

## SCIENCE UPDATE

Please join us in welcoming three new members to the APMRF Scientific Advisory Board.

### Ed Holson, Ph.D., Entrepreneur in Residence and Chief Scientific Officer at KDAC Therapeutics.

Dr. Holson is the director of the medicinal chemistry group in the Stanley Center for Psychiatric Research and a director of chemistry in the Chemical Biology Platform at the Broad Institute. He joined the Stanley Center to design and implement a range of biologic strategies toward developing novel therapies in central nervous system related disorders including schizophrenia, bipolar disorder, and cognitive impairment. Prior to that, Ed worked in medicinal chemistry and pharmaceutical development roles for Infinity Pharmaceuticals and Merck & Co. Dr. Holson received his Ph.D. in chemistry from the University of Michigan where he worked with William R. Roush on developing novel methods for the synthesis of complex natural products.

### Dan S. Ory, M.D., Director, Washington University Metabolomics Facility, Washington University School of Medicine

Dr. Daniel S. Ory was trained at the Whitehead Institute for Biomedical Research at the Massachusetts Institute of Technology, USA, and graduated from Harvard College and Harvard Medical School. In 1995 he joined the faculty at Washington University, St. Louis and studied cholesterol biology and Niemann-Pick disease type C (NPC). He is internationally recognized for this research and has served on numerous boards, including as Chair of the Scientific Advisory Board of the National Niemann-Pick Disease Foundation (NNPDF) in the USA. Dr. Ory is Director of the Washington University Metabolomics Facility and leads SOAR-NPC, an international collaborative group aimed at identifying biomarkers of NP-C and developing new therapies. His work has been instrumental in the development of new diagnostic NPC biomarkers, and clinical assays based on these biomarkers have been implemented in more than two dozen clinical labs on five continents. Dr. Ory has worked closely with the NIH Therapies for Rare and Neglected Diseases (TRND) team to develop clinical trials for cyclodextrin, a potential new treatment for NPC. He is a member of the Preclinical SAB for Vtesse, the sponsor of the Phase 2b/3 trial for cyclodextrin. Dr. Ory is also leading a Phase 1 trial at the NIH Clinical Center to assess the potential of histone deacetylase inhibition as a treatment for NPC.

### Suzanne Pfeffer, Ph.D., Emma Pfeiffer Merner Professor of Medical Sciences and the Professor and Chairman of Biochemistry, Stanford University.

Suzanne Pfeffer received her A.B. in biochemistry from the University of California, Berkeley and her doctoral degree in biochemistry and biophysics from the University of California, San Francisco (UCSF). She carried out postdoctoral training at UCSF before arriving at Stanford in 1984 for additional postdoctoral work. Pfeffer joined the Stanford and became a full professor in 1998. She served as Chairman of the Department of Biochemistry from 1998 to 2006, and was reappointed Department Chairman in 2013.



Ed Holson, Ph.D.



Dan S. Ory, M.D.



Suzanne Pfeffer, Ph.D.

## SCIENTIFIC UPDATE continued

Dr. Pfeffer studies the roles of NPC1 and NPC2 proteins in cholesterol export from lysosomes. She also studies the Golgi complex, which is a central sorting station for most secreted and membrane-associated proteins. This basic research is important for our understanding of the molecular basis of Niemann Pick Type C disease.

Dr. Pfeffer is a former Helen Hay Whitney Postdoctoral Fellow and Presidential Young Investigator, the recipient of a Merit Award from the National Institute of Diabetes and Digestive and Kidney Diseases and an elected fellow of the American Association for the Advancement of Science and the American Academy of Arts and Sciences. She served as president of the American Society for Cell Biology in 2003 and as president of the American Society for Biochemistry and Molecular Biology from 2010 to 2012.

## MARK YOUR CALENDAR

### March 11, 2016

The University of Notre Dame Rugby Team will host the fourth annual "One More Victory, Ara! Cup", a rugby series between the University of Arizona and the University of Notre Dame on March 11, 2016. Dedicated to raising money for NP-C research this game will be played in the San Francisco Bay Area and is played in honor of Dave Sitton, the UA Rugby coach and longtime friend of the Parseghian Family and Foundation who passed away in 2013. More information will be available by contacting APMRF (520) 577-5106.

### June 4-7, 2016

The annual "Michael, Marcia & Christa Parseghian Scientific Conference" for Niemann Pick Type C research will be held on June 4-7, 2016 at the Westin La Paloma Resort, Tucson, Arizona. Researchers will come together 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. Dr. Suzanne Pfeffer and Dr. Bill Pavan are spearheading this very important conference. For more information please contact Jenna.Rangel@nd.edu.

### June 24- 27, 2016

The fifth annual "Parseghian Classic", a 3 day golf tournament, will be held at the beautiful Pebble Beach Golf Resort. Golfers will play a round of golf at Spyglass Hill Golf Course and another at Pebble Beach Golf Links. This 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. Come join us for a weekend of golf on championship courses, enjoy the unparalleled accommodations at Pebble Beach and experience the camaraderie of this special weekend. Contact Kim Kirkpatrick at Kimberly.Kirkpatrick.18@nd.edu or go to niemannpick.nd.edu for information.



## HEARTFELT THANKS

**"Gratitude can transform common days into thanksgiving, turn routine jobs into joy, and change ordinary opportunities into blessings." William Arthur Ward**

From galas to golf tournaments, raffles to rides - caring people are hosting events throughout the year and businesses are providing goods and services. The donations given to APMRF ensure that the magnitude of NP-C research continues. We are most grateful for this remarkable support. Every dollar, every donation brings us closer to finding a treatment for Niemann-Pick Type C disease.

## THE MILLIONS FOR TUCSON — Jim Click Limited Edition Mustang Raffle



Jim Click at the Mustang Raffle Drawing.

Jim Click, owner of Jim Click Automotive, is a well-known and highly respected philanthropist in Tucson. He has once again provided Tucson charities with an opportunity to raise money through his most recent car raffle – a 50th Anniversary Limited Edition Ford Mustang. Over 484 local charities participated, raising more than \$1.2 million. The Parseghian Foundation exceeded all expectations by selling over 1600 tickets and again we are proud to be the top seller of this raffle.

We wish to thank all who purchased tickets and we thank Jim Click and family for their ongoing support and generosity. Winners of the Parseghian Foundation drawing are listed on our website ([www.parseghian.org](http://www.parseghian.org)).



**Macks Ride 2015 – July 18, 2015** For the past 2 summers, the T'ville Moose Riders, members of the Thompsonville Moose Lodge 1525, in Enfield, CT, has hosted "Motoring for Macks" a motorcycle ride in honor of Macks Thompson, age 4 who has NP-C disease. The event also included food, live music and raffles with the proceeds going to APMRF. We thank family friend, Frank Silver and Mack's mother, Laura Thompson for organizing this and we appreciate and thank all who participated. "There will be no cure without research and no research without funding."

**Enstrom Candies**, the creators of the delicious and famous almond toffee has been a highly anticipated tradition at many of the Parseghian Foundation's golf tournaments. We wish to thank, Jamee & Doug Simons, owners of Enstrom Candies – a confectionary business in Grand Junction, Colorado, started by Jamie's grandfather over 50 years ago, for their generosity in providing this sweet treat for many, many years.

## HEARTFELT THANKS continued

**United Airlines Employees** have for many years taken on the fight for NP-C disease. Beginning in 1996 when Kevin Eadie, the son of United Airlines pilot, Bob Eadie and flight attendant, Brenda Eadie, was diagnosed with NP-C. Through the incredible amount of responses from fellow employees wanting to help, APMRF qualified to be one of the agencies to be listed on the employee pledge form. Since that time the total amount donated has reached a staggering sum of well over \$1 million dollars. Sadly, Kevin lost his courageous battle with NP-C disease in November 2008 but the outpouring of generosity and support continues and provides hope to all families living with this devastating disease.



Kevin Eadie



The students of Ms. Pam Porter, Science teacher at **Lake Middle School**, Stevensville, MI, are involved in a variety of fundraising activities to support NP-C research. Since the 2014 school year they have continuously pursued fun and creative ways to raise money. Recently a group of these students met with Cindy Parseghian at the College of Science at the University of Notre Dame and presented her with a check from their recent activities. The students were taken on a tour of campus, conducted experiments in the DNA Learning Center and enjoyed an astronomy presentation at the Digital Visualization Theatre. We thank them for their dedication and enthusiasm to take the time to make a difference in the lives of those suffering from rare diseases.

**The Greater La Grange YMCA of Metro Chicago** helps their community and a variety of organizations each year through sale of Christmas trees. For the past 9 years APMRF has received a donation that was inspired by local resident, Mrs. Lorraine Donegan whose grandson, Blake Donegan, age 19, suffers from the ravages of NP-C disease. We thank the Y Men's Club for their support of the Donegan family and for their longtime support of the Foundation.



**UNDER ARMOUR.**

**Under Armour** – As we prepare each year for the Parseghian Classic Golf Tournament we reach out and ask for gifts for our attendees...for the past 2 years Under Armour has graciously provided a wonderful variety of wearables for both the men and women that were enthusiastically received by all of our guests and added great comfort as well as style to the weekend. We can't thank Under Armour enough for their help and generosity in making this event so successful.



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## A GOAL FOR LIFE

For Friends and Supporters of the Ara Parseghian Medical Research Foundation



*The gift of love.*

*The gift of peace.*

*The gift of happiness.*

*May all these be yours this holiday season.*

*Dear Friends,*

*During this season we look back on the many acts of kindness that we have received throughout the year. We take this time to reflect on all that we are thankful for and count our many blessings.*

*This newsletter is dedicated to you....and we wish you a heart filled with peace, love and hope in the New Year.*

*The Parseghian Family*



**"Christmas is not as much about opening our presents as opening our hearts."  
Janice Maeditere**



### TEMMERMAN/DONOVAN FAMILY CHRISTMAS



Every year during the holiday season, APMRF receives an envelope of donations from the Temmerman/Donovan family in honor of Christmas. In lieu of gifts to each other they have chosen to give a gift to support NP-C research. Judy & Joe Donovan, friends of the Parseghians since their college days at Notre Dame, have reached out to their family members Mary Jo, Bob, Trese and Becky and father, Bob, Sr. Since the beginning of the Foundation they continue to be ardent supporters. We are so grateful and appreciative of their friendship and thoughtfulness.

"As the holidays unfold around us we are reminded of that which is most important....family, friends and the wish to make the world a better place. The Parseghian family holds a very special place in our hearts not only during the holiday season but all year long....and our greatest hope is to find a cure for Niemann Pick Type C disease." Judy Donovan