The Mary Tyler Moore Vision Initiative

(Formerly: The Restoring Vision Moonshot)

JDRF Improving Lives. Curing Type 1 Diabetes. | The Mary Tyler Moore & S. Robert Levine, MD Charitable Foundation | Elizabeth Weiser Caswell Diabetes Institute, University of Michigan

Diabetic Retinal Disease (DRD) Clinical Endpoints Workshop

Elizabeth Weiser Caswell Diabetes Institute (CDI) – University of Michigan
Ann Arbor Marriott Ypsilanti at Eagle Crest
October 25, 2022

AGENDA

0800 **Breakfast | Served in *Pre-function A*** (take food and drink into ***Salon IV*** to enjoy)
Note: dishes will be cleared by hotel staff when we take our break.

0825 **Welcome | *Salon IV***

- **Martin G. Myers, Jr., MD, PhD** | Director, Elizabeth Weiser Caswell Diabetes Institute

0830 **Why are we here? | *Salon IV***

- Setting the stage- why we are here:

  S Robert Levine, MD | Chairman, Mary Tyler Moore Vision Initiative Steering Committee

- Patient perspective on DRD

  Ryan Barunas | Board member, JDRF Northeast Ohio & Michigan Chapter

- Industry Speaker - Unmet need re: Clinical Endpoints (and Therapeutics)

  Dolly Chang, MD, **PhD** | Senior Medical Director, Early Clinical Development, Genentech

- The importance of consortia (rethinking endpoints for T1D)

  Sanjoy Dutta, PhD | Chief Scientific Officer, JDRF

- Questions for the day

  Thomas Gardner, MD, MS | Prof. of Ophthalmology
  Co-Director, JDRF Center of Excellence at the University of Michigan

0910 How Do We Prioritize Endpoints for Development and Validation? | Salon IV

- Challenges in Measuring Visual Function: Standardized Effect Sizes in pre-DR patients, reproducibility/capacity to track changes, and interoperability of structural and functional changes

  Ted Maddess, PhD, FNAI, Eccles, Institute for Neuroscience
  John Curtin School of Medical Research – Australia National University

- Lessons from visual function testing in glaucoma

  Jeffrey Liebmann, MD | Vice Chair Ophthalmology, Columbia University

- Path to developing a PRO in DRD

  Steven Sherman, **MPH** | Director, Health Economics and Outcomes Research, Regeneron Pharmaceuticals

- Regulatory perspective on PRO development

  Malvina Eydelman, MD | Director, Office of Health Technology 1, FDA

- DRD Staging Update: Starting the discussion on priority endpoints

  Jennifer Sun, MD, MPH | Chair, Diabetes Initiatives for the Diabetic Retinopathy Clinical Research (DRCR) Retina Network

1010 **Break and Move to Breakout Group Rooms**

1020 **AM Breakouts: Prioritizing Clinically Useful Endpoints for Development and Validation**

Group 1*:* *Visual function and Retinal Physiology Endpoints* | ***Salon III***
Leader: **Adam Glassman, MS** | Executive Director at the Jaeb Center for Health Research

Group 2*:* *Patient-Reported Outcomes Measures* | ***Salon I***
Leader: **Stela Vujosevic, MD, PhD** | University of Milan, Eye Clinic, IRCCS MultiMedica, Milan, Italy

Group 3: *Systemic, Biochemical, and Cellular Markers* | ***Salon II***
Leader: **Lloyd Paul Aiello, MD, PhD** | Director, Beetham Eye Institute, Joslin Diabetes Center

Group 4*:* *Retinal Imaging Endpoints* | ***Salon V***
Leader: **Jennifer Sun, MD, MPH** | Chief of the Center for Clinical Eye Research, Beetham Eye Institute, Joslin Diabetes Center

Questions for *AM Breakout* Discussion:

1. What are the criteria by which we judge a new endpoint's potential for use in DRD clinical care and research?
2. In each area, what are the priority clinically relevant potential prognostic and predictive endpoints?
    i. What are the clinically relevant endpoints which have potential to become registerable endpoints?
3. How do these endpoints relate to stages of DRD where there is need and opportunity for intervention to preserve, restore, protect vision?
4. What is the patient burden of these endpoints and can/how do they take into consideration the patient voice and priorities?

1120 AM Breakout Reports to Workshop: ***Recommended targets for validation*** (5 minutes per group)

1155 Group Picture *(Please meet in the hallway outside of Pre-function A for directions)*

1200 **Lunch | Served in *Pre-function A*** (take food and drink into ***Salon IV*** to enjoy)
Note: dishes will be cleared by hotel staff when we take our next break

1245 **How Do We Validate Endpoints? | *Salon IV***

- Regulatory perspective on endpoint development

  Wiley Chambers, MD | Director of the Division of Ophthalmology, Center for Drug Evaluation and Research, FDA

  Kerstin Wickström, PhD | Expert, Lyfjastofun, the Icelandic medicines agency

- Lessons from visual function testing in clinical trials & natural history studies in AMD and DR

  Ulrich Luhmann, PhD | Biomarker Experimental Medicine Leader Ophthalmology & Rare Disease, Roche

- Incorporating PROs and passive (patient) sensor data into clinical studies via IoT devices, mobile connectivity, and cloud-based platforms

  Martin Pellinat, MBA | President and Founder, Visiontree Software, Inc.

- Clinical trial considerations in endpoint validation

  Adam Glassman, MS | Executive Director at the Jaeb Center for Health Research

1345 **PM Breakouts: Developing an action plan for endpoint validation and approval**

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Questions for *PM Breakout* Discussion:

1. What are the initial studies proposed to validate the most important endpoints identified in the morning breakout sessions?
    i. What is the general study design
    ii. Who will conduct/sponsor such studies?
    iii. What is the study duration?

2. What are concrete actions we can take to encourage collaboration?

3. Can we develop a consortium of stakeholders in this area and how would it function?
    i. Can we start, as a group, with cross sectional studies in a "pre-competitive space" by numerous stakeholders to develop insights that may be applicable to later longitudinal assessment by individual entities (in a possibly competitive space)?
    ii. Can we identify a "basket" of clinically relevant and potentially registerable endpoints that could be recommended for inclusion in on-going DRD trials or new trials for further assessment, validation, and comparative analysis?

1445 **Break | Food will be served in *Pre-function A***
(Please feel free to take snacks and drinks with you to the airport as well.)

1455 **Action Plans coming out of breakout groups, including next steps on consortium development and first trial design** (5 minutes per group)

1535 **Synthesis and Summation | *Salon IV***

- **Lloyd Paul Aiello, MD, PhD** | Director, Beetham Eye Institute, Joslin Diabetes Center

1550 **Next Steps** (and Expression of Gratitude) | ***Salon IV***

- **S. Robert Levine, MD** | Chairman, MTM Vision Initiative Steering Committee

1600 **Adjourn - *Bus departs to DTW airport from Conference Center Patio Exit***