



## Dominos

### Sarah Ziegler speech during the 2009 FUNTASIA Research Banquet

Have you ever seen videos, where there are rows & rows of dominos all lined up? Tap the first and watch the chain reaction. This is what the MHE Research Foundation stands for, through research and education, we line up the rows of dominos and medical & research professionals knock them down! Row after row time after time! When the last domino falls we will have accomplished our goal a treatment a CURE for Multiple Hereditary Exostoses.

There has been a huge amount of progress since the last time we've gathered together to feel the magic of FUNTASIA. For the last decade researchers have faced a perplexing problem with the MHE mice models, the vast majority of them only developed tumors in the ribcage, unlike people who suffer from MHE where they develop everywhere. I am happy to report the row of dominos concerning this issue has fallen and a huge breakthrough has been made in the advancement of MHE Research.

Yu Yamaguchi, MD, PhD, Professor at the Sanford Children's Health Research Center, Burnham Institute for Medical Research. The 2007 award recipient of the Humanitarian Scientific Achievement Award and co-organizer along with our foundation of the Third International MHE Research conference has made this tremendous breakthrough. Yu has over this past year developed a mice model for MHE; these mice develop tumors in the same locations and have the same bone deformity as people who suffer from MHE. Sorry Mickey Mouse now ranks second in my heart.

Dr. Benjamin Alman, Canada Research Chair

A.J. Latner Professor and Chair of Orthopaedic Surgery Vice Chair Research, Department of Surgery, University of Toronto Head, Division of Orthopaedic Surgery and Senior Scientist, Program in Developmental and Stem Cell Biology Hospital for Sick Children and member of our advisory board has just published a research paper. Multiple Hereditary Exostosis and Hedgehog Signaling: Implications for Novel Therapies.

This signaling factor can now truly be explored using this new MHE mouse model. In a few short weeks the Third International MHE Research Conference will take place. 35 of the best Hedgehog Signaling, Glycobiologists, Bone development, Orthopaedic and Genetic researchers in the world from 8 countries will converge in Boston to think tank on this new progress and the direction MHE Research needs to take. The funds raised today will help support this conference and indeed the progress MHE Research will take in the future. Needless to say how important each and every one of you are attending FUNTASIA and showing your support here today Thank you.

I do not want to mislead anyone, simply put for the first time the key components are being revealed for the future development of a cure. This is going to take years and much depends on the funding of MHE Research by "The National Institute of Health" and also the support of our foundation.

Mark my words piece by piece, row by row these dominos will continue to fall. One day operating rooms will be replaced by our children playing on Baseball fields, the endless barrage of hospital room stays will be replaced with these children spending their time in the class room! So they can follow their dreams of becoming everything they want to be, without having their lives filled with pain, surgeries and all the burdens MHE places upon them!

This brings the introduction and the awarding of the MHE Research Foundation's 2009 "The Humanitarian Scientific Achievement Award" to Dr. John P. Dormans the Chief of Orthopaedic surgery at the Children's Hospital of Philadelphia, Professor of Orthopaedic Surgery at the University of Pennsylvania School of Medicine. Authoring an astonishing 260 peer-reviewed journal publications, a number of these on MHE. Dr. Dormans serves on the editorial boards of the Journal of Pediatric Orthopaedics & Clinical Orthopaedics and Related Research and a founding member of our foundations advisory board. He's been a visiting professor both nationally & internationally at 50 institutions. His resume is a book in and of itself being 164 pages long, listing his accomplishments.

Dr. Dormans has spent the majority of his career caring for children with musculoskeletal tumors and sees about 15 MHE children a week, 90% of these children require surgery. Dr. Dormans is currently President of the Pediatric Orthopaedic Society of North America (POSNA); the leading internationally recognized Society for Pediatric Orthopaedics. Committed to the care and treatment of children with orthopaedic conditions and leading the way in the education of Orthopaedic surgeons around the world.

There is always a beginning and for me it was reading the Orthopaedic publications on MHE Dr. Dormans authored. Looking back writing my first emails concerning our organizations efforts; I thought this man is just too huge he's never going to respond. I was dead wrong within an hour I received his reply saying count me in, how can I help, how can I be of service!

First order of business was the lack of lay patient information; the dominos fell without hesitation and has continued. 19 pages the Standards of care the corner stone to the Connection Corner Guide book to MHE. Dr. Dormans input lead to others in the field authoring other chapters of the book as well.

Again they fell; working jointly with our foundation on a research project, providing investigators from EuroBoNet the tumor samples, blood samples and clinical information to continuing the progress into the etiology of MHE. This project now serves as template for many other hospitals to use around the United States. Examples: Dr. Alex Arkader you trained who is now Director of the musculoskeletal tumors program at Children's Hospital Los Angeles and Dr. Harish Hosalkar at the Rady Children's Hospital, UCSD.

Another row went down; Together our initiative, the first ever session entirely dedicated to MHE surgery and clinical care our kids receive, conducted during last Dec's International Pediatric Orthopaedic Symposium.

Your support as well as the entire POSNA board of directors, illustrating the concern about the education of rare bone diseases by granting my request for the donation of an educational exhibit & poster display at the next conference to take place this May; to be held in Hawaii. I am looking forward to representing the United States Bone and Joint, Rare Bone Disease Patient Network and the MHE Research Foundation.

The countless rows of dominos that have fallen as a result of Dr. Dormans efforts has been seen and felt around the world.

I felt the weight of the world being placed on my shoulders the day my son was diagnosed with MHE at a year old, simply being handed two paragraphs about a disease I had never heard of. 10 years of searching for a surgeon that could care for my son took a huge toll concerning his health and the never ending feeling was I making the correct decisions? Dr. Dormans when I asked you to take over his care, it was not simply because of your skills as a surgeon or your knowledge concerning MHE. It was also your ability to communicate with my family.

Walking into your office and watching you speak with Robert. You looking in his eyes showing Robert your concern and letting him know you were there to help no matter what and would stand by him. Your assurance that we were in this together, I felt you willingly lift the weight of the world off my shoulders and place it on your own. You gave me sanity and brought balance into Robert's care and our lives. This allowed me to simply be MOM support my son through his painful journey.

Dr. Dormans you are not only a huge part of the medical & research professional family we created together, our advisory board 17 members strong, countless other professionals always willing to help thanks to your dedication to our foundation. You are a huge part of the reason why my son at 18 can now chase his goals and dreams.

The day my son was born I whispered my hopes and dreams for his future in his ear as he lay close to my heart.

Today I would like to whisper my hopes and dreams for you Dr. Dormans from our hearts. May you look upon this award and see every child's face whose life you have changed for the better. May you see not only the faces of the children you have performed surgery on, but also see the faces of children treated by the Orthopaedic surgeons you have trained and your efforts on behalf of all children from around the world. May you see/experience their joy as these children REACH for their goals and aspirations.

You have not only provided countless residents and fellows the Orthopaedic knowledge and skill to care for children. Every day, you illustrate to them how to live a life with broad shoulders ready and willing to take the weight of the world on, in the most ethical, caring and humanitarian manner, you teach by example.

May this award help provide the strength to continue to keep knocking down the rows of dominos placed before you both medical & research, so we can see the results of our dreams, our efforts to one day watch our kids play someday on that baseball field! Never having to see an operating room again! Dr. Dormans you are a paragon of all that medicine can be and should be! It is my profound honor to

have Robert Ziegler and Vincent Eaton present you with the MHE Research Foundation “The Humanitarian Scientific Achievement Award”. Thank you for your life’s dedication and working together with us to change the world knocking down one domino at a time!

Sarah Ziegler

The MHE Research Foundation Vice President & National Director of Research

Executive Director, The MHE National Research Registry & Coordinator of Clinical Information

Liaison Officer, University of Antwerp, Department of Genetics, Belgium

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Co-organizer Third International MHE Research Conference

USBJD Rare Bone Disease Patient Network member

Website: [MHEResearchFoundation.org](http://MHEResearchFoundation.org)

Website: [MHEResearchFoundation.org/MHE\\_Conference.html](http://MHEResearchFoundation.org/MHE_Conference.html)

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