HRG Leadership & Consultants
Martin Pietrucka, MD, JD Clinical Laboratory Director, HRG
Yadira Velazquez-Acevedo, MD, PhD Director of ADA, Clinical Molecular Laboratory, HRG
Debra Dankovich, MD Director and Clinical Consultant, HRG
Madeline E. Griffin MD MPH Director, Preventive Medicine
Affiliates, American Board of Preventive Medicine, Washington, DC
Former Director of FDA Office of Orphan Products Development
Wayne Grody, MD, PhD Professor, Division of Molecular Pathology
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Peter Shambayei, MD, PhD Director, InnoPharma Ventures
InnoPharma Ventures, Los Angeles, CA
Timothy P. Komanow, PhD Founder/CEO InnoPharma Ventures

HRG Expansion
For the past two years, HRG has collaborated with local universities to send outstanding students to HRG. This collaborative group brings together academia and a research community through mutually beneficial basic and applied research endeavors. HRG believes that university collaboration is integral in fostering innovative work in the biomedical research field to develop hands-on skills and knowledge. HRG has set up a satellite laboratory at California State University, Northridge so students will be able to work, learn, and contribute to helping HRG find a cure for HRG. Current Students at HRG are: Rosangela Carabaj, Saghi Esfandiarzand, Zashna Khokhar, Daniel Yu, Chiu Satchin, Khita Shah, Pedram Sinaei.

HRG Current Projects
1. Mouse model development for HRG. Over the past few years, HRG was involved with development of an HRM mouse model that was useful for proof of efficacy studies on Na-Acetylmannosaminidase (ManNAc) as potential therapeutic for HRG (Shahin, Kowtechnik et al 2007). The effectiveness of this potential therapy was also verified using another mouse model developed in Japan (Mallan, Noguchi et al 2009). Due to non-muscle pathology, late onset muscle disease, and unpredictable survival, neither mouse model was optimal for rapid testing of all potential therapeutic drugs. Thus, HRG, with collaboration of WCMC Medical Center, is developing another mouse model with hope that it will develop muscle disease at an earlier age. This model will hopefully allow for more rapid translational research towards clinical trials. The development of this mouse model was partially funded by the Marfan Foundation (MFA USA).

2. ManNAc Toxicology Study. Following proof of efficacy in animal studies (Shahin, Kowtechnik et al 2007), an investigational new drug (IND) application was filed with FDA for ManNAc for clinical trials for 20 patients affected with HRG. Due to lack of adequate safety/toxicology data, the clinical trial was placed on hold pending performance of animal toxicology studies on both inbred and non-inbred models for the duration of 3 months. This toxicology study is estimated to cost $50.0M – $15.0M. ARM and HRG are currently working closely to ensure enough funds become available to perform this urgent study in the most cost effective and efficient manner possible.

3. Gene Therapy vector development. HRG is involved in both developing and validating promising gene therapy vectors for potential HRG treatment. This work is in the cooperation of Julie Ogden (Texas trial) for which the GNE expression vector was developed in collaboration with Gradowski and McManus (Jay Neumai et al 2008). This single patient gene therapy trial is currently ongoing, and we hope to see promising results soon.

4. Biomarker Development for HRG Clinical Trials. One of the most important needs for HRG clinical trials is development of robust, reproducible, and reliable clinical assays that can be used to determine if a therapy being tested in human trials is biochemically effective. HRG is currently active in improving assays for clinical use. One current priority develops a reliable Stabild of Skeletal Acid levels from a very small amount of human muscle samples obtained by needle-core biopsies. An initial progress of this work was presented at the American Society of Human Genetics (ASHG) meeting in October 2009 and the Society for Glycobiology meeting in November 2009.

What does HRG do?
- Accelerate research toward HRG clinical trial by developing needed biomarkers (e.g. mouse model, gene driven, GNE antibody)
- Develop key collaborations required for trials (e.g. with NIH/NIDDK, etc.)
- Provide patient referrals (e.g. for the HRG Trial I/II)
- Develop/provide patient services (e.g. genetic testing/patient counseling)

HRG has published collaborative scientific papers with investigators at NHI, Harvard University, Ohio State University, and others. HRG provides genetic testing services internationally as well as domestically for doctors and patients at Cedars-Sinai, Garvey Genetics, Johns Hopkins University, UCLA, Mayo Clinic, and others.

Our Vision
The vision of ARM is to unite communities worldwide in our fight against HRG. Together, we can significantly shorten the path to the development of an effective treatment. Please join us in this fight.

Who is ARM and What is HRG?
ARM (Advancement of Research for Marfan’s) is a 501(c)(3) non-profit organization with the primary goal of spending scientific research on HRG. The Autosomal Recessive form of Hereditary Inclusion Body Myopathy (HRM) is the purpose of ARM to accelerate biomedical research aimed at developing treatments for HRG and skeletal muscle degeneration. ARM’s mission is to accomplish this goal in the most efficient manner possible. The vision of ARM is to unite communities worldwide in the fight against HRG. ARM’s collaborative genetic testing for HRG is available at the HRG lab in Redwood for a nominal fee. Together, we can significantly shorten the path to development of an effective treatment.

On Sunday June 27, 2010, ARM will host its 10 Year Anniversary Gala to celebrate accomplishments and share the future of HRG research. We are at an unprecedented moment in the search for a cure for HRG. It is truly no longer about finding the cure, it is only about funding the care. All necessary knowledge is available and all necessary research has been done to move towards clinical trials with multiple patients. To be able to do all that is needed is funding. ARM has been working closely with HRG, HRG Research Group, and the only non-profit laboratory working full-time on HRG and the laboratory with the most detailed knowledge and understanding of HRG research.

ARM’s 10 Year Anniversary will be celebrated with excellent music, food, and entertainment, and ARM hopes to celebrate with you! For information and tickets please contact the ARM office or visit the ARM website.

ARM Fortuately Invites You To Their 10th Year Anniversary Gala Dinner on Sunday, June 27th, 2010

Larry Klug Jazz Band Come Celebrate With Us! Comedian Maz Jobrani The Gypsy Kings
And the promies of the new ARM promotions’ cards by David Vlack
Time: 6:00pm
Location: Marriott Los Angeles Airport, Marquis Ballroom 1855 West Century Boulevard, Los Angeles, CA 90045
$150.00 per ticket (open seating) $175.00 reserved seating
Purchase tickets by phone or online: 1-818 789-1044 / www.arm.org
Koshar Catering by Sharon

Newsletter Program
ARM has begun two periodic newsletter programs: 1) Update, the newsletter for donors, and 2) Review, the newsletter for healthcare professionals and students. These newsletters will include important information regarding scientific updates, ARM funded research, activities, and ARM’s financial position. If you or someone you know, would like to receive one or both of the newsletters, please email a request to arm@hrg.org. If you would like to include other specific information regarding HRG or ARM as the next newsletter, please email a request to arm@hrg.org.

ARM needs more funds to be able to support new research projects vital for funding clinical trials for HRG. If every household makes a donation, regardless of the amount, we will be able to fund more research towards developing an effective treatment. Please take a moment and donate as much as you can afford. Your donations are crucial for developing a treatment for HRG.
ARM Dollar Campaign
one minute, one dollar, pass it on.

You can make a REAL difference with very LITTLE money and very LITTLE time. We’re asking for two things: give $1 and pass this email on to people you know. We’re trying to raise $1,000,000 in 3 months to enable human therapeutic trials to fight HBV, a debilitating disease. We’re asking for two things:

First, go to WEBSITE (www.hbmonline.org) to donate $1. Second, send this campaign on to all your friends and family.

In the Internet age, reaching one million people in that amount of time is surely doable. If you are interested in becoming a bigger influence in this campaign, host your own dollar collecting campaign in your community, church, workplace, among your friends and family and donate what you’ve collected as one donation.

I-Give
Support ARM while you shop at your favorite stores.

Log on to www.igive.com/forARM and register to shop at more than 700 stores. Corporations will donate a portion of your purchases to ARM. Among them, you will find brand names such as: Staples, eBay, GAP, Egidia, Barnes & Noble, Toys ‘R’ Us, Overstock.com, Office Depot, Home Depot, Lancôme and Victoria’s Secret.

Thank you for supporting ARM and HBV research by shopping at www.igive.com/forARM. ARM will be rewarded if you choose http://www.hbmonline.org/forARM as your search engine.

www.igive.com/forARM

Join ARM as a Volunteer!

ARM needs volunteers of all ages with different skills to organize parties, gala dinners, or informative seminars, to write articles, to design and mail newsletters, to produce documentaries. Various groups representing different demographics are currently active in forwarding ARM’s objectives. If you would like to be involved as a volunteer, and join our great journey towards a cure for HBV, please contact ARM at 1-800-ARM-200.

Visit www.hbmonline.org for more ways to help.

Calendar of Events

The following events are planned for 2010:

May 13, 2010 – ARM’s 10th Year Anniversary Gala (NY)

July 17, 2010 – ARM’s 10th Year Anniversary Gala (LA)

October 28, 2010 – Young ARM’s Costume Party (LA)

December 5, 2010 – Annual Telethon (LA)

To receive detailed information, call 1-800-ARM-200.

Young ARM Party (Vanguard)

The annual Young ARM Costume Party was on Thursday October 28th, 2010 at Club Vanguard in Hollywood.

ARM would like to thank the Young ARM group for organizing the event and the dedication throughout the year. ARM strives to improve the event and make it bigger and better. For 2010, Young ARM will give a new look to ARM’s annual Costume Party by using a different venue and bringing new entertainment and surprises to the event. We thank everyone who attended the event and hope to see you again next year.

For more information and to submit your suggestions on ways to improve the costume party please email ARM@bmonline.org.

Roya Nourrifi – An Incredible Journey

My name is Roya Nourrifi and I have had HBV for 13 years. I was born in Iran. After graduating from high school in Iran, I was banned from attending college there due to my religion. That did not dissuade me from pursuing my dream in regard to the study of art. I trained prowly with famous artists in Iran, studying their techniques and style. It was during this time that I was medically diagnosed with hepatitis B and became progressively fatigued. Very few doctors and writers were able to correctly diagnose my condition for years. After numerous consultations, the shocking news came that I had Hereditary Inclusion Body Myopathy (HIBM). HIBM is a group of genetic disorders, which are very rare in the general population, but cause progressive muscle wasting and weakness leading to severe disability.

At times, my dreams were shattered. It seemed my whole life was changing and I would surely have to abandon my dream of being an artist. This idea was unacceptable to me and I pushed even harder to hone my skills as an artist. Finally, my family and I decided to come to America to have an easier life.

When I came to America three years ago, I met doctors David and Babak Davarri at ARM. Meeting with other patients helped me not to give up. However, it has not been easy as my health has progressed and I am now forced to use a wheelchair. Despite this setback, I have been attending college ever since arriving in America. Living in Los Angeles has allowed me the incredible opportunity to have a better life. I am in the final stages of graduation and am going to a work of poetry and art that will be my second book of poems. Thus far, my life has been an incredible journey of life experience and lessons, hardships and struggles, sadness and major hope forward. I am grateful for every moment and hope to continue my dream of becoming an artist. I wrote a novel in Farsi, entitled “Main.” It is based on my own life story, and has been published 5 times in Iran. I wrote the 520 page work, and designed the cover. My second book is ready to be published in America, which is a collection of 200 poems. Four years ago, I realized that my hands started to become weak, and I could not paint on a huge canvas as I used to. But I do not make use of a wheelchair and paint at any opportunity and hope someday a cure will be found.

I have learned to communicate with others through various languages. I find communication in art that transcends language and spoken word, and is a way for me to express my deepest thoughts and emotions. These feelings are shared by others and take normal human interactions and gain inspiration for a poem, painting or even a sculpture. It connects me to a world that sometimes feels distant due to my disability.

Financial Report

For the year that ended Sep. 30, 2008, the total revenue was $624,628 which included 67.14% research grants awarded, 26.61% fundraising and 6.25% management cost.

Young ARM New York

Young ARM of New York has successfully ended its 2009 Telethon raising over $150,000 for ARM through their efforts since 2006. We truly appreciate their hard work and dedication. The Young ARM of New York Committee has done an amazing job in both spreading awareness, and raising funds for HBV/HBMONLINE research. We applaud the members for their organization skills and hard work for more information you can contact ARM by email.

The Young ARM New York members include the following dedicated individuals:

David Amiran
Michael Aizazi
Ben Hijazi
Mipa Hijazi
Melody Khindardish

Teleton 2009

Major Contributors
Mrs. Mahrokh Eshaghi
Mrs. Mahrokh Eshaghi is the President of ARM in New York and has donated over $150,000 to ARM over the past two years. She has been responsible for many more contributions to ARM and has actively been involved in organizing ARM events in New York.

The Yasuhalfar Family (Millbank Real Estate)
The Yasuhalfar family has been a solid and generous contributor to ARM for many years, and continues to support ARM in every way they can. The location of the 2009 ARM Telethon was donated by the Yasuhalfar family, which resulted in a very successful fundraising event.

ARM Board members extend their warmest gratitude to Mrs. Eshaghi and her loving family, and the Yasuhalfar Family, for their commitment and continued support of ARM. It is with the generosity of active members of our community that ARM is able to continue its fight against HBV.

ARM Supporters / Sponsors Telethon 2009

- The Yasuhalfar Family – Millbank Real Estate
- Mr. Bijan Pakzad – Bijan Menswear Perfumes Jewelry www.bijanbeins.com
- Mr. Bruce Haimi – Cybernet Communication Inc. www.cybernetinc.com
- 90 Medical Supplies (312) 542-6575
- Sharon Giltz Kosher Catering (312) 342-7204
- Mr. Simon P Eshkar – Eshkar Law Firm www.eshkarrlawfirm.com
- Bar Market (312) 655-7070
- Beverly Blossoms (332) 655-7117
- UnitedFabrics International, Inc.

- Video Audio Center www.audiovisualaudiocenter.com
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- Sadaf – www.sadaf.com
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- Omid Travel & Tours (312) 477-2001
- Imperial Party Rentals (323) 931-5566

It is no longer about FINDING the cure; it is about FUNDING the cure.