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Catherine Bowes Rickman Matthew M. LaVail Robert E. Anderson Christian Grimm Joe Hollyfield John Ash *Editors*



Mechanisms and Experimental Therapy



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Retinal Degenerative Diseases

Mechanisms and Experimental Therapy



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Dedication



Holly Jo Whiteside

Holly Whiteside has been an extraordinary RD Symposium Coordinator for 16 years, from RD2000 through the RD2014 meeting. For most of these symposia, she managed all aspects of the meetings, their selection sites. the design and maintenance of the meeting website, all interactions with participants and Travel Awardees, as well as assisting the preparation and submission of the conference grant from the NEI and the proceedings volume. For many, Holly has been the face of the meetings, and she showed remarkable dedication to the meetings and their participants, often giving much of her personal time to be sure the symposia were successful. In so doing, she helped mostly during the period of doubling the size of the biennial meeting. Holly has decided to step down from her involvement with the RD Symposia to devote her time to other aspects of her research and administrative tasks and her personal interests. We will miss her and are honored to dedicate this proceedings volume to her.

Preface

The International Symposia on Retinal Degeneration have been held in conjunction with the biennial meeting of the International Society of Eye Research (ISER) since 1984. These RD Symposia have allowed basic and clinician scientists from around the world to convene and present their new research findings. They have been organized to allow substantial time for discussions and one-on-one interactions in a relaxed atmosphere, where international friendships and collaborations can be fostered

The XVI International Symposium on Retinal Degeneration (also known as RD2014) was held from July 13–18, 2014 at the Asilomar Conference Center in the beautiful city of Pacific Grove, California, USA. The meeting brought together 272 basic and clinician scientists, retinal specialists in ophthalmology, and trainees in the field from all parts of the world. In the course of the meeting, 43 platform and 159 poster presentations were given, and a majority of these are presented in this proceedings volume. New discoveries and state of the art findings from most research areas in the field of retinal degenerations were presented. This was the largest of all of the RD Symposia, with the greatest number of attendees and presentations.

The RD2014 meeting was highlighted by three special keynote lectures. The first was given by **John Flannery**, PhD, of the University of California, Berkeley, Berkeley, CA. Dr. Flannery discussed "Engineering AAV vectors to target specific functional subclasses of retinal neurons and glia." Dr. Flannery's talk was the first named keynote lecture of the RD Symposia in 32 years, the Edward H. Gollob Lecture, named for the President of the Foundation Fighting Blindness. The second keynote lecture was given by **Sally Temple**, PhD, Director of the Neural Stem Cell Institute, Regenerative Research Foundation, Rensselaer, NY. Dr. Temple discussed "Endogenous RPE stem cells, their surprising plasticity and implications for therapeutic applications." The third keynote lecture was given by **Samuel G. Jacobson**, MD, PhD, of the University of Pennsylvania, Philadelphia, PA. Dr. Jacobson discussed "A treatment trial for an inherited retinal degeneration: what have we learned?"

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The scientific meeting ended with a "Welcome to RD2016" by Prof. Nagahisa Yoshimura of Kyoto, Japan, along with the organizers primarily responsible for the meeting, Drs. John Ash and Robert E. Anderson.

We thank the outstanding management and staff of the beautiful Asilomar Conference Center for their assistance in making this an exceptionally smooth-running conference and a truly memorable experience for all of the attendees. These included, in particular, **Suzan Carabarin**, **Vivian Garcia**, **Sammy Ramos** and **Carlene Miller**. We also thank **Kelly Gilford** and **Jason McIntosh** for providing audio/visual equipment and services that resulted in a flawless flow of platform presentations. We thank **Steve Henry** of Associated Hosts, Inc. for planning and implementing transportation of most of the attendees to and from the Asilomar meeting venue, the memorable whale watching excursion, as well as for providing the dynamic "Beach Boys Band" for the end-of-meeting Gala for a truly California experience. Lastly, we thank **Franz Badura** of Pro Retina Germany for serenading the attendees at the Gala with his beautiful trumpet solos.

The Symposium received international financial support from a number of organizations. We are particularly pleased to thank The Foundation Fighting Blindness, Columbia, Maryland, for its continuing support of this and all previous biennial Symposia, without which we could not have held these important meetings. In addition, for the seventh time, the National Eye Institute of the National Institutes of Health contributed in a major way to the meeting. In the past, funds from these two organizations allowed us to provide 25–35 Travel Awards to young investigators and trainees working in the field of retinal degenerations. However, the response to the Travel Awards program was extraordinary, with 110 applicants, many more than in the past. For this reason, we sought additional support for the Travel Awards program. We are extremely appreciative for the contributions from Pro Retina Germany, the Fritz Tobler Foundation Switzerland and from Ed and Sandy Gollob. In total, we were able to fund 49 Travel Awards, the largest number ever an RD Symposia held in North America. We are grateful to the BrightFocus Foundation, which supported the important poster sessions. Many of the contributing foundations sent members of their organizations to attend the meeting. Their participation and comments in the scientific sessions were instructive to many, offering new perspectives to some of the problems being discussed. The Travel Awardees were selected on the basis of 9 independent scores of their submitted abstracts, 6 from each of the organizers and 3 from the other members of the Travel Awards Committee for RD2014, Drs. Jacque Duncan, Machelle Pardue and XianJie Yang.

We also acknowledge the diligent and outstanding efforts of Ms. Holly White-side, who along with Dr. John Ash, carried out most of the administrative aspects of the RD2014 Symposium, and designed and maintained the meeting website. Holly is the Administrative Manager of Dr. Anderson's laboratory at the University of Oklahoma Health Sciences Center. For this Symposium, Ms. Melody Marcum, Director of Development of the Dean McGee Eye Institute, worked closely and extensively in selecting and negotiating the meeting venue, and in planning the meals, entertainment and various events. Melody and Holly were crucial to the success of the RD2014 symposium. Also, Dr. Michael Matthes in Dr. LaVail's laboratory

Preface

played a major role in all aspects in the production of this volume, along with the assistance of Ms. **Cathy Lau-Villacorta**, also in Dr. LaVail's laboratory.

Finally, we honor the monumental efforts of Holly Whiteside. Holly has been the RD Symposium Coordinator since 2000, and during that time she has been the "face" of the RD Symposia. She has been responsible for virtually all of the administrative aspects of the RD Symposia for 16 years, and most repeat attendees feel a close relationship with Holly. She is now stepping back from the efforts of the RD Symposia to pursue personal and professional avenues. We have valued Holly's efforts enormously over these years, and we are proud to dedicate this volume to her.

Catherine Bowes Rickman Matthew M. LaVail Robert E. Anderson Christian Grimm Joe G. Hollyfield John D. Ash

Travel Awards

We gratefully acknowledge National Eye Institute, NIH, USA; the Foundation Fighting Blindness, USA; Pro Retina Germany; the Fritz Tobler Foundation, Switzerland; and Ed and Sandy Gollob for their generous support of 49 Travel Awards to allow young investigators and trainees to attend this meeting. Eligibility was restricted to graduate students, postdoctoral fellows, instructors and assistant professors actively involved in retinal degeneration research. These awards were based on the quality of the abstract submitted by each application. Catherine Bowes Rickman chaired the Travel Awards Committee of 9 senior retinal degeneration investigators, the 6 organizers and Drs. Jacque Duncan, Machelle Pardue and Xian-Jie Yang. The travel awardees are listed below

Carolina Abrahan

University of Florida, Gainesville, USA

Martin-Paul Agbaga

University of Oklahoma HSC, Oklahoma City, USA

Monica Aguila

University College of London, London, United Kingdom

Marcel Alavi

University of California, San Francisco, San Francisco, USA

Seifollah Azadi

University of Oklahoma HSC, Oklahoma City, USA

Emran Bashar

University of British Columbia, Vancouver, Canada

Lea Bennett

Retina Foundation of the Southwest, Dallas, USA

Manas Biswal

University of Florida, College of Medicine, Gainesville, USA

Shannon Boye

University of Florida, Gainesville, USA

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Melissa Calton

Stanford University School of Medicine, San Francisco, USA

Livia Carvalho

Schepens Eye Research Institute/MEEI, Boston, USA

Wei-Chieh Chiang

University of California, San Diego, LaJolla, USA

Rob Collin

Radboud University Medical Centre, Nijmegen, Netherlands

Janise Deming

University of Southern California, Los Angeles, USA

Louise Downs

University of Pennsylvania, Philadelphia, USA

Lindsey Ebke

Cleveland Clinic Cole Eye Institute, Cleveland, USA

Michael Elliott

University of Oklahoma HSC, Oklahoma City, USA

Michael Gale

Oregon Health and Science University, Portland, USA

Xavier Gerard

Institut Imagine, Paris, France

Rosario Fernandez Godino

MEEI-Harvard Medical School, Boston, USA

Christin Hanke

University of Utah, Salt Lake City, USA

Stefanie Hauck

Helmholtz Zentrum Müchen, Neuherberg, Germany

Roni Hazim

University of California, Los Angeles, Los Angeles, USA

Claire Hippert

UCL Institute of Ophthalmology, London, United Kingdom

John Hulleman

Univ. of Texas Southwestern Medical Center, Dallas, USA

Xiaojie Ji

The Jackson Laboratory, Bar Harbor, USA

Mark Kleinman

University of Kentucky, Lexington, USA

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Elod Kortvely

Universität Tübingen, Tübingen, Germany

Ruanne Lai

University of British Columbia, Vancouver, Canada

Christopher Langlo

Medical College of Wisconsin, Milwaukee, USA

Jennifer Lentz

Louisiana State University HSC, New Orleans, USA

Yao Li

Columbia University, New York City, USA

Hongwei Ma

University of Oklahoma HSC, Oklahoma City, USA

Alexander Marneros

Massachusetts General Hospital, Charlestown, USA

Alex McKeown

University of Alabama at Birmingham, Birmingham, USA

Claudia Müller

Fordham University, New York City, USA

Celia Parinot

Institut de la Vision, Paris, France

David Parfitt

UCL Institute of Ophthalmology, London, United Kingdom

Diana Pauly

Universität Regensburg, Regensburg, Germany

Beryl Royer-Bertrand

University of Lausanne, Lausanne, Switzerland

Matt Rutar

The Australian National University, Canberra, Australia

Marijana Samardzija

University of Zurich, Schlieren, Switzerland

Kimberly Toops

University of Wisconsin-Madison, Madison, USA

Christopher Tracy

University of Missouri, School of Medicine, Columbia, USA

Mallika Valapala

Johns Hopkins University School of Medicine, Baltimore, USA

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Lei Wang

Johns Hopkins University, Baltimore, USA

Qingjie Wang

Regenerative Research Foundation, Rensselaer, USA

Wenjun Xiong

Harvard Medical School, Boston, USA

Lei Xu

University of Florida, Gainesville, USA

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