic. Merck has graciously agreed to provide the drug supply for the trial.

## Mark Your Calendar

### May 15, 2014

The eighth annual, **Irish Legends** event takes on a new look this year. Plans are underway for the Coaches Cup Golf Tournament to be played at Olympia Fields with a lovely reception, auction and dinner to follow in the evening at the Drake Hotel in downtown Chicago. For information contact Angela Monger at (800) 628-9922, ext. 503 or email [acmonger@sap-corp.com](mailto: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 by singer/songwriter Amy Grant. Full golf and non-golf packages are available. Visit [parseghianclassic.nd.edu](http://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 direction of NP-C research.



*Irish Legends: Coaches Parseghian, Kelly and Holtz*



# Heartfelt Thanks

## Pennies add up...

The Dave Iaconis family of Tucson, AZ decided to put their pennies to work...they donated the family piggy bank to APMRF knowing that it would go to an important cause in their hometown. Filled with 18 pounds of pennies, the family had a contest to see who could estimate the closest amount. Pennies do make a difference....please donate yours to APMRF today!



## Notre Dame Cycling Team – Pedaling for NP-C disease!

The ND Cycling Team helped raise funds by transporting visitors around the ND campus in “rickshaws” during the home football weekends. A percentage of the donations received were given to APMRF. We thank the team for their enthusiasm, energy and for their ongoing support. Pedal on!

## Girls Night Out

14 year old Ashton Friedl is now a sixth grader at Chickahominy Middle School in Mechanicsville, VA. This year the school chose Ashton to be the honoree of the annual, Girls Night Out event. Led by Mrs. Cindy Keener and the PE Dept., this special night is set aside for girls and their moms, aunts, grandmothers, neighbors or any significant female in their life, to come together for an evening of a myriad of wonderful activities.

The theme PINK OUT, fit Ashton’s passion for pink perfectly. The participants decked out in pink were treated to their school transformed into a pink wonderland!

The event was a huge success, best turnout EVER. The proceeds were sent to the Foundation to support NP-C research.

To those who created and enjoyed this memorable evening, we thank you.



Ashton enjoying Girls Night Out teacher Miss Amy Gregory.

# In Memory of Riley Smith

**November 14, 1998 – January 19, 2014**

We wish to extend our deepest sympathies to the Smith family. Riley Smith, age 15, lost her courageous battle with NP-C disease on January 19, 2014. Riley’s parents, Trent & Julie Smith created the Smith Family BReak Thru fund at the APMRF in honor of their 3 children with NP-C disease and have worked tirelessly to raise funds and awareness.

Riley is preceded in death by her brother, Braden, who passed away in 2006 at the age of 10. We ask you all to keep Trent & Julie Smith, son Keaton, who also struggles with NP-C, and his older sister Chandler, in your thoughts and prayers.





## Ara Parseghian Medical Research Foundation

4729 E. Sunrise Drive  
Suite 327  
Tucson, AZ 85718-4535  
Phone: (520) 577-5106  
Fax: (520) 577-5212  
www.parseghian.org

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## Infiniti Coaches' Charity Challenge...Caring for Causes

Infiniti, once again, partnered with the National Association of Basketball Coaches, the NCAA and ESPN as the sponsor of the 2014 Infiniti Coaches' Challenge. Purdue University Head Basketball Coach, Matt Painter, participated for the second year in a row representing the Smith Family BReaK Thru Fund at APMRF.

The Challenge encouraged fans to vote daily for the coach of their choice over a ten week period. Out of 48 coaches, Coach Painter made it to the round of the Final Four winning \$15,000 for the BReaK Thru Fund.

We wish to thank Infiniti and partners, and we send our gratitude to Coach Matt Painter whose generosity has offered hope to all families struggling with NP-C disease. His dedication and support on and off the court are greatly appreciated.

