



CLINICAL TRIALS AT THE SUMMIT 2024

JUNE 8, 2024

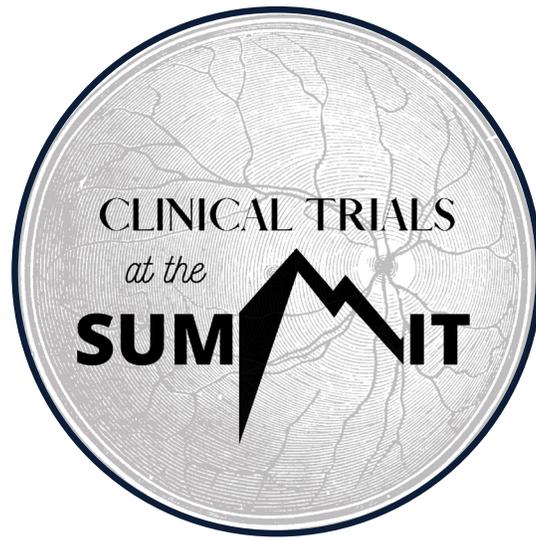
MEETING CO-CHAIRS

Arshad M. Khanani, MD, MA
Jeffrey S. Heier, MD
Peter K. Kaiser, MD

PROGRAM COMMITTEE MEMBERS

David S. Boyer, MD; Diana V. Do, MD; Frank G. Holz, MD;
Anat Loewenstein, MD; Srinivas R. Satta, MD;
Ramin Tadayoni, MD, PhD 

2024



WELCOME TO CLINICAL TRIALS AT THE SUMMIT 2024!

Dear Clinical Trials at the Summit (CTS) Attendees,

Thank you for joining us in Park City! Following the immense success of our past three meetings, we are delighted to host you at our 2024 event.

The inception of *Clinical Trials at the Summit* (CTS) was driven by the aspiration to establish a platform where eminent clinician-scientists and trialists could engage substantively with the key medical leaders of the companies propelling our field forward. The gathering is centered on discussions, aimed at dissecting the multifaceted stages required to guide a product from its conceptualization to commercial success.

We extend a warm welcome and express our gratitude for your participation in CTS 2024. We hope you enjoy the experience!

Sincerely,

Meeting Co-Chairs, on Behalf of the CTS Program Committee



Arshad M. Khanani, MD, MA
Meeting Founder



Jeffrey S. Heier, MD



Peter K. Kaiser, MD

Program Committee Members



David S. Boyer, MD



Diana V. Do, MD



Frank G. Holz, MD



Anat Loewenstein, MD



Srinivas R. Satta, MD



Ramin Tadayoni, MD, PhD

AGENDA

FRIDAY JUNE 7

4:00 – 6:00 PM Registration

6:00 – 9:00 PM **Dinner in Pendry Grand Ballroom**
Sponsored by Apellis Pharmaceuticals

SATURDAY JUNE 8

6:00 AM – 5:45 PM Registration

6:00 – 7:00 AM **Breakfast**

7:00 – 7:05 AM Introduction Jeffrey Heier, Peter Kaiser, Arshad Khanani

7:05 – 7:37 AM **Imaging and Functional Biomarkers for Clinical Trials**
Moderator: Srinivas Sadda
Panelists: Eleonora Lad & Philip Rosenfeld

7:05 – 7:09 AM Dry AMD: Structural and Functional Biomarkers, and the Future of Clinical Trials Eleonora Lad

7:09 – 7:13 AM MACUSTAR Study: Clinical Endpoints and Study Design for Intermediate AMD Trials Frank Holz

7:13 – 7:17 AM Consensus Clinical Trial Design for the Treatment of Intermediate AMD Philip Rosenfeld

7:17 – 7:21 AM Diagnostic Approaches to Geographic Atrophy in 2024 Srinivas Sadda

7:21 – 7:25 AM Mapping Localized Functional Impairment Using Patient-Tailored Fundus-Controlled Perimetry Steffen Schmitz-Valckenberg

7:25 – 7:29 AM Addressing the challenges of the ETDRS chart with Bayesian Active Learning Platform for Visual Function Endpoints Karl Csaky

7:29 – 7:35 AM Discussion

7:35 – 7:37 AM Transition

7:37 – 8:14 AM **Clinical Trials Addressing DR, DME and UME**
Moderator: Daniel Martin
Panelists: Courtney Crawford & Raj Maturi

7:37 – 7:41 AM Update on OCS-01: A Novel Topical Drop for DME in Phase 3 Trials Courtney Crawford

7:41 – 7:45 AM Clinical Update on Oral APX3330 for Diabetic Retinopathy Veeral Sheth

7:45 – 7:49 AM Current and Planned Trials in the DRCR Retina Network Daniel Martin

7:49 – 7:53 AM Bispecific Anti-VEGF Anti-IL-6 Programs: KSI-501 in Retinal Vascular Disease and KSI-101 in Macular Edema Secondary to Inflammation Quan Nguyen

7:53 – 7:57 AM Suprachoroidal Delivery of Investigational ABBV-RGX-314 Gene Therapy for DR: The Phase 2 ALTITUDE Study Raj Maturi

7:57 – 8:01 AM Suprachoroidal Drug Delivery in the Real World Glenn Yiu

8:01 – 8:05 AM Outcomes of the HORNBILL Study: Investigating BI 754524 in Patients with Diabetic Macular Ischemia Quan Nguyen

8:05 – 8:12 AM Discussion

8:12 – 8:14 AM Transition

8:14 – 8:34 AM	DR and DME: Past, Present and the Future <i>Moderator: Arshad Khanani</i> <i>Panelists: Margaret Chang, Dhaval Desai, Kevin Douglas, Jason Ehrlich, George Magrath, Carlos Quezada-Ruiz, Eric Quinones, Riad Sherif</i>	
8:34 – 8:36 AM	Transition	
8:36 – 8:56 AM	Steroids for Retinal Diseases: When and How to Use them? <i>Moderator: Baruch Kuppermann</i> <i>Panelists: David Almeida, Sophie Bakri, Mark Barakat, Chirag Jhaveri, Anat Loewenstein, Michael Singer, Paulo Stanga</i>	
8:56 – 8:58 AM	Transition	
8:58 – 9:03 AM	Sponsor Talk: AbbVie	
9:03 – 9:13 AM	Break	
9:13 – 9:45 AM	Clinical Trials Addressing Dry AMD #1 <i>Moderator: Frank Holz</i> <i>Panelists: Dilsher Dhoot & Margaret Chang</i>	
9:13 – 9:17 AM	Efficacy of Avacincaptad Pegol in the GATHER Clinical Trial Program	Margaret Chang
9:17 – 9:21 AM	Safety of Avacincaptad Pegol in the GATHER Clinical Trial Program	Erin Henry
9:21 – 9:25 AM	Long Term Efficacy and Safety of Pegcetacoplan: GALE 36 Months Results	Frank Holz
9:25 – 9:29 AM	Analysis of Functional Parameters in Pegcetacoplan Treated Eyes	Dilsher Dhoot
9:29 – 9:33 AM	Designing GA Studies of the Future	Sharon Bakalash
9:33 – 9:37 AM	Study Endpoints and Patient Impact	Brian Guthrie
9:37 – 9:43 AM	Discussion	
9:43 – 9:45 AM	Transition	
9:45 – 9:55 AM	Sponsor Talk: Astellas	
9:55 – 10:27 AM	Clinical Trials Addressing Dry AMD #2 <i>Moderator: Glenn Jaffe</i> <i>Panelists: David Boyer & Steffen Schmitz-Valckenberg</i>	
9:55 – 9:59 AM	Prevention of Visual Acuity Loss and Preservation of Photoreceptors by ANX007 in ARCHER Phase 2 Trial	Karl Csaky
9:59 – 10:03 AM	Regional Effects of ANX007 on RPE and Photoreceptor Loss and Relationship to Visual Acuity in ARCHER Phase 2 Trial	Glenn Jaffe
10:03 – 10:07 AM	The Role of Mitochondria in the Treatment of Dry Macular Degeneration	David Boyer
10:07 – 10:11 AM	Glyco-immune Modulation of Macrophage Activity to Treat Geographic Atrophy Associated with AMD	Tarek Hassan
10:11 – 10:15 AM	Effect of AVD-104 on Visual Acuity and GA Lesions in the SIGLEC Trial	Arshad Khanani
10:15 – 10:19 AM	Suprachoroidal Delivery of KRIYA-825, A Gene Therapy Expressing a CR2-CR1 Fusion Protein for the Treatment of Geographic Atrophy	Diana Do
10:19 – 10:25 AM	Discussion	
10:25 – 10:27 AM	Transition	

AGENDA

10:27 - 10:47 AM	Geographic Atrophy: Current Treatments and Future Opportunities <i>Moderator: Jeffrey Heier</i> <i>Panelists: Christopher Brittain, David Callanan, Doug Love, Reenie McCarthy, Shankar Ramaswamy, Philip Rosenfeld, Carolyn Sasse</i>	
10:47 - 10:49 AM	Transition	
10:49 - 11:26 AM	Clinical Trials Utilizing Surgical Delivery, Devices and Home OCT <i>Moderator: Christina Weng</i> <i>Panelists: Thomas Aaberg, Jr. & Veeral Sheth</i>	
10:49 - 10:53 AM	Sub-retinal Delivery of Investigational ABBV-RGX-314 Gene Therapy for nAMD: A Phase 2 Pharmacodynamic Study	Chirag Jhaveri
10:53 - 10:57 AM	Update on Port Delivery Platform for Retinal Diseases	Carlos Quezada-Ruiz
10:57 - 11:01 AM	Update on EXG-102: A Multimechanistic Gene Therapy	David Almeida
11:01 - 11:05 AM	KH631 Gene Therapy for wAMD	Jeffrey Heier
11:05 - 11:09 AM	Efficacy, Safety and Durability of Revakinagene Taroretcel for Macular Telangiectasia	Thomas Aaberg, Jr.
11:09 - 11:13 AM	Role of Home OCT in Reducing Office Visit Burden	Jeffrey Heier
11:13 - 11:17 AM	Home OCT and DRCR Retina Network Protocol AO	Christina Weng
11:17 - 11:24 AM	Discussion	
11:24 - 11:26 AM	Transition	
11:26 - 11:36 AM	Prof. R. Tadayoni "Summit of Excellence" Award - Physician <i>Presented by Peter Kaiser & Arshad Khanani</i>	
11:36 - 11:45 AM	Transition	
11:45 AM - 12:30 PM	Lunch Symposium Cases and Conversations: Approaching Patient Discussions and Integrating a GA Treatment into Clinical Practice <i>Lunch Sponsored by Astellas</i>	
12:30 - 12:40 PM	Transition	
12:40 - 1:00 PM	Retina Innovation 2024 and Beyond: An Investor View <i>Moderator: Ram Palanki</i> <i>Panelists: Matthew Caufield, Emmett Cunningham, Jr., Anupam Dalal, Aaron Osborne, Ronil Patel, Kim Smith, Gena Wang</i>	
1:00 - 1:02 PM	Transition	
1:02 - 1:39 PM	Clinical Trials Addressing nAMD <i>Moderator: Anat Loewenstein</i> <i>Panelists: Robert Avery & Lejla Vajzovic</i>	
1:02 - 1:06 PM	Implications for Targeting VEGF-C/D: Potential for Vision Gain Beyond VEGF-A Inhibition	Megan Baldwin
1:06 - 1:10 PM	Superior Functional Outcomes Beyond Standard of Care: Update on Sozinibercept Clinical Trials	Julie Clark
1:10 - 1:14 PM	Update on KSI Programs for Retinal Diseases	Victor Perlroth
1:14 - 1:18 PM	Clinical Update on Axpaxli for nAMD	Anat Loewenstein
1:18 - 1:22 PM	EYP1901 for nAMD: Results from DAVIO2 Trial	Carl Regillo
1:22 - 1:26 PM	Update on CLS-AX for nAMD	Lejla Vajzovic
1:26 - 1:30 PM	Clinical Update on ONS-501: An Investigational Ophthalmic Formulation of Bevacizumab	Robert Avery

1:30 – 1:37 PM	Discussion	
1:37 – 1:39 PM	Transition	
1:39 – 1:49 PM	Sponsor Talk: Opthea	
1:49 – 2:09 PM	Designing Registration Trials with the Latest FDA Guidance <i>Moderator: Peter Kaiser</i> <i>Panelists: Arshad Khanani, Robert Kim, Eliot Lazar, Jennifer Lim, Raj Maturi, Steve Pakola, Adam Turpcu</i>	
2:09 – 2:11 PM	Transition	
2:11 – 2:16 PM	Sponsor Talk: Ocular Therapeutix	
2:16 – 2:48 PM	Gene Therapy and Optogenetics <i>Moderator: Lejla Vajzovic</i> <i>Panelists: Mark Barakat & Carl Danzig</i>	
2:16 – 2:20 PM	24-week Results from the Randomized Phase 2 Dose Expansion Stage of PRISM Trial Evaluating 4D-150 in High Need nAMD Patients	Carl Danzig
2:20 – 2:24 PM	Ixoberogene Soroparvec IntraVitreAl Gene Therapy for nAMD: Preliminary Results from the Phase 2 LUNA Study	Mark Barakat
2:24 – 2:28 PM	Clinical Update on JNJ-1887 for GA	Paulo Stanga
2:28 – 2:32 PM	AGTC-501 Gene Therapy in XLRP: Clinical Update and Cases	Darin Curtiss
2:32 – 2:36 PM	Efficacy and Safety of MCO-010 Optogenetic Therapy for Retinitis Pigmentosa: Phase 2b RESTORE Study	Peter Kaiser
2:36 – 2:40 PM	Clinical Endpoints for Optogenetic Vision Restoration	Peter Francis
2:40 – 2:46 PM	Discussion	
2:46 – 2:48 PM	Transition	
2:48 – 2:53 PM	Sponsor Talk: Adverum Biotechnologies	
2:53 – 2:58 PM	Sponsor Talk: 4D Molecular Therapeutics	
2:58 – 3:08 PM	Break	
3:08 – 3:40 PM	Molecules with New Mechanism of Action <i>Moderator: Diana Do</i> <i>Panelists: Ramanath Bhandari & Baruch Kuppermann</i>	
3:08 – 3:12 PM	Measures of Clinical Trial Outcome Efficacy - The Importance of Looking Past P-Values	Emmett Cunningham, Jr.
3:12 – 3:16 PM	EB-105: When Three is Not a Crowd - A Novel Trispecific Antibody for Retinal Disease	Charles Semba
3:16 – 3:20 PM	RO-104: A Novel Trispecific Mono-Therapy for Retinal Diseases	Ramanath Bhandari
3:20 – 3:24 PM	A New Potential Option for Uveal Melanoma - The Science of Bel-Sar and Phase 3 CoMpass Trial	Ross Goldstein
3:24 – 3:28 PM	Gildeuretinol Slows Progression of Stargardt Disease: TEASE-3 Study	Baruch Kuppermann
3:28 – 3:32 PM	IL-6 Inhibition with Vamikibart in Patients with UME: DOVETAIL, MEERKAT and SANDCAT Studies	Sophie Bakri
3:32 – 3:38 PM	Discussion	
3:38 – 3:40 PM	Transition	

AGENDA

3:40 – 4:00 PM	Challenges of Bringing New Treatment Options to Market <i>Moderator: Arshad Khanani</i> <i>Panelists: Kelly Giuffrida, Fred Guerard, David Kirn, Keith Lane, Star Seyedkazemi, Russ Trenary, Jonathan Weyne</i>	
4:00 – 4:02 PM	Transition	
4:02 – 4:34 PM	Updates on Faricimab and Aflibercept 8mg <i>Moderator: Patricio Schlottmann</i> <i>Panelists: Jeremiah Brown & Carl Danzig</i>	
4:02 – 4:06 PM	Clinical Benefits of Dual Inhibition of Ang-2 and VEGF-A with Faricimab	Patricio Schlottmann
4:06 – 4:10 PM	Faricimab vs Aflibercept in the Reduction of Exudates in Y/R Trials	Jennifer Lim
4:10 – 4:14 PM	ELEVATUM: A Phase 4 Trial of Faricimab in Underrepresented Patients with DME	Jeremiah Brown
4:14 – 4:18 PM	Faricimab to Vabysmo - Innovative Pipeline to Global Impact	Carol Hoang-Roller
4:18 – 4:22 PM	Population PK Modeling and Simulation of Ocular Clearance for Aflibercept 8mg and Association with Durability	David Eichenbaum
4:22 – 4:26 PM	Aflibercept 8mg: Bridging Pharmacokinetic to Durability of VEGF Suppression	Diana Do
4:26 – 4:32 PM	Discussion	
4:32 – 4:34 PM	Transition	
4:34 – 4:44 PM	Sponsor Talk: Genentech, Inc.	
4:44 – 5:11 PM	First-Time Results of Clinical Trials <i>Moderator: David Boyer</i> <i>Panelists: David Almeida & Glenn Yiu</i>	
4:44 – 4:48 PM	First Time Results from Proof of Concept Phase 2a Study with ANXV in Treatment Naïve Patients with RVO	Michael Singer
4:48 – 4:52 PM	RZ402: Initial results from a Phase 2 study of a novel, orally administered PKal Inhibitor in patients with DME	Rajat Agrawal
4:52 – 4:56 PM	Learnings from the Phase 1 Axpaxli HEALIOS Diabetic Retinopathy Study	Dilsher Dhoot
4:56 – 5:00 PM	Real World Efficacy and Safety of PDS in nAMD: The SUMMIT Study	Arshad Khanani
5:00 – 5:04 PM	Early Real-World Experience with Aflibercept 8mg	David Eichenbaum
5:04 – 5:08 PM	Latest Updates from the TRUCKEE Study	David Almeida
5:08 – 5:12 PM	Real-World Experience with Intravitreal Pegcetacoplan for the Treatment of GA	Philip Rosenfeld
5:12 – 5:16 PM	The CALM Registry: 36 Months Data	Michael Singer
5:16 – 5:24 PM	Discussion	
5:24 – 5:26 PM	Transition	
5:26 – 5:36 PM	“Summit of Excellence” Award - Industry <i>Presented by Jeffrey Heier & Arshad Khanani</i>	
5:36 – 5:45 PM	Video Awards and Closing Remarks	Jeffrey Heier, Peter Kaiser, Arshad Khanani
5:45 – 6:45 PM	Reception <i>Location: Terrace</i>	



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