

Road to
DISCOVERY
Riding for the Lives of Children



Road to Discovery Blog

Riding for the Lives of Children

2,200 miles

26 Days

25 Cities

12 States

2 Bikes

1 Goal

A Goal for Life

BOSTON
6.13.11

DALLAS
7.8.11

Greg Crawford, dean of the College of Science at the University of Notre Dame, wrote the following blog entries each day of the 2,200 mile trip. He and his wife, Renate, rode to raise awareness and funds for Niemann Pick Type C disease, a neurodegenerative disease that usually affects children.



The 2,300-mile Desert to Dome bike trip last summer from Tucson to Notre Dame was so inspiring that Renate and I decided to ride again. This time, we're bringing awareness of Niemann-Pick disease, the devastating toll on families and the determination to defeat it through research, to another corner of the country. We'll be cycling from Boston to Dallas via New York, Philadelphia, Washington, Richmond and Nashville. On this "Road to Discovery," we'll meet families, researchers, doctors and Notre Dame friends and develop a video to help spread the word about research to find a cure or treatment for NPC.

Desert to Dome, dramatizing the strengthened partnership between Notre Dame and the Ara Parseghian Medical Research Foundation, was an amazing experience. Working with the Parseghians, who have transformed their personal loss into hope for thousands, is a great privilege. Cindy and Mike's son, Ara Parseghian, who is a medical student in Boston, will join us this time for the early leg of the ride — the best sendoff I can imagine.

The support of the Notre Dame family will also be with us as we take this trip. President John Jenkins and Provost Tom Burish, as well as my colleagues and staff and the alumni network, are always a great encouragement. We like to think the ride is another piece of the great Notre Dame commitment to healing and serving in the world, like the work of our students in Haiti and Uganda and at the Center for the Homeless and Hospice in South Bend and so many other places.

I was so moved after last year's ride when Notre Dame students Michael Dean and Joe Magro came to me to share their passion for

this cause and their determination to raise awareness. Michael ran the Boston Marathon and raised money for the Foundation. Joe and the Notre Dame biking team dedicated their race to raising money for NPC. What incredible students we have at Notre Dame! Their individual and collective efforts, their unity of purpose, their passion and compassion for humanity show the world the true fighting spirit of our University.

This road goes through some bigger cities and some higher mountains than we encountered last year. We hope you'll join us here, day by day, and spread the word so the cure will come soon to end the suffering of these precious children and their families.

Thanks so much for being a part of this life-changing mission.

**— Greg Crawford,
Dean of the College of Science**

“

Next to the children and families who inspire us all, we find ourselves energized in the fight against NPC by the dedication and determination of the researchers that are on the front line for us.

”

We had the perfect day today! The weather was a bit chilly at first, but then it turned cool and sunny, and we had a favorable wind most of the day. The start was very special as we departed from Ara Parseghian's home in the Boston area. Ara is the grandson of Coach Ara Parseghian, one of Notre Dame's most successful football coaches; the son of Cindy and Mike Parseghian; and the brother of Michael, Marcia and Christa, whose diagnosis with NPC led to the creation of the Ara Parseghian Medical Research Foundation and who still inspire our efforts. Getting to know Ara and his wife Cicely was such a pleasure, and it was very special to have Ara bike with us on this first leg. He's a strong biker and runner — he finished the Boston Marathon this year. Wow, what an accomplishment! What a privilege to have him as part of our ride. We left Ara and Cicely's home around 9 a.m. and arrived in Providence around 1 p.m. Apart from a detour in Pawtucket, Rhode Island, the day was flawless. Thank you, Ara, for riding with us — we really enjoyed being with you.

day 1





day 2

We had a really wonderful day with families. Judy met us at our hotel in Mystic. Like most people, she seemed impressed by the brightness of the van — the matching vehicle and bike gear

attract a lot of attention. Judy shared her stories and a picture album of Bryanna with us. In all the photos, Bryanna was smiling, being silly and living life to the fullest. We learned about their travels, about Bryanna's creativity and about her love of ballet, music and being with friends. Judy had some wise advice for other families: "Keep on living." Little things like vacations and time spent with family and friends are so precious. We feel very honored to hear about these precious children and their love for life, and they inspire us so much! We continue to be so inspired by the families who have children fighting NPC or who have lost children to NPC. Their strength and perseverance are incredible. The families who have lost their children continue to support other NPC families and to be actively involved in the fight to find a cure so no other family will have to go through what they suffered. We've always seen this so clearly in all the families we met in the last few days share that dedication and generosity.

June 14, 2011

Connecticut Parents Committed to the Cure

“

Judy had some wise advice for other families: “Keep on living.”

”



Judy

I can't imagine a more inspiring way to spend my summer than meeting with researchers, physicians, health-care professionals and parents of NPC children. After meeting so many families on our first few days of our trip, I thought about the NPC families I met at the 2011 Michael, Marcia, and Christa Parseghian Scientific Conference for Niemann-Pick Type C Research last week at Notre Dame. Their stories, so fresh in our minds, will keep us going all the way from Boston to Dallas. What the families gave us is even greater, and not just the wristbands we wear every day — the blue "A Goal for Life" from Cindy and Mike and the Ara Parseghian Medical Research Foundation, the red "Race for Adam" from Sean and the Race for Adam Foundation, the light blue "Persevere" from Karen and Gene and the National Niemann-Pick Disease Foundation. The children have always been our

inspiration in this fight, and now we have more names and faces for "the children" — Ty and Riley and Keaton and Braden and Adam and Natalia and Marcela and Caroline and Roy and Murray and Isaiah and Joshua and Dana and Andrew and Scott and Graham and Erin and Anna and Michael and Marcia and Christa, and many others.

I am so fortunate to have met these wonderful families. They are our partners, our friends, and their hope, their faith and their dreams for their children keep us inspired and motivated, at the University of Notre Dame, in the College of Science, and on this Road to Discovery.

day 3

Mystic, Connecticut to Long Island, New York



June 15, 2011

day 4



Today I had the opportunity to visit Cold Spring Harbor Laboratory (CSHL) on Long Island. John, one of my advisory council members, has been instrumental in setting up an internship for Notre Dame students at the world-famous Cold Spring Harbor Laboratory each year. The institution's collaborative approach to research, covering everything from cancer and plant biology to neuroscience, quantitative biology, and bioinformatics and genomics, is a cutting-edge example of how to solve big problems by bringing scientific minds together across disciplines. The research is aimed at giving us better tools to diagnose and treat such illnesses as cancer and neurological disorders.

I always tell our students that we are seeking to train them to be creators of knowledge, not just absorbers of it. There is no better way to be a creator of knowledge than to work as a researcher at one of the top laboratories in the world.

After the Cold Spring Harbor Laboratory visit, we attended a Notre Dame event, hosted by John and Heidi at their beautiful home on Long Island, with about 30 Notre Dame alumni, parents, and Cold Spring Harbor Laboratory researchers. It was a wonderful time. We spoke for about 15 minutes to the group about the ride for Niemann-Pick Type C, our partnership with Cold Spring Harbor, and some of our new research and pedagogy programs in Notre Dame Science.

Our first stop today was at the University of Pennsylvania to visit the Vite Laboratory. Dr. Paula met us at the School of Veterinary Medicine, and two NPC families came — Sean and Amy with their children, Katie and Adam, and Brian and Kim with their children, David and Andrew. Adam and Andrew have Niemann-Pick Type C. The families had not met each other before, and we were very happy to get to know them. Adam and Andrew were so outgoing, friendly and kind, and it was wonderful to spend time with them. The boys hit it off right away, and it was touching how Andrew took to Adam. Both boys are sports enthusiasts. Adam loves to play soccer and football, and Andrew, who had been a pitcher, loves to watch baseball. They both root for the Phillies. These boys have so much spirit and positive attitude — we were very impressed.

day 5

While Renate spent time with the families, Dr. Paula took me on a tour of the genetics laboratories. I met with the Niemann-Pick Type C researchers — Jessica, Maria, Su, Tracey, Therese, and others. They are doing amazing work on cyclodextrin to stop the neurological progression of NPC. The results are very promising in their animal model. They were very passionate about their research, deeply focused on the mission of their work, and truly excited to be part of such an important project.

Thank you to both families for coming out to meet us. The perseverance, love and positive attitude that we see in the children and

their families always inspires us. Your energy keeps us going, and we will carry your inspiration and your stories to Dallas and beyond; they are so uplifting, full of hope and spirit.

We try to put our experiences with them into words, but words alone cannot convey how amazing these people are.





day 6

Another excellent ride today — nice weather, warm temperatures and favorable winds adding up to a smooth first six days. We covered four states in one day — New Jersey, Pennsylvania, Delaware and Maryland — spanning a little bit of everything from ocean views to pine trees to corn fields to marshes, little villages and a nuclear power plant.

Today, we visited Dr. Denny Porter, who is performing a Niemann-Pick Type C clinical study, at the National Institutes of Health (NIH) in Bethesda, Maryland. Darille and Mark joined us there with their son Dillon, who has NPC, along

with Dillon's grandma. Dillon is outgoing and loving, with a strong compassion for others. Dr. Porter is conducting a clinical study with Niemann-Pick Type C children that started six years ago. He has a terrific nurse practitioner, Nicole, who coordinates the study. It was very clear to us why the children love coming to the NIH — they look forward to their visits with Dr. Porter and Nicole. Dr. Porter's National History Trial is looking for biomarkers that can ultimately help track the effectiveness of drugs and therapies, as well as a more rapid way to diagnose Niemann-Pick Type C. We were so impressed by the facilities and labs at the NIH, but even more so by the dedication of Dr. Porter and Nicole. Watching their interaction with Dillon, it was clear that we were seeing so much more than clinical science — this is the human and compassionate side of healthcare.

“

It is the energy that we gain from these children and families that carries us on to our next stop and all the way to Dallas, so we may tell their story all along the way and beyond.

”



Dillon & his family



day 7

We rode the first 40 miles today in a sprinkling rain that felt pleasant in the warm air. Then the sun came out for the rest of the day. Nearly a week into the ride, we had our first and second flat tires — both times it was Greg's front tire.

After arriving in Fredericksburg, we drove back to Washington, D.C., through the heavy traffic on Interstate 95 that we had forgotten in the quieter parts of the country. We had a great evening at the home of John and Mary. They also invited Norb and Linda, and Paul, Dana, Phil and Ed. Norb is on the Science Advisory Council and actively involved with Niemann-Pick research developments.

Just seven days ago, we started in Boston with Ara. We had a phenomenal week meeting with the families and NPC children. On the way, many of them have given us necklaces, wristbands and t-shirts associated with their own fundraising and awareness campaigns for NPC in their local communities. We are very touched and honored to know these members of the big team fighting NPC, and we proudly wear these items on the ride, showing them off and telling the story that goes with each one.

We left Fredericksburg today in the rain, and the weather stayed steady the entire way down to Richmond. We were soaked through and through, but the landscape was beautiful. We would have seen more if we had windshield wipers for our glasses, but I think we saw every shade of green along the road today, including Kelly green! Very few cars interrupted our tranquil surroundings until we entered Richmond, and by then we were close to our hotel. Tomorrow we are expecting a hot, sunny and humid ride — typical Southern weather.

This evening, Chuck met us at our hotel to tell us about his granddaughter Ashton, who has Niemann-Pick. He affectionately told us about her love of family, friends and school – she prefers school over summer days. Ashton deeply cares for others, and she and her younger sister are very protective of each other. It was great to hear about such a sweet girl. He mentioned some of the fundraisers at which he helps raise money for NPC research within the framework of the Ara Parseghian Medical Research Foundation. One is a BBQ chicken dinner, a very successful and growing event. Students at Ashton's previous school hold a walk-a-thon for her every year. Chuck was very clear that the answer lies in research, something we also firmly believe.



day 8

June 20, 2011



day 9

Today we followed the route of “Lee’s Retreat” from Richmond into Appomattox Court House. We packed a good amount of Civil War history into our day. We were able to go into the McClean house where Robert E. Lee surrendered to Ulysses S. Grant.

It was fascinating to walk around there, and see a snapshot of how things were in 1865. Afterwards, on the road, we encountered some nice rolling hills, so our legs got a good workout. This evening we met with Dwayne, who told his about his 15-year-old son Raiden with NPC. Raiden is a real jokester with a vibrant personality; his numerous friends care for him a great deal and often come by the house. They seem to understand better what he goes through than some adults. Until he was 10 years old, Raiden played Little League. He always wanted to be a pitcher, and at one of his games, the umpire gave him that opportunity by moving the mound closer to home plate. He was able to get the count to full, and the final pitch was “Strike Three!” One of his favorite things to do is go watch the Mariners, the local minor league team Through the Make-A-Wish Foundation, he was able to have a

very memorable trip to watch the Dallas Cowboys play.

We have heard lots of stories of how families organize fundraisers for Niemann-Pick. Dwayne has a unique approach to fundraising. He started Fishing for Charities, a bass fishing tournament trail in Virginia, North Carolina and Tennessee. Every competition on the trail supports a different charity, with at least one competition for Niemann-Pick every year. Dwayne hopes that this bass fishing tournament trail continues to grow and that others will start such events in their parts of the country, so all may contribute to NPC. Thank you, Dwayne and Raiden, for all your hard work raising money and awareness for NPC. Every day, we are in awe of the collective work of parents, families and communities raising money for research in search for a cure or therapy.

Today we had a very challenging ride on rolling hills, punctuated by several encounters with dogs that were enough to give even dog lovers like us some second thoughts. Even though the terrain was hilly, the scenery was just gorgeous. Fortunately, our friends at Knollwood Country Club in Granger — Kelly, Greg, Tom and Cheryl — had us prepared for the hills.

Today was the first evening on the trip when we did not have an event or meeting scheduled. We stayed with our Destination Cycling expert Peter's brother Tim and Tim's wife Susan in Winston-Salem. Peter's sister Lizzy took us to the pool for a nice relaxing swim. They were all very hospitable. We had a wonderful evening, fabulous food, great conversations and many laughs. Thank you.

Today was a day of reflection — not having meetings tonight enabled us to think more about our trip and what is going on at Notre



Dame. The NPC children, of course, are the chief inspiration for our ride, the faces we remember when the pedaling gets tough on the road in the summer heat and the dogs show up. But we are surrounded with so many kinds of inspiration, including the terrific researchers at Notre Dame. For example, I am very proud of the work in Professor Paul Helquist's laboratory in Chemistry and Biochemistry that is making great strides against Niemann-Pick Type C. In addition to Professor Helquist, the team includes undergraduates Nathan Farley, Tyler Hulett, Christopher Mariani

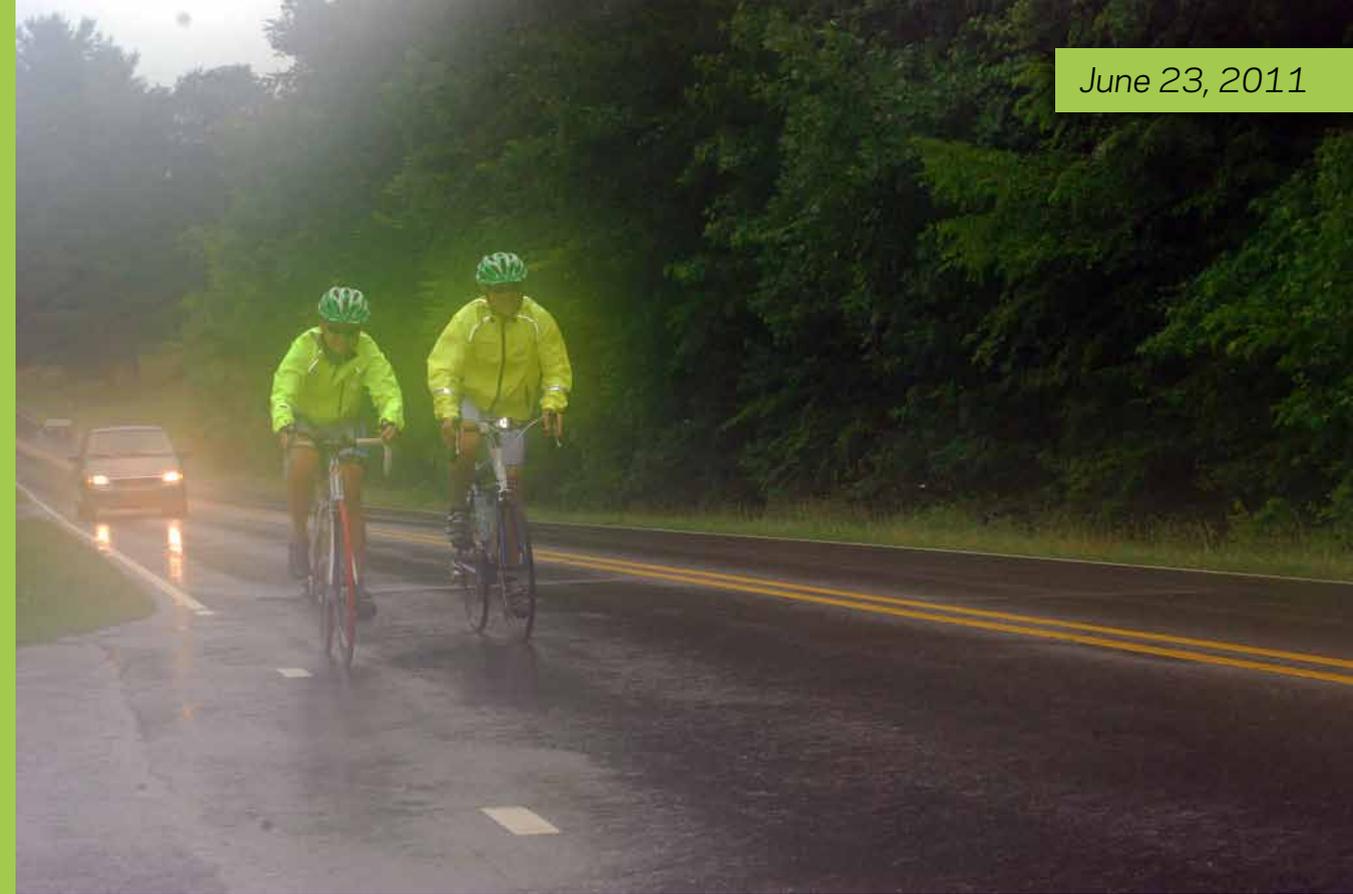
and Luke Westby as well as graduate students Pauline Bourbon, Katherine Byrd, Casey Cosner, John Markiewicz, Jacob Plummer and Douglas Schauer. It is great to be a small part of such a large overall effort to fight NPC. In the homes where families care for the wonderful children, in the labs where the scientists are discovering such hopeful drugs, in the clinics where trials are moving us closer to the goal — and on the Road to Discovery where we make our uphill climbs (and fend off dogs) — each of us contributes what we can to the common cause.

day 10

Today was wet, to put it mildly. After a taxing ride on Wednesday, up and down hills in the heat of the sun, we were a bit sore and sluggish to start the day. First there were on-and-off rain showers, and then they just stayed ON. The rest of the day, the heavy downpours continued, making the overall ride very challenging. We are looking forward to Asheville because we have heard that it is beautiful, but most importantly because our youngest daughter will be joining us there to become part of the support team. She feels strongly about being a part of the effort to raise awareness of Niemann-Pick and to help with the drive for a cure.

This evening we had a nice dinner with Dick and Barbara in Hickory. Dick is a Notre Dame alumnus, and many of his children are also Domers. We had a lot of great conversations about the ride, the cause, dentistry, economic development and entrepreneurship. After a day like today, it was certainly nice to sit down for a good meal and pleasant talk. Thank you so much for a wonderful evening.

So many people at Notre Dame have joined us in the fight against Niemann-Pick Type C, each with different skills and different interests, all working together for the same goals. Tyler Hulett, who graduated last year and took my course on scientific



entrepreneurship when he was a junior, has done amazing work in a behind-the-scenes task involving a test for drug effectiveness against NPC, and also works with Professors Paul Helquist and Holly Goodson and Dr. Norbert Wiech to advance the application of epigenetic medicine to NPC. Like the rest of us, Tyler is moved with compassion for the children and families suffering with

NPC. He knows that clinical trials take time. The need is urgent. To Tyler, that makes his contribution all the more significant and rewarding. Each of us is doing our part in this fight. For today, mine is riding a bicycle a few more miles, even when it's raining, and Tyler is hard at work in the laboratory. Thanks, Tyler, and best of luck next year in

day 11

“

The need is urgent.
Each of us is doing
our part in this fight.

”

The sun came out to greet us again today. After a soaking wet day yesterday, we were very pleased to have favorable weather conditions. We entered the Smokies today — they were everything people told us they would be, and more. At some point, Old Hwy 70 ended and left us to detour on a dirt road that went up the hill switch-back style. It was quite the adventure. You could say we took “the road less travelled” and “the path of most resistance.” The effort was rewarded, though, as we rode through Sandlin Park. Having beautiful scenery certainly makes a huge difference on these physically challenging days.

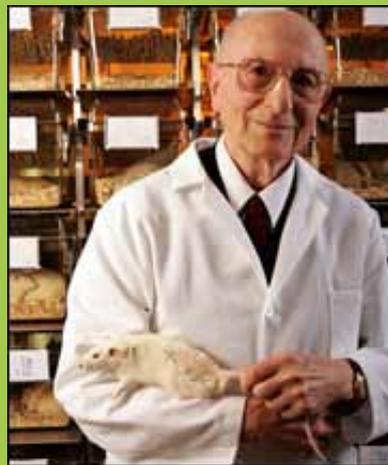
We don't take short cuts on this bike ride, but a short cut in Niemann-Pick Type C research could get help to children much quicker than expected. The laboratory of Professor Olaf Wiest in the Department of Chemistry and Biochemistry at the University of Notre Dame is using a drug repurposing approach to develop drugs for treating NPC and other rare diseases. The aim of the approach is to identify small molecules that are already FDA-approved or in late-stage clinical trials for other diseases and use them for rare diseases, including NPC. We're going the distance in the fight against NPC — and if we find short cuts to help the children along the way, we'll take them to speed up development efforts.

Hickory to Asheville: Sunny Skies, Steep Slopes



day **12**

June 24, 2011



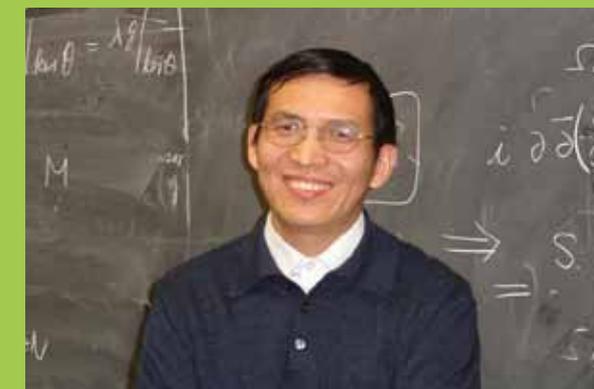
day 13

The Notre Dame family has suffered two very significant losses while we have been on the road. Professor Morris Pollard, one of the true Notre Dame legends in the Department of Biological sciences, and Professor Jianguo Cao, a distinguished mathematician who had been with us for 15 years, passed away within the last week. It is always hard to lose a colleague, and these great scholars and teachers will be sorely missed in their departments, in the college and across the University, as well as in their larger fields.

Professor Morris Pollard was 95 years old and had been at Notre Dame for 50 years when he passed away on June 18. Morris was professor emeritus of biological sciences and director of our Lobund Laboratory. When he — a prominent Jewish researcher — came to Notre Dame — a Catholic university — he helped establish our community as a place where people of different backgrounds could join in the common purpose of serving humanity. In addition to directing medical research at Lobund, he published more than 300 scientific articles and developed a unique breed of germ-free “Lobund-Wistar” rats to study the mechanisms of disease. His research on virology and cancer led to bone marrow transplants to treat leukemia and sarcomas, and the American Cancer Society bestowed its Hope Award on him.

Professor Jianguo Cao, a native of China who came to us from Cornell in 1996, was only 51 years old when he passed away on June 23. His career was tragically brief, but remarkably productive. He published more than 30 research articles, authored a book on Riemannian geometry, and delivered more than 100 lectures. Jianguo was a world expert in his field, differential geometry, and often spoke at international conferences. He received a Changjiang Chair at Nanjing University in 2007. He was a visiting member of the Institute of Advanced Studies in Princeton, the Mathematical Sciences Research Institute in Berkeley, Max-Planck Institute for Mathematics in Leipzig, and Institut des Hautes Etudes Scientifiques in Paris, as well as a visiting professor at the University of Michigan and at Capital Normal University in Beijing. In the Department of Mathematics, he often served on departmental committees and in recent years ran the colloquium and short-term visitor programs.

Like any family, when we suffer loss, we all mourn — and we find strength in each other. We have been riding for nearly two weeks, with miles to go, to do our small part in the fight against NPC. We feel the support of the Notre Dame family that responded so strongly when this disease brought loss to the Parseghian family. We want the Pollard family and the Cao family to feel that same kind of solidarity from us and all of the Notre Dame community. Our sincere sympathy and condolences go out to the families, friends, and colleagues of Professor Morris Pollard and Professor Jianguo Cao.



day 14

Today we added a new member to the support team — our daughter Ally, who flew to Asheville over the weekend to be with us. She has learned a lot about NPC, and because it affects children her age, she wanted to be a part of the trip. So we brought her down for the final two weeks. On Saturday night, Greg had dinner with John and his son Joseph, both ND alums, and Ann and Robert, ND parents, at the home of John's friend Bo in Waynesville, North Carolina. It was a great evening of conversation. John told us about his path into Notre Dame, and Ann and Robert told us about their four children who graduated from Notre Dame. Now they have a grandchild there. We continue to hear wonderful stories from our Notre Dame parents and alumni.

Well, today's ride started off beautifully, sunny and uphill and down, and through a long tunnel. Toward the end, however, a pouring rain reduced visibility and made for a tough final 20 miles. The good news today is that this marks the halfway point in our ride. We have had a fabulous experience so far, meeting the families and children with NPC — the really special part of this ride for us — along with researchers and health care professionals. We are all in this fight together to find a cure or therapy for NPC. It has been a real pleasure to get to know so many people on this great team fighting against the disease. Their passion and commitment is incredible — it comes out in our

meetings with them time and time again. Those new friends are in our hearts and minds, and the memories of the meetings and discussions are on our wrists and around our necks. So many people have given us NPC bracelets and necklaces, and we wear them all the time. When the hill is steep, or the deer is really close, or the barking dog is chasing us, the summer heat or the rain is pouring down, one glance at

the collection renews our energy and keeps us going. They're visible reminders of each one who gave them, and of all those with us on this drive to cross the goal line. We look forward to taking you all with us on the second half of our Road to Discovery.



“ We showed our Irish pride by wearing Notre Dame athletics t-shirts in the evenings. ”

e ride
2200
miles can make
a world of
a difference



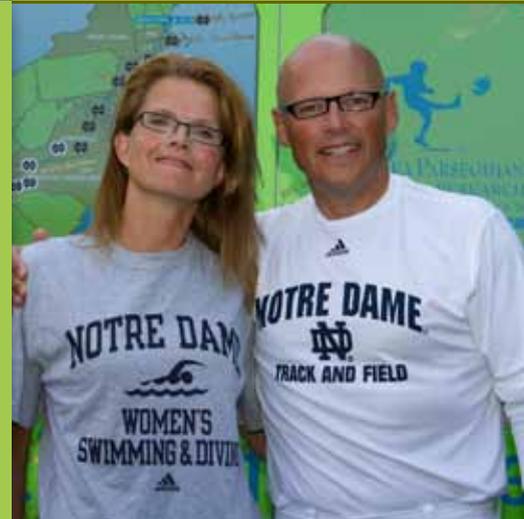
Cindy, Mike - How
You Journey with
Been Quite a Ride
I love you All



Persevere!
Thank you,
Karen Quandt



RIDE LIKE
A CHAMPION
TODAY!





day 15

Today was a challenge – a short day in terms of miles, but about 25 of them were up and over the mountains in Great Smoky Mountains National Park. We left Bryson City very early, passed by the Cherokee Reservation, and climbed up the Smoky Mountains to more than 5,000 feet. It was a tough ride up because of the relentless incline, but the views were spectacular — from whitewater rapids to mountaintops and gaps.

This evening we had dinner with Susan,

a Saint Mary's alumna, and John, a Notre Dame alumnus and double Domer — also Notre Dame parents. John wanted to know how Coach Ara Parseghian was doing, and we told him that he was just great — he spoke at our Niemann-Pick conference at Notre Dame a few weeks ago and gave the scientists a great pep talk.

It was a wonderful opportunity to reflect on the conference. As many already know, the College of Science recently hosted the 2011 Michael, Marcia, and Christa

Parseghian Conference on Niemann-Pick Type C Research, the first of a series of conferences permanently endowed at Notre Dame by the Ara Parseghian Medical Research Foundation. More than 80 researchers, students and NPC-affected family members from around the world attended the three-day event. Researchers told about hopeful discoveries and potential progress toward a drug treatment, and NPC parents told inspiring stories about their remarkable children.

Coach Parseghian, grandfather of Michael, Marcia and Christa whose NPC diagnosis in the 1990s inspired the foundation, kicked off the event. He thanked researchers for the remarkable progress — from the 1-yard line in our own territory, as he put it — and urged them to “cross the goal line and spike the ball.”

The 26 oral presentations and 11 posters at the conference also included research aimed at understanding the biological processes behind NPC. Several scientists are

using animal and cellular models and studying changes at the molecular and biochemical level.

To borrow Coach Parseghian's football analogy, the conference was a huge pep rally that energizes us with hope from the researchers and inspires us with the urgent stories of the beautiful NPC families. We look forward to pressing ahead for the next year, getting together again for the conference next June, and driving across that goal line soon for the sake of these precious children and everyone who will benefit from our fight.



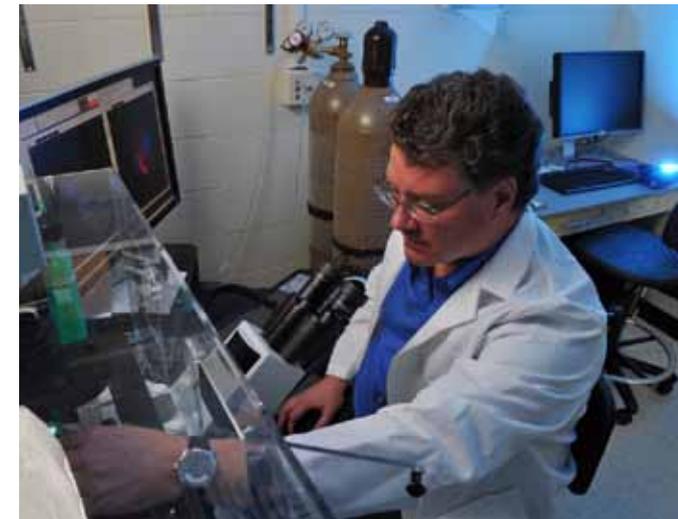


Today we put it all out on the Road to Discovery — we had nothing left after the 102-mile ride and 6,000-foot climbing. We got lucky with the weather, but the mountain we crossed in the last 20 miles was really something else. The first 50 miles were smooth sailing.

As for the last 20 miles — well, let's say it took everything we had to make it over into Crossville. However, on these challenging days, we feel a real sense of accomplishment when we are finished — and we have those fantastic NPC families in our hearts and minds to help us across the tough parts.

Pigeon Forge to Crossville, Tennessee: Hilly 100

Organizing a big road trip like this takes a lot of planning, and a lot of people. But it's just a little piece of the huge fight against Niemann-Pick on the Notre Dame campus. Take just one example: in Professor Kevin Vaughan's lab, undergraduates, graduate students and even the latest mass spectrometry instruments are part of the project, with help from the Ara Parseghian Medical Research Foundation. Professor Vaughan's lab is focused on the NPC1 genetic mutation that is responsible for about 95 percent of all cases of NPC. Using cell biology approaches, they've developed a new model to



explain the consequences of the mutations, measuring the impact on the membranes where the NPC1 protein accumulates.

day **16**

June 28, 2011

day 17



June 29, 2011

We are counting down to July 8, our last day. Today marks our 17th day, so we only have 9 days left — down to single digits! Today the ride was spectacular. The temperature was perfect, the sun was shining over the hilly landscape, the pavement was smooth, the shoulders were broad, and the winds were behind us — they pushed us all the way to Manchester. This was a great active recovery day after our hilly 100 yesterday. It's funny how fast the day goes when conditions are perfect. We did run across a town called Pleasant Hill — we did not think there were any pleasant hills after yesterday — and we got a big laugh out of it.

We had a wonderful dinner in Nashville with Gregg, a Notre Dame alumnus, and Louise and their daughter Abigail; Pat, a Notre Dame alumnus, and Sue and their daughter Annie, who is studying Science Business and will graduate in 2014; Christopher, a Notre Dame alumnus; and Katie, a Saint Mary's alumna. We had great food and terrific conversations about ND, the Science Business major, start-up companies from universities, and of course our ride and cause.

Crossville to Manchester, TN: Down to Single Digits

Today we had the tremendous honor and pleasure of meeting Amy Grant after a fine ride across the beautiful Tennessee countryside. We were a bit nervous on the way to meet such an accomplished singer and songwriter — 30 million albums sold, 25 Dove Awards, six Grammy Awards. Amy is a friend and longtime supporter of the Ara Parseghian Medical Research Foundation. We were so excited to meet with her today.

As soon as we met Amy, we weren't nervous any more: she was so welcoming and down to earth and so generous with her time that we felt at ease right away. When we asked her how she got involved with the Ara Parseghian Medical Research Foundation, she explained that she read Cindy and Mike's story in People magazine not long before Cindy invited her to perform at an event. She told us about the Parseghian children, Marcia and Christa, and her relationship with them.

Amy is a firm believer that there is an upside to every hard thing, even when the difficulty is so intense that the positive effect takes time to appear. The Parseghian family's immense suffering — the loss of three beautiful children — had a ripple effect throughout the medical profession and the Notre Dame community. Cindy and Mike are a beacon to all the families struggling with NPC, and the Ara Parseghian Medical Research Foundation is a great place for people to find connection and support, as well as a vital contributor to the search for a cure. We talked about Amy's music and how some of her songs have been embraced by the NPC community, especially "Everybody Needs A Helping Hand" and "Children of the World." Amy said the connection fulfills her dream as a songwriter to touch people's lives in a meaningful way. She took a look at those young children in the magazine years ago and reached out a helping hand. She's still doing it for the hundreds of children waiting



for us to find a cure. We are so fortunate to have Amy as a friend and supporter in the fight against NPC. From now on, that song in our ears will have even more meaning as we ride on the Road to Discovery. Thank you, Amy, for all you do for the NPC community.

day 18

“
Everybody needs
a helping hand.
”



Singer/Songwriter Amy Grant



day 19

July 1, 2011

Today was our longest day — 150 miles! We started very early, at 4 a.m., to get in as many miles as possible before the heat set in. By the end of our ride, the temperature was 93. In addition, we faced over 5,000 feet of climbing for the first 125 miles. As we approached Collierville, Tennessee, just east of Memphis, the landscape flattened out a bit. All in all, it was a challenging day because of the large number of miles, but the weather, although hot, was just spectacular. Renate pointed out that I have my own city in Tennessee — Deanburg! It's a very small farming community.

We had a wonderful dinner with Rick and Lisa — Rick is a Notre Dame alumnus and very active in the Notre Dame Memphis club. We ate at a local café in Collierville, enjoying great food and great conversations. Rick is a real ND football fan and could have gone on for hours about Irish football. Historic Collierville is very charming and quaint — a wonderful place to wind up our longest day.

Nashville to Collierville, Tennessee: The Longest Day

Today was a long, hot day. It was a lot of fun since we crossed into Mississippi from just outside Memphis for about 30 miles, then over the Mississippi River and into Arkansas — three states in one day. Although very hot, there is probably very little climbing left until Dallas. Today's ride was flat, flat, flat. We had dinner at Blues Bayou, a restaurant with alligator bites as a specialty. We weren't feeling particularly adventurous and went with the streak instead. Overall, a very uneventful day. After dinner, we went to bed early, as we had the night off for the first time in a long time, giving us time to rest for what promises to be a hot and long (>100 miles) day tomorrow.

day 20



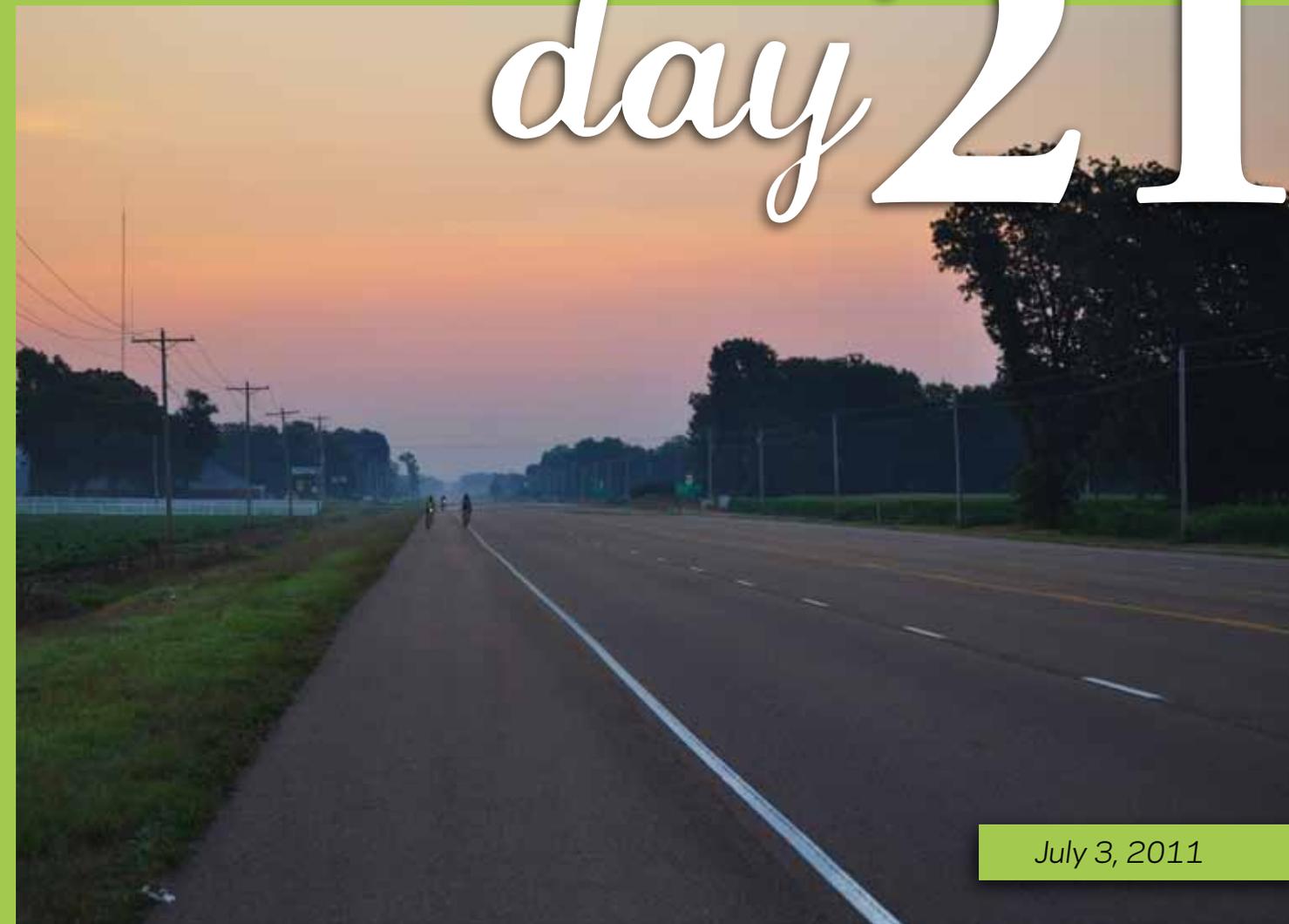
Today was a long day — over 105 miles across flat land — and very hot, with temperatures above 100 degrees by day's end. To avoid as much of the heat as possible, we started very early, putting all of our reflectors and lights on our bikes and bodies in the pre-dawn darkness. Getting to watch the phenomenal sunrise was a great reward for the early rising. As the heat kicked in, we drank a lot of water to stay hydrated.

This evening we met an NPC family in Little Rock - Janice, Richard, and Judy (Richard's sister). They talked about their son, Jon D., who was diagnosed with NPC when he was 5 and died at the age of 23. Jon D. was easy to get along with and never had any harsh words for anyone. They told us that he was always a happy child. He loved blondes and the color purple. Janice and Richard shared with us their memory albums of Jon D.

There were a few passages from our conversations that stuck in our minds tonight. Richard told us about discussion with the doctors after the diagnosis: they said to him that there was no treatment or cure and nothing could be done. But Richard said: "Yes, there is something we can do. We can raise funds to support research on NPC." And that they did. The family organized raffles, softball games, BBQ events, and so on. They funded a few researchers around the country to study NPC. Richard and Janice went on to discuss their deep appreciation for the work of Coach Parseghian, Cindy and Mike, and for the Foundation bringing together people and families to help each other out. They talked at length about the bonds made with other NPC families, and they told us that they thought of the NPC community as a large family. When they heard that a child succumbed to NPC, they felt the sorrow as if one of their own had passed on.

Jon D. had an impact on many — his schoolmates, the NPC community, his local community, politicians, his doctors, etc. Richard, Janice and Judy through their initiatives raised money and funded research on NPC. We had a wonderful evening with them, hearing stories about Jon D. and sharing stories other NPC families had shared with us.

day 21





John D's family

“

Yes, there is something we can do. We can raise funds to support research on NPC.

”

No fireworks for us on the road today, which is just fine as we make steady progress toward our goal. It was really warm, with a bright, sunny start to the ride, and temperatures reached into the 90s, but the ground was level, and the thunderstorms held off until we had arrived in Camden — so, a very pleasant day overall! We passed through Kingsland, Arkansas, which was home to Johnny Cash.

With time for reflection during the easy ride today, I was reminded of how lucky we are to have such wonderful students at Notre Dame: students in Professor Michelle Whaley's course working on an NPC project mentored by Professor Kevin Vaughan, students working on clinical scores in Professor Kasturi Halder's course, and graduate students working on NPC in a number of laboratories for their Ph.D. theses.

Biking today reminded me of two students who also organized and performed in some physically intense fundraisers this year for NPC — Michael Dean and Joe Magro. Michael, who qualified for the Boston Marathon, dedicated his race to NPC. Joe, a member of the Notre Dame Cycling Team, arranged for the Midwest Collegiate Cycling Conference race in downtown South Bend to benefit the Ara Parseghian Medical Research Foundation. Joe asked me to say a few words about NPC at the beginning of the race in March, which I gladly did. We have outstanding students at Notre Dame. From the classroom to the laboratory, they're working to understand NPC in order to provide better health care and treatment for NPC children. Even those who do not have a direct scientific connection have dedicated their efforts, through athletic feats, to the cause.

Out here on the Road to Discovery, we're proud to be doing our part to join them in that life-changing fight.

Happy Fourth of July!

day 22



Today was a pleasant ride into Texarkana — the Arkansas side, but within sight of Texas. After a major storm last night, with a lot of rain, lightning and thunder, the weather was just beautiful. As usual, we started early to avoid the heat. Later on we encountered what might have been the biggest surprise of the trip. We were traveling along at a pretty good speed, making good time, when I glanced into the farm field on my right and saw a zebra — yes, a zebra in Arkansas! There was no explanation or way to figure out what a zebra was doing there, but when you see something that far from a place you would expect it, you'll remember it! Overall, it was a wonderful day.



Yesterday I spoke about our wonderful Notre Dame students and how accomplished they are. Today I would like to tell you about a unique course, created and taught by Professor Kasturi Haldar, titled “A Course in Developing Health Networks in Rare and Neglected Diseases.” NPC is actually a pilot disease studied in this interdisciplinary course, designed to help students become better advocates for research and treatment of rare diseases like NPC and neglected diseases like malaria and lymphatic filariasis. Students, including both upper-level undergraduates and graduate students, not only learn about clinical research in those areas but also conduct research themselves. For example, they score medical records of patients with NPC, which help develop a clinical score to be used to test the promising tools of diagnosis and treatment for this disease. The students also make a medical summary in the form



day 23

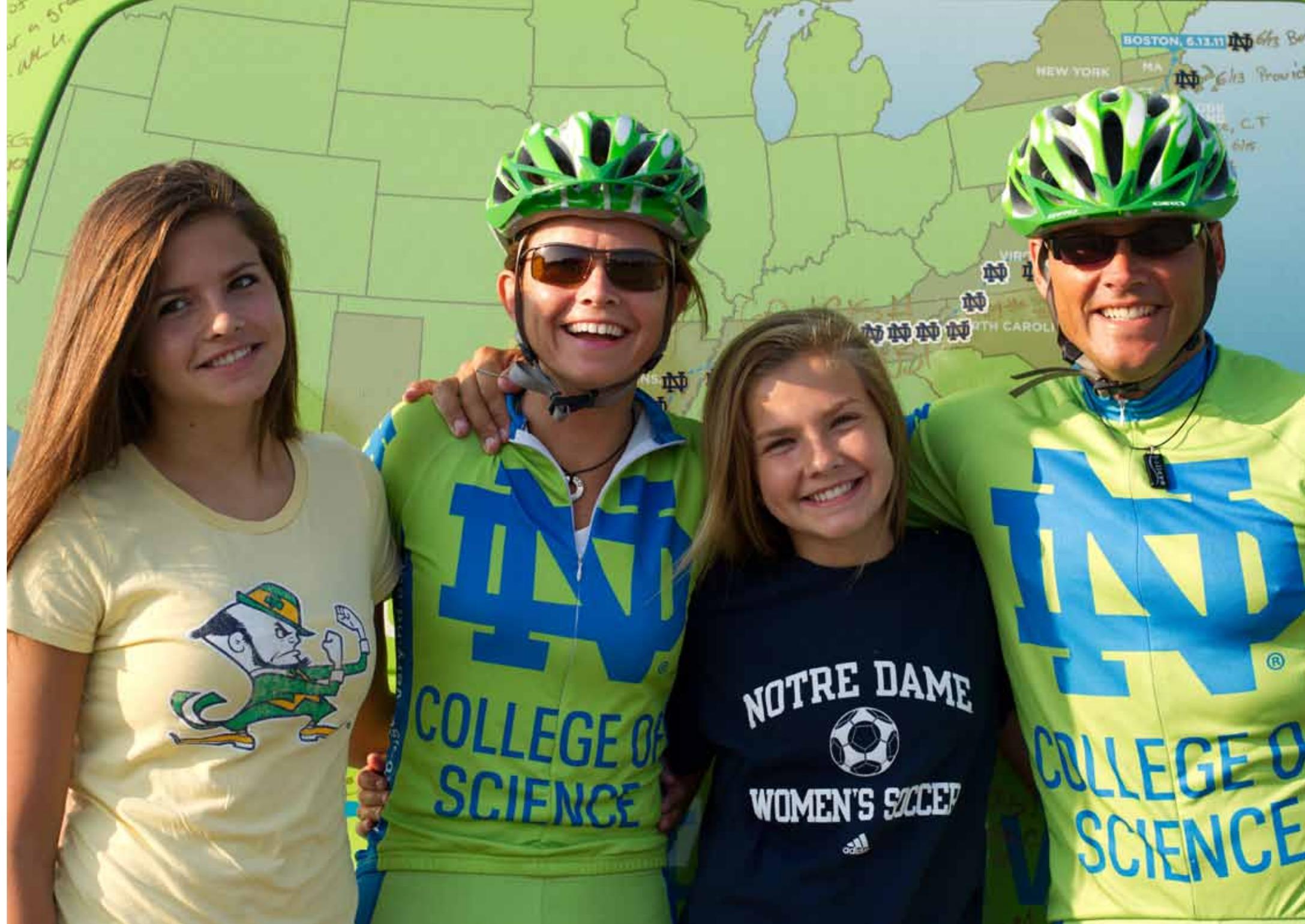
of a notebook, which the families could use when they have to explain their situation to a new specialist. The course involves participation in the Center’s Clinical Translational Seminar Series, so they have a platform for interacting with top investigators and experts in the field. The students get really interested and involved in this work, and we help them find more opportunities for service and learning in the area beyond the course.

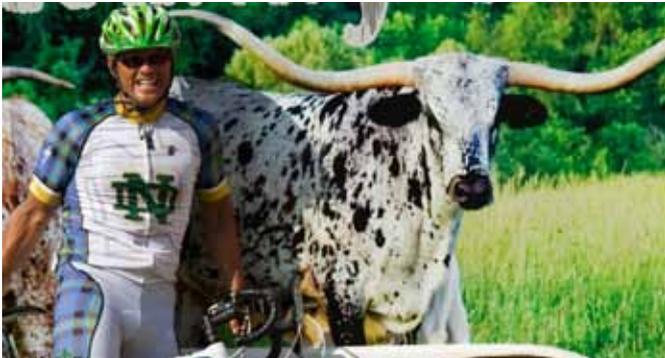
Wherever you look, the fight against NPC is going on with a great team of committed people, and more and more people are joining us. We are so proud to be a part of this effort on the Road to Discovery.

“

We were fortunate to have our two daughters Michaela and Alexandra along for part of our ride. They felt close to the cause since many of the children are so close to their age.

”





The ride directions were pretty simple today: turn left out of the hotel parking lot in Texarkana and go straight until you hit Mount Pleasant, Texas. As we crossed the state line into Texas, we found longhorns just about everywhere, and Greg fit right in with the cowboy boots he had worn in the “Dancing with Our Stars” fundraiser earlier this year for the Center for the Homeless in South Bend.

Tonight we met with an NPC family — Tracy, the mother of Tristan, and her two sisters and cousin. Tristan is 9 years old. She had a big smile, and she seemed to like bald guys. Tracy told us that Tristan’s physical therapist has a shaved head and she really likes

it — so she took a liking to Greg as well. Everyone signed the van tonight, and all the girls were already sporting their bright green Road to Discovery t-shirts. Tracy has been following our blog. She found out about the ride through one of the NPC email exchanges, and they drove an hour to meet us and see the van. We are so grateful they were willing to make the trip — it was such a pleasure to meet them.

As we were riding today on a relatively flat, straight road to Mount Pleasant, I started to think more about our discoveries and entrepreneurship efforts at Notre Dame. Many of the drugs currently being used to treat rare diseases were discovered in academic or university laboratories. In order to make these discoveries available to patients, the drugs needed to be evaluated for safety and efficacy, which required a transfer of responsibility from the University to a drug development company. The same

is true of NPC. Funded by the Parseghian Foundation, efforts by scientists from the Chemistry Department at Notre Dame and the Biochemistry Department at Weill Cornell Medical College recently resulted in the breakthrough discovery of a compound with potential for treating NPC. The next step in the development process has begun with the formation of Lysomics, LLC., whose sole purpose is to take this compound to the clinic. Lysomics has taken the first step by opening consultations with the FDA. We are moving closer and closer to the goal line.

Out here on the Road to Discovery, we are moving closer to our goal line, too — only two days away from finishing the trip. We are so fortunate to have met so many great families, children, researchers, doctors, nurses, Notre Dame alumni and friends, and total strangers whose hearts were moved when they heard the story of our mission. The ride has been a challenge at times, but we remember the great team we’ve joined, fighting for the lives of those beautiful children, and the miles fly by. We can’t wait to get back home to tell everyone about these amazing experiences. See you soon!

day 24



“

The ride has been a challenge at times, but we remember the lives of those beautiful children and the miles fly by.

”



July 7, 2011

day 25



Mount Pleasant to Greenville, Texas: 55 Miles To Go

Today we left early to avoid the heat. Some of the roads were rough, but the weather was favorable for an early ride and the morning sky was beautiful.

For the past 25 days, we have been meeting with NPC families and children. We have been interviewing them with the goal in mind of creating a documentary on NPC after we return home. Yesterday we interviewed Tracy, the mother of Tristan. We are so grateful to the parents for sharing their thoughts and experiences related to NPC for our project. The Charles Edison Fund has provided funding for our documentary. We are so appreciative of their support.

This evening, we had a welcoming reception and dinner in Dallas at Maggiano's Little Italy Italian Restaurant. We were warmly welcomed by the Dallas Notre Dame Club.

Renate gave a talk on the ride, and I spoke on some of our new programs in the College of Science. Several researchers from the University of Texas Southwestern who are working on NPC also attended. Some of them recently visited Notre Dame for the NPC conference. It was great to see them again, and we will visit them tomorrow at the University. We had a wonderful evening, with everyone signing the van. Thank you, Notre Dame Club of Dallas, and Calvin, the club president, for organizing this wonderful event.

We have a short ride into Dallas tomorrow, only 55 miles. It will be a quick day — we are excited to finish up the ride. Thank you all for your support and inspirational emails and comments over the past few weeks. We really appreciate your thoughtfulness. Your support kept us going on the Road to Discovery.

Our last day turned out to be much more challenging than we anticipated. For the first 20 miles or so the pavement was rougher than any we had experienced on the entire trip. Then came the sun and heat, with over 100 degree temperatures as we arrived in downtown Dallas. But we were so excited to be completing the 2,200-mile ride, and we are so eager to tell everyone about the experience — the NPC families, children, doctors and researchers — that the few hours on the bike seemed to fly by. We finished in downtown Dallas, and Jim, one of my advisory council members, and his wife were there to greet us with Sean from Development.

After we arrived, we had the opportunity to meet one more NPC family, a fitting reminder of our reason for the whole ride. Thad is the father of two NPC children — Katie, who is five, and Andrew, who succumbed to the

disease five years ago. This was the second family we met on our trip with more than one NPC child, and Thad said something we will never forget: "For parents with multiple NPC children, you are going through the hardest thing in your life and know you have to go through it again."

Thad told us about Andrew's love for baseball and basketball, and how Andrew was very social and liked to imitate Barney to introduce people he met. He lost his battle with NPC at the age of nine. Thad spoke about Katie as one of the funniest people he knows: she jokes around and loves to do and try anything, even karate.



day 26

Thad was active on the board and research subcommittee for the National Niemann-Pick Disease Foundation (<http://www.nnpdf.org>). He also told us that Niemann-Pick A, B and C get very little attention in medical school textbooks — in some cases, only one paragraph for all three diseases.

Shortly after our meeting with Thad, we had the opportunity to visit the University of Texas Southwestern, an institution with a lot of NPC research taking place. We were

thrilled to see the facilities, a reminder of how much progress we are making against NPC. Professor Joyce Repa toured us around the building and introduced us to her colleagues and many of the students. We were so excited. The UTSW effort is significant and very innovative — we were very fortunate to learn more about the forward-thinking NPC science taking place there.

A long day, a great ending to our ride, and we are very happy to be on our way back to Notre Dame. We have a lot of great stories to tell you all. We had a journey of a lifetime. We will always remember all of those we met along the way, and we will certainly keep in touch.

Crossing the Finish Line



“

We are inspired by the Parseghian Family and the Ara Parseghian Medical Research Foundation. They have turned their personal hardship into hope for all NPC families.

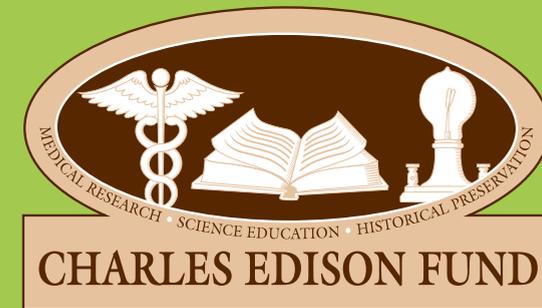
”



“

Thank you to the Charles Edison Fund. Without your help, our wonderful experience on the Road to Discovery would not have been possible.

”



roadtodiscovery.nd.edu



UNIVERSITY OF
NOTRE DAME

College of Science