



2021 APBD Biomarkers Symposium

November 18, 2021, 11 AM – 3 PM ET
Virtual Zoom Meeting

Symposium Description

The 2021 APBD Biomarkers Symposium brings together APBD clinicians and researchers, related disease and technology experts, and industry partners to discuss promising biomarker research. Short presentations will be followed by interactive discussions with all symposium participants. Our goals are to learn about and build consensus around promising biomarker research, and to collectively catalyze the development of biomarkers for APBD and related diseases.

Symposium Agenda

Time (Eastern Time)	Session
11:00 AM – 11:30 AM	<p>Welcome and opening remarks Catherine Fanning, APBD Research Foundation</p> <p>Sharing of the patient and caregiver experience Harriet Saxe, APBD Research Foundation</p> <p>Setting the stage for a discussion of biomarkers Berge Minassian, MD, UT Southwestern</p>
11:30 AM – 12:30 PM	<p>Session 1: Exploring metabolic biomarkers <i>10 min. presentations</i></p> <p>Felix Nitschke, PhD, UT Southwestern <i>CSF α-Glucans and Glycogen Synthase - Potential for biomarker and target engagement in APBD</i></p> <p>Matthew Gentry, PhD, University of Kentucky <i>Utilizing metabolic profiles as biomarkers for glycogen-centric diseases</i></p> <p>Gerald Shulman, MD, PhD, Yale University <i>Application of ^{13}C magnetic resonance spectroscopy to assess liver and skeletal muscle glycogen synthesis and degradation</i></p> <p>Facilitated, interactive discussion</p>
12:30 PM – 12:40 PM	Break
12:40 PM – 1:50 PM	Session 2: Novel approaches to neurological biomarkers

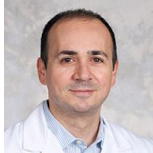
	<p><i>15-20 min. presentations</i></p> <p>Michael Benatar, MD, PhD, University of Miami <i>Biomarkers for ALS therapy development: Relevance to adult polyglucosan body disease?</i></p> <p>Q&A, facilitated discussion</p> <p>Erez Eitan, PhD, NeuroDex <i>Neuronal exosomes - liquid biopsy for brain tissues</i></p> <p>Q&A, facilitated discussion</p>
1:50 PM – 2:00 PM	Break
2:00 PM – 2:50 PM	<p>Session 3: Biorepositories and collaboration <i>15-20 min. presentation</i></p> <p>Tatiana Foroud, PhD, Indiana University <i>Biospecimens, Biomarkers and Biorepositories</i></p> <p>Moving APBD biomarkers and data sharing forward Discussion facilitated by Jesse Cedarbaum, MD, Yale University and Berge Minassian, MD, UT Southwestern</p>
2:50 PM – 3:00 PM	Closing remarks

Recording Disclaimer

This session will be recorded on Zoom for transcription and notetaking purposes only. The recording will not be shared outside of the APBD Research Foundation.

Presenter and Participant Profiles

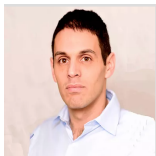
Presenters



Michael Benatar, MD, PhD
Professor of Neurology
Chief, Neuromuscular Division
University of Miami



Jesse M. Cedarbaum, MD
Founder and Head, Coeruleus Clinical Sciences LLC
Chief Medical Officer, Immunobrain Checkpoint
Professor (Adjunct) of Neurology and Psychiatry,
Yale University School of Medicine



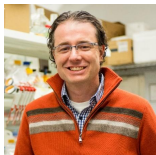
Erez Eitan, PhD
Chief Scientific Officer, Co-founder
NeuroDex



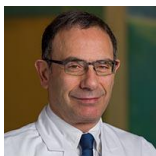
Catherine Fanning
Pharmaceutical Development Committee Chair
APBD Research Foundation



Tatiana Foroud, PhD
Executive Associate Dean for Research Affairs
Department of Medical and Molecular Genetics
Indiana University School of Medicine

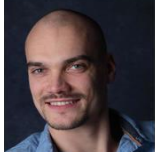


Matthew Gentry, PhD
Professor, Molecular & Cellular Biochemistry
Director, Lafora Epilepsy Cure Initiative
University of Kentucky College of Medicine

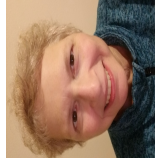


Chief, Division of Child Neurology
UT Southwestern Medical Center

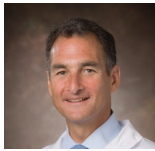
Felix Nitschke, PhD
Assistant Professor
Departments of Pediatrics and Biochemistry
UT Southwestern Medical Center



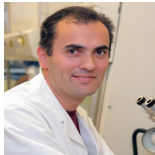
Harriet Saxe
Board Member, Patient Engagement
APBD Research Foundation



Gerald I Shulman, MD, PhD, MACP, MACE, FRCP
Professor of Medicine Cellular and Molecular Physiology
Co-Director, Yale Diabetes Research Center
Yale School of Medicine



Participants



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Columbia University Medical Center



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Project Manager
Reference Center for Leukodystrophies
Pitié-Salpêtrière University Hospital



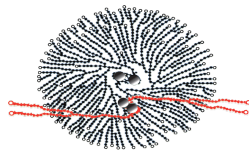
David Brenner, MD
Neurologist
Division of Neurodegeneration, Department of Neurology
Mannheim Center for Translational Neurosciences
Medical Faculty Mannheim, Heidelberg University



Sanjay Chandriani, PhD
Director of Translational Sciences
Maze Therapeutics



Andrew Cunningham, MD, MBA
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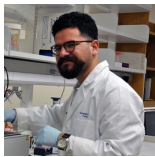
Florian Eichler, MD
Director, Center for Rare Neurological Diseases
Associate Professor, Neurology
Massachusetts General Hospital
Harvard Medical School



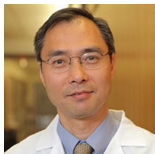
Cheryl Fitzer-Attas, PhD, MBA
Senior Advisor to GHF



Rebecca Gibson
Medical Scientist Training Program
Molecular Genetics and Microbiology
Duke University Medical Center



Emrah Gumusgoz, MD, PhD
Research Associate
UT Southwestern Medical Center



Michio Hirano, MD,
Lucy G. Moses Professor of Neurology
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Director, H. Houston Merritt Neuromuscular Research Center
Columbia University Irving Medical Center
Vagelos College of Physicians and Surgeons



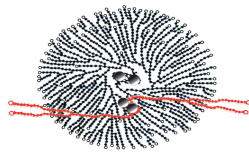
Or Kakhlon, PhD
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Priya Sunil Kishnani, MD, MBBS
C.L. and Su Chen Professor of Pediatrics
Medical Director, YT and Alice Chen Pediatrics Genetics and Genomics Center
Division Chief, Medical Genetics
Professor, Molecular Genetics and Microbiology
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Rebecca Koch, PhD, RDN
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Department of Pediatrics
Duke University Medical Center



APBD
ADULT POLYGLUCOSAN BODY DISEASE
RESEARCH FOUNDATION



Holly Kordasiewicz, PhD
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Ionis Pharmaceuticals, Inc.



Roger Lane, MD, MPH
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Department of Neurology
Hadassah-Hebrew University Medical Center



Jennifer L Orthmann-Murphy, MD, PhD
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Chanchal Sadhu
Vice President, Translational Sciences
Taysha Gene Therapies



Terrence Satterfield, PhD
Biomarker Discovery Scientist
Maze Therapeutics



Shlomi Schneider
Founder
GHF



Rob Shaffer, PhD
Founder/CEO
Enable Therapeutics



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Senior Scientist & Project Team Leader
Maze Therapeutics



Mayank Verma, MD, PhD
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Sarah Phyllis Young, PhD
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Wyatt Yue, PhD
Professor of Structural Biology
Newcastle University Medical School

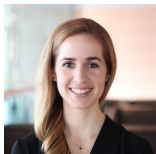
Coordination and Technical Support



Caliopy Glaros
Facilitator & Consultant
Philanthropy Without Borders



Gabriella Tost
Tech Support
Philanthropy Without Borders



Erin Chown, MSc
Research Coordinator
APBD Research Foundation

Participant Roles and Expectations

Presenters	Participants	Tech Support
<ul style="list-style-type: none"> ● Present materials ● Utilize timers to track allotted time ● Share slide deck ● Answer questions as applicable ● Turn video on/off as needed ● Mute and unmute as needed 	<ul style="list-style-type: none"> ● Turn video on/off as needed ● Participate in the discussion ● Answer questions as applicable 	<ul style="list-style-type: none"> ● Answer tech questions as applicable ● Assist with audio and video issues ● Share slides in the event that the presenter cannot ● Mute and unmute participants as needed

Presenting Virtually: Best Practices

- Try to connect via a wired Ethernet jack, if possible. If on wifi, confirm connection is strong enough to stay connected.
- If connecting from a laptop, plug in the laptop to wall power. Battery use can adversely affect video quality.
- Be aware of your surroundings and how you appear visually. Have a look at the items behind you and check if you can see clutter or distracters behind you in the video image.
- Try to join from a quiet location with minimal background noise; use headphones or headset when possible.
- Have the lighting come from in front of you or from the side, not the back.
- Utilize verbal prompts and cues to keep yourself focused and others in tune.

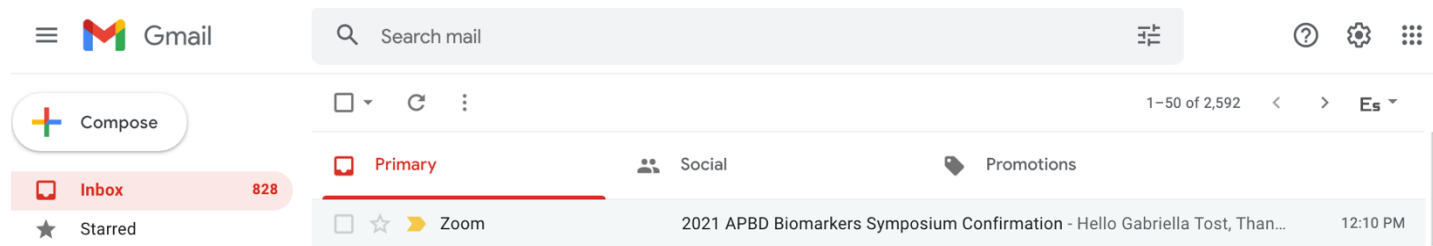
Zoom Instructions

Meeting link: <https://us02web.zoom.us/j/88926740921>

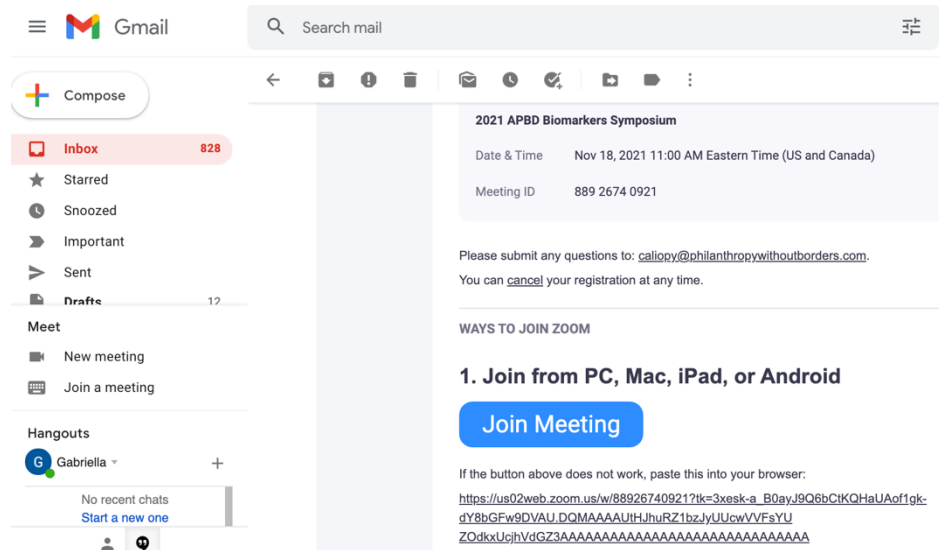
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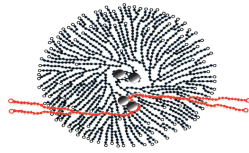
STEP #1: Confirm You Received the Meeting Link

To locate that link, open your email account and look for an email from “Zoom” entitled “2021 APBD Biomarkers Symposium Confirmation.” It should look like this:



Open the email and click “Click Here to Join”





STEP #2: Download Zoom and Test Your Device Before the Meeting

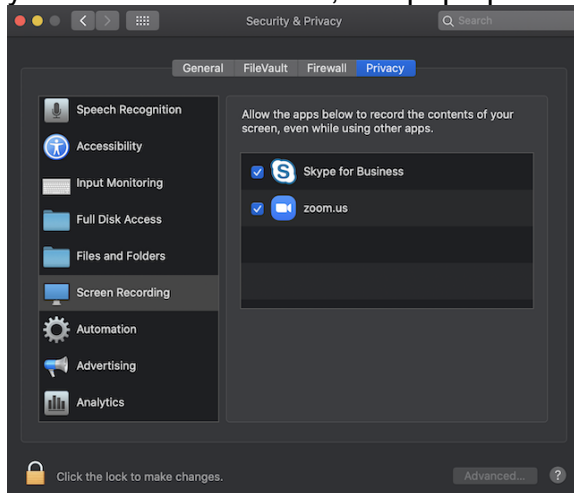
Please review the following helpful links:

Join a Test Meeting: <https://zoom.us/test>

Updating your Zoom: <https://support.zoom.us/hc/en-us/articles/201362233>

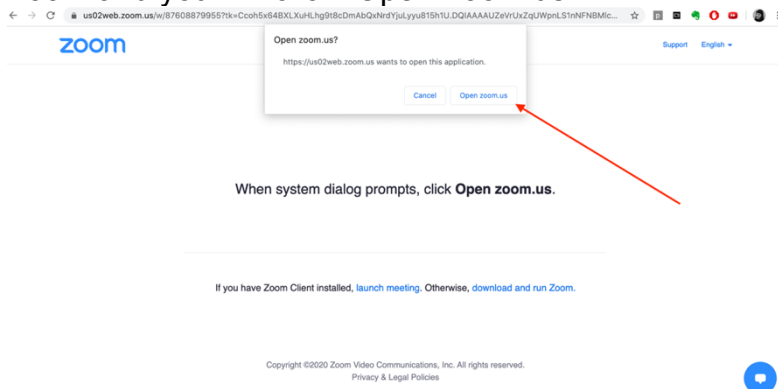
Testing your audio and video: <https://support.zoom.us/hc/en-us/articles/201362283-How-Do-I-Join-or-Test-My-Computer-Audio->

Please also test your ability to share your screen. Depending on your version of Zoom, browser, and privacy settings, you may need to grant Zoom access to share or record your screen. On a Mac, this pop up window looks like this:



STEP #3: Join the Meeting

When you click the meeting link in your email, a new window will open that will launch Zoom and you will click “Open Zoom.us”





STEP #4: Contact Meeting Tech Support if you need help

If you still experience trouble with opening and using Zoom, contact:

Gabriella Tost

Email: gabriellatost@gmail.com

Phone: +1-503-853-8375

WhatsApp: +593-999-939-389

Time zone: Central (CDT)