

A GOAL FOR LIFE

For Friends and Supporters of the Ara Parseghian Medical Research Foundation



Dear Friends,

It's always bittersweet to meet a new child afflicted with Niemann-Pick Type C.

Bitter...because it breaks your heart that another innocent child has been struck with this terrible disease. Sweet...because these children are so precious and full of love and joy.

I had the honor of meeting Calum Burdon, a little fellow from England, at the National Institutes of Health (NIH) in Maryland while attending a medical research conference last month.

His mom and dad have enrolled him in the important NP-C Natural History Study for which the Parseghian Foundation has been providing funding and recruiting patients over the past two years.

Like many other NP-C parents, they are doing all that they can to help Calum and to move medical research ahead toward future clinical trials. Families like the Burdons around the world look to the APMRF and the 25 NP-C researchers we are funding to save the lives of their children.

Thanks to so many wonderful donors and friends, we have been able to push NP-C research light years ahead. I think you will see by reading this newsletter that great strides are being made.

We trust that even with the downturn in the economy, this incredible support will continue. We simply must find a treatment soon. Thanks so much.

Sincerely,

Cindy Parseghian



Cindy Parseghian hugs Calum Burdon.

Parseghian Funded Researchers Identify Promising Compound

The Parseghian Foundation is funding four researchers at UT Southwestern, two of whom (Dr. Benny Liu, lead author, and Dr. Joyce Repa) were key researchers involved with this discovery. The compound CYCLO will be the subject of several presentations and great discussion at the upcoming NP-C Scientific Conference hosted by the APMRF in May.

DALLAS – Jan. 26, 2009 – Researchers at UT Southwestern Medical Center have identified in mice a compound that liberates cholesterol that has inappropriately accumulated to excessive levels inside cells.

The findings shed light on how cholesterol is transported through the cells of the body and suggest a possible therapeutic target for Niemann-Pick type C disease (NP-C), a disorder characterized by abnormally high cholesterol levels in every organ.

“What we’ve shown is that very quickly after administration of this compound, the huge pool of cholesterol that has accumulated in cells is suddenly released and metabolized normally,” said Dr. John Dietschy, professor of internal medicine at UT Southwestern and senior author of the

Promising Compound (cont'd. from page 1)

study which appeared in the *Proceedings of the National Academy of Sciences*. "With just one dose, the mice excrete a large portion of the cholesterol."

Researchers injected a single dose of a cholesterol-binding agent known as CYCLO into 7-day-old NP-C mice. Shortly after administration, these mice processed cholesterol just as their healthy counterparts did. After 49 days, the mice treated with a single injection continued to show substantially lower tissue cholesterol levels than the untreated mice, as well as improved liver function and decreased neurodegeneration.

Dr. John Dietschy, who has been studying cholesterol metabolism for nearly 50 years, cautioned that the findings in no way represent a NP-C disease cure. "We do not yet understand what is happening at the molecular level, but it is clear that this compound somehow overcomes the genetic defect that causes individuals to accumulate cholesterol."

The next step is to determine the concentration of CYCLO needed to trigger the cholesterol's release. Researchers also hope to determine in animals the additional lifespan CYCLO administration provides, as well as how long the drug's affects lasts.

There is great hope about the potential of CYCLO with NP-C disease. Subsequent articles will appear in "A Goal For Life" about the progress being made with this compound.

Excerpts reprinted with permission from UT Southwestern Medical Center



Drs. Benny Liu, Joyce Repa and John Dietschy discussing their latest NP-C research discoveries.

Zavesca Approved in Europe

The APMRF has been intimately involved in helping to fund and speed development of the drug Zavesca, which led to a clinical drug trial.

We are very excited to announce that Zavesca has been approved for treatment of patients with NP-C disease in Europe. Our hope is that the Food and Drug Administration (FDA) in the US will soon follow the lead of their European counterpart and approve the drug for US patients.

Through funding provided by the Parseghian Foundation, Dr. Steven Walkley of Albert Einstein College of Medicine in Bronx, NY administered Zavesca to NP-C mice. He found a positive affect and after a number of years, this led to the first-ever clinical trial with NP-C patients.

A number of NP-C patients in the US are taking Zavesca off label as the FDA has approved use of this drug for patients with another lysosomal disease called type 1 Gaucher. The drug is very expensive and many insurance companies will not cover drugs that are prescribed off label. Consequently, until the drug is approved in the US for NP-C patients, many of them are not able to take Zavesca due to the prohibitive costs for most parents.

Actelion Ltd with headquarters in Switzerland developed Zavesca and funded the clinical trials. Jean-Paul Clozel, MD and CEO of Actelion commented, "I am very proud that Actelion—together with the scientific community—has been able to demonstrate the role of Zavesca in reducing the progression of clinically relevant neurological symptoms in patients with NP-C. I would like to thank both the patients and their families who, over the years, have been involved in our clinical program with so much dedication, as well as all the clinical experts for their continuous support."

We feel strongly that it will be a combination of therapies that will ultimately stop the progress of this disease. Zavesca is the first of several therapies that are in the development pipeline. The Parseghian Foundation is working diligently with many researchers, organizations and pharmaceutical companies to bring a number of other therapies to the forefront.

We hope to make many more announcements like this in the near future. Thanks for your support which makes this all possible.

Heartfelt Thanks...

Tucson Originals, Tucson's leading group of independent restaurants, invited the APMRF to be the beneficiary of its sixth annual event, **Tucson Culinary Festival Brunch**, on October 26. It was a great way to spend a Sunday—fabulous gourmet food, entertaining and talented chefs facing off in a cooking challenge, with a room full of appreciative food and beverage connoisseurs!

Tucson Originals does a wonderful job of bringing together great talent and providing Tucsonans with a delightful 4-day festival celebrating fine food, wines and the diversity of the Tucson culinary culture. We applaud them for their passion and commitment not only to providing quality restaurant experiences for Tucson but for their charitable spirit as they generously support organizations such as the Ara Parseghian Medical Research Foundation.



Pia and Bill Park enjoying the Tucson Culinary Festival Brunch.

David and Bonnie Mehl along with **Tim and Casey Bollinger** are co-developers of the new Ritz-Carlton Golf Club, Dove Mountain. They graciously extended the opportunity to the APMRF along with three other Tucson charities to be the beneficiary of the **"Fore For Four"** Grand Opening of the new Club.

Friends of the Parseghian Foundation were honored to play this stunning new course, a few weeks prior to when the pros teed off for the World Golf Championship – Accenture Match Play Championship. It was a wonderful day of golf coupled with an exquisite evening of special food and beverages representing the restaurants that will be part of the new Ritz-Carlton Resort opening in late 2009.



David and Bonnie Mehl, and Casey and Tim Bollinger toasting the opening of the new Ritz-Carlton Golf Club at Dove Mountain.

This beautiful development including the Golf Club, resort and lovely private homes is the largest Ritz-Carlton project in all of North America, and is simply stunning.

Verna Burrows, grandmother to 12 year-old Ty who has NP-C, did another wonderful job of organizing, promoting and hosting the 4th annual **"Touch-down for Ty"** fundraising event on January 24 to benefit the work of the APMRF.



Ty Quandt having fun at the event in his honor with grandparents, Verna and Roy Burrows.

Held in Las Vegas, this event has now raised in excess of \$45,000 for NP-C research. Verna and her committee again rallied the support of individuals, businesses and organizations in the Las Vegas area to provide the community with an evening of entertainment, food and an auction.



Adam Recke announces "Start Your Engines" with encouragement from event organizer, Casey Stengel, and father Sean Recke.

Casey Stengel rallied his biker friends as well as more than 700 other motorcycle enthusiasts from Eastern Pennsylvania to take part in the 2nd Annual **"Poker Ride for Adam."** Adam Recke who has NP-C disease has won the heart of Casey who is very committed to helping in the

fight against NP-C disease. The Poker Ride raised \$11,000 in its first year and nearly \$70,000 in its second! Casey is hoping to top the \$100,000 mark this summer. Our sincere thanks to Casey Stengel and his committee for their enthusiasm and kindness.

Auction Donations Welcome

Our 2009 "One More Victory, Ara!" Auction will feature many unique items including a 2009 Toyota donated by Precision Toyota, a Windstar cruise, a European Mercedes Road Rally donated by Chapman Automotive, as well as Super Bowl and Indy 500 tickets.

We welcome donations of all kinds for the auction, large and small. The auction accounts for a large portion of our event proceeds. Giving to the auction is a way for many to donate other than giving cash.

The following are a few suggestions of items that you, your family and/or friends may have available to donate to our silent auction:

- Stay packages at hotels, resorts, private homes, etc.
- Artwork and antiques
- Jewelry and fashion
- Signed sports and entertainment memorabilia
- Tickets to national sporting or music events
- Health and beauty product baskets
- Priceless wine and other food products

We welcome your donation of auction items at this time!

Please mail the item(s) to the Foundation office by April 1, or email Peg Romano at promano@parseghian.org if you have questions or comments about the item(s) you wish to donate. Thank you for your consideration.

In Loving Memory of Cindy Wool

This issue of
"A Goal for Life"
is dedicated to our
dear friend and volunteer,
Cindy Wool.



Mark Your Calendar

April 11, 2009 – Catalina Foothills High School Student Council in Tucson will host their 5th Annual "One More Victory, Ara! 5K Walk/Fun Run" in memory of Marcia Parseghian. For information contact www.cfsd16.org/schools/foothillshigh

April 17-18, 2009 – The 14th annual "One More Victory, Ara!" Celebrity Golf and Gala Weekend Presented by University Medical Center will take place at The Westin La Paloma Resort & Spa in Tucson, AZ. For information call the APMRF: (520) 577-5106.

April 25, 2009 - Allentown, PA will be the site of the fourth annual "5K Run/Walk for a Cure" hosted by family and friends of Adam Recke, a nine year-old boy with NP-C. Call Matt Tobias: (610) 390-5602.

April 29, 2009 – Sponsored by The College of Science and The Center for Rare and Neglected Diseases at the University of Notre Dame, "Run for a New Ara" 5K Run/1 Mile Walk on campus will benefit the APMRF. Contact: Jenny at jshin@nd.edu

May 8, 2009 - Fiesta Fantastica at La Encantada in Tucson, AZ sponsored by Tucson Lifestyle and La Encantada, and presented by Chapman Automotive. A festive evening of delicious food, beverages and music to benefit the APMRF. To purchase tickets call: (520) 577-5106.

July 13, 2009 – Notre Dame Club of Milwaukee's "Ara's Outing 2009," Westmoor Country Club in Brookfield, Wisconsin. Join Notre Dame members and celebrities for golf, dinner and an auction. Contact Tom or Terry Mulcahy: 1-800-711-6132.

August 5, 2009 – The third annual "Irish Legends" Golf Tournament will take place at the Lost Dunes Golf Club in Bridgman, MI to benefit the APMRF and The Lou Holtz Foundation. For information on this fun golf outing, dinner and auction contact Angela Monger: (800) 628-9922 Ext. 503.

A CELEBRITY
GOLF TOURNAMENT & GALA WEEKEND

ONE
MORE
VICTORY,
ARA!



PRESENTED BY
UNIVERSITY
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FEATURING
AMY GRANT



April 17-18, 2009

The Westin La Paloma Resort and Country Club

Title Sponsor





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Remembering Her Twin Brother

Bob and Deb Semple, parents of Scott Semple who sadly passed away from complications of NP-C in 2005, shared a heartwarming story about their daughter Tiffany's wedding.

Scott and Tiffany were born twins, one with NP-C and the other disease free. One can only imagine the heartache Tiffany felt to see her twin die from this terrible disease, and not be with her and her husband Brian to celebrate their wedding day.

In his memory and with all those afflicted with NP-C disease in their hearts, they wanted their wedding to also be a celebration of Scott's life.

Since Scott was first diagnosed with NP-C the Semple



family has been helping raise awareness and funds in the fight against this disease. They've been wonderful partners, working in collaboration with the APMRF on many occasions.

Typical of this positive and loving family—Tiffany, Brian and the Semples decided to give the APMRF a nice donation in lieu of wedding favors and in memory of Scott. Printed on the scroll each guest received was the following:

We have chose a special way to say thank you for sharing our wedding day. In lieu of a favor, a donation has been made to Ara Parseghian Medical Research Foundation for Niemann-Pick Type C, in loving memory of Scott F. Semple. We feel the finest gift is one which helps others live longer and healthier lives. We truly appreciate your being part of our special day.

*With Deepest Love,
Tiffany and Brian
01.10.09*

"We feel the finest gift is one which helps others live longer and healthier lives."