

# A GOAL FOR LIFE

For Friends and Supporters of the Ara Parseghian Medical Research Foundation



Dear Friends,

We are pleased to announce a new partnership with the University of Notre Dame which will help to ensure APMRF's momentum in pursuing a treatment for Niemann Pick Type-C disease. Notre Dame has launched the Ara Parseghian Medical Research Fund which will take on the administrative functions of the APMRF including fundraising, recruiting of researchers world-wide, grant administration, marketing, interaction with other NP-C families, and hosting of the annual science conference. The Parseghian family will continue to be intimately involved with the fundraising efforts and will act as advisors to the new Fund. A great deal of attention has been focused on this transition to ensure that the work we started over 22 years ago continues advancing the field of NP-C research.

Sean Kassen has been appointed the Director of the Fund at the University of Notre Dame. Sean is uniquely qualified for this position. Sean has been the Academic Advancement Program Director for the ND College of Science for the last 6 years. In this role, he has overseen Greg Crawford's 5 bike

rides for NP-C research and has been instrumental in raising over \$2,000,000, including \$1,200,000 in 2015. He has also been involved in our annual Pebble Beach golf tournament and rugby events which have cultivated new donors and supporters for NP-C research. Through these and other fundraising events, Sean has had the opportunity to meet many NP-C patients and families. Meeting with NP-C families has had a lasting impact on Sean and his desire to make a difference in their lives. Sean has a PhD in genetics which adds to his understanding of NP-C disease and the challenges of research. We could not hope for a better individual to take on this leadership role.

We can look back at the last 22 years and recognize the huge gains that have been accomplished in NP-C research. We have been blessed with incredible financial supporters who have contributed over \$42 million towards research. This has given hope to all NP-C families by unraveling many aspects of the disease and developing 3 potential therapies. In addition, our efforts have expanded research into other lysosomal storage disorders, and increased the scientific understanding of other neurodegenerative diseases and cholesterol related disorders.

We have had one drug, Zavesca, approved in 27 countries, including Canada and Europe, although not in the USA. Another drug, cyclodextrin, was recently granted "Breakthrough Therapy" designation by the FDA. Cyclodextrin is currently in a Phase II / III study and is showing a great deal of promise. This "Breakthrough Therapy" designation is given to help accelerate development and review of drug candidates only when preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies. And, a third drug, an H-DAC inhibitor, is also in a clinical study. When we began the Foundation in 1994, very little was known about the disease. Our efforts have shed great light on the disease to the point where it is now supported by the National Institutes of Health as one of its TRND projects, injecting millions into the research efforts every year.

It has been a long arduous journey since we began the APMRF. It is with deep gratitude that I extend my thanks to each of you for the role you have played in making the Foundation a success. Your devotion has made an enormous change in the understanding of NP-C disease and has brought hope to NP-C patients and their families. I hope you will continue your support by backing the work of the new Fund at Notre Dame.

Forever grateful!  
The Parseghian Family

# WELCOME Sean Kassen, Director

## Sean Kassen, PhD Director of the Ara Parseghian Medical Research Fund

Sean Kassen is the Director of the Ara Parseghian Medical Research Fund at the University of Notre Dame. In this role he will be responsible for administering all of the functions of the Fund which includes advancing Niemann-Pick Type C (NPC) disease research, fundraising, managing the grants program, and raising awareness of NPC disease.

He has been intimately involved in our efforts to find a cure for NPC disease for the past 7 years. Prior to this new position, he was the Director of Academic Advancement for the College of Science at the University of Notre Dame where he worked directly with the University Relations office to externally promote the priorities of the College of Science to individuals, companies, foundations and NP-C families. Additionally, he coordinated many events including the Road to Discovery cross-country bike ride, the Parseghian Classic and the Parseghian Cup Rugby Match to raise awareness and funds for NPC disease.

Sean also possesses a background in Biology, having received his Ph.D. in cell and molecular biology from the University of Notre Dame. He has published ten manuscripts in leading peer-reviewed scientific journals, ranging in topics from identifying the mechanisms behind retinal development and regeneration to using zebrafish as a model for developing genetic tools. As a graduate student he also taught an undergraduate genetics lab for four years and trained many students, four of whom have been coauthors on publications. Prior to attending Notre Dame, Sean conducted research for Evanston Hospital in Evanston, IL, identifying causes of a rare premature infant disease called necrotizing enterocolitis.

He received his undergraduate degree in biochemistry from Alma College in Alma, MI. Sean currently lives in Granger, IN and is married with two daughters...who run his life!



## Science Update



### **Vtesse, Inc. Announces FDA's Granting of Breakthrough Therapy Designation for VTS-270 in Niemann-Pick Type C1 Disease**

Vtesse, Inc. announced on January 6, 2016 that the U.S. Food and Drug Administration (FDA) has granted its drug candidate, VTS-270 for treatment of Niemann-Pick Type C1 Disease (NPC), Breakthrough Therapy designation status. Both the FDA and the European Medicines Agency (EMA) had previously granted Orphan Drug status to VTS-270, which is currently in a pivotal Phase 2b/3 clinical trial. VTS-270 is a form of cyclodextrin.

The FDA grants Breakthrough Therapy designation to companies to help accelerate development and review of drug candidates when preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies. The designation is designed to ensure that patients can benefit from therapies as soon as possible, without changing FDA standards for new drug approval.

The Ara Parseghian Medical Research Foundation (APMRF) supported the researchers responsible for the ground-breaking recognition of cyclodextrin as a potential therapy for NPC. The APMRF contributed several million dollars to the research and development of this promising drug.

"It is both rewarding and validating to receive the FDA's Breakthrough Therapy designation for VTS-270, which we believe may provide the first effective treatment for slowing the progress or stabilizing the devastating impacts of NPC in children and adolescents," said Ben Machielse, Drs., President and Chief Executive Officer of Vtesse, Inc. "This designation is supported by strong preclinical and early clinical data with VTS-270, including that from the Phase 1 study conducted by the National Institutes of Health (NIH). It is our hope that this designation will help to expedite the development and regulatory review process, getting the drug to patients who can benefit sooner."

The FDA Breakthrough Therapy designation comes as Vtesse is in the midst of conducting its pivotal Phase 2b/3 clinical trial of VTS-270 for treatment of NPC. Vtesse expects to enroll a total of 51 patients at up to 20 sites (across the United States, the European Union, and other countries) to participate in this clinical trial. For more information on Vtesse's pivotal Phase 2b/3 clinical trial, visit [www.theNPCstudy.com](http://www.theNPCstudy.com).

"Breakthrough Therapy designation means that the FDA will provide intensive guidance and an organizational commitment to expedited development of VTS-270 for the treatment of NPC," said Dr. Johnson. "These multidisciplinary interactions with the FDA will support Vtesse's efficient design and conduct of its regulatory strategy with the goal of driving VTS-270 towards FDA approval."

Vtesse, Inc. is a rare disease company dedicated to developing drugs for patients suffering from diseases that are underserved. Vtesse is working collaboratively with the NIH and other leading academic centers to advance clinical study of VTS-270 for NPC, and to conduct pre-clinical discovery and development of other novel drugs for NPC and other lysosomal storage diseases (LSDs).

To experience what it is like to be a patient in the VTS-270 clinical trial go to: <https://vimeo.com/160183927>

## Heartfelt Thanks



The 4th annual **Parseghian Cup Rugby Match** was played between the University of Notre Dame and the University of Arizona rugby teams on March 12, 2016 at Steuber Rugby Stadium in Stanford, CA. The focus on the field was on rugby but off the field the coaches and players shared their commitment to raising funds to help fight Niemann Pick Type C disease. The UA Wildcats prevailed but the real winner was the outcome - raising over \$30,000 for NP-C research.

Many thanks to UND Coach Lonnie Heeter, UA Coach Sean Duffy and to all of the players for taking the time and effort to continue this event that was lovingly launched by Dave Sitton, a highly respected, and greatly missed UA Rugby coach who passed away suddenly in 2013.

We extend our sincere appreciation to Phil and Susan Calandra who organized this spirit-filled vent and to the many sponsors who helped to make this a great success.

**Tucson Orthopaedic Institute**, a Tucson based group of orthopedic surgeons and their staff, have supported their colleague Mike Parseghian and his family since 1994 when three of their four children were diagnosed with NP-C disease. This dedicated group again raised funds through a variety of fundraisers in 2015 and we wish to thank them for their caring support that provides hope for all families who are living with this challenging disease.



### Infiniti Coaches Charity Challenge

We wish to thank Infiniti for their dedication to supporting charities through this opportunity. For 6 years, the Infiniti Coaches Charity Challenge has provided NCAA college basketball coaches with the chance to win significant dollars for the charity of their choice. Purdue University basketball coach, Matt Painter, has participated for the last 4 years, representing the Smith Family BReaK Thru Fund, a fund held at the Parseghian Foundation. Through the many votes that have been placed by NP-C families and supporters, \$135,000 has been raised for NP-C research. Our sincere thanks to Coach Painter for his enthusiasm throughout the Challenge and for his dedication and support of the Smith family and the hope he has given to NP-C families everywhere.

### Students Who Care

The Stanley Clark School in South Bend, IN, has been a longtime friend of the Foundation. Since 1998, the students have participated in a variety of fundraising activities and have donated these funds to APMRF. Their continued efforts are greatly appreciated and we thank them for their thoughtful and compassionate support.



**“How beautiful a day can be when kindness touches it.”**

George Elliston

## Mark Your Calendar



### May 8 – 11, 2016

This year's Road To Discovery bike ride, in keeping with the ambitious legacy set by Greg Crawford, is being organized by the University of Notre Dame's ESTEEM Program; Engineering, Science and Technology Entrepreneurship Excellence Master's Program. This 4-day bike ride, with a team of 16 cyclists, will begin in Louisville, KY on May 8th and will conclude in Notre Dame, IN on May 11th. The goal is to raise \$150,000 for NP-C research. For information go to: <http://niemannpick.nd.edu>

### 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, contact Kimberly.Kirkpatrick.18@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](http://niemannpick.nd.edu) for information.

### July 23, 2016

The "15th annual Kevin Eadie Night at the Peninsula Pilots" will be held at the War Memorial Stadium in Hampton, VA. This event is held in loving memory of Kevin who touched the lives of many and left a tremendous legacy to all who knew him. An enthusiastic baseball fan and the Peninsula Pilots Batboy from 2002-2008, Kevin's spirit continues to live on in the hearts of this team. Kevin passed away from the complications of NP-C disease at the age of 14. This event, organized by his mom, Brenda Eadie, continues the quest to find a cure for this life robbing illness. For information about this event, contact Brenda Eadie at [Kevinsmom@helpkevin.org](mailto:Kevinsmom@helpkevin.org). Go Pilots!





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